UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

FORM 10-K

(Mark One)

☒ **ANNUAL REPORT UNDER SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended September 30, 2025

☐ **TRANSITION REPORT UNDER SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from to

Commission file number 001-38042

ARROWHEAD PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**46-0408024**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**

(626) 304-3400
177 E. Colorado Blvd, Suite 700
Pasadena, California 91105
(Address and telephone number of principal executive offices)

Securities registered pursuant to Section 12(b) of the Exchange Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.001 par value	**ARWR**	**The Nasdaq Global Select Market**

Securities registered pursuant to Section 12(g) of the Exchange Act: None

Indicate by a check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☐ No ☒

Indicate by a check mark if the registrant is not required to file reports pursuant to Section 13 or 15(d) of the Act. Yes ☐ No ☒

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer ☒	Accelerated filer ☐	Non-accelerated filer ☐	Smaller reporting company ☐	Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☒

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act). Yes ☐ No ☒

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to §240.10D-1(b). ☐

The aggregate market value of issuer's voting and non-voting outstanding common stock held by non-affiliates was approximately $1.4 billion based upon the closing stock price of issuer's common stock on March 31, 2025. Shares of common stock held by each officer and director and by each person who is known to own 10% or more of the outstanding common stock have been excluded in that such persons may be deemed to be affiliates of the Company. This determination of affiliate status is not necessarily a conclusive determination for other purposes.

As of November 19, 2025, 135,809,558 shares of the issuer's Common Stock were issued and outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the Definitive Proxy Statement to be filed for Arrowhead Pharmaceuticals, Inc.'s 2026 Annual Meeting of Stockholders are incorporated by reference into Part III hereof.

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FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains certain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, and we intend that such forward-looking statements be subject to the safe harbors created thereby. For this purpose, any statements contained in this Annual Report on Form 10-K except for historical information may be deemed to be forward-looking statements. Without limiting the generality of the foregoing, words such as "may," "might," "will," "expect," "believe," "anticipate," "goal," "endeavor," "strive," "intend," "plan," "project," "could," "estimate," "target," "might," "forecast," or "continue" or the negative of these words or other variations thereof or comparable terminology are intended to identify forward-looking statements. In addition, any statements that refer to projections of our future financial performance, trends in our business, or other characterizations of future events or circumstances are forward-looking statements. These forward-looking statements include, but are not limited to, statements about the initiation, timing, progress and results of our preclinical studies and clinical trials, and our research and development programs; our expectations regarding our commercialization efforts for REDEMPLO; our expectations regarding the potential benefits of the partnership, licensing and/or collaboration arrangements and other strategic arrangements and transactions we have entered into or may enter into in the future; our beliefs and expectations regarding the amount and timing of future milestone, royalty or other payments that could be due to or from third parties under existing agreements; and our estimates regarding future revenues, research and development expenses, capital requirements and payments to third parties.

The forward-looking statements included herein are based on current expectations of our management based on available information and involve a number of risks and uncertainties, all of which are difficult or impossible to predict accurately, and many of which are beyond our control. As such, our actual results and outcomes may differ materially from those discussed, projected, anticipated or indicated in any forward-looking statements. Forward-looking statements are not guarantees of future performance and our actual results or outcomes may differ materially. Factors that may cause or contribute to such differences include, but are not limited to, those discussed in more detail in "Item 1. Business" and "Item 1A. Risk Factors" of Part I and "Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations" of Part II of this Annual Report on Form 10-K. Readers should carefully review these risks, as well as the additional risks described in other documents we file from time to time with the Securities and Exchange Commission (the "SEC"). In light of the significant risks and uncertainties inherent in the forward-looking information included herein, the inclusion of such information should not be regarded as a representation by us or any other person that such results will be achieved, and readers are cautioned not to place undue reliance on such forward-looking information. Statements made herein are as of the date of the filing of this Annual Report on Form 10-K with the SEC and should not be relied upon as of any subsequent date. Except as may be required by law, we disclaim any intent to revise the forward-looking statements contained herein to reflect events or circumstances after the date hereof or to reflect the occurrence of unanticipated events.

PART I

Unless otherwise noted, (1) the term "Arrowhead" refers to Arrowhead Pharmaceuticals, Inc., a Delaware corporation and its Subsidiaries, (2) the terms "Company," "we," "us," and "our," refer to the ongoing business operations of Arrowhead and its Subsidiaries, whether conducted through Arrowhead or a subsidiary of Arrowhead, (3) the term "Subsidiaries" refers to Arrowhead Madison Inc. ("Arrowhead Madison"), Arrowhead Australia Pty Ltd ("Arrowhead Australia"), Arrowhead Pharmaceuticals NZ Limited ("Arrowhead New Zealand"), Arrowhead Pharmaceuticals Ireland Limited ("Arrowhead Ireland"), and Visirna Therapeutics Inc. ("Visirna") (4) the term "common stock" refers to Arrowhead's common stock, (5) the term "preferred stock" refers to Arrowhead's preferred stock and (6) the term "stockholder(s)" refers to the holders of Arrowhead common stock.

ITEM 1. BUSINESS

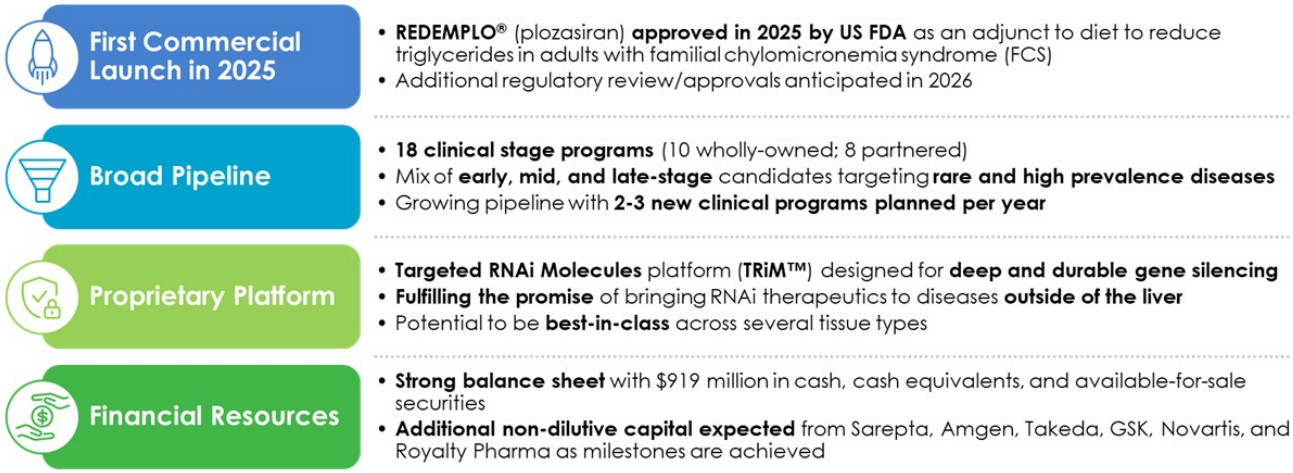
A. Overview

The Company develops medicines that treat intractable diseases by silencing the genes that cause them. Using a broad portfolio of RNA chemistries and modes of delivery, the Company's therapies trigger the RNA interference mechanism to induce rapid, deep and durable knockdown of target genes.

There are currently 18 Arrowhead discovered drug candidates in clinical trials ranging from early stage (Phase 1) to late stage (Phase 3). In addition, the company has a robust discovery stage pipeline which is capable of generating multiple new clinical candidates each year.

The Company recently achieved a transformational milestone with its first commercial launch in 2025, when the U.S. Food and Drug Administration ("FDA") approved REDEMPLO® (plozasiran) as an adjunct to diet to reduce triglycerides in adults with Familial Chylomicronemia Syndrome ("FCS"). Additionally, phase 3 studies (SHASTA-3, SHASTA-4 and SHASTA-5) for severe hypertriglyceridemia ("sHTG") have been fully enrolled and the Company plans to file a supplemental NDA for this indication in 2026, pending successful completion of Phase 3 clinical studies. The Company has built a commercial organization to support marketing in FCS, a rare disease, and plans to progressively build its commercial capabilities to also support marketing in sHTG, a higher prevalence disease which will require a larger commercial footprint.

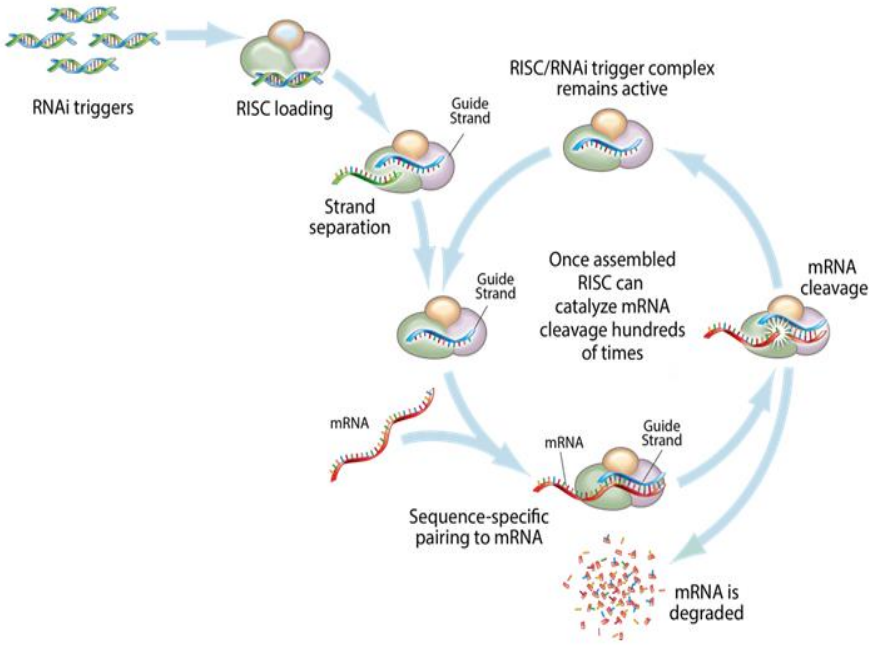
The Company has entered into multiple license and collaboration agreements with leading biotech and pharmaceutical companies, including Sarepta Therapeutics, Inc., Amgen Inc., Takeda Pharmaceutical Company Limited, Glaxosmithkline Intellectual Property (No. 3) Limited and Novartis Pharma AG, for programs that the Company does not intend to commercialize independently. This approach aims to expand the reach of the Company's technology and provides a source of non-dilutive capital to support REDEMPLO and other wholly-owned programs through commercial stage.



RNA Interference and the Benefits of RNAi Therapeutics

RNA interference ("RNAi") is a mechanism present in living cells that inhibits the expression of a specific gene, thereby affecting the production of a specific protein. RNAi-based therapeutics may leverage this natural pathway of gene silencing to target and shut down specific disease-causing genes.

Small molecule and antibody drugs have proven effective at inhibiting certain cell surface, intracellular, and extracellular targets. However, other drug targets have proven difficult to inhibit with traditional drug-based and biologic therapeutics. Developing effective drugs for these targets would have the potential to address large underserved markets for the treatment of many diseases. Using the ability to specifically silence any gene, RNAi therapeutics may be able to address previously "undruggable" targets, unlocking the market potential of such targets.



This figure depicts the mechanism by which gene silencing occurs. Double stranded RNAi triggers (commonly referred to as small interfering RNAs ("siRNAs")) are introduced into a cell and are loaded into the RNA-induced silencing complex ("RISC"). The strands are then separated, leaving an active RISC/RNAi trigger complex. This complex can then pair with and degrade the complementary messenger RNAs ("mRNA") and stop the production of the target proteins. RNAi is a catalytic process, so each RNAi trigger can degrade mRNA hundreds of times, which results in a relatively long duration of effect for RNAi therapeutics.

Key Benefits of RNAi and proprietary TRiM™ platform as a Therapeutic Modality:

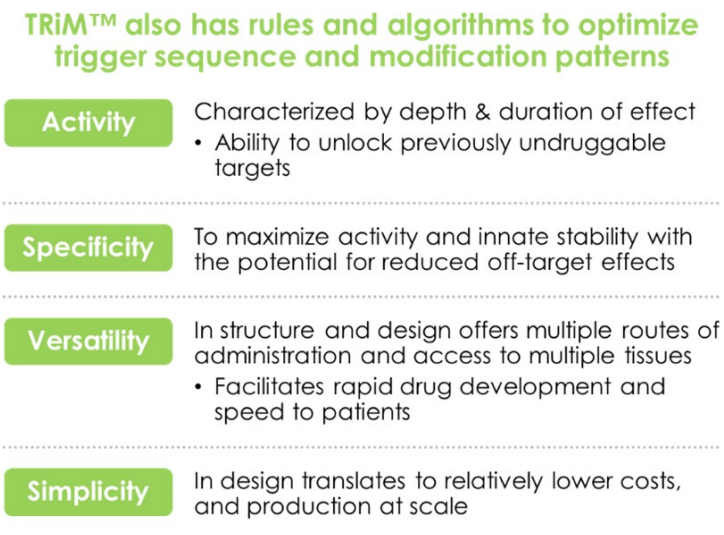
- Silences the expression of disease associated genes;
- Potential to address any target in the transcriptome including previously "undruggable" targets;
- Rapid lead identification;
- High specificity;
- Opportunity to use multiple RNA sequences in one drug product for synergistic silencing of multiple targets; and
- RNAi therapeutics are uniquely suited for personalized medicine through target and cell specific delivery and gene knockdown.

Targeted RNAi Molecule (TRiM™) Platform

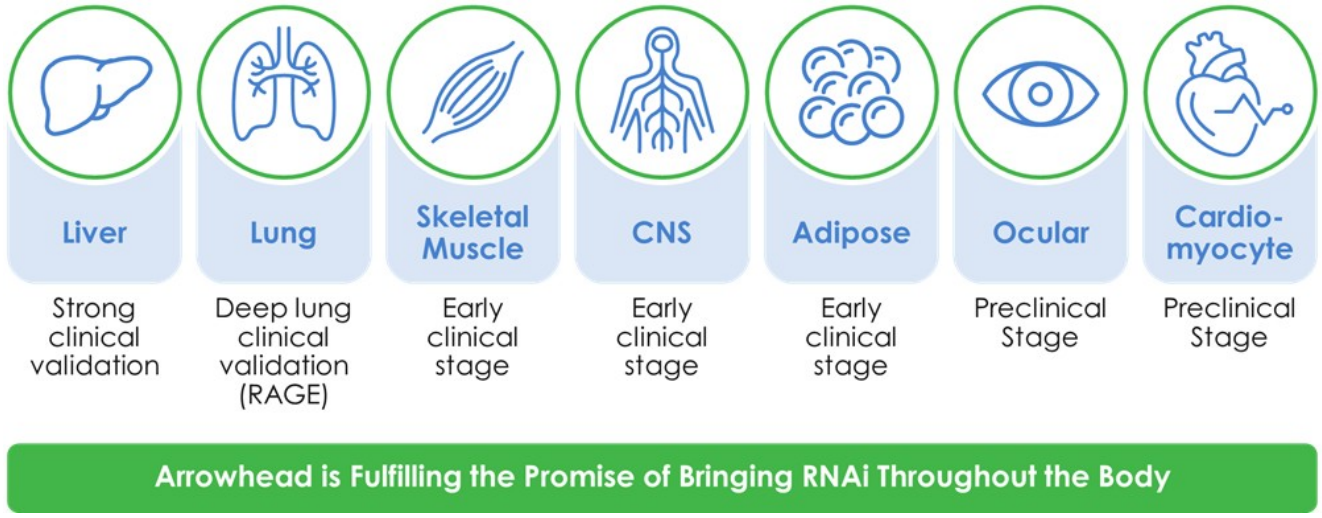
The Company's TRiM platform utilizes ligand-mediated delivery and is designed to enable tissue-specific targeting while being structurally simple. Targeting has been core to the Company's development philosophy and the TRiM platform builds on more than a decade of work on actively targeted drug delivery vehicles. The Company's scientists have discovered ways to progressively "TRiM" away extraneous features and chemistries and retain optimal pharmacologic activity.

The TRiM platform is comprised of a highly potent RNA trigger identified using the Company's proprietary trigger selection rules and algorithms with the following components optimized, as needed, for each drug candidate: a high affinity targeting ligand; various linker chemistries; structures that enhance pharmacokinetics; and highly potent RNAi triggers with sequence specific stabilization chemistries.

Therapeutics developed with the TRiM platform offer several advantages: simplified manufacturing and reduced costs; multiple routes of administration; and potential for improved safety because there are less metabolites from smaller molecules, thereby reducing the risk of intracellular buildup.



The Company believes that for RNAi to reach its true potential, it must target organs outside the liver. The Company is leading this expansion with the TRiM platform, which has shown the potential to reach multiple tissues throughout the body. The TRiM platform currently enables delivery of siRNA to seven cell types:



RNA Chemistries

The structure and chemistries of the oligonucleotide molecules used to trigger the RNAi mechanism can be tailored for optimal activity. The Company's broad portfolio of RNA trigger structures and chemistries, including certain proprietary structures, enable the Company to optimize each drug candidate on a target-by-target basis and utilize the combination of structure and chemical modifications that yield the most potent RNAi therapeutic candidate.

As a component of the TRiM platform, the Company's design philosophy for RNA chemical modifications is to start with a structurally simple molecule and add only selective modification and stabilization chemistries as necessary to achieve the desired level of target knockdown and duration of effect. The conceptual framework for the stabilization strategy starts with a more sophisticated RNAi trigger screening and selection process that identifies potent sequences rapidly in locations that others may miss.

Approved Products

REDEMPLO (plozasiran) is approved by the U.S. Food and Drug Administration as an adjunct to diet to reduce triglycerides for adults with Familial Chylomicronemia Syndrome (FCS). REDEMPLO is an siRNA therapeutic designed to suppress the production of apolipoprotein C-III (APOC3), a protein produced in the liver that raises triglyceride levels by slowing their breakdown and clearance. By targeting the *APOC3* gene with sustained silencing, REDEMPLO delivers significant reductions in triglyceride levels. REDEMPLO is the first and only FDA-approved siRNA treatment studied in both genetically confirmed and clinically diagnosed patients living with FCS.

For more information about REDEMPLO, visit Our Medicines.

IMPORTANT SAFETY INFORMATION

CONTRAINDICATIONS

None.

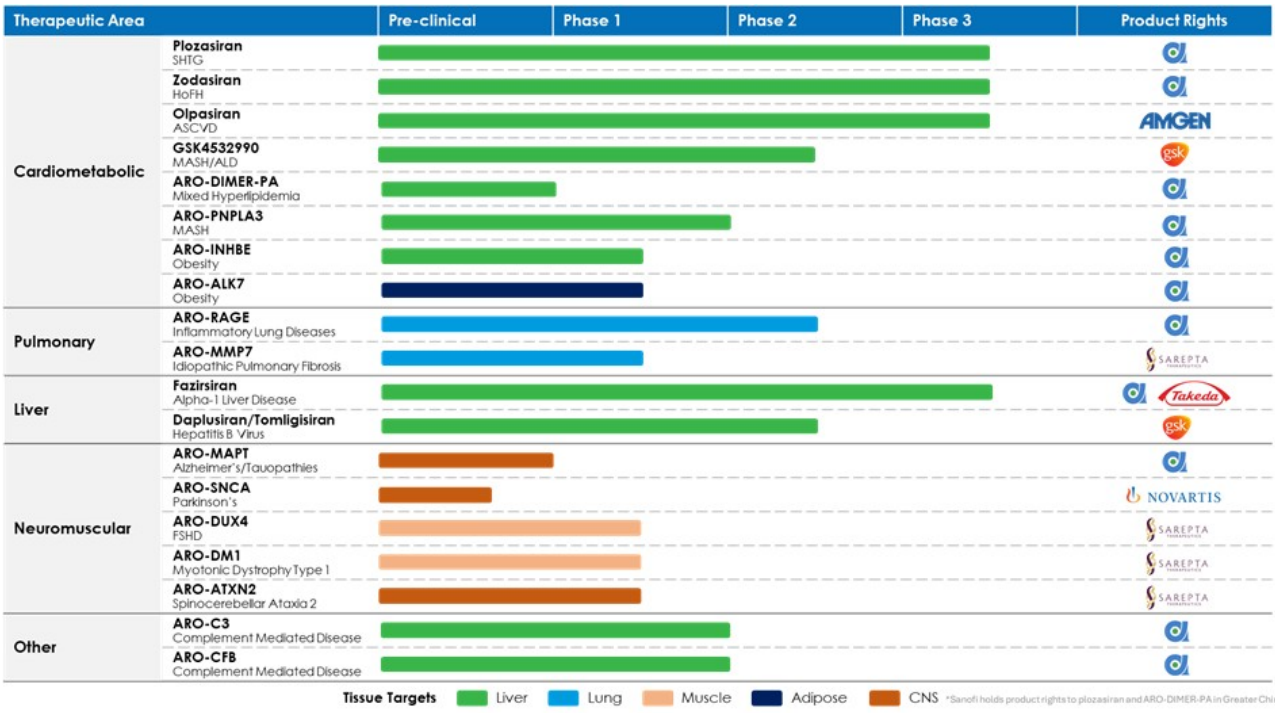
ADVERSE REACTIONS

Most common adverse reactions in REDEMPLO treated patients (incidence ≥10% of patients treated with REDEMPLO and >5% more frequently than with placebo) are hyperglycemia, headache, nausea, and injection site reaction.

Please see full Prescribing Information for REDEMPLO.

B. Pipeline

The Company is focused on developing innovative drugs for diseases with a genetic basis, typically characterized by the overproduction of one or more proteins that are involved with disease. The depth and versatility of the Company's RNAi technologies enables the Company to potentially address conditions across many therapeutic areas and pursue disease targets that are not otherwise addressable by small molecules and biologics.



(1) Greater China rights for plozasiran are out-licensed to Sanofi.

Plozasiran (ARO-APOC3) - Severe Hypertriglyceridemia (sHTG)

Severe Hypertriglyceridemia is a disease characterized by elevated triglyceride levels > 500 mg/dL. Severely elevated triglycerides in patients with severe hypertriglyceridemia (sHTG) or familial chylomicronemia syndrome (FCS), a rare genetic disorder, can result in potentially fatal acute pancreatitis.

Plozasiran (formerly ARO-APOC3) is designed to reduce production of apolipoprotein C-III (APOC3), a component of triglyceride rich lipoproteins (TRLs) including Very Low Density Lipoprotein (VLDL) and chylomicrons, a key regulator of triglyceride metabolism. The Company believes that knocking down the hepatic production of APOC3 may result in reduced VLDL synthesis and assembly, enhanced breakdown of TRLs, and better clearance of VLDL and chylomicron remnants.

We are conducting multiple Phase 3 studies to potentially support regulatory filings, pending successful completion, for approval in sHTG including SHASTA-3, SHASTA-4, SHASTA-5, and MUIR-3.

Zodasiran (ARO-ANG3) - Homozygous Familial Hypercholesterolemia (HoFH)

Dyslipidemia and Hypertriglyceridemia are risk factors for atherosclerotic coronary heart disease and cardiovascular events.

Zodasiran (formerly ARO-ANG3) is designed to reduce production of angiopoietin-like protein 3 (ANGPTL3), a liver synthesized inhibitor of lipoprotein lipase and endothelial lipase. ANGPTL3 inhibition has been shown to lower serum LDL, serum and liver triglyceride and has genetic validation as a novel target for cardiovascular disease. The Company is currently investigating zodasiran in one Phase 3 clinical trial (YOSEMITE) to evaluate the efficacy and safety of zodasiran in adolescent and adult subjects with HoFH.

ARO-DIMER-PA - Mixed Hyperlipidemia

Mixed Hyperlipidemia is a highly prevalent disorder characterized by elevated low-density lipoprotein cholesterol (LDL-C) and triglyceride (TG) levels and is a major risk factor for ASCVD, which is the leading cause of mortality worldwide and associated with substantial morbidity and healthcare costs.

ARO-DIMER-PA is a dual functional RNAi molecule designed to silence expression of the proprotein convertase subtilisin kexin 9 (PCSK9) and apolipoprotein C3 (APOC3) genes in hepatocytes. Prior clinical experience with other investigational and approved agents suggests that PCSK9 and APOC3 inhibition may lead to robust reductions in LDL-C, TGs, triglyceride rich lipoprotein remnants, and total atherogenic lipoproteins. This represents an important step forward for the RNAi field as it is the first clinical candidate to target two genes simultaneously in one molecule, enabled by Arrowhead's innovative and proprietary TRiM platform.

The Company has filed for regulatory clearance to initiate a Phase 1/2a clinical trial of ARO-DIMER-PA.

ARO-PNPLA3 - Metabolic-dysfunction Associated Steatohepatitis (MASH)

MASH is a subgroup of steatotic liver disease (MASLD) in which hepatic cell injury and inflammation has developed over background steatosis. The I148M genetic variant in the PNPLA3 gene is involved with the underlying pathophysiology and is a known risk factor for hepatic steatosis, steatohepatitis, elevated plasma liver enzyme levels, hepatic fibrosis and cirrhosis. The rising prevalence of MASH presents a significant health burden in many developed countries.

ARO-PNPLA3 (formerly JNJ-75220795) is an investigational RNAi therapeutic designed to reduce liver expression of patatin-like phospholipase domain containing 3 (PNPLA3) as a potential treatment for patients with metabolic-dysfunction associated steatohepatitis (MASH). PNPLA3 has strong genetic and preclinical validation as a driver of fat accumulation and damage in the livers of patients who carry the common I148M mutation. Former licensee Janssen Pharmaceuticals, Inc. investigated ARO-PNPLA3 in two Phase 1 clinical trials.

ARO-INHBE - Obesity

ARO-INHBE is designed to reduce the hepatic expression of the INHBE gene and its secreted gene product, Activin E. INHBE is a promising genetically validated target in which loss-of-function INHBE variants in humans are associated with lower risk of obesity and metabolic diseases, such as type 2 diabetes. The Company is currently investigating ARO-INHBE in a Phase 1/2a clinical trial.

ARO-ALK7- Obesity

ARO-ALK7 is designed to silence adipocyte expression of the ACVR1C gene to reduce the production of Activin receptor-like kinase 7 (ALK7), which acts as a receptor in a pathway that regulates energy homeostasis in adipose tissue. In large genetic datasets, reduced ACVR1C expression has been associated with healthier adipose distribution and reduced

risk of obesity-related metabolic complications. The Company is currently investigating ARO-ALK7 in a Phase 1/2a clinical trial.

ARO-RAGE - Inflammatory Pulmonary Disease

ARO-RAGE is designed to reduce production of the Receptor for Advanced Glycation End products (RAGE) as a potential treatment for various inflammatory pulmonary diseases. The Company is currently investigating ARO-RAGE in a Phase 1/2a clinical trial.

ARO-MAPT - Alzheimer's and Tauopathies

Aggregation of the toxic tau protein is believed to be a key driver in multiple tauopathies, including Alzheimer's disease. By preventing or potentially reversing tau protein accumulation in subjects with mild cognitive impairment due to Alzheimer's disease and mild Alzheimer's disease dementia, ARO-MAPT has the potential to prevent or slow disease progression.

ARO-MAPT is Arrowhead's first investigational RNAi-based therapy to utilize a new proprietary delivery system which, in preclinical studies, has achieved blood-brain-barrier penetration and deep knockdown of target genes across the central nervous system (CNS), including deep brain regions, after subcutaneous injections. ARO-MAPT is designed to silence CNS expression of the microtubule associated protein tau (MAPT) gene, which encodes the tau protein. The Company has filed for regulatory clearance to initiate a phase 1/2a clinical trial of ARO-MAPT.

ARO-C3 - Complement Mediated Renal Disease

A number of rare renal diseases result from uncontrolled activation of the alternative pathway of complement, leading to progressive glomerular damage, proteinuria, hematuria, and impaired kidney function, and often resulting in end-stage renal disease (ESRD). In addition, dysregulation of the alternative complement pathway has been shown to play a role in the pathogenesis and progression of disease in some of the more common glomerulopathies. Silencing complement component 3 (C3) may be a therapeutic approach for treatment of these conditions.

ARO-C3 is designed to reduce production of C3 as a potential therapy for patients with various complement mediated or complement associated renal diseases. The Company is currently investigating ARO-C3 in a Phase 1/2a clinical trial.

ARO-CFB - Complement Mediated Disease

A number of rare renal diseases result from uncontrolled activation of the alternative pathway of complement, leading to progressive glomerular damage, proteinuria, hematuria, and impaired kidney function, and often resulting in end-stage renal disease (ESRD). In addition, dysregulation of the alternative complement pathway has been shown to play a role in the pathogenesis and progression of disease in some of the more common glomerulopathies. Silencing complement factor B (CFB) may be a therapeutic approach for treatment of these conditions.

ARO-CFB is designed to reduce hepatic expression of CFB, which plays an important regulatory role in amplifying complement alternative pathway activation and has been identified as a promising therapeutic target. ARO-CFB is being developed as a potential treatment for complement mediated kidney diseases such as immunoglobulin A nephropathy (IgAN), which is the most common glomerular disease worldwide and carries a high lifetime risk of progression to end-stage renal disease. Additionally, ARO-CFB may have clinical applications in non-renal diseases involving complement activation. The Company is currently investigating ARO-CFB in a Phase 1/2a clinical trial.

Collaboration and License Agreements

Glaxosmithkline Intellectual Property (No. 3) Limited ("GSK")

GSK-HSD License Agreement

On November 22, 2021, GSK and the Company entered into an Exclusive License Agreement (the "GSK-HSD License Agreement"). Under the GSK-HSD License Agreement, GSK has received an exclusive license for GSK-4532990 (formerly ARO-HSD). The exclusive license is worldwide with the exception of greater China. GSK is wholly responsible for all clinical development and commercialization of GSK-4532990 in its territory.

GSK-4532990 - Metabolic-Dysfunction Associated Steatohepatitis (MASH)

MASH is liver inflammation and damage caused by a buildup of fat in the liver. This can cause scarring of the liver and in advanced cases can lead to cirrhosis. Alcochol-related liver disease (ALD) represents a spectrum of liver injury

resulting from alcohol use, ranging from hepatic steatosis to more advances forms including alcoholic hepatitis (AH), alcohol-associated cirrhosis (AC), and acute AH presenting as acute-on-chronic liver failure.

GSK-4532990 (formerly ARO-HSD) is designed to reduce production of HSD17B13, a hydroxysteroid dehydrogenase involved in the metabolism of hormones, fatty acids and bile acids. Published human genetic data indicate that a loss of function mutation in HSD17B13 provides strong protection against metabolic-dysfunction associated steatohepatitis (MASH) cirrhosis and alcoholic hepatitis and cirrhosis. GSK is conducting Phase 2b clinical trials in patients with MASH and alcohol-related liver disease (ALD).

GSK-HBV Agreement - Chronic Hepatitis B Virus Infection

On December 11, 2023, the Company entered into an Amended and Restated License Agreement with GSK (the "GSK-HBV Agreement") pursuant to which GSK received a worldwide, exclusive license to develop and commercialize daplusiran/tomligisiran (GSK5637608, formerly JNJ-3989), the Company's third-generation subcutaneously administered RNAi therapeutic candidate being developed as a potential therapy for patients with chronic hepatitis B virus infection. daplusiran/tomligisiran had previously been licensed to Janssen Pharmaceuticals, Inc. ("Janssen") in October 2018. GSK is currently in the process of initiating a Phase 2 study of daplusiran/tomligisiran followed by bepirovirsen in patients with chronic hepatitis B.

Takeda Pharmaceutical Company Limited ("Takeda")

On October 7, 2020, Takeda and the Company entered into an Exclusive License and Co-Funding Agreement (the "Takeda License Agreement"). Under the Takeda License Agreement, Takeda and the Company co-develop the Company's fazirsiran program (formerly TAK-999 and ARO-AAT), the Company's second-generation subcutaneously administered RNAi therapeutic candidate being developed as a treatment for liver disease associated with alpha-1 antitrypsin deficiency. Within the United States, fazirsiran, if approved, will be co-commercialized under a 50/50 profit sharing structure. Outside the United States, Takeda received an exclusive license to commercialize fazirsiran and will lead the global commercialization strategy, while the Company will be eligible to receive tiered royalties of 20% to 25% on net sales.

Fazirsiran - Alpha-1 Antitrypsin Deficiency (AATD)

AATD is a genetic disorder associated with liver disease in children and adults, and pulmonary disease in adults. AAT is a circulating glycoprotein protease inhibitor that is primarily synthesized and secreted by liver hepatocytes. Its physiologic function is the inhibition of neutrophil protease to protect healthy lung tissues during inflammation and prevent tissue damage. The most common disease variant, the Z mutant, has a single amino acid substitution that results in improper folding of the protein. The mutant protein cannot be effectively secreted and accumulates in globules in the hepatocytes. This triggers continuous hepatocyte injury, leading to fibrosis, cirrhosis, and increased risk of hepatocellular carcinoma.

Individuals with the homozygous PiZZ genotype have severe deficiency of functional AAT leading to pulmonary disease and hepatocyte injury and liver disease. Lung disease in this patient population is frequently treated with AAT augmentation therapy. However, augmentation therapy does nothing to treat liver disease, and there is no specific therapy for hepatic manifestations. There is a significant unmet need as liver transplant, with its attendant morbidity and mortality, is currently the only available treatment.

Fazirsiran is a subcutaneously administered RNAi therapeutic being developed as a treatment for liver disease associated with alpha-1 antitrypsin deficiency (AATD), which is a rare genetic disorder that severely damages the liver and lungs of affected individuals. Fazirsiran is designed to reduce production of the mutant Z-AAT protein by silencing the AAT gene in order to prevent accumulation of Z-AAT in the liver, allow clearance of the accumulated Z-AAT protein, prevent repeated cycles of cellular damage, and possibly prevent or even reverse the progression of liver fibrosis.

The goal of fazirsiran treatment is prevention and potential reversal of Z-AAT accumulation-related liver injury and fibrosis. Reduction of inflammatory Z-AAT protein, which has been clearly defined as the cause of progressive liver disease in AATD patients, is important as it is expected to halt the progression of liver disease and allow fibrotic tissue repair.

Takeda is conducting multiple Phase 3 studies for the treatment of AATD liver disease including the REDWOOD study.

Amgen Inc. ("Amgen")

On September 28, 2016, Amgen and the Company entered into two collaboration and license agreements and a common stock purchase agreement. Under the Second Collaboration and License Agreement (the "Olpasiran Agreement"), Amgen received a worldwide, exclusive license to the Company's novel RNAi olpasiran (previously referred to as AMG

890 or ARO-LPA) program. These RNAi molecules are designed to reduce elevated lipoprotein(a), which is a genetically validated, independent risk factor for atherosclerotic cardiovascular disease. Under the Olpasiran Agreement, Amgen is wholly responsible for clinical development and commercialization.

In November 2022, Royalty Pharma Investments 2019 ICAV ("Royalty Pharma") and the Company entered into a Royalty Purchase Agreement (the "Royalty Pharma Agreement"). In consideration for the payments under the Royalty Pharma Agreement, Royalty Pharma is entitled to receive all royalties otherwise payable by Amgen to the Company under the Olpasiran Agreement. The Company remains eligible to receive up to an additional $485.0 million in remaining development, regulatory and sales milestone payments payable from Amgen and Royalty Pharma.

Olpasiran - Atherosclerotic Cardiovascular Disease (ASCVD)

Olpasiran is designed to reduce production of apolipoprotein A, a key component of lipoprotein(a), which has been genetically linked with increased risk of cardiovascular diseases, independent of cholesterol and LDL levels. Amgen completed a Phase 2 clinical study evaluating the efficacy, safety, and tolerability of olpasiran in subjects with elevated levels of lipoprotein(a). Amgen reported Phase 2 clinical results at the American Heart Association (AHA) Scientific Sessions in November 2022 and simultaneously published in the New England Journal of Medicine. Amgen began evaluating olpasiran in a Phase 3 study (OCEAN) to assess the impact of olpasiran on major cardiovascular events in participants with atherosclerotic cardiovascular disease and elevated lipoprotein(a), in a double-blind, randomized, placebo-controlled, multi center study in December 2022.

Sarepta Therapeutics, Inc. ("Sarepta")

On November 25, 2024, Sarepta and Company entered into a global licensing and collaboration agreement. The Sarepta Agreement covers multiple clinical and preclinical programs in rare, genetic diseases of the muscle, central nervous system (CNS), and the lungs, as well as allows Sarepta to select up to six new targets for Company to conduct discovery and preclinical development activities in areas complementary to Sarepta's leadership in precision genetic medicine for rare diseases, which can utilize Arrowhead's proprietary and differentiated TRiM platform. Clinical stage programs under the license and collaboration agreement include SRP-1001 (ARO-DUX4), SRP-1003 (ARO-DM1), SRP-1002 (ARO-MMP7), and SRP-1004 (ARO-ATXN2). Preclinical stage programs under the Sarepta Agreement include ARO-HTT for patients with Huntington's disease, ARO-ATXN1 for patients with spinocerebellar ataxia 1 (SCA1), and ARO-ATXN3 for patients with spinocerebellar ataxia 3 (SCA3).

SRP-1001 (ARO-DUX4) - Facioscapulohumeral Muscular Dystrophy (FSHD)

Facioscapulohumeral muscular dystrophy (FSHD) is an autosomal dominant disease associated with the failure to maintain complete epigenetic suppression of DUX4 expression in differentiated skeletal muscle, leading to overexpression of DUX4, which is myotoxic and can lead to muscle degeneration. As DUX4 expression is recognized as the cause of muscle pathology in FSHD patients, the Company believes that the selective targeting and knockdown of DUX4 using RNAi may prevent or reverse downstream myotoxicity and lead to muscle repair and improvement in muscle function in patients. There are currently no effective treatments specifically for FSHD.

ARO-DUX4 is designed to target the gene that encodes human double homeobox 4 (DUX4) protein as a potential treatment for patients with facioscapulohumeral muscular dystrophy. The Company is currently investigating ARO-DUX4 in a Phase 1/2a clinical trial.

SRP-1003 (ARO-DM1) - Type 1 Myotonic Dystrophy

Type 1 myotonic dystrophy is an autosomal dominant, debilitating, chronic progressive multisystem disorder characterized by an expansion of a highly unstable CUGexp in the DMPK gene. Patients with DM1 have muscle weakness and wasting, myotonia, cataracts, and often have cardiac conduction abnormalities, and may become physically disabled and have a shortened life span.

ARO-DM1 is designed to reduce expression of the dystrophia myotonica protein kinase (DMPK) gene. There is currently no approved disease-modifying therapy for type 1 myotonic dystrophy (DM1). Treatments have focused on symptomatic management, including physical therapy, exercise, ankle-foot orthoses, wheelchairs, and other assistive devices. The Company is currently investigating ARO-DM1 in a Phase 1/2a clinical trial.

SRP-1002 (ARO-MMP7) - Idiopathic Pulmonary Fibrosis (IPF)

Idiopathic Pulmonary Fibrosis (IPF) is a chronic interstitial lung disease characterized by progressive fibrosis.

ARO-MMP7 is designed to reduce expression of matrix metalloproteinase 7 (MMP7) as a potential treatment for IPF. The Company is currently investigating ARO-MMP7 in a Phase 1/2a clinical trial.

SRP-1004 (ARO-ATXN2) - Spinocerebellar Ataxia 2 (SCA2)

SCA2 is a progressive cerebellar ataxia with instability of stance, speech and swallow disorder, pain, spasticity, and ocular signs, caused by gain of function of mutant expanded polyQ ATXN2 protein.

ARO-ATXN2 is designed to reduce the expression of the ATXN2 gene as a potential treatment for spinocerebellar ataxia 2 (SCA2). The Company is currently investigating ARO-ATXN2 in a Phase 1 clinical trial.

Novartis Pharma AG ("Novartis")

On August 29, 2025, Novartis and the Company entered into a global licensing and collaboration agreement. The agreement covers ARO-SNCA, Arrowhead's preclinical stage siRNA therapy against alpha-synuclein for the treatment of synucleinopathies, such as Parkinson's Disease, and other additional collaboration targets that will utilize Arrowhead's proprietary TRiM platform. The transaction closed in October 2025.

C. Intellectual Property and Other Key Agreements

The Company controls approximately 643 issued patents (including 404 directed to RNAi trigger molecules and 159 directed to targeting groups or targeting compounds), including European validations, and approximately 833 currently pending patent applications worldwide from 103 different patent families. The Company's patent applications have been filed throughout the world, including, in the United States, Argentina, ARIPO (Africa Regional Intellectual Property Organization), Australia, Brazil, Canada, Chile, China, Eurasian Patent Organization, Europe, GCC (Gulf Cooperation Council), Hong Kong, Israel, India, Indonesia, Iraq, Jordan, Japan, Lebanon, Mexico, New Zealand, OAPI (African Intellectual Property Organization), Peru, Philippines, Russian Federation, South Africa, Saudi Arabia, Singapore, South Korea, Thailand, Taiwan, Uruguay, Venezuela, and Vietnam.

***RNAi Triggers*:** The Company owns issued patents or has filed patent applications directed to RNAi trigger molecules, which serve as the foundation of the Company's TRiM platform, and are targeted to reduce expression of various gene targets. However, the Company cannot guarantee that issued patents will be enforceable or provide adequate protection for the Company, or that pending patent applications will result in issued patents. These patents and patent applications that relate to partnered and non-partnered products in the Company's clinical pipeline include the following:

Patent Group	Estimated Year(s) of Expiration*
AAT	2035, 2038
ALK7	2044
ANGPTL3	2038
APOC3	2038
APOC3/PCSK9 Dimer	2045
ATXN2	2044
C3	2043
CFB	2044
DM1	2043
DUX4	2041
HBV	2032, 2036, 2037
HSD17B13	2039, 2043
INHBE	2044
LPA	2036
MMP7	2042
PNPLA3	2041
RAGE (AGER)	2042

*Assuming issuance of any pending patent applications, and excluding any patent term adjustments or patent term extensions.

***Delivery Technologies*:** The delivery technology-related patents and patent applications, which include components used in the Company's TRiM platform, have been filed and/or issued in various jurisdictions worldwide including the United States, Argentina, Australia, Brazil, Canada, China, Eurasian Patent Organization, Europe (including validations in France, Germany, Italy, Spain, Switzerland, United Kingdom), GCC (Gulf Cooperation Council),

Israel, India, Japan, Lebanon, Mexico, New Zealand, Philippines, Russia, South Africa, South Korea, Singapore, Taiwan, and Uruguay. However, the Company cannot guarantee that issued patents will be enforceable or provide adequate protection for the Company, or that pending patent applications will result in issued patents. These various groups of patents and applications that relate to partnered and non-partnered products in the Company's clinical pipeline are set forth below:

Patent Group	Estimated Year(s) of Expiration*
Targeting ligands and other RNAi delivery and platform technologies	
CNS Intrathecal Delivery Platform	2043
Adipose Delivery Platform	2044
BBB Shuttle Delivery Platform	2045
Muscle delivery platform	2041
PK/PD lipid modifiers	2041
RNAi agent design (5′-phosphate mimic)	2037
Targeting groups (αvβ6 integrin ligands)	2037, 2038, 2041
Targeting groups (N-acetylgalactosamine ligands)	2037
Trialkyne linkers	2039

*Assuming issuance of any pending patent applications, and excluding any patent term adjustments or patent term extensions.

The RNAi and drug delivery patent landscapes are complex and rapidly evolving. As such, the Company may need to obtain additional patent licenses prior to commercialization of its candidates. Please see “Risk Factors” in Part I, Item 1A of this Annual Report on Form 10-K.

Acquisition of Assets from Novartis

On March 3, 2015, Novartis and the Company entered into an Asset Purchase and Exclusive License Agreement (the “RNAi Purchase Agreement”) pursuant to which the Company acquired Novartis’s RNAi assets and rights thereunder. Pursuant to the RNAi Purchase Agreement, the Company acquired or was granted a license to certain patents and patent applications owned or controlled by Novartis related to RNAi therapeutics, was assigned Novartis’s rights under a license from Alnylam Pharmaceuticals, Inc. (“Alnylam”) (the “Alnylam-Novartis License”) and acquired a license to certain additional Novartis assets (the “Licensed Novartis Assets”). The patents acquired from Novartis include multiple patent families covering delivery technologies and RNAi-trigger design rules and modifications. The Licensed Novartis Assets include an exclusive, worldwide right and license, solely in the RNAi field, with the right to grant sublicenses through multiple tiers under or with respect to certain patent rights and know how relating to delivery technologies and RNAi-trigger design rules and modifications. Under the assigned Alnylam-Novartis License, the Company acquired a worldwide, royalty-bearing, exclusive license with limited sublicensing rights to existing and future Alnylam intellectual property (including intellectual property that came under Alnylam’s control on or before March 31, 2016), excluding intellectual property concerning delivery technology, to research, develop and commercialize 30 undisclosed gene targets.

Non-Exclusively Licensed Patent Rights from Roche

On October 21, 2011, the Company acquired the RNAi therapeutics business of Hoffmann-La Roche, Inc. and F. Hoffmann-La Roche Ltd. (collectively, “Roche”). The acquisition provided the Company with two primary sources of value:

- Broad freedom to operate with respect to key patents directed to the primary RNAi-trigger formats: canonical, unlocked nucleotide analogs (“UNA”), meroduplex, and dicer substrate structures; and
- A large team of scientists experienced in RNAi and oligonucleotide delivery.

Pursuant to this acquisition, Roche assigned to the Company its entire rights under certain licenses including: the License and Collaboration Agreement between Roche and Alnylam dated July 8, 2007; the Non-Exclusive Patent License Agreement between Roche and MDRNA, Inc. dated February 12, 2009 (“MDRNA License”); and the Non-Exclusive License Agreement between Roche and City of Hope dated September 19, 2011 (collectively the “RNAi Licenses”).

The RNAi Licenses included licenses to patents related to modifications of double-stranded oligonucleotides, including modifications to the base, sugar, or internucleoside linkage, nucleotide mimetics, and end modifications, which do not abolish the RNAi activity of the double-stranded oligonucleotides. Also included are patents relating to modified

double-stranded oligonucleotides, such as meroduplexes described in U.S. Patent No. 9,074,205 assigned to Marina Biotech (f/k/a MDRNA, Inc.), as well as U.S. Patent Nos. 8,314,227, 9,051,570, and 9,303,260 related to UNA. The UNA patents were assigned by Marina Biotech to Arcturus Therapeutics, Inc., but remain part of the MDRNA License.

D. Government Regulation

Government authorities in the United States, at the federal, state, and local levels, and in other countries and jurisdictions, including the European Union ("EU"), extensively regulate, among other things, the research, development, testing, product approval, manufacture, quality control, manufacturing changes, packaging, storage, recordkeeping, labeling, promotion, advertising, sales, distribution, marketing, and import and export of drugs and biologic products. All of the Company's current product candidates are expected to be regulated as drugs. The processes for obtaining regulatory approval in the United States and in foreign countries and jurisdictions, along with compliance with applicable statutes and regulations and other regulatory authorities both pre- and post-commercialization, are a significant factor in the production and marketing of the Company's products and its R&D activities and require the expenditure of substantial time and financial resources.

Review and Approval of Drugs in the United States

The FDA and other government entities regulate drugs under the Federal Food, Drug, and Cosmetic Act (the "FDCA"), and the regulations promulgated under those statutes, as well as other federal and state statutes and regulations. Failure to comply with applicable legal and regulatory requirements in the United States at any time during the product development process, approval process, or after approval, may subject us to a variety of administrative or judicial sanctions, such as a delay in approving or refusal by the FDA to approve pending applications, withdrawal of approvals, delay or suspension of clinical trials, issuance of warning letters and other types of regulatory letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil monetary penalties, refusals of or debarment from government contracts, exclusion from the federal healthcare programs, restitution, disgorgement of profits, civil or criminal investigations by the FDA, U.S. Department of Justice, State Attorneys General, and/or other agencies, False Claims Act suits and/or other litigation, and/or criminal prosecutions.

An applicant seeking approval to market and distribute a new drug in the United States must typically undertake the following:

(1) completion of preclinical laboratory tests, which may include animal and *in vitro* studies, and formulation studies in compliance with the FDA's good laboratory practice ("GLP") regulations;

(2) submission to the FDA of an Investigational New Drug application ("IND") for human clinical testing, which must become effective without FDA objection before human clinical trials may begin;

(3) approval by an independent institutional review board ("IRB"), representing each clinical site before each clinical trial may be initiated;

(4) performance of adequate and well-controlled human clinical trials in accordance with the FDA's current good clinical practice ("cGCP") regulations, to establish the safety and effectiveness of the proposed drug product for each indication for which approval is sought;

(5) preparation and submission to the FDA of an NDA;

(6) satisfactory review of the NDA by an FDA advisory committee, where appropriate or if applicable;

(7) satisfactory completion of one or more FDA inspections of the manufacturing facility or facilities at which the drug product, and the active pharmaceutical ingredient or ingredients thereof, are produced to assess compliance with current good manufacturing practice ("cGMP") regulations and to assure that the facilities, methods, and controls are adequate to ensure the product's identity, strength, quality, and purity;

(8) payment of user fees, as applicable, and securing FDA approval of the NDA; and

(9) compliance with any post-approval requirements, such as any Risk Evaluation and Mitigation Strategies ("REMS") or post-approval studies required by the FDA.

Preclinical Studies and an IND

Preclinical studies can include *in vitro* and animal studies to assess the potential for adverse events and, in some cases, to establish a rationale for therapeutic use, which studies are subject to federal regulations and requirements, including GLP regulations. Other studies include laboratory evaluation of the purity, stability and physical form of the manufactured drug substance or active pharmaceutical ingredient and the physical properties, stability and reproducibility of the formulated drug or drug product. An IND sponsor must submit the results of the preclinical tests, together with

manufacturing information, analytical data, any available clinical data or literature and plans for clinical studies, among other things, to the FDA as part of an IND. Some preclinical testing, such as longer-term toxicity testing, animal tests of reproductive adverse events and carcinogenicity, may continue after the IND is submitted. However, submission of an IND may not result in the FDA allowing clinical trials to commence, and clinical trials that have commenced may be paused, if the FDA has any concerns and places the trial on hold.

A clinical hold is an order issued by the FDA to the sponsor to delay a proposed clinical investigation or to suspend an ongoing investigation in part or in full. Following issuance of a full or partial clinical hold, an investigation may only resume after the FDA has notified the sponsor that the investigation may proceed, which determination will be based on information provided by the sponsor correcting the deficiencies previously cited or otherwise satisfying the FDA that the investigation can proceed.

Human Clinical Studies in Support of an NDA

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators in accordance with cGCP requirements, which include, among other things, the requirement that all research subjects provide their informed consent in writing before their participation in any clinical trial. Clinical trials are conducted under written study protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. The plan for, and protocols and informed consent processes and documentation for, any clinical trial is also subject to certain requirements for approval, and periodic review and reapproval, by an IRB representing each institution participating in the clinical trial. Information about certain clinical trials must be submitted within specific timeframes to the National Institutes of Health for public dissemination on its ClinicalTrials.gov website.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

Phase 1: The product candidate is initially introduced into healthy human subjects or patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early indication of its effectiveness.

Phase 2: The product candidate is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.

Phase 3: The product candidate is administered to an expanded patient population, generally at geographically dispersed clinical trial sites, in well-controlled clinical trials to generate enough data to statistically evaluate the efficacy and safety of the product for approval, to establish the overall risk-benefit profile of the product, and to provide adequate information for the labeling of the product.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if serious adverse events occur. Phase 1, Phase 2, and Phase 3 clinical trials may not be completed successfully within any specified period, or at all. Furthermore, the FDA or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. An IRB also may suspend or terminate a clinical trial under certain circumstances. The FDA will typically inspect one or more clinical sites in late-stage clinical trials to assure compliance with cGCP and the integrity of the clinical data submitted.

A sponsor may choose, but is not required, to conduct a foreign clinical study under an IND. When a foreign clinical study is conducted under an IND, all IND requirements must be met unless waived. When the foreign clinical study is not conducted under an IND, the sponsor must ensure that the study complies with certain FDA regulatory requirements in order to use the study as support for an IND or application for marketing approval or licensure, including that the study was conducted in accordance with cGCP, including review and approval by an independent ethics committee and use of proper procedures for obtaining informed consent from subjects, and the FDA is able to validate the data from the study through an onsite inspection if the FDA deems such inspection necessary. The cGCP requirements encompass both ethical and data integrity standards for clinical studies.

Submission of an NDA to the FDA

Assuming successful completion of required clinical testing and other requirements, the results of the preclinical and clinical studies, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA requesting approval to market the drug product for one or more indications. Under federal law, the submission of most NDAs is additionally subject to an application user fee, currently approximately $4.682 million for fiscal year 2026, for applications requiring clinical data, and the sponsor of

an approved NDA is also subject to an annual program fee, currently approximately $0.442 million for fiscal year 2026. These fees are adjusted annually.

The FDA conducts a preliminary review of an NDA within 60 days of its receipt and informs the sponsor by the 74th day after the FDA's receipt of the submission to determine whether the application is sufficiently complete to permit substantive review. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA has agreed to specified performance goals in the review process of NDAs. Most such applications are meant to be reviewed within ten months from the date of filing, and most applications for "priority review" products are meant to be reviewed within six months of filing. The review process may be extended by the FDA for three additional months to consider new information or clarification provided by the applicant to address an outstanding deficiency identified by the FDA following the original submission.

Before approving an NDA, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with cGCP.

The FDA also may require submission of a REMS plan to mitigate any identified or suspected serious risks. The REMS plan could include medication guides, physician communication plans, assessment plans, and elements to assure safe use, such as restricted distribution methods, patient registries, or other risk minimization tools.

The FDA is required to refer an application for a novel drug to an advisory committee or explain why such referral was not made. Typically, an advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

The FDA's Decision on an NDA

On the basis of the FDA's evaluation of the NDA and accompanying information, including the results of the inspection of the manufacturing facilities, the FDA may issue an approval letter or a complete response letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If and when those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

If the FDA approves a product, it may limit the approved indications for use for the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies be conducted to further assess the drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution restrictions or other risk management mechanisms, including REMS, which can materially affect the potential market and profitability of the product. After approval, the FDA may seek to prevent or limit further marketing of a product based on the results of post-market studies or surveillance programs. Some types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Under the Orphan Drug Act, the FDA may grant orphan drug designation to a drug intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making available in the United States a drug for this type of disease or condition will be recovered from sales in the United States for that drug. Orphan drug designation entitles the applicant to incentives such as grant funding towards clinical study costs, tax advantages, and waivers of FDA user fees. Orphan drug designation must be requested before submitting an NDA, and both the drug and the disease or condition must meet certain criteria specified in the Orphan Drug Act and FDA's regulations. The granting of an orphan drug designation does not alter the standard regulatory requirements and process for obtaining marketing approval. Safety and effectiveness of a drug must be established through adequate and well-controlled studies.

After the FDA grants orphan drug designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. If a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the

FDA may not approve any other application to market the same drug for the same indication, except in very limited circumstances, for seven years. Orphan drug exclusivity does not prevent the FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition.

The FDA's interpretation of the scope of orphan drug exclusivity may change. The FDA's longstanding interpretation of the Orphan Drug Act is that exclusivity is specific to the orphan indication for which the drug was actually approved. As a result, the scope of exclusivity has been narrow and protected only against competition from the same "use or indication" rather than the broader "disease or condition." Recent court decisions have created uncertainty in the application and interpretation of orphan drug exclusivity, which may limit the drugs that can receive orphan drug exclusivity.

Expedited Review and Accelerated Approval Programs

A sponsor may seek approval of its product candidate under programs designed to accelerate the FDA's review and approval of NDAs. For example, Fast Track Designation may be granted to a drug intended for treatment of a serious or life-threatening disease or condition and data demonstrate its potential to address unmet medical needs for the disease or condition. The key benefits of Fast Track Designation are the eligibility for priority review, rolling review (submission of portions of an application before the complete marketing application is submitted), and accelerated approval, if relevant criteria are met.

The FDA may approve an NDA under the accelerated approval program if the drug treats a serious condition, provides a meaningful advantage over available therapies, and demonstrates an effect on either (1) a surrogate endpoint that is reasonably likely to predict clinical benefit, or (2) on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. Post-marketing studies or completion of ongoing studies after marketing approval, which the FDA may require to be underway prior to approval, are generally required to verify the drug's clinical benefit in relationship to the surrogate endpoint or ultimate outcome in relationship to the clinical benefit. In addition, promotional materials is required for drugs granted accelerated approval.

In addition, the Food and Drug Administration Safety and Innovation Act of 2012 ("FDASIA") established the Breakthrough Therapy designation. A sponsor may seek FDA designation of its product candidate as a breakthrough therapy if the drug is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. If a drug is designated as breakthrough therapy, FDA will provide more intensive guidance on the drug development program and expedite its review.

Post-Approval Requirements

Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing, annual user fee requirements for any marketed products and the establishments at which such products are manufactured, as well as new application fees for supplemental applications with clinical data.

In addition, drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and state agencies and are subject to periodic unannounced inspections by the FDA and these state agencies for compliance with cGMP requirements. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance.

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events or problems with manufacturing processes of unanticipated severity or frequency, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to

add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning, untitled, or it has come to our attention letters, or holds on post-approval clinical trials;
- refusal of the FDA to approve pending NDAs or supplements to approved NDAs, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Drugs may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

In addition, the distribution of prescription pharmaceutical products is subject to the Prescription Drug Marketing Act ("PDMA"), which regulates the distribution of drugs and drug samples at the federal level and sets minimum standards for the registration and regulation of drug distributors by the states. Both the PDMA and state laws limit the distribution of prescription pharmaceutical product samples and impose requirements to ensure accountability in distribution.

Abbreviated New Drug Applications for Generic Drugs

In 1984, with passage of the Drug Price Competition and Patent Term Restoration Act of 1984 (commonly referred to as the "Hatch-Waxman Amendments") amending the FDCA, Congress authorized the FDA to approve generic drugs that are the same as drugs previously approved by the FDA under the NDA provisions of the statute. To obtain approval of a generic drug, an applicant must submit an abbreviated new drug application ("ANDA") to the agency. In support of such applications, a generic manufacturer may rely on the preclinical and clinical testing previously conducted for a drug product previously approved under an NDA, known as the reference listed drug ("RLD"). In order for an ANDA to be approved, the FDA must find that the generic version is identical to the RLD with respect to the active ingredients, the route of administration, the dosage form, and the strength of the drug. However, an applicant may submit an ANDA suitability petition to request the FDA's prior permission to submit an abbreviated application for a drug that differs from the RLD in route of administration, dosage form, or strength, or for a drug that has one different active ingredient in a fixed combination drug product (i.e., a drug product with multiple active ingredients).

At the same time, the FDA must also determine that the generic drug is "bioequivalent" to the innovator drug. Under the statute, a generic drug is bioequivalent to a RLD if the rate and extent of absorption of the generic drug do not show a significant difference from the rate and extent of absorption of the RLD. Upon approval of an ANDA, the FDA indicates that the generic product is "therapeutically equivalent" to the RLD and it assigns a therapeutic equivalence rating to the approved generic drug in its publication "Approved Drug Products with Therapeutic Equivalence Evaluations," also referred to as the "Orange Book." Physicians and pharmacists consider the therapeutic equivalence rating to mean that a generic drug is fully substitutable for the RLD. In addition, by operation of certain state laws and numerous health insurance programs, the FDA's designation of a therapeutic equivalence rating often results in substitution of the generic drug without the knowledge or consent of either the prescribing physician or patient.

Under the Hatch-Waxman Amendments, the FDA may not approve an ANDA until any applicable period of nonpatent exclusivity for the RLD has expired. The FDCA provides a period of five years of data exclusivity for NDAs containing a new chemical entity. In cases where such exclusivity has been granted, an ANDA may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification, in which case the applicant may submit its application four years following the original product approval. The FDCA also provides for a period of three years of exclusivity if the NDA includes reports of one or more new clinical investigations, other than bioavailability or bioequivalence studies, that were conducted by or for the applicant and are essential to the approval of the application. This three-year exclusivity period often protects changes to a previously approved drug product, such as a new dosage form, route of administration, combination or indication.

Hatch-Waxman Patent Certification and the 30 Month Stay

Upon approval of an NDA or a supplement thereto, NDA sponsors are required to list with the FDA each patent with claims that cover the applicant's product or a method of using the product. Each of the patents listed by the NDA sponsor is published in the Orange Book. When an ANDA applicant files its application with the FDA, the applicant is required to certify to the FDA concerning any patents listed for the referenced product in the Orange Book, except for patents covering methods of use for which the ANDA applicant is not seeking approval.

A certification that the new product will not infringe the already approved product's listed patents or that such patents are invalid or unenforceable is called a Paragraph IV certification. If the applicant does not challenge the listed patents or indicate that it is not seeking approval of a patented method of use, the ANDA application will not be approved until all the listed patents for the referenced product have expired. If the ANDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days after the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months after the receipt of the Paragraph IV certification, expiration of the patent, settlement of the lawsuit or a decision in the infringement case that is favorable to the ANDA applicant.

505(b)(2) New Drug Applications

As an alternative path to FDA approval for modifications to formulations or uses of products previously approved by the FDA pursuant to an NDA, an applicant may submit an NDA under Section 505(b)(2) of the FDCA. Section 505(b)(2) permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by, or for, the applicant, and for which the applicant has not obtained a right of reference. If the 505(b)(2) applicant can establish that reliance on the FDA's previous findings of safety and effectiveness is scientifically and legally appropriate, it may eliminate the need to conduct certain preclinical studies or clinical trials of the new product. The FDA may also require companies to perform additional bridging studies or measurements, including clinical trials, to support the change from the previously approved reference drug. The FDA may then approve the new drug candidate for all, or some, of the label indications for which the reference drug has been approved, as well as for any new indication sought by the 505(b)(2) applicant.

To the extent that a Section 505(b)(2) applicant is relying on studies conducted for an already approved product, the applicant is required to certify to the FDA concerning any patents listed for the approved product in the Orange Book to the same extent that an ANDA applicant would. As a result, approval of a 505(b)(2) NDA can be stalled until all the listed patents claiming the referenced product have expired, until any non-patent exclusivity, such as exclusivity for obtaining approval of a new chemical entity, listed in the Orange Book for the referenced product has expired, and, in the case of a Paragraph IV certification and subsequent patent infringement suit, until the earlier of 30 months, settlement of the lawsuit or a decision in the infringement case that is favorable to the Section 505(b)(2) applicant.

Pediatric Studies and Exclusivity

Under the Pediatric Research Equity Act of 2003, an NDA or supplement thereto must contain data that are adequate to assess the safety and effectiveness of the drug product for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. With the enactment of FDASIA, sponsors must also submit pediatric study plans prior to the assessment data. Those plans must contain an outline of the proposed pediatric study or studies the applicant plans to conduct, including study objectives and design, any deferral or waiver requests, and other information required by regulation. The applicant, the FDA, and the FDA's internal review committee must then review the information submitted, consult with each other, and agree upon a final plan. The FDA or the applicant may request an amendment to the plan at any time.

The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. Additional requirements and procedures relating to deferral requests and requests for extension of deferrals are contained in FDASIA.

Pediatric exclusivity is another type of non-patent marketing exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the non-patent and orphan exclusivity. This six-month exclusivity may be granted if an NDA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or patent protection cover the product are extended by six months. This is not a patent term extension under 35 U.S.C. § 156, but it effectively extends the regulatory period during which the FDA cannot accept or approve another application.

Patent Term Restoration and Extension

A patent claiming a new drug product may be eligible for a limited patent term extension under the Hatch-Waxman Amendments. Those Amendments permit a patent restoration of up to five years for patent term lost during product development and the FDA regulatory review. The restoration period granted is typically one-half the time between the

effective date of an IND and the submission date of a NDA, plus the time between the submission date of a NDA and ultimate approval. Patent term restoration cannot be used to extend the remaining term of a patent past a total of 14 years from the product's approval date. Only one patent applicable to an approved drug product is eligible for the extension, and the application for the extension must be submitted prior to the expiration of the patent in question. The U.S. Patent and Trademark Office reviews and approves the application for any patent term extension or restoration in consultation with the FDA.

Review and Approval of Drugs in the European Union and United Kingdom

In order to market any pharmaceutical product outside of the United States, a company must also comply with numerous and varying regulatory requirements of other countries and jurisdictions governing, among other things, research and development, testing, manufacturing, quality control, safety, efficacy, labeling, clinical trials, marketing authorization, packaging, storage, record keeping, reporting, export and import, advertising, marketing and other promotional practices involving pharmaceutical products, as well as commercial sales, distribution, authorization, approval and post-approval monitoring and reporting of its products. Whether or not a company obtains FDA approval for a pharmaceutical product, the company would need to obtain the necessary approvals by the comparable foreign regulatory authorities before it can commence clinical trials or marketing of the pharmaceutical product in those countries or jurisdictions. The approval process ultimately varies between countries and jurisdictions and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries and jurisdictions might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country or jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country or jurisdiction may negatively impact the regulatory process in others.

The United Kingdom ("UK") formally left the EU on January 31, 2020 ("Brexit") and EU laws now only apply to the UK in respect of Northern Ireland as laid out in the Protocol on Ireland and Northern Ireland. The EU and the UK have agreed on a trade and cooperation agreement ("TCA") which includes provisions affecting the life sciences sector (including on customs and tariffs). There are some specific provisions concerning pharmaceuticals, including the mutual recognition of Good Manufacturing Practice ("GMP") and issued GMP documents. The TCA does not, however, contain wholesale mutual recognition of UK and EU pharmaceutical regulations and product standards.

The UK government has enacted the Medicines and Medical Devices Act 2021. The purpose of the act is to enable the existing regulatory frameworks in relation to human medicines and clinical trials of human medicines, among others, to be updated. The powers under the act may only be exercised in relation to specified matters and must safeguard public health.

The Medicines and Medical Devices Act 2021 supplements the UK Medical Devices Regulations 2002 ("UK Regulations"), which are based on the EU Medical Devices Directive as amended to reflect the UK's post-Brexit regulatory regime. Notably, the UK Regulations do not include any of the revisions that have been made by the EU Medical Devices Regulation (EU) 2017/745, which, since May 26, 2021, applies in all EU member states.

In June 2025, new post-market surveillance requirements for medical devices entered into force under the Medical Devices (Post-market Surveillance Requirements (Amendment) (Great Britain) Regulations 2024. These requirements represent a significant tightening of post-market surveillance obligations, including the introduction of mandatory post-market surveillance plans and periodic safety update reports, reduced incident reporting deadlines, and new requirements to report and analyze incident trends.

In July 2025, the UK's Medicines and Healthcare products Regulatory Agency ("MHRA") published its responses to the consultation on *Medical Device Regulations: Routes to market and in vitro diagnostic devices* carried out in 2024. In its responses, the MHRA confirmed that it will be moving forward with a number of the proposed changes, and intends to table 'Pre-Market Regulations' in Parliament later this year, with implementation aimed for 2026. Among other things, MHRA will extend the sunset period for four key EU-derived regulations (covering IVDs, electronic instructions, animal tissue devices, and approved bodies) beyond May 26, 2025 while new UK-specific laws are established. MHRA also plans to rely on approvals from Australia, Canada, EU, and US, given their comparable regulatory systems, and will implement three routes to market. Also, manufacturers will be required to assign a Unique Device Identification (UDI) to devices before they are placed on the Great Britain market. Once UDI is operational, the requirement for mandatory UKCA (UK Conformit Assessed) marking on devices or packaging will be removed. Under the Medical Devices (Amendment) (Great Britain) Regulations 2023, CE (Conformité Européene, or European Conformity) marked European medical devices will continue to be accepted for sale in the UK until 2028 or 2030 (depending on the type of device).

Drug and Biologic Development Process

The conduct of clinical trials in the EU is governed by the EU Clinical Trials Regulation (EU) No. 536/2014 ("CTR") which became applicable on January 31, 2022. The CTR replaced the Clinical Trials Directive 2001/20/EC,

(Clinical Trials Directive) and introduced a complete overhaul of the existing regulation of clinical trials for medicinal products in the EU.

Under the new CTR, a sponsor is able to submit a single application for approval of a clinical trial through a centralized EU clinical trials portal, the Clinical Trials Information System ("CTIS"). One national regulatory authority (the reporting EU member state proposed by the applicant) takes the lead in validating and evaluating the application, and consults and coordinates with the other concerned EU member states. If an application is rejected, it may be amended and resubmitted through the CTIS. If an approval is issued, the sponsor may start the clinical trial in all concerned EU member states. However, a concerned EU member state may in limited circumstances declare an "opt-out" from an approval and prevent the clinical trial from being conducted in such EU member state. The CTR also aims to streamline and simplify the rules on safety reporting and introduces enhanced transparency requirements such as mandatory submission of a summary of the clinical trial results to the CTIS. The CTR included a three-year transition period. Since January 31, 2023, submission of initial Clinical Trial Applications "CTA") via CTIS has been mandatory and CTIS serves as the single entry point for submission of clinical trial-related information and data. As of January 31, 2025, all ongoing trials approved under the former Clinical Trials Directive need to comply with the CTR and have to be transitioned to CTIS.

Under both the former regime and the new CTR, national laws, regulations, and the applicable GCP and Good Laboratory Practice standards must also be respected during the conduct of the trials, including the International Council for Harmonization of Technical Requirements for Pharmaceuticals for Human Use guidelines on GCP, and the ethical principles that have their origin in the Declaration of Helsinki.

During the development of a medicinal product, the European Medicines Agency ("EMA") and national regulators within the EU provide the opportunity for dialogue and guidance on the development program. At the EMA level, this is usually done in the form of scientific advice, which is given by the Committee for Medicinal Products for Human Use ("CHMP") on the recommendation of the Scientific Advice Working Party ("SAWP"). A fee is incurred with each scientific advice procedure, but is significantly reduced for designated orphan medicines. Advice from the EMA is typically provided based on questions concerning, for example, quality (chemistry, manufacturing and controls testing), nonclinical testing and clinical studies, and pharmacovigilance plans and risk-management programs. Advice is not legally binding with regard to any future Marketing Authorization Application ("MAA") of the product concerned.

Marketing Authorization Procedures

In the EU and in Iceland, Norway and Liechtenstein (together the European Economic Area or "EEA"), after completion of all required clinical testing, pharmaceutical products may only be placed on the market after obtaining a Marketing Authorization ("MA"). To obtain an MA of a drug under EU regulatory systems, an applicant can submit a MAA through, amongst others, a centralized or decentralized procedure.

The centralized procedure provides for the grant of a single MA by the European Commission ("EC") that is valid for all EU member states and, after respective national implementing decisions which must be rendered within 30 days, in the three additional member states of the EEA. The centralized procedure is compulsory for specific pharmaceutical products, including for medicines developed by means of certain biotechnological processes, products designated as orphan pharmaceutical products, advanced therapy pharmaceutical products and pharmaceutical products with a new active substance indicated for the treatment of certain diseases (AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune diseases and other immune dysfunctions and viral diseases). For pharmaceutical products containing a new active substance not yet authorized in the European Economic Area before May 20, 2004 and indicated for the treatment of other diseases, pharmaceutical products that constitute significant therapeutic, scientific or technical innovations or for which the grant of a MA through the centralized procedure would be in the interest of public health at EU level, an applicant may voluntarily submit an application for a marketing authorization through the centralized procedure.

Under the centralized procedure, the CHMP established at the EMA is responsible for conducting the initial assessment of a drug. The CHMP is also responsible for several post-authorization and maintenance activities, such as the assessment of modifications or extensions to an existing marketing authorization. Under the centralized procedure, the timeframe for the evaluation of an MAA by the EMA's CHMP is, in principle, 210 days from receipt of a valid MAA. However, this timeline excludes clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP, so the overall process typically takes a year or more, unless the application is eligible for an accelerated assessment. Accelerated assessment might be granted by the CHMP in exceptional cases when a pharmaceutical product is expected to be of major public health interest, particularly from the point of therapeutic innovation. On request, the CHMP can reduce the time frame to 150 days if the applicant provides sufficient justification for an accelerated assessment. The CHMP will provide a positive opinion regarding the application only if it meets certain quality, safety and efficacy requirements. However, the EC has final authority for granting the MA within 67 days after receipt of the CHMP opinion.

The decentralized procedure permits companies to file identical MA applications for a pharmaceutical product to the competent authorities in various EU member states simultaneously if such pharmaceutical product has not received marketing approval in any EU member state before. This procedure is available for pharmaceutical products not falling within the mandatory scope of the centralized procedure. The competent authority of a single EU member state, known as the reference EU member state, is appointed to review the application and provide an assessment report. Under this procedure, an applicant submits an application based on identical dossiers and related materials, including a draft summary of product characteristics, and draft labeling and package leaflet, to the reference EU member state and concerned EU member states. The reference EU member state prepares a draft assessment report and drafts of the related materials within 120 days after receipt of a valid application. Subsequently, each concerned EU member state must decide whether to approve the assessment report and related materials.

If an EU member state cannot approve the assessment report and related materials on the grounds of potential serious risk to public health, the disputed points are subject to a dispute resolution mechanism and may eventually be referred to the EC, whose decision is binding for all EU member states.

All new MAAs must include a Risk Management Plan ("RMP"), describing the risk management system that the company will put in place and documenting measures to prevent or minimize the risks associated with the product. RMPs are continually modified and updated throughout the lifetime of the medicine as new information becomes available. New RMPs are required to be submitted (i) at the request of EMA or a national competent authority, or (ii) whenever the risk-management system is modified, especially as the result of new information being received that may lead to a significant change to the benefit-risk profile or as a result of an important pharmacovigilance or risk-minimization milestone being reached. The regulatory authorities may also impose specific obligations as a condition of the MA. Since October 20, 2023, all RMPs for centrally authorized products are published by the EMA subject to only limited redactions.

Marketing Authorizations have an initial duration of five years. After these five years, the authorization may subsequently be renewed on the basis of a reevaluation of the risk-benefit balance. Once renewed, the MA is valid for an unlimited period unless the EC or the national competent authority decides, on justified grounds relating to pharmacovigilance, to proceed with only one additional five-year renewal. Applications for renewal must be made to the EMA at least nine months before the five-year period expires.

Data and Market Exclusivity in the European Union

As in the United States, it may be possible to obtain a period of market and / or data exclusivity in the EU that would have the effect of postponing the entry into the marketplace of a competitor's generic, hybrid or biosimilar product (even if the pharmaceutical product has already received an MA) and prohibiting another applicant from relying on the MA holder's pharmacological, toxicological and clinical data in support of another MA for the purposes of submitting an application, obtaining MA or placing the product on the market. New Chemical Entities ("NCE") approved in the EU qualify for eight years of data exclusivity and ten years of marketing exclusivity. The overall ten-year period can be extended to a maximum of eleven years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are deemed to bring a significant clinical benefit in comparison with existing therapies.

The data exclusivity period begins on the date of the product's first MA in the EU. After eight years, a generic product application may be submitted and generic companies may rely on the MA holder's data. However, a generic product cannot launch until two years later (or a total of 10 years after the first MA in the EU of the innovator product), or three years later (or a total of 11 years after the first MA in the EU of the innovator product) if the MA holder obtains MA for a new indication with significant clinical benefit within the eight-year data exclusivity period. Additionally, another noncumulative one-year period of data exclusivity can be added to the eight years of data exclusivity where an application is made for a new indication for a well-established substance, provided that significant preclinical or clinical studies were carried out in relation to the new indication. Another year of data exclusivity may be added to the eight years, where a change of classification of a pharmaceutical product has been authorized on the basis of significant pre-trial tests or clinical trials (when examining an application by another applicant for or holder of market authorization for a change of classification of the same substance the competent authority will not refer to the results of those tests or trials for one year after the initial change was authorized).

Products may not be granted data exclusivity since there is no guarantee that a product will be considered by the EU's regulatory authorities to include an NCE. Even if a compound is considered to be an NCE and the MA applicant is able to gain the prescribed period of data exclusivity, another company nevertheless could also market another version of the pharmaceutical product if such company can complete a full MAA with their own complete database of pharmaceutical tests, preclinical studies and clinical trials and obtain MA of its pharmaceutical product.

On April 26, 2023, the EC submitted a proposal for the reform of the European pharmaceutical legislation. After the European Parliament adopted an approving position on the reform on April 10, 2024, the Council of the European Union took a position and commented on the draft on June 4, 2025, clearing the way for the next legislative step, the trilogue proceedings. The first trilogue meeting took place on June 17, 2025, and negotiations are still ongoing. Finalization of these negotiations and / or entry into force are yet unclear; however, conclusion of the legislative process is not expected within the current Council Presidency, which ends on December 31, 2025.

The current drafts envisage:

- a shortening of the periods of data exclusivity from eight to six years (with transferrable vouchers for an additional year of market protection as an incentive for the development of new antibiotics),
- earlier regulatory guidance and extension of market exclusivity for orphan medicines (depending on certain conditions),
- four-year data exclusivity for additional indications of existing products, and
- rules governing the availability of products (including shortage prevention plans and some supply obligations for manufacturers).

There is currently no final version of the draft package. All details, in particular dates and timeframes within the package, are still subject to negotiations between the parties involved and vary between drafts.

Orphan Designation and Exclusivity

The criteria for designating an orphan medicinal product in the EU are similar in principle to those in the United States. The EMA's Committee for Orphan Medicinal Products ("COMP") evaluates applications for orphan drug designation within 90 days and will issue a recommendation if the medicinal product is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting no more than five in 10,000 persons in the EU (prevalence criterion). In addition, Orphan Drug Designation can be granted if, for economic reasons, the medicinal product would be unlikely to be developed without incentives and if there is no other satisfactory method approved in the EU of diagnosing, preventing, or treating the condition, or if such a method exists, the proposed medicinal product is a significant benefit to patients affected by the condition. Orphan drug designations are granted by the EC. An application for orphan drug designation (which is not a marketing authorization, as not all orphan-designated medicines reach the authorization application stage) must be submitted first before an application for marketing authorization of the medicinal product is submitted. The applicant will receive a fee reduction for the marketing authorization application if the orphan drug designation has been granted, but not if the designation is still pending at the time the marketing authorization is submitted, and sponsors must submit an annual report to EMA summarizing the status of development of the medicine. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. Designated orphan medicines are eligible for conditional marketing authorization.

COMP reassesses the orphan drug designation of a product in parallel with the review for a marketing authorization; for a product to benefit from market exclusivity it must maintain its orphan drug designation at the time of marketing authorization review by the EMA and approval by the EC. Additionally, any marketing authorization granted for an orphan medicinal product must only cover the therapeutic indication(s) that are covered by the orphan drug designation. Upon the grant of a marketing authorization, orphan drug designation provides up to ten years of market exclusivity in the orphan indication.

During the 10-year period of market exclusivity, with a limited number of exceptions, the regulatory authorities of the EU member states and the EMA may not accept applications for marketing authorization, accept an application to extend an existing marketing authorization or grant marketing authorization for other similar medicinal products for the same therapeutic indication. A similar medicinal product is defined as a medicinal product containing a similar active substance or substances as contained in a currently authorized orphan medicinal product, and which is intended for the same therapeutic indication. An orphan medicinal product can also obtain an additional two years of market exclusivity for an orphan-designated condition when the results of specific studies are reflected in the Summary of Product Characteristics ("SmPC"), addressing the pediatric population and completed in accordance with a fully compliant Pediatric Investigation Plan ("PIP"). No extension to any supplementary protection certificate can be granted on the basis of pediatric studies for orphan indications.

The 10-year market exclusivity may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation, i.e. the condition prevalence or financial returns criteria under Article 3 of Regulation (EC) No. 141/2000 on orphan medicinal products. When the period of orphan market exclusivity for an indication ends, the orphan drug designation for that indication expires as well. Orphan exclusivity runs in parallel

with normal rules on data exclusivity and market protection. Additionally, a marketing authorization may be granted to a similar medicinal product (orphan or not) for the same or overlapping indication subject to certain requirements.

Pediatric Development

In the EU, companies developing a new pharmaceutical product are obligated to study their product in children and must therefore submit a PIP together with a request for agreement to the EMA. The EMA issues a decision on the PIP based on an opinion of the EMA's Pediatric Committee ("PDCO"). Companies must conduct pediatric clinical trials in accordance with the PIP approved by the EMA, unless a deferral (*e.g.* until enough information to demonstrate its effectiveness and safety in adults is available) or waiver (*e.g.* because the relevant disease or condition occurs only in adults) has been granted by the EMA. The MAA for the pharmaceutical product must include the results of all pediatric clinical trials performed and details of all information collected in compliance with the approved PIP, unless a waiver or a deferral has been granted, in which case the pediatric clinical trials may be completed at a later date. Pharmaceutical products that are granted a marketing authorization on the basis of the pediatric clinical trials conducted in accordance with the approved PIP are eligible for a six month extension of the protection under a supplementary protection certificate (if any is in effect at the time of approval) or, in the case of orphan pharmaceutical products, a two year extension of the orphan market exclusivity. This pediatric reward is subject to specific conditions and is not automatically available when data in compliance with the approved PIP are developed and submitted. An approved PIP is also required when a marketing authorization holder wants to add a new indication, pharmaceutical form or route of administration for a medicine that is already authorized and covered by intellectual property rights.

Post-Approval Regulation

Similar to the United States, both MA holders and manufacturers of pharmaceutical products are subject to comprehensive regulatory oversight by the EMA, the EC and/or the competent regulatory authorities of the EU member states. This oversight applies both before and after grant of manufacturing licenses and marketing authorizations. It includes control of compliance with EU good manufacturing practices rules, manufacturing authorizations, pharmacovigilance rules and requirements governing advertising, promotion, sale, and distribution, recordkeeping, importing and exporting of pharmaceutical products.

Failure by us or by any of our third-party partners, including suppliers, manufacturers and distributors to comply with EU laws and the related national laws of individual EU member states governing the conduct of clinical trials, manufacturing approval, MA of pharmaceutical products and marketing of such products, both before and after grant of MA, manufacturing of pharmaceutical products, statutory health insurance, bribery and anti-corruption or other applicable regulatory requirements may result in administrative, civil or criminal penalties. These penalties could include delays or refusal to authorize the conduct of clinical trials or to grant MA, product withdrawals and recalls, product seizures, suspension, withdrawal or variation of the MA, total or partial suspension of production, distribution, manufacturing or clinical trials, operating restrictions, injunctions, suspension of licenses, fines and criminal penalties.

The holder of an EU MA for a pharmaceutical product must also comply with EU pharmacovigilance legislation and its related regulations and guidelines, which entail many requirements for conducting pharmacovigilance, or the assessment and monitoring of the safety of pharmaceutical products.

These pharmacovigilance rules can impose on holders of MAs the obligation to conduct a labor intensive collection of data regarding the risks and benefits of marketed pharmaceutical products and to engage in ongoing assessments of those risks and benefits, including the possible requirement to conduct additional clinical studies or post-authorization safety studies to obtain further information on a medicine's safety, or to measure the effectiveness of risk-management measures, which may be time consuming and expensive and could impact our profitability. MA holders must establish and maintain a pharmacovigilance system and appoint an individual qualified person for pharmacovigilance who is responsible for oversight of that system. Key obligations include expedited reporting of suspected serious adverse reactions and submission of Periodic Safety Update Reports ("PSURs") in relation to pharmaceutical products for which they hold MAs. The EMA reviews PSURs for pharmaceutical products authorized through the centralized procedure. If the EMA has concerns that the risk-benefit profile of a product has varied, it can adopt an opinion advising that the existing MA for the product be suspended, withdrawn or varied. The agency can advise that the MA holder be obliged to conduct post-authorization Phase 4 safety studies. If the EC agrees with the opinion, it can adopt a decision varying the existing MA. Failure by the MA holder to fulfill the obligations for which the European Commission's decision provides can undermine the on-going validity of the MA.

More generally, non-compliance with pharmacovigilance obligations can lead to the variation, suspension or withdrawal of the marketing authorization for the pharmaceutical product or imposition of financial penalties or other enforcement measures.

The manufacturing process for pharmaceutical products in the EU is highly regulated and regulators may shut down manufacturing facilities that they believe do not comply with regulations. Manufacturing requires a manufacturing authorization, and the manufacturing authorization holder must comply with various requirements set out in the applicable EU laws, regulations and guidance, including Directive 2001/83/EC, Directive 2003/94/EC, Regulation (EC) No 726/2004 and the European Commission Guidelines for Good Manufacturing Practice ("GMP"). These requirements include compliance with EU GMP standards when manufacturing pharmaceutical products and active pharmaceutical ingredients, including the manufacture of active pharmaceutical ingredients outside of the EU with the intention to import the active pharmaceutical ingredients into the EU. Amendments or replacements of Directive 2001/83/EC and Regulation (EC) No 726/2004 are part of the reform proposal for European pharmaceutical legislation.

Similarly, the distribution of pharmaceutical products into and within the EU is subject to compliance with the applicable EU laws, regulations and guidelines, including the requirement to hold appropriate authorizations for distribution granted by the competent authorities of the EU member states. The manufacturer or importer must have a qualified person who is responsible for certifying that each batch of product has been manufactured in accordance with GMP, before releasing the product for commercial distribution in the EU or for use in a clinical trial. Manufacturing facilities are subject to periodic inspections by the competent authorities for compliance with GMP.

Advertising and Promotion

The advertising and promotion of our products is also subject to EU laws concerning promotion of pharmaceutical products, interactions with physicians, misleading and comparative advertising and unfair commercial practices. In addition, other national legislation of individual EU member states may apply to the advertising and promotion of pharmaceutical products and may differ from one country to another. These laws require that promotional materials and advertising in relation to pharmaceutical products comply with the product's SmPC as approved by the competent regulatory authorities. The SmPC is the document that provides information to physicians concerning the safe and effective use of the pharmaceutical product. It forms an intrinsic and integral part of the marketing authorization granted for the pharmaceutical product. Promotion of a pharmaceutical product that does not comply with the SmPC is considered to constitute off-label promotion. All advertising and promotional activities for the product must be consistent with the approved SmPC and therefore all off-label promotion of pharmaceutical products is prohibited in the EU. Direct-to-consumer advertising of prescription-only pharmaceutical products is prohibited in the EU. Violations of the rules governing the promotion of pharmaceutical products in the EU could be penalized by administrative measures, fines and imprisonment. These laws may further limit or restrict the advertising and promotion of our products to the general public and may also impose limitations on its promotional activities with healthcare professionals.

Pricing and Reimbursement Environment

Even if a pharmaceutical product obtains a marketing authorization in the EU, there can be no assurance that reimbursement for such product will be secured on a timely basis or at all. The EU member states are free to restrict the range of pharmaceutical products for which their national health insurance systems provide reimbursement, and to control the prices and reimbursement levels of pharmaceutical products for human use. An EU member state may approve a specific price or level of reimbursement for the pharmaceutical product, or alternatively adopt a system of direct or indirect controls on the profitability of the company responsible for placing the pharmaceutical product on the market, including volume-based arrangements, caps and reference pricing mechanisms.

Reference pricing used by various EU member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. In some countries, we may be required to conduct a clinical study or other studies that compare the cost-effectiveness of our product candidates, if any, to other available therapies in order to obtain or maintain reimbursement or pricing approval. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products. Historically, pharmaceutical products launched in the EU do not follow price structures of the United States and generally published and actual prices tend to be significantly lower. Publication of discounts by third-party payers or authorities and public tenders may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries.

The so-called health technology assessment ("HTA") of pharmaceutical products is becoming an increasingly common part of the pricing and reimbursement procedures in some EU member states, including France, Germany, Ireland, Italy and Sweden. The HTA process, which is governed by the national laws of these countries, is the procedure according to which the assessment of the public health impact, therapeutic impact, and the economic and societal impact of use of a given pharmaceutical product in the national healthcare systems of the individual country is conducted. HTA generally focuses on the clinical efficacy and effectiveness, safety, cost, and cost-effectiveness of individual pharmaceutical products as well as their potential implications for the healthcare system. Those elements of pharmaceutical products are compared with other treatment options available on the market. The outcome of HTA regarding specific

pharmaceutical products will often influence the pricing and reimbursement status granted to pharmaceutical products by the regulatory authorities of individual EU member states. A negative HTA of one of our products by a leading and recognized HTA body could not only undermine our ability to obtain reimbursement for such product in the EU member state in which such negative assessment was issued, but also in other EU member states. For example, EU member states that have not yet developed HTA mechanisms could rely to some extent on the HTA performed in other countries with a developed HTA framework, when adopting decisions concerning the pricing and reimbursement of a specific pharmaceutical product.

On January 31, 2018, the European Commission adopted Regulation (EU) 2021/2282 on health technology assessment ("HTAR"). HTAR entered into force on January 11, 2022 and applies from January 12, 2025 onwards, followed by a further three-year transitional period during which EU member states must fully adapt to the new system. HTAR intends to boost EU level cooperation among EU member states in assessing health technologies, including new pharmaceutical products, and to provide the basis for cooperation at the EU level for joint clinical assessments in these areas. Under HTAR, EU member states will be able to use common HTA tools, methodologies and procedures across the EU, working together in four main areas: the joint clinical assessment of the innovative health technologies with the most potential impact for patients; joint scientific consultations whereby developers can seek advice from HTA authorities; identification of emerging health technologies to identify promising technologies early; and continuing voluntary cooperation in other areas. Individual EU member states will continue to be responsible for assessing non-clinical (*e.g.*, economic, social, ethical) aspects of health technology, and making decisions on pricing and reimbursement. While EU member states can choose to delay participation in the joint network until three years after the rules enter into force, it will become mandatory after six years. The European Commission has stated that the role of the HTA regulation is not to influence pricing and reimbursement decisions in the individual EU member states, but there can be no assurance that the HTA regulation will not have effects on pricing and reimbursement decisions.

To obtain reimbursement or pricing approval in some countries, including the EU member states, we may be required to conduct studies that compare the cost-effectiveness of our product candidates to other therapies that are considered the local standard of care. There can be no assurance that any country will allow favorable pricing, reimbursement and market access conditions for any of our products, or that we will be feasible to conduct additional cost-effectiveness studies, if required.

In certain EU member states, pharmaceutical products designated as orphan pharmaceutical products may be exempted or waived from having to provide certain clinical, cost-effectiveness and other economic data in connection with their filings for pricing/reimbursement approval.

On October 27, 2025, the Council of the European Union approved a framework for compulsory licensing of crisis-relevant products (including medicinal products) in crisis situations. While the proposal focuses on voluntary agreements with intellectual property rights holders, it includes rules on compulsory licensing as a measure of last resort upon declaration of a crisis or emergency. The European Parliament is yet to vote on the proposal.

Data Privacy and Security Laws

There are numerous U.S. federal, state, and local laws and regulations, as well as foreign legislation, in particular in the EU and UK, which regulate personal information, including how that information may be used, processed, and disclosed. These regulations also cover sensitive personal information, including medical and health information, and impose requirements on entities that handle such information to implement certain privacy and security measures. We and/or our partners may be subject to these laws.

In the United States, at the federal level, the Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 ("HITECH Act"), and the regulations promulgated thereunder, impose data privacy, security and data breach reporting obligations with respect to protected health information ("PHI") on covered entities—which include health plans, healthcare clearinghouses and certain healthcare providers—and business associates—which include persons or entities that perform certain functions or activities that involve the use or disclosure of PHI on behalf of, or in connection with providing a service for, a covered entity.

There are also a number of U.S. state privacy laws, such as the California Consumer Privacy Act of 2018 ("CCPA"), as amended by the California Privacy Rights Act of 2020 ("CPRA"), that govern the privacy and security of personal information in certain circumstances. The CCPA/CPRA applies to personal data of consumers (which is defined to include business representatives and employees) who are California residents, imposes obligations on certain businesses that do business in California, including to provide specific disclosures in privacy notices, and affords rights to California residents in relation to their personal information. Health information falls under the CCPA/CPRA's definition of personal information where it identifies, relates to, describes, is reasonably capable of being associated with or could reasonably be

linked, directly or indirectly, with a particular consumer or household and is considered "sensitive personal information," which is offered greater protection. However, the CCPA/CPRA, like other U.S. state privacy laws, does not apply to PHI, and most other U.S. state laws exempt covered entities and business associates altogether. Some of these laws and regulations impose different, and in certain instances, more stringent requirements than HIPAA. Failing to comply with these laws and regulations can result in significant civil and/or criminal penalties, as well as, in some cases, exposure to private litigation, all of which can result in financial and reputational risks.

The processing of personal data, including health-related personal data, in the EEA is mainly governed by the provisions of the European General Data Protection Regulation (EU) 2016/679 ("GDPR"), and by data protection related national laws, which supplement, interpret, and in some cases go beyond the GRDP. In the UK, the processing of personal data is mainly governed by the GDPR as incorporated into UK law pursuant to the European Union (Withdrawal) Act 2018 (the "UK GDPR"). Compliance with the GDPR and UK GDPR requirements has a number of significant practical consequences, including for international data transfers and enforcement of the GDPR by competent data protection authorities. The GDPR and UK GDPR imposed specific responsibility and liability in relation to personal data that we process, particularly when we act as a controller.

The GDPR and UK GDPR imposes a number of strict obligations and restrictions on the ability to process (processing means any operation or set of operations which is performed on personal data or on sets of personal data, whether or not by automated means, such as collection, storage, use and transfer of) personal data of individuals in the EEA, including health data from clinical trials and adverse event reporting. The GDPR and UK GDPR also includes requirements relating to the consent of the individuals to whom the personal data relates, the information provided to the individuals prior to processing their personal data (including health data), data breach notification obligations to the national data protection authorities and obligations relating to the security and confidentiality of the personal data. EEA countries may also impose additional requirements in relation to processing of health, genetic and biometric data through their national legislation.

Failure to comply with the requirements of the GDPR or UK GDPR and the related national data protection laws of the EEA countries may result in significant monetary fines for noncompliance of up to €20 million or 4% of the annual global revenues of the noncompliant company – whichever is greater. In addition, violations of national laws can trigger additional administrative penalties and investigations, corrective orders, temporary or definitive bans and, in some jurisdictions, a number of criminal offenses (punishable by capped or uncapped fines) for organizations and in certain cases their directors and officers as well as civil liability claims from individuals whose personal data was processed. Data protection authorities from the different EEA countries may still implement certain variations, enforce the GDPR and national data protection laws differently, and introduce additional national regulations and guidelines, which adds to the complexity of processing personal data in the EEA. Guidance developed at both EEA level and at the national level in individual EEA countries concerning implementation and compliance practices are often updated or otherwise revised.

There is, moreover, a growing trend towards required public disclosure of clinical trial data in the EU which adds to the complexity of obligations relating to processing health data from clinical trials. Such public disclosure obligations are provided in the EU Clinical Trials Regulation, EMA disclosure initiatives and voluntary commitments by industry. Failing to comply with these obligations could lead to government enforcement actions and significant penalties against us, harm to our reputation, and adversely impact our business and operating results. The uncertainty regarding the interplay between different regulatory frameworks, such as the Clinical Trials Regulation and the GDPR, further adds to the complexity that we face with regard to data protection regulation.

The GDPR also imposes specific restrictions on the transfer of personal data to countries outside of the EEA that are not considered by the European Commission to provide an adequate level of data protection. Appropriate safeguards are required to enable such transfers to countries outside the EEA that are not considered to provide an adequate level of data protection. Among the appropriate safeguards that can be used, the data exporter may use the standard contractual clauses ("SCCs"). In this respect, on June 4, 2021, the EU Commission issued a new set of SCCs which replace the old sets of SCCs that were adopted under the previous European Data Protection Directive 95/46. In addition, when relying on SCCs, the data exporters are required to conduct a transfer risk assessment to verify if anything in the law and/or practices of the third country may impinge on the effectiveness of the SCCs in the context of the transfer at stake and, if so, to identify and adopt supplementary measures that are necessary to ensure compliance with the EU level of protection of personal data. Where no supplementary measure is suitable, the data exporter should avoid, suspend or terminate the transfer. On June 18, 2021, the European Data Protection Board adopted recommendations to assist data exporters with such assessment and their duty to identify and implement supplementary measures where they are needed to ensure compliance with the EU level of protection to the personal data they transfer to third countries. With regard to the transfer of data from the EEA to the US, on July 10, 2023, the European Commission adopted its adequacy decision for the EU-US Data Privacy Framework. On the basis of the new adequacy decision, personal data can flow from the EEA to US companies participating in the Data Privacy Framework. With regard to the transfer of data from the EEA to the UK, based on the

European Commission's adequacy decision of June 28, 2021, personal data may now flow from the EU to the UK until June 27, 2025. In May 2025, the Commission adopted a decision to extend the validity of such adequacy decision for six more months, from June until December 2025. In July 2025, the Commission issued draft decisions on the extension of the validity of the UK adequacy decisions until December 2031 – if not adopted, as of December 2025, transfer of personal data from the EEA to the UK will be restricted and further measures will have to be implemented to comply with the GDPR. With respect to transfers from the UK to other countries, these transfers are also subject to specific transfer rules under the UK regime. These UK international transfer rules broadly mirror the EU GDPR rules. On February 2, 2022, the UK Secretary of State laid before the UK Parliament the international data transfer agreement (IDTA) and the international data transfer addendum to the European Commission's standard contractual clauses for international data transfers (Addendum) and a document setting out transitional provisions. The IDTA and Addendum came into force on March 21, 2022 and replaced the EU SCCs for the purposes of international transfers under the UK GDPR regime. With regard to the transfer of data from the UK to the US, the UK government has adopted an adequacy decision for the UK Extension to the EU-US Data Privacy Framework, the UK-US Data Bridge, which came into force on October 12, 2023. The UK-US Data Bridge recognizes the US as offering an adequate level of data protection where the transfer is to a US company listed on the EU-US Data Privacy Framework and participating in the UK Extension to the EU-US Data Privacy Framework.

Promotional Activities

In the EU, interactions between pharmaceutical companies and physicians are also governed by strict laws, regulations, industry self-regulation codes of conduct and physicians' codes of professional conduct both at EU level and in the individual EU member states. The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of pharmaceutical products is prohibited in the EU. The provision of benefits or advantages to physicians is also governed by the national anti-bribery laws of the EU member states. Violation of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain EU member states must be publicly disclosed. Moreover, agreements with physicians must often be the subject of prior notification and approval by the physician's employer, their regulatory professional organization, and/or the competent authorities of the individual EU member states. These requirements are provided in the national laws, industry codes, or professional codes of conduct, applicable in the individual EU member states. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

While the UK has left the EU, as mentioned above, it should be noted that the UK still has the strictest anti-bribery regime in Europe, the UK Bribery Act 2010. The Act is applicable English law and continues to apply to any company incorporated in or "carrying on business" in the UK, irrespective of where in the world the alleged bribery activity occurs.

Other Legislation Regarding Marketing, Authorization and Pricing of Pharmaceutical Products in the European Union

Other core legislation relating to the marketing, authorization and pricing of pharmaceutical products in the EU exists as regulations and directives, while the implementing acts and guidelines based on these may vary in each EU member state. In addition, the respective national provisions of the member states, as well as self-committed codes of the pharmaceutical industry, must be observed. Such regulations and directives include the following:

- Directive 2001/83/EC, establishing the requirements and procedures governing the marketing authorization for medicinal products for human use, as well as the rules for the constant supervision of products following authorization. This Directive has been amended several times, most recently by Directive 2012/26/EU regarding pharmacovigilance, and the Falsified Medicines Directive 2011/62/EU.
- Regulation (EC) 726/2004, as amended, establishing procedures for the authorization, supervision and pharmacovigilance of medicinal products for human and veterinary use and establishing the EMA.
- Regulation (EC) 469/2009, establishing the requirements necessary to obtain a Supplementary Protection Certificate, which extends the period of patent protection applicable to medicinal products at the EU-level.
- Directive 89/105/EEC, ensuring the transparency of measures taken by the EU member states to set the prices and reimbursements of medicinal products. Specifically, while each member state has competence over the pricing and reimbursement of medicines for human use, they must also comply with this Directive, which establishes procedures to ensure that member state decisions and policies do not obstruct trade in medicinal products. The European Commission proposed to repeal and replace Directive 89/105/EEC, but this proposal was withdrawn in 2015.
- Directive 2003/94/EC, laying down the principles of good manufacturing practice in respect of medicinal products and investigational medicinal products for human use (the "GMP Directive"); repealed by Directive

2017/1572 on January 31, 2022; this directive also lays out standards and principles for manufacturing practices of medicinal products for human use and investigational medicinal products for human use.

- Directive 2005/28/EC of April 8, 2005, laying down principles and detailed guidelines for good clinical practice as regards investigational medicinal products for human use, as well as the requirements for authorization of the manufacturing or importation of such products (the "GCP Directive").
- Directives 2004/9/EC and 2004/10/EC laying down principles of GLP including on the organizational process under which non-clinical health and safety studies are performed.
- Directive 2010/84/EU and Regulation (EU) 1235/2010 on pharmacovigilance laying down procedures for the authorization and supervision of medicinal products for human and veterinary use.
- Directive 2006/114/EC concerning misleading and comparative advertising.
- Directive 2005/29/EC regulating unfair business-to-consumer commercial practices that occur before, during and after a business-to-consumer transaction.
- Regulation (EC) 1223/2009 on Cosmetic Products, setting mandatory requirements for cosmetics which are available on the market within the EU.
- Regulation (EC) 1901/2006 on Pediatric Use, laying down rules to ensure that medicines for use in children are researched, developed and authorized appropriately.
- Directive (2004/109/EC) on Transparency laying down rules to improves the harmonization of information duties of issuers, whose securities are listed at a regulated market at a stock exchange within the EU; amended by Directive (EU) 2022/2464 with effect from May 1, 2023 as regards corporate sustainability reporting.

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of products approved by the FDA and other government authorities. Sales of products will depend, in part, on the extent to which the costs of the products will be covered by third-party payers, including government health programs such as, in the United States, Medicare and Medicaid, commercial health insurers and managed care organizations. The process for determining whether a payer will provide coverage for a product may be separate from the process for setting the price or reimbursement rate that the payer will pay for the product once coverage is approved. Third-party payers may limit coverage to specific products on an approved list, or formulary, which might not include all of the approved products for a particular indication.

In order to secure coverage and reimbursement for any product that might be approved for sale, a company may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of the product, in addition to the costs required to obtain FDA or other comparable regulatory approvals. A payer's decision to provide coverage for a drug product does not necessarily imply that an adequate reimbursement rate will be approved. Third-party reimbursement may not be sufficient to maintain price levels high enough to realize an appropriate return on our investment in product development.

The containment of healthcare costs has become a priority of federal, state and foreign governments, and the prices of drugs have been a focus in this effort. Third-party payers are increasingly challenging the prices charged for medical products and services and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. If these third-party payers do not consider a product to be cost effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to sell its products at a profit. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost containment programs to limit the growth of government-paid health care costs, including price controls, risk sharing, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. For example, in 2022, the U.S. government passed the Inflation Reduction Act ("IRA"), which authorizes the U.S. Department of Health and Human Services to negotiate prices of certain drugs with participating manufacturers in federal healthcare programs. In 2025, an executive order issued by the White House directed the Department of Health and Human Services (HHS) to implement a "Most Favored Nation" drug pricing policy; the federal Centers for Medicare and Medicaid Services has announced a pilot program in this regard for the Medicaid program. Adoption of these and other controls and measures and tightening of restrictive policies in jurisdictions with existing controls and measures, could limit payments for pharmaceuticals. As a result, the marketability of any product which receives regulatory approval for commercial sale may suffer if the government and third-party payers fail to provide adequate coverage and reimbursement.

In addition, an increasing emphasis on managed care in the United States has increased and will continue to increase the pressure on drug pricing. Coverage policies, third-party reimbursement rates and drug pricing regulation may change at any time. In particular, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education

Affordability Reconciliation Act, contains provisions that may reduce the profitability of drug products, including, for example, increased rebates for drugs sold to Medicaid programs, extension of Medicaid rebates to Medicaid managed care plans, mandatory discounts for certain Medicare Part D beneficiaries and annual fees based on pharmaceutical companies' share of sales to federal health care programs. Even if favorable coverage and reimbursement status is attained for one or more products that receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

In the EU, pricing and reimbursement schemes vary widely between member states. Some countries provide that drug products may be marketed only after a reimbursement price has been agreed. Some member states may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies. For example, the EU provides options for its member states to restrict the range of drug products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. EU member states may approve a specific price for a drug product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the drug product on the market. Other member states allow companies to fix their own prices for drug products but monitor and control company profits. The downward pressure on health care costs in general, particularly prescription drugs, has become intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert competitive pressure that may reduce pricing within a country. Any country that has price controls or reimbursement limitations for drug products may not allow favorable reimbursement and pricing arrangements for any of our products.

Healthcare Laws and Regulations

Healthcare providers, physicians and third-party payers play important roles in the recommendation and prescription of drug products that are granted marketing approval. Arrangements with healthcare providers, physicians, third-party payers and customers are subject to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which the Company markets, sells and distributes products for which it obtains marketing approval. Restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the federal Anti-Kickback Statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing any remuneration (in cash or in kind), directly or indirectly, to induce or reward either the referral of an individual for, or the purchase, lease, order or recommendation of, any item, facility or service for which payment may be made in whole or in part under a federal healthcare program such as Medicare and Medicaid;
- the federal Foreign Corrupt Practices Act prohibits, among other things, U.S. corporations and persons acting on their behalf from offering, promising, authorizing or making payments to any foreign government official (including certain healthcare professionals in many countries), political party, or political candidate in an attempt to obtain or retain business or otherwise seek preferential treatment abroad;
- the federal False Claims Act, which may be enforced by the U.S. Department of Justice or private whistleblowers who bring civil actions (qui tam actions) on behalf of the federal government, imposes civil penalties, as well as liability for treble damages and for attorneys' fees and costs, on individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent, making a false statement material to a false or fraudulent claim, or improperly avoiding, decreasing, or concealing an obligation to pay money to the federal government;
- the U.S. Department of Health and Human Services' Civil Monetary Penalty authorities, which imposes administrative sanctions for, among other things, presenting or causing to be presented false claims for government payment and providing remuneration to government health program beneficiaries to influence them to order or receive healthcare items or services;
- HIPAA imposes criminal and civil liability for, among other conduct, executing a scheme to defraud any healthcare benefit program and making false statements relating to healthcare matters;
- HIPAA, as amended by the HITECH Act and its implementing regulations, also imposes criminal and civil liability and penalties on those who violate requirements, including mandatory contractual terms, intended to safeguard the privacy, security, transmission and use of individually identifiable health information;
- the federal false statements statute relating to healthcare matters imposes criminal liability for knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;
- the federal Physician Payment Sunshine Act requires manufacturers of drugs (among other products) to report to the Centers for Medicare and Medicaid Services within the U.S. Department of Health and Human

Services information related to payments and other transfers of value to various healthcare professionals including physicians, physician assistants, nurse practitioners, clinical nurse specialists, certified nurse anesthetists, certified nurse-midwives and teaching hospitals, as well as physician ownership and investment interests in the reporting manufacturers;

- similar state and foreign laws and regulations, such as state anti-kickback and false claims laws, may apply (e.g., in the EU, where the implementation of EU-wide regulations as well as independent national legislation may vary for each EU member state) to sales or marketing arrangements and claims involving healthcare items or services reimbursed by nongovernmental third-party payers, including private insurers; and
- certain state laws require pharmaceutical companies to comply with voluntary compliance guidelines promulgated by a pharmaceutical industry association and relevant compliance guidance issues by the U.S. Department of Health and Human Services Office of Inspector General; bar drug manufacturers from offering or providing certain types of payments or gifts to physicians and other health care providers; and/or require disclosure of gifts or payments to physicians and other healthcare providers.

Various state and foreign laws also govern the privacy and security of health information in some circumstances; many of these laws differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

E. Facilities and Resources

The Company's principal executive offices are located in Pasadena, California. During the first quarter of fiscal 2025, the Company further expanded its footprint with a new manufacturing and laboratory facility to manufacture drug substance (API) under current Good Manufacturing Practices (GMP) in Verona, Wisconsin.

Research and Development Facilities

The Company operates research laboratory facilities in San Diego, California and Madison, Wisconsin, where its pre-clinical research and development activities, including the discovery and early development of RNAi therapeutics, take place. A summary is provided below:

- State-of-the-art laboratories with supporting office space that comprise more than 251,000 total square feet;
- Cell culture laboratories;
- Animal efficacy models for numerous diseases, including cardio metabolic, viral, liver, skeletal muscle, ocular, central nervous system (CNS), metabolic, renal, obesity and lung diseases;
- Animal safety screening and assessment;
- Clinical pathology laboratories and in-house histopathology and pathology evaluation capabilities;
- Integrated in-house expertise in clinical biomarker assay development and analytical evaluation;
- Advanced Artificial Intelligence-leveraged data science capabilities dedicated to analyzing human genetics databases for target discovery and validation;
- Drug metabolism and pharmacokinetics (DMPK), bioanalytical, biodistribution, and clearance assessment and methodology capabilities;
- Primate colony housed at the Wisconsin National Primate Research Center, an affiliate of the University of Wisconsin, and at other contract research organizations (CROs).
- Pharmacodynamic method development and analysis and translational biomarker development capabilities;
- Conventional and confocal microscopy, flow cytometry, Luminex platform, qRT-PCR and clinical chemistry analytics; and
- Oligonucleotide, peptide, antibody, and small molecule discovery, synthesis, production, and analytics capabilities (for example, HPLC, NMR, and LCMS).

GMP Manufacturing and Related Development Laboratory Facility

During the first quarter of fiscal 2025, the Company further expanded into a new, state-of-the-art GMP manufacturing facility in Verona, Wisconsin that includes related laboratories and office space to support chemistry, manufacturing, and controls (CMC) and quality activities. A summary is provided below:

- State-of-the-art, custom-designed GMP oligonucleotide manufacturing facility with related support laboratories for process development and analytical development, comprising approximately 300,000 total square feet;
- Full certificate of occupancy for laboratory, office & manufacturing spaces obtained August 2024;
- Full analytical chemistry capabilities including method development and validation, transfer of methods, and support of in-process and final product analysis;
- Drug product formulation development capabilities;
- In-house capabilities to release GMP drug substance and finished drug product;
- Multiple equipment scales for oligonucleotide manufacturing with maximum capacity to manufacture hundreds of kilograms of GMP drug substance annually; and
- Drug substance manufacturing capabilities to produce and release GMP material (API) and capabilities to release finished drug product pending ongoing commissioning, qualification, and validation (CQV) activities, allowing for the manufacture of GMP drug substance at the facility.

F. Human Capital Management

As of September 30, 2025, the Company employed 711 full-time employees based at four facilities in the United States, including Pasadena and San Diego, California, and Madison and Verona, Wisconsin.

In fiscal year 2025, the Company continued to expand its workforce, focusing on increasing in-house manufacturing capacity, as well as enhancing expertise and throughput in clinical and preclinical research and development and commercialization preparation. The Company continually evaluates the business need and opportunity and balances in-house expertise and capacity with outsourced expertise and capacity. Currently, the Company outsources substantial clinical trial work to clinical research organizations and certain drug manufacturing to contract manufacturers.

Drug development is a complex endeavor which requires deep expertise and experience across a broad array of disciplines. Pharmaceutical companies both large and small compete for a limited number of qualified applicants to fill specialized positions. To attract qualified applicants to the Company, it offers a total compensation package consisting of base salary and cash target bonus targeting the 50th to 75th percentile of market, and offers a comprehensive benefit package and equity compensation to every employee. Bonus opportunity and equity compensation increase as a percentage of total compensation based on level of responsibility. Actual bonus payout is based on performance.

A significant portion of the Company's employees have obtained advanced degrees in their professions. The Company supports its employees' further development with individualized development plans, mentoring, coaching, group training, conference attendance and financial support including tuition reimbursement.

Inclusion

The Company is dedicated to fostering a welcoming, healthy and equitable environment where all employees can thrive and contribute to its mission of delivering safe and effective medicine to patients in need. Ongoing efforts include programs and processes that promote awareness of inclusion and belonging, such as anti-bias training and employee engagement initiatives.

G. Investor Information

The Company's website address is http://www.arrowheadpharma.com. The Company's website address is not intended to function as a hyperlink and the information contained on its website is not, and should not be considered part of, and is not incorporated by reference into, this Annual Report on Form 10-K. The Company's reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), including its Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K, Proxy Statements, and amendments to such periodic reports and Proxy Statements, are accessible through its website, free of charge, as soon as reasonably practicable after these reports are filed electronically with, or otherwise furnished to, the SEC. These SEC reports can be accessed through the "Investors" section of the Company's website.

The SEC maintains an Internet website that contains reports, proxy and information statements, and other information regarding the Company and other issuers that file electronically with the SEC. The SEC's Internet website address is http://www.sec.gov.

ITEM 1A. RISK FACTORS

The Company's business involves various risks and uncertainties in addition to the normal risks of business, some of which are discussed in this section. It should be noted that the Company's business may be adversely affected by general economic conditions and other factors beyond the Company's control. In addition, other risks and uncertainties not presently known or that the Company currently believes to be immaterial may also adversely affect the Company's business. Some of the factors, events, and contingencies discussed below may have occurred in the past, but the disclosures below are not representations as to whether or not the factors, events, or contingencies have occurred in the past, and instead reflect our beliefs and opinions as to the factors, events or contingencies that could materially and adversely affect us in the future. Any such risks or uncertainties, or any of the following risks or uncertainties, that develop into actual events could result in a material and adverse effect on the Company's business, financial condition, results of operations, or liquidity.

The information discussed below should be considered carefully with the other information contained in this Annual Report on Form 10-K and the other documents and materials filed by the Company with the SEC, as well as news releases and other information publicly disseminated by the Company from time to time.

Risk Factors Summary

Risks Related to Our Discovery, Development, and Commercialization of Medicines

- Our prospects substantially depend on the success of our clinical-stage product candidates. If we and our licensees are unable to obtain approval for and commercialize these product candidates, or successfully commercialize REDEMPLO, our business could be materially harmed.
- There are substantial risks inherent in attempting to commercialize our new drugs, and, as a result, we may not be able to successfully develop additional products for commercial use.
- Our product candidates are in clinical development, which is a lengthy and expensive process with uncertain outcomes and the potential for substantial delays. There can be no assurance that our product candidates will obtain regulatory approval, which is necessary before they can be commercialized.
- Our clinical trials may not yield successful results for the product candidates that we may identify and pursue for their intended uses, which would prevent, delay or limit the scope of regulatory approval and commercialization.
- Our clinical trials may reveal significant adverse events, toxicities or other side effects and may result in a safety profile that could impede regulatory approval or market acceptance of any of our product candidates.
- Results of earlier studies or clinical trials may not be predictive of future clinical trial results, and initial studies or clinical trials may not establish an adequate safety or efficacy profile for our product candidates to justify proceeding to advanced clinical trials or an application for regulatory approval.
- We face potential product liability exposure, and if successful claims are brought against us, we may incur substantial liability from REDEMPLO, or any other approved product candidate, and may have to limit its commercialization.
- The successful commercialization of REDEMPLO, or any other product candidates, will depend in part on the extent to which government authorities and health insurers establish adequate reimbursement levels and pricing policies.
- Our commercialization, collaborative and other arrangements may give rise to disputes over commercial terms, contract interpretation, and ownership or protection of our intellectual property and may adversely affect the commercial success of our product candidates.

Risks Related to Regulatory Review and Approval of Our Candidates

- A Fast Track or Breakthrough Therapy product designation may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.
- We and our licensees conduct clinical trials for product candidates outside the United States, and the FDA and comparable foreign regulatory authorities may not accept data from such trials.
- Even if we obtain FDA approval for products in the United States, we may never obtain approval to commercialize any product candidates outside of the United States, which would limit our ability to realize their full market potential.
- If the FDA or comparable foreign regulatory authorities approve generic versions of REDEMPLO, or any other potential products that receive marketing approval, or such authorities do not grant REDEMPLO appropriate periods of data or market exclusivity before approving a generic version, the sales of REDEMPLO could be adversely affected.
- Failure to comply with regulatory requirements or unanticipated problems with our products may result in various adverse actions such as the suspension or withdrawal of one or more of our products, closure of a facility or enforcement of substantial penalties or fines.

- Pharmaceutical and biological product marketing is subject to substantial regulation in the U.S. and any failure by us or our commercial and collaborative partners to comply with applicable statutes or regulations can adversely affect our business.

Risks Related to Our Intellectual Property

- Our ability to protect our patents and other proprietary rights is uncertain, exposing us to the possible loss of competitive advantage.
- We are party to technology license agreements with third parties that require us to satisfy obligations to keep them effective and, if these agreements are terminated, our technology and our business could be seriously and adversely affected.
- We may be and have been subject to patent infringement claims, which could result in substantial costs and liability and prevent us from commercializing our potential products.

Risks Related to Our Business Model

- Our business model assumes we will generate revenue by, among other activities, marketing or out-licensing the products we develop. Our product candidates are in various stages of development and we have only one product based on RNAi and our delivery technologies approved in November 2025. Accordingly, there is a limited amount of information about us upon which you can evaluate our business and prospects.
- We may need to establish additional relationships with strategic and development partners to fully develop our product candidates and market any approved products.
- Our ability to generate milestone and royalty payments under our current and potential future licensing and collaboration agreements is substantially controlled by our partners, and as such, we will likely need other sources of financing to continue to develop our internal product candidates.
- We may lose a considerable amount of control over our intellectual property and may not receive anticipated revenues in strategic transactions, particularly where the consideration is contingent on the achievement of development or sales milestones.
- We will need to achieve commercial acceptance of REDEMPLO and our other product candidates to generate revenues and achieve profitability.
- If the market opportunities for REDEMPLO, or any other approved product candidates, are smaller than we expect, it could materially and adversely affect our financial condition and results of operations.
- We have limited manufacturing capability and capacity and must rely on third-party manufacturers to manufacture certain of our clinical supplies and our commercial products, if and when approved, and if they fail to meet their obligations, the development and commercialization of our products could be adversely affected.
- We rely on third parties to conduct our clinical trials, and if they fail to fulfill their obligations, the development of our products may be adversely affected.
- We may have difficulty expanding our operations successfully as we evolve our pipeline and move toward commercializing drugs.
- Because we use biological materials, hazardous materials, chemicals and radioactive compounds, if we do not comply with laws regulating the protection of the environment and health and human safety, our business could be adversely affected.
- Our operations, including our relationships with healthcare providers, physicians, and third-party payers are subject to applicable anti-kickback, fraud and abuse, and other healthcare laws and regulations, which, in the event of a violation, exposes us to liability for criminal sanctions, civil penalties, contractual damages, and reputational harm and diminished profits and future earnings.
- The actions of distributors and specialty pharmacies could affect our ability to sell or market products profitably. Fluctuations in buying or distribution patterns by such distributors and specialty pharmacies could adversely affect our revenues, financial condition, or results of operations.

Risks Related to Our Financial Condition

- We have a history of net losses, and we expect to continue to incur net losses and may not achieve or maintain profitability.
- We will require substantial additional funds to complete our research and development activities.
- The terms of our Sixth Street Financing Agreement and our indebtedness could adversely affect our operations and limit our ability to plan for or respond to changes in our business. If we are unable to comply with restrictions in our Sixth Street Financing Agreement, the repayment of our existing indebtedness could be accelerated.
- Our operating results may fluctuate significantly, which makes our future operating results difficult to predict and could cause our operating results to fall below expectations or our guidance.
- Our ability to utilize net operating loss carryforwards and other tax benefits may be limited.

Risks Related to Investment and Securities

- The market for purchases and sales of our common stock may be limited, and the sale of a limited number of shares could cause the price to fall sharply.
- Our common stock price has fluctuated significantly over the last several years and may continue to do so in the future, without regard to our results of operations and prospects.

Economic and Industry Risks

- Drug development is time consuming, expensive and risky.
- Evolving regulatory standards make it difficult to accurately predict the likelihood of marketing approval even when clinical trials meet their endpoints.

Risks Related to Our Discovery, Development, and Commercialization of Medicines

Our prospects substantially depend on the success of our clinical-stage product candidates. If we and our licensees are unable to obtain approval for and commercialize these product candidates, or successfully commercialize REDEMPLO, our business could be materially harmed.

Our future success is substantially dependent on the ability of the Company and our licensees to timely complete clinical trials and obtain marketing approval for, and then successfully commercialize our clinical-stage product candidates. We are not permitted to market or promote our product candidates before we receive marketing approval from the FDA and comparable foreign regulatory authorities, and we may never receive such marketing approvals.

The success of developing and commercializing our product candidates will depend on several factors, including the following:

- obtaining positive data that supports demonstration of efficacy, safety and tolerability profiles and durability of effect for our product candidates that are satisfactory to the FDA or any comparable foreign regulatory authority for marketing approval;
- successful inspections by FDA or any comparable foreign regulatory authority of our manufacturing facilities and clinical trial sites, including as part of the review process for marketing approval;
- successful and timely enrollment of appropriate patients for the indications included in our current and future clinical trials;
- potential variability of patient outcomes;
- the extent of any required post-marketing approval commitments, potentially including post-marketing clinical trials or other studies, to applicable regulatory authorities;
- the establishment of and maintenance of sufficient internal manufacturing capabilities;
- the maintenance of existing or the establishment of new supply arrangements with third-party drug product suppliers and manufacturers for clinical development and, if approved, commercialization of our product candidates;
- the maintenance of existing or the establishment of new scaled production arrangements with third-party manufacturers to obtain finished products that are appropriately packaged for sale;
- obtaining and maintaining patent protection, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- protecting our rights in our intellectual property portfolio, including our licensed intellectual property;
- establishing sales, marketing and distribution capabilities and the successful launch of commercial sales of our product candidates if and when approved for marketing, whether alone or in collaboration with others;
- a continued acceptable safety profile following any marketing approval;
- commercial acceptance by patients, the medical community and third-party payers; and
- our ability to compete with other therapies.

We do not have complete control over many of these factors, including certain aspects of clinical development and the regulatory submission process, potential threats to our intellectual property rights and the manufacturing, marketing, distribution and sales efforts of any collaborator or licensee. For development programs that are licensed to third parties, we generally do not have control over the design or conduct of clinical trials and will not have discretion over marketing decisions. If we are not successful with respect to one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize any product candidates from our lead programs, which would materially harm our business. If we do not receive marketing approvals for such product candidates, we may not be able to continue our operations.

There are substantial risks inherent in attempting to commercialize our new drugs, and, as a result, we may not be able to successfully develop additional products for commercial use.

Scientific research and development requires significant amounts of capital and even approved drug candidates take a long time to reach commercial viability if it can be achieved at all. During the research and development process, we may experience technological barriers that we may be unable to overcome. Because we use platform technology to develop drug candidates, toxicology signals that may emerge in the course of testing of one particular candidate may apply broadly across our drug candidate platform. Further, certain underlying premises in our development programs are not proven and many of the drug targets that we are pursuing have not yet been validated clinically. For instance, ARO-RAGE has demonstrated the ability to reduce the expression of RAGE in the lung, however it has not been established that this will have an anti-inflammatory effect sufficient for a meaningful clinical benefit in patients with inflammatory lung disease. Further, it is also unknown at this time what may be required to gain adequate reimbursement by either commercial or government payers. With respect to fazirsiran, it is also unknown at this time what changes in the liver may be required to gain regulatory approval and/or adequate reimbursement for a drug that reduces the production of mutant alpha-1 antitrypsin in the liver. Similar uncertainties and risks exist that are specific to each of our development programs. If we are unable to successfully develop commercial products, we will be unable to generate revenue or build a sustainable or profitable business.

Our product candidates are in clinical development, which is a lengthy and expensive process with uncertain outcomes and the potential for substantial delays. There can be no assurance that our product candidates will obtain regulatory approval, which is necessary before they can be commercialized.

The sale of human therapeutic products in the United States and foreign jurisdictions is subject to extensive and time-consuming regulatory approval which requires, among other things:

- controlled research and human clinical testing;
- establishment of the safety and efficacy of the product;
- government review and approval of a submission containing manufacturing, preclinical and clinical data; and
- adherence to cGMP regulations during production and storage.

Since 2011, we have focused substantially all of our efforts and financial resources on identifying, acquiring and developing our product candidates, including conducting lead optimization, nonclinical studies, preclinical studies and clinical trials, and providing general administrative support for these operations. And, the clinical-stage product candidates we currently have under development will require significant development, preclinical and clinical testing and investment of significant funds to gain regulatory approval before they can be approved for commercialization. The results of our research and human clinical testing of our products may not meet regulatory requirements. Some of our product candidates, if approved, may require the completion of post-market studies. There can be no assurance that any of our products will be further developed and approved. The process of completing clinical testing and obtaining required approvals will take several years and require the use of substantial resources. Further, there can be no assurance that product candidates employing a new technology will be shown to be safe and effective in clinical trials or receive applicable regulatory approvals. If we fail to obtain regulatory approvals for any or all of our products, we will not be able to market such product and our operations may be adversely affected.

Our clinical trials may not yield successful results for the product candidates that we may identify and pursue for their intended uses, which would prevent, delay or limit the scope of regulatory approval and commercialization.

We must demonstrate our product candidates' safety and efficacy in humans for each target indication through extensive clinical testing. We may experience numerous unforeseen events during, or as a result of, the testing process that could delay or prevent commercialization of any products, including the following:

- the results of preclinical studies may be inconclusive, or they may not be indicative of results that will be obtained in human clinical trials;
- safety and efficacy results attained in early human clinical trials may not be indicative of results that are obtained in later clinical trials;
- after reviewing test results, we may abandon projects that we might previously have believed to be promising;
- we or our regulators may suspend or terminate clinical trials because the participating subjects or patients are being exposed to unacceptable health risks; and
- our product candidates may not have the desired effects or may include undesirable side effects or other characteristics that preclude regulatory approval or limit their commercial use if approved.

We cannot be certain that current clinical trials or any future clinical trials, whether conducted by us or our licensees, will be successful. Additionally, any safety concerns observed in any one of our clinical trials in our targeted indications could limit the prospects for regulatory approval of our product candidates in those and other indications, which could have a material adverse effect on our business, financial condition and results of operation. Success in clinical trials in a particular indication does not ensure that a product candidate will be successful in other indications. Similarly, approval of a product candidate in a particular indication does not ensure that the product candidate will be successful in other indications. For instance, although REDEMPLO's Phase 3 PALISADE trial for patients with FCS was successful in achieving its primary endpoint and all multiplicity-controlled key secondary endpoints, and we have filed for regulatory approval with global regulatory authorities, there can be no guarantee that global regulatory authorities that have not already approved REDEMPLO for the treatment of FCS will do so, and REDEMPLO may not succeed in achieving its clinical trial endpoints or be approved for the treatment of larger indications such as sHTG because the endpoints and clinical data required for approval in a rare disease indication are different from what is required for a broader patient population. Moreover, results acceptable to support approval in one jurisdiction may be deemed inadequate by another regulatory authority to support regulatory approval in that other jurisdiction. To the extent that the results of the trials are not satisfactory to the FDA or comparable foreign regulatory authorities for support of a marketing application, we may be required to expend significant resources, which may not be available to us, to conduct additional trials in support of potential approval of our product candidates. Even if regulatory approval is secured for a product candidate, the terms of such approval may limit the scope and use of the specific product candidate, which may also limit its commercial potential.

Our clinical trials may reveal significant adverse events, toxicities or other side effects and may result in a safety profile that could inhibit regulatory approval or market acceptance of any of our product candidates.

In order to obtain marketing approval for any of our product candidates, we must demonstrate the safety and efficacy of the product candidate for the relevant clinical indication or indications through preclinical studies and clinical trials as well as additional supporting data. As is the case with pharmaceuticals generally, it is likely that there may be side effects and adverse events ("AEs") associated with the use of our products or product candidates. If our product candidates are associated with undesirable side effects in preclinical studies or clinical trials, or have unexpected characteristics, we may need to interrupt, delay or abandon their development or limit development to more narrow uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective.

If further significant adverse events or other side effects are observed in any of our current or future clinical trials, we may have difficulty recruiting patients to the clinical trials, patients may drop out of our trials, or we may be required to abandon the trials or our development efforts of that product candidate altogether. We, the FDA, the EMA, other applicable regulatory authorities or an institutional review board may suspend clinical trials of a product candidate at any time for various reasons, including a belief that subjects in such trials are being exposed to unacceptable health risks or adverse side effects. Some potential therapeutics developed in the biotechnology industry that initially showed therapeutic promise in early-stage studies have later been found to cause side effects that prevented their further development. Even if the side effects do not preclude the drug from obtaining or maintaining marketing approval, undesirable side effects may inhibit market acceptance of the approved product due to its tolerability relative to other therapies. Any of these developments could materially harm our business, financial condition and prospects.

Clinical trials of our product candidates may not uncover all possible adverse events that patients may experience.

Clinical trials are conducted in representative samples of the potential patient population, which may have significant variability. By design, clinical trials are based on a limited number of subjects and are of limited duration of exposure to the product, to determine whether the product candidate demonstrates the substantial evidence of efficacy and safety necessary to obtain regulatory approval. As with the results of any statistical sampling, we cannot be sure that all side effects of our product candidates may be uncovered. It may be the case that only with a significantly larger number of patients exposed to the product candidate for a longer duration may a more complete safety profile be identified. Further, even larger clinical trials may not identify rare significant AEs, and the duration of such studies may not be sufficient to identify when those events may occur. Other products have been approved by the regulatory authorities for which safety concerns have been uncovered following approval. Such safety concerns have led to labeling changes, restrictions on distribution through use of a REMS, or withdrawal of products from the market, and any of our product candidates may be subject to similar risks.

Although to date our current product candidates have generally evidenced an acceptable safety profile in clinical trials, patients treated with REDEMPLO or any of our product candidates, if approved, may experience previously unreported adverse reactions or minor incidences of adverse reactions may manifest with greater frequency in subsequent larger trials, and it is possible that the FDA or other regulatory authorities may ask for additional safety data as a condition of, or in connection with, our efforts to obtain approval of our product candidates. If toxicities, adverse events or any other

safety problems occur or are identified after our products reach the market, we may make the decision or be required by regulatory authorities to conduct additional clinical safety trials, amend the labeling of our products or add additional warnings to the labeling, recall our products, or even withdraw approval for our products.

Topline data may not accurately reflect the complete results of a particular study or trial.

We may publicly disclose topline or interim data from time to time, which is based on a preliminary analysis of then-available efficacy and safety data which are based on preliminary analysis of key efficacy and safety data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the topline results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Topline data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, topline data should be viewed with caution until the final data are available. Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimations, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular drug candidate or drug and the Company in general. In addition, the information we may publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure, and any information we determine not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular drug, drug candidate or our business. If the topline data that we report differ from a future analysis of results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for and commercialize our product candidates, our business, operating results, prospects or financial condition may be harmed.

Results of earlier studies or clinical trials may not be predictive of future clinical trial results, and initial studies or clinical trials may not establish an adequate safety or efficacy profile for our product candidates to justify proceeding to advanced clinical trials or an application for regulatory approval.

The results of nonclinical and preclinical studies and clinical trials may not be predictive of the results of later-stage clinical trials, and interim results of clinical trials do not necessarily predict final results. The results of preclinical studies and clinical trials in one set of patients or disease indications, or from preclinical studies or clinical trials that we did not lead, may not be predictive of those obtained in another. In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. In addition, preclinical and clinical data are often susceptible to various interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy profile despite having progressed through nonclinical studies and initial clinical trials. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in early-stage development, or after achieving positive results in pivotal trials, and we cannot be certain that we will not face similar setbacks. Even if early-stage clinical trials are successful, we may need to conduct additional clinical trials of our product candidates in additional patient populations or under different treatment conditions before we are able to seek approvals from the FDA and regulatory authorities outside the United States to market and sell these product candidates. Our failure to obtain marketing approval for our product candidates for commercially viable indications, or at all, would substantially harm our business, prospects, financial condition and results of operations.

It may take us longer than we project to complete clinical trials, and we may not be able to complete them at all.

Although for planning purposes we project the commencement, continuation and completion of our clinical trials, a number of factors, including scheduling conflicts with participating clinicians and clinical institutions, and difficulties in identifying or enrolling patients who meet trial eligibility criteria, may cause significant delays. Enrollment of clinical trials may be particularly difficult in orphan diseases or limited-sized patient populations. The FDA or other regulatory bodies may require additional, longer or broader clinical trials to establish safety and effectiveness, notwithstanding guidance the Company may have received from those bodies during clinical trial planning and execution. Further, the cost for conducting clinical trials is significant and if our cash resources become limited we may not be able to commence, continue and/or complete our clinical trials. We may not commence or complete clinical trials involving any of our product candidates as projected or may not conduct them successfully.

We face potential product liability exposure, and if successful claims are brought against us, we may incur substantial liability from REDEMPLO, or any other approved product candidate, and may have to limit its commercialization.

The use of our product candidates in clinical trials and the sale of REDEMPLO, or any other products for which we obtain marketing approval expose us to the risk of product liability claims. Product liability claims might be brought against us by clinical trial participants, consumers, healthcare providers, pharmaceutical companies, or others selling our products. If we cannot successfully defend ourselves against these claims, we may incur substantial liabilities. Regardless of merit or eventual outcomes of such claims, product liability claims may result in:

- decreased demand for our product candidates;
- impairment of our business reputation;
- withdrawal of clinical trial participants;
- costs of litigation;
- substantial monetary awards to patients or other claimants; and
- loss of revenues.

Our insurance coverage may not be sufficient to reimburse us for all expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive and, in the future, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses.

The successful commercialization of REDEMPLO, or any other approved product candidates, will depend in part on the extent to which government authorities and health insurers establish adequate reimbursement levels and pricing policies.

Sales of REDEMPLO, or any other approved drug candidate, will depend in part on the availability of coverage and reimbursement from third-party payers such as government insurance programs, including Medicare and Medicaid, private health insurers, health maintenance organizations and other health care related organizations, who are increasingly challenging the price of medical products and services. Accordingly, coverage and reimbursement may be uncertain. Adoption of any drug by the medical community may be limited if third-party payers will not offer adequate coverage. Additionally, significant uncertainty exists as to the reimbursement status of newly-approved drugs, including REDEMPLO. Cost control initiatives may decrease coverage and payment levels for any drug and, in turn, the price that we will be able to charge and/or the volume of our sales. We are unable to predict all changes to the coverage or reimbursement methodologies that will be applied by private or government payers. Any denial of private or government payer coverage or inadequate reimbursement could harm our business and reduce our revenue. With respect to our partnered product candidates, we will be reliant on that partner to obtain reimbursement from government and private payers for the drug, if approved, and any failure of that partner to establish adequate reimbursement could have a negative impact on our revenues and profitability.

In addition, both the federal and state governments in the United States and foreign governments continue to propose and pass new legislation, regulations, and policies affecting coverage and reimbursement rates, which are designed to contain or reduce the cost of health care. Further federal and state proposals and healthcare reforms are likely, which could limit the prices that can be charged for the product candidates that we develop and may further limit our commercial opportunity. For example, the IRA includes several measures intended to lower the cost of prescription drugs and related healthcare reforms, including limits on price increases and subjecting an escalating number of drugs to annual price negotiations with the Centers for Medicare & Medicaid Services ("CMS"). In addition, an executive order issued by the White House on May 12, 2025, directs the Department of Health and Human Services (the "HHS") to implement a "Most Favored Nation" drug pricing policy. See also "*The healthcare system is under significant financial pressure to reduce costs, which could reduce payment and reimbursement rates for drugs.*" And the recently-enacted One Big Beautiful Bill Act (the "OBBBA") imposes new restrictions on funding for government health care programs and on individual eligibility for coverage under those programs, which may lead to lower reimbursements for drugs covered by those programs. We cannot be sure whether additional legislation or rulemaking related to these developments will be issued or enacted, or what impact, if any, such changes will have on the profitability of any of our drug candidates, if approved for commercial use, in the future. There also may be future changes unrelated to these that result in reductions in potential coverage and reimbursement levels for our product candidates, if approved and commercialized, and we cannot predict the scope of any future changes or the impact that those changes would have on our operations.

If future reimbursement for approved product candidates, if any, is substantially less than we project, or rebate obligations associated with them are substantially greater than we expect, our future net revenue and profitability could be materially diminished.

We may not enjoy the market exclusivity benefits of our orphan drug designations.

Although we may obtain orphan designations in the treatment of certain diseases our products are intended to treat, the designation may not be applicable to any particular product we might get approved and that product may not be the first product to receive approval for that indication. Under the Orphan Drug Act, the first approved product with an orphan designation receives market exclusivity, which prohibits the FDA from approving the "same" drug for the same indication. The FDA has stated that drugs can be the "same" even when they are not identical but has not provided guidance with respect to how it will determine "sameness" for RNAi drugs. It is possible that another RNAi drug could be approved for the treatment of a disease that one of our orphan products is intended to treat before our product is approved, which means that we may not obtain orphan drug exclusivity and could also potentially be blocked from approval until the first product's orphan drug exclusivity period expires or we demonstrate, if we can, that our product is superior. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved and granted orphan drug exclusivity, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. In addition, a designated Orphan Drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the disease for which it received orphan designation. Recent court decisions have created uncertainty in the application and interpretation of orphan drug exclusivity. Further, orphan drug exclusivity can be lost if the FDA later determines that the request for designation was materially defective or if the applicant is unable to assure the availability of sufficient quantities of the drug to meet the needs of patients with the disease or condition for which the drug was designated. Orphan Drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

Our success depends on the attraction and retention of senior management and scientists with relevant expertise.

Our future success depends to a significant extent on the continued services of our key employees, including our senior scientific, technical and managerial personnel. We do not maintain key person life insurance for any of our executives and we do not maintain employment agreements with many senior employees. Competition for qualified employees in the pharmaceutical industry is high, and our ability to execute our strategy will depend in part on our ability to continue to attract and retain qualified scientists, management and other employees. This will depend in part on our ability to create and maintain a desirable workplace culture, which may be impacted by employee preferences for remote working. In addition, the market for qualified employees in the pharmaceutical industry is experiencing labor shortages and inflationary pressures are causing salaries and wages to increase, all of which exacerbates these competitive dynamics. If we are unable to find, hire and retain qualified individuals, we will have difficulty implementing our business plan in a timely manner, or at all.

Our commercialization, collaborative and other arrangements may give rise to disputes over commercial terms, contract interpretation and ownership or protection of our intellectual property and may adversely affect the commercial success of our product candidates.

We have in the past and may again in the future enter into collaboration or license arrangements, including commercialization or collaborative arrangements, some of which may be based on less definitive agreements, such as memoranda of understanding, material transfer agreements, options or feasibility agreements.

Commercialization and collaborative relationships are generally complex and can give rise to disputes regarding the relative rights, obligations and revenues of the parties, including the ownership of intellectual property and associated rights and obligations, especially when the applicable collaborative provisions have not been fully negotiated and documented. Such disputes have arisen in the past from time to time and, if they arise again could delay collaborative research, development or commercialization of potential product candidates, and can lead to lengthy, expensive litigation or arbitration. The terms of such arrangements may also limit or preclude us from commercializing products or technologies developed pursuant to such collaborations. Additionally, the commercialization or collaborative partners under these arrangements might breach the terms of their respective agreements or fail to maintain, protect or prevent infringement of the licensed patents or our other intellectual property rights by third parties. Moreover, negotiating commercialization and collaborative arrangements often takes considerably longer to conclude than the parties initially anticipate, which could cause us to enter into less favorable agreement terms that delay or defer recovery of our development costs and reduce the funding available to support key programs. Any failure by our commercialization or collaborative partners to abide by the terms of their respective agreements with us (including their failure to accurately calculate, report or pay any royalties payable to either us or a third party or their failure to repay, in full or in part, either any outstanding receivables or any other amounts for which we are entitled to reimbursement) may adversely affect our results of operations.

We are not always able to enter into commercialization or collaborative arrangements on acceptable terms, which can harm our ability to develop and commercialize our current and potential future products and technologies. Other factors relating to collaborations that may adversely affect the commercial success of our product candidates include:

- any parallel development by a commercialization or collaborative partner of competitive technologies or products;
- arrangements with commercialization or collaborative partners that limit or preclude us from developing products or technologies;
- premature termination of a commercialization or collaboration agreement or the inability to renegotiate existing agreements on favorable terms; or
- failure by a commercialization or collaborative partner to devote sufficient resources to the development and commercial sales of products using our current and potential future products and technologies.

Our commercialization or collaborative arrangements do not necessarily restrict our commercialization or collaborative partners from competing with us or restrict their ability to market or sell competitive products. Our current and any future commercialization or collaborative partners may pursue existing or other development-stage products or alternative technologies in preference to those being commercialized or developed in collaboration with us.

In addition, contract disputes with customers or other third parties may arise from time to time. Our commercialization or collaborative partners, or customers or other third parties, may also terminate their relationships with us or otherwise decide not to proceed with the development, commercialization or purchase of our product candidates.

Risks Related to Regulatory Review and Approval of Our Product Candidates

Breakthrough Therapy designation for Fazirsiran (formerly ARO-AAT) and other of our current or future product candidates and other of our current or future product candidates may not lead to a faster development or review process.

We have been granted a Breakthrough Therapy designation for fazirsiran in the United States for the treatment of liver disease associated with AATD, and we may seek Breakthrough Therapy designation for other current or future product candidates. Breakthrough Therapy designation is intended to facilitate the development and expedite the review of new therapies to treat serious conditions with unmet medical needs by providing sponsors with the opportunity for frequent interactions and additional drug development guidance with the FDA and its senior managers. Breakthrough Therapy designation applies to the combination of the product candidate and the specific indication for which it is being studied. Product candidates that receive Breakthrough Therapy designation may receive more frequent interactions with the FDA regarding the product candidate's development plan and clinical trials and may be eligible for the FDA's Rolling Review.

Despite receiving Breakthrough Therapy designation, fazirsiran or other product candidates may not actually benefit from faster clinical development or regulatory review or approval any sooner than other product candidates that do not have such designation, or at all. For example, although we received Breakthrough Therapy designation for REDEMPLO in the United States for the treatment of FCS, REDEMPLO was ultimately reviewed on a standard timeline. Furthermore, such a designation does not increase the likelihood that fazirsiran or other product candidates will receive marketing approval in the United States. The FDA may also rescind Breakthrough Therapy designation if it determines that fazirsiran or other product candidates no longer meets the relevant criteria.

A Fast Track product designation may not lead to faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We have received a Fast Track product designation for fazirsiran in the United States for the treatment of liver disease associated with AATD, and we may seek Fast Track designation for other of our current or future product candidates. The Fast Track designation is a program offered by the FDA designed to facilitate drug development and to expedite the review of new drugs that are intended to treat serious or life-threatening conditions. Compounds selected must demonstrate the potential to address unmet medical needs. The FDA's Fast Track designation allows for close and frequent interaction with the FDA. A designated Fast Track drug may also be considered for priority review with a shortened review time, rolling submission, and accelerated approval if applicable.

A Fast Track designation does not, however, guarantee FDA approval or expedited approval of any application for the product candidate. The receipt of such a designation for a product candidate may not result in a faster development process, review, or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate marketing approval by the FDA. For example, although we received Fast Track designation for REDEMPLO in the United States for the treatment of FCS, REDEMPLO was ultimately reviewed on a standard timeline. In addition, the FDA may later decide that the products no longer meet the designation conditions.

We may seek priority review designation for one or more of our product candidates, but we might not receive such designation, and even if we do, such designation may not lead to faster development or regulatory review or approval process.

If the FDA determines that a product candidate offers a treatment for a serious condition and, if approved, the product would provide a significant improvement in safety or effectiveness, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months from the filing date, rather than the standard review period of ten months. We may request priority review for our product candidates. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster development or regulatory review or approval process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or at all.

We intend to deliver some of our product candidates via drug delivery devices that will have their own regulatory, development, supply and other risks.

We intend to deliver some of our product candidates via drug delivery devices, such as an autoinjector or nebulizer. There may be unforeseen technical complications related to the development activities required to bring such a product to market, including container compatibility and/or dose volume requirements. If our product candidates are intended to be used with drug delivery devices, we expect to utilize drug delivery devices authorized for marketing under clearances of approvals held by third parties. Our product candidates may not be approved or may be substantially delayed in receiving approval if the devices do not gain and/or maintain their own regulatory approvals or clearances. Where approval of the drug product and device is sought under a single application, the increased complexity of the review process may delay approval. In addition, some drug delivery devices are provided by single-source unaffiliated third-party companies. We may be dependent on the sustained cooperation and effort of those third-party companies both to supply the devices and, in some cases, to conduct the studies required for approval or other regulatory clearance of the devices. Even if approval is obtained for our products, we may also be dependent on those third-party companies continuing to maintain such approvals or clearances, if required, for their drug delivery devices once they have been received. Failure of third-party companies to supply the devices, to successfully complete studies on the devices in a timely manner, or to obtain or maintain required approvals or clearances of the devices could result in increased development costs, delays in or failure to obtain regulatory approval and delays in product candidates reaching the market or in gaining approval or clearance for expanded labels for new indications.

We and our licensees conduct clinical trials for product candidates outside the United States, and the FDA and comparable foreign regulatory authorities may not accept data from such trials.

We and our licensees currently conduct clinical trials outside the United States. The acceptance by the FDA or comparable foreign regulatory authority of study data from clinical trials conducted outside the United States or another jurisdiction may be subject to certain conditions or may not be accepted at all. In cases where data from foreign clinical trials are intended to serve as the basis for marketing approval in the United States, the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the U.S. population and U.S. medical practice; (ii) the trials were performed by clinical investigators of recognized competence and pursuant to GCP regulations; and (iii) the data may be considered valid without the need for an on-site inspection by the FDA or, if the FDA considers such as inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means. Additionally, the FDA's clinical trial requirements, including sufficient size of patient populations and statistical powering, must be met. Many foreign regulatory authorities have similar approval requirements. In addition, such foreign trials would be subject to the applicable local laws of the foreign jurisdictions where the trials are conducted. There can be no assurance that the FDA or any comparable foreign regulatory authority will accept data from trials conducted outside of the United States or the applicable jurisdiction. Most of our clinical trials involve study subjects outside of the United States, including most of our Phase 1 clinical trials (which often enroll study subjects in Australia and New Zealand), and our Phase 3 clinical trials of plozasiran and zodasiran, for which we have enrolled and plan to enroll cohorts outside the United States. If the FDA or any comparable foreign regulatory authority does not accept such data, it would result in the need for additional trials, which would be costly and time-consuming and delay aspects of our business plan, and which may result in product candidates that we may develop not receiving approval or clearance for commercialization in the applicable jurisdiction.

Even if we obtain FDA approval for products in the United States, we may never obtain approval to commercialize any product candidates outside of the United States, which would limit our ability to realize their full market potential.

In order to market any products outside of the United States, we must establish and comply with numerous and varying regulatory requirements of other countries regarding safety and effectiveness, and the chemistry, manufacturing and controls for the product. Clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not mean that regulatory approval will be obtained in any other country. Approval processes and requirements vary among countries and can involve additional product testing and validation and additional or different administrative review periods from those in the United States, including additional preclinical studies or clinical trials. In many jurisdictions outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our products is also subject to approval before a product can be marketed in that jurisdiction, even after establishing safety and efficacy in a clinical setting.

Seeking foreign regulatory approval could result in difficulties and costs and require additional nonclinical studies or clinical trials which could be costly and time-consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our product candidates in those countries. The foreign regulatory approval process may include all of the risks associated with obtaining FDA approval. We do not have any product candidates approved for sale in international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approval in international markets is delayed, our target market will be reduced and our ability to realize the full market potential of our products will be harmed.

If the FDA or comparable foreign regulatory authorities approve generic versions of REDEMPLO, or any other potential products that receive marketing approval, or such authorities do not grant REDEMPLO appropriate periods of data or market exclusivity before approving a generic version, the sales of REDEMPLO could be adversely affected.

Once an NDA is approved, the drug covered thereby becomes a "reference-listed drug" in the FDA's publication, "Approved Drug Products with Therapeutic Equivalence Evaluations." Manufacturers may seek marketing approval of generic versions of reference-listed drugs through submission of ANDAs in the United States. Generic drugs may be significantly less costly to bring to market than the reference-listed drug and companies that produce generic drugs are generally able to offer them at lower prices. Thus, following the introduction of a generic drug, a significant percentage of the sales of any branded product or reference-listed drug is typically lost to the generic drug.

The FDA may not approve an ANDA or a 505(b)(2) NDA for a generic drug until any applicable period of non-patent exclusivity for the reference-listed drug has expired. The FDCA provides a period of five years of non-patent exclusivity for a new drug containing a new chemical entity. During the exclusivity period, the FDA may not accept for review an ANDA or a 505(b)(2) NDA submitted by another company for another version of such product candidate where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement. The FDCA also provides three years of marketing exclusivity for an NDA, 505(b)(2) NDA or supplement to an approved NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example, for new indications, dosages or strengths of an existing product candidate. This three-year exclusivity covers only the conditions associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for product candidates containing the original active agent for other conditions of use. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the nonclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness. Manufacturers may seek to launch generic drugs following the expiration of the marketing exclusivity period, even if we still have patent protection for such drugs.

Competition that REDEMPLO, or any other potential product candidates, may face from generic drugs could materially and adversely impact our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on the investments we have made in those products. Our future revenues, profitability and cash flows could also be materially and adversely affected and our ability to obtain a return on the investments we have made in REDEMPLO or other product candidates may be substantially limited if REDEMPLO, or any other potential products, product candidates, are not afforded the appropriate periods of non-patent exclusivity.

Failure to comply with regulatory requirements or unanticipated problems with our products may result in various adverse actions such as the suspension or withdrawal of one or more of our products, closure of a facility or enforcement of substantial penalties or fines.

Regulatory agencies subject any marketed product(s), such as REDEMPLO, and the facilities where they are manufactured to continual review and periodic inspection. If previously unknown problems with a product, manufacturing and laboratory facilities or regulatory requirements are discovered, such as AEs of unanticipated severity or frequency,

problems with a manufacturing process or laboratory facility, or failure to comply with applicable regulatory approval requirements, a regulatory agency may impose restrictions or penalties on that product or on us. Such restrictions or penalties may include, among other things:

- restrictions on the marketing or manufacturing of the product, the withdrawal of the product from the market or product recalls;
- warning, untitled, or it has come to our attention letters, or holds on clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or suspension or revocation of approvals;
- product seizure or detention, or refusal to permit the import or export of our product candidates; and
- closure of the facility, enforcement of substantial fines, injunctions, or the imposition of civil or criminal penalties.

Pharmaceutical and biological product marketing is subject to substantial regulation in the U.S. and any failure by us or our commercial and collaborative partners to comply with applicable statutes or regulations can adversely affect our business.

Any marketing activities associated with REDEMPLO, or any other product candidates approved for commercialization, will be subject to numerous federal and state laws governing the marketing and promotion of pharmaceutical and biological products. The FDA regulates post-approval promotional labeling and advertising to ensure that they conform to statutory and regulatory requirements. In addition to FDA restrictions, the marketing of prescription drugs is subject to laws and regulations prohibiting fraud and abuse under government healthcare programs. Similarly, many states have similar statutes or regulations that apply to items and services reimbursed under Medicaid and other state programs, and, in some states, such statutes or regulations apply regardless of the payer. In addition, government authorities may also seek to hold us responsible for any failure of our commercialization or collaborative partners to comply with applicable statutes or regulations. If we, or our commercial or collaborative partners, fail to comply with applicable FDA regulations or other laws or regulations relating to the marketing of REDEMPLO, or any other approved product candidates, we could be subject to criminal prosecution, civil penalties, seizure of products, injunctions and exclusion of our product candidates from reimbursement under government programs, as well as other regulatory or investigatory actions against our future product candidates, our commercial or collaborative partners or us. See also "*Our operations, including our relationships with healthcare providers, physicians and third-party payers, are subject to applicable anti-kickback, fraud and abuse, and other healthcare laws and regulations, which, in the event of a violation, exposes us to liability for criminal sanctions, civil penalties, and contractual damages, and reputational harm and diminished profits and future earnings.*"

Risks Related to Our Intellectual Property

Our ability to protect our patents and other proprietary rights is uncertain, exposing us to the possible loss of competitive advantage.

We have licensed rights to patents and have filed and expect to continue to file patent applications. Researchers sponsored by us may also file patent applications that we may need to license. Such patent applications may not be available for licensing or may not be economically feasible to license. Certain of our patents may not be granted or may not contain claims of the necessary breadth because, for example, prior patents or publications exist. If a particular patent is not granted, the value of the invention described in the patent would be diminished. Further, even if these patents are granted, they may be difficult to enforce. Patent prosecution and maintenance is expensive, and we may be forced to curtail prosecution or maintenance if our cash resources are limited. Thus, the patents held by or licensed to us may not afford us any meaningful competitive advantage. Even if ultimately successful in obtaining patent protection, efforts to enforce our patent rights could be expensive, distracting for management, cause our patents to be invalidated or held unenforceable, and thus frustrate commercialization of products. Even if patents are issued and are enforceable, others may develop similar, superior or parallel technologies to any technology developed by us and not infringe on our patents. Additionally, our technology may be accused of infringing and may ultimately prove to infringe upon patents or rights owned by others. The Company may be subject to intellectual property litigation that could negatively impact our ability to commercialize REDEMPLO and our product candidates, if approved. For example, on September 10, 2025, the Company filed a Complaint for Declaratory Judgment in the United States District Court for the District of Delaware against Ionis Pharmaceuticals, Inc. ("Ionis") to declare that the United States Patent No. 9,593,333 ("the '333 patent") is invalid and not infringed by the Company's planned commercialization of investigational plozasiran, and on September 11, 2025, Ionis filed a Complaint for Patent Infringement against the Company in the United States District Court for the Central District of California alleging patent infringement of the '333 patent. In addition, the laws of some foreign countries in which we do business, including through our joint ventures, do not protect intellectual property rights to the same extent or in the

same manner as the laws of the United States. Moreover, if we or our licensors fail to maintain the patents and patent applications covering our product candidates or technologies, including as a result of geopolitical events such as civil or political unrest (including the ongoing conflicts between Ukraine and Russia and Israel and Palestine), we may not be able to use such patents and patent applications or stop a competitor from marketing products that are the same as or similar to our product candidates. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. If we are unable to adequately protect our owned intellectual property or derive sufficient value from our licensed or owned intellectual property, the value of your investment may decline.

In addition, patent grant standards by the USPTO and its foreign counterparts are not always uniform or predictable, and subject to change. For example, the America Invents Act enacted a number of changes to U.S. patent laws, which may prevent us from adequately protecting our inventions and discoveries, including our ability to seek injunctive relief, pursue infringement claims, and obtain substantial damage awards. An example of a major provision of the America Invents Act is the change in the U.S. patent policy from a first-to-invent to a first-to-file practice. Additionally, the USPTO and patent offices in other jurisdictions have often required that patent applications directed to pharmaceutical and/or biotechnology-related inventions be limited or narrowed substantially to cover only the specific innovations exemplified in the patent application, thereby limiting the scope of protection against competitive challenges. Accordingly, even if we or our licensors are able to obtain patents, the patents might be substantially narrower than anticipated. Thus, there is no assurance as to the degree and range of protections any of our patents, if issued, may afford us or whether patents will be issues. Foreign counterparts to this law are also not uniform, and there is no worldwide policy governing the subject matter and scope of claims granted in a pharmaceutical or biotechnology patent. Uncertainty, arising from changing laws, can impact our ability to protect our patents and other proprietary rights.

We are party to technology license agreements with third parties that require us to satisfy obligations to keep them effective and, if these agreements are terminated, our technology and our business could be seriously and adversely affected.

We are party to license agreements to incorporate third-party proprietary technologies into our drug products under development or our manufacturing processes. These license agreements require us to pay royalties and satisfy other conditions. If we fail to satisfy our obligations under these agreements, the terms of the licenses may be materially modified, such as by rendering currently exclusive licenses non-exclusive, or may give our licensors the right to terminate their respective agreement with us, which could limit our ability to implement our current business plan and harm our business and financial condition.

We may be and have been subject to patent infringement claims, which could result in substantial costs and liability and prevent us from commercializing our potential products.

Because the intellectual property landscape in the fields in which we participate is rapidly evolving and interdisciplinary, it is difficult to conclusively assess our freedom to operate without infringing on third-party rights. However, if granted marketing approval, we are currently aware of certain patent rights held by third parties that, if found to be valid and enforceable, could be alleged to render one or more of our drug products or candidates infringing. For example, as the Company previously reported in Form 8-K filed on September 12, 2025, Ionis Pharmaceuticals, Inc. (“Ionis”) filed a complaint for patent infringement against the Company in the United States District Court for the Central District of California alleging patent infringement of United States Patent No. 9,593,333 (“the ’333 patent”) (the "Ionis Claim"), which may impact our planned commercialization of REDEMPLO. See also “*Our ability to protect our patents and other proprietary rights is uncertain, exposing us to the possible loss of competitive advantage.”* If the Ionis Claim or any potential future claim by another party is successful, we may be required to pay substantial damages, be forced to abandon any affected drug product and/or product candidates and/or seek a license from the patent holder. In addition, the Ionis Claim and any future patent infringement claims brought against us, whether or not successful, may cause us to incur significant expenses and divert the attention of our management and key personnel from other business concerns. These could negatively affect our results of operations and prospects. We cannot be certain that patents owned or licensed by us will not be challenged, potentially successfully, by others.

In addition, if our products and/or product candidates are found to infringe the intellectual property rights of third parties, these third parties may assert infringement claims against our customers, licensees and other parties with whom we have business relationships, and we may be required to indemnify those parties for any damages they suffer as a result of these claims. The claims may require us to initiate or defend protracted and costly litigation on behalf of customers, licensees, and other parties regardless of the merits of these claims. If any of these claims succeed, we may be forced to pay damages on behalf of those parties or may be required to obtain licenses for the products they use. If we cannot obtain all necessary licenses on commercially reasonable terms, we may be unable to continue selling such products.

We license patent rights from third-party owners and we rely on such owners to obtain, maintain and enforce the patents underlying such licenses.

We are a party to a number of licenses that give us rights to third-party intellectual property that is necessary or useful for our business. We also expect to enter into additional licenses to third-party intellectual property in the future.

Our success may depend in part on the ability of our licensors to obtain, maintain and enforce patent protection for our licensed intellectual property, in particular, those patents to which we have secured exclusive rights. Our licensors may not successfully prosecute the patent applications to which we are licensed. Even if patents are issued in respect of these patent applications, our licensors may fail to maintain these patents, may determine not to pursue litigation against other companies that are infringing these patents, or may pursue such litigation less aggressively than we would. Without protection for the intellectual property we license, other companies might be able to offer substantially identical products for sale, which could adversely affect our competitive business position and harm our business prospects.

Our technology licensed from various third parties may be subject to retained rights.

Our licensors often retain certain rights under their agreements with us, including the right to use the underlying technology for noncommercial academic and research use, to publish general scientific findings from research related to the technology, and to make customary scientific and scholarly disclosures of information relating to the technology. It is difficult to monitor whether our licensors limit their use of the technology to these uses, and we could incur substantial expenses to enforce our rights to our licensed technology in the event of misuse.

Confidentiality agreements with employees and others may not adequately prevent disclosure of trade secrets and other proprietary information.

In order to protect our proprietary technology and processes, we rely in part on confidentiality agreements with our collaborators, employees, consultants, outside scientific collaborators and sponsored researchers, and other advisors. These agreements may not effectively prevent disclosure of confidential information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. As our organization grows, so does the risk of unauthorized disclosure of confidential information. In addition, while we undertake efforts to protect our trade secrets and other confidential information from disclosure, others may independently discover trade secrets and proprietary information, and in such cases, we may not be able to assert any trade secret rights against such party. Enforcing a claim that a party illegally obtained and is using our trade secrets is challenging and the outcome is unpredictable. In addition, courts outside of the U.S. may be less willing to protect trade secrets. Costly and time-consuming litigation could be necessary to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

We may not be able to effectively secure first-tier technologies when competing against other companies or investors.

Our future success may require that we acquire patent rights and know-how to new or complimentary technologies. However, we also compete with a substantial number of other companies that are working to develop novel drugs using technology that compete directly with us. We are aware of several other companies that are working to develop RNAi therapeutic products and any one of these companies may develop its RNAi technology more rapidly and more effectively than us may also compete for technologies we desire. In addition, many venture capital firms and other institutional investors, as well as other pharmaceutical and biotech companies, invest in companies seeking to commercialize various types of emerging technologies. Many of these companies have greater financial, scientific and commercial resources than us. Therefore, we may not be able to secure the technologies we desire or to otherwise effectively compete. Furthermore, should any commercial undertaking by us prove to be successful, there can be no assurance competitors with greater financial resources will not offer competitive products and/or technologies.

We may not be able to protect our intellectual property rights throughout the world, which could negatively impact our business.

Filing, prosecuting and defending patents covering our current and any future product candidates in all countries throughout the world would be prohibitively expensive. Competitors may use our technologies in jurisdictions where we or our licensors have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we may obtain patent protection but where patent enforcement is not as strong as that in the United States. These products may compete with our products in jurisdictions where we do not have any issued or licensed patents, and any future patent claims or other intellectual property rights may not be effective or sufficient to prevent them from competing. Issued patents may be challenged by third parties in the courts or patent offices in various countries throughout the world. Invalidation proceedings may result in patent claims being narrowed, invalidated or held unenforceable. Uncertainties regarding the outcome of such proceedings, as well as any resulting losses of patent protection, could harm our business.

Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. Some countries do not enforce patents related to medical treatments, or limit enforceability in the case of a public emergency. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

The intellectual property systems in other countries can be destabilized or unpredictable as a result of geopolitical events such as civil or political unrest (including the ongoing conflicts between Ukraine and Russia and Israel and Palestine). Therefore, during such geopolitical events, the ability to obtain, retain and enforce intellectual property protection in the affected countries may be uncertain and evolve during the course of such geopolitical event. The U.S. government's response to geopolitical events may also negatively affect our ability to obtain, retain and enforce intellectual property protection in the affected countries. Uncertainties regarding geopolitical events, as well as any resulting losses of intellectual property protection, could harm our business.

Risks Related to Our Business Model

Our business model assumes we will generate revenue by, among other activities, marketing or out-licensing the products we develop. Our product candidates are in various stages of development and we currently have only one product based on RNAi and our delivery technologies which was approved in November 2025. Accordingly, there is a limited amount of information about us upon which you can evaluate our business and prospects.

We have not begun to generate revenues from the commercialization of REDEMPLO or any product candidates. As such, we have not yet demonstrated an ability to successfully overcome many of the risks and uncertainties frequently encountered by companies in new and rapidly evolving fields, particularly in the biopharmaceutical area. For example, to execute our business plan, we will need to successfully:

- execute product development activities using technologies that have not yet generated revenues;
- build, maintain, and protect a strong intellectual property portfolio;
- demonstrate safety and efficacy of our product candidates in multiple human clinical studies;
- receive FDA approval and approval from similar foreign regulatory bodies;
- gain market acceptance for the development and commercialization of any drugs we develop;
- ensure our products are reimbursed by commercial and/or government payers at a rate that permits commercial viability;
- develop and maintain successful strategic relationships with suppliers, distributors, and commercial licensing partners;
- manage our spending and cash requirements as our expenses will increase in the near term if we add programs and additional preclinical and clinical trials; and
- effectively market any products for which we obtain marketing approval.

If we are unsuccessful in accomplishing these objectives, we may not be able to develop products, raise capital, expand our business or continue our operations.

We may need to establish additional relationships with strategic and development partners to fully develop our product candidates and market any approved products.

Over the past several years we have entered into license and collaboration agreements with Takeda, Janssen, Amgen, Horizon, GSK, Sarepta, Visirna and Novartis Pharma AG ("Novartis"). Our business strategy includes securing additional collaborations with other pharmaceutical and biotech companies to support the development of our RNAi therapeutics and other product candidates. We do not possess all of the financial and development resources necessary to develop and commercialize all of the products that may result from our technologies. Unless we expand our own product development capacity and enhance our own internal marketing capability, we may need to make arrangements with other strategic partners to develop and commercialize any product candidates that may be approved. We may not be able to attract such partners, and even if we are able to enter into such partnerships, the terms may be less favorable than anticipated. Further, entering into partnership agreements may limit our commercialization options and/or require us to share revenues and profits with our partners. If we do not find appropriate partners, or if our existing arrangements or future agreements are not successful, our ability to develop and commercialize products could be adversely affected. Even if we are able to find collaborative partners, the overall success of the development and commercialization of product candidates in those programs will depend largely on the efforts of other parties and will be beyond our control, particularly

as partnered programs progress and our licensees may elect to assume greater control over these programs. In addition, in the event we pursue our commercialization strategy through collaboration or licenses to third parties, there are a variety of technical, business and legal risks, including:

- we may not be able to control the amount and timing of resources that our collaborators may be willing or able to devote to the development or commercialization of our product candidates or to their marketing and distribution; and
- disputes may arise between us and our collaborators that result in the delay or termination of the research, development or commercialization of our product candidates or that result in costly litigation or arbitration that diverts our management's resources.

The occurrence of any of the above events or other related events could impair our ability to generate revenues and harm our business and financial condition.

Our ability to generate milestone and royalty payments under our current and potential future licensing and collaboration agreements is substantially controlled by our partners, and as such, we will likely need other sources of financing to continue to develop our internal product candidates.

Under our licensing and collaboration agreements with Amgen, Takeda, GSK, Sarepta, Visirna, Sanofi, and Novartis, our partners substantially control clinical development and commercialization for all of the candidates covered under those agreements in their relevant territories. To the extent that (i) our partners' interests in advancing these candidates or targets changes, (ii) unforeseen scientific issues with the candidates arise, or (iii) the pace at which our partners move the candidates through clinical trials toward commercialization slows, our ability to collect milestones and royalties may be significantly diminished. This would further cause us to rely upon other sources of financing to continue to develop our other internal product candidates.

We may lose a considerable amount of control over our intellectual property and may not receive anticipated revenues in strategic transactions, particularly where the consideration is contingent on the achievement of development or sales milestones.

Our business model has been to develop new technologies and to utilize the intellectual property created through the research and development process to develop commercially successful products. If the acquirers of our technologies fail to achieve performance milestones, we may not receive a significant portion of the total value of any sale, license or other strategic transaction.

We will need to achieve commercial acceptance of REDEMPLO and our product candidates to generate revenues and achieve profitability.

Even if our research and development efforts yield technologically feasible applications, we may not successfully develop commercial products. Drug development takes years of study in human clinical trials prior to regulatory approval, and, even if we are successful in getting regulatory approval of our product candidates, it may not be on a timely basis. During our drug development period, superior competitive technologies may be introduced which could diminish or extinguish the potential commercial uses for our product candidates. Additionally, the degree to which the medical community and consumers will adopt any product we develop is uncertain. The rate and degree of market acceptance of our products will depend on a number of factors, including the establishment and demonstration in the medical community of the clinical efficacy and safety of our products, their potential advantage over alternative treatments, and the costs to patients and third-party payers, including commercial insurance companies and government health care programs. Recent efforts in the United States and abroad to reduce overall healthcare spending has put significant pressure on the price of prescription drugs and certain companies have been publicly criticized for the relatively high cost of their therapies. These pressures may force us to sell any approved drugs at a lower price than we or analysts may anticipate or may result in lower levels of reimbursement and coverage from third parties.

Moreover, we have not yet generated any revenue from product sales. Our ability to generate significant revenue and achieve profitability depends on our ability, alone or with potential strategic collaboration partners, to complete the development of and obtain the regulatory and marketing approvals necessary to commercialize our product candidates and introduce products that will be accepted by the medical community. The commercial success of REDEMPLO or any of our product candidates, if approved, will depend on many factors, including, but not limited to:

- the availability of coverage and adequate and timely reimbursement from managed care plans, private insurers, government payers (such as Medicare and Medicaid and similar foreign authorities) and other third-party payers for our products;
- patients' ability and willingness to pay out-of-pocket for our products in the absence of coverage and/or adequate reimbursement from third-party payers;

- patient demand for our products;
- the overall health benefits and costs savings that patients derive from REDEMPLO;
- our ability to establish and enforce intellectual property rights in and to our products; and
- our ability to avoid third-party patent interference, intellectual property challenges or intellectual property infringement claims.

We cannot predict whether significant commercial market acceptance for REDEMPLO or any of our products, if approved, will ever develop, and we cannot reliably estimate the projected size of any such potential market. Our revenue growth and achievement of consistent profitability will depend substantially on our ability to introduce products that will be accepted by the medical community. If we are unable to cost-effectively achieve acceptance of our technology among the medical establishment and patients, or if the associated products do not achieve wide market acceptance, our business will be materially and adversely affected.

If the market opportunities for REDEMPLO or any other approved product candidates, are smaller than we expect, it could materially adversely affect our financial condition and results of operation.

If the market opportunity for REDEMPLO or any other approved products, is smaller than we expect, we may never become or remain profitable nor generate sufficient revenue growth to sustain our business even if we obtain significant market share for them. The potentially addressable patient population for our products may be limited or may not be amenable to treatment with our products, and new patients may become increasingly difficult to identify or access, which would adversely affect our results of operations and our business.

We rely on outside sources for various components and processes for our products.

We rely on third parties for various components and processes for REDEMPLO and our product candidates. We may not be able to achieve multiple sourcing because there may be no acceptable second source, other companies may choose not to work with us, or the component or process sought may be so new that a second source does not exist or does not exist on acceptable terms. For instance, drug substance and drug product for REDEMPLO are each currently sourced from a single, third-party manufacturer, and many of our pulmonary product candidates are administered using a proprietary delivery device which is currently sourced from a single manufacturer. There may be a disruption or delay in the performance of our third-party contractors, suppliers or collaborators which is beyond our control. If such third parties are unable to satisfy their commitments to us, the development of our products would be adversely affected. Therefore, it is possible that our development plans will have to be slowed down or stopped completely at times due to our inability to obtain required raw materials, components, and outsourced processes at an acceptable cost, if at all, or to get a timely response from vendors, particularly as a result of recent labor market and global supply chain constraints.

We have limited manufacturing capability and must rely on third-party manufacturers to manufacture our clinical supplies and commercial products, if and when approved, and if they fail to meet their obligations, the development and commercialization of our products could be adversely affected.

Although we have developed our own internal manufacturing capabilities which allow us to manufacture oligonucleotide drug substance for our clinical product candidates, we do not currently have internal manufacturing capabilities beyond such clinical-stage oligonucleotide drug substance. We rely, and expect to continue to rely, on third-party manufacturers for the production of REDEMPLO and our drug product candidates for clinical trials and commercialization. We may choose to utilize third-party manufacturers to produce some or all of our development candidates, even if we have internal manufacturing capabilities to do so. Further, we have not developed the ability to manufacture drug product ourselves, nor have we developed the capabilities to manufacture biologics. If we were to experience an unexpected loss or interruption of supply for REDEMPLO or any of our product candidates, whether as a result of manufacturing, supply or storage issues or otherwise, we could experience disruptions in our commercial sales or delays, disruptions, suspensions or terminations of, or be required to restart or repeat, any pending or ongoing clinical trials. Further, our product candidates are composed of multiple components and require specific formulations for which scale-up and manufacturing could be difficult. For certain products, we have limited experience in such scale-up and manufacturing which may require us to depend on a limited number of third parties, who may not be able to deliver in a timely manner, or at all. In order to develop products, apply for regulatory approvals, and commercialize our products, we will need to develop, contract for, or otherwise arrange for the necessary manufacturing capabilities. We have increased our GMP drug substance manufacturing capacity following the substantially completed build out and integration of our manufacturing facility in Verona, Wisconsin. There are a limited number of manufacturers that supply synthetic oligonucleotides. There are risks inherent in pharmaceutical manufacturing that could affect the ability of our contract manufacturers to meet our delivery time requirements or provide adequate amounts of material to meet our needs. Included in these risks are synthesis and purification failures and contamination during the manufacturing process, which could result in unusable product and cause delays in our development process, as well as additional expense to us.

Additionally, as more of our product candidates become approved for commercial sale, we will need to establish either internal or third-party manufacturing and analytical capacity. For example, we have entered into third-party agreements for the manufacturing of REDEMPLO for drug substance and drug product, in conjunction with its commercial launch in 2025. Further, some manufacturing partners may require us to fund capital improvements, perhaps on behalf of third parties, to support the scale-up of manufacturing and related activities. We may not be able to establish scaled manufacturing capacity for an approved product in a timely or economic manner, if at all. If we or our third-party manufacturers are unable to provide commercial quantities of such an approved product, we will have to successfully transfer manufacturing technology to a different or additional manufacturer. Engaging a new manufacturer for such an approved product could require us to conduct comparative studies or utilize other means to determine bioequivalence of the new and prior manufacturers' products, which could delay or prevent our ability to commercialize such an approved product. If we or any of these manufacturers is unable or unwilling to increase its manufacturing capacity or if we are unable to establish alternative arrangements on a timely basis or on acceptable terms, the development and commercialization of such an approved product may be delayed or there may be a shortage in supply. Any inability to manufacture our product candidates or future approved drugs in sufficient quantities when needed would seriously harm our business. While we are exploring alternative suppliers for certain critical materials, some of which are sole sourced, and there can be no assurance that our efforts will be successful. Accordingly, there is a risk that supplies of our products and product candidates may be significantly delayed by, or may become unavailable as a result of manufacturing, equipment, process, regulatory or business-related issues affecting that company.

Manufacturers of our approved products (including us, if we chose to internally manufacture) must comply with cGMP requirements relating to methods, facilities and controls used in the manufacturing, processing and packaging of the product, which are intended to ensure that drug products are safe and that they consistently meet applicable requirements and specifications. These requirements include quality control, quality assurance, and the maintenance of records and documentation. These manufacturers (including us, if we chose to internally manufacture) may be unable to comply with these cGMP requirements and with other FDA, state and foreign regulatory requirements. These requirements are enforced by the FDA and other health authorities through periodic announced and unannounced inspections of manufacturing facilities. A failure to comply with these requirements or to provide adequate and timely corrective actions in response to deficiencies identified in an inspection may result in enforcement action, including warning letters, fines and civil penalties, suspension of production, suspension or delay in product approval, product seizure or recall, plant shutdown, or the delay, withholding, or withdrawal of product approval. If the safety of any quantities supplied is compromised due to a manufacturer's failure to adhere to applicable laws or for other reasons, we may not be able to obtain regulatory approval for or successfully commercialize our products, which would seriously harm our business.

We rely on third parties to conduct our clinical trials, and if they fail to fulfill their obligations, the development of our products may be adversely affected.

We rely on independent clinical investigators, contract research organizations (CROs) and other third-party service providers to assist us in managing, monitoring and otherwise carrying out our clinical trials. We contract with certain third-parties to provide certain services, including site selection, enrollment, monitoring and data management services. We rely on these parties to carry out our clinical trials in compliance with GCP and other relevant requirements. Although we depend heavily on these parties, we do not control them and therefore we cannot be assured that these third parties will adequately perform all of their contractual obligations to us. If our third-party service providers cannot adequately and timely fulfill their obligations to us, or if the quality and accuracy of our clinical trial data is compromised due to failure by such third parties to adhere to our protocols, GCP, or other regulatory requirements or if such third parties otherwise fail to meet deadlines or quality requirements, our development plans may be delayed or terminated. Further, if clinical study results are compromised, then we may need to repeat the affected studies, which could result in significant additional costs and delays to us.

We face competition from various entities including large pharmaceutical companies, small biotech companies, private companies, and research institutions.

Many of our competitors have greater financial resources and may have more experience in research and development, manufacturing, managing clinical trials, commercialization and/or regulatory compliance than we do. Our competitors may compete with us for lead clinical trial investigators, clinical trial site locations and patient enrollment. These competitors may also compete with us in recruiting scientific and management personnel. Because our products are in various stages of preclinical and clinical development, along with many of the competing products, and given unpredictability inherent in drug development, it is difficult to predict which third party may be our most direct competitor, and on what factors that competition may be focused. Moreover, we also face increased competition from other companies that are using artificial intelligence, some of whom may be able to more quickly and effectively identify and develop novel product candidates compared to us and our business partners, which could impair our ability to compete effectively and have a material adverse effect on our business, results of operations or financial condition.

We may have difficulty expanding our operations successfully as we evolve our pipeline and move toward commercializing drugs.

Our future financial performance and our ability to commercialize products and compete effectively will depend, in part, on our ability to effectively manage future growth. We expect that as we increase the number of product candidates we are developing we will also need to expand our operations. This expected growth may place a strain on our administrative and operational infrastructure and information technology systems. As product candidates we develop enter and advance through clinical trials, we will need to expand our development, regulatory, manufacturing, marketing, sales, and customer support capabilities or contract with other organizations to provide these capabilities for us. We are currently establishing a sales, customer support, and marketing infrastructure for REDEMPLO and certain of our product candidates, and although we have hired individuals with significant experience in the sales, customer support, marketing, and distribution of pharmaceutical products, the Company does not have prior experience in commercialization of a product. To achieve commercial success for REDEMPLO or any approved product for which we retain sales and marketing rights, we must continue to develop a sales and marketing organization or outsource these functions to third parties. If we or our collaborators are unable to establish sales, marketing, customer support, and distribution capabilities or successfully enter into or maintain agreements with third parties to market and sell our products, we may not be successful in commercializing our products. Further, as our operations expand due to our development progress, we expect that we will need to manage additional relationships with various collaborators, suppliers, and other organizations. Our ability to manage our operations and future growth will require us to continue to improve our operational, financial, information technology and management controls, reporting systems and procedures. We may not be able to effectively manage the expansion of our operations or implement improvements to our management information and control systems in an efficient or timely manner and may discover deficiencies in existing systems and controls.

Our business and operations could suffer in the event of a cybersecurity incident or other information technology system failures.

Our internal computer systems and those of our contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, ransomware and other cyber-attacks, human error, natural disasters, terrorism, war, and telecommunication and electrical failures. Such events could cause interruption of our operations and loss of intellectual property. For example, the loss of preclinical trial data or data from completed or ongoing clinical trials for our product candidates could result in delays in our regulatory filings and development efforts and significantly increase our costs. Further, cybersecurity breaches or other cybersecurity incidents may allow hackers access to our preclinical compounds, strategies, discoveries, trade secrets, and/or other confidential information. Additionally, sensitive data could be leaked, disclosed, or revealed as a result of or in connection with our employees', personnel's, vendors' or partners' use of generative AI technologies. To the extent that any disruption or cybersecurity incident were to result in a loss of or damage to our data, or inappropriate disclosure of confidential, proprietary or private information, we could incur liability or regulatory penalties, including under laws and regulations governing the protection of protected health information and other personal data, we could be subject to litigation (including class-action claims), we could lose valuable trade secret rights, the development of our product candidates could be delayed, and we could suffer reputational damage and damage to key business relationships. The risk of a cybersecurity incident or other informational technology disruption, particularly through cyber-attacks, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. We, and certain of the third parties for which we depend on to operate our business, have experienced cyber-security attacks in the past, which to date have not had a material impact on our operations or development programs; however; there is no assurance that such impacts will not be material in the future.

Because we use biological materials, hazardous materials, chemicals and radioactive compounds, if we do not comply with laws regulating the protection of the environment and health and human safety, our business could be adversely affected.

Our research, development and manufacturing activities involve the use of potentially harmful biological materials as well as materials, chemicals and various radioactive compounds that could be hazardous to human health and safety or the environment. We store most of these materials and various wastes resulting from their use at our facilities in Madison, Wisconsin, Verona, Wisconsin, and San Diego, California pending ultimate use and disposal. We cannot completely eliminate the risk of contamination, which could cause interruption to our research and development and manufacturing efforts, injury to our employees and others, environmental damage, and liabilities under federal, state and local law. In such an event, we may be held liable for any resulting damages, and any liability could exceed our resources. Although we carry insurance in amounts and types that we consider commercially reasonable, we do not have insurance coverage for losses relating to an interruption of our research, development or manufacturing efforts caused by contamination, and the coverage or coverage limits of our insurance policies may not be adequate. If our losses exceed our insurance coverage, our financial condition would be affected.

Securities litigation claims may result in financial losses or harm our reputation and may divert management resources.

When the market price of a stock is volatile, holders of that stock have often initiated securities class action litigation against the company that issued the stock. We cannot predict with certainty the eventual outcome of such litigation, arbitration or third-party inquiry. We may not be successful in defending ourselves or asserting our rights in current or future lawsuits, investigations, or claims that have been or may be brought against us and, as a result, our business could be materially harmed. These lawsuits, arbitrations, investigations or claims may result in large judgments or settlements against us, any of which could have a negative effect on our financial performance and business. Additionally, lawsuits, arbitrations and investigations can be expensive to defend, whether or not the lawsuit, arbitration or investigation has merit, and the defense of these actions may divert the attention of our management and other resources that would otherwise be engaged in running our business.

Our operations, including our relationships with healthcare providers, physicians and third-party payers, are subject to applicable anti-kickback, fraud and abuse, and other healthcare laws and regulations, which, in the event of a violation, exposes us to liability for criminal sanctions, civil penalties, and contractual damages, and reputational harm and diminished profits and future earnings.

Our operations, including any arrangements that we enter into with healthcare providers, physicians, and third-party payers, are subject to broadly applicable fraud and abuse and other healthcare laws and regulations. Such laws and regulations, including applicable U.S. federal and state healthcare laws and regulations, as well as foreign laws, such as the federal Anti-Kickback Statute, the False Claims Act, the Health Insurance Portability and Accountability Act of 1996, or the Foreign Corrupt Practices Act, may constrain our operation and the business or financial arrangements through which we can market, sell and distribute any product candidates for which we obtain marketing approval.

Efforts to confirm that our business arrangements with third parties comply with applicable healthcare laws and regulations involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may become subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of products from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations, and compliance and reporting obligations that could adversely affect our revenues, financial condition or results of operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

The actions of distributors and specialty pharmacies could affect our ability to sell or market products profitably. Fluctuations in buying or distribution patterns by such distributors and specialty pharmacies could adversely affect our revenues, financial condition, or results of operations.

We have an exclusive agreement with Vanscoy Rare Pharmacy for drug delivery services, and we expect to rely on this pharmacy for a considerable portion of our sales for REDEMPLO. The financial failure of Vanscoy Rare Pharmacy could adversely affect our revenues, financial condition or results of operations. Our revenues, financial condition or results of operations may also be affected by fluctuations in their buying or distribution patterns. These fluctuations may result from seasonality, pricing, wholesaler inventory objectives, or other factors.

Risks Related to Our Financial Condition

We have a history of net losses, and we expect to continue to incur net losses and may not achieve or maintain profitability.

We have incurred net losses since our inception and we expect that our operating losses will continue for the foreseeable future as we continue our drug development efforts and begin commercialization of our product candidates. To achieve profitability, we must, either directly or through licensing and/or partnering relationships, meet certain milestones, successfully develop and obtain regulatory approval for drug candidates and effectively manufacture, market and sell any drugs we successfully develop. Even if we successfully commercialize product candidates that receive regulatory approval, we may not be able to realize revenues at a level that would allow us to achieve or sustain profitability. Accordingly, we may never generate significant revenue and, even if we do generate significant revenue, we may never achieve consistent profitability.

We will require substantial additional funds to complete our research and development activities.

Our business currently does not generate the cash that is necessary to finance our operations. Subject to the success of the research and development programs of the Company and our partners, and potential licensing or partnering transactions, we may need to raise additional capital to:

- fund research and development infrastructure and activities relating to the development of our product candidates, including preclinical and clinical trials and manufacturing to support these efforts;
- fund a commercialization infrastructure and activities related to the sale, marketing, customer support, and distribution of our drug products ;
- fund our general and administrative infrastructure and activities;
- pursue business development opportunities for our technologies;
- add to and protect our intellectual property; and
- retain our management and technical staff.

Our future capital needs depend on many factors, including:

- the scope, duration, and expenditures associated with our research and development, including the progression of our clinical trials, with late-stage trials generally requiring greater capital than early-stage trials;
- regulatory requirements for our clinical trials;
- the extent to which our research and development and clinical efforts are successful;
- expenditures to build out or contract for sales, marketing and distribution capabilities ;
- the outcome of potential partnering or licensing transactions, if any, and the extent to which our business development efforts result in the acquisition of new programs or technologies;
- competing technological developments;
- our intellectual property positions, if any, in our products; and
- the regulatory approval process and regulatory standards for our product candidates.

We will need to raise additional funds through public or private equity offerings, debt financings or additional strategic alliances and licensing arrangements in the future to continue our operations. We may not be able to obtain additional financing on terms favorable to us, if at all. General market conditions may make it very difficult for us to seek financing from the capital markets, and the terms of any financing may adversely affect the holdings or the rights of our stockholders. For example, if we raise additional funds by issuing equity securities, further dilution to our stockholders will result, which may substantially dilute the value of investment. In addition, as a condition to providing additional funds to us, future investors may demand, and may be granted, rights superior to those of existing stockholders. Debt financing, if available, may involve restrictive covenants that could limit our flexibility in conducting future business activities and, in the event of insolvency, would be paid before holders of equity securities receive any distribution of corporate assets. In order to raise additional funds through partnerships, joint ventures or licensing arrangements, we may be required to relinquish rights to our technologies or product candidates or grant licenses on terms that are not favorable to us. If adequate funds are not available, we may have to further delay, reduce or eliminate one or more of our planned activities. These actions would likely reduce the market price of our common stock.

The terms of our financing agreement with Sixth Street Lending Partners and our indebtedness could adversely affect our operations and limit our ability to plan for or respond to changes in our business. If we are unable to comply with restrictions in the financing agreement, the repayment of our existing indebtedness could be accelerated.

On August 7, 2024, we entered into a financing agreement with Sixth Street Lending Partners, as the administrative agent and collateral agent for several lenders. The financing agreement establishes a senior secured term loan facility of $500.0 million (the "Credit Facility"), consisting of $400.0 million funded on the closing date and an additional $100.0 million available at our option, subject to mutual agreement with Sixth Street, over a seven-year term. On November 26, 2024, the Company entered into an amendment to the Financing Agreement to modify, amongst other things, some of the prepayment terms of the loans under the Credit Facility, including the prepayment terms related to the Sarepta Collaboration Agreement.

The financing agreement requires us to make certain payments over time and contains several other negative covenants that, subject to certain exceptions, restrict indebtedness, liens, investments (including acquisitions), fundamental changes, asset sales and licensing transactions, dividends, modifications to material agreements, payment of subordinated indebtedness, and other matters customarily restricted in such agreements. Among other requirements of the financing agreement, we and our subsidiaries party to the financing agreement must maintain certain liquidity thresholds based on our market capitalization. We are also subject to restrictions on sales and licensing transactions with respect to our core intellectual property and product assets, including, but not limited to, olpasiran, REDEMPLO, zodasiran, fazirsiran,

GSK4532990, and daplusiran/tomligisiran, subject to certain exceptions. These and other terms in the financing agreement could restrict our ability to grow our business or enter into transactions that we believe would be beneficial to our business.

Our indebtedness could affect our business in the following ways, among other things: make it more difficult for us to satisfy our contractual and commercial commitments; require us to use a substantial portion of our cash flow subject to mandatory prepayments to pay interest and principal when due, which would reduce funds available for working capital, capital expenditures and other general corporate purposes; limit our ability to obtain additional financing for working capital, capital expenditures, acquisitions and other investments or general corporate purposes; heighten our vulnerability to downturns in our business, our industry or in the general economy; place us at a disadvantage compared to those of our competitors that may have proportionately less debt; limit management's discretion in operating our business; and limit our flexibility in planning for, or reacting to, changes in our business, the industry in which we operate or the general economy.

Our business may not generate cash flows from operations in the future that are sufficient to service our debt and support our growth strategies. In addition, our ability to generate sufficient cash flows to meet our debt obligations depends upon several factors, such as the ability of the Company and our licensees to timely complete clinical trials and obtain marketing approval for our clinical-stage product candidates, to successfully commercialize REDEMLPO and our clinical-stage product candidates, and our future performance, which is subject to financial, business, and other impacts on our operations, many of which are beyond our control. If we are unable to generate sufficient cash flows, we may be required to adopt one or more alternatives, such as obtaining additional equity capital on terms that may be onerous or highly dilutive, selling assets, or restructuring debt. Our ability to refinance our indebtedness will depend on the capital markets and our financial condition at such time. We may not be able to engage in any of these activities or engage in these activities on desirable terms, which could result in a default on our debt obligations.

If the estimates we make, or the assumptions on which we rely, in preparing our consolidated financial statements prove inaccurate, our actual results may vary from those reflected in our accruals.

Our consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America. The preparation of these consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities, revenues and expenses, the amounts of charges accrued by us and related disclosure of contingent assets and liabilities. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances. We cannot assure you, however, that our estimates, or the assumptions underlying them, will be correct.

Our operating results may fluctuate significantly, which makes our future operating results difficult to predict and could cause our operating results to fall below expectations or our guidance.

Our quarterly and annual operating results have fluctuated and may continue to fluctuate significantly in the future, which makes it difficult for us to predict our future operating results. From time to time, we may enter into license or collaboration agreements or strategic partnerships with other companies that include development funding and significant upfront and milestone payments and/or royalties. These upfront and milestone payments may vary significantly from period to period and any such variance could cause a significant fluctuation in our operating results from one period to the next.

In addition, we measure compensation cost for stock-based awards made to employees at the grant date of the award, based on the fair value of the award, and recognize the cost as an expense over the employee's requisite service period. As the variables that we use as a basis for valuing these awards change over time, including our underlying stock price and stock price volatility, the magnitude of the expense that we must recognize may vary significantly.

Furthermore, our operating results may fluctuate due to a variety of other factors, many of which are outside of our control and may be difficult to predict, including the following:

- our ability to successfully commercialize REDEMPLO or any of our product candidates, if approved;
- the timing and cost of, and level of investment in, research and development activities relating to our current and any future product candidates, which will change from time to time;
- our ability to enroll patients in clinical trials and the timing of enrollment;
- the cost of manufacturing our current and any future product candidates, which may vary depending on FDA guidelines and requirements, the quantity of production and the terms of our agreements with manufacturers and other suppliers;
- expenditures that we will or may incur to acquire or develop additional product candidates and technologies;
- the timing and outcomes of clinical trials for product candidates;
- the need to conduct unanticipated clinical trials or trials that are larger or more complex than anticipated;

- competition from existing and potential future products that compete with REDEMPLO or any of our product candidates, and changes in the competitive landscape of our industry, including consolidation among our competitors or partners;
- any delays in regulatory review or approval of any of our product candidates;
- the level of demand for REDEMPLO or any of our product candidates, of approved, which may fluctuate significantly and be difficult to predict;
- the risk/benefit profile, cost and reimbursement policies with respect to REDEMPLO or any of our product candidates, if approved, and existing and potential future products that compete with our products;
- our ability to commercialize any of our products inside and outside of the United States, either independently or working with third parties;
- our ability to establish and maintain collaborations, licensing or other arrangements;
- our ability to adequately support future growth;
- potential unforeseen business disruptions that increase our costs or expenses;
- future accounting pronouncements or changes in our accounting policies; and
- the changing and volatile global economic environment.

The cumulative effect of these factors could result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, comparing our operating results on a period-to-period basis may not be meaningful. Investors should not rely on our past results as an indication of our future performance. This variability and unpredictability could also result in our failing to meet the expectations of industry or financial analysts or investors for any period. If our revenue or operating results fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if the forecasts we provide to the market are below the expectations of analysts or investors, the price of our common stock could decline substantially. Such a stock price decline could occur even when we have met any previously publicly stated guidance we may provide.

The investment of our cash, cash equivalents and fixed income securities is subject to risks which may cause losses and affect the liquidity of these investments.

At September 30, 2025, we had $919.4 million in cash, cash equivalents, restricted cash and available-for-sale securities. Our investments may also include commercial paper, securities issued by the U.S. government obligations, and money market funds meeting the criteria of our investment policy, which is focused on the preservation of our capital. These investments are subject to general credit, liquidity, and market and interest rate risks, particularly in the current economic environment. We may realize losses in the fair value of these investments or a complete loss of these investments, which would have a negative effect on our consolidated financial statements. In addition, should our investments cease paying or reduce the amount of interest paid to us, our interest income would suffer. The market risks associated with our investment portfolio may have an adverse effect on our results of operations, liquidity and financial condition.

Our ability to utilize net operating loss carryforwards and other tax benefits may be limited.

We have historically incurred net losses. Under the Internal Revenue Code of 1986, as amended (the "Code"), a corporation is generally allowed a deduction for net operating losses (NOLs) carried forward from a prior taxable year. Under that provision, we can carryforward our NOLs to offset our future taxable income, if any, until such NOLs are used or expire. As a result of the Coronavirus Aid, Relief, and Economic Security Act of 2020 ("CARES Act") and legislation commonly referred to as the Tax Cuts and Jobs Act of 2017 ("2017 Tax Act"), NOLs arising before January 1, 2018, and NOLs arising after January 1, 2018, are subject to different rules. Under the CARES Act and 2017 Tax Act, federal NOLs incurred in 2018, 2019 and 2020 can generally be carried back five years, carried forward indefinitely and can offset 100% of future taxable income for tax years before January 1, 2021 and up to 80% of future taxable income for tax years after December 31, 2020. Any NOLs arising on or after January 1, 2021, cannot be carried back, but can generally be carried forward indefinitely and can offset up to 80% of future taxable income. It is uncertain if and to what extent various states will conform to the newly enacted federal tax law. These NOL carryforwards could expire unused before offsetting potential future income tax liabilities.

In addition, under Section 382 and 383 of the Code and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50 percent change, by value, in its equity ownership over a three-year period, the corporation's ability to use its pre-change NOL carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be limited. It is possible that we have experienced an ownership change limitation. We may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control.

If an ownership change occurs and our ability to use our NOL carryforwards is materially limited, it would harm our future operating results by effectively increasing our future tax obligations.

We could be subject to additional tax liabilities.

We are subject to U.S. federal, state, and local taxes in the United States and other countries. Significant judgment is required in evaluating our tax positions. During the ordinary course of business, there are many activities and transactions for which the ultimate tax determination is uncertain. In addition, our tax obligations and effective tax rates could be adversely affected by changes in the relevant tax, accounting and other laws, regulations, principles and interpretations, including those relating to income tax nexus, by recognizing tax losses or lower than anticipated earnings in jurisdictions where we have lower statutory rates and higher than anticipated earnings in jurisdictions where we have higher statutory rates, by changes in foreign currency exchange rates, or by changes in the valuation of our deferred tax assets and liabilities. For instance, beginning in 2022, the 2017 Tax Act eliminated the option of expensing all research and development expenditures in the current year, instead requiring amortization over five years for expenditures in the U.S. and over fifteen years for foreign-based expenditures. The One Big Beautiful Bill Act, enacted in 2025, permits the expensing of certain research and development expenditures in the U.S. incurred in tax years beginning in 2025, while amortization over fifteen years continues to be required for foreign-based expenditures. We continue to monitor new tax legislation or other developments since significant changes in tax legislation, or in the interpretation of existing legislation, could materially and adversely affect our financial condition and operating results.

Additionally, we may be audited in various jurisdictions, and such jurisdictions may assess additional taxes, sales taxes and value-added taxes against us. Although we believe our tax estimates are reasonable, the final determination of any tax audits or litigation could be materially different from our historical tax provisions and accruals, which could have a material adverse effect on our operating results or cash flows in the period for which a determination is made.

Our business is subject to changing regulations for corporate governance and public disclosure that have increased both our costs and the risk of noncompliance.

Each year we are required to evaluate our internal controls systems in order to allow management to report on and our Independent Registered Public Accounting Firm to attest to, our internal controls as required by Section 404 of the Sarbanes-Oxley Act. As a result, we continue to incur additional expenses and divert our management's time to comply with these regulations. In addition, if we cannot continue to comply with the requirements of Section 404 in a timely manner, we might be subject to sanctions or investigation by regulatory authorities, such as the SEC, the Public Company Accounting Oversight Board or The Nasdaq Global Select Market. Any such action could adversely affect our financial results and the market price of our common stock.

Risks Related to Investment and Securities

Our Board of Directors has the authority to issue shares of "blank check" preferred stock, which may make an acquisition of the Company by another company more difficult.

We have adopted and may in the future adopt certain measures that may have the effect of delaying, deferring or preventing a takeover or other change in control of the Company that a holder of our common stock might consider in its best interest. For example, our Board of Directors, without further action by our stockholders, currently has the authority to issue up to 5,000,000 shares of preferred stock and to fix the rights (including voting rights), preferences and privileges of these shares ("blank check" preferred). Such preferred stock may have rights, including economic rights, senior to our common stock. These factors could also reduce the price that certain investors might be willing to pay for shares of our common stock and result in the market price being lower than it would be without these provisions.

We do not intend to declare cash dividends on our common stock.

We will not distribute cash to our stockholders unless and until we can develop sufficient funds from operations to meet our ongoing needs and implement our business plan. The time frame for that is unpredictable and investors should not expect dividends in the near future, if at all.

If securities or industry analysts do not publish research reports about our business or if they make adverse recommendations regarding an investment in our stock, our stock price and trading volume may decline.

The trading market for our common stock can be influenced by the research and reports that industry or securities analysts publish about our business. Investors have many investment opportunities and may limit their investments to companies that receive greater coverage from analysts. If additional industry or securities analysts do not commence coverage of the Company, the trading price of our stock could be negatively impacted. If one or more of the analysts downgrade our stock or comment negatively on our prospects, our stock price may decline. If one or more of these analysts cease to cover our industry or us or fail to publish reports about the Company regularly, our common stock could lose

visibility in the financial markets, which could also cause our stock price or trading volume to decline. Further, incorrect judgments, estimates or assumptions made by research analysts may adversely affect our stock price, particularly if subsequent performance falls below the levels that were projected by the research analyst(s), even if we did not set or endorse such expectations. Any of these events could cause further volatility in our stock price and could result in substantial declines in the value of our stock.

The market for purchases and sales of our common stock may be limited, and the sale of a limited number of shares could cause the price to fall sharply.

Although our common stock is listed for trading on the Nasdaq Global Select Market, at various times our securities are relatively thinly traded. Investor trading patterns could serve to exacerbate the volatility of the price of our stock. For example, mandatory sales of our common stock by institutional holders could be triggered if an investment in our common stock no longer satisfies their investment standards and guidelines. It may be difficult to sell shares of our common stock quickly without significantly depressing the value of the stock. Unless we are successful in developing continued investor interest in our stock, sales of our stock could result in major fluctuations in the price of the stock.

Our common stock price has fluctuated significantly over the last several years and may continue to do so in the future, without regard to our results of operations and prospects.

The stock market in general and the market for smaller pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. Consequently, we expect that the market price of our common stock will continue to fluctuate significantly. We may not generate substantial revenue from the sale of our products for several years, if at all. In the absence of product revenue as a measure of our operating performance, we anticipate that investors and market analysts will assess our performance by considering factors such as:

- announcements of developments related to our business;
- our ability to enter into or extend investigation phase, development phase, commercialization phase and other agreements with new and/or existing partners;
- announcements regarding the status of any or all of our collaborations or products, including clinical trial results;
- market perception and/or investor sentiment regarding our technology;
- the success of competitive products or technologies;
- announcements of actions taken by regulatory authorities, such as the FDA;
- announcements regarding developments in the RNAi, antisense technologies, gene editing technologies or biotechnology fields in general;
- announcements regarding clinical trial results with our products or competitors' products;
- market perception and/or announcements regarding other companies developing products in the field of biotechnology generally or specifically RNAi;
- the issuance of competitive patents or disallowance or loss of our patent rights;
- the addition or departure of key executives; and
- variations in our operating results.

We will not have control over many of these factors but expect that they may influence our stock price. As a result, our stock price may be volatile and such volatility could result in the loss of all or part of your investment.

Stockholder equity interest may be substantially diluted in any additional equity issuances.

We expect that significant additional capital will be needed in the future to continue our planned operations. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. These sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders.

We are subject to stringent and evolving U.S. and foreign laws, regulations, rules, contractual obligations, policies and other obligations related to data privacy and security. Our actual or perceived failure to comply with such obligations could lead to regulatory investigations or actions; litigation and mass arbitration demands; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; and other adverse business consequences.

In the ordinary course of business, we collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share confidential, proprietary, and sensitive information, including personal information, business data, trade secrets, intellectual property, information we collect about trial participants in connection with clinical trials, sensitive third-party data, business plans, transactions, and financial information.

These activities may subject us to numerous data privacy and security obligations governing the collection, use, disclosure, protection, and other processing of personal data, such as various laws, regulations, guidance, industry standards, external and internal data privacy and security policies, contractual requirements, and other obligations relating to data privacy and security.

In the United States, there are both state and federal data privacy and security laws, including data breach notification laws, data privacy laws (including biometric privacy laws and wiretapping laws), consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), and the Health Insurance Portability and Accountability Act ("HIPPA"), as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 ("HITECH Act"), and the regulations promulgated thereunder. For example, the California Consumer Privacy Act of 2018, as amended by the California Privacy Rights Act of 2020 (together, the "CCPA"), applies to personal data of consumers, including business representatives and employees, who are California residents, and requires businesses to provide specific disclosures in their privacy notices and affords certain rights to California residents with respect to their personal data. However, the CCPA does not apply to protected health information that is subject to HIPAA/HITECH. The CCPA provides for civil penalties of up to $7,500 per intentional violation and $2,500 per unintentional violation and allows private litigants affected by certain data breaches to recover significant statutory damages. In addition, state health information privacy laws, such as California's Confidentiality of Medical Information Act, Washington's My Health My Data Act and the Nevada Consumer Health Data Privacy Law, govern the privacy and security of health-related information, specifically, may apply even when HIPAA/HITECH does not and impose additional requirements.

Outside the United States there are additional laws, regulations, and industry standards governing data privacy and security. For example, in the EEA, the General Data Protection Regulation ("GDPR") and, in the UK, the GDPR as incorporated into UK law pursuant to the European Union (Withdrawal) Act 2018 (the "UK GDPR") impose strict requirements for processing personal data, including health-related data which is subject to specific requirements. Under the GDPR and UK GDPR, companies may face fines of up to 20 million Euros or 4% of annual global revenue, whichever is greater, , as well as claims from individuals who suffered damages as a result of an infringement. In addition, the GDPR and UK GDPR impose specific restrictions on the transfer of personal data to countries outside of the EEA and UK that are not considered to provide an adequate level of data protection. Although there are currently various mechanisms that may be used to ensure appropriate safeguards to the personal data, such as the EEA and UK's standard contractual clauses, these mechanisms are subject to further requirements, in particular the conduct of transfer risk/impact assessments to verify if anything in the law and/or practices of the third country may impinge on the effectiveness of the safeguards in the context of the transfer at stake and, if so, to identify and adopt supplementary measures. Additionally, companies that transfer personal data out of the EEA and UK to other jurisdictions are subject to potential scrutiny from regulators, individuals and associations. In the EEA, beyond the GDPR framework, companies must also comply with national data protection laws, which supplement, interpret, and in some cases go beyond the GDPR or other EEA-level requirements, including with respect to health-related data. Data protection authorities from the different member states may also implement certain variations, enforce data protection laws differently, and introduce additional national regulations and guidelines, which adds to the complexity of processing personal data in the EEA. Guidance developed at both EEA level and at the national level in each member state concerning implementation and compliance practices is often updated or otherwise revised. Violations of national laws can trigger administrative investigations, corrective orders, temporary or definitive bans on processing, and, in some jurisdictions, criminal penalties.

Additionally, our use of artificial intelligence may be subject to laws and evolving regulations regarding the use of artificial intelligence, including as they relate to controlling for data bias and antidiscrimination.

Preparing for and complying with these obligations require us to devote resources and may necessitate changes to our services, information technologies, systems, and practices and to those of any third parties that process personal data on our behalf.

If we or the third parties on which we rely fail, or are perceived to have failed, to address or comply with applicable data privacy and security obligations, we could face significant consequences, including but not limited to: government regulatory enforcement actions (e.g., investigations, fines, penalties, audits, inspections, and similar); litigation (including class-action claims); additional reporting requirements and/or oversight; bans on processing personal data; and orders to destroy or not use personal data. Any of these events could have a material adverse effect on our reputation, business, or financial condition, including but not limited to: loss of customers; inability to process personal data or to operate in certain

jurisdictions; limited ability to develop or commercialize our products; expenditure of time and resources to defend any claim or inquiry; adverse publicity; or substantial changes to our business model or operations.

Economic and Industry Risks

Unfavorable global economic conditions, whether brought about by material global crises, health epidemics, military conflicts or war, geopolitical and tariffs or other trade disputes or other factors, may adversely affect our business and financial results.

Our business is sensitive to global economic conditions, which can be adversely affected by epidemics and other public health crises (such as the COVID-19 pandemic), political and military conflict, tariffs or other trade restrictions or the threat of such actions and retaliatory actions, and other international disputes, significant natural disasters (including as a result of climate change) or other events that disrupt macroeconomic conditions. Adverse macroeconomic conditions, including inflation, slower growth or recession, new or increased tariffs and other trade restrictions or the threat of such actions and retaliatory actions, changes to fiscal and monetary policy or government budget dynamics (particularly in the pharmaceutical and biotech areas), tighter credit, higher interest rates, volatility in financial markets, high unemployment, labor availability constraints, currency fluctuations and other challenges in the global economy have in the past adversely affected, and may in the future adversely affect, us and our business partners and suppliers.

Trade policies and geopolitical disputes (including as a result of China-Taiwan relations) and other international conflicts can result in tariffs, sanctions and other measures that restrict international trade, and can materially and adversely affect our business, particularly if these measures occur in regions where we source components or raw materials. For example, tensions between the United States and China have led to a series of tariffs being imposed by the United States on imports from mainland China, as well as other business restrictions. In response to tariffs, countries have implemented retaliatory tariffs on U.S. goods. Furthermore, in September 2025, the current Presidential Administration announced plans to impose up to 100% tariffs on imported branded or patented pharmaceutical products, subject to certain exceptions. There is substantial uncertainty as to when such tariffs may go into effect and whether such tariffs would apply to the importation of active pharmaceutical ingredients or bulk drug products that are intended for use in clinical trials, and, more generally,

about the duration of existing tariffs, implementation of announced tariffs, litigation challenging tariffs and whether additional tariffs or other retaliatory measures may be imposed, modified or suspended. Tariffs increase the costs of the components and raw materials we source. Countries may also adopt other measures, such as controls on imports or exports of goods, technology or data, that could adversely impact the Company's operations and supply chain. These geopolitical risks could also adversely affect Visirna. Political tensions as a result of trade policies could reduce trade volume, investment, technological exchange and other economic activities between major international economies, resulting in a material adverse effect on global economic conditions and the stability of global financial markets.

Further, military conflicts or wars (such as the ongoing conflicts between Russia and Ukraine and in the Middle East) can cause exacerbated volatility and disruptions to various aspects of the global economy. The uncertain nature, magnitude, and duration of hostilities stemming from such conflicts, including the potential effects of sanctions and counter-sanctions, or retaliatory cyber-attacks on the world economy and markets, have contributed to increased market volatility and uncertainty, which could have an adverse impact on macroeconomic factors that affect our business and operations, such as worldwide supply chain issues. Additionally, the ongoing conflict between Russia and Ukraine has impacted our business decisions with respect to potential clinical trial sites in Europe. For example, a number of our clinical trial sites we had previously planned to use in Russia, Ukraine, and Belarus were shut down and we had to seek alternatives in other geographies. Moreover, the conflict between Israel and Palestine could impact future business decisions to locate potential clinical trials in Israel. It is not possible to predict the short and long-term implications of military conflicts or wars or geopolitical tensions which could include further sanctions, uncertainty about economic and political stability, increases in inflation rate and energy prices, cyber-attacks, supply chain challenges and adverse effects on currency exchange rates and financial markets.

Additionally, our operations and facilities, as well as operations of our suppliers and manufacturers, may be located in areas that are prone to earthquakes, wildfires and other natural disasters. Such operations and facilities are also subject to the risk of interruption by drought, power shortages, nuclear power plant accidents and other industrial accidents, terrorist attacks and other hostile acts, ransomware and other cybersecurity attacks, labor disputes, public health crises, and other events beyond the Company's control. Global climate change is resulting in certain types of natural disasters occurring more frequently or with more intense effects. Such events can create delays or interruptions to the Company's development efforts and inefficiencies in the Company's supply and manufacturing chain. Significant delays in our development efforts could materially impact our ability to obtain regulatory approval and to commercialize our products. Any insurance we maintain against damage to our property and the disruption of our business due to disaster may not be sufficient to cover all of our potential losses and may not continue to be available to us on acceptable terms, or at all. Further, because the

Company relies on single or limited sources for the supply and manufacture of many critical components, a business interruption affecting such sources would exacerbate any negative consequences to the Company.

Any future public health crises may affect our operations and those of third parties on which we rely, including our business partners and suppliers. We may in the future experience:

- delays in receiving authorization from regulatory authorities to initiate any planned clinical trials, inspections, reviews and approvals of products;
- delays or difficulties enrolling patients in our clinical trials;
- delays in or disruptions to the conduct of preclinical programs and clinical trials;
- constraints on the movement of products and supplies through the supply chain, which can disrupt our ability to conduct clinical trials and develop our products;
- price increases in raw materials and capital equipment, as well as increasing price competition in our markets;
- adverse impacts on our workforce and/or key employees; and
- increased risk that counterparties to our contractual arrangements will become insolvent or otherwise unable to fulfill their contractual obligations.

Drug development is time consuming, expensive and risky.

We are focused on technology related to new and improved pharmaceutical candidates. Product candidates that appear promising in the early phases of development, such as in animal and early human clinical trials, often fail to reach the market for a number of reasons, such as:

- clinical trial results may be unacceptable, even though preclinical trial results were promising;
- inefficacy and/or harmful side effects in humans or animals;
- the necessary regulatory bodies, such as the FDA, may not approve our potential product for the intended use, or at all; and/or
- manufacturing and distribution may be uneconomical.

For example, any positive preclinical results in animals may not be replicated in human clinical studies. These programs may be also found to be unsafe in humans, particularly if higher doses are needed to achieve the desired levels of efficacy. Also, the positive safety results from single dose human clinical studies may not be replicated in other human studies, including multiple dose studies. Clinical and preclinical study results are frequently susceptible to varying interpretations by scientists, medical personnel, regulatory personnel, statisticians and others, which often delays, limits, or prevents further clinical development or regulatory approvals of potential products. Clinical trials can take many years to complete, including the process of study design, clinical site selection and the recruitment of patients. As a result, we can experience significant delays in completing clinical studies, which can increase the cost of developing a drug candidate and shorten the time that an approved product may be protected by patents. If our drug candidates are not successful in human clinical trials, we may be forced to curtail or abandon certain development programs. If we experience significant delays in commencing or completing our clinical studies, we could suffer from significant cost overruns, which could negatively affect our capital resources and our ability to complete these studies.

The healthcare system is under significant financial pressure to reduce costs, which could reduce payment and reimbursement rates for drugs.

Throughout the world and particularly in the United States, the healthcare system is under significant financial pressure to reduce costs. The price of pharmaceuticals has been a topic of considerable public discussion that could lead to price controls or other price-limiting strategies by payers that have the effect of lowering payment and reimbursement rates for drugs or otherwise making the commercialization of pharmaceuticals less profitable. Many federal and state legislatures have considered, and adopted, healthcare policies intended to curb rising healthcare costs, such as the Inflation Reduction Act of 2022. These cost-containment measures may include, among other measures: requirements for pharmaceutical companies to negotiate prescription drug prices with government healthcare programs; controls on government-funded reimbursement for drugs; new or increased requirements to pay prescription drug rebates to government healthcare programs, including if drug prices increase at a higher rate than inflation; controls on healthcare providers; challenges to or limits on the pricing of drugs, including pricing controls or limits or prohibitions on reimbursement for specific products through other means; requirements to try less expensive products or generics before a more expensive branded product; and public funding for cost effectiveness research, which may be used by government and private third-party payers to make coverage and payment decisions. In addition, in May 2025, the Trump administration issued an executive order entitled "Delivering Most-Favored-Nation Prescription Drug Pricing to American Patients," which, among other things, directs the HHS and other agencies to communicate most-favored-nation ("MFN") price targets to pharmaceutical manufacturers to

bring prices for U.S. patients in line with comparably developed nations and to facilitate direct-to-consumer purchasing programs. The HHS subsequently issued guidance indicating the MFN target price is the lowest price paid in an Organization for Economic Co-operation and Development country with a gross domestic product ("GDP") per capita of at least 60% of the U.S. GDP per capita. It is currently unclear whether and to what extent these measures will be implemented and what impact any such implementation would have on our business. Further, there can be no assurance that the current administration or future administrations will not pursue different or additional measures that could impact drug pricing in the U.S. Political, economic and regulatory developments may further complicate developments in healthcare systems and pharmaceutical drug pricing. These developments could, for example, impact our potential licensing agreements as commercial and collaborative partners may also consider the impact of these pressures on their licensing strategies.

Any new laws or regulations that have the effect of imposing additional costs or regulatory burden on pharmaceutical manufacturers, or otherwise negatively affect the industry, could adversely affect our ability to successfully commercialize our product candidates. The implementation of any price controls, caps on prescription drugs or price transparency requirements could adversely affect our business, operating results and financial condition.

Evolving regulatory standards, including as a result of changes in government leadership, make it difficult to accurately predict the likelihood of marketing approval even when clinical trials meet their endpoints.

Regulatory standards are promulgated by various government entities and are subject to change based on factors such as scientific developments, public perceptions of risk, and political forces. Because clinical trials often take years to complete, it is sometimes possible for standards that exist during the conception and initiation of a clinical trial to change before the clinical trial is completed or reviewed by government regulators. For example, we may initiate clinical trials that are designed to show benefits on relatively short-term endpoints, but ultimately be required to show benefits in longer-term outcome studies. While some government entities have safeguards intended to ensure standards agreed upon by sponsors and regulators at the outset of a clinical trial are applied during regulatory review processes, those safeguards generally permit regulators to apply more rigorous standards where regulators believe doing so is necessary. As such, there can be no assurance that regulatory standards that are appropriate at the outset of a clinical trial program will not become more rigorous during the regulatory approval process and could potentially result in a delayed approval or denial of marketing authorization.

In addition, the FDA, EMA and other regulatory authorities may change their policies, issue additional regulations or revise existing regulations, or take other actions, including as a result of changes in leadership at the FDA and other federal agencies under the current U.S. administration, which may prevent or delay approval of our future products under development on a timely basis. Such policy or regulatory changes could impose additional requirements upon us that could delay our ability to obtain approvals, increase the costs of compliance or restrict our ability to maintain any marketing authorizations we may have obtained. In June 2024, the Supreme Court overruled the *Chevron* doctrine, which had given deference to regulatory agencies' statutory interpretations of ambiguous regulations in litigation against federal government agencies, such as the FDA. The overruling of the *Chevron* doctrine may significantly increase the number of challenges brought by companies and other stakeholders against federal agencies such as the FDA and its longstanding decisions and policies, including the FDA's statutory interpretations of market exclusivities and the "substantial evidence" requirements for drug approvals, which could undermine the FDA's authority, lead to uncertainties in the industry, and disrupt the FDA's normal operations, any of which could delay the FDA's review of our regulatory submissions. We cannot predict the full impact of this decision, future judicial challenges brought against the FDA, or the nature or extent of government regulation that may arise from future legislation or administrative action.

Further, under the new leadership at the HHS under the current administration, agency reorganization, mass layoffs due to the reduction in force initiative and other measures implemented by the Department of Government Efficiency may impact the normal operations of the FDA as well as other federal agencies. FDA may lack adequate staff and resources to meet current review, approval, and inspection schedules, which could delay our anticipated timelines and may have a material impact on the industry and our clinical development plans. For example, average review times at the FDA have fluctuated in recent years as a result. Our business depends upon the ability of the FDA to accept and review our potential regulatory filings. If a prolonged government shutdown occurs or if a significant number of federal employees are laid off or leave federal agencies, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our ability to advance clinical development of our product candidates.

In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable. In January 2025, an executive order entitled "Unleashing Prosperity Through Deregulation", was issued which calls for at least 10 existing regulations to be repealed whenever an executive department or agency publicly proposes for notice and comment or otherwise promulgates a new regulation. Recent developments at the FDA include announcement of a plan to phase out animal testing for monoclonal antibodies

and certain other drugs, the proposed rare disease evidence principles (RDEP) program to facilitate approval of drugs to treat rare diseases with very small patient populations with significant unmet medical need and with a known genetic defect that is the major driver of the pathophysiology, and the announcement of a new Commissioner's National Priority Voucher program for companies supporting certain U.S. national health priorities and interests. To the extent our competitors are selected for this new voucher pilot program, or are otherwise able to participate in any of these initiatives intended to accelerate drug development and application review, and obtain faster approval than us, our competitive position may be harmed. FDA has also increased its scrutiny of foreign drug manufacturing facilities and other contractors based in China, especially with respect to the transfer of biological materials, genetic data, and other sensitive data of American patients to parties located in China. It is unclear how our industry and our clinical programs will be impacted by policies and regulations implemented under the current administration and FDA leadership, or other executive orders. There is significant uncertainty in the industry and how federal agencies like the FDA will change in the coming years under the current administration. To the extent the agency reorganization and other agency changes lead to disruptions in FDA's operations, our correspondence and regulatory review processes with the FDA may be materially delayed.

ITEM 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 1C. CYBERSECURITY

The Company maintains a cybersecurity program, with direct oversight from senior management and the Audit Committee (the "Audit Committee") of the Board of Directors (the "Board"), to manage information, data, and technology security. The cybersecurity program is informed in part by the National Institute of Standards and Technology Cybersecurity Framework (NIST CSF) and is designed to help identify, assess, and manage cybersecurity risks relevant to the Company's business. The Company's cybersecurity program has been developed in light of the nature of the Company's business, resource availability, requirements from stakeholders, and industry trends. The Company has formed an internal cross-functional Technology Risk Management Committee comprised of representative leaders from various aspects of the Company's business to broadly implement its cybersecurity program.

The Company's cybersecurity program prioritizes vulnerability management, risk reduction, detection, and prevention to help protect against material risks from cybersecurity threats to its information systems. The Company periodically conducts internal and third-party cybersecurity risk assessments and penetration tests and incorporates relevant findings and recommendations into its overall cybersecurity strategy, as appropriate. Through these assessments, the Company develops targeted strategies intended to address the most significant cybersecurity risks.

The Company's cybersecurity program emphasizes defense, rapid detection, and remediation of cybersecurity threats and incidents, including the use of various security tools and systems based on defense-in-depth and zero-trust principles that are intended to meet control requirements. The cybersecurity program also encompasses crisis incident response guidelines that detail the processes for the detection, response, mitigation, and remediation of cybersecurity incidents, in order to support the effective management of, response to, communication during, and recovery from any such incidents.

A key element of the Company's strategy is fostering training and awareness through annual cybersecurity training and role-based phishing tests for employees and certain third parties having access to the Company's information systems. The Company also utilizes a third-party cybersecurity operations monitoring center to help identify threats and incidents to the Company's servers and computers. The Company's cybersecurity program includes specific requirements and guidelines for the information security team relating to the Company's computer emergency response preparedness, intrusion response preparedness, and incident response preparedness.

When a potential cybersecurity threat or incident is identified, our processes require that the Senior Director of Information Security be promptly notified of the incident, who then is to conduct an initial investigation to determine the probability and potential of the threat or incident to have a material impact on key business systems and processes. If there is a reasonable possibility for a material impact to the Company's business or information systems, the cybersecurity program requires that the Technology Risk Management Committee be promptly notified, which then assigns a risk level to the threat or incident. All threats and incidents identified as high-risk are promptly escalated to Company leadership and the legal department, who, in collaboration with the Company's Information Security department, are tasked with activating and implementing a high-risk information security incident mitigation and response plan, which details the roles, responsibilities, and strategies to respond. Our cybersecurity program also requires that high-risk cybersecurity incidents or threats be reported to the Company's Materiality Committee and the Audit Committee within 24 hours of their designation as high-risk by the Technology Risk Management Committee.

Cybersecurity risks are incorporated into our overall risk management program. If a cybersecurity risk is identified as high-risk, a response and mitigation plan is developed, and progress updates on the plan are routinely reported to the Technology Risk Management Committee and tracked by the Audit Committee as part of our overall risk management process.

The Company is not aware of any cybersecurity threats or incidents in the last fiscal year, including as a result of any prior cybersecurity incidents, that have had a material impact on the Company, including its business strategy, operations, or financial condition. However, we face certain ongoing cybersecurity risks and threats that, if realized, are reasonably likely to materially affect us. Additional information on cybersecurity risks we face is discussed in Part I, Item 1A "Risk Factors," under the heading "Our business and operations could suffer in the event of a cybersecurity incident or other information technology system failures."

Execution of the Company's cybersecurity program is delegated by the Board to the Senior Director of Information Security, who has over 25 years of relevant experience in information security, including 14 years at the Company, and is further supported by a team of security professionals within the Information Systems & Informatics department. The Senior Director of Information Security reports to the Vice President, Treasury & Head of Information Systems, and they meet periodically with senior leadership and the Audit Committee to review metrics on cybersecurity preparedness, incidents, mitigations and remediation efforts.

The Company has also established a management-level Technology Risk Management Committee, which includes leaders from finance, legal, operations, quality & compliance, and information systems & informatics, who are responsible for overseeing the execution of high-risk incident response and mitigation plans. This committee actively reviews technology strategies, physical and cybersecurity threat assessment, and emerging issues and related initiatives. It is also responsible for evaluating the materiality of information for SEC filings and, as required or as otherwise appropriate, coordinates with the Company's Materiality Committee to support timely disclosure of relevant information.

ITEM 2. PROPERTIES

The following table summarizes the Company's leased facilities as of November 19, 2025.

	Approximate Square Footage	Primary Use	Lease Expiration	Remaining Lease Term (year)
Pasadena, California	49,000	Corporate Headquarters	April 2027	1.5
Madison, Wisconsin	110,956	Research Facility	September 2031	5.9
San Diego, California	144,000	Research and Office Facility	April 2038	12.5

The Company owns land in the Verona Technology Park in Verona, Wisconsin, which has been developed into an approximately 160,000 square foot drug manufacturing facility and an approximately 140,000 square foot laboratory and office facility which will support the Company's manufacturing process development and analytical activities. The Company completed the build out of one of its laboratory and office facilities during the first quarter of fiscal year 2024 and substantially completed the build out of its manufacturing facility during the first quarter of fiscal year 2025.

ITEM 3. LEGAL PROCEEDINGS

Legal Proceedings are set forth in the Company's financial statement schedules in Part IV, Item 15 of this Annual Report on Form 10-K and are incorporated herein by reference. See Note 7 — Commitments and Contingencies of Notes to Consolidated Financial Statements of Part IV, "Item 15. Exhibits and Financial Statement Schedules."

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES

Market Information

Shares of the Company's common stock are traded on The Nasdaq Global Select Market under the symbol "ARWR." There were 86 holders of record of the Company's common stock as of November 19, 2025.

Dividends

The Company has never paid dividends on its common stock and does not anticipate that it will do so in the foreseeable future.

Recent Sales of Unregistered Securities

None.

Repurchases of Equity Securities

The following table contains information relating to the repurchases of our common stock during the three months ended September 30, 2025 :

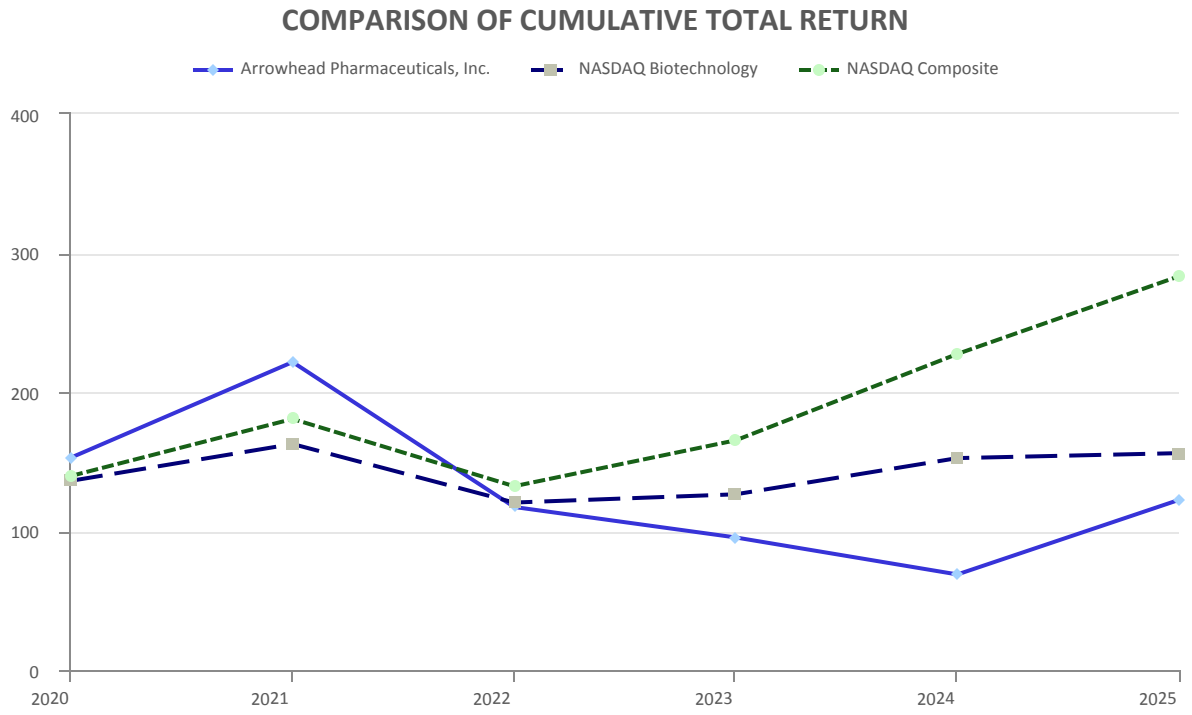
Period	Total Number of Shares Purchased	Average Price Per Share	Total Number of Shares Purchase as Part of Publicly Announced Plans or Programs	Approximate Dollar Value of Shares that May Yet Be Purchase Under the Plans or Programs
July 1 - July 31, 2025	—	—	—	—
August 1 - August 31, 2025	2,660,989	$ 18.79	—	$ —
September 1 - September 30, 2025	—	—	—	—
Total	2,660,989	$ 18.79	—	$ —

In August 2025, the Company repurchased 2,660,989 shares of our common stock from Sarepta in accordance with the share repurchase agreement related to the first development milestone payment for SRP-1003 under the Sarepta Agreement.

Performance Graph

The following performance graph shall not be deemed "soliciting material" or to be "filed" with the SEC, nor shall such information be incorporated by reference into any future filing under the Securities Act of 1933 or Securities Exchange Act of 1934, each as amended, except to the extent that we specifically incorporate it by reference into such filing. The graph compares the cumulative 5-year total return to stockholders on the Company's common stock relative to the cumulative total returns of the Nasdaq Composite Index and the Nasdaq Biotechnology Index. The Company selected the Nasdaq Biotechnology Index because it believes the index reflects the market conditions within the industry in which the Company primarily operates. The comparison of total return on investment, defined as the change in year-end stock price plus reinvested dividends, for each of the periods assumes that $100 was invested on September 30, 2020 in each of the Company's common stock, the Nasdaq Composite Index and the Nasdaq Biotechnology Index, with investment weighted on the basis of market capitalization.

The comparisons in the following graph are based on historical data and are not intended to forecast the possible future performance of the Company's common stock.



$100 investment in stock or index	Ticker	2020	2021	2022	2023	2024	2025
Arrowhead Pharmaceuticals, Inc.	ARWR	$ 152.80	$ 221.54	$ 117.28	$ 95.35	$ 68.74	$ 122.39
NASDAQ Biotechnology Index	^NBI	$ 136.10	$ 162.58	$ 120.46	$ 126.41	$ 152.44	$ 156.02
NASDAQ Composite Index	^IXIC	$ 139.61	$ 180.62	$ 132.21	$ 165.26	$ 227.38	$ 283.27

ITEM 6. RESERVED

ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

OVERVIEW

The Company develops medicines that treat intractable diseases by silencing the genes that cause them. Using a broad portfolio of RNA chemistries and modes of delivery, the Company's therapies trigger the RNAi interference mechanism to induce rapid, deep and durable knockdown of target genes. RNAi is a mechanism present in living cells that inhibits the expression of a specific gene, thereby affecting the production of a specific protein. RNAi-based therapeutics may leverage this natural pathway of gene silencing to target and shut down specific disease-causing genes.

The Company believes that TRiM enabled therapeutics offer several potential advantages over prior generations and competing technologies, including: simplified manufacturing and reduced costs; multiple routes of administration including subcutaneous injection and inhaled administration; the ability to target multiple tissue types including liver, lung, skeletal muscle, central nervous system (CNS), adipose tissue, ocular, and cardio-myocytes; and the potential for improved safety and reduced risk of intracellular buildup, because there are fewer metabolites from smaller, simpler molecules.

The Company's pipeline includes:

- Severe Hypertriglyceridemia - plozasiran (formerly ARO-APOC3, Greater China rights out-licensed to Sanofi);

- Homozygous familial hypercholesterolemia (HoFH) - zodasiran (formerly ARO-ANG3);
- Cardiovascular disease - olpasiran (formerly AMG 890 or ARO-LPA, out-licensed to Amgen);
- Mixed hyperlipidemia – ARO-DIMERPA (Greater China rights out-licensed to Sanofi);
- Inflammatory pulmonary conditions - ARO-RAGE;
- Idiopathic pulmonary fibrosis - SRP-1002 (formerly ARO-MMP7, out-licensed to Sarepta);
- Metabolic-dysfunction associated steatohepatitis (MASH) - GSK4532990 (formerly ARO-HSD, out licensed to GSK and Visirna);
- Alpha-1 antitrypsin deficiency (AATD) - fazirsiran (formerly ARO-AAT, a collaboration with Takeda);
- Chronic Hepatitis B virus - daplusiran/tomligisiran - GSK5637608 (formerly JNJ-3989 and ARO-HBV, out-licensed to GSK);
- Complement mediated diseases - ARO-C3 and ARO-CFB;
- Metabolic-dysfunction associated steatohepatitis (MASH) - ARO-PNPLA3 (formerly JNJ-75220795 or ARO-JNJ1);
- Obesity - ARO-INHBE and ARO-ALK7
- Facioscapulohumeral muscular dystrophy - SRP-1001 (formerly ARO-DUX4, out-licensed to Sarepta);
- Myotonic Dystrophy Type 1 - SRP-1003 (formerly ARO-DM1 out-licensed to Sarepta; and
- Spinocerebellar ataxia 2 - SRP-1004 (formerly ARO-ATXN2, out-licensed to Sarepta).
- Parkinson’s disease – ARO-SNCA (out-licensed to Novartis)
- Alzheimer’s disease – ARO-MAPT

The Company operates lab facilities in California and Wisconsin, where its research and development activities, including the development of RNAi therapeutics, take place. The Company’s principal executive offices are located in Pasadena, California.

The Company continues to develop other clinical candidates for future clinical trials. Clinical candidates are tested internally and through Good Laboratory Practice (GLP) toxicology studies at outside laboratories. Drug materials for such studies and clinical trials are either manufactured internally or contracted to third-party manufacturers. The Company engages third-party contract research organizations (CROs) to manage clinical trials and works cooperatively with such organizations on all aspects of clinical trial management, including plan design, patient recruiting, and follow up. These outside costs, including toxicology/efficacy testing and manufacturing costs, as well as the preparation for and administration of clinical trials, are referred to as “candidate costs.” As clinical candidates progress through clinical development, candidate costs will increase.

2025 Business Highlights

During fiscal year 2025 and through the date of filing, the Company continued to develop and advance its pipeline and partnered candidates and expand its facilities to support its growing programs. The bullets below highlight some of these key developments; however, this list is not all-inclusive and is meant to be read in conjunction with the entirety of management’s discussion and analysis, the Company’s Consolidated Financial Statements and notes thereto, and all other items contained within this Annual Report on Form 10-K.

- On November 20, 2025, the Company earned a $200.0 million milestone payment from Sarepta. The milestone was earned when Arrowhead achieved the second development milestone event in a Phase 1/2 clinical study of ARO-DM1, also called SRP-1003, an investigational RNAi therapeutic for the treatment of type 1 myotonic dystrophy (DM1), the most common adult-onset muscular dystrophy. The second milestone event included the achievement of a patient enrollment target, drug safety committee review and subsequent authorization to dose escalate and proceed, and completion of day 105 study visit by at least one patient in the clinical trial;
- The FDA approved the Company's New Drug Application (NDA) for REDEMPLO (plozasiran) injection for Familial Chylomicronemia Syndrome (FCS), on November 18, 2025. This approval was supported by clinical data from the Phase 3 PALISADE study, a randomized, double-blind, placebo-controlled trial in adults with clinically diagnosed or genetically confirmed FCS. The PALISADE study met its primary endpoint and all multiplicity-controlled key secondary endpoints, including demonstrating significant reductions in triglycerides and APOC3. In PALISADE, 25 mg REDEMPLO achieved deep and durable reductions in triglycerides, with a median change from baseline of -80% versus -17% in the pooled placebo group, and a lower numerical incidence of acute pancreatitis compared with placebo;
- Filed a request for regulatory clearance to initiate a Phase 1/2a clinical trial of ARO-DIMER-PA, the Company’s investigational RNA interference (RNAi) therapeutic being developed as a potential treatment for atherosclerotic cardiovascular disease (ASCVD) due to mixed hyperlipidemia. ARO-DIMER-PA is designed to silence expression of the proprotein convertase subtilisin kexin 9 (PCSK9) and apolipoprotein C3

(APOC3) genes. This represents an important step forward for the RNAi field as it is the first clinical candidate to target two genes simultaneously in one molecule, enabled by Arrowhead's innovative and proprietary TRiM platform;

- Filed a request for regulatory clearance to initiate a Phase 1/2a clinical trial of ARO-MAPT, the Company's investigational RNAi therapeutic being developed as a potential treatment for tauopathies including Alzheimer's disease, a progressive neurodegenerative disease characterized by cognitive and functional decline. Alzheimer's disease is the most common cause of dementia and is estimated to affect 32 million people worldwide and is part of a group of neurodegenerative diseases called tauopathies that are marked by the abnormal accumulation and formation of tau tangles in neurons.
- On August 29, 2025, the Company entered into a global licensing and collaboration agreement with Novartis for ARO-SNCA, Arrowhead's preclinical stage siRNA therapy against alpha-synuclein for the treatment of synucleinopathies, such as Parkinson's Disease, and for other additional collaboration targets that will utilize Arrowhead's proprietary TRiM platform. Closing of the transaction was subject to the expiration or termination of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 and other customary conditions. Upon closing in October 2025, the Company received $200.0 million as an upfront payment and is eligible to receive up to $2.0 billion in potential milestone payments plus royalties on commercial sales.
- Triggered a $100.0 million milestone payment from Sarepta, which was achieved on July 27, 2025, when the Company reached the first of two prespecified enrollment targets and subsequent authorization to dose escalate in a Phase 1/2 clinical study of ARO-DM1, an investigational RNAi therapeutic for the treatment of type 1 myotonic dystrophy (DM1). Arrowhead received $53.2 million worth of Arrowhead common stock and $50.0 million in cash from Sarepta Therapeutics, satisfying the payment of the $100.0 million milestone owed to Arrowhead.
- Announced the signing of an asset purchase agreement between Sanofi and Visirna, a majority-owned subsidiary of the Company, created to develop and commercialize four of the Company's investigational cardiometabolic candidates in Greater China. Under the terms of the agreement, Sanofi will acquire rights to develop and commercialize investigational plozasiran, the Company's first-in-class RNAi therapeutic candidate designed to reduce production of apolipoprotein C-III (APOC3) as a potential treatment for familial chylomicronemia syndrome (FCS) and severe hypertriglyceridemia (sHTG), in Greater China;
- Initiated and dosed the first subject in the YOSEMITE Phase 3 clinical trial of zodasiran, the Company's investigational RNAi therapeutic being developed as a potential treatment for homozygous familial hypercholesterolemia (HoFH), a rare genetic condition that leads to severely elevated LDL-cholesterol and early onset cardiovascular disease;
- Completed enrollment of SHASTA-3, SHASTA-4, and MUIR-3 Phase 3 clinical trials of REDEMPLO. The Company's global Phase 3 clinical studies are designed to support regulatory submissions for approval of investigational REDEMPLO in the treatment of severe hypertriglyceridemia.
- Initiated a Phase 1/2a clinical trial of ARO-ALK7 for the treatment of obesity. ARO-ALK7 is the first RNAi-based therapy designed to silence adipocyte expression of the ACVR1C gene to reduce the production of Activin receptor-like kinase 7 (ALK7), which acts as a receptor in a pathway that regulates energy homeostasis in adipose tissue;
- Announced Topline results from Part 2 of a Phase 1/2 clinical study of ARO-C3, the Company's investigational RNAi therapeutic designed to reduce liver production of complement component 3 (C3) as a potential therapy for various complement mediated diseases. ARO-C3 achieved reductions in alternative pathway complement activity and proteinuria;
- Entered into a global licensing and collaboration agreement with Sarepta on November 25, 2024, which closed on February 7, 2025. Upon closing, the Company received $325.0 million through the purchase of 11,926,301 shares of Company common stock by Sarepta, at a price per share of $27.25, and received $500.0 million as an upfront payment on February 24, 2025. The Company will also receive $250.0 million to be paid in equal installments over five years and is eligible to receive an additional $300.0 million in near-term payments. Additionally, the Company is eligible to receive royalties on commercial sales and up to approximately $10.0 billion in future potential milestone payments;
- GSK dosed its fifth patient in a Phase 2 trial in December 2024, triggering a $2.5 million milestone payment to the Company which was paid in the second quarter of fiscal 2025;

- Announced that the Company dosed the first subjects in a Phase 1/2a clinical trial of ARO-INHBE; and
- Presented interim results from a Phase 1/2a clinical study of ARO-CFB at the 8th Complement-Based Drug Development Summit. The study resulted in multiple findings including: (1) ARO-CFB led to dose dependent reductions in circulating CFB protein by up to 90% with greater than 3 months duration, (2) single and multiple doses of ARO-CFB led to near complete inhibition of alternative pathway activity based on Wieslab AP, and (3) single and multiple doses of ARO-CFB led to near complete inhibition of alternative pathway hemolytic activity, measured by AH50.

Critical Accounting Estimates

Management makes certain judgments and uses certain estimates and assumptions when applying U.S. generally accepted accounting principles ("GAAP") in the preparation of the Company's Consolidated Financial Statements. On an ongoing basis, the Company evaluates its estimates, judgments and assumptions. The Company bases its estimates on historical experience and on various other assumptions that it believes are reasonable, the results of which form the basis for making judgments about the carrying values of assets, liabilities and equity and the amount of revenue and expense. Actual results may vary from what the Company anticipates and different assumptions or estimates about the future could change its reported results. The Company believes the following accounting policies are the most critical to it, in that they require its most difficult, subjective or complex judgments in the preparation of the Company's Consolidated Financial Statements. For further information, see Note 1, Organization and Significant Accounting Policies of the Notes to the Company's Consolidated Financial Statements in Part IV, "Item 15. Exhibits and Financial Statement Schedules."

Revenue Recognition—The Company has adopted Financial Accounting Standards Board ("FASB") Topic 606 – *Revenue for Contracts from Customers*. The Company has not yet achieved commercial sales of its drug candidates to date; however, this standard is applicable to its licensing and collaboration agreements. This is discussed further in Note 2, Collaboration and License Agreements of the Notes to the Company's Consolidated Financial Statements in Part IV, "Item 15. Exhibits and Financial Statement Schedules."

At contract inception, the Company assesses whether the goods or services promised within each contract are distinct and, therefore, represent a separate performance obligation, or whether they are not distinct and are combined with other goods and services until a distinct bundle is identified. The Company then determines the transaction price, which typically includes upfront payments and any variable consideration that it determines is probable to not cause a significant reversal in the amount of cumulative revenue recognized when the uncertainty associated with the variable consideration is resolved. The Company then allocates the transaction price to each performance obligation and recognizes the associated revenue when (or as) each performance obligation is satisfied.

The Company recognizes the transaction price allocated to upfront license payments as revenue upon delivery of the license to the customer and resulting ability of the customer to use and benefit from the license, if the license is determined to be distinct from the other performance obligations identified in the contract. These other performance obligations are typically to perform research and development services for the customer, often times relating to the candidate that the customer is licensing. If the license is not considered to be distinct from other performance obligations, the Company assesses the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied at a point in time or over time. If the performance obligation is satisfied over time, the Company then determines the appropriate method of measuring progress for purposes of recognizing revenue from license payments. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the related revenue recognition.

Typically, the Company's collaboration agreements entitle it to additional payments upon the achievement of milestones or royalties on sales. The milestones are generally categorized into three types: development milestones, generally based on the initiation of toxicity studies or clinical trials; regulatory milestones, generally based on the submission, filing or approval of regulatory applications such as a NDA in the United States; and sales-based milestones, generally based on meeting specific thresholds of sales in certain geographic areas. The Company evaluates whether it is probable that the consideration associated with each milestone or royalty will not be subject to a significant reversal in the cumulative amount of revenue recognized. Amounts that meet this threshold are included in the transaction price using the most-likely-amount method, whereas amounts that do not meet this threshold are excluded from the transaction price until they meet this threshold. At the end of each subsequent reporting period, the Company re-evaluates the probability of a significant reversal of the cumulative revenue recognized for its milestones and royalties, and, if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect revenues and net income in the Company's consolidated statements of operations and comprehensive income (loss). Typically, milestone payments and royalties are achieved after the Company's performance obligations associated with the collaboration agreements have been completed and after the customer has assumed responsibility for the respective clinical or preclinical program. Milestones or royalties achieved after the Company's performance obligations have been completed are recognized as revenue in the period the milestone or royalty was achieved. If a milestone payment is achieved during

the performance period, the milestone payment would be recognized as revenue to the extent performance had been completed at that point, and the remaining balance would be recorded as deferred revenue.

The revenue standard requires the Company to assess whether a significant financing component exists in determining the transaction price. The Company performs this assessment at the onset of its licensing or collaboration agreements. Typically, a significant financing component does not exist because the customer is paying for a license or services in advance with an upfront payment. Additionally, future royalty payments are not substantially within the control of the Company or the customer.

The revenue standard requires the Company to allocate the arrangement consideration on a relative standalone selling price basis for each performance obligation after determining the transaction price of the contract and identifying the performance obligations to which that amount should be allocated. The relative standalone selling price is defined in the revenue standard as the price at which an entity would sell a promised good or service separately to a customer. If other observable transactions in which the Company has sold the same performance obligation separately are not available, the Company estimates the standalone selling price of each performance obligation. The estimates includes forecasted revenues and expenses, phase dates, probability of success, development timelines, and the discount rate. The estimates of the stand-alone selling price for research and development or other service-related performance obligations generally include forecasting the expected costs of satisfying a performance obligation at market rates.

Whenever we determine that goods or services promised in a contract should be accounted for as a combined performance obligation over time, the Company determines the period over which the performance obligations will be performed and revenue will be recognized. Revenue is recognized using the input method. Labor hours, costs incurred or patient visits in clinical trials are typically used as the measure of performance. Significant management judgment is required in determining the level of effort required under an arrangement and the period over which the Company is expected to complete its performance obligations. If the Company determines that the performance obligation is satisfied over time, any upfront payment received is initially recorded as deferred revenue on the Company's consolidated balance sheets.

Collaborative Arrangements—The Company analyzes its collaborative arrangements to assess whether such arrangements involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards, and therefore an appropriate recognition method is determined and applied consistently, either by analogy to appropriate accounting literature or by applying a reasonable accounting policy election. For collaborative arrangements that are within the scope of FASB Topic 808—*Collaborative Arrangements*, the Company evaluates the income statement classification for presentation of amounts due to or owed from other participants associated with multiple units of account in a collaborative arrangement based on the nature of each activity. Payments or reimbursements that are the result of a collaborative relationship instead of a customer relationship, such as co-development and co-commercialization activities, are recorded as increases or decreases to research and development expense or general and administrative expense, as appropriate.

Clinical Accruals—The Company accrues liabilities for products received or services incurred, particularly for ongoing clinical trials, where service providers have not yet billed or where billing terms do not align with the timing of the work performed as of the period-end. These costs mainly include third-party clinical management or clinical research organization (CRO), laboratory analysis, and investigator fees. Accrual estimates may be based on vendor communications to obtain pending invoices and/or estimates for services performed during the period. In some cases, these estimates require significant judgment, drawing on an understanding of research and development programs, services provided during the period, prior experience, and, where applicable, the expected duration of third-party contracts. Actual costs upon settlement may differ significantly from the accrued amounts in the Company's consolidated financial statements, though historical estimates have not differed materially from actual costs.

Liability Related to the Sale of Future Royalties—Based on its evaluation of the agreement terms, the Company classifies the liability related to the sale of future royalties as a debt financing. The Company records the obligations at their carrying value using the effective interest method. In order to amortize the sale of future royalties, the Company utilizes the prospective method to estimate the future royalties to be paid by the Company to the counterparty over the life of the arrangement. Under the prospective method, a new effective interest rate is determined based on the revised estimate of remaining cash flows. The new rate is the discount rate that equates the present value of the revised estimate of remaining cash flows with the carrying amount of the debt, and it will be used to recognize non-cash interest expense for the remaining periods. The Company periodically assesses the amount and the timing of expected royalty payments using a combination of internal projections and forecasts from external sources. The estimates of future net product sales (and resulting royalty payments) are based on key assumptions including population, penetration, probability of success and sales price, among others. To the extent such payments are greater or less than the Company's initial estimates or the

timing of such payments is different than its original estimates, the Company will prospectively adjust the amortization of the royalty financing obligations and the effective interest rate.

RESULTS OF OPERATIONS

The following data summarizes the Company's results of operations for the following periods indicated:

	Year Ended September 30,		
	2025	2024	2023
	(in thousands, except per share amounts)		
Revenue	$ 829,448	$ 3,551	$ 240,735
Operating income (loss)	$ 98,346	$ (601,080)	$ (205,002)
Net loss attributable to Arrowhead	$ (1,631)	$ (599,493)	$ (205,275)
Net loss per share (diluted) attributable to Arrowhead	$ (0.01)	$ (5.00)	$ (1.92)

Year Ended September 30, 2025 Compared to Year Ended September 30, 2024

Revenue

Total revenue for the year ended September 30, 2025 increased by $825.9 million, from the same period of 2024. The change was primarily driven by increased revenue recognition associated with the Sarepta, Sanofi, and GSK license agreements as discussed below.

The Company has evaluated each agreement in accordance with FASB Topic 808–*Collaborative Arrangements* and Topic 606-*Revenue for Contracts from Customers*. See Note 2 — Collaboration and License Agreements of the Notes to Consolidated Financial Statements of Part IV, "Item 15. Exhibits and Financial Statement Schedules."

Sarepta: On November 25, 2024, the Company entered into the Sarepta Collaboration Agreement and Stock Purchase Agreement with Sarepta for the development and commercialization of multiple clinical and preclinical programs in rare, genetic diseases of the muscle, central nervous system, and lungs. During the fourth quarter of fiscal 2025, a $100.0 million milestone payment from Sarepta Therapeutics, Inc. was triggered, when the Company reached the first of two prespecified enrollment targets and subsequent authorization to dose escalate in a Phase 1/2 clinical study of ARO-DM1, an investigational RNAi therapeutic for the treatment of type 1 myotonic dystrophy (DM1). The Company received $53.2 million of Arrowhead common stock and $50.0 million cash from Sarepta to satisfy the milestone payment. During the year ended September 30, 2025, the Company recorded $696.8 million in revenue.

Visirna: On August 1, 2025, Visirna Therapeutics HK Limited ("Visirna HK"), a wholly owned subsidiary of Visirna Therapeutics, Inc, a majority owned subsidiary of the Company, entered into an Asset Purchase Agreement (the "Asset Purchase Agreement") with Genzyme Corporation ("Sanofi"), a wholly owned subsidiary of Sanofi S.A., pursuant to which Visirna HK sold all of its assets and rights in investigational plozasiran to Sanofi, which included an assignment of Visirna HK's rights (as successor by assignment from Visirna) to develop and commercialize investigational plozasiran in Greater China pursuant to that certain License Agreement by and between the Company and Visirna dated, April 25, 2022 (the "Visirna License Agreement"). During the year ended September 30, 2025, the Company recorded $130.0 million in revenue.

GSK: On December 11, 2023, the Company entered into the GSK-HBV Agreement pursuant to which GSK received a worldwide, exclusive license to develop and commercialize daplusiran/tomligisiran (GSK5637608, formerly JNJ-3989), the Company's third-generation subcutaneously administered RNAi therapeutic candidate being developed as a potential therapy for patients with chronic hepatitis B virus infection.

Under the terms of the GSK-HBV Agreement, the Company received $2.7 million in December 2023, upon signing the GSK-HBV Agreement. Further, GSK dosed the fifth patient in a Phase 2 trial in December 2024, triggering a $2.5 million milestone payment to the Company which was paid in the second quarter of fiscal 2025. During the year ended September 30, 2025, the Company recorded $2.6 million revenue.

Operating Expenses

The analysis below details the operating expenses and discusses the expenditures of the Company within the major expense categories. For purposes of comparison, the amounts for the years ended September 30, 2025 and 2024 are shown in the tables below.

Research and Development (R&D) Expenses

Research and development expenses consist of expenses for drug candidate and drug discovery costs, which are comprised primarily of outsourced costs related to the manufacturing of clinical supplies, toxicity/efficacy studies and clinical trial expenses. Internal costs primarily relate to discovery operations at the Company's research facilities in California and Wisconsin, including facility costs and laboratory-related expenses. The Company operates in a cross-functional manner across projects and does not separately allocate facilities-related costs, candidate costs, discovery costs, compensation expenses, depreciation and amortization expenses, and other expenses related to research and development activities. The Company does not separately track research and development expenses by individual research and development projects, or by individual drug candidates.

The following table provides details of research and development expenses for the period indicated:

(in thousands)	Year Ended September 30, 2025	% of Expense Category	Year Ended September 30, 2024	% of Expense Category	Increase (Decrease) $	Increase (Decrease) %
Candidate costs	$ 347,571	57 %	$ 259,280	51 %	$ 88,291	34 %
R&D discovery costs	66,788	11 %	74,150	15 %	(7,362)	(10)%
Salaries	109,085	18 %	96,418	19 %	12,667	13 %
Facilities related	29,233	5 %	25,782	5 %	3,451	13 %
Total research and development expense, excluding non-cash expense	$ 552,677	91 %	$ 455,630	90 %	$ 97,047	21 %
Stock compensation	32,582	5 %	33,586	7 %	(1,004)	(3)%
Depreciation and amortization	21,900	4 %	16,654	3 %	5,246	31 %
Total research and development expense	$ 607,159	100 %	$ 505,870	100 %	$ 101,289	20 %

Candidate costs increased $88.3 million, or 34%, for the year ended September 30, 2025 compared to the same period of 2024. The increase was primarily due to the additional progression of the Company's pipeline of candidates into and through clinical trials, which resulted in higher manufacturing, outsourced clinical trial, and toxicity study costs.

R&D discovery costs decreased $7.4 million, or (10)%, for the year ended September 30, 2025 compared to the same period of 2024. This decrease was primarily driven by strategic shifts toward clinical development and commercial launch. R&D discovery costs are influenced by the Company's ongoing discovery efforts, continued advancements into novel therapeutic areas and tissue types.

Salaries consist of salary, bonuses, payroll taxes, and related benefits for the Company's R&D personnel. Salaries expense increased $12.7 million, or 13%, for the year ended September 30, 2025 compared to the same period of 2024. The increase was primarily due to an increase in headcount that has occurred as the Company has expanded its pipeline of candidates, in addition to annual salary increases.

Facilities-related expense includes lease costs for the Company's research and development facilities in San Diego, California and Madison and Verona, Wisconsin. These expenses increased $3.5 million, or 13%, for the year ended September 30, 2025 compared to the same period of 2024. The increase was primarily due to full-year expenses such as utilities and repair and maintenance charges associated with the new facilities in Verona, Wisconsin, which completed their build out during the first quarter of fiscal 2024.

Stock compensation expense, a non-cash expense, is primarily based on the valuation of restricted stock units granted to employees, which is based on the closing stock price on the grant date. Stock compensation expense decreased $1.0 million, or 3%, for the year ended September 30, 2025 compared to the same period of 2024. The decrease was primarily due to the cancellation of awards upon the departure of employees.

Depreciation and amortization expense, a non-cash expense, relates to depreciation on buildings, lab equipment and leasehold improvements. Depreciation and amortization expense increased $5.2 million, or 31% for the year ended September 30, 2025 compared to the same period of 2024. The increase was primarily attributable to completion of the build out of facilities in Verona, Wisconsin, and the commencement of depreciation.

General & Administrative Expenses

The following table provides details of general and administrative expenses for the periods indicated:

(in thousands)	Year Ended September 30, 2025	% of Expense Category	Year Ended September 30, 2024	% of Expense Category	Increase (Decrease) $	Increase (Decrease) %
Salaries	$ 31,916	26 %	$ 27,589	28 %	$ 4,327	16 %
Professional, outside services, and other	53,589	42 %	24,733	25 %	28,856	117 %
Facilities related	5,625	5 %	4,116	4 %	1,509	37 %
Total general & administrative expense, excluding non-cash expense	$ 91,130	73 %	$ 56,438	57 %	$ 34,692	61 %
Stock compensation	30,785	25 %	40,382	41 %	(9,597)	(24)%
Depreciation/amortization	2,028	2 %	1,941	2 %	87	4 %
Total general & administrative expense	$ 123,943	100 %	$ 98,761	100 %	$ 25,182	25 %

Salaries expense increased $4.3 million, or 16%, for the year ended September 30, 2025 compared to the same period of 2024. The increase was driven by the combination of annual salary increases and an increase in headcount required to support the Company's growth as the Company prepares for commercialization.

Professional, outside services, and other expenses include costs related to legal, audit, consulting, patent filings, business insurance, other external services, as well as travel, communication, and technology expenses. These expenses increased $28.9 million, or 117%, for the year ended September 30, 2025 compared to the same period of 2024. The increase was mainly due to professional services associated with commercialization and business development efforts as the Company prepares for a product launch, including costs for data analytics, marketing and commercial launch support.

Facilities related expense primarily includes rental costs and other facilities-related costs for the Company's corporate headquarters in Pasadena, California. These expenses increased $1.5 million, or 37%, for the year ended September 30, 2025 compared to the same periods of 2024. The increase was primarily driven by higher common area maintenance charges, increased staff amenities expenses.

Stock compensation expense, a non-cash expense, is based on the valuation of restricted stock units granted to employees, which is based on the closing stock price on the grant date. This expense decreased by $9.6 million, or 24%, for the year ended September 30, 2025 compared to the same period of 2024. The decrease was primarily due to lower compensation costs related to performance awards, as the timing of these expenses can vary based on the achievement of related performance targets.

Depreciation and amortization expense, a noncash expense, was primarily related to amortization of leasehold improvements for the Company's corporate headquarters.

Other (Expense) Income

Other (expense) income is primarily related to interest income and expense. Other expense increased $35.4 million for the year ended September 30, 2025 compared to the same period of 2024. The increase was primarily due to non-cash interest expense associated with the liability related to the sale of future royalties and the Credit Facility, partially offset by higher income from increased investment yields.

Net loss attributable to Arrowhead Pharmaceuticals, Inc. was $1.6 million for the year ended September 30, 2025 compared to a net loss attributable to Arrowhead Pharmaceuticals, Inc. of $599.5 million for the year ended September 30, 2024. Net loss per share – diluted was $0.01 for the year ended September 30, 2025 compared to net loss per share – diluted $5.00 for the year ended September 30, 2024. The decrease in net loss attributable to Arrowhead Pharmaceuticals, Inc. for the year ended September 30, 2025 compared to the same period of 2024 was primarily due to an increase in revenue from the Sarepta Collaboration Agreement, partially offset by higher research and development expenses, associated with the expansion of the Company's pipeline and progression through clinical trial phases.

Income Tax Expense (Benefit)

Income tax expense was $21.4 million for the year ended September 30, 2025, compared to an income tax benefit of $2.8 million for the same period in 2024. The change of $24.2 million was primarily due to higher taxable income in fiscal 2025

resulting from the recognition of $696.8 million in revenue under the Sarepta Collaboration Agreement and $130.0 million in revenue recognized by Visirna.

Non-controlling Interest

Net income attributable to non-controlling interest was $31.7 million for the year ended September 30, 2025, compared to a loss attributable to non-controlling interest of $10.2 million for the same period in 2024. The change of $41.9 million was primarily due to Visirna's recognition of $130.0 million in revenue during fiscal 2025, resulting in a significant increase in net income, whereas Visirna incurred a net loss in fiscal 2024.

Year Ended September 30, 2024 Compared to Year Ended September 30, 2023

See "Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations" of the Company's Form 10-K for the year ended September 30, 2024 for a discussion of changes in its results of operations from the year ended September 30, 2024 to the year ended September 30, 2023.

LIQUIDITY AND CAPITAL RESOURCES

The Company has historically financed its operations through the sale of its equity securities, credit facility, revenue from its licensing and collaboration agreements, and the sale of certain future royalties. Research and development activities have required significant capital investment since the Company's inception and are expected to continue to require significant cash expenditure as the Company's pipeline continues to expand and matures into later stage clinical trials, including commercialization efforts. For further information on the Company's capital needs, see the section titled "Risks Related to Our Financial Condition" in "Item 1A. Risk Factors" of this Annual Report on Form 10-K.

The Company's cash, cash equivalents and restricted cash was $226.5 million at September 30, 2025 compared to $102.7 million at September 30, 2024. Cash invested in available-for-sale securities was $692.8 million at September 30, 2025 compared to $578.3 million at September 30, 2024.

On December 2, 2022, the Company entered into an open market sale agreement ("the Open Market Sale Agreement"), pursuant to which the Company may, from time to time, sell up to $250.0 million in shares of the Company's common stock through Jefferies LLC, acting as the sales agent and/or principal, in an at-the-market offering. As of September 30, 2025, no shares have been issued under the Open Market Sale Agreement.

In August 2024, the Company entered into the Credit Facility, which provides for a senior secured term loan facility of $500.0 million, which includes $400.0 million funded on the closing date with an additional $100.0 million at the Company's option during the seven-year term of the agreement. The Company received net proceeds of $388.9 million, after issuance costs as of September 30, 2024.

On November 25, 2024, the Company entered into a licensing and collaboration agreement with Sarepta. Upon closing, the Company received $325.0 million for the purchase of 11,926,301 shares of common stock, at a price per share of $27.25, and received $500.0 million as an upfront payment on February 24, 2025. During the fourth quarter of fiscal 2025, a $100.0 million milestone payment from Sarepta was triggered, when the Company reached the first of two prespecified enrollment targets and subsequent authorization to dose escalate in a Phase 1/2 clinical study of ARO-DM1, an investigational RNAi therapeutic for the treatment of type 1 myotonic dystrophy (DM1). The Company received $53.2 million of Arrowhead common stock and $50.0 million cash from Sarepta to satisfy the milestone payment. The Company is eligible to receive additional milestones of up to $250.0 million over the 12 months from the date of this report, inclusive of the second DM1 milestone payment of $200.0 million earned in November 2025.

On August 29, 2025, the Company entered into a licensing and collaboration agreement with Novartis. Upon closing in October 2025, the Company received $200.0 million as an upfront payment. The Company projects it will be eligible to receive additional milestones of up to $25.0 million over the 12 months from the date of this report.

Based upon the Company's current cash and investment resources and operating plan, the Company expects to have sufficient liquidity to fund its operations through at least the next twelve months from the date of the issuance of these consolidated financial statements.

The following table presents a summary of cash flows:

	Year Ended September 30,		
	2025	2024	2023
	(in thousands)		
Cash Flow from:			
Operating activities	$ 179,552	$ (462,851)	$ (153,890)
Investing activities	(129,294)	(420,072)	(96,155)
Financing activities	74,006	870,520	253,053
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 124,264	$ (12,403)	$ 3,008
Cash, cash equivalents and restricted cash at end of period	$ 226,548	$ 102,685	$ 110,891

During the year ended September 30, 2025, cash flow provided by operating activities was $179.6 million, which was primarily due to $500.0 million upfront payment and $50.0 million milestone payment received as part of the Sarepta agreement, partially offset by the ongoing expenses related to the Company's research and development programs and general and administrative expenses. Cash used in investing activities amounted to $129.3 million, which was primarily attributable to capital expenditures of $22.7 million and investment purchases of $796.3 million, partially offset by proceeds from sales and maturities of investments of $689.6 million. Cash provided by financing activities of $74.0 million was related to cash received from the issuance of common stock in the Sarepta agreement, pre-funded warrants, and stock

option exercises. (See Note 6 — Stockholders' Equity of Notes to Consolidated Financial Statements of Part IV, “Item 15. Exhibits and Financial Statement Schedules.”), partially offset by $201.6 million repayments of the Credit Facility.

During the year ended September 30, 2024, cash flow used in operating activities was $462.9 million, which was primarily due to the ongoing expenses related to the Company’s research and development programs and general and administrative expenses, Cash used in investing activities amounted to $420.1 million, which was primarily attributable to capital expenditures of $141.5 million and investment purchases of $720.9 million, offset by proceeds from sales and maturities of investments of $442.3 million. Cash provided by financing activities of $870.5 million was related to cash received from the issuance of common stock, the Credit Facility, a milestone payment from Royalty Pharma, and stock option exercises.

See “Item 7. Management’s Discussion and Analysis of Financial Condition and Results of Operations” of the Company’s Form 10-K for the year ended September 30, 2024 for a discussion of cash flows from the year ended September 30, 2023.

Contractual Obligations

Based on the Company’s current operating plan, it believes that cash, cash equivalents and short-term investments as of September 30, 2025 will be sufficient to satisfy its near-term capital and operating needs. Recent and expected working and other capital requirements include the items described below.

- For information related to the Company’s future commitments for its collaboration and licensing agreements, see Note 2 of Notes to the Company’s Consolidated Financial Statements of Part IV, “Item 15. Exhibits and Financial Statement Schedules.”
- Amounts related to future lease payments for operating lease obligations at September 30, 2025 totaled $111.4 million, with $7.3 million expected to be paid within the next 12 months.
- Cash outflows for capital expenditures related to the manufacturing facility build-out at Verona, Wisconsin were $12.5 million in 2025 and $136.9 million in 2024. The Company expects to spend an additional $0.1 million to complete the build out of the facilities.
- A secured term loan facility of $500.0 million, which includes $400.0 million funded on the closing date with an additional $100.0 million at the Company’s option during the seven-year term of the agreement. The Company expects to make $40.0 million of prepayments on the facility within the next 12 months in additional to the $66.7 million prepayment made in November 2025 relating to the receipt of the upfront payment under the terms of the Novartis Collaboration Agreement. See Note 14 of Notes to the Company’s Consolidated Financial Statements of Part IV, “Item 15. Exhibits and Financial Statement Schedules.”
- Commitments related to the Company’s clinical, manufacturing and business operation related agreements totaled $665.5 million as of September 30, 2025. However, many of these agreements are cancellable.
- The Company has not entered into, nor does it currently have, any off-balance sheet arrangements (as defined under SEC rules).

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

The Company is subject to market risk exposures primarily due to its investing activities. The primary market risk exposure is change in interest rates. Adverse changes to rates may occur due to changes in the liquidity of a market or to changes in market perceptions of creditworthiness and risk tolerance.

The Company’s investment criteria are governed by its Investment Policy. The Company primarily invests its excess cash in securities of reputable financial institutions, corporations, and US government agencies with strong credit ratings. On September 30, 2023, the Company changed the classification of its investment securities from held-to-maturity to available-for-sale. This change enables the Company to sell securities to diversify its portfolio, reduce exposure to market risks, and provide flexibility to meet cash flow needs and new investment opportunities. Due to the relatively short-term nature of the investments that the Company holds, a hypothetical 100 basis point change in interest rates during any of the periods presented would not have had a material impact on the Company’s investment portfolio as of September 30, 2025.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

The information required by this item is included in Item 15 of this Annual Report on Form 10-K.

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE

None.

ITEM 9A. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

The Company maintains disclosure controls and procedures designed to ensure that information required to be disclosed in its reports filed under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the SEC rules and forms, and that such information is accumulated and communicated to its management, including its Chief Executive Officer and Chief Financial Officer, as appropriate, to allow for timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and management necessarily was required to apply its judgment in evaluating the cost benefit relationship of possible controls and procedures.

As required by Rule 13a-15(b) of the Exchange Act, the Company carried out an evaluation, under the supervision and with the participation of its management, including its Chief Executive Officer and Chief Financial Officer, of the effectiveness of the design and operation of the Company's disclosure controls and procedures as of the end of the period covered by this Annual Report on Form 10-K. Based on the foregoing, the Company's Chief Executive Officer and Chief Financial Officer concluded that the Company's disclosure controls and procedures were effective at the reasonable assurance level.

Management's Annual Report on Internal Control over Financial Reporting

The Company's management is responsible for establishing and maintaining adequate internal control over financial reporting as defined in Rules 13a-15(f) and 15d-15(f) of the Exchange Act. The Company's internal control over financial reporting is designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of its Consolidated Financial Statements for external purposes in accordance with GAAP.

This process includes those policies and procedures that:

(i) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the Company's assets;

(ii) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with GAAP, and that receipts and expenditures are being made only in accordance with authorizations of the Company's management and directors; and

(iii) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of the Company's assets that could have a material effect on the Company's financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of the internal control over financial reporting to future periods are subject to risk that controls may become inadequate because either conditions change or the degree of compliance with policies or procedures may deteriorate.

Management has assessed the effectiveness of the Company's internal control over financial reporting as of September 30, 2025. In making this assessment, the Company used the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in Internal Control-Integrated Framework (2013). Based on this assessment, management concluded that the Company's internal control over financial reporting was effective as of September 30, 2025.

KPMG LLP, the independent registered public accounting firm that audited the Consolidated Financial Statements included in this 2025 Annual Report on Form 10-K, has issued an audit report on the effectiveness of the Company's internal control over financial reporting as of September 30, 2025, which is included herein.

Changes in Internal Control Over Financial Reporting

There has been no change in the Company's internal control over financial reporting during the Company's most recent fiscal quarter that has materially affected, or is reasonably likely to materially affect, the Company's internal control over financial reporting. The Company regularly evaluates its controls and procedures and makes improvements in the

design and effectiveness of established controls and procedures and the remediation of any deficiencies which may be identified during this process.

ITEM 9B. OTHER INFORMATION

(b) Trading Plans

During the fiscal quarter ended September 30, 2025, the following directors and officers (as defined in Exchange Act Rule 16a-1(f)) adopted certain trading plans intended to satisfy Rule 10b5-1(c):

Name	Title	Adoption or Termination Date	Plan End Date	Shares Vesting and Subject to Sell-To-Cover [1]	Other Shares Being Sold (Subject to Certain Conditions)
Adeoye Olukotun	Board Member	09/04/2025	12/24/2025	n/a	10,000
Christopher Anzalone	President and Chief Executive Officer	09/19/2025	12/24/2026	50,000	n/a
Daniel Apel	Chief Financial Officer	09/16/2025	04/30/2026	25,000	n/a
James Hamilton	Chief Medical Officer, Head of R&D	09/03/2025	01/16/2026	72,500	n/a
Mauro Ferrari	Board Member	09/25/2025	12/31/2025	n/a	7,530
Patrick O'Brien	Chief Operating Officer & General Counsel	09/03/2025	01/07/2026	77,500	20,000
Victoria Vakiener	Board Member	09/03/2025	12/31/2025	n/a	10,040
William Waddill	Board Member	09/03/2025	12/31/2025	n/a	8,367

(1) This column indicates the total number of shares vesting, but the 10b5-1 Plan provides for the sale of only those shares necessary to satisfy payment of applicable withholding taxes.

ITEM 9C. DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS

Not applicable.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

The information called for by this Item will be incorporated by reference from the Company's Definitive Proxy Statement, under the headings Proposal One — Election of Directors, Equity Compensation Plan Information, Corporate Governance, Environmental and Social Commitment, Executive Compensation, and, if applicable, Delinquent Section 16(a) Reports — to be filed for the Company's 2026 Annual Meeting of Stockholders (the "Definitive Proxy Statement").

ITEM 11. EXECUTIVE COMPENSATION

The information called for by this Item will be incorporated by reference from the Definitive Proxy Statement, under the heading Executive Compensation.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

The information called for by this Item will be incorporated by reference from the Definitive Proxy Statement, under the heading Voting Securities of Principal Stockholders and Management.

ITEM 13. CERTAIN RELATIONSHIPS, RELATED TRANSACTIONS AND DIRECTOR INDEPENDENCE

The information called for by this Item will be incorporated by reference from the Definitive Proxy Statement, under the headings Review and Approval of Related-Party Transactions and Certain Relationships and Related Transactions, and Director Independence.

ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES

The information called for by this Item will be incorporated by reference from the Definitive Proxy Statement, under the heading Audit Fees.

PART IV

ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

The following documents are filed as part of this Annual Report on Form 10-K:

(1) Financial Statements.

See Index to Financial Statements and Schedule on page F-1.

(2) Financial Statement Schedules.

See Index to Financial Statements and Schedule on page F-1. All other schedules are omitted as the required information is not present or is not present in amounts sufficient to require submission of the schedule, or because the information required is included in the Consolidated Financial Statements or notes thereto.

SIGNATURE

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the Registrant has duly caused this report on Form 10-K to be signed on its behalf by the undersigned, thereunto duly authorized.

Dated: November 25, 2025

ARROWHEAD PHARMACEUTICALS, INC.

By: /s/ Christopher Anzalone
Christopher Anzalone
Chief Executive Officer

POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Christopher Anzalone and Daniel Apel, and each of them, as true and lawful attorneys-in-fact and agents, with full powers of substitution and resubstitution, for them and in their name, place and stead, in any and all capacities, to sign in any and all capacities (including, without limitation, the capacities listed below), this Annual Report on Form 10-K, any and all amendments thereto, and to file the same, with all exhibits thereto, and all other documents in connection therewith, with the Securities and Exchange Commission, and hereby grants to such attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and anything necessary to be done to enable the registrant to comply with the provisions of the Securities Exchange Act and all the requirements of the Securities and Exchange Commission, as fully to all intents and purposes as the undersigned might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents, or any of them, or their or his or her substitute, or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report on Form 10-K has been signed below by the following persons on behalf of the Registrant and in the capacities and on the dates indicated:

Signature	Title	Date
/s/ Christopher Anzalone **Christopher Anzalone**	Chief Executive Officer, President and Director, Chairman of the Board of Directors (Principal Executive Officer)	November 25, 2025
/s/ Daniel Apel **Daniel Apel**	Chief Financial Officer (Principal Financial and Accounting Officer)	November 25, 2025
/s/ Mauro Ferrari **Mauro Ferrari**	Director	November 25, 2025
/s/ Douglass Ingram **Douglass Ingram**	Director	November 25, 2025
/s/ Hongbo Lu **Hongbo Lu**	Director	November 25, 2025
/s/ Adeoye Olukotun **Adeoye Olukotun**	Director	November 25, 2025
/s/ Michael S. Perry **Michael S. Perry**	Director	November 25, 2025
/s/ Victoria Vakiener **Victoria Vakiener**	Director	November 25, 2025
/s/ William Waddill **William Waddill**	Director	November 25, 2025

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Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders
Arrowhead Pharmaceuticals, Inc.:

Opinion on the Consolidated Financial Statements

We have audited the accompanying consolidated balance sheets of Arrowhead Pharmaceuticals, Inc. and subsidiaries (the Company) as of September 30, 2025 and 2024, the related consolidated statements of operations and comprehensive income (loss), stockholders' equity, and cash flows for each of the years in the two-year period ended September 30, 2025, and the related notes (collectively, the consolidated financial statements). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of September 30, 2025 and 2024, and the results of its operations and its cash flows for each of the years in the two-year period ended September 30, 2025, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of September 30, 2025, based on criteria established in Internal Control – Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission, and our report dated November 25, 2025 expressed an unqualified opinion on the effectiveness of the Company's internal control over financial reporting.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these consolidated financial statements based on our audits. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matters

The critical audit matters communicated below are matters arising from the current period audit of the consolidated financial statements that were communicated or required to be communicated to the audit committee and that: (1) relate to accounts or disclosures that are material to the consolidated financial statements and (2) involved our especially challenging, subjective, or complex judgments. The communication of critical audit matters does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matters below, providing separate opinions on the critical audit matters or on the accounts or disclosures to which they relate.

Sufficiency of audit evidence over valuation of future royalty sales liability

As discussed in Note 13 to the consolidated financial statements, the Company records the obligations under the Royalty Pharma Agreement with Royalty Pharma Investments (RPI) at carrying value using the effective interest method. The Company amortizes the sale of future royalties utilizing the prospective method to estimate future royalties to be paid by the Company to RPI over the life of the arrangement. The Company periodically assesses the amount and timing of expected royalty payments using a combination of internal projections and forecasts from external sources. To the extent such payments differ from the Company's initial estimates, the Company will prospectively adjust the amortization of the royalty obligation and the effective interest rate. The estimate of the carrying value of the liability related to the sale of future royalties is derived from the estimate of future sales of olpasiran and the probability of success assumption. The estimate of future sales of olpasiran is based on key assumptions such as patient population, market penetration, olpasiran sales price, and the comparable guideline drug. The liability related to the sale of future royalties was $367,397 thousand as of September 30, 2025.

We identified the evaluation of the sufficiency of audit evidence over the determination of the carrying value of the liability related to the sale of future royalties as a critical audit matter. Subjective auditor judgment was required to evaluate the sufficiency of audit evidence obtained because of the level of audit effort associated with evaluating the carrying value of the liability related to the sale of future royalties.

The following are the primary procedures we performed to address this critical audit matter. We applied auditor judgment to determine the nature and extent of procedures to be performed over the evaluation of the carrying value of the liability related to the sale of future royalties. We evaluated the design and tested the operating effectiveness of certain internal controls related to management's valuation process, including the determination of the key assumptions into the carrying value of the liability related to the sale of future royalties as described above. We assessed the patient population and market penetration assumptions by comparing to independently sourced external market and industry data. We performed sensitivity analyses over the estimated olpasiran sales price and probability of success using independently sourced external market and industry data and evaluated the impact of changes in those assumptions on the carrying value of the liability related to the sale of future royalties. We assessed the reasonableness of the comparable guideline drug by evaluating against drugs similar to olpasiran in the marketplace. We evaluated the sufficiency of audit evidence obtained by assessing the cumulative results of the audit procedures performed and potential bias in the accounting estimate, including the appropriateness of the nature and extent of such evidence.

Evaluation of distinct performance obligations related to the license and collaboration agreement with Sarepta Therapeutics, Inc

As discussed in Notes 1 and 2 to the consolidated financial statements, the Company entered into an Exclusive License and Collaboration Agreement with Sarepta Therapeutics, Inc. (Sarepta). At contract inception, the Company assesses whether the goods or services promised within the contract are distinct and, therefore, represent a separate performance obligation, or whether they are not distinct and are combined with other goods and services until a distinct bundle is identified. The Company then determines the transaction price and allocates it to each performance obligation. The Company identified 17 performance obligations under the license and collaboration agreement with Sarepta. Fixed consideration of $833.6 million and an estimated variable consideration of $71.2 million were allocated to all performance obligations based on their relative standalone selling price.

We identified the evaluation of distinct performance obligations, including understanding the nature and significance of the contractual obligations and their standalone selling prices, related to the license and collaboration agreement with Sarepta as a critical audit matter. Subjective and complex auditor judgment was required to assess the Company's identification of distinct performance obligations, including evaluating the rights and obligations described in the agreement, their benefit to the customer, and the level of modification or customization among the performance obligations. In addition, subjective auditor judgment was required to evaluate certain significant assumptions in the discounted cash flow model used by management to determine the standalone selling prices of the distinct performance obligations, including the discount rate and certain forecasted expenses. The audit effort associated with assessing the discount rate assumption required specialized skills and knowledge.

The following are the primary procedures we performed to address this critical audit matter. We evaluated the design and tested the operating effectiveness of certain internal controls related to the Company's revenue recognition process, including management's identification of distinct performance obligations in the license and collaboration agreement and determination of the standalone selling prices. We evaluated management's identification of distinct performance obligations and the significance of the contractual obligations by obtaining and reading the license and collaboration agreement to gain an understanding of the contractual terms and conditions and the commitments being made in the agreement. We conducted inquiries with personnel responsible for clinical development to understand the nature of the research and development activities specific to the clinical, preclinical, and discovery stage programs to evaluate the nature of the commitments made to the customer. We evaluated certain forecasted expenses within the discounted cash flow model by comparing such amounts to external market and industry data. We involved valuation professionals with specialized skills and knowledge, who assisted in the assessment of the discount rate within the discounted cash flow model by comparing it to a discount rate that was independently developed using publicly available market data for comparable companies.

KPMG LLP
We have served as the Company's auditor since 2024.

San Diego, California
November 25, 2025

Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders
Arrowhead Pharmaceuticals, Inc.:

Opinion on Internal Control Over Financial Reporting

We have audited Arrowhead Pharmaceuticals, Inc. and subsidiaries' (the Company) internal control over financial reporting as of September 30, 2025, based on criteria established in Internal Control – Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission. In our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of September 30, 2025, based on criteria established in Internal Control – Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the consolidated balance sheets of the Company as of September 30, 2025 and 2024, the related consolidated statements of operations and comprehensive income (loss), stockholders' equity, and cash flows for each of the years in the two-year period ended September 30, 2025, and the related notes (collectively, the consolidated financial statements), and our report dated November 25, 2025 expressed an unqualified opinion on those consolidated financial statements.

Basis for Opinion

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting, included in the accompanying Management's Annual Report on Internal Control over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. Our audit of internal control over financial reporting included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, and testing and evaluating the design and operating effectiveness of internal control based on the assessed risk. Our audit also included performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

Definition and Limitations of Internal Control Over Financial Reporting

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

KPMG LLP
San Diego, California
November 25, 2025

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Board of Directors and
Stockholders of Arrowhead Pharmaceuticals, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated statements of operations and comprehensive income (loss), stockholders' equity, and cash flows for period ended September 30, 2023 of Arrowhead Pharmaceuticals, Inc. and Subsidiaries (the Company), and the related notes (collectively referred to as the financial statements). In our opinion, the consolidated financial statements present fairly, in all material respects, the consolidated results of the Company's operations and cash flows for the period ended September 30, 2023, in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's consolidated financial statements based on our audits. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Rose, Snyder & Jacobs LLP
We have served as the Company's auditor from 2004 to 2023.

Encino, California
November 29, 2023

Arrowhead Pharmaceuticals, Inc.
Consolidated Balance Sheets
(in thousands, except per share amounts)

	September 30,	
	2025	**2024**
ASSETS		
Current assets:		
Cash, cash equivalents and restricted cash	$ 88,706	$ 76,208
Cash at variable interest entity	137,842	26,477
Accounts receivable	6,824	—
Available-for-sale securities, at fair value	692,818	578,276
Prepaid expenses	10,933	9,537
Other current assets	13,516	4,973
Total current assets	950,639	695,471
Property, plant and equipment, net	382,515	386,032
Intangible assets, net	6,861	8,562
Right-of-use assets	43,891	45,255
Other assets	1,389	4,482
Total Assets	**$ 1,385,295**	**$ 1,139,802**
LIABILITIES, NONCONTROLLING INTEREST AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 17,674	$ 11,388
Accrued expenses	90,419	63,017
Accrued payroll and benefits	26,895	21,989
Lease liabilities	7,289	6,342
Deferred revenue	2,399	—
Credit facility	40,000	—
Other liabilities	10,811	432
Total current liabilities	195,487	103,168
Long-term liabilities:		
Lease liabilities, net of current portion	104,112	111,027
Liability related to the sale of future royalties	367,397	341,361
Credit facility, net of current portion	214,883	393,183
Total long-term liabilities	686,392	845,571
Commitments and contingencies (Note 7)		
Noncontrolling interest and stockholders' equity:		
Common stock, $0.001 par value: Authorized 290,000 shares; issued and outstanding 135,702 and 124,376 shares at September 30, 2025 and 2024, respectively	231	217
Additional paid-in capital	2,139,725	1,806,000
Accumulated other comprehensive income	6,443	4,750
Accumulated deficit	(1,627,154)	(1,625,523)
Treasury stock; at cost; 2,661 and 0 shares of common stock at September 30, 2025 and 2024, respectively	(53,193)	—
Total Arrowhead Pharmaceuticals, Inc. stockholders' equity	466,052	185,444
Noncontrolling interest	37,364	5,619
Total noncontrolling interest and stockholders' equity	503,416	191,063
Total Liabilities, Noncontrolling Interest and Stockholders' Equity	**$ 1,385,295**	**$ 1,139,802**

The accompanying notes are an integral part of these consolidated financial statements.

Arrowhead Pharmaceuticals, Inc.
Consolidated Statements of Operations and Comprehensive Income (Loss)
(in thousands, except per share amounts)

	Year Ended September 30,		
	2025	**2024**	**2023**
Revenue	$ 829,448	$ 3,551	$ 240,735
Operating expenses:			
Research and development	607,159	505,870	353,188
General and administrative	123,943	98,761	92,549
Total operating expenses	731,102	604,631	445,737
Operating income (loss)	98,346	(601,080)	(205,002)
Other (expense) income:			
Interest income	37,289	22,720	15,299
Interest expense	(89,361)	(32,352)	(18,326)
Other, net	5,259	(1,748)	1,538
Total other expense	(46,813)	(11,380)	(1,489)
Income (loss) before income tax expense and noncontrolling interest	51,533	(612,460)	(206,491)
Income tax expense (benefit)	21,419	(2,767)	2,784
Net income (loss) including noncontrolling interest	30,114	(609,693)	(209,275)
Net income (loss) attributable to noncontrolling interest, net of tax	31,745	(10,200)	(4,000)
Net loss attributable to Arrowhead Pharmaceuticals, Inc.	**$ (1,631)**	**$ (599,493)**	**$ (205,275)**
Net loss per share attributable to Arrowhead Pharmaceuticals, Inc.:			
Basic	$ (0.01)	$ (5.00)	$ (1.92)
Diluted	$ (0.01)	$ (5.00)	$ (1.92)
Weighted-average shares used in calculating			
Basic	133,758	119,784	106,750
Diluted	133,758	119,784	106,750
Other comprehensive loss, net of tax:			
Unrealized gains (losses) on available-for-sale securities	2,125	3,775	(2,964)
Foreign currency translation adjustments	(432)	4,197	(122)
Comprehensive income (loss) attributed to noncontrolling interest	31,745	(10,200)	(4,000)
Other comprehensive income (loss)	$ 31,807	$ (601,721)	$ (212,361)

The accompanying notes are an integral part of these consolidated financial statements.

Arrowhead Pharmaceuticals, Inc.
Consolidated Statements of Stockholders' Equity
(in thousands)

	Common Stock	Amount ($)	Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Common Stock in Treasury	Amount ($)	Non-controlling Interest	Totals
Balance at September 30, 2022	**105,960**	**$ 198**	**$ 1,219,213**	**$ (136)**	**$ (820,755)**	**—**	**$ —**	**$ 19,819**	**$ 418,339**
Stock-based compensation	—	—	78,130	—	—	—	—	—	78,130
Exercise of stock options	439	1	3,053	—	—	—	—	—	3,054
Common stock - restricted stock units vesting	913	1	(1)	—	—	—	—	—	—
Unrealized losses on available-for-sale securities	—	—	—	(2,964)	—	—	—	—	(2,964)
Foreign currency translation adjustments	—	—	—	(122)	—	—	—	—	(122)
Net loss	—	—	—	—	(205,275)	—	—	(4,000)	(209,275)
Balance at September 30, 2023	**107,312**	**$ 200**	**$ 1,300,395**	**$ (3,222)**	**$ (1,026,030)**	**—**	**$ —**	**$ 15,819**	**$ 287,162**

	Common Stock	Amount ($)	Additional Paid-In Capital	Accumulated Other Comprehensive (Loss) Income	Accumulated Deficit	Common Stock in Treasury	Amount ($)	Non-controlling Interest	Totals
Balance at September 30, 2023	**107,312**	**$ 200**	**$ 1,300,395**	**$ (3,222)**	**$ (1,026,030)**	**—**	**$ —**	**$ 15,819**	**$ 287,162**
Stock-based compensation	—	—	73,968	—	—	—	—	—	73,968
Exercise of stock options	226	—	2,389	—	—	—	—	—	2,389
Common stock - restricted stock units vesting	1,048	1	(1)	—	—	—	—	—	—
Common stock issued, net of offering costs	15,790	16	429,249	—	—	—	—	—	429,265
Unrealized gains on available-for-sale securities	—	—	—	3,775	—	—	—	—	3,775
Foreign currency translation adjustments	—	—	—	4,197	—	—	—	—	4,197
Net loss	—	—	—	—	(599,493)	—	—	(10,200)	(609,693)
Balance at September 30, 2024	**124,376**	**$ 217**	**$ 1,806,000**	**$ 4,750**	**$ (1,625,523)**	**—**	**$ —**	**$ 5,619**	**$ 191,063**

	Common Stock	Amount ($)	Additional Paid-In Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Common Stock in Treasury	Amount ($)	Non-controlling Interest	Totals
Balance at September 30, 2024	**124,376**	**$ 217**	**$ 1,806,000**	**$ 4,750**	**$ (1,625,523)**	**—**	**$ —**	**$ 5,619**	**$ 191,063**
Stock-based compensation	—	—	63,366	—	—	—	—	—	63,366
Exercise of stock options	535	—	3,983	—	—	—	—	—	3,983
Common stock - restricted stock units vesting	1,526	2	—	—	—	—	—	—	2
Common stock issued	11,926	12	241,376	—	—	—	—	—	241,388
Common stock - repurchase	—	—	—	—	—	(2,661)	(53,193)	—	(53,193)
Issuance of pre-funded warrants	—	—	25,000	—	—	—	—	—	25,000
Unrealized gains on available-for-sale securities	—	—	—	2,125	—	—	—	—	2,125
Foreign currency translation adjustments	—	—	—	(432)	—	—	—	—	(432)
Net (loss) income	—	—	—	—	(1,631)	—	—	31,745	30,114
Balance at September 30, 2025	**138,363**	**$ 231**	**$ 2,139,725**	**$ 6,443**	**$ (1,627,154)**	**(2,661)**	**$ (53,193)**	**$ 37,364**	**$ 503,416**

The accompanying notes are an integral part of these consolidated financial statements.

Arrowhead Pharmaceuticals, Inc.
Consolidated Statements of Cash Flows
(in thousands)

	Year Ended September 30,		
	2025	**2024**	**2023**
CASH FLOWS FROM OPERATING ACTIVITIES:			
Net income (loss)	$ 30,114	$ (609,693)	$ (209,275)
Adjustments to reconcile net income (loss) to net cash flow from operating activities:			
Stock-based compensation	63,366	73,968	78,130
Depreciation and amortization	23,928	18,595	12,493
Accretion of note premiums/discounts	(5,789)	(3,244)	(2,017)
Non-cash interest expense on liability related to the sale of future royalties	26,036	23,035	18,326
Non-cash interest expense on credit facility	63,325	9,317	—
Non-cash gain on treasury stock received	(3,193)	—	—
Realized loss on investments	—	80	—
Changes in operating assets and liabilities:			
Accounts receivable	(56,967)	—	1,410
Prepaid expenses and other current assets	(9,939)	(1,664)	11,603
Accounts payable	6,286	(5,536)	32,998
Accrued expenses	41,497	32,117	(14,965)
Deferred revenue	2,399	(866)	(129,183)
Operating lease, net	(4,605)	2,240	46,590
Other	3,094	(1,200)	—
Net cash provided by (used in) operating activities	179,552	(462,851)	(153,890)
CASH FLOWS FROM INVESTING ACTIVITIES:			
Purchases of property, plant and equipment	(22,666)	(141,469)	(176,737)
Purchases of investments	(796,258)	(720,947)	(246,141)
Proceeds from sales and maturities of investments	689,630	442,344	326,723
Net cash used in investing activities	(129,294)	(420,072)	(96,155)
CASH FLOWS FROM FINANCING ACTIVITIES:			
Proceeds from the exercises of stock options	3,983	2,389	3,053
Proceeds from the issuance of common stock, net of offering costs	—	429,265	—
Proceeds from the sale of future royalties	—	50,000	250,000
Proceeds from the issuance of warrants	25,000	—	—
Payment of debt issuance costs	(5,000)	(3,134)	—
Proceeds from the issuance of common stock	241,388	—	—
Proceeds from credit facility	—	392,000	—
Repayments of credit facility	(201,625)	—	—
Proceeds from Visirna credit agreement	10,260	—	—
Net cash provided by financing activities	74,006	870,520	253,053
Net increase (decrease) in cash, cash equivalents and restricted cash	124,264	(12,403)	3,008
Effect of exchange rate on cash, cash equivalents and restricted cash	(401)	4,197	(122)
CASH, CASH EQUIVALENTS AND RESTRICTED CASH:			
BEGINNING OF PERIOD	102,685	110,891	108,005
END OF PERIOD	$ 226,548	$ 102,685	$ 110,891
Supplementary disclosure of cash flows:			
Interest paid	$ (19)	$ —	$ —
Income taxes (refund) paid	$ 814	$ (3,744)	$ —
Treasury stock received to settle accounts receivable	$ 50,000	$ —	$ —
Supplementary disclosure of non-cash investing activities:			
Capital expenditures included in accrued expenses	$ 277	$ 4,206	$ 14,044

The accompanying notes are an integral part of these consolidated financial statements.

NOTE 1. ORGANIZATION AND SIGNIFICANT ACCOUNTING POLICIES

General

Arrowhead Pharmaceuticals, Inc. and its subsidiaries (referred to herein collectively as the "Company") are primarily engaged in developing medicines that treat intractable diseases by silencing the genes that cause them. Using a broad portfolio of RNA chemistries and efficient modes of delivery, the Company's therapies trigger the RNA interference mechanism to induce rapid, deep and durable knockdown of target genes. RNA interference ("RNAi") is a mechanism present in living cells that inhibits the expression of a specific gene, thereby affecting the production of a specific protein. The Company's RNAi-based therapeutics may leverage this natural pathway of gene silencing to target and shut down specific disease-causing genes.

The following table presents the Company's current pipeline:

Therapeutic Area	Name	Stage	Product Rights
Cardiometabolic	plozasiran	Phase 3	Arrowhead[(1)]
	zodasiran	Phase 3	Arrowhead
	olpasiran	Phase 3	Amgen
	ARO-PNPLA3	Phase 1	Arrowhead
	GSK4532990	Phase 2b	GSK
	ARO-INHBE	Phase 1/2a	Arrowhead
	ARO-ALK7	Phase 1/2a	Arrowhead
	ARO-DIMERPA	Phase 1/2a	Arrowhead
Pulmonary	ARO-RAGE	Phase 1/2a	Arrowhead
	SRP-1002 (ARO-MMP7)	Phase 1/2a	Sarepta
Liver	fazirsiran	Phase 3	Takeda and Arrowhead
	daplusiran/tomligisiran	Phase 2	GSK
Neuromuscular	SRP-1001 (ARO-DUX4)	Phase 1/2a	Sarepta
	SRP-1003 (ARO-DM1)	Phase 1/2a	Sarepta
	SRP-1004 (ARO-ATXN2)	Phase 1/2a	Sarepta
	ARO-SNCA	Pre-clinical	Novartis
	ARO-MAPT	Phase 1/2a	Arrowhead
Other	ARO-C3	Phase 1/2a	Arrowhead
	ARO-CFB	Phase 1/2a	Arrowhead

(1) Greater China rights for plozasiran are out-licensed to Sanofi.

The Company operates lab facilities in California and Wisconsin, where its research and development activities, including the development of RNAi therapeutics, take place. The Company also operates an active pharmaceutical ingredient manufacturing and supporting laboratory facility in Verona, Wisconsin. The Company's principal executive offices are located in Pasadena, California.

Consolidation and Basis of Presentation

The Consolidated Financial Statements include the accounts of Arrowhead Pharmaceuticals, Inc. and its subsidiaries (wholly-owned subsidiaries and a variable interest entity for which the Company is the primary beneficiary). Subsidiaries refer to Arrowhead Madison, Inc., Arrowhead Australia Pty Ltd., Arrowhead Pharmaceuticals Ireland Limited, Arrowhead Pharmaceuticals NZ Limited, and Visirna Therapeutics, Inc. ("Visirna"). For subsidiaries in which the Company owns or is exposed to less than 100% of the economics, the Company records net loss attributable to noncontrolling interests in its consolidated statements of operations equal to the percentage of the economic or ownership interests retained in such entity by the respective noncontrolling party.

The Consolidated Financial Statements have been prepared in conformity with U.S. generally accepted accounting principles ("GAAP"). All intercompany transactions and balances have been eliminated.

The Company operates as a single segment as the chief operating decision maker, or CODM, reviews operating results on an aggregate basis and manages the operations as a single operating segment. Refer to Note 16, *Segment Reporting*, for further details on the segment information.

Liquidity

The Company's primary sources of financing have been through the sale of its equity securities, credit facility, revenue from its licensing and collaboration agreements and the sale of certain future royalties. Research and development activities have required significant investment since the Company's inception and are expected to continue to require significant cash expenditure in the future, particularly as the Company's pipeline of drug candidates and its headcount have both expanded. Additionally, significant investment will be required as the Company's pipeline matures into later stage clinical trials and commercialization efforts.

As of September 30, 2025, the Company had $226.5 million in cash, cash equivalents and restricted cash ($1.9 million in restricted cash) and $692.8 million in available-for-sale securities to fund operations. During the year ended September 30, 2025, the Company's cash, cash equivalents and restricted cash and investments balance increased by $238.4 million, which was primarily due to the $500.0 million as an upfront payment under the Sarepta agreement and $325.0 million in the form of an equity investment under the Sarepta agreement, $100.0 million relating to the achievement of partnership milestone achievement of which $50.0 million was settled in cash and the remaining $50.0 million was settled through the repurchase of Company's common stock , $37.3 million interest income earned on investments, and $25.0 million in the form of pre-funded warrants, partially offset by ongoing expenses related to the Company's research and development programs, $201.6 million payments on its credit facility and $22.7 million relating to capital expenditure.

In total, the Company is eligible to receive up to $13.4 billion in additional developmental, regulatory and sales milestones, and may receive various royalties on net sales from its licensing and collaboration agreements, subject to the terms and conditions of those agreements.

Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements, and the reported amounts of revenues and expenses during the reporting period. On an ongoing basis, the Company evaluates its estimates, judgments and assumptions. The Company bases its estimates on historical experience and on various other assumptions that it believes are reasonable, the results of which form the basis for making judgments about the carrying values of assets, liabilities and equity and the amount of revenue and expense. Actual results could materially differ from those estimates.

Cash, Cash Equivalents and Restricted Cash

All highly liquid interest-bearing investments are classified as cash equivalents. These investments mainly include term deposits, money market instruments, corporate debt securities, and certificate of deposits with maturities of three months or less when purchased. The carrying value of these cash equivalents approximate fair value.

There was $1.9 million and $3.5 million restricted cash at September 30, 2025 and 2024, respectively, that is primarily held as collateral associated with letters of credit for the Company's facility leases.

Investments

The Company classified all of its investments in debt securities as available-for-sale and as current assets as they represent the investment of funds available for current operations as of September 30, 2025 and 2024. The available-for-sale investments may consist of investment-grade interest bearing instruments, primarily corporate debt securities, U.S. government and agency securities, commercial notes, certificate of deposits, and municipal securities, which are accounted for at fair value. Dividends from these funds were automatically re-invested. Changes in fair values are reported as unrealized gains or losses and are recorded in the Company's consolidated statement of operations and comprehensive income (loss).

The Company evaluates its investments for impairment based on a security-specific analysis as of each balance sheet date. If the fair value of a security is below its amortized cost, the Company first assesses whether it intends to sell the security or is more likely than not required to sell it before recovery of its amortized cost. If neither condition is met, the Company evaluates whether a portion of the decline is attributable to credit loss. Any credit-related impairment is recorded as an allowance for credit losses through earnings, with non-credit-related unrealized losses recorded in other

comprehensive income (loss). The Company did not recognize any credit loss relating to its investment for the years ended September 30, 2025, 2024, and 2023.

Concentration of Credit Risk

Financial instruments that potentially expose the Company to concentration of credit risk primarily consist of cash, cash equivalents and restricted cash and investments. As of September 30, 2025 and 2024, the Company's investments were primarily invested in money market funds, U.S. Government, commercial paper, and corporate debt securities and term deposit through highly rated financial institutions. The Company has established guidelines relative to diversification and maturities that maintain safety and liquidity. The Company periodically reviews and modifies these guidelines to maximize trends in yields and interest rates without compromising safety and liquidity. The Company also maintains several bank accounts at two financial institutions for its operations. These accounts are insured by the Federal Deposit Insurance Corporation (FDIC) for up to $250,000 per institution.

In addition, Visirna maintains several deposit and term accounts in mainland China, Hong Kong, and Singapore. Cash balances are held with various local and international financial institutions, which are subject to their respective jurisdictional deposit insurance programs. The Deposit Insurance Fund Management Corporation in China provides coverage of up to RMB 500,000 per depositor per institution, and the Hong Kong Deposit Protection Scheme provides protection of up to HKD 800,000 per depositor per bank, and the Singapore Deposit Insurance Corporation provides coverage of up to SGD 100,000 per depositor per member bank.

Property, Plant and Equipment

Property, plant and equipment are recorded at cost, net of accumulated depreciation. Depreciation expense is recorded on a straight-line basis over the estimated useful lives of the assets. Leasehold improvements are amortized over the shorter of the asset life or lease term. Construction in progress reflects amounts incurred for construction or improvements of property, plant and equipment that have not been placed in service. Upon disposition, the cost and accumulated depreciation of assets retired or sold are removed from the respective asset category, and any gain or loss is recognized in the Company's consolidated statement of operations and comprehensive income (loss).

The estimated useful lives of property, plant and equipment are as follows (in years):

	Estimated Useful Lives
Buildings	39
Research equipment	5
Manufacturing equipment	7 to 10
Furniture	7
Computers and software	3 to 5
Leasehold improvements	Shorter of asset life or lease term

The Company periodically assesses long-lived assets or asset groups, including property, plant and equipment, for recoverability when events or changes in circumstances indicate that their carrying amounts may not be recoverable. If the Company identifies an indicator of impairment, the Company assesses recoverability by comparing the carrying amount of the asset to the sum of the undiscounted cash flows expected to result from the use and the eventual disposal of the asset. An impairment loss is recognized when the carrying amount is not recoverable and is measured as the excess of carrying value over fair value. There were no impairment charges during the years ended September 30, 2025, 2024, and 2023.

Intangible Assets Subject to Amortization

Intangible assets subject to amortization include certain patents and license agreements. The Company evaluates intangible assets for impairment annually or whenever events or changes in circumstances indicate that it is more likely than not that the carrying amount of intangible assets may exceed their implied fair values. No impairment charges were recorded during the years ended September 30, 2025, 2024, and 2023.

Leases

The Company determines whether a contract is, or contains, a lease at inception. All of the Company's leases are classified as operating leases. Leases with terms greater than one-year are recognized on the Company's consolidated balance sheets as right-of-use assets that represent the Company's right to use an underlying asset for the lease term, and lease liabilities that represent its obligation to make lease payments arising from the lease. Lease assets and liabilities are recognized at the lease commencement date based on the estimated present value of lease payments over the expected lease term. As of September 30, 2025 and 2024, the Company is not reasonably certain that it will exercise renewal options for any lease facilities. Therefore, these options are not included in the right-of-use assets and liabilities.

The interest rate implicit in lease contracts is typically not readily determinable. As such, the Company utilizes the appropriate incremental borrowing rate, which is the rate incurred to borrow on a collateralized basis an amount equal to the lease payments over a similar term and in a similar economic environment. The Company records expense to recognize lease payments on a straight-line basis over the expected lease term. Costs determined to be variable and not based on an index or rate are not included in the measurement of the lease liability and are expensed as incurred.

Clinical Accruals

The Company accrues liabilities for products received or services incurred, particularly for ongoing clinical trials, where service providers have not yet billed or where billing terms do not align with the timing of the work performed as of the period-end. These costs mainly include third-party clinical management or clinical research organization (CRO), laboratory analysis, and investigator fees. Accrual estimates may be based on vendor communications to obtain pending invoices and/or estimates for services performed during the period. In some cases, these estimates require significant judgment, drawing on an understanding of research and development programs, services provided during the period, prior experience, and, where applicable, the expected duration of third-party contracts. Actual costs upon settlement may differ significantly from the accrued amounts in the Company's consolidated financial statements, though historical estimates have not differed materially from actual costs.

Revenue Recognition

The revenue standard provides a five-step framework for recognizing revenue as control of promised goods or services is transferred to a customer at an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. To determine revenue recognition for arrangements that it determines are within the scope of the revenue standard, the Company performs the following five steps: (i) identify the contract; (ii) identify the performance obligations; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the Company satisfies a performance obligation. At contract inception, the Company assesses whether the goods or services promised within each contract are distinct and, therefore, represent a separate performance obligation, or whether they are not distinct and are combined with other goods and services until a distinct bundle is identified. The Company then determines the transaction price, which typically includes upfront payments and any variable consideration that it determines is probable to not cause a significant reversal in the amount of cumulative revenue recognized when the uncertainty associated with the variable consideration is resolved. The Company then allocates the transaction price to each performance obligation and recognizes the associated revenue when (or as) each performance obligation is satisfied.

The Company recognizes the transaction price allocated to upfront license payments as revenue upon delivery of the license to the customer and resulting ability of the customer to use and benefit from the license, if the license is determined to be distinct from the other performance obligations identified in the contract. These other performance obligations are typically to perform research and development services for the customer, often times relating to the candidate that the customer is licensing. If the license is not considered to be distinct from other performance obligations, the Company assesses the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied at a point in time or over time. If the performance obligation is satisfied over time, the Company then determines the appropriate method of measuring progress for purposes of recognizing revenue from license payments. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the related revenue recognition.

Typically, the Company's collaboration agreements entitle it to additional payments upon the achievement of milestones or royalties on sales. The milestones are generally categorized into three types: development milestones, generally based on the initiation of toxicity studies or clinical trials; regulatory milestones, generally based on the submission, filing or approval of regulatory applications such as a New Drug Application ("NDA") in the United States; and sales-based milestones, generally based on meeting specific thresholds of sales in certain geographic areas. The Company evaluates whether it is probable that the consideration associated with each milestone or royalty will not be subject to a significant reversal in the cumulative amount of revenue recognized. Amounts that meet this threshold are included in the transaction price using the most likely amount method, whereas amounts that do not meet this threshold are excluded from the transaction price until they meet this threshold. At the end of each subsequent reporting period, the Company re-evaluates the probability of a significant reversal of the cumulative revenue recognized for its milestones and royalties, and, if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect revenues and net income in the Company's consolidated statements of operations and comprehensive income (loss). Typically, milestone payments and royalties are achieved after the Company's performance obligations associated with the collaboration agreements have been completed and after the customer has assumed responsibility for the respective clinical or preclinical program. Milestones or royalties achieved after the Company's performance obligations have been completed are recognized as revenue in the period the milestone or royalty was achieved. If a milestone payment is achieved during the performance period, the milestone payment would be

recognized as revenue to the extent performance had been completed at that point, and the remaining balance would be recorded as deferred revenue.

The revenue standard requires the Company to assess whether a significant financing component exists in determining the transaction price. The Company performs this assessment at the onset of its licensing or collaboration agreements. Typically, a significant financing component does not exist because the customer is paying for a license or services in advance with an upfront payment. Additionally, future royalty payments are not substantially within the control of the Company or the customer.

Further, the revenue standard requires the Company to allocate the arrangement consideration on a relative standalone selling price basis for each performance obligation after determining the transaction price of the contract and identifying the performance obligations to which that amount should be allocated. The relative standalone selling price is defined in the revenue standard as the price at which an entity would sell a promised good or service separately to a customer. If other observable transactions in which the Company has sold the same performance obligation separately are not available, the Company estimates the standalone selling price of each performance obligation. Key assumptions to determine the standalone selling price may include forecasted revenues, development timelines and costs, reimbursement rates for personnel costs, discount rates and probabilities of technical and regulatory success.

Whenever the Company determines that goods or services promised in a contract should be accounted for as a combined performance obligation over time, the Company determines the period over which the performance obligations will be performed and revenue will be recognized. Revenue is recognized using the input method; Labor hours, costs incurred or patient visits in clinical trials are typically used as the measure of performance. Significant management judgment is required in determining the level of effort required under an arrangement and the period over which the Company is expected to complete its performance obligations. If the Company determines that the performance obligation is satisfied over time, any upfront payment received is initially recorded as deferred revenue on its consolidated balance sheets.

Certain judgments affect the application of the Company's revenue recognition policy. For example, the Company records short-term (less than one year) and long-term (over one year) deferred revenue based on its best estimate of when such revenue will be recognized. This estimate is based on the Company's current operating plan and, the Company may recognize a different amount of deferred revenue over the next 12-month period if its plan changes in the future.

Collaborative Arrangements

The Company analyzes its collaborative arrangements to assess whether such arrangements involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards, and therefore are within the scope of Financial Accounting Standards Board ("FASB") Topic 808 - *Collaborative Arrangements*. For collaborative arrangements that contain multiple elements, the Company determines which units of account are deemed to be within the scope of Topic 808 and which units of account are more reflective of a vendor-customer relationship, and therefore are within the scope of Topic 606 - *Revenue for Contracts from Customers*. For units of account that are accounted for pursuant to Topic 808, an appropriate recognition method is determined and applied consistently, either by analogy to appropriate accounting literature or by applying a reasonable accounting policy election. For collaborative arrangements that are within the scope of Topic 808, the Company evaluates the income statement classification for presentation of amounts due to or owed from other participants associated with multiple units of account in a collaborative arrangement based on the nature of each activity. Payments or reimbursements that are the result of a collaborative relationship instead of a customer relationship, such as co-development and co-commercialization activities, are recorded as increases or decreases to research and development expense or general and administrative expense, as appropriate.

Research and Development Expenses

Research and development costs are charged to expense as incurred. Included in research and development costs are operating costs, facilities, supplies, external services, clinical trial and manufacturing costs, overhead directly related to the Company's research and development operations, and costs to acquire technology licenses.

Stock-Based Compensation

Share-based compensation expense for all stock grants are based on their estimated grant-date fair value. The fair value of stock option awards is estimated using the Black-Scholes option valuation model which requires the input of subjective assumptions to calculate the value of stock options. The Company uses historical data and other information to estimate the expected price volatility and the expected forfeiture rate for stock option awards. For restricted stock units, the value of the award is based on the Company's stock price at the grant date. For performance-based restricted stock unit awards, the value of the award is based on the Company's stock price at the grant date, with consideration given to the

probability of the performance condition being achieved. Expense is recognized over the vesting period for all awards and commences at the grant date for time-based awards and upon the Company's determination that the achievement of such performance conditions is probable for performance-based awards. This determination requires significant judgment by management.

Income Taxes

Deferred tax assets and liabilities are recognized for the estimated future tax consequences attributable to differences between the financial reporting basis and the respective tax basis of the Company's assets and liabilities, and expected benefits of utilizing net operating loss, capital loss, and tax-credit carryforwards. The Company assesses the likelihood that its deferred tax assets will be realized and, to the extent management does not believe these assets are more likely than not to be realized, a valuation allowance is established. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates or laws is recognized in earnings in the period that includes the enactment date.

Variable Interest Entity ("VIE")

A VIE is an entity that, by design, either (i) lacks sufficient equity to permit the entity to finance its activities without additional subordinated financial support from other parties; or (ii) has equity investors that do not have the ability to make significant decisions relating to the entity's operations through voting rights, or do not have the obligation to absorb the expected losses, or do not have the right to receive the residual returns of the entity. The primary beneficiary of a VIE is required to consolidate the assets and liabilities of the VIE. The primary beneficiary is the party that has both (i) the power to direct the activities of the VIE that most significantly impact the VIE's economic performance, and (ii) the obligation to absorb losses or the right to receive benefits from the VIE that could potentially be significant to the VIE through its interest in the VIE.

On April 25, 2022, the Company entered into a license agreement with Visirna (Note 2) and consolidated Visirna's financial statements in which the Company has a direct controlling financial interest based on the VIE model.

The Company considers all the facts and circumstances, including its role in establishing Visirna and its ongoing rights and responsibilities to assess whether the Company has the power to direct the activities of Visirna. In general, the parties that make the most significant decisions affecting a VIE and have the right to unilaterally remove those decision-makers are deemed to have the power to direct the activities of a VIE.

The Company also considers all of its economic interests to assess whether the Company has the obligation to absorb losses of Visirna or the right to receive benefits from it that could potentially be significant to Visirna. This assessment requires the Company to apply judgment in determining whether these interests, in the aggregate, are considered potentially significant to Visirna. Factors considered in assessing the significance include: the design of Visirna, including its capitalization structure, subordination of interests, payment priority, and the reasons why the interests are held by the Company.

At Visirna's inception, the Company determined it was the primary beneficiary and that Visirna should be consolidated based on the facts and circumstances. The Company performs ongoing reassessments of the VIE based on reconsideration events and reevaluates whether a change to the consolidation conclusion is required. As of September 30, 2025, there were no events to be reconsidered in the consolidation.

Net Loss per Share

Net loss per share is computed using the weighted-average number of common shares outstanding during the period. Diluted earnings per share is computed using the weighted-average number of common shares and dilutive potential common shares outstanding during the period. Dilutive potential common shares primarily consist of stock options and restricted stock units outstanding.

During the years ended September 30, 2025, 2024 and 2023, the calculation of the effect of dilutive stock options and restricted stock units excluded all stock options and restricted stock units outstanding during the period due to their anti-dilutive effect.

Foreign Currency Translation Adjustments

Three of the Company's wholly-owned subsidiaries' functional currencies is not the United States dollar, which is the Company's reporting currency. Assets and liabilities are translated at the exchange rate in effect at the balance sheet date. Revenues and expenses are translated at the average rate of exchange prevailing during the reporting period. Translation adjustments arising from the use of different exchange rates from period to period are included in the accumulated other comprehensive income (loss).

Segment Information

The Company operates as a single segment because its CODM reviews operating results on an aggregate basis and manages its operations as a single operating segment.

Recent Accounting Pronouncements

In January 2025, the Financial Accounting Standards Board ("FASB") issued Accounting Standard Update ("ASU") 2024-03, *Income Statement—Reporting Comprehensive Income—Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses,* in November 2024, and ASU 2025-01, *Clarifying the Effective Date*. These updates require entities to provide disaggregated disclosures of income statement expenses. The ASUs do not affect the expense captions presented on the face of the income statement but instead require the disaggregation of certain expense captions into specified categories within the footnotes to the financial statements. The ASUs will become effective for the Company beginning October 1, 2027, and the Company is currently evaluating the impact on its consolidated financial statements and related disclosures.

In December 2023, the FASB issued Accounting Standard Update ("ASU") 2023-09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures*, to improve its income tax disclosure requirements. Under the guidance, entities must annually (1) disclose specific categories in the rate reconciliation and (2) provide additional information for reconciling items that meet a quantitative threshold. This guidance became effective for the Company beginning on October 1, 2025. The Company does not expect any material impact on its consolidated financial statements and related disclosures resulting from applying this ASU.

In November 2023, the FASB issued ASU 2023-07, *Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures*, which is intended to improve reportable segment disclosure requirements, primarily through additional disclosures about significant segment expenses. The guidance requires public companies with a single reportable segment to provide all disclosures required under ASC 280. In addition, the guidance requires public companies to include in interim reports all disclosures related to a reportable segment's profit or loss and assets that are currently required in annual reports. The standard is effective for fiscal years beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024, with early adoption permitted. The amendments should be applied retrospectively to all prior periods presented in the financial statements. This ASU is applicable to the Company's Annual Report on Form 10-K for the year ending September 30, 2025, and subsequent interim periods. Refer to Note 16, *Segment Reporting*, for further details on segment information.

On July 4, 2025, the One Big Beautiful Bill Act ("OBBBA") was enacted in the U.S. The OBBBA includes significant provisions, such as the permanent extension of certain expiring provisions of the Tax Cuts and Jobs Act, modifications to the international tax framework and the restoration of favorable tax treatment for certain business provisions. The legislation has multiple effective dates, with certain provisions effective in 2025 and others implemented through 2027. ASC 740, "Income Taxes", requires the effects of changes in tax rates and laws to be recognized in the period in which the legislation is enacted. The Company has implemented OBBBA in the fourth quarter of fiscal 2025. Refer to Note 11, *Income Taxes*, for further details.

NOTE 2. COLLABORATION AND LICENSE AGREEMENTS

The following table provides a summary of revenue recognized:

	Year Ended September 30,		
	2025	2024	2023
		(in thousands)	
GSK	$ 2,645	$ 2,685	$ 29,657
Horizon	—	—	23,206
Takeda	—	866	162,516
Janssen	—	—	356
Amgen	—	—	25,000
Sarepta	696,803	—	—
Sanofi	$ 130,000	$ —	$ —
Total	$ 829,448	$ 3,551	$ 240,735

The following table summarizes the balance of receivables and contract liabilities related to the Company's collaboration and license agreements:

	September 30,	
	2025	2024
	(in thousands)	
Receivables included in accounts receivable	$ 6,824	$ —
Contract liabilities included in deferred revenue	$ 2,399	$ —

GlaxoSmithKline Intellectual Property (No. 3) Limited ("GSK")

GSK-HSD License Agreement

On November 22, 2021, GSK and the Company entered into an Exclusive License Agreement (the "GSK-HSD License Agreement"). Under the GSK-HSD License Agreement, GSK has received an exclusive license for GSK-4532990 (formerly ARO-HSD). The exclusive license is worldwide with the exception of greater China. GSK is wholly responsible for all clinical development and commercialization of GSK-4532990 in its territory.

The Company has completed its performance obligation related to this agreement, and the upfront payment of $120.0 million was fully recognized in the year ended September 30, 2022. Further, GSK dosed the first patient in a Phase 2b trial in March 2023 and paid a $30.0 million milestone payment to the Company in the third quarter of fiscal 2023.

The Company is eligible for an additional payment of $100.0 million upon achieving the first patient dosed in a Phase 3 trial. Furthermore, should the Phase 3 trial read out positively, and the potential new medicine receives regulatory approval in major markets, the deal provides for commercial milestone payments to the Company of up to $190.0 million at first commercial sale, and up to $590.0 million in sales-related milestone payments. The Company is further eligible to receive tiered royalties on net product sales in a range of mid-teens to twenty percent.

GSK-HBV Agreement

On December 11, 2023, the Company entered into an Amended and Restated License Agreement with GSK (the "GSK-HBV Agreement") pursuant to which GSK received a worldwide, exclusive license to develop and commercialize daplusiran/tomligisiran (GSK5637608, formerly JNJ-3989), the Company's third-generation subcutaneously administered RNAi therapeutic candidate being developed as a potential therapy for patients with chronic hepatitis B virus infection.

Under the terms of the GSK-HBV Agreement, the Company received $2.7 million in December 2023, upon signing the amended GSK-HBV Agreement. Further, GSK dosed the fifth patient in a Phase 2 trial in December 2024, triggering a $2.5 million milestone payment to the Company which was paid in the second quarter of fiscal 2025. The Company is eligible to receive up to $830.0 million in development and sales milestone payments under the GSK-HBV Agreement.

There were no contract assets and liabilities recorded as of September 30, 2025.

Horizon Therapeutics Ireland DAC ("Horizon")

In June 2021, Horizon and the Company entered into a collaboration and license agreement (the "Horizon License Agreement"). Under the terms of the Horizon License Agreement, Horizon received a worldwide exclusive license for HZN-457, a clinical-stage medicine being developed by Horizon as a potential treatment for people with uncontrolled gout.

On October 6, 2023, Amgen completed its acquisition of Horizon and subsequently notified the Company of Amgen's intent to terminate the HZN-457 license. Horizon exercised its right to terminate the Horizon License Agreement for convenience, which took effect on December 21, 2023.

Takeda Pharmaceutical Company Limited ("Takeda")

In October 2020, Takeda and the Company entered into an Exclusive License and Co-Funding Agreement (the "Takeda License Agreement"). Under the Takeda License Agreement, Takeda and the Company will co-develop the Company's fazirsiran program (formerly TAK-999 and ARO-AAT), the Company's second-generation subcutaneously administered RNAi therapeutic candidate being developed as a treatment for liver disease associated with alpha-1 antitrypsin deficiency. Within the United States, fazirsiran, if approved, will be co-commercialized under a 50/50 profit sharing structure. Outside the United States, Takeda received an exclusive license to commercialize fazirsiran and will lead the global commercialization strategy, while the Company will be eligible to receive tiered royalties of 20% to 25% on net sales.

The Company determined that the key deliverables included the license and certain research and development services including the Company's responsibilities to complete the initial portion of the SEQUOIA study, to complete the ongoing Phase 2 AROAAT2002 study, and to ensure certain manufacturing of fazirsiran drug product is completed and delivered to Takeda (the "Takeda R&D Services"). Due to the specialized and unique nature of these Takeda R&D Services and their direct relationship with the license, the Company determined that these deliverables represent one distinct bundle and, thus, one performance obligation. Takeda is responsible for managing clinical development and commercialization outside the United States. Within the United States, the Company and Takeda are responsible in the co-development and co-commercialization efforts. The Company considers the collaborative activities, including the co-development and co-commercialization, to be a separate unit of account within Topic 808, and as such, these co-funding amounts are recorded as research and development expenses or general and administrative expenses, as appropriate.

Under the terms of the Takeda License Agreement, the Company received $300.0 million as an upfront payment in January 2021 and an additional $40.0 million upon Takeda's initiation of a Phase 3 REDWOOD clinical study of fazirsiran in March 2023, and is eligible to receive up to $527.5 million in additional potential development, regulatory and commercial milestones.

The Company allocated the total $300.0 million initial transaction price to its one distinct performance obligation for the fazirsiran license and the associated Takeda R&D Services. The Company has substantially completed its performance obligation under the Takeda License Agreement by December 31, 2023. As such, all revenue has been fully recognized as of December 31, 2023. There were no further deferred revenue and contract liabilities as of September 30, 2025.

The Company recorded $31.3 million as accrued expenses as of September 30, 2025 that was primarily driven by co-development and co-commercialization activities.

Janssen Pharmaceuticals, Inc. ("Janssen")

On April 7, 2023, Janssen voluntarily terminated its collaboration agreement with the Company and the Company regained full rights to ARO-PNPLA3, formerly called JNJ-75220795. There are no currently active trials for ARO-PNPLA3.

Further, on December 11, 2023, the Company entered into the GSK-HBV Agreement, as discussed above, pursuant to which GSK received an exclusive license for JNJ-3989 (formerly ARO-HBV). JNJ-3989 had previously been licensed to Janssen in October 2018.

Amgen Inc. ("Amgen")

In September 2016, Amgen and the Company entered into two collaboration and license agreements and a common stock purchase agreement. Under the Second Collaboration and License Agreement (the "Olpasiran Agreement"), Amgen received a worldwide, exclusive license to the Company's novel RNAi olpasiran (previously referred to as AMG-890 or ARO-LPA) program. These RNAi molecules are designed to reduce elevated lipoprotein(a), which is a genetically validated, independent risk factor for atherosclerotic cardiovascular disease. Under the Olpasiran Agreement, Amgen is wholly responsible for clinical development and commercialization.

The Company has substantially completed its performance obligations under the Olpasiran Agreement. There were no contract assets and liabilities recorded as of September 30, 2025.

In November 2022, Royalty Pharma Investments 2019 ICAV ("Royalty Pharma") and the Company entered into a Royalty Purchase Agreement with Royalty Pharma (the "Royalty Pharma Agreement"). In consideration for the payments under the Royalty Pharma Agreement, Royalty Pharma is entitled to receive all royalties otherwise payable by Amgen to the Company under the Olpasiran Agreement. The Company remains eligible to receive up to an additional $485.0 million in remaining development, regulatory and sales milestone payments payable from Amgen and Royalty Pharma. See Note 13.

Sarepta Therapeutics, Inc.

On November 25, 2024, the Company entered into an Exclusive License and Collaboration Agreement (the "Sarepta Collaboration Agreement") with Sarepta for the development and commercialization of multiple clinical and preclinical programs in rare, genetic diseases of the muscle, central nervous system, and lungs. The Company concurrently entered into a Stock Purchase Agreement (the "Stock Purchase Agreement") with Sarepta (see Note 6).

Under the Sarepta Collaboration Agreement, Sarepta received an exclusive sublicensable worldwide license to SRP-1001 (formerly ARO-DUX4), SRP-1003 (formerly ARO-DM1), SRP-1002 (formerly ARO-MMP7), and SRP-1004 (formerly ARO-ATXN2) clinical stage programs (the "C1" programs). Sarepta also received an exclusive sublicensable

worldwide license to the Company's ARO-HTT, ARO-ATXN1, and ARO-ATXN3 preclinical stage programs (the "C2" programs). The Company will perform certain research and development activities for the C1 and C2 programs.

Further, Sarepta may select up to six gene targets for which the Company will perform discovery, optimization and preclinical development activities to identify RNAi compounds against each selected target (the "C3" programs). Upon target acceptance, Sarepta will receive an exclusive license to the Company's intellectual property rights to exploit compounds directed to those targets and is wholly responsible for clinical development and commercialization of each compound after the Company delivers a Clinical Trial Application ready data package (the "CTA package").

The Company identified 17 performance obligations under the Sarepta Collaboration Agreement. The four C1 licenses are distinct performance obligations from the four C1 research and development performance obligations since the customer can use and benefit from the licenses separately. The performance obligations for the licenses were satisfied in the second quarter of fiscal 2025 upon delivery and the research and development performance obligations will be satisfied as the work is performed. The remaining nine performance obligations include three C2 preclinical stage program licenses and research and development activities, and six C3 unidentified discovery target licenses and research and development activity. Each of the three C2 programs and the six C3 programs were determined to represent one performance obligation, as the customer cannot benefit from the use of the product license at the point of transfer until the specified research and development activities are performed. As such, each of the C2 and C3 product licenses and respective research and development work will be combined to form one performance obligation. For these nine performance obligations, revenue is recognized over time as the work is performed.

For performance obligations recognized over time, the estimated performance period over which revenue will be recognized is determined to be the period over which the Company estimates it will perform the research and development activities. The Company determined that the most appropriate method of measuring progress for these performance obligations is an input method based on research and development costs in the program budget. Accordingly, the Company has estimated the total cost required to complete its obligation and recognized an amount of revenue equal to the proportion of services performed, which is reassessed on an ongoing basis as the program progresses. In the period an agreement expires or is terminated, remaining deferred revenue, if any, is recognized as revenue.

Under the terms of the Sarepta Collaboration Agreement, the Company received an upfront payment of $500.0 million on February 14, 2025. In addition, on February 7, 2025, the Company received $325.0 million in the form of an equity investment under the Stock Purchase Agreement. Based upon the Company's share price on February 7, 2025, (the "Closing Date"), the difference between the $325.0 million and the fair value of the shares on the Closing date resulted in a premium of $83.6 million. The premium is included as part of the total consideration of the Sarepta Collaboration Agreement for revenue recognition purposes. The Company is entitled to receive $250.0 million to be paid in annual installments of $50.0 million over the first five years of the agreement. The Company is also eligible receive reimbursement of certain costs related to carrying out the research and development activities for the C1 programs. The fixed consideration of $833.6 million and an estimated variable consideration of $71.2 million for a total of $904.9 million were allocated to all performance obligations based on their relative standalone selling price. Standalone selling prices for the product licenses were determined using an adjusted market-based approach through the net present value of the expected future cash flows for each program. The standalone selling prices for the research and development work were determined based on an expected cost plus margin approach.

The Company estimates the stand-alone selling price for each distinct performance obligation, which involves assumptions that may require significant judgment. The Company's estimates of the stand-alone selling price for license-related performance obligations includes forecasted revenues and expenses, phase dates, probability of success, development timelines, and the discount rate. The estimates of the stand-alone selling price for research and development performance obligations generally include forecasting the expected costs of satisfying a performance obligation at market rates. The Company identified a discount based on the difference between the aggregate stand-alone selling price and the transaction price for accounting revenue recognition purposes. The Company allocated the discount proportionally to each of the performance obligations based upon their standalone selling price.

The Company receives reimbursement of certain costs related to carrying out the research and development activities for the C1 programs and may receive development milestone payments of up to $300.0 million. Further, for each of the 13 programs, the Company is eligible to receive regulatory milestone payments between $110.0 million and $180.0 million per program. Variable consideration associated with the milestones that may be achieved will be allocated to the performance obligation to which it is determined to be related, which will be the respective development work that is being reimbursed and the respective programs to which the milestones relate. ARO-DM1 development milestones were allocated between the license and development work based on the allocation of the standalone selling price. The Company will recognize the ARO-DM1 development milestones and other development milestones as revenue in the periods the underlying milestone events are achieved as achievement of the milestone events are highly susceptible to factors outside of the entity's influence and therefore there is a possibility that the milestone event will not be achieved.

The Company is also eligible to receive sales milestone payments between $500.0 million and $700.0 million per program as well as tiered royalties on net sales of licensed products of up to the low double digits, subject to the terms and conditions of the Sarepta Collaboration Agreement. The Company has applied the sales-based scope exception to the sales milestones and the royalty-based payments.

The Sarepta Collaboration Agreement commenced in February 2025 and may be terminated by either party in the event of a material breach as defined therein. In addition, Sarepta may voluntarily terminate the Sarepta Collaboration Agreement with 30 days' written notice to the Company if terminated prior to any regulatory approval of a licensed product. Unless earlier terminated, the Sarepta Collaboration Agreement expires on a product-by-product and country-by-country basis, upon the date of expiration of the relevant royalty term for such product in such country.

In August 2025, the Company repurchased 2,660,989 shares of its common stock from Sarepta in connection with the $100.0 million DM1 first development milestone under the Sarepta Collaboration Agreement. The repurchase satisfied $50.0 million of the milestone payment through delivery of the Company's common stock, with the remaining $50.0 million settled in cash. The shares were recorded as treasury stock at their fair value of $53.2 million, resulting in a $3.2 million gain on settlement. The repurchased shares are presented as a reduction to total stockholders' equity in accordance with ASC 505-30.

As of September 30, 2025, the Company recorded $696.8 million in revenue from Sarepta and $6.8 million in accounts receivable. The recognition of the remaining revenue for the performance obligations is dependent upon the time it takes to complete the respective research and development activities and in consideration of the timing of the selection of the C3 programs.

Visirna Therapeutics Inc. ("Visirna") and Genzyme Corporation ("Sanofi")

On August 1, 2025, Visirna Therapeutics HK Limited ("Visirna HK"), a wholly owned subsidiary of Visirna Therapeutics, Inc, a majority owned subsidiary of the Company, entered into an Asset Purchase Agreement (the "Asset Purchase Agreement") with Genzyme Corporation ("Sanofi"), a wholly owned subsidiary of Sanofi S.A., pursuant to which Visirna HK sold all of its assets and rights in investigational plozasiran to Sanofi, which included an assignment of Visirna HK's rights (as successor by assignment from Visirna) to develop and commercialize investigational plozasiran in Greater China pursuant to that certain License Agreement by and between the Company and Visirna dated, April 25, 2022 (the "Visirna License Agreement").

In connection with the Asset Purchase Agreement, the Company consented to the partial assignment of the Visirna License Agreement by Visirna HK to Sanofi (as so assigned, the "Sanofi License Agreement"), amongst other agreements between the Company and Visirna, effective as of the closing of the Asset Purchase Agreement. This agreement was not deemed a legal sale of intellectual property from the consolidated perspective of the Company. After giving effect to the Asset Purchase Agreement, Visirna HK retains rights to develop and commercialize in Greater China three other cardiometabolic drugs licensed to it pursuant to the Visirna License Agreement.

Upon closing of the Asset Purchase Agreement, Visirna received an upfront payment of $130.0 million from Sanofi and is eligible to receive further development milestone payments of up to $265.0 million upon approval of plozasiran across various indications in mainland China. The Company is also eligible to receive royalties from Sanofi on net commercial product sales in Greater China under the Sanofi License Agreement. During the year ended September 30, 2025, the Company recorded $130.0 million in revenue.

Visirna identified the licenses as defined in the agreement as the performance obligations under the Asset Purchase Agreement. The performance obligations for the licenses was satisfied in the fourth quarter of fiscal 2025 upon delivery. The fixed consideration of $130.0 million was allocated to the performance obligations. The Company will recognize the development milestones as revenue in the periods the underlying milestone events are achieved as achievement of the milestone events are highly susceptible to factors outside of the entity's influence and therefore there is a possibility that the milestone events will not be achieved. The Company has also applied the sales-based scope exception to the royalty-based payments.

The Sanofi License Agreement may be terminated by either party in the event of a material breach as defined therein. Unless earlier terminated, the Sanofi License Agreement expires on a product-by-product basis, upon the date of expiration of the relevant royalty term for such product in Greater China.

NOTE 3. BALANCE SHEET ACCOUNTS

Property, Plant and Equipment

The following table summarizes the Company's major classes of property, plant and equipment:

	September 30,	
	2025	2024
	(in thousands)	
Land	$ 2,996	$ 2,996
Buildings	251,317	75,988
Research equipment	62,758	65,353
Manufacturing equipment	18,588	—
Furniture	5,594	5,594
Computers and software	1,064	981
Leasehold improvements	104,425	104,410
Construction in progress	15,942	188,731
	462,684	444,053
Less: Accumulated depreciation and amortization	(80,169)	(58,021)
Property, plant and equipment, net	$ 382,515	$ 386,032

Depreciation and amortization expense for property, plant and equipment for the years ended September 30, 2025, 2024, and 2023 was $22.2 million, $16.9 million and $10.7 million, respectively.

During the first quarter of fiscal 2025, the Company substantially completed the build out of its manufacturing facility in Verona, Wisconsin, leading to the reclassification of $162.7 million from construction in progress to buildings and $2.6 million from construction in progress to manufacturing equipment. The Company subsequently incurred and capitalized $10.6 million to buildings and $16.0 million to manufacturing equipment during the remainder of fiscal 2025. Furthermore, the Company began depreciating the newly completed manufacturing facility over a 39-year period and the manufacturing equipment over 7- or 10-year periods.

During the first quarter of fiscal 2024, the Company completed the build out of its laboratory and office facilities in Verona, Wisconsin, which resulted in the reclassification of $71.8 million from construction in progress to buildings. The Company subsequently incurred and capitalized $4.2 million from construction in progress to buildings in fiscal 2024.

Accrued Expenses

Accrued expenses consisted of the following:

	September 30,	
	2025	2024
	(in thousands)	
Accrued research and development expenses	$ 30,330	$ 28,069
Accrued research and development expenses; co-development	31,296	23,351
Accrued capital expenditures	277	4,206
Accrued income taxes (benefits)	20,799	—
Other	7,717	7,391
Total accrued expenses	$ 90,419	$ 63,017

As of September 30, 2025, the Company's accrued research and development expenses was primarily attributable to ongoing clinical trial operations, preclinical animal studies, and associated toxicology assessments. In addition, accrued research and development expenses; co-development relates to the co-development and co-commercialization activities under the Takeda License Agreement (see Note 2).

NOTE 4. INVESTMENTS

The Company's investments consisted of the following:

	As of September 30, 2025 (in thousands)			
	Adjusted Basis	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Available-for-sale securities	$ 689,882	$ 2,956	$ (20)	$ 692,818
Total current investments	$ 689,882	$ 2,956	$ (20)	$ 692,818

	As of September 30, 2024 (in thousands)			
	Adjusted Basis	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Available-for-sale securities	$ 577,465	$ 837	$ (26)	$ 578,276
Total current investments	$ 577,465	$ 837	$ (26)	$ 578,276

The following table summarizes the contract maturity of the available-for-sale securities as of:

	September 30, 2025	September 30, 2024
	(in thousands)	
Within one year	$ 224,328	$ 578,276
After one to two years	468,490	—
After two to three years	—	—
Total	$ 692,818	$ 578,276

As of September 30, 2025, the gross unrealized losses were immaterial. The Company has determined that the available-for-sale securities that were in an unrealized loss position did not have any credit loss impairment as of September 30, 2025 and 2024.

NOTE 5. INTANGIBLE ASSETS

Intangible assets subject to amortization include patents and a license agreement capitalized as part of the Novartis RNAi asset acquisition in March 2015. The following table presents the components of intangible assets:

	Gross Carrying Amount	Accumulated Amortization	Impairment	Net Carrying Amount	Useful Lives
		(in thousands)			(in years)
As of September 30, 2025					
Patents	$ 21,728	$ 16,426	$ —	$ 5,302	14
License	3,129	1,570	—	1,559	21
Total intangible assets, net	$ 24,857	$ 17,996	$ —	$ 6,861	
As of September 30, 2024					
Patents	$ 21,728	$ 14,873	$ —	$ 6,855	14
License	3,129	1,422	—	1,707	21
Total intangible assets, net	$ 24,857	$ 16,295	$ —	$ 8,562	

Intangible assets are reviewed annually for impairment and more frequently if potential impairment indicators exist. No impairment indicators were identified during fiscal 2025 and 2024.

Intangible assets with definite useful lives are amortized on a straight-line basis over their useful lives. Intangible assets amortization expense in each of fiscal 2025, 2024, and 2023 was $1.7 million. None of the intangible assets with definite useful lives are anticipated to have a residual value.

The following table presents the estimated future amortization expense related to intangible assets as of September 30, 2025:

Year Ending September 30,	Amortization Expense (in thousands)
2026	$ 1,700
2027	1,700
2028	1,700
2029	795
2030	149
Thereafter	817
Total	$ 6,861

NOTE 6. STOCKHOLDERS' EQUITY

The following table summarizes the Company's shares of common stock and preferred stock:

	Par Value	Shares		
		Authorized	Issued	Outstanding
			(in thousands)	
As of September 30, 2025				
Common stock [1]	$ 0.001	290,000	135,702	135,702
Preferred stock	$ 0.001	5,000	—	—
As of September 30, 2024				
Common stock	$ 0.001	290,000	124,376	124,376
Preferred stock	$ 0.001	5,000	—	—

(1) Does not include shares of common stock into which the Avoro Pre-Funded Warrants may be exercised.

As of September 30, 2025 and 2024, respectively, 9,851,400 and 11,492,293 shares of common stock were reserved for issuance upon exercise of options and vesting of restricted stock units granted or available for grant under the Company's 2013 and 2021 Incentive Plans, as well as for other inducement grants made to new employees under Rule 5635(c)(4) of the Nasdaq Listing Rules.

On November 25, 2024, the Company entered into a Securities Purchase Agreement (the "Securities Purchase Agreement") with an institutional and accredited investor for a private placement of pre-funded warrants to purchase shares of common stock with an exercise price of $0.001 per share ("Avoro Pre-Funded Warrants"). Pursuant to the Securities Purchase Agreement, the Company sold pre-funded warrants to purchase up to 917,441 shares of common stock at a purchase price of $27.25 per pre-funded warrant, for an aggregate value of approximately $25.0 million. The outstanding Avoro Pre-Funded Warrants are exercisable at any time and do not have an expiration date.

The Company determined that the Avoro Pre-Funded Warrants are freestanding financial instruments because they (i) are immediately exercisable, (ii) do not embody an obligation for the Company to repurchase its shares, (iii) permit the holders to receive a fixed number of shares of common stock upon exercise, and (iv) are indexed to the Company's common stock. As such, the Company evaluated the Avoro Pre-Funded Warrants to determine whether they represent instruments that require liability classification pursuant to the guidance in ASC 480. However, the Company concluded that the Avoro Pre-Funded Warrants are not a liability within the scope of ASC 480 due to their characteristics. Further, the Company determined that the Avoro Pre-Funded Warrants do not meet the definition of a derivative under ASC 815 because they do not meet the criteria regarding no or little initial net investment. Accordingly, the Company assessed the Avoro Pre-Funded Warrants relative to the guidance in ASC 815-40, Contracts in Entity's Own Equity, to determine the appropriate treatment. The Company concluded that the Avoro Pre-funded Warrants are both indexed to its own stock and meet all other conditions for equity classification. Accordingly, the Company has classified the Avoro Pre-funded Warrants as permanent equity. As of September 30, 2025, no shares underlying the Avoro Pre-Funded Warrants had been exercised.

In connection with the Sarepta Collaboration Agreement, on November 25, 2024, the Company entered into the Stock Purchase Agreement with an affiliate of Sarepta for a private placement of shares of common stock of the Company (the "Private Placement"). Pursuant to the Stock Purchase Agreement, the Company sold 11,926,301 shares of common stock, at a price per share of $27.25, for an aggregate value of approximately $325.0 million. The Private Placement closed on February 7, 2025. On August 13, 2025, the Company subsequently entered into an agreement with Sarepta to repurchase 2,660,989 common stock of the Company from Sarepta at a price per share of $18.79 for an aggregate value of approximately $50.0 million and approximately $50.0 million in cash to satisfy the milestone payment of $100.0 million due from Sarepta. The shares were recorded as treasury stock at their fair value of $53.2 million, resulting in a $3.2 million gain on settlement. As of the end of fiscal 2025, Sarepta no longer holds an equity position in the Company.

On December 2, 2022, the Company entered into an open market sale agreement (the "Open Market Sale Agreement"), pursuant to which the Company may, from time to time, sell up to $250,000,000 in shares of the Company's common stock through Jefferies LLC, acting as the sales agent and/or principal, in an at-the-market offering ("ATM Offering"). The Company is not required to sell shares under the Open Market Sale Agreement. The Company will pay Jefferies LLC a commission of up to 3.0% of the aggregate gross proceeds received from all sales of the common stock under the Open Market Sale Agreement. Unless otherwise terminated, the ATM Offering shall terminate upon the earlier of (i) the sale of all shares of common stock subject to the Open Market Sale Agreement and (ii) the termination of the Open Market Sale Agreement as permitted therein. The Company and Jefferies may each terminate the Open Market Sale Agreement at any time upon prior notice. As of September 30, 2025, no shares have been issued under the Open Market Sale Agreement.

NOTE 7. COMMITMENTS AND CONTINGENCIES

Litigation

From time to time, the Company may be subject to various claims and legal proceedings in the ordinary course of business. If the potential loss from any claim, asserted or unasserted, or legal proceeding is considered probable and the amount is reasonably estimable, the Company will accrue a liability for the estimated loss. There were no contingent liabilities recorded as of September 30, 2025 and 2024.

On September 10, 2025, the Company filed a Complaint for Declaratory Judgment in the United States District Court for the District of Delaware against Ionis Pharmaceuticals, Inc. ("Ionis") to declare that the United States Patent No. 9,593,333 ("the '333 patent") is invalid and not infringed by the Company's planned commercialization of investigational plozasiran. On September 11, 2025, Ionis filed a Complaint for Patent Infringement against the Company in the United States District Court for the Central District of California alleging patent infringement of the '333 patent by the Company's planned commercialization of investigational plozasiran and seeking damages. There were no contingent liabilities recorded related to this litigation.

Commitments

The Company owns land in the Verona Technology Park in Verona, Wisconsin, where it has constructed an approximately 160,000 square foot drug manufacturing facility and an approximately 140,000 square foot laboratory and office facility to support the Company's manufacturing, process development, and analytical activities.

As of September 30, 2025, the build-out of these facilities was substantially completed, with total costs incurred of $296.4 million. These costs included $173.4 million capitalized to building related to the drug manufacturing facility, $78.0 million capitalized to building related to the laboratory and office facility, $18.6 million capitalized to manufacturing equipment, $15.9 million in construction in progress and $7.9 million capitalized to research equipment and $2.6 million capitalized to furniture.

NOTE 8. LEASES

Pasadena, California: The Company leases 49,000 square feet of office space located at 177 East Colorado Blvd. for its corporate headquarters from 177 Colorado Owner, LLC, which lease expires on April 30, 2027. The lease contains an option to renew for one additional five-year term. The Company is not reasonably certain that it will exercise this option to renew and therefore it is not included in right-of-use assets and liabilities as of September 30, 2025.

San Diego, California: The Company leases 144,000 square feet of office and research and development laboratory space located at 10102 Hoyt Park from 11404 & 11408 Sorrento Valley Owner, LLC, which lease expires on April 30, 2038. Pursuant to the lease, within twelve months of the expiration of the initial 15-year term, the Company has the option to extend the lease for up to one additional ten-year term, with certain annual increases in base rent. The Company is not reasonably certain that it will exercise this option to renew and therefore it is not included in right-of-use assets and liabilities as of September 30, 2025.

The lease agreement, as amended, granted the Company the right to receive an Additional Tenant Improvement Allowance ("ATIA") funded by the lessor. The Company received $30.8 million in ATIA, including a final payment of $3.1 million during the first quarter of fiscal 2024. As a result, the Company remeasured its lease liability and right-of-use assets to reflect these additional allowances and the related increased lease payments. The Company has further concluded that these ATIAs have no effects on the classification of the lease.

Madison, Wisconsin: The Company leases 110,956 square feet space, which it increased from 107,000 square feet on June 30, 2025, located at 502 South Rosa Road for its office and laboratory facilities, which lease expires on September 30, 2031. The lease contains options to renew for two terms of five years. The Company is not reasonably certain that it will exercise this option and therefore it is not included in right-of-use assets and liabilities as of September 30, 2025.

The components of lease assets and liabilities along with their classification on the Company's consolidated balance sheets were as follows:

Lease Assets and Liabilities	Classification	September 30, 2025	September 30, 2024
		(in thousands)	
Operating lease assets	Right-of-use assets	$ 43,891	$ 45,255
Current operating lease liabilities	Lease liabilities	7,289	6,342
Non-current operating lease liabilities	Lease liabilities, net of current portion	104,112	111,027

Lease Cost	Classification	Year Ended September 30, 2025	2024	2023
			(in thousands)	
Operating lease cost	Research and development	$ 10,483	$ 11,035	$ 10,350
	General and administrative expense	1,952	2,006	1,730
Variable lease cost[(1)]	Research and development	4,076	3,648	1,179
	General and administrative expense	—	—	—
Total		$ 16,511	$ 16,689	$ 13,259

(1) Variable lease cost is primarily related to operating expenses associated with the Company's operating leases.

There was $0, $0 and $1.4 million in short-term lease cost during the years ended September 30, 2025, 2024, and 2023, respectively.

The following table presents maturities of operating lease liabilities on an undiscounted basis as of September 30, 2025:

Year	Amounts
	(in thousands)
2026	$ 15,873
2027	15,050
2028	13,696
2029	13,985
2030	14,282
2031 and thereafter	100,672
Total	$ 173,558
Less imputed interest	(62,157)
Total operating lease liabilities	$ 111,401

Supplemental cash flow and other information related to leases was as follows:

	Year Ended September 30, 2025	2024	2023
		(in thousands)	
Cash received for amounts included in the measurement of lease liabilities:			
Operating cash flows from operating leases	$ —	$ 3,099	$ 48,391
Right-of-use assets adjusted in exchange for new/amended operating lease liabilities	$ —	$ (29)	$ 17,071
Cash paid for amounts included in the measurement of lease liabilities:			
Operating cash flows from operating leases	$ 15,474	$ 11,038	$ 5,204
Weighted-average remaining lease term (in years)	11.70	12.5	13.5
Weighted-average discount rate	8.0 %	8.0 %	8.0 %

NOTE 9. STOCK-BASED COMPENSATION

The Company has three plans that provide for equity-based compensation.

Under the 2013 Incentive Plan (the "2013 Plan"), 2,276,279 awards are granted and outstanding, relating to stock options and restricted stock awards to employees and directors as of September 30, 2025.

Under the 2021 Incentive Plan (the "2021 Plan"), 8,000,000 shares (subject to certain adjustments) of the Company's common stock are authorized for grants of stock options, stock appreciation rights, restricted and unrestricted stock, performance awards, cash awards and other awards convertible into or otherwise based on shares of the Company's common stock. The maximum number of shares authorized under the 2021 Plan will be (i) reduced by any shares subject to awards made under the 2013 Plan after January 1, 2021, and (ii) increased by any shares subject to outstanding awards under the 2013 Plan as of January 1, 2021 that, after January 1, 2021, are canceled, expired, forfeited or otherwise not issued under such awards (other than as a result of being tendered or withheld to pay the exercise price or withholding taxes in connection with any such awards) or settled in cash. As of September 30, 2025, 6,231,559 shares have been granted under the 2021 Plan. The total number of shares available for issuance was 2,378,770 shares, which includes 170,898 and 439,431 shares that were forfeited under the 2013 and 2021 Plans, respectively.

Under the Company's Inducement Plan (the "Inducement Plan"), 832,950 shares of the Company's common stock are authorized for issuance pursuant to grants of stock options, stock appreciation rights, restricted and unrestricted stock, stock units (including restricted stock units), performance awards, cash awards, and other awards convertible into or otherwise based on shares of the Company's common stock. Awards under the Inducement Plan may only be granted to new employees of the Company in accordance with the provisions of Rule 5635(c)(4) of the Nasdaq Listing Rules. As of September 30, 2025, 660,020 shares have been granted under the Inducement Plan. The total number of shares remaining available for issuance was 255,244 shares.

In addition, prior to adoption of the Inducement Plan, the Company previously granted stand-alone inducement awards in the form of stock options and restricted stock units outside of the Company's equity plans to new employees under Rule 5635(c)(4) of the Nasdaq Listing Rules. As of September 30, 2025, there were 598,605 and 53,713 shares underlying outstanding stand-alone inducement options and restricted stock units, respectively.

The following table presents a summary of awards outstanding attributable to Arrowhead Pharmaceuticals, Inc.:

	As of September 30, 2025			
	2013 Plan	2021 Plan	Inducement Awards	Total
Granted and outstanding awards:				
Options	776,279	32,151	598,605	1,407,035
Restricted stock units	1,500,000	3,750,071	560,280	5,810,351
Total	2,276,279	3,782,222	1,158,885	7,217,386

The following table summarizes stock-based compensation expenses included in operating expenses attributable to Arrowhead Pharmaceuticals, Inc.:

	Year Ended September 30,		
	2025	2024	2023
		(in thousands)	
Research and development	$ 27,320	$ 29,527	$ 34,332
General and administrative	27,528	37,570	43,798
Total	$ 54,848	$ 67,097	$ 78,130

Stock Option Awards

The following table presents a summary of the stock option activity for the year ended September 30, 2025:

	Shares	Weighted-Average Exercise Price Per Share	Weighted-Average Remaining Contractual Term (Years)	Aggregate Intrinsic Value
Outstanding at September 30, 2024	1,978,516	$ 23.39		
Granted	—	—		
Cancelled or expired	(36,341)	45.44		
Exercised	(535,140)	7.41		
Outstanding at September 30, 2025	1,407,035	$ 28.90	3.4	$ 19,099,141
Exercisable at September 30, 2025	1,407,035	$ 28.90	3.4	$ 19,099,141

The aggregate intrinsic values represent the amount by which the market price of the underlying stock exceeds the exercise price of the option. The total intrinsic value of the options exercised during the years ended September 30, 2025, 2024, and 2023 was $6.4 million, $4.2 million and $12.2 million, respectively.

Stock-based compensation expense related to stock options outstanding for the years ended September 30, 2025, 2024, and 2023 was $0.1 million, $2.8 million and $8.4 million, respectively.

As of September 30, 2025, the pre-tax compensation expense for all outstanding unvested stock options is considered nominal.

The fair value of each stock option award is estimated on the date of grant using the Black-Scholes option pricing model. The Black-Scholes option pricing valuation model was developed for use in estimating the fair value of traded options, which do not have vesting restrictions and are fully transferable. The determination of the fair value of each stock option is affected by the Company's stock price on the date of grant, as well as assumptions regarding a number of highly complex and subjective variables. No options were granted during the years ended September 30, 2025 and 2024.

The following table provides the assumptions used in the calculation of grant-date fair values of these stock options based on the Black-Scholes option pricing model:

	Year Ended September 30,		
	2025[5]	2024[5]	2023
Expected dividend yield[1]	—	—	—
Risk-free interest rate[2]	N/A	N/A	3.69% – 4.57%
Expected volatility[3]	N/A	N/A	86.4 %
Expected term (in years)[4]	N/A	N/A	6.25
Weighted-average grant date fair value per share	N/A	N/A	25.61

(1) The dividend yield is zero as the Company currently does not pay a dividend.
(2) The risk-free interest rate is based on that of the U.S. Treasury yields with equivalent terms in effect at the time of the grant.
(3) Volatility is estimated based on volatility average of the Company's common stock price.
(4) The computation of expected term was determined based on safe harbor rules, considering the contractual terms of the awards and vesting schedules.
(5) No options were granted during the year ended September 30, 2025 and September 30, 2024.

Visirna ESOP: As of September 30, 2025, Visirna, a subsidiary of the Company, granted 14,612,000 stock options to its employees from the Employee Stock Option Plan (the "Visirna ESOP"), which authorizes 20,000,000 shares for issuance. The Visirna ESOP is independently managed by Visirna, including the valuation process. For the years ended September 30, 2025 and 2024, stock-based compensation expense related to the Visirna ESOP was $8.5 million and $6.9 million, respectively.

Restricted Stock Units

Restricted Stock Units ("RSUs"), including market-based, time-based and performance-based awards, have been granted under the Company's 2013 and 2021 Plans, the Inducement Plan and as inducements awards granted outside of the Company's equity-based compensation plans. At vesting, each outstanding RSU will be exchanged for one share of the

Company's common stock. RSU awards generally vest subject to the satisfaction of service requirements or the satisfaction of both service requirements and achievement of certain performance targets.

The following table summarizes the activity of the Company's RSUs:

	Number of RSUs	Weighted-Average Grant Date Fair Value
Outstanding as of September 30, 2024	4,913,312	$ 49.61
Granted	2,779,689	19.38
Vested	(1,525,693)	47.15
Forfeited	(356,957)	30.19
Outstanding as of September 30, 2025	5,810,351	$ 36.97

The fair value of RSUs was determined based on the closing price of the Company's common stock on the grant date, with consideration given to the probability of achieving service and/or performance conditions for awards.

For the years ended September 30, 2025, 2024 and 2023, the Company recorded stock-based compensation expense of $54.9 million, $64.3 million and $69.7 million, respectively, related to shares of RSUs. As of September 30, 2025, there was $73.3 million of total unrecognized compensation cost related to RSUs that is expected to be recognized over a weighted-average period of 1.7 years.

NOTE 10. FAIR VALUE MEASUREMENTS

The Company employs a fair value hierarchy that prioritizes the inputs to valuation techniques used to measure fair value. The fair value of a financial instrument is the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date using the exit price. Accordingly, when market observable data are not readily available, the Company's own assumptions are used to reflect those that market participants would be presumed to use in pricing the asset or liability at the measurement date.

Assets and liabilities recorded at fair value on the consolidated balance sheets are categorized based on the level of judgment associated with inputs used to measure their fair values and the level of market price observability, as follows:

Level 1 Unadjusted quoted prices are available in active markets for identical assets or liabilities as of the reporting date.

Level 2 Pricing inputs are other than quoted prices in active markets, which are based on the following:

- Quoted prices for similar assets or liabilities in active markets;
- Quoted prices for identical or similar assets or liabilities in non-active markets; or
- Either directly or indirectly observable inputs as of the reporting date.

Level 3 Pricing inputs are unobservable and significant to the overall fair value measurement, and the determination of fair value requires significant management judgment or estimation.

In certain cases, inputs used to measure fair value may fall into different levels of the fair value hierarchy. In such cases, the level in the fair value hierarchy within which the fair value measurement in its entirety falls has been determined based on the lowest level input that is significant to the fair value measurement in its entirety. Thus, a Level 3 fair value measurement may include inputs that are observable (Level 1 or Level 2) and unobservable (Level 3). The Company's assessment of the significance of a particular input to the fair value measurement in its entirety requires judgment and consideration of factors specific to the asset or liability.

The Company uses prices and inputs that are current as of the measurement date, including during periods of market disruption. In periods of market disruption, the ability to observe prices and inputs may be reduced for many instruments. This condition could cause an instrument to be reclassified from Level 1 to Level 2, or from Level 2 to Level 3. The Company recognizes transfers between levels at either the actual date of the event or a change in circumstances that caused the transfer. At September 30, 2025 and 2024, the Company did not have any financial assets or financial liabilities based on Level 3 measurements.

The following tables present information about the Company's assets and liabilities measured at fair value on a recurring basis, and indicate the fair value hierarchy of the valuation techniques utilized by the Company:

	September 30, 2025			
	Level 1	Level 2	Level 3	Total
	(in thousands)			
Available-for-sale securities				
U.S. government and agency securities	$ —	$ 150,695	$ —	$ 150,695
Certificate of deposits	—	12,019	—	12,019
Municipal securities	—	7,046	—	7,046
Commercial notes	—	13,801	—	13,801
Corporate debt securities	—	509,257	—	509,257
Total available-for-sale securities	—	692,818	—	692,818
Cash equivalents				
Money market instruments	64,460	—	—	64,460
Term deposit	—	134,357	—	134,357
Certificate of deposits	—	3,001	—	3,001
Corporate debt securities	—	16,182	—	16,182
Total cash equivalents	64,460	153,540	—	218,000
Total financial assets	$ 64,460	$ 846,358	$ —	$ 910,818

	September 30, 2024			
	Level 1	Level 2	Level 3	Total
	(in thousands)			
Available-for-sale securities				
U.S. government and agency securities	$ —	$ 160,723	$ —	$ 160,723
Commercial notes	—	179,714	—	179,714
Corporate debt securities	—	237,839	—	237,839
Total available-for-sale securities	—	578,276	—	578,276
Cash equivalents				
Money market instruments	66,966	—	—	66,966
Total cash equivalents	66,966	—	—	66,966
Total financial assets	$ 66,966	$ 578,276	$ —	$ 645,242

NOTE 11. INCOME TAXES

Income Tax Provision (Benefit)

The components of the income (loss) before income tax expense and noncontrolling interest are as follows:

	Year Ended September 30,		
	2025	2024	2023
		(in thousands)	
Domestic	$ (42,573)	$ (582,333)	$ (194,639)
Foreign	94,106	(30,127)	(7,852)
Total	$ 51,533	$ (612,460)	$ (202,491)

Income tax provision (benefit) consisted of the following components:

	Year Ended September 30,		
	2025	2024	2023
	(in thousands)		
Current:			
Federal	$ 21,440	$ 148	$ 1,074
State	(56)	375	1,710
Foreign	35	(3,290)	—
Total current tax	$ 21,419	$ (2,767)	$ 2,784
Deferred:			
Federal	$ —	$ —	$ —
State	—	—	—
Foreign	—	—	—
Total deferred tax	$ —	$ —	$ —
Income tax provision	$ 21,419	$ (2,767)	$ 2,784

The following table presents a reconciliation of the tax expense based on the statutory rate to the Company's actual tax expense in the consolidated statements of operations and comprehensive income (loss). A notional 21% tax rate was applied as follows:

	September 30,		
	2025	2024	2023
U.S. federal statutory income tax	21.0 %	21.0 %	21.0 %
State income taxes, net of federal tax benefit	(3.1)%	2.6 %	0.4 %
Tax credits	(44.4)%	3.0 %	6.8 %
Permanent and other items	(37.9)%	2.5 %	(4.6)%
Non-deductible compensation	5.1 %	(0.9)%	(4.6)%
Foreign-derived intangible income deduction	(16.2)%	— %	1.2 %
Other income	11.1 %	— %	— %
Stock compensation	10.7 %	(0.7)%	(1.1)%
Valuation allowance	95.4 %	(27.0)%	(20.5)%
Effective income tax rate	41.7 %	0.5 %	(1.4)%

Deferred Income Taxes

The following table presents the significant components of the Company's net deferred tax assets and liabilities:

	September 30,	
	2025	2024
	(in thousands)	
Deferred tax assets:		
Net operating loss carryforwards	$ 70,858	$ 102,716
Capitalized research and development	232,360	156,015
Tax credits	48,867	85,428
Deferred revenue	171,262	81,556
Lease liabilities	23,919	27,999
Stock compensation	9,184	10,989
Accrued compensation	4,254	4,078
Intangible assets	1,034	1,384
Other	948	2,843
Total gross deferred tax assets	$ 562,686	$ 473,008
Valuation allowance	$ (497,543)	$ (448,867)
Deferred tax liabilities:		
Fixed assets	$ (30,503)	$ (13,155)
Right-of-use assets	(9,424)	(10,792)
Unrealized gains	(630)	(194)
Original Issue Discount	(24,586)	—
Total gross deferred tax liability	$ (65,143)	$ (24,141)
Net deferred tax assets (liabilities)	$ —	$ —

A valuation allowance is recorded to reduce deferred tax assets to the amount that is more likely than not to be realized based on an assessment of positive and negative evidence, including estimates of future taxable income necessary to realize future deductible amounts. A significant piece of objective negative evidence evaluated was the cumulative loss incurred over the three-year period ended September 30, 2025. Such objective evidence limits the ability to consider other subjective evidence such as its projections for future growth. On the basis of this evaluation at September 30, 2025 and 2024, a valuation allowance of $497.5 million and $448.9 million, respectively, has been recorded.

As of September 30, 2025, the Company had accumulated federal, state, and foreign net operating loss ("NOL") carryforwards of $20.6 million, $815.5 million and $46.3 million, respectively. Of the $20.6 million in federal NOL carryforwards, $20.6 million was generated before January 1, 2018, and is subject to a 20-year carryforward period ("pre-Tax Act losses"), with expiration beginning in 2031. Of the $815.5 million in state NOL carryforwards, $2.7 million can be carried forward indefinitely, while the remaining balance begins to expire in 2031. The Company also has foreign NOL carryforwards totaling $46.3 million, which begin to expire in 2027. Additionally, the Company has federal and state income tax credits of $49.4 million and $26.3 million, respectively. The federal credits begin to expire in 2041. Of the state income tax credits, $13.6 million begins to expire in 2035, while the remaining credits can be carried forward indefinitely.

Pursuant to Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (the "Code"), the annual use of an entity's NOL and research and development credit carryforwards may be limited if there is a cumulative ownership change of greater than 50% within a three-year period. The annual limitation is determined based on the entity's value immediately prior to the ownership change. Future ownership changes could further affect the limitation. If a limitation is applied, the related tax asset would be removed from the deferred tax asset schedule, with a corresponding reduction in the valuation allowance. To date, the Company has completed an analysis pursuant to Sections 382 and 383 through September 30, 2024. Ownership Changes may have occurred since then, and future changes could potentially limit the Company's ability to utilize these attributes.

Uncertainty in Income Taxes

The Company has adopted guidance issued by the FASB that clarifies the accounting for uncertainty in income taxes recognized in an enterprise's financial statements and prescribes a recognition threshold of more-likely-than not and a measurement process for financial statement recognition and measurement of a tax position taken or expected to be taken in a tax return. In making this assessment, a company must determine whether it is more-likely-than not that a tax position

will be sustained upon examination, based solely on the technical merits of the position and must assume that the tax position will be examined by taxing authorities.

The following table summarizes the Company's gross unrecognized tax benefits:

	Year Ended September 30,		
	2025	2024	2023
		(in thousands)	
Beginning balance of unrecognized tax benefits	$ 16,613	$ 14,536	$ 3,481
Gross increase for prior period tax positions	1,160	654	9,495
Gross decrease for prior period tax positions	—	—	(1,489)
Gross increase for current period tax positions	4,379	3,415	3,049
Lapse of statue of limitations	—	(1,992)	—
Ending balance of unrecognized tax benefits	$ 22,152	$ 16,613	$ 14,536

The Company has recorded income tax (benefit) expense of $0 and $3.3 million for the years ended September 30, 2025 and 2024, respectively, related to uncertain tax positions inclusive of interest and penalties. The Company's policy is to recognize potential interest and penalties related to unrecognized tax benefits associated with uncertain tax positions, if any, in the income tax provision. As of September 30, 2025, the Company has not accrued any interest or penalties.

If the unrecognized tax benefit as of September 30, 2025 is ultimately recognized, there would be no reduction in the Company's income tax expense or effective tax rate, excluding the impact of U.S. Tax benefits netted against deferred taxes that are subject to a valuation allowance. The Company does not anticipate any changes in its unrecognized tax benefits over the next 12 months.

The Company is subject to taxation in the U.S. and various states along with other foreign countries. Due to the presence of NOL carryforwards, all of the income tax years remain open for examination. The Company is currently under audit by the IRS for September 30, 2023. California income tax examination has been closed. There are no other audits in any other jurisdictions.

The Company analyzes undistributed earnings of each foreign subsidiary and has determined that no withholding taxes are applicable to earnings which are currently available for distribution. No additional deferred tax liability has been recorded as the parent entity would not be required to include the distribution into income under the current law.

The Tax Cuts and Jobs Act subjects a U.S. shareholder to tax on Global Intangible Low-Taxed Income ("GILTI") earned by certain foreign subsidiaries. The FASB Staff Q&A, Topic 740 No. 5. Accounting for GILTI, states that an entity can make an accounting policy election to either recognize deferred taxes for temporary basis differences expected to reverse as GILTI in future years or to provide for the tax expense related to GILTI in the year that the tax is incurred as a period expense only. The Company has elected to account for GILTI in the year the tax is incurred.

On July 4, 2025, the One Big Beautiful Bill Act ("OBBBA") was enacted in the U.S. The OBBBA includes significant provisions, such as the permanent extension of certain expiring provisions of the Tax Cuts and Jobs Act, modifications to the international tax framework and the restoration of favorable tax treatment for certain business provisions. The legislation has multiple effective dates, with certain provisions effective in 2025 and others implemented through 2027. ASC 740, "Income Taxes", requires the effects of changes in tax rates and laws to be recognized in the period in which the legislation is enacted. The Company has implemented OBBBA in the fourth quarter of the current year.

NOTE 12. EMPLOYEE BENEFIT PLANS

The Company sponsors a defined contribution retirement plan which is under Section 401(k) of the Internal Revenue Code and is designed to adhere to ERISA Fiduciary standards. All of the Company's full-time employees are eligible to participate this plan. Under the terms of the plan, an eligible employee may elect to contribute a portion of their salary on a pre-tax basis, subject to federal statutory limitations. The plan allows for a discretionary match in an amount up to 100% of each participant's first 3% of compensation contributed plus 50% of each participant's next 2% of compensation contributed.

For the years ended September 30, 2025, 2024, and 2023, the Company recorded expenses for the matching contributions under this plan of $3.9 million, $3.4 million and $2.2 million, respectively.

The Company also provides certain employee benefit plans, including those which provide health and life insurance benefits to employees.

NOTE 13. LIABILITY RELATED TO THE SALE OF FUTURE ROYALTIES

In November 2022, the Company and Royalty Pharma entered into the Royalty Pharma Agreement, pursuant to which Royalty Pharma agreed to pay up to $410.0 million in cash to the Company in consideration for the Company's future royalty interest in olpasiran, a siRNA originally developed by the Company and licensed to Amgen in September 2016 under the Olpasiran Agreement.

Pursuant to the Royalty Pharma Agreement, Royalty Pharma paid $250.0 million upfront and agreed to pay up to an additional $160.0 million in aggregate one-time milestone payments due if and when the following milestone events occur: (i) $50.0 million on completion of enrollment in the OCEAN Phase 3 clinical trial for olpasiran, (ii) $50.0 million upon receipt of FDA approval of olpasiran for an approved indication (reduction in the risk of myocardial infarction, urgent coronary revascularization, or coronary heart disease death in adults with established cardiovascular disease and elevated Lp(a)), and (iii) $60.0 million upon Royalty Pharma's receipt of at least $70.0 million of royalty payments under the Royalty Pharma Agreement in any single calendar year. During the third quarter of fiscal 2024, Amgen completed enrollment of the Phase 3 OCEAN(a) outcomes trial of olpasiran, which triggered a $50.0 million milestone payment that the Company received in the same quarter.

In consideration for the payment of the foregoing amounts under the Royalty Pharma Agreement, Royalty Pharma is entitled to receive all royalties otherwise payable by Amgen to the Company under the Olpasiran Agreement. The Company remains eligible to receive any milestone payments potentially payable by Amgen under the Olpasiran Agreement.

The Company has evaluated the terms of the Royalty Pharma Agreement and concluded in accordance with the relevant accounting guidance that the Company accounted for the transaction as debt and the funding of $250.0 million and $50.0 million from Royalty Pharma were recorded as liabilities related to the sale of future royalties on its consolidated balance sheets. The Company is not obligated to repay these funds received under the Royalty Pharma Agreement.

The Company records the obligations at their carrying value using the effective interest method. In order to amortize the sale of future royalties, the Company utilizes the prospective method to estimate the future royalties to be paid by the Company to the counterparty over the life of the arrangement. Under the prospective method, a new effective interest rate is determined based on the revised estimate of remaining cash flows. The new rate is the discount rate that equates the present value of the revised estimate of remaining cash flows with the carrying amount of the debt, and it will be used to recognize non-cash interest expense for the remaining periods. The Company periodically assesses the amount and the timing of expected royalty payments using a combination of internal projections and forecasts from external sources. The estimates of future net product sales (and resulting royalty payments) are based on key assumptions including population, penetration, probability of success and sales price, among others. To the extent such payments are greater or less than the Company's initial estimates or the timing of such payments is different than its original estimates, the Company will prospectively adjust the amortization of the royalty financing obligations and the effective interest rate. As of September 30, 2025, the estimated effective interest rate was 8.3%.

The following table presents the activity with respect to the liability related to the sale of future royalties.

	September 30,	
	2025	2024
	(in thousands)	
Beginning carrying value	$ 341,361	$ 268,326
Upfront payment received	—	—
Milestone payment received	—	50,000
Non-cash interest expense recognized	26,036	23,035
Ending carrying value	$ 367,397	$ 341,361

NOTE 14. FINANCING AGREEMENT

On August 7, 2024 (the "Closing Date"), the Company entered into a Financing Agreement with the guarantors party thereto, the lenders party thereto (the "Lenders"), and Sixth Street Lending Partners ("Sixth Street"), as the administrative agent and collateral agent for the Lenders (the "Financing Agreement"). The Financing Agreement establishes a senior secured term loan facility of $500.0 million (the "Credit Facility"), consisting of $400.0 million funded on the Closing Date and an additional $100.0 million available at the Company's option, subject to mutual agreement with Sixth Street. The loans under the Credit Facility bear interest at an annual rate of 15.0%, which is paid in kind and added to the outstanding principal balance of the Credit Facility each period. The outstanding principal balance of this Credit

Facility, including amounts representing accrued but unpaid interest previously paid in kind, is due and payable on August 7, 2031.

The Company is permitted to use the net proceeds for working capital, capital expenditures and general corporate purposes of the Company and its subsidiaries.

The Company will have the right to prepay loans under the Credit Facility at any time. The Company is required to partially repay loans under the Credit Facility with proceeds from certain asset sales, condemnation events and extraordinary receipts, subject, in some cases, to reinvestment rights. If the Company repays in full the aggregate principal outstanding under the Credit Facility and such payment in full occurs on or prior to August 7, 2028, the Company will be required to make an additional payment to the lenders under the Credit Facility on such date in an amount necessary for the lenders to achieve a two times multiple of invested capital ("MOIC") of the aggregate principal amount funded on the Closing Date (the "MOIC Payment"). If such payment in full occurs after August 7, 2028, the Company will be required to make a payment to the lenders under the Credit Facility on such date in an amount necessary for the lenders to achieve the greater of the MOIC Payment and the present value of all interest payments that would have been payable from such date through the maturity date of the Credit Facility discounted at the Treasury Rate (as defined in the Financing Agreement) plus 0.5%; provided that such payment amount in this instance will not exceed the amount necessary for the lenders to achieve a 2.5 times MOIC.

On November 26, 2024, the Company entered into an amendment to the Financing Agreement (the "Amendment") to modify, amongst other things, some of the prepayment terms of the loans under the Credit Facility, including, the prepayment terms related to the Sarepta Collaboration Agreement. The Amendment was effective on February 14, 2025, following the closing of the Sarepta Collaboration Agreement and receipt of the $500.0 million upfront payment from Sarepta. The Amendment added an additional prepayment clause that requires certain contractual prepayments of principle and MOIC payments throughout the life of the loans under the Credit Facility. Additionally, any prepayment will be split with 50% of any such prepayment paying down the principle balance of the loans under the Credit Facility and the other 50% being applied to prepay the MOIC Payment. In the event the prepayment amounts result in fees being prepaid in excess of the actual amounts required to be paid, the excess fees shall be reallocated and applied to reduce the amount of the principal balance upon repayment in full of the loans under the Credit Facility. As of September 30, 2025, the Company has paid $100.0 million in MOIC payments of which $25.3 million is expected to be applied to principal upon repayment in full. To date, the Company has paid $201.6 million of the loans under the Credit Facility during fiscal 2025.

The Amendment was accounted for as a debt modification under ASC 470-50, "Debt—Modification and extinguishments" since the Amendment did not result in substantially different terms. In connection with the Amendment, the Company did not incur significant third-party fees.

All obligations under the Financing Agreement are secured on a first-priority basis by security interests in substantially all assets of the Company and material subsidiaries of the Company, including its intellectual property, subject to certain exceptions, and is guaranteed by material subsidiaries of the Company, including foreign subsidiaries, subject to certain exceptions.

The Financing Agreement contains customary covenants, including, without limitation, a financial covenant to maintain liquidity (cash, cash equivalents and investments) of at least $100.0 million if the Company's market capitalization is above $1.5 billion, and negative covenants that, subject to certain exceptions, restrict indebtedness, liens, investments (including acquisitions), fundamental changes, asset sales and licensing transactions, dividends, modifications to material agreements, payment of subordinated indebtedness, distributions from certain parties, and other matters customarily restricted in such agreements. The Company is subject to restrictions on sales and licensing transactions with respect to certain core intellectual property, subject to certain exceptions, including certain transactions related to areas outside the United States, United Kingdom, European Union, Japan and China.

The Financing Agreement contains certain embedded features that were identified and evaluated as not material to the consolidated financial statements.

On August 13, 2025, the Company entered into second amendment to the Financing Agreement (the "Second Amendment") that permitted the share repurchase of the Company's common stock from Sarepta and required the Company to pay a nominal administrative fee.

The outstanding balance of the Credit Facility consisted of the following:

	September 30,	
	2025	2024
	(in thousands)	
Initial Term Loan	$ 400,000	$ 400,000
Accumulated interest on the Initial Term Loan	66,942	9,000
Accumulated accretion of the MOIC Payment	3,478	—
Less: Unamortized debt issuance costs	(13,912)	(15,817)
Less: Current portion of credit facility	(40,000)	—
Less: Payments	(201,625)	—
Credit facility, net of current portion	$ 214,883	$ 393,183

The following table sets forth total interest expense recognized related to the Credit Facility:

	Year Ended September 30,		
	2025	2024	2023
		(in thousands)	
Amortization of debt discount and issuance costs	$ 1,906	$ 317	$ —
Accretion of the MOIC Payment	3,478	—	—
Contractual interest expense	57,941	9,000	—
Total interest expense	$ 63,325	$ 9,317	$ —

The amounts shown in the table below, related to the Credit Facility, represent the expected repayments of principle and accrued interest balance as of September 30, 2025 as well as any mandatory prepayments that the Company is obligated to make to the Lenders during the indicated periods. The principal balance will increase from accrued paid in kind interest and the table does not include MOIC payments beyond those contractually determined. Actual payments on current principal may vary from the amounts presented in the table.

Year	Amounts
	(in thousands)
2026	$ 40,000
2027	40,000
2028	15,000
2029	15,000
2030	15,000
Thereafter	214,990
Total	$ 339,990

In May 2025, Visirna entered into the Revolving Credit Agreement with Bank of Zhejiang. The maximum aggregate credit facility is 72.9 million Chinese Yuan ($10.3 million) bearing an annual interest rate of 4.1%. The term of each loan is twelve months. The amount outstanding as of September 30, 2025 was 72.9 million Chinese Yuan ($10.3 million) on the credit facility which was classified as other current liabilities.

NOTE 15. NET LOSS PER SHARE

The following table presents the computation of basic and diluted net loss per share for the years ended September 30, 2025, 2024 and 2023.

	Year Ended September 30,		
	2025	2024	2023
	(in thousands, except per share amounts)		
Numerator:			
Net loss attributable to Arrowhead Pharmaceuticals, Inc.	$ (1,631)	$ (599,493)	$ (205,275)
Denominator:			
Weighted-average basic shares outstanding [(1)]	133,758	119,784	106,750
Effect of dilutive securities	—	—	—
Weighted-average diluted shares outstanding [(1)]	133,758	119,784	106,750
Basic net loss per share	$ (0.01)	$ (5.00)	$ (1.92)
Diluted net loss per share	$ (0.01)	$ (5.00)	$ (1.92)

(1) Includes shares of common stock into which the Avoro Pre-Funded Warrants may be exercised. See Note 6.

The following table sets forth the potentially dilutive securities that have been excluded from the calculation of diluted net loss per share because to include them would be anti-dilutive.

	Year Ended September 30,		
	2025	2024	2023
	(in thousands)		
Options	744	707	633
Restricted stock units	4,276	4,030	3,420
Total	5,020	4,737	4,053

NOTE 16. SEGMENT INFORMATION

We operate in a single segment dedicated to the discovery, development, manufacturing and commercialization of RNAi therapeutics. The Company's RNAi therapeutics are comprised of siRNAs that function upstream of conventional medicines by potently silencing messenger RNA ("mRNA") that encode for proteins implicated in the cause or pathway of disease, thus preventing them from being made. Consistent with our operational structure, our Chief Executive Officer ("CEO"), as the CODM, manages and allocates resources on a consolidated basis at the global corporate level. Our global research and development and technical operations and quality organizations are responsible for the discovery, development, and supply of products. Commercial efforts that coordinate the marketing, sales and distribution of these products are organized by geographic region and therapeutic area. All of these activities are supported by corporate staff functions. Managing and allocating resources at the corporate level enables our CEO to assess the overall level of resources available and how to best deploy these resources in line with our overarching long-term, corporate-wide strategic goals. The determination of a single segment is consistent with the consolidated financial information regularly reviewed by the CODM for the purposes of evaluating performance, forecasting future period financial results, allocating resources and setting incentive targets.

Consistent with our management reporting, results of our operations are reported on a consolidated basis for purposes of segment reporting. The CEO evaluates performance and decides how to allocate resources based on consolidated net loss that is reported on the consolidated statements of operations and comprehensive income (loss). The measure of segment assets is reported on the consolidated balance sheets as total assets. The CEO uses consolidated net loss to evaluate income generated from the Company's business activities in deciding how to allocate company resources (such as pursuing clinical development or entering a strategic collaboration), monitoring budget versus actual results, and establishing management's compensation. Please refer to the consolidated financial statements for further information related to these measures of segment performance. In addition, research and development and selling, general and administrative expenses are significant segment expenses regularly provided to the CEO with the following categories:

Research and Development

	Year Ended September 30,		
	2025	2024	2023
		(in thousands)	
Candidate costs	$ 347,571	$ 259,280	$ 162,459
R&D discovery costs	66,788	74,150	55,586
Salaries	109,085	96,418	73,668
Facilities related	29,233	25,782	16,267
Total research and development expense, excluding non-cash expense	$ 552,677	$ 455,630	$ 307,980
Stock compensation	32,582	33,586	34,332
Depreciation and amortization	21,900	16,654	10,876
Total research and development expense	$ 607,159	$ 505,870	$ 353,188

General & Administrative

	Year Ended September 30,		
	2025	2024	2023
		(in thousands)	
Salaries	$ 31,916	$ 27,589	$ 22,999
Professional, outside services, and other	53,589	24,733	20,720
Facilities related	5,625	4,116	3,415
Total general and administrative expense, excluding non-cash expense	$ 91,130	$ 56,438	$ 47,134
Stock compensation	30,785	40,382	43,798
Depreciation/amortization	2,028	1,941	1,617
Total general and administrative expense	$ 123,943	$ 98,761	$ 92,549

NOTE 17. SUBSEQUENT EVENTS

Novartis

On August 29, 2025, the Company entered into an Exclusive License and Collaboration Agreement (the "Novartis Collaboration Agreement") with Novartis Pharma AG ("Novartis") for the co-development and commercialization of multiple preclinical programs in rare, genetic diseases. Under the Novartis Collaboration Agreement, Novartis has received an exclusive worldwide license to the Company's ARO-SNCA preclinical stage program. The Novartis Collaboration Agreement closed on October 17, 2025 subsequent to clearance under the Hart-Scott-Rodino Antitrust Improvement Act. Under the terms of the Novartis Collaboration Agreement, the Company received $200.0 million as an upfront payment. The Company is also eligible to receive $30.0 million associated with certain target nominations. Further, for each of the 4 programs, the Company is eligible to receive development milestone payments between $175.0 million and $245.0 million per program and sales milestone payments between $285.0 million and $370.0 million per program. The Company is also eligible to receive tiered royalties on net sales of licensed products of up to the low double digits.

Sarepta DM1 Milestone

On November 20, 2025, the Company earned a $200.0 million milestone payment from Sarepta. The milestone was earned when Arrowhead achieved the second development milestone event in a Phase 1/2 clinical study of ARO-DM1, also called SRP-1003, an investigational RNAi therapeutic for the treatment of type 1 myotonic dystrophy (DM1), the most common adult-onset muscular dystrophy. The second milestone event included the achievement of a patient enrollment target, drug safety committee review and subsequent authorization to dose escalate and proceed, and completion of day 105 study visit by at least one patient in the clinical trial.

REDEMPLO Commercial Launch

The FDA approved the Company's New Drug Application (NDA) for REDEMPLO (plozasiran) injection for Familial Chylomicronemia Syndrome (FCS), on November 18, 2025. This approval, which was based on the results of the Phase 3 PALISADE clinical trial, was completed within the Prescription Drug User Fee Act (PDUFA) VI timeframe. This approval is a significant milestone for the Company, and the commercial launch of REDEMPLO is in progress. We expect to begin generating revenue from sales of REDEMPLO in the upcoming fiscal year.

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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

SCHEDULE 14A

Proxy Statement Pursuant to Section 14(a) of the Securities Exchange Act of 1934

(Amendment No.)

Filed by the Registrant ☒ Filed by a Party other than the Registrant ☐

Check the appropriate box:

☐ Preliminary Proxy Statement
☐ Confidential, For Use of the Commission Only (as permitted by Rule 14a-6(e)(2))
☒ Definitive Proxy Statement
☐ Definitive Additional Materials
☐ Soliciting Material Under §240.14a-12

ARROWHEAD PHARMACEUTICALS, INC.

(Name of Registrant as Specified in Its Charter)

(Name of Person(s) Filing Proxy Statement if other than the Registrant)

Payment of filing fee (Check all boxes that apply):

☒ No fee required.

☐ Fee paid previously with preliminary materials.

☐ Fee computed on table in exhibit required by Item 25(b) per Exchange Act Rules 14a-6(i)(1) and 0-11.

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Notice of Annual Meeting of Stockholders

To Be Held on Thursday, March 19, 2026

Your vote is important, whether or not you expect to attend the Annual Meeting of Stockholders. Stockholders of record are urged to vote via the Internet or telephone as instructed, or if you are voting by mail, to mark, sign and date and promptly return the proxy in the postage-prepaid return envelope provided.

Voting promptly will help avoid the additional expense of further solicitation to assure a quorum at the meeting.

Important Notice Regarding the Availability of Proxy Materials for the Stockholder Meeting to be Held on Thursday, March 19, 2026:

You may access the following proxy materials at www.proxyvote.com before the meeting and at www.virtualshareholdermeeting.com/ARWR2026 during the meeting.

- Notice of the 2026 Annual Meeting of Stockholders;
- Company's 2026 Proxy Statement; and
- Company's Annual Report on Form 10-K for the year ended September 30, 2025

TO THE STOCKHOLDERS OF ARROWHEAD PHARMACEUTICALS, INC.:

NOTICE IS HEREBY GIVEN that the 2026 Annual Meeting of Stockholders of Arrowhead Pharmaceuticals, Inc., a Delaware corporation (the "Company" or "Arrowhead"), will be held on Thursday, March 19, 2026, at 10:00 a.m., Pacific Time (the "Annual Meeting"). This year's meeting will be a completely "virtual" meeting of stockholders. If you are a stockholder of record, you can attend the Annual Meeting online, vote your shares electronically, and submit your questions during the Annual Meeting by visiting www.virtualshareholdermeeting.com/ARWR2026. Prior to the Annual Meeting, you will be able to vote at www.proxyvote.com or, if you requested printed copies of the proxy materials, by telephone or mail. The Annual Meeting will be held for the purpose of considering and voting upon the following proposals, as more fully described in the accompanying Proxy Statement:

1. To elect the seven directors named in the Proxy Statement to serve as members of the Company's Board of Directors until the next Annual Meeting or until their successors are duly elected and qualified;
2. To conduct an advisory (non-binding) vote to approve executive compensation;
3. To approve the Arrowhead Pharmaceuticals, Inc. Amended and Restated 2021 Incentive Plan;
4. To ratify the selection of KPMG LLP as independent auditors of the Company for the fiscal year ending September 30, 2026; and
5. To transact any other matters that may properly come before the Annual Meeting or any adjournments or postponements thereof.

The foregoing items of business are more fully described in the Proxy Statement accompanying this Notice. The Board of Directors has fixed January 22, 2026 as the record date for the meeting. Only stockholders of record at the close of business on that date will be entitled to notice of, and to vote at, the Annual Meeting and any adjournment or postponement thereof.

All stockholders of record are cordially invited to attend the Annual Meeting by visiting www.virtualshareholdermeeting.com/ARWR2026. Instructions for accessing the virtual Annual Meeting are provided in the Proxy Statement. In the event of a technical malfunction or other situation that the meeting chair determines may affect the ability of the Annual Meeting to satisfy the requirements for a meeting of stockholders to be held by means of remote communication under the Delaware General Corporation Law, or that otherwise makes it advisable to adjourn the Annual Meeting, the chair or secretary of the Annual Meeting will convene the meeting at 11:00 a.m., Pacific Time on the date specified above and at the Company's address specified below solely for the purpose of adjourning the meeting to reconvene at a date, time and physical or virtual location announced by the meeting chair or secretary. Under either of the foregoing circumstances, we will post information regarding the announcement on the Investors page of the Company's website at ir.arrowheadpharma.com.

If you prefer to receive paper copies of our proxy materials, please follow the instructions included in the Notice of Internet Availability. To ensure your representation at the meeting, you are urged to vote via the Internet, or if you requested a printed copy of the proxy materials, by telephone or mail by marking, signing, dating and returning the proxy card or voting instruction form provided with the printed proxy materials as promptly as possible in the postage-prepaid envelope enclosed for that purpose. Any stockholder of record attending the Annual Meeting may vote at the Annual Meeting even if such stockholder has previously returned a proxy.

/s/ Patrick O'Brien
Patrick O'Brien
Secretary

Pasadena, California
January 26, 2026

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ARROWHEAD PHARMACEUTICALS, INC.
177 E. Colorado Blvd., Suite 700
Pasadena, California 91105
(626) 304-3400

PROXY STATEMENT FOR ANNUAL MEETING OF STOCKHOLDERS

To be held on Thursday, March 19, 2026

General Information Concerning Solicitation and Voting

The enclosed Proxy is solicited on behalf of the Board of Directors (the "**Board**") of Arrowhead Pharmaceuticals, Inc. (the "**Company**" or "**Arrowhead**") for use at the 2026 Annual Meeting of Stockholders (the "**Annual Meeting**") to be held on Thursday, March 19, 2026 at 10:00 a.m., Pacific Time, and at any adjournment(s) or postponement(s) thereof, for the purposes set forth herein and in the accompanying Notice of Annual Meeting of Stockholders (the "**Notice**"). The Company anticipates that the Notice Regarding the Availability of Proxy Materials (the "**Notice of Internet Availability**") in connection with these proxy solicitation materials will first be made available on or about January 26, 2026 to all stockholders entitled to vote at the Annual Meeting and we will post our proxy materials on the website referenced in the Notice of Internet Availability. As more fully described in the Notice of Internet Availability, stockholders may choose to access our proxy materials on the website referred to in the Notice of Internet Availability or may request to receive a printed set of our proxy materials. You will not receive a printed copy of the proxy materials unless you request one.

This year's meeting will be a completely "virtual" meeting of stockholders. If you were a stockholder as of the close of business on the Record Date (as defined below), you can attend the Annual Meeting online, vote your shares electronically, and submit your questions during the Annual Meeting, by visiting www.virtualshareholdermeeting.com/ARWR2026. You will need to have your 16-digit Control Number included on your Notice of Internet Availability or your proxy card (if you received a printed copy of the proxy materials) to join the Annual Meeting. If your shares are held in street name and your voting instruction form or Notice of Internet Availability indicates that you may vote those shares through www.proxyvote.com, then you may access, participate in, and vote at the Annual Meeting with the 16-digit access code indicated on that voting instruction form or Notice. Otherwise, stockholders who hold their shares in street name should contact their bank, broker or other nominee (preferably at least five days before the Annual Meeting) and obtain a "legal proxy" in order to be able to attend, participate in or vote at the Annual Meeting.

The meeting webcast will begin promptly at 10:00 a.m. Pacific Time. Online check-in will begin approximately 15 minutes before then and we encourage you to allow ample time for check-in procedures. If you experience technical difficulties during the check-in process or during the meeting, please call the number listed on the meeting website for technical support.

We will endeavor to answer as many stockholder-submitted questions as time permits that comply with the Annual Meeting rules of conduct. We reserve the right to edit profanity or other inappropriate language and to exclude questions regarding topics that are not pertinent to meeting matters or Company business. If we receive substantially similar questions, we may group such questions together and provide a single response to avoid repetition. Additional information regarding the rules and procedures for participating in the Annual Meeting will be set forth in our meeting rules of conduct, which stockholders can view during the meeting at the meeting website.

By hosting the Annual Meeting virtually, we believe we can expand access, improve communication and lower costs, while reducing the environmental impact of the meeting.

Record Date

Only holders of record of our common stock at the close of business on January 22, 2026 (the "**Record Date**") are entitled to notice of the Annual Meeting and to vote at the Annual Meeting. On that date, the Company had outstanding 140,010,690 shares of common stock ("**Common Stock**").

Revocability of Proxies

Any proxy given by a stockholder of record pursuant to this solicitation may be revoked by the person giving it at any time before its use by delivering to the Secretary of the Company, at or before the taking of the vote at the Annual Meeting, a written notice of revocation or a duly executed proxy bearing a later date or by attending the Annual Meeting and voting electronically. Stockholders may also revoke their proxy by entering a new vote over the Internet or by telephone. Stockholders who hold their shares in street name should contact their bank, broker or other nominee for instructions on how to change their vote.

Voting and Solicitation

Each share of the Company's Common Stock is entitled to one vote on all matters presented at the Annual Meeting. Stockholders do not have the right to cumulate their votes in the election of directors. Shares of Common Stock represented by properly executed proxies will, unless such proxies have been previously revoked, be voted in accordance with the instructions indicated thereon. In the absence of specific instructions to the contrary, properly executed proxies will be voted FOR each of the nominees in Proposal 1 and FOR Proposals 2, 3, and 4 at the Annual Meeting. No business other than that set forth in the accompanying Notice of Annual Meeting of Stockholders is expected to come before the Annual Meeting. Should any other matter requiring a vote of stockholders properly arise, the persons named in the enclosed form of proxy will vote in accordance with their best judgment.

If you cannot attend the Annual Meeting to vote, you may vote your shares prior to the meeting via the Internet, or if you requested printed copies of the proxy materials, by telephone or by mail.

The Company has engaged a proxy solicitor, Okapi Partners, LLC, to encourage voting by our stockholders. The total cost for the solicitation campaign is estimated at about $15,000, plus campaign services expenses. Proxies may also be solicited by certain of the directors, officers and employees of the Company, without additional compensation. The Company will bear the costs of solicitation. In addition, the Company expects to reimburse brokerage firms and other persons representing beneficial owners of shares for their expenses in forwarding solicitation materials to such beneficial owners.

If your shares are held in a street name, the voting instruction form sent to you by your broker, bank or other nominee should indicate whether the institution has a process for beneficial holders to provide voting instructions over the Internet or by telephone. If your bank or brokerage firm gives you this opportunity, the voting instructions from your broker, bank or other nominee that accompany this Proxy Statement will tell you how to use the Internet or telephone to direct the vote of shares held in your account. If your voting instruction form does not include Internet or telephone information, please complete, and return the voting instruction form in the addressed, postage-paid envelope provided by your broker. Stockholders who vote by proxy over the Internet or by telephone need not return a proxy card or voting instruction form by mail.

Quorum; Abstentions; Broker Non-Votes

The required quorum for the transaction of business at the Annual Meeting is the presence, either virtually or represented by proxy, of the holders of shares representing a majority of the votes entitled to be cast by holders of shares of Common Stock issued and outstanding on the Record Date and entitled to vote at the Annual Meeting. If there is no quorum, the holders of a majority of shares of common stock virtually present at the Annual Meeting, either personally or by proxy, may adjourn the Annual Meeting to another time or date. Shares that are voted "FOR," "AGAINST" or "ABSTAIN" on any proposal are treated as being present at the meeting for purposes of establishing a quorum for the conduct of business at the meeting. If you are the beneficial owner and do not direct your broker, fiduciary, or custodian how to vote your shares, your broker, fiduciary, or custodian will only be able to vote your shares with respect to proposals considered to be "routine." Your broker, fiduciary, or custodian is not entitled to vote your shares with respect to "non-routine" proposals (resulting in "**broker non-votes**" for the matters on which the broker, fiduciary, or custodian does not vote). Whether a proposal is considered routine or non-routine is subject to

stock exchange rules. Even with respect to routine matters, some brokers are choosing not to exercise discretionary voting authority. As a result, we urge you to direct your broker, fiduciary, or custodian how to vote your shares on all proposals to ensure that your vote is counted. Shares subject to a broker non-vote will be counted as present for the purpose of determining the presence or absence of a quorum for the transaction of business at the Annual Meeting; the effect of abstentions and broker non-votes on the proposals presented herein is discussed below.

With regard to the election of directors, votes may be cast "FOR," "AGAINST" or "ABSTAIN" for each director nominee. Because directors are elected by a majority of votes cast in an uncontested election, abstentions and broker non-votes, if any, will have no effect on the outcome of the election of directors. If a quorum is present at the meeting, the nominees receiving more "FOR" votes than "AGAINST" votes will be elected. Because Proposal Nos. 2, 3 and 4 must be approved by the affirmative vote of a majority of the shares of Common Stock entitled to vote thereon and present in person or by proxy at the Annual Meeting (the "**Required Vote**"), abstentions will have the same effect as a vote "AGAINST" the proposal, whereas broker non-votes, if any, will have no effect on its outcome.

Deadline for Receipt of Stockholder Proposals and Nominations

To be considered for inclusion in the Proxy Statement and proxy card for the Company's 2027 Annual Meeting of Stockholders, proposals submitted by stockholders pursuant to Rule 14a-8 of the Securities Exchange Act of 1934, as amended (the "**Exchange Act**"), and stockholders' director nominations pursuant to the proxy access provisions of the Company's Amended and Restated Bylaws ("**Bylaws**"), must be submitted in writing to our Corporate Secretary at the address set forth on the first page of this Proxy Statement. Such proposals and nominations must be received by us not later than our close of business (6:00 p.m. Pacific Time) on September 28, 2026, and, in the case of a proxy access nomination, no earlier than August 29, 2026, and must satisfy the requirements of Rule 14a-8 and our Bylaws, as applicable. The submission of a stockholder proposal or proxy access nomination does not guarantee that it will be included in our proxy materials.

Additionally, our Bylaws provide for advance notice procedures to nominate a person for director (other than pursuant to our Bylaws' proxy access provisions) or to propose business to be considered by stockholders at a meeting (other than pursuant to Rule 14a-8). To be considered timely under these provisions, the stockholder's notice must provide the information set forth in the Bylaws (which includes information required under Rule 14a-19 of the Exchange Act) and be received by the Corporate Secretary at our principal executive offices at the address set forth on the first page of this Proxy Statement between November 19, 2026 and our close of business (6:00 p.m. Pacific Time) on December 19, 2026; *provided, however*, that if the 2027 Annual Meeting date is advanced by more than 30 days before or delayed by more than 60 days after the anniversary date of the 2026 Annual Meeting, then stockholders must provide notice within time periods specified in our Bylaws. Our Bylaws also specify requirements as to the form and content of a stockholder's notice. If a stockholder fails to meet these deadlines or fails to satisfy the requirements of Rule 14a-4 under the Exchange Act, we may exercise discretionary voting authority under proxies we solicit to vote on any such proposal as we determine appropriate.

We reserve the right to reject, rule out of order, or take other appropriate action regarding any nomination or proposal that does not comply with these and other applicable requirements.

Proposal One — Election of Directors

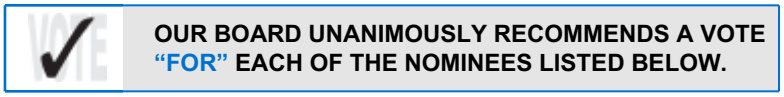
The Board has nominated the following seven persons as directors to serve until the 2027 Annual Meeting or until their successors have been duly elected and qualified. Each of the nominees is currently a director of Arrowhead and was elected most recently by stockholders at the 2025 Annual Meeting. None of the nominees has a familial relationship with any other nominee or any executive officer of the Company.

Douglas Ingram, a current director, is not standing for re-election and will retire from the Board at the annual meeting, at which point the size of the Board will be reduced to seven directors. Mr. Ingram's departure is not the result of any disagreement with the Company, the Board or the management team. The Board thanks Mr. Ingram for his contributions to the Company.

The nominees receiving more "FOR" votes than "AGAINST" votes at the Annual Meeting will be elected. Unless otherwise instructed, the proxy holders will vote the proxies received by them "FOR" the nominees named below. Under Delaware law, a director not receiving a majority of votes cast in an uncontested election could continue to serve as a "holdover director" until the director resigns or is replaced. Under the Company's director resignation policy, each director is expected to submit an advance, contingent irrevocable resignation that the Board may accept if stockholders to not re-elect that director. In that event, the Board will then decide whether to accept or reject the resignation, or whether other action is required.

The table below sets forth, with respect to each nominee for election, the nominee's age, current position with Arrowhead and biographical and other information, including the primary skills and experiences considered by our Nomination Committee in determining to recommend them as nominees. The director nominees have indicated that they are willing and able to serve as directors. However, if any of the director nominees becomes unable or, for good cause, unwilling to serve, proxies may be voted for the election of such other person as shall be designated by our Board, or the Board may decrease the size of the Board.

Nominees for Election as Directors. The Board unanimously adopted a resolution proposing the nominees set forth below for election as directors of the Company to serve until the 2027 Annual Meeting.



Christopher Anzalone, PhD
Chief Executive Officer, President, Director & Board Chair

Age: 56

Director since: 2007

Experience & Expertise
Dr. Anzalone has been President, Chief Executive Officer and Director of the Company since December 1, 2007 and has led the Company's business and technical development since then. Prior to joining Arrowhead, Dr. Anzalone formed and served as CEO of the Benet Group LLC, a private equity firm focused on creating and building new nano-biotechnology companies from university-generated science. Before his tenure at the Benet Group, from 1999 to 2003, he was a partner at the Washington, DC-based private equity firm Galway Partners, LLC, where he was responsible for sourcing, structuring and building new business ventures. Dr. Anzalone holds a Ph.D in Biology from UCLA and a B.A. in Government from Lawrence University.

Qualifications
Dr. Anzalone's qualifications to serve on the Board include his deep understanding of the business through his role as Chief Executive Officer; in addition, Dr. Anzalone has extensive experience in business development, biotechnology, drug development, company-building and venture capital.

Mauro Ferrari, PhD
Independent Director

Age: 66

Director since: 2010

Serves on:
- Science Committee (Co-Chair)
- Audit Committee
- Nomination Committee

Experience & Expertise

Dr. Ferrari has served as Affiliate Professor of Pharmaceutics at the University of Washington in Seattle, WA since 2019. He has also served as CEO and Chairman of the Board of BrYet US, Inc., a biotech company, since 2019. From 2010 to 2019, Dr. Ferrari served at the Houston Methodist Hospital in multiple roles, including: President and CEO of The Houston Methodist Hospital Research Institute (TMHRI), Executive Vice President of Houston Methodist Hospital, and Senior Associate Dean of Weill Cornell Medical College in New York. He also previously served as President of the European Research Council. Dr. Ferrari was formerly a Professor and chair of the Department of NanoMedicine and Biomedical Engineering at the University of Texas Health Science Center at Houston, Professor of Experimental Therapeutics at the MD Anderson Cancer Center, Adjunct Professor of Bioengineering at Rice University, and Adjunct Professor of Biomedical Engineering at the University of Texas in Austin. His previous academic appointments include tenured professorships at his graduate Alma Mater U.C. Berkeley and The Ohio State University. From 2003 to 2005, Dr. Ferrari served as Special Expert on Nanotechnology and Eminent Scholar at The National Cancer Institute. He previously served on the board of the University of St. Thomas in Houston. He has also served as CEO of DXT Therapeutics.

Dr. Ferrari is an internationally recognized expert in innovative therapeutics, nanomedicine and biomedical engineering. For his work as a biomedical and pharmaceutical researcher, Dr Ferrari has received numerous recognitions such as induction into the National Academy of Inventors, the American Society of Medical and Biological Engineers, the American Association for the Advancement of Science, the European Academy of Sciences, and awards including the Founders Award of the Controlled Release Society, Blaise Pascal Medal of the European Academy of Sciences, the James Shannon Director's Award of the NIH, and the Coulter Award for Innovation and Entrepreneurship. He holds degrees from the University of Padova Italy (Dottore in Matematica), and the University of California, Berkeley (MS and PhD in Mechanical Engineering). He has studied Medicina at The Ohio State University (no degree), and completed executive education programs at Harvard Business School and the Wharton School of the University of Pennsylvania. He has received honorary degrees from the University of Naples "Federico II", (Biotechnology), the University of Palermo (Electrical Engineering) and the University of St Thomas in Houston (Letters).

Qualifications:

Dr. Ferrari's qualifications to serve on the Board include his extensive training and experience in the fields of innovative therapeutics, nanotechnology, biotechnology and their biomedical applications. Dr. Ferrari has significant technical training, held several academic appointments, was author of over 500 published articles, was awarded over 30 issued patents and is the recipient of most prestigious academic awards in the biomedical sciences, nanomedicine and drug delivery technology. Additionally, Dr. Ferrari has extensive experience in developmental stage organizations having founded several startup companies. He also has experience supervising CFOs in several of his current and prior positions.

Hongbo Lu
Independent Director

Age: 55

Director since: 2024

Serves on:
- Science Committee
- Nomination Committee
- Compensation Committee

Other Public Company Boards:
- Terns Pharmaceuticals, Inc.
- Zenas Biopharma Inc

Experience & Expertise

Hongbo Lu, Ph.D. has served as a founding member of NEXTBio Capital, a biotech investment firm, since 2024. Dr. Lu has over 20 years of healthcare investment experience in both public securities and private companies, including her tenure as a Managing Partner of Vivo Capital, LLC, an investment firm, from 2020 to 2024, as a Managing Partner at Lilly Asia Ventures (LAV), a venture capital firm, from 2017 to 2020, and as a Managing Director at OrbiMed Advisors, an investment firm, from 2011 to 2016. Over her investment career, Dr. Lu served on the boards of over 20 healthcare companies, including Turning Point Therapeutics, Crown Biosciences, Avedro, BlossomHill, Createrna, Avistone, Echosens, and was extensively involved in the company formations of ADARx, Ribox, Visirna, Ablaze, Elpiscience, and Ronovo. She currently serves on the boards of Terns Pharmaceuticals (Nasdaq: TERN) and Zenas (Nasdaq: ZBIO). Dr. Lu started her Wall Street career as a biotechnology analyst at Piper Jaffray & Co. and prior to that, her scientific career in biotechnology startups in the Bay Area. Dr. Lu earned her Ph.D. in Bioengineering from the University of Washington, M.B.A. from the Haas School of Business at the University of California, Berkeley, and graduated with honors from Tsinghua University.

Qualifications: Dr. Lu's qualifications to serve on the Board include her deep experience in international business and the pharmaceutical industry, her expertise with venture and capital markets, and her executive leadership experience.

Adeoye Olukotun, MD, MPH
Independent Director

Age: 81

Director since: 2020

Serves on:
- Science Committee (Co-Chair)
- Nomination Committee

Other Public Company Boards:
- Tonix Pharmaceuticals Holding Corp.

Experience & Expertise

Dr. Olukotun is a Mayo Clinic trained cardiologist who has served as Chief Executive Officer of CR Strategies, LLC, which consults on clinical trial design and FDA strategy for pharmaceutical development, since 2001. Dr. Olukotun currently serves on the board of directors of Tonix Pharmaceuticals Holding Corp. (Nasdaq: TNXP), a commercial-stage biopharmaceuticals company. He served as CEO of Epigen Pharmaceuticals, Inc., a discovery phase biotechnology company, from 2014 to 2017, and as Vice Board Chair of CardioVax, Inc., a clinical-stage biopharmaceutical company, from 2012 to 2016. He spent the first 20 years of his career in roles of increasing responsibility in clinical development, including multiple product approvals, at Pfizer, Bristol-Myers Squibb, and Mallinckrodt. He has over 35 years of experience in the pharmaceutical industry and has been instrumental in the approval and success of numerous cardiology and metabolic medicines, including the first daily beta blocker and the first approved ACE inhibitor, among others. Dr. Olukotun received his Medical Doctor degree from the Albert Einstein College of Medicine in New York, and a Masters in Public Health from Harvard University School of Public Health.

Qualifications

Dr. Olukotun's qualifications to serve on the Board include his extensive background in biopharmaceutical development, particularly in the cardiometabolic field, his scientific and public health expertise, and his board and executive leadership experience.

Michael S. Perry, DVM, Ph.D.

Independent Director

Age: 66

Director since: 2011

Serves on:

- Compensation Committee (Chair)
- Audit Committee
- Nomination Committee
- Science Committee

Experience and Expertise

Dr. Perry is currently a Venture Partner with Bioscience Managers, a global venture capital firm. Dr. Perry was Chief Executive Officer of Avita Medical, Inc., a regenerative medicine company based in Valencia, CA (Nasdaq: RCEL) from 2017 to 2022. From 2014 to 2017, he served as Chief Scientific Officer of Novartis' Cell and Gene Therapy Unit, and from 2012 to 2014 he served as Vice President and Global Head of Stem Cell Therapy for Novartis Pharmaceuticals Corp., the US affiliate of Switzerland-based Novartis AG, a global pharmaceutical company. Dr. Perry has also served as SVP and Global Head of R&D at Baxter Healthcare, and as President and as CEO of Cell & Gene Therapy at Novartis AG. Earlier in his career he served as VP Regulatory Affairs at Novartis Pharmaceuticals, Sandoz Pharmaceuticals, and Syntex Corporation. He also served as Director of Regulatory Affairs at Schering-Plough Corporation. Dr. Perry also served as a Venture Partner with Bay City Capital, LLC for eight years. Dr. Perry has previously served as a board member for the following companies: Ampliphi Bioscience Corp., Gamida Cell Ltd., Targeted Genetics, Inc., American Xeno, Inc., 7 Hills Pharma, BioTransplant, Inc., Itamar Biomedical Ltd., Systemix, Inc., Genetic Therapy, Inc., Extropy Pharmaceuticals, Inc., and Pharsight Corp. Dr. Perry served as Chair of the Compensation Committee at Ampliphi Bioscience Corp., Genetic Therapy Inc., and at Gamida Cell Ltd. He has also served as a member of the Audit Committee at several biotech companies and as Chairman or member of the Nomination Committee. Dr. Perry additionally served as board Chairman at American Xeno Inc., Extropy Pharmaceuticals Inc., and at 7 Hills Pharma, Inc. Dr. Perry holds an Honors Bachelor of Science in Physics and Engineering and a Ph.D. in Biomedical Pharmacology from the University of Guelph. He also holds a Doctor of Veterinary Medicine & Surgery from the Ontario Veterinary College and is a graduate of the International Advanced Management Program at Harvard Business School. Dr. Perry currently serves as Adjunct Professor at the Gates Center for Regenerative Medicine at the University of Colorado Anschutz Medical Campus and as Faculty at Houston Methodist and Chair of the Translational Medicine Advisory Board of the Houston Methodist Research Institute.

Qualifications

Dr. Perry's qualifications to serve on the board include his medical expertise and his extensive experience in preclinical and clinical drug development, including executive level leadership roles and directorships in several publicly held biotech companies.

Victoria Vakiener
Independent Director

Age: 62

Director since: 2022

Serves on:
- Nomination Committee (Chair)
- Audit Committee

Experience & Expertise
Ms. Vakiener served on the board of directors and chaired the nominating committee at Chimerix, Inc., a clinical-stage biopharmaceutical company, until its acquisition by Jazz Pharmaceuticals in April 2025. From November 2018 through September 2021, she served as Chief Commercial Officer of Epizyme, Inc., a commercial-stage biopharmaceutical company, where she built the commercial organization and launched TAZVERIK for two indications within six months. Prior to joining Epizyme, Ms. Vakiener was an executive at Johnson & Johnson, a pharmaceutical company, for more than 20 years where she held positions of leadership with increasing responsibility across the company's pharmaceutical and diagnostics businesses. Ms. Vakiener began her pharmaceutical career at Schering-Plough, where she spent nine years in both scientific and commercial roles. Ms. Vakiener received a B.S. in Biochemistry from Albright College.

Qualifications
Ms. Vakiener's qualifications to serve on the board include her extensive experience in drug development and commercialization, including executive level leadership roles and directorships in publicly held biotech companies.

William Waddill
Lead Independent Director

Age: 68

Director since: 2018

Serves on:
- Audit Committee (Chair)
- Compensation Committee
- Nomination Committee

Other Public Company Boards:
- Protagonist Therapeutics, Inc.
- Annexon, Inc.

Experience & Expertise
Mr. Waddill began his career over 35 years ago in commercial banking and public accounting and has been in the biotechnology industry for over 30 years. He currently serves on the boards of Protagonist Therapeutics (Nasdaq: PTGX), and Annexon, Inc. (Nasdaq: ANNX), both clinical-stage biopharmaceutical companies, and he previously served on the board of Turnstone Biologics. Mr. Waddill was Senior Vice President and CFO of Calithera Biosciences, from 2014 to 2016 and Senior Vice President and CFO at OncoMed Pharmaceuticals from 2007 to 2014, both of which were then-public biopharmaceutical companies. Prior to that, he served as the Senior Vice President and CFO of Ilypsa, Inc., a biotechnology company that was acquired in 2007 by Amgen, Inc. Before joining Ilypsa, he served as the founder and principal at Square One Finance, a financial consulting business. Mr. Waddill received a B.S. in accounting from the University of Illinois, Chicago, and certification as a public accountant (inactive) after working at PriceWaterhouseCoopers and Deloitte in Boston.

Qualifications
Mr. Waddill's qualifications to serve on the Board include his extensive background in the biopharma industry, his financial and audit expertise, executive leadership roles and experience as a director of other public companies.

Board Composition and Nominating Process

The Nomination Committee of the Board annually considers the size, composition and needs of the Board and, as appropriate, recommends the nominees for directors to the Board for approval. The Nomination Committee considers and evaluates suggestions from many sources regarding possible candidates for directors. Below are general criteria for the evaluation of current and proposed directors:

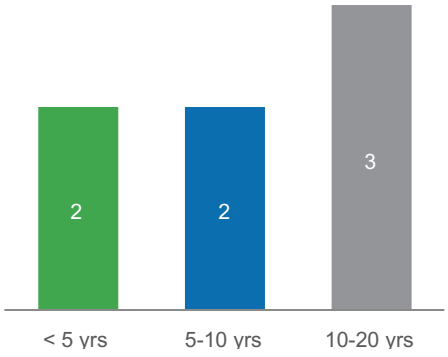
- The highest ethical character and shared values with our Code of Conduct
- Reputation, both personal and professional, consistent with our image and reputation
- Accomplishment within a candidate's field, with superior credentials and recognition
- Relevant expertise and experience and the ability to offer advice and guidance to the Chief Executive Officer based on such expertise and experience
- Independence, without the appearance of any conflict in serving as a director, and independence of any particular constituency with the ability to represent all stockholders
- Ability to exercise sound business judgment

The Nomination Committee believes that the Board should represent a diverse mix of skills, regional and industry experience and backgrounds with a goal of assembling a Board with complementary skills, experiences and backgrounds for the benefit of the Company. In furtherance of this goal, the Committee seeks to include highly qualified candidates with diverse backgrounds in the pool from which the Nominating Committee selects the nominees with the skills, experience and qualifications that it believes best support the Company in the context of the Board as a whole. The Nominating Committee assesses its effectiveness in balancing these considerations as part of its annual assessment of the composition of the Board. In this regard, two of our directors are female and one of our directors is from an underrepresented community.

Listed below are selected key contributions of each current Board member. The table is not intended to be an exhaustive summary of all the contributions of each Board member.

Expertise	Anzalone	Ferrari	Lu	Olukotun	Perry	Vakiener	Waddill
Biopharma Research & Development	X	X	X	X	X	X	X
Healthcare	X	X	X	X	X	X	X
Drug Development	X	X		X	X	X	
Executive Leadership	X	X	X	X	X	X	X
Public Company Governance	X	X	X	X	X	X	X
Accounting/Audit		X	X			X	X
Capital Markets	X		X		X		X
Commercial				X	X	X	

Director Tenure



Corporate Governance, Environmental and Social Commitment

The following is a summary of our corporate governance, environmental, and social commitment policies and practices:

- **Lead Independent Director:** Because our CEO serves as the Board Chair, we have elected a Lead Independent Director to lead discussions and decision-making of the Board when it would not be appropriate for the CEO to do so, including during executive sessions of the independent directors of the Board.

- **Combined Chair and CEO:** Dr. Anzalone currently serves as Board Chair and Chief Executive Officer. Our Bylaws do not require that our Board Chair and Chief Executive Officer positions be separate, and our Board believes that having a combined CEO and Chair is the appropriate leadership structure for us at this time as it helps promote unified leadership and direction and provides management a single, clear focus to execute our strategy and business plans.

- **Majority Independent Board:** A majority of the members of the Board are independent directors, as defined under the listing rules of the Nasdaq Stock Market (the "**Nasdaq Marketplace Rules**"). The Nasdaq Marketplace Rules require that a majority of the Board be comprised of independent directors who, in the opinion of the Board, do not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. The Board undertook a review of its composition and the independence of each director. Based upon information requested from and provided by each director concerning his or her background, employment and affiliations, the Board has determined that all of the Company's directors and director nominees are independent, except Dr. Anzalone, due to his employment relationship with the Company. Douglass Given was also determined to be not independent during the period he served on the Board. In making such determinations, our Board considered the relationships that each such non-employee director has with our Company and all other facts and circumstances our Board deemed relevant in determining independence, including the beneficial ownership of our capital stock by each non-employee director.

- **Board Oversight of Risk:** The Board has overall responsibility for the oversight of the Company's risk management process, which is designed to support the achievement of organizational objectives, including strategic objectives, to improve long-term organizational performance and enhance stockholder value. Risk management includes not only understanding company-specific risks and the steps management implements to manage those risks, but also what level of risk is acceptable and appropriate for the Company. Management is responsible for establishing our business strategy, identifying and assessing the related risks and implementing appropriate risk management practices. The Board regularly reviews our business strategy and management's assessment of the related risk and discusses with management the appropriate level of risk for the Company. The Board believes its programs for overseeing risk would be effective under a variety of leadership frameworks.

Accordingly, the Board's risk oversight function did not significantly impact its selection of the current leadership structure.

- **Code of Conduct:** All of the Company's employees, officers, and directors are subject to the Company's Code of Conduct, which is available on the Company's website at www.arrowheadpharma.com. The code meets the requirements of Nasdaq Marketplace Rules, as well as the code of ethics requirements of the SEC. We intend to disclose future amendments to certain provisions of the Code of Conduct, and waivers of the Code of Conduct granted to officers and directors, on the Company's website within four business days following the date of the amendment or waiver.
- **Independent Committees:** The Audit, Compensation, and Nomination Committees consist entirely of independent directors, and each of the directors currently serving on the Audit Committee and the Compensation Committee satisfy the additional independence criteria applicable to directors on such committees.
- **Regularly Held Executive Sessions:** The independent directors meet separately in executive session on a regular basis to discuss matters relating to the Company and the Board, without members of the management team present.
- **Proxy Access:** Stockholders have a proxy access right with market-standard terms (3% for 3 years, up to 20% of the Board).
- **Board Oversight of Strategy:** The Board reviews at least annually the Company's business initiatives, capital projects and budget matters.
- **Environmental and Social Responsibility Oversight:** The Board has designated one of its members, Adeoye Olukotun, with responsibility for confirming the Company's environmental and social programs align with the Board's expectations in these matters.
- **Cybersecurity:** The Company maintains a cybersecurity program, with direct oversight from senior management and the Audit Committee of the Board of Directors, to manage information, data, and technology security. The Company has formed an internal cross-functional Technology Risk Management Committee comprised of representative leaders from various aspects of the Company's business to broadly implement its cybersecurity program.
- **Related Party Transaction Oversight:** The Audit Committee is responsible for reviewing and approving all disclosable related-party transactions or, if the size and nature of the transaction warrants, a special committee of non-related Board members is formed to negotiate and approve the transaction.
- **Sustainability:** The Company continues to assess its environmental impact and ways in which it can operate more responsibly and sustainably. The Company's research and development facilities in San Diego, California and Verona, Wisconsin were built to meet LEED certification standards. The Company has contracted with a third party to operate a solar power generation plant at our San Diego facility with a generating capacity of approximately 1,406kW, which is comprised of a rooftop system, parking lot solar canopies, and a 1,200 kW battery storage system to supply power to the building. Additionally, the Company has continued its efforts to minimize use of paper records in favor of electronic records and has a robust recycling program for paper, batteries, and electronic equipment.
- **Human Capital Management:** Arrowhead has a vibrant and growing culture and we are committed to the health and welfare of our employees. Our important work developing advanced drugs for patients requires a specialized and dedicated workforce. Arrowhead supports the development of our employees with a competitive compensation and benefits package, internal advancement, and individualized development opportunities. We are committed to training young scientists and businesspeople and offer multiple internships and entry level positions each year.

Stockholder Communications with Directors

Stockholders who wish to communicate with the Board or any individual director can write to: Corporate Secretary, Arrowhead Pharmaceuticals, Inc., 177 E. Colorado Blvd., Suite 700, Pasadena, CA 91105. Your letter should indicate that you are an Arrowhead stockholder. Depending on the subject matter, management will:

- Forward the communication to the director or directors to whom it is addressed;
- Forward the communication to the Board Chair, if addressed to the board of directors; or
- If not addressed to, or otherwise appropriate for, any director or directors, attempt to handle the inquiry directly.

The purpose of this screening is to allow the Board to avoid having to consider irrelevant or inappropriate communications (such as advertisements, solicitations and hostile communications).

Board Meetings and Committees

The Board held a total of six meetings during the fiscal year ended September 30, 2025. The Board has three standing committees: Audit Committee, Compensation Committee, and Nomination Committee.

The functions of the Audit Committee are to select and oversee the independent registered public accounting firm, to review the scope and results of the year-end audit with management and the independent auditors, to review the Company's accounting principles and its system of internal accounting controls, to review and oversee the Company's response to any cybersecurity incidents, to review the Company's annual and quarterly reports before filing with the SEC, and to review any related-party transactions. The Audit Committee met four times during fiscal 2025. The current members of the Audit Committee are William Waddill (Committee Chair), Mauro Ferrari, Mike Perry and Victoria Vakiener. The Board has determined that all members of the Audit Committee are independent directors under the Nasdaq Marketplace Rules, satisfy the additional independence criteria applicable to directors on the Audit Committee under the rules and regulations of the Securities and Exchange Commission ("**SEC**") and the Marketplace Rules and are "financially literate" under the Nasdaq Marketplace Rules. The Board has determined that Mr. Waddill is an "audit committee financial expert" in accordance with the applicable rules and regulations established by the SEC, based on his experience as noted above. The Audit Committee Charter is available on the Company's website at www.arrowheadpharma.com.

The functions of the Compensation Committee are to review and approve the corporate goals and objectives relevant to the Chief Executive Officer's compensation, to review salaries, bonuses, equity awards, and other benefits payable to the Company's executive officers and to administer the Company's equity incentive compensation plans. The Compensation Committee is specifically responsible for determining the compensation of the Chief Executive Officer and the other executive officers. The Compensation Committee reviews compensation recommendations made by the Chief Executive Officer for other senior executives of the Company and the compensation of the Chief Executive Officer at least annually; the Chief Executive Officer is not present during discussions or deliberations regarding his compensation. In fiscal 2025, the Compensation Committee engaged Compensia, Inc. ("**Compensia**"), a national compensation consulting firm, to provide advice and guidance with regard to compensation for our NEOs. The decision to engage the consultant was not made or recommended by management and the Compensation Committee has the sole discretion to engage or change the consultant. The Compensation Committee met four times during fiscal 2025. The current members of the Compensation Committee are Michael Perry (Committee Chair), Hongbo Lu, and William Waddill. The Board has determined that all members of the Compensation Committee are independent directors under the Nasdaq Marketplace Rules and satisfy the additional independence criteria applicable to directors on the Compensation Committee under the rules and regulations of the SEC and the Nasdaq Marketplace Rules. The Compensation Committee Charter is available on the Company's website at www.arrowheadpharma.com. The Compensation Committee has not delegated any of its responsibilities or authorities granted under its charter but has delegated authority under the 2021 Incentive Plan to the Chief Executive Officer to grant equity awards to non-executive employees within prescribed limits set by the Compensation Committee.

The Nomination Committee is responsible for proposing a slate of directors for election by the stockholders at each annual meeting and for proposing candidates to fill any vacancies. The Nomination Committee met once during fiscal 2025. The current members of the Nomination Committee are Victoria Vakiener (Committee Chair), Mauro Ferrari, Adeoye Olukotun, Michael Perry, Hongbo Lu, and William Waddill. The Board has determined that all members of the Nomination Committee are independent directors under the Nasdaq Marketplace Rules. The Nomination Committee's charter is available on the Company's website at www.arrowheadpharma.com. The Nomination Committee manages the process for evaluating current Board members at the time they are considered for re-nomination. After considering the appropriate skills and characteristics required on the Board, the current makeup of the Board, the results of the evaluations and the wishes of the Board members to be re-nominated, the Nomination Committee recommends to the Board whether those individuals should be re-nominated.

On at least an annual basis, the Nomination Committee reviews with the Board whether it believes the Board would benefit from adding new members and, if so, the appropriate skills and characteristics required for any new members. If the Board determines that a new member would be beneficial, the Nomination Committee solicits and receives recommendations for candidates and manages the process for evaluating candidates. All potential candidates, regardless of their source, are reviewed under the same process. The Nomination Committee (or the Committee Chair) screens the available information about the potential candidate(s). Based on the results of the initial screening, interviews with candidates are scheduled with Nomination Committee members, other members of the Board and

senior members of management. Upon completion of these interviews and other due diligence, the Nomination Committee may recommend a candidate to the Board for appointment.

Candidates for independent Board member positions are identified through recommendations from directors or others associated with the Company, as well as, from time to time, through a formal search process managed by a third-party search firm. Arrowhead stockholders may also recommend candidates by sending the candidate's name and resume to the Nomination Committee pursuant to the procedures set forth above for communication with the Board. The Nomination Committee evaluates director candidates recommended by stockholders in the same way that it evaluates candidates recommended by its members, other members of the Board, or other persons. As described above, our Bylaws also provide for separate notice procedures to recommend a person for nomination as a director to be considered by stockholders at a meeting, including requirements as to the timing, form and content of a stockholder's notice.

The Nomination Committee has no predefined minimum criteria for selecting Board nominees, although it believes that all directors should share qualities such as governance and business experience at the corporate level, relevant competitive experience, and strong communication and analytical skills. Independent directors must meet the criteria for independence set forth by the Nasdaq Marketplace Rules and, as applicable, the SEC. In any given search, the Nomination Committee may also define particular characteristics for candidates to balance the overall mix of skills, backgrounds and characteristics of the Board and the needs of the Company. During any search, the Nomination Committee reserves the right to modify its stated search criteria for exceptional candidates.

In 2018, the Board established a Science Committee to review and advise on science and technology topics of interest to the Company. The current members of the Science & Technology Committee are Mauro Ferrari (Committee Co-Chair), Adeoye Olukotun (Committee Co-Chair), Hongbo Lu, and Michael Perry.

Each of our incumbent directors attended at least 75% of the aggregate of (i) the total number of meetings of the Board held during fiscal 2025, and (ii) the total number of meetings held by all committees of the Board during fiscal 2025 on which such person served, in each case during the period in which such person served on the Board or committee and was expected to attend such meeting(s) in accordance with the Company's governance policies and practices.

In addition, all of the directors then serving on the Board and standing for re-election attended the virtual 2025 Annual Meeting of Stockholders. It is the Company's policy to encourage, but not require, that all directors attend our annual stockholder meetings.

Director Compensation

Directors who are also employees of the Company receive no separate compensation from the Company for their service as members of the Board. For 2025, the Company maintained the structure of director compensation it adopted in 2019 to provide a base retainer for each director with higher base retainers for service by the Board Chair and committee leadership as well as an annual equity award of restricted stock units that vests one year from the date of grant or, in the case of a newly-appointed director, an initial equity award of restricted stock units that vests in three equal annual installments on each anniversary of the date of grant, subject, in each case, to continued service through the applicable vesting date. The average total compensation paid to the Company's non-executive directors for service in 2025 is at or below the 60th percentile of the total compensation paid to non-executive directors of its peer group as described later in this proxy statement. The Compensation Committee believes the structure aligns compensation according to the level of service contributions by each director. The fees payable to directors for service on the Board and for service on each committee of the Board on which the director serves are as follows:

Board of Directors:	2025 Annual Retainer:	2024 Annual Retainer:
All non-employee directors	$80,000	$80,000
Additional retainer for Lead Independent Director	$15,000	$15,000
Audit Committee:		
Chairman	$5,000	$5,000
Compensation Committee:		
Chairman	$10,000	$5,000
Nomination Committee		
Chairman	$5,000	$—

The following table sets forth the total compensation paid to our non-employee directors in fiscal 2025. Dr. Anzalone's compensation is set forth in the discussion of Executive Compensation and in the Summary Compensation Table. He did not receive any additional compensation for his services as a director.

Name	Fee Earned or Paid in Cash ($)	Stock Awards ($) (1) (2)	Total ($)
Douglass Given (3)	$23,750	$—	$23,750
Michael S. Perry	$87,500	$340,684	$428,184
Mauro Ferrari	$80,000	$340,684	$420,684
William Waddill	$97,500	$340,684	$438,184
Hongbo Lu	$80,000	$340,684	$420,684
Douglas Ingram (4)(5)	$60,000	$786,350	$846,350
Adeoye Olukotun	$80,000	$340,684	$420,684
Victoria Vakiener	$83,750	$340,684	$424,434

(1) This column represents the total grant date fair value, computed in accordance with ASC 718, of RSUs granted during fiscal year 2025 to each director. The assumptions used to calculate the value of the stock underlying the RSU awards are set forth in Note 9 of the Notes to the Consolidated Financial Statements included with the Company's Annual Report on Form 10-K.

(2) The RSUs granted to non-employee directors vest one year from the date of grant, subject to continued service through the vesting date, with the exception of the RSUs granted to Mr. Ingram in connection with his appointment to the Board, which vest in three equal installments on each anniversary of the date of grant, subject to continued service through each such vesting date.

(3) Douglass Given retired from the Board effective as of December 31, 2024.

(4) Mr. Ingram joined the Board during fiscal year 2025 and his cash compensation was pro-rated for such fiscal year.

(5) In connection with his appointment, Mr. Ingram received a sign-on grant of restricted stock units valued at $786,350.

As of the last day of fiscal year 2025, the directors held the following outstanding restricted stock unit ("**RSU**") grants in the aggregate: Douglass Given — 0; Michael S. Perry — 16,733; Mauro Ferrari —16,733; William Waddill — 16,733; Adeoye Olukotun — 16,733; Victoria Vakiener — 16,733; Hongbo Lu — 32,786; and Douglas Ingram — 38,396.

As of the last day of fiscal year 2025, the directors held the following outstanding option ("**Options**") grants in the aggregate: Douglass Given — 0; Michael S. Perry — 4,593; Mauro Ferrari — 4,593; William Waddill — 4,593; Adeoye Olukotun — 4,593; Victoria Vakiener — 4,593; Hongbo Lu — 0; and Douglas Ingram — 0.

Vote Required; Recommendation of the Board

The nominees listed above receiving more "FOR" votes than "AGAINST" votes, assuming a quorum is present, will be elected as directors to serve until the 2027 Annual Meeting or until their successors have been duly elected and qualified. Because directors are elected by a majority of votes cast, abstentions from voting and broker non-votes, if any, will have no effect on the outcome of this Proposal.

THE BOARD UNANIMOUSLY RECOMMENDS A VOTE "FOR" EACH OF THE NOMINEES FOR DIRECTOR IN PROPOSAL ONE.

Proposal Two — Advisory Vote to Approve Executive Compensation

The compensation paid to our Named Executive Officers ("**NEOs**") is described below in the Compensation Discussion and Analysis of this Proxy Statement for the year ended September 30, 2025. As required pursuant to Section 14A of the Exchange Act, the Board is asking stockholders to cast a non-binding, advisory vote "FOR" the following resolution:

> "RESOLVED, that the compensation paid to the Company's named executive officers, as disclosed pursuant to Item 402 of Regulation S-K, as set forth in the compensation tables and narrative discussion in this Proxy Statement, is hereby APPROVED, on an advisory basis."

Although the vote we are asking you to cast is non-binding, the Compensation Committee and the Board value the views of our stockholders and will consider the outcome of the vote when determining future compensation arrangements for our NEOs.

The Board has adopted a policy providing for annual advisory votes to approve executive compensation. Unless the Board modifies its policy on the frequency of holding advisory votes to approve executive compensation, the next such advisory vote will occur at the 2027 Annual Meeting.

Vote Required; Recommendation of the Board

Proposal Two must be approved by the Required Vote, assuming a quorum is present. For this purpose, abstentions will be counted as a vote "AGAINST" the Proposal, while broker non-votes, if any, will have no effect on the outcome of the vote.

THE BOARD UNANIMOUSLY RECOMMENDS A VOTE "FOR" PROPOSAL TWO.

Proposal Three —Approval of the Arrowhead Pharmaceuticals, Inc. Amended and Restated 2021 Incentive Plan

On January 26, 2021, the Board adopted the Arrowhead Pharmaceuticals, Inc. 2021 Incentive Plan (the "**Original Plan**"), which was subsequently approved by the Company's stockholders on March 18, 2021. The Original Plan replaced the Company's 2013 Incentive Plan (the "**Prior Plan**") with respect to the grant of new awards from and after March 18, 2021. In order to continue to provide employees, directors, consultants and key advisors with stock-based incentives, on January 21, 2026, the Board approved, subject to stockholder approval, the Arrowhead Pharmaceuticals, Inc. Amended and Restated 2021 Incentive Plan (the "**Plan**") to increase the total number of shares of the Company's common stock available for stock-based awards by 10,500,000 shares, extend the term of the Plan to January 21, 2036, and make certain other administrative changes. The Plan will become effective only after approval of stockholders at the Annual Meeting. We believe that employees, directors, consultants and key advisors should have a significant stake in the Company under programs that link compensation to stockholder return. As a result, equity compensation is considered to be an integral part of the Company's compensation program. The Board approved the Plan because the Original Plan has very limited remaining shares available for grant and the Company does not have any other plans under which equity awards can be granted to existing employees. If stockholders do not approve this proposal, we will continue to have the authority to grant awards under the Original Plan, but the proposed 10,500,000 share increase will not be effective, which could result in a serious disruption of our compensation programs and will limit our ability to provide retention incentives to our executives and other employees. Equity awards are a significant component of total compensation for our executive officers and other employees and are vital to our ability to attract and retain outstanding and highly skilled individuals in the extremely competitive labor markets in which we must compete. If stockholders do not approve the proposal, we would need to grant cash and other non-equity rewards to these individuals. We believe that such alternative forms of compensation do not align employee interests with those of stockholders as efficiently as equity-based awards, and we think it is important to provide compensation that continues to effectively align employees with stockholders and which provides a total compensation package that is competitive with other companies. We strongly believe that the approval of this proposal is instrumental to our continued success.

The Board believes that the Plan will continue to promote the interests of stockholders and is consistent with principles of good corporate governance, including:

- Independent Committee. The Plan will continue to be administered by our Compensation Committee, which is composed entirely of independent directors who meet the Nasdaq standards for independence and who meet the definition of "non-employee directors" under Rule 16b-3(b)(3).
- No Discounted Stock Options or SARs. All stock option and stock appreciation rights ("**SAR**") awards under the Plan must continue to have an exercise or base price that is not less than the fair market value of the underlying common stock on the date of grant.
    - No Repricing. Other than in connection with a corporate transaction affecting the Company, the Plan prohibits any repricing of stock options or SARs without stockholder approval.
    - No Dividends on Unvested Awards. Dividends or dividend equivalent amounts payable in respect of awards are subject to the same vesting and forfeiture restrictions as apply to the underlying awards.
- Minimum Vesting. All awards are still subject to a one-year minimum vesting requirement, provided that (i) we may grant up to a maximum of 5% of the aggregate number of shares available for issuance under the Plan without such a minimum vesting period, (ii) awards to non-employee directors need not comply with the one year minimum vesting requirement so long as such awards do not vest earlier than the date of the annual meeting following the grant date, provided that such annual meeting is at least 50 weeks after the immediately preceding year's annual meeting, and (iii) awards granted in connection with a merger or acquisition as a substitute or replacement award for awards held by grantees of the acquired business need not comply with the one (1) year minimum vesting requirements.
- Holding Period. All awards granted under the Plan to our Chief Executive Officer will continue to be subject to a 12-month holding period requirement pursuant to which the Chief Executive Officer may not transfer or dispose of any shares received upon exercise or settlement of an award (net of tax withholding) for a period of 12 months following the date of such exercise or settlement.

Existing Equity Plan Information

We currently have three plans that provide for equity-based compensation. Under the Prior Plan and Original Plan, as of January 15, 2026, 1,688,374 and 5,385,095 shares, respectively, of Arrowhead's common stock are reserved for the grant of stock options, vesting of restricted stock awards and performance unit/share awards to employees, consultants and others. No further grants may be made under the Prior Plan. As of January 15, 2026, there were outstanding options to purchase 588,374 and 32,151 shares of common stock under the Prior Plan and the Original Plan, respectively, and there were 1,100,000 and 4,656,512 outstanding restricted stock units under the Prior Plan and the Original Plan, respectively. Also, as of January 15, 2026, there were outstanding stand-alone inducement awards granted outside of any equity compensation plan consisting of 440,713 outstanding options and 52,125 outstanding restricted stock units. In addition, the Board established the Inducement Plan during fiscal year 2024 to advance the interests of the Company by providing for the grant of stock-based awards. In accordance with Nasdaq rules, the Inducement Plan is used to offer equity awards as material inducements for new employees to join the Company. The maximum aggregate number of shares that may be issued under the inducement plan is 832,950. As of January 15, 2026, there were no outstanding options to purchase shares of common stock and there were 682,980 outstanding restricted stock units under the Inducement Plan.

The following table includes aggregated information regarding awards outstanding as of January 15, 2026 under the Prior Plan, the Original Plan, the Inducement Plan, and stand-alone inducement awards, and the proposed number of additional shares issuable under the Plan:

Outstanding SAR Awards	1,061,238
Weighted Average Exercise Price	$32.91
Weighted Average Remaining Term	3.5
Outstanding Full Value Awards	6,491,617
Number of Shares Available for Grant Under Inducement Plan	12,855
Number of Shares Available for Grant Under Original Plan	696,432
Total Shares of Common Stock Outstanding	139,794,665
Number of additional Shares Requested for Plan	10,500,000

Reasons for Seeking Stockholder Approval

The Board believes that equity grants are a critical part of the Company's compensation program. Stockholder approval of the Plan would allow us to continue to attract and retain employees, directors, consultants and key advisors with equity incentives. The Board believes that the additional number of shares authorized to be issued under the Plan is necessary in order to continue to meet our compensation objectives. Stock-based awards comprise a significant component of the compensation of our executive management team and are important tool in attracting and retaining key employees. Furthermore, the Board believes the grant of equity based compensation awards aligns the long-term interests of stockholders and key employees.

When approving the Plan and the number of shares available for issuance thereunder, the Board considered the burn rate with respect to the equity awards granted by the Company, as well as the Company's overhang. The burn rate is equal to the total number of equity awards the Company granted in a fiscal year divided by the weighted average common stock outstanding during the year. Overhang is equal to the total number of equity awards outstanding plus the total number of shares available for grant under the Company's equity plans, divided by the sum of the total common stock outstanding, the number of equity awards outstanding and the total number of shares available for grant under the Company's equity plans. The Company's three-year average burn rate for fiscal years 2025, 2024, and 2023, was approximately 1.63%. The Company's overhang as of January 15, 2026 was 5.6%. If the Plan is approved, the Company's overhang would increase to 11.8% and the Company will be expected to have sufficient shares to fund future equity grants under the Plan for approximately the next five years.

Summary of the Plan

On January 21, 2026, the Board adopted the Plan, subject to approval by our stockholders. This summary is not a complete description of all provisions of the Plan and is qualified in its entirety by reference to the Plan, which is attached as Exhibit A to this Proxy Statement.

Purpose. The purpose of the Plan is to advance the Company's interests by providing for additional shares of stock for the grant to participants of stock-based and other incentive awards.

Plan Administration. The Plan is administered by our Compensation Committee, who has the authority to, among other things, interpret the Plan, determine eligibility for, grant and determine the terms of awards under the Plan, and to do all things necessary or appropriate to carry out the purposes of the Plan. Subject to the limitations and

requirements set forth in the Plan, the Compensation Committee may authorize one or more officers of the Company to perform any of its duties, power and responsibilities. Our Compensation Committee's determinations under the Plan are conclusive and binding.

Term. No awards will be made after the tenth anniversary of the Plan's adoption, but previously granted awards may continue beyond that date in accordance with their terms.

Authorized Shares. Subject to adjustment, the maximum number of shares of our common stock that may be delivered in satisfaction of awards under the Plan is 18,500,000, reduced by any shares of common stock subject to awards made under the Prior Plan after January 1, 2021. Shares of common stock subject to outstanding awards under the Prior Plan as of January 1, 2021 that, after January 1, 2021, are canceled, expired, forfeited or otherwise not issued under such award (other than as a result of being tendered or withheld to pay the exercise price or withholding taxes in connection with any such awards) or settled in cash will be added to the number of shares of common stock issuable under the Plan.

Any shares of common stock underlying awards that are settled in cash or that otherwise expire, terminate or are forfeited prior to the issuance of stock, would again be available for issuance under the Plan. Shares of common stock that are tendered to or withheld by the Company from an award in payment of the exercise price of any award requiring exercise or in satisfaction of the tax withholding requirements with respect to any award and shares of stock purchased by the Company with proceeds from the exercise of stock options will not again be available for issuance under the Plan. Stock delivered under the Plan may be authorized but unissued stock or previously issued stock acquired by the Company. On January 15, 2026, the closing price of our common stock as reflected on Nasdaq was $66.25.

Eligibility. Our Compensation Committee selects participants from among the key employees, directors, consultants and advisors of the Company and its affiliates. Eligibility for options intended to be incentive stock options ("ISOs") is limited to employees of the Company or certain affiliates. As of January 15, 2026, approximately 796 employees and 8 directors were eligible to participate in the Plan.

Types of Awards. The Plan provides for grants of options, SARs, restricted and unrestricted stock and stock units, performance awards, cash awards and other awards convertible into or otherwise based on shares of our stock. Dividend equivalents may also be provided in connection with awards under the Plan.

Restricted and Unrestricted Stock	A restricted stock award is an award of common stock subject to forfeiture restrictions, while an unrestricted stock award is not subject to restrictions.
Stock Options and SARs	The Plan provides for the grant of ISOs, non-qualified stock options ("NSOs"), and SARs. The exercise price of an option, and the base price against which a SAR is to be measured, may not be less than the fair market value (or, in the case of an ISO granted to a ten percent stockholder, 110% of the fair market value) of a share of common stock on the date of grant. Subject to the minimum vesting requirement described below, our Compensation Committee determines when stock options or SARs become exercisable and the terms on which such awards remain exercisable. Stock options and SARs will generally have a maximum term of ten years. SARs are payable in cash, in shares of our common stock or in a combination of cash and shares.
Stock Units	A stock unit award is denominated in shares of common stock and entitles the participant to receive stock or cash measured by the value of the shares in the future. The delivery of stock or cash under a stock unit may be subject to the satisfaction of performance or other vesting conditions.
Performance Awards	A performance award is an award the vesting, settlement or exercisability of which is subject to specified performance criteria.
Cash Awards	A cash award is an award denominated in cash.

Vesting. Subject to the minimum vesting requirement described in the next sentence, our Compensation Committee has the authority to determine the vesting schedule applicable to each award, and to accelerate the vesting or exercisability of any award. No award may be granted with terms providing for any right of vesting, exercise or lapse of vesting requirements earlier than a date that is at least one (1) year following the date of grant (or, in the case of a performance award, earlier than the one (1) year anniversary of the commencement of the performance period). Notwithstanding the foregoing, the Compensation Committee may grant up to a maximum of 5% of the aggregate number of shares available for issuance under the Plan without such a minimum vesting period. In addition, (i) awards granted to non-employee directors may vest earlier than one (1) year from the date of grant so long as such

awards do not vest earlier than the date of the next annual meeting of stockholders following the grant date (provided such annual meeting is at least 50 weeks after the immediately preceding year's annual meeting) and (ii) awards granted in connection with a merger or other acquisition as a substitute or replacement award for awards held by grantees of the acquired business need not comply with the one (1) year minimum vesting and exercise requirements.

In addition, all awards granted under the Plan to our Chief Executive Officer will be subject to a 12-month holding period requirement, pursuant to which the Chief Executive Officer may not transfer or dispose of any shares received upon exercise or settlement of an award (net of tax withholding) for a period of 12 months following the date of such exercise or settlement.

Termination of Employment. Our Compensation Committee determines the effect of termination of employment or service on an award. Unless otherwise provided by our Compensation Committee or in an award agreement, upon a termination of employment or service all unvested options and other awards requiring exercise will terminate and all other unvested awards will be forfeited.

Performance Criteria. The Plan provides that grants of performance awards may be made based upon, and subject to achieving, "performance objectives" over a specified performance period. Performance objectives may include, without limitation, a measure of performance relating to any, or any combination of, the following (measured either absolutely or by reference to an index or indices and determined either on a consolidated basis or, as the context permits, on a divisional, subsidiary, line of business, project or geographical basis or in combinations thereof): sales; revenues; assets; expenses; earnings before or after deduction for all or any portion of interest, taxes, depreciation, or amortization, whether or not on a continuing operations or an aggregate or per share basis; return on equity, investment, capital or assets; one or more operating ratios; borrowing levels, leverage ratios or credit rating; market share; capital expenditures; cash flow; stock price; stockholder return; sales of particular products or services; customer acquisition or retention; acquisitions and divestitures (in whole or in part); joint ventures and strategic alliances; spin-offs, split-ups and the like; reorganizations; or recapitalizations, restructurings, financings (issuance of debt or equity) or refinancings.

Transferability. Awards under the Plan may not be transferred except by will or by the laws of descent and distribution, unless (for awards other than ISOs) otherwise provided by our Compensation Committee.

Corporate Transactions. In the event of a consolidation, merger or similar transaction, a sale or transfer of all or substantially all of the Company's assets or a dissolution or liquidation of the Company, our Compensation Committee may, among other things, provide for the continuation or assumption of outstanding awards, for new grants in substitution of outstanding awards, for the accelerated vesting or delivery of shares under awards or for a cash-out of outstanding awards, in each case on such terms and with such restrictions as it deems appropriate.

Change in Control. Unless otherwise expressly provided for in the award agreement or another contract, including an employment or severance agreement or severance plan, or under the terms of a transaction constituting a Change in Control (as defined in the Plan), in the event of a Change in Control, each outstanding award will fully vest and become exercisable, including to the maximal value of performance awards. Except as our Compensation Committee may otherwise determine awards not assumed or exercised will terminate in connection with such corporate transaction.

Adjustment. In the event of certain corporate transactions (including, but not limited to, a stock dividend, stock split or combination of shares, recapitalization or other change in the Company's capital structure that constitutes an equity restructuring within the meaning of ASC 718), the Compensation Committee will make appropriate adjustments to the maximum number of shares that may be delivered under the Plan, and will also make appropriate adjustments to the number and kind of shares of stock or securities subject to awards, the exercise prices of such awards or any other terms of awards affected by such change. Our Compensation Committee may also make the types of adjustments described above to take into account distributions to stockholders and events other than those listed above if it determines that such adjustments are appropriate to avoid distortion in the operation of the plan.

Amendment and Termination. Our Compensation Committee can amend the Plan or outstanding awards, or terminate the Plan as to future grants of awards, except that our Compensation Committee will not be able alter the terms of an award if it would affect materially and adversely a participant's rights under the award without the participant's consent (unless expressly provided in the Plan or reserved by our Compensation Committee at the time of grant). Stockholder approval will be required for any amendment to the extent such approval is required by law, including the Code or applicable stock exchange requirements.

Federal Income Tax Consequences Under the Plan

The following is a summary of some of the material federal income tax consequences associated with the grant and exercise of awards under the Plan under current federal tax laws and certain other tax considerations associated with awards under the Plan. The summary does not address tax rates or non-U.S., state, or local tax consequences, nor does it address employment-tax or other federal tax consequences except as noted.

Restricted Stock. A participant who is awarded or purchases shares subject to a substantial risk of forfeiture generally does not have income until the risk of forfeiture lapses. When the risk of forfeiture lapses, the participant has ordinary income equal to the excess of the fair market value of the shares at that time over the purchase price, if any, and a corresponding deduction is generally available to the Company. However, a participant may make an election under Section 83(b) of the Code to be taxed on restricted stock when it is acquired rather than later, when the substantial risk of forfeiture lapses. An 83(b) election must be made not later than thirty days after the transfer of the shares to the participant and must satisfy certain other requirements. A participant who makes an effective 83(b) election will realize ordinary income equal to the fair market value of the shares as of the time of acquisition less any price paid for the shares. A corresponding deduction will generally be available to the Company. Fair market value for this purpose is determined without regard to the forfeiture restrictions. If a participant makes an effective 83(b) election, no additional income results by reason of the lapsing of the restrictions.

For purposes of determining capital gain or loss on a sale of shares awarded under the Plan, the holding period in the shares begins when the participant realizes taxable income with respect to the transfer. The participant's tax basis in the shares equals the amount paid for the shares plus any income realized with respect to the transfer. However, if a participant makes an effective 83(b) election and later forfeits the shares, the tax loss realized as a result of the forfeiture is limited to the excess of what the participant paid for the shares (if anything) over the amount (if any) realized in connection with the forfeiture.

ISOs. In general, a participant realizes no taxable income upon the grant or exercise of an ISO. However, the exercise of an ISO may result in an alternative minimum tax liability to the participant. With some exceptions, a disposition of shares purchased under an ISO within two years from the date of grant or within one year after exercise produces ordinary income to the participant (and generally a deduction to the Company) equal to the value of the shares at the time of exercise less the exercise price. Any additional gain recognized in the disposition is treated as a capital gain for which the Company is not entitled to a deduction. If the participant does not dispose of the shares until after the expiration of these one and two-year holding periods, any gain or loss recognized upon a subsequent sale is treated as a long-term capital gain or loss for which the Company is not entitled to a deduction.

NSOs. In general, a participant has no taxable income upon the grant of an NSO but realizes income in connection with exercise of the option in an amount equal to the excess (at time of exercise) of the fair market value of the shares acquired upon exercise over the exercise price. A corresponding deduction is generally available to the Company. Upon a subsequent sale or exchange of the shares, any recognized gain or loss is treated as a capital gain or loss for which the Company is not entitled to a deduction. An ISO that is exercised more than three months after termination of employment (other than termination by reason of death) is generally treated as an NSO. ISOs are also treated as NSOs to the extent they first become exercisable by an individual in any calendar year for shares having a fair market value (determined as of the date of grant) in excess of $100,000.

SARs. The grant of a SAR does not itself result in taxable income, nor does taxable income result merely because a SAR becomes exercisable. In general, a participant who exercises a SAR for shares of stock or receives payment in cancellation of a SAR will have ordinary income equal to the amount of any cash and the fair market value of any stock received. A corresponding deduction is generally available to the Company.

Restricted Stock Units. The grant of a restricted stock unit does not itself generally result in taxable income. Instead, the participant is taxed upon vesting (and a corresponding deduction is generally available to the Company), unless he or she has made a proper election to defer receipt of the shares (or cash if the award is cash settled) under Section 409A of the Code ("Section 409A"). If the shares delivered are restricted for tax purposes, the participant will instead be subject to the rules described above for restricted stock.

Section 409A. Section 409A imposes an additional 20% income tax, plus, in some cases, a further income tax in the nature of interest, on nonqualified deferred compensation that does not comply with deferral, payment-timing and other formal and operational requirements specified in Section 409A and related regulations and that is not exempt from those requirements. Stock options and SARs granted under the Plan are intended to be exempt from Section 409A. The Plan gives the Compensation Committee the flexibility to prescribe terms for other awards that are consistent with the requirements of, or an exemption from, Section 409A.

Certain Change of Control Payments. Under Section 280G of the Code, the vesting or accelerated exercisability of options or the vesting and payments of other awards in connection with a change of control of a corporation may be required to be valued and taken into account in determining whether participants have received compensatory payments, contingent on the change in control, in excess of certain limits. If these limits are exceeded, a substantial

portion of amounts payable to the participant, including income recognized by reason of the grant, vesting or exercise of awards, may be subject to an additional 20% federal tax and may be non-deductible to the Company.

Plan Benefits; Market Value of Securities

The benefits that will be awarded or paid in the future under the Plan are not currently determinable as awards under the Plan are within the discretion of the Compensation Committee, and the Compensation Committee has not determined future awards or who might receive them. As of January 15, 2026, awards covering 7,926,708 shares of our common stock have been granted under the Original Plan. The following table shows information regarding the distribution of awards covering such shares as of such date among the persons and groups identified below. The closing market price of our common stock as reflected on Nasdaq on January 15, 2026 was $66.25.

Name and Position	Number of Options Granted (#)	Number of Shares Subject to Stock Awards (#)
Christopher Anzalone, President and Chief Executive Officer	—	1,625,753
James Hamilton, Chief Medical Officer	—	365,000
Daniel Apel, Chief Financial Officer	—	75,000
Patrick O'Brien, Chief Operating Officer	—	385,000
All current executive officers as a group (4 persons)	—	2,450,753
Kenneth Myszkowski, Former Chief Financial Officer	—	295,000
Tracie Oliver, Former Chief Commercial Officer	—	75,500
All current directors who are not executive officers as a group (7 persons)	22,965	372,909
Each nominee for election as a director	—	—
Each associate of any such directors, executive officers, or nominees	—	—
Each other person who received or is to receive 5% of such options, warrants, or rights	—	—
All current employees, including all current officers who are not executive officers, as a group	9,186	4,545,845

Registration

If our stockholders approve the Plan, we will file with the SEC a registration statement on Form S-8, as soon as reasonably practicable after the approval, to register the additional shares available for issuance under the plan.

The Board is asking stockholders to cast a vote "FOR" the following resolution:

"RESOLVED, that the stockholders of the Company approve the Arrowhead Pharmaceuticals, Inc. Amended & Restated 2021 Incentive Plan in a form substantially as provided in Exhibit A to this Proxy Statement."

Vote Required; Recommendation of the Board

Proposal Three must be approved by the Required Vote, assuming a quorum is present. For this purpose, abstentions will be counted as a vote "AGAINST" the Proposal, while broker non-votes, if any, will have no effect on the outcome of the vote.

THE BOARD UNANIMOUSLY RECOMMENDS A VOTE "FOR" PROPOSAL THREE.

Executive Compensation

COMPENSATION DISCUSSION AND ANALYSIS

The following compensation discussion and analysis contains statements regarding future individual and Company performance targets and goals. These targets and goals are disclosed in the limited context of Arrowhead's executive compensation program and should not be understood to be statements of management's expectations or guidance. Arrowhead cautions investors not to apply these statements to other contexts. Fiscal years are denoted as fiscal years, all other year references refer to calendar years.

This Compensation Discussion and Analysis describes the compensation program for our NEOs. During fiscal 2025, these individuals were:

- Christopher Anzalone, our President and Chief Executive Officer (our "**CEO**");
- James Hamilton, our Chief Medical Officer (our "**CMO**");
- Daniel Apel, our Chief Financial Officer (our "**CFO**" after April 2025)
- Patrick O'Brien, our Chief Operating Officer (our "**COO**")*;
- Kenneth Myszkowski, our former Chief Financial Officer (our "**Former CFO**"); and
- Tracie Oliver, our former Chief Commercial Officer (our "**Former CCO**").

*Mr. O'Brien also served as our General Counsel until December 17, 2025

This Compensation Discussion and Analysis describes the material elements of our executive compensation program during fiscal 2025. It also provides an overview of our executive compensation philosophy and objectives and summarizes our executive compensation policies and practices. Finally, it analyzes how and why the Compensation Committee of our Board arrived at the specific compensation decisions for our executive officers, including our NEOs, for fiscal 2025, including the key factors that the Compensation Committee considered in determining their compensation.

Our Company

We develop medicines that treat intractable diseases by silencing the genes that cause them. Using a broad portfolio of RNA chemistries and efficient modes of delivery, our therapies trigger the RNA interference mechanism to induce rapid, deep and durable knockdown of target genes. RNA interference, or RNAi, is a mechanism present in living cells that inhibits the expression of a specific gene, thereby affecting the production of a specific protein. Arrowhead's RNAi-based therapeutics leverage this natural pathway of gene silencing.

Arrowhead is focused on developing innovative drugs for diseases with a genetic basis, typically characterized by the overproduction of one or more proteins. The depth and versatility of our RNAi technologies enable us to potentially address conditions in virtually any therapeutic area and pursue disease targets that are not otherwise addressable by small molecules and biologics.

Business Highlights

During fiscal year 2025 and through the date of this Proxy Statement, the Company continued to develop and advance its pipeline and partnered candidates and expand its facilities to support its growing programs. The bullets below highlight some of these key developments; however, this list is not all-inclusive.

- Completed upsized offerings of convertible senior notes, common stock, and pre-funded warrants with gross proceeds of $930 million meaningfully strengthening the company's balance sheet and expected to provide funding through multiple potential commercial launches.

- Announced interim results from two Phase 1/2a clinical trials of ARO-INHBE and ARO-ALK7, the company's investigational RNA interference (RNAi) therapeutics being developed as potential treatments for obesity, showing:

- ARO-INHBE in combination with tirzepatide, a GLP-1/GIP receptor co-agonist, doubled weight loss and tripled reductions in visceral fat, total fat, and liver fat versus tirzepatide alone in obese patients with type 2 diabetes mellitus.

- ARO-ALK7, the first RNAi-therapeutic to show adipocyte gene target silencing in a clinical trial, achieved dose dependent reductions in adipose ALK7 mRNA with a mean reduction of -88% at the 200 mg dose at week 8 with a maximum reduction of -94%.

- On December 2, 2025, the United States Food and Drug Administration (FDA) granted Breakthrough Therapy designation to investigational plozasiran as an adjunct to diet to reduce triglyceride (TG) levels in adults with severe hypertriglyceridemia (SHTG) (TG levels greater than or equal to 500 mg/dL);

- On November 20, 2025, the Company earned a $200.0 million milestone payment from Sarepta, which was paid on January 15, 2026. The milestone was earned when Arrowhead achieved the second development milestone event in a Phase 1/2 clinical study of ARO-DM1, also called SRP-1003, an investigational RNAi therapeutic for the treatment of type 1 myotonic dystrophy (DM1), the most common adult-onset muscular dystrophy. The second milestone event included the achievement of a patient enrollment target, drug safety committee review and subsequent authorization to dose escalate and proceed, and completion of day 105 study visit by at least one patient in the clinical trial;

- The FDA approved the Company's New Drug Application (NDA) for REDEMPLO (plozasiran) injection for Familial Chylomicronemia Syndrome (FCS), on November 18, 2025. This approval was supported by clinical data from the Phase 3 PALISADE study, a randomized, double-blind, placebo-controlled trial in adults with clinically diagnosed or genetically confirmed FCS. The PALISADE study met its primary endpoint and all multiplicity-controlled key secondary endpoints, including demonstrating significant reductions in triglycerides and APOC3. In PALISADE, 25 mg REDEMPLO achieved deep and durable reductions in triglycerides, with a median change from baseline of -80% versus -17% in the pooled placebo group, and a lower numerical incidence of acute pancreatitis compared with placebo. The Company subsequently received approvals for REDEMPLO for the treatment of FCS from the Chinese National Medical Products Administration and Health Canada;

- Filed a request for regulatory clearance to initiate a Phase 1/2a clinical trial of ARO-DIMER-PA, the Company's investigational RNA interference (RNAi) therapeutic being developed as a potential treatment for atherosclerotic cardiovascular disease (ASCVD) due to mixed hyperlipidemia. ARO-DIMER-PA is designed to silence expression of the proprotein convertase subtilisin kexin 9 (PCSK9) and apolipoprotein C3 (APOC3) genes. This represents an important step forward for the RNAi field as it is the first clinical candidate to target two genes simultaneously in one molecule, enabled by Arrowhead's innovative and proprietary TRiM platform;

- Dosed the first subjects in a Phase 1/2a clinical trial of ARO-MAPT, the Company's investigational RNAi therapeutic being developed as a potential treatment for tauopathies including Alzheimer's disease, a progressive neurodegenerative disease characterized by cognitive and functional decline. Alzheimer's disease is the most common cause of dementia and is estimated to affect 32 million people worldwide and is part of a group of neurodegenerative diseases called tauopathies that are marked by the abnormal accumulation and formation of tau tangles in neurons;

- On August 29, 2025, the Company entered into a global licensing and collaboration agreement with Novartis for ARO-SNCA, Arrowhead's preclinical stage siRNA therapy against alpha-synuclein for the treatment of synucleinopathies, such as Parkinson's Disease, and for other additional collaboration targets that will utilize Arrowhead's proprietary TRiM™ platform. Upon closing of the transaction in October 2025, the Company received $200.0 million as an upfront payment and is eligible to receive up to $2.0 billion in potential milestone payments plus royalties on commercial sales;

- Triggered a $100.0 million milestone payment from Sarepta, which was achieved on July 27, 2025, when the Company reached the first of two prespecified enrollment targets and subsequent authorization to dose escalate in a Phase 1/2 clinical study of ARO-DM1, an investigational RNAi therapeutic for the treatment of type 1 myotonic dystrophy (DM1). Arrowhead received $53.2 million worth of Arrowhead common stock and $50.0 million in cash from Sarepta Therapeutics, satisfying the payment of the $100.0 million milestone owed to Arrowhead.

- Announced the signing of an asset purchase agreement between Sanofi and Visirna, a majority-owned subsidiary of the Company, created to develop and commercialize four of the Company's investigational cardiometabolic candidates in Greater China. Under the terms of the agreement, Sanofi acquired rights to develop and commercialize investigational plozasiran, the Company's first-in-class RNAi therapeutic candidate

designed to reduce production of apolipoprotein C-III (APOC3) as a potential treatment for familial chylomicronemia syndrome (FCS) and severe hypertriglyceridemia (sHTG), in Greater China;

- Initiated and dosed the first subject in the YOSEMITE Phase 3 clinical trial of zodasiran, the Company's investigational RNAi therapeutic being developed as a potential treatment for homozygous familial hypercholesterolemia (HoFH), a rare genetic condition that leads to severely elevated LDL-cholesterol and early onset cardiovascular disease;

- Completed enrollment of SHASTA-3, SHASTA-4, and MUIR-3 Phase 3 clinical trials of plozasiran. The Company's global Phase 3 clinical studies are designed to support regulatory submissions for approval of investigational plozasiran in the treatment of severe hypertriglyceridemia;

- Initiated a Phase 1/2a clinical trial of ARO-ALK7 for the treatment of obesity. ARO-ALK7 is the first RNAi-based therapy designed to silence adipocyte expression of the ACVR1C gene to reduce the production of Activin receptor-like kinase 7 (ALK7), which acts as a receptor in a pathway that regulates energy homeostasis in adipose tissue;

- Announced Topline results from Part 2 of a Phase 1/2 clinical study of ARO-C3, the Company's investigational RNAi therapeutic designed to reduce liver production of complement component 3 (C3) as a potential therapy for various complement mediated diseases. ARO-C3 achieved reductions in alternative pathway complement activity and proteinuria;

- Entered into a global licensing and collaboration agreement with Sarepta on November 25, 2024, which closed on February 7, 2025. Upon closing, the Company received $325.0 million through the purchase of 11,926,301 shares of Company common stock by Sarepta, at a price per share of $27.25, and received $500.0 million as an upfront payment on February 24, 2025. The Company will also receive $250.0 million to be paid in equal installments over five years and was eligible to receive the DM1 milestones described above which were already earned. Additionally, the Company is eligible to receive royalties on commercial sales and up to approximately $10.0 billion in future potential milestone payments;

- GSK dosed its fifth patient in a Phase 2 trial in December 2024, triggering a $2.5 million milestone payment to the Company which was paid in the second quarter of fiscal 2025;

- Announced that the Company dosed the first subjects in a Phase 1/2a clinical trial of ARO-INHBE; and

- Presented interim results from a Phase 1/2a clinical study of ARO-CFB at the 8th Complement-Based Drug Development Summit. The study resulted in multiple findings including: (1) ARO-CFB led to dose dependent reductions in circulating CFB protein by up to 90% with greater than 3 months duration, (2) single and multiple doses of ARO-CFB led to near complete inhibition of alternative pathway activity based on Wieslab AP, and (3) single and multiple doses of ARO-CFB led to near complete inhibition of alternative pathway hemolytic activity, measured by AH50.

Business Development

Sarepta Therapeutics, Inc. ("Sarepta")

On November 25, 2024, the Company entered into an Exclusive License and Collaboration Agreement (the "Sarepta Collaboration") with Sarepta Therapeutics, Inc. ("Sarepta") for the co-development and commercialization of ARO-DUX4, ARO-DM1, ARO-MMP7, and ARO-ATXN2 clinical stage programs. Sarepta also received an exclusive sublicensable worldwide license to the Company's ARO-HTT, ARO-ATXN1, and ARO-ATXN3 preclinical stage programs.

Pursuant to the Sarepta Collaboration, Sarepta may select up to six gene targets for which the Company will perform discovery, optimization and preclinical development activities to identify RNAi compounds against each selected target. Upon completion of the Company's preclinical activities, Sarepta will receive an exclusive license to the Company's intellectual property rights to exploit those compounds and be wholly responsible for clinical development and commercialization of each compound.

In connection with the Sarepta Collaboration, on November 25, 2024, the Company entered into a Stock Purchase Agreement (the "Stock Purchase Agreement") with an affiliate of Sarepta for a private placement of shares of common stock of the Company (the "Private Placement"). Pursuant to the Stock Purchase Agreement, the Company

sold 11,926,301 shares of common stock, at a price per share of $27.25, for an aggregate value of approximately $325.0 million.

The Sarepta Collaboration and Private Placement closed concurrently in February 2025.

On July 27, 2025, the Company triggered a $100.0 million milestone payment from Sarepta when the Company reached the first of two prespecified enrollment targets and subsequent authorization to dose escalate in a Phase 1/2 clinical study of ARO-DM1. In satisfaction of the amount owed by Sarepta, the Company elected to receive $53.2 million worth of Arrowhead common stock, at a price per share of $18.79, and $50 million in cash from Sarepta.

On November 20, 2025, the Company triggered a $200.0 million milestone payment when the Company achieved the second of two prespecified enrollment targets and subsequent authorization to dose escalate and proceed in a Phase 1/2 clinical study of ARO-DM1, which was paid on January 15, 2026.

Taken together, the Company received $500.0 million as an upfront payment under the Sarepta Collaboration, $325.0 million in the form of an equity investment under the Stock Purchase Agreement, and $250.0 million to be paid in annual installments of $50.0 million over 5 years. The Company has also triggered $300.0 million in near-term payments associated with the continued enrollment of certain cohorts of a Phase 1/2 study, discussed above. Further, for each of the 13 programs, the Company is eligible to receive development milestone payments between $110.0 million and $180.0 million per program and sales milestone payments between $500.0 million and $700.0 million per program. The Company is also eligible to receive tiered royalties on net sales of licensed products of up to the low double digits.

Sanofi

On August 1, 2025, Visirna Therapeutics HK Limited (“Visirna HK”), a wholly owned subsidiary of Visirna Therapeutics, Inc, a majority owned subsidiary of the Company, entered into an Asset Purchase Agreement with Genzyme Corporation (“Sanofi”), a wholly owned subsidiary of Sanofi S.A., pursuant to which Visirna HK sold all of its assets and rights in investigational plozasiran to Sanofi, which included an assignment of Visirna HK’s rights (as successor by assignment from Visirna) to develop and commercialize investigational plozasiran in Greater China pursuant to that certain License Agreement by and between the Company and Visirna dated, April 25, 2022. As part of this transaction with Sanofi, the Company recorded $130.0 million in revenue during fiscal 2025. Further, pursuant to the agreement, Visirna earned a $10.0 million milestone from Sanofi for the approval of REDEMPLO(R) (plozasiran) for the treatment of FCS in the People's Republic of China.

Novartis Pharma AG ("Novartis")

On August 29, 2025, the Company entered into a global licensing and collaboration agreement with Novartis for ARO-SNCA, Arrowhead’s preclinical stage siRNA therapy against alpha-synuclein for the treatment of synucleinopathies, such as Parkinson’s Disease, and for other additional collaboration targets that will utilize Arrowhead’s proprietary TRiM™ platform. Upon closing on October 17, 2025, the Company received $200.0 million as an upfront payment and is eligible to receive up to $2.0 billion in potential milestone payments plus royalties on commercial sales.

Platform

In fiscal 2025, the Company continued to develop and deploy its Targeted RNAi Molecule platform (“**TRiM**™”) to identify and develop new therapeutics. TRiM™ utilizes ligand-mediated delivery and is designed to enable tissue-specific targeting, while being structurally simple. Targeting has been core to the Company’s development philosophy and the TRiM™ platform builds on more than a decade of work on actively targeted drug delivery vehicles. The TRiM™ platform is designed to offer several potential competitive advantages including:

- A more sophisticated RNAi trigger selection and screening process that identifies potent sequences rapidly in locations that RNAi competitors may miss;
- Multiple routes of administration including subcutaneous, intravenous and inhaled;
- Faster time to clinical candidates;
- Optimal pharmacologic activity and long duration-of-effect;
- Potentially wide safety margins;
- Simplified manufacturing at reduced cost; and

- The ability to take RNAi to tissues beyond the liver, including seven different tissue types with ongoing clinical programs.

Approved Products

REDEMPLO (plozasiran) is approved by the U.S. Food and Drug Administration as an adjunct to diet to reduce triglycerides for adults with Familial Chylomicronemia Syndrome (FCS). REDEMPLO (plozasiran) is also approved by the Chinese National Medical Products Administration (NMPA). In addition, Health Canada has issued a Notice of Compliance (NOC) authorizing REDEMPLO™ (plozasiran) as an adjunct to diet to reduce triglycerides in adults with familial chylomicronemia syndrome (FCS) for whom standard triglyceride lowering therapies have been inadequate. REDEMPLO is an siRNA therapeutic designed to suppress the production of apolipoprotein C-III (APOC3), a protein produced in the liver that raises triglyceride levels by slowing their breakdown and clearance. By targeting the APOC3 gene with sustained silencing, REDEMPLO delivers significant reductions in triglyceride levels. REDEMPLO is the first and only FDA-approved siRNA treatment studied in both genetically confirmed and clinically diagnosed patients living with FCS.

Pipeline

Arrowhead is focused on developing innovative drugs for diseases with a genetic basis, typically characterized by the overproduction of one or more proteins that are involved with disease. The depth and versatility of Arrowhead's RNAi technologies enables Arrowhead to potentially address conditions in many therapeutic areas and pursue disease targets that are not otherwise addressable by small molecules and biologics. Arrowhead is focused on bringing the promise of RNAi to address diseases outside of the liver, and its pipeline now includes disease targets in the liver, lung, muscle and CNS.

*The timing of our planned and already filed clinical trial applications ("**CTA**") discussed below are based on calendar years, not fiscal years.*

Plozasiran (ARO-APOC3) - Severe Hypertriglyceridemia (sHTG)

Severe Hypertriglyceridemia is a disease characterized by elevated triglyceride levels > 500 mg/dL. Severely elevated triglycerides in patients with severe hypertriglyceridemia (sHTG) or familial chylomicronemia syndrome (FCS), a rare genetic disorder, can result in potentially fatal acute pancreatitis.

Plozasiran (formerly ARO-APOC3) is designed to reduce production of apolipoprotein C-III (APOC3), a component of triglyceride rich lipoproteins (TRLs) including Very Low Density Lipoprotein (VLDL) and chylomicrons, a key regulator of triglyceride metabolism. The Company believes that knocking down the hepatic production of APOC3 may result in reduced VLDL synthesis and assembly, enhanced breakdown of TRLs, and better clearance of VLDL and chylomicron remnants.

We are conducting multiple Phase 3 studies to potentially support regulatory filings for approval in sHTG, pending successful completion, including SHASTA-3, SHASTA-4, SHASTA-5, and MUIR-3.

Zodasiran (ARO-ANG3) - Homozygous Familial Hypercholesterolemia (HoFH)

Dyslipidemia and Hypertriglyceridemia are risk factors for atherosclerotic coronary heart disease and cardiovascular events.

Zodasiran (formerly ARO-ANG3) is designed to reduce production of angiopoietin-like protein 3 (ANGPTL3), a liver synthesized inhibitor of lipoprotein lipase and endothelial lipase. ANGPTL3 inhibition has been shown to lower serum LDL, serum and liver triglyceride and has genetic validation as a novel target for cardiovascular disease. The Company is currently investigating zodasiran in one Phase 3 clinical trial (YOSEMITE) to evaluate the efficacy and safety of zodasiran in adolescent and adult subjects with HoFH.

ARO-INHBE - Obesity

ARO-INHBE is designed to reduce the hepatic expression of the INHBE gene and its secreted gene product, Activin E. INHBE is a promising genetically validated target in which loss-of-function INHBE variants in humans are associated with lower risk of obesity and metabolic diseases, such as type 2 diabetes. The Company is currently investigating ARO-INHBE in a Phase 1/2a clinical trial.

ARO-ALK7- Obesity

ARO-ALK7 is designed to silence adipocyte expression of the ACVR1C gene to reduce the production of Activin receptor-like kinase 7 (ALK7), which acts as a receptor in a pathway that regulates energy homeostasis in adipose

tissue. In large genetic datasets, reduced ACVR1C expression has been associated with healthier adipose distribution and reduced risk of obesity-related metabolic complications. The Company is currently investigating ARO-ALK7 in a Phase 1/2a clinical trial.

ARO-C3 - Complement Mediated Renal Disease

A number of rare renal diseases result from uncontrolled activation of the alternative pathway of complement, leading to progressive glomerular damage, proteinuria, hematuria, and impaired kidney function, and often resulting in end-stage renal disease (ESRD). In addition, dysregulation of the alternative complement pathway has been shown to play a role in the pathogenesis and progression of disease in some of the more common glomerulopathies. Silencing complement component 3 (C3) may be a therapeutic approach for treatment of these conditions.

ARO-C3 is designed to reduce production of C3 as a potential therapy for patients with various complement mediated or complement associated renal diseases. The Company is currently investigating ARO-C3 in a Phase 1/2a clinical trial.

ARO-CFB - Complement Mediated Disease

A number of rare renal diseases result from uncontrolled activation of the alternative pathway of complement, leading to progressive glomerular damage, proteinuria, hematuria, and impaired kidney function, and often resulting in end-stage renal disease (ESRD). In addition, dysregulation of the alternative complement pathway has been shown to play a role in the pathogenesis and progression of disease in some of the more common glomerulopathies. Silencing complement factor B (CFB) may be a therapeutic approach for treatment of these conditions.

ARO-CFB is designed to reduce hepatic expression of CFB, which plays an important regulatory role in amplifying complement alternative pathway activation and has been identified as a promising therapeutic target. ARO-CFB is being developed as a potential treatment for complement mediated kidney diseases such as immunoglobulin A nephropathy (IgAN), which is the most common glomerular disease worldwide and carries a high lifetime risk of progression to end-stage renal disease. Additionally, ARO-CFB may have clinical applications in non-renal diseases involving complement activation. The Company is currently investigating ARO-CFB in a Phase 1/2a clinical trial.

ARO-RAGE - Inflammatory Pulmonary Disease

ARO-RAGE is designed to reduce production of the Receptor for Advanced Glycation End products (RAGE) as a potential treatment for various inflammatory pulmonary diseases. The Company is currently investigating ARO-RAGE in a Phase 2a clinical trial.

ARO-PNPLA3 - Metabolic-dysfunction Associated Steatohepatitis (MASH)

MASH is a subgroup of steatotic liver disease (MASLD) in which hepatic cell injury and inflammation has developed over background steatosis. The I148M genetic variant in the PNPLA3 gene is involved with the underlying pathophysiology and is a known risk factor for hepatic steatosis, steatohepatitis, elevated plasma liver enzyme levels, hepatic fibrosis and cirrhosis. The rising prevalence of MASH presents a significant health burden in many developed countries.

ARO-PNPLA3 (formerly JNJ-75220795) is an investigational RNAi therapeutic designed to reduce liver expression of patatin-like phospholipase domain containing 3 (PNPLA3) as a potential treatment for patients with metabolic-dysfunction associated steatohepatitis (MASH). PNPLA3 has strong genetic and preclinical validation as a driver of fat accumulation and damage in the livers of patients who carry the common I148M mutation. Former licensee Janssen Pharmaceuticals, Inc. investigated ARO-PNPLA3 in two Phase 1 clinical trials.

ARO-MAPT - Alzheimer's and Tauopathies

Aggregation of the toxic tau protein is believed to be a key driver in multiple tauopathies, including Alzheimer’s disease. By preventing or potentially reversing tau protein accumulation in subjects with mild cognitive impairment due to Alzheimer’s disease and mild Alzheimer’s disease dementia, ARO-MAPT has the potential to prevent or slow disease progression.

ARO-MAPT is Arrowhead’s first investigational RNAi-based therapy to utilize a new proprietary delivery system which, in preclinical studies, has achieved blood-brain-barrier penetration and deep knockdown of target genes across the central nervous system (CNS), including deep brain regions, after subcutaneous injections. ARO-MAPT is designed to silence CNS expression of the microtubule associated protein tau (MAPT) gene, which encodes the tau protein.

The Company has dosed the first subjects in a Phase 1/2a clinical trial of ARO-MAPT.

ARO-DIMER-PA - Mixed Hyperlipidemia

Mixed Hyperlipidemia is a highly prevalent disorder characterized by elevated low-density lipoprotein cholesterol (LDL-C) and triglyceride (TG) levels and is a major risk factor for ASCVD, which is the leading cause of mortality worldwide and associated with substantial morbidity and healthcare costs.

ARO-DIMER-PA is a dual functional RNAi molecule designed to silence expression of the proprotein convertase subtilisin kexin 9 (PCSK9) and apolipoprotein C3 (APOC3) genes in hepatocytes. Prior clinical experience with other investigational and approved agents suggests that PCSK9 and APOC3 inhibition may lead to robust reductions in LDL-C, TGs, triglyceride rich lipoprotein remnants, and total atherogenic lipoproteins. This represents an important step forward for the RNAi field as it is the first clinical candidate to target two genes simultaneously in one molecule, enabled by Arrowhead's innovative and proprietary TRiM platform.

The Company has filed for regulatory clearance to initiate a Phase 1/2a clinical trial of ARO-DIMER-PA

Partnered Programs

Sarepta Therapeutics, Inc.

SRP-1001 (formerly ARO-DUX4) - Facioscapulohumeral Muscular Dystrophy (FSHD)

Facioscapulohumeral muscular dystrophy (FSHD) is an autosomal dominant disease associated with the failure to maintain complete epigenetic suppression of DUX4 expression in differentiated skeletal muscle, leading to overexpression of DUX4, which is myotoxic and can lead to muscle degeneration. As DUX4 expression is recognized as the cause of muscle pathology in FSHD patients, the Company believes that the selective targeting and knockdown of DUX4 using RNAi may prevent or reverse downstream myotoxicity and lead to muscle repair and improvement in muscle function in patients. There are currently no effective treatments specifically for FSHD.

SRP-1001 is designed to target the gene that encodes human double homeobox 4 (DUX4) protein as a potential treatment for patients with facioscapulohumeral muscular dystrophy. The Company is currently investigating SRP-1001 in a Phase 1/2a clinical trial.

SRP-1003 (formerly ARO-DM1) - Type 1 Myotonic Dystrophy

Type 1 myotonic dystrophy is an autosomal dominant, debilitating, chronic progressive multisystem disorder characterized by an expansion of a highly unstable CUGexp in the DMPK gene. Patients with DM1 have muscle weakness and wasting, myotonia, cataracts, and often have cardiac conduction abnormalities, and may become physically disabled and have a shortened life span.

SRP-1003 is designed to reduce expression of the dystrophia myotonica protein kinase (DMPK) gene. There is currently no approved disease-modifying therapy for type 1 myotonic dystrophy (DM1). Treatments have focused on symptomatic management, including physical therapy, exercise, ankle-foot orthoses, wheelchairs, and other assistive devices. The Company is currently investigating SRP-1003 in a Phase 1/2a clinical trial.

SRP-1002 (formerly ARO-MMP7) - Idiopathic Pulmonary Fibrosis (IPF)

Idiopathic Pulmonary Fibrosis (IPF) is a chronic interstitial lung disease characterized by progressive fibrosis.

SRP-1002 is designed to reduce expression of matrix metalloproteinase 7 (MMP7) as a potential treatment for IPF. The Company is currently investigating SRP-1002 in a Phase 1/2a clinical trial.

SRP-1004 (formerly ARO-ATXN2) - Spinocerebellar Ataxia 2 (SCA2)

SCA2 is a progressive cerebellar ataxia with instability of stance, speech and swallow disorder, pain, spasticity, and ocular signs, caused by gain of function of mutant expanded polyQ ATXN2 protein.

SRP-1004 is designed to reduce the expression of the ATXN2 gene as a potential treatment for spinocerebellar ataxia 2 (SCA2). The Company is currently investigating SRP-1004 in a Phase 1 clinical trial.

SRP-1005 (formerly ARO-HTT) is designed to reduce the expression of the Huntingtin gene as a potential treatment for Huntington's disease. Sarepta is currently investigating SRP-1005 in a Phase 1 clinical trial.

ARO-ATXN1 is designed to reduce the expression of the ATXN1 gene as a potential treatment for spinocerebellar ataxia 1 (SCA1).

ARO-ATXN3 is designed to reduce the expression of the ATXN3 gene as a potential treatment for spinocerebellar ataxia 3 (SCA3).

Takeda Pharmaceuticals U.S.A., Inc.

Fazirsiran (formerly ARO-AAT) is a subcutaneously administered RNAi therapeutic being developed as a treatment for liver disease associated with alpha-1 antitrypsin deficiency (AATD), which is a rare genetic disorder that severely damages the liver and lungs of affected individuals.

AATD is a genetic disorder associated with liver disease in children and adults, and pulmonary disease in adults. AAT is a circulating glycoprotein protease inhibitor that is primarily synthesized and secreted by liver hepatocytes. Its physiologic function is the inhibition of neutrophil protease to protect healthy lung tissues during inflammation and prevent tissue damage. The most common disease variant, the Z mutant, has a single amino acid substitution that results in improper folding of the protein. The mutant protein cannot be effectively secreted and accumulates in globules in the hepatocytes. This triggers continuous hepatocyte injury, leading to fibrosis, cirrhosis, and increased risk of hepatocellular carcinoma.

Individuals with the homozygous PiZZ genotype have severe deficiency of functional AAT leading to pulmonary disease and hepatocyte injury and liver disease. Lung disease in this patient population is frequently treated with AAT augmentation therapy. However, augmentation therapy does nothing to treat liver disease, and there is no specific therapy for hepatic manifestations. There is a significant unmet need as liver transplant, with its attendant morbidity and mortality, is currently the only available treatment.

Fazirsiran is designed to reduce production of the mutant Z-AAT protein by silencing the AAT gene in order to prevent accumulation of Z-AAT in the liver, allow clearance of the accumulated Z-AAT protein, prevent repeated cycles of cellular damage, and possibly prevent or even reverse the progression of liver fibrosis.

The goal of fazirsiran treatment is prevention and potential reversal of Z-AAT accumulation-related liver injury and fibrosis. Reduction of inflammatory Z-AAT protein, which has been clearly defined as the cause of progressive liver disease in AATD patients, is important as it is expected to halt the progression of liver disease and allow fibrotic tissue repair.

Takeda is conducting multiple Phase 3 studies for the treatment of AATD liver disease including the REDWOOD study.

Amgen Inc.

Olpasiran (formerly AMG 890 and ARO-LPA) is a subcutaneously administered RNAi therapeutic designed to reduce production of apolipoprotein A, a key component of lipoprotein(a), which has been genetically linked with increased risk of cardiovascular diseases, independent of cholesterol and LDL levels.

Amgen completed a Phase 2 clinical study evaluating the efficacy, safety, and tolerability of olpasiran in subjects with elevated levels of lipoprotein(a). Amgen reported Phase 2 clinical results at the American Heart Association (AHA) Scientific Sessions in November 2022 and simultaneously published in the New England Journal of Medicine. Amgen began evaluating olpasiran in a Phase 3 study (OCEAN) to assess the impact of olpasiran on major cardiovascular events in participants with atherosclerotic cardiovascular disease and elevated lipoprotein(a), in a double-blind, randomized, placebo-controlled, multi center study in December 2022.

GlaxoSmithKline Intellectual Property (No. 3) Limited ("GSK")

GSK-4532990 (**formerly ARO-HSD**) is designed to reduce production of hydroxysteroid 17-beta dehydrogenase 13 (HSD17B13). Published human genetic data indicate that a loss of function mutation in HSD17B13 provides strong protection against metabolic-dysfunction associated steatohepatitis (MASH) cirrhosis and alcoholic hepatitis and cirrhosis. GSK is conducting Phase 2b clinical trials in patients with MASH and alcohol-related liver disease (ALD).

GSK5637608 (formerly JNJ-3989 and ARO-HBV) is designed to silence all HBV gene products and intervenes upstream of the reverse transcription process where current standard-of-care nucleotide and nucleoside analogues act. GSK initiated a Phase 2 study of daplusiran/tomligisiran followed by bepirovirsen in patients with chronic hepatitis B.

Financial Results

- ***Revenue*** — Generated revenue of $829.4 million in fiscal 2025, $3.6 million in fiscal 2024 and $240.7 million in fiscal 2023;
- ***Net Loss attributable to Arrowhead Pharmaceuticals, Inc.*** — Recorded net loss of $1.6 million in fiscal 2025, $599.5 million in fiscal 2024 and $205.3 million in fiscal 2023;
- ***Net Loss Per Share attributable to Arrowhead Pharmaceuticals, Inc.*** — Recorded net loss per share (basic and diluted) of ($0.01) in fiscal 2025, ($5.00) in fiscal 2024 and ($1.92) in fiscal 2023;
- ***Cash at end of fiscal 2025*** — Cash, cash equivalents, restricted cash and available-for-sale securities totaled $919.4 million at September 30, 2025; and
- ***Total Stockholder Return*** — Achieved a five-year total stockholder return ("**TSR**") at the 48th percentile of our peer group as measured as of September 30th, 2025.

Executive Compensation Highlights

Say-on-Pay Vote and Ongoing Response to Stockholder Feedback

At our 2025 Annual Meeting of Stockholders, 91% of the stockholder votes cast on our non-binding, advisory proposal (the "**Say-on-Pay**" vote) on the executive compensation program were in favor of the program. Our Board of Directors was pleased with this result.

We value the opinions of our stockholders and will continue to consider the outcome of future Say-on-Pay votes, as well as feedback received throughout the year, when making compensation decisions for our executive officers. The Compensation Committee is committed to being responsive to stockholder feedback regarding our executive compensation program, policies, and practices, including concerns expressed through the Say-on-Pay vote.

Stockholder Engagement

Based on our stockholder vote during our 2025 Annual Meeting of Stockholders, we believe stockholders are pleased with our current compensation approach.

Aggressive Performance Fueled by Incentives

The Compensation Committee expects and has observed aggressive performance from the entire executive management team. Our philosophy has been to foster this expectation with reasonably aggressive incentive compensation. Based on our overall operating environment, feedback from our stockholders, and stockholder return results, the Compensation Committee took the following key actions and maintained key policies with respect to the compensation of all of our NEOs for fiscal 2025:

- ***Base Salary —*** Approved base salary increases for our current NEOs based on performance and market adjustments.
- ***Annual Incentive Compensation*** — Certified performance and approved annual cash bonuses for our NEOs for fiscal 2025 in amounts of up to 125% of their target annual cash incentive compensation opportunities, including an annual cash bonus for our CEO in the amount of $1,236,000, equal to 125% of his target annual cash incentive compensation opportunity.
- ***Equity Compensation*** — On the basis of stockholder feedback, the Compensation Committee granted our CEO a combination of time-based RSUs and performance share unit (PRSU) awards. The PRSU awards granted to our CEO for this fiscal year are described more fully below. Our other NEOs were granted long-term incentive compensation opportunities in the form of time-based RSU awards. The RSU awards vest in four equal annual installments beginning in 2026.
- ***Clawback Policy*** — Maintained our clawback policy which allows Arrowhead to recover incentive compensation from our executive officers, on a non-fault basis, in the event a financial restatement is required to correct any accounting errors made by any such executive officer.
- ***Stock Ownership Guidelines*** — Maintained guidelines mandating ownership of Arrowhead stock in amounts equal to, for our CEO, six times annual base salary and, for our CFO and COO, two times annual base salary.
- ***"Double Trigger" Feature for Acceleration of Equity Awards*** — Maintained the agreements for outstanding equity awards granted to our CEO pursuant to our 2013 and 2021 Incentive Plans to provide that, upon a change in control of Arrowhead, the vesting of such awards will accelerate only in the event of a subsequent involuntary termination of employment (i.e., on a "double-trigger" basis).

Pay-for-Performance Analysis

We believe our executive compensation program is reasonable, competitive, and appropriately balances the goals of attracting, motivating, rewarding, and retaining our executive officers with the objective of aligning their interests with those of our stockholders. To ensure this alignment and to motivate and reward individual initiative and effort, a significant portion of our executive officers' target total direct compensation opportunity is both performance-based and "at-risk."

We emphasize performance-based compensation that appropriately rewards our executive officers, including our NEOs, through two separate compensation elements:

- First, we provide the opportunity to participate in our annual incentive compensation plan which provides cash payments if executive officers produce short-term financial, operational, and strategic results that meet or exceed the objectives set forth each year in our annual operating plan.
- In addition, we grant equity awards as long-term incentive compensation. These require substantial performance and are themselves substantial awards, designed to drive our financial and operational performance and long-term growth. In the case of our CEO, these awards are heavily performance weighted, and in the case of our other executive officers, are either dependent on the future appreciation in value of our common stock or are subject to the risk of fluctuations in the value of our common stock and, therefore, are "at risk."

We believe that, ultimately, the creation of sustainable long-term stockholder value will depend on our ability to successfully bring to market the products we develop or our success in partnering with strategic collaborators to bring them to market. Consequently, the Compensation Committee strives to incentivize our executive officers to create that value through the discovery and development of a robust and attractive pipeline of drug candidates and subsequent commercialization of those products if and when approved by regulatory agencies (e.g. Redemplo (plozasiran), which was approved by FDA in November 2025, and by Health Canada and China National Medical Products Administration in January 2026. To achieve that end, our executive compensation program is designed to provide incentives that facilitate these efforts. Particularly for our CEO, the Compensation Committee has awarded a mix of time-based RSUs (approximately 30%) and performance share units (PRSUs) (approximately 70%). The Compensation Committee closely tracks the progress against these objectives and, in conjunction with the independent members of our Board, ensures the objectives are met using sound, ethical business practices before certifying performance achievement of the awards.

To ensure that we remain faithful to our compensation philosophy, the Compensation Committee regularly evaluates the relationship between the reported values of the equity awards granted to our executive officers, the amount of compensation realizable (and, ultimately, realized) from such awards in subsequent years, and our total stockholder return over this period.

Executive Compensation Policies and Practices

We endeavor to maintain sound governance standards consistent with our executive compensation policies and practices. The Compensation Committee evaluates our executive compensation program on a regular basis to ensure that it is consistent with our short-term and long-term goals given the dynamic nature of our business and the market in which we compete for executive talent. The following summarizes our executive compensation and related policies and practices:

WHAT WE DO	WHAT WE DON'T DO
✓ ***Maintain an Independent Compensation Committee.*** The Compensation Committee consists solely of independent directors.	✘ ***No Executive Retirement Plans.*** We do not offer pension arrangements or retirement plans or arrangements to our executive officers that are different from or in addition to those offered to our other employees.
✓ ***Retain an Independent Compensation Advisor.*** The Compensation Committee engaged its own compensation advisor to provide information and analysis with its fiscal 2025 compensation review, and other advice on executive compensation independent of management. This consultant performed no consulting or other services for us in fiscal 2025.	✘ ***No Special Welfare or Health Benefits.*** Our executive officers participate in broad-based Company-sponsored health and welfare benefits programs generally on the same basis as our other full-time, salaried employees.
✓ ***Annual Executive Compensation Review.*** The Compensation Committee conducts an annual review and approval of our compensation strategy, including a review and determination of our compensation peer group and a review of our compensation-related risk profile to ensure that our compensation programs do not encourage excessive or inappropriate risk-taking.	✘ ***No Post-Employment Tax Payment Reimbursement.*** We do not provide any tax reimbursement payments (including "gross-ups") on any severance or change-in-control payments or benefits.
✓ ***Compensation At-Risk.*** Our executive compensation program is designed so that a significant portion of compensation is "at risk" based on our performance, as well as short-term cash and long-term equity incentives to align the interests of our executive officers and stockholders.	✘ ***No Hedging and Limit on Pledging of Our Equity Securities.*** We prohibit our employees, executive officers and the non-employee members of our Board from hedging our equity securities. Our board members and executive officers may pledge up to 75% of owned and vested shares with the approval of our Board.
✓ ***CEO Annual Incentive Compensation Cap.*** Our CEO's annual cash incentive compensation opportunity is capped at 200% of his base salary.	✘ ***No Dividends or Dividend Equivalents Payable on Unvested Equity Awards.*** We do not pay dividends or dividend equivalents on unvested RSU awards or PRSU awards.
✓ ***Stock Ownership Policy.*** We maintain a stock ownership policy that requires our CEO and CFO to maintain a minimum ownership level of our common stock.	✘ ***No Stock Option Re-pricing.*** Our employee stock plan does not permit options to purchase shares of our common stock to be repriced to a lower exercise or strike price without the approval of our stockholders.
✓ ***Compensation Recovery ("Clawback") Policy.*** We maintain a clawback policy to allow Arrowhead to recover incentive compensation from our executive officers, on a non-fault basis, in the event a financial restatement is required to correct any accounting errors made by any such executive officer.	
✓ ***Conduct an Annual Stockholder Advisory Vote on NEO Compensation.*** We conduct an annual stockholder advisory vote on the compensation of our NEOs.	
✓ ***Use a Pay-for-Performance Philosophy.*** The majority of our CEO's compensation is directly linked to achievement of milestones to the benefit of all stakeholders; we also structure target total direct compensation opportunities with a significant long-term equity component, thereby making a substantial portion of our CEO's and each additional executive officer's target total direct compensation dependent upon our stock price and/or total stockholder return.	
✓ ***"Double Trigger" Feature for Acceleration of CEO Equity Awards*** — The outstanding equity awards granted to our CEO provide that, upon a change in control of the Company, the vesting of such awards will accelerate only in the event of a subsequent involuntary termination of employment (a "double-trigger" arrangement).	

Executive Compensation Philosophy

Our executive compensation philosophy reflects our two fundamental objectives:

- to attract, motivate and retain a highly skilled team of executives; and
- to align our executive officers' interests with those of our stockholders by rewarding short-term and long-term performance and aligning compensation to increases in stockholder value.

We believe that the compensation of our executive officers should be directly linked to the achievement of specific objectives that are expected to increase stockholder value. In furtherance of this goal, the Compensation Committee has established the following guidelines as a foundation for compensation decisions:

- provide a competitive total compensation package that enables us to attract, retain and motivate highly-qualified executives with the skills and experience required for the achievement of business goals;
- promote the achievement of key strategic and financial performance measures by linking short-term and long-term compensation to the achievement of measurable goals;
- reward significant achievements outside of pre-established goals;
- recognize that pharmaceutical research, development and commercialization require sustained and focused effort over many years, and involve a high degree of risk and therefore balance incentives for short-term and long-term compensation;
- employ external compensation expertise and market data from industry peers to help assure that our compensation policies and practices are consistent with industry practice and meet our goals for our compensation program;
- consider our cash resources and cost of capital to balance cash and equity compensation; and
- align our executives' incentives with the creation of stockholder value.

Executive Compensation Program Design

Our practice is to combine a mixture of compensation elements that balance achievement of our short-term goals with our longer-term performance. Currently, our executive compensation program consists of three principal elements:

- base salary;
- an annual cash incentive compensation opportunity; and
- long-term incentive compensation in the form of equity awards.

We believe that cash compensation in the form of base salary and an annual incentive compensation opportunity provides our executive officers with short-term rewards for success in operations, and that long-term incentive compensation in the form of RSU and PRSU awards align the objectives of our executive officers with those of our stockholders with respect to long-term performance and success.

The Compensation Committee takes into consideration, among other things, our financial and working capital condition when approving performance objectives and making compensation decisions for our executive officers. Since we seek to invest our cash prudently and until November 2025 did not have marketed products, overall target total direct compensation opportunities are weighted more heavily toward long-term incentive compensation in the form of equity awards. Thus, a significant portion of each executive officer's target total direct compensation opportunity is "at risk," and dependent on the increase in the value of our common stock. The Compensation Committee periodically reassesses the appropriate weighting of cash and equity compensation.

In the case of long-term incentive compensation, typically the Compensation Committee designs these awards to vest, or be earned, over a multi-year period, meaning that long-term value creation, contrasted with short-term gain, presents the best opportunity for our executive officers to benefit from their awards.

We do not maintain a specific policy on the percentage allocation between short-term and long-term incentive compensation elements.

Governance of Executive Compensation Program

Role of the Compensation Committee

The Compensation Committee discharges many of the responsibilities of our Board relating to the compensation of our executive officers, including our NEOs, and the non-employee members of our Board. The Compensation Committee has overall responsibility for overseeing our compensation and benefits philosophy and policies generally, overseeing and evaluating the compensation plans, policies and practices applicable to our CEO and our other executive officers, and ensuring that the target total direct compensation opportunities of our executive officers,

including our NEOs, are consistent with our compensation philosophy, policies and objectives.

The Compensation Committee reviews our executive compensation program annually on a calendar year basis, generally in December. The Compensation Committee draws on a number of resources to assist in the evaluation of the various elements of our executive compensation program including, but not limited to, feedback from our stockholders, input from our CEO, the advice of an external compensation consultant (as identified below) retained by the Compensation Committee, information provided in the public filings of industry peers and industry data compiled by AON, which represents a nationally-based assessment of executive compensation widely used within the pharmaceutical and biotechnology industry sectors.

The Compensation Committee relies upon the judgment of its members in making compensation decisions. In addition, the Compensation Committee incorporates its members' judgment in the assessment process to respond to and adjust for the evolving business environment. The members of the Compensation Committee have extensive experience in executive management, as well as compensation practices and policies.

Compensation-Setting Process

The Compensation Committee develops recommendations for the target total direct compensation opportunities for our executive officers, including our NEOs. The Compensation Committee does not use a single method or measure in making its compensation decisions, nor does it ordinarily position compensation levels based upon a specific or target level relative to a compensation peer group or other companies. Nonetheless, the pay practices at other companies are an important factor that the Compensation Committee considers in assessing the reasonableness of compensation and ensuring that our compensation practices are competitive in the marketplace.

Generally, the Compensation Committee evaluates the compensation of our executive officers relative to the median of the competitive market. However, as discussed hereafter, various other factors are taken into consideration in determining our executive officers' compensation and the Compensation Committee does not target compensation at any specific level relative to the competitive market. When reviewing our current executive compensation arrangements and approving each compensation element and the target total direct compensation opportunity for our executive officers, the Compensation Committee considers the following factors:

- Our performance against the financial and operational objectives established by the Compensation Committee and our Board;
- Each individual executive officer's skills, experience and qualifications relative to other similarly-situated executives at the companies in our compensation peer group and in selected broad-based compensation surveys;
- The scope of each executive officer's role compared to other similarly-situated executives at the companies in our compensation peer group and in selected broad-based compensation surveys;
- The performance of each individual executive officer, based on a subjective assessment of his or her contributions to our overall performance, ability to lead his or her business unit or function and work as part of a team, all of which reflect our core values;
- The compensation practices of our compensation peer group and the companies in selected broad-based compensation surveys and the positioning of each executive officer's compensation in a ranking of peer company compensation levels; and
- The recommendations provided by our CEO with respect to the compensation of our other executive officers.

These factors provide the framework for compensation decision-making and final decisions regarding the compensation opportunity for each executive officer. No single factor is determinative in setting pay levels, nor was the impact of any factor on the determination of pay levels quantifiable.

Role of Chief Executive Officer

In discharging its responsibilities, the Compensation Committee works with members of our management, including our CEO. Our management assists the Compensation Committee by providing information on corporate and individual performance, market compensation data and management's perspective on compensation matters. The Compensation Committee solicits and reviews our CEO's recommendations with respect to the compensation levels for individual executive officers other than himself based on his performance evaluation of each executive officer.

The Compensation Committee reviews and discusses these recommendations and proposals with our CEO and considers them as one factor in determining the compensation for our executive officers, including our other NEOs. Our CEO recuses himself from all discussions and recommendations regarding his own compensation.

Role of Compensation Consultant

The Compensation Committee engages an external compensation consultant to assist it by providing information, analysis and other advice relating to our executive compensation program and the decisions resulting from its annual executive compensation review. The Compensation Committee has the final authority to engage and terminate the engagement of any compensation consultant that it retains.

Since October 2018, the Compensation Committee has engaged Compensia as its external compensation consultant. Compensia assisted the Compensation Committee in its review of executive officer and non-employee director compensation practices for fiscal 2025, including the competitiveness of compensation levels, executive compensation design, comparisons with our industry peers, and other technical considerations. Such assistance included:

- Reviewing and updating our compensation peer group;
- Reviewing and analyzing the compensation arrangements for our executive officers, including our NEOs;
- Reviewing and analyzing the compensation arrangements for the non-employee members of our Board;
- Reviewing and updating of the Compensation Discussion and Analysis section of our proxy statement for our 2026 Annual Meeting of Stockholders; and
- Supporting on other ad hoc matters.

The terms of Compensia's engagement include reporting directly to the Compensation Committee and to the Compensation Committee Chair.

In fiscal 2025, Compensia did not provide any services to us other than those described above. The Compensation Committee has evaluated Compensia's independence pursuant to the listing standards of Nasdaq and the relevant SEC rules and has determined that no conflict of interest has arisen as a result of the work performed by Compensia.

Competitive Positioning

Each year, the Compensation Committee directs its external compensation consultant to conduct a comparative study and report on compensation levels and practices relative to industry peers, including a competitive assessment of our executive compensation program as compared to the market data for base salaries, target total cash compensation, long-term incentive compensation and target total direct compensation. Typically, the findings of this study are presented to the Compensation Committee by the compensation consultant in conjunction with the Compensation Committee's annual review of our executive compensation program.

Because the biotechnology sector is dynamic, the comparator group used by the Compensation Committee to assess the competitive positioning of the compensation of our executive officers is updated annually to ensure that peer companies continue to meet the established criteria. For purposes of its review of our executive compensation program in fiscal 2025, the Compensation Committee directed Compensia to update the compensation peer group reflecting the competitive market for executive talent based on the following criteria:

- Publicly-held, U.S. biotechnology/pharmaceutical companies;
- Companies with lead assets that are in late clinical stage (Phase III) through early commercialization stage (less than $1 billion in revenue); and
- Companies with market capitalizations between 0.25x to 4.0x our market capitalization at the time of the peer selection.

The compensation peer group was selected in such a manner that our market capitalization was very near the median for all peer companies. Consideration was also given to the frequency or infrequency with which a company was identified as a peer with other peer companies.

For fiscal 2025, the compensation peer group was generated in the first quarter of fiscal 2025 and consisted of the following companies:

ACADIA Pharmaceuticals, Inc.	Denali Therapeutics
Amicus Therapeutics	Halozyme Therapeutics
AnaptysBio	IDEAYA Biosciences
Apellis Pharmaceuticals	Intellia Therapeutics
Arcus Biosciences	Ionis Pharmaceuticals
BioCryst Pharmaceuticals	Ironwood Pharmaceuticals
Blueprint Medicines	Madrigal Pharmaceuticals
BridgeBio Pharma	Prothena
CRISPR Therapeutics AG	Ultragenyx Pharmaceuticals
Day One Biopharmacueticals	Vir Biotechnology

AnaptysBio, Day One Biopharmaceuticals, IDEAYA Biosciences, and Prothena were added to the compensation peer group and Deciphera Pharmaceuticals, Insmed, Mirati Therapeutics, REGENXBIO, and Sarepta Therapeutics were removed from the compensation peer group due to changes in our business complexity, employee base and market capitalization, as well as mergers, changes to business complexity, employee base and market capitalization among our prior peer group.

The compensation study prepared by Compensia and presented in October 2024 provided an assessment of our compensation practices as compared to industry peers. Compensation levels for our executive officers, in the aggregate, were determined to be within the range of compensation provided to similarly placed executives and consistent with our compensation philosophy.

Individual Compensation Elements

In 2025, the principal elements of our executive compensation program were as follows:

- base salary;
- an annual cash incentive compensation opportunity;
- long-term incentive compensation in the form of equity awards;
- welfare and health benefits; and
- post-employment compensation arrangements.

Base Salary

Base salary represents the fixed portion of the compensation of our executive officers, including our NEOs, and is an important element of compensation intended to attract and retain highly-talented individuals.

The initial base salaries for our executive officers were negotiated on an individual basis at the time of hire. Thereafter, using the competitive market data provided by its external compensation consultant, the Compensation Committee reviews and determines adjustments to the base salaries for each of our executive officers, including our NEOs, as part of its annual executive compensation review. In addition, the base salaries of our executive officers may be adjusted by the Compensation Committee in the event of a promotion or significant change in responsibilities. Generally, the Compensation Committee sets base salaries with reference to the competitive range of the market median of our compensation peer group and applicable executive compensation survey data, as well as its assessment of the factors described in “Governance of Executive Compensation Program — Compensation-Setting Process” above.

The base salaries of our NEOs for fiscal 2025 and fiscal 2024 were as follows:

Named Executive Officer	Fiscal 2025 Base Salary	Fiscal 2024 Base Salary	Percentage Adjustment
Christopher Anzalone President & CEO	$988,800	$960,000	3%
Daniel Apel (1) Chief Financial Officer	$575,000	$—	N/A
Patrick O'Brien Chief Operating Officer	$650,000	$600,000	8%
James Hamilton Chief Medical Officer	$630,000	$582,400	8%
Kenneth Myszkowski (2) Former Chief Financial Officer	$606,000	$582,400	4%
Tracie Oliver (3) Former Chief Commercial Officer	$488,880	$488,880	—%

(1) Mr. Apel joined the Company in April 2025.
(2) Mr. Myszkowski served as the Company's Chief Financial Officer until May 2025, at which time he transitioned to the role of employee advisor until January 31, 2026 in order to assist with the CFO transition with a base salary of $200,000 in such role.
(3) Ms. Oliver served as the Company's Chief Commercial Officer until October 2024.

The actual base salaries paid to our NEOs in fiscal 2025 are set forth in the "Fiscal 2025 Summary Compensation Table" below.

Annual Cash Incentive Compensation

We provide our executive officers, including our NEOs, with the opportunity to earn performance-based annual incentive awards, payable in cash, which are designed to reward them for our overall corporate performance as well as their individual performance. Generally, our executive officers are evaluated each year for eligibility to receive an annual cash incentive compensation opportunity. Through a collaborative planning process involving our Board and management, corporate performance objectives are established at the beginning of each year and evaluated regularly by our Board for their continued relevance to our status.

Target Annual Cash Incentive Award Opportunities

For purposes of the fiscal 2025 performance-based incentive awards, each of our NEOs was assigned a target annual cash incentive award opportunity based upon a percentage of his or her base salary. The target annual cash incentive award opportunities for our executive officers, including our NEOs, were recommended by our CEO (except with respect to his own target annual cash incentive award opportunity) based on each executive officer's accountability, scope of responsibilities, and potential impact on our performance, and approved by the Compensation Committee. The determination of target annual cash incentive award opportunities was also based on the factors described in "Governance of Executive Compensation Program — Compensation-Setting Process" above. Our NEOs' target annual cash incentive award opportunities did not change from fiscal 2024.

The target cash annual incentive award opportunities for our NEOs were as follows:

Named Executive Officer	Fiscal 2025 Target Annual Incentive Award Opportunity (as a percentage of base salary)	Fiscal 2024 Target Annual Incentive Award Opportunity (as a percentage of base salary)
Dr. Anzalone (1)	100%	100%
Mr. Apel (2)	45%	—%
Mr. O'Brien	45%	45%
Dr. Hamilton	45%	45%
Mr. Myszkowski	45%	45%
Ms. Oliver (3)	—%	40%

(1) Dr. Anzalone's annual cash incentive compensation opportunity is capped at 200% of his base salary.
(2) Mr. Apel joined the Company in April 2025.
(3) Ms. Oliver served as the Company's Chief Commercial Officer until October 2024.

Performance Objectives

In determining the amount of the annual cash incentive award for each of our executive officers, including each of our NEOs, the Compensation Committee evaluated the corporate performance objectives that had been established at the beginning of the calendar year (as set forth below) as well as other corporate and individual achievements and performance throughout the year. These performance objectives addressed milestones for our lead products, research and development milestones for our drug pipeline and business development objectives. In December 2025, the Compensation Committee determined our performance against our primary business objectives set for calendar 2025, as described below.

Goal	Achievement Highlights
Corporate/ Business Development - Weight: 35% *Meet certain goals related to business development, capitalization, CFO recruitment, BoD expansion, and utilization of recent capital improvements*	Met Substantial business development transactions leading to significant new capital. Hired new CFO and expanded the board.
Clinical Development - Weight: 35% *Meet certain goals relating to clinical programs.*	Met Achieved 20 in '25 corporate goal. Fully enrolled plozasiran SUMMIT program clinical trials. Initiated phase 3 clinical trials for zodasiran in HoFH. Met other goals related to early stage clinical trials, including trial recruitment or initiation milestones for ARO-INHBE, ARO-ALK7, ARO-MAPT and ARO-RAGE.
Discovery - Weight: 15% *Meet certain goals with regard to progress on our pre-clinical programs.*	Met. Executed on plan to in-source portions of BBB platform manufacturing Nominated at least four new candidates for wholly-owned use.
Commercial - Weight: 15% *Meet certain goals with regard to REDEMPLO's approval and product revenue.*	Did not meet because these goals assumed priority review instead of standard review for REDEMPLO's launch.

Annual Incentive Award Payments

The Compensation Committee determined that the corporate objectives were achieved at 125% and the actual annual cash incentive award payments earned by our incumbent NEOs are set forth in the table below. Except for the annual incentive award for our CEO, these awards were recommended by our CEO and approved by the Compensation Committee based on the overall achievement of our goals, their contributions to the goals and the overall performance of each executive officer during the year. The following table sets forth the target annual cash incentive award opportunities as a percentage of base salary, the achievement level expressed as a percentage of target and the actual award payment made in cash or cash equivalents to each of our NEOs based on their performance in fiscal 2025:

Named Executive Officer	Target Annual Incentive Award Opportunity (as a percentage of base salary)	Achievement of Target Bonus	Actual Annual Incentive Award ($)
Dr. Anzalone	100%	125%	$1,236,000
Mr. Apel (1)	45%	87.5%	$226,406
Mr. O'Brien	45%	125%	$365,638
Dr. Hamilton	45%	125%	$354,627
Mr. Myszkowski (2)	45%	57%	$155,776
Ms. Oliver (3)	40%	—%	$—

(1) Mr. Apel's annual bonus was prorated as he joined the Company in April 2025.
(2) Mr. Myszkowski served as the Company's Chief Financial Officer until May 2025 and received a prorated target bonus.
(3) Ms. Oliver served as the Company's Chief Commercial Officer until October 2024 and did not receive an annual incentive award.

The annual cash incentive award payments made to our NEOs for fiscal 2025 are set forth in the "Fiscal 2025 Summary Compensation Table" below.

Long-Term Incentive Compensation

We view long-term incentive compensation in the form of equity awards as a critical element of our executive compensation program. The realizable value of these equity awards over time bears a direct relationship to our stock price, and, therefore, these awards are an incentive for our executive officers, including our NEOs, to create value for our stockholders. Equity awards also help us retain qualified executive officers in a competitive market.

Long-term incentive compensation opportunities in the form of equity awards are granted to our executive officers by the Compensation Committee. The amount and forms of such equity awards are determined by the Compensation Committee after considering the factors described in "Governance of Executive Compensation Program — Compensation-Setting Process" above.

2025 Long-Term Incentive Awards

Equity Awards for Chief Executive Officer

Our CEO's fiscal 2025 award was designed as a combination of time-based RSUs and performance share unit (PRSU) awards, which were designed to be valued so as to give our CEO compensation equal to approximately the median of our peer group. Specifically, the award consisted of:

- A time-based RSU award of 100,000 RSUs to vest in four equal, annual amounts of 25,000 RSUs;
- PRSU awards subject to the following performance goals:
  - 75,000 shares to vest upon filing of an sNDA for the sHTG indication of plozasiran;
  - 75,000 shares to vest upon the first patient to be screened in a Phase 2 clinical trial for a drug targeting the central nervous system (whether wholly owned or partnered); and
  - 75,000 shares to vest upon the first patient to be screened in a Phase 2 clinical trial for an obesity drug (whether wholly owned or partnered).

In addition to the fiscal 2025 awards described above, the Board determined that it was in the best interest of the Company to extend the CEO's performance awards expiring at the end of calendar year 2024 or beginning of calendar year 2025 for an additional four years. The Compensation Committee discussed the performance of the CEO and considered whether the expiration of the awards would result in equitable and fair treatment of the CEO's performance. After considering various factors including the status of and the progress toward the performance milestones, and in consideration of external market forces during the period beyond the CEO's control, as well as the additional time needed to achieve the milestones, the Compensation Committee determined that it was in the best interest of the Company to have the CEO continue to be incentivized to meet the goals given the substantial progress that had already been made toward their achievement. Although these awards are shown in the compensation table as part of the value of the CEO's long term incentive compensation for Fiscal 2025 in accordance with SEC disclosure rules, the Compensation Committee notes that these awards were fully disclosed as compensation in previous proxy statements and therefore does not deem this to be a core part of his fiscal 2025 compensation.

Results from PRSU Awards Previously Granted

In fiscal 2025, the Compensation Committee certified achievement of the performance milestones relating to the following PRSU awards:

Performance Goal	Achievement	No. of Shares Certified as Vested
Achievement of 15 Arrowhead-Discovered drugs in clinical study or clinical use, inclusive of wholly-owned and out licensed drugs. *Granted 1/2022 and Modified 7/2022*	Certified in July 2025.	49,760 shares pursuant to a PRSU award

RSU Awards for Other Named Executive Officers

The Compensation Committee approved the following aggregate RSU awards for our other NEOs for 2025:

Named Executive Officer	Restricted Stock Unit Awards (number of shares)	Restricted Stock Unit Awards ($)(1)
Mr. Apel (2)	100,000	$1,287,000
Mr. O'Brien	100,000	$1,979,000
Dr. Hamilton	100,000	$1,979,000
Mr. Myszkowski	100,000	$1,979,000
Ms. Oliver (3)	0	$—

(1) This column represents the total grant date fair value, computed in accordance with ASC 718, of RSUs granted during fiscal years 2025. The assumptions used to calculate the value of the stock underlying the RSU awards are set forth in Note 9 of the Notes to the Consolidated Financial Statements included with the Company's Annual Report on Form 10-K.
(2) Mr. Apel was granted RSU awards in April 2025 when he joined the Company.
(3) Ms. Oliver served as the Company's Chief Commercial Officer until October 2024 and did not receive restricted stock unit awards in fiscal 2025.

RSUs granted to our NEOs in fiscal 2025 vest over four years in equal annual installments, subject to the NEO's continued employment on each applicable vesting date.

The equity awards granted to our NEOs in fiscal 2025 are set forth in the "Fiscal 2025 Summary Compensation Table" and the "Fiscal 2025 Grants of Plan-Based Awards Table" below.

Welfare and Health Benefits

Our executive officers, including our NEOs, are eligible to participate in all of our employee benefit plans, including medical, dental, vision, life and disability insurance, in each case on the same basis as our other employees, subject to applicable law. In addition, we provide an additional life insurance benefit to our CEO for the benefit of his heirs. We also provide vacation and other paid holidays to all our employees, including our executive officers, all of which we believe to be comparable to those provided the companies in our compensation peer group. These benefit programs are designed to enable us to attract and retain our workforce in a competitive marketplace. Our health, welfare and vacation benefits are designed to ensure that we have a productive and focused workforce through reliable and competitive health and other benefits.

Our retirement savings plan ("401(k) plan") is a tax-qualified retirement savings plan, pursuant to which qualified employees, including our NEOs, are able to contribute certain amounts of their annual compensation, subject to limits prescribed by the Internal Revenue Service. Historically, we have made matching contributions of 100% of the first 3% of base salary and of 50% of the next 2% of base salary contributed to the plan. The value of these benefits for each of our NEOs is reflected in the "All Other Compensation" column of the "Fiscal 2025 Summary Compensation Table" below.

Perquisites and Other Personal Benefits

Currently, we do not view perquisites or other personal benefits as a significant component of our executive compensation program. Accordingly, we do not provide significant perquisites or other personal benefits to our executive officers, including our NEOs, except as generally made available to our employees, or in situations where we believe it is appropriate to assist an individual in the performance of his or her duties, to make our executive officers more efficient and effective and for recruitment and retention purposes.

In the future, we may provide perquisites or other personal benefits in limited circumstances, such as those described in the preceding paragraph. All future practices with respect to perquisites or other personal benefits will be approved and subject to periodic review by the Compensation Committee.

Employment Arrangements

We have entered into a written employment agreement with our CEO and have written employment offer letters with our other executive officers. In filling each of our executive positions, we recognized the need to develop competitive compensation packages to attract qualified candidates in a dynamic labor market. At the same time, in formulating these compensation packages, we were sensitive to the need to integrate new executive officers into the executive compensation structure that we were seeking to develop, balancing both competitive and internal equity considerations. Each of these arrangements provides for "at will" employment.

For detailed descriptions of the employment arrangements we maintained with our NEOs during fiscal 2025, see "Termination Benefits — Potential Payments Upon Termination or Change in Control" below.

Post-Employment Compensation Arrangements

We have entered into Severance and Change of Control Agreements with our CEO, CFO, CMO and COO that provide for certain payments and benefits in the event of certain involuntary terminations of employment, including in connection with a Change of Control. We believe that it is necessary to provide each of these executive officers with certain benefits upon termination of their employment, which benefits are intended to provide the executive officers with financial security and provide sufficient income and, in the case of a Change of Control, encouragement for them to remain employed by the Company through the Change of Control process. These agreements are designed to provide reasonable compensation to these executive officers if they were to leave our employ under certain circumstances to facilitate their transition to new employment. We seek to mitigate any potential employer liability and avoid future disputes or litigation by requiring a departing executive officer to sign a separation and release agreement acceptable to us as a condition to receiving such post-employment compensation payments or benefits.

The Compensation Committee does not consider the specific amounts payable under these agreements when establishing annual compensation. We do believe, however, that these arrangements are necessary to offer compensation packages that are competitive.

In addition, our 2013 Incentive Plan and our 2021 Incentive Plan each provides for the acceleration of vesting of outstanding and unvested equity awards in the event of a change in control of the Company, as defined in the plans, except as otherwise determined by our Board. However, the agreements for equity awards granted to our CEO pursuant to our 2013 Incentive Plan and our 2021 Incentive Plan provide that, upon a change in control of the Company, the vesting of such awards will accelerate only in the event of a subsequent involuntary termination of employment (a "double-trigger" arrangement).

For detailed descriptions of the post-employment compensation arrangements we maintained with our NEOs during fiscal 2025, as well as an estimate of the potential payments and benefits payable under these arrangements, see "Termination Benefits — Potential Payments Upon Termination or Change in Control" below.

Other Compensation Policies and Practices

Equity Awards Grant Policy

We do not have any program, plan, or obligation that requires us to grant equity awards on specified dates, although historically we have granted such awards to our existing executive officers and employees at least annually and to newly-hired employees upon the commencement of their employment. We do not have any program, plan or practice to grant equity awards of our common stock to our executive officers in coordination with the release of material

nonpublic information and during fiscal 2025, the Compensation Committee did not time the disclosure of material nonpublic information for the purpose of affecting the value of executive compensation. Equity awards may occasionally be granted following a significant change in job responsibilities or to meet other special retention or performance objectives.

Authority to grant equity awards to our employees rests with the Compensation Committee, although the Compensation Committee has delegated authority to our CEO to grant equity awards to non-executive employees within prescribed limits set by the Compensation Committee. With respect to our executive officers, except for our CEO, recommendations for equity awards are made by our CEO and reviewed and approved by the Compensation Committee.

Under the terms of our 2021 Incentive Plan and the Inducement Plan, pursuant to which new equity awards are granted, the exercise price of any option to purchase shares of our common stock awarded under the plans must be equal to at least 100% of the fair market value of our common stock (which is determined based on the closing sales price of our common stock on the Nasdaq Global Market) on the date of grant.

Stock Ownership Policy

We maintain a stock ownership policy for our CEO, CFO and COO to further align their respective interests with the interests of our stockholders, and to further promote our commitment to sound corporate governance. This policy requires our CEO to own a minimum number of shares of our common stock equal to a value of six times his annualized base salary and our CFO and COO to own a minimum number of shares of our common stock equal to a value of twice their annualized base salaries. Our CEO, CFO and COO have each achieved the respective required ownership level.

Compensation Recovery ("Clawback") Policy

In November 2023, we updated our compensation recovery ("Clawback") policy, in accordance with the Nasdaq Marketplace Rules. Under the updated policy, in the event the Company is required to prepare an accounting restatement of the Company's financial statements due to material non-compliance with any financial reporting requirement under the federal securities laws, the Company will recover, on a reasonably prompt basis, the excess incentive-based compensation received by any covered executive, including each of the NEOs, during the prior three fiscal years that exceeds the amount that the executive otherwise would have received had the incentive-based compensation been determined based on the restated financial statements.

Additionally, our 2013 Incentive Plan and our 2021 Incentive Plan each provides for the recovery of awards made under the plan in accordance with any applicable compensation recovery or recoupment policy, including as required by law, regulation or national securities exchange rule.

Insider Trading Policy

Our insider trading policy governs the purchase, sale and other transactions in our securities and the securities of related companies by our directors, officers and employees, and other covered persons, as well as the Company itself, and is designed to promote compliance with insider trading laws, rules and regulations, and Nasdaq Marketplace Rules, as applicable. As part of this policy, we prohibit our directors, officers, and employees, and other covered persons, from engaging in (a) short-term trading; (b) short sales; (c) options trading; (d) trading on margin; (e) pledging our common stock as collateral (except as noted below); and (f) all hedging transactions with respect to our securities. Subject to approval of our Board, directors and executive officers may pledge up to 75% owned and vested stock as collateral for a loan.

Tax and Accounting Considerations

Deductibility of Executive Compensation

Section 162(m) of the Internal Revenue Code limits the federal income tax deductibility of certain compensation amounts in excess of $1 million paid to certain executive officers. While the Compensation Committee generally seeks to pay compensation that is tax-deductible, it reserves the right to pay non-deductible compensation to the extent it deems appropriate.

Accounting for Stock-Based Compensation

We follow the Financial Accounting Standard Board's Accounting Standards Codification Topic 718 ("FASB ASC Topic 718") for our stock-based compensation awards. FASB ASC Topic 718 requires us to measure the compensation expense for all share-based payment awards made to our employees and non-employee members of our Board, including options to purchase shares of our common stock and other stock awards, based on the grant date "fair value" of these awards. This calculation is performed for accounting purposes and reported in the executive compensation tables required by the federal securities laws, even though the recipient of the awards may never realize any value from their awards.

Compensation Risk Assessment

In reviewing our various compensation programs, the Compensation Committee considers how our compensation policies and practices may affect our risk profile and whether such policies and practices may encourage undue risk-taking by our employees. More specifically, the Compensation Committee considers the general design philosophy of our policies and practices for our employees whose conduct would be most affected by incentives established pursuant to these compensation policies. In considering these issues, the Compensation Committee concluded that the use of a performance-based annual incentive compensation plan and long-term incentive compensation opportunities in the form of equity awards did not appear to create undue risks for us or encourage excessive risk-taking behavior on the part of our NEOs.

With respect to the annual incentive awards for our executive officers, the amount of an individual's award depends principally on overall Company performance, as determined by the Compensation Committee, which reduces the ability and incentive for an individual to take undue risks at the expense of our performance in an effort to increase the amount of his or her annual incentive award. Our performance objectives are reviewed regularly by the Compensation Committee and our Board and are considered to be generally of the nature that promote the steady progression of our development programs and would not encourage or reward excessive risk-taking. In addition, our Board has the ability to intervene in instances where actions by our executive officers vis-à-vis Company performance objective attainment would be considered unduly risky to prevent or penalize such actions.

Compensation Committee Report

The Compensation Committee of the Company has reviewed and discussed with management the Compensation Discussion and Analysis required by Item 402(b) of Regulation S-K. Based on this review and discussion, the Compensation Committee recommended to our Board that the foregoing Compensation Discussion and Analysis be included in this Proxy Statement and incorporated by reference in the Company's Annual Report on Form 10-K for the fiscal year ended September 30, 2025.

Submitted by the Compensation
Committee of the Board of Directors

Michael Perry, Committee Chair
Hongbo Lu
William Waddill

Compensation Committee Interlocks and Insider Participation

During fiscal year 2025, Dr. Perry, Mr. Waddill, and Dr. Lu served on the Compensation Committee. During fiscal year 2025 and through December 2025, there were no compensation committee interlocks between the Company and other entities involving the Company's executive officers and directors. For information regarding a transaction involving the brother of Dr. Douglass Given, the Company's former Director and Chairman of the Board, which is required to be disclosed under Item 404 of Regulation S-K, see "Certain Relationships and Related Transactions, and Director Independence" below.

Fiscal 2025 Summary Compensation Table

The following table summarizes compensation earned for services rendered during fiscal 2025, 2024, and 2023 by our Chief Executive Officer, our Chief Financial Officer, our Chief Operating Officer, our Chief Medical Officer, and our former Chief Financial Officer and former Chief Commercial Officer, collectively our "Named Executive Officers":

Name and Principal Position	Year	Salary ($)	Bonus ($)	Stock Awards (1) ($)		Non-Equity Incentive Plan Compensation (2) ($)	All Other Compensation (3) ($)	Total ($)
Christopher Anzalone	2025	981,046		6,810,292	(4)	1,236,000	2,523	9,029,861
President and Chief Executive Officer	2024	951,012		10,319,000	(5)	1,152,000	2,406	12,424,418
	2023	902,522		8,314,056	(6)	700,000	1,515	9,918,093
Daniel Apel (7)	2025	232,212	200,000	1,287,000		226,406	9,294	1,754,912
Chief Financial Officer								
Patrick O'Brien (8)	2025	653,940		1,979,000		365,638	31,617	3,030,195
Chief Operating Officer	2024	605,260		2,935,050		378,000	31,261	3,949,571
	2023	568,423		2,475,200		252,000	30,715	3,326,338
James Hamilton	2025	617,512		1,979,000		354,627	15,074	2,966,213
Chief Medical Officer	2024	567,938		2,589,750		314,496	15,261	3,487,445
	2023	511,178		2,284,800		236,250	14,715	3,046,943
Kenneth Myszkowski (9)	2025	496,783		1,979,000		155,776	28,601	2,660,161
Former Chief Financial Officer	2024	592,369		2,589,750		314,496	31,761	3,528,376
	2023	568,129		2,284,800		252,000	30,215	3,135,144
Tracie Oliver (10)	2025	43,193		—		—	—	43,193
Former Chief Commercial Officer	2024	484,065		2,589,750		—	2,965	3,076,780
	2023	647,261		19,040		188,000	19,521	873,822

(1) This column represents the total grant date fair value, computed in accordance with ASC 718, of RSUs (including those subject to the achievement of performance goals) granted during fiscal years 2025, 2024 and 2023. The assumptions used to calculate the value of the stock underlying the RSU awards are set forth in Note 9 of the Notes to the Consolidated Financial Statements included with the Company's Annual Report on Form 10-K.

(2) These bonus amounts represent the amounts earned for performance under the Company's Annual Bonus Incentive Plan during calendar years 2025, 2024 and 2023 and paid in fiscal years 2026, 2025 and 2024, respectively. The Annual Bonuses are described in more detail in the "Bonus Incentive" section.

(3) Amounts consist of 401(k) matching contribution, as well as life insurance and disability premiums for the benefit of each executive officer as well as tax and financial advisory services for certain officers.

(4) The amount reported for Christopher Anzalone in the Stock Awards column includes the grant date fair value of three fiscal 2025 RSU awards that are subject to vesting upon the achievement of specific performance conditions. We determined the performance conditions were 100% probable of being achieved as of the grant date, as defined under applicable accounting guidance, and assigned a grant date fair value of $4,581,000 based on this evaluation. The amount reported in the Summary Compensation Table for this

award may not represent the amount that Christopher Anzalone will realize from the award. Whether, and to what extent, an NEO realizes the value will depend on our actual operating performance, stock price fluctuations and the NEO's continued employment.

(5) The amount reported for Christopher Anzalone in the Stock Awards column includes the grant date fair value of a fiscal 2024 RSU award that is subject to vesting upon the achievement of a specific performance condition. We determined the performance condition was 100% probable of being achieved as of the grant date, as defined under applicable accounting guidance, and assigned a grant date fair value of $10,319,000 based on this evaluation. The amount reported in the Summary Compensation Table for this award may not represent the amount that Christopher Anzalone will realize from the award. Whether, and to what extent, an NEO realizes the value will depend on our actual operating performance, stock price fluctuations and the NEO's continued employment.

(6) The amount reported for Christopher Anzalone in the Stock Awards column includes the grant date fair value of a fiscal 2023 RSU award that is subject to vesting upon the achievement of specific performance conditions. We determined the performance conditions that were probable and not probable of being achieved as of the grant date, as defined under applicable accounting guidance, and assigned a grant date fair value of $4,625,270 based on this evaluation. If we had determined that as of the date of the grant it was probable that 100% of the performance conditions would be achieved, we would have assigned a grant date fair value of $8,314,056 for the performance-based RSUs. The amount reported in the Summary Compensation Table for this award may not represent the amount that Christopher Anzalone will realize from the award. Whether, and to what extent, an NEO realizes the value will depend on our actual operating performance, stock price fluctuations and the NEO's continued employment.

(7) Mr. Apel joined the Company in April 2025 and received a $200,000 relocation bonus.

(8) Mr. O'Brian received tax and financial advisory services of $16,543 in fiscal year 2025.

(9) Mr. Myszkowski served as the Company's Chief Financial Officer until May 2025, at which time he transitioned to the role of employee advisor until January 31, 2026 in order to assist with the CFO transition with a base salary of $200,000 in such role. The amounts reported in the table above include amounts he received in both roles. Mr. Myszkowski received tax and financial advisory services of $16,543 in fiscal year 2025.

(10) Ms. Oliver served as the Company's Chief Commercial Officer until October 2024.

Fiscal 2025 Grants of Plan Based Awards Table

The following table sets forth cash bonus and equity grants made to the NEOs in fiscal 2025:

Name	Grant Date	Estimated Future Payouts Under Non-Equity Incentive Plan Awards (1)		Estimated Future Payouts Under Equity Incentive Plan Awards (2)	All Other Stock Awards: Number of Shares of Stock or Units (#) (3)	Grant Date Fair Value
		Target ($)	Maximum ($)	Target (#)		
Christopher Anzalone						
Cash Bonus		$988,800	$1,977,600	—	—	—
PRSUs	12/18/2024	—		225,000	—	$4,581,000
RSUs	12/18/2024				100,000	$2,036,000
RSUs	02/07/2025				9,550	$193,292
Daniel Apel						
Cash Bonus		$258,750		—	—	—
RSUs	04/15/2025	—		—	100,000	$1,287,000
Patrick O'Brien						
Cash Bonus		$292,500		—	—	—
RSUs	01/04/2025	—		—	100,000	$1,979,000
James Hamilton						
Cash Bonus		$283,500		—	—	—
RSUs	01/04/2025	—		—	100,000	$1,979,000
Kenneth Myszkowski						
Cash Bonus		$272,700		—	—	—
RSUs	01/04/2025	—		—	100,000	$1,979,000
Tracie Oliver (4)						
Cash Bonus		$—		—	—	—
RSUs	N/A	—		—	—	$—

(1) Amounts listed represent cash award targets for our NEOs in fiscal 2025. Actual payments were made in fiscal 2026 and the amounts are reported in the Summary Compensation Table above. There are no thresholds or maximum levels applicable under our annual cash incentive awards except with respect to Dr. Anzalone, whose target opportunity is capped at 200%.

(2) These PRSUs are described above in the "Compensation Discussion and Analysis" under the heading "Equity Compensation".

(3) RSUs granted in fiscal 2025 vest in four equal annual installments beginning 1 year from the grant date.

(4) Ms. Oliver served as the Company's Chief Commercial Officer until October 2024 and did not receive any awards.

Fiscal 2025 Outstanding Equity Awards at Fiscal Year End Table

The following table provides information, with respect to the NEOs, concerning the outstanding equity awards covering shares of the Company's common stock as of September 30, 2025.

		Option Awards			Stock Awards			
Name	Grant Date	Number of Securities Underlying Unexercised Options (# Exercisable) (1)	Option Exercise Price ($)	Option Expiration Date	Number of Shares or Units of Stock That Have Not Vested (#) (2)	Market Value of Shares or Units of Stock That Have Not Vested ($) (3)	Equity Incentive Plan Awards: Number of Unearned Shares or Units of Stock That Have Not Vested (#) (4)	Equity Incentive Plan Awards: Market Value of Unearned Shares or Units of Stock That Have Not Vested ($) (3)
Christopher Anzalone	01/01/2016	51,726	6.15	1/1/2026	—	—	—	—
	01/01/2020				—	—	700,000	24,143,000
	01/01/2021				—	—	800,000	27,592,000
	07/08/2022				24,881	858,146	49,761	1,716,257
	12/20/2022				46,805	1,614,304	93,610	3,228,609
	12/18/2024				100,000	3,449,000	225,000	7,760,250
Daniel Apel	04/15/2025				100,000	3,449,000	—	—
Patrick O'Brien	01/01/2022				15,000	517,350	—	—
	01/04/2023				32,500	1,120,925	—	—
	01/04/2024				63,750	2,198,738	—	—
	01/04/2025				100,000	3,449,000	—	—
James Hamilton	01/01/2022				13,750	474,238	—	—
	01/04/2023				30,000	1,034,700	—	—
	01/04/2024				56,250	1,940,063	—	—
	01/04/2025				100,000	3,449,000	—	—
Kenneth Myszkowski	01/01/2022				15,000	517,350	—	—
	01/04/2023				30,000	1,034,700	—	—
	01/04/2024				56,250	1,940,063	—	—
	01/04/2025				100,000	3,449,000	—	—
Tracie Oliver	N/A				—	—	—	—

(1) Options are priced at the market closing price on the date of grant. Options have various vesting parameters, but generally vest over 48 months after the award is granted.

(2) RSUs have various vesting parameters but generally vest in four equal annual installments beginning one year from the grant date.
(3) Value is based on our Company's Common Stock closing price of $34.49 on September 30, 2025.
(4) The amounts reported for Christopher Anzalone in this column reflect the January 2020, January 2021, July 2022, December 2022, and December 2024 awards that contain performance-based vesting conditions. These awards and their vesting conditions are described above in the "Compensation Discussion and Analysis" under the heading "Equity Compensation" in this year's proxy statement and in prior proxy statements, as applicable.

Fiscal 2025 Options Exercises and Stock Vested Table

The following table provides information, with respect to the NEOs, concerning options exercised or RSUs or PRSUs vested during fiscal 2025.

	Option Awards		Stock Awards	
Name	Number of Shares Acquired on Exercise	Value Realized on Exercise (1)	Number of Shares Acquired on Vesting	Value Realized on Vesting (2)
Christopher Anzalone	300,000	$ 2,948,360	497,354	$ 8,091,313
Daniel Apel	—	—	—	$ —
Patrick O'Brien	—	—	67,500	$ 1,335,825
James Hamilton	—	—	60,000	$ 1,187,400
Kenneth Myszkowski	—	—	63,750	$ 1,261,613
Tracie Oliver	—	—	—	$ —

(1) Value is calculated as the price of our Common Stock upon exercise, less the exercise price, multiplied by the number of shares exercised.

(2) Value is calculated as the price of our Common Stock upon vesting, multiplied by the number of shares vested.

Termination Benefits — Potential Payments Upon Termination or Change in Control

The Company has entered into Severance and Change of Control Agreements with each of Christopher Anzalone, Daniel Apel, Patrick O'Brien and James Hamilton (the "**Severance Agreements**"). Under the Severance Agreements, upon a termination of the executive's employment by the Company without "**Cause**" (and not as a result of the executive's death or disability) or due to the executive's "**Resignation for Good Reason**" (each quoted term as defined in each Severance Agreement) (such a termination, an "**Involuntary Termination**"), the Company will provide the following severance benefits: (i) payment of accrued but unpaid base salary, accrued but unused vacation, and any annual cash bonus earned but unpaid for the preceding fiscal year; (ii) a lump sum severance payment equal to six months' worth of the executive's base salary (or, for Dr. Anzalone, 12 months' worth of his base salary, plus a pro-rated portion of his target annual cash bonus for the year in which the termination occurs) (the "**Severance Payment**"); and (iii) reimbursement of health care continuation premiums for the 12-month period (or, for Dr. Anzalone, the 18-month period) following the termination date.

If the termination of employment described above occurs within the period beginning three months prior to the signing of a definitive agreement that would result in a "**Change of Control**" (as defined in each Severance Agreement) through the first anniversary of such Change of Control (the "**CIC Period**"), the Company will provide the following severance benefits in lieu of the benefits described above: (i) payment of accrued but unpaid base salary, accrued but unused vacation, and any annual cash bonus earned but unpaid for the preceding fiscal year; (ii) a lump sum severance payment equal to (a) 12 months' worth (or, for Dr. Anzalone, 24 months' worth) of the executive's base salary, plus (b) one and one-half times (or, for Dr. Anzalone, two times) the executive's target annual cash bonus for the year in which the termination occurs (the "**COC Severance Payment**"); provided, however, that if the executive has received a Severance Payment, the COC Severance Payment shall be reduced by the amount of such Severance Payment; (iii) reimbursement of health care continuation premiums for the 18-month period following the termination date; and (iv) accelerated vesting of all outstanding equity awards, with any performance-based vesting criteria deemed achieved at the target level of performance (and, if the termination occurs within the permitted period prior to the Change of Control, a lump sum cash payment equal to the value of any equity awards that would have vested had the executive remained employed through the Change of Control).

The severance benefits provided under the Severance Agreements are subject to each executive's execution and non-revocation of a release of claims in favor of the Company and its affiliates. Further, in the event that any payments or benefits provided under the Severance Agreements or otherwise constitute "**parachute payments**" within the meaning of Section 280G of the Internal Revenue Code of 1986, as amended (the "Code"), the executive will receive the portion of such payments and benefits that results in the greatest after-tax benefit to the executive (including after payment of any excise tax under Section 4999 of the Code).

The Company executed a Retirement Letter with Mr. Ken Myszkowski, dated May 9, 2025, stating that after May 13, 2025, and through January 31, 2026, Mr. Myszkowski will remain an Arrowhead employee, assuming the position of Assistant to the Chief Financial Officer. In that role, Mr. Myszkowski is expected to be reasonably available for consultation at the CFO's request, and to assist the CFO with certain regulatory filings. As Assistant to the CFO, Mr. Myszkowski will be considered a full-time employee and will draw a salary at the annualized rate of $200,000. Pursuant to the Retirement Letter, if Arrowhead were to terminate Mr. Myszkowski's employment without cause prior to January 31, 2026, Mr. Myszkowski would be entitled to a lump sum payment equal to three months of his salary and an amount equal to the cost of three months of healthcare insurance under his post-employment COBRA rights.

Ms. Oliver passed away in November 2024.

Additionally, pursuant to the Inducement Plan, 2013 Incentive Plan and the 2021 Incentive Plan, any unvested awards held by plan participants, including the NEOs, become fully vested upon a change of control of the Company, except as otherwise determined by the Board and except with respect to the outstanding awards held by the CEO whose awards will only become fully vested if he experiences a qualifying termination of employment following a change of control.

The following tables set forth information regarding potential termination and change of control arrangements with our NEOs (other than Ms. Oliver) had their employment been terminated or a change in control of the Company taken place on September 30, 2025:

Termination Payments

Triggering Event	Salary ($)	Bonus ($)	Benefits ($)	Stock Awards (1)($)	Total
Involuntary Termination Outside of the CIC Period					
Christopher Anzalone	988,800	988,800	66,286	—	2,043,886
Daniel Apel	287,500	—	47,383	—	334,883
Patrick O'Brien	325,000	—	49,069	—	374,069
James Hamilton	315,000	—	32,114	—	347,114
Kenneth Myszkowski	50,000	—	12,107	—	62,107
Change in Control					
Christopher Anzalone	—	—	—	—	—
Daniel Apel	—	—	—	3,449,000	3,449,000
Patrick O'Brien	—	—	—	7,286,013	7,286,013
James Hamilton	—	—	—	6,898,000	6,898,000
Kenneth Myszkowski	—	—	—	6,941,113	6,941,113
Involuntary Termination During the CIC Period					
Christopher Anzalone	1,977,600	1,977,600	66,286	71,827,481	75,848,967
Daniel Apel	575,000	388,125	71,075	3,449,000	4,483,200
Patrick O'Brien	650,000	438,750	73,604	7,286,013	8,448,367
James Hamilton	630,000	425,250	48,170	6,898,000	8,001,420
Kenneth Myszkowski	50,000	—	72,643	6,941,113	7,063,756

(1) For stock awards the value is calculated as the number of unvested and unearned shares multiplied by the Company's closing stock price at September 30, 2025 of $34.49, plus any exercisable options exercised at the Company's closing stock price at September 30, 2025 of $34.49. Ms. Oliver did not receive any payments or benefits in connection with her departure from the Company.

CEO Pay Ratio

Pursuant to Item 402(u) of Regulation S-K, we are required to calculate and disclose the median of the annual total compensation of all of our employees (excluding our CEO, Dr. Anzalone), the annual total compensation of Dr. Anzalone, and the ratio of these two amounts.

Based on the fact that we had a significant number of new hires during fiscal 2025, we did not elect to use the same median employee as the prior year. Our median employee was identified using the entire population of our employees as of September 30, 2025 based on a consistently applied compensation measure, or CACM, that reasonably reflects the annual compensation of our employees. The CACM selected by us for our disclosure included annual base salary, the cash bonus amount for fiscal 2025, the grant-date fair value for stock-based awards (calculated in accordance with requirements for the Summary Compensation Table), and welfare and health benefits for fiscal 2025.

Based on the CACM methodology described above, we identified the median employee and calculated the fiscal 2025 compensation for this selected employee in the same manner we determine the annual total compensation of our NEOs for purposes of the Summary Compensation Table. The median of the annual total compensation of all our employees was $163,062. Dr. Anzalone's fiscal 2025 annual total compensation as disclosed in the Fiscal 2025 Summary Compensation Table was $9,029,861. As a result, our CEO to median employee pay ratio for fiscal 2025 is 55:1.

This pay ratio is a reasonable estimate calculated by a method consistent with the SEC requirements, described above, based on our payroll and employment records. As a result of a variety of factors, including employee populations, potential differences in the components used for the CACM, compensation philosophies and certain assumptions, pay ratios reported by other companies may not be comparable to our pay ratio. The pay ratio is not utilized by our management or our compensation committee for compensation-related decisions.

Pay Versus Performance

As required by Section 953(a) of the Dodd-Frank Wall Street Reform and Consumer Protection Act, and Item 402(v) of Regulation S-K, we are providing the following information about the relationship between executive "compensation actually paid" and certain financial performance of the Company. This information has been prepared in accordance with Item 402(v) and does not necessarily reflect the actual amount of compensation earned by or paid to our named executive officers ("NEOs") for the applicable year. Please refer to the Compensation Discussion and Analysis section of this proxy statement for a discussion of our executive compensation program objectives and the ways in which we align executive compensation with performance.

Year (a)	Summary Compensation Table Total for PEO (1)(b)	Compensation Actually Paid to PEO (2)(c)	Average Summary Compensation Table Total for Non-PEO NEOs (1)(d)	Average Compensation Actually Paid to Non-PEO NEOs (2) (e)	Value of Initial Fixed $100 Investment Based On: Total Shareholder Return (3)(f)	Peer Group Total Shareholder Return (g)	Net Loss (4) (h) (in thousands)	Company Selected Measure (5)(i) (in thousands)
2025	$9,029,861	$42,599,705	$2,090,935	$4,674,785	$80	$146	($1,631)	$ —
2024	$12,424,418	($5,357,049)	$3,499,747	$1,787,414	$31	$94	($599,493)	$ —
2023	$9,918,093	($2,171,064)	$3,151,102	$1,873,139	$62	$49	($205,275)	$ —
2022	$12,031,969	($40,073,641)	$4,133,800	($386,119)	$77	$67	($176,063)	$ —
2021	$24,703,855	$69,061,855	$3,324,746	$6,080,785	$145	$116	($140,848)	$ —

(1) The dollar amounts reported are the amounts reported for Christopher Anzalone (the Company's Chief Executive Officer or Principal Executive Officer (PEO)) for each of the corresponding years in the "Total" column in our Summary Compensation Table. The dollar amounts reported in column (d) represent the average of the amounts reported for the Company's named executive officers (NEOs) as a group (excluding Christopher Anzalone) in the "Total" column of the Summary Compensation Table in each applicable year. The names of each of the NEOs included for these purposes in each applicable year are as follows: (i) for fiscal year 2025, Daniel Apel, Kenneth Myszkowski, Patrick O'Brien, James Hamilton and Tracie Oliver, (ii) for fiscal year 2024, Kenneth Myszkowski, Patrick O'Brien, James Hamilton, Javier San Martin and Tracie Oliver, (iii) for fiscal year 2023, Kenneth Myszkowski, Patrick O'Brien, James Hamilton, and Javier San Martin; (iv) for fiscal year 2022, Kenneth Myszkowski, Patrick O'Brien, James Hamilton, and Tracie Oliver; and (v) for fiscal year 2021, Kenneth Myszkowski, Patrick O'Brien, James Hamilton, Javier San Martin, and James Hassard.

(2) The dollar amounts reported in column (c) represent the amount of "compensation actually paid" to Christopher Anzalone, and the dollar amounts reported in column (e) represent the average amount of "compensation actually paid" to our other NEOs as a group, each as computed in accordance with Item 402(v) of Regulation S-K and do not reflect the total compensation actually realized or received by Christopher Anzalone or the other NEOs on average, as applicable. In accordance with these rules, these amounts reflect "Total Compensation" as set forth in the Summary Compensation Table for each year, adjusted as shown below. Equity values are calculated in accordance with FASB ASC Topic 718, and the valuation assumptions used to calculate fair values did not materially differ from those disclosed at the time of grant.

Compensation Actually Paid	2025		2024		2023		2022		2021	
	PEO	Other NEOs*	PEO	Other NEOs*	PEO	Other NEOs*	PEO	Other NEOs*	PEO	Other NEOs*
Summary Compensation Table Total	$ 9,029,861	$ 2,090,935	$ 12,424,418	$ 3,499,747	$ 9,918,093	$ 3,151,102	$ 12,031,969	$ 4,133,800	$ 24,703,855	$ 3,324,746
Less, value of "Stock Awards" and "Option Awards" reported in Summary Compensation Table	$ (6,810,292)	$ (1,444,800)	$ (10,319,000)	$ (2,658,810)	$ (8,314,056)	$ (2,332,400)	$ (10,382,549)	$ (3,529,925)	$ (23,019,000)	$ (2,608,820)
Plus, year-end fair value of outstanding and unvested equity awards granted in the year	$ 11,209,250	$ 2,759,200	$ 6,585,800	$ 1,200,940	$ 6,288,252	$ 1,645,788	$ 8,222,939	$ 1,578,138	$ 49,944,000	$ 3,537,700
Plus, fair value as of vesting date of equity awards granted and vested in the year	$ 193,292	$ —	$ —	$ —	$ 583,774	$ —	$ —	$ —	$ —	$ —
Plus (less), year over year change in fair value of outstanding and unvested equity awards granted in prior years	$ 25,931,662	$ 945,000	$ (13,971,458)	$ (456,188)	$ (10,653,844)	$ (847,819)	$ (49,946,000)	$ (2,754,375)	$ 17,433,000	$ 2,015,691
Plus (less), change in fair value from last day of prior fiscal year to vesting date for equity awards granted in prior years that vested in the year	$ 3,045,932	$ 324,450	$ (76,809)	$ 201,724	$ 6,718	$ 256,469	$ —	$ 186,244	$ —	$ (188,531)
Compensation Actually Paid	$ 42,599,705	$ 4,674,785	$ (5,357,049)	$ 1,787,414	$ (2,171,064)	$ 1,873,139	$ (40,073,641)	$ (386,119)	$ 69,061,855	$ 6,080,785

* Amounts presented are averages for the entire group of Other NEOs in each respective year.

(3) Total Shareholder Return (TSR) is calculated by dividing (a) the sum of (i) the cumulative amount of dividends for the measurement period, assuming dividend reinvestment, and (ii) the difference between the Company's share price at the end of each fiscal year shown and the beginning of the measurement period, by (b) the Company's share price at the beginning of the measurement period. The beginning of the measurement period for each year in the table is September 30, 2020.

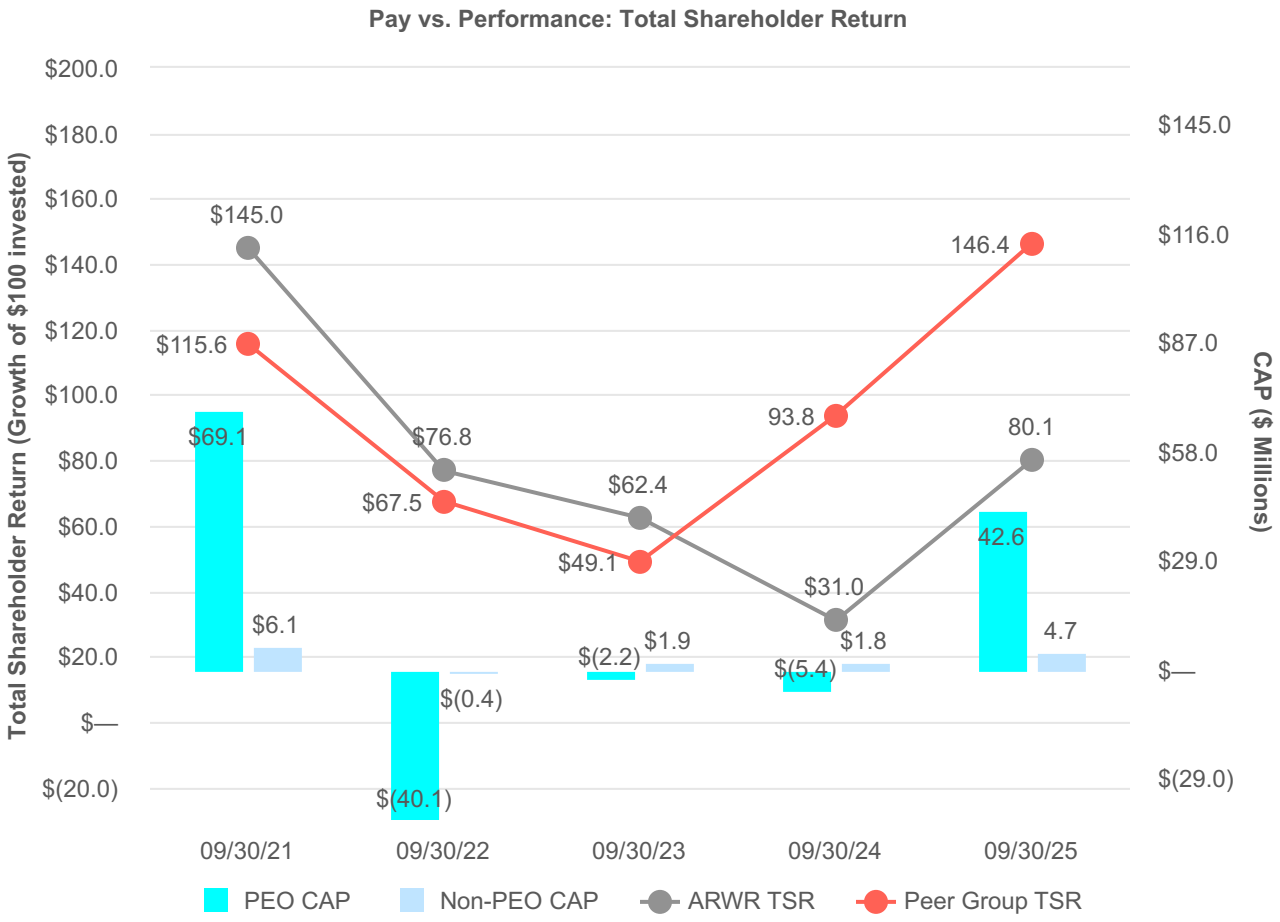
(4) The dollar amounts reported represent the amount of net income reflected in the Company's audited financial statements for the applicable year.

(5) The Company does not use any financial performance measures to link executive compensation actually paid to company performance. Consequently, no "Company Selected Measure" is included in the table above.

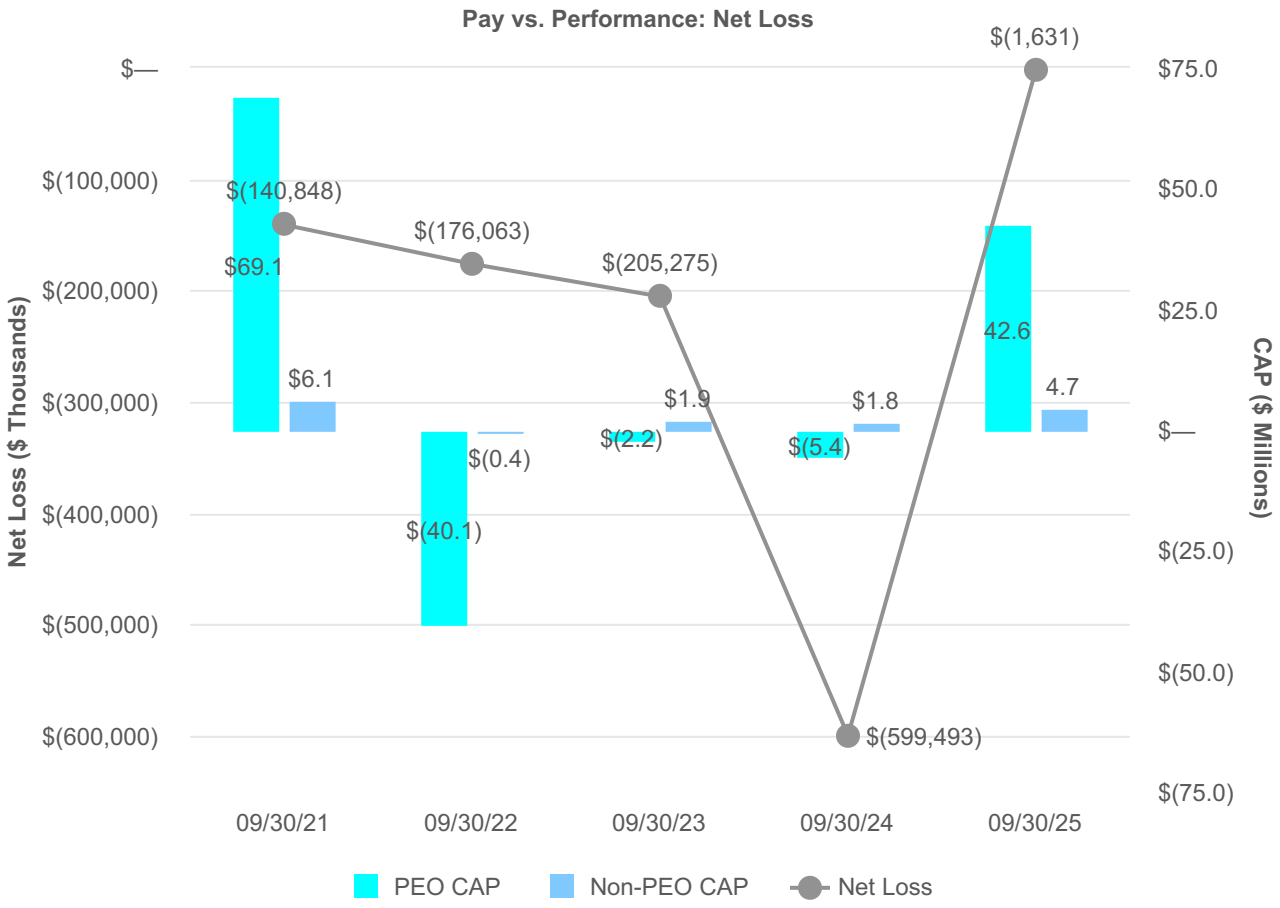
Analysis of Information Presented in the Pay versus Performance Table

As described in more detail in the Compensation Discussion and Analysis section, the Company's executive compensation program reflects a variable pay-for-performance philosophy. While the Company utilizes several performance measures to align executive compensation with Company performance, all of those Company measures are not presented in the Pay versus Performance table. Moreover, the Company generally seeks to incentivize long-term performance, and therefore does not specifically align the Company's performance measures with compensation that is actually paid (as computed in accordance with SEC rules) for a particular year. In accordance with SEC rules, the Company is providing the following descriptions of the relationships between information presented in the Pay versus Performance table.

Compensation Actually Paid, Cumulative TSR and Peer Group TSR



Compensation Actually Paid and Net Loss



Financial Performance Measures

As described in greater detail in the Compensation Discussion and Analysis section, the Company's executive compensation program reflects a variable pay-for-performance philosophy. The metrics that the Company uses for both our long-term and short-term incentive awards are selected based on an objective of incentivizing our NEOs to increase the value of our enterprise for our shareholders. The Company does not currently use any financial performance measures to link executive compensation actually paid to our performance. However, the most important performance measures used by the Company to link executive compensation actually paid to the Company's NEOs, for the most recently completed fiscal year, to the Company's performance are as follows:

- Corporate goals related to meeting certain objectives with respect to business development, accessing new capital, market capitalization, commercialization, and and utilization of recent capital improvements;
- Discovery and early development goals related to meeting certain goals with regard to progress on our pre-clinical programs; and
- Clinical development goals involving meeting certain goals relating to our clinical programs;

All information provided above under the "Item 402(v) Pay Versus Performance" heading will not be deemed to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date hereof and irrespective of any general incorporation language in any such filing, except to the extent the Company specifically incorporates such information by reference.

Proposal Four — Ratification of Selection of Independent Auditors

Our Audit Committee, with the ratification of our Board, selected the accounting firm of KPMG LLP ("**KPMG**") as the Company's independent auditors for the fiscal year ending September 30, 2026, and that selection is now being submitted to the stockholders for their ratification.

A representative of KPMG is expected to be present at the Annual Meeting and available to respond to appropriate stockholder questions. The representative will also have an opportunity to make any other statements such representative deems appropriate.

Stockholders are not required to ratify the selection of KPMG as our independent auditors. However, we are submitting the selection for ratification as a matter of good corporate practice. If stockholders fail to ratify the selection, the Audit Committee will consider whether or not to retain KPMG. Even if the selection is ratified, the Audit Committee may direct the selection of different independent auditors at any time during the year if it determines that such a change would be in the best interests of the Company and our stockholders.

Vote Required; Recommendation of the Board

In order to be ratified, Proposal Four must be approved by the Required Vote, assuming a quorum is present. For this purpose, abstentions will be counted as a vote "AGAINST" the Proposal, while broker non-votes, if any, will have no effect on the outcome of the vote.

THE BOARD UNANIMOUSLY RECOMMENDS A VOTE "FOR" PROPOSAL FOUR.

Audit Fees

The Audit Committee regularly reviews and determines whether specific projects or expenditures with our independent auditors may potentially affect their independence. The Audit Committee's policy is to pre-approve all audit and permissible non-audit services provided by our independent auditors. Pre-approval is generally provided by the Audit Committee for up to one year, detailed to the particular service or category of services to be rendered and is generally subject to a specific budget. The Audit Committee may also pre-approve additional services of specific engagements on a case-by-case basis. All engagements of our independent registered public accounting firm in 2025 and 2024 were pre-approved by the audit committee.

The following table sets forth the aggregate audit fees and fees for all other services provided during the indicated fiscal years. These include amounts billed and expected to be billed by KPMG for fiscal year 2025 and for fiscal year 2024:

	Year Ended September 30,	
	2025	2024
Audit fees (1)	$1,310,425	$1,010,000
Audit-related fees	—	—
Tax Fees	—	—
All other fees	—	—
Total	$1,310,425	$1,010,000

(1) Fees invoiced by KPMG include year-end audit, periodic reviews of Forms 10-Q and 10-K, comfort letters and consents for financings and registration statements, and other agreed-upon procedures.

Report of the Audit Committee

The following is the report of the Audit Committee with respect to the Company's audited financial statements for fiscal 2025, which include the consolidated balance sheets of the Company as of September 30, 2025 and September 30, 2024, and the related consolidated statements of operations, stockholders' equity and cash flows for the fiscal years ended September 30, 2025, September 30, 2024 and September 30, 2023, and the notes thereto.

Review with management and independent registered public accounting firm. The Audit Committee met separately to review the Company's consolidated audited financial statements and held discussions with management and KPMG. Management represented to the Audit Committee that the Company's consolidated financial statements were prepared in accordance with generally accepted accounting principles. The members of the Audit Committee discussed with KPMG matters required to be discussed under the applicable standards of the Public Company Accounting Oversight Board ("PCAOB") and the SEC. The Company's independent registered public accounting firm also provided to the Audit Committee the written disclosures and the letter required by the applicable requirements of the PCAOB regarding the independent registered public accounting firm's communications with the Audit Committee concerning independence and the Audit Committee discussed the firm's independence with KPMG.

Conclusion. Based upon the Audit Committee's review of the financial statements and discussions with management and KPMG, the Audit Committee's review of the representations of management and the report of KPMG to the Audit Committee, the Audit Committee recommended that the Board include the audited consolidated financial statements in the Company's Annual Report on Form 10-K for the fiscal year ended September 30, 2025, as filed with the SEC.

This report is submitted by the Audit Committee of the Board.

William Waddill, Committee Chair
Mauro Ferrari
Mike Perry
Victoria Vakiener

Security Ownership of Certain Beneficial Owners and Management

The following table sets forth certain information regarding the ownership of the Company's Common Stock as of January 15, 2026 (except as otherwise indicated in the footnotes to the table), by (i) each of our NEOs, (ii) each director and director nominee, (iii) all current directors and executive officers as a group, and (iv) the holders of greater than 5% of our total shares outstanding known to us.

Beneficial ownership is determined in accordance with the rules of the SEC, and thus represents voting or investment power with respect to our securities. Under such rules, beneficial ownership includes any shares over which the individual has sole or shared voting power or investment power as well as any shares that the individual has the right to acquire within 60 days after the date of this table. Unless otherwise specified in the footnotes to the table below, to our knowledge, (i) the persons and entities named in the table have sole voting and investment power with respect to all shares beneficially owned, subject to community property laws, where applicable, and (ii) the address of each stockholder is c/o Arrowhead Pharmaceuticals, Inc., 177 E. Colorado Blvd., Suite 700, Pasadena, CA 91105.

	Number and Percentage of Shares Beneficially Owned (1)	
	Shares	Percentage
5% Beneficial Owners		
BlackRock Inc (2) 50 Hudson Yards, New York, NY 10001	19,012,855	13.1%
The Vanguard Group (3) 100 Vanguard Blvd., Malvern, PA 19355	15,525,072	10.7%
Entities affiliated with Avoro Capital Advisors LLC (4) 110 Greene Street, Suite 800, New York, NY 10012	13,985,269	9.7%
Named Executive Officers and Directors		
Christopher Anzalone (5)	3,792,739	2.6%
Patrick O'Brien	547,408	*
James Hamilton	246,958	*
Daniel Apel	176,200	*
Michael S. Perry (6)	127,652	*
Mauro Ferrari (6)	73,646	*
William Waddill (6)	61,156	*
Hongbo Lu (7)	63,009	*
Douglas Ingram (8)	59,016	
Victoria Vakiener (6)	40,316	*
Adeoye Olukotun (6)	38,193	*
All current Executive Officers and Directors as a group (11 persons) (9)	5,226,293	3.6%

* Less than 1%

(1) Based on 142,262,493 shares of Common Stock issued and outstanding as of January 15, 2026. Shares not outstanding but deemed beneficially owned by virtue of the right of a person to acquire them as of January 15, 2026, or within sixty days of such date are treated as outstanding only when determining the percentage owned by such individual and when determining the percentage owned by the group.

(2) Based on Amendment No. 3 to Schedule 13G filed April 23, 2025. According to Amendment No. 3, BlackRock Inc. has sole voting power and sole dispositive power over 18,877,477 shares and 19,012,855 shares, respectively.

(3) Based on Amendment No. 8 to Schedule 13G filed July 29, 2025. According to Amendment No. 8, The Vanguard Group has sole dispositive power over 15,243,118 shares and has shared voting power and shared dispositive power over 142,962 shares and 281,954 shares, respectively.

(4) Based on Amendment No. 2 to Schedule 13G filed August 14, 2025. Includes 917,441 shares of Common Stock issuable upon the exercise of warrants. According to Amendment No. 2, Avoro Capital Advisors LLC and Behzad Aghazadeh, who serves as

the portfolio manager and controlling person of Avoro Capital Advisors LLC, have sole voting power and sole dispositive power over 11,517,441 shares. Subsequent to the Amendment No. 2 to Schedule 13G filing, Avoro Capital Advisors LLC received additional warrants that can be exercised to receive 1,550,387 shares of Common Stock.

(5) Chris Anzalone has pledged 1,192,897 shares of Common Stock as collateral for a line of credit.

(6) Includes 4,593 shares of Common Stock issuable upon the exercise of options exercisable as of January 15, 2026 or within 60 days of January 15, 2026.

(7) Includes 8,027 shares of RSUs that vest within 60 days of January 15, 2026.

(8) Includes 12,801 shares of RSUs that vest within 60 days of January 15, 2026.

(9) Includes 22,965 shares of Common Stock issuable upon the exercise of options exercisable as of January 15, 2026 or within 60 days of January 15, 2026, and 20,828 shares of RSUs that vest within 60 days of January 15, 2026.

Equity Compensation Plan Information

The following table provides information as of September 30, 2025 with respect to shares of our Common Stock that may be issued under our equity compensation plans.

	Equity Compensation Plan Information		
	Number of shares to be issued upon exercise of outstanding options, warrants and rights	Weighted average exercise price of outstanding options, warrants and rights	Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a))
Equity compensation plans approved by security holders (1)	7,217,386	$35.40	2,378,770
Equity compensation plans not approved by security holders (2)	506,567	$49.61	255,244
Total	7,723,953	$35.50	2,634,014

(1) Includes options outstanding representing 776,279 and 32,151 shares of Common Stock under the 2013 Incentive Plan and the 2021 Incentive Plan, respectively. Also includes 1,500,000 and 3,750,071 RSUs subject to the 2013 Incentive Plan and the 2021 Incentive Plan, respectively. There is no exercise price associated with a RSU award. Accordingly, these have been excluded from the column in the table reporting the weighted-average exercise price of outstanding awards.

(2) Includes 598,605 inducement option grants and 53,713 inducement RSU grants issued to newly hired employees granted outside of the Company's Inducement Plan and 0 inducement option grants and 506,567 inducement RSU grants issued to newly hired employees pursuant to the Company's Inducement Plan.

Material Features of the Inducement Plan and Stand-Alone Inducement Awards

The Company's Inducement Plan was established by the Board during fiscal year 2024 to advance the interests of the Company by providing for the grant of stock-based awards. The Company has also granted inducement awards outside of the inducement plan. In accordance with Nasdaq rules, this plan and the stand-alone awards are used to offer equity awards as material inducements for new employees to join the Company. Subject to adjustment for certain changes in our capitalization, the maximum aggregate number of shares that may be issued under the inducement plan is 832,950. The equity awards granted as inducement awards both under and outside of the inducement plan are typically in the form of stock options with exercise prices equal to the fair market value of our common stock on the date of grant and/or restricted stock units. The inducement plan also provides for the granting of other types of equity awards, including stock appreciation rights and restricted stock awards.

Current Executive Officers of the Registrant

The names, ages, and positions of our current executive officers serving as of January 26, 2026 are provided below. Biographical information regarding these officers is set forth under the following table, except for Dr. Anzalone, whose biography is set forth above with our other directors. Each executive officer holds office until the first meeting of the Board of Directors after the annual meeting of stockholders next succeeding their election, and until his/her successor is elected and qualified or until his/her earlier resignation or removal.

Name	Age	Position with Arrowhead
Christopher Anzalone	56	Chief Executive Officer, President, Director and Board Chair
Daniel Apel	60	Chief Financial Officer
James Hamilton	48	Chief Medical Officer
Patrick O'Brien	62	Chief Operating Officer

Daniel Apel, Chief Financial Officer, joined Arrowhead as Chief Financial Officer in April 2025. Mr. Apel brings over 25 years of US and global financial experience to Arrowhead. Prior to joining Arrowhead he served as Global Head of Financial Planning and Analysis from June 2019 through June 2024 at Walgreens Boots Alliance WBA, a healthcare, pharmacy and retail company, where he was responsible for global financial planning and analysis. Mr. Apel also served in various capacities in his nearly 20-year career at Bayer, including as Chief Financial Officer for Bayer U.S. from 2016 through 2019, as Chief Financial Officer for Bayer Canada and as global Head of Accounting for the Bayer Healthcare Segment, based in Germany. He served on the boards of the Biotechnology Innovation Organization (BIO) and the Organization for International Investment, as well as a Trustee for the Health Institute of New Jersey. Mr. Apel earned his Master's degree in Business Administration from the University of California, Berkeley, a Bachelor's of Arts Degree from the University of Pennsylvania, and is a licensed Certified Public Accountant.

James Hamilton, Chief Medical Officer and Head of R&D, joined the Company in 2014, where he has served as Chief Medical Officer since February 2025. He previously served as Chief of Discovery & Translational Medicine, and was responsible for target discovery as well as non-clinical and early clinical development. Prior to that, Dr. Hamilton served as Vice President, Clinical Development, and was responsible for clinical strategy, clinical trial design and execution including early translational and mid-stage development of all Arrowhead programs. He is experienced in multiple disease areas including virology, hepatology, cardiovascular disease, rare disease and oncology. Dr. Hamilton led the clinical development of ARO-HBV (now JNJ-3989), which was licensed to Janssen Pharmaceuticals. In parallel, Dr. Hamilton served as Head of Corporate Development and led Arrowhead's in-licensing transaction of Novartis's RNAi assets, as well as the out-licensing of ARO-LPA (now AMG890) to Amgen and the ARO-AAT partnership with Takeda. Dr. Hamilton started his employment at Arrowhead as Medical Director and Head of Corporate development. He holds both M.D. and M.B.A. degrees from The Ohio State University. He is a licensed physician and completed residency training with board certification in emergency medicine.

Patrick C. O'Brien, Chief Operating Officer, joined the Company in December 2014, where he has served as Chief Operating Officer since July 2022 and previously served as General Counsel from 2014 to December 2025. Mr. O'Brien has practiced in the healthcare legal field for over 30 years. Before joining the Company, from 2012 to 2014, Mr. O'Brien was with Shire, a global pharmaceutical company, where he was Group Vice President, Law. Immediately prior to working with Shire, he was a partner with the international law firm of Holland & Knight LLP in its Washington, DC office. In 2010, Mr. O'Brien co-founded the law firm O'Brien Gould PLLC which joined Holland & Knight in 2011. From 2009 to 2010, Mr. O'Brien was a partner in Burke O'Neil LLC. From 2001 to 2009, Mr. O'Brien served in several legal roles with Johnson & Johnson, including serving as Vice President of Law for J&J's Centocor Ortho-Biotech unit. Mr. O'Brien previously served as Regulatory Counsel with the United States Food & Drug Administration. Mr. O'Brien was awarded a B.S. in Pharmacy and a Pharm.D. from the University of Arizona before completing a residency in Clinical Pharmacy with the University of Illinois at Chicago Hospital. He was also awarded his J.D. from the University of Arizona.

Review and Approval of Related-Party Transactions

Our Board has adopted written policies and procedures for the review and approval of related-party transactions and has delegated to the Audit Committee the authority to review and approve the material terms of any proposed related-party transactions. To the extent that a proposed related-party transaction may involve a non-employee director or nominee for election as a director and may be material to a consideration of that person's independence, the matter may also be considered by the other disinterested directors.

Pursuant to our Corporate Code of Conduct and our Nomination Committee Charter, each of our officers, directors and employees must disclose related-party transactions to our Board. In order to avoid conflicts of interest, our executive officers and directors may not acquire any ownership interest in any supplier, customer or competitor (other than nominal amounts of stock in publicly traded companies), enter into any consulting or employment relationship with any customer, supplier or competitor, or engage in any outside business activity that is competitive with any of our businesses, without first disclosing the proposed transaction. After the proposed transaction has been disclosed, a determination will be made by our Board or Audit Committee as to what course to follow, depending on the nature or extent of the conflict. Furthermore, our executive officers and directors may not serve on any board of directors of any customer, supplier or competitor unless such board service has been disclosed to us and approved by our Board.

In determining whether to approve or ratify a related-party transaction, the Board and/or Audit Committee may consider, among other factors it deems appropriate, the potential benefits to the Company, the impact on a director's or nominee's independence or an executive officer's relationship with or service to the Company, whether the related-party transaction is on terms no less favorable than terms generally available to an unaffiliated third party under the same or similar circumstances, and the extent of the related party's interest in the transaction. In deciding to approve a transaction, the Board or Audit Committee may, in its sole discretion, impose such conditions as it deems appropriate on us or the related party in connection with its approval of any transaction. Any transactions involving the compensation of executive officers, however, are reviewed and approved by the Compensation Committee. If a related-party transaction will be ongoing, the Audit Committee may establish guidelines to be followed in our ongoing dealings with the related party. Thereafter, the Audit Committee reviews and assesses the ongoing relationship with each related party to see that it is in compliance with the Audit Committee's guidelines and that the related-party transaction remains appropriate.

Certain Relationships and Related Transactions

The following is a summary of each transaction or series of similar transactions since November 1, 2024, or any currently proposed transaction, to which we were or are a party in which:

- the amount involved exceeds $120,000; and
- any related person (including our directors, executive officers, beneficial owners of more than 5% of our common stock and any affiliates or members of their immediate family) had or will have a direct or indirect material interest, other than compensation and other arrangements that are described under the section titled “Executive Compensation” or that were approved by our Compensation Committee.

Beneficial ownership of securities is determined in accordance with the rules of the SEC.

Dr. Bruce Given was the Company's Chief Medical Scientist, and is the brother of Dr. Douglass Given, the Company's former Director and Chairman of the Board, who stepped down effective as of December 31, 2024. Dr. Bruce Given transitioned to being a consultant to the Company on August 1, 2025 for a yearly fee of $280,000. Dr. Bruce Given earned base salary, bonus and consulting fees of $887,227 during fiscal year 2025. In fiscal 2025, Dr. Bruce Given was awarded 100,000 RSUs, and this award vests in four annual tranches from the grant date.

Vincent Anzalone is the Company’s Senior Vice President, Investor Relations and Corporate Communications and the brother of Christopher Anzalone, the Company’s Chief Executive Officer, President, Director and Board Chair. Vincent Anzalone earned base salary and bonus of $417,568 during fiscal year 2025. In fiscal 2025, as part of the Company's annual stock grants to employees, Vincent Anzalone was awarded 25,000 RSUs, which vest in four annual tranches from the grant date and 5,000 RSUs, which vests two years after the grant date. In January 2026, as part of the Company's annual stock grants to employees, Vincent Anzalone was awarded 21,000 RSUs, and this award vests in four annual tranches from the grant date.

Annual Report on Form 10-K

The Company will mail, without charge to any stockholder solicited upon written request, a copy of the Company’s Annual Report on Form 10-K for the year ended September 30, 2025 including the financial statements, schedules and a list of exhibits. Requests should be sent to Arrowhead Pharmaceuticals, Inc., 177 E. Colorado Blvd., Suite 700, Pasadena, CA 91105, Attn: Corporate Secretary, Phone (626) 304-3400.

Stockholders Sharing the Same Address

We may satisfy SEC rules regarding delivery of proxy statements including the proxy statement, annual report and Notice, by delivering a single Notice and, if applicable, a single set of proxy materials to an address shared by two or more of our stockholders. This delivery method can result in meaningful cost savings for us. To take advantage of this opportunity, we may deliver only one Notice, and if applicable, a single set of proxy materials to multiple stockholders who share an address, unless contrary instructions are received prior to the mailing date. Similarly, if you share an address with another stockholder and have received multiple copies of our Notice and/or other proxy materials, you may write or call us at the address and phone number below to request delivery of a single copy of these materials in the future. We undertake to deliver promptly upon written or oral request a separate copy of the Notice and/or other proxy materials to a stockholder at a shared address to which a single copy of these documents was delivered. If you hold stock as a record stockholder and prefer to receive separate copies of a Notice, and if applicable, other proxy materials either now or in the future, please contact us at the address provided below. If your stock is held through a brokerage firm or bank and you prefer to receive separate copies of a Notice and, if applicable, other proxy materials either now or in the future, please contact your brokerage firm or bank.

Arrowhead Pharmaceuticals, Inc.
177 E. Colorado Blvd., Suite 700
Pasadena CA 91105
Attn: Corporate Secretary
Phone (626) 304-3400

Delinquent Section 16(a) Reports

Section 16(a) of the Exchange Act requires our directors, executive officers, and greater-than-10% stockholders to file forms with the SEC to report their ownership of Company shares and any changes in ownership. We have reviewed all forms filed electronically with the SEC. Based on that review and on written information given to us by our executive officers and directors, we believe that all of our directors, executive officers and greater-than-10% stockholders filed the required reports under Section 16(a) on a timely basis during fiscal 2025, except for the inadvertent late filing of one Form 3 the initial beneficial ownership of Company securities for Mr. Daniel Apel.

Legal Matters

Forward-Looking Statements. The Proxy Statement may contain "forward-looking statements" within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995, which statements are subject to substantial risks and uncertainties and are based on estimates and assumptions. All statements other than statements of historical fact included in the Proxy Statement are forward-looking statements, including statements about the Company's Board of Directors; corporate governance practices; executive compensation program; equity compensation utilization; corporate responsibility initiatives; our future financial performance; trends in our business; the initiation, timing, progress and results of our preclinical studies and clinical trials, and our research and development programs; our expectations regarding our commercialization efforts for REDEMPLO; our expectations regarding the potential benefits of the partnership, licensing and/or collaboration arrangements and other strategic arrangements and transactions we have entered into or may enter into in the future; our beliefs and expectations regarding the amount and timing of future milestone, royalty or other payments that could be due to or from third parties under existing agreements; and our estimates regarding future revenues, research and development expenses, capital requirements and payments to third parties. In some cases, you can identify forward-looking statements by terms such as "may," "might," "will," "objective," "intend," "should," "could," "can," "would," "expect," "believe," "design," "estimate," "predict," "potential," "plan" or the negative of these terms, and similar expressions intended to identify forward-looking statements. These statements involve known and unknown risks, uncertainties and other factors that could cause our actual results or outcomes to differ materially from the forward-looking statements expressed or implied in the Proxy Statement. Such risks, uncertainties and other factors include those identified in the Company's Annual Report on Form 10-K for the year ended September 30, 2025 filed with the SEC and other subsequent documents we file with the SEC. The Company expressly disclaims any obligation to update or alter any statements whether as a result of new information, future events or otherwise, except as required by law.

Website References. Website references throughout this document are inactive textual references and provided for convenience only, and the content on the referenced websites is not incorporated herein by reference and does not constitute a part of the Proxy Statement.

Other Matters

The Company knows of no other matters to be submitted at the Annual Meeting. If any other matters properly come before the meeting, it is the intention of the persons named in the proxy card to vote on such matters in accordance with their best judgment.

BY ORDER OF THE BOARD OF DIRECTORS

/s/ Patrick O'Brien
Patrick O'Brien,
Secretary

Pasadena, California
January 26, 2026

Exhibit A

Arrowhead Pharmaceuticals, Inc. Amended and Restated 2021 Incentive Plan

1. DEFINED TERMS

Exhibit A, which is incorporated by reference, defines the terms used in the Plan and sets forth certain operational rules related to those terms.

2. PURPOSE

The Plan has been established to advance the interests of the Company by providing for the grant to Participants of Stock-based and other incentive Awards.

3. ADMINISTRATION

The Administrator has discretionary authority, subject only to the express provisions of the Plan, to interpret the Plan; determine eligibility for and grant Awards; determine, modify or waive the terms and conditions of any Award; prescribe forms, rules and procedures relating to the Plan; determine whether Awards should be settled in cash and/or shares of Stock; and otherwise do all things necessary or appropriate to carry out the purposes of the Plan. Determinations of the Administrator made under the Plan will be conclusive and will bind all parties.

4. LIMITS ON AWARDS UNDER THE PLAN

(a) *Number of Shares*. The maximum number of shares of Stock that may be delivered in satisfaction of Awards under the Plan is (i) 18,500,000, which includes (A) 8,000,000 shares of Stock previously approved by the Company's stockholders prior to this amendment and restatement and (B) 10,500,000 additional shares of Stock that shall become available for grant under the Plan upon stockholder approval of this amendment and restatement, (ii) reduced by any shares of Stock subject to awards made under the Prior Plan after January 1, 2021. Shares of Stock subject to outstanding awards under the Prior Plans as of January 1, 2021 that, after January 1, 2021, are canceled, expired, forfeited or otherwise not issued under such award (other than as a result of being tendered or withheld to pay the exercise price or withholding taxes in connection with any such awards) or settled in cash shall be added to the number of shares of Stock issuable under the Plan. No more than 18,500,000 shares of Stock may be delivered in satisfaction of ISOs awarded under the Plan. Nothing in this Section 4(a) will be construed as requiring that any, or any fixed number of, ISOs be awarded under the Plan. For purposes of this Section 4(a), the number of shares of Stock delivered in satisfaction of Awards will be determined (i) by treating as having been delivered any shares of Stock underlying the portion of any Award that is settled in Stock, (ii) by treating as having been delivered any shares of Stock tendered to or withheld by the Company from any Award in payment of the exercise price of any Award requiring exercise or in satisfaction of the tax withholding requirements with respect to any Award, and (iii) by treating as having been delivered any shares of Stock purchased by the Company with proceeds from the exercise of Stock Options. Stock underlying the portion of any Award that expires, terminates or is forfeited or is settled in cash prior to the issuance of Stock thereunder shall not be counted as having been delivered under the Plan. To the extent consistent with the requirements of Section 422 and the regulations thereunder, and with other applicable legal requirements (including applicable stock exchange requirements), Stock issued under awards of an acquired company that are converted, replaced or adjusted in connection with the acquisition shall not reduce the number of shares of Stock available for Awards under the Plan.

(b) *Type of Shares*. Stock delivered by the Company under the Plan may be authorized but unissued Stock or previously issued Stock acquired by the Company. No fractional shares of Stock will be delivered under the Plan.

5. ELIGIBILITY AND PARTICIPATION

The Administrator will select Participants from among key Employees and directors of, and consultants and advisors to, the Company and its Affiliates. Eligibility for ISOs is limited to individuals described in the first sentence of this Section 5 who are employees of the Company or of a "parent corporation" or "subsidiary corporation" of the Company

as those terms are defined in Section 424 of the Code. Eligibility for Stock Options other than ISOs is limited to individuals described in the first sentence of this Section 5 who are providing direct services on the date of grant of the Stock Option to either the Company or to a subsidiary of the Company that would be described in the first sentence of Treas. Regs. §1.409A-1(b)(5)(iii)(E).

6. RULES APPLICABLE TO AWARDS

(a) *All Awards*.

(1) *Award Provisions*. The Administrator will determine the terms of all Awards, subject to the limitations provided herein. By accepting (or, under such rules as the Administrator may prescribe, being deemed to have accepted) an Award, the Participant will be deemed to have agreed to the terms of the Award and the Plan. Notwithstanding any provision of this Plan to the contrary, awards of an acquired company that are converted, replaced or adjusted in connection with the acquisition may contain terms and conditions that are inconsistent with the terms and conditions specified herein, as determined by the Administrator.

(2) *Term of Plan*. No Awards may be made after ten years from the Date of Adoption, but previously granted Awards may continue beyond that date in accordance with their terms.

(3) *Transferability*. Neither ISOs nor, except as the Administrator otherwise expressly provides in accordance with the third sentence of this Section 6(a)(3), other Awards may be transferred other than by will or by the laws of descent and distribution. During a Participant's lifetime, ISOs (and, except as the Administrator otherwise expressly provides in accordance with the third sentence of this Section 6(a)(3), SARs and NSOs) may be exercised only by the Participant. The Administrator may permit the gratuitous transfer (*i.e.*, transfer not for value) of Awards other than ISOs to any transferee eligible to be covered by the provisions of Form S-8 (under the Securities Act of 1933, as amended), subject to such limitations as the Administrator may impose.

(4) *Vesting, etc*. Subject to Section 6(a)(5), the Administrator will determine the time or times at which an Award will vest or become exercisable and the terms on which a Stock Option or SAR will remain exercisable. Without limiting the foregoing, the Administrator may at any time accelerate the vesting or exercisability of an Award, regardless of any adverse or potentially adverse tax or other consequences resulting from such acceleration. Unless the Administrator expressly provides otherwise, however, the following rules will apply if a Participant's Employment ceases:

(A) Immediately upon the cessation of the Participant's Employment and except as provided in (B), (C), and (D) below, each Stock Option and SAR that is then held by the Participant or by the Participant's permitted transferees, if any, will cease to be exercisable and will terminate and all other Awards that are then held by the Participant or by the Participant's permitted transferees, if any, to the extent not already vested will be forfeited.

(B) Subject to (C), (D) and (E) below, all Stock Options and SARs held by the Participant or the Participant's permitted transferees, if any, immediately prior to the cessation of the Participant's Employment, to the extent then exercisable, will remain exercisable for the lesser of (i) a period of three months and (ii) the period ending on the latest date on which such Stock Option or SAR could have been exercised without regard to this Section 6(a)(4), and will thereupon immediately terminate.

(C) All Stock Options and SARs held by a Participant or the Participant's permitted transferees, if any, immediately prior to the cessation of the Participant's Employment due to his or her death, to the extent then exercisable, will remain exercisable for the lesser of (i) a period of twelve months and (ii) the period ending on the latest date on which such Stock Option or SAR could have been exercised without regard to this Section 6(a)(4), and will thereupon immediately terminate.

(D) All Stock Options and SARs held by a Participant or the Participant's permitted transferees, if any, immediately prior to the cessation of the Participant's Employment due to his or her "permanent and total disability" (within the meaning of Section 22(e)(3) of the Code, to the extent then exercisable, will remain exercisable for the lesser of (i) a period of six months and (ii) the period ending on the latest date on which such Stock Option or SAR could have been exercised without regard to this Section 6(a)(4), and will thereupon immediately terminate.

(E) All Awards (whether or not exercisable) held by a Participant or the Participant's permitted transferees, if any, immediately prior to the cessation of the Participant's Employment will immediately terminate upon such cessation of Employment if the termination is for Cause or occurs in circumstances that in the sole determination of the Administrator would have constituted grounds for the Participant's Employment to be terminated for Cause.

(5) *Minimum Vesting; Holding Period*. Except as otherwise provided in this Section 6(a)(5), no Award shall be granted with terms providing for any right of vesting, exercise or lapse of vesting requirements earlier than a date that is at least one (1) year following the date of grant (or, in the case of vesting based upon performance objectives, exercise and vesting restrictions cannot lapse earlier than the one (1) year anniversary measured from the date of the commencement of the period over which performance is measured). Notwithstanding the foregoing, the Administrator may grant up to a maximum of five percent (5%) of the aggregate number of shares available for issuance under the Plan (for purposes of counting shares against the five percent (5%) limitation, the share counting rules under Section 4(a) shall apply) that do not comply with the one (1) year minimum vesting and exercise requirements set forth in the preceding sentence. In addition, (i) Awards granted to non-employee directors need not comply with the one (1) year minimum vesting and exercise requirements so long as the Awards provide for a right of exercise or lapse of any vesting obligations no earlier than the next annual stockholder meeting date following the grant date (provided such annual meeting is at least 50 weeks after the immediately preceding year's annual meeting) and (ii) Awards granted in connection with a merger or other acquisition as a substitute or replacement award for awards held by grantees of the acquired business need not comply with the one (1) year minimum vesting and exercise requirements. In addition to the minimum vesting and exercise requirements in this Section 6(a)(5), any Awards granted to the Company's Chief Executive Officer shall be granted with terms providing that the shares issued upon exercise or settlement of any such Award (net of tax withholding) may not be transferred or otherwise disposed of by the Company's Chief Executive Officer for at least twelve (12) months following the date of such exercise or settlement.

(6) *Additional Restrictions*. The Administrator may cancel, rescind, withhold or otherwise limit or restrict any Award at any time if the Participant is not in compliance with all applicable provisions of the Award agreement and the Plan, or if the Participant breaches any agreement with the Company or its Affiliates with respect to non-competition, non-solicitation or confidentiality. Without limiting the generality of the foregoing, the Administrator may recover Awards made under the Plan and payments under or gain in respect of any Award in accordance with any applicable Company clawback or recoupment policy, as such policy may be amended and in effect from time to time, or as otherwise required by law or applicable stock exchange listing standards, including, without limitation, Section 10D of the Exchange Act, or any stock exchange or similar rule adopted under said Section.

(7) *Taxes*. The grant of an Award and the delivery, vesting and retention of Stock, cash or other property under an Award are conditioned upon full satisfaction by the Participant of all tax withholding requirements with respect to the Award. The Administrator will prescribe such rules for the withholding of taxes as it deems necessary or appropriate. The Administrator may, but need not, hold back shares of Stock from an Award or permit a Participant to tender previously owned shares of Stock in satisfaction of tax withholding requirements (only up to the amount permitted that will not cause an adverse accounting consequence or cost).

(8) *Dividend Equivalents, Etc*. The Administrator may provide for the payment of amounts (on terms and subject to conditions established by the Administrator) in lieu of cash dividends or other cash distributions with respect to Stock subject to an Award whether or not the holder of such Award is otherwise entitled to share in the actual dividend or distribution in respect of such Award. Any entitlement to dividend equivalents or similar entitlements will be established and administered either consistent with an exemption from, or in compliance with, the requirements of Section 409A. Dividends or dividend equivalent amounts payable in respect of Awards that are subject to restrictions will be subject to the same vesting and forfeiture restrictions as apply to the Awards to which they relate.

(9) *Rights Limited*. Nothing in the Plan or in any Award will be construed as giving any person the right to continued employment or service with the Company or its Affiliates, or any rights as a stockholder except as to shares of Stock actually issued under the Plan; nor will anything in the Plan or in any Award affect the right of the Company or its Affiliates to discharge or discipline a Participant at any time. The loss of existing or potential profit in Awards will not constitute an element of damages in the event of termination of Employment for any reason, even if the termination is in violation of an obligation of the Company or any Affiliate to the Participant.

(10) *Coordination with Other Plans*. Awards under the Plan may be granted in tandem with, or in satisfaction of or substitution for, other Awards under the Plan or awards made under other compensatory plans or programs of the Company or its Affiliates. For example, but without limiting the generality of the foregoing, awards under other compensatory plans or programs of the Company or its Affiliates may be settled in Stock (including, without limitation, Unrestricted Stock) if the Administrator so determines, in which case the shares delivered will be treated as awarded under the Plan (and will reduce the number of shares thereafter available under the Plan in accordance with the rules set forth in Section 4).

(11) *Section 409A*. Each Award will contain such terms as the Administrator determines, and will be construed and administered, such that the Award either qualifies for an exemption from the requirements of Section 409A or satisfies such requirements.

(b) *Stock Options and SARs*.

(1) *Time And Manner Of Exercise*. Unless the Administrator expressly provides otherwise, no Stock Option or SAR will be deemed to have been exercised until the Administrator receives a notice of exercise (in form acceptable to the Administrator), which may be an electronic notice, signed (including electronic signature in form acceptable to the Administrator) by the appropriate person and accompanied by any payment required under the Award. A Stock Option or SAR exercised by any person other than the Participant will not be deemed to have been exercised until the Administrator has received such evidence as it may require that the person exercising the Award has the right to do so.

(2) *Exercise Price*. The exercise price (or the base value from which appreciation is to be measured) of each Award requiring exercise will be no less than 100% (or in the case of an ISO granted to a ten-percent shareholder within the meaning of subsection (b)(6) of Section 422, 110%) of the Fair Market Value of the Stock subject to the Award, determined as of the date of grant, or such higher amount as the Administrator may determine in connection with the grant. Except in connection with a corporate transaction involving the Company (which term shall include, without limitation, any stock dividend, stock split, extraordinary cash dividend, recapitalization, reorganization, merger, consolidation, split-up, spin-off, combination, or exchange of shares) or as otherwise contemplated by Section 7 of the Plan, the Company may not, without obtaining stockholder approval, (A) amend the terms of outstanding Stock Options or SARs to reduce the exercise price or base value of such Stock Options or SARs, (B) cancel outstanding Stock Options or SARs in exchange for Stock Options or SARs with an exercise price or base value that is less than the exercise price or base value of the original Stock Options or SARs, or (C) cancel outstanding Stock Options or SARs that have an exercise price or base value greater than the Fair Market Value of a share of Stock on the date of such cancellation in exchange for cash or other consideration.

(3) *Payment Of Exercise Price*. Where the exercise of an Award is to be accompanied by payment, payment of the exercise price will be by cash or check acceptable to the Administrator or by such other legally permissible means, if any, as may be acceptable to the Administrator.

(4) *Maximum Term*. Stock Options and SARs will have a maximum term not to exceed ten (10) years from the date of grant (or five (5) years from the date of grant in the case of an ISO granted to a ten-percent shareholder described in Section 6(b)(2) above); provided, however, that, if a Participant still holding an outstanding but unexercised NSO or SAR ten (10) years from the date of grant (or, in the case of an NSO or SAR with a maximum term of less than ten (10) years, such maximum term) is prohibited by applicable law or a written policy of the Company applicable to similarly situated employees from engaging in any open-market sales of Stock, and if at such time the Stock is publicly traded (as determined by the Administrator), the maximum term of such Award will instead be deemed to expire on the thirtieth (30th) day following the date the Participant is no longer prohibited from engaging in such open market sales.

7. EFFECT OF CERTAIN TRANSACTIONS

(a) *Mergers, etc*. Except as otherwise provided in an Award agreement, the following provisions will apply in the event of a Covered Transaction:

(1) *Assumption or Substitution*. If the Covered Transaction is one in which there is an acquiring or surviving entity, the Administrator may (but, for the avoidance of doubt, need not) provide (i) for the assumption or continuation of some or all outstanding Awards or any portion thereof or (ii) for the grant of new awards in substitution therefor by the acquiror or survivor or an affiliate of the acquiror or survivor.

(2) *Cash-Out of Awards*. Subject to Section 7(a)(5) below, the Administrator may (but, for the avoidance of doubt, need not) provide for payment (a "cash-out"), with respect to some or all Awards or any portion thereof, equal in the case of each affected Award or portion thereof to the excess, if any, of (A) the Fair Market Value of one share of Stock (as determined by the Administrator in its reasonable discretion) times the number of shares of Stock subject to the Award or such portion, over (B) the aggregate exercise or purchase price, if any, under the Award or such portion (in the case of an SAR, the aggregate base value above which appreciation is measured), in each case on such payment terms (which need not be the same as the terms of payment to holders of Stock) and other terms, and subject to such conditions, as the Administrator determines, it being understood that if the exercise or purchase price (or base value) of an Award is equal to or greater than the Fair Market Value of one share of Stock (as determined in accordance with this Section 7(a)(2)), the Award may be cancelled with no payment due hereunder.

(3) *Acceleration of Certain Awards*. Subject to Section 7(a)(6) below, the Administrator may (but, for the avoidance of doubt, need not) provide that any Award requiring exercise will become exercisable, in full or in part and/or that the

delivery of any shares of Stock remaining deliverable under any outstanding Award of Stock Units (including Restricted Stock Units and Performance Awards to the extent consisting of Stock Units) will be accelerated in full or in part, in each case on a basis that gives the holder of the Award a reasonable opportunity, as determined by the Administrator, following exercise of the Award or the delivery of the shares, as the case may be, to participate as a stockholder in the Covered Transaction.

(4) *Change in Control*. Notwithstanding anything herein to the contrary, unless otherwise expressly provided for in the Award agreement or another contract, including an employment or severance agreement or severance plan, or under the terms of a transaction constituting a Change in Control, in the event of a Change in Control, each outstanding Award will fully vest (which, in the case of Performance Awards shall be to their maximal value) and become exercisable immediately prior to the consummation of such Change in Control, and the Administrator shall notify the Participant in writing or electronically that the Award will be fully vested and exercisable for a period of at least fifteen (15) days from the date of such notice, (which notice may be delivered prior to the consummation of the Change in Control) and the Award will terminate upon the expiration of such period if not otherwise assumed or substituted pursuant to Section 7(a)(1) above or cashed-out pursuant to Section 7(a)(2) above.

(5) *Termination of Awards Upon Consummation of Covered Transaction*. Except as the Administrator may otherwise determine in any case and except for the 15-day exercise period set forth above in Section 7(a)(4), each Award will automatically terminate (and in the case of outstanding shares of Restricted Stock, will automatically be forfeited) upon consummation of the Covered Transaction, other than Awards assumed pursuant to Section 7(a)(1) above.

(6) *Additional Limitations*. Any share of Stock and any cash or other property delivered pursuant to Section 7(a)(2) or Section 7(a)(3) above with respect to an Award may, in the discretion of the Administrator, contain such restrictions, if any, as the Administrator deems appropriate to reflect any performance or other vesting conditions to which the Award was subject and that did not lapse (and were not satisfied) in connection with the Covered Transaction. For purposes of the immediately preceding sentence, a cash-out under Section 7(a)(2) above or acceleration under Section 7(a)(3) above will not, in and of itself, be treated as the lapsing (or satisfaction) of a performance or other vesting condition. In the case of Restricted Stock that does not vest and is not forfeited in connection with the Covered Transaction, the Administrator may require that any amounts delivered, exchanged or otherwise paid in respect of such Stock in connection with the Covered Transaction be placed in escrow or otherwise made subject to such restrictions as the Administrator deems appropriate to carry out the intent of the Plan.

(b) *Changes in and Distributions With Respect to Stock*.

(1) *Basic Adjustment Provisions*. In the event of a stock dividend, stock split or combination of shares (including a reverse stock split), recapitalization, reclassification or other distribution of the Company's equity securities without the receipt of consideration by the Company, or other change in the Company's capital structure that constitutes an equity restructuring within the meaning of FASB ASC 718, the Administrator will make appropriate adjustments to the maximum number of shares specified in Section 4(a) that may be delivered under the Plan and will also make appropriate adjustments to the number and kind of shares of stock or securities subject to Awards then outstanding or subsequently granted, any exercise or purchase prices (or base values) relating to Awards and any other provision of Awards affected by such change.

(2) *Certain Other Adjustments*. The Administrator may also make adjustments of the type described in Section 7(b)(1) above to take into account distributions to stockholders other than those provided for in Section 7(a) and 7(b)(1), or any other event, if the Administrator determines that adjustments are appropriate to avoid distortion in the operation of the Plan, having due regard for the qualification of ISOs under Section 422 and the requirements of Section 409A, where applicable.

(3) *Continuing Application of Plan Terms*. References in the Plan to shares of Stock will be construed to include any stock or securities resulting from an adjustment pursuant to this Section 7.

8. LEGAL CONDITIONS ON DELIVERY OF STOCK

The Company will not be obligated to deliver any shares of Stock pursuant to the Plan or to remove any restriction from shares of Stock previously delivered under the Plan until: (i) the Company is satisfied that all legal matters in connection with the issuance and delivery of such shares have been addressed and resolved; (ii) if the outstanding Stock is at the time of delivery listed on any stock exchange or national market system, the shares to be delivered have been listed or authorized to be listed on such exchange or system upon official notice of issuance; and (iii) all conditions of the Award have been satisfied or waived. The Company may require, as a condition to exercise of the Award, such representations or agreements as counsel for the Company may consider appropriate to avoid violation

of the Securities Act of 1933, as amended, or any applicable state or non-U.S. securities law. Any Stock required to be issued to Participants under the Plan will be evidenced in such manner as the Administrator may deem appropriate, including book-entry registration or delivery of stock certificates. In the event that the Administrator determines that Stock certificates will be issued to Participants under the Plan, the Administrator may require that certificates evidencing Stock issued under the Plan bear an appropriate legend reflecting any restriction on transfer applicable to such Stock, and the Company may hold the certificates pending lapse of the applicable restrictions.

9. AMENDMENT AND TERMINATION

The Administrator may at any time or times amend the Plan or any outstanding Award for any purpose which may at the time be permitted by law, and may at any time terminate the Plan as to any future grants of Awards; provided, that except as otherwise expressly provided in the Plan the Administrator may not, without the Participant's consent, alter the terms of an Award so as to affect materially and adversely the Participant's rights under the Award, unless the Administrator expressly reserved the right to do so at the time the Award was granted or unless the Administrator determines in its sole discretion and prior to the date of any Covered Transaction that such amendment or alteration either (i) is required or advisable in order for the Company, the Plan or the Award to satisfy any law or regulation or to meet the requirements of or avoid adverse financial accounting consequences under any accounting standard, or (ii) is not reasonably likely to significantly diminish the benefits provided under such Award, or that any such diminishment has been adequately compensated. Any amendments to the Plan will be conditioned upon stockholder approval only to the extent, if any, such approval is required by law (including the Code and applicable stock exchange requirements), as determined by the Administrator.

10. OTHER COMPENSATION ARRANGEMENTS

The existence of the Plan or the grant of any Award will not in any way affect the Company's right to Award a person bonuses or other compensation in addition to Awards under the Plan.

11. MISCELLANEOUS

(a) *Waiver of Jury Trial*. By accepting an Award under the Plan, each Participant waives any right to a trial by jury in any action, proceeding or counterclaim concerning any rights under the Plan and any Award, or under any amendment, waiver, consent, instrument, document or other agreement delivered or which in the future may be delivered in connection therewith, and agrees that any such action, proceedings or counterclaim will be tried before a court and not before a jury. By accepting an Award under the Plan, each Participant certifies that no officer, representative, or attorney of the Company has represented, expressly or otherwise, that the Company would not, in the event of any action, proceeding or counterclaim, seek to enforce the foregoing waivers. Notwithstanding anything to the contrary in the Plan, nothing herein is to be construed as limiting the ability of the Company and a Participant to agree to submit disputes arising under the terms of the Plan or any Award made hereunder to binding arbitration or as limiting the ability of the Company to require any eligible individual to agree to submit such disputes to binding arbitration as a condition of receiving an Award hereunder.

(b) *Limitation of Liability*. Notwithstanding anything to the contrary in the Plan, neither the Company, nor any Affiliate, nor the Administrator, nor any person acting on behalf of the Company, any Affiliate, or the Administrator, will be liable to any Participant or to the estate or beneficiary of any Participant or to any other holder of an Award by reason of any acceleration of income, or any additional tax (including any interest and penalties), asserted by reason of the failure of an Award to satisfy the requirements of Section 422 or Section 409A or by reason of Section 4999 of the Code, or otherwise asserted with respect to the Award.

12. ESTABLISHMENT OF SUB-PLANS

The Administrator may from time to time establish one or more sub-plans under the Plan for purposes of satisfying applicable blue sky, securities or tax laws of various jurisdictions. The Administrator will establish such sub-plans by adopting supplements to the Plan setting forth (i) such limitations on the Administrator's discretion under the Plan as it deems necessary or desirable and (ii) such additional terms and conditions not otherwise inconsistent with the Plan as it deems necessary or desirable. All supplements so established will be deemed to be part of the Plan, but each supplement will apply only to Participants within the affected jurisdiction (as determined by the Administrator).

13. GOVERNING LAW

The laws of the State of Delaware will govern all questions concerning the construction, validity and interpretation of this Plan, without regard to that state's conflict of laws rules.

EXHIBIT A

Definition of Terms

The following terms, when used in the Plan, will have the meanings and be subject to the provisions set forth below:

"Administrator": The Compensation Committee, except that the Compensation Committee may delegate (i) to one or more of its members (or one or more other members of the Board (including the full Board)) such of its duties, powers and responsibilities as it may determine; (ii) to one or more officers of the Company the power to grant Awards or perform any of its other duties, power and responsibilities as it may determine, to the extent permitted by Section 152(b) or 157(c) of the Delaware General Corporation Law and Nasdaq Listing Rule 5635(c)(4), provided, however, that the resolution so authorizing such officer or officers shall specify the total number of Awards (if any) such officer or officers may award pursuant to such delegated authority, the time period during which such Awards may be granted and the time period during which the Shares issuable upon exercise or vesting of an Award may be issued, a minimum amount of consideration (if any) for which such Awards may be issued and a minimum amount of consideration for the Stock issuable upon the exercise of an Award, and, unless provided otherwise in such authorizing resolutions, that any such Award shall be subject to the form of Award agreement theretofore approved by the Compensation Committee and, provided, further, that such authorization shall not provide for the grant of Awards to officers or directors of the Company who are subject to the reporting requirements of Section 16(a) of the Exchange Act and no such officer shall designate himself or herself as a recipient of any Awards granted under authority delegated to such officer; and (iii) to such Employees or other persons as it determines such ministerial tasks as it deems appropriate. In the event of any delegation described in the preceding sentence, the term "Administrator" will include the person or persons so delegated to the extent of such delegation.

"Affiliate": Any corporation or other entity that stands in a relationship to the Company that would result in the Company and such corporation or other entity being treated as one employer under Section 414(b) and Section 414(c) of the Code.

"Award": Any or a combination of the following:

(i) Stock Options.

(ii) SARs.

(iii) Restricted Stock.

(iv) Unrestricted Stock.

(v) Stock Units, including Restricted Stock Units.

(vi) Performance Awards.

(vii) Cash Awards.

(viii) Awards (other than Awards described in (i) through (vii) above) that are convertible into or otherwise based on Stock.

"Board": The Board of Directors of the Company.

"Cash Award": An Award denominated in cash.

"Cause": In the case of any Participant who is party to an employment or severance-benefit agreement that contains a definition of "Cause," the definition set forth in such agreement will apply with respect to such Participant under the Plan for so long as such agreement is in effect. In the case of any other Participant, "Cause" will mean, as determined by the Administrator in its reasonable judgment, (i) a substantial failure of the Participant to perform the Participant's duties and responsibilities to the Company or subsidiaries or substantial negligence in the performance of such duties and responsibilities; (ii) the commission by the Participant of a felony or a crime involving moral turpitude; (iii) the commission by the Participant of theft, fraud, embezzlement, material breach of trust or any material act of dishonesty

involving the Company or any of its subsidiaries; (iv) a significant violation by the Participant of the code of conduct of the Company or its subsidiaries of any material policy of the Company or its subsidiaries, or of any statutory or common law duty of loyalty to the Company or its subsidiaries; (v) material breach of any of the terms of the Plan or any Award made under the Plan, or of the terms of any other agreement between the Company or subsidiaries and the Participant; or (vi) other conduct by the Participant that could be expected to be harmful to the business, interests or reputation of the Company.

"Change in Control": The first to occur of (i) a "person," as such term is used in Sections 13(d) and 14(d) of the Exchange Act (other than the Company or one of its subsidiaries or an employee benefit plan of the Company or any of its subsidiaries, including any trustee of such plan acting as trustee) becoming the "beneficial owner" (as defined in Rule 13d-3 under the Exchange Act), directly or indirectly, of securities of the Company representing fifty percent (50%) or more of the combined voting power of the Company's then outstanding securities entitled to vote generally in the election of directors; (ii) a consummation of (x) a merger or consolidation of the Company with any other corporation, other than a merger or consolidation that would result in the voting securities of the Company outstanding immediately prior thereto continuing to represent (either by remaining outstanding or by being converted into voting securities of the surviving entity) more than fifty percent (50%) of the total voting power represented by the voting securities of the Company or such surviving entity outstanding immediately after such merger or consolidation, or (y) the sale or disposition by the Company of all or substantially all the Company's assets; or (iii) a change in the composition of the Board, as a result of which fewer than a majority of the directors are Incumbent Directors. "Incumbent Directors" shall mean directors who either (A) are directors of the Company as of the date the Plan is approved by the stockholders, or (B) are elected, or nominated for election, to the Board with the affirmative votes of at least a majority of the Incumbent Directors at the time of such election or nomination (but shall not include an individual whose election or nomination is in connection with an actual or threatened proxy contest relating to the election of directors to the Company). For the avoidance of doubt, a transaction will not constitute a Change in Control if: (i) its sole purpose is to change the jurisdiction of the Company's incorporation, or (ii) its sole purpose is to create a holding company that will be owned in substantially the same proportions by the persons who held the Company's securities immediately before such transaction.

"Code": The U.S. Internal Revenue Code of 1986 as from time to time amended and in effect, or any successor statute as from time to time in effect.

"Compensation Committee": The Compensation Committee of the Board.

"Company": Arrowhead Pharmaceuticals, Inc.

"Covered Transaction": Any of (i) a consolidation, merger, or similar transaction or series of related transactions, including a sale or other disposition of stock, in which the Company is not the surviving corporation or which results in the acquisition of all or substantially all of the Company's then outstanding common stock by a single person or entity or by a group of persons and/or entities acting in concert; (ii) a sale or transfer of all or substantially all the Company's assets; or (iii) a dissolution or liquidation of the Company. Where a Covered Transaction involves a tender offer that is reasonably expected to be followed by a merger described in clause (i) (as determined by the Administrator), the Covered Transaction will be deemed to have occurred upon consummation of the tender offer.

"Date of Adoption": The earlier of the date the Plan (for avoidance of doubt, as most recently amended and restated) was approved by the Company's stockholders or adopted by the Board, as determined by the Compensation Committee.

"Employee": Any person who is employed by the Company or an Affiliate.

"Employment": A Participant's employment or other service relationship with the Company and its Affiliates. Employment will be deemed to continue, unless the Administrator expressly provides otherwise, so long as the Participant is employed by, or otherwise is providing services in a capacity described in Section 5 to the Company or an Affiliate. If a Participant's employment or other service relationship is with an Affiliate and that entity ceases to be an Affiliate, the Participant's Employment will be deemed to have terminated when the entity ceases to be an Affiliate unless the Participant transfers Employment to the Company or its remaining Affiliates. Notwithstanding the foregoing and the definition of "Affiliate" above, in construing the provisions of any Award relating to the payment of "nonqualified deferred compensation" (subject to Section 409A) upon a termination or cessation of Employment, references to termination or cessation of employment, separation from service, retirement or similar or correlative terms will be construed to require a "separation from service" (as that term is defined in Section 1.409A-1(h) of the Treasury Regulations, after giving effect to the presumptions contained therein) from the Company and from all other corporations and trades or businesses, if any, that would be treated as a single "service recipient" with the Company

under Section 1.409A-1(h)(3) of the Treasury Regulations. The Company may, but need not, elect in writing, subject to the applicable limitations under Section 409A, any of the special elective rules prescribed in Section 1.409A-1(h) of the Treasury Regulations for purposes of determining whether a "separation from service" has occurred. Any such written election will be deemed a part of the Plan.

"Exchange Act": The Securities Exchange Act of 1934, as amended.

"Fair Market Value": As of any date, the value of the Stock determined as follows:

i. If the Stock is listed on any established stock exchange or traded on any established market, the Fair Market Value of a share of Stock as of any date of determination will be, unless otherwise determined by the Board, the closing sales price for such stock as quoted on such exchange or market (or the exchange or market with the greatest volume of trading in the Stock) on the date of determination, as reported in a source the Board deems reliable.

ii. Unless otherwise provided by the Board, if there is no closing sales price for the Stock on the date of determination, then the Fair Market Value will be the closing selling price on the last preceding date for which such quotation exists.

iii. In the absence of such markets for the Stock, the Fair Market Value will be determined by the Board in good faith and in a manner that complies with Sections 409A and 422 of the Code.

"ISO": A Stock Option intended to be an "incentive stock option" within the meaning of Section 422. Each Stock Option granted pursuant to the Plan will be treated as providing by its terms that it is to be an NSO unless, as of the date of grant, it is expressly designated as an ISO.

"NSO": A Stock Option that is not intended to be an "incentive stock option" within the meaning of Section 422.

"Participant": A person who is granted an Award under the Plan.

"Performance Award": An Award subject to Performance Criteria.

"Performance Criteria": Specified criteria, other than the mere continuation of Employment or the mere passage of time, the satisfaction of which is a condition for the grant, exercisability, vesting or full enjoyment of an Award. Such criteria may include, without limitation, a measure of performance relating to any or any combination of the following (measured either absolutely or by reference to an index or indices and determined either on a consolidated basis or, as the context permits, on a divisional, subsidiary, line of business, project or geographical basis or in combinations thereof): sales; revenues; assets; expenses; earnings before or after deduction for all or any portion of interest, taxes, depreciation, or amortization, whether or not on a continuing operations or an aggregate or per share basis; return on equity, investment, capital or assets; one or more operating ratios; borrowing levels, leverage ratios or credit rating; market share; capital expenditures; cash flow; stock price; stockholder return; sales of particular products or services; customer acquisition or retention; acquisitions and divestitures (in whole or in part); joint ventures and strategic alliances; spin-offs, split-ups and the like; reorganizations; or recapitalizations, restructurings, financings (issuance of debt or equity) or refinancings. A Performance Criterion and any targets with respect thereto determined by the Administrator need not be based upon an increase, a positive or improved result or avoidance of loss.

"Plan": The Arrowhead Pharmaceuticals, Inc. 2021 Incentive Plan as from time to time amended and in effect.

"Prior Plan": The Arrowhead Research Corporation 2013 Incentive Plan.

"Restricted Stock": Stock subject to restrictions requiring that it be redelivered or offered for sale to the Company if specified conditions are not satisfied.

"Restricted Stock Unit": A Stock Unit that is, or as to which the delivery of Stock or cash in lieu of Stock is, subject to the satisfaction of specified performance or other vesting conditions.

"SAR": A right entitling the holder upon exercise to receive an amount (payable in cash and/or in shares of Stock of equivalent value) equal to the excess of the Fair Market Value of the shares of Stock subject to the right over the base value from which appreciation under the SAR is to be measured.

"Section 409A": Section 409A of the Code.

"Section 422": Section 422 of the Code.

"Stock": Common stock of the Company, par value $0.001 per share.

"Stock Option": An option entitling the holder to acquire shares of Stock upon payment of the exercise price.

"Stock Unit": An unfunded and unsecured promise, denominated in shares of Stock, to deliver Stock or cash measured by the value of Stock in the future.

"Unrestricted Stock": Stock not subject to any restrictions under the terms of the Award.

ARROWHEAD PHARMACEUTICALS, INC.
177 EAST COLORADO BLVD, SUITE 700
PASADENA, CA 91105

SCAN TO VIEW MATERIALS & VOTE

VOTE BY INTERNET
Before The Meeting - Go to **www.proxyvote.com or scan the QR Barcode above**

Use the Internet to transmit your voting instructions and for electronic delivery of information. Vote by 11:59 P.M. ET on March 18, 2026. Have your proxy card in hand when you access the web site and follow the instructions to obtain your records and to create an electronic voting instruction form.

During The Meeting - Go to **www.virtualshareholdermeeting.com/ARWR2026**

You may attend the meeting via the Internet and vote during the meeting. Have the information that is printed in the box marked by the arrow available and follow the instructions.

VOTE BY PHONE - 1-800-690-6903
Use any touch-tone telephone to transmit your voting instructions. Vote by 11:59 P.M. ET on March 18, 2026. Have your proxy card in hand when you call and then follow the instructions.

VOTE BY MAIL
Mark, sign and date your proxy card and return it in the postage-paid envelope we have provided or return it to Vote Processing, c/o Broadridge, 51 Mercedes Way, Edgewood, NY 11717.

TO VOTE, MARK BLOCKS BELOW IN BLUE OR BLACK INK AS FOLLOWS:

V83337-P42564

KEEP THIS PORTION FOR YOUR RECORDS
DETACH AND RETURN THIS PORTION ONLY

THIS PROXY CARD IS VALID ONLY WHEN SIGNED AND DATED.

ARROWHEAD PHARMACEUTICALS, INC.

The Board of Directors recommends you vote FOR each of the following:

1. Election of Directors

Nominees:	**For**	**Against**	**Abstain**
1a. Christopher Anzalone	☐	☐	☐
1b. Mauro Ferrari	☐	☐	☐
1c. Hongbo Lu	☐	☐	☐
1d. Adeoye Olukotun	☐	☐	☐
1e. Michael S. Perry	☐	☐	☐
1f. Victoria Vakiener	☐	☐	☐
1g. William Waddill	☐	☐	☐

The Board of Directors recommends you vote FOR the following proposal:	**For**	**Against**	**Abstain**
2. Advisory Vote to Approve Executive Compensation.	☐	☐	☐

The Board of Directors recommends you vote FOR the following proposal:	**For**	**Against**	**Abstain**
3. Approval of the Arrowhead Pharmaceuticals, Inc. Amended and Restated 2021 Incentive Plan.	☐	☐	☐

The Board of Directors recommends you vote FOR the following proposal:	**For**	**Against**	**Abstain**
4. Ratification of the selection of KPMG LLP as independent auditors of the Company for the fiscal year ending September 30, 2026.	☐	☐	☐

NOTE: Such other business as may properly come before the meeting or any adjournment or postponement thereof.

Please sign exactly as your name(s) appear(s) hereon. When signing as attorney, executor, administrator, or other fiduciary, please give full title as such. Joint owners should each sign personally. All holders must sign. If a corporation or partnership, please sign in full corporate or partnership name by authorized officer.

Signature [PLEASE SIGN WITHIN BOX] Date

Signature (Joint Owners) Date

Important Notice Regarding the Availability of Proxy Materials for the Annual Meeting:

The Notice and Proxy Statement, Form 10-K are available at www.proxyvote.com

V83338-P42564

ARROWHEAD PHARMACEUTICALS, INC.
Annual Meeting of Stockholders
March 19, 2026 10:00 AM PT
This proxy is solicited by the Board of Directors

The undersigned stockholder(s) hereby appoint(s) Christopher Anzalone and Patrick O'Brien or either of them, as proxies, each with the power to appoint his substitute, and hereby authorize(s) them to represent and to vote, as designated on the reverse side of this ballot, all of the shares of Common Stock of ARROWHEAD PHARMACEUTICALS, INC. that the stockholder(s) is/are entitled to vote at the Annual Meeting of Stockholders to be held at 10:00 AM, PT on March 19, 2026, online at www.virtualshareholdermeeting.com/ARWR2026, and any adjournment or postponement thereof.

This proxy, when properly executed, will be voted in the manner directed herein and in the discretion of the proxies with respect to such other business that may properly come before the meeting and any adjournments or postponements thereof. If no such direction is made but the card is signed, this proxy will be voted in accordance with the Board of Directors' recommendations. In the event that any of the nominees named on the reverse side of this form are unavailable for election or unable to serve, the shares represented by this proxy may be voted for a substitute nominee selected by the Board of Directors.

Continued and to be signed on reverse side