Ovid® Therapeutics

2025 ANNUAL REPORT

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

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

For the Fiscal Year Ended December 31, 2025

OR

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

For the Transition Period from to

Commission File Number: 001-38085

Ovid Therapeutics Inc.

(Exact name of Registrant as specified in its charter)

Delaware	2834	46-5270895
(State or Other Jurisdiction of Incorporation or Organization)	(Primary Standard Industrial Classification Code Number)	(I.R.S. Employer Identification Number)

441 Ninth Avenue, 14th Floor
New York, New York 10001
(646) 661-7661
(Address, Including Zip Code, and Telephone Number, Including Area Code, of Registrant's Principal Executive Offices)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.001 per share	OVID	The Nasdaq Stock Market LLC

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

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

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or 15(d) of the Securities 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 the 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, 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. ☐

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). ☐

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

As of June 30, 2025, the last day of the registrant's most recently completed second fiscal quarter, the aggregate market value of the Common Stock held by non-affiliates of the registrant was approximately $22.2 million based on the closing price of the registrant's common stock on June 30, 2025. The calculation excludes shares of the registrant's common stock held by current executive officers, directors and stockholders that the registrant has concluded are affiliates of the registrant. This determination of affiliate status is not a determination for other purposes.

As of March 16, 2026, there were 131,874,634 shares of common stock outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive proxy statement for its 2026 Annual Meeting of Stockholders, which the registrant intends to file pursuant to Regulation 14A with the Securities and Exchange Commission not later than 120 days after the registrant's fiscal year ended December 31, 2025, are incorporated by reference into Part III of this Annual Report on Form 10-K.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. All statements other than statements of historical fact are "forward-looking statements" for purposes of this Annual Report on Form 10-K. In some cases, you can identify forward-looking statements by terminology such as "aim," "anticipate," "assume," "believe," "contemplate," "continue," "could," "design," "due," "estimate," "expect," "goal," "intend," "may," "objective," "plan," "positioned," "potential," "predict," "project," "should," "target," "will," "would," or the negative or plural of those terms, and similar expressions.

Forward-looking statements include, but are not limited to, statements about:

- our ability to identify additional novel compounds with significant commercial potential to acquire or in-license;
- our ability to successfully acquire or in-license additional drug candidates on reasonable terms;
- the potential use, development and therapeutic potential of the drug candidates in our pipeline;
- our ability to develop medical therapies that deliver preferable safety and tolerability profiles relative to approved drugs;
- our estimates regarding expenses, future revenue, including any royalty or milestone payments, capital requirements and needs for additional financing;
- our ability to obtain regulatory approval of our current and future drug candidates;
- our expectations regarding the timing of initiation, completion, and results and data from clinical trials and potential regulatory filings;
- our expectations regarding the potential market size for drug candidates and the rate and degree of market acceptance of such drug candidates;
- our ability to fund our working capital requirements;
- our expectations regarding the duration of our cash runway and the expectation that it will support our operations and development programs;
- the implementation of our business model and strategic plans for our business and drug candidates, including the programs in our planned pipeline;
- developments or disputes concerning our intellectual property or other proprietary rights;
- our ability to maintain and establish collaborations or obtain additional funding;
- our expectations regarding government and third-party payor coverage and reimbursement;
- our ability to compete in the markets we serve;
- the impact of government laws and regulations;
- developments relating to our competitors and our industry;
- announced or published interim topline and preliminary data;
- the impact of macroeconomic conditions and geopolitical tensions; and
- the factors that may impact our financial results.

Factors that may cause actual results to differ materially from current expectations include, among other things, those set forth in Part I, Item 1A. "Risk Factors," herein and for the reasons described elsewhere in this Annual Report on Form 10-K. Any forward-looking statement in this Annual Report on Form 10-K reflects our current view with respect to future events and is subject to these and other risks, uncertainties and assumptions relating to our operations, results of operations, industry and future growth. Given these uncertainties, you should not rely on these forward-looking statements as predictions of future events. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity, performance or achievements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

This Annual Report on Form 10-K also contains estimates, projections and other information concerning our industry, our business and the markets for certain drugs and consumer products, including data regarding the estimated size of those markets, their projected growth rates and the incidence of certain medical conditions. Information that is based on estimates, forecasts, projections or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained these industry, business, market and other data from reports, research surveys, studies and similar data prepared by third parties, industry, medical and general publications, government data and similar sources and we have not independently verified the data from third party sources. In some cases, we do not expressly refer to the sources from which these data are derived.

In this Annual Report on Form 10-K, unless otherwise stated or as the context otherwise requires, references to "Ovid," "the Company," "we," "us," "our" and similar references refer to Ovid Therapeutics Inc. and its wholly owned subsidiaries. This Annual Report on Form 10-K also contains references to our trademarks and to trademarks belonging to other entities. Solely for convenience, trademarks and trade names referred to, including logos, artwork and other visual displays, may appear without the ® or ™ symbols, but such references are not intended to indicate, in any way, that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto. We do not intend our use or display of other companies' trade names or trademarks to imply a relationship with, or endorsement or sponsorship of us, by any other companies.

PART I

Item 1. BUSINESS

Overview

Ovid is a biopharmaceutical company that is dedicated to developing small molecule medicines for brain disorders with significant unmet need. These potential medicines targeting the central nervous system ("CNS") are designed to potentially halt the course of brain disease by quelling neuronal hyperexcitation and alleviating the most impactful patient symptoms. We seek to address fundamental biological targets in the brain that are implicated in neuronal hyperexcitability, such as neurotransmitters and dysregulated ion channels. By mitigating excessive neuronal hyperexcitation with differentiated medicines, we believe we can unlock substantial scientific, therapeutic and commercial opportunities across a range of neurological and psychiatric conditions that have few therapeutic options today, both impacting patients' lives and creating long-term stockholder value.

Over the last decade, scientific understanding of the underlying biology of neuronal hyperexcitability and the related pathophysiology of epilepsies, psychoses, and other brain disorders has significantly advanced. Today, the underpinnings of many monogenic epilepsies and seizure disorders are becoming better understood and can be linked to mutations in specific molecular transporters, ion channels and receptors. Additionally, science is beginning to illuminate the systemic and damaging effects of over-excitation on neuronal networks, including its relationship to cell inflammation, stress and apoptosis.

This improved understanding of the genesis and pathophysiology of disease, coupled with advances in preclinical research tools, is enhancing the predictive potential of translational research, and thereby improving the probability of successful clinical development of CNS medicines. Additionally, emerging scientific evidence suggests that hyperexcitability of neurons is implicated in a broad range of conditions well beyond seizures and psychoses. Therefore, ameliorating excessive neuronal hyperexcitability may offer therapeutic relevance and applications in a broad array of brain disorders, including certain psychiatric, neurodegenerative and neurodevelopmental conditions, and other brain traumas.

Despite the scientific advances mentioned above, the unmet need for people living with seizures, epilepsies, and psychiatric diseases remains significant. Relatively few therapeutics utilizing new mechanisms of action ("MoAs") have been approved in recent decades, and most patients are not 'cured' of these conditions. Therefore, the need and opportunity for medicines that act upon fundamental biological targets that impact neuronal excitatory/inhibitory balance remain substantial.

Our vision and focus

Our vision is to create sustained long-term value by advancing a differentiated pipeline of small molecule medicines intended to culminate in a fully integrated neurotherapeutics company with multiple clinical-stage programs and commercial medicines. We believe the lack of new classes of medicine in neurology and neuropsychiatry represents both an unmet need and a significant opportunity.

Through our research and development ("R&D") and business development strategies, we have in-licensed and clinically developed potential medicines that may offer first-in-class or best-in-class MoAs. Our programs are intended to act upon fundamental biological targets associated with neuronal hyperexcitability in a mechanistically precise fashion. It is our intent to eventually market a franchise of unique medicines that mitigate patient symptoms, such as seizures and psychoses, that stem from hyper-excited neurons. Over time, our efforts have resulted in five clinical-stage drug development programs, including two programs that advanced to late-stage trials. Our current clinical programs are indicated for the potential treatment of drug-resistant focal onset seizures ("FOS"), developmental and epileptic encephalopathies ("DEEs"), including tuberous sclerosis complex ("TSC") seizures and infantile spasms ("IS"), psychosis associated with Parkinson's disease and Lewy body dementia ("LBD"), and schizophrenia. Several preclinical programs are also anticipated to be advanced for other forms of psychosis and mood disorders.

Importantly, we seek to develop medical therapies that are more efficacious and/or have 'gentler' profiles for patients, meaning drugs that are intended to deliver preferable safety and tolerability profiles relative to approved drugs. Improved product profiles in neurology and psychiatry are needed, given that many patients require polypharmacy regimens that create cumulative tolerability issues and drug-drug interactions, significantly impacting quality of life and adherence to therapy.

Our near-term strategy is focused on clinical advancement of potential small molecule medicines to treat specific epilepsies and psychoses that are manifestations of neuronal excitatory/inhibitory imbalance. This cohesive scientific focus, reinforced by our deep professional experience and pipeline of differentiated assets, gives us confidence that we can succeed in our mission.

Additionally, we seek to create stockholder value by establishing multiple sources of potential revenue via clinical and commercial milestones from our pipeline, strategic collaborations and partnerships.

Unmet need and opportunity

While seizures and forms of psychosis are some of the earliest maladies documented by humanity, they remain common, and often intractable, medical conditions.

The global epilepsy and psychosis market opportunities reflect massive medical need and economic potential. Epilepsy and antipsychotic pharmacologic therapies respectively represent approximately $8 billion and $22 billion markets globally and are expected to grow. Reinforcing the scale of the commercial opportunity is the number and size of recent acquisitions of epilepsy and psychiatric medicines companies, which have been acquired for values ranging from $2.6 billion to $14.6 billion. Moreover, the regulatory environment appears increasingly amenable to new approaches in epilepsy and psychiatric conditions. For example, the U.S. Food and Drug Administration ("FDA") has encouraged developers to seek "basket labels" for single therapeutic agents that cover more than one underlying disease, such as DEE. For the first time in 50 years, a therapeutic with a novel MoA was approved in schizophrenia in 2024.

The unmet need of people affected by seizures and psychosis is substantial. Today, managing patient symptoms commonly requires chronic drug therapy, which is not curative, and may not treat the whole disease.

Unmet need in epilepsy

Approximately 50 million people globally live with epilepsy, including an estimated three million adults and half a million children in the United States.

While modern drug discovery efforts have produced more than 30 anti-seizure medications ("ASMs") over the last 100 years, a substantial number of epilepsy patients continue to experience breakthrough seizures that can cause enduring damage to the brain. Individuals who suffer from rare epilepsies may experience persistent refractory, or drug-resistant, seizures with rates ranging from 50% to 90%. The seizures they suffer can have a devastating impact both upon patients and their families, by triggering permanent motor, cognitive and developmental delays. Some patients with DEEs experience even greater rates of refractory seizures.

With an estimated 70% of epilepsy diagnoses occurring in people younger than 20 years of age, the need to treat seizures early and effectively is critical to mitigate worsening and permanent later-life disabilities. In the search for seizure control, approximately half of patients take a polypharmacy regimen of five or more ASMs, requiring careful management of drug side effects and interactions. The large population of patients requiring multiple drug therapies to control seizures, as well as persistent rates of breakthrough seizures, signal the urgent need for effective new medicines. For these patients, MoAs that demonstrate improved efficacy, safety and tolerability profiles are optimal, as they may be more easily incorporated into existing treatment regimens.

Unmet need in psychosis and mood disorders

Current standards of care in psychosis rely largely on modulation of dopaminergic and serotonergic pathways. While these approaches provide benefit for some patients, they often fail to adequately control symptoms and carry tolerability burdens. We believe potassium-chloride cotransporter 2 ("KCC2") modulation represents a differentiated, upstream mechanism designed to restore physiologic inhibitory tone and reestablish network stability, with the potential to improve both efficacy and safety outcomes.

While Ovid's KCC2 direct activator portfolio has potential applications across a range of psychoses and mood disorders, the focus for our first oral program is psychosis associated with Parkinson's disease and LBD. These populations share a common pathological link, which is intraneuronal accumulation of alpha-synuclein, which leads to neurodegeneration.

Today, there are more than 4.3 million people globally living with psychosis associated with Parkinson's disease and LBD. In the United States LBD is the second most common dementia (psychoses affect 80% of the LBD population), affecting 1.5 million Americans, and psychosis associated with Parkinson's disease impacts approximately another one

million Americans. These progressive syndromes are characterized clinically by movement disorders, cognitive impairment, sleep dysregulation and autonomic instability. Changes in perception are common, manifesting in visual illusions, misperceptions of visual stimuli, and hallucinations, which can impact health and quality of life.

These conditions lead to high morbidity, mortality and healthcare costs. Psychosis in these populations is also a high risk factor for hospitalization and nursing home placement. Atypical antipsychotics are contraindicated for psychosis associated with Parkinson's disease and LBD as they may worsen motor features, their anti-dopaminergic pharmacology is untenable in Parkinson's disease, and many carry a black box warning for mortality.

The current standard of care for psychosis related to Parkinson's disease is Nuplazid® (pimavanserin), to which only a small proportion of patients fully respond.

Differentiated potential first-in-class or best-in-class programs

The science underlying the discovery and development of new drugs for the brain has changed fundamentally over the last decade. We believe that major developments in the understanding of the biology of these diseases now make it possible to address key areas of unmet need, including many neurological and psychiatric disorders, offering significant therapeutic potential.

We are specifically cultivating a pipeline of potential first-in-class or potential best-in-class MoAs to treat the underlying causes of neuronal imbalance which can lead to manifestations such as seizures, psychosis, schizophrenia, and mood disorders. Collectively, our differentiated pipeline has produced multiple potential value-creating drug programs. Several of these therapeutic development programs — OV329, OV4071 and other KCC2 activators — aim to affect signaling and enzymatic pathways that modulate hyperexcitability of neurons.

Through our scientific expertise and strategic business development, we have performed translational research and in-licensed compounds to build a robust development pipeline of potential medicines, including:

- **OV329, a highly potent next-generation GABA-aminotransferase inhibitor ("GABA-AT").** OV329 is intended to deliver preferable seizure reduction, safety profile and dosing relative to prior medicines in the class, which have known safety challenges. OV329 is an oral therapy intended to optimally regulate GABA, the inhibitory neurotransmitter. We announced the results of the OV329 Phase 1 study which evaluated safety, tolerability, and pharmacokinetics ("PK"), as well as biomarkers that offer insight into target engagement, pharmacodynamic ("PD"), and clinical effects. OV329 demonstrated a favorable safety profile in healthy subjects and was well-tolerated with no serious adverse events ("SAEs") reported. Biomarker data suggest encouraging directional signs of target engagement and clinical effects as measured by magnetic resonance spectrometry ("MRS") and transcranial magnetic stimulation ("TMS"). These indicate signs of increased GABAergic activity consistent with the intended mechanism of action by inhibiting GABA-AT.
    - *New data from an additional OV329 Phase 1 cohort shows continued differentiated safety and tolerability profile:*
        - 7 mg SAD and MAD cohort (n=11) shows all adverse events reported as unrelated, mild and transient, no SAEs and continued clean ocular safety profile, with a predictable PK effect
        - Regulatory discussions underway in support of planned Phase 2 patient studies
    - *Launching additive studies to expand OV329 into TSC and IS, areas where GABA-AT inhibition has been shown as a validated mechanism and OV329 potentially offers a differentiated safety profile in comparison to current standard of care, enabling earlier and longer use*
        - In TSC, POC safety and signal-finding study to initiate as early as Q4 2026
        - In IS, infant formulation and enabling studies are ongoing
        - Studies are going to be run in parallel to FOS program
- **KCC2 library, a portfolio of potential first-in-class direct activators of potassium-chloride cotransporter 2.** KCC2 is a fundamental biological target, solely expressed in the CNS, that enables synaptic inhibition. KCC2 dysregulation has been implicated in a broad range of neuropathologies. We are actively advancing multiple unique drug development programs that emerged from our KCC2 direct activator library containing more than 100 molecules. We were the first to report data in humans from a KCC2 direct activator from our intravenous (IV) program, OV350, a first-in-human KCC2 direct activator. Results of the Phase 1 first-in-human study of OV350 showed no treatment-related laboratory findings, no safety findings, and no treatment-related SAEs. The PK were

as predicted, and will inform dosing strategies for future KCC2 development programs. These data support development of the Company's oral direct activator programs, which are the focus of future development efforts. The KCC2 direct activator pipeline includes OV4071 and other undisclosed molecules. Each program has potential distinct therapeutic and potency characteristics as demonstrated in varying phenotypic screens and animal disease models, reflecting multiple therapeutic opportunities and optionality for co-development. The portfolio is being evaluated for a range of therapeutic indications that have symptoms associated with psychoses and mood disorders.

- **OV4071,** a first-in-human oral KCC2 direct activator approved for clinical trial initiation. OV4071 is the most advanced program in the KCC2 library. We intend to initiate a Phase 1 clinical study in the second quarter of 2026 following receipt of Human Research Ethics Committee (HREC) approval and acknowledgement of our Clinical Trial Notification (CTN) from the Therapeutic Goods Administration (TGA). The Phase 1 clinical trial will study multiple dose levels in an ascending single and multiple dose trial.
    - As part of the OV4071 clinical development plan, Ovid intends to conduct a ketamine challenge study in mid-2026 to further characterize potential PD effects and establish proof-of-mechanism.
- Collectively, these development programs and other KCC2 direct activators in preclinical studies are expected to generate a range of value-creating milestones for investors in the near- and mid-term.

R&D strategy: Differentiated MoAs to precisely target the causes of neuronal hyperexcitability

Our R&D strategy is dedicated to designing medicines that can ameliorate excessive neuronal excitation and return neurons to a state of homeostasis, or electrophysiological "balance." Many factors can contribute to neuronal hyperexcitability, including those that are intrinsic or extrinsic to the cell. Extrinsic factors can include an imbalance or dysfunction of neurotransmitters, traumas, and infections. Other factors are intrinsic to the neuron (e.g., genetic conditions or the disruption of neuronal metabolism). Whatever its origin, electrophysiological imbalance and resultant neuronal hyperexcitability manifests in a range of debilitating symptoms, including seizures and psychiatric symptoms such as psychosis, behavioral, mood disorders and more. Such symptoms are prevalent across a range of diagnoses, including: epilepsies, neurodegenerative diseases and neurodevelopmental disorders. Therefore, drugging targets that lead to excessive neuronal excitation may have widespread therapeutic utility.

While some drug development companies focus exclusively on a single biological target or MoA, we believe that multiple MoAs will be necessary to effectively treat the heterogeneous causes of hyperexcitability. Accordingly, our pipeline seeks to curate and develop a unique set of molecules that can be effective as monotherapies and within the context of polypharmacy regimens. We believe this approach will create a differentiated and leading epilepsy and psychosis franchise.

Core tenets of our approach include a focus on:

- **Small molecule compounds.** Ovid's pipeline consists of small molecule programs that can potentially be delivered orally, by subcutaneous or intramuscular injection, or intravenously. The compounds we seek to develop are mechanistically designed to activate or modulate specific biological targets. We plan to take advantage of the versatility small molecules have to offer in terms of manufacturing, chemistry and dosing, with the ultimate goal of delivering medicines that can be easily taken by patients for chronic conditions.
- **Target validation and indication selection.** Our therapeutic development programs focus on precise MoAs for biological targets that are confirmed to be relevant in excessive neuronal excitation with established relevance to hyperexcitability through in vitro and in vivo animal models. Prior to advancing our drug candidates from nonclinical evaluation into clinical trials, we apply a systematic approach to de-risk molecules using emerging tools, phenotypic screens and animal disease biology models. Additionally, we prioritize targets that are either uniquely (1) expressed in the CNS, such as KCC2 cotransporters, or are (2) over-expressed in a pathological state, such as GABA-AT.
- **Prioritization of the total drug profile.** We strive to develop drug candidates that deliver therapeutic efficacy while maintaining safe and well-tolerated side effect profiles. Our preclinical and toxicology work seeks to screen for and respectively avert drug or dose dependent effects, such as sedation or drug-drug interactions, that could impact patient tolerability.

Business development strategy: Pipeline built through disciplined business development and enhanced with academic collaborations

Ovid has primarily built our pipeline through strategic business development. We identify molecules with untapped potential value and seek to in-license or enter into collaborative agreements to secure such assets and advance

clinical development. This strategy directs our efforts where we excel in creating value and shaping translational and clinical stage development. An integral part of our process is establishing collaborations with academic research centers to support translational expertise for our programs.

The multiple programs in our diversified pipeline provide optionality to pursue out-bound business development to expand our opportunities. As the pipeline progresses, we may endeavor to partner the development of our compounds in non-core indications or extend regional market rights outside the United States. We believe that we are well-positioned to execute on our business development strategy due to the extensive experience and networks of our management team. Collectively, our senior management has transacted hundreds of in-licensing deals and collaborations.

We continue to enhance and expand our pipeline via two complementary strategies: (1) internal R&D efforts in collaboration with external leaders in the field and academic collaborators; and (2) business development activities to partner with collaborators that have promising programs or assets in our chosen therapeutic areas.

Clinical development approach

We take a scientifically driven and evidence-based approach to translation and clinical development of our programs. We are building our portfolio based on the existence of known biological rationales that are associated with targets, and which can be evaluated using validated biomarkers and clear endpoints that are meaningful to patients, clinicians and regulators. Our early clinical development efforts focus on time- and cost-efficient trials that address key questions critical to de-risking future development.

Our approach is driven by the following scientific principles:

- **Clinically translatable preclinical models inform indication selection**. Recent advances in genetics and artificial intelligence enable us to employ predictive in vitro and in vivo models of specific brain diseases and symptoms. We believe these predictive disease biology models will allow us to evaluate and observe a drug candidate's potential phenotypic and therapeutic activity prior to initiation of human trials.
- **Sentinel indications.** Our drug development approach generally pursues rare, resistant conditions as initial "sentinel" indications. Pursuing rare, resistant conditions can enable us to demonstrate rapid proof-of-concept ("POC") for our compounds while potentially exploring efficient regulatory pathways and incentives. Case studies of the life cycle management for prior ASMs suggest that demonstration of refractory seizure reduction is often indicative of therapeutic effect in more common and tractable seizure types. Similarly, many ASMs were later proven to have clinical efficacy in other neurological conditions.
- **Biomarker strategies for early PD and clinical insight.** Whenever possible we integrate biomarker strategies beginning early in translational and clinical development to help identify whether our programs are achieving target engagement and having a PD effect on clinical parameters that are relevant to disease processes and manifestations. Such biomarkers can help identify whether we are achieving biologically active doses to inform patient studies. Using such biomarkers may provide early POC in clinical development and, in turn, guide capital allocation toward projects with a higher probability of later stage success.
- **Meaningful endpoints and scales.** We focus on clear, observable endpoints that are meaningful to patients, caregivers, clinicians and regulators. Our clinical development experts have extensive experience identifying and using validated scales and designing scales to measure symptoms that are common among seizure and psychiatric disorders, such as cognitive declines, movement deficiencies, hallucinations and behavioral manifestations. These skills support our ability to develop medicines that may provide clinical benefit across multiple aspects of patient health.
- **Motivated and accessible patient populations.** We seek to develop medicines for disorders with motivated and accessible patient populations. Patients and caregivers affected by intractable brain disorders have increasing access to diagnostics and genetic testing. Additionally, many are social media users, through which they learn new insights about their conditions and share relevant information and experiences. We conduct patient community outreach and activities to inform our clinical design, educate patients about trial opportunities and support efficient study enrollment.

Fit-for-purpose infrastructure

We have built a highly specialized, efficient, and focused infrastructure that supports our chosen areas of neurotherapeutics development. This infrastructure spans the critical domains of R&D and market access strategy. The scale and design of the organization reflects the needs of our pipeline programs and our status as a publicly-traded company.

We have recruited a team of 23 professionals, many with deep subject matter expertise in seizures and neurological conditions. This includes physicians, academic scientists, and commercial and biopharmaceutical industry leaders. We have three individuals with MD degrees and eight professionals with PhD degrees specializing in the sciences.

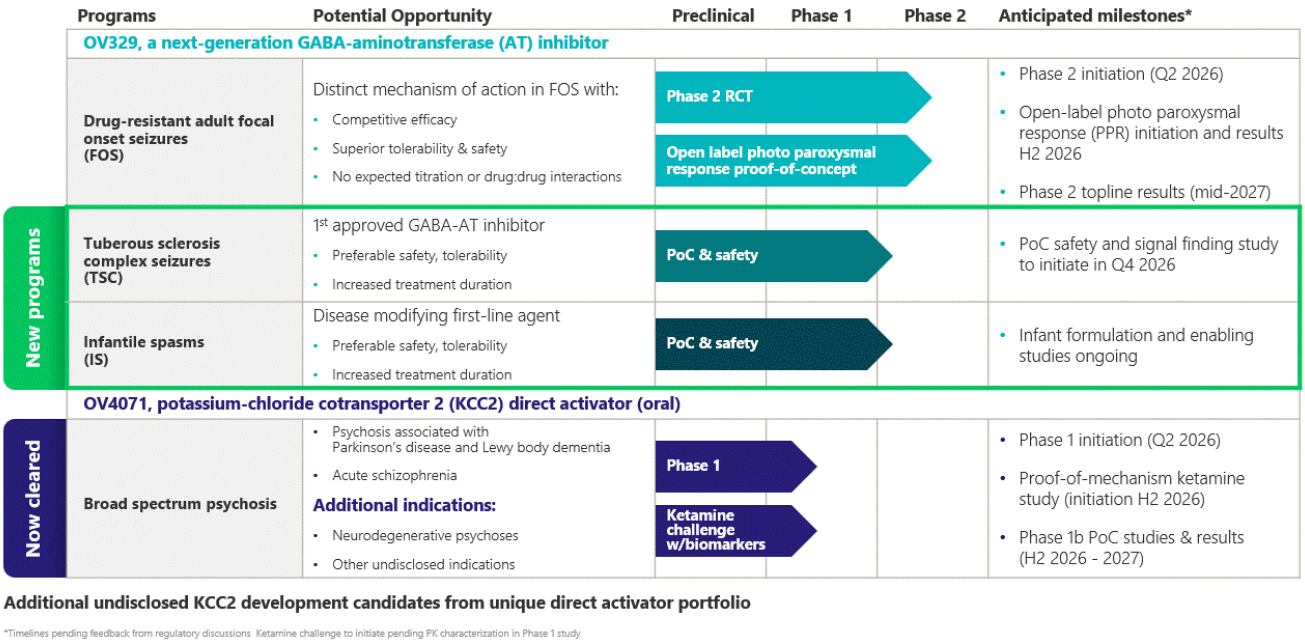
Our operational leaders have extensive experience developing, formulating, manufacturing, regulatory controls and implementing market access strategies for leading neurological medicines. In total, our team's collective professional experience has resulted in the successful development or commercial launch of more than 25 medicines, including many CNS products. Over time, we have built upon our core strength in the clinical development of medicines for anti-seizure and genetic neurodevelopmental conditions and expanded into adjacent therapeutic areas of drug development in the CNS where strong mechanistic evidence exists.

Ovid pipeline

Our efforts have already brought drug candidates from POC into late-stage patient clinical trials. Today, we are one of the few companies that has researched and developed three distinct MoAs to target seizures and we believe we are the only company that holds a portfolio of direct activators of KCC2. We believe this pipeline of potential first-in-class or best-in-class mechanisms differentiates us and provides the foundation for a productive franchise of small molecule neurotherapeutics for epilepsies, psychoses, schizophrenia and mood disorders.

The following table (Figure 1) sets forth our drug candidate programs and their development status, respective MoAs, and anticipated near-term milestones.

Figure 1. Ovid Therapeutics Pipeline



OV329 - A next-generation GABA-AT inhibitor

OV329 is a clinical-stage, next-generation GABA-AT inhibitor that we are developing for the treatment of adult and pediatric DREs. OV329 represents a potential best-in-class GABA-AT inhibitor and was designed to supplant vigabatrin ("VGB"), which is an approved therapeutic globally for the treatment of infantile spasms. VGB was a first-generation medicine that demonstrated substantial seizure reduction; however, its clinical and commercial use was limited by lack of a therapeutic window. Specifically, VGB was proven to generate deleterious and irreversible ocular effects in some patients, including retinal degradation and irreversible vision loss that led to significant post-market restrictions and monitoring.

We believe OV329 to be an improved GABA-AT inhibitor with a different chemical structure, potency, binding, PK and PD profile as compared to VGB. OV329 has been shown to deliver increased potency and efficacy in the target binding site. In preclinical research it was demonstrated to be 100-fold more potent than VGB. An oral formulation of OV329 was assessed in a Phase 1 study with multiple biomarkers to measure clinical effect and target engagement, in addition to evaluating safety, tolerability and PK. Following the successful completion of the initial cohorts, we are conducting additional higher dose cohorts of OV329 evaluating safety, tolerability and PK. We believe that OV329 has the potential to be a therapeutic option for adult DREs, including FOS, and DEEs, including TSC and IS. The data from the Phase 1 study support advancing OV329 into a Phase 2 trial.

A benefit of our OV329 program is that it acts upon a validated drug target for seizures. Specifically, it works by substantially reducing the activity of GABA-AT, a key enzyme responsible for the degradation of the brain's major inhibitory neurotransmitter, GABA. OV329 leads to increased concentrations of GABA by inhibiting its metabolism.

Given that epilepsy is characterized by excessive neuronal excitation, the increased levels of GABA may suppress this excitatory signaling and thus reduce seizures.

OV329 profile

Based upon preclinical data supporting OV329, we believe it has the potential to provide (in comparison to VGB) greater seizure reduction efficacy, improved tolerability and safety profiles without sedation, and lower dosing in comparison to existing therapeutics.

To support OV329's anti-convulsant profile, nine animal seizure models have demonstrated its seizure reducing effects (see Figure 2 below). These findings from both chronic and acute seizure models provide additional confidence about the therapeutic potential of OV329 in humans. The PD profile of OV329 is differentiated from VGB. Preclinical research has shown that OV329 induces phasic (synaptic) and tonic (extra-synaptic) inhibition of GABA-AT. This produced more GABA in the synapse and environmental milieu, potentially contributing to more durable inhibitory effects.

Figure 2. Nine preclinical animal models reaffirm OV329 seizure reduction activity, including resistant seizure models

Anti-convulsant activity demonstrated in 9 seizure models

Seizure reduction seen in chronic & acute seizure models

	i.v. (ivPTZ)	NMDA-Induced Infantile Spasm model	Audiogenic Seizure	Amygdala Kindled	Corneal Kindled	Intrahippocampal Kainate Model of Mesial-Temporal Lobe Epilepsy (MTLE)	Intraamygdala Kainate Model of Mesial-Temporal Lobe Epilepsy (MTLE)	Lithium-Pilocarpine	Dravet *Scn1a* $^{A1783V/WT}$
Injury Model	Acute/ seizure	Acute/ seizure	Acute	Chronic/ epilepsy	Chronic/ epilepsy	Chronic/ epilepsy	Epilepsy prevention/ modification	Acute/ seizure	Chronic/ Genetic epilepsy
Clinical Correlate	Nonconvulsive Seizures (e.g., absence, myoclonic)	Infantile spasms	Generalized seizures	Chronic focal to bilateral tonic-clonic seizure/ Pharmacoresistant seizures	Chronic focal to bilateral tonic-clonic seizure	Focal Mesial temporal lobe epilepsy/ Pharmacoresistant seizures; Status Epilepticus	Focal Mesial temporal lobe epilepsy/ Pharmacoresistant seizures; Status Epilepticus	Like human, rodents exhibit EEG abnormalities, convulsions, and cognitive impairment	Spontaneous seizures, higher rate of SUDEP. Hyperthermia-induced Pharmcoresistant seizures.
Species	Rat	Mouse	Mouse	Rat	Mouse	Mouse	Mouse	Rat	Mouse
Dosing	Acute (5, 20, 40 mg/kg i.p.)	Acute (0.0025, 0.01, 0.1, 1 mg/kg, p.o.)	Acute (0.01, 0.05, 0.1 mg/kg, p.o.)	Acute (30, 40 mg/kg, i.p.)	Acute (1, 3, 10, 20, 30, 40, 60 mg/kg, p.o.)	Acute, single dose (0.01, 0.1, 1, 10 mg/kg, p.o.; 10 mg/kg, i.p.) Subacute (8 days q.d.) 0.3, 1.0 and 3.0 mg/kg/day (p.o.)	Acute (40 mg/kg p.o.)	Acute (15mg/kg, IV)	Repeat (10mg/kg x 4d, IP)
Activity	+	+	+	+	+	+	+	+	+

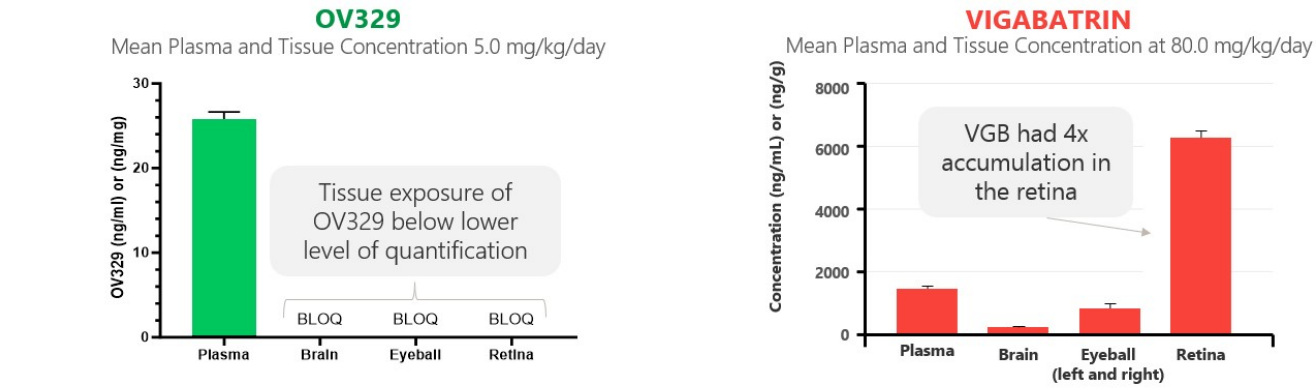
OV329 safety profile

To date, OV329 has been well tolerated in humans in our Phase 1 study. There have been no treatment-related serious adverse events reported, and only mild and transient treatment-related adverse events reported, such as headache. Additionally, to characterize OV329's potential safety profile relative to VGB, our preclinical efforts sought to extensively study safety and tolerability, including any potential ocular changes.

We have demonstrated that the tissue clearance of OV329 is rapid, which when coupled with its potency and irreversible binding, leads us to believe that the accumulation in the back of the eye does not occur as it does with VGB, which has a longer half-life.

We presented results at the American Epilepsy Society meeting of a head-to-head animal study evaluating whether OV329 could be found to accumulate in mouse retinas and brains, as has been previously shown to occur with VGB. The preferential accumulation of VGB in the eye is thought to be a contributing factor in VGB's ocular toxicity. The findings (summarized in Figure 3 below), were that OV329 cleared and remained undetectable in the retinas, eyes, and brains of mice after 48 hours of continuous exposure via a sub-cutaneous osmotic pump, suggesting a lack of accumulation. In contrast, ocular accumulation of VGB was confirmed within this period. These results replicate previously published findings that indicate VGB preferentially and rapidly accumulates within mouse tissue and plasma, including retina, visual cortex, and brain at sub-therapeutic doses (70 mg/kg). In contrast, a therapeutic dose of OV329 in animals (5 mg/kg) did not show signs of ocular accumulation in the same study design.

Figure 3. OV329 clears brain and eye tissue rapidly and does not accumulate like VGB



FINDINGS[1]

- OV329 was present in the plasma and then cleared the tissue
- No accumulation detected of OV329 in the eye or retina
- 4x greater exposure of VGB in retina as compared to plasma
- Suggests vigabatrin, but not OV329, preferentially partitions into retina when plasma exposure is kept at a relatively constant level

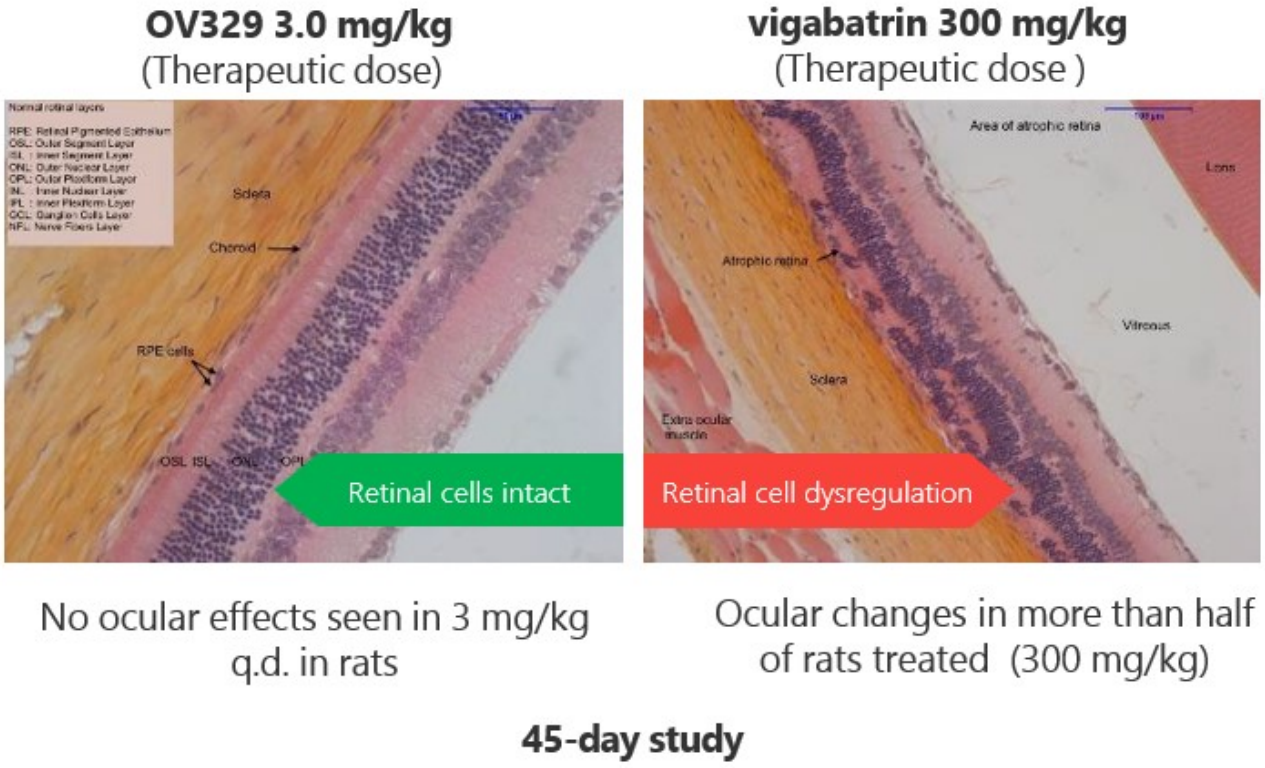
[1]Tsai, J., et al. (2024). *Evaluation of the Potential Accumulation of OV329 in the Brain, Retina, and Eye Following Continuous Infusion.* Poster presented at the 2024 Epilepsy Pipeline Conference

These results complement previously presented studies which showed that therapeutic doses of OV329 (3 mg/kg) did not result in retinal tissue pathology at 45 days in Sprague-Dawley rats, an animal model that investigates structural and functional ocular toxicity (see Figure 4). In contrast, VGB did show retinal cell degradation at the therapeutic dose in animals of 300 mg/kg at 45 days.

We applied a clinically translatable rodent model of albino Sprague-Dawley rats to determine if any ocular changes could be observed associated with the predicted therapeutic doses of OV329 and VGB, as compared to placebo. This rodent model is an accepted proxy by the FDA for the ocular effects seen in humans treated with VGB. Figure 4 (below) demonstrates the results of our research.

After 45 days of dosing with the therapeutic dose of VGB and an expected therapeutic dose of OV329 (3 mg/kg), the model showed no ocular effect in animals taking OV329, whereas disruption in retinal cells was seen in animals taking the therapeutic dose of VGB (300 mg/kg). In this short-term model, OV329's ocular profile appears similar to placebo, and no disruption to the retina was seen at the anticipated therapeutic dose. These models must be confirmed in human studies, though they lead us to believe that OV329 may offer significant seizure reduction benefit with a therapeutic window not provided by VGB.

Figure 4. No ocular changes seen in rodents treated with expected therapeutic dose of OV329 (3 mg/kg)



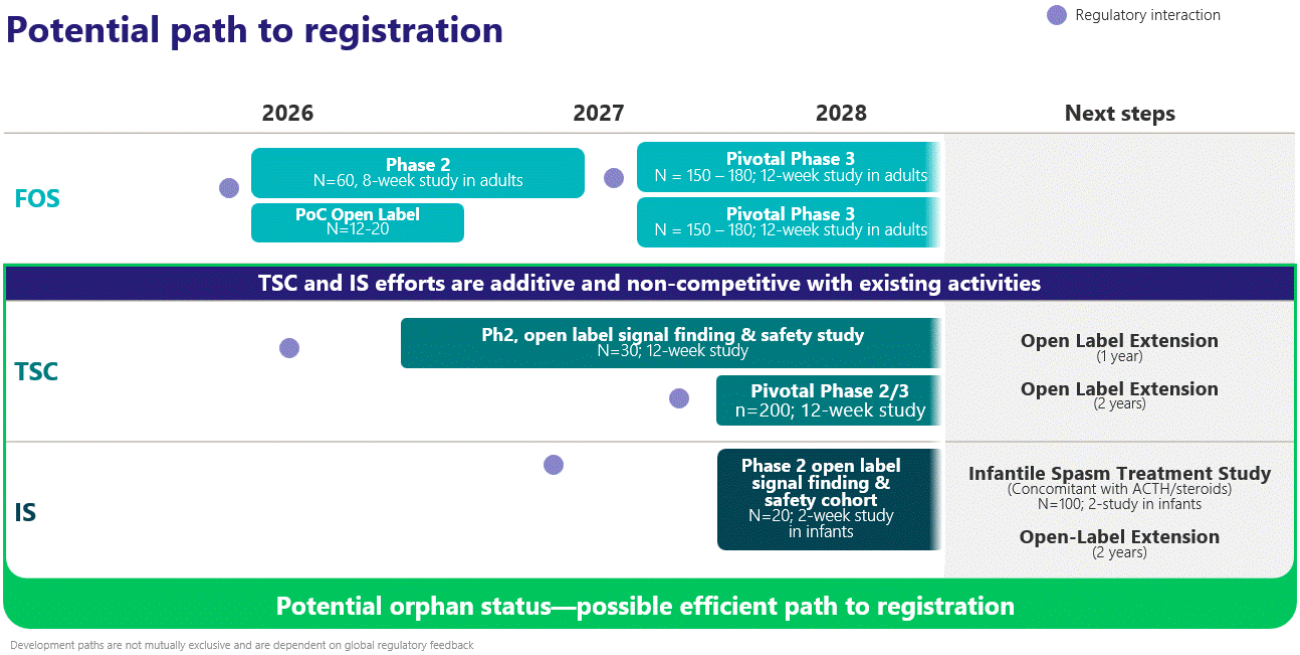
Human studies and biomarker strategy

We announced topline results of our Phase 1 trial of OV329 in the third quarter of 2025 and presented these data at the 2025 American Epilepsy Society Meeting. The Phase 1 trial evaluated PK profile, safety, tolerability and target engagement associated with escalating doses of OV329 in healthy volunteers. Two surrogate biomarkers, TMS and MRS, were included as exploratory biomarkers in the study to measure a corollary for clinical biological effect and target engagement. Biomarker data suggest encouraging directional signs of target engagement and clinical effects as measured by the utilized biomarkers. These findings indicate signs of increased GABAergic activity consistent with the intended mechanism of action by inhibiting GABA-aminotransferase. Previous studies have reported that MRS measurement of GABA concentration levels increase following treatment with GABA-AT inhibitors, which has been shown to correlate with seizure reduction efficacy in existing GABA-AT inhibitor programs. These metrics coupled with safety, data and pharmacokinetic data may inform mid- to late-stage development of the program. OV329 will be further studied for the potential treatment of DREs, including FOS.

We announced that we will be pursuing additional POC studies in TSC and IS, which will be run in parallel to our program in FOS. OV329 has the potential to have differentiated safety profile as compared to current standard of care; enabling earlier and longer use. Our confidence in these programs comes from validated novel MOA, which offers the transformative potential to alter the course of disease. These additional indications open a large opportunity for OV329, as it has a differentiated safety and tolerability profile, along with robust target engagement and potential flexibility of use with no anticipated titration and no drug-drug interactions. Preclinical data support OV329's potential efficacy with compelling anti-convulsant effects in a mouse model of infantile spasm, and in animal models of focal seizures. Importantly, we have a robust body of clinical and preclinical data demonstrating that OV329 does not accumulate in the retina, greatly improving upon the ophthalmic risk associated with VGB.

OV329 Development Timelines

With the addition of TSC and IS, we have expanded an additional path to registration, and have also supplemented a catalyst-rich pipeline that potentially unlocks the full value of OV329.



KCC2 direct activator portfolio

In late 2021, we in-licensed a portfolio of more than 100 molecules from AstraZeneca AB ("AstraZeneca"), which are direct activators of KCC2. Since that time, we have extensively characterized the library for bioavailability, formulation amenability and therapeutic potential. As a result, we now have three unique programs that we intend to successively progress into human clinical studies. These include OV4071 and other undisclosed programs. Based upon our phenotype screens, disease model studies, and published evidence, we believe this portfolio offers broad therapeutic potential in a range of brain disorders and symptoms including psychosis, schizophrenia, other behavior and mood disorders, and seizures. This may include neurodegenerative and neurodevelopmental diseases that exhibit the above mentioned symptoms. Given the broad therapeutic relevance of KCC2 in many brain disorders, it is our desire to unlock the full value of KCC2 for stockholders and patients.

KCC2: A fundamental target in the CNS

KCC2 is a fundamental biological target expressed exclusively in the CNS and is central to maintaining synaptic inhibition. Hundreds of publications link KCC2 dysregulation directly or indirectly to various medical conditions and symptoms associated with excessive neuronal excitation. KCC2 is an ion cotransporter that regulates chloride extrusion in neurons. A functioning chloride gradient is essential to GABA being inhibitory in neuronal synapses. By directly activating KCC2, our development programs seek to restore GABAergic inhibition and bring hyper-excited neurons into homeostasis.

We believe that our KCC2 portfolio represents the only small molecule library within the broad biopharmaceutical industry that directly activates this unique ion cotransporter. Other companies have attempted to activate KCC2; to our knowledge, no others have been successful. However, some may potentiate the cotransporter, which will likely have different clinical effects.

KCC2 direct activator library

Our KCC2 direct activator portfolio includes two programs in active characterization and development, which we believe are suitable for pharmaceutical development. We have characterized and translated multiple candidates from the KCC2 portfolio for development in epilepsy as well as other possible neurological conditions associated with psychiatric, neurodegeneration and neurodevelopmental disorders. Phenotypic screens and confirmatory animal disease models suggest that our programs, including: OV4071 and other undisclosed molecules, have potential therapeutic properties associated with antipsychotic, anxiolytic and anticonvulsant response. We have also determined that the unique molecules in the library are amenable to a range of formulations, including intravenous, oral and intramuscular injections.

Over the next three years, we anticipate filing regulatory submissions for human trials annually for successive programs emerging from our KCC2 portfolio. As noted above, these programs represent opportunities for in-house

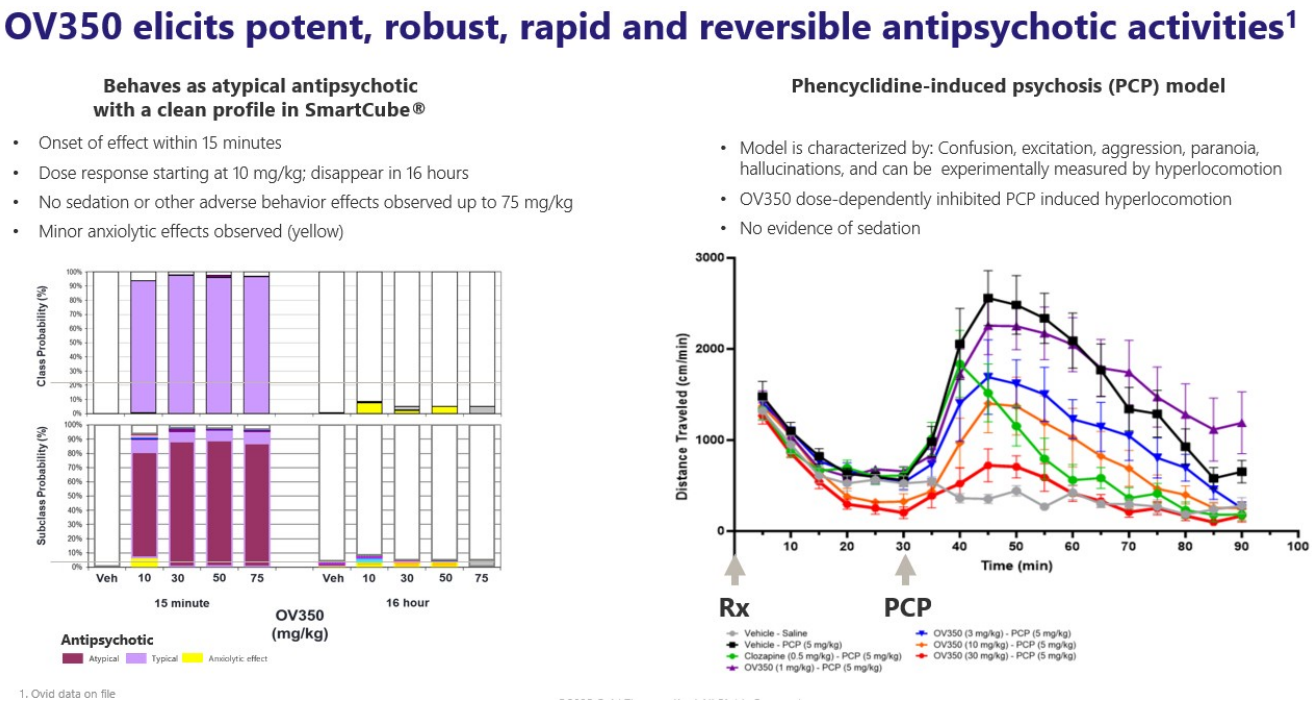
development, co-development or out-licensing. The library of early-stage small molecules that target KCC2, including OV4071, are included in a pending composition-of-matter application that was filed globally and, if issued, will expire in 2041 excluding any potential regulatory extensions.

OV4071

In 2025, we completed first-in-human studies of OV350 IV, for which topline data results were announced in the fourth quarter. The data demonstrate a favorable safety profile, no treatment-related SAEs, and indicate central activity and spectral power consistent with expected physiological effects of KCC2 modulation aligned with potential drug exposure in the brain. In 2025, we elected to prioritize and direct our capital resources to accelerate chronic (oral) formulations of our oral direct activator programs, including OV4071. Accordingly, we do not intend to advance OV350 further in the clinic. We believe the results from OV350 support the advancement of our portfolio of KCC2 direct activators, including OV4071. We intend to initiate a Phase 1 clinical study of OV4071 in the second quarter of 2026 following receipt of HREC approval and acknowledgment of our CTN from the TGA. The Phase 1 clinical trial will study multiple dose levels in as ascending single and multiple dose trial.

As noted above, these conditions have deep unmet patient need and are underserved by the current standard of care. Furthermore, in vivo POC studies have established that restoring KCC2 activity reduced psychotic behaviors in animals (see Figure 5 below). Preclinical mechanistic studies have also demonstrated that OV4071 was well-tolerated and did not induce sedation.

Figure 5. OV350 demonstrates antipsychotic effects in schizophrenia model



License and Collaboration Agreements

Research Collaboration and Equity Investment in Gensaic (2022)

Under the terms of an equity agreement, we invested a total of $5.1 million in exchange for convertible preferred stock in Gensaic, Inc. ("Gensaic"). Dr. Jeremy M. Levin, our Executive Chairman, is a director of Gensaic. We also entered into a collaboration agreement with Gensaic (the "Gensaic Collaboration Agreement") to potentially develop up to three genetic medicines for neurological indications of interest to us, harnessing Gensaic's proprietary tissue-selective intracellular delivery platform. Gensaic retains full rights to its platform technology. We will have commercial rights to license and develop any resulting therapies and delivery technologies that emerge from this collaboration subject to agreed-upon terms. We also retained rights to invest in future equity financing rounds. In January 2026, the Gensaic Collaboration Agreement was amended to allow for identification of a new research project target in order to utilize the remaining prepaid funds from the original agreement, with additional immaterial provisions to the agreed-upon terms.

Royalties Associated with Marinus Out-License Agreement (2022)

In March 2022, we entered into an exclusive patent license agreement with Marinus Pharmaceuticals, Inc. ("Marinus") ("Marinus License Agreement"). Under the Marinus License Agreement, we granted Marinus an exclusive, non-transferable (except as expressly provided therein), royalty-bearing right and license under certain Ovid patents relating to ganaxolone to develop, make, have made, commercialize, promote, distribute, sell, offer for sale and import licensed products in the territory (which consists of the United States, the European Economic Area, United Kingdom and Switzerland) for the treatment of CDKL5 deficiency disorder. Following the date of regulatory approval by the FDA of the first licensed product in the territory, which was received on March 18, 2022, Marinus issued, at the Company's option, 123,255 shares of Marinus common stock, par value $0.001 per share. The Marinus License Agreement also provided for payment of royalties from Marinus to us in single digits on net sales of each such licensed product sold. In January 2025, Marinus was acquired by Immedica Pharma, S.A. ("Immedica"). In June 2025, we entered into an amendment to the Marinus License Agreement and received $7.0 million in cash to replace ongoing royalty payment obligations on sales of ganaxolone, as well as the right to add additional patents to the licensed portfolio within the following six months. In December 2025, the right to add additional patents to the licensed portfolio lapsed without any additions.

Exclusive In-Licensing Agreement with AstraZeneca (2021)

In December 2021, we entered into an exclusive license agreement ("AstraZeneca Exclusive License Agreement") with AstraZeneca. Under the terms of the AstraZeneca Exclusive License Agreement, we have obtained worldwide rights to a portfolio of early-stage, small molecule compounds targeting the KCC2 transporter, including our lead compound, OV4071. In exchange for an upfront payment of $5.0 million in cash and $7.3 million in shares of our common stock to AstraZeneca, we are responsible for using commercially reasonable efforts to carry out all future development and commercialization of KCC2 transporter activators in epilepsies and potentially other neuropathic conditions. We are obligated to pay AstraZeneca potential clinical development milestones of up to $8.0 million, regulatory milestones of up to $45.0 million and total commercial milestones of up to $150.0 million, as well as tiered royalty payments ranging from the single digits up to ten percent on net sales. At the time of proof of clinical efficacy, AstraZeneca will have the right of first negotiation to opt in to co-develop and co-commercialize KCC2 transporter activators with Ovid. The license option will continue until the expiration of all relevant royalty terms.

Northwestern University License for OV329 (2016)

In December 2016, we entered into a license agreement ("Northwestern Agreement") with Northwestern University ("Northwestern"), pursuant to which Northwestern granted us an exclusive, worldwide license to patent rights in certain inventions ("Northwestern Patent Rights") which relate to a specific compound (OV329) and related methods of use for such compound, along with certain know-how related to the practice of the inventions claimed in the Northwestern Patent Rights. Under the Northwestern Agreement, we were granted exclusive rights to research, develop, manufacture and commercialize products utilizing the Northwestern Patent Rights for all uses, other than cancer.

Upon entry into the Northwestern Agreement, we paid an upfront non-creditable one-time license issuance fee of $75,000, and we are required to pay an annual license maintenance fee of $20,000, which will be creditable against any royalties payable to Northwestern following first commercial sale of licensed products under the agreement. We are responsible for all ongoing costs of filing, prosecuting and maintaining the Northwestern Patent Rights, but we also have the right to control such activities using our own patent counsel. In consideration for the rights granted to us under the Northwestern Agreement, we are required to pay to Northwestern up to an aggregate of $5.3 million upon the achievement of certain development and regulatory milestones for the first product covered by the Northwestern Patent Rights, and, upon commercialization of any such products, will be required to pay to Northwestern a tiered royalty on net sales of such products by Ovid, its affiliates or sublicensees, at percentages in the low to mid-single-digits, subject to standard reductions and offsets. Our royalty obligations continue on a product-by-product and country-by-country basis until the later of the expiration of the last-to-expire valid claim in a licensed patent covering the applicable product in such country and ten years following the first commercial sale of such product in such country. If Ovid sublicenses a Northwestern Patent Right, it will be obligated to pay to Northwestern a specified percentage of sublicense revenue received by us, ranging from the high single-digits to the low-teens.

The Northwestern Agreement requires that we use commercially reasonable efforts to develop and commercialize at least one product that is covered by the Northwestern Patent Rights. Unless earlier terminated, the Northwestern Agreement will remain in force until the expiration of our payment obligations thereunder. We have the right to terminate the agreement for any reason upon prior written notice or for an uncured material breach by Northwestern. Northwestern may terminate the agreement for our uncured material breach or insolvency.

License Agreement with H. Lundbeck A/S (2015)

In March 2015, we entered into a license agreement with H. Lundbeck A/S ("Lundbeck"), which we subsequently amended in May 2019, and July 2020 (collectively, the "Lundbeck Agreement"). As part of the Lundbeck Agreement, we obtained from Lundbeck an exclusive (subject to certain reserved non-commercial rights), worldwide license to develop,

manufacture and commercialize OV101, also known as gaboxadol. We subsequently closed our OV101 (gaboxadol) program in Angelman syndrome in early 2021.

Sales and Marketing

Given our stage of development, we have not yet established commercial and distribution infrastructures. However, we do have internal market access and commercial capabilities that inform our pipeline strategy and execution. As our pipeline assets advance in the clinic, we intend to build focused capabilities to commercialize our programs. In markets for which commercialization may be less capital efficient for us, we may selectively pursue strategic collaborations with third parties in order to maximize the commercial potential of our drug candidates.

Manufacturing and Supply

We currently outsource all manufacturing, and intend to continue utilizing collaborators and contract manufacturers for the foreseeable future. However, members of our management have broad experience in manufacturing, which we believe may provide a competitive advantage.

Competition

The fields of epilepsy, including FOS and DEEs such as TSC and IS, and psychiatric medicines are highly fragmented. There is no one direct competitor, though there are others in the field of epilepsy who market to similar indications as we may explore. Those include: UCB, Jazz Pharmaceuticals plc, SK Biopharmaceuticals Inc., Harmony Biosciences, and Xenon Pharmaceuticals, Inc. These are our most direct competitors with respect to OV329. Our KCC2 programs are potentially intended for the treatment of psychosis associated with Parkinson's disease and LBD, schizophrenia, and mood disorders. The only existing medicine indicated for psychosis associated with Parkinson's disease and LBD is marketed by Acadia Pharmaceuticals Inc. We are aware of one other company seeking to develop KCC2 potentiators, which is Axonis Therapeutics, Inc.

Drug development is highly competitive and subject to rapid and significant technological advancements. Our ability to compete will significantly depend upon our ability to complete necessary clinical trials and regulatory approval processes, and effectively market any drug that we may successfully develop. Our current and potential future competitors include pharmaceutical and biotechnology companies, academic institutions and government agencies. The primary competitive factors that will affect the commercial success of any drug candidate for which we may receive marketing approval include efficacy, safety and tolerability profile, dosing convenience, price, coverage and reimbursement. Many of our existing or potential competitors have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of drug candidates, as well as in obtaining regulatory approvals of those drug candidates in the United States and in other regions.

Our current and potential future competitors also have significantly more experience commercializing drugs that have been approved for marketing. Mergers and acquisitions in the pharmaceutical and biotechnology industries could result in even more resources being concentrated among a small number of our competitors.

Accordingly, our competitors may be more successful than us in obtaining regulatory approval for therapies and in achieving widespread market acceptance of their drugs. It is also possible that the development of a cure or more effective treatment method for the disorders we are targeting by a competitor could render our current or future drug candidates non-competitive or obsolete or reduce the demand for our drug candidates before we can recover our development and commercialization expenses.

Intellectual Property

Our commercial success depends in part on our ability to obtain and maintain proprietary protection for our current and future drug candidates, novel discoveries, product development technologies and know-how, to operate without infringing on the proprietary rights of others, and to prevent others from infringing upon our proprietary rights. Our policy is to seek to protect our proprietary position by, among other methods, filing or in-licensing U.S. and foreign patents and patent applications related to technology, inventions and improvements that are important to the development and implementation of our business. We also rely on trademarks, trade secrets, copyright protection, know-how, continuing technological innovation and potential in-licensing opportunities to develop and maintain our proprietary position.

While we seek broad coverage under our existing patent applications, there is always a risk that an alteration to the product or process may provide sufficient basis for a competitor to avoid infringement claims. In addition, the coverage claimed in a patent application can be significantly reduced before a patent is issued and courts can reinterpret patent scope after issuance. Moreover, many jurisdictions, including the United States, permit third parties to challenge issued patents in administrative proceedings, which may result in further narrowing or even cancellation of patent claims. We cannot

provide assurance that any patents will be issued from our pending or future applications or that any potentially issued patents will adequately protect our intellectual property.

We exclusively licensed a portfolio of issued U.S. and international patents from Lundbeck directed to polymorphic forms of OV101 and their preparation and methods of manufacturing OV101. We have also filed, and own, multiple patent families directed to methods of treatment and formulations with OV101. Subsequently, we have licensed much of this portfolio to Healx Ltd, and are not further developing OV101.

OV329 was in-licensed from Northwestern. OV329's composition of matter patent expires in 2036, excluding regulatory extensions. We have also filed, and own, multiple patent families involving the synthesis of OV329 and methods of treatment with OV329.

A library of early-stage small molecules that target KCC2 was in-licensed from AstraZeneca. The molecules are included in a pending composition-of-matter application that was filed globally and, if issued, will expire in 2041, excluding any potential regulatory extensions. We have also filed, and own, multiple patent families directed to methods of treatment with KCC2 direct activation.

We continue to expand our intellectual property portfolio to protect our library of potential development candidates. Individual patents extend for varying periods depending on the date of filing of the patent application or the date of patent issuance and the legal term of patents in the countries in which they are obtained. Generally, utility patents issued for applications filed in the United States are granted a term of 20 years from the earliest effective filing date of a non-provisional patent application. In addition, in certain instances, a patent term can be extended to recapture a portion of the U.S. Patent and Trademark Office (the "USPTO") delay in issuing the patent as well as a portion of the term effectively lost as a result of the FDA regulatory review period. However, as to the FDA component, the restoration period cannot be longer than five years and the total patent term including the restoration period must not exceed 14 years following FDA approval. The duration of foreign patents varies in accordance with provisions of applicable local law, but typically is also 20 years from the earliest effective filing date. The actual protection afforded by a patent may vary on a product-by-product basis, from country to country and can depend upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory-related extensions, the availability of legal remedies in a particular country and the validity and enforceability of the patent.

Furthermore, we rely upon trade secrets and know-how and continuing technological innovation to develop and maintain our competitive position. We seek to protect our proprietary information, in part, using confidentiality agreements with our employees and consultants and any potential commercial partners and collaborators, as well as invention assignment agreements with our employees. We also have or intend to implement confidentiality agreements or invention assignment agreements with our selected consultants and any potential commercial partners. These agreements are designed to protect our proprietary information and, in the case of the invention assignment agreements, to grant us ownership of technologies that are developed through a relationship with a third party. These agreements may be breached, and we may not have adequate remedies for any such breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our commercial partners, collaborators, employees and consultants use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

Our commercial success will also depend in part on not infringing upon the proprietary rights of third parties. It is uncertain whether the issuance of any third-party patent would require us to alter our development or commercial strategies, or our drugs or processes, obtain licenses or cease certain activities. Our breach of any license agreements or failure to obtain a license to proprietary rights that we may require to develop or commercialize our future drugs may have an adverse impact on us. Since patent applications in the United States and certain other jurisdictions are maintained in secrecy for 18 months or potentially longer, and since publication of discoveries in the scientific or patent literature often lags behind actual discoveries, we cannot be certain of the priority of inventions covered by pending patent applications. Moreover, we may have to participate in Interference, Derivation, Ex Parte Reexamination, Post-Grant Review, Inter Partes Review or Opposition proceedings, brought by third parties or declared by the USPTO.

Government Regulation

The FDA and regulatory authorities in state and local jurisdictions and in other countries impose substantial and burdensome requirements upon companies involved in the clinical development, manufacture, marketing and distribution of drugs such as those we are developing. These agencies and other federal, state and local entities regulate, among other things, the research and development, testing, manufacture, quality control, safety, effectiveness, labeling, storage, record keeping, approval, advertising and promotion, distribution, post-approval monitoring and reporting, sampling and export and import of drugs and drug candidates.

U.S. Government Regulation

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act and its implementing regulations. The process of obtaining regulatory approvals and the subsequent compliance with applicable federal, state, local and foreign statutes and regulations requires the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant to a variety of administrative or judicial sanctions, such as the FDA's refusal to approve pending New Drug Applications ("NDAs"), withdrawal of an approval, imposition of a clinical hold, issuance of warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties.

The process required by the FDA before a drug product may be marketed in the United States generally involves the following:

- completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's Good Laboratory Practice ("GLP") regulations;
- submission to the FDA of an IND, which must become effective before human clinical trials may begin;
- approval by an independent institutional review board ("IRB") at each clinical site before each trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with current Good Clinical Practice ("cGCP") requirements to establish the safety and efficacy of the proposed drug product for each indication;
- submission to the FDA of an NDA;
- satisfactory completion of an FDA advisory committee review, if applicable;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product is produced to assess compliance with current Good Manufacturing Practice ("cGMP"), requirements and to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity, and;
- FDA review and approval of the NDA.

Preclinical Studies

Preclinical studies include laboratory evaluation of product chemistry, toxicity and formulation, as well as animal studies to assess potential safety and efficacy. An IND sponsor must submit the results of the preclinical studies, together with manufacturing information, analytical data and any available clinical data or literature, among other things, to the FDA as part of an IND. Some preclinical testing may continue even after the IND is submitted. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to one or more proposed clinical trials and places the trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. As a result, submission of an IND may not result in the FDA allowing clinical trials to commence.

Clinical Trials

Clinical trials involve the administration of the investigational new drug to human patients under the supervision of qualified investigators in accordance with cGCP requirements, which include the requirement that all research patients provide their informed consent in writing for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the trial, 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. In addition, an IRB at each institution participating in the clinical trial must review and approve the plan for any clinical trial before it commences at that institution. Information about certain clinical trials must be submitted within specific timeframes to the National Institutes of Health ("NIH") for public dissemination on their website www.clinicaltrials.gov.

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

- Phase 1 clinical trial: The drug is initially introduced into healthy human volunteers, or patients without the targeted 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 clinical trial: The drug 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 or conditions and to determine dosage tolerance and optimal dosage.
- Phase 3 clinical trial: The drug 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 are observed. Each of 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 patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients.

Marketing Approval

Assuming successful completion of the required clinical testing, the results of the preclinical studies and clinical trials, 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 product for one or more indications. In most cases, the submission of an NDA is subject to a substantial application user fee. Under the Prescription Drug User Fee Act ("PDUFA") guidelines that are currently in effect, the FDA has a goal of ten months from the date of "filing" of a standard NDA for a new molecular entity to review and act on the submission. This review typically takes twelve months from the date the NDA is submitted to the FDA because the FDA has approximately two months to make a "filing" decision.

The FDA conducts a preliminary review of all NDAs within the first 60 days after submission, before accepting them for filing, to determine whether they are sufficiently complete to permit substantive review. The FDA may request additional information rather than accepting an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA reviews an NDA to determine, among other things, whether the drug is safe and effective and whether the facility in which it is manufactured, processed, packaged or held meets standards designed to assure the product's continued safety, quality and purity.

In addition, under the Pediatric Research Equity Act of 2003, as amended and reauthorized, certain research must contain data that are adequate to assess the safety and effectiveness of the drug 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. 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.

The FDA also may require submission of a risk evaluation and mitigation strategy ("REMS") plan to ensure that the benefits of the drug outweigh its risks. A REMS plan could include medication guides, physician communication plans, assessment plans, or elements to assure safe use, such as restricted distribution methods, patient registries or other risk minimization tools.

The FDA may refer an application for a novel drug to an advisory committee. 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.

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 may inspect one or more clinical trial sites to assure compliance with cGCP requirements.

After evaluating the application and all related information, including the advisory committee recommendation, if any, and inspection reports regarding the manufacturing facilities and clinical trial sites, the FDA may issue an approval letter, or, in some cases, a complete response letter. A complete response letter generally contains a statement of specific

conditions that must be met in order to secure final approval of the NDA and may require additional clinical or preclinical testing in order for the FDA to reconsider the application. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. If and when those conditions have been met to the FDA's satisfaction, the FDA will typically issue an approval letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications.

Even if the FDA approves a product, it may limit the approved indications for use of the product, require that particular contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs. After approval, 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.

Orphan Drug Act

Under the Orphan Drug Act of 1983, the FDA may grant orphan 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 must be requested before submitting an NDA. After the FDA grants orphan drug designation, the name of the sponsor, identity of the drug and its potential orphan use are disclosed publicly by the FDA. The orphan drug designation does not shorten the duration of the regulatory review or approval process, but does provide certain advantages, such as a waiver of PDUFA fees, enhanced access to FDA staff and potential waiver of pediatric research requirements.

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 applications, including a full NDA, or an abbreviated NDA ("ANDA"), to market a drug with the same active moiety for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity. 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. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the application user fee. A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. In addition, exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

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.

The FDA may impose a number of post-approval requirements as a condition of approval of a marketing authorization. For example, the FDA may require post-marketing testing, including Phase 4 clinical trials and surveillance to further assess and monitor the product's safety and effectiveness after commercialization.

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 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 requirements 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 of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in mandatory 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 letters or holds on post-approval clinical trials;
- refusal of the FDA to approve related pending applications or supplements to approved applications, or suspension or revocation of product 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.

Coverage and Reimbursement

Sales of our drug candidates, if approved, will depend, in part, on the extent to which such products will be covered by third-party payors, such as government health care programs, commercial insurance and managed healthcare organizations. These third-party payors are increasingly limiting coverage or reducing reimbursements for medical products and services. In addition, the U.S. government, state legislatures, and foreign governments have continued implementing cost-containment programs, including price controls, restrictions on reimbursement and requirements for substitution of generic products. For example, the United States Department of Health and Human Services ("HHS") imposes rebates on many Medicare Part B and Medicare Part D products to penalize price increases that outpace inflation on an annual basis. In addition, HHS has been empowered to negotiate the price of certain single-source drugs that have been on the market for at least seven years and single-source biologics that have been on the market for at least 11 years covered under Medicare as part of the Medicare Drug Price Negotiation Program. Each year up to 20 products will be selected by HHS for the Medicare Drug Price Negotiation Program. Products subject to the Medicare Drug Price Negotiation Program are expected to experience a significant reduction in reimbursement from the Medicare program on a per unit basis. If coverage and adequate reimbursement are not available, or are available only to limited levels, we may not be able to successfully commercialize any product candidates for which we receive approval, which could have an adverse effect on our operating results and our overall financial condition. Third-party payors decide which therapies they will pay for and establish reimbursement levels. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own coverage and reimbursement policies. Further, no uniform policy for coverage and reimbursement exists in the United States. Therefore, decisions regarding the extent of coverage and amount of reimbursement to be provided for any drug candidates that we develop will be made on a payor-by-payor basis. Each payor determines whether or not it will provide coverage for a therapy, what amount it will pay the manufacturer for the therapy, and on what tier of its formulary it will be placed. The position on a payor's list of covered drugs, or formulary, generally determines the copayment that a patient will need to make to obtain the therapy and can strongly influence the adoption of such therapy by patients and physicians. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit our net revenue and financial results. Decreases in third-party reimbursement for our drug candidates or a decision by a third-party payor to not cover our drug candidates could reduce physician usage of our drug candidates, once approved, and have a material adverse effect on our sales, results of operations and financial condition. Coverage policies and third-party payor reimbursement rates may change at any time. Therefore, even if favorable coverage and reimbursement status is attained, less favorable coverage policies and reimbursement rates may be implemented in the future.

Other Healthcare Laws

Because of our current and future arrangements with healthcare professionals, principal investigators, consultants, customers and third-party payors, we will also be subject to healthcare regulation and enforcement by the federal government and the state and foreign governments in which we will conduct our business, including clinical research, proposed sales, marketing and educational programs.

The U.S. laws that may affect our ability to operate, among others, include: the Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), which created federal criminal statutes that prohibit, among other things, knowingly and willfully executing a scheme to defraud any healthcare benefit program, including private third-party payors and knowing and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services, and governs the conduct of "covered entities," including certain healthcare providers, health plans and healthcare clearinghouses, as well as their respective "business associates," including their covered subcontractors, that create, receive, maintain or transmit individually identifiable health information for or on behalf of a covered entity, with respect to certain electronic healthcare transactions and protecting the security and privacy of protected health information; certain state laws governing the privacy and security of health information in certain circumstances, some of which are more stringent than HIPAA and many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts; the federal Anti-Kickback Statute, which prohibits, among other things, individuals or entities from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, in exchange for or to induce either the referral of an individual for, or the purchase, order or recommendation of, any good or service for which payment may be made under federal healthcare programs such as the Medicare and Medicaid programs; federal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid or other third-party payors that are false or fraudulent; federal criminal laws that prohibit executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters; the Physician Payments Sunshine Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies to report annually to HHS information related to payments and other transfers of value to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other healthcare professionals (such as physician assistants and nurse practitioners) and teaching hospitals, and ownership and investment interests held by physicians and their immediate family members.

In addition, many states have similar laws and regulations, such as anti-kickback and false claims laws that may be broader in scope and may apply regardless of payor, in addition to items and services reimbursed under Medicare, Medicaid and other state programs. Additionally, to the extent that our product is sold in a foreign country, we may be subject to similar foreign laws.

Failure to comply with these laws, where applicable, can result in the imposition of significant penalties, including civil, criminal, and administrative penalties; damages, disgorgement, monetary fines, exclusion from participation in Medicare, Medicaid and other federal or state healthcare programs, imprisonment, and integrity oversight and reporting obligations.

Healthcare Reform

Current and future legislative proposals to further reform healthcare or reduce healthcare costs may result in lower reimbursement for our products. The cost containment measures that payors and providers are instituting and the effect of any healthcare reform initiative implemented in the future could significantly reduce our revenues from the sale of our products.

For example, implementation of the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, collectively the Affordable Care Act ("PPACA"), has substantially changed healthcare financing and delivery. Since its enactment there have been executive, judicial and congressional challenges to certain aspects of the PPACA. For example, on July 4, 2025, the One Big Beautiful Bill Act ("OBBBA") was signed into law, which narrowed access to PPACA marketplace exchange enrollment and declined to extend the PPACA enhanced advanced premium tax credits that expired at the end of 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. The OBBBA also is expected to reduce Medicaid spending and enrollment by implementing work requirements for some beneficiaries, capping state-directed payments, reducing federal funding, and limiting provider taxes used to fund the program. Congress is considering proposed legislation intended to further reduce healthcare costs with alternatives to replace the expired PPACA subsidies. In the United States, we expect that additional federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the federal government will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

The current administration is pursuing policies to reduce regulations and expenditures across government agencies including at HHS, the FDA, the United States Centers for Medicare & Medicaid Services ("CMS") and related agencies. These actions, presently directed by executive orders or memoranda from the Office of Management and Budget, may propose policy changes that create additional uncertainty for our business. For example, the current administration has announced agreements with several pharmaceutical companies that require the drug manufacturers to offer, through a

direct-to-consumer platform, U.S. patients in the United States and Medicaid programs prescription drug Most-Favored Nation pricing equal to or lower than those paid in other developed nations, with additional mandates for direct-to-patient discounts and repatriation of foreign revenues. Other recent actions, for example, include (1) directing agencies to reduce agency workforce and cut programs; (2) directing HHS and other agencies to lower prescription drug costs through a variety of initiatives, including by improving upon the Medicare Drug Price Negotiation Program and establishing Most-Favored-Nation pricing for pharmaceutical products; (3) imposing tariffs on imported pharmaceutical products; and (4) as part of the Make America Healthy Again ("MAHA") Commission's Strategy Report released in September 2025, working across government agencies to increase enforcement on direct-to-consumer pharmaceutical advertising. Additionally, the current administration recently called on Congress to enact "The Great Healthcare Plan," to codify and expand Most-Favored Nation pricing, lower government subsidies to private insurance companies, increase healthcare price transparency, expand pharmaceutical drugs available for over-the-counter purchase, and enact restrictions on pharmacy benefit manager ("PBM") payment methodologies, among other things. These actions and policies may significantly reduce drug prices in the United States, potentially impacting manufacturers' global pricing strategies and profitability, while increasing their operational costs and compliance risks. In June 2024, in Loper Bright Enterprises v. Raimondo, the United States Supreme Court greatly reduced judicial deference to regulatory agencies, which could increase successful legal challenges to federal regulations affecting our operations. Congress may introduce and ultimately pass health care related legislation that could impact the drug approval process and make changes to the Medicare Drug Price Negotiation Program.

These and other reform initiatives may, among other things, result in modifications to the aforementioned laws and/or the implementation of new laws affecting the healthcare industry.

Human Capital Management

As of December 31, 2025, we had 23 full-time employees, the majority of whom were primarily engaged in research and development activities, including three individuals with MD degrees and eight professionals with PhD degrees specializing in the sciences. Many of these professionals have extensive epilepsy and neurology experience. In total, within our management team, we have colleagues who worked to shape the development or commercialization of a number of important marketed neurology drugs and ASMs, including: Zolgensma, Ztalmy, Fintepla, Brineura, Gilenya, Tysabri and Tecfidera.

We believe that our future success largely depends upon our continued ability to attract and retain highly skilled employees. We emphasize a number of measures and objectives in managing our human capital assets, including, among others: employee engagement, development and training, talent acquisition and retention, employee wellness, diversity and inclusion, and compensation, benefits and equity.

We believe that developing a diverse and inclusive culture is central to continuing to attract and retain the top talent necessary to deliver on our growth strategy. As such, we are investing in a work environment in which our employees feel inspired, included and enjoy a strong sense of belonging.

We value our employees' curiosity to translate scientific discoveries into innovative medicines and their courage and perseverance to overcome obstacles and operate with a sense of purpose and urgency on behalf of patients. Grounded in these guiding principles, we believe we have developed a collaborative and highly engaged environment where our colleagues feel respected and valued, and can contribute to their fullest potential.

We maintain equity incentive plans that are designed to attract, retain and motivate selected employees, consultants and directors through the granting of equity-based compensation awards and cash-based compensation awards, in order to increase stockholder value and the success of our company by motivating such individuals to perform to the best of their abilities and achieve our objectives.

In addition, our governance is overseen by an independent board of directors who provide and complement our expertise to execute the strategy and performance of our company. Among our board of directors, five out of seven members are independent. Collectively, our board of directors provides insight and expertise in areas of importance to the performance and growth of our enterprise, including experience as: senior operators of public companies; financial, transactional and oversight experience at public companies; proven biopharmaceutical experience; research and regulatory acumen in drug development; and corporate governance.

Facilities

We lease the space for our principal executive offices, located in Hudson Yards, New York City. In 2022, we formally instituted hybrid work policies. Our office facilities have received LEED Platinum certification.

Corporate and Other Information

We were incorporated in Delaware in April 2014. Our principal executive offices are located at 441 Ninth Avenue, 14th Floor, New York, New York 10001 and our telephone number is (646) 661-7661. Our corporate website address is www.ovidrx.com. Information contained on or accessible through our website is not a part of this Annual Report on Form 10-K, and the inclusion of our website address in this Annual Report is an inactive textual reference only.

We file electronically with the Securities and Exchange Commission ("SEC") our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act. We make these filings available on our website (www.ovidrx.com) under "Investors," free of charge, copies of these reports as soon as reasonably practicable after filing or furnishing these reports with the SEC.

Item 1A. Risk Factors

An investment in our securities involves a high degree of risk. You should carefully consider the following information about these risks, together with the other information appearing elsewhere in this Annual Report on Form 10-K, including our audited consolidated financial statements and related notes hereto, before deciding to invest in our common stock. The occurrence of any of the following risks could have a material adverse effect on our business, financial condition, results of operations and future growth prospects or cause our actual results to differ materially from those contained in forward-looking statements we have made in this report and those we may make from time to time. In these circumstances, the market price of our common stock could decline and stockholders may lose all or part of their investment. We cannot assure you that any of the events discussed below will not occur.

Summary of Select Risks Associated with Our Business

Our business faces significant risks and uncertainties. If any of the following risks are realized, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected. Some of the more significant risks we face include the following:

- We will require additional capital to finance our operations, which may not be available on acceptable terms, if at all. Failure to obtain this necessary capital when needed will force us to delay, limit or terminate certain of our drug development efforts or other operations.
- We expect to continue to incur substantial operating losses for the foreseeable future and may never achieve or maintain profitability.
- Our operating history may make it difficult to evaluate the success of our business to date and to assess our future viability.
- Our business could be adversely affected by economic downturns, changes in inflation and interest rates, changes in trade policy, natural disasters, political crises, geopolitical events, or other macroeconomic conditions, which may in the future negatively impact our business and financial performance.
- Our ability to raise capital may be limited by applicable laws and regulations.
- We may sell additional equity or debt securities or enter into other arrangements to fund our operations, which may result in dilution to our stockholders and impose restrictions or limitations on our business.
- Sale of a substantial number of shares of our common stock in the public market could cause the market price of our common stock to drop significantly.
- We are early in our drug development efforts of our current drug candidates and all of our drug candidates are in clinical trials or preclinical development. If we are unable to successfully develop, receive regulatory approval for and commercialize our drug candidates, or successfully develop any other drug candidates, or experience significant delays in doing so, our business will be harmed.
- Our future success is dependent on the successful clinical development, regulatory approval and commercialization of our current and future drug candidates. If we, or our licensees or collaborators, are not able to obtain the required regulatory approvals, we, or our licensees or collaborators, will not be able to commercialize our drug candidates, and our ability to generate revenue will be adversely affected.
- Because the results of preclinical studies or earlier clinical trials are not necessarily predictive of future results, our drug candidates may not have favorable results in planned or future preclinical studies or clinical trials, or may not receive regulatory approval.

- Interim topline and preliminary results from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures, which could result in material changes in the final data.
- Preclinical studies and clinical trials are very expensive, time-consuming and difficult to design and implement and involve uncertain outcomes. Further, we may encounter substantial delays in our clinical trials or we may fail to demonstrate safety and efficacy in our preclinical studies and clinical trials to the satisfaction of applicable regulatory authorities.
- If we are not successful in discovering, developing and commercializing additional drug candidates, our ability to expand our business and achieve our strategic objectives would be impaired.
- Our drug candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial potential or result in significant negative consequences following any potential marketing approval.
- Even if our current or future drug candidates receive marketing approval, they may fail to achieve market acceptance by physicians, patients, third-party payors or others in the medical community necessary for commercial success.
- Our relationships with customers, physicians, and third-party payors may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws, and other healthcare laws and regulations. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.
- Coverage and adequate reimbursement may not be available for our current or any future drug candidates, which could make it difficult for us to sell profitably, if approved.
- If we are unable to obtain and maintain patent protection for our current or any future drug candidates, or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.
- We may be involved in lawsuits to protect or enforce our patents, the patents of our licensors or our other intellectual property rights, which could be expensive, time consuming and unsuccessful.
- We do not have our own manufacturing capabilities and will rely on third parties to produce clinical and commercial supplies of our current and any future drug candidates.
- We rely on third parties to conduct, supervise and monitor our preclinical studies and clinical trials, and if those third parties perform in an unsatisfactory manner, it may harm our business.
- We may need to expand our organization, and we may experience difficulties in managing this growth, which could disrupt our operations.
- We are subject to numerous and varying privacy and security laws, and our failure to comply could result in penalties and reputational damage.
- We have previously identified material weaknesses in our internal control over financial reporting. In the future, if we are unable to identify or remediate material weaknesses, or if we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial condition, results of operations or cash flows, which may adversely affect investor confidence in us and, as a result, the value of our common stock.

Risks Related to Our Financial Position and Need for Additional Capital

We will require additional capital to finance our operations, which may not be available on acceptable terms, if at all. Failure to obtain this necessary capital when needed will force us to delay, limit or terminate certain of our drug development efforts or other operations.

Our operations have consumed substantial amounts of cash since our inception. As of December 31, 2025, our cash, cash equivalents and marketable securities were $90.4 million and we had an accumulated deficit of $321.7 million. We believe that our cash, cash equivalents and marketable securities will be sufficient to fund our operating and capital expenditure requirements through at least 12 months from the issuance of the financial statements contained in this Annual Report on Form 10-K. However, our operating plans may change because of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, third-party

funding, marketing and distribution arrangements, as well as other collaborations, strategic alliances and licensing arrangements, or any combination of these approaches.

We expect our expenses to continue to increase in connection with our ongoing activities, particularly as we conduct clinical trials of, and seek marketing approval for, our product candidates and advance our other programs. Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is a very time-consuming, expensive and uncertain process that takes years to complete. Other unanticipated costs may also arise. Because the design and outcome of our ongoing and anticipated clinical trials are highly uncertain, we cannot reasonably estimate the actual amount of resources and funding that will be necessary to successfully complete the development and commercialization of any product candidate we develop.

We may never generate the necessary data or results required to obtain regulatory approval in order to generate revenue from product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we do not expect to be commercially available for several years, if at all. Adequate additional financing may not be available to us on acceptable terms, or at all. Our ability to raise additional capital may be adversely impacted by potential worsening global economic conditions, inflation expectations, and the recent disruptions to and volatility in the credit and financial markets in the United States and worldwide resulting from public health crises and geopolitical tensions.

There have been and may continue to be significant disruptions to the global financial markets and general global economic slowdown due to macroeconomic and geopolitical factors. If disruptions and slowdown deepen or persist, we may not be able to access additional capital on favorable terms, or at all, which could in the future negatively affect our financial condition and our ability to pursue our business strategy. If we are unable to raise additional capital when needed, we will be required to delay, limit, reduce or terminate our drug development or future commercialization efforts, or grant rights to develop and market drug candidates that we would otherwise develop and market ourselves. If we are unable to obtain adequate financing when needed, we will be required to implement cost reduction measures, such as reducing operating expenses, or otherwise be required to delay, reduce the scope of or suspend one or more of our preclinical studies, clinical trials, research and development programs or commercialization efforts, or terminate our operations.

For additional details, see elsewhere in these Risk Factors, including "*Our business could be adversely affected by economic downturns, changes in inflation and interest rates, changes in trade policy (including tariffs), natural disasters, political crises, geopolitical events, or other macroeconomic conditions, which may in the future negatively impact our business and financial performance*."

We expect to continue to incur substantial operating losses for the foreseeable future and may never achieve or maintain profitability.

We have historically incurred significant operating losses. Our net loss was $17.4 million for the year ended December 31, 2025, and we had an accumulated deficit of $321.7 million as of that date. We expect to incur operating losses in the future. Since inception, we have devoted substantially all of our efforts to research and preclinical and clinical development of our drug candidates, as well as hiring employees and building our infrastructure.

We have no drugs approved for commercialization and have never generated any revenue from drug sales. Most of our drug candidates are still in the preclinical testing stage. It could be several years, if ever, before we have a commercialized drug. We expect to incur significant expenses and operating losses in the future, and the net losses we incur may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially if, and as, we:

- continue the ongoing and planned preclinical and clinical development of our drug candidates;
- continue to build a portfolio of drug candidates through the acquisition or in-license of drugs, drug candidates or technologies;
- initiate preclinical studies and clinical trials for any additional drug candidates that we may pursue in the future;
- seek marketing approvals for our current and future drug candidates that successfully complete clinical trials;
- establish a sales, marketing and distribution infrastructure to commercialize any drug candidate for which we may obtain marketing approval;

- develop, maintain, expand and protect our intellectual property portfolio;
- implement operational, financial and management systems; and
- attract, hire and retain additional administrative, clinical, regulatory and scientific personnel.

Even if we complete the development and regulatory processes described above, we anticipate incurring significant costs associated with launching and commercializing our current and future drug candidates.

If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations.

Our operating history may make it difficult to evaluate the success of our business to date and to assess our future viability.

Our operations have consumed substantial amounts of cash since our inception, primarily due to research and development of our drug candidates, organizing and staffing our company, business planning, raising capital, and acquiring assets. We have not yet demonstrated the ability to obtain marketing approvals, manufacture a commercial-scale drug or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we had more experience developing drug candidates.

We expect our financial condition and operating results to continue to fluctuate from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. We will need to eventually transition from a company with a research and development focus to a company capable of undertaking commercial activities. We may encounter unforeseen expenses, difficulties, complications and delays and may not be successful in such a transition.

Our business could be adversely affected by economic downturns, changes in inflation and interest rates, changes in trade policy (including tariffs), natural disasters, political crises, geopolitical events, or other macroeconomic conditions, which may in the future negatively impact our business and financial performance.

The global economy, including credit and financial markets, has experienced extreme volatility and disruptions, including, among other things, severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, supply chain shortages, fluctuations in inflation and interest rates, and uncertainty about economic stability. For example, during 2022 and 2023, the Federal Reserve raised interest rates multiple times in response to concerns about inflation. Higher interest rates, coupled with reduced government spending, tariffs, and volatility in financial markets may increase economic uncertainty and affect consumer spending. Trade policies and geopolitical disputes and conflicts can result in tariffs, sanctions and other measures that restrict international trade, and may adversely affect our costs of doing business, particularly if these measures occur in regions where our suppliers source components or raw materials. Similarly, geopolitical tensions and wars have created volatility in the global capital markets and are expected to have further global economic consequences, including disruptions of the global supply chain and energy markets. Increased inflation may result in increased operating costs (including labor costs) and may affect our operating budgets.

Trade disputes, tariffs, restrictions and other political tensions between the U.S. and other countries may also exacerbate unfavorable macroeconomic conditions, including inflationary pressures, foreign exchange volatility, financial market instability, and economic recessions or downturns, which may also limit our access to capital, or otherwise negatively impact our business and operations. The U.S. government has enacted, and continues to enact, a series of new tariffs, including a tariff on all imports and additional "reciprocal" tariffs targeting imports from specified countries. Additionally, current or future tariffs may result in increased research and development expenses, including with respect to increased costs associated with APIs, raw materials, laboratory equipment and research materials and components. In addition, such tariffs may increase our supply chain complexity and could also potentially disrupt our existing supply chain. Trade policies, geopolitical disputes and other trade restrictions affecting the import of materials necessary for clinical trials could result in delays to our development timelines. With the current administration in the U.S., additional and higher tariffs and sanctions may be imposed on goods imported from other countries which could increase the cost of goods needed to commercialize our products and continue development of our product candidates. Further, such actions by the U.S. could result in retaliatory action by those countries which could impact our ability to profitably commercialize our products in those jurisdictions. As a result, our business, operations and financial condition could be materially harmed.

Any such volatility and disruptions may adversely affect our business or the third parties on whom we rely. If the equity and credit markets deteriorate, including as a result of political unrest or war, it may make any necessary debt or

equity financing more difficult to obtain in a timely manner or on favorable terms, more costly or more dilutive. Increased inflation rates can adversely affect us by increasing our costs, including labor and employee benefit costs.

The ultimate impact of current or future tariffs and trade restrictions remains uncertain and could materially and adversely affect our business, financial condition, and prospects. While we actively monitor these risks, any prolonged economic downturn, escalation in trade tensions, or deterioration in international perception of U.S.-based companies could materially and adversely affect our business, ability to access the capital markets or other financing sources, results of operations, financial condition and prospects. In addition, tariffs and other trade developments have and may continue to heighten the risks related to the other risk factors described elsewhere in this Annual Report on Form 10-K.

We may sell additional equity or debt securities or enter into other arrangements to fund our operations, which may result in dilution to our stockholders and impose restrictions or limitations on our business.

Until such time as we can generate substantial revenue from drug sales, if ever, we expect to finance our cash needs through a combination of equity and debt financings, strategic alliances, and license and development agreements in connection with any collaborations. We do not have any committed external source of funds. To the extent that we issue additional equity securities, our stockholders may experience substantial dilution, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our stockholders. In addition, we may issue equity or debt securities as consideration for obtaining rights to additional compounds.

Debt and equity financings, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as redeeming our shares, making investments, issuing additional equity, incurring additional debt, making capital expenditures, declaring dividends or placing limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could negatively impact our ability to conduct our business. If we raise additional capital through future collaborations, strategic alliances or third-party licensing arrangements, we may have to relinquish valuable rights to our intellectual property, future revenue streams, research programs or drug candidates, or grant licenses on terms that may not be favorable to us. Any of these events could significantly harm our business, financial condition and prospects.

Sales of a substantial number of shares of our common stock in the public market could cause the market price of our common stock to drop significantly.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. Some of the holders of our securities have rights, subject to certain conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. Registration of these shares would result in the shares becoming freely tradable without restriction under the Securities Act except for shares held by our affiliates. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

Risks Related to the Development and Commercialization of Our Drug Candidates

We are early in our drug development efforts. If we are unable to successfully develop, receive regulatory approval for and commercialize our drug candidates, or successfully develop any other drug candidates, or experience significant delays in doing so, our business will be harmed.

We are early in our drug development efforts. In order to commercialize any product that achieves regulatory approval, we will need to build a commercial organization or successfully outsource commercialization, all of which will require substantial investment and significant marketing efforts before we have the ability to generate any revenue from drug sales. We do not have any drugs that are approved for commercial sale, and we may never be able to develop or commercialize marketable drugs.

Our ability to generate revenue from drug sales and achieve profitability depends on our ability, alone or with any current or future collaborative partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, our current and future drug candidates. We do not anticipate generating revenue from drug sales for the next several years, if ever. Our ability to generate revenue from drug sales depends heavily on our, or any current or future collaborators', success in the following areas, including but not limited to:

- timely and successfully completing preclinical and clinical development of our current and future drug candidates;

- obtaining regulatory approvals for our current and future drug candidates for which we successfully complete clinical trials;
- launching and commercializing any drug candidates for which we obtain regulatory approval by establishing a sales force, marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- qualifying for coverage and adequate reimbursement by government and third-party payors for any drug candidates for which we obtain regulatory approval, both in the United States and internationally;
- developing, validating and maintaining a commercially viable, sustainable, scalable, reproducible and transferable manufacturing process for our current and future drug candidates that is compliant with cGMP;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide an adequate amount and quality of drugs and services to support clinical development, as well as the market demand for our current and future drug candidates, if approved;
- obtaining market acceptance, if and when approved, of our current or any future drug candidates as a viable treatment option by physicians, patients, third-party payors and others in the medical community;
- effectively addressing any competing technological and market developments;
- implementing additional internal systems and infrastructure, as needed;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter and performing our obligations pursuant to such arrangements;
- obtaining and maintaining orphan drug exclusivity for any of our current and future drug candidates for which we obtain regulatory approval;
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how;
- avoiding and defending against third-party interference or infringement claims; and
- securing appropriate pricing in the United States, the European Union and other regions.

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 the drug candidates we develop, which would materially harm our business. If we do not receive marketing approvals for any drug candidate we develop, we may not be able to continue our operations.

Our future success is dependent on the successful clinical development, regulatory approval and commercialization of our current and future drug candidates. If we, or our licensees or collaborators, are not able to obtain the required regulatory approvals, we, or our licensees or collaborators, will not be able to commercialize our drug candidates, and our ability to generate revenue will be adversely affected.

We do not have any drugs that have received regulatory approval. Our business is dependent on our ability to successfully complete preclinical and clinical development of, obtain regulatory approval for, and, if approved, successfully commercialize our current and future drug candidates in a timely manner. Activities associated with the development and commercialization of our current and future drug candidates are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and similar regulatory authorities outside the United States. Failure to obtain regulatory approval in the United States or other jurisdictions would prevent us from commercializing and marketing our current and future drug candidates. An inability to effectively develop and commercialize our current and future drug candidates could have an adverse effect on our business, financial condition, results of operations and growth prospects.

Further, activities associated with the development and commercialization of our current and future drug candidates are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and similar regulatory authorities outside the United States. Failure to obtain regulatory approval in the United States or other jurisdictions would prevent us from commercializing and marketing our current and future drug candidates.

Even if we obtain approval from the FDA and comparable foreign regulatory authorities for our current and future drug candidates, any approval might contain significant limitations related to use restrictions for specified age groups,

warnings, precautions or contraindications, or may be subject to burdensome post-approval study or risk management requirements. If we are unable to obtain regulatory approval, or any approval contains significant limitations, we may not be able to obtain sufficient funding or generate sufficient revenue to continue the development of that drug candidate or any other drug candidate that we may in-license, develop or acquire in the future. In certain circumstances, our third-party licensees or collaborators are responsible for obtaining regulatory approvals in the countries covered by the license, and we are dependent on their efforts in order to achieve the necessary approvals in order to commercialize our products. If any future licensees or collaborators fail to perform their obligations to develop and obtain regulatory approvals for the licensed products, we may not be able to commercialize our products in the affected countries, or our ability to do so may be substantially delayed.

Furthermore, even if we obtain regulatory approval for our current and future drug candidates, we will still need to develop a commercial organization, establish a commercially viable pricing structure and obtain approval for adequate reimbursement from third-party and government payors. If we are unable to successfully commercialize our current and future drug candidates, we may not be able to generate sufficient revenue to continue our business.

Because the results of preclinical studies or earlier clinical trials are not necessarily predictive of future results, our drug candidates may not have favorable results in planned or future preclinical studies or clinical trials, or may not receive regulatory approval.

Success in preclinical testing and early clinical trials does not ensure that subsequent clinical trials will generate similar results or otherwise provide adequate data to demonstrate the efficacy and safety of a drug candidate. Frequently, drug candidates that have shown promising results in early clinical trials have subsequently suffered significant setbacks in later clinical trials. The results from preclinical studies of our current and future drug candidates may not be predictive of the effects of these compounds in later stage clinical trials. For example, we co-developed soticlestat with Takeda Company Limited ("Takeda") through completion of Phase 2 clinical trials and subsequently sold our rights back to Takeda under a royalty, license and termination agreement ("RLT Agreement"). Although we successfully completed Phase 1b/2a and Phase 2 clinical trials of soticlestat, in June 2024, Takeda reported that soticlestat failed to meet its primary endpoints in two Phase 3 trials evaluating soticlestat for the treatment of Dravet and Lennox-Gastaut syndromes and, in January 2025, Takeda announced discontinuation of the program. If we do not observe favorable results in clinical trials of one of our drug candidates, we may decide to delay or abandon clinical development of that drug candidate. Any such delay or abandonment could harm our business, financial condition, results of operations and prospects.

Interim topline and preliminary results from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures, which could result in material changes in the final data.

From time to time, we have and may in the future publish or report preliminary or interim data from our clinical trials. Preliminary or interim data from our clinical trials and those of our partners may not be indicative of the final results of the trial and are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and/or more patient data become available. Preliminary or topline results 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 or reported. As a result, preliminary or interim data should be considered carefully and with caution until final data are available. Differences between preliminary or interim data and final data could significantly harm our business prospects and may cause the trading price of our common stock to fluctuate significantly.

Preclinical studies and clinical trials are very expensive, time-consuming and difficult to design and implement and involve uncertain outcomes. Further, we may encounter substantial delays in our clinical trials or we may fail to demonstrate safety and efficacy in our preclinical studies and clinical trials to the satisfaction of applicable regulatory authorities.

All of our current drug candidates are in early clinical or preclinical development and their risk of failure is high. We must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that each of our drug candidates are safe and effective for its intended indications before we are prepared to submit an NDA for regulatory approval. We cannot predict with any certainty if or when we might submit an NDA for any of our product candidates or whether any such application will be approved by the FDA. Human clinical trials are very expensive and difficult to design and implement, in part because they are subject to rigorous review and regulatory requirements by numerous government authorities in the United States and in other countries where we intend to test and market our product candidates. For instance, the FDA or similar regulatory authorities in other countries may not agree with our proposed endpoints for any future clinical trial of our product candidates, which may delay the commencement of such clinical trial.

We estimate that the successful completion of clinical trials of our product candidates will take at least several years to complete, if not longer or at all. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. Furthermore, failure can occur at any stage and we could encounter problems that cause us to abandon or repeat clinical trials. Events that may prevent successful or timely completion of clinical development include:

- our inability to generate sufficient preclinical, toxicology or other data to support the initiation of clinical trials;
- our inability to develop and validate disease-relevant clinical endpoints;
- delays in reaching a consensus with regulatory authorities on trial design;
- delays in reaching agreement on acceptable terms with prospective contract research organizations ("CROs") and clinical trial sites;
- delays in opening investigational sites;
- delays or difficulty in recruiting and enrollment of suitable patients to participate in our clinical trials;
- imposition of a clinical hold by regulatory authorities because of a serious adverse event, concerns with a class of drug candidates or after an inspection of our clinical trial operations or trial sites;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- occurrence of serious adverse events associated with the drug candidate that are viewed to outweigh its potential benefits;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols; or
- business interruptions resulting from global geopolitical tensions and wars, or the perception that hostilities may be imminent, terrorism, natural disasters or public health crises.

Further, clinical endpoints for certain diseases we are targeting, including brain disorders with significant unmet need, have not been established, and accordingly, we may have to develop new modalities or modify existing endpoints to measure efficacy, which may increase the time it takes for us to commence or complete clinical trials. In addition, we believe investigators in this area may be inexperienced in conducting trials in this area due to the current lack of drugs to treat these disorders, which may result in increased time and expense to train investigators and open clinical sites.

Any inability to successfully complete preclinical and clinical development could result in additional costs to us or impair our ability to generate revenue from future drug sales and regulatory and commercialization milestones. In addition, if we make manufacturing or formulation changes to our drug candidates, we may need to conduct additional testing to bridge our modified drug candidate to earlier versions. Clinical trial delays could also shorten any periods during which we may have the exclusive right to commercialize our drug candidates, if approved, or allow our competitors to bring comparable drugs to market before we do, which could impair our ability to successfully commercialize our drug candidates and may harm our business, financial condition, results of operations and prospects.

Additionally, if the results of our clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with our drug candidates, we may:

- be delayed in obtaining marketing approval, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to additional post-marketing testing requirements;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the drug or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation strategy ("REMS");
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or

- experience damage to our reputation.

Our drug development costs will also increase if we experience delays in testing or obtaining marketing approvals. We do not know whether any of our preclinical studies or clinical trials will begin as planned, need to be restructured or be completed on schedule, if at all.

Further, we, the FDA or an IRB may suspend our clinical trials at any time if it appears that we or our collaborators are failing to conduct a trial in accordance with regulatory requirements, including the FDA's current Good Clinical Practice ("cGCP") regulations, that we are exposing participants to unacceptable health risks, or if the FDA finds deficiencies in our IND applications or the conduct of these trials. Therefore, we cannot predict with any certainty the schedule for commencement and completion of future clinical trials. If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of our drug candidates could be negatively impacted, and our ability to generate revenues from our drug candidates may be delayed.

If we are not successful in discovering, developing and commercializing additional drug candidates, our ability to expand our business and achieve our strategic objectives would be impaired.

A key element of our current strategy is to discover, develop and potentially commercialize a portfolio of drug candidates to treat certain brain disorders with significant unmet need. However, our business development activities and research activities may present attractive opportunities outside of our current areas of focus and we may choose to pursue drug candidates in other areas of interest including other disorders that we believe would be in the best interest of the Company and our stockholders. We plan to continuously review our strategies and modify as necessary based on attractive areas of interest and assets that we choose to pursue. We intend to develop our portfolio of drug candidates by in-licensing and entering into collaborations with biopharmaceutical companies or academic institutions for new drug candidates. Identifying new drug candidates requires substantial technical, financial and human resources, whether or not any drug candidates are ultimately identified. Even if we identify drug candidates that initially show promise, we may fail to in-license or acquire these assets and may also fail to successfully develop and commercialize such drug candidates for many reasons, including the following:

- the research methodology used may not be successful in identifying potential drug candidates;
- competitors may develop alternatives that render any drug candidate we develop obsolete;
- any drug candidate we develop may nevertheless be covered by third parties' patents or other exclusive rights;
- a drug candidate may, on further study, be shown to have harmful side effects or other characteristics that indicate it is unlikely to be effective or otherwise does not meet applicable regulatory criteria;
- a drug candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a drug candidate may not be accepted as safe and effective by physicians, patients, the medical community or third-party payors, even if approved.

We have limited financial and management resources and, as a result, we may forego or delay the pursuit of opportunities with other drug candidates or for other indications that later prove to have greater market potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial drugs or profitable market opportunities. If we do not accurately evaluate the commercial potential or target market for a particular drug candidate, we may relinquish valuable rights to that drug candidate through collaboration, licensing or other royalty arrangements in circumstances under which it would have been more advantageous for us to retain sole development and commercialization rights to such drug candidate.

If we are unsuccessful in identifying and developing additional drug candidates or are unable to do so, our key growth strategy and business will be harmed.

Enrollment and retention of patients in clinical trials is an expensive and time-consuming process and could be made more difficult or rendered impossible by multiple factors outside our control.

We focus our research and drug development on treatments for certain brain disorders with significant unmet need. This specifically includes medicines to treat symptoms of excess neuronal excitation such as seizures and psychoses that manifest in specific epilepsies, psychiatric, neurodegenerative and neurodevelopmental conditions. We are developing medicines for rare and broader conditions. Identifying and qualifying patients to participate in our clinical trials is critical

to our success. The number of patients suffering from the disorders that we are targeting is small and has not been established with precision. If the actual number of patients with these disorders is smaller than we anticipate, we may encounter difficulties in enrolling patients in our clinical trials, thereby delaying or preventing development and approval of our drug candidates. Even once enrolled, we may be unable to retain a sufficient number of patients to complete any of our trials. Patient enrollment and retention in clinical trials depends on many factors, including the size of the patient population, the nature of the trial protocol, the existing body of safety and efficacy data, the number and nature of competing treatments and ongoing clinical trials of competing therapies for the same indication, the proximity of patients to clinical sites and the eligibility criteria for the trial, any such enrollment issues could cause delays or prevent development and approval of our drug candidates. Because we are focused on addressing rare neurological disorders, there are limited patient pools from which to draw in order to complete our clinical trials in a timely and cost-effective manner. Furthermore, our efforts to build relationships with patient communities may not succeed, which could result in delays in patient enrollment in our clinical trials. In addition, any negative results we may report in clinical trials of our drug candidate may make it difficult or impossible to recruit and retain patients in other clinical trials of that same drug candidate. Delays or failures in planned patient enrollment or retention may result in increased costs, program delays or both, which could have a harmful effect on our ability to develop our drug candidates, or could render further development impossible.

Our drug candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial potential or result in significant negative consequences following any potential marketing approval.

During the conduct of clinical trials, patients report changes in their health, including illnesses, injuries and discomforts, to their doctor. Often, it is not possible to determine whether or not the drug candidate being studied caused these conditions. Regulatory authorities may draw different conclusions or require additional testing to confirm these determinations, if they occur. In addition, it is possible that illnesses, injuries, discomforts or other adverse events that were observed in earlier trials, as well as conditions that did not occur or went undetected in previous trials will be reported by subjects as we test our drug candidates in larger, longer and more extensive clinical programs, or, as use of these drug candidates becomes more widespread if they receive regulatory approval. Many times, side effects are only detectable after investigational drugs are tested in large-scale, Phase 3 trials or, in some cases, after they are made available to patients on a commercial scale after approval. If clinical experience indicates that any of our drug candidates causes adverse events or serious or life-threatening adverse events, the development of that drug candidate may fail or be delayed, or, if the drug candidate has received regulatory approval, such approval may be revoked, which would harm our business, prospects, operating results and financial condition.

Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of our drug candidates, the commercial prospects of our drug candidates may be harmed and our ability to generate revenue through their sale may be delayed or eliminated. Any of these occurrences may harm our business, financial condition and prospects significantly.

Additionally, if any of our drug candidates receive marketing approval, the FDA could require us to include a black box warning in our label or adopt REMS to ensure that the benefits outweigh its risks, which may include, among other things, a medication guide outlining the risks of the drug for distribution to patients and a communication plan to health care practitioners. Furthermore, if we or others later identify undesirable side effects caused by our drug candidates, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such drug candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a drug candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients;
- we may need to conduct a recall; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of our drug candidates and could significantly harm our business, prospects, financial condition and results of operations.

If the market opportunities for our drug candidates are smaller than we believe they are, even assuming approval of a drug candidate, our business may suffer. Because the patient populations in the market for our drug candidates may be

small and difficult to assess, we must be able to successfully identify patients and acquire a significant market share to achieve profitability and growth.

We focus our research and drug development on treatments for certain brain disorders with significant unmet need. This specifically includes medicines to treat symptoms of excess neuronal excitation such as seizures and psychoses that manifest in specific epilepsies, psychiatric, neurodegenerative and neurodevelopmental conditions. We are developing medicines for both rare and broader conditions. As a result of the disorders that we are targeting for drug development, our eligible patient population and pricing estimates may differ significantly from the actual market addressable by our drug candidates. Our projections of both the number of people who have these disorders, as well as the subset of people with these disorders who have the potential to benefit from treatment with our drug candidates, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including scientific literature, patient foundations, or market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these disorders. The number of patients may turn out to be lower than expected. Likewise, the potentially addressable patient population for each of our drug candidates may be limited or may not be amenable to treatment with our drug candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business.

We face substantial competition, which may result in others developing or commercializing drugs before or more successfully than us.

The development and commercialization of new drugs is highly competitive. We face competition with respect to our current drug candidates and will face competition with respect to any other drug candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell drugs or are pursuing the development of drug candidates for the treatment of the indications that we are pursuing. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

More established companies may have a competitive advantage over us due to their greater size, resources and institutional experience. In particular, these companies have greater experience and expertise in securing collaboration or partnering relationships, reimbursement, government contracts, relationships with key opinion leaders, conducting testing and clinical trials, obtaining and maintaining regulatory approvals and distribution relationships to market products, and marketing approved drugs. These companies also have significantly greater research and marketing capabilities than we do. If we are not able to compete effectively against existing and potential competitors, our business and financial condition may be harmed.

As a result of these factors, our competitors may obtain regulatory approval of their drugs before we are able to, which may limit our ability to develop or commercialize our drug candidates. Our competitors may also develop therapies that are safer, more effective, more widely accepted and cheaper than ours, and may also be more successful than us in manufacturing and marketing their drugs. These appreciable advantages could render our drug candidates obsolete or non-competitive before we can recover the expenses of such drug candidates' development and commercialization.

Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific, management and commercial personnel, establishing clinical trial sites and subject registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Even if our current or future drug candidates receive marketing approval, they may fail to achieve market acceptance by physicians, patients, third-party payors or others in the medical community necessary for commercial success.

Even if our current or future drug candidates receive marketing approval, they may fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If they do not achieve an adequate level of acceptance, we may not generate significant drug revenue and may not become profitable. The degree of market acceptance of our current or future drug candidates, if approved for commercial sale, will depend on a number of factors, including but not limited to:

- the efficacy and potential advantages compared to alternative treatments and therapies;

- the safety profile of our drug candidate compared to alternative treatments and therapies;
- effectiveness of sales and marketing efforts;
- the strength of our relationships with patient communities;
- the cost of treatment in relation to alternative treatments and therapies, including any similar generic treatments;
- our ability to offer such drug for sale at competitive prices;
- the convenience and ease of administration compared to alternative treatments and therapies;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- the availability of third-party coverage and adequate reimbursement;
- the prevalence and severity of any side effects; and
- any restrictions on the use of the drug together with other medications.

Our efforts to educate physicians, patients, third-party payors and others in the medical community on the benefits of our drug candidates may require significant resources and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of our drug candidates. Because we expect sales of our drug candidates, if approved, to generate substantially all of our drug revenues for the foreseeable future, the failure of our drugs to find market acceptance would harm our business and could require us to seek additional financing.

Even if we obtain and maintain approval for our current or future drug candidates from the FDA, we may never obtain approval for our current or future drug candidates outside of the United States, which would limit our market opportunities and could harm our business.

Approval of a drug candidate in the United States by the FDA does not ensure approval of such drug candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Sales of our current and future drug candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a drug candidate, comparable regulatory authorities of foreign countries also must approve the manufacturing and marketing of the drug candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, which may require additional preclinical studies or clinical trials. In many countries outside the United States, a drug candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for any drug candidates, if approved, is also subject to approval. Obtaining approval for our current and future drug candidates in the European Union from the European Commission following the opinion of the European Medicines Agency, if we choose to submit a marketing authorization application there, would be a lengthy and expensive process. The FDA and comparable foreign regulatory authorities have the ability to limit the indications for which the drug may be marketed, require extensive warnings on the drug labeling or require expensive and time-consuming additional clinical trials or reporting as conditions of approval. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our current and future drug candidates in certain countries. In certain cases, we are dependent on third parties to obtain such foreign regulatory approvals, and any delay or failure of performance of such third parties could delay or prevent our ability to commercialize our products in the affected countries.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Also, regulatory approval for our drug candidates may be withdrawn. If we fail to comply with the regulatory requirements, our target market will be reduced and our ability to realize the full market potential of our current and future drug candidates will be harmed and our business, financial condition, results of operations and prospects could be harmed.

If we seek approval to commercialize our current or future drug candidates outside of the United States, a variety of risks associated with international operations could harm our business.

If we seek approval of our current or future drug candidates outside of the United States, we expect that we will be subject to additional risks in commercialization including:

- different regulatory requirements for approval of therapies in foreign countries;
- reduced protection for intellectual property rights;
- the potential requirement of additional clinical studies in international jurisdictions;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- foreign reimbursement, pricing and insurance regimes;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical tensions and wars, or the perception that hostilities may be imminent, terrorism, natural disasters or public health crises.

We have no prior experience in these areas. In addition, there are complex regulatory, tax, labor and other legal requirements imposed by many of the individual countries in and outside of Europe with which we will need to comply. Many biopharmaceutical companies have found the process of marketing their own products in foreign countries to be very challenging.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit commercialization of any drug candidate that we may develop.

We face an inherent risk of product liability exposure related to the testing of our current and any future drug candidates in clinical trials and may face an even greater risk if we commercialize any drug candidate that we may develop. If we cannot successfully defend ourselves against claims that any such drug candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any drug candidate that we may develop;
- loss of revenue;
- substantial monetary awards to trial participants or patients;
- significant time and costs to defend the related litigation;
- withdrawal of clinical trial participants;
- the inability to commercialize any drug candidate that we may develop; and
- injury to our reputation and significant negative media attention.

Although we maintain product liability insurance coverage, such insurance may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage each time we commence a clinical trial and if we successfully commercialize any drug candidate. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

Risks Related to Licensing and Collaboration Arrangements

We may be required to relinquish important rights to and control over the development and commercialization of our drug candidates to any future collaborators.

Our current and future collaborations could subject us to a number of risks, including:

- we may be required to undertake the expenditure of substantial operational, financial and management resources;
- we may be required to issue equity securities that would dilute our stockholders' percentage of ownership;
- we may be required to assume substantial actual or contingent liabilities;
- we may not be able to control the amount and timing of resources that our strategic collaborators devote to the development or commercialization of our drug candidates;
- strategic collaborators may delay clinical trials, provide insufficient funding, terminate a clinical trial or abandon a drug candidate, repeat or conduct new clinical trials or require a new version of a drug candidate for clinical testing;
- strategic collaborators may not pursue further development and commercialization of products resulting from the strategic collaboration arrangement or may elect to discontinue research and development programs;
- strategic collaborators may not commit adequate resources to the marketing and distribution of our drug candidates, limiting our potential revenues from these products;
- we rely on our current collaborators to manufacture drug substance and drug product and may do so with respect to future collaborators, which could result in disputes or delays;
- disputes may arise between us and our strategic collaborators that result in the delay or termination of the research, development or commercialization of our drug candidates or that result in costly litigation or arbitration that diverts management's attention and consumes resources;
- disputes may arise between us and our current or future collaborators regarding any termination of any collaboration, license, or other business development arrangement in which we may enter;
- strategic collaborators may experience financial difficulties;
- strategic collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in a manner that could jeopardize or invalidate our proprietary information or expose us to potential litigation;
- business combinations or significant changes in a strategic collaborator's business strategy may also adversely affect a strategic collaborator's willingness or ability to complete its obligations under any arrangement;
- strategic collaborators could decide to move forward with a competing drug candidate developed either independently or in collaboration with others, including our competitors; and
- strategic collaborators could terminate the arrangement or allow it to expire, which would delay the development and may increase the cost of developing our drug candidates.

If we engage in future acquisitions or strategic partnerships, this may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

Our business plan is to continue to evaluate various acquisitions and strategic partnerships, including licensing or acquiring complementary drugs, intellectual property rights, technologies, or businesses. Any potential acquisition or strategic partnership may entail numerous risks, including:

- increased operating expenses and cash requirements;
- the assumption of additional indebtedness or contingent liabilities;
- assimilation of operations, intellectual property and drugs of an acquired company, including difficulties associated with integrating new personnel;

- the diversion of our management's attention from our existing drug programs and initiatives in pursuing such a strategic partnership, merger or acquisition;
- retention of key employees, the loss of key personnel, and uncertainties in our ability to maintain key business relationships;
- risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their existing drugs or drug candidates and regulatory approvals;
- our inability to generate revenue from acquired technology and/or drugs sufficient to meet our objectives in undertaking the acquisition or even to offset the associated acquisition and maintenance costs;
- challenges related to integrating acquired businesses or entering into or realizing the benefits of strategic transactions generally; and
- risks associated with potential international acquisition transactions, including in countries where we do not currently have a material presence.

In addition, if we engage in future acquisitions or strategic partnerships, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense. Moreover, we may not be able to locate suitable acquisition opportunities and this inability could impair our ability to grow or obtain access to technology or drugs that may be important to the development of our business.

We may explore additional strategic collaborations that may never materialize or may fail.

Our business strategy is based on acquiring or in-licensing compounds directed at certain epilepsies, seizure-related disorders, and other brain neurological disorders. As a result, we intend to periodically explore a variety of possible additional strategic collaborations in an effort to gain access to additional drug candidates or resources. At the current time, we cannot predict what form such a strategic collaboration might take. We are likely to face significant competition in seeking appropriate strategic collaborators, and strategic collaborations can be complicated and time consuming to negotiate and document. We may not be able to negotiate strategic collaborations on acceptable terms, or at all. We are unable to predict when, if ever, we will enter into any additional strategic collaborations because of the numerous risks and uncertainties associated with establishing them, and we cannot predict the success of any collaborations that we enter into. We may enter into strategic collaborations that we subsequently no longer wish to pursue.

If our strategic collaborations do not result in the successful development and commercialization of products, or if one of our collaborators or licensees terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration or license, as appropriate. If we do not receive the potential funding under these agreements, our development of product candidates could be delayed, and we may need additional resources to develop product candidates. For example, in March 2021, we entered into the RLT Agreement, pursuant to which Takeda secured rights to our global share in soticlestat and had sole discretion over the conduct of the development and commercialization of soticlestat. In June 2024, Takeda reported that soticlestat failed to meet its primary endpoints in two Phase 3 trials evaluating soticlestat for the treatment of Dravet and Lennox-Gastaut syndromes and, in January 2025, Takeda announced discontinuation of the program. In addition, if one of our collaborators or licensees terminates its agreement with us, we may find it more difficult to find a suitable replacement collaborator or licensee or attract new collaborators or licensees, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected.

Risks Related to Regulatory Compliance

Our relationships with customers, physicians, and third-party payors may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws, and other healthcare laws and regulations. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

Healthcare providers and third-party payors in the United States and elsewhere will play a primary role in the recommendation and prescription of any drug candidates for which we obtain marketing approval. Our current and future arrangements with healthcare professionals, principal investigators, consultants, customers and third-party payors may subject us to various federal and state fraud and abuse laws and other healthcare laws, including, without limitation, the federal Anti-Kickback Statute, the federal civil and criminal false claims laws and the law commonly referred to as the Physician Payments Sunshine Act and regulations. These laws will impact, among other things, our clinical research,

proposed sales, marketing and educational programs. In addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct or may conduct our business. The laws that will affect our operations include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, in return for the purchase, recommendation, leasing or furnishing of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers and formulary managers on the other. The Patient Protection and Affordable Care Act, as amended ("PPACA"), amended the intent requirement of the federal Anti-Kickback Statute. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation;
- federal civil and criminal false claims laws, including, without limitation, the False Claims Act, and civil monetary penalty laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid or other government payors that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. The PPACA provides, and recent government cases against pharmaceutical and medical device manufacturers support, the view that federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, may implicate the False Claims Act;
- HIPAA, which created additional federal criminal statutes that prohibit a person from knowingly and willfully executing a scheme or making false or fraudulent statements to defraud any healthcare benefit program, regardless of the payor (e.g., public or private);
- HIPAA, as amended by HITECH, and their implementing regulations, and as amended again by the final HIPAA omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach Notification Rules Under HITECH and the Genetic Information Nondiscrimination Act, published in January 2013, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, healthcare clearinghouses and certain healthcare providers, known as covered entities, and their respective business associates, individuals or entities that perform certain services on behalf of a covered entity that involves the use or disclosure of individually identifiable health information and their subcontractors that use, disclose or otherwise process individually identifiable health information;
- Physician Payments Sunshine Act, which is part of the PPACA, requires that certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, information related to: (i) payments or other "transfers of value" made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other healthcare professionals (such as physician assistants and nurse practitioners), and teaching hospitals; and (ii) ownership and investment interests held by physicians and their immediate family members;
- state and foreign law equivalents of each of the above federal laws, state laws that require manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures and/or information regarding drug pricing, state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or to adopt compliance programs as prescribed by state laws and regulations, or that otherwise restrict payments that may be made to healthcare providers, state laws and regulations that require drug manufacturers to file reports relating to drug pricing and marketing information, and state and local laws that require the registration of pharmaceutical sales representatives; and
- state and foreign laws that govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws.

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 be subject to significant civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from participation in government funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws and the curtailment or restructuring of our operations.

The risk of our being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. The shifting compliance environment and the need to build and maintain robust and expandable systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the possibility that a healthcare company may run afoul of one or more of the requirements.

Coverage and adequate reimbursement may not be available for our current or any future drug candidates, which could make it difficult for us to sell profitably, if approved.

Market acceptance and sales of any drug candidates that we commercialize, if approved, will depend in part on the extent to which coverage and adequate reimbursement for these drugs and related treatments will be available from third-party payors, including government health administration authorities, managed care organizations and other private health insurers. Third-party payors decide which therapies they will pay for and establish reimbursement levels. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own coverage and reimbursement policies. However, decisions regarding the extent of coverage and amount of reimbursement to be provided for any drug candidates that we develop will be made on a payor-by-payor basis. One third-party payor's determination to provide coverage for a drug does not assure that other payors will also provide coverage, and adequate reimbursement, for the drug. Additionally, a third-party payor's decision to provide coverage for a therapy does not imply that an adequate reimbursement rate will be approved. Each third-party payor determines whether or not it will provide coverage for a therapy, what amount it will pay the manufacturer for the therapy, and on what tier of its formulary it will be placed. The position on a third-party payor's list of covered drugs, or formulary, generally determines the co-payment that a patient will need to make to obtain the therapy and can strongly influence the adoption of such therapy by patients and physicians. Patients who are prescribed treatments for their conditions and providers prescribing such services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Patients are unlikely to use our drugs unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our drugs.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. For example, HHS imposes rebates on many Medicare Part B and Medicare Part D products to penalize price increases that outpace inflation on an annual basis. In addition, HHS has been empowered to negotiate the price of certain single-source drugs that have been on the market for at least seven (7) years and single-source biologics that have been on the market for at least eleven (11) years covered under Medicare as part of the Medicare Drug Price Negotiation Program. Each year up to twenty (20) products will be selected by HHS for the Medicare Drug Price Negotiation Program. Products subject to the Medicare Drug Price Negotiation Program are expected to experience a significant reduction in reimbursement from the Medicare program on a per unit basis. If coverage and adequate reimbursement are not available, or are available only to limited levels, we may not be able to successfully commercialize our current and any future product candidates that we develop, which could have an adverse effect on our operating results and our overall financial condition. Third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. We cannot be sure that coverage and reimbursement will be available for any drug that we commercialize and, if reimbursement is available, what the level of reimbursement will be. Inadequate coverage and reimbursement may impact the demand for, or the price of, any drug for which we obtain marketing approval. If coverage and adequate reimbursement are not available, or are available only to limited levels, we may not be able to successfully commercialize our current and any future drug candidates that we develop. Further, coverage policies and third-party payor reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained, less favorable coverage policies and reimbursement rates may be implemented in the future.

Healthcare legislative reform measures may have a negative impact on our business and results of operations.

In the United States and some foreign jurisdictions, there have been, and continue to be, several legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of drug candidates, restrict or regulate post-approval activities, and affect our ability to profitably sell any drug candidates for which we obtain marketing approval.

Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and/or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. In March 2010, the PPACA was passed, which substantially changed the way healthcare is financed by both the government and private insurers and significantly impacts the U.S. pharmaceutical industry.

There have been amendments to and executive, judicial, congressional and executive branch challenges to certain aspects of the PPACA. For example, on July 4, 2025, OBBBA, was signed into law, which narrowed access to PPACA marketplace exchange enrollment and declined to extend the PPACA enhanced advanced premium tax credits that expired at the end of 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. The OBBBA also is expected to reduce Medicaid spending and enrollment by implementing work requirements for some beneficiaries, capping state-directed payments, reducing federal funding, and limiting provider taxes used to fund the program. Congress is considering proposed legislation intended to further reduce healthcare costs with alternatives to replace the expired PPACA subsidies. In the United States, we expect that additional federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the federal government will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

The current administration is pursuing policies to reduce regulations and expenditures across government agencies including at HHS, the FDA, CMS and related agencies. These actions, presently directed by executive orders or memoranda from the Office of Management and Budget, may propose policy changes that create additional uncertainty for our business. For example, the current administration has announced agreements with several pharmaceutical companies that require the drug manufacturers to offer, through a direct to consumer platform, U.S. patients and Medicaid programs prescription drug Most-Favored Nation pricing equal to or lower than those paid in other developed nations, with additional mandates for direct-to-patient discounts and repatriation of foreign revenues. Other recent actions, for example, include (1) directing agencies to reduce agency workforce and cut programs; (2) directing HHS and other agencies to lower prescription drug costs through a variety of initiatives, including by improving upon the Medicare Drug Price Negotiation Program and establishing Most-Favored-Nation pricing for pharmaceutical products; (3) imposing tariffs on imported pharmaceutical products; and (4) as part of the MAHA Commission's Strategy Report released in September 2025, working across government agencies to increase enforcement on direct-to-consumer pharmaceutical advertising. Additionally, the current administration recently called on Congress to enact "The Great Healthcare Plan," to codify and expand Most-Favored Nation pricing, lower government subsidies to private insurance companies, increase healthcare price transparency, expand pharmaceutical drugs available for over-the-counter purchase, and enact restrictions on PBM payment methodologies, among other things. These actions and policies may significantly reduce U.S. drug prices, potentially impacting manufacturers' global pricing strategies and profitability, while increasing their operational costs and compliance risks. In June 2024, in Loper Bright Enterprises v. Raimondo, the United States Supreme Court greatly reduced judicial deference to regulatory agencies, which could increase successful legal challenges to federal regulations affecting our operations. Congress may introduce and ultimately pass health care related legislation that could impact the drug approval process and make changes to the Medicare Drug Price Negotiation Program.

At the state level, legislatures have increasingly passed and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or judicial action in the United States or any other applicable jurisdiction. If we or any third parties we may engage are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or such third parties are not able to maintain regulatory compliance, we may not achieve or sustain profitability.

We may not be able to obtain or maintain orphan drug designations or exclusivity for our drug candidates, which could limit the potential profitability of our drug candidates.

Regulatory authorities in some jurisdictions, including the United States, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a drug as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States. Generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for an indication for which it receives the designation, then the drug is entitled to a period of marketing exclusivity that precludes the applicable regulatory authority from approving another marketing application for the same drug for the same indication for the exclusivity period except in limited situations. For purposes of small molecule drugs, the FDA defines "same drug" as a drug that contains the same active moiety and is intended for the same use as the drug in question. A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation.

Obtaining orphan drug designations is important to our business strategy; however, obtaining an orphan drug designation can be difficult and we may not be successful in doing so. Even if we were to obtain orphan drug designation for a drug candidate, we may not obtain orphan exclusivity and that exclusivity may not effectively protect the drug from the competition of different drugs for the same condition, which could be approved during the exclusivity period. Additionally, after an orphan drug is approved, the FDA could subsequently approve another application for the same drug for the same indication if the FDA concludes that the later drug is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug exclusive marketing rights in the United States also may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. The failure to obtain an orphan drug designation for any drug candidates we may develop, the inability to maintain that designation for the duration of the applicable period, or the inability to obtain or maintain orphan drug exclusivity could reduce our ability to make sufficient sales of the applicable drug candidate to balance our expenses incurred to develop it, which would have a negative impact on our operational results and financial condition.

Even if we obtain regulatory approval for our current or future drug candidates, they will remain subject to ongoing regulatory oversight.

Even if we obtain any regulatory approval for our current or future drug candidates, such approvals will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping and submission of safety and other post-market information. Any regulatory approvals that we receive for our current or future drug candidates may also be subject to a REMS, limitations on the approved indicated uses for which the drug may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 trials, and surveillance to monitor the quality, safety and efficacy of the drug.

In addition, drug manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP requirements and adherence to commitments made in the NDA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with a drug, such as adverse events of unanticipated severity or frequency, or problems with the facility where the drug is manufactured or if a regulatory authority disagrees with the promotion, marketing or labeling of that drug, a regulatory authority may impose restrictions relative to that drug, the manufacturing facility or us, including requesting a recall or requiring withdrawal of the drug from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements following approval of our current or future drug candidates, a regulatory authority may:

- issue an untitled letter or warning letter asserting that we are in violation of the law;
- seek an injunction or impose administrative, civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending NDA or comparable foreign marketing application (or any supplements thereto) submitted by us or our strategic partners;
- restrict the marketing or manufacturing of the drug;
- seize or detain the drug or otherwise require the withdrawal of the drug from the market;

- refuse to permit the import or export of drug candidates; or
- refuse to allow us to enter into supply contracts, including government contracts.

Moreover, the FDA strictly regulates the promotional claims that may be made about drug products. In particular, a product may not be promoted for uses that are not approved by the FDA as reflected in the product's approved labeling. 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 civil, criminal and administrative penalties.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our current or future drug candidates and harm our business, financial condition, results of operations and prospects.

In addition, the FDA's policies, and those of equivalent foreign regulatory agencies, may change and additional government regulations may be enacted that could cause changes to or delays in the drug review process, or suspend or restrict regulatory approval of our drug candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would harm our business, financial condition, results of operations and prospects.

Disruptions at the FDA and other government agencies caused by layoffs, funding shortages or global health concerns could negatively impact our business.

The ability of the FDA to review proposed clinical trials or approve new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory, and policy changes, the FDA's ability to hire and retain key personnel and accept payment of user fees, and other events that may otherwise affect the FDA's ability to perform routine functions. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, including executive and congressional priorities, the impacts of which are inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may slow the time necessary for new product candidates to be reviewed and/or approved, which would adversely affect our business. For example, over the last several years, including in October 2025, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities. If a prolonged government shutdown occurs, 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 business. In addition, the current administration has implemented substantial reductions in force at various government agencies including the FDA, and has implemented layoffs at the FDA, which could significantly reduce the FDA's capacity to perform its functions in a manner consistent with its past practices and could delay reviews and negatively impact our business.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain patent protection for our current or any future drug candidates, or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our development programs and drug candidates. Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our current and any future drug candidates. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our current and future development programs and drug candidates. The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our current or any future drug candidates in the United States or in other foreign countries. There is

no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found, which can invalidate a patent or prevent a patent from issuing from a pending patent application. Even if patents do successfully issue and even if such patents cover our current or any future drug candidates, third parties may challenge their validity, enforceability or scope, which may result in such patents being narrowed, invalidated, or held unenforceable. Any successful opposition to these patents or any other patents owned by or licensed to us could deprive us of rights necessary for the successful commercialization of any drug candidates or companion diagnostic that we may develop. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a drug candidate and companion diagnostic under patent protection could be reduced.

If the patent applications we hold or have in-licensed with respect to our development programs and drug candidates fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our current or any future drug candidates, it could dissuade companies from collaborating with us to develop drug candidates, and threaten our ability to commercialize, future drugs. Any such outcome could have a negative effect on our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does. Publications of discoveries in scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or drugs, in whole or in part, or which effectively prevent others from commercializing competitive technologies and drugs. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. In December 2011, the Leahy-Smith America Invents Act ("Leahy-Smith Act") was signed into law. The Leahy-Smith Act includes a number of significant changes to United States patent law. These include provisions that affect the way patent applications are prosecuted and may also affect patent litigation. The United States Patent Office recently developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, only became effective in March 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could harm our business and financial condition.

Moreover, we may be subject to a third-party pre-issuance submission of prior art to the USPTO or become involved in opposition, derivation, reexamination, inter partes review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or drugs and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize drugs without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future drug candidates.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. An adverse determination in any such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and drugs, or limit the duration of the patent protection of our technology and drugs. Moreover, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years from the earliest filing date of a non-provisional patent application. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Without patent protection for our current or future drug candidates, we may be open to competition from generic versions of such drugs. Given the amount of time required for the development, testing and regulatory review of new drug candidates, patents protecting such candidates might expire before or shortly after such

candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing drugs similar or identical to ours.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the USPTO and various government patent agencies outside of the United States over the lifetime of our owned and licensed patents and/or applications and any patent rights we may own or license in the future. We rely on our outside counsel or our licensing partners to pay these fees due to non-U.S. patent agencies. The USPTO and various non-U.S. government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply and we are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market and this circumstance could harm our business.

Patent terms may be inadequate to protect our competitive position on our drug candidates for an adequate amount of time.

Given the amount of time required for the development, testing and regulatory review of new drug candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. We expect to seek extensions of patent terms in the United States and, if available, in other countries where we are prosecuting patents. In the United States, the Drug Price Competition and Patent Term Restoration Act of 1984 permits a patent term extension of up to five years beyond the normal expiration of the patent, which is limited to the approved indication (or any additional indications approved during the period of extension). However, the applicable authorities, including the FDA and the USPTO in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. If this occurs, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data and launch their drug earlier than might otherwise be the case.

Intellectual property rights do not necessarily address all potential threats to our business.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business. The following examples are illustrative:

- others may be able to make compounds or formulations that are similar to our drug candidates but that are not covered by the claims of any patents, should they issue, that we own or control;
- we or any strategic partners might not have been the first to make the inventions covered by the issued patents or pending patent applications that we own or control;
- we might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own or control may not provide us with any competitive advantages, or may be held invalid or unenforceable because of legal challenges;
- our competitors might conduct research and development activities in the United States and other countries that provide a safe harbor from patent infringement claims for certain research and development activities, as well as in countries where we do not have patent rights and then use the information learned from such activities to develop competitive drugs for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable; and

- the patents of others may have an adverse effect on our business.

The proprietary map of disease-relevant biological pathways underlying orphan disorders of the brain that we developed would not be appropriate for patent protection and, as a result, we rely on trade secrets to protect this aspect of our business.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a negative impact on the success of our business.

Our commercial success depends, in part, upon our ability and the ability of our current or future collaborators to develop, manufacture, market and sell our current and any future drug candidates and use our proprietary technologies without infringing the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may in the future become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our current and any future drug candidates and technology, including interference proceedings, post grant review and inter partes review before the USPTO. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could have a negative impact on our ability to commercialize our current and any future drug candidates. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. If we are found to infringe a third party's valid and enforceable intellectual property rights, we could be required to obtain a license from such third party to continue developing, manufacturing and marketing our drug candidate(s) and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing technology or drug candidate. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from manufacturing and commercializing our current or any future drug candidates or force us to cease some or all of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business, financial condition, results of operations and prospects. See the section herein titled "Item 3. Legal Proceedings" for additional information.

We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Certain of our employees, consultants or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property.

We may be involved in lawsuits to protect or enforce our patents, the patents of our licensors or our other intellectual property rights, which could be expensive, time consuming and unsuccessful.

Competitors may infringe or otherwise violate our patents, the patents of our licensors or our other intellectual property rights. To counter infringement or unauthorized use, we may be required to file legal claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing. The initiation of a claim against a third party may also cause the third party to bring counter claims against us such as claims asserting that our patents are invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, non-enablement or lack of statutory subject matter. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant material information from the USPTO, or made a materially misleading statement, during prosecution. Third parties may also raise similar validity claims before the USPTO in post-grant proceedings such as ex parte reexaminations, inter partes review, or post-grant review, or oppositions or similar proceedings outside the United States, in parallel with litigation or even outside the context of litigation. The outcome following legal assertions of invalidity and unenforceability is unpredictable. We cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. For the patents and patent applications that we have licensed, we may have limited or no right to participate in the defense of any licensed patents against challenge by a third party. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of any future patent protection on our current or future drug candidates. Such a loss of patent protection could harm our business.

We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States. Our business could be harmed if in litigation the prevailing party does not offer us a license on commercially reasonable terms. Any litigation or other proceedings to enforce our intellectual property rights may fail, and even if successful, may result in substantial costs and distract our management and other employees.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have an adverse effect on the price of our common stock.

Changes in U.S. patent law or the patent law of other countries or jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our current and any future drug candidates.

The United States has recently enacted and implemented wide-ranging patent reform legislation. The U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on actions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce patents that we have licensed or that we might obtain in the future. Similarly, changes in patent law and regulations in other countries or jurisdictions or changes in the governmental bodies that enforce them or changes in how the relevant governmental authority enforces patent laws or regulations may weaken our ability to obtain new patents or to enforce patents that we have licensed or that we may obtain in the future.

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 drug candidates throughout the world would be prohibitively expensive. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own drugs and, further, may export otherwise infringing drugs to territories where we may obtain patent protection, but where patent enforcement is not as strong as that in the United States. These drugs may

compete with our drugs 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 so competing.

Reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

If we rely on third parties to manufacture or commercialize our current or any future drug candidates, or if we collaborate with additional third parties for the development of our current or any future drug candidates, we must, at times, share trade secrets with them. We may also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure could have an adverse effect on our business and results of operations.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third parties, independent development or publication of information by any third-party collaborators. A competitor's discovery of our trade secrets would harm our business.

Risks Related to Our Dependence on Third Parties

We do not have our own manufacturing capabilities and will rely on third parties to produce clinical and commercial supplies of our current and any future drug candidates.

We do not own or operate, and we do not expect to own or operate, facilities for drug manufacturing, drug formulation, storage and distribution or testing. We have been in the past, and will continue to be, dependent on third parties to manufacture the clinical supplies of our drug candidates.

Further, we also will rely on third-party manufacturers to supply us with sufficient quantities of our drug candidates to be used, if approved, for commercialization. Any significant delay in the supply of a drug candidate, or the raw material components thereof, for an ongoing clinical trial due to the need to replace a third-party manufacturer could considerably delay completion of our clinical trials, product testing and potential regulatory approval of our drug candidates.

Further, our reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured drug candidates ourselves, including:

- inability to meet our drug specifications and quality requirements consistently;
- delay or inability to procure or expand sufficient manufacturing capacity;
- issues related to scale-up of manufacturing;
- costs and validation of new equipment and facilities required for scale-up;
- failure to comply with cGMP and similar foreign standards;
- inability to negotiate manufacturing agreements with third parties under commercially reasonable terms, if at all;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us;
- reliance on single sources for drug components;
- lack of qualified backup suppliers for those components that are currently purchased from a sole or single source supplier;

- operations of our third-party manufacturers or suppliers could be disrupted by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier; and
- carrier disruptions or increased costs that are beyond our control.

Any of these events could lead to clinical trial delays, failure to obtain regulatory approval or impact our ability to successfully commercialize our current or any future drug candidates once approved. Some of these events could be the basis for FDA action, including injunction, request for recall, seizure, or total or partial suspension of production.

We rely on third parties to conduct, supervise and monitor our preclinical studies and clinical trials, and if those third parties perform in an unsatisfactory manner, it may harm our business.

We do not currently have the ability to independently conduct any preclinical studies or clinical trials. We rely on CROs and clinical trial sites to ensure the proper and timely conduct of our preclinical studies and clinical trials, and we expect to have limited influence over their actual performance. We rely upon CROs to monitor and manage data for our clinical programs, as well as the execution of future preclinical studies. We are able to control only certain aspects of our CROs' activities. Nevertheless, we are responsible for ensuring that each of our preclinical studies or clinical trials are conducted in accordance with the applicable protocol, legal, regulatory and scientific standards and our reliance on the CROs does not relieve us of our regulatory responsibilities.

We and our CROs are required to comply with GLPs and cGCPs, which are regulations and guidelines enforced by the FDA and are also required by the Competent Authorities of the Member States of the European Economic Area and comparable foreign regulatory authorities in the form of International Council for Harmonization guidelines for any of our drug candidates that are in preclinical and clinical development. The regulatory authorities enforce cGCPs through periodic inspections of trial sponsors, principal investigators and clinical trial sites. Although we rely on CROs to conduct cGCP-compliant clinical trials, we remain responsible for ensuring that each of our GLP preclinical studies and clinical trials is conducted in accordance with its investigational plan and protocol and applicable laws and regulations, and our reliance on the CROs does not relieve us of our regulatory responsibilities. If we or our CROs fail to comply with cGCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. Accordingly, if our CROs fail to comply with these regulations or fail to recruit a sufficient number of subjects, we may be required to repeat clinical trials, which would delay the regulatory approval process.

While we have agreements governing their activities, our CROs are not our employees, and we do not control whether or not they devote sufficient time and resources to our future clinical and preclinical programs. These CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials, or other drug development activities which could harm our business. We face the risk of potential unauthorized disclosure or misappropriation of our intellectual property by CROs, which may reduce our trade secret protection and allow our potential competitors to access and exploit our proprietary technology. If our CROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for any other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize any drug candidate that we develop. As a result, our financial results and the commercial prospects for any drug candidate that we develop would be harmed, our costs could increase, and our ability to generate revenue could be delayed.

In addition, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA. The FDA may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the trial. The FDA may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA and may ultimately lead to the denial of marketing approval of our current and future drug candidates.

If our relationship with these CROs terminates, we may not be able to enter into arrangements with alternative CROs or do so on commercially reasonable terms. Switching or adding additional CROs involves substantial cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can negatively impact our ability to meet our desired clinical development timelines. Though we intend to carefully manage our relationships with our CROs, there can be no assurance that we will not

encounter challenges or delays in the future or that these delays or challenges will not have a negative impact on our business, financial condition and prospects.

Risks Related to Our Business Operations, Employee Matters and Managing Growth

We are highly dependent on the services of our senior management team, including our President and Chief Executive Officer, Margaret "Meg" Alexander, and our Executive Chairman, Dr. Jeremy M. Levin, and if we are not able to retain these members of our management team or recruit and retain additional management, clinical and scientific personnel, our business will be harmed.

We are highly dependent on our senior management team, including our President and Chief Executive Officer, Margaret "Meg" Alexander, and our Executive Chairman, Dr. Jeremy M. Levin. The employment agreements we have with these officers do not prevent such persons from terminating their employment with us at any time. The loss of the services of any of these persons could impede the achievement of our research, development, operational, financial and commercialization objectives.

In addition, we are dependent on our continued ability to attract, retain and motivate highly qualified additional management, clinical and scientific personnel. If we are not able to retain our management and to attract, on acceptable terms, additional qualified personnel necessary for the continued development of our business, we may not be able to sustain our operations or grow. This risk may be further amplified given the particularly competitive hiring market in New York City, the location of our corporate headquarters.

We may not be able to attract or retain qualified personnel in the future due to the intense competition for qualified personnel among biotechnology, pharmaceutical and other businesses. Many of the other pharmaceutical companies that we compete against for qualified personnel and consultants have greater financial and other resources, different risk profiles and a longer history in the industry than we do. They also may provide more diverse opportunities and better chances for career advancement. Some of these characteristics may be more appealing to high-quality candidates and consultants than what we have to offer. If we are unable to continue to attract, retain and motivate high-quality personnel and consultants to accomplish our business objectives, the rate and success at which we can discover and develop drug candidates and our business will be limited and we may experience constraints on our development objectives.

Our future performance will also depend, in part, on our ability to successfully integrate newly hired executive officers into our management team and our ability to develop an effective working relationship among senior management. Our failure to integrate these individuals and create effective working relationships among them and other members of management could result in inefficiencies in the development and commercialization of our drug candidates, harming future regulatory approvals, sales of our drug candidates and our results of operations. Additionally, we do not currently maintain "key person" life insurance on the lives of our executives or any of our employees.

We may need to expand our organization, and we may experience difficulties in managing this growth, which could disrupt our operations.

In connection with our future growth plans, we may need to hire additional employees. We may have difficulties in connection with identifying, hiring, integrating and retaining new personnel. Future growth would impose significant additional responsibilities on management, including the need to identify, recruit, maintain, motivate and integrate additional employees, consultants and contractors. Also, our management may need to divert a disproportionate amount of its attention away from our day-to-day operations and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational inefficiencies, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of our current and potential future drug candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and grow revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance, our ability to commercialize drug candidates, develop a scalable infrastructure and compete effectively will depend, in part, on our ability to effectively manage any future growth.

Many of the other pharmaceutical and healthcare companies that we compete against for qualified personnel and consultants have greater financial and other resources, different risk profiles and a longer operating history in the industry than us. They also may provide more diverse opportunities and better chances for career advancement. Some of these opportunities may be more appealing to high-quality candidates and consultants than what we have to offer. If we are

unable to continue to attract and retain high-quality personnel and consultants, the rate and success at which we can discover and develop our products and product candidates will be harmed, which could negatively impact our financial condition, results of operations and cash flows.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk that our employees, consultants, distributors, principal investigators, collaborators and commercial partners may engage in fraudulent or illegal activity. Misconduct by these parties could include intentional, reckless or negligent conduct or disclosure of unauthorized activities to us that violates the regulations of the FDA and non-U.S. regulators, including those laws requiring the reporting of true, complete and accurate information to such regulators, manufacturing standards, healthcare fraud and abuse laws and regulations in the United States and abroad or laws that require the true, complete and accurate reporting of financial information or data. In particular, sales, marketing and business arrangements in the healthcare industry, including the sale of pharmaceuticals, are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. It is not always possible to identify and deter misconduct by our employees and other third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. Further, because of our hybrid-work policies, information that is normally protected, including confidential company information, may be less secure. If actions are instituted against us and we are not successful in defending ourselves or asserting our rights, those actions could result in the imposition of significant fines or other sanctions, including the imposition of civil, criminal and administrative penalties, damages, monetary fines, imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, contractual damages, reputational harm, diminished profits and future earnings and curtailment of operations, any of which could adversely affect our ability to operate our business and our results of operations. Whether or not we are successful in defending against such actions or investigations, we could incur substantial costs, including legal fees, and divert the attention of management in defending ourselves against any of these claims or investigations.

If our information technology systems or data, or those of the third parties upon with whom we work, are or were compromised, we could experience adverse impacts resulting from such compromise, including, but not limited to, regulatory investigations or actions, litigation, fines and penalties, interruptions to our operations such as our clinical trials, claims that we breached our data protection obligations, harm to our reputation, and a loss of future customers or sales and other adverse consequences.

We are increasingly dependent on information technology systems and infrastructure, including mobile technologies, to operate our business. In the ordinary course of our business, we receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, process) large amounts of sensitive data, and, as a result, we and the third parties with whom we work face a variety of continuously evolving threats that have caused and could cause future security incidents. Cyberattacks, malicious internet-based activity, online and offline fraud, and other similar activities are increasing in their frequency, levels of persistence, sophistication and intensity, and are also being conducted by sophisticated and organized groups and individuals with a wide range of motives (including, but not limited to, industrial espionage) and expertise, including organized criminal groups, "hacktivists," nation states and others. Such attacks could include the deployment of harmful malware (including as a result of advanced persistent threat intrusions), ransomware attacks, denial-of-service attacks, credential stuffing and/or harvesting, social engineering (including through deep fakes, which may be increasingly more difficult to identify as fake, and phishing attacks), supply-chain attacks, software bugs, server malfunctions, software or hardware failures, loss of sensitive data or other information technology assets, adware, attacks enhanced or facilitated by AI, telecommunications failures, earthquakes, fires, floods and other means to affect service reliability and threaten the confidentiality, integrity and availability of our information systems and sensitive data. In particular, severe ransomware attacks are becoming increasingly prevalent and can lead to significant interruptions in our operations, ability to provide our products or services, loss of sensitive data and income, reputational harm, and diversion of funds. Extortion payments may alleviate the negative impact of a ransomware attack, but we may be unwilling or unable to make such payments due to, for example, applicable laws or regulations prohibiting such payments.

In addition, many of those third parties in turn subcontract or outsource some of their responsibilities to other third parties. While all information technology operations are inherently vulnerable to inadvertent or intentional security

breaches, incidents, attacks and exposures, the accessibility and distributed nature of our information technology systems, and the sensitive data stored on those systems, make such systems vulnerable to unintentional or malicious, internal and external attacks on our technology environment. We have also outsourced elements of our operations (including elements of our information technology infrastructure) to third parties, and as a result, certain third parties with whom we work have access to our computer networks and/or our sensitive data. In addition, many of those third parties in turn subcontract or outsource some of their responsibilities to other third parties. Our ability to monitor the third parties' information security practices is limited, and these third parties may not have adequate information security measures in place. When the third parties with whom we work experience a security incident or other interruption, which has occurred in the past, we could experience adverse consequences. For example, in the third quarter of 2024, we were notified of a business email compromise impacting a development collaborator, which resulted in our payment being misdirected to a fraudulent account. The funds were fully recovered in January 2025. In the absence of recovery of funds or other remuneration, we may be entitled to damages if our third-party service providers fail to satisfy their privacy or security-related obligations to us, any award may be insufficient to cover our damages, or we may be unable to recover such award. In addition, supply chain attacks have increased in frequency and severity, and we cannot guarantee that third parties' infrastructure in our supply chain or our third-party partners' supply chains have not been compromised. Increasing geopolitical tensions are likely to increase the frequency of cybersecurity attacks.

In addition, hybrid work has increased risks to our information technology systems and data as our employees utilize network connections, computer and devices outside our premises or network, including working at home, while in transit and in public locations. Additionally, future or past business transactions (such as acquisitions or integrations) could expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. Furthermore, we may discover security issues that were not identified during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

We take steps designed to detect, mitigate, and remediate vulnerabilities in our information systems (such as our hardware and/or software, including that of third parties upon which we rely); however, we may not detect and remediate all such vulnerabilities on a timely basis. Further, we may and have experienced delays in deploying remedial measures and patches designed to address identified vulnerabilities. For example, we have had situations in which a vulnerability has been identified, but the remediating patch download and installation was delayed due to the user being offline or the computer not being rebooted in a timely manner to finalize the installation. Non-remediated vulnerabilities could be exploited and result in a security incident.

Significant disruptions of our, our third-party vendors' and/or business partners' information technology systems or other similar data security incidents could adversely affect our business operations and/or result in the loss, misappropriation, and/or unauthorized access, use or disclosure of, or the prevention of access to, sensitive data, which could result in financial, legal, regulatory, business and reputational harm to us. In addition, information technology system disruptions, whether from attacks on our technology environment or from computer viruses, natural disasters, terrorism, war and telecommunication and electrical failures, could result in a material disruption of our development programs and our business operations. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data.

It may be difficult and/or costly to detect, investigate, mitigate, contain and remediate a security incident. Our efforts to do so may not be successful. Actions taken by us or the third parties with whom we work to detect, investigate, mitigate, contain, and remediate a security incident could result in outages, data losses and disruptions of our business. For example, we utilize a phishing reporting feature that allows our IT function to analyze and assess whether suspicious emails are phishing attempts. This system is reliant on AI and employee awareness and diligence, both of which may be exploited or not always successful. Actions taken by us or the third parties with whom we work to detect, investigate, mitigate, contain and remediate a security incident could result in outages, data losses, and disruptions of our business. Threat actors may also gain access to other networks and systems after a compromise of our networks and systems. For example, threat actors may use an initial compromise of one part of our environment to gain access to other parts of our environment, or leverage a compromise of our networks or systems to gain access to the networks or systems of third parties with whom we work, such as through phishing or supply chain attacks. We have in the past and will in the future expend significant resources or modify our business activities to try to protect against current and constantly evolving security incidents. Additionally, certain data privacy and security obligations will require us to implement and maintain specific security measures or industry-standard or reasonable security measures to protect our information technology systems and sensitive data.

Applicable data privacy and security obligations may require us to notify relevant stakeholders, including affected individuals, customers, regulators, and investors, of security incidents. Such disclosures are costly, and the disclosure or the failure to comply with such requirements could lead to adverse consequences.

If we (or a third party with whom we work) experience a security incident or are perceived to have experienced a security incident, including but not limited to a security incident involving personal information regarding our patients or employees, we may experience adverse consequences, such as disruptions to our business, harm to our reputation, government enforcement actions (for example, investigations, fines, penalties, audits, and inspections), additional reporting requirements, and/or oversight, or we may otherwise be subject to liability under laws, regulations and contractual obligations, including those that protect the privacy and security of personal information. This could result in increased costs to us, and result in significant legal and financial exposure and/or reputational harm. In addition, any failure or perceived failure by us or our vendors or business partners to comply with our privacy, confidentiality or data security-related legal or other obligations to third parties, or any further security incidents or other inappropriate access events that result in the unauthorized access, release or transfer of sensitive data, may result in governmental investigations, enforcement actions, regulatory fines, litigation, or public statements against us by advocacy groups or others, and could cause third parties, including clinical sites, regulators or current and potential partners, to lose trust in us or we could be subject to claims by third parties that we have breached our privacy- or confidentiality-related obligations, which could materially and adversely affect our business and prospects. Moreover, data security incidents and other inappropriate access can be difficult to detect, and any delay in identifying them may lead to increased harm of the type described above.

While we have implemented security measures intended to protect our information technology systems and infrastructure, there can be no assurance that such measures will be effective. Our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our data privacy and security obligations.

While we are seeking cybersecurity insurance coverage to cover certain aspects of the cyber risks described above, there is no guarantee that we will obtain cybersecurity insurance coverage sufficient to cover the risks described here. Furthermore, any losses suffered by the company may not be adequately covered by insurance or other contractual rights available to us. The successful assertion of one or more large claims against us that exceed or are not covered by our insurance coverage or changes in our insurance policies, including premium increases or the imposition of large deductible or co-insurance requirements, could make us unable to acquire such insurance and may have an adverse effect on our business, financial condition, and results of operations.

In addition to experiencing a security incident, third parties may gather, collect or infer sensitive information about us from public sources, data brokers or other means that reveal competitively sensitive details about our organization and could be used to undermine our competitive advantage or market position. Additionally, sensitive company information could be leaked, disclosed or revealed as a result of or in connection with our employees', personnel's, or vendors' use of generative AI technologies.

We and the third parties with whom we work are subject to stringent and evolving privacy and security laws, regulations, contractual obligations, industry standards, policies, and other obligations, and our failure or perceived failure to comply with such obligations could result in regulatory investigations or actions, litigation (including class actions), fines and penalties, disruptions of our business operations, loss of revenue or profits, reputational damage and other adverse business consequences.

In the ordinary course of business, we and the third parties with whom we work process personal data and other sensitive information, including proprietary and confidential business data, trade secrets, intellectual property, sensitive third-party data, business plans, transactions, clinical trial data and financial information (collectively, sensitive data).

Our data processing activities subject us to laws and regulations covering data privacy and the protection of personal information and other sensitive data. The legislative and regulatory landscape for privacy and data protection continues to evolve, and there has been an increasing focus on privacy and data protection issues which may affect our business. In the United States, there are state security breach notification laws, state health information privacy laws and federal and state consumer protections laws which impose requirements for the collection, use, disclosure and transmission of personal information. Each of these laws is subject to varying interpretations by courts and government agencies. If we fail to comply with applicable laws and regulations we could be subject to penalties or sanctions, including criminal penalties if we knowingly obtain individually identifiable health information from a covered entity in a manner that is not authorized or permitted by HIPAA or for aiding and abetting the violation of HIPAA.

Numerous U.S. states have enacted comprehensive privacy laws that impose certain obligations on covered businesses, including providing specific disclosures in privacy notices and affording residents with certain rights concerning their personal data. As applicable, such rights may include the right to access, correct, or delete certain personal data, and to opt-out of certain data processing activities, such as targeted advertising, profiling, and automated decision-making. The exercise of these rights may impact our business and ability to provide our products and services if we become subject to these laws. For example, California enacted the California Consumer Privacy Act ("CCPA") which applies to personal information of consumers, business representatives, and employees, and requires businesses to provide specific disclosures in privacy notices and honor requests of California residents to exercise certain privacy rights The CCPA provides for civil penalties for violations, as well as a private right of action for certain data breaches. Although there are limited exemptions for clinical trial data under the CCPA (and the other similar state privacy laws), the CCPA and other similar laws may impact (possibly significantly) our business activities depending on how it is interpreted, should we become subject to the CCPA and other state comprehensive privacy laws in the future.

Numerous other countries have, or are developing, laws governing the collection, use and transmission of personal information as well. For example, the European Union's General Data Protection Regulation ("EU GDPR"), the United Kingdom's GDPR ("UK GDPR"), impose strict requirements for processing personal data. The EU GDPR provides for fines of up to 20 million Euros or 4% of annual global revenue, whichever is greater.

In the ordinary course of business, we transfer personal data from Europe and other jurisdictions to the United States or other countries. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal data to other countries. In particular, the European Economic Area (EEA) and the United Kingdom (UK) have significantly restricted the transfer of personal data to the United States and other countries whose privacy laws it believes are inadequate. Other jurisdictions have and may continue to adopt similarly stringent interpretations of their data localization and cross-border data transfer laws. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and UK to the United States in compliance with law, such as the EEA and UK's standard contractual clauses, these mechanisms are subject to legal challenges, and there is no assurance that we can satisfy or rely on these measures to lawfully transfer personal data to the United States. If there is no lawful manner for us to transfer personal data from the EEA, the UK or other jurisdictions to the United States, or if the requirements for a legally-compliant transfer are too onerous, we could face significant adverse consequences, including the interruption or degradation of our operations, the need to relocate part of or all of our business or data processing activities to other jurisdictions at significant expense, increased exposure to regulatory actions, substantial fines and penalties, the inability to transfer data and work with partners, vendors and other third parties, and injunctions against our processing or transferring of personal data necessary to operate our business. Additionally, companies that transfer personal data out of the EEA and UK to other jurisdictions, particularly to the United States, are subject to increased scrutiny from regulators, individual litigants, and activist groups. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers out of Europe for allegedly violating the GDPR's cross-border data transfer limitations. Additionally, the U.S. Department of Justice issued a rule entitled the Preventing Access to U.S. Sensitive Personal Data and Government-Related Data by Countries of Concern or Covered Persons, which places additional restriction on certain data transactions involving countries of concern (e.g., China, Russia, Iran) and covered individuals (i.e., individuals and entities who are designated as such by the U.S. Attorney General or considered "foreign persons" and are majority owned by, organized under the laws of, a primary resident, or a contract of, a covered person or country of concern, as applicable) that may impact certain business activities such as vendor engagements, employment of certain individuals, and investor agreements. Violations of the rule could lead to significant civil and criminal fines and penalties. The rule applies regardless of whether data is anonymized, key-coded, pseudonymized, de-identified or encrypted, which presents particular challenges for companies like ours and may impact our ability to transfer data in connection with certain transactions or agreements.

Our employees and personnel use generative AI technologies to perform their work, and the disclosure and use of personal data in generative AI technologies is subject to various privacy laws and other privacy obligations. Governments have passed and are likely to pass additional laws regulating generative AI. Our use of this technology could result in additional compliance costs, regulatory investigations and actions, and lawsuits. If we are unable to use generative AI, it could make our business less efficient and result in competitive disadvantages.

In addition to data privacy and security laws, we are bound by certain contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. We also publish privacy policies, marketing materials, and other statements regarding data privacy and security. Regulators are increasingly scrutinizing these statements, and if these policies, materials, or statements are found to be deficient, lacking in transparency, deceptive, unfair, misleading or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators, or other adverse consequences.

We may at times fail (or be perceived to have failed) in our efforts to comply with our data privacy and security obligations. Moreover, despite our efforts, our personnel or third parties with whom we work may fail to comply with such obligations, which could negatively impact our business operations. If we or the third parties with whom we work 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 enforcement actions (e.g., investigations, fines, penalties, audits, inspections and similar); litigation (including class-action claims) and mass arbitration demands; additional reporting requirements and/or oversight; bans on processing personal data (including clinical trial data); and orders to destroy or not use personal data.

Our ability to use our net operating loss ("NOL") carryforwards and certain other tax attributes to offset future taxable income may be subject to limitation.

Our NOL carryforwards could expire unused and be unavailable to offset future income tax liabilities because of their limited duration or because of restrictions under U.S. tax law. Under applicable U.S. tax law, federal NOLs generated in tax years beginning on or before December 31, 2017 are permitted to be carried forward for only 20 years, and federal NOLs incurred in taxable years beginning after December 31, 2017 may be carried forward indefinitely, but the utilization of such federal NOLs is limited.

In addition, under Section 382 and Section 383 of the Internal Revenue Code of 1986, as amended (the "Code"), and corresponding provisions of state law, if a corporation undergoes an "ownership change," its ability to use its pre-change NOL carryforwards and other pre-change tax attributes (such as research tax credits) to offset its post-change income may be limited. A Section 382 "ownership change" generally occurs if one or more stockholders or groups of stockholders who own at least 5% of our stock increase their ownership by more than 50 percentage points (by value) over their lowest ownership percentage over a rolling three-year period. On August 10, 2015 and February 22, 2019, we experienced ownership changes, and we may have experienced other ownership changes in the past and may experience ownership changes in the future as a result of shifts in our stock ownership (some of which are outside our control). In October 2025 and March 2026, we completed financings but did not conduct a formal Section 382 study. As a result, if we earn net taxable income, our ability to use our pre-change NOLs and certain other tax attributes to offset such taxable income may be subject to limitations. Similar provisions of state tax law may also apply to limit our use of accumulated state tax attributes. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

For the years ended December 31, 2025 and 2024, we recorded no U.S. federal or state income tax provision, based on pre-tax losses of $17.4 million and $26.4 million, respectively. As of December 31, 2025, we had approximately $214.9 million of unused NOL carryforwards for U.S. federal income tax purposes, $202.7 million of unused NOL carryforwards for New York and other state income tax purposes, and $163.8 million of unused NOL carryforwards for New York City income tax purposes, that may be applied against future taxable income. Our NOL carryforwards are significantly limited such that even if we achieve profitability in future periods, we may not be able to utilize most of the NOL carryforwards, which could have a material adverse effect on cash flow and results of operations.

Changes in tax laws or regulations that are applied adversely to us or our customers may have a material adverse effect on our business, cash flow, financial condition, or results of operations.

New tax laws, statutes, rules, regulations, or ordinances could be enacted at any time. For example, the Tax Cuts and Jobs Act, the Coronavirus Aid, Relief, and Economic Security Act, the IRA, and OBBBA made many significant changes to U.S. tax laws, including the imposition by the IRA of, among other rules, a 15% minimum tax on the book income of certain large corporations and a 1% excise tax on certain corporate stock repurchases. Further, existing tax laws, statutes, rules, regulations, or ordinances could be interpreted differently, changed, repealed, or modified at any time. Any such enactment, interpretation, change, repeal, or modification could adversely affect us, possibly with retroactive effect. In particular, changes in corporate tax rates, the realization of net deferred tax assets, the taxation of foreign earnings, and the deductibility of expenses could have a material impact on the value of our deferred tax assets, resulting in significant one-time charges, and increase our future tax expenses.

Risks Related to Being a Public Company

We are a "smaller reporting company" and the reduced disclosure requirements applicable to such companies may make our common stock less attractive to investors.

We are currently a "smaller reporting company" as defined in the Securities Exchange Act of 1934, as amended. We will be a smaller reporting company and may take advantage of the scaled-back disclosures available to smaller

reporting companies for so long as (i) the market value of our voting and non-voting ordinary shares held by non-affiliates is less than $250.0 million measured on the last business day of our most recently completed second fiscal quarter or (ii) (a) our annual revenue is less than $100.0 million during the most recently completed fiscal year and (b) the market value of our voting and non-voting ordinary shares held by non-affiliates is less than $700.0 million measured on the last business day of our most recently completed second fiscal quarter.

As a smaller reporting company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation. If investors consider our common stock less attractive as a result of our election to use the scaled-back disclosure permitted for smaller reporting companies, there may be a less active trading market for our common shares and our stock price may be more volatile.

We have previously identified material weaknesses in our internal control over financial reporting. If we fail to maintain an effective system of internal control over financial reporting in the future, we may not be able to accurately report our financial condition, results of operations or cash flows, which may adversely affect investor confidence in us and, as a result, the value of our common stock.

During the three month period ended September 30, 2024, we identified material weaknesses in our internal control over financial reporting, which were subsequently remediated as of December 31, 2024. In the future, if we are unable to identify or remediate material weaknesses, or if we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial condition, results of operations or cash flows, which may adversely affect investor confidence in us and, as a result, the value of our common stock.

The Sarbanes-Oxley Act requires, among other things, that we maintain effective internal controls over financial reporting and disclosure controls and procedures. We are required, under Section 404, to furnish a report by management on, among other things, the effectiveness of our internal control over financial reporting. This assessment will need to include disclosure of any material weaknesses identified by our management in our internal control over financial reporting. Section 404 also generally requires an attestation from our independent registered public accounting firm on the effectiveness of our internal control over financial reporting. We will not be required to have our auditors formally attest to the effectiveness of our internal control over financial reporting unless we cease to qualify as a non-accelerated filer.

During the three month period ended September 30, 2024, we were the victim of a criminal scheme involving a business email compromise at one of our development collaborators which led to a fraudulent transfer of $1.8 million to a third-party impersonating one of our development collaborators. The funds were subsequently fully recovered. As a result of the misdirection of funds, management re-evaluated the effectiveness of our disclosure controls and procedures and internal control over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act. Based on this assessment, management identified material weaknesses related to fund transfers and vendor-related information updates. A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting such that a reasonable possibility exists that a material misstatement of our annual or condensed interim financial statements would not be prevented or detected on a timely basis.

Effective internal controls are necessary for us to provide reliable financial reports and prevent fraud. Immediately following the incident, we initiated a reassessment of our processes and controls related to fund transfers and vendor-related information updates and developed an action plan that remediated this matter.

Our compliance with Section 404 in future periods may require that we incur substantial expense and expend significant management efforts. We currently do not have an internal audit group and rely on experienced consultants to support this function. We may need to hire additional consultants or accounting and financial staff with appropriate public company experience and technical accounting knowledge in order to continually comply with Section 404. We may not be able to complete our evaluation, testing and any required remediation in a timely fashion. We cannot assure you that there will not be additional material weaknesses or significant deficiencies in our internal control over financial reporting in the future. Any failure to maintain internal control over financial reporting could severely inhibit our ability to accurately report our financial condition, results of operations or cash flows. If we are unable to conclude that our internal control over financial reporting is effective, or if our independent registered public accounting firm determines we have any additional material weakness in our internal control over financial reporting, we could lose investor confidence in the accuracy and completeness of our financial reports, the market price of our common stock could decline, and we could be subject to sanctions or investigations by The Nasdaq Stock Market LLC, the SEC or other regulatory authorities. Failure to

remedy material weaknesses in our internal control over financial reporting, or to implement or maintain other effective control systems required of public companies, could also restrict our future access to the capital markets.

Risks Related to the Ownership of Our Common Stock and Other General Matters

The market price of our common stock has been and will likely remain volatile and fluctuate substantially, which could result in substantial losses for holders of our common stock.

The market price of our common stock has been and likely will remain volatile. For example, during the year ended December 31, 2025, the closing price of our common stock on the Nasdaq Global Select Market and Nasdaq Capital Markets ranged from $0.26 per share to $1.84 per share. In June 2024, we experienced a stock price drop following our announcement of Takeda's release of topline Phase 3 study results for soticlestat, noting that soticlestat narrowly missed its primary endpoint and showed clinically meaningful and significant effects in multiple key secondary efficacy endpoints with respect to Dravet syndrome and missed its primary endpoint with respect to Lennox-Gastaut syndrome. In addition, the stock market in general and the market for biopharmaceutical or pharmaceutical companies in particular, has experienced extreme volatility that has often been unrelated to the operating performance of particular companies, which has resulted in decreased stock prices for many companies notwithstanding the lack of a fundamental change in their underlying business models or prospects. Broad market and industry factors, including potentially worsening economic conditions and other adverse effects or developments relating to new or ongoing public health crises or other inflationary factors, may negatively affect the market price of our common stock, regardless of our actual operating performance. As a result of this volatility, stockholders may lose all or part of their investment in our common stock since they may be unable to sell their shares at or above the price paid for the shares. The market price for our common stock may be influenced by many factors, including:

- results of clinical trials of our current and any future drug candidates or those of our competitors;
- the success of competitive drugs or therapies;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to our current and any future drug candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional drug candidates;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- our inability to obtain or delays in obtaining adequate drug supply for any approved drug or inability to do so at acceptable prices;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions, including supply chain disruptions and inflationary pressure; and
- the other factors described in this "Risk Factors" section.

In addition, in the past stockholders have initiated class action lawsuits against companies following periods of volatility in the market prices of these companies' stock. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management's attention and resources.

Concentration of ownership of our common stock among our executive officers, directors and principal stockholders may prevent new investors from influencing significant corporate decisions.

Based upon shares of our common stock outstanding as of December 31, 2025, our executive officers, directors and stockholders who owned more than 5% of our outstanding common stock, in the aggregate, beneficially own shares representing approximately 62.0% of our outstanding common stock.

Takeda, a greater than 5% holder, has agreed to, among other things, (i) a standstill provision, (ii) restrictions on its ability to sell or otherwise transfer its shares of our stock, (iii) vote its shares on certain matters in accordance with the holders of a majority of shares of our common stock, and (iv) restrictions on the percentage of our outstanding common stock it may own, in accordance with the terms of the RLT Agreement.

If our executive officers, directors and stockholders who owned more than 5% of our outstanding common stock acted together, they may be able to significantly influence all matters requiring stockholder approval, including the election and removal of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. The concentration of voting power, Takeda standstill provisions, voting obligations and transfer restrictions could delay or prevent an acquisition of our company on terms that other stockholders may desire or result in the management of our company in ways with which other stockholders disagree.

If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock relies, in part, on the research and reports that industry or financial analysts publish about us or our business. We currently have research coverage offered by several industry or financial analysts. We do not have any control over these analysts. If one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If additional analysts cease to cover our stock or fail to regularly publish reports, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

Because we do not anticipate paying any dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be stockholders' sole source of gain.

We have never declared or paid dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be stockholders' sole source of gain for the foreseeable future.

Provisions in our organizational documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our amended and restated certificate of incorporation ("Certificate of Incorporation") and our amended and restated bylaws ("Bylaws") may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which they might otherwise receive a premium for their shares. These provisions also could limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that not all members of the board of directors are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board of directors;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;

- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a stockholder rights plan, or so-called "poison pill," that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 66 2/3% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our Certificate of Incorporation or Bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law (the "DGCL"), which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

Additionally, the Takeda standstill provisions and transfer restrictions in the RLT Agreement may delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which stockholders might otherwise receive a premium for their shares.

We may be subject to securities litigation, which is expensive and could divert management attention.

The market price of our common stock may be volatile. In the past, companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. We may be the target of this type of litigation in the future. Securities litigation against us could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business.

Some provisions of our organizational documents and the DGCL may have anti-takeover effects that could discourage an acquisition of us by others, even if an acquisition would benefit our stockholders and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our Certificate of Incorporation and Bylaws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us or increase the cost of acquiring us, even if doing so would benefit our stockholders, or remove our current management. These provisions include:

- authorizing the issuance of "blank check" preferred stock, the terms of which we may establish and shares of which we may issue without stockholder approval;
- prohibiting cumulative voting in the election of directors, which would otherwise allow for less than a majority of stockholders to elect director candidates;
- prohibiting stockholder action by written consent, thereby requiring all stockholder actions to be taken at a meeting of our stockholders;
- eliminating the ability of stockholders to call a special meeting of stockholders; and
- establishing advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted upon at stockholder meetings.

These provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors, who are responsible for appointing the members of our management. Because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the DGCL, which may discourage, delay or prevent someone from acquiring us or merging with us whether or not it is desired by or beneficial to our stockholders. Under the DGCL, a corporation may not, in general, engage in a business combination with any holder of 15% or more of its capital stock unless the holder has held the stock for three years or, among other things, the board of directors has approved the transaction. Any provision of our Certificate of Incorporation or Bylaws or Delaware law that has the effect of delaying or deterring a change of control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock and could also affect the price that some investors are willing to pay for our common stock.

Item 1B. Unresolved Staff Comments

None.

Item 1C. Cybersecurity

Risk Management and Strategy

We have implemented and maintain various information security processes designed to identify, assess and manage material risks from cybersecurity threats to our critical computer networks, third-party hosted services, communications systems, hardware and software, and our critical data, including intellectual property and confidential information that is proprietary, strategic or competitive in nature ("Information Systems and Data"). Our information security function is led by our Director of Information Technology, overseen by our Senior Vice President of Finance and Financial Planning, with the support of our third-party specialists. Our information security function helps identify, assess and manage material risks from cybersecurity threats by monitoring and evaluating our threat environment using various methods, including automated tools, domain name system filtering, antivirus protection, vulnerability scanning and penetration testing.

Depending on the environment, systems and data, we implement and maintain various technical, physical and organizational measures, processes, standards and policies designed to manage and mitigate material risks from cybersecurity threats to our Information Systems and Data. These measures include an incident response policy, encryption of certain data, network security controls, segregation of certain data and system monitoring. Our incident response policy provides the framework for the execution of cybersecurity incident response procedures and internal communications while adhering to applicable legal reporting and disclosure requirements.

Our assessment and management of material risks from cybersecurity threats are integrated into our overall risk management processes. Our information security function works with management to prioritize risk management processes and mitigate cybersecurity threats that are more likely to lead to a material impact to our business.

We use third-party service providers to assist us from time to time to identify, assess and manage material risks from cybersecurity threats. In addition, we use third-party service providers to perform a variety of functions throughout our business, including, for example, hosting and processing data, manufacturing our product candidates and assisting with R&D and clinical trial activities. Depending on the nature of the services provided, the sensitivity of the systems and data at issue, and the identity of the provider, our vendor contracting processes may include imposing certain contractual provisions related to privacy and cybersecurity.

For a description of our cybersecurity risks, refer to the risk factor in Item 1A. "Risk Factors," to this Annual Report on Form 10-K titled "*If our information technology systems or data, or those of the third parties with whom we work, are or were compromised, we could experience adverse impacts resulting from such compromise, including, but not limited to, regulatory investigations or actions, litigation, fines and penalties, interruptions to our operations such as our clinical trials, claims that we breached our data protection obligations, harm to our reputation, and a loss of future customers or sales and other adverse consequences*."

Governance

Our board of directors maintains oversight of our most significant risks and our processes to identify, manage and mitigate those risks. The audit committee of our Board of Directors (the "Audit Committee") has responsibility for oversight of our management of cybersecurity risks.

Our cybersecurity risk assessment and management processes are implemented and maintained by certain management, including our Director of Information Technology, who reports to our Senior Vice President of Finance and Financial Planning. Our Director of Information Technology is responsible for monitoring third-party specialists, helping to integrate cybersecurity risk considerations into our overall risk management strategy and communicating key priorities to relevant personnel. Our Senior Vice President of Finance and Financial Planning is responsible for approving budgets, helping prepare for cybersecurity incidents, approving cybersecurity processes and reviewing security assessments and other security-related reports.

Our incident response policy is designed to escalate certain cybersecurity incidents to members of management depending on the circumstances, including our Senior Vice President of Finance and Financial Planning. Our Senior Vice President of Finance and Financial Planning and, if escalated, other senior management, work with our incident response team to help mitigate and remediate cybersecurity incidents. In addition, our incident response policy includes reporting to the Audit Committee for certain cybersecurity incidents. The incident response policy is managed and maintained by our Director of Information Technology, with oversight from our Senior Vice President of Finance and Financial Planning. The Audit Committee receives periodic updates from our Senior Vice President of Finance and Financial Planning regarding the status of our cybersecurity program and our posture to identify and mitigate risks to our information security.

Item 2. Properties

We currently lease the space for our principal executive offices, which are located at 441 Ninth Avenue, New York, New York, under a ten-year lease agreement which commenced in March 2022. We believe our facilities are adequate to meet current needs.

Item 3. Legal Proceedings

From time to time, we may become subject to various legal proceedings and claims that arise in the ordinary course of our business activities. We are not currently subject to any material legal proceedings.

Item 4. Mine Safety Disclosures

Not applicable.

PART II

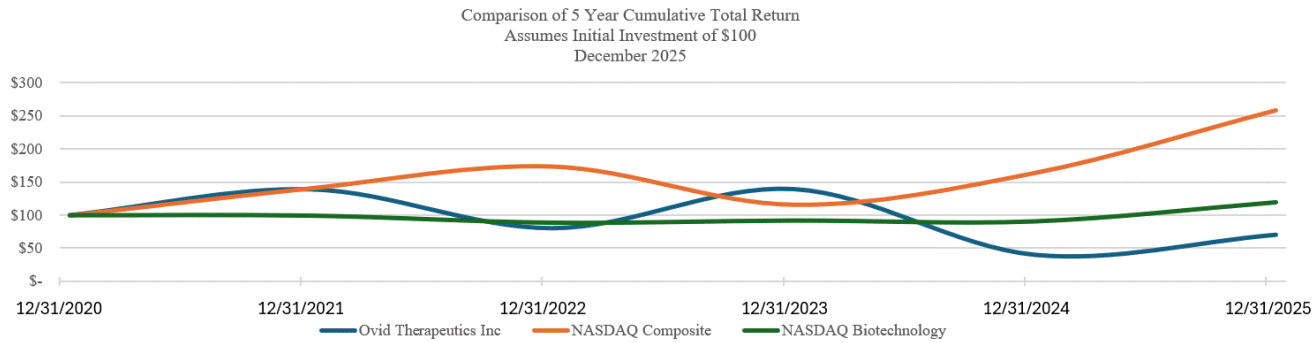
Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities

Market Information

Our common stock began trading on The Nasdaq Global Select Market on May 5, 2017, under the symbol "OVID." In August 2025, we transferred our listing to the Nasdaq Capital Market.

Comparative Stock Performance Graph

The following performance graph and related information 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 or Exchange Act. The following graph shows a comparison from December 31, 2020 through December 31, 2025, of the cumulative total return for our common stock, the Nasdaq Composite Index and the Nasdaq Biotechnology Index.



The graph assumes an initial investment of $100 on December 31, 2020. The comparisons in the graph are not intended to forecast or be indicative of possible future performance of our common stock.

Holders of Record

As of March 16, 2026, we had approximately 18 holders of record of our common stock. Certain shares are held in "street name" and accordingly, the number of beneficial owners of such shares is not known or included in the foregoing number. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

Dividend Policy

We have never declared or paid any dividends on our common stock. We currently intend to retain future earnings to fund the development and growth of our business. We do not expect to pay any dividends in the foreseeable future. Any future determination to pay dividends will be made at the discretion of our board of directors and will depend on then-existing conditions, including our financial conditions, operating results, contractual restrictions, capital requirements, business prospects and other factors our board of directors may deem relevant.

Recent Sales of Unregistered Securities

None.

Item 6. [Reserved]

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following discussion and analysis should be read in conjunction with our financial statements and related notes included elsewhere in this Annual Report on Form 10-K. This discussion and analysis and other parts of this Annual Report on Form 10-K contain forward-looking statements based upon current beliefs, plans and expectations that involve risks, uncertainties and assumptions, such as statements regarding our plans, objectives, expectations, intentions and projections. Our actual results and the timing of selected events could differ materially from those anticipated in these forward-looking statements as a result of several factors, including those set forth under "Risk Factors" and elsewhere in

this Annual Report on Form 10-K. You should carefully read the "Risk Factors" section of this Annual Report on Form 10-K to gain an understanding of the important factors that could cause actual results to differ materially from our forward-looking statements. Please also see the section entitled "Special Note Regarding Forward-Looking Statements."

Overview

We are a biopharmaceutical company dedicated to developing small molecule medicines for brain disorders with significant unmet need. Our approach to achieve this goal is scientifically driven, patient-focused, and coupled with an integrated and disciplined approach to research, clinical development and business development. Our team has significant experience with and understanding of epilepsies and other neurological conditions, and we continue to gain insight into the ways the different molecular mechanisms and pathways underlying these disorders impact the symptoms patients experience. We have developed a differentiated pipeline of drug candidates containing novel mechanisms of action ("MoAs") to target seizures and believe we are the only company that holds a portfolio of direct activators of potassium-chloride cotransporter 2 ("KCC2"). One of our programs are in clinical trials in humans, and a second is expected to begin a clinical trial in the first half of 2026. We are initially pursuing therapeutic drug candidates for the potential treatment of drug-resistant focal onset seizures ("FOS"), developmental and epileptic encephalopathies ("DEEs"), including tuberous sclerosis complex ("TSC") seizures and infantile spasms ("IS"), psychosis associated with Parkinson's disease and Lewy body dementia ("LBD"), and schizophrenia. If successfully developed and marketed to treat these conditions, we intend to explore these drug candidates for broader neurologic indications. Our cohesive focus in brain disorders with significant unmet need reinforces our belief that we can develop and produce multiple novel medicines, scale our infrastructure, positively impact patients' lives and create long-term stockholder value.

Since our inception in April 2014, we have devoted substantially all of our efforts to organizing and planning our business, building our management and technical team, acquiring assets and raising capital.

During the years ended December 31, 2025 and 2024, we generated $7.3 million and $0.6 million of royalty and licensing revenue, respectively. We have otherwise primarily funded our business through the sale of our capital stock and through the entry into the royalty, license and termination agreement ("RLT Agreement") with Takeda Company Limited ("Takeda"), which resulted in a one-time up-front payment of $196.0 million in 2021 and the entry into a Royalty Monetization Agreement (the "Ligand Agreement") with Ligand Pharmaceuticals Incorporated ("Ligand"), which resulted in a one-time up-front payment of $30.0 million in 2023. Through December 31, 2025, we have raised net proceeds of $350.5 million from the sale of our capital stock. As of December 31, 2025, we had $90.4 million in cash, cash equivalents and marketable securities. We recorded net losses of $17.4 million and $26.4 million for the years ended December 31, 2025 and 2024, respectively. As of December 31, 2025, we had an accumulated deficit of $321.7 million.

We expect to incur significant expenses and operating losses for at least the next several years. Our net losses may fluctuate significantly from period to period, depending on the timing of our clinical trials and expenditures on our other research and development and commercial development activities. We expect our expenses will increase substantially over time as we:

- continue the ongoing and planned preclinical and clinical development of our drug candidates;
- build a portfolio of drug candidates through the development, acquisition or in-license of drugs, drug candidates or technologies;
- initiate preclinical studies and clinical trials for any additional drug candidates that we may pursue in the future;
- seek marketing approvals for our current and future drug candidates that successfully complete clinical trials;
- establish a sales, marketing and distribution infrastructure to commercialize any drug candidate for which we may obtain marketing approval;
- develop, maintain, expand and protect our intellectual property portfolio;
- implement operational, financial and management systems; and
- attract, hire and retain additional administrative, clinical, regulatory, manufacturing, commercial and scientific personnel.

2025 Private Placement

In October 2025, we entered into a Securities Purchase Agreement with the purchasers named therein (the "Investors"), pursuant to which we issued and sold an aggregate of (i) 57,722 shares of our Series B convertible preferred stock, par value $0.001 per share (the "Series B Preferred Stock"), (ii) Series A warrants (the "Series A Warrants") to purchase up to 38,481,325 shares of our common stock and/or pre-funded warrants to purchase common stock (the "Pre-Funded Warrants") and (iii) Series B warrants to purchase up to 28,861,000 shares of common stock and/or Pre-Funded Warrants (the "Series B Warrants" and, together with the Series A Warrants, the "Warrants") to the Investors in a private placement (the "2025 Private Placement"). Each share of Series B Preferred Stock was sold together with a Series A Warrant to purchase up to 666.66 shares of common stock and/or Pre-Funded Warrants (rounded down to next whole share based on each investor's aggregate purchase) and a Series B Warrant to purchase 500 shares of common stock and/or Pre-Funded Warrants (together, a "Security"). The Securities were sold at a purchase price of $1,400.00 per Security to the Investors, which included the purchase of 71 shares of Series B Preferred Stock, 47,333 Series A Warrants, and 35,500 Series B Warrants by our Executive Chairman.

We received initial net proceeds of $75.1 million from the 2025 Private Placement, after deducting placement agent fees and offering expenses. We may further receive up to $94.0 million in additional gross proceeds, assuming exercise in full of the Warrants.

In December 2025, following the approval by our stockholders at a special meeting held on December 11, 2025 of specified proposals, all of the shares of the Series B Preferred Stock automatically converted into an aggregate of 57,722,000 shares of our common stock.

The Warrants each have an exercise price of $1.40 per share (the "Exercise Price"). The Series A Warrants are exercisable and expire on the earlier of (i) October 6, 2030 or (ii) the 30th calendar day following the date on which we publicly announce the clearance of the first of any investigational new drug application, clinical trial application or other foreign equivalent with respect to the clinical development of our OV4071 product candidate. On March 18, 2026, we publicly announced that we received Human Research Ethics Committee (HREC) approval of the Phase 1 clinical trial protocol for OV4071 and acknowledgement of our Clinical Trial Notification (CTN) from the Therapeutic Goods Administration (TGA). Accordingly, pursuant to the terms of the Series A Warrants, the Series A Warrants will expire on April 17, 2026, if not exercised in full. If all Series A Warrants are exercised in full, we would anticipate receiving an additional $53.9 million in gross proceeds, prior to deducting placement agent fees. In the event that beneficial ownership limitations prevent the exercise by an Investor of all or a portion of the Series A Warrants held thereby, such Investor may purchase shares of common stock up to the specified limit and, for the remainder, purchase Pre-Funded Warrants in lieu of shares of common stock.

The Series B Warrants are exercisable and expire on October 6, 2030. In the event that the closing price of our common stock equals or exceeds 300% of the Exercise Price (subject to customary adjustments) for 20 of any 30 consecutive trading days, the Company may elect to require exercise of the Series B Warrants for cash. In the event that beneficial ownership limitations prevent the exercise by an Investor of all or a portion of the Series B Warrants held thereby upon any such mandatory exercise demand, such Investor will purchase shares of common stock up to the specified limit, and for the remainder, purchase Pre-Funded Warrants in lieu of shares of common stock.

Recent Developments

2026 Private Placement

On March 17, 2026, we entered into a Securities Purchase Agreement with the purchasers named therein (the "Investors"), pursuant to which we agreed to issue and sell an aggregate of 19,154,321 shares of our common stock at a purchase price of $2.01 per share and, in lieu of common stock, pre-funded warrants to purchase up to 10,701,710 shares of common stock, at a purchase price $2.009 for each pre-funded warrant, to the Investors in a private placement (the "2026 Private Placement"). The pre-funded warrants will have an exercise price of $0.001 per share and will be immediately exercisable.

We intend to use the net proceeds from the 2026 Private Placement, together with our existing cash, cash equivalents and marketable securities, to provide financing to support the expansion of the development of OV329 into additional indications, including TSC and IS, as well as for general research and development expenses.

We will receive gross proceeds from the 2026 Private Placement of $60.0 million, before placement agent fees and offering expenses. The closing of the 2026 Private Placement is expected to occur on March 19, 2026, subject to customary closing conditions.

Significant Risks and Uncertainties

The global economic slowdown, the overall disruption of global healthcare systems and other risks and uncertainties associated with public health crises and global geopolitical tensions may have a material adverse effect on our business, financial condition, results of operations and growth prospects. The resulting fluctuations in inflation rates may materially affect our business and corresponding financial position and cash flows. Inflationary factors, such as increases in the cost of our clinical trial materials and supplies, interest rates and overhead costs may adversely affect our operating results. Relatively high interest rates also present a challenge impacting the U.S. economy and could make it more difficult for us to obtain traditional financing on acceptable terms, if at all, in the future. Furthermore, economic conditions have produced downward pressure on share prices. Although we do not believe that inflation has had a material impact on our financial position or results of operations to date, we may experience increases in the future on our operating costs, including our labor costs and research and development costs, due to supply chain constraints, global geopolitical tensions, worsening global macroeconomic conditions, and employee availability and wage increases, which may result in additional stress on our working capital resources. Moreover, there is great uncertainty with respect to potential changes in trade regulations, ongoing changes to U.S. and international tariffs and other trade restrictions and trade barriers, renegotiation of international trade agreements or further escalation of trade tension, sanctions and export controls which also increase volatility in the global economy.

In addition, we are subject to other challenges and risks specific to our business and our ability to execute on our strategy, as well as risks and uncertainties common to companies in the pharmaceutical industry with development and commercial operations, including, without limitation, risks and uncertainties associated with: identifying, acquiring or in-licensing products or product candidates; obtaining regulatory approval of product candidates; pharmaceutical product development and the inherent uncertainty of clinical success; the challenges of protecting and enhancing our intellectual property rights; and complying with applicable regulatory requirements.

Financial Operations Overview

Revenue

We have generated revenue under various licensing and collaboration agreements. We have not generated any revenue from commercial drug sales, and we do not expect to generate any further revenue unless or until we obtain regulatory approval and commercialize one or more of our current or future drug candidates. In the future, we may also seek to generate revenue from a combination of research and development payments, license fees and other upfront or milestone payments.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our product discovery efforts and the development of our product candidates, which include, among other things:

- employee-related expenses, including salaries, benefits and stock-based compensation expense;
- fees paid to consultants for services directly related to our drug development and regulatory efforts;
- expenses incurred under agreements with contract research organizations, as well as contract manufacturing organizations and consultants that conduct preclinical studies and clinical trials;
- costs associated with preclinical activities and development activities;
- costs associated with technology and intellectual property licenses;
- milestone payments and other costs and payments under licensing agreements, research agreements and collaboration agreements; and
- depreciation expense for assets used in research and development activities.

Costs incurred in connection with research and development activities are expensed as incurred. Costs for certain development activities, such as clinical trials, are recognized based on an evaluation of the progress to completion of

specific tasks using data such as patient enrollment, clinical site activations or other information provided to us by our vendors.

Research and development activities are and will continue to be central to our business model. We expect our research and development expenses to increase for the foreseeable future as we advance our current and future drug candidates through preclinical studies and clinical trials. The process of conducting preclinical studies and clinical trials necessary to obtain regulatory approval is costly and time-consuming. It is difficult to determine with certainty the duration and costs of any preclinical study or clinical trial that we may conduct. The duration, costs and timing of clinical trial programs and development of our current and future drug candidates will depend on a variety of factors that include, but are not limited to, the following:

- number of clinical trials required for approval and any requirement for extension trials;
- per patient trial costs;
- number of patients who participate in the clinical trials;
- number of sites included in the clinical trials;
- countries in which the clinical trials are conducted;
- length of time required to enroll eligible patients;
- number of doses that patients receive;
- drop-out or discontinuation rates of patients;
- potential additional safety monitoring or other studies requested by regulatory agencies;
- duration of patient follow-up; and
- efficacy and safety profile of the drug candidates.

In addition, the probability of success for any of our current or future drug candidates will depend on numerous factors, including competition, manufacturing capability and commercial viability. We will determine which programs to pursue and how much to fund each program in response to the scientific and clinical success of each drug candidate, as well as an assessment of each drug candidate's commercial potential.

General and Administrative Expenses

General and administrative expenses consist primarily of employee-related expenses, including salaries, benefits and stock-based compensation expense, related to our executive, finance, business development and support functions. Other general and administrative expenses include costs associated with operating as a public company, travel expenses, conferences, professional fees for auditing, tax and legal services, and facility-related costs.

Other Income (Expense), net

Other income (expense), net, primarily consists of interest income and accretion of discount on investments in marketable securities, unrealized gains (losses) on long-term equity investments, changes in the fair value of the royalty monetization liability under the Ligand Agreement, and the impact of a fraudulent funds transfer.

Results of Operations

Comparison of the Years Ended December 31, 2025 and 2024

The following table summarizes the results of our operations for the periods indicated:

(in thousands)	Year Ended December 31, 2025	Year Ended December 31, 2024	Change $
Revenue:			
License and other revenue	$ 7,252	$ 566	$ 6,686
Total revenue	7,252	566	6,686
Operating expenses:			
Research and development	25,582	36,767	(11,185)
General and administrative	24,109	25,684	(1,575)
Total operating expenses	49,691	62,451	(12,760)
Loss from operations	(42,439)	(61,885)	19,446
Other income (expense), net	25,026	35,452	(10,426)
Loss before provision for income taxes	(17,414)	(26,433)	9,019
Provision for income taxes	—	—	—
Net loss	$ (17,414)	$ (26,433)	$ 9,019

Revenue

Revenue of $7.3 million and $0.6 million was recognized for the years ended December 31, 2025 and 2024, respectively, related to royalty agreements.

Research and Development Expenses

(in thousands)	Year Ended December 31, 2025	Year Ended December 31, 2024	Change $
Preclinical and clinical development expenses	$ 15,726	$ 23,965	$ (8,239)
Payroll and payroll-related expenses	7,765	9,889	(2,124)
Other expenses	2,091	2,913	(822)
Total research and development	$ 25,582	$ 36,767	$ (11,185)

Research and development expenses were $25.6 million for the year ended December 31, 2025 compared to $36.8 million for the year ended December 31, 2024. The decrease of $8.2 million in preclinical and development expenses was primarily due to activities related to the pause of the OV888 (GV101) program in late 2024. The decrease in payroll and payroll-related expenses was primarily due to the impact of an organizational restructuring in 2024, which resulted in approximately $1.7 million in severance costs during the period ended December 31, 2024.

General and Administrative Expenses

(in thousands)	Year Ended December 31, 2025	Year Ended December 31, 2024	Change $
Payroll and payroll-related expenses	$ 10,043	$ 13,835	$ (3,792)
Legal and professional fees	8,280	6,573	1,707
General office expenses	5,786	5,276	510
Total general and administrative	$ 24,109	$ 25,684	$ (1,575)

General and administrative expenses were $24.1 million for the year ended December 31, 2025 compared to $25.7 million for the year ended December 31, 2024. The decrease in payroll and related expenses was primarily due to prior year organizational restructuring that reduced non-severance payroll and related expenses by $2.0 million and by $1.8 million of severance costs recorded in 2024. Legal and professional fees increased between the periods in relation to the 2025 Private Placement as well as other non-routine business development related professional services fees.

Other Income (Expense), net

Other income (expense), net was $25.0 million for the year ended December 31, 2025, comprised primarily of $21.0 million unrealized gain on a long-term equity investment, interest and accretion income on investments in U.S. treasuries and gain from the full recovery of funds on a fraudulent funds transfer. For the year ended December 31, 2024, other income (expense), net of $35.5 million was comprised of a $30.0 million decrease in fair value of the royalty monetization liability resulting from Takeda's reported negative soticlestat Phase 3 study results and announcement of program discontinuation, $3.9 million in interest and accretion income on investments in U.S. treasuries, $1.8 million loss on a fraudulent funds transfer and net $3.3 million unrealized gain on long-term equity investments.

Liquidity and Capital Resources

Overview

As of December 31, 2025 and 2024, we had total cash, cash equivalents and marketable securities of $90.4 million and $53.1 million, respectively. We believe that our cash, cash equivalents and marketable securities as of December 31, 2025 are sufficient to fund our current operating plans through at least 12 months from the issuance of the financial statements contained in our Annual Report on Form 10-K.

Similar to other development-stage biotechnology companies, we have generated limited revenue. We have incurred losses and experienced negative operating cash flows in most years since our inception and anticipate that we will continue to incur losses for at least the next several years. We incurred net losses of $17.4 million and $26.4 million for the years ended December 31, 2025 and 2024, respectively. As of December 31, 2025, we had an accumulated deficit of $321.7 million and working capital of $66.1 million.

2025 Private Placement

In October 2025, we sold an aggregate of (i) 57,722 shares of Series B Preferred Stock, (ii) Series A Warrants to purchase up to 38,481,325 shares of our common stock and/or Pre-Funded Warrants and (iii) Series B Warrants to purchase up to 28,861,000 shares of common stock and/or Pre-Funded Warrants to the Investors in the 2025 Private Placement. The Securities were sold at a purchase price of $1,400.00 per Security to the Investors, which included the purchase of 71 shares of Series B Preferred Stock, 47,333 Series A Warrants, and 35,500 Series B Warrants by our Executive Chairman. We received initial net proceeds of $75.1 million from the 2025 Private Placement, after deducting placement agent fees and offering expenses. We may further receive up to $94.0 million in additional gross proceeds, assuming exercise in full of the Warrants.

The Warrants each have an exercise price of $1.40 per share. The Series A Warrants are exercisable and expire on the earlier of (i) October 6, 2030 or (ii) the 30th calendar day following the date on which we publicly announce the clearance of the first of any investigational new drug application, clinical trial application or other foreign equivalent with respect to the clinical development of our OV4071 product candidate. On March 18, 2026, we publicly announced that we received HREC approval of the Phase 1 clinical trial protocol for OV4071 and acknowledgement of our CTN from the TGA. Accordingly, pursuant to the terms of the Series A Warrants, the Series A Warrants will expire on April 17, 2026, if not exercised in full. If all Series A Warrants are exercised in full, we would anticipate receiving an additional $53.9 million in gross proceeds, prior to deducting placement agent fees. The Series B Warrants are exercisable and expire on October 6, 2030. In the event that the closing price of the Company's common stock equals or exceeds 300% of the Exercise Price (subject to customary adjustments) for 20 of any 30 consecutive trading days, the Company may elect to require exercise of the Series B Warrants for cash. If all Series B Warrants are exercised in full, we would expect to receive an additional $40.1 million in gross proceeds, prior to deducting placement agent fees.

2026 Private Placement

On March 17, 2026, we entered into a Securities Purchase Agreement related to the 2026 Private Placement. We expect to receive gross proceeds from the 2026 Private Placement of $60.0 million, before placement agent fees and offering expenses. The closing of the 2026 Private Placement is expected to occur on March 19, 2026, subject to customary

closing conditions. We intend to use the net proceeds from the 2026 Private Placement, together with our existing cash, cash equivalents and marketable securities, to provide financing to support the expansion of the development of OV329 into additional indications, including TSC and IS, as well as for general research and development expenses.

At-the-Market Offering Program

In November 2023, we filed a shelf registration statement on Form S-3 (Registration No. 333-275307) that allows us to sell up to an aggregate of $250.0 million of our common stock, preferred stock, convertible debt securities and/or warrants, which includes a prospectus covering the issuance and sale of up to $75.0 million of common stock pursuant to our at-the-market ("ATM") program. During the years ended December 31, 2025 and 2024, we did not sell any shares under our ATM program. Since December 31, 2025, we have sold 1,500,000 shares through our ATM program for gross proceeds of approximately $2.4 million before deducting sales agent fees and other offering expenses.

Future Funding Requirements

We expect our expenses to continue to increase in connection with our ongoing activities, particularly as we conduct clinical trials of, and seek marketing approval for, our product candidates and advance our other programs. Our primary uses of capital are, and we expect will continue to be, compensation and related expenses, third-party clinical research and development services, clinical trial costs, legal and other regulatory expenses and general overhead costs. We have based our estimates on assumptions that may prove to be incorrect, and we could use our capital resources sooner than we expect. Additionally, the process of testing drug candidates in clinical trials is costly, and the timing of progress in these trials is uncertain. We cannot estimate the actual amounts necessary to successfully complete the development and commercialization of our drug candidates or whether, or when, we may achieve profitability.

We have no products approved for commercial sale and have not generated any revenues from product sales to date. Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings and additional funding from license and collaboration arrangements. Except for any obligations of our collaborators to reimburse us for research and development expenses or to make milestone or royalty payments under our agreements with them, we will not have any committed external source of liquidity. To the extent that we raise additional capital through future equity offerings or debt financings, ownership interests may be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Debt and equity financings, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. There can be no assurance that such financings will be obtained on terms acceptable to us, if at all.

Additionally, while the long-term economic impact of geopolitical tensions is difficult to assess or predict, such events have caused significant disruptions to the global financial markets and contributed to a general global economic slowdown. Furthermore, inflation rates have increased recently to levels not seen in decades, which may also be impacted by the implementation of tariffs by the United States and other countries. Moreover, there is great uncertainty with respect to potential changes in trade regulations, tariffs, sanctions and export controls which also increase volatility in the global economy. In addition, the U.S. Federal Reserve has raised interest rates in the past in response to concerns about inflation. Relatively high interest rates and fluctuations in inflation, especially if coupled with a significant change in government spending and volatility in financial markets, may further increase economic uncertainty and heighten these risks. If the disruptions and slowdown deepen or persist, we may not be able to access additional capital on favorable terms, or at all, which could in the future negatively affect our ability to pursue our business strategy.

If we raise additional funds through collaborations, strategic alliances or licensing agreements with third parties for one or more of our current or future drug candidates, we may be required to relinquish valuable rights to our technologies, future revenue streams, research programs or drug candidates, or to grant licenses on terms that may not be favorable to us. Our failure to raise capital as and when needed would have a material adverse effect on our financial condition and our ability to pursue our business strategy. We may be required to take additional actions beyond the cost preservation measures initiated to address our liquidity needs, including exploring other strategic options, continuing to further reduce operating expense or delaying, reducing the scope of, discontinuing or altering our research and development activities.

See "*Item 1A. Risk Factors*" for additional risks associated with our capital requirements.

Material Cash Requirements

As of December 31, 2025, we had no material non-cancelable purchase commitments with service providers, as we have generally contracted on a cancelable, purchase order basis. We cannot estimate whether we will receive or the timing of any potential contingent payments upon the achievement by us of clinical, regulatory and commercial events, as applicable, or royalty payments that we may be required to make under license agreements we have entered into with various entities pursuant to which we have in-licensed certain intellectual property as contractual obligations or commitments, including agreements with AstraZeneca, Gensaic and Northwestern. Pursuant to these license agreements, we have agreed to make milestone payments up to an aggregate of $660.3 million upon the achievement of certain development, regulatory and sales milestones. We excluded these contingent payments from the consolidated financial statements given that the timing, probability and amount, if any, of such payments cannot be reasonably estimated at this time.

In September 2021, we entered into a ten-year lease agreement for our corporate headquarters with a term commencing in March 2022 for approximately 19,000 square feet of office space at Hudson Commons in New York, New York. The lease provides for monthly rental payments over the lease term. The base rent under the lease is currently $2.3 million per year. Rent payments commenced in January 2023, and will continue for ten years following the rent commencement date. We issued a letter of credit in the amount of $1.9 million in association with the execution of the lease agreement, which is reflected as restricted cash on the consolidated balance sheets. Payment obligations under the lease agreement include approximately $2.3 million in the 12 months subsequent to December 31, 2025 and approximately $17.0 million over the remainder of the agreement. For additional information see Note 5 to our consolidated financial statements under the heading ‘Leases.’

Cash Flows

The following table summarizes our cash flows for the periods indicated:

(in thousands)	Year Ended December 31, 2025	Year Ended December 31, 2024
Net cash (used in) provided by:		
Operating activities	$ (38,334)	$ (55,956)
Investing activities	(49,854)	54,594
Financing activities	75,207	622
Effect of exchange rates on cash, cash equivalents and restricted cash	$ (167)	$ —
Net decrease in cash, cash equivalents, and restricted cash	$ (13,148)	$ (740)

Net Cash Used in Operating Activities

Net cash used in operating activities was $38.3 million for the year ended December 31, 2025, which consisted of net loss of $17.4 million offset by various noncash charges, most significantly an adjustment to fair value of one of our long-term equity investments of $21.0 million which was reflected in other income (expense), net. Net cash used in operating activities was $56.0 million for the year ended December 31, 2024, which consisted of net loss of $26.4 million offset by $29.5 million, net, of various noncash charges, most significantly a $30.0 million change in fair value of the royalty monetization liability with pursuant to the Ligand Agreement.

Net Cash (Used in) Provided by Investing Activities

Net cash used in investing activities was $49.9 million for the year ended December 31, 2025, which was primarily related to our purchases of investments in U.S. treasuries. For the year ended December 31, 2024, $54.6 million was provided by investing activities, primarily comprised of net sales/maturities of investments in U.S. treasuries.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $75.2 million for the year ended December 31, 2025, which was comprised of net proceeds from the 2025 Private Placement as well as proceeds from the exercise of options and purchases made under our employee stock purchase plan. For the same period in 2024, cash provided by financing activities was

$0.6 million, comprised of proceeds from the exercise of options and purchases made under the employee stock purchase plan.

Critical Accounting Policies and Estimates

Our management's discussion and analysis of financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States. The preparation of these consolidated financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, as well as the revenue and expenses incurred during the reported periods. On an ongoing basis, we evaluate our estimates and judgments. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not apparent from other sources. Changes in estimates are reflected in reported results for the period in which they become known.

Actual results may differ from these estimates under different assumptions or conditions. In making estimates and judgments, management employs critical accounting policies. Our critical accounting policies are described in greater detail in Note 2 to our audited consolidated financial statements included in this Annual Report on Form 10-K.

We have listed below our critical accounting estimates that we believe to have the greatest potential impact on our consolidated financial statements. Historically, our assumptions, judgments and estimates relative to our critical accounting estimates have not differed materially from actual results.

Revenue Recognition

We recognize revenue under sublicense agreements in accordance with ASC 606, Revenue Recognition. The terms of the agreements within this scope may contain multiple performance obligations, including but not limited to licenses and research and development activities. ASC 606 requires that we evaluate these agreements to determine the distinct performance obligations. Nonrefundable, upfront fees that are not contingent on any future performance and require no consequential continuing involvement by us, are recognized as revenue when the license term commences and the licensed data, technology or product is delivered. We defer recognition of nonrefundable upfront fees if the performance obligations are not satisfied.

During the year ended December 31, 2025, we recognized revenue of approximately $7.3 million related to royalty agreements.

Research and Development Accrual

When preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and communicating with our personnel, consultants and vendors to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of the actual cost. Payments under certain contracts we have with third parties depend on factors, such as the successful enrollment of certain numbers of patients, site initiation and the completion of clinical trial milestones.

When accruing research and development expenses, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If possible, we obtain information regarding unbilled services directly from our service providers. However, we may be required to estimate the cost of these services based only on information available to us. If we underestimate or overestimate the cost associated with a trial or service at a given point in time, adjustments to research and development expenses may be necessary in future periods. Historically, our estimated accrued research and development expenses have approximated actual expense incurred.

Smaller Reporting Company Status

We are a smaller reporting company as defined in the Exchange Act. We may take advantage of certain of the scaled disclosures available to smaller reporting companies and will be able to take advantage of these scaled disclosures for so long as (i) the value of our voting and non-voting common stock held by non-affiliates is less than $250.0 million measured on the last business day of our most recently completed second fiscal quarter or (ii) our annual revenue is less than $100.0 million during the most recently completed fiscal year and the value of our voting and non-voting common

stock held by non-affiliates is less than $700.0 million measured on the last business day of our most recently completed second fiscal quarter.

As a smaller reporting company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk

The primary objectives of our investment activities are to ensure liquidity and preserve capital. As of December 31, 2025, we had cash, cash equivalents and marketable securities of $90.4 million. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the short-term maturities of our cash equivalents and marketable securities and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our cash equivalents and marketable securities. To minimize the risk now and in the future, we intend to continue to maintain our portfolio of cash equivalents and marketable securities in institutional market funds that are comprised of U.S. Treasury and U.S. Treasury-backed repurchase agreements as well as treasury notes and high quality short-term corporate bonds.

Item 8. Financial Statements and Supplementary Data

Our financial statements, together with the report of our independent registered public accounting firm, appear in this Annual Report on Form 10-K beginning on page F-1.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure

None.

Item 9A. Controls and Procedures

Management's Evaluation of our Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Securities Exchange Act of 1934, as amended is (1) recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms and (2) accumulated and communicated to our management, including our principal executive officer and principal financial officer, to allow timely decisions regarding required disclosure.

As of December 31, 2025, our management, with the participation of our principal executive officer and principal financial and accounting officer, evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act). Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our principal executive officer and principal financial officer have concluded based upon the evaluation described above, that as of December 31, 2025, our disclosure controls and procedures were effective at the reasonable assurance level.

Management's Annual Report on Internal Control over Financial Reporting

Our 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. Our management, under the supervision and with the participation of our principal executive officer and principal financial and accounting officer, conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2025 based on the framework in Internal Control-Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 Framework). Based on the results of its evaluation, management concluded that our internal control over financial reporting was effective as of December 31, 2025.

Attestation Report of the Registered Public Accounting Firm

This Annual Report on Form 10-K does not include an attestation report on our internal control over financial reporting from our registered public accounting firm due to an exemption as a smaller reporting company and as a non-accelerated filer for the year ended December 31, 2025.

Changes in Internal Control over Financial Reporting

There have been no changes in internal controls over financial reporting during our most recent quarter ended December 31, 2025, that materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information

During the three months ended December 31, 2025, no director or officer of the Company adopted or terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement," as each term is defined in Item 408(a) of Regulation S-K.

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections

Not Applicable.

PART III

Certain information required by Part III is omitted from this Annual Report because we will file with the SEC a definitive proxy statement pursuant to Regulation 14A, or the 2026 Proxy Statement, no later than 120 days after the end of our fiscal year, and certain information included therein is incorporated herein by reference.

Item 10. Directors, Executive Officers, and Corporate Governance

The information required by this item is incorporated by reference to the information set forth in the sections titled "Proposal 1 – Election of Directors," "Executive Officers," and "Information Regarding the Board and Corporate Governance" and "Delinquent Section 16(a) Reports," if any, in our 2026 Proxy Statement.

Information regarding our Code of Business Conduct and Ethics, or the Code of Conduct, required by this item will be contained in our 2026 Proxy Statement under the caption "Information Regarding the Board and Corporate Governance – Code of Business Conduct and Ethics," and is hereby incorporated by reference. We intend to promptly disclose on our website or in a Current Report on Form 8-K in the future (i) the date and nature of any amendment (other than technical, administrative or other non-substantive amendments) to the Code of Conduct that applies to our principal executive officer, principal financial officer, principal accounting officer or controller or persons performing similar functions and relates to any element of the code of ethics definition enumerated in Item 406(b) of Regulation S-K and (ii) the nature of any waiver, including an implicit waiver, from a provision of the Code of Conduct that is granted to one of these specified individuals that relates to one or more of the elements of the code of ethics definition enumerated in Item 406(b) of Regulation S-K, the name of such person who is granted the waiver and the date of the waiver. The full text of our Code of Conduct is available at the Investors section of our website at *www.ovidrx.com*. The reference to our website address does not constitute incorporation by reference of the information contained at or available through our website, and you should not consider it to be a part of this Annual Report.

Item 11. Executive Compensation

The information required by this item is incorporated by reference to the information set forth in the section titled "Executive Officer and Director Compensation" in our 2026 Proxy Statement.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

The information required by this item is incorporated by reference to the information set forth in the section titled "Security Ownership of Certain Beneficial Owners and Management" and "Equity Compensation Plan Information" in our 2026 Proxy Statement.

Item 13. Certain Relationships and Related Transactions and Director Independence

The information required by this item is incorporated by reference to the information set forth in the section titled "Transactions with Related Persons" and "Information Regarding the Board and Corporate Governance – Board Independence" in our 2026 Proxy Statement.

Item 14. Principal Accountant Fees and Services

The information required by this item is incorporated by reference to the information set forth in Proposal 3 under the section titled "Independent Registered Public Accounting Firm Fees" and "Pre-Approval Policies and Procedures" contained in our 2026 Proxy Statement.

PART IV

Item 15. Exhibits and Financial Statements and Schedules

(a)(1) Financial Statements.

(a)(2) Financial Statement Schedules.

All schedules have been omitted because they are not required or because the required information is given in the Financial Statements or Notes thereto.

(a)(3) Exhibits.

The exhibits listed below are filed as part of this Annual Report on Form 10-K.

Number	Description
3.1	Amended and Restated Certificate of Incorporation (incorporated herein by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 001-38085), filed with the Commission on May 10, 2017).
3.2	Certificate of Amendment to the Amended and Restated Certificate of Incorporation (incorporated by reference to Exhibit 4.3 to the Company's Registration Statement on Form S-3 (File No. 333-292151), filed with the Commission on December 15, 2025).
3.3	Corrected Amended and Restated Certificate of Designation of Series A Convertible Preferred Stock (incorporated herein by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 001-38085), filed with the Commission on September 24, 2019).
3.4	Certificate of Designation of Preferences, Rights and Limitations of Series B Convertible Preferred Stock (incorporated herein by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 001-38085), filed with the Commission on October 3, 2025).
3.5	Amended and Restated Bylaws (incorporated herein by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K (File No. 001-38085), filed with the Commission on May 10, 2017).
4.1	Form of Common Stock Certificate of the Company (incorporated herein by reference to Exhibit 4.1 to the Company's Registration Statement on Form S-1/A (File No. 333-217245), filed with the Commission on April 25, 2017).
4.2**	Description of the Securities of Ovid Therapeutics Inc.
4.3	Form of Series A Preferred Stock Certificate (incorporated herein by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K (File No. 001-38085), filed with the Commission on February 21, 2019).
4.4	Form of Series A Warrant (incorporated herein by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K (File No. 001-38085), filed with the Commission on October 3, 2025).
4.5	Form of Series B Warrant (incorporated herein by reference to Exhibit 4.2 to the Company's Current Report on Form 8-K (File No. 001-38085), filed with the Commission on October 3, 2025).
4.6**	Form of Pre-Funded Warrant.
10.1+	Form of Indemnity Agreement by and between the Company and its directors and officers (incorporated herein by reference to Exhibit 10.1 to the Company's Registration Statement on Form S-1 (File No. 333-217245), filed with the Commission on April 10, 2017).
10.2+	2017 Equity Incentive Plan (incorporated herein by reference to Exhibit 4.12 to the Company's Registration Statement on Form S-8 (File No. 001-38085), filed with the Commission on May 22, 2017).

10.3+	Forms of Option Grant Notice and Option Agreement under 2017 Equity Incentive Plan (incorporated herein by reference to Exhibit 10.3 to the Company's Registration Statement on Form S-1 (File No. 333-217245), filed with the Commission on April 10, 2017).
10.4+	Form of Restricted Stock Unit Grant Notice and Award Agreement under the 2017 Equity Incentive Plan (incorporated herein by reference to Exhibit 10.4 to the Company's Annual Report on Form 10-K (File No. 001-38085), filed with the Commission on March 11, 2025).
10.5+	2014 Equity Incentive Plan, as amended (incorporated herein by reference to Exhibit 10.5 to the Company's Registration Statement on Form S-1 (File No. 333-217245), filed with the Commission on April 10, 2017).
10.6+	Amendment to 2014 Equity Incentive Plan, effective as of March 9, 2015 (incorporated herein by reference to Exhibit 10.6 to the Company's Registration Statement on Form S-1 (File No. 333-217245), filed with the Commission on April 10, 2017).
10.7+	Amendment to 2014 Equity Incentive Plan, effective as of June 4, 2015 (incorporated herein by reference to Exhibit 10.7 to the Company's Registration Statement on Form S-1 (File No. 333-217245), filed with the Commission on April 10, 2017).
10.8+	Amendment to 2014 Equity Incentive Plan, effective as of July 28, 2015 (incorporated herein by reference to Exhibit 10.8 to the Company's Registration Statement on Form S-1 (File No. 333-217245), filed with the Commission on April 10, 2017).
10.9+	Amendment to 2014 Equity Incentive Plan, effective as of February 11, 2016 (incorporated herein by reference to Exhibit 10.9 to the Company's Registration Statement on Form S-1 (File No. 333-217245), filed with the Commission on April 10, 2017).
10.10+	2017 Employee Stock Purchase Plan (incorporated herein by reference to Exhibit 4.14 to the Company's Registration Statement on Form S-8 (File No. 001-38085), filed with the Commission on May 22, 2017).
10.11+	Amended Non-Employee Director Compensation Policy, effective February 22, 2024 (incorporated herein by reference to Exhibit 10.11 to the Company's Annual Report on Form 10-K (File No. 001-38085), filed with the Commission on March 11, 2025).
10.12+	Amended Non-Employee Director Compensation Policy, effective February 20, 2025 (incorporated herein by reference to Exhibit 10.12 to the Company's Annual Report on Form 10-K (File No. 001-38085), filed with the Commission on March 11, 2025).
10.13+**^^	Amended and Restated Executive Employment Agreement between the Company and Margaret Alexander, dated November 11, 2025.
10.14+**^^	Executive Employment Agreement between the Company and Jeremy M. Levin, dated November 11, 2025.
10.15+	Executive Employment Agreement between the Company and Jeff Rona, effective June 2, 2021 (incorporated herein by reference to Exhibit 10.17 to the Company's Annual Report on Form 10-K (File No. 001-38085), filed with the Commission on March 15, 2021).
10.16^	License Agreement by and between Northwestern University and the Company, dated December 15, 2016. (incorporated herein by reference to Exhibit 4.3 to the Company's Quarterly Report on Form 10-Q (File No. 001-38085), filed with the Commission on May 14, 2024).
10.17†	License Agreement, dated as of December 30, 2021, by and between the Ovid Therapeutics Inc. and AstraZeneca AB (incorporated herein by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 001-38085), filed with the Commission on January 3, 2022).
10.18^	Purchase and Sale Agreement, dated as of October 17, 2023, by and between the Company and Ligand Pharmaceuticals Incorporated. (incorporated herein by reference to Exhibit 10.21 to the Company's Annual Report on Form 10-K (File No. 001-38085), filed with the Commission on March 8, 2024.

10.19	Form of Registration Rights Agreement (incorporated herein by reference to Exhibit 10.2 to the Company's Current Report on Form 8-K (File No. 001-38085), filed with the Commission on October 3, 2025).
19.1	Registrant's Insider Trading Policy (incorporated herein by reference to Exhibit 19.1 to the Company's Annual Report on Form 10-K (File No. 001-38085), filed with the Commission on March 11, 2025).
23.1	Consent of Independent Registered Public Accounting Firm.
24.1	Power of Attorney (included on the signature page to this report).
31.1	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1*	Certification of Principal Executive Officer and Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
97	Incentive Compensation Recoupment Policy (incorporated herein by reference to Exhibit 97 to the Company's Annual Report on Form 10-K (File No. 001-38085), filed with the Commission on March 8, 2024).
101.INS*	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File as its XBRL tags are embedded within the XBRL document.
101.SCH*	Inline XBRL Taxonomy Extension Schema with Embedded Linkbase Documents.
104*	Cover Page formatted as Inline XBRL and contained within Exhibit 101.

* Furnished herewith and not deemed to be "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and shall not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act (whether made before or after the date of the Form 10-K), irrespective of any general incorporation language contained in such filing.

** Filed herewith.

+ Indicates a management contract or compensatory plan.

† Confidential treatment has been granted for certain portions of this exhibit. These portions have been omitted and filed separately with the SEC.

^ Pursuant to Item 601(b)(10)(iv) of Regulation S-K promulgated by the Securities and Exchange Commission, certain portions of this exhibit have been redacted. The Registrant hereby agrees to furnish supplementally to the Securities and Exchange Commission, upon its request, an unredacted copy of this exhibit.

^^ Certain of the exhibits and schedules to this exhibit have been omitted in accordance with Regulation S-K Item 601(a)(5). The Registrant agrees to furnish a copy of all omitted exhibits and schedules to the Securities and Exchange Commission upon its request.

Item 16. Form 10-K Summary

Not applicable.

SIGNATURES

Pursuant to the requirements 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.

OVID THERAPEUTICS INC.

Date: March 18, 2026

By: /s/ Margaret Alexander

Margaret Alexander

President, Chief Executive Officer and Director
(Principal Executive Officer)

Date: March 18, 2026

By: /s/ Jeffrey Rona

Jeffrey Rona
Chief Business and Financial Officer
(Principal Financial and Accounting Officer)

POWER OF ATTORNEY

Each person whose individual signature appears below hereby authorizes and appoints Margaret Alexander and Jeffrey Rona, and each of them, with full power of substitution and resubstitution and full power to act without the other, as his or her true and lawful attorney-in-fact and agent to act in his or her name, place and stead and to execute in the name and on behalf of each person, individually and in each capacity stated below, and to file any and all amendments to this report on Form 10-K, and to file the same, with all exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing, 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 thereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report 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/ Margaret Alexander Margaret Alexander	President, Chief Executive Officer and Director *(Principal Executive Officer)*	March 18, 2026
/s/ Jeffrey Rona Jeffrey Rona	Chief Business and Financial Officer *(Principal Financial and Accounting Officer)*	March 18, 2026
/s/ Jeremy M. Levin, DPhil, MB BChir Jeremy M. Levin, DPhil, MB BChir	Executive Chairman	March 18, 2026
/s/ Karen Bernstein, PhD Karen Bernstein, PhD	Director	March 18, 2026
/s/ Barbara Duncan Barbara Duncan	Director	March 18, 2026
/s/ Kevin Fitzgerald, PhD Kevin Fitzgerald, PhD	Director	March 18, 2026
/s/ Bart Friedman Bart Friedman	Director	March 18, 2026
/s/ Stelios Papadopoulos Stelios Papadopoulos	Director	March 18, 2026

OVID THERAPEUTICS INC.

Index to Consolidated Financial Statements

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors
Ovid Therapeutics Inc.:

Opinion on the Consolidated Financial Statements

We have audited the accompanying consolidated balance sheets of Ovid Therapeutics Inc. and subsidiaries (the Company) as of December 31, 2025 and 2024, the related consolidated statements of operations, comprehensive loss, changes in stockholders' equity, and cash flows for the years then ended, 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 December 31, 2025 and 2024, and the results of its operations and its cash flows for the years then ended, in conformity with U.S. generally accepted accounting principles.

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 Public Company Accounting Oversight Board (United States) (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. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits, we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

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 Matter

The critical audit matter communicated below is a matter arising from the current period audit of the consolidated financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates 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 a critical audit matter 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 matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Valuation of warrants

As discussed in Note 2 to the consolidated financial statements, the Company has issued freestanding warrants to purchase shares of its common stock in connection with financing activities and accounts for them as either liabilities or as equity instruments depending on the specific terms of the warrant agreements. As discussed in Note 7, the Company issued Series A common warrants and Series B common warrants in October 2025. The Series A common warrants were valued using a probability-weighted expected return method based on two different expiry periods valued using a Black-Scholes model with corresponding probability for the likelihood of each scenario and Level 3 inputs. The Series B common warrants were valued using a Monte Carlo simulation model since the timing and payoff are dependent on the Company's trailing stock price and level 3 inputs. The relative fair value allocated to Series A common warrants and Series B common warrants were $13.8 million and $17.7 million, respectively.

We identified the evaluation of the fair value of the Series A common warrants and the Series B common warrants as a critical audit matter. Subjective auditor judgment was required to evaluate the estimated fair values due to the degree of subjectivity associated with the expected volatility assumptions and the sensitivity of these assumptions to variation.

Additionally, the evaluation of the fair value of the Series A common warrants and the Series B common warrants required specialized skills and knowledge.

The following are the primary procedures we performed to address this critical audit matter. We involved valuation professionals with specialized skills and knowledge who assisted in evaluating the fair value of the Series A common warrants and Series B common warrants by:

- developing independent expectations of the expected volatility assumptions for both the Series A common warrants and Series B common warrants based on consideration of historical and implied share price volatility information for the Company and publicly available market information for guideline public companies
- developing an independent estimate of the fair value of the Series A common warrants using the independently developed expected volatility assumption for the Series A common warrants and comparing to the fair value used by management for the Series A common warrants
- developing an independent estimate of the fair value of the Series B common warrants using independently developed assumptions, including the independently developed expected volatility assumption for the Series B common warrants, and comparing to the fair value used by management for the Series B common warrants

/s/ KPMG LLP

We have served as the Company's auditor since 2015.

New York, New York
March 18, 2026

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

OVID THERAPEUTICS INC.
Consolidated Balance Sheets

(in thousands, except share and per share data)	December 31, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 13,153	$ 26,301
Marketable securities	56,482	26,774
Prepaid expenses and other current assets	4,733	2,865
Total current assets	74,368	55,940
Marketable securities - noncurrent	20,812	—
Long-term equity investments	41,961	20,974
Restricted cash	1,931	1,931
Right-of-use asset, net	11,610	12,797
Property and equipment, net	252	433
Other assets	—	92
Total assets	$ 150,934	$ 92,167
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,956	$ 3,192
Accrued expenses	4,899	5,994
Current portion, lease liability	1,433	1,336
Total current liabilities	8,288	10,522
Long-term liabilities:		
Lease liability	11,986	13,419
Total liabilities	20,274	23,941
Stockholders' equity:		
Preferred stock, $0.001 par value; 10,000,000 shares authorized; Series A convertible preferred stock, 10,000 shares designated, 0 and 1,250 shares issued and outstanding at December 31, 2025 and 2024, respectively	—	—
Common stock, $0.001 par value; 315,000,000 and 125,000,000 shares authorized; 130,184,353 and 71,009,866 shares issued and outstanding at December 31, 2025 and 2024, respectively	130	71
Additional paid-in-capital	452,445	372,489
Accumulated other comprehensive loss	(202)	(35)
Accumulated deficit	(321,713)	(304,299)
Total stockholders' equity	130,660	68,226
Total liabilities and stockholders' equity	$ 150,934	$ 92,167

See accompanying notes to these consolidated financial statements

OVID THERAPEUTICS INC.
Consolidated Statements of Operations

(in thousands, except share and per share data)	For the Year Ended December 31, 2025	For the Year Ended December 31, 2024
Revenue:		
License and other revenue	$ 7,252	$ 566
Total revenue	7,252	566
Operating expenses:		
Research and development	25,582	36,767
General and administrative	24,109	25,684
Total operating expenses	49,691	62,451
Loss from operations	(42,439)	(61,885)
Other income (expense), net	25,026	35,452
Loss before provision for income taxes	(17,414)	(26,433)
Provision for income taxes	—	—
Net loss	$ (17,414)	$ (26,433)
Net loss per share of Series A preferred stock, basic and diluted	$ (232.47)	$ (366.33)
Weighted-average Series A preferred stock shares outstanding, basic and diluted	1,171	1,250
Net loss per share of common stock, basic and diluted	$ (0.23)	$ (0.37)
Weighted-average common stock shares outstanding, basic and diluted	73,735,606	70,905,422

See accompanying notes to these consolidated financial statements

OVID THERAPEUTICS INC.
Consolidated Statements of Comprehensive Loss

(in thousands)	For the Year Ended December 31, 2025	For the Year Ended December 31, 2024
Net loss	$ (17,414)	$ (26,433)
Other comprehensive (loss) income:		
Cumulative translation adjustment	(140)	(42)
Unrealized (loss) gain on available-for-sale securities	(27)	7
Comprehensive loss	$ (17,581)	$ (26,468)

See accompanying notes to these consolidated financial statements

OVID THERAPEUTICS INC.
Consolidated Statement of Changes in Stockholders' Equity

(in thousands, except shares)	Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total
	Shares	Amount	Shares	Amount				
Balance, December 31, 2024	1,250	$ —	71,009,866	$ 71	$ 372,489	$ (35)	$ (304,299)	$ 68,226
Conversion of Series A convertible preferred stock to common stock	(1,250)	—	1,250,000	1	(1)	—	—	—
Conversion of Series B convertible preferred stock to common stock	—	—	57,722,000	58	49,251	—	—	49,309
Issuance of Series A warrants	—	—	—	—	13,802	—	—	13,802
Issuance of Series B warrants	—	—	—	—	17,700	—	—	17,700
Expenses related to the sale of Series B preferred stock and Series A and Series B warrants	—	—	—	—	(5,694)	—	—	(5,694)
Issuance of common stock from exercise of stock options and purchases from employee stock purchase plan	—	—	202,487	—	91	—	—	91
Stock-based compensation expense	—	—	—	—	4,807	—	—	4,807
Other comprehensive loss	—	—	—	—	—	(167)	—	(167)
Net loss	—	—	—	—	—	—	(17,414)	(17,414)
Balance, December 31, 2025	—	$ —	130,184,353	$ 130	$ 452,445	$ (202)	$ (321,713)	$ 130,660

(in thousands, except shares)	Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total
	Shares	Amount	Shares	Amount				
Balance, December 31, 2023	1,250	$ —	70,691,992	$ 71	$ 365,591	$ 1	$ (277,866)	$ 87,797
Issuance of common stock from exercise of stock options and purchases from employee stock purchase plan	—	—	317,874	—	622	—	—	622
Stock-based compensation expense	—	—	—	—	6,276	—	—	6,276
Other comprehensive loss	—	—	—	—	—	(36)	—	(36)
Net loss	—	—	—	—	—	—	(26,433)	(26,433)
Balance, December 31, 2024	1,250	$ —	71,009,866	$ 71	$ 372,489	$ (35)	$ (304,299)	$ 68,226

See accompanying notes to these consolidated financial statements

OVID THERAPEUTICS INC.
Consolidated Statements of Cash Flows

(in thousands)	For the Year Ended December 31, 2025	For the Year Ended December 31, 2024
Cash flows from operating activities:		
Net loss	$ (17,414)	$ (26,433)
Adjustments to reconcile net loss to cash used in operating activities:		
Change in fair value of royalty monetization liability	—	(30,000)
Unrealized gain on equity investments	(21,052)	(3,349)
Change in accrued interest and accretion of discount on marketable securities	(695)	(2,765)
Stock-based compensation expense	4,807	6,276
Depreciation and amortization expense	273	613
Noncash operating lease expense	1,187	1,097
Change in lease liability	(1,336)	(1,246)
Change in operating assets and liabilities:		
Prepaid expenses and other current assets	(1,802)	899
Accounts payable	(1,234)	(512)
Accrued expenses	(1,068)	(537)
Net cash used in operating activities	(38,334)	(55,956)
Cash flows from investing activities:		
Purchase of marketable securities	(91,854)	(73,246)
Sales/maturities of marketable securities	42,000	128,000
Purchases of property and equipment	—	(71)
Software development and other costs	—	(90)
Net cash (used in) provided by investing activities	(49,854)	54,594
Cash flows from financing activities:		
Proceeds from private placement financing, net of transaction costs	75,116	—
Proceeds from exercise of options and employee stock purchase plan	91	622
Net cash provided by financing activities	75,207	622
Effect of exchange rates on cash, cash equivalents and restricted cash	(167)	—
Net decrease in cash, cash equivalents and restricted cash	(13,148)	(740)
Cash, cash equivalents and restricted cash, at beginning of period	28,232	28,972
Cash, cash equivalents and restricted cash, at end of period	$ 15,084	$ 28,232

See accompanying notes to these consolidated financial statements

NOTE 1 – NATURE OF OPERATIONS

Ovid Therapeutics Inc. (the "Company") was incorporated under the laws of the state of Delaware, commenced operations on April 1, 2014, and maintains its principal executive office in New York, New York. The Company is a biopharmaceutical company that is dedicated to developing small molecule medicines for brain disorders with significant unmet need. The Company is currently focused on developing OV329, OV4071, and other undisclosed potential medicines. See Part I, Item 1 of the annual report on Form 10-K for the period ended December 31, 2025 for detailed program descriptions.

Since its inception, the Company has devoted substantially all of its efforts to business development, research and development, recruiting management and technical staff, and raising capital, and has financed its operations through the issuance of convertible preferred stock, common stock and other equity instruments, the sale and/or licensing of certain assets and the licensing of certain intellectual property. The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development and regulatory success, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations, and the ability to secure additional capital to fund operations.

The Company's major sources of cash have been licensing revenue, proceeds from various public and private offerings of its capital stock, option exercises and interest income. As of December 31, 2025, the Company had approximately $90.4 million in cash, cash equivalents and marketable securities. Since inception, the Company has generated $230.7 million in revenue, primarily from the Company's royalty, license and termination agreement ("RLT Agreement") with Takeda Pharmaceutical Company Limited ("Takeda"). Historically, the Company has incurred recurring losses, has experienced recurring negative operating cash flows and has required significant cash resources to execute its business plans, which the Company expects will continue for the foreseeable future. The Company has an accumulated deficit of $321.7 million as of December 31, 2025, working capital of $66.1 million and used $38.3 million of cash in operating activities for the year ended December 31, 2025.

The Company recorded a net loss of $17.4 million during the year ended December 31, 2025 and expects to incur losses in subsequent periods for at least the next several years. The Company is highly dependent on its ability to find additional sources of funding through either equity offerings, debt financings, collaborations, strategic alliances, licensing agreements or a combination of any such transactions. Management believes that the Company's existing cash, cash equivalents and marketable securities as of December 31, 2025 will be sufficient to fund its current operations through at least 12 months from the date of the issuance of these consolidated financial statements. Adequate additional funding may not be available to the Company on acceptable terms or at all. The failure to raise capital as and when needed will have a negative impact on the Company's financial condition and ability to pursue its business strategy. If the Company is unable to raise capital on acceptable terms, the Company will be required to delay, reduce the scope of or eliminate research and development programs, or obtain funds through arrangements with collaborators or others that may require the Company to relinquish rights to certain drug candidates that the Company might otherwise seek to develop or commercialize independently.

The Company is subject to other challenges and risks specific to its business and its ability to execute on its strategy, as well as risks and uncertainties common to companies in the pharmaceutical industry with development and commercial operations, including, without limitation, risks and uncertainties associated with: delays or problems in the supply of the Company's product candidates, loss of single source suppliers or failure to comply with manufacturing regulations; identifying, acquiring or in-licensing additional products or product candidates; pharmaceutical product development and the inherent uncertainty of clinical success; the challenges of protecting and enhancing intellectual property rights; complying with applicable regulatory requirements; and obtaining regulatory approval of any of the Company's product candidates, among others.

On February 10, 2025, the Company received a notification letter from the Listing Qualifications Department of the Nasdaq Stock Market LLC notifying the Company that the average closing bid price of the Company's shares of common stock was below the closing bid price of $1.00 per share during the last 31 consecutive trading days. The Company had an initial period of 180 calendar days, or until August 11, 2025, to regain compliance with the minimum bid price requirement. On August 12, 2025, the Company received approval from the Listing Qualifications Department of Nasdaq to transfer the listing of the Company's common stock from the Nasdaq Global Select Market to the Nasdaq Capital Market (the "Approval"). In connection with the Approval, the Company was granted an additional 180-day grace

period, or until February 9, 2026, to regain compliance with the minimum bid price requirement. On September 11, 2025, the Company received formal notification from Nasdaq that it had regained compliance with the minimum bid price requirement.

NOTE 2 – SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

(A) Basis of Presentation and Consolidation

The accompanying consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP") and include the accounts of Ovid Therapeutics Inc. and its wholly owned subsidiaries, Ovid Therapeutics Australia Pty Ltd and Ovid Therapeutics Hong Kong Limited. All material intercompany transactions and balances have been eliminated in consolidation.

(B) Use of Estimates

The preparation of financial statements in conformity with 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 expenses during the reporting period. Actual results could differ materially from those estimates.

(C) Marketable Securities

Marketable securities consist of investments in U.S. treasury instruments which are considered available-for-sale securities. The Company classifies its marketable securities with maturities of less than one year from the balance sheet date as current assets on its consolidated balance sheets. The Company classifies its marketable securities with original maturities of less than three months as cash equivalents on its consolidated balance sheets. The Company classifies its marketable securities with maturities of greater than twelve months as noncurrent assets on it consolidated balance sheets. Unrealized gains and losses on these securities that are determined to be temporary are reported as a separate component of accumulated other comprehensive income (loss) in stockholders' equity.

(D) Restricted Cash

The Company classifies as restricted cash all cash pledged as collateral to secure long-term obligations and all cash for which use is otherwise limited by contractual provisions. Amounts are reported as non-current unless restrictions are expected to be released in the next 12 months.

(E) Long-term Equity Investments

Long-term equity investments consist of equity investments in the preferred shares of Gensaic, Inc., formerly M13 Therapeutics, Inc. ("Gensaic"), and Graviton Bioscience Corporation ("Graviton"), both privately held corporations. The preferred shares are not considered in-substance common stock, and the investments are accounted for at cost, with adjustments for observable changes in prices or impairments, and are classified within long-term equity investments on the consolidated balance sheets with adjustments recognized in other income (expense), net on the consolidated statements of operations. The Company has determined that these equity investments do not have a readily determinable fair value and elected the measurement alternative. Therefore, the carrying amount of the equity investments will be adjusted to fair value at the time of the next observable price change for the identical or similar investment of the same issuer, or when an impairment is recognized. Each reporting period, the Company performs a qualitative assessment to evaluate whether the investments are impaired. The assessment includes a review of recent operating results and trends, recent sales/acquisitions of the investees' securities, and other publicly available data. If an investment is determined to be impaired, the Company will then write it down to its estimated fair value. As of December 31, 2025 and 2024, the equity investment in Gensaic had a carrying value of $5.1 million. As of December 31, 2025 and 2024, the equity investment in Graviton had a carrying value of $36.8 million and $15.8 million, respectively, which reflect unrealized gains of $21.0 million and $4.6 million recorded in other income (expense), net, in the consolidated statements of operations for the periods ended December 31, 2025 and 2024, respectively, due to observable changes in price. The cumulative unrealized gain on the equity investment in Graviton is $26.9 million.

Long-term equity investments also consist of an equity investment in the common shares of Marinus Pharmaceuticals, Inc. ("Marinus") that was received as noncash consideration via the terms of a licensing agreement executed between the two companies effective March 2022. The equity shares are marked-to-market at each reporting date with changes in the fair value being reflected in the carrying value of the investment on the Company's consolidated balance sheets and other income (expense), net on the Company's consolidated statements of operations. As of

December 31, 2025 and 2024, the equity investment in Marinus had a carrying value of zero and $0.1 million, respectively. In January 2025, Immedica Pharma, S.A. purchased Marinus in an all-cash tender offer, resulting in the Company's sale of its position in Marinus for $0.07 million.

No impairments were recognized in the years ending December 31, 2025 and 2024.

(F) Fair Value of Financial Instruments

Financial Accounting Standards Board guidance specifies a hierarchy of valuation techniques based on whether the inputs to those valuation techniques are observable or unobservable. Observable inputs reflect market data obtained from independent sources, while unobservable inputs reflect market assumptions. The hierarchy gives the highest priority to unadjusted quoted prices in active markets for identical assets or liabilities (Level 1 measurement) and the lowest priority to unobservable inputs (Level 3 measurement).

The three levels of the fair value hierarchy are as follows:

- Level 1—Unadjusted quoted prices in active markets for identical assets or liabilities that the reporting entity has the ability to access at the measurement date. Level 1 primarily consists of financial instruments whose value is based on quoted market prices such as exchange-traded instruments and listed equities. The Company's Level 1 assets consisted of investments in a U.S. treasury money market fund and equity securities totaling approximately $7.6 million and $25.8 million, respectively, as of December 31, 2025 and 2024.
- Level 2—Inputs other than quoted prices included within Level 1 that are observable for the asset or liability, either directly or indirectly (e.g., quoted prices of similar assets or liabilities in active markets, or quoted prices for identical or similar assets or liabilities in markets that are not active). Level 2 includes financial instruments that are valued using models or other valuation methodologies. The Company's Level 2 assets consisted of U.S. treasury bills totaling approximately $82.3 million and $26.8 million, respectively, as of December 31, 2025 and 2024.
- Level 3—Unobservable inputs for the asset or liability. Financial instruments are considered Level 3 when the fair values are determined using pricing models, discounted cash flows or similar techniques and at least one significant model assumption or input is unobservable. There were no Level 3 assets or liabilities as of December 31, 2025 and 2024. Previously, the Company's Level 3 liabilities consisted of a royalty monetization liability. In 2024, the Company recorded reduction of the fair value of the royalty monetization liability to zero as a result of the improbability that the program would be further developed into a commercial product.

The carrying amounts reported in the consolidated balance sheets for cash, cash equivalents and marketable securities, other current assets, accounts payable, and accrued expenses approximate their fair values based on the short-term maturity of these instruments.

(G) Leases

The Company determines if an arrangement is a lease at inception and recognizes the lease in accordance with ASC 842, Lease Accounting. Operating leases are included in right-of-use ("ROU") assets, current portion, lease liability and long-term lease liability in the Company's consolidated balance sheets. ROU assets represent the right to use an underlying asset for the lease term and lease liabilities represent the Company's obligation to make lease payments arising from the lease. Operating lease ROU assets and liabilities are recognized at the lease commencement date based on the present value of the lease payments over the lease term. The Company determines the portion of the lease liability that is current as the difference between the calculated lease liability at the end of the current period and the lease liability that is projected 12 months from the current period.

(H) Property and Equipment

Property and equipment are stated at cost and depreciated over their estimated useful lives of three years, or in the case of leasehold improvements, over the remaining life of the relevant lease, using the straight-line method. Repair and maintenance costs are expensed. The Company reviews the recoverability of all long-lived assets, including the related useful life, whenever events or changes in circumstances indicate that the carrying amount of a long-lived asset might not be recoverable.

(I) Research and Development Expenses

The Company expenses the cost of research and development as incurred. Research and development expenses are comprised of costs incurred in performing research and development activities, including clinical trial costs, manufacturing costs for both clinical and preclinical materials as well as contracted services, license fees, and other external costs. Research and development expenses also include the cost of licensing agreements acquired from third-parties. Nonrefundable advance payments for goods and services that will be used in future research and development activities are expensed when the activity is performed or when the goods have been received in accordance with ASC 730, Research and Development.

(J) Stock-based Compensation

The Company accounts for its stock-based compensation in accordance with ASC 718, Compensation—Stock Compensation, which establishes accounting for stock-based awards granted to employees for services and requires companies to expense the estimated fair value of these awards over the requisite service period. The Company estimates the fair value of all awards granted using the Black-Scholes valuation model. Key inputs and assumptions include the expected term of the option, stock price volatility, risk-free interest rate, dividend yield, stock price and exercise price. Many of the assumptions require judgment and any changes could have an impact in the determination of stock-based compensation expense. The Company elected to record forfeitures as they occur. The Company recognizes employee stock-based compensation expense based on the fair value of the award on the date of the grant. The compensation expense is recognized over the vesting period using the straight-line method. The Company aggregates employee and nonemployee awards for certain disclosures since nonemployee awards are not material.

(K) Royalty Monetization Liability

The Company accounted for its sale to Ligand Pharmaceuticals Incorporated ("Ligand") of a 13% share of royalties and milestones owed to the Company related to the potential approval and commercialization of soticlestat ("Ligand Agreement") in accordance with ASC 470, Debt, classifying the proceeds received from the sale to Ligand as debt as the Company determined that it had significant continuing involvement in the generation of the cash flows to Ligand. The Company further elected to account for the debt at fair value with changes in the fair value of the debt classified as other income (expense) in the consolidated statements of operations. In June 2024, Takeda issued a press release indicating the soticlestat trials missed their primary endpoints and noted that while Takeda would discuss the program with FDA, Takeda fully impaired the asset representing soticlestat. In 2024, the Company recorded a gain of $30.0 million due to reducing the fair value of the Ligand Agreement debt to zero as a result of the improbability that the program would be further developed into a commercial product by Takeda or another party. In January 2025, Takeda announced the discontinuation the program.

(L) Income Taxes

The Company accounts for income taxes under the asset and liability method, which requires deferred tax assets and liabilities to be recognized for the estimated future tax consequences attributable to differences between financial statement carrying amounts and respective tax bases of existing assets and liabilities, as well as for net operating loss carryforwards and research and development credits. Valuation allowances are provided if it is more likely than not that some portion or all of the deferred tax assets will not be realized. The impact of a change in tax laws is recorded in the period in which the law is enacted.

(M) Net Loss per Share

The rights and preferences of the Series A Preferred stock are negligible relative to common stock, therefore the Series A Preferred stock is treated as in-substance common stock on an as-converted basis when allocating net loss to actual and in-substance shares of common stock. The Company applies the two-class method to allocate earnings between common stock, Series A Preferred stock as well as other securities deemed in-substance common stock and participating securities, if any.

Net loss per share of common stock is determined by dividing net loss attributable to common stockholders by the basic and diluted weighted-average shares of common stock outstanding during the period. Net loss per share of Series A Preferred stock is determined by dividing net loss attributable to Series A Preferred stockholders on an as-converted basis by the basic and diluted weighted-average shares of Series A Preferred stock outstanding during the period.

Net loss per diluted share attributable to common stockholders adjusts the basic earnings per share attributable to common stockholders and the weighted-average number of shares of common stock outstanding for the potential dilutive impact of stock options using the treasury-stock method and the potential impact of any preferred stock using the if-converted method. Net loss per diluted attributable to common stockholders omits the inclusion of options and common stock issuable upon conversion of our preferred stock as these securities would be anti-dilutive.

(N) Retirement Plan

The Company maintains a 401(k)-retirement plan for its employees that is intended to qualify under Sections 401(a) and 501(a) of the U.S. Internal Revenue Code of 1986, as amended ("Code"). The Company provides all active employees with a 100% matching contribution equal to 3% of an employee's eligible deferred compensation and a 50% matching contribution on employee contributions that are between 3% and 5% of an employee's eligible deferred compensation. These safe harbor contributions vest immediately. For the years ended December 31, 2025 and 2024 the Company contributed $0.2 million and $0.3 million, respectively.

(O) Revenue Recognition

Under ASC 606, Revenue from Contracts with Customers, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration that the entity expects to receive in exchange for those goods or services. In applying ASC 606, the Company performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the promises and performance obligations in the contract; (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 performance obligations are satisfied. The Company only applies the five-step model to contracts when it is probable that it will collect the consideration to which it is entitled in exchange for the goods or services transferred to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, the Company assesses the goods or services promised within each contract, determines those that are performance obligations and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

Prior to recognizing revenue, the Company makes estimates of the transaction price, including variable consideration that is subject to a constraint. Amounts of variable consideration are included in the transaction price to the extent that it is probable that a significant reversal in the amount of cumulative revenue recognized will not occur and when the uncertainty associated with the variable consideration is subsequently resolved.

If there are multiple distinct performance obligations, the Company allocates the transaction price to each distinct performance obligation based on its relative standalone selling price. The standalone selling price is generally determined using expected cost and comparable transactions. Revenue for performance obligations recognized over time is recognized by measuring the progress toward complete satisfaction of the performance obligations using an input measure.

Nonrefundable upfront fees allocated to licenses that are not contingent on any future performance and require no consequential continuing involvement by the Company, are recognized as revenue when the license term commences and the licensed data, technology or product is delivered. The Company defers recognition of upfront license fees if the performance obligations are not satisfied.

(P) Segment Reporting

Under Accounting Standards Update ("ASU") 2023-07, Segment Reporting (Topic 280): "Improvements to Reportable Segment Disclosures" ("ASU 2023-07"), the Company discloses significant segment expenses regularly provided to the chief operating decision maker ("CODM") and discloses the title and position of the CODM.

(Q) Common Warrants

The Company has issued freestanding warrants to purchase shares of its common stock in connection with financing activities and accounts for them in accordance with applicable accounting guidance as either liabilities or as equity instruments depending on the specific terms of the warrant agreements.

(R) Recent Accounting Pronouncements

The Company has reviewed recently issued accounting standards and plans to adopt those that are applicable. The Company does not expect the adoption of those standards to have a material impact on its financial position, results of operations or cash flows.

In November 2024, the FASB issued ASU 2024-03, Income Statement – Reporting Comprehensive Income – Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses, which requires the disaggregation of certain expense captions into specified categories in disclosures within the notes to the consolidated financial statements to provide enhanced transparency into the expense captions presented on the face of the statements of income and comprehensive income. ASU 2024-03 is effective for annual reporting periods beginning after December 15, 2026, with early adoption permitted, and may be applied either prospectively or retrospectively to financial statements issued for reporting periods after the effective date of ASU 2024-03 or retrospectively to any or all prior periods presented in the financial statements. The Company is currently evaluating the impact that the adoption of ASU 2024-03 will have on its related disclosures.

In December 2023, the FASB issued ASU 2023-09, Improvements to Income Tax Disclosures, ("ASU 2023-09"), which is effective for annual periods beginning after December 15, 2024. ASU 2023-09 intends to enhance the transparency as well as usefulness of income tax disclosures, primarily related to the rate reconciliation and income taxes paid. The Company has adopted ASU 2023-09 retrospectively and the related disclosures are reflected in Note 9.

The Company adopts new pronouncements relating to GAAP applicable to the Company as they are issued, and based upon the effective dates included in the pronouncements. Management does not believe that any recently issued, but not yet effective accounting standards, if currently adopted, would have a material effect on the accompanying consolidated financial statements.

NOTE 3 – CASH, CASH EQUIVALENTS AND MARKETABLE SECURITIES

The following tables summarize the fair value of cash, cash equivalents and marketable securities as well as gross unrealized holding gains and losses as of December 31, 2025 and 2024:

	December 31, 2025			
(in thousands)	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Fair Value
Cash	$ 516	$ —	$ —	$ 516
Cash equivalents	12,636	1	—	12,637
Marketable securities	77,315	—	(21)	77,294
Total cash, cash equivalents and marketable securities	$ 90,467	$ 1	$ (21)	$ 90,447

(in thousands)	December 31, 2024			
	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Fair Value
Cash	$ 522	$ —	$ —	$ 522
Cash equivalents	25,779	—	—	25,779
Marketable securities	26,767	7	—	26,774
Total cash, cash equivalents and marketable securities	$ 53,068	$ 7	$ —	$ 53,075

The Company did not hold any securities that were in an unrealized loss position for more than 12 months as of December 31, 2025 and 2024.

There were no material realized gains or losses on available-for-sale securities during the years ended December 31, 2025 and 2024.

NOTE 4 – PROPERTY AND EQUIPMENT AND INTANGIBLE ASSETS

Property and equipment is summarized as follows:

(in thousands)	December 31, 2025	December 31, 2024
Furniture and equipment	$ 1,534	$ 1,534
Leasehold improvements	306	306
Less accumulated depreciation	(1,588)	(1,407)
Total property and equipment, net	$ 252	$ 433

Depreciation expense was $0.2 million and $0.4 million for the years ended December 31, 2025 and 2024, respectively.

Intangible assets, net of accumulated amortization, were zero and $0.1 million as of December 31, 2025 and 2024, respectively, and are included in other assets. Amortization expense was $0.1 million and $0.2 million for the years ended December 31, 2025 and 2024, respectively.

NOTE 5 – LEASES

In September 2021, the Company entered into a 10-year lease agreement for its corporate headquarters, with a term commencing in March 2022, for approximately 19,000 square feet of office space at Hudson Commons in New York, NY (“Hudson Commons Lease”). The lease provides for monthly rental payments over the lease term. The base rent under the lease is currently $2.3 million per year. Rent payments commenced ten months following the commencement of the lease, or January 2023, and continue for ten years following the rent commencement date. The Company issued a letter of credit in the amount of $1.9 million in association with the execution of the lease agreement; the letter of credit is characterized as restricted cash on the Company’s consolidated balance sheets.

The Hudson Commons Lease has a remaining lease term of approximately eight years and includes a single renewal option for an additional five years. The Company did not include the renewal option in the lease term when calculating the lease liability as the Company is not reasonably certain that it will exercise the renewal option. The present value of the lease payments is calculated using an incremental borrowing rate of 7.02%. Lease expense is included in general and administrative and research and development expenses in the consolidated statements of operations.

ROU asset and lease liabilities related to the Company's operating lease are as follows:

(in thousands)	December 31, 2025	December 31, 2024
ROU asset, net	$ 11,610	$ 12,797
Current lease liability	$ 1,433	$ 1,336
Long-term lease liability	$ 11,986	$ 13,419

The components of operating lease cost for the year ended December 31, 2025 and 2024 were as follows:

(in thousands)	December 31, 2025	December 31, 2024
Operating lease cost	$ 2,167	$ 2,167
Variable lease cost	—	—
Short-term lease cost	—	—

Future minimum commitments under the non-cancelable operating lease are as follows:

(in thousands)	
2026	$ 2,316
2027	2,316
2028	2,469
2029	2,469
2030	2,469
Thereafter	4,939
	$ 16,978

NOTE 6 – ACCRUED EXPENSES

Accrued expenses consist of the following:

(in thousands)	December 31, 2025	December 31, 2024
Payroll and bonus accrual	$ 3,106	$ 2,959
Research and development accrual	1,191	2,779
Professional fees accrual	350	168
Other	252	88
Total	$ 4,899	$ 5,994

NOTE 7 – STOCKHOLDERS' EQUITY

The Company's capital structure consists of common stock and preferred stock. Pursuant to the Company's amended and restated certificate of incorporation, as amended, the Company is authorized to issue up to 315,000,000 shares of common stock and 10,000,000 shares of preferred stock. The Company has designated 10,000 of the authorized shares of preferred stock as non-voting Series A convertible preferred stock ("Series A Preferred Stock") and 57,722 of the authorized shares of preferred stock as non-voting Series B convertible preferred stock ("Series B Preferred Stock").

The holders of common stock are entitled to one vote for each share held. The holders of common stock have no preemptive or other subscription rights, and there are no redemption or sinking fund provisions with respect to such shares. Subject to preferences that may apply to any outstanding series of preferred stock, holders of the common stock are entitled to receive ratably any dividends declared on a non-cumulative basis. The common stock is subordinate to all series of Preferred Stock with respect to rights upon liquidation, winding up and dissolution of the Company. The holders of common stock are entitled to liquidation proceeds after all liquidation preferences for the preferred stock are satisfied.

There were 1,250 shares of Series A Preferred Stock outstanding as of December 31, 2024 and 1,250 shares of Series A Preferred Stock optionally converted into a total of 1,250,000 shares of common stock in December 2025. The Series A Preferred Stock was non-voting and had a liquidation preference such that in the event of a liquidation, dissolution, or winding up of the Company, holders of Series A Preferred Stock would receive a payment equal to $0.001 per share of Series A Preferred Stock before any proceeds were distributed to the holders of common stock. Holders of Series A Preferred Stock were entitled to receive dividends paid to holders of common stock at an equal rate, in the same form, and in the same manner on an as-if-converted basis.

In October 2025, the Company entered into a Securities Purchase Agreement with the purchasers named therein (the "Investors"), pursuant to which the Company issued and sold an aggregate of (i) 57,722 shares of Series B Preferred Stock, (ii) Series A warrants (the "Series A Warrants") to purchase up to 38,481,325 shares of the Company's common stock and/or pre-funded warrants to purchase common stock (the "Pre-Funded Warrants"), and (iii) Series B warrants (the "Series B Warrants" and together with the Series A Warrants, the "Warrants") to purchase up to 28,861,000 shares of common stock and/or Pre-Funded Warrants to the investors in a private placement (the "2025 Private Placement"). Each share of Series B Preferred Stock was sold together with a Series A Warrant to purchase up to 666.66 shares of common stock and/or Pre-Funded Warrants (rounded down to next whole share based on each such Investor's aggregate purchase) and a Series B Warrant to purchase up to 500 shares of common stock and/or Pre-Funded Warrants (together, one "Security"). The Securities were sold at a purchase price of $1,400.00, which included the purchase of 71 shares of Series B Preferred Stock, 47,333 Series A Warrants, and 35,500 Series B Warrants by the Company's Executive Chairman, which was approved by the Company's stockholders at a special meeting in December 2025 (the "Special Meeting").

Each share of Series B Preferred Stock is convertible into 1,000 shares of common stock and on receipt of stockholder approval at the Special Meeting of an increase of sufficient authorized shares of common stock to enable issuance of common stock on conversion of all of the Series B Preferred Stock, the shares of Series B Preferred Stock became mandatorily convertible, subject to beneficial ownership limitations. Had any investor been unable to convert all of their Series B Preferred Stock due to beneficial ownership limitations, those shares would be optionally convertible at any time after the mandatory conversion event when their ownership limitation allowed. The Series B Preferred Stock is non-voting, except for customary protective provisions, and has rights to receive dividends *pari passu,* on an as-converted basis, if and when the shareholder of common stock received dividend payment. The Series B Preferred Stock has a liquidation preference equal to the greater of a) $1,400 per share plus any declared but unpaid dividends due to stockholders of Series B Preferred Stock or b) the amount due on an as-converted basis while maintaining the seniority of the stock class.

The Warrants each have an exercise price of $1.40 per share (the "Exercise Price"). The Series A Warrants became exercisable upon stockholder approval at the Special Meeting and expire on the earlier of October 6, 2030 or the 30th calendar day following the date on which the Company publicly announces the clearance of the first of any investigational new drug application, clinical trial application or other foreign equivalent with respect to the clinical development of the Company's OV4071 product candidate. In the event that beneficial ownership limitations prevent the exercise by an Investor of all or a portion of the Series A Warrants held thereby, such Investor may purchase shares of common stock up to the specified limit and, for the remainder, purchase Pre-Funded Warrants in lieu of shares of common stock. The Series A Warrants meet the requirements to be recorded in permanent equity. The Series A Warrants are entitled to dividends on an as-converted basis if and when dividends are paid to common stock, therefore the Series A Warrants meet the definition of participating securities for the purpose of computing earnings per share.

The Series B Warrants expire on October 6, 2030. In the event that the closing price of the Company's common stock equals or exceeds 300% of the Exercise Price (subject to customary adjustments) for 20 of any 30 consecutive trading days, the Company may elect to require exercise of the Warrant for cash. In the event that beneficial ownership limitations prevent the exercise of all or a portion of the Series B Warrants held thereby upon any such mandatory exercise demand, such Investor will purchase shares of common stock up to the specified limit and, for the remainder, purchase Pre-Funded Warrants. The Series B Warrants are entitled to dividends on an as-converted basis if and when dividends are paid to common stock, therefore the Series B Warrants meet the definition of participating securities for the purpose of computing earnings per share.

The Company received initial net proceeds of $75.1 million from the 2025 Private Placement, after deducting placement agent fees and offering expenses of $5.7 million. The Company may further receive up to $94.0 million in additional gross proceeds, assuming exercise in full of the Warrants.

In December 2025, the Company held a special meeting of stockholders, at which the Company's stockholders approved (a) an amendment to the Company's amended and restated certificate of incorporation, as amended to date, to increase the number of authorized shares from 125,000,000 to 315,000,000 shares, (b) the issuance of shares of common

stock upon the conversion of the Series B Preferred Stock and the exercise of Series A Warrants and Series B Warrants, and (c) the issuance and sale of Securities to our Executive Chairman. Stockholder approval of the increase in the number of authorized shares of the Company's common stock and the issuance of common stock on the conversion of Series B Preferred Stock triggered the mandatory conversion of all of the shares of the Series B Preferred Stock into an aggregate of 57,722,000 shares of common stock. At December 31, 2025, there were no outstanding shares of Series B Preferred Stock. The Series A Warrants and the Series B Warrants are now exercisable.

The Company valued the components of the Securities and allocated the issuance costs using relative fair values. The Series B Preferred Stock was valued at $1.79 per underlying common share, which was the close price of the common stock on the financing close date of October 6, 2025 due to the high probability of mandatory conversion within a short period of time.

The Series A Warrants were valued at $0.75 per share using a probability-weighted expected return method based on two different expiry periods valued using a Black-Scholes model with corresponding probability for the likelihood of each scenario and Level 3 model inputs.

The Series B Warrants were valued at $1.29 using a Monte Carlo simulation model since the timing and payoff are dependent on the Company's trailing stock price and level 3 inputs. It is assumed that the Company would trigger a mandatory conversion at the earliest triggering event.

The following table presents the level 3 inputs used in the valuation models:

	Series A Warrants		Series B Warrants
	6-month expiry	5-year expiry	
Probability	97.5 %	2.5 %	
Stock price	$ 1.79	$ 1.79	$ 1.79
Strike price	$ 1.40	$ 1.40	$ 1.40
Expected volatility	115 %	115 %	115 %
Expected term in years	0.5	5.0	
Dividend rate	—	—	—
Risk-free interest rate	3.81 %	3.75 %	3.75 %
Fair value	$ 0.73	$ 1.51	$ 1.29

The fair value of the Series B Preferred Stock and the associated allocation of issuance costs was recorded in temporary equity until such time as the stockholder approvals were obtained. On gaining stockholder approvals the Series B Preferred Stock converted automatically and the temporary equity balances were moved to permanent equity. The Series A Warrants and Series B Warrants do not have features that disallowed equity treatment and were recorded at relative fair value along with the allocated issuance costs in permanent equity.

The following table summarizes the number of Warrants outstanding and the weighted average exercise price:

	Warrants	Weighted Average Exercise Price	Aggregate Intrinsic Value (in thousands)
Outstanding, December 31, 2024	—	$ —	$ —
Granted	67,342,325	$ 1.40	$ 15,489
Exercised	—	$ —	$ —
Outstanding, December 31, 2025	67,342,325	$ 1.40	$ 15,489

Dividends

Through December 31, 2025, the Company has not declared or paid any dividends. No dividends on the common stock shall be declared and paid unless dividends on the preferred stock have been declared and paid.

NOTE 8 – STOCK-BASED COMPENSATION

The Company's Board of Directors (the "Board") adopted, and the Company's stockholders approved, the 2017 Equity Incentive Plan ("2017 Plan"), which became effective on May 4, 2017. The initial reserve of shares of common stock under the 2017 Plan was 3,052,059 shares. The 2017 Plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock, restricted stock units ("RSUs"), stock appreciation rights, performance-based stock awards and other forms of stock-based awards. Additionally, the 2017 Plan provides for the grant of performance cash awards. The Company's employees, officers, directors, consultants and advisors are eligible to receive awards under the 2017 Plan. Upon the adoption of the 2017 Plan, no further awards were granted under the prior plan. Pursuant to the terms of the 2017 Plan, on each January 1st, the plan limit shall be increased by the lesser of (x) 5% of the number of shares of common stock outstanding as of the immediately preceding December 31 and (y) such lesser number as the Board may determine in its discretion. An additional 6,509,217, 0, and 3,534,600 shares were reserved for issuance under the 2017 Plan on January 1, 2026, 2025, and 2024, respectively. As of December 31, 2025, there were 4,652,938 shares of the Company's common stock reserved for issuance under the 2017 Plan.

The Board adopted, and the Company's stockholders approved, the 2017 employee stock purchase plan ("ESPP"), which became effective on May 4, 2017. The initial reserve of shares of common stock that may be issued under the ESPP was 279,069 shares. The ESPP allows employees to purchase common stock of the Company at a 15% discount to the market price on designated purchase dates. During the years ended December 31, 2025 and 2024, 76,976 and 69,850 shares were purchased under the ESPP and the Company recorded expense of $38,000 and $65,000, respectively. The number of shares of common stock reserved for issuance under the ESPP will automatically increase on January 1 of each year, beginning on January 1, 2018 and continuing through and including January 1, 2027, by the lesser of (i) 1% of the total number of shares of the Company's capital stock outstanding on December 31 of the preceding calendar year, (ii) 550,000 shares or (iii) such lesser number of shares determined by the Board. The Board acted prior to January 1, 2026, 2025 and 2024 to provide that there be no increase in the number of shares reserved for issuance under the ESPP. As of December 31, 2025 and 2024, there were 206,020 and 282,996 shares of the Company's common stock reserved for issuance under the ESPP, respectively.

The Board adopted, and the Company's stockholders approved, the 2014 Equity Incentive Plan ("2014 Plan"), which authorized the Company to grant shares of common stock in the form of incentive stock options, nonstatutory stock options, stock appreciation rights, restricted stock and restricted stock units. The 2014 Plan was terminated as to future awards in May 2017, although it continues to govern the terms of options that remain outstanding under the 2014 Plan. No additional stock awards will be granted under the 2014 Plan, and all outstanding stock awards granted under the 2014 Plan that are repurchased, forfeited, expire or are cancelled will become available for grant under the 2017 Plan in accordance with its terms. As of December 31, 2025 and 2024, options to purchase 793,833 and 1,328,715 shares of common stock were outstanding under the 2014 Plan, respectively.

Unless specified otherwise in an individual option agreement, stock options granted under the 2014 Plan and 2017 Plan have a ten-year term and a four-year graded vesting period. The vesting requirement is generally conditioned upon the grantee's continued service with the Company during the vesting period. Once vested, all options granted are exercisable from the date of grant until they expire. The option grants are non-transferable. Vested options remain exercisable for 90 days under the 2017 Plan and 30 days under the 2014 Plan subsequent to the termination of the option holder's service with the Company. In the event of the option holder's death or disability while employed by or providing service to the Company, the exercisable period extends to 18 months or 12 months, respectively, under the 2017 Plan and 6 months under the 2014 Plan.

Performance-based option awards generally have similar vesting terms, with vesting occurring on the date the performance condition is achieved and expire in accordance with the specific terms of the agreement. At December 31, 2025 and 2024, there were no performance-based options outstanding and unvested that include options to vest upon the achievement of certain research and development milestones.

The fair value of options granted during the years ended December 31, 2025 and 2024 was estimated using the Black-Scholes option valuation model. The inputs for the Black-Scholes option valuation model require assumptions made by management and are detailed in the table below. The risk-free interest rates were based on the rate for U.S. Treasury securities at the date of grant with maturity dates approximately equal to the expected life at the grant date. The expected life was based on the simplified method in accordance with the SEC Staff Accounting Bulletin No. Topic 14D. The expected volatility was estimated based on the Company's published historical stock prices.

The Company granted 4,152,650 and 4,699,810 stock options during the years ended December 31, 2025 and 2024, respectively. There were 6,570,487 and 5,688,743 unvested options outstanding as of December 31, 2025 and 2024, respectively. Total expense recognized related to the stock options for the years ended December 31, 2025 and 2024 was $3.2 million and $6.2 million, respectively. Total unrecognized compensation expense related to stock options was $8.3 million and $9.1 million as of December 31, 2025 and 2024, respectively.

The Company granted 327,326 and 348,575 RSUs during the years ended December 31, 2025 and 2024. The RSUs vest in equal installments over three years on the grant date anniversary for all executive awards and 2025 non-executive awards and annually on January 1st for the 2024 non-executive awards.

The Company's stock-based compensation expense was recognized in operating expenses as follows:

	For the Year Ended December 31,	
(in thousands)	2025	2024
Research and development	$ 1,497	$ 1,631
General and administrative	3,310	4,645
Total	$ 4,807	$ 6,276

	For the Year Ended December 31,	
(in thousands)	2025	2024
Stock options and RSUs	$ 4,769	$ 6,212
ESPP	38	64
Total	$ 4,807	$ 6,276

The fair value of stock options granted during the years ended December 31, 2025 and 2024, respectively, was estimated by utilizing the following assumptions:

	For the Year Ended December 31,	
	2025	2024
	Weighted Average	Weighted Average
Volatility	97.02 %	87.18 %
Expected term in years	6.02	5.93
Dividend rate	0.00 %	0.00 %
Risk-free interest rate	4.29 %	4.18 %
Fair value of option on grant date	$ 0.55	$ 1.88

The following table summarizes the number of options outstanding and the weighted average exercise price:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life in Years	Aggregate Intrinsic Value (in thousands)
Options outstanding December 31, 2023	15,124,546	$ 3.87	6.90	$ 5,213
Vested and exercisable December 31, 2023	9,649,094	$ 4.47	5.97	$ 2,465
Granted	4,699,810	2.58	5.87	
Exercised	(248,024)	3.13		
Forfeited or expired	(4,234,976)	3.77		
Options outstanding December 31, 2024	15,341,356	$ 3.49	5.87	$ —
Vested and exercisable December 31, 2024	9,652,613	$ 4.07	5.87	$ —
Granted	4,152,650	0.64		
Exercised	(60,372)	1.05		
Forfeited or expired	(1,820,282)	4.32		
Options outstanding December 31, 2025	17,613,352	$ 2.68	6.56	$ 5,332
Vested and exercisable December 31, 2025	11,042,865	$ 3.49	5.25	$ 1

At December 31, 2025, there was $8.3 million of unamortized stock-based compensation expense, which is expected to be recognized over a remaining average vesting period of 2.39 years. At December 31, 2024, there was $9.1 million of unamortized stock-based compensation expense, which is expected to be recognized over a remaining average vesting period of 2.23 years.

NOTE 9 – INCOME TAXES

At December 31, 2025, the Company has available $214.9 million and $202.7 million of unused net operating loss ("NOL") carryforwards for federal and state tax purposes, respectively, that may be applied against future taxable income. The Company also has $163.8 million of unused NOL carryforwards for New York City purposes. The NOL carryforwards will begin to expire in the year 2037 if not utilized prior to that date.

Under Section 382 and Section 383 of the Internal Revenue Code of 1986, if a corporation undergoes an ownership change, the corporation's ability to use its pre-change NOL carryforwards and other pre-change tax attributes to offset its post-change income may be limited. On each of August 10, 2015 and February 22, 2019 the Company experienced an ownership change. The Company anticipates a significant portion of its pre-change NOLs to be limited, however has not completed a formal Section 382 analysis subsequent to the last ownership change.

The Company maintains a full valuation allowance against its net deferred tax assets. The valuation allowance increased by $1.6 million for the year ended December 31, 2025 and decreased by $3.2 million for the year ended December 31, 2024. The increase in valuation allowance in 2025 was primarily due to increase in net operating loss carryovers, offset by decrease in various other temporary and permanent differences.

Loss before income taxes resulting from operations is as follows:

(in thousands)	December 31, 2025	December 31, 2024
Domestic	$ (17,775)	$ (25,058)
Foreign	361	(1,375)
Pretax loss from operations	$ (17,414)	$ (26,433)

The tax effects of temporary differences that gave rise to significant portions of the deferred tax assets and liabilities were as follows:

(in thousands)	December 31, 2025	December 31, 2024
Deferred tax assets/liabilities:		
Net operating loss carryovers	$ 70,229	$ 62,120
Intangible assets	4,550	5,406
Capitalized research and experimental costs	14,289	14,939
Stock-based compensation	5,089	5,534
Lease liability	2,890	3,324
Research and development tax credits	2,206	2,229
Charitable contributions	2	2
Depreciation	(48)	(93)
Right-of-use asset	(2,501)	(2,883)
Unrealized gain on long-term equity investment	(5,612)	(1,103)
Total net deferred tax assets/liabilities	91,094	89,474
Valuation allowance	(91,094)	(89,474)
Net deferred tax assets (liabilities)	$ —	$ —

A reconciliation of the amounts at the U.S. federal statutory rate to the Company's effective income tax rate is as follows:

(in thousands)	December 31, 2025		December 31, 2024	
	Amount	Percent	Amount	Percent
U.S. federal statutory tax rate	$ (3,657)	21.0 %	$ (5,525)	21.0 %
State and local income taxes, net of federal income tax effect	2	— %	—	— %
Foreign tax effects				
Australia				
Changes in valuation allowance	(55)	0.3 %	370	(1.4)%
Other	(21)	0.1 %	(81)	0.3 %
Effect of changes in tax laws or rates enacted in the current period				
Effect of cross-border tax laws				
Other	—	— %	99	(0.4)%
Tax credits				
Changes in valuation allowances	2,686	(15.4)%	3,391	(12.9)%
Nontaxable or nondeductible items				
Stock-based awards	1,188	(6.8)%	1,526	(5.8)%
Other	7	— %	164	(0.6)%
Changes in unrecognized tax benefits				
Other adjustments				
Other	(52)	0.3 %	55	(0.2)%
Effective income tax rate	$ 98	(0.5)%	$ —	0.0 %

The Company's reserves related to taxes are based on a determination of whether and how much of a tax benefit taken by the Company in its tax filings or positions is more likely than not to be realized following resolution of any potential contingencies related to the tax benefit. For the years ended December 31, 2025 and 2024, the Company had no unrecognized tax benefits or related interest and penalties accrued. The Company would recognize both accrued interest

and penalties related to unrecognized benefits in provision for income taxes. The Company's uncertain tax positions yet to be determined would be related to years that remain subject to examination by relevant tax authorities. Since the Company is in a loss carryforward position, the Company is generally subject to examination by the U.S. federal, state and local income tax authorities for all tax years in which a loss carryforward is available.

NOTE 10 – COMMITMENTS AND CONTINGENCIES

License Agreements

Northwestern University License Agreement

In December 2016, the Company entered into a license agreement ("Northwestern Agreement") with Northwestern University ("Northwestern"), pursuant to which Northwestern granted the Company an exclusive, worldwide license to patent rights of certain inventions ("Northwestern Patent Rights") which relate to a specific compound and related methods of use for such compound, along with certain know-how related to the practice of the inventions claimed in the Northwestern Patent Rights. The Company is developing OV329 under this agreement.

Under the Northwestern Agreement, the Company was granted exclusive rights to research, develop, manufacture and commercialize products utilizing the Northwestern Patent Rights for all uses. The Company has agreed that it will not use the Northwestern Patent Rights to develop any products for the treatment of cancer, but Northwestern may not grant rights in the technology to others for use in cancer. The Company also has an option, exercisable during the term of the agreement to an exclusive license under certain intellectual property rights covering novel compounds with the same or similar mechanism of action as the primary compound that is the subject of the license agreement. Northwestern has retained the right, on behalf of itself and other non-profit institutions, to use the Northwestern Patent Rights and practice the inventions claimed therein for educational and research purposes and to publish information about the inventions covered by the Northwestern Patent Rights.

Upon entry into the Northwestern Agreement, the Company paid an upfront non-creditable one-time license issuance fee of $75,000 and is required to pay an annual license maintenance fee of $20,000, which will be creditable against any royalties payable to Northwestern following first commercial sale of licensed products under the agreement. The Company is responsible for all ongoing costs of filing, prosecuting and maintaining the Northwestern Patent Rights, but also has the right to control such activities using its own patent counsel. In consideration for the rights granted to the Company under the Northwestern agreement, the Company is required to pay to Northwestern up to an aggregate of $5.3 million upon the achievement of certain development and regulatory milestones for the first product covered by the Northwestern Patent Rights, and upon commercialization of any such products, will be required to pay to Northwestern a tiered royalty on net sales of such products by the Company, its affiliates or sublicensees, at percentages in the low to mid-single-digits, subject to standard reductions and offsets. The Company's royalty obligations continue on a product-by-product and country-by-country basis until the later of the expiration of the last-to-expire valid claim in a licensed patent covering the applicable product in such country and 10 years following the first commercial sale of such product in such country. If the Company sublicenses a Northwestern Patent Right, it will be obligated to pay to Northwestern a specified percentage of sublicense revenue received by the Company, ranging from the high single-digits to the low-teens.

The Northwestern agreement requires that the Company use commercially reasonable efforts to develop and commercialize at least one product that is covered by the Northwestern Patent Rights.

Unless earlier terminated, the Northwestern agreement will remain in force until the expiration of the Company's payment obligations thereunder. The Company has the right to terminate the agreement for any reason upon prior written notice or for an uncured material breach by Northwestern. Northwestern may terminate the agreement for the Company's uncured material breach or insolvency.

The Company incurred licensing expenses related to Northwestern of $20,000 and $100,000 in the years ended December 31, 2025 and 2024, respectively.

The second milestone of $100,000 due under the license agreement was triggered by a time limit to start a phase 2 trial and was recorded as research and development expense in 2024.

AstraZeneca AB License Agreement

In December 2021, the Company entered into an exclusive license agreement with AstraZeneca AB ("AstraZeneca"), for a library of early-stage small molecules targeting the KCC2 transporter, including OV350. Upon execution of the agreement, the Company was obligated to pay an upfront cash payment of $5.0 million and issued shares of the Company's common stock in an amount that equaled $7.3 million based on the volume-weighted average price of shares of the Company's common stock for the 30 business days immediately preceding the execution date of the

transaction. Since the intangibles acquired in the AstraZeneca license agreement do not have an alternative future use, all costs incurred were treated as research and development expense.

Pursuant to the AstraZeneca license agreement, the Company agreed to potential milestone payments of up to $203.0 million upon the achievement of certain developmental, regulatory and sales milestones. The first payment of $3.0 million is due upon the successful completion of the first Phase 2 clinical study of a licensed product following a positive biomarker readout in a Phase 1 clinical study.

Gensaic Equity Agreement and Collaboration and Option Agreement

In August 2022, the Company entered into an equity agreement and a collaboration and option agreement with Gensaic ("Gensaic Collaboration Agreement"). Under the terms of the equity agreement, the Company invested a total of $5.1 million in exchange for convertible preferred stock in Gensaic. The Company also retained rights to invest in future equity financing rounds. Dr. Jeremy Levin, the Company's Executive Chairman, is currently the Chairman of Gensaic's board of directors. The Gensaic Collaboration Agreement involves the research and development of Gensaic's proprietary platform for certain rare central nervous system ("CNS") disorder targets. Under the Gensaic Collaboration Agreement, Gensaic granted the Company an option to obtain an exclusive license with respect to certain identified lead phage-derived particle ("PDP") products, which are exercisable at any time prior to the expiration of the option period. Once a product is identified by the Company that demonstrates sufficient efficacy, the Company may exercise its option with respect to the specific research program for that PDP product.

The Company shall reimburse Gensaic for Gensaic's research costs related to the specific research plan for PDP products identified. The research plan and budget shall be mutually agreed upon by the parties and shall not exceed $3.0 million in any research year. The Company will record these reimbursement payments as research and development costs in the period the research costs are incurred. In May 2023, the Company identified a lead PDP candidate for further research and provided $3.5 million to Gensaic to support the approved research plan and budget. The amount is expensed as the research and development occurs with the remaining amount included in prepaid expenses and other current assets in the consolidated balance sheets. The balance of the previously provided research funds was $1.0 million as of December 31, 2025 and 2024. Research and development expense was zero and $1.5 million during the years ended December 31, 2025 and 2024, respectively.

If a product is ultimately commercialized under the Gensaic Collaboration Agreement, the Company is required to make tiered royalty payments to Gensaic in the mid-single to low double-digit range based on the net sales of all licensed PDP products during the royalty term. The Company is also responsible for potential tiered milestone payments of up to $452.0 million based upon the achievement of certain sales milestone events and developmental milestone approvals for three or more products. Gensaic also has the option to become a collaborative partner in the development and commercialization of PDP products in exchange for a fee based on a percentage of the costs incurred by the Company through the date Gensaic exercises its option. The Company would no longer be required to pay Gensaic royalty or milestone payments if Gensaic elects to exercise its option. The Company may terminate the Gensaic Collaboration Agreement by providing written notice to Gensaic 90 days in advance of the termination date.

As of December 31, 2025, none of the contingent payments are considered probable.

In January 2026, the Company and Gensaic executed an amendment which provided, among other changes to the original agreement, (a) the ability for the Company to apply the remaining prepaid research balance of $1.0 million to a new research project that may or may not be limited to PDP products and (b) provide an option at the end of the research project to enter into exclusive negotiations to enter into a license and development agreement with associated compensation. Any newly negotiated license and development agreement and compensation would supersede the original milestone and royalty schedules.

Non-Operating Loss

During the quarter ended September 30, 2024, the Company was the victim of a criminal scheme involving a business email compromise at one of its development collaborators, which led to a fraudulent transfer totaling $1.8 million to a third-party impersonating one of the Company's development collaborators. A loss was recorded in Other income (expense) in the Consolidated Statement of Operations. The Company subsequently recovered the funds in full and recorded a gain in Other income (expense) in the Consolidated Statement of Operations in 2025.

Contingencies

Liabilities for loss contingencies arising from claims, assessments, litigation, fines, and penalties and other sources are recorded when it is probable that a liability has been incurred and the amount can be reasonably estimated. Legal costs

incurred in connection with loss contingencies are expensed as incurred. The Company is not currently involved in any legal matters arising in the normal course of business.

Under the terms of their respective employment agreements, each of the Company's named executive officers is eligible to receive severance payments and benefits upon a termination without "cause" or due to "permanent disability," or upon "resignation for good reason," contingent upon the named executive officer's delivery to the Company of a satisfactory release of claims, and subject to the named executive officer's compliance with non-competition and non-solicitation restrictive covenants for two years following the termination date.

NOTE 11 – COLLABORATION AGREEMENTS

Takeda Collaboration

In January 2017, the Company entered into a license and collaboration agreement with Takeda under which the Company licensed from Takeda certain exclusive rights to develop and commercialize soticlestat in certain territories.

In March 2021, the Company entered into the Royalty, License, and Termination ("RLT") Agreement with Takeda, pursuant to which Takeda secured rights to the Company's 50% global share in soticlestat, and the Company granted to Takeda an exclusive worldwide license under the Company's relevant intellectual property rights to develop and commercialize the investigational medicine soticlestat for the treatment of developmental and epileptic encephalopathies, including Dravet syndrome and Lennox-Gastaut syndrome.

Under the RLT Agreement, all rights in soticlestat are owned by Takeda or exclusively licensed to Takeda by the Company. Takeda assumed all responsibility for, and costs of, both development and commercialization of soticlestat, and the Company no longer had any financial obligation to Takeda under the original collaboration agreement. In March 2021, upon the closing of the RLT Agreement, the Company received a nonrefundable upfront payment of $196.0 million and was eligible to receive up to an additional $660.0 million upon Takeda achieving developmental, regulatory and sales milestones. Additionally, the Company was entitled to receive tiered royalties beginning in the low double-digits, and up to 20% on sales of soticlestat if regulatory approval was achieved. In 2023, the Company sold a 13% stake in the royalty, regulatory and commercial milestone payments that the Company was eligible to receive under the RLT Agreement to Ligand Pharmaceuticals, Inc. for $30.0 million.

During the years ended December 31, 2025 and 2024, no income or expense was recognized pursuant to the RLT Agreement. In June 2024, Takeda issued a press release indicating the soticlestat trials missed their primary endpoints and noted that while Takeda would discuss the program with FDA, Takeda fully impaired the asset representing soticlestat. In January 2025, Takeda discontinued the program.

Marinus Pharmaceuticals Out-License Agreement

In March 2022 the Company entered into an exclusive patent license agreement with Marinus ("Marinus License Agreement"). Under the Marinus License Agreement, the Company granted Marinus an exclusive, non-transferable (except as expressly provided therein), royalty-bearing right and license under certain Ovid patents relating to ganaxolone to develop, make, have made, commercialize, promote, distribute, sell, offer for sale and import licensed products in the territory (which consists of the United States, the European Economic Area, United Kingdom and Switzerland) for the treatment of CDKL5 deficiency disorders. Following the date of regulatory approval by the FDA of the first licensed product in the territory which was received on March 18, 2022, Marinus issued, at the Company's option, 123,255 shares of Marinus common stock, par value $0.001 per share, as payment. The Marinus License Agreement also provides for payment of royalties from Marinus to the Company in single-digits on net sales of each such licensed product sold.

The Company recorded unrealized losses in other income (expenses), net in the consolidated statements of operations reflecting changes in the value of the Company's equity holding of $1.3 million for the year ended December 31, 2024. In February 2025, Immedica closed a cash purchase of Marinus, resulting in the sale of the Company's equity position in Marinus for $70,000.

In June 2025, the Company entered into an amendment to the Marinus License Agreement with Immedica wherein the parties agreed to replace ongoing royalty payment obligations and add additional licensing for a one-time payment of $7.0 million, which was remitted to the Company pursuant to the agreement within 10 days of execution and recognized as revenue in 2025. The Company immediately recognized $6.3 million of the $7.0 million revenue related to the royalties and existing licenses. The remaining $0.7 million was related to a six-month option for Marinus to include additional patent assignments or expansion of the territory or field of use. The Company initially recorded the value of the option as deferred revenue until the option period lapsed in 2025 without the addition or transfer of any additional licenses. On expiry of the option, the Company recorded the deferred revenue as revenue.

Graviton License Agreement and Equity Purchase

In April 2023, the Company entered into a collaboration and license agreement with Graviton ("Graviton Agreement"), whereby it secured from Graviton an exclusive license to develop and commercialize Graviton's library of ROCK2 inhibitors including their lead program GV101 (OV888) in rare CNS disorders (excluding amyotrophic lateral sclerosis) worldwide (excluding China, Hong Kong, Macau and Taiwan). Under the Graviton Agreement, the Company and Graviton plan to investigate GV101 in cerebral cavernous malformations as well as Graviton's library of ROCK2 inhibitors in other rare CNS disorders. The Company will be responsible for all development and commercialization costs of the products. Should the Company receive regulatory approval and commercialize any of Graviton's ROCK2 inhibitors, it will pay Graviton tiered royalties on net sales ranging from the mid to high teens. As part of the Graviton Agreement, the Company also purchased shares of Graviton's preferred stock for $10.0 million. The Company recorded the purchase of the preferred stock as a long-term equity investment on its consolidated balance sheets. In December 2023 and March 2024, the Company recognized unrealized gains on the investment due to an observable change in price, and recorded the gain in other income (expense), net, in the consolidated statements of operations. The program related to this collaboration agreement is currently paused, pending regulatory feedback on another competitive clinical-stage development program.

NOTE 12 – RELATED PARTY TRANSACTIONS

In March 2021, the Company entered into the RLT Agreement with Takeda. For a description of the RLT Agreement, see Note 11.

In the 2025 Private Placement, Dr. Levin, our Executive Chairman, purchased 71 shares of Series B Preferred Stock, 47,333 Series A Warrants, and 35,500 Series B Warrants for an aggregate purchase price of approximately $99,000. For additional information on the 2025 Private Placement, see Note 7.

NOTE 13 – NET LOSS PER SHARE

The basic and diluted net loss per common share is presented in conformity with the two-class method required for participating securities and multiple classes of shares. The Company considers its Series A Preferred Stock to be in-substance common stock (Note 2), and is reflected as a class of common stock for purposes of calculating net loss per share. While there were no shares of Series A Preferred Stock outstanding at December 31, 2025, the conversion of the Series A Preferred Stock occurred in December 2025 and the weighted average shares outstanding for 2025 are used in calculating net loss per share. The Series B Preferred Stock and the Warrants are participating securities.

Basic net loss per common share is calculated based upon the allocation of net loss to the weighted-average number of common shares outstanding during the period, excluding outstanding stock options that have not yet vested, and weighted-average number of shares of Series A Preferred Stock outstanding during the period on an as-converted basis. For any period in which the Company records net income, diluted net income per share is calculated in the same manner as basic net loss per share, except that the Series B Preferred Stock and the Warrants are participating and are therefore included in the allocation of net income and the calculation of net income per share. Diluted net income per common share includes outstanding common stock, common shares underlying outstanding options and unvested RSUs in the number of shares used to allocate net loss to share classes and as the denominator in calculating net loss per common share - diluted.

Diluted net loss per common share is equivalent to the basic net loss per common share due to the exclusion of outstanding stock options because the inclusion of these securities would result in an anti-dilutive effect on per common share amounts.

The following tables summarize the calculation of basic and diluted net loss per share:

(in thousands, except share and per share data)	For the Year Ended December 31, 2025	
	Series A Preferred Stock	Common Stock
Net loss per share, basic and diluted		
Allocation of loss	$ (272)	$ (17,141)
Weighted-average shares outstanding, basic and diluted	1,171	73,735,606
Net loss per share, basic and diluted	$ (232.47)	$ (0.23)

(in thousands, except share and per share data)	For the Year Ended December 31, 2024	
	Series A Preferred Stock	Common Stock
Net loss per share, basic and diluted		
Allocation of loss	$ (458)	$ (25,975)
Weighted average shares outstanding, basic and diluted	1,250	70,905,422
Net loss per share, basic and diluted	$ (366.33)	$ (0.37)

The following potentially dilutive securities have been excluded from the computations of diluted weighted-average shares outstanding as they would be anti-dilutive:

	For the Year Ended December 31,	
	2025	**2024**
Stock options to purchase common stock	17,613,352	15,341,356
Common stock issuable upon conversion of Series A Preferred Stock	—	1,250,000
Common stock issuable upon exercise of Series A Warrants	38,481,325	—
Common stock issuable upon exercise of Series B Warrants	28,861,000	—
Unvested restricted stock units	420,080	194,075
	85,375,757	16,785,431

NOTE 14 – SEGMENT REPORTING

The Company has determined that it operates as one segment focused on developing medicines for brain disorders with significant unmet need. The Company's pre-commercial development drug candidates have similar economic and other characteristics, including all being in the small molecule therapeutic class that share target markets, development pathways, and regulatory environments. The Chief Operating Decision Maker ("CODM") is the Chief Executive Officer ("CEO"), who reviews profit and loss information on a consolidated basis to assess performance and make operating and planning decisions, including resource allocations among active programs. The determination of the single segment is consistent with the information provided to the CODM. As the Company's operations are comprised of a single reporting segment, the segment assets are reflected on the accompanying consolidated balance sheet as "total assets." Segment asset information is not used by the CODM to allocate resources.

The following tables summarize the Company's segment information as presented to the CODM for the periods indicated:

(in thousands)	For the Year Ended December 31, 2025	2024
Revenue	$ 7,252	$ 566
Payroll and payroll-related expenses	7,765	9,889
Direct program expenses		
KCC2 library	8,567	8,785
OV329	5,744	4,485
OV888 (GV101)	(224)	8,212
Gensaic projects	—	1,493
Other programs	1,639	990
Total direct program expenses	15,726	23,965
Other research and development expenses	2,091	2,913
Total research and development expenses	25,582	36,767
Payroll and payroll-related expenses	10,043	13,835
Legal and professional fees	8,280	6,573
General office expenses	5,786	5,275
Total general and administrative expenses	24,110	25,684
Total operating expenses	49,691	62,451
Operating loss	(42,439)	(61,885)
Other (income) expense, net	25,026	35,452
Net loss	$ (17,414)	$ (26,433)

The program expense for OV888 is negative for the period ended December 31, 2025 because the Company recognized a contra-expense upon settlement of the amounts due to its collaboration partner and on reversal of an immaterial accrual estimate on final determination of amounts owed.

Other research and development expenses include general office expenses allocated to research and development, including costs related to rent and depreciation of leasehold improvements, and nonclinical contract labor. Other income/expense includes decrease in fair value of royalty monetization liability, gain/loss on fraudulent funds transfer, unrealized net gain on equity investments and interest/accretion income on securities.

Other significant segment information includes:

(in thousands)	For the Year Ended December 31, 2025	2024
Decrease in fair value of royalty monetization liability	$ —	$ 30,000
Stock-based compensation expense	4,807	6,276
Interest/accretion income on securities	2,026	3,915
Severance expense	882	3,508
Gain from recovery of fraudulent funds transfer	1,800	—
Loss on fraudulent funds transfer	—	1,800
Unrealized net gain on equity investments	21,052	3,337
Depreciation and amortization	273	613

NOTE 15 – SUBSEQUENT EVENTS

Subsequent to December 31, 2025, Ovid sold 1,500,000 shares of common stock through the Company's at-the-market offering program, resulting in gross proceeds of $2.4 million before deducting sales agent fees and other offering expenses.

On March 18, 2026, the Company publicly announced that it received approval from Human Research Ethics Committee and acknowledgment of the Company's Clinical Trial Notification from the Australian Therapeutic Goods Administration to initiate the Phase 1 clinical trial of OV4071 in Australia. Accordingly, pursuant to the terms of the Series A Warrants, the Series A Warrants will expire on April 17, 2026, if not exercised in full.

On March 17, 2026, the Company entered into a Securities Purchase Agreement with the purchasers named therein (the "Investors"), pursuant to which the Company agreed to issue and sell an aggregate of 19,154,321 shares of common stock at a purchase price of $2.01 per share and, in lieu of common stock, pre-funded warrants to purchase up to 10,701,710 shares of common stock at a purchase price of $2.009 for each pre-funded warrant. The pre-funded warrants have an exercise price of $0.001 per share and will be immediately exercisable. Subject to customary closing conditions, the Company expects to receive gross proceeds of $60.0 million before deducting placement agent fees and transaction costs.