Catalyst pharmaceuticals

2024 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, 2024

OR

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

Commission File No. 001-33057

CATALYST PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**76-0837053**
(State of jurisdiction of incorporation or organization)	**(IRS Employer Identification No.)**
355 Alhambra Circle, Suite 801 Coral Gables, Florida	**33134**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (305) 420-3200

Securities Registered Pursuant to Section 12(b) of the Exchange Act:

Title of Each Class	Ticker Symbol	Name of Exchange on Which Registered
Common Stock, par value $0.001 per share	**CPRX**	**NASDAQ Capital Market**

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

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

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

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company or an emerging growth company. See the definitions of "large accelerated filer", "accelerated filer" and "smaller reporting 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 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, 2024, the last business day of the Registrant's most recently completed second quarter, the aggregate market value of all voting and non-voting common equity held by non-affiliates was $1,720,853,867.

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date: 121,449,655 shares of common stock, $0.001 par value per share, were outstanding as of February 24, 2025.

Part III incorporates certain information by reference from the registrant's definitive proxy statement for the 2025 annual meeting of stockholders. The proxy statement with respect to the 2025 annual meeting of stockholders will be filed no later than 120 days after the close of the registrant's fiscal year ended December 31, 2024.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the Definitive Proxy Statement to be filed for Catalyst Pharmaceuticals, Inc.'s 2025 Annual Meeting of Stockholders are incorporated by reference into Part III hereof.

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EXHIBITS FILED WITH FORM 10-K

PART I

You are urged to read this Annual Report on Form 10-K (Form 10-K) in its entirety. This Form 10-K contains forward-looking statements that involve risks and uncertainties. Our actual results may differ significantly from the projected results discussed in these forward-looking statements. Factors that may cause such a difference include, but are not limited to, those discussed below and in Item 1A, "Risk Factors."

"We," "our," "ours," "us," "Catalyst," or the "Company," when used herein, refers to Catalyst Pharmaceuticals, Inc., a Delaware corporation, and its wholly owned subsidiary, Catalyst Pharmaceuticals Ireland, Ltd., a corporation organized in the Republic of Ireland.

Forward-Looking Statements

This Annual Report on Form 10-K contains "forward-looking statements", as that term is defined in the Private Securities Litigation Reform Act of 1995. These include statements regarding our expectations, beliefs, plans or objectives for future operations and anticipated results of operations. For this purpose, any statements contained herein that are not statements of historical fact may be deemed to be forward-looking statements. Without limiting the foregoing, "believes", "anticipates", "proposes", "plans", "expects", "intends", "may", and other similar expressions are intended to identify forward-looking statements. Such statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or other achievements to be materially different from any future results, performances or achievements expressed or implied by such forward-looking statements. Factors that might cause such differences include, but are not limited to, those discussed in the section entitled "Risk Factors."

The continued successful commercialization of FIRDAPSE® (amifampridine), FYCOMPA® (perampanel) CIII, and AGAMREE® (vamorolone) are highly uncertain. Factors that will affect our success include the uncertainty of:

- Whether we will be able to continue to successfully market and sell FIRDAPSE®, FYCOMPA®, and AGAMREE® while maintaining full compliance with applicable federal and state laws, rules and regulations;
- Whether we will be able to continue to attract and retain the qualified personnel necessary to run our business;
- Whether our estimates of the size of the market for FIRDAPSE® for the treatment of Lambert-Eaton myasthenic syndrome (LEMS) will prove to be accurate;
- Whether the daily dose of FIRDAPSE® taken by patients changes over time and affects our results of operations;
- Whether we will continue to be able to locate LEMS patients who are undiagnosed or are misdiagnosed with another disease;
- Whether patients will discontinue from the use of our products at rates that are higher than historically experienced or are higher than we project;
- Whether new FIRDAPSE®, FYCOMPA®, and AGAMREE® patients can be successfully titrated to stable therapy;
- Whether we can continue to market our products on a profitable and cash flow positive basis;
- Whether we will be able to demonstrate, to the satisfaction of the U.S. Food and Drug Administration (FDA) and third party payors, whether AGAMREE® offers advantages compared to other corticosteroids or competitor's products;
- Whether DMD patients transitioning to current or future gene therapy treatments will delay initiating use of AGAMREE® while waiting for access to such gene therapy, or stop their AGAMREE® therapy during the course of their gene therapy treatment;
- Whether we will be able to continue to successfully commercialize FYCOMPA® after its patents expire starting in May 2025 and generic competition for FYCOMPA® enters the market;
- Whether any revenue or earnings guidance that we provide to the public market will turn out to be accurate;
- Whether payors will continue to provide coverage and reimburse for our products at the price that we charge for our products;
- The ability of our third party suppliers and contract manufacturers to continue to supply sufficient product to meet our customers' needs in a timely manner;
- The ability of our third party suppliers and contract manufacturers to maintain compliance with current cGMP;

- The ability of those third parties that distribute our products to maintain compliance with applicable law;
- Our ability to maintain compliance with applicable rules relating to our patient assistance programs for our products;
- Our ability to maintain compliance with the applicable rules that relate to our contributions to 501(c)(3) organizations that support patients in financial need;
- The scope of our intellectual property and the outcome of challenges to our intellectual property, and, conversely, whether any third party intellectual property presents unanticipated obstacles for FIRDAPSE®, FYCOMPA®, or AGAMREE®;
- Whether there will be a post-closing review by antitrust regulators of our previous acquisition transactions, and the outcome of any such reviews if they occur;
- Whether we will be able to acquire additional drug products under development, complete development required to commercialize such products, and thereafter, if such products are approved for commercialization, successfully market such products;
- Whether we will be successful in our litigation to enforce our patents against the Paragraph IV challengers who have filed Abbreviated New Drug Applications (ANDAs) seeking to introduce generic versions of FIRDAPSE®;
- Whether our patents will be sufficient to prevent generic competition for FIRDAPSE® and AGAMREE® after our orphan drug exclusivity for each product expires;
- The impact on our profits and cash flow of adverse changes in reimbursement and coverage policies or regulations from government and private payors such as Medicare, Medicaid, insurance companies, health maintenance organizations and other plan administrators, or the impact of pricing pressures enacted by industry organizations, the federal government or the government of any state, including as a result of increased scrutiny over pharmaceutical pricing or otherwise;
- Changes in the healthcare industry and the effect of political pressure from and actions by the President, Congress and/or medical professionals seeking to reduce prescription drug costs, and changes to the healthcare industry occasioned by any future changes in laws relating to the pricing of drug products, including changes made in the Inflation Reduction Act of 2022, changes (if any) to be made by the current President and/or the current Congressional administrations, changes to the review and approval process at the FDA, or changes in the healthcare industry generally;
- Whether we and Santhera can successfully develop additional indications for AGAMREE® and obtain the ability to commercialize the product for these additional indications;
- The state of the economy generally and its impact on our business;
- The scope, rate of progress and expense of future clinical trials and studies, pre-clinical studies, proof-of-concept studies, and our other drug development activities, and whether any trials and studies we undertake will be successful;
- Our ability to complete any clinical trials and studies that we may undertake on a timely basis and within the budgets we establish for such trials and studies;
- Whether FIRDAPSE® can be successfully commercialized in Canada on a profitable basis through KYE Pharmaceuticals (KYE), our collaboration partner in Canada;
- Whether AGAMREE® will be approved by Health Canada for commercialization in Canada and whether, if the product is approved, KYE can successfully commercialize it in Canada;
- Whether KYE will successfully file an application seeking to commercialize AGAMREE® in Canada during the first quarter of 2025 or at all;
- Now that FIRDAPSE® has been approved for commercialization in Japan and launched, whether DyDo will be successful in commercializing the product in Japan;
- The impact on sales of FIRDAPSE® in the U.S. if an amifampridine product is purchased in Canada for use in the U.S.;
- Whether our plans to expand the reach of FIRDAPSE® and AGAMREE® into other global regions will be successful;

- System failures or security or data breaches due to cyber-attacks, or cyber intrusions, including ransomware, phishing attacks and other malicious intrusions whether it occurs directly to us or indirectly through third parties; and
- Our ability to enhance our systems, processes and procedures to appropriately support the growing complexity and scale of our business.

Our current plans and objectives are based on assumptions relating to the continued commercialization of FIRDAPSE®, FYCOMPA®, and AGAMREE® and on our plans to seek to acquire or in-license additional products. Although we believe that our assumptions are reasonable, any of our assumptions could prove inaccurate. Considering the significant uncertainties inherent in the forward-looking statements we have made herein, which reflect our views only as of the date of this report, you should not place undue reliance upon such statements. We undertake no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

Item 1. Business

Overview

We are a commercial-stage, patient-centric biopharmaceutical company focused on in-licensing, developing, and commercializing novel high-quality medicines for patients living with rare and difficult to treat diseases. We currently market three drug products, FIRDAPSE® (amifampridine), FYCOMPA® (perampanel), and AGAMREE® (vamorolone). We are also currently seeking to further expand our drug portfolio, with a focus on acquiring the rights to late-stage products to treat orphan, rare diseases across therapeutic areas. With an unwavering patient focus embedded in everything we do, we are committed to providing innovative, best-in-class medications with the hope of making a meaningful impact on those affected by these conditions.

FIRDAPSE®

On November 28, 2018, we received approval from the FDA for our new drug application, (NDA) for FIRDAPSE® Tablets 10 mg for the treatment of adult patients (ages 17 and above) with LEMS, and in January 2019, we launched FIRDAPSE® in the U.S. Further, on September 29, 2022, the FDA approved our supplemental NDA (sNDA) to expand the indicated age range for FIRDAPSE® Tablets 10 mg for the treatment of LEMS to include pediatric patients six years of age and older. Finally, on May 30, 2024, the FDA approved our sNDA increasing the indicated maximum daily dosage of FIRDAPSE® tablets for the treatment of patients with LEMS from 80 mg to 100 mg. We believe that this recent sNDA approval offers healthcare providers and patients greater flexibility in treatment regimens for the management of LEMS.

We sell FIRDAPSE® in the U.S. through a field-based force experienced in neurologic, central nervous system or rare disease products consisting at this time of approximately 41 field personnel, including sales (Regional Account Managers), thought leader liaisons and patient assistance and insurance navigation support (Patient Access Liaisons). These field personnel have also supported AGAMREE® since its commercial launch in March 2024. We also have a field-based force of 10 medical science liaisons who are helping educate the medical community about scientific literature concerning LEMS and FIRDAPSE®. Additionally, we use non-personal promotion to reach the 20,000 neurologists who are potential LEMS treaters and the 16,000 oncologists who might be treating a LEMS patient who also has small cell lung cancer. Further, we continue to make available at no-cost a LEMS voltage gated calcium channel antibody diagnostic testing program for use by physicians who suspect that one of their patients may have LEMS and wish to reach a definitive diagnosis.

When we launched AGAMREE®, in March 2024, we utilized the FIRDAPSE® commercial and medical field-based forces to market AGAMREE® as well. In early 2025, we made a strategic decision to split each of these field-based forces into two units, one for each function expressly focused on supporting FIRDAPSE® and one for each function expressly focused on supporting AGAMREE®. This change is being made in an effort to allow us to better focus our sales teams on the market for each product. We expect to complete this division of our field-based forces into two units early in the second quarter of 2025.

Finally, we are continuing to expand our digital and social media activities to introduce our products and services to potential patients and their healthcare providers. We also work with several rare disease advocacy organizations (including the Myasthenia Gravis Foundation of America, the National Organization for Rare Disorders, and the LEMS Family Association) to help increase awareness and level of support for patients living with LEMS and to provide education for the physicians who treat these rare diseases and the patients they treat.

We are supporting the distribution of FIRDAPSE® through Catalyst Pathways®, our personalized treatment support program for patients who enroll in it. Catalyst Pathways® is a single source for personalized treatment support, education and guidance through the challenging dosing and titration regimen required to reach an effective therapeutic dose. The program also includes distributing the drug through a very small group of exclusive specialty pharmacies (primarily AnovoRx), which is consistent with the way that most drug products for ultra-orphan diseases are distributed and dispensed to patients. We believe that by

using specialty pharmacies in this way, the difficult task of navigating the health care system is far better for the patient needing treatment for their rare disease and the health care community in general.

In order to help patients with LEMS afford their medication, we, like other pharmaceutical companies which market drug products for ultra-orphan conditions, have developed an array of financial assistance programs to reduce out-of-pocket costs that makes FIRDAPSE® accessible and affordable. A co-pay assistance program has been designed to reduce commercial patients' out of pocket costs to $0 whenever possible. Our FIRDAPSE® co-pay assistance program is not available to patients enrolled in state or federal healthcare programs, including Medicare, Medicaid, Department of Veterans Affairs (VA), Department of Defense (DoD), or TRICARE. However, we are donating, and committing to continue to donate, money to qualified, independent charitable foundations dedicated to providing assistance to any U.S. LEMS patients in financial need, who meet those independent organizations' guidelines. In addition, we have a safety net program in place for patients who are uninsured and underinsured. Subject to compliance with regulatory requirements, our goal is that no LEMS patient is ever denied access to their medication for financial reasons.

FIRDAPSE® is currently marketed for the treatment of LEMS in Canada through our exclusive sublicensee, KYE. We supply product to KYE at agreed upon prices and we are also eligible to earn sales milestones and sales royalties based on net revenues from sales of the product in Canada.

In December 2023, DyDo Pharma, Inc. (DyDo), our sub-licensee for FIRDAPSE® in Japan, filed a NDA with the Pharmaceuticals and Medical Devices Agency (PMDA) in Japan seeking approval to commercialize FIRDAPSE® for the treatment of LEMS in Japan. Upon acceptance of the Japan NDA by the PMDA in December 2023, our license for FIRDAPSE® automatically expanded to include other key markets in Asia and Latin America, and we are currently seeking opportunities to expand FIRDAPSE®'s global footprint through strategic partnerships (with the current focus on the Asia Pacific and Latin American regions). Further, in September 2024, DyDo advised us that the Ministry of Health, Labour and Welfare (MHLW) had approved DyDo's Japan NDA to commercialize FIRDAPSE® for the treatment of patients with LEMS in Japan. Finally, DyDo began commercialization of FIRDAPSE® in Japan on January 21, 2025. We will generate revenue through additional milestone payments and a transfer price on the product supplied by us to DyDo, in lieu of royalties.

We control six U.S. patents for FIRDAPSE® that are listed in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (Orange Book), the earliest of which expires in 2032 and the latest of which expires in 2037. We also have orphan drug exclusivity (ODE) for the product that will not expire until November 2025, and no Abbreviated New Drug Application (ANDA) for the product can be finally approved by the FDA until the ODE exclusivity period has expired. Nevertheless, generic drug manufacturers were permitted to submit applications for the product challenging our patents starting in 2023.

In that regard, in January 2023, we received Paragraph IV Certification Notice Letters from three generic drug manufacturers (Teva Pharmaceuticals USA, Inc. and Teva Pharmaceuticals, Inc. (collectively Teva), Hetero USA, Inc. (Hetero), and Lupin Pharmaceuticals, Inc. (Lupin)) advising that they had each submitted an ANDA to the FDA seeking authorization from the FDA to manufacture, use or sell a generic version of FIRDAPSE® in the U.S. The notice letters each alleged that the six patents protecting FIRDAPSE® that are listed in the Orange Book in connection with FIRDAPSE® are not valid, not enforceable, and/or will not be infringed by the commercial manufacture, use or sale of the proposed product described in these ANDA submissions. Under the Federal Food, Drug, and Cosmetic Act (FDCA), as amended by the Drug Price Competition and Patent Term Restoration Act of 1984, we had 45 days from receipt of the notice letters to determine if there were grounds to bring a lawsuit and, if so, to commence patent infringement lawsuits against these generic drug manufacturers in a federal district court, which would trigger a statutory stay precluding the FDA from final approval of the subject ANDA until May 2026 or entry of judgment holding the patents invalid, unenforceable, or not infringed, whichever occurs first in all cases (but not earlier than the expiration of orphan drug exclusivity on November 28, 2025). In that regard, after conducting the necessary due diligence, we filed lawsuits on March 1, 2023 in the U.S. District Court for the District of New Jersey against each of the three generic drug manufacturers who notified us of their ANDA submissions, thus triggering the stay.

In June 2024, Lupin converted five of its Paragraph IV Certifications in its ANDA to Paragraph III certifications acknowledging the validity and their ANDA's infringement of five of those patents, the latest ending in 2034. We subsequently dismissed all of our claims against Lupin related to those five patents but maintain our claims against Lupin for the remaining Paragraph IV certification for U.S. Patent No. 10,626,088, which is the patent expiring in 2037, accordingly the litigation continues.

Further, on January 8, 2025, we reached a settlement with Teva in which Teva agreed not to market a generic version of FIRDAPSE® in the U.S. any earlier than February 25, 2035, if approved by the FDA, unless certain limited circumstances customarily included in these types of agreements occur. In accordance with the settlement agreement, the parties terminated all ongoing patent litigation between us and Teva regarding FIRDAPSE® patents pending in the U.S. District Court for the District of New Jersey.

The pending FIRDAPSE® patent litigation against the remaining defendants, Hetero (for FIRDAPSE®'s Orange Book-listed patents expiring in 2032, 2034 and 2037) and Lupin (only for FIRDAPSE® patent expiring in 2037), remains ongoing, and

there can be no assurance as to whether the currently ongoing litigation with Hetero and Lupin will allow a generic version of FIRDAPSE® to be marketed in the U.S. prior to Teva's licensed entry into the market on February 25, 2035.

Finally, in October 2023, we received a Paragraph IV Certification Notice Letter from a fourth generic drug manufacturer (Inventia Life Science Pty Ltd (Inventia)), and we filed a similar lawsuit against that manufacturer in November 2023 in the U.S. District Court for the District of New Jersey. On July 30, 2024, we settled this patent litigation with Inventia for FIRDAPSE®. In this settlement, Inventia acknowledged both the validity of our FIRDAPSE® patents and also the infringement by the ANDA filer's product of our patents. As part of the settlement, Inventia also agreed not to commercialize its product until the earlier of all FIRDAPSE® patents expiration or the entry into the market of another ANDA product meeting certain conditions.

The outcome of patent litigation with Paragraph IV challengers is always uncertain and there can be no assurance as to whether we will prevail in these litigations.

FYCOMPA®

On December 17, 2022, we entered into an agreement with Eisai Co., Ltd. (Eisai) for the acquisition of the U.S. rights to FYCOMPA® (perampanel) CIII. FYCOMPA® is a selective non-competitive antagonist of AMPA receptors, the major subtype of ionotropic glutamate receptors. It was the first, and still the only, drug of its class to be approved for epilepsy. Studies suggest that AMPA receptor antagonism can lead to reduced overstimulation and anticonvulsant effects, as well as inhibiting seizure generation and spread. FYCOMPA® is a controlled substance and is approved with a box warning label. FYCOMPA® is used to treat certain types of focal onset seizures (seizures that involve only one part of the brain) in adults and children four years of age and older. It is also used in combination with other medications to treat certain types of primary generalized tonic-clonic seizures (also known as a “grand mal” seizure, a seizure that involves the entire body) in adults and children 12 years of age or older. Perampanel is in a class of medications called anticonvulsants. It works by decreasing abnormal electrical activity in the brain.

On January 24, 2023, we closed our acquisition of the U.S. rights to FYCOMPA®. In connection with the acquisition, we purchased Eisai’s regulatory approvals and documentation, product records, intellectual property, inventory, and other matters relating to the U.S. rights for FYCOMPA®, in exchange for an upfront payment of $160 million in cash. We also agreed to pay Eisai royalty payments after patent protection for FYCOMPA® expires, which royalty payments will be reduced upon generic equivalents to FYCOMPA® entering the market.

In conjunction with the closing of the asset purchase, we entered into two additional agreements, a Transition Services Agreement (TSA) and a Supply Agreement. Under the Supply Agreement, Eisai agreed to manufacture FYCOMPA® for us for at least seven years at prices listed in the Supply Agreement (to be updated on a yearly basis), and under the TSA, a U.S. subsidiary of Eisai provided us with certain transitional services (which transition services ended on December 31, 2023).

We sell FYCOMPA® in the U.S. through a field-based force experienced in epilepsy products consisting at this time of approximately 27 field personnel, including sales (Regional Account Managers) and payor reimbursement (National Account Managers). We also have a field-based force of four medical science liaisons who are helping educate the medical community who treat epilepsy about scientific literature regarding epilepsy and FYCOMPA®. Further, since January 1, 2024, FYCOMPA® is being sold and distributed through a 3PL organization under our contracts.

We are currently taking steps to prepare for the loss of exclusivity for FYCOMPA®, which, assuming a timely ANDA approval of the respective generic applications, will take place on or after May 23, 2025 for the tablet version of the product and will take place on or after December 15, 2025 for the oral suspension version of the product. We expect to continue to market the product following the loss of patent exclusivity.

We are supporting patients using FYCOMPA® through an Instant Savings Card Program. Through the program, eligible commercially insured patients could pay as little as $5 for their FYCOMPA® co-pay (with a maximum savings of $2,500 per year). The FYCOMPA® Instant Savings Card Program is not available to patients enrolled in state or federal healthcare programs, including Medicare, Medicaid, VA, DoD, or TRICARE.

Patent protection for FYCOMPA® tablets and oral solution is primarily derived from two patents listed in the FDA’s Orange Book. The first, U.S. patent no. 6,949,571 (the ‘571 patent), will expire on May 23, 2025, including patent term extension. The second FYCOMPA® patent in the Orange Book is U.S. Patent No. 8,772,497 (the ‘497 patent), which will expire on July 1, 2026. The ‘497 patent, which covers the API used in both FYCOMPA® tablets and oral solution, has been the subject of previous Paragraph IV certifications from three ANDA filers for the tablet formulation, which were not contested by Eisai prior to our acquisition of the drug. Following our acquisition of the drug, we attempted to obtain an extension of the patent term for the '571 patent, which was ultimately unsuccessful. As a result, the '571 patent will expire on May 23, 2025 and the initial ANDA filers who did not challenge this patent may seek approval of their ANDA applications on or after that date.

In February 2023 we received a Paragraph IV certification for the '497 patent from an ANDA filer for two applications, one for the FYCOMPA® tablets and another for the FYCOMPA® oral suspension. After due diligence we filed lawsuits on April 5, 2023 in the U.S. District Court for the District of New Jersey against the drug manufacturer who notified us of their ANDA submissions alleging infringement of both patents. In June 2024, we settled the pending Paragraph IV litigation with the Paragraph IV filer for both ANDAs. As part of that settlement, this Paragraph IV filer agreed not to commercialize their proposed ANDA products for both the oral suspension formulation of FYCOMPA® and for FYCOMPA® tablets until at least December 15, 2025.

In January 2024, we received a Paragraph IV certification for the ’571 and ’497 patents from an ANDA filer for an application for the FYCOMPA® oral suspension. After due diligence, we determined that the circumstances and timeline did not warrant a lawsuit against this Paragraph IV filer.

AGAMREE®

On June 19, 2023, we entered into a License and Collaboration Agreement (AGAMREE® License Agreement) and an Investment Agreement (Investment Agreement) with Santhera Pharmaceuticals Holding, Inc. (Santhera). Under the AGAMREE® License Agreement, we contracted to obtain an exclusive North America license, manufacturing and supply agreement for Santhera’s investigational product candidate, AGAMREE® (vamorolone), a novel corticosteroid for the treatment of DMD. Under the Investment Agreement, we agreed to make a strategic investment into Santhera.

Both transactions closed on July 18, 2023. Under the AGAMREE® License Agreement, upon closing we made a $75 million payment to Santhera in return for the exclusive North American license for AGAMREE®. In addition to the rights to commercialize the product in North America, the AGAMREE® License Agreement provides us with the right of first negotiation for AGAMREE® in Japan should Santhera pursue partnership opportunities in that territory. Additionally, we will hold the North American rights to any future approved indications for AGAMREE®. Finally, under our AGAMREE® License Agreement with Santhera, we have agreed to purchase commercial supply of AGAMREE® from Santhera at agreed upon prices.

Concurrent with the closing of the AGAMREE® License Agreement, we made a strategic investment into Santhera in which we acquired 1,414,688 of Santhera’s ordinary shares (representing approximately 11.26% of Santhera’s outstanding ordinary shares immediately following the transaction) at an investment price of CHF 9.477 per share, with the approximately $15.7 million USD in equity investment proceeds to be used by Santhera for Phase IV studies of AGAMREE® in DMD and future development of additional indications for AGAMREE®. On February 24, 2025, the closing price of Santhera’s common shares on the SIX Swiss Exchange was CHF 16.08 per share (approximately $17.90 USD based on then-current exchange rates).

On October 26, 2023, the U.S. FDA approved Santhera’s NDA for AGAMREE® for use in treating DMD in patients aged two years and older. Shortly thereafter, as part of the previously described transaction, Santhera transferred the approved NDA to us. Additionally, following approval of the NDA for the drug, we became obligated to make a milestone payment of $36 million to Santhera, which we paid during the fourth quarter of 2023. We may also be obligated to pay future regulatory and commercial milestone payments to Santhera tied to calendar year sales of AGAMREE®, as well as commercial royalties.

On March 13, 2024, utilizing our FIRDAPSE® field-based force, we launched AGAMREE® for the treatment of DMD in the U.S. for patients aged two years or older. During the first quarter of 2024, in connection with our preparation for the commercial launch of AGAMREE®, we incurred substantial commercialization expenses, including sales, marketing, analytical infrastructure, patient services, patient advocacy, and other commercialization related expenses. Initially, we added approximately 10 additional members to our FIRDAPSE® team, and our commercial team marketed both products. However, in early 2025, we made a strategic decision to split our commercial field-based force into two units, one expressly focused on the marketing of FIRDAPSE® and one expressly focused on the marketing of AGAMREE®. This change is expected to allow us to better focus on the market for each product. We expect to complete this division of our field-based force into two units early in the second quarter of 2025.

We are further supporting the distribution of AGAMREE® through our Catalyst Pathways® patient services program to ensure that patients have access to a dedicated, personalized support team that assists families through the AGAMREE® patient journey, from answering questions to coordinating financial assistance programs for eligible patients. Finally, we have donated and intend to continue to donate funds to one or more qualified, independent charitable financial foundations who assist U.S. DMD patients in financial need for paying the costs of care including medication, to the extent permitted by each such organization's guidelines.

DMD, the most common form of muscular dystrophy, is a rare and life-threatening neuromuscular disorder characterized by progressive muscle dysfunction, ultimately leading to loss of ambulation, respiratory failure, and fatality. Current standard treatment for DMD involves corticosteroids, which often come with significant side effects. It is estimated that between 11,000 and 13,000 patients in the U.S. are affected by DMD, with approximately 70% of patients currently receiving a corticosteroid

treatment. Steroids are expected to remain the foundation of therapy for DMD patients and dosed concomitantly with other therapies.

AGAMREE®'s unique mode of action is based on differential effects on glucocorticoid and mineralocorticoid receptors and modifying further downstream activity. As such, it is considered a novel corticosteroid that we hope has the potential to demonstrate comparable efficacy to corticosteroids, with the potential for a better-tolerated side effect profile. This mechanism of action may allow vamorolone to emerge as an effective alternative to the current standard of care corticosteroids in children, adolescents, and adult patients with DMD. In that regard, we have launched our SUMMIT study to evaluate data about long-term patient safety and quality of life data from the use of our product, with the hope of offering a deeper understanding of the product's potential long-term benefits for patients.

On October 13, 2023, Santhera announced that the European Union's Committee for Medicinal Products for Human Use (CHMP) adopted a positive position in favor of AGAMREE® for the treatment of DMD patients aged four and older. In its recommendation for approval, CHMP acknowledged that there was a positive benefit-risk profile of AGAMREE® in such patient population, including certain safety benefits of AGAMREE® compared to standard of care corticosteroids in the treatment of DMD. Further, on December 18, 2023, the European Commission (EC) granted to Santhera marketing authorization for AGAMREE® for the treatment of DMD in patents ages four years and older and on January 12, 2024 Santhera announced that AGAMREE® had received approval by the Medicines and Healthcare products Regulatory Agency (MHRA) in the United Kingdom. Further, on January 15, 2024, Santhera announced that AGAMREE® was commercially launched in Germany. Finally, On January 16, 2025, the National Institute for Health and Care Excellence (NICE) issued positive Final Guidance that recommends AGAMREE® for use in the National Health Service (NHS) in England, Wales and Northern Ireland for the treatment of DMD in patients four years of age and older and on February 13, 2025, Santhera announced an agreement with the German National Association of Statutory Health Insurance Funds (GKV-SV) on the reimbursement for AGAMREE® (vamorolone) for the treatment of DMD. This milestone makes AGAMREE® the first product to receive an agreed federal price in Germany for the treatment of DMD in patients 4 years of age and older, independent of the underlying genetic mutation.

We are currently taking the first steps to seek to expand the number of diseases that can be treated with AGAMREE®. In furtherance of that objective, we are currently conducting a Phase 1 study in healthy adults comparing a single dose of vamorolone, prednisone, and deflazacort, and studying the immunosuppressive effect of multiple ascending doses of AGAMREE®, which study will attempt to define the immunosuppressive dose of vamorolone for future indications and for the use of our product in conjunction with gene and cell therapies that are approved to treat DMD and require a concurrent immunosuppressive regimen of a corticosteroid when administered. We expect to have the results of this study around the middle of this year and hope that the results of this study will provide important information for use in marketing our product to healthcare practitioners in an effort to help with the treatment of patients. We have also begun a long-term project to undertake long-term research and development efforts for the product.

Further, we have established a joint steering committee with Santhera that is overseeing the lifecycle management and development of AGAMREE®. There can be no assurance that we can develop our product for the treatment of diseases other than DMD.

In the U.S., AGAMREE® has New Chemical Entity exclusivity that expires in October 2028. AGAMREE® also has Orphan Drug Exclusivity expiring in October 2030. AGAMREE® is further protected by seven Orange Book listed patents expiring as early as May 28, 2029 and as late as July 16, 2040. The Company has also requested Patent Term Extension and will update the relevant expiration date in the Orange Book upon a final determination by the USPTO. The earliest a generic manufacturer could submit an ANDA is October 26, 2027. If we were to pursue a patent infringement action if any such ANDA challenges any of AGAMREE®'s Orange Book patents, then the automatic statutory 30-month stay would prevent FDA approval of such ANDA until April 26, 2031.

Finally, on July 23, 2024 we entered into a license, supply and commercialization agreement with KYE, which is already our sublicensee for FIRDAPSE® in Canada, granting KYE the exclusive Canadian commercial rights to market AGAMREE® in Canada for DMD and other indications. Under the agreement, KYE is responsible for obtaining regulatory approval of the product from Health Canada (of which there can be no assurance), and we will supply product to KYE. Further, KYE has advised us that they expect to file an application with Health Canada seeking approval to commercialize AGAMREE® in Canada during the first quarter of 2025.

There can be no assurance that any such application when and if filed will be approved, and even if such application is approved that KYE will be successful in commercializing AGAMREE® in Canada.

Business Development

We continue to advance our strategic initiatives and portfolio expansion efforts, focusing on broadening and diversifying our rare (orphan) neurology product portfolio with innovative therapies that address critical unmet medical needs and expanding the geographical footprint of our existing products. In that regard, we are currently exploring clinically differentiated and

adequately de-risked opportunities, with a keen focus on orphan, rare disease products across therapeutic areas. These prospects include evaluating companies with existing commercial drug products or drugs in development, for potential partnerships, licensing, geographical expansion opportunities with our existing products, and/or asset acquisitions. We continue to employ a disciplined, comprehensive, and exhaustive approach to identifying and evaluating opportunities that we believe will add significant value to our company over the near, mid, and long term. However, other than the recent sublicense agreement described above between the Company and KYE for AGAMREE® in Canada, no definitive agreements have been entered into to-date, and there can be no assurance that any of the Company's business development initiatives will be successful.

Capital Resources

At December 31, 2024, we had cash and investments of approximately $517.6 million. Based on our current financial condition, including our profitability, cash flows generated from operations and forecasts of available cash, we believe that we have sufficient funds to support our operations for at least the next 12 months. There can be no assurance that we will continue to be successful in commercializing FIRDAPSE® and AGAMREE®, that our projections about the commercialization of FYCOMPA® after the expiration of its patents will be correct, or that we will continue to be profitable and cash flow positive. See "Management's Discussion and Analysis of Financial Condition and Results of Operations - Liquidity and Capital Resources" below for further information on our liquidity and cash flow.

Our Strategy

Our goal is to implement our buy-and-build strategy, acquiring and integrating high-value, orphan designated, synergistic assets. We are dedicated to making a meaningful impact on the lives of those suffering from rare diseases, and we believe in putting patients first in everything we do. Specifically, we intend to:

- Continue to commercialize FIRDAPSE® for the treatment of LEMS and improve disease awareness. We are currently commercializing FIRDAPSE® in the U.S. and Canada. We are working to expand awareness of the disease, including to physicians treating LEMS patients who also have small-cell lung cancer, and helping health care providers and their patients understand the benefits of FIRDAPSE®. A cornerstone of our strategy is our continuing development of Catalyst Pathways®, our personalized treatment support program, and our development of the patient assistance programs that are required to further our goal that no LEMS patient be denied access to FIRDAPSE® for financial reasons within existing legal restrictions.
- Continue to commercialize AGAMREE® for the treatment of DMD and seek to develop additional indications for the product. We are currently marketing AGAMREE® in the U.S. for the treatment of DMD. We are also hoping to evaluate our product for the treatment of other diseases and in furtherance of that objective we are currently taking steps that will help us in that goal, including our current Phase 1 trial to evaluate AGAMREE® against other steroids in an effort to first evaluate the dose equivalence between AGAMREE®, prednisone and deflazacort) and the immunosuppressive dose of AGAMREE®. We are also conducting the SUMMIT study in an effort to obtain real world data about the benefits of AGAMREE® over other corticosteroids.
- Seek to expand the market for FIRDAPSE® and AGAMREE®. We are continuing our efforts to expand the footprint for FIRDAPSE® into Asia and Latin America and maintain our Right of First Negotiation with Santhera for rights to AGAMREE® in Japan.
- Seek to acquire additional products. We intend to continue our efforts to broaden and diversify our product portfolio through acquisitions of early and/or late-stage products or companies or technology platforms in orphan, rare disease therapeutic categories. To accomplish these priorities, we are continuing to employ a disciplined approach to evaluating assets and we believe that this strategic expansion will better position our company to build out a broader, more diversified portfolio of drug candidates, which should add greater value to our company over the near and long-term.

FIRDAPSE® Product Overview

FIRDAPSE® is Catalyst's registered trade name in the U.S. for amifampridine phosphate tablets. Amifampridine is the WHO (World Health Organization) registered INN (International Nonproprietary Name) and U.S. Adopted Name (USAN) for the chemical entity, 3,4-diaminopyridine, often abbreviated as 3,4-DAP or DAP. FIRDAPSE® contains the phosphate salt of amifampridine, hence the name "amifampridine phosphate." We will refer to our drug by its trade name in the U.S. (FIRDAPSE®), by the INN and USAN (amifampridine), or by the specific salt in our product (amifampridine phosphate), throughout this report.

Amifampridine has been recommended as the first-line symptomatic treatment for LEMS by the European Federation of Neurological Societies (now known as the European Academy of Neurology). In December 2009, amifampridine phosphate received marketing approval from the European Commission (with the trade name FIRDAPSE®) for the symptomatic treatment of patients with LEMS. Safety data from clinical data published over the last 30 years in patients with LEMS or other

neurological disorders treated with amifampridine show that amifampridine is well tolerated at doses up to 80 mg per day. Among the 1,279 patients or healthy subjects assessed in the literature, the most frequently reported adverse events (AEs) were perioral and peripheral paresthesias (unusual sensations like pins and needles), and gastrointestinal disorders (abdominal pain, nausea, diarrhea, and epigastralgia (pain around the upper part of the stomach)). These events were typically mild or moderate in severity, and transient, seldom requiring dose reduction or withdrawal from treatment.

Lambert-Eaton myasthenic syndrome (LEMS)

LEMS is a rare autoimmune neuromuscular disorder characterized primarily by muscle weakness of the limbs. The disease is caused by an autoimmune reaction where antibodies are formed against voltage-gated calcium channels on nerve endings, which damages the channels. These calcium channels are responsible for the transport of charged calcium atoms that activate the biochemical machinery responsible for releasing acetylcholine. Acetylcholine is the neurotransmitter responsible for causing muscles to contract and the failure to release enough of this neurotransmitter results in muscle weakness in LEMS patients. Additionally, LEMS is often associated with an underlying malignancy, most commonly small-cell lung cancer (SCLC), and in some individuals, LEMS is the first symptom of such malignancy.

LEMS generally affects individuals' extremities, especially the legs. As LEMS most affects the parts of limbs closest to the trunk, difficulties with climbing stairs or rising from a sitting position are commonly reported. Physical exercise and high temperatures tend to worsen the symptoms. Other symptoms often seen include weakness of the muscles of the mouth, throat, and eyes. Individuals affected with LEMS also may have a disruption of the autonomic nervous system, including dry mouth, constipation, blurred vision, impaired sweating, and/or hypotension.

LEMS is managed by treating the symptoms or treating the underlying autoimmune attack on voltage gated calcium channels. Unapproved treatments include steroids, azathioprine and intravenous immunoglobulin, which work by suppressing the immune system; and pyridostigmine and amifampridine, which enhance neuromuscular transmission. Plasma exchange has also been used to attempt to remove antibodies from the body. FIRDAPSE® is a symptomatic treatment and does not alter the underlying autoimmune condition. As a voltage gated potassium blocker, FIRDAPSE® prevents charged potassium atoms from leaving the nerve cells, which prolongs the period of depolarization. This allows more charged calcium atoms to enter the nerves, which enables the nerves to release acetylcholine and causes muscles to contract and to restore lost muscle strength in LEMS patients.

Based on currently available information, we estimate that there are at least 3,600 LEMS patients in the U.S., approximately half of which we believe are presently diagnosed and identified and the remainder of which we believe are undiagnosed or misdiagnosed. However, until awareness of the disease is increased, it is unlikely that the total number of LEMS patients in the U.S. can be determined with better certainty (as is typical of rare diseases), and the actual number of patients in the U.S. with LEMS may be higher or lower than our estimate.

Some of the factors that affect the addressable size of the population with a rare disease such as LEMS include the number of patients actually diagnosed with the disease, the number of patients who are misdiagnosed with other diseases, and the number of patients who are simply undiagnosed. Additionally, while there is an antibody test that positively identifies patients with LEMS which we offer at no cost to health-care providers to be used to definitively determine whether a patient has LEMS, the test is not particularly well known or utilized at this time by many neurologists. Further, many LEMS patients who have small cell lung cancer (SCLC) are not currently being treated for LEMS because many oncology medical professionals who treat SCLC patients are generally unfamiliar with how to diagnose and treat LEMS. All of these factors affect the ultimate number of patients who will benefit from treatment with FIRDAPSE®.

License Agreements for FIRDAPSE®

License Agreement with BioMarin

On October 26, 2012, we licensed the exclusive North American rights to FIRDAPSE® pursuant to a License Agreement (the License Agreement) between us and BioMarin Pharmaceutical Inc. (BioMarin). Under the License Agreement, we make the following royalty payments on our net sales of FIRDAPSE®:

- Royalties to the licensor for seven years from the first commercial sale of FIRDAPSE® equal to 7% of net sales (as defined in the License Agreement) in North America for any calendar year for sales up to $100 million, and 10% of net sales in North America in any calendar year in excess of $100 million; and
- Royalties to the third party licensor of the rights sublicensed to us for seven years from the first commercial sale of FIRDAPSE® equal to 7% of net sales (as defined in the License Agreement between BioMarin and the third party licensor) in any calendar year for the duration of any pending or issued patents or regulatory exclusivity within a territory and 3.5% of net sales in any calendar year in territories without pending or issued patents or regulatory exclusivity.

On May 29, 2019, we entered into an amendment to the License Agreement. Under the amendment, we expanded our commercial territory for FIRDAPSE®, which originally was comprised of North America, to include Japan. Additionally, in December 2023 our territory automatically expanded to include most of Asia, as well as Central and South America, upon the acceptance by the MHLW in Japan of an application to market our product in Japan. Under the amendment, we will pay royalties on net sales in Japan of a similar percentage to the royalties that we are currently paying under our original License Agreement for North America.

In January 2020, we were advised that BioMarin had sold certain rights under the License Agreement to SERB SA. We believe that we remain in compliance with our obligations under the License Agreement.

License Agreement with Jacobus

In May 2019, the FDA approved an NDA for RUZURGI®, Jacobus Pharmaceuticals' version of amifampridine (3,4-DAP), for the treatment of pediatric LEMS patients (ages 6 to under 17). In June 2019 we filed suit against the FDA and several related parties challenging this approval and related drug labeling. Jacobus later intervened in the case. We ultimately prevailed in our litigation in September 2021, when U.S. Court of Appeals for the 11th Circuit determined that the FDA's approval of RUZURGI® violated our rights to Orphan Drug Exclusivity.

On July 11, 2022, we settled our disputes with Jacobus. In connection with the settlement, we licensed the rights to develop and commercialize RUZURGI® in the U.S. and Mexico. Simultaneously, we purchased, among other intellectual property rights, Jacobus' U.S. patents related to RUZURGI®, its NDAs in the U.S. for RUZURGI®, and certain RUZURGI® inventory previously manufactured by Jacobus. At the same time, we received a license from Jacobus for use of its know-how related to the manufacture of RUZURGI®. Further, we settled our pending patent lawsuit against Jacobus, which has been dismissed without prejudice. Finally, Jacobus agreed that until the later of (i) the expiration of the royalty term or (ii) December 31, 2034, Jacobus and its affiliates, will not, directly or indirectly, research, develop, manufacture, commercialize, distribute, use or otherwise exploit any product competitive to FIRDAPSE® or RUZURGI® in the U.S. and Mexico, and Laura Jacobus, the sole shareholder of Jacobus, and two of Jacobus' other officers, also signed individual non-competition agreements containing the same terms.

In connection with the settlement with Jacobus, we paid Jacobus $30 million of cash, of which $10 million was paid at the closing of the settlement on July 11, 2022, $10 million was paid on the first anniversary of the closing of the settlement, and the remainder was paid on the second anniversary of closing. We also pay Jacobus an annual royalty on our net sales (as defined in the License and Asset Purchase Agreement between us and Jacobus) of amifampridine products in the U.S. equal to: (a) for calendar years 2022 through 2025, 1.5% (with a minimum annual royalty of $3.0 million per year), and (b) for calendar years 2026 through the expiration of the last to expire of Catalyst's FIRDAPSE® patents in the U.S., 2.5% (with a minimum annual royalty of $5 million per year); provided, however, that the royalty rate may be reduced and the minimum annual royalty may be eliminated under certain circumstances. Royalties will be trued up at the end of the year to the extent that royalties on net sales are below the minimum royalty.

Clinical Trials Supporting our NDA for FIRDAPSE® for LEMS and Approval of our NDA

We conducted two successful Phase 3 double-blind, placebo-controlled clinical trials evaluating FIRDAPSE® for the treatment of LEMS. The results of the first trial published in 2016 in Muscle & Nerve (Muscle Nerve, 2016, 53(5):717-725). The results of the second trial were published in March 2019 in the Journal of Clinical Neuromuscular Disease (J. Clin Neuromusc Dis 2019; 20:111-119). In March 2018, we submitted an NDA seeking approval of FIRDAPSE® for the treatment of LEMS. Our NDA was accepted for filing in May 2018 and, on November 28, 2018, the FDA granted approval of FIRDAPSE® for the treatment of LEMS in adult patients.

On September 29, 2022, the FDA approved our sNDA to expand the indicated age range for FIRDAPSE® for the treatment of LEMS to include pediatric patients, six years of age and older. Further, on May 30, 2024, the FDA approved our sNDA increasing the indicated maximum daily dose of FIRDAPSE® for adults and pediatric patients weighing more than 45 kg from 80 mg to 100 mg for the treatment of LEMS.

Required Post-Approval Studies

As part of the approval of our NDA for FIRDAPSE® for LEMS, the FDA required us to conduct two studies. The first was a clinical trial to evaluate the effect of hepatic impairment on the exposure of amifampridine after oral administration of FIRDAPSE® relative to that in subjects with normal hepatic function. The second was to perform a second carcinogenicity study of amifampridine phosphate in mice. Both have been completed and submitted to the FDA which considers these commitments complete. For a third commitment, we have also established a pregnancy surveillance program to collect and analyze information for a minimum of 10 years on pregnancy complications and birth outcomes related to FIRDAPSE®. Finally, in connection with the approval of our sNDA for FIRDAPSE® for the treatment of children ages six through seventeen with LEMS, we are currently conducting a pediatric safety study of juvenile toxicity in a rodent.

Compassionate Use Programs

We continue to make FIRDAPSE® available to a limited number of patients diagnosed with congenital myasthenic syndromes or Downbeat Nystagmus through investigator-sponsored compassionate use programs. Further, when we acquired the U.S. rights to RUZURGI® in July 2022, we agreed to continue to supply RUZURGI® to these patients with neuromuscular conditions other than LEMS who are without access to an approved drug and were being treated with RUZURGI® under investigator-sponsored INDs at the time of our settlement with Jacobus. However, this program has recently been discontinued because the API for RUZURGI® is no longer available and we are therefore unable to manufacture additional product.

Sales, Marketing and Distribution

Marketing of FIRDAPSE®

In January 2019, we launched FIRDAPSE® in the U.S. We currently sell FIRDAPSE® in the U.S. through a field-based force experienced in neurologic, central nervous system or rare disease products consisting at this time of approximately 40 field personnel, including sales (Regional Account Managers), thought leader liaisons and patient assistance and insurance navigation support (Patient Access Liaisons). We also have a field-based force of nine medical science liaisons who are helping educate the medical community about scientific literature concerning LEMS and FIRDAPSE®. Additionally, we use non-personal promotion to reach the 20,000 neurologists who are potential LEMS treaters and the 16,000 oncologists who might be treating a LEMS patient who also has small cell lung cancer. Further, we continue to make available at no-cost a LEMS voltage gated calcium channel antibody diagnostic testing program for use by physicians who suspect that one of their patients may have LEMS and wish to reach a definitive diagnosis.

When we launched AGAMREE®, in March 2024, we utilized the FIRDAPSE® commercial field-based force for AGAMREE® as well. In early 2025, we made a strategic decision to split our commercial field-based force into two units, one expressly focused on the marketing of FIRDAPSE® and one expressly focused on the marketing of AGAMREE®. This change will allow us to better focus on the market for each product. We expect to complete this division of our field-based force into two units by early in the second quarter of 2025.

We are supporting the distribution of FIRDAPSE® through Catalyst Pathways®, our personalized treatment support program. Catalyst Pathways® is a single source for personalized treatment support, education and guidance through the challenging dosing and titration regimen to an effective therapeutic dose. It also includes distributing the drug through a very small group of exclusive specialty pharmacies (primarily AnovoRx), which is consistent with the way that most pharmaceutical products for ultra-orphan diseases are distributed and dispensed to patients. By using specialty pharmacies in this way, the difficult task of navigating the health care system is far better for the patient needing treatment for their rare disease and the health care community in general.

In addition, Catalyst Pathways® is the gateway for our free bridge medication for patients transitioning from investigational product while they are waiting for a coverage determination or, later on, for patients whose access is threatened by the bureaucratic complications arising from a change of insurer. The Catalyst Pathways® program is also the access point for our Patient Assistance Program, which provides longer-term free medication for those who are uninsured or functionally uninsured with respect to FIRDAPSE® because they may be unable to obtain coverage from their payer despite having health insurance.

We are continuing efforts on the challenging process to identify patients and their physicians who have diagnosed LEMS, but have not had access, awareness or understanding of this treatment for their rare disease. These patients often do not see their physician frequently, have many questions about changing treatment(s), and may not perceive the need to change to a new therapy. Further, we have begun to focus our commercial efforts to locate misdiagnosed and undiagnosed LEMS patients and provide educational and sales activities to help improve the diagnosis, understanding of the treatment, and information on the prescribing process. We plan to continue to support LEMS and rare disease patient organizational groups' efforts to generate awareness and educate patients and physicians on the diagnosis of LEMS, the impact of the disease, and the support services and treatments available.

Access to FIRDAPSE®

In order to help patients afford their medication, we, like other pharmaceutical companies who are marketing drugs for ultra-orphan conditions, have developed an array of financial assistance programs that are available to reduce patient co-pays and deductibles to a nominal affordable amount. For eligible patients with commercial coverage, a co-pay assistance program designed to keep out-of-pocket costs to $10 or less per month (currently $0.00 per month) is available for all LEMS patients prescribed FIRDAPSE®. We are also donating, and committing to continue to donate, money to qualified, independent charitable foundations dedicated to providing assistance to LEMS patients in financial need. Our goal is to ensure that no LEMS patient is ever denied access to their medication for financial reasons.

To date, FIRDAPSE® has been widely covered and reimbursed by private and public payors for the indicated small population of adult and pediatric LEMS patients.

Canadian Market

We market FIRDAPSE® for the treatment of LEMS in Canada through a sublicensee, KYE. Our product was approved for commercialization in Canada in 2020.

Japanese Market

In May 2019, we entered into an amendment to our license agreement for FIRDAPSE® with BioMarin. Under the amendment, we expanded our commercial territory for FIRDAPSE®, which originally was comprised of North America, to include Japan. Further, we were granted orphan drug designation in Japan for FIRDAPSE® for the symptomatic treatment of LEMS.

On June 28, 2021, we entered into a sub-license agreement with DyDo Pharma, Inc., or DyDo, pursuant to which we sub-licensed to DyDo the Japanese rights for FIRDAPSE® for the treatment of LEMS. In December 2021, we announced that DyDo had initiated a Phase 3 registrational study in Japan to evaluate the efficacy and safety of FIRDAPSE® for the treatment of LEMS. In December 2023, DyDo submitted a Japan NDA for FIRDAPSE® to the PMDA. On September 24, 2024, DyDo informed us that the MHLW of Japan has approved their New Drug Application for FIRDAPSE® for the treatment of LEMS and DyDo began commercialization of FIRDAPSE® in Japan on January 21, 2025.

Future Markets for FIRDAPSE®

Under the amendment to our license agreement that added Japan to our territory, our territory in which we have the right to seek to commercialize FIRDAPSE® has automatically expanded to include several countries in Asia-Pacific and South and Central America, and we are working to expand our FIRDAPSE® activities into some of these.

Intellectual property and regulatory exclusivity protections for FIRDAPSE®

The bulk of our patent rights related to FIRDAPSE® are derived from our license agreement with BioMarin, which was transferred to SERB in 2020. In August 2020, the U.S. Patent and Trademark Office (USPTO) allowed Patent No. 10,793,893 (the ’893 patent) to our licensor and thereby to us, and the patent issued on October 6, 2020. The patent is directed to the use of suitable doses of amifampridine to treat patients, regardless of the therapeutic indication, that are slow metabolizers of amifampridine. Any drug product containing amifampridine with a label that states the patented dosing regimens and doses in the Dosing and Administration section prior to May 26, 2034, the expiration date of the patent, could possibly infringe this patent. Generic drug product labels would necessarily have to do this, and we intend to take all appropriate actions to protect our intellectual property.

In April 2021, the USPTO also allowed a further application in the family of licensed intellectual property, and this second patent, Patent No. 11,060,128 (the ’128 patent), issued on July 13, 2021. The patent is directed to the use of suitable doses of amifampridine to treat patients suffering with LEMS that are slow metabolizers of amifampridine. Any drug product containing amifampridine with a label for the treatment of LEMS, that states the patented dosing regimens and doses in the Dosing and Administration section of a product label, including generic drug product labels, could possibly infringe this patent prior to this patent’s expiration date.

Additional patents claiming priority from the patents noted above issued in March 2022 as Patent Nos. 11,268,128, 11,274,332, and 11,274,331, and extended our patent coverage to include fast metabolizers of amifampridine. These patents are now listed in the Orange Book for FIRDAPSE®.

As part of our transaction with Jacobus Pharmaceuticals, we also acquired two patents. One of these patents, 10,626,088 issued by the USPTO on April 21, 2020, was suitable for listing in the Orange Book and has now been listed in further support of FIRDAPSE®.

On December 19, 2023 and January 16, 2024, the USPTO issued Patent Nos. 11,845,977 and 11,873,525, respectively. These new patents cover methods of treating LEMS with FIRDAPSE® under fasting and fed conditions of dosing. We are also pursuing additional patent applications for FIRDAPSE® in an effort to further protect our drug product.

There can be no assurance that we do not or will not infringe on patents held by third parties or that third parties in the future will not claim that we have infringed on their patents. In the event that our products or technologies infringe or violate the patent or other proprietary rights of third parties, we may be prevented from pursuing product development, manufacturing or commercialization of our products that utilize such technologies. For example, there may be patents or patent applications held by others that contain claims that our products or operations might be determined to infringe or that may be broader than we believe them to be. Given the complexities and uncertainties of patent laws, there can be no assurance as to the impact that future patent claims against us may have on our business, financial condition, results of operations, or prospects.

Until FIRDAPSE® was approved in November 2018, no drug product containing amifampridine for any indication had been approved by the FDA such that we received five-year “new chemical entity” exclusivity from the FDA. New chemical entity exclusivity provides a five-year period of marketing exclusivity for all indications and in the absence of an Orange Book listed

patent, precludes a generic from submitting an ANDA until that five-year period has expired. Further, when FIRDAPSE® was approved for the treatment of LEMS patients, we received seven-year orphan drug exclusivity (ODE) for our product for the treatment of LEMS, precluding a generic filer from receiving final FDA approval until the ODE exclusivity period has expired. Because we have Orange Book listed patents for FIRDAPSE®, potential generic filers were permitted to submit ANDA filings to the FDA starting on the "NCE-1" date (November 28, 2022).

For a description of currently ongoing Paragraph IV litigation related to FIRDAPSE®, see "Item 1. Business – Overview – FIRDAPSE®." The outcome of patent litigation with Paragraph IV challengers is always uncertain and there can be no assurance to whether we will prevail in this litigation.

We have also in-licensed the FIRDAPSE® trademark, which was registered in the U.S. in March 2015.

Protection of our intellectual property and regulatory exclusivities is a strategic priority for our business. Our ability to protect and use our intellectual property rights and regulatory exclusivity in the future development and commercialization of our products, operate without infringing the proprietary rights of others, and prevent others from infringing our proprietary rights, is crucial to our future success. See Item 1A. "Risk Factors - Risks Related to Our Intellectual Property."

FYCOMPA® Product Overview

Epilepsy is a serious neurological condition that affects more than 50 million individuals globally, 80% of whom live in developing countries. An estimated 1.7% of U.S. adults have been diagnosed with the condition. From prominent historical figures to friends or family members, most people probably know someone affected by epilepsy.

The FDA approved FYCOMPA® in October 2012 as an adjunctive agent for the treatment of focal onset seizures with or without secondary generalization in patients with epilepsy at least 4 years of age. In June 2015, the agency approved a second indication for primary generalized tonic-clonic seizures in patients with epilepsy who are at least 12 years of age.

FYCOMPA® is a novel non-competitive selective antagonist at the postsynaptic ionotropic alpha-amino-3-hydroxy-5-methyl-4-isoxazolepropionic acid (AMPA) glutamate receptor. In the nervous system, glutamate is known to be a major excitatory neurotransmitter, but the exact antiepileptic mechanism of perampanel in humans is unknown. Studies suggest that AMPA receptor antagonism can lead to reduced overstimulation and anticonvulsant effects, as well as inhibiting seizure generation and spread. In addition, AMPA receptor antagonists may prevent neuronal death.

At the time of its approval, the FDA included specific significant warnings for FYCOMPA® in the prescribing information that it required to be included prominently in all communications about the product. Such warnings are known as "black box" warnings because they are traditionally surrounded by a black box to emphasize their significance. For FYCOMPA®, the warning addresses rare but serious behavioral changes that occur in some patients using FYCOMPA® including aggression (up to and including homicidal behavior), hostility, anger, distrust and other extreme behavioral changes; visual and auditory hallucinations; and difficulty with memory. In addition, FYCOMPA® was classified as a Class III controlled substance prior to its approval due to evidence of prolonged use creating a physical dependence in some patients and the possibility of abuse.

Epilepsy

Epilepsy is a long-term (chronic) disease that causes repeated seizures due to abnormal electrical signals produced by damaged brain cells. A burst of uncontrolled electrical activity within brain cells causes a seizure. Epilepsy is generally diagnosed after an individual suffers two seizures within a 24-hour period. Generally, cells in the brain send messages to and receive messages from all areas of the body. These messages are transmitted via a continuous electrical impulse that travels from cell to cell. Epilepsy disrupts this rhythmic electrical impulse pattern. Instead, there are bursts of electrical energy — like an unpredictable lightning storm — between cells in one or more areas of your brain. This electrical disruption causes changes in awareness (including loss of consciousness), sensations, emotions and muscle movements. In the U.S., about 3.4 million people have epilepsy. Of this number, approximately 3 million are adults and 470,000 are children. There are 150,000 new cases of epilepsy in the U.S. each year. Worldwide, about 65 million people have epilepsy.

Epileptic seizures (defined by two or more unprovoked seizures separated by more than 24 hours, or one unprovoked seizure with high probability of an additional seizure in the next 10 years, or as better defined by an epileptic syndrome) are separated into two broad categories: partial-onset seizures (POS) and generalized seizures, which affect one or both hemispheres of the brain, respectively. While many risk factors (e.g., infection, genetics, prenatal injury, or structural or metabolic abnormalities) have been elucidated, more than half of all cases of epilepsy are due to unknown causes. Regardless of the causative factor, epileptic seizures result from a persistent and uncontrolled increase in hypersynchronous neuronal excitability implicating various receptors (e.g., sodium, calcium, potassium, gamma-aminobutyric acid, or glutamate) involved in normal neurotransmission. Antiepileptic drugs (AEDs) target the various receptors to reduce neuronal excitability and control seizures, thus reducing the risk of seizure-related injuries and death. Although monotherapy is ideal for treating epileptic seizures, only about 49% of patients achieve seizure freedom while using their first appropriately selected AED. Subsequently, 62% to 66% of patients might only be able to achieve seizure freedom with a second or third appropriately selected AED, respectively,

leaving up to one-third of patients with inadequate control of their seizures. In addition, patients may have a higher risk of toxicity if AEDs with similar mechanisms of action are used concomitantly. In the last two decades, the number of agents commercially available in the armamentarium against epilepsy has risen fourfold, few with a novel mechanism of action like FYCOMPA®.

Access to FYCOMPA®

We are supporting patients using FYCOMPA® through an Instant Savings Card Program. Through the program, eligible commercially insured patients could pay as little as $5 for their FYCOMPA® co-pay (with a maximum savings of $2,500 per year). The FYCOMPA® instant savings card program is not available to patients enrolled in state or federal healthcare programs, including Medicare, Medigap, VA, DoD, or TRICARE.

Acquisition of U.S. rights to FYCOMPA®

On December 17, 2022, we entered into an Asset Purchase Agreement with Eisai, pursuant to which we acquired the U.S. rights to FYCOMPA®. Pursuant to the Asset Purchase Agreement entered into with Eisai for FYCOMPA®, we purchased Eisai's regulatory approvals and documentation, product records, intellectual property, inventory, and other matters relating to the U.S. rights for FYCOMPA®, in exchange for an up-front cash payment of $160 million; and royalty payments on net sales post-expiration of the patents for FYCOMPA®, which royalty payments will be reduced upon generic equivalents to FYCOMPA® entering the market. Finally, we have agreed to pay Eisai an additional cash payment of $25 million if a patent extension for FYCOMPA® was approved by the USPTO (which did not occur).
In conjunction with the Asset Purchase Agreement, at the closing of our purchase on January 24, 2023 we entered into two additional agreements with Eisai:

- A Transition Services Agreement under which a U.S. subsidiary of Eisai provided us with certain services for certain periods, including but not limited to, FDA Post-Marketing study requirements for FYCOMPA® and Transitional Services pursuant to which Eisai's U.S. subsidiary assisted us with the transition of commercial, market asset, finance, medical information, and supply issues; and
- A Supply Agreement under which Eisai has agreed to manufacture FYCOMPA® for us for at least seven years at prices to be updated on a yearly basis.

These additional agreements became effective upon the closing of the transaction with Eisai on January 24, 2023. The transition services under the Transition Services Agreement ended on December 31, 2023.

Clinical Trials Supporting FYCOMPA®

Partial Onset Seizures

The efficacy of FYCOMPA® in partial-onset seizures, with or without secondary generalization, was studied in patients who were not adequately controlled with 1 to 3 concomitant AEDs in 3 randomized, double-blind, placebo-controlled, multicenter trials (Studies 1, 2, and 3) in adult and pediatric patients (12 years of age and older). All trials had an initial 6-week Baseline Period, during which patients were required to have more than five seizures in order to be randomized. The Baseline Period was followed by a 19-week Treatment Period consisting of a 6-week Titration Phase and a 13-week Maintenance Phase. Patients in these 3 trials had a mean duration of epilepsy of approximately 21 years and a median baseline seizure frequency ranging from 9 to 14 seizures per 28 days. During the trials, more than 85% of patients were taking 2 to 3 concomitant AEDs with or without concurrent vagal nerve stimulation, and approximately 50% were on at least one AED known to induce CYP3A4, an enzyme critical to the metabolism of FYCOMPA® (i.e., carbamazepine, oxcarbazepine, or phenytoin), resulting in a significant reduction in FYCOMPA®'s serum concentration.

Each study evaluated placebo and multiple FYCOMPA® dosages. During the Titration period in all 3 trials, patients on FYCOMPA® received an initial 2 mg once daily dose, which was subsequently increased in weekly increments of 2 mg per day to the final dose. Patients experiencing intolerable adverse reactions were permitted to have their dose reduced to the previously tolerated dose.

The primary endpoint in Studies 1, 2, and 3 was the percent change in seizure frequency per 28 days during the Treatment Period as compared to the Baseline Period. The criterion for statistical significance was $p<0.05$. A statistically significant decrease in seizure rate was observed at doses of 4 to 12 mg per day. Dose response was apparent at 4 to 8 mg with little additional reduction in frequency at 12 mg per day.

Primary Generalized Tonic-Clonic Seizures

The efficacy of FYCOMPA® as adjunctive therapy in patients 12 years of age and older with idiopathic generalized epilepsy experiencing primary generalized tonic-clonic seizures was established in one multicenter, randomized, double-blind, placebo-controlled study (Study 4), conducted at 78 sites in 16 countries. Eligible patients on a stable dose of 1 to 3 AEDs experiencing at least 3 primary generalized tonic-clonic seizures during the 8-week baseline period were randomized to either FYCOMPA®

or placebo. Efficacy was analyzed in 162 patients (FYCOMPA® N=81, placebo N=81) who received medication and at least one post-treatment seizure assessment. Patients were titrated over 4 weeks up to a dose of 8 mg per day or the highest tolerated dose and treated for an additional 13 weeks on the last dose level achieved at the end of the titration period. The total treatment period was 17 weeks. Study drug was given once per day.

The primary endpoint was the percent change from baseline in primary generalized tonic-clonic seizure frequency per 28 days during the treatment period as compared to the baseline period. The criterion for statistical significance was $p<0.05$. A statistically significant decrease in seizure rate was observed with FYCOMPA® compared to placebo.

Intellectual Property Protections for FYCOMPA®

Patent protection for FYCOMPA® is primarily from two patents listed in the Orange Book. The first, U.S. patent no. 6,949,571 (the '571 patent) will expire no earlier than May 23, 2025, inclusive of patent term extension. A request for reconsideration of patent term extension to extend the period until June 8, 2026 was denied. The second FYCOMPA® patent in the Orange Book is U.S. Patent No. 8,772,497 (the '497 patent), which expires on July 1, 2026.

Expiration of Patent Protection for FYCOMPA®

With the anticipated loss of exclusivity for FYCOMPA® tablets on May 23, 2025 and for the FYCOMPA® oral suspension on December 15, 2025, we expect that one or more generic versions of each FYCOMPA® formulation will come onto the market in 2025. We are currently executing our strategy in preparation for the patent expiry of the solid dose of FYCOMPA® in the face of generic competition. The level of generic competition we will face is uncertain and we are currently exploring our options for future commercialization.

AGAMREE® Product Overview

AGAMREE® is a structurally unique steroidal anti-inflammatory drug to treat children and adults living with DMD. In clinical studies, AGAMREE® showed evidence of inhibition of pro-inflammatory NF-kB (nuclear factor kappa light chain enhancer of activated B cells) pathways, which are, a family of highly conserved transcription factors that regulate many important cellular behaviors, in particular, inflammatory responses and cellular growth. This inhibition is achieved through high-affinity binding to the glucocorticoid receptor, high-affinity antagonism for the mineralocorticoid receptor, and membrane stabilization properties. It is hoped that AGAMREE® will demonstrate similar efficacy to traditional corticosteroids with reduced negative downstream impacts and side effects.

The FDA approved AGAMREE® for the treatment of DMD in patients aged 2 and older on October 26, 2023.

Clinical Trials Supporting AGAMREE®

A randomized, double-blind, placebo and prednisone-controlled trial of vamorolone was carried out in patients with DMD. The trial met the primary (time to stand velocity after 24 weeks for vamorolone, 6 mg/kg per day vs placebo) and first 4 sequential secondary motor function endpoints. Study participants receiving vamorolone, 2 mg/kg per day, and vamorolone, 6 mg/kg per day, showed improvements in multiple functional endpoints over the 24-week treatment period as compared to placebo. The statistical thresholds for the primary outcome and first 4 secondary outcomes for vamorolone treatment were met, and vamorolone demonstrated efficacy across both dose ranges. The differences in time to stand from supine velocity (TTSTAND) were clinically meaningful. The differences in 6-minute walk test (6MWT) were also clinically meaningful.

The trial validated previous open-label findings where a more normal growth trajectory was observed over 18-month and 30-month periods in vamorolone treated boys with DMD. Furthermore, bone turnover markers supported the improved profile of vamorolone on bone health.

Access to AGAMREE®

We are supporting AGAMREE® through our Catalyst Pathways® Program, which includes a dedicated, personalized support team that assists families through the AGAMREE® treatment journey, from answering questions to coordinating financial assistance programs for eligible patients.

Acquisition of AGAMREE®

On June 19, 2023, we entered into a License and Collaboration Agreement (License Agreement) and an Investment Agreement (Investment Agreement) with Santhera. Under the License Agreement, we contracted to obtain an exclusive North America license, manufacturing and supply agreement for Santhera's investigational product candidate, AGAMREE® (vamorolone), a novel corticosteroid for the treatment of DMD. Under the Investment Agreement, we agreed to make a strategic investment into Santhera.

Both transactions closed on July 18, 2023. Under the License Agreement, upon closing, we made a $75 million payment to Santhera in return for the exclusive North American license for AGAMREE®. Additionally, following approval of the NDA for the drug, on October 26, 2023, we became obligated to make milestone payments of $36 million to Santhera, $26 million

of which will be used by Santhera to make milestone payments to third parties. This payment were made in the fourth quarter of 2023. We may also be obligated to pay future regulatory and commercial milestone payments to Santhera tied to calendar year sales of AGAMREE®, as well as commercial royalties.

In addition to the rights to commercialize the product in North America, the License Agreement provides us with the right of first negotiation for AGAMREE® in Japan should Santhera pursue partnership opportunities in that region. Additionally, we will hold the North American rights to any future approved indications for AGAMREE®

Concurrently with the closing of the License Agreement, we made a strategic investment into Santhera in which we acquired 1,414,688 of Santhera's post-reverse split ordinary shares (representing approximately 11.26% of Santhera's outstanding ordinary shares following the transaction) at an investment price of CHF 9.477 per share (corresponding to a mutually agreed volume weighted average price prior to signing), with the approximately $15.7 million investment to be used by Santhera for Phase IV studies of AGAMREE® in DMD and future development of additional indications for AGAMREE®

Intellectual Property Protections for AGAMREE®

AGAMREE® is protected by multiple patents with expiration dates from August 2028 to July 2040. The Orange Book listed patents granted by the USPTO are as follows:

U.S. Patent No.	Indication	Expiration Date
10,857,161	Non-hormonal steroid modulators of NF-kB for treatment of disease	May 28, 2029
8,334,279	Non-hormonal steroid modulators of NF-kB for treatment of disease	May 28, 2029
11,471,471	Aqueous oral pharmaceutical suspension compositions	March 17, 2040
11,382,922	Aqueous oral pharmaceutical suspension compositions	July 16, 2040
11,690,853	Non-hormonal steroid modulators of NF-κB for treatment of disease	March 7, 2033
11,833,159	Non-hormonal steroid modulators of NF-κB for treatment of disease	May 28, 2029
12,201,639	Aqueous oral pharmaceutical suspension compositions	March 17, 2040

Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a genetic disorder characterized by progressive muscle degeneration and weakness due to the alterations of a protein called dystrophin that helps keep muscle cells intact. DMD is the most common of four conditions known as dystrophinopathies, with the others being Becker Muscular Dystrophy, or BMD, a mild form of DMD; an intermediate clinical presentation between BMD and DMD, and DMD-associated dilated cardiomyopathy (heart disease), with little or no clinical skeletal, or voluntary, muscle disease.

DMD symptom onset is in early childhood, usually between the ages of 2 and 3. The disease primarily affects boys, but in rare cases can affect girls. For girls who are carriers, about 2.5 to 20 percent may have mild symptoms. In the U.S., the incidence of DMD is estimated to be about 1 in 3,600 live male births.

DMD causes muscle weakness that worsens over time, and common symptoms include:

- Progressive muscle weakness and atrophy that begins in the legs and pelvis, occurring less severely in the arms, neck, and other areas of the body.
- Calf muscle hypertrophy (increase in muscle size).
- Difficulty climbing up stairs.
- Difficulty walking that gets worse over time.
- Frequent falls.

- Waddling gait (walk).
- Toe walking.
- Fatigue.

Other common symptoms of DMD include:

- Cardiomyopathy.
- Breathing difficulties and shortness of breath.
- Cognitive impairment and learning difficulty.
- Delayed speech and language development.
- Developmental delay.
- Scoliosis.
- Short stature.

DMD is caused by a mutation in the gene that gives instructions for a protein called dystrophin. Dystrophin is a critical part of the dystrophin-glycoprotein complex (DGC), which plays an important role as a structural unit of muscle. In DMD, both dystrophin and DGC proteins are missing, which ultimately leads to the death of muscle cells. People with DMD have less than 5% of the normal quantity of dystrophin needed for healthy muscles. As people with DMD age, their muscles can't replace dead cells with new ones, and connective and adipose (fat) tissue gradually replace muscle fibers.

DMD has X-linked recessive inheritance, but about 30% of cases happen spontaneously with no family history of the condition. X-linked diseases are located on the X chromosome. Recessive inheritance means that when there are two copies of the responsible gene, both copies must have a pathogenic variant or mutation in order for a person to have the condition. Since males only have one X chromosome, any mutation on that chromosome will cause DMD.

Manufacturing and Supply

We are licensed in Florida as a virtual drug manufacturer, which means that we have no in-house manufacturing capacity and we are obligated to rely on contract manufacturers and packagers. We have no plans to build or acquire the manufacturing capability needed to manufacture any of our research materials or commercial products, and we expect that our drug products and drug substances will be prepared by contractors with suitable capabilities for these tasks and that we will enter into appropriate supply agreements with these contractors at appropriate times in the development and commercialization of our products. Because we will use contractors to manufacture and supply our products, we will be reliant on such contractors. Further, the contractors selected would have to be inspected by the FDA and found to be in substantial compliance with federal regulations in order for an application for one of our drug candidates to be approved, and there can be no assurance that the contractors we select would pass such an inspection.

FIRDAPSE®

We have entered into agreements with a supplier of the active pharmaceutical ingredient (API) contained in FIRDAPSE® for future requirements and we have contracted with third party contract manufacturers who are manufacturing FIRDAPSE® tablets for us.

FYCOMPA®

Under our Supply Agreement with Eisai, Eisai has agreed to manufacture and supply to us finished bulk FYCOMPA® tablets for us for a seven-year period that will run through at least the end of 2029. In addition, Eisai has assigned to us third party manufacturing contracts related to final packaging of bulk FYCOMPA® tablets and also the manufacture of the oral solution formulation.

AGAMREE®

Under our License and Collaboration Agreement with Santhera, we have agreed to purchase supplies of AGAMREE® from Santhera until January 1, 2026, after which we have the right, but not an obligation, to contract with outside, third party manufacturers for the manufacture and supply of AGAMREE®. In that regard, we have begun the process of preparing to add additional third party manufacturers for the manufacture and supply of AGAMREE®.

Manufacturing Changes

Any significant change that we make for any of our drug products must be approved by the FDA in an sNDA. If the manufacturing plan and data are insufficient, any sNDA we submit will not be approved. Before an sNDA can be approved,

our manufacturers must also demonstrate compliance with FDA's cGMP regulations and policies. Further, even if we receive approval of any sNDAs for our drug product(s), if our manufacturers do not follow cGMPs in the manufacture of our products, it may delay product launches or shipments and adversely affect our business.

Since we contract with third parties to manufacture our products, our contract manufacturers are required to comply with all applicable environmental laws and regulations that affect the manufacturing process. As a result, we do not believe that we will have any significant direct exposure to environmental issues.

Competition

The pharmaceutical industry is intensely competitive, and any product candidate developed or licensed by us would likely compete with currently marketed and potentially new drugs and therapies even though they are not indicated for these conditions. We also may face generic competition upon the loss of exclusivity and/or patent protection for our products. There are many pharmaceutical companies, biotechnology companies, public and private universities, government agencies and research organizations that compete with us in developing various approaches to the treatment of orphan diseases. Many of these organizations have substantially greater financial, technical, marketing and manufacturing resources than we have.

FIRDAPSE®

Before the approval of FIRDAPSE®, LEMS was generally treated with unapproved drugs and therapies including steroids, azathioprine, other immunosuppressants and intravenous immunoglobulin, which work by suppressing the immune system, and pyridostigmine. Plasma exchange has also been used in an attempt to remove antibodies from the body. Further, one other product, guanidine HCl tablets, was approved many years ago (during a period when drugs were not required to be reviewed by the FDA for both safety and effectiveness) for use in the treatment of LEMS. However, this drug has significant side effects and is not currently viewed as an effective treatment for LEMS. Notwithstanding, drugs may be prescribed by physicians for the treatment of LEMS whether or not they are considered effective.

Finally, we are aware that amifampridine has been available in the past from compounding pharmacies and may remain available, even though FIRDAPSE® has been approved and is commercially marketed in the U.S.

FYCOMPA®

FYCOMPA® is the first and only AED that targets a specific receptor in the brain called "AMPA". The receptor plays a role in allowing seizures to occur. Seizures have historically been treated with benzodiazepines such as clonazepam (Klonopin) and lorazepam (Ativan), GABA inhibitors such as gabapentin (Neurontin), phenobarbital (Luminal), and pregabalin (Lyrica), and sodium channel blockers such as carbamazepine (Tegretol) and lacosamide (Vimpat). Additionally, surgical options such as deep brain stimulation have been used in patients who have failed polypharmacy. Finally, there are multiple compounds that have been recently approved or are in late-stage development for focal epilepsy.

After the expiration of patent protection for FYCOMPA®, we expect that we will also face competition from generic manufacturers. At this time, we are not yet aware of the scope of this competition, including the identity of such manufacturers and pricing.

AGAMREE®

The market for DMD treatment is highly competitive, with multiple lines of treatment and medications, both name brand and generic. The first-line treatment has long been and continues to be corticosteroids. On February 9, 2017, the FDA approved Emflaza (deflazacort), a corticosteroid marketed by PTC Therapeutics for the treatment of DMD in patients 5 years old and older.

In addition, there are many companies which have announced plans for pre-clinical candidates and clinical development for the treatment of DMD, including gene transfer and/or gene editing therapies. One of those gene therapy treatments, Sarepta's Elevidys has been approved for the treatment of DMD and, in June 2024, received an expanded label for treatment of DMD patients over age five, even though the drug failed the primary endpoint in a Phase 3 clinical trial in that population. Since the drug was only evaluated with co-treatment with prednisone as an immunosuppressant, we expect that DMD patients transitioning to Elevidys may (in the short-term) delay initiating use of AGAMREE® while waiting for access to such gene therapy, or stop their AGAMREE® therapy during the course of their gene therapy treatment. We believe that patients are likely to return to a differentiated steroid like AGAMREE® post the immunosuppression period due to its potential superior clinical profile.

Additionally, we believe that in the long term, corticosteroids like AGAMREE®, will continue to be the foundational therapy for the treatment of DMD.

Factors affecting competition generally

In general, our ability to compete depends in large part upon:

- our ability to complete clinical development and obtain regulatory approvals for our drug candidates;
- the demonstrated efficacy, safety and reliability of our drug candidates;
- the timing and scope of regulatory approvals;
- product acceptance by physicians and other health care providers;
- the willingness of payors to reimburse for our product;
- protection of our proprietary rights and the level of generic competition;
- the speed at which we develop drug candidates;
- our ability to supply commercial quantities of a product to the market;
- our ability to obtain reimbursement from private and/or public insurance entities for product use in approved indications;
- our ability to recruit and retain skilled employees; and
- the availability of capital resources to fund our development and commercialization activities.

Business Development

Following our acquisition of the U.S. rights to FYCOMPA® and the North American rights for AGAMREE®, we are continuing to work to broaden and diversify our product portfolio through acquisitions of early and/or late-stage products or companies or technology platforms in rare disease therapeutic categories. To accomplish these priorities, we are continuing to employ a disciplined approach to evaluating assets, and we believe that this strategic expansion will better position our company long term to build out a broader more diversified portfolio of drug candidates (which should add greater value to our company over the near and long-term). In that regard, we are currently exploring several additional potential opportunities to acquire companies with commercial drug products and/or drug products in development or to in-license or acquire commercialized drug products or drug products in development. However, other than the recent License Agreement with KYE for AGAMREE®, no additional definitive agreements have been entered into to date and there can be no assurance that our efforts to continue to broaden and diversify our product portfolio will be successful.

Regulatory Matters

Government Regulation and Product Approval

Government authorities in the U.S., at the federal, state and local level, and in other countries extensively regulate, among other things, the research, development, testing, manufacture, labeling, record-keeping, promotion, storage, advertising, distribution, marketing and export and import of products such as those we are developing. Our drugs must be approved by the FDA through the NDA process before they may be legally marketed in the U.S.

In the U.S., drugs are subject to rigorous regulation by the FDA under the FDCA and implementing regulations, as well as other federal and state statutes. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local, and foreign statutes and regulations require 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 administrative or judicial sanctions. These sanctions could include the FDA's refusal to approve pending applications, license suspension or revocation, withdrawal of an approval, a clinical hold, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties or criminal prosecution. Any agency or judicial enforcement action could have a material adverse effect on us.

The process required by the FDA before a drug may be marketed in the U.S. generally involves the following: completion of pre-clinical laboratory tests, animal studies and formulation studies according to the FDA's good laboratory practice (GLP) regulations:

- submission of an investigational new drug application (IND) which must become effective before human clinical trials may begin and which must include approval by an institutional review board, or IRB, at each clinical site before the trials are initiated;
- performance of adequate and well-controlled human clinical trials to establish the safety and efficacy of the proposed drug for its intended use conducted in compliance with federal regulations and good clinical practice

(GCP), an international standard meant to protect the rights and health of patients and to define the roles of clinical trial sponsors, administrators, and monitors;

- submission to, and acceptance by, the FDA of an NDA;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the drug is produced to assess compliance with cGMP regulations to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity;
- potential FDA audit of the non-clinical and clinical trial sites that generated the data in support of the NDA; and
- FDA review and approval of the NDA.

The testing and approval process requires substantial time, effort and financial resources, and the receipt and timing of any approval is uncertain.

U.S. Drug Development Process

Once a pharmaceutical candidate is identified for development it enters the pre-clinical testing stage. Pre-clinical tests include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies. Prior to beginning human clinical trials, an IND sponsor must submit an IND to the FDA. The IND sponsor must submit the results of the pre-clinical tests, together with manufacturing information and analytical data, to the FDA as part of the IND. Some pre-clinical or non-clinical testing may continue even after the IND is submitted. In addition to including the results of the pre-clinical studies, the IND will also include a protocol detailing, among other things, the objectives of the clinical trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated, if the trial lends itself to an efficacy evaluation. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30–day time period, raises concerns or questions about the conduct of the trial. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. The FDA may, at any time, impose a clinical hold on ongoing clinical trials. If the FDA imposes a clinical hold, clinical trials cannot commence or recommence without FDA authorization and then only under terms authorized by the FDA.

Clinical trials involve the administration of the investigational new drug to healthy volunteers or patients under the supervision of one or more qualified investigators in accordance with federal regulations and GCP.

Clinical trials must be conducted under protocols detailing the objectives of the trial and the safety and effectiveness criteria to be evaluated. Each protocol must be submitted to the FDA as part of the IND. Further, an Institutional Review Board (IRB) at each institution participating in the clinical trial must review and approve each protocol before any clinical trial commences at that institution. All research subjects must provide informed consent, and informed consent information must be submitted to the IRB for approval prior to initiation of the trial. Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if adverse events or other certain types of other changes occur.

Human clinical trials are typically conducted in three phases. A fourth, or post-approval, phase may include additional clinical studies. These phases generally include the following, and may be sequential, or may overlap or be combined:

- Phase 1 clinical trials involve the initial introduction of the drug into human subjects. These studies are designed to determine the safety of usually single doses of the compound and determine any dose limiting intolerance, as well as evidence of the metabolism and pharmacokinetics of the drug in humans.
- Phase 2 clinical trials usually involve studies in a limited patient population to evaluate the safety and efficacy of the drug for specific, targeted indications, to determine dosage tolerance and optimal dosage, and to identify possible adverse effects and safety risks.
- In Phase 3, if a compound is found to be potentially effective and to have an acceptable safety profile in Phase 2 (or occasionally Phase 1) studies, the Phase 3 studies will be conducted to further confirm clinical efficacy, optimal dosage and safety within an expanded population which may involve geographically diverse clinical trial sites. Generally, but not always, two adequate and well-controlled Phase 3 clinical trials are required by the FDA for approval of an NDA.
- Phase 4 clinical trials are studies required of or agreed to by a sponsor that are conducted after the FDA has approved a product for marketing. These studies are used to gain additional experience from the treatment of patients in the intended therapeutic indication and to document a clinical benefit in the case of drugs approved under accelerated approval regulations. If the FDA approves a product while a company has ongoing clinical trials that were not necessary for approval, a company may be able to use the data from these clinical trials to meet all or part of any Phase 4 clinical trial requirement. Failure to promptly conduct Phase 4 clinical trials where necessary could result in withdrawal of approval for products approved under accelerated approval regulations.

While Phase 1, Phase 2, and Phase 3 tests are generally required for approval of an NDA, certain drugs may not require one or more steps in the process depending on other testing and the situation involved. Additionally, the FDA, an IRB, or the sponsor may stop testing at any time if results show patients being exposed to unnecessary health risks or overly dangerous side effects.

In addition, the manufacturer of an investigational drug in a Phase 2 or Phase 3 clinical trial for a serious or life-threatening disease is required to make available, such as by posting on its website, its policy on evaluating and responding to requests for expanded access to such investigational drug.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the drug and finalize a process for manufacturing the product in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the drug candidate and, among other requirements, the manufacturer must develop methods for testing the identity, strength, quality and purity of the final drug. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the drug candidate does not undergo unacceptable deterioration over its shelf life.

U.S. Review and Approval Process

FDA approval of an NDA is required before marketing of the product may begin in the U.S. The NDA must include the results of product development, pre-clinical studies and clinical studies, together with other detailed information, including information on the chemistry, manufacture and composition of the product. The FDA has 60 days from its receipt of the NDA to review the application to ensure that it is sufficiently complete for substantive review before filing it. The FDA may request additional information rather than file an NDA. In this event, the NDA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA files it. Once the submission is filed, the FDA begins an in-depth substantive review. The submission of an NDA is also subject to the payment of a substantial application fee (for FDA fiscal year 2025 this fee is $4,310,002), although a waiver of such fee may be obtained under certain limited circumstances, including when the drug that is subject of the application has received Orphan Drug Designation for the indication sought. Further, the sponsor of an approved NDA is subject to an annual program fee, which for FDA fiscal year 2025 is $403,889 per prescription drug product. User fees typically increase annually. The approval process is lengthy and difficult, and the FDA may refuse to approve an NDA if the applicable regulatory criteria are not satisfied or may require additional clinical or other data and information. Even if such data and information is submitted, the FDA may ultimately decide that the NDA does not satisfy the criteria for approval. The FDA may also refer applications for novel drug products or drug products which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee. The FDA reviews an NDA to determine, among other things, whether a product is safe and effective for its intended use. Before approving an NDA, the FDA will inspect the facility or facilities where the product is manufactured to determine whether its manufacturing is cGMP–compliant to assure and preserve the product's identity, strength, quality, purity and stability.

If the FDA's evaluation of the NDA submission or manufacturing facilities is not favorable, the FDA will issue a complete response letter. The complete response letter outlines the deficiencies in the submission and often requires additional testing or information in order for the FDA to reconsider the application. Even after submitting this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. With limited exceptions, the FDA may withhold approval of an NDA regardless of prior advice it may have provided or commitments it may have made to the sponsor.

Once an NDA is approved, changes to the conditions of approval, including additional indications, are made by the submission of a supplement to the NDA. The supplemental NDA (sNDA) must contain all of the information necessary to support the change. In the case of a new indication, that information usually consists of at least one clinical trial, and often more. Like an NDA, FDA determines whether the sNDA is sufficiently complete to permit review before it files the sNDA. FDA then reviews the sNDA. Like an NDA, FDA can either approve the sNDA or issue a complete response letter outlining the deficiencies in the sNDA.

Post-approval requirements

Once an NDA is approved, a product will be subject to certain post-approval requirements. For instance, the FDA closely regulates the post-approval marketing and promotion of drugs, including standards and regulations for direct-to-consumer advertising, off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the internet. As a condition of NDA approval, the FDA may also require a risk evaluation and mitigation strategy (REMS), to help ensure that the benefits of the drug outweigh the potential risks. REMS can include medication guides, communication plans for the healthcare professionals, and other Elements To Assure Safe Use (ETASU). ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring, and the use of patient registries. The requirement for a REMS can materially affect the potential market and profitability of the drug.

Drugs may be marketed only for the approved indications and in accordance with the provisions of the approved labeling. Changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, require submission and FDA approval of a new NDA or sNDA before the change can be implemented. An sNDA for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing NDA supplements as it does in reviewing NDAs.

Adverse event reporting and submission of periodic reports is required following FDA approval of an NDA. The FDA also may require post-marketing testing, known as Phase 4 testing, and surveillance to monitor the effects of an approved product or place conditions on an approval that could restrict the distribution or use of the product. In addition, quality control as well as drug manufacture, packaging, and labeling procedures must continue to conform to cGMPs after approval. Drug manufacturers and certain of their subcontractors are required to register their establishments with the FDA and certain state agencies and are subject to periodic unannounced inspections by the FDA during which the agency inspects manufacturing facilities to assess compliance with cGMPs. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality control to maintain compliance with cGMPs. Regulatory authorities may withdraw product approvals or request product recalls if a company fails to comply with regulatory standards, if it encounters problems following initial marketing, or if previously unrecognized problems are subsequently discovered.

Controlled Substance Regulations

A drug product approved by FDA may be subject to scheduling as a controlled substance under the Controlled Substances Act (CSA) depending on the drug's potential for abuse. Controlled substances that are pharmaceutical products are subject to a high degree of regulation under the CSA, which establishes, among other things, certain registration, manufacturing quotas, security, recordkeeping, reporting, import, export and other requirements administered by the U.S. Drug Enforcement Administration (DEA). The DEA classifies controlled substances into five schedules. Schedule I substances by definition have a high potential for abuse, have no currently "accepted medical use" in the U.S., lack accepted safety for use under medical supervision, and may not be prescribed, marketed or sold in the U.S. Pharmaceutical products approved for use in the U.S. may be listed as Schedule II, III, IV or V, with Schedule II substances considered to present the highest potential for abuse or dependence and Schedule V substances the lowest relative risk of abuse. FYCOMPA® is a Schedule III drug (DEA Controlled Substance Code 2261), which means that the DEA has determined that (i) it has a potential for abuse less than the drugs or other substances in Schedules I and II, (ii) it has a currently accepted medical use in treatment in the U.S., and (iii) abuse may lead to moderate or low physical dependence or high psychological dependence.

Schedule III drugs are subject to certain DEA import volume limits and state regulations relating to manufacturing, storage, distribution and physician prescription procedures, including limitations on prescription refills. In addition, the third parties who perform our clinical and commercial manufacturing, distribution, and dispensing for FYCOMPA® are required to maintain necessary DEA registrations and state licenses. The DEA periodically inspects facilities for compliance with its rules and regulations.

The Hatch-Waxman Amendments

Orange Book Listing

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent with claims covering the applicant's product or approved methods of using the product. Upon approval of a drug, each of the patents listed in the application for the drug are then published in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential generic competitors in support of approval of an ANDA. An ANDA provides for marketing of a drug product that has the same active ingredients in the same strengths and dosage form as the listed drug and has been shown to be bioequivalent to the listed drug. Other than the requirement for bioequivalence testing, ANDA applicants are not required to conduct, or submit results of, pre-clinical or clinical tests to prove the safety or effectiveness of their drug product. Drugs approved in this way are commonly referred to as "generic equivalents" to the listed drug and can often be substituted by pharmacists under prescriptions written for the original listed drug.

The ANDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA's Orange Book. Specifically, the applicant must certify that: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired but will expire on a particular date and approval is sought after patent expiration; or (iv) the listed patent is invalid or will not be infringed by the new product. The ANDA applicant may also elect to submit a section viii statement certifying that its proposed ANDA label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent. If the applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

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

The ANDA application also will not be approved until any applicable non-patent exclusivity listed in the Orange Book for the referenced product has expired.

Exclusivity

Upon NDA approval of a new chemical entity (NCE), which is a drug product that contains an active moiety that has never been approved by FDA in any other NDA, that drug receives five years of marketing exclusivity during which FDA cannot receive any ANDA seeking approval of a generic version of that drug. A drug may obtain a three-year period of exclusivity for a particular condition of approval, or change to a marketed product, such as a new formulation for the previously approved product, if one or more new clinical studies (other than bioavailability or bioequivalence studies) was essential to the approval of the application and was conducted/sponsored by the applicant. During this period of exclusivity, FDA cannot approve an ANDA for a generic drug that includes the change.

An ANDA may be submitted one year before NCE exclusivity expires if a Paragraph IV certification is filed. If there is no listed patent in the Orange Book, there cannot be a Paragraph IV certification, and, thus, no ANDA can be filed before the expiration of the exclusivity period.

Section 505(b)(2) NDAs

Most drug products obtain FDA marketing approval pursuant to an NDA or an ANDA. A third alternative is a special type of NDA, commonly referred to as a Section 505(b)(2), or 505(b)(2), NDA, which enables the applicant to rely, in part, on FDA's previous approval of a similar product, or published literature, in support of its application.

505(b)(2) NDAs often provide an alternate path to FDA approval for new or improved formulations or new uses of previously approved products. Section 505(b)(2) permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by, or for, the applicant and for which the applicant has not obtained a right of reference. If the Section 505(b)(2) applicant can establish that reliance on FDA's prior findings of safety and effectiveness or published literature is scientifically appropriate, it may eliminate the need to conduct certain pre-clinical or clinical studies of the new product.

The FDA may also require companies to perform additional studies or measurements to support the change from the approved product. The FDA may then approve the new product candidate for all, or some, of the label indications for which the referenced product has been approved, as well as for any new indication sought by the Section 505(b)(2) applicant.

To the extent that the Section 505(b)(2) applicant is relying on studies conducted on previously approved drug product, the Section 505(b)(2) applicant must submit patent certifications with respect to any patents for the approved product on which the application relies that are listed in the FDA's publication, Approved Drug Products with Therapeutic Equivalence Evaluations, commonly referred to as the Orange Book. Specifically, the applicant must certify for each listed patent that either: (1) the required patent information has not been filed; or (2) the listed patent has expired; or (3) the listed patent has not expired but will expire on a particular date, and approval is not sought until after patent expiration; or (4) the listed patent is invalid, unenforceable or will not be infringed by the proposed new product. A certification that the new product will not infringe the previously approved product's listed patent or that such patent is invalid or unenforceable is known as a Paragraph IV certification.

If the applicant does not challenge one or more listed patents through a Paragraph IV certification, the FDA will not approve the Section 505(b)(2) NDA application until all the listed patents claiming the referenced product have expired. Further, the FDA also will not approve, as applicable, a Section 505(b)(2) NDA application until any non-patent exclusivity has expired, for example: five-year exclusivity period for obtaining approval of an NCE; or three-year exclusivity period for an approval based on new clinical trials; or pediatric exclusivity, listed in the Orange Book for the referenced product.

A section 505(b)(2) NDA applicant must send notice of the Paragraph IV certification to the owner of the referenced NDA for the previously approved product and relevant patent holders within 20 days after the Section 505(b)(2) NDA has been accepted for filing by the FDA. If the relevant patent holder elects to initiate litigation, the Section 505(b)(2) applicant may invest a significant amount of time and expense in the development of its product, only to be subject to significant delay and patent litigation before its product may be commercialized. Alternatively, if the NDA applicant or relevant patent holder does not file

a patent infringement lawsuit within the specified 45-day period, the FDA may approve the Section 505(b)(2) application at any time.

ANDAs

Generic drugs may enter the market after the approval of an ANDA. The ANDA development process typically does not require new pre-clinical or clinical studies, but it does typically require one or more bioequivalence studies to show that the ANDA drug is bioequivalent to the previously approved brand name reference listed drug. Bioequivalence studies compare the bioavailability of the proposed drug product with that of the approved listed product containing the same active ingredient. Bioavailability is a measure of the rate and extent to which the active ingredient or active moiety is absorbed from a drug product and becomes available at the site of action. A demonstration of bioequivalence means that the rate and extent of absorption of the ANDA drug is not significantly different from the rate and extent of absorption of the brand name reference listed drug when administered at the same molar dose under similar experimental conditions.

As noted above, generic drug products are generally introduced to the marketplace at the expiration of patent protection and non-patent market exclusivity for the reference listed drug. However, if an ANDA applicant is the first ANDA applicant to submit an ANDA containing a Paragraph IV certification, that ANDA may be eligible for a period of generic marketing exclusivity on approval. This exclusivity, which under certain circumstances must be shared with other ANDA applicants with Paragraph IV certifications, lasts for 180 days, during which the FDA cannot grant final approval to other ANDA sponsors of an application for a generic equivalent to the same reference drug. Under certain circumstances, eligibility for 180-day exclusivity may be forfeited.

Various types of changes to an approved ANDA must be requested in a prior approval supplement. In addition, some changes may only be approved after new bioequivalence studies are conducted or other requirements are satisfied. In addition, the ANDA applicant must demonstrate that manufacturing procedures and operations conform to FDA cGMP requirements. Facilities, procedures, operations, and/or testing of products are subject to periodic inspection by the FDA and other authorities. In addition, the FDA conducts pre-approval and post-approval reviews and inspections to determine whether the systems and processes are in compliance with cGMP and other FDA regulations.

There are also user fees for ANDA applicants, sponsors, and manufacturers. For fiscal year 2025, the application fees are $321,920 per ANDA application and the facility fees are $231,952 per domestic finished dosage form facility, $246,952 per foreign finished dosage form facility, $41,580 per domestic active pharmaceutical ingredient facility, and $56,580 per foreign active pharmaceutical ingredient facility. In addition, there is a new annual program fee based on the size of the generic drug applicant. These user fees typically increase each fiscal year.

Other Regulatory Requirements

In addition to regulation by the FDA and certain state regulatory agencies, we are also subject to a variety of foreign regulations governing clinical trials and the marketing of other products. Outside of the U.S., our ability to market a product depends upon receiving a marketing authorization from the appropriate regulatory agencies. The requirements governing the conduct of clinical trials, marketing authorization, pricing and reimbursement vary widely from country to country. In any country, however, we will only be permitted to commercialize our products if the appropriate regulatory agency is satisfied that we have presented adequate evidence of safety, quality and efficacy. Whether or not FDA approval has been obtained, approval of a product by the comparable regulatory authorities of foreign countries must be obtained prior to the commencement of marketing of the product in those countries. The regulatory approval and oversight process in other countries includes all of the risks associated with regulation by the FDA and certain state regulatory agencies as described above.

Under the European Union regulatory system, applications for drug approval may be submitted either in a centralized or decentralized manner. Under the centralized procedure, a single application to the European Medicines Agency leads to an approval granted by the European Commission which permits marketing of the product throughout the European Union. The decentralized procedure provides for mutual recognition of nationally approved decisions and is used for products that do not comply with requirements for the centralized procedure. Under the decentralized procedure, the holders of national marketing authorization in one of the countries within the European Union may submit further applications to other countries within the European Union, who will be requested to recognize the original authorization based on an assessment report provided by the country in which marketing authorization is held.

Pharmaceutical pricing and reimbursement

In both the U.S. and foreign markets, our ability to commercialize our products successfully, and to attract commercialization partners for our products, depends in significant part on the availability of adequate financial coverage and reimbursement from third party payors, including, in the U.S., governmental payors such as Medicare and Medicaid, managed care organizations, private commercial health insurers and PBMs. Third party payors are increasingly challenging the prices charged for medicines and examining their cost effectiveness, in addition to their safety and efficacy. We may need to conduct expensive pharmacoeconomic or other studies in order to further demonstrate the value of our products. Even with the availability of such

studies, our products may be considered less safe, less effective or less cost-effective than alternative products, and third party payors may not provide coverage and reimbursement for our drug candidates, in whole or in part.

Political, economic and regulatory influences are subjecting the health care industry in the U.S. to fundamental changes. There have been, and we expect there will continue to be, legislative and regulatory proposals to change the healthcare system in ways that could significantly affect our business, including the Patient Protection and Affordable Care Act of 2010 (the Affordable Care Act) and the Inflation Reduction Act of 2022 (IRA).

We anticipate that in the U.S., Congress, state legislatures, and private sector entities will continue to consider and may adopt healthcare policies intended to curb rising healthcare costs. These cost-containment measures could include:

- public transparency on qualifying price increases and/or discounting to better inform purchasers;
- additional controls on government-funded reimbursement for drugs;
- controls on healthcare providers;
- challenges to the pricing of drugs or limits or prohibitions on reimbursement for specific products through other means;
- reform of drug importation laws;
- entering into contractual agreements with payors; and
- expansion of use of managed-care systems in which healthcare providers contract to provide comprehensive healthcare for a fixed cost per person.

We are unable to predict what additional legislation, regulations or policies, if any, relating to the healthcare industry or third party coverage and reimbursement may be enacted in the future or what effect such legislation, regulations or policies would have on our business. Any cost containment measures, including those listed above, or other healthcare system reforms that are adopted may have a material adverse effect on our business prospects.

Further, the pricing of pharmaceutical products generally, and particularly the pricing of orphan drugs, has received scrutiny from the press, and from members of Congress in both parties. Some members of the medical community and some politicians have also made statements in the press on the potential pricing of orphan drugs generally and on the pricing of our product specifically. The impact of this scrutiny on us and on the pricing of orphan drugs and other pharmaceutical products generally cannot be determined at this time.

Third Party Reimbursement in the U.S.

Sales of pharmaceutical products depend in significant part on the availability of coverage and adequate reimbursement by third party payors, such as state and federal governments, including Medicare and Medicaid, managed care providers, private commercial insurance plans and pharmacy benefit management (PBM) plans. Decisions regarding the extent of coverage and the amount of reimbursement are expected to be made on a plan-by-plan, and in some cases, on a patient-by-patient basis. Particularly given the rarity of LEMS and DMD, our experience has been that securing coverage and appropriate reimbursement from third party payors requires targeted education and highly skilled insurance navigation experts that have experience with rare disease launches and medical exception processes at insurance companies to provide patient coverage for important rare disease therapies. To that end, we have engaged a dedicated team of field-based market access account managers and reimbursement experts as well as a patient service center staffed with experienced personnel focused on ensuring that clinically-qualified patients have access to our products.

There can be no assurance, however, as to whether payors will continue to cover our products, and if so, at what level of reimbursement. In that regard, we have advised payors that we will provide free medication to support titration and confirm patient therapeutic benefit. Further, when necessary, we provide patients with access to therapy at no charge while those patients are awaiting coverage decisions.

Orphan Drug Exclusivity

Some jurisdictions, including the U.S. and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983 (ODA), the FDA may grant Orphan Drug Designation to drugs intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the U.S., or more than 200,000 individuals in the U.S. and for which there is no reasonable expectation that the cost of developing and making available in the U.S. a drug for this type of disease or condition will be recovered from sales in the U.S. for that drug. In the U.S., Orphan Drug Designation must be requested before submitting an application for marketing approval. An Orphan Drug Designation does not shorten the duration of the regulatory review and approval process. The grant of an Orphan Drug Designation request does not alter the standard regulatory requirements and process for obtaining marketing approval. Safety and efficacy of a compound must be established through adequate and well-controlled studies. If a product which has been granted Orphan Drug Designation

subsequently receives the first FDA approval for the indication for which it has such designation, the product is entitled to an orphan drug exclusivity period, which means the FDA may not approve any other application to market the same drug for the same disease or condition for a period of seven years, except in limited circumstances, such as where an alternative product demonstrates clinical superiority to the product with orphan exclusivity. In addition, holders of exclusivity for orphan drugs are expected to assure the availability of sufficient quantities of their orphan drugs to meet the needs of patients. Failure to do so could result in the withdrawal of marketing exclusivity for the drug.

The orphan drug exclusivity contained in the ODA has been the subject of recent scrutiny from the press, from some members of Congress and from some in the medical community. There can be no assurance that the exclusivity granted in the ODA to orphan drugs approved by the FDA will not be modified in the future, and as to how any such change might affect our products, if approved.

The European Orphan Drug Regulation is considered for drugs intended to diagnose, prevent or treat a life-threatening or very serious condition afflicting five or fewer per 10,000 people in the EU, including compounds that for serious and chronic conditions would likely not be marketed without incentives due to low market return on the sponsor's development investment. The medicinal product considered should be of significant benefit to those affected by the condition. Benefits of being granted Orphan Medicinal Product Designation are significant, including eight years of data exclusivity, two years of marketing exclusivity and a potential one-year extension of both. The EU Community and Member States may not accept or grant for ten years a new marketing authorization or application for another drug for the same therapeutic indication as the orphan drug, although the ten-year period can be reduced to six years if, after the end of the fifth year, available evidence establishes that the product is sufficiently profitable not to justify maintenance of the marketing exclusivity. A supplementary protection certificate may extend the protection six months beyond patent expiration if that is later than the orphan drug exclusivity period. To apply for the supplementary protection, a pediatric investigation plan, or PIP, must be included in the market application. In Europe all drugs now seeking marketing authorization need to have a PIP agreed with the European Medicines Agency (EMA) before it can be approved, even if it is a drug being developed specifically for a pediatric indication. If a product is developed solely for use in the pediatric population, then a Pediatric Use Marketing Authorization, or PUMA, may provide eight years of data exclusivity and ten years of marketing exclusivity.

Breakthrough Therapy Designation

Breakthrough therapy designation is intended to expedite the development and review of drugs for serious or life-threatening conditions. The criteria for breakthrough therapy designation require preliminary clinical evidence that demonstrates the drug may have substantial improvement on at least one clinically significant endpoint over available therapy. A breakthrough therapy designation conveys all of the fast track program features (see below for more details on fast track designation), as well as more intensive FDA guidance on an efficient drug development program. The FDA also has an organizational commitment to involve senior management in such guidance. Actions taken to expedite development may include the following actions, as appropriate holding meetings with the sponsor and review team throughout the development of the drug;

- providing timely advice to, and interactive communication with, the sponsor regarding the development of the drug to ensure that the development program to gather the non-clinical and clinical data necessary for approval is as efficient as possible;
- taking steps to ensure that the design of the clinical trials is as efficient as practicable, when scientifically appropriate, such as by minimizing the number of patients exposed to a potentially less efficacious treatment;
- assigning a cross-disciplinary project lead for the FDA review team to facilitate an efficient review of the development program and to serve as a scientific liaison between the cross-discipline members of the review team (i.e., clinical, pharmacology-toxicology, chemistry, manufacturing and control (CMC), compliance) for coordinated internal interactions and communications with the sponsor through the review division's Regulatory Health Project Manager; and
- involving senior managers and experienced review staff, as appropriate, in a collaborative, cross-disciplinary review.

Fast Track Designation and Accelerated Approval

FDA is required to facilitate the development, and expedite the review, of drugs that are intended for the treatment of a serious or life-threatening disease or condition for which there is no effective treatment and which demonstrate the potential to address unmet medical needs for the condition. Under the fast track program, the sponsor of a new drug candidate may request that FDA designate the drug candidate for a specific indication as a fast track drug concurrent with, or after, the filing of the IND for the drug candidate. FDA must determine if the drug candidate qualifies for fast track designation within 60 days of receipt of the sponsor's request.

Under the fast track program and FDA's accelerated approval regulations, FDA may approve a drug for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments.

In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions, or survives. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A drug candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, will allow FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by FDA. The Food and Drug Omnibus Reform Act (FDORA) included provisions related to the accelerated approval pathway. Pursuant to FDORA, the FDA is authorized to require a post-approval study to be underway prior to approval or within a specified time period following approval. FDORA also requires the FDA to specify conditions of any required post-approval study, which may include milestones such as a target date of study completion and requires sponsors to submit progress reports for required post-approval studies and any conditions required by the FDA not later than 180 days following approval and not less frequently than every 180 days thereafter until completion or termination of the study. FDORA enables the FDA to initiate enforcement action for the failure to conduct with due diligence a required post-approval study, including a failure to meet any required conditions specified by the FDA or to submit timely reports.

In addition to other benefits such as the ability to use surrogate endpoints and engage in more frequent interactions with FDA, FDA may initiate review of sections of a fast track drug's NDA before the application is complete. This rolling review is available if the applicant provides, and FDA approves, a schedule for the submission of the remaining information and the applicant pays applicable user fees. However, FDA's time period goal for reviewing an application does not begin until the last section of the NDA is submitted. Additionally, the fast track designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

Priority Review

Under FDA policies, a drug candidate is eligible for priority review, or review within a six to eight-month time frame from the time a complete NDA is submitted, if the drug candidate is intended for the treatment, diagnosis, or prevention of a serious or life-threatening condition, demonstrates the potential to address an unmet medical need, or provides a significant improvement compared to marketed drugs.

Disclosure of Clinical Trial Information

Sponsors of clinical trials of FDA-regulated products, including drugs, are required to register and disclose certain clinical trial information. Information related to the product, patient population, phase of investigation, study sites and investigators, and other aspects of the clinical trial is then made public as part of the registration. Sponsors are also obligated to disclose the results of their clinical trials after completion. Disclosure of results of these trials can be delayed in certain circumstances for up to two years after the date of completion of the clinical trial. Competitors may use this publicly available information to gain knowledge regarding the progress of development programs.

Anti-Kickback, False Claims Laws & the Prescription Drug Marketing Act

In addition to FDA restrictions on marketing of drug products, other state and federal laws have been applied to restrict certain marketing practices in the pharmaceutical industry in recent years. These laws include anti-kickback statutes and false claims statutes. The federal healthcare program anti-kickback statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and patients, prescribers, purchasers and formulary managers on the other. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties, and exclusion from participation in federal healthcare programs. Although there are a number of statutory exemptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor.

Federal false claims laws prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. Recently,

several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly inflating drug prices they report to pricing services, which in turn were used by the government to set Medicare and Medicaid reimbursement rates, and for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws. The majority of states also have statutes or regulations similar to the federal anti-kickback law and false claims laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payer.

The Centers for Medicare & Medicaid Services (CMS) has issued a final rule that requires manufacturers of approved prescription drugs to collect and report information on payments or transfers of value to physicians, physician assistants, certain types of advanced practice nurses and teaching hospitals, as well as investment interests held by physicians and their immediate family members. The information reported each year is made publicly available on a searchable website. Failure to submit required information may result in civil monetary penalties.

In addition, several states now require prescription drug companies to report expenses relating to the marketing and promotion of drug products, to report gifts and payments to individual physicians in these states and to report certain pricing information, including price increases. Other states prohibit various other marketing-related activities. Still other states require the posting of information relating to clinical studies and their outcomes. In addition, California, Connecticut, Nevada, and Massachusetts require pharmaceutical companies to implement compliance programs and/or marketing codes. Several additional states are considering similar proposals. Compliance with these laws is difficult and time consuming, and companies that do not comply with these state laws face civil penalties.

Prescription drug advertising is subject to federal, state and foreign regulations. In the U.S., the FDA regulates prescription drug promotion, including direct-to-consumer advertising. Prescription drug promotional materials must be submitted to the FDA in conjunction with their first use. Any distribution of prescription drug products and pharmaceutical samples must comply with the U.S. Prescription Drug Marketing Act (PDMA), a part of the FDCA. In addition, Title II of the Federal Drug Quality and Security Act of 2013, known as the Drug Supply Chain Security Act (DSCSA), has imposed "track and trace" requirements on the distribution of prescription drug products by manufacturers, distributors, and other entities in the drug supply chain. The DSCSA requires product identifiers (i.e., serialization) on prescription drug products in order to eventually establish an electronic interoperable prescription product to system to identify and trace certain prescription drugs distributed in the U.S. and preempts existing state drug pedigree laws and regulations on this topic. The DSCSA also establishes new requirements for the licensing of wholesale distributors and third party logistic providers, although FDA regulations addressing wholesale distributors and third party logistics providers have not yet been promulgated. We serialize our product at both the package and homogeneous case level, pass serialization and required transaction information to our customers, and believe that we comply with all such requirements.

Government Programs for Marketed Drugs

Medicaid, the 340B Drug Pricing Program, and Medicare

Federal law requires that a pharmaceutical manufacturer, as a condition of having its products receive federal reimbursement under Medicaid and Medicare Part B, must pay rebates to state Medicaid programs for all units of its covered outpatient drugs dispensed to Medicaid beneficiaries and paid for by a state Medicaid program under either a fee-for-service arrangement or through a managed care organization. This federal requirement is effectuated through a Medicaid drug rebate agreement between the manufacturer and the Secretary of Health and Human Services (HHS). CMS administers the Medicaid drug rebate agreements, which provide, among other things, that the drug manufacturer will pay rebates to each state Medicaid agency on a quarterly basis and report certain price information on a monthly and quarterly basis. The rebates are based on prices reported to CMS by manufacturers for their covered outpatient drugs. For innovator products, that is, drugs that are marketed under approved NDAs, the basic rebate amount is generally the greater of 23.1% of the average manufacturer price (AMP) for the quarter or the difference between such AMP and the best price for that same quarter. The AMP is the weighted average of prices paid to the manufacturer (1) directly by retail community pharmacies and (2) by wholesalers for drugs distributed to retail community pharmacies. The best price is essentially the lowest price available to non-governmental entities. Innovator products are also subject to an additional inflation rebate that is based on the amount, if any, by which the product's current AMP has increased over the baseline AMP, which is the AMP for the first full quarter after launch, adjusted for inflation. To date, the rebate amount for a drug has been capped at 100% of the AMP; however, effective January 1, 2024, this cap was eliminated, which means that a manufacturer could pay a rebate amount on a unit of the drug that is greater than the average price the manufacturer receives for the drug. For non-innovator products, generally generic drugs marketed under approved ANDAs, the basic rebate amount is 13% of the AMP for the quarter. Non-innovator products are also subject to an additional inflation rebate. The additional rebate is similar to that discussed above for innovator products, except that the baseline AMP quarter is the fifth full quarter after launch (for non- innovator multiple source drugs launched on April 1, 2013 or later) or the third quarter of 2014 (for those launched before April 1, 2013). The terms of participation in the Medicaid drug rebate program impose an obligation to correct the prices reported in previous quarters, as may be necessary. Any such corrections could result in additional or lesser rebate liability, depending on the direction of the correction. In addition to retroactive rebates, if a

manufacturer were found to have knowingly submitted false information to the government, federal law provides for civil monetary penalties for failing to provide required information, late submission of required information, and false information. CMS may also impose penalties against a manufacturer that is notified by CMS about a "misclassification", defined as an incorrectly reported drug attribute (for example, an erroneous classification of non-innovator rather than innovator), and fails to correct the error within 30 days. The penalties include monetary penalties and/or suspension of the drug from Medicaid coverage until the error is corrected. CMS may also suspend a manufacturer's Medicaid rebate agreement if a manufacturer fails to submit the required pricing reports or fails to correct a misclassification within 90 calendar days after receiving notice from CMS.

A manufacturer must also participate in a federal program known as the 340B drug pricing program in order for federal funds to be available to pay for the manufacturer's drugs under Medicaid and Medicare Part B. Under this program, the participating manufacturer agrees to charge certain federally funded clinics and safety net hospitals no more than an established discounted price for its covered outpatient drugs. The formula for determining the discounted price is defined by statute and is based on the AMP and the unit rebate amount as calculated under the Medicaid drug rebate program, discussed above. Manufacturers are required to report pricing information to the Health Resources and Services Administration (HRSA) on a quarterly basis. HRSA has also issued regulations relating to the calculation of the ceiling price as well as imposition of civil monetary penalties for each instance of knowingly and intentionally overcharging a 340B covered entity.

Federal law also requires that manufacturers report data on a quarterly basis to CMS regarding the pricing of drugs that are separately reimbursable under Medicare Part B. These are generally drugs, such as injectable products, that are administered "incident to" a physician service and are not generally self-administered. The pricing information submitted by manufacturers is the basis for reimbursement to physicians and hospital outpatient departments for drugs covered under Medicare Part B. Under the IRA, manufacturers are also required to provide quarterly rebates for certain single-source drugs and biologics (including biosimilars) covered under Medicare Part B with prices that increase faster than the rate of inflation, and in November 2024, CMS finalized regulations pertaining to the Medicare Part B inflation rebates. As with the Medicaid drug rebate program, federal law provides for civil monetary penalties for failing to provide required information, late submission of required information, and false information.

Medicare Part D provides prescription drug benefits for individuals at least 65 years of age and certain people with disabilities. Enrollees in Part D plans, after they meet a deductible, pay a co-insurance of 25% until they reach the out-of-pocket limit, after which they have no co-insurance. The out-of-pocket limit is $2,000 in 2025 and is to be adjusted for inflation thereafter. Each manufacturer of a drug approved under an NDA. in order for the drugs to be reimbursed by Medicare Part D, is required to enter into a manufacturer discount agreement with HHS and provide a discount on those drugs dispensed to Medicare enrollees. The discount is 10% of Part D enrollees' prescription costs for brand drugs above the deductible and below the out-of-pocket limit, and 20% once the out-of-pocket limit has been reached. The IRA also requires manufacturers to provide annual Medicare Part D rebates for single-source drugs and biological products with prices that increase faster than the rate of inflation, and in November 2024, CMS finalized regulations pertaining to Medicare Part D inflation rebates.

The IRA also allows HHS to negotiate the selling price of certain drugs and biologics that CMS reimburses under Medicare Part B and Part D. The negotiated price may not exceed a statutory ceiling price. Only high-expenditure single-source drugs (excluding drugs and biologics that are designated and approved for only one rare disease or condition) that have been approved for at least 7 years (11 years for biologics) can be selected by CMS for negotiation, with the negotiated price taking effect two years after the selection year. For 2026, the first year in which negotiated prices become effective, CMS selected 10 high-cost Medicare Part D products in 2023, negotiations began in 2024, and the negotiated maximum fair price for each product has been announced. CMS has selected 15 additional Medicare Part D drugs for negotiated maximum fair pricing in 2027. For 2028, an additional 15 drugs, which may be covered under either Medicare Part B or Part D, will be selected, and for 2029 and subsequent years, 20 Part B or Part D drugs will be selected.

Federal Contracting/Pricing Requirements

Manufacturers are also required to make their covered drugs, which are generally drugs approved under NDAs, available to authorized users of the Federal Supply Schedule (FSS), which is administered by the VA. The law also requires manufacturers to offer deeply discounted FSS contract pricing for purchases of their covered drugs by the VA, the DoD, the Coast Guard, and the Public Health Service (including the Indian Health Service) in order for the manufacturer's covered drugs to be eligible for purchase by these agencies and also in order for federal funding to be available for reimbursement of the manufacturer's drugs under Medicaid and Medicare Part B. FSS pricing to those four federal agencies for covered drugs must be no more than the Federal Ceiling Price (FCP), which is at least 24% below the Non-Federal Average Manufacturer Price (Non-FAMP) for the prior government fiscal year. The Non-FAMP is the average price for covered drugs sold to wholesalers or other middlemen, net of any price reductions.

The accuracy of a manufacturer's reported Non-FAMPs, FCPs, or FSS contract prices may be audited by the government. Among the remedies available to the government for inaccuracies is recoupment of any overcharges to the four specified federal

agencies based on those inaccuracies. If a manufacturer were found to have knowingly reported false prices, in addition to other penalties available to the government, the law provides for civil monetary penalties of $100,000 per incorrect item.

Finally, manufacturers are required to disclose in FSS contract proposals all commercial pricing that is equal to or less than the proposed FSS pricing, and subsequent to award of an FSS contract, manufacturers are required to monitor certain commercial price reductions and extend commensurate price reductions to the government, under the terms of the FSS contract Price Reductions Clause. Among the remedies available to the government for any failure to properly disclose commercial pricing and/or to extend FSS contract price reductions is recoupment of any FSS overcharges that may result from such omissions, and civil penalties under the Federal False Claims Act if such failures are knowing.

Tricare Retail Pharmacy Network Program

The DoD provides pharmacy benefits to current and retired military service members and their families through the Tricare healthcare program. When a Tricare beneficiary obtains a prescription drug through a retail pharmacy, the DoD reimburses the pharmacy at the retail price for the drug rather than procuring it from the manufacturer at the discounted FCP discussed above. In order for the DoD to realize discounted prices for covered drugs (generally drugs approved under NDAs), federal law requires manufacturers to pay refunds on utilization of their covered drugs sold to Tricare beneficiaries through retail pharmacies in DoD's Tricare network. These refunds are generally the difference between the Non-FAMP and the FCP and are due on a quarterly basis. Absent an agreement from the manufacturer to provide such refunds, DoD will designate the manufacturer's products as Tier 3 (non-formulary) and require that beneficiaries obtain prior authorization in order for the products to be dispensed at a Tricare retail network pharmacy. However, refunds are due whether or not the manufacturer has entered into such an agreement.

Branded Pharmaceutical Fee

A branded pharmaceutical fee is imposed on manufacturers and importers of branded prescription drugs, generally drugs approved under NDAs. In each year between 2011 and 2018, the aggregate fee for all such manufacturers ranged from $2.5 billion to $4.1 billion, and has remained at $2.8 billion in 2019 and subsequent years. This annual fee is apportioned among the participating companies based on each company's sales of qualifying products to or utilization by certain U.S. government programs during the preceding calendar year. The fee is not deductible for U.S. federal income tax purposes. Utilization of generic drugs, generally drugs approved under ANDAs, is not included in a manufacturer's sales used to calculate its portion of the fee. Further, orphan drugs with only one indication are generally exempt from this fee.

Human Capital Management

We are dedicated to making a meaningful impact on the lives of those suffering from rare diseases, and we believe in putting patients first in everything we do. To facilitate talent attraction and retention, we strive to make Catalyst an inclusive, safe, and healthy workplace, with opportunities to grow and develop in their careers, supported by strong compensation, benefits, health and welfare programs. Our goal in selecting employees is to retain high quality personnel with substantial prior experience who understand and support our mission as a company to develop and commercialize innovative therapies for people with rare, debilitating, chronic neuromuscular and neurological diseases and who are willing to work hard and in a collaborative manner to further that mission.

On February 13, 2025, Gregg Russo was promoted from VP, Head of Human Resources to Chief Human Resources Officer.

Employee Profile

As of February 24, 2025, we had 181 employees, 127 of whom are in our commercial organization, 11 of whom are in our R&D organization, and the rest of whom are in our G&A organization. We also utilize the services of several full-time consultants who primarily work with our commercial organization. None of our employees are covered by a collective bargaining agreement. We believe our relationship with our employees and consultants is good.

Compensation and Benefits

Our compensation philosophy is to provide pay and benefits that are competitive in the biotechnology and pharmaceutical industry where we compete for talent. We monitor our compensation programs closely and review them at least annually to provide what we consider to be a very competitive mix of compensation and health, welfare and retirement benefits for all our employees. Our compensation package includes market-competitive base salaries, annual performance bonuses and stock option grants. Our benefits programs include company sponsored medical, dental and vision health care coverage, life and AD&D insurance, and a 401(k) plan with a matching employer contribution, among others benefits.

Our Workforce Goals

We believe a diverse workforce is critical to our success and we are fundamentally committed to creating and maintaining a work environment in which employees are treated fairly, with dignity, decency, respect and in accordance with all applicable laws. We strive to create a professional work environment that is free from all forms of harassment, discrimination and bullying

in the workplace, including sexual harassment and any form of retaliation. We are an equal opportunity employer and we strive to administer all human resources actions and policies without regard to race, color, religion, sex, national origin, ethnicity, age, disability, sexual orientation, gender identification or expression, past or present military or veteran status, marital status, familial status, or any other status protected by applicable law. Our management team and employees are expected to exhibit and promote honest, ethical, and respectful conduct in the workplace. All employees must adhere to a code of business conduct and ethics and our employee handbook, which combined, define standards for appropriate behavior and are annually trained to help prevent, identify, report, and stop any type of discrimination and harassment. Our recruitment, hiring, development, training, compensation, and advancement is based on qualifications, performance, skills, and experience without regard to gender, race, or ethnicity.

Communication and Engagement

We focus on engagement with our employees as we believe an engaged workforce is key to our success and to the success and wellbeing of our employees. In addition, we are always looking for new and different ways to engage our staff further as a team and individually.

Health, Wellness and Safety

The health, safety, and wellness of our employees is a priority in which we have always invested and will continue to do so. We provide our employees and their families with access to a variety of innovative, flexible, and convenient health and wellness programs. Program benefits are intended to provide protection and security, so employees can have peace of mind concerning events that may require time away from work or that may impact their financial well-being.

Company Culture

We are committed to instilling a company culture that is focused on integrity, transparency, quality and respect. We expect our employees to observe the highest levels of business ethics, integrity, mutual respect, tolerance and inclusivity. Our Code of Business Conduct and Ethics sets forth policies reflecting these values and provide direction for registering complaints in the event of any violation of our policies. We maintain an "open door" policy at all levels of our organization and any form of retaliation against an employee is strictly prohibited.

Available Information

We make available free of charge on or through our website our Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and all amendments to those reports as soon as reasonably practicable after such material is electronically filed with or furnished to the Securities and Exchange Commission (SEC). Our Internet address is www.catalystpharma.com. The content on our website is not, nor should it be deemed to be, incorporated by reference into this report.

Item 1A. Risk Factors

Risk Factors Summary

We are providing the following summary of the risk factors contained in our Form 10-K to enhance the readability and accessibility of our risk factor disclosures. We encourage our stockholders to carefully review the full risk factors contained in this Form 10-K in their entirety for additional information regarding the risks and uncertainties that could cause our actual results to vary materially from our recent results or from our anticipated future results.

Risks Related to the Marketing of Approved Products

- Our success depends on the successful commercialization of our products. To the extent that our drug products are not commercially successful, our business, financial condition and results of operations will be materially harmed.
- Our drug products may fail to receive the degree of market acceptance by physicians, patients, third party payers or others in the medical community necessary for commercial success, which would negatively impact our business.
- Our strategy of seeking to acquire or in-license innovative technical platforms or earlier stage drug development programs outside of the neuromuscular disease space may not be successful.
- Because the target patient populations for FIRDAPSE® and AGAMREE® are small, we must achieve significant market share and obtain relatively high per-patient prices for our products to achieve meaningful gross margins.
- Because of risks associated with taking FYCOMPA®, potential patients may be reluctant to start treatment with FYCOMPA® or may discontinue use.

Risks Related to the Development of Additional Drug Products and Indications

- Failure can occur at any stage of our drug development efforts.
- We rely on third parties to conduct our pre-clinical studies and clinical studies and trials, and if they do not perform their obligations to us we may not be able to obtain approval for additional indications.
- We will need to continue to develop and maintain distribution and production capabilities or relationships to be successful.
- We could be impacted by the viability of our suppliers.
- We are dependent on our licensing partners for supplies of FYCOMPA® and AGAMREE®.
- We may encounter difficulties in managing our growth, which would adversely affect our results of operations.
- Pressure on drug product third party payor coverage, reimbursement and pricing may impair our ability to be reimbursed at prices or on terms sufficient to provide a viable financial outcome.
- Our internal computer systems, or those of our contract research organizations and other key vendors or consultants, may fail or suffer security breaches, which could result in a material disruption of our business and/or our product development programs.
- Our employees, sales agents and consultants may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.
- We may face significant delays in our clinical studies and trials due to an inability to recruit patients for our clinical studies and trials or to retain patients in the clinical studies and trials we may perform.

Risks Related to Government Regulation

- The healthcare industry is highly regulated, subject to stringent regulatory standards and other applicable laws, and we may be the subject of unexpected changes in interpretation or enforcement, any of which may adversely impact our business.
- The regulatory approval process is lengthy, and we may not be able to obtain all of the regulatory approvals required to manufacture and commercialize our drug products in which we are licensed to them.
- If our pre-clinical studies or our clinical studies and trials are unsuccessful or significantly delayed, our ability to commercialize our products will be impaired.
- If our third party suppliers or contract manufacturers do not maintain appropriate standards of manufacturing in accordance with cGMP and other manufacturing regulations, our development and commercialization activities could suffer significant interruptions or delays.
- Our drug products are subject to continuing regulatory review. If we fail to comply with continuing U.S. and applicable foreign regulations, we could lose those approvals, and our business would be severely harmed.
- Enacted and future legislation or judicial action may increase the difficulty and cost for us to market our approved products or commercialize any other drug candidates we may acquire or license and affect the prices we may obtain.
- If we fail to obtain or subsequently maintain orphan drug exclusivity or regulatory exclusivity for FIRDAPSE®, AGAMREE®, and any other orphan drug candidates we may acquire or license, our competitors may sell products to treat the same conditions at greatly reduced prices, and our revenues would be significantly adversely affected.
- If the FDA or comparable foreign regulatory authorities approve generic versions of any of our products, or such authorities do not grant our products sufficient periods of exclusivity before approving generic versions of our products, the sales of our products could be adversely affected.
- Changes to the Orphan Drug Act or successful legal challenges to the FDA's interpretation of the Orphan Drug Act may affect our ability to obtain or subsequently maintain orphan drug exclusivity or may affect the scope orphan drug exclusivity for our products.
- Our operations and relationships with healthcare providers, healthcare organizations, customers and third party payors are subject to applicable anti-bribery, anti-kickback, fraud and abuse, transparency and other healthcare laws and regulations, which could expose us to, among other things, enforcement actions, criminal sanctions,

civil penalties, contractual damages, reputational harm, administrative burdens and diminished profits and future earnings.

- We are subject to environmental, health, and safety laws and regulations, which could increase our costs or restrict our operations.

Risks Related to our Intellectual Property

- If we are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.
- There is a risk that our patents may not protect our products from generic competition.
- Our success will depend significantly on our ability to operate without infringing the patents and other proprietary rights of third parties.
- We may incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights.

There are also general risk factors relating to us that you should consider that relate to our business and to our common stock.

Risk Factors

Our business involves a high degree of risk. You should carefully consider the risks and uncertainties described below, and all of the other information contained in this Form 10-K in assessing the risks relating to ownership of our common stock. The risks described below could cause our business, results of operations, financial condition and prospects to materially suffer and the market price of our stock to decline.

Risks related to Our Business

Our success depends on the successful commercialization of our products. To the extent that our drug products are not commercially successful, our business, financial condition and results of operations will be materially harmed.

We received approval for FIRDAPSE® for the treatment of LEMS from the FDA in November 2018; in January 2023, we completed our acquisition of FYCOMPA® for the treatment of (i) partial-onset seizures with or without secondary generalized seizures in people with epilepsy four years of age and older, and (ii) for the treatment of primary generalized tonic-clonic seizures in people with epilepsy twelve years of age and older from Eisai; and in October 2023, we received approval for AGAMREE® for the treatment of DMD. We invest a significant amount of effort and financial resources in the commercialization of these drug products in the U.S., and, in the case of FIRDAPSE®, Canada and now Japan and in the case of AGAMREE®, Canada. The ability for us to generate net product revenues from our drug products will depend on the size of the markets, the numbers of competitors in such markets and numerous other factors, including:

- successfully establishing and maintaining effective sales, marketing, and distribution systems in jurisdictions in which our drug products are approved for sale;
- successfully establishing and maintaining commercial third party manufacturers and having adequate commercial quantities of our drug products manufactured at acceptable cost and quality levels, including maintaining cGMP and quality systems regulation standards required by various regulatory agencies;
- broad acceptance of our drug products by physicians, patients and the healthcare community;
- the acceptance of pricing and placement of our drug products on payers' formularies and the associated tiers;
- effectively competing with other approved or used medicines and future compounds in development;
- continued demonstration of safety and efficacy of our drug products in comparison to competing products; and
- obtaining, maintaining, enforcing, and defending intellectual property rights and claims.

Our drug products may fail to receive the degree of market acceptance by physicians, patients, third party payers or others in the medical community necessary for commercial success, which would negatively impact our business.

Our drug products may fail to gain sufficient market acceptance by physicians, patients, third party payers, or others in the medical community. If any of our drug products do not achieve an adequate level of acceptance, we may not generate significant net product revenue or become profitable. The degree of market acceptance of our drug products is dependent on a number of factors, including but not limited to:

- the efficacy and potential advantages compared to alternative treatments, including the convenience and ease, or duration of administration;

- the competition pressure from other products can be particularly pronounced at launch when having to establish market share against older, more established products;
- a separate competitive pressure also occurs at loss of exclusivity when drug products like ours to face significant price erosion and loss of market share upon the launch of generic alternatives – with the effect typically being more severe the greater the number of generic entrants;
- the prevalence and severity of any side effects;
- the acceptability of the price of our drug products relative to other treatments;
- the content of the approved product labels and our ability to make compelling product claims;
- the effectiveness and adequacy of our and our collaboration partner's sales and marketing efforts;
- the patients' out-of-pocket costs in relation to alternative treatments;
- the breadth and cost of distribution support;
- the effectiveness of our patient assistance and support programs;
- the availability of third party payer coverage and adequate reimbursement; and
- any restrictions on the use of our drug products together with other medications.

Our business is subject to substantial competition.

The biotechnology and pharmaceutical industries are highly competitive. Many of our competitors have substantially greater financial and other resources, larger research and development staffs and more experience developing products, obtaining FDA and other regulatory approvals of products and manufacturing and marketing products than we have. We compete against pharmaceutical companies that are developing or currently marketing therapies that will compete with us. In addition, we compete against biotechnology companies, universities, government agencies, and other research institutions in the development of pharmaceutical products. Our business could be negatively impacted if our competitors' present or future offerings are more effective, safer or less expensive than ours, or more readily accepted by regulators, healthcare providers or third party payors. Further, we may also compete with respect to manufacturing efficiency and marketing capabilities. For all of these reasons, we may not be able to compete successfully.

Our strategy of seeking to acquire or in-license innovative technical platforms or earlier stage drug development programs may not be successful.

We continue to seek to broaden and diversify our product portfolio through acquisitions of both early and late-stage products or companies or technology platforms in orphan, rare disease therapeutic categories. To accomplish these priorities, we are employing a disciplined approach to evaluating assets and we believe that this strategic expansion will better position our company to build out a broader more diversified portfolio of drug candidates, which should add greater value to our company over the near-term and the long-term. However, there can be no assurance that whatever product candidates or technology platforms we acquire, if any, will be successfully developed or commercialized.

The process of proposing, negotiating and implementing a license or acquisition of a product candidate is lengthy and complex, and we may be unable to in-license or acquire the rights to any such products, product candidates or technologies from third parties for several reasons. Further, even if we identify acquisition or in-licensing targets, we may not be able to close those deals or we may determine after diligence not to pursue identified targets. The success of this strategy depends partly upon our ability to identify, select and acquire or in-license promising product candidates and technologies.

In addition, acquisitions and in-licenses may entail numerous operational, financial and legal risks, including:

- exposure to known and unknown liabilities, including possible intellectual property infringement claims, violations of laws, tax liabilities and commercial disputes;
- incurrence of substantial debt, dilutive issuances of securities or depletion of cash to pay for acquisitions;
- higher than expected acquisition and integration costs;
- difficulty in combining the operations and personnel of any acquired businesses with our operations and personnel;
- inability to maintain uniform standards, controls, procedures and policies;
- restructuring charges related to eliminating redundancies or disposing of assets as part of any such combination;

- large write-offs and difficulties in assessing the relative percentages of in-process research and development expense that can be immediately written off as compared to the amount that must be amortized over the appropriate life of the asset;
- increased amortization expenses or, in the event that we write down the value of acquired assets, impairment losses;
- potential failure of the due diligence process to identify significant problems, liabilities or other shortcomings or challenges of an acquired or licensed product candidate or technology, including problems, liabilities or other shortcomings or challenges with respect to intellectual property, product quality, revenue recognition or other accounting practices, partner disputes or issues and other legal and financial contingencies and known and unknown liabilities; and
- entry into therapeutic modalities, indications or markets in which we have no or limited direct prior development or commercial experience and where competitors in such markets have stronger market positions.

Because the target patient population for certain of our products is small, we must achieve significant market share and obtain relatively high per-patient prices for our products to achieve meaningful gross margins.

Our products target diseases with a small patient population. A key component of the successful commercialization of a drug product for these indications includes identification of patients and a targeted prescriber base for the drug product. Due to small patient populations for certain of our products, we believe that we would need to have significant market penetration to achieve meaningful revenues and identifying patients and targeting the prescriber base are key to achieving significant market penetration. Typically, drugs for conditions with small prevalence have higher prices in order to generate a return on investment, and as a result, the per-patient prices at which we sell our products are relatively high in order for us to generate an appropriate return for the investment in these product development programs and achieve meaningful gross margins.

There can be no assurance that we will be successful in achieving a sufficient degree of market penetration and/or obtaining or maintaining high per-patient prices for FIRDAPSE® and AGAMREE® for diseases with small patient populations. Further, even if we obtain significant market share for FIRDAPSE® and AGAMREE®, because the potential target populations are very small, we may not be able to maintain profitability despite obtaining such significant market share. Additionally, patients who discontinue therapy or do not fill prescriptions are not easily replaced by new patients, given the limited patient population.

Because of risks associated with taking FYCOMPA®, potential patients may be reluctant to start treatment with FYCOMPA® or may discontinue use.

FYCOMPA®'s labeling has a boxed warning noting that some people taking the drug have undergone serious psychiatric and behavioral changes. These events occurred in people who had no history of such issues, as well as people who had such a history. The psychiatric changes included mood changes like euphoric mood, anger, irritability, aggression, belligerence, agitation, and anxiety, as well as psychosis (acute psychosis, hallucinations, delusions, paranoia) and delirium (delirium, confusional state, disorientation, memory impairment). Behavioral changes included physical assault and homicidal ideation and/or threats. While these side effects are rare, their existence may cause reluctance on the part of patients or providers to start or continue treatment.

Other serious side effects include suicidal thoughts or behavior (like all anti-epileptic drugs), dizziness and gait disturbance, somnolence and fatigue, risk of falls, and increased risk of seizures if the drug is quickly withdrawn. In clinical trials, dizziness, somnolence, vertigo, aggression, anger, loss of coordination, blurred vision, irritability, and slurred speech were the side effects that most commonly led people to leave the trial. Use of FYCOMPA® is also contraindicated in women who are pregnant or breastfeeding.

Risks Related to the Development of Drug Products

Failure can occur at any stage of our drug development efforts.

We will only obtain regulatory approval to commercialize our future drug candidates if we can demonstrate to the satisfaction of the FDA (or the equivalent foreign regulatory authorities) in adequate and well-controlled clinical studies and trials that the drug is safe and effective for its intended use, that the clinical and other benefits outweigh the safety risks and that it otherwise meets approval requirements. As we have experienced in the past, a failure of one or more pre-clinical or clinical trials or studies can occur at any stage of drug development. We may experience numerous unforeseen events during, or as a result of, testing that could delay or prevent us from obtaining regulatory approval for, or commercializing our drug candidates, including but not limited to:

- regulators or Institutional Review Boards (IRBs) may not authorize us to commence a clinical trial or conduct a clinical trial at a prospective trial site;

- conditions may be imposed upon us by the FDA regarding the scope or design of our clinical trials, or we may be required to resubmit our clinical trial protocols to IRBs for review due to changes in the regulatory environment;
- the number of subjects required for our clinical trials may be larger, patient enrollment may take longer, or patients may drop out of our clinical trials at a higher rate than we anticipate;
- we may have to suspend or terminate one or more of our clinical trials if we, regulators, or IRBs determine that the participants are being subjected to unreasonable health risks;
- our third party contractors, clinical investigators or contractual collaborators may fail to comply with regulatory requirements or fail to meet their contractual obligations to us in a timely manner;
- the FDA may not accept clinical data from trials that are conducted at clinical sites in countries where the standard of care is potentially different from the U.S.;
- our tests may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional testing; and
- the costs of our pre-clinical and/or clinical trials may be greater than we anticipate.

We rely on third parties to conduct our pre-clinical studies and clinical studies and trials, and if they do not perform their obligations to us we may not be able to obtain approval for additional indications.

We do not have the ability to independently conduct pre-clinical studies or clinical studies and trials, and we rely on third parties, such as third party contract research and governmental organizations, medical institutions and clinical investigators (including academic clinical investigators), to conduct studies and trials for us. Our reliance on third parties for development activities reduces our control over these activities. These third parties may not complete activities on schedule or may not conduct our pre-clinical studies and our clinical studies and trials in accordance with regulatory requirements or our study design. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may be adversely affected, and our efforts to obtain regulatory approvals for and commercialize our product candidates may be delayed.

If we conduct studies with other parties, we may not have control over all decisions associated with that trial. To the extent that we disagree with the other party on such issues as study design, study timing and the like, it could adversely affect our drug development plans.

Although we also rely on third parties to manage the data from our studies and trials, we are responsible for confirming that each of our studies and trials is conducted in accordance with its general investigational plan and protocol. Moreover, the FDA and foreign regulatory agencies will require us to comply with applicable regulations and standards, including Good Laboratory Practice (GLP) and Good Clinical Practice (GCP), for conducting, recording and reporting the results of such studies and trials to assure that the data and the results are credible and accurate and that the human study and trial participants are adequately protected. Our reliance on third parties does not relieve us of these obligations and requirements, and we may fail to obtain regulatory approval for any additional indications if these requirements are not met.

We will need to continue to develop and maintain distribution and production capabilities or relationships to be successful.

We are licensed in Florida as a virtual drug manufacturer, which means we have no in-house manufacturing capacity and we will be obligated to rely on contract manufacturers and packagers. We cannot be sure that we will successfully manufacture any product, either independently or under manufacturing arrangements, if any, with third party manufacturers. Moreover, if any manufacturer should cease doing business with us or experience delays, shortages of supply or excessive demands on their capacity, we may not be able to obtain adequate quantities of product in a timely manner, or at all. Manufacturers, and in certain situations their suppliers, are required to comply with current NDA commitments and cGMP requirements enforced by the FDA, and similar requirements of other countries. The failure by a manufacturer to comply with these requirements could affect its ability to provide us with product. Although we intend to rely on third party contract manufacturers, we are ultimately responsible for ensuring that our products are manufactured in accordance with cGMP. In addition, if, during a preapproval inspection or other inspection of our third party manufacturers' facility or facilities, the FDA determines that the facility is not in compliance with cGMP, any of our marketing applications that lists such facility as a manufacturer may not be approved or approval may be delayed until the facility comes into compliance with cGMP and completes a successful re-inspection by the FDA.

Any manufacturing problem, natural disaster, or epidemic, affecting manufacturing facilities, or the loss of a contract manufacturer could be disruptive to our operations and result in lost sales. Additionally, we will be reliant on third parties to supply the raw materials needed to manufacture our products. Any reliance on suppliers may involve several risks, including a potential inability to obtain critical materials and reduced control over production costs, delivery schedules, reliability and

quality. Any unanticipated disruption to future contract manufacture caused by problems at suppliers could delay shipment of products, increase our cost of goods sold and result in lost sales.

If our suppliers were to be unable to supply us with adequate supply of our drugs, it could have a material adverse effect on our ability to successfully commercialize our drug candidates.

We could be impacted by the viability of our suppliers.

We source FIRDAPSE® from more than one supplier, and we have entered into contracts with our suppliers that contractually obligate them to meet our requirements. However, if our suppliers cannot or will not meet our requirements (for whatever reason), our business could be adversely impacted.

We are dependent on our licensing partners for supplies of FYCOMPA® and AGAMREE®.

Through our agreements with Eisai for FYCOMPA® and Santhera for AGAMREE®, we have agreed to purchase our supplies of each product through such companies. If either company were unable to supply sufficient supplies of drug product, our business would be adversely impacted, whether we would be required to work with these companies to resume supplies or whether we would be required to search for a sufficient third party supplier.

We may encounter difficulties in managing our growth, which would adversely affect our results of operations.

To manage future growth, we will likely need to hire, train, and manage additional employees. Concurrent with expanding our operational and marketing capabilities, we will also need to increase our product development activities. We may not be able to support, financially or otherwise, future growth, or hire, train, motivate, and manage the required personnel. Our failure to manage growth effectively could limit our ability to achieve our goals.

Our success in managing our growth will depend in part on the ability of our executive officers to continue to implement and improve our operational, management, information and financial control systems, and to expand, train and manage our employee base, and particularly to expand, train and manage a specially-trained field-based force to market our products. We may not be able to attract and retain personnel on acceptable terms given the intense competition for such personnel among biotechnology, pharmaceutical and healthcare companies, universities and non-profit research institutions. Our inability to manage growth effectively could cause our operating costs to grow at a faster pace than we currently anticipate and could have a material adverse effect on our business, financial condition, results of operations and prospects.

Pressure on drug product third party payor coverage, reimbursement and pricing may impair our ability to be reimbursed at prices or on terms sufficient to provide a viable financial outcome.

The commercial success of our drug products will depend substantially on the extent to which the cost of those products will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities (such as Medicare and Medicaid), private health coverage insurers and other third party payors. If reimbursement is not available, or is available only to limited levels, we may not be able to continue to successfully commercialize our products. Even if coverage is provided, the approved reimbursement amount may not be high enough to establish and maintain pricing sufficient to realize a meaningful return on our investment.

The healthcare industry is acutely focused on cost containment, both in the U.S. and elsewhere. Government authorities and third party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications, which could affect our ability to sell our product candidates profitably. These payors may not view our products as cost-effective, and coverage and reimbursement may not be available to our customers, or may not be sufficient to allow our products, if any, to be marketed on a competitive basis. Cost-control initiatives could cause us to decrease the price we might establish for products, which could result in lower than anticipated product revenues. If the prices for our products decrease or if governmental and other third party payors do not provide adequate coverage or reimbursement, our prospects for revenue and profitability will suffer.

There may also be delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the indications for which the drug is approved by the FDA. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Reimbursement rates may vary, by way of example, according to the use of the drug and the clinical setting in which it is used. Reimbursement rates may also be based on reimbursement levels already set for lower cost drugs or may be incorporated into existing payments for other services.

In addition, increasingly, third party payors are requiring higher levels of evidence of the benefits and clinical outcomes of new technologies and are challenging the prices charged. We cannot be sure that coverage will be available for any product candidate that we commercialize and, if available, that the reimbursement rates will be adequate. Further, the net reimbursement for drug products may be subject to additional reductions if there are changes to laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the U.S. An inability to promptly obtain coverage and adequate

payment rates from both government-funded and private payors for any of our product candidates for which we obtain marketing approval could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

The pricing of pharmaceutical products, in general, and of specialty drugs, in particular, has been a topic of concern in the U.S. Congress, where hearings have been held on the topic, and several bills have been introduced proposing a variety of actions to restrain the prices of drugs. Healthcare reform proposals culminated in the enactment of the IRA, which eliminates, beginning in 2025, the coverage gap under Medicare Part D by significantly lowering the enrollee maximum out-of-pocket cost and requiring manufacturers to subsidize, through a newly established manufacturer discount program, 10% of Part D enrollees' prescription costs for brand drugs below the out-of-pocket limit, and 20% once the out-of-pocket limit has been reached. The IRA also allows the Department of Health and Human Services (HHS) to negotiate the selling price of certain drugs and biologics that the CMS reimburses under Medicare Part B and Part D (excluding drugs and biologics that are designated and approved for only one rare disease or condition), although only high-expenditure single-source drugs that have been approved for at least 7 years (11 years for biologics) can be selected by CMS for negotiation, with the negotiated price taking effect two years after the selection year. The negotiated prices, which will first become effective in 2026, will be capped at a statutory ceiling price. For 2026, the first year in which negotiated prices become effective, CMS selected 10 high-cost Medicare Part D products in 2023, negotiations began in 2024, and the negotiated maximum fair price for each product has been announced. CMS has selected 15 additional Medicare Part D drugs for negotiated maximum fair pricing in 2027. For 2028, an additional 15 drugs, which may be covered under either Medicare Part B or Part D, will be selected, and for 2029 and subsequent years, 20 Part B or Part D drugs will be selected. Beginning in October 2022 for Medicare Part D and January 2023 for Medicare Part B, the IRA began penalizing drug manufacturers that increased prices of Medicare Part D and Part B drugs at a rate greater than the rate of inflation. There have also been indications by some, including President Trump's Secretary of HHS, that the government should seize the patents of "high priced drugs" and transfer them to other manufacturers to lower drug prices. It is unclear to what extent other statutory, regulatory, and administrative initiatives will be enacted and implemented in the future and to what extent these or any future legislation or regulations will have on our business, including market acceptance, and sales, of our products and product candidates.

We cannot predict how any such laws or regulations, or new laws or regulations that have yet to be proposed, will affect the pricing of our product, of orphan drugs generally, or of pharmaceutical products generally.

Our internal computer systems, or those of our contract research organizations and other key vendors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs.

Our internal computer systems and those of our contract research organizations and other key vendors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our programs. For example, the loss of clinical trial data from completed or ongoing clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development of our drug candidates could be delayed.

Our employees, sales agents and consultants may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk of fraud or other misconduct by our employees, sales agents or consultants. Misconduct could include failures to comply with FDA regulations, provide accurate information to the FDA, comply with manufacturing standards, comply with federal and state healthcare fraud and abuse laws and regulations, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, 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. Misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter such misconduct, 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 be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

Risks Related to Government Regulation

The healthcare industry is highly regulated, subject to stringent regulatory standards and other applicable laws, and we may be the subject of unexpected changes in interpretation or enforcement, any of which may adversely impact our business.

The healthcare industry is highly regulated. We, and our customers, are subject to various local, state, federal, national, and transnational laws and regulations, which include the operating, quality, and security standards of the FDA, the DEA, various state boards of pharmacy, state health departments, similar bodies of the U.K., the E.U. and its member states, and other comparable agencies around the world, and, in the future, any change to such laws and regulations or the interpretation or application thereof could adversely affect us. Among other rules affecting us, we are subject to laws and regulations concerning cGMP and drug safety. New public health orders or best practice guidelines may increase our costs to operate or reduce our productivity, thereby affecting our business, financial condition, or results of operations.

We cannot ensure that our compliance controls, policies, and procedures will in every instance protect us from acts of our employees, agents, contractors, or collaborators that turn out to violate the laws or regulations of the jurisdictions in which we operate, including, without limitation, healthcare, employment, foreign corrupt practices, trade restrictions and sanctions, environmental, competition, and privacy laws and regulations. Failure by us or by our customers to comply with the requirements of applicable laws and regulations or requests from regulatory authorities could result in warning letters, product recalls or seizures, monetary sanctions, injunctions to halt manufacture or distribution, restrictions on our operations, civil or criminal sanctions, or withdrawal of existing or denial of pending approvals, permits, or registrations, including those relating to products or facilities. In addition, any such failure relating to the products or services we provide could expose us to contractual or product liability claims as well as claims from our customers, including claims for reimbursement for lost or damaged active pharmaceutical ingredients, which cost could be significant. Our business activities outside the U.S. are subject to the U.S. Foreign Corrupt Practices Act, the U.K. Anti-Bribery Act, and other anti-bribery or anti-corruption laws, regulations, or rules. Our business is heavily regulated and therefore involves significant interaction with public officials, including officials of non-U.S. governments. There is no certainty that all of our employees, agents, contractors, or collaborators, or those of our affiliates, will comply with all applicable laws and regulations, particularly given the high level of complexity of these laws. Violations of these laws and regulations may have a material adverse impact on our business, prospects, financial condition, or results of operations.

In addition, any new offering or product classified as a pharmaceutical or medical device must undergo lengthy and rigorous clinical testing and other extensive, costly, and time-consuming procedures mandated by the FDA, the EMA, and other equivalent local, state, federal, national, and transnational regulatory authorities in the jurisdictions that regulate our offerings and products.

Our results depend on our ability to execute and improve when necessary our quality management strategy and systems, and effectively train and maintain our workforce with respect to quality management. Quality management plays an essential role in determining and meeting customer requirements, preventing defects, and improving our offerings, and, despite our network of quality systems, a quality or safety issue, including with respect to a high-revenue product, could have an adverse effect on our business, financial condition, stock price, or results of operations and may subject us to regulatory action, including a product recall, product seizure, injunction to halt manufacture or distribution, or restriction on our operations; monetary fines; or other civil or criminal sanctions. In addition, such an issue could subject us to adverse publicity and costly litigation, including claims from our customers for reimbursement for the cost of lost or damaged active pharmaceutical ingredients or other related losses, the cost of which could be significant.

The regulatory approval process is lengthy, and we may not be able to obtain all of the regulatory approvals required to manufacture and commercialize our drug products in which we are licensed to them.

We will not be able to commercialize our products in other countries or for additional indications until we have obtained the requisite regulatory approvals from applicable governmental authorities. To obtain regulatory approval of a drug candidate for an indication, we must demonstrate to the satisfaction of the applicable regulatory agency that such drug candidate is safe and effective for that indication. The type and magnitude of the testing required for regulatory approval varies depending on the drug candidate and the disease or condition for which it is being developed. In addition, in the U.S. we must show that the facilities used to manufacture our drug candidates are in compliance with cGMP requirements. We will also have to meet similar regulations in any foreign country where we may seek to commercialize our drug candidates. In general, these requirements mandate that manufacturers follow elaborate design, testing, control, documentation, and other quality assurance procedures throughout the entire manufacturing process. The process of obtaining regulatory approvals typically takes several years and requires the expenditure of substantial capital and other resources. Despite the time, expense and resources invested by us in the approval process, we may not be able to demonstrate that our drug candidate is safe and effective for such indications, in which event we would not receive the regulatory approval required to market it.

If our pre-clinical studies or our clinical studies and trials are unsuccessful or significantly delayed, our ability to commercialize our products will be impaired.

Before we can obtain future regulatory approval for the sale of our drug candidates for an indication, we may have to conduct, at our own expense, pre-clinical tests in animals in order to support the safety of our drug candidates. Pre-clinical testing is expensive, difficult to design and implement, can take several years to complete, and is uncertain as to outcome. Our pre-clinical tests may produce negative or inconclusive results, and on the basis of such results, we may decide, or regulators may require us, to halt ongoing clinical trials or conduct additional pre-clinical testing.

In other countries where FIRDAPSE®, FYCOMPA®, AGAMREE®, or any other product we may acquire or license may be marketed, we will also be subject to regulatory requirements governing human clinical studies, trials and marketing approval for drugs. The requirements governing the conduct of clinical studies, trials, product licensing, pricing and reimbursement varies widely from country to country.

We may face significant delays in our clinical studies and trials due to an inability to recruit patients for our clinical studies and trials or to retain patients in the clinical studies and trials we may perform.

We may encounter difficulties in our current and future clinical studies and trials recruiting patients, particularly since the conditions we are studying are rare, orphan conditions. The availability of approved therapies can also make enrollment difficult. We compete for study and trial subjects with others conducting clinical trials testing other treatments for the indications we are studying for our drug candidates. Further, unrelated third parties and investigators in the academic community have in the past and we expect will continue in the future to test our drug products and/or drug candidates. If these third party tests are unsuccessful, or if they show significant health risk to the test subjects, our development efforts may also be adversely affected.

Clinical trials in orphan diseases are often difficult to enroll given the small number of patients with these diseases. Completion of orphan clinical trials may take considerably more time than other trials, sometimes years, depending on factors such as type, complexity, novelty and intended use of a product candidate. As a result of the uncertainties described above, there can be no assurance that we will meet timelines that we establish for any of our clinical trials.

If our third party suppliers or contract manufacturers do not maintain appropriate standards of manufacturing in accordance with cGMP and other manufacturing regulations, our development and commercialization activities could suffer significant interruptions or delays.

We rely, and intend to continue to rely, on third party suppliers and contract manufacturers to provide us with materials for our clinical trials and commercial-scale production of our products. These suppliers and manufacturers must continuously adhere to cGMP as well as any applicable corresponding manufacturing regulations outside of the U.S. In complying with these regulations, we and our third party suppliers and contract manufacturers must expend significant time, money and effort in the areas of design and development, testing, production, record-keeping, and quality control to assure that our products meet applicable specifications and other regulatory requirements. Failure to comply with these requirements could result in an enforcement action against us, including warning letters, the seizure of products, suspension or withdrawal of approvals, shutting down of production, and criminal prosecution. Any of these third party suppliers or contract manufacturers will also be subject to inspections by the FDA and other regulatory agencies. If any of our third party suppliers or contract manufacturers fail to comply with cGMP or other applicable manufacturing regulations, our ability to develop and commercialize our products could suffer significant interruptions and delays.

Reliance on third party manufacturers entails risks to which we would not be subject if we manufactured the product ourselves, including:

- reliance on the third party for regulatory compliance and quality assurance;
- reliance on the continued financial viability of the third parties;
- limitations on supply availability resulting from capacity and scheduling constraints of the third parties;
- impact on our reputation in the marketplace if manufacturers of our products fail to meet the demands of our customers;
- the possible breach of the manufacturing agreement by the third party because of factors beyond our control; and
- the possible termination or nonrenewal of the agreement by the third party, based on its own business priorities, at a time that is costly or inconvenient for us.

If any of our contract manufacturers fail to achieve and maintain appropriate manufacturing standards, patients using our products could be injured or die, resulting in product liability claims. Even absent patient injury, we may be subject to product

recalls, product seizures or withdrawals, delays or failures in testing or delivery, cost overruns, or other problems that could seriously harm our business or profitability.

Our drug products are subject to continuing regulatory review. If we fail to comply with continuing U.S. and applicable foreign regulations, we could lose those approvals, and our business would be severely harmed.

We are and will continue to be subject to continuing regulatory review for our approved products, including the review of our required nonclinical and clinical post-marketing studies, and other clinical results which are reported after our drug candidates become commercially available approved drugs. As greater numbers of patients use a drug following its approval, side effects and other problems may be observed after approval that were not seen or anticipated during preapproval clinical studies and trials. In addition, the manufacturer, and the manufacturing facilities we use to make any approved drugs, will also be subject to periodic review and inspection by the FDA. The subsequent discovery of previously unknown problems with the drug, manufacturer or facility may result in restrictions on the drug, manufacturer or facility, including withdrawal of the drug from the market. If we fail to comply with applicable continuing regulatory requirements, we may be subject to fines, suspension, or withdrawal of regulatory approval, product recalls and seizures, operating restrictions, and criminal prosecutions.

Our product promotion and advertising are also subject to regulatory requirements and continuing regulatory review. In particular, the marketing claims we will be permitted to make in labeling or advertising regarding our marketed products will be limited by the terms and conditions of the FDA-approved labeling and available scientific data. We must submit copies of our advertisements and promotional labeling to the FDA at the time of initial publication or dissemination. If the FDA believes these materials or statements promote our products for unapproved indications, or with unsubstantiated claims, or if we fail to provide appropriate safety related information, the FDA could allege that our promotional activities misbrand our products. Specifically, the FDA could issue an untitled letter or warning letter, which may demand, among other things, that we cease such promotional activities and issue corrective advertisements and labeling to all recipients of the misbranded materials. The FDA also could take enforcement action including seizure of allegedly misbranded product, injunction, or criminal prosecution against us and our officers or employees. If we repeatedly or deliberately fail to submit such advertisements and labeling to the agency, the FDA could withdraw our approvals. Moreover, the Department of Justice can bring civil or criminal actions against companies and executives that promote drugs or biologics for unapproved uses, based on the FDCA, the False Claims Act, and other federal laws governing the marketing and reimbursement for such products under federally supported healthcare programs such as Medicare and Medicaid. Monetary penalties in such cases have often been substantial, and civil penalties can include costly mandatory compliance programs and potential exclusion of a company's products from federal healthcare programs.

Enacted and future legislation or judicial action may increase the difficulty and cost for us to commercialize FIRDAPSE®, FYCOMPA®, AGAMREE®, or any other drug candidates we may acquire or license and affect the prices we may obtain.

In the U.S., there have been a number of court cases, legislative and regulatory changes, and other potential changes relating to the healthcare system that restrict or regulate post-approval activities, which may affect our ability to profitably sell FIRDAPSE®, AGAMREE®, or any other drug candidates for which we obtain marketing approval.

Legislative and regulatory proposals have been made to expand post-approval requirements, restrict sales and promotional activities for pharmaceutical products, and with respect to orphan drug designation and exclusivity. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may subject us to more stringent product labeling and post-marketing testing and other requirements. Delays in feedback from the FDA may affect our ability to quickly update or adjust our label in the interest of patient adherence and tolerability. We cannot predict whether other legislative changes will be adopted or how such changes would affect the pharmaceutical industry generally and specifically the commercialization of FIRDAPSE® and any other products we develop.

While it is difficult to quantify in the absence of specific proposals, we assess the risk of a material financial impact from potential new tariffs to be low due to a significant portion of our supply chain being U.S. based; however, this may not prove to be the case.

If we fail to obtain or subsequently maintain orphan drug exclusivity or regulatory exclusivity for FIRDAPSE®, AGAMREE®, and any other orphan drug candidates we may acquire or in-license, our competitors may sell products to treat the same conditions at greatly reduced prices, and our revenues would be significantly adversely affected.

In the U.S., orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and user fee waivers. The company that first obtains FDA approval for a designated orphan drug for a given rare disease receives marketing exclusivity for use of that drug for the stated disease or condition for a period of seven years, with an additional six months of exclusivity if the product also qualifies for pediatric exclusivity. Orphan drug exclusive marketing rights may be lost if the FDA later determines that the request for designation was materially defective, a subsequent product is deemed clinically superior, or if the manufacturer is unable to deliver sufficient quantity of the drug.

Because the extent and scope of patent protection for some of our drug products may be particularly limited, orphan drug designation – and ultimately, orphan drug exclusivity – is especially important for our products that are eligible for orphan drug

designation. For eligible drugs, we plan to rely on the orphan exclusivity period to maintain a competitive position. However, if we do not obtain orphan drug exclusivity for our drug candidates or we cannot maintain orphan exclusivity for our drug candidates, our competitors may then sell the same drug to treat the same condition and our revenues will be reduced. Also, without strong patent protection, competitors may sell a generic version upon the expiration of orphan exclusivity if our patent position is not upheld.

Even if we obtain orphan drug designation for our future drug candidates, we may not fulfill the criteria for exclusivity or we may not be the first to obtain marketing approval for any orphan indication. Further, even if we obtain orphan drug exclusivity for a particular product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition, and FDA can approve the same drug for a different patient population. Even after an orphan drug is approved, the FDA can subsequently approve a drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. The FDA can discontinue orphan drug exclusivity after it has been granted if the orphan drug cannot be manufactured in sufficient quantities to meet demand.

Finally, there can be no assurance that the exclusivity provisions currently in the law may not be changed in the future and the impact of any such changes (if made) on us. The orphan drug exclusivity contained in the Orphan Drug Act has been the subject of recent scrutiny from the press, from some members of Congress and from some in the medical community. There can be no assurance that the exclusivity granted in the Orphan Drug Act to orphan drugs approved by the FDA will not be modified in the future, and as to how any such change might affect our products, if approved.

If the FDA or comparable foreign regulatory authorities approve generic versions of any of our products, or such authorities do not grant our products sufficient periods of exclusivity before approving generic versions of our products, the sales of our products could be adversely affected.

Once an NDA is approved, the product covered becomes a "reference listed drug" in the FDA's publication, "Approved Drug Products with Therapeutic Equivalence Evaluations", commonly known as the Orange Book. Manufacturers may seek approval of generic versions of reference listed drugs through submission of ANDAs in the U.S. In support of an ANDA, a generic manufacturer need not conduct clinical trials. Rather, the applicant generally must show that its product has the same active ingredient(s), dosage form, strength, route of administration and conditions of use or labeling as the reference listed drug and that the generic version is bioequivalent to the reference listed drug, meaning it is absorbed in the body at the same rate and to the same extent. Generic products may be significantly less costly to bring to market than the reference listed drug and companies that produce generic products are generally able to offer them at lower prices. Thus, following the introduction of a generic drug, a significant percentage of the sales of any branded product or reference listed drug is typically lost to the generic product.

The FDA may not approve an ANDA for a generic product until any applicable period of non-patent exclusivity for the reference listed drug has expired. The U.S. Federal Food, Drug, and Cosmetic Act (FDCA) provides a period of five years of non-patent exclusivity for a new drug containing a new chemical element (NCE). Specifically, in cases where such exclusivity has been granted, an ANDA may not be submitted to the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification that a patent covering the reference listed drug is either invalid or will not be infringed by the generic product, in which case the applicant may submit its application four years following approval of the reference listed drug.

Patent protection for FYCOMPA® will expire during 2025, and the FDA may approve an ANDA for a generic version at any time after that patent expires. Additionally, while we believe that FIRDAPSE® and AGAMREE® are protected both by patent and NCE, manufacturers may seek to launch generic versions of these products following the expiration of the applicable marketing exclusivity period, even if we still have patent protection for such product.

Competition that FYCOMPA® or our other products may face from generic versions could materially and adversely affect our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on investment for such products.

Changes to the Orphan Drug Act or successful legal challenges to the FDA's interpretation of the Orphan Drug Act may affect our ability to obtain or subsequently maintain orphan drug exclusivity or affect the scope of orphan drug exclusivity for our products.

There can be no assurance that the designation and/or exclusivity provisions currently in the law may not be changed in the future and the impact of any such changes (if made) on us. For example, the U.S. Congress could pass, and the President could sign, legislation to effectively overturn the decision of the U.S. Court of Appeals for the 11th Circuit overturning the FDA's approval of RUZURGI®, and such legislation, if passed and signed into law, could retroactively affect the outcome of the 11th Circuit's decision. Notwithstanding, since we now hold the U.S. rights to RUZURGI®, these legislative efforts will have no effect on our FIRDAPSE® business.

In that regard, in January 2023, the FDA reported that while it is complying with the 11th Circuit decision in Catalyst's favor with respect to FIRDAPSE®, going forward the FDA intends to continue to apply its regulations tying the scope of orphan

drug exclusivity to the uses or indications for which a drug is approved with respect to other orphan drugs. We will not be affected by the FDA's position, as the FDA's announcement confirms the FDA's previous decision to set aside the approval of RUZURGI® as a result of the 11th Circuit's decision.

The orphan drug exclusivity contained in the Orphan Drug Act has been the subject of scrutiny from the press, from some members of Congress and from some in the medical community. Furthermore, the FDA's interpretations of the Orphan Drug Act have been successfully challenged in court and future court decisions could continue that trend. There can be no assurance that the exclusivity granted in the Orphan Drug Act to orphan drugs approved by the FDA will not be modified in the future, and as to how any such change might affect our products, if approved.

Our operations and relationships with healthcare providers, healthcare organizations, customers and third party payors are subject to applicable anti-bribery, anti-kickback, fraud and abuse, transparency and other healthcare laws and regulations, which could expose us to, among other things, enforcement actions, criminal sanctions, civil penalties, contractual damages, reputational harm, administrative burdens and diminished profits and future earnings.

Our current and future arrangements with healthcare providers, healthcare organizations, third party payors, customers, and patients expose us to broadly applicable anti-bribery, fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we research, market, sell and distribute our drug candidates. In addition, we may be subject to patient data privacy and security regulation by the U.S. federal government and the states and the foreign governments in which we conduct our business. Restrictions under applicable federal and state anti-bribery and healthcare laws and regulations include the following:

- the Federal health care program Anti-Kickback Statute, which prohibits individuals and entities from, among other things, knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, 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 a federal and state healthcare program such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- the federal criminal and civil false claims and civil monetary penalties laws, including the federal False Claims Act, which can be imposed through civil whistleblower or qui tam actions against individuals or entities, prohibits, among other things, knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent, knowingly making, using or causing to be made or used, a false record or statement material to a false or fraudulent claim, or from knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws. Moreover, the government may assert that a claim including items and services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal False Claims Act;
- HIPAA, which imposes criminal and civil liability, prohibits, among other things, knowingly and willfully executing, or attempting to execute a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services; similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- HIPAA, as amended by HITECH, which impose obligations on certain healthcare providers, health plans, and healthcare clearinghouses, known as covered entities, as well as their business associates that perform certain services involving the storage, use or disclosure of individually identifiable health information, including mandatory contractual terms, with respect to safeguarding the privacy, security, and transmission of individually identifiable health information, and require notification to affected individuals and regulatory authorities of certain breaches of security of individually identifiable health information;
- the federal legislation commonly referred to as the Physician Payments Sunshine Act, enacted as part of the ACA, and its implementing regulations, which requires certain manufacturers of covered drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children's Health Insurance Program, with certain exceptions, to report annually to CMS information related to certain payments and other transfers of value to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), physician assistants, certain types of advanced care practice nurses and teaching hospitals, as well as ownership and investment interests held by the physicians described above and their immediate family members, with the information made publicly available on a searchable website;

- the U.S. Foreign Corrupt Practices Act of 1977, as amended, which prohibits, among other things, U.S. companies and their employees and agents from authorizing, promising, offering, or providing, directly or indirectly, corrupt or improper payments or anything else of value to foreign government officials, employees of public international organizations and foreign government owned or affiliated entities, candidates for foreign political office, and foreign political parties or officials thereof;
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, that may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third party payors, including private insurers; and
- certain state and local laws that, among other things, require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government; require drug and therapeutic biologics manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures; require manufacturers to report price increases that exceed a statutory threshold, as well as information on the reasons for the price increase; require manufacturers to report the introduction into the market of costly drugs; require the registration of pharmaceutical sales representatives; and govern the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

If we or our collaborators, manufacturers or service providers fail to comply with applicable federal, state or foreign laws or regulations, we could be subject to enforcement actions, which could affect our ability to develop, market and sell our products successfully and could harm our reputation and lead to reduced acceptance of our products by the market. These enforcement actions include, not only civil and criminal penalties, but also exclusion from participation in government-funded healthcare programs, and exclusion from eligibility for the award of government contracts for our products.

Efforts to ensure that our current and future business arrangements with third parties comply with applicable healthcare laws and regulations could involve substantial costs. It is possible that government authorities will conclude that our business practices do not comply with current or future statutes, regulations, agency guidance 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 such requirements, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, the curtailment or restructuring of our operations, loss of eligibility to obtain approvals from the FDA, exclusion from participation in government contracting, healthcare reimbursement or other government programs, including Medicare and Medicaid, integrity oversight and reporting obligations, or reputational harm, any of which could adversely affect our financial results. Although effective compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, these risks cannot be entirely eliminated. Any action against us for an alleged or suspected violation could cause us to incur significant legal expenses and could divert our management's attention from the operation of our business, even if our defense is successful. In addition, achieving and sustaining compliance with applicable laws and regulations may be costly to us in terms of money, time and resources.

Disruptions at the FDA or other comparable foreign regulatory authorities may also slow the time necessary for new products to be reviewed and/or approved, which would adversely affect our business. In addition, there is substantial uncertainty regarding the new Administration's initiatives and how these might impact the FDA, its implementation of laws, regulations, policies and guidance and its personnel. These initiatives could prevent, limit or delay development and regulatory approval of our product candidates, which would adversely affect our business.

Disruptions at the FDA or other comparable foreign regulatory authorities may also slow the time necessary for new products to be reviewed and/or approved, which would adversely affect our business. Changes in FDA staffing could result in delays in the FDA's responsiveness or in its ability to review submissions or applications, issue regulations or guidance, or implement or enforce regulatory requirements in a timely fashion or at all. Similar consequences would also result in the event of another significant shutdown of the federal government. For example, in 2024, the U.S. government was on the verge of a shutdown and has previously shut down several times, and certain regulatory agencies, such as the FDA, had to furlough critical employees and stop critical activities. If a prolonged government shutdown occurs, or if geopolitical or global health concerns prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns or delays could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations. If the FDA is constrained in its ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

In addition, FDA-regulated industries, such as ours, face substantial uncertainty in regard to the regulatory environment we will face as we proceed with research and development efforts following the inauguration of President Trump in January 2025.

Some of these efforts have manifested to date in the form of personnel measures that could impact the FDA's ability to hire and retain key personnel, which could result in delays or limitations on our ability to obtain guidance from the FDA on our product candidates in development and obtain the requisite regulatory approvals in the future. There remains general uncertainty regarding future activities. The new Administration could issue or promulgate executive orders, regulations, policies or guidance that adversely affect us or create a more challenging or costly environment to pursue the development of new therapeutic products. Alternatively, state governments may attempt to address or react to changes at the federal level with changes to their own regulatory frameworks in a manner that is adverse to our operations. If we become negatively impacted by future governmental orders, regulations, policies or guidance as a result of the new Administration, there could be a material adverse effect on us and our business.

We are subject to environmental, health, and safety laws and regulations, which could increase our costs or restrict our operations.

Our operations are subject to a variety of environmental, health, and safety laws and regulations, including those of the EPA, OSHA, and equivalent local, state, and national regulatory agencies in the jurisdictions in which we operate. Any failure by us to comply with environmental, health, and safety requirements could result in the limitation or suspension of production or subject us to monetary fines, civil or criminal sanctions, or other future liabilities in excess of our reserves. In particular, we are subject to laws and regulations governing the destruction and disposal of raw materials, byproducts of our manufacturing operations, and non-compliant products, the handling of regulated material included in our offerings, and the disposal of our products or their components at the end of their useful lives. In addition, compliance with environmental, health, and safety requirements could restrict our ability to expand our facilities or require us to acquire costly environmental or safety control equipment, incur other significant expenses, or modify our manufacturing processes. Our manufacturing facilities may use, in varying degrees, hazardous substances in their processes. These substances include, among others, chlorinated solvents, and in the past chlorinated solvents were used at one or more of our facilities, including a number we no longer own or operate. As at our current facilities, contamination at such formerly owned or operated properties can result and has resulted in liability to us. In the event of the discovery of new or previously unknown contamination either at our facilities, facilities we acquire in the future, or at third party locations, including facilities we formerly owned or operated, the issuance of additional requirements with respect to existing contamination, or the imposition of other cleanup obligations for which we are responsible, we may be required to take additional, unplanned remedial measures for which we have not recorded reserves. We are conducting monitoring and cleanup of contamination at certain facilities currently or formerly owned or operated by us, and such activities may result in unanticipated costs or management distraction.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain patent protection for our technology and products, 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, trademarks, trade secret protection, and confidentiality agreements to protect the intellectual property related to our drug development programs, products, and product candidates. Our success depends in large part on our ability to obtain and maintain patent protection in the U.S. and other countries with respect to our drug candidates. We seek to protect our proprietary position by filing patent applications in the U.S. and abroad related to our development programs and product 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.

The patent applications that we own or have licensed may fail to result in issued patents with claims that protect our drug products in the U.S. 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 prevent a patent from issuing from a pending patent application or be used to invalidate a patent. Even if patents do successfully issue and even if such patents cover our drug products, 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 product 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 product candidate under patent protection could be reduced.

If the patent applications we hold or have in-licensed with respect to our development programs, products, and product 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 products or candidates, it could dissuade companies from collaborating with us to develop product candidates, and threaten our ability to commercialize future drugs. Any such outcome could have a materially adverse effect on our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions, and has been and will continue to be the subject of litigation and new legislation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the U.S. For example, many countries restrict the

patentability of methods of treatment of the human body. Publications of discoveries in scientific literature often lag behind the actual discoveries, and patent applications in the U.S. 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 of these and other factors, 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 products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the U.S. and other countries may diminish the value of our patents or narrow the scope of our patent protection.

Moreover, we may be subject to a third party pre-issuance submission of prior art to the U.S. Patent and Trademark Office (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. The costs of defending our patents or enforcing our proprietary rights in post-issuance administrative proceedings and litigation can be substantial and the outcome can be uncertain. 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 products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products 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 product 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 U.S. and abroad. Such challenges may result in loss of exclusivity or freedom to operate 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 products, or limit the duration of the patent protection of our technology and products. Generally, issued patents are granted a term of 20 years from the earliest claimed non-provisional filing date. In certain instances, patent term can be adjusted to recapture a portion of delay by the USPTO in examining the patent application (patent term adjustment) or extended to account for term effectively lost as a result of the FDA regulatory review period (patent term extension), or both. The scope of patent protection may also be limited. Without patent protection for our current or future product candidates, we may be open to competition from generic versions of such products. Given the amount of time required for the development, testing, and regulatory review of new product 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 products similar or identical to ours.

Our success will depend significantly on our ability to operate without infringing the patents and other proprietary rights of third parties.

Our commercial success depends in part on our avoiding infringement and other violations of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the U.S., involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, derivation, and administrative law proceedings, inter partes review, and post-grant review before the USPTO, as well as oppositions and similar processes in foreign jurisdictions. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we and our collaborators are developing product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, and as we gain greater visibility and market exposure as a public company, the risk increases that our products, product candidates or other business activities may be subject to claims of infringement of the patent and other proprietary rights of third parties. Third parties may assert that we are infringing their patents or employing their proprietary technology without authorization. Also, there may be third party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our products or product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our products or product candidates may infringe.

In addition, third parties may obtain patent rights in the future and claim that use of our technologies infringes upon rights. If any third party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our products or product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such products or product candidates unless we obtained a license under the applicable patents, or until such patents expire. Similarly, if any third party patent were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patent may be able to block our ability to develop and commercialize the applicable products or product candidates unless we obtained a license or until such patent expires. In either case, such a license may not be available on

commercially reasonable terms or at all. In addition, we may be subject to claims that we are infringing other intellectual property rights, such as trademarks or copyrights, or misappropriating the trade secrets of others, and to the extent that our employees, consultants or contractors use intellectual property or proprietary information owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our products or product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful infringement or other intellectual property claim against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our affected products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of our products or product candidates, and we may do so from time to time. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize one or more of our products or product candidates, which could harm our business significantly. We cannot provide any assurances that third party patents do not exist which might be enforced against our products or product candidates, resulting in either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties or other forms of compensation to third parties.

We may incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights.

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 U.S., 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, enablement, written description, or patentable 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 U.S., 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 products, or current or future product 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 U.S. 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 shares.

General Risk Factors

Our business may require additional capital.

We may need to raise additional capital in the future in order to fund our business (particularly to fund potential company or product acquisitions that are intended to expand our product offerings). If necessary, we would likely raise additional funds in

the future through public or private equity offerings, debt financings, corporate collaborations, or other means. We may also seek governmental grants to support our clinical and pre-clinical trials. However, there is no assurance that any such funding will be available, and, even if it is available, whether it will be available on terms that are favorable to us. We may also seek to raise additional capital to fund additional product development efforts, even if we have sufficient funds for our planned operations.

Any sale by us of additional equity or debt securities convertible into additional equity could result in dilution to our stockholders. Further, to the extent that we raise funds through collaborative arrangements, it may be necessary to relinquish some rights to our technologies or grant sublicenses on terms that are not favorable to us. If we are not able to secure funding when needed, we may have to delay, reduce the scope of or eliminate one or more research and development programs, which could have an adverse effect on our business.

The obligations incident to being a public company place significant demands on our management.

As a public reporting company, we are required to comply with the Sarbanes-Oxley Act of 2002 and the related rules and regulations of the SEC, including periodic reports, disclosures and more complex accounting rules. As directed by Section 404 of the Sarbanes-Oxley Act, the SEC adopted rules requiring public companies to include a report of management on a company's internal control over financial reporting in their Annual Report on Form 10-K. Based on current rules, we are required to annually report under Section 404(a) of the Sarbanes-Oxley Act regarding our management's assessment as to the effectiveness of our internal control over financial reporting. Further, under Section 404(b) of the Sarbanes-Oxley Act, our auditors are required to report on their assessment as to the effectiveness of our internal control over financial reporting. If we or our auditors are unable to conclude that we have effective internal control over our financial reporting, investors could lose confidence in the reliability of our consolidated financial statements, which could result in a decrease in the value of our common stock.

Our business and operations could suffer in the event of system failures or security or data breaches due to cyber-attacks, or cyber intrusions, including ransomware, phishing attacks and other malicious intrusions.

In recent years, cybersecurity threats have become a greater risk and focus for companies. In particular, ransomware attacks, where a hacker locks and threatens to delete or disclose the victim's data unless a ransom is paid, has become a major risk. We and our third party service providers are at risk of cyber-attacks or cyber intrusions via the Internet, computer viruses, break-ins, malware, ransomware, phishing attacks, hacking, denial-of-service attacks or other attacks and similar disruptions from the unauthorized use of, or access to, computer systems (including from internal and external sources). These types of incidents continue to be prevalent and pervasive across industries, including in our industry. In addition, we expect information security risks to continue to increase due to the proliferation of new technologies and the increased sophistication and activities of organized crime, hackers, terrorists and other external parties, including foreign state actors.

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 collect, process, store and transmit large amounts of confidential information, including intellectual property, proprietary business information and personal information. It is critical that we do so in a secure manner to maintain the confidentiality and integrity of such information. The size and complexity of our information technology systems, and those of third party vendors with whom we contract, and the volume of data we retain, make such systems potentially vulnerable to breakdown, malicious intrusion, security breaches, ransomware, phishing, and other cyber-attacks. Our information security systems and those of our third party vendors are subject to laws and regulations, or may become subject to new laws and regulations, requiring that we enact certain measures to protect the privacy and security of certain information we collect or use in our business. A security breach or privacy violation that leads to disclosure or modification of, or prevents access to, personal information or other protected information, whether caused by internal or external parties, could harm our reputation, compel us to comply with federal and/or state breach notification laws and foreign law equivalents, subject us to notification requirements under certain agreements with third parties, subject us to mandatory corrective action, require us to verify the correctness of database contents and otherwise subject us to liability under laws and regulations that protect personal information, resulting in increased costs or loss of revenue. Similarly, the loss or unauthorized disclosure of clinical trial data from completed, ongoing or planned clinical trials could prevent us from obtaining regulatory approval or delay our regulatory approval efforts and significantly increase our costs to recover or reproduce the data.

If we are unable to prevent such security breaches or privacy violations or implement satisfactory remedial measures, our operations could be disrupted, and we may suffer negative impact to our reputation, financial loss and be subject to regulatory fines and penalties. In addition, breaches and other unauthorized data access can be difficult to detect, and any delay in identifying them may lead to increased harm of the type described above. Moreover, the reliance on remote working technologies by our employees and third party partners due to COVID-19 and related public health safety measures and the prevalent use of mobile devices that access confidential and personal information increases the risk of data security breaches, which could lead to the loss of confidential information, personal information, trade secrets or other intellectual property. As cyber threats continue to evolve, we may be required to expend significant additional resources to continue to modify or

enhance our protective measures or to investigate and remediate any information security vulnerabilities. While we have implemented security measures to protect our data security and information technology systems, such measures may not prevent such events. Significant disruptions of our information technology systems or breaches of data security could have a material adverse effect on our business, financial condition and results of operations.

We are highly dependent on our small number of key personnel and advisors.

We are highly dependent on our executive officers and key employees, and on our Board of Directors. The loss of the services of one or more of these individuals could significantly impede the achievement of our scientific and business objectives. We have no employment or retention agreements with any of our other officers or key employees. If we lose the services of any of our existing executive officers or key employees, or if we were unable to recruit qualified replacements on a timely basis for persons who leave our employ, our efforts to develop our drug candidates might be significantly delayed. We do not carry key-man insurance on any of our personnel.

We face a risk of product liability claims and may not be able to obtain adequate insurance.

Our business exposes us to potential risks that may arise from the clinical testing, manufacture, and/or sale of our pharmaceutical products. Patients have received substantial damage awards in some jurisdictions against pharmaceutical companies based on claims for injuries allegedly caused by the use of pharmaceutical products used in clinical trials or after FDA approval.

Liability claims may be expensive to defend and may result in large judgments against us. We currently carry liability insurance that we believe to be adequate. Our insurance may not reimburse us for certain claims or the coverage may not be sufficient to cover claims made against us. We cannot predict all of the possible harms or side effects that may result from the use of our current drug candidates, or any potential future products we may acquire and use in clinical trials or after FDA approval and, therefore, the amount of insurance coverage we currently hold may not be adequate to cover all liabilities we might incur. If we are sued for any injury allegedly caused by our products, our liability could exceed our ability to pay the liability. Whether or not we are ultimately successful in any adverse litigation, such litigation could consume substantial amounts of our financial and managerial resources, all of which could have a material adverse effect on our business, financial condition, results of operations, prospects and stock price.

Business or economic disruptions or global health concerns could seriously harm our development efforts and increase our costs and expenses.

Broad-based business or economic disruptions could adversely affect our ongoing or planned research and development activities. Global health concerns could also result in social, economic, and labor instability in the countries in which we or the third parties with whom we engage operate. We cannot presently predict the scope and severity of any potential business shutdowns or disruptions, but if we or any of the third parties with whom we engage, including the suppliers, clinical trial sites, regulators and other third parties with whom we conduct business, were to experience shutdowns or other business disruptions, our ability to conduct our business in the manner and on the timelines presently planned could be materially and negatively impacted. It is also possible that global health concerns could disproportionately impact the hospitals and clinical sites in which we conduct any of our clinical trials, which could have a material adverse effect on our business and our results of operation and financial condition.

We anticipate being subject to increasing focus by our investors, regulators, customers, and stakeholders on environmental, social & governance (ESG) matters.

Our investors, regulators, and other stakeholders have in the last few years increasingly focused on ESG matters. Certain investors, particularly institutional investors, may use third party benchmarks or scores to measure our ESG practices, and to decide whether to invest in our shares, engage with us regarding our practices, or engage or continue to use our services. If our ESG scores or practices do not meet desired standards, we may face reputational challenges. There can be no assurance that we will be able to accomplish any particular ESG goal or commitment, including any additional or revised commitment that we may announce in the future, as statements regarding such goals and commitments reflect our plans and aspirations at the time of announcement and do not guarantee achievement of such plans and aspirations within the timelines we announce or at all.

Different stakeholder groups have divergent views on ESG matters, which increases the risk that any action or lack thereof with respect to ESG matters will be perceived negatively by at least some stakeholders and adversely impact our reputation and business. Anti-ESG sentiment has gained some momentum across the U.S., with several states having enacted or proposed "anti-ESG" policies or legislation, or issued related legal opinions. If we do not successfully manage ESG-related expectations across these varied stakeholder interests, it could erode stakeholder trust, impact our reputation, and constrain our business. Globally, a lack of harmonization in relation to ESG legal and regulatory reform across the jurisdictions in which we may operate may affect our future implementation of, and compliance with, rapidly developing ESG standards and requirements. Generally, we expect stakeholder demands and the prevailing legal environment to require us to devote additional resources to

ESG matters in our review of prospective acquisitions. Additionally, collecting, measuring, and reporting ESG information and metrics can be costly, difficult, and time-consuming, are subject to evolving reporting standards, and can present numerous operational, reputational, financial, legal, and other risks. Compliance with ESG-related rules and efforts to meet investor expectations on ESG matters may place strain on our personnel, systems, and resources, and we may incur significant compliance costs. Additionally, failure to comply with such rules or meet investor expectations may have a material adverse impact on our business, prospects, financial condition, or results of operations.

The trading price of the shares of our common stock has been and could in the future be highly volatile.

The market price of our common stock has fluctuated in the past and is likely to fluctuate in the future. Market prices for biopharmaceutical companies have historically been particularly volatile. Some of the factors that may cause the market price of our common stock to fluctuate include:

- developments concerning our clinical studies and trials and our pre-clinical studies;
- status of regulatory requirements for approval of our drug candidates;
- adverse publicity regarding the pricing our drug products;
- announcements of product development successes and failures by us or our competitors;
- new products introduced or announced by us or our competitors;
- adverse changes in the abilities of our third party manufacturers to provide drug or product in a timely manner or to meet FDA requirements;
- challenges to our intellectual property which could affect our products, such as the currently pending litigation involving Paragraph IV challenges to FIRDAPSE®;
- changes in reimbursement levels;
- changes in financial estimates by securities analysts;
- actual or unanticipated variations in operating results;
- changes in laws regarding FDA approval;
- expiration or termination of licenses (particularly our License Agreement for FIRDAPSE®), research contracts, or other collaboration agreements;
- conditions or trends in the regulatory climate and the biotechnology and pharmaceutical industries;
- intellectual property, product liability or other litigation against us;
- changes in the market valuations of similar companies;
- changes in pharmaceutical company regulations or reimbursements for pharmaceutical products as a result of healthcare reform or other legislation;
- changes in economic conditions; and
- sales of shares of our common stock, particularly sales by our officers, directors and significant stockholders, or the perception that such sales may occur.

In addition, equity markets in general, and the market for emerging pharmaceutical and life sciences companies in particular, have experienced substantial price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of companies traded in those markets. Further, changes in economic conditions in the U.S., Europe, or globally could impact our ability to grow profitably. Adverse economic changes are outside our control and may result in material adverse impacts on our business or financial results. These broad market and industry factors may materially affect the market price of our shares, regardless of our own development and operating performance. In the past, following periods of volatility in the market price of a company's securities, securities class-action litigation has often been instituted against that company. Any such litigation that we become involved in could cause us to incur substantial costs and divert our management's attention and resources, which could have a material adverse effect on our business, financial condition, and results of operations.

Delaware law and our certificate of incorporation and by-laws contain provisions that could delay and discourage takeover attempts that stockholders may consider favorable.

Certain provisions of our certificate of incorporation and by-laws, and applicable provisions of Delaware corporate law, may make it more difficult for or prevent a third party from acquiring control of us or changing our Board of Directors and management. These provisions include:

- the ability of our Board of Directors to issue preferred stock with voting or other rights or preferences;
- limitations on the ability of stockholders to amend our charter documents, including stockholder supermajority voting requirements;
- the inability of stockholders to act by written consent or to call special meetings;
- requirements that special meetings of our stockholders may only be called by the Board of Directors; and
- advance notice procedures our stockholders must comply with in order to nominate candidates for election to our Board of Directors or to place stockholders' proposals on the agenda for consideration at meetings of stockholders.

In addition, Section 203 of the Delaware General Corporation Law generally prohibits us from engaging in a business combination with any person who owns 15% or more of our common stock for a period of three years from the date such person acquired such common stock, unless Board or stockholder approval is obtained. These provisions could make it difficult for a third party to acquire us, or for members of our Board of Directors to be replaced, even if doing so would be beneficial to our stockholders.

Any delay or prevention of a change of control transaction or changes in our Board of Directors or management could deter potential acquirers or prevent the completion of a transaction in which our stockholders could receive a substantial premium over the then current market price for their shares.

Future sales of our common stock may cause our stock price to decline.

As of February 24, 2025, we had 121,449,655 shares of our common stock outstanding, of which 7,642,725 shares were held by our executive officers and directors. We also had outstanding: (i) stock options to purchase an aggregate of 13,077,867 shares at exercise prices ranging from $2.11 to $22.90 (7,582,868 of which are currently exercisable); and (ii) restricted stock units for 575,049 shares of common stock (none of which are currently vested).

Sales of shares, 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.

We do not intend to pay cash dividends on our common stock in the foreseeable future.

We have never declared or paid any cash dividends on our common stock or other securities, and we currently do not anticipate paying any cash dividends in the foreseeable future. Accordingly, investors should not invest in our common stock if they require dividend income. Our stockholders will not realize a return on their investment unless the trading price of our common stock appreciates, which is uncertain and unpredictable.

Item 1B. Unresolved Staff Comments

None.

Item 1C. Cybersecurity

Risk management and strategy

We recognize the critical importance of developing, implementing, and maintaining robust cybersecurity measures to safeguard our information systems and protect the confidentiality, integrity, and availability of our data.

Managing Material Risks & Integrated Overall Risk Management

We have integrated cybersecurity risk management into our broader risk management framework to promote a company-wide culture of cybersecurity risk management. This integration ensures that cybersecurity considerations are a part of our decision-making processes at every level. Our risk management team works closely with our Information Technology (IT) team including our IT and cybersecurity vendors to continuously evaluate and address cybersecurity risks in alignment with our business objectives and operational needs.

Engage Third Parties on Risk Management

Recognizing the complexity and evolving nature of cybersecurity threats, we engage with a range of external experts in evaluating and testing our risk management systems. These partnerships enable us to leverage specialized knowledge and

insights, ensuring our cybersecurity strategies and processes remain at the forefront of industry best practices. Our collaboration with these third parties includes regular audits, threat assessments, and consultation on security enhancements.

Oversee Third Party Risk

Because we are aware of the risks associated with third party service providers, we implement stringent processes to oversee and manage these risks. We conduct thorough security assessments of all third party providers before engagement and maintain ongoing monitoring to ensure compliance with our cybersecurity standards. The monitoring includes quarterly assessments by our Chief Legal and Compliance Officer (CLCO) and our Chief Operating Officer (COO) and on an ongoing basis by our IT professionals. This approach is designed to mitigate risks related to data breaches or other security incidents originating from third parties.

Risks from Cybersecurity Threats

We have not encountered, to date, cybersecurity challenges that have materially impaired our operations or financial standing.

Governance

The Board of Directors is acutely aware of the critical nature of managing risks associated with cybersecurity threats. The Board has established robust oversight mechanisms to ensure effective governance in managing risks associated with cybersecurity threats because we recognize the significance of these threats to our operational integrity and stakeholder confidence.

Risk Management Personnel

Primary responsibility for assessing, monitoring and managing our cybersecurity risks rests with our CLCO, our COO, and our IT personnel. In their time with our company, our CLCO and our COO have become increasingly involved in investigating, responding to, and mitigating cybersecurity incidents and intrusion attempts. Their in-depth knowledge and experience are instrumental in developing and executing our cybersecurity strategies. Our CLCO and our COO oversee our governance programs, test our compliance with standards, remediate known risks, and oversee or lead our employee training program.

The CLCO and the COO regularly inform our CEO and CFO of all aspects related to cybersecurity risks and incidents. This ensures that the highest levels of management are kept abreast of the cybersecurity posture and potential risks facing our company. Furthermore, significant cybersecurity matters, and strategic risk management decisions are escalated to the Board of Directors, ensuring that they have comprehensive oversight and can provide guidance on critical cybersecurity issues.

Monitor Cybersecurity Incidents

Our CLCO, our COO, and our IT personnel are periodically informed about the latest developments in cybersecurity, including potential threats and innovative risk management techniques. This ongoing knowledge acquisition is crucial for the effective prevention, detection, mitigation, and remediation of cybersecurity incidents. Our CLCO and COO implement and oversee processes for the regular monitoring of our information systems. This includes the deployment of advanced security measures and regular system audits to identify potential vulnerabilities. In the event of a cybersecurity incident, the CLCO and COO, along with our IT personnel, are equipped with a well-defined incident response plan. This plan includes immediate actions to mitigate the impact and long-term strategies for remediation and prevention of future incidents.

Management's Role Managing Risk

The CLCO and the COO play a pivotal role in informing the Board of Directors about cybersecurity risks. They provide comprehensive briefings to the Board of Directors, with a minimum frequency of not less than once per year. These briefings encompass a broad range of topics, including:

- Current cybersecurity landscape and emerging threats;
- Status of ongoing cybersecurity initiatives and strategies;
- Incident reports and learnings from any cybersecurity events; and
- Compliance with regulatory requirements and industry standards.

In addition, at regular meetings of the Board, the Board members, including the CEO, and the CLCO and COO maintain an ongoing dialogue regarding emerging or potential cybersecurity risks. Together, they receive updates on any significant developments in the cybersecurity domain, ensuring the Board's oversight is proactive and responsive. The Board members actively participate in strategic decisions related to cybersecurity, offering guidance and approval for major initiatives. This involvement ensures that cybersecurity considerations are integrated into our broader strategic objectives. The Board of Directors conducts an annual review of the company's cybersecurity posture and the effectiveness of its risk management strategies. This review helps in identifying areas for improvement and ensuring the alignment of cybersecurity efforts with the overall risk management framework.

Board of Directors Oversight

The Board of Directors as a group is responsible for oversight of cybersecurity risks and bears the primary responsibility for oversight of this domain. The Board has delegated to the Audit Committee the primary responsibility of overseeing risk management relating to cybersecurity. The Board of Directors is composed of board members with diverse expertise including, risk management, technology, and finance, equipping them to oversee cybersecurity risks effectively.

Item 2. Properties

We currently operate our business in 10,700 square feet of leased office space in Coral Gables, Florida. Our current annual rent in the new space is approximately $0.5 million.

Item 3. Legal Proceedings

Paragraph IV Patent Litigation

For a description of currently ongoing Paragraph IV litigation related to FIRDAPSE®, see "Item 1. Business – Overview – FIRDAPSE®." The outcome of patent litigation with Paragraph IV challengers is always uncertain and there can be no assurance as to whether we will prevail in this litigation.

Other Litigation

From time to time we may become involved in legal proceedings arising in the ordinary course of business. Other than as set forth above, we believe that there is no litigation pending at this time that could have, individually or in the aggregate, a material adverse effect on our results of operations, financial condition or cash flows.

Item 4. Mine Safety Disclosure

Not applicable.

PART II

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

Market Information

Shares of the Company's common stock are traded on the Nasdaq Capital Market under the symbol "CPRX". There were 17 holders of record of the Company's common stock as of February 24, 2025.

Dividends

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

Recent Sales of Unregistered Securities

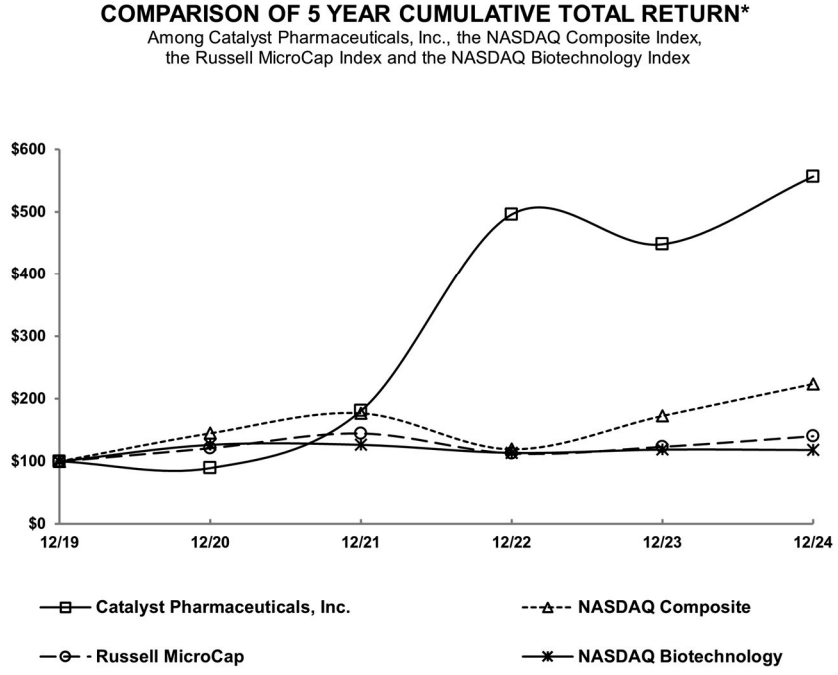
None.

Repurchases of Equity Securities

None.

Performance Graph

The following performance graph shall not be deemed "soliciting material" or to be "filed" with the SEC, nor shall such information be incorporated by reference into any future filing under the Securities Act of 1933 or Securities Exchange Act of 1934, each as amended, except to the extent that we specifically incorporate it by reference into such filing. The graph compares the cumulative 5-year total return to stockholders on the Company's common stock relative to the cumulative total returns of the Nasdaq Composite Index and the Nasdaq Biotechnology Index. The Company selected the Nasdaq Biotechnology Index because it believes the index reflects the market conditions within the industry in which the Company primarily operates. The comparison of total return on investment, defined as the change in year-end stock price plus reinvested dividends, for each of the periods assumes that $100 was invested on January 1, 2020, in each of the Company's common stock, the Nasdaq Composite Index and the Nasdaq Biotechnology Index, with investment weighted on the basis of market capitalization.



	12/19	12/20	12/21	12/22	12/23	12/24
Catalyst Pharmaceuticals, Inc.	**100.00**	**89.07**	**180.53**	**496.00**	**448.27**	**556.53**
NASDAQ Composite	**100.00**	**144.92**	**177.06**	**119.45**	**172.77**	**223.87**
Russell MicroCap	**100.00**	**120.96**	**144.35**	**112.66**	**123.17**	**140.05**
NASDAQ Biotechnology	**100.00**	**126.42**	**126.45**	**113.65**	**118.87**	**118.20**

The stock price performance included in this graph is not necessarily indicative of future stock price performance.

Securities Authorized for Issuance under Equity Compensation Plans

The following table presents information as of December 31, 2024 with respect to compensation plans under which shares of our common stock may be issued.

	Equity Compensation Plan Information		
Plan Category	**Number of securities to be issued upon exercise of outstanding options, warrants, and rights**	**Weighted-average exercise price of outstanding options, warrants, and rights**	**Number of securities remaining available for equity compensation plans**
Equity compensation plans approved by security holders [(1)]	13,076,789	$ 10.76	2,254,102[(2)]
Equity compensation plans not approved by security holders	—	—	—
Total	**13,076,789**	**$ 10.76**	**2,254,102**

(1) Includes our 2014 Stock Incentive Plan and our 2018 Stock Incentive Plan

(2) Remaining shares are only under our 2018 Stock Incentive Plan

Item 6. Selected Financial Data

Not applicable.

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

The following discussion of our financial condition and results of operations should be read together with the Consolidated Financial Statements and related notes included elsewhere in this Annual Report on Form 10-K. This discussion and analysis may contain forward-looking statements that involve certain risks, assumptions and uncertainties that could cause actual results to differ materially from those implied or described by the forward-looking statements. Future results could differ materially from the discussion that follows for many reasons, including the factors described in Part I, Item 1A. "Risk Factors" in this Annual Report on Form 10-K, as well as those described in future reports filed with the SEC.

Introduction

Management's Discussion and Analysis of Financial Condition and Results of Operations (MD&A) is intended to provide an understanding of our financial condition, changes in financial condition and results of operations. The discussion and analysis is organized as follows:

- Overview. This section provides a general description of our business and information about our business that we believe is important in understanding our financial condition and results of operations.
- Basis of Presentation. This section provides information about key accounting estimates and policies that we followed in preparing our consolidated financial statements for the 2024 fiscal year.
- Critical Accounting Policies and Estimates. This section discusses those accounting policies that are both considered important to our financial condition and results of operations and require significant judgment and estimates on the part of management in their application. All of our significant accounting policies, including the critical accounting policies, are also summarized in the notes to our accompanying consolidated financial statements.
- Results of Operations. This section provides an analysis of our results of operations for the three fiscal years presented in the accompanying consolidated statements of operations and comprehensive income.
- Liquidity and Capital Resources. This section provides an analysis of our cash flows, capital resources, off-balance sheet arrangements, and our outstanding commitments, if any.
- Caution Concerning Forward-Looking Statements. This section discusses how certain forward-looking statements made throughout this MD&A and in other sections of this report are based on management's present expectations about future events and are inherently susceptible to uncertainty and changes in circumstance.

Overview

We are a commercial-stage, patient-centric biopharmaceutical company focused on in-licensing, developing, and commercializing novel high-quality medicines for patients living with rare and difficult to treat diseases. We currently market three drug products, FIRDAPSE® (amifampridine), FYCOMPA® (perampanel), and AGAMREE® (vamorolone). We are also currently seeking to further expand our drug portfolio, with a focus on acquiring the rights to late-stage products to treat orphan, rare diseases across therapeutic areas. With an unwavering patient focus embedded in everything we do, we are committed to providing innovative, best-in-class medications with the hope of making a meaningful impact on those affected by these conditions.

FIRDAPSE®

On November 28, 2018, we received approval from the FDA for our new drug application, (NDA) for FIRDAPSE® Tablets 10 mg for the treatment of adult patients (ages 17 and above) with LEMS, and in January 2019, we launched FIRDAPSE® in the U.S. Further, on September 29, 2022, the FDA approved our supplemental NDA (sNDA) to expand the indicated age range for FIRDAPSE® Tablets 10 mg for the treatment of LEMS to include pediatric patients six years of age and older. Finally, on May 30, 2024, the FDA approved our sNDA increasing the indicated maximum daily dosage of FIRDAPSE® tablets for the treatment of patients with LEMS from 80 mg to 100 mg. We believe that this recent sNDA approval offers healthcare providers and patients greater flexibility in treatment regimens for the management of LEMS.

We sell FIRDAPSE® in the U.S. through a field-based force experienced in neurologic, central nervous system or rare disease products consisting at this time of approximately 41 field personnel, including sales (Regional Account Managers), thought leader liaisons and patient assistance and insurance navigation support (Patient Access Liaisons). These field personnel have also supported AGAMREE® since its commercial launch in March 2024. We also have a field-based force of 10 medical science liaisons who are helping educate the medical community about scientific literature concerning LEMS and FIRDAPSE®. Additionally, we use non-personal promotion to reach the 20,000 neurologists who are potential LEMS treaters and the 16,000 oncologists who might be treating a LEMS patient who also has small cell lung cancer. Further, we continue to make available at no-cost a LEMS voltage gated calcium channel antibody diagnostic testing program for use by physicians who suspect that one of their patients may have LEMS and wish to reach a definitive diagnosis.

When we launched AGAMREE®, in March 2024, we utilized the FIRDAPSE® commercial and medical field-based forces to market AGAMREE® as well. In early 2025, we made a strategic decision to split each of these field-based forces into two units, one for each function expressly focused on supporting FIRDAPSE® and one for each function expressly focused on supporting AGAMREE®. This change is being made in an effort to allow us to better focus our sales teams on the market for each product. We expect to complete this division of our field-based forces into two units early in the second quarter of 2025.

Finally, we are continuing to expand our digital and social media activities to introduce our products and services to potential patients and their healthcare providers. We also work with several rare disease advocacy organizations (including the Myasthenia Gravis Foundation of America, the National Organization for Rare Disorders, and the LEMS Family Association) to help increase awareness and level of support for patients living with LEMS and to provide education for the physicians who treat these rare diseases and the patients they treat.

We are supporting the distribution of FIRDAPSE® through Catalyst Pathways®, our personalized treatment support program for patients who enroll in it. Catalyst Pathways® is a single source for personalized treatment support, education and guidance through the challenging dosing and titration regimen required to reach an effective therapeutic dose. The program also includes distributing the drug through a very small group of exclusive specialty pharmacies (primarily AnovoRx), which is consistent with the way that most drug products for ultra-orphan diseases are distributed and dispensed to patients. We believe that by using specialty pharmacies in this way, the difficult task of navigating the health care system is far better for the patient needing treatment for their rare disease and the health care community in general.

In order to help patients with LEMS afford their medication, we, like other pharmaceutical companies which market drug products for ultra-orphan conditions, have developed an array of financial assistance programs to reduce out-of-pocket costs that makes FIRDAPSE® accessible and affordable. A co-pay assistance program has been designed to reduce commercial patients' out of pocket costs to $0 whenever possible. Our FIRDAPSE® co-pay assistance program is not available to patients enrolled in state or federal healthcare programs, including Medicare, Medicaid, Department of Veterans Affairs (VA), Department of Defense (DoD), or TRICARE. However, we are donating, and committing to continue to donate, money to qualified, independent charitable foundations dedicated to providing assistance to any U.S. LEMS patients in financial need, who meet those independent organizations' guidelines. In addition, we have a safety net program in place for patients who are uninsured and underinsured. Subject to compliance with regulatory requirements, our goal is that no LEMS patient is ever denied access to their medication for financial reasons.

FIRDAPSE® is currently marketed for the treatment of LEMS in Canada through our exclusive sublicensee, KYE. We supply product to KYE at agreed upon prices and we are also eligible to earn sales milestones and sales royalties based on net revenues from sales of the product in Canada.

In December 2023, DyDo Pharma, Inc. (DyDo), our sub-licensee for FIRDAPSE® in Japan, filed a NDA with the Pharmaceuticals and Medical Devices Agency (PMDA) in Japan seeking approval to commercialize FIRDAPSE® for the treatment of LEMS in Japan. Upon acceptance of the Japan NDA by the PMDA in December 2023, our license for FIRDAPSE® automatically expanded to include other key markets in Asia and Latin America, and we are currently seeking opportunities to expand FIRDAPSE®'s global footprint through strategic partnerships (with the current focus on the Asia Pacific and Latin American regions). Further, in September 2024, DyDo advised us that the Ministry of Health, Labour and Welfare (MHLW) had approved DyDo's Japan NDA to commercialize FIRDAPSE® for the treatment of patients with LEMS in Japan. Finally, DyDo began commercialization of FIRDAPSE® in Japan on January 21, 2025. We will generate revenue through additional milestone payments and a transfer price on the product supplied by us to DyDo, in lieu of royalties.

We control six U.S. patents for FIRDAPSE® that are listed in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (Orange Book), the earliest of which expires in 2032 and the latest of which expires in 2037. We also have orphan drug exclusivity (ODE) for the product that will not expire until November 2025, and no ANDA for the product can be finally approved by the FDA until the ODE exclusivity period has expired. Nevertheless, generic drug manufacturers were permitted to submit applications for the product challenging our patents starting in 2023.

In that regard, in January 2023, we received Paragraph IV Certification Notice Letters from three generic drug manufacturers (Teva Pharmaceuticals USA, Inc. and Teva Pharmaceuticals, Inc. (collectively Teva), Hetero USA, Inc. (Hetero), and Lupin Pharmaceuticals, Inc. (Lupin)) advising that they had each submitted an ANDA to the FDA seeking authorization from the FDA to manufacture, use or sell a generic version of FIRDAPSE® in the U.S. The notice letters each alleged that the six patents protecting FIRDAPSE® that are listed in the Orange Book in connection with FIRDAPSE® are not valid, not enforceable, and/or will not be infringed by the commercial manufacture, use or sale of the proposed product described in these ANDA submissions. Under the Federal Food, Drug, and Cosmetic Act (FDCA), as amended by the Drug Price Competition and Patent Term Restoration Act of 1984, we had 45 days from receipt of the notice letters to determine if there were grounds to bring a lawsuit and, if so, to commence patent infringement lawsuits against these generic drug manufacturers in a federal district court, which would trigger a statutory stay precluding the FDA from final approval of the subject ANDA until May 2026 or entry of judgment holding the patents invalid, unenforceable, or not infringed, whichever occurs first in all cases (but not earlier

than the expiration of orphan drug exclusivity on November 28, 2025). In that regard, after conducting the necessary due diligence, we filed lawsuits on March 1, 2023 in the U.S. District Court for the District of New Jersey against each of the three generic drug manufacturers who notified us of their ANDA submissions, thus triggering the stay.

In June 2024, Lupin converted five of its Paragraph IV Certifications in its ANDA to Paragraph III certifications acknowledging the validity and their ANDA's infringement of five of those patents, the latest ending in 2034. We subsequently dismissed all of our claims against Lupin related to those five patents but maintain our claims against Lupin for the remaining Paragraph IV certification for U.S. Patent No. 10,626,088, which is the patent expiring in 2037, accordingly the litigation continues.

Further, on January 8, 2025, we reached a settlement with Teva in which Teva agreed not to market a generic version of FIRDAPSE® in the U.S. any earlier than February 25, 2035, if approved by the FDA, unless certain limited circumstances customarily included in these types of agreements occur. In accordance with the settlement agreement, the parties terminated all ongoing patent litigation between us and Teva regarding FIRDAPSE® patents pending in the U.S. District Court for the District of New Jersey.

The pending FIRDAPSE® patent litigation against the remaining defendants, Hetero (for FIRDAPSE®'s Orange Book-listed patents expiring in 2032, 2034 and 2037) and Lupin (only for FIRDAPSE® patent expiring in 2037), remains ongoing, and there can be no assurance as to whether the currently ongoing litigation with Hetero and Lupin will allow a generic version of FIRDAPSE® to be marketed in the U.S. prior to Teva's licensed entry into the market on February 25, 2035.

Finally, in October 2023, we received a Paragraph IV Certification Notice Letter from a fourth generic drug manufacturer (Inventia Life Science Pty Ltd (Inventia)), and we filed a similar lawsuit against that manufacturer in November 2023 in the U.S. District Court for the District of New Jersey. On July 30, 2024, we settled this patent litigation with Inventia for FIRDAPSE®. In this settlement, Inventia acknowledged both the validity of our FIRDAPSE® patents and also the infringement by the ANDA filer's product of our patents. As part of the settlement, Inventia also agreed not to commercialize its product until the earlier of all FIRDAPSE® patents expiration or the entry into the market of another ANDA product meeting certain conditions.

The outcome of patent litigation with Paragraph IV challengers is always uncertain and there can be no assurance as to whether we will prevail in these litigations.

FYCOMPA®

On December 17, 2022, we entered into an agreement with Eisai Co., Ltd. (Eisai) for the acquisition of the U.S. rights to FYCOMPA® (perampanel) CIII. FYCOMPA® is a selective non-competitive antagonist of AMPA receptors, the major subtype of ionotropic glutamate receptors. It was the first, and still the only, drug of its class to be approved for epilepsy. Studies suggest that AMPA receptor antagonism can lead to reduced overstimulation and anticonvulsant effects, as well as inhibiting seizure generation and spread. FYCOMPA® is a controlled substance and is approved with a box warning label. FYCOMPA® is used to treat certain types of focal onset seizures (seizures that involve only one part of the brain) in adults and children four years of age and older. It is also used in combination with other medications to treat certain types of primary generalized tonic-clonic seizures (also known as a "grand mal" seizure, a seizure that involves the entire body) in adults and children 12 years of age or older. Perampanel is in a class of medications called anticonvulsants. It works by decreasing abnormal electrical activity in the brain.

On January 24, 2023, we closed our acquisition of the U.S. rights to FYCOMPA®. In connection with the acquisition, we purchased Eisai's regulatory approvals and documentation, product records, intellectual property, inventory, and other matters relating to the U.S. rights for FYCOMPA®, in exchange for an upfront payment of $160 million in cash. We also agreed to pay Eisai royalty payments after patent protection for FYCOMPA® expires, which royalty payments will be reduced upon generic equivalents to FYCOMPA® entering the market.

In conjunction with the closing of the asset purchase, we entered into two additional agreements, a Transition Services Agreement (TSA) and a Supply Agreement. Under the Supply Agreement, Eisai agreed to manufacture FYCOMPA® for us for at least seven years at prices listed in the Supply Agreement (to be updated on a yearly basis), and under the TSA, a U.S. subsidiary of Eisai provided us with certain transitional services (which transition services ended on December 31, 2023).

We sell FYCOMPA® in the U.S. through a field-based force experienced in epilepsy products consisting at this time of approximately 27 field personnel, including sales (Regional Account Managers) and payor reimbursement (National Account Managers). We also have a field-based force of four medical science liaisons who are helping educate the medical community who treat epilepsy about scientific literature regarding epilepsy and FYCOMPA®. Further, since January 1, 2024, FYCOMPA® is being sold and distributed through a 3PL organization under our contracts.

We are currently taking steps to prepare for the loss of exclusivity for FYCOMPA®, which, assuming a timely ANDA approval of the respective generic applications, will take place on or after May 23, 2025 for the tablet version of the product and will

take place on or after December 15, 2025 for the oral suspension version of the product. We expect to continue to market the product following the loss of patent exclusivity.

We are supporting patients using FYCOMPA® through an Instant Savings Card Program. Through the program, eligible commercially insured patients could pay as little as $5 for their FYCOMPA® co-pay (with a maximum savings of $2,500 per year). The FYCOMPA® Instant Savings Card Program is not available to patients enrolled in state or federal healthcare programs, including Medicare, Medicaid, VA, DoD, or TRICARE.

Patent protection for FYCOMPA® tablets and oral solution is primarily derived from two patents listed in the FDA's Orange Book. The first, U.S. patent no. 6,949,571 (the '571 patent), will expire on May 23, 2025, including patent term extension. The second FYCOMPA® patent in the Orange Book is U.S. Patent No. 8,772,497 (the '497 patent), which will expire on July 1, 2026. The '497 patent, which covers the API used in both FYCOMPA® tablets and oral solution, has been the subject of previous Paragraph IV certifications from three ANDA filers for the tablet formulation, which were not contested by Eisai prior to our acquisition of the drug. Following our acquisition of the drug, we attempted to obtain an extension of the patent term for the '571 patent, which was ultimately unsuccessful. As a result, the '571 patent will expire on May 23, 2025 and the initial ANDA filers who did not challenge this patent may seek approval of their ANDA applications on or after that date.

In February 2023 we received a Paragraph IV certification for the '497 patent from an ANDA filer for two applications, one for the FYCOMPA® tablets and another for the FYCOMPA® oral suspension. After due diligence we filed lawsuits on April 5, 2023 in the U.S. District Court for the District of New Jersey against the drug manufacturer who notified us of their ANDA submissions alleging infringement of both patents. In June 2024, we settled the pending Paragraph IV litigation with the Paragraph IV filer for both ANDAs. As part of that settlement, this Paragraph IV filer agreed not to commercialize their proposed ANDA products for both the oral suspension formulation of FYCOMPA® and for FYCOMPA® tablets until at least December 15, 2025.

In January 2024, we received a Paragraph IV certification for the '571 and '497 patents from an ANDA filer for an application for the FYCOMPA® oral suspension. After due diligence, we determined that the circumstances and timeline did not warrant a lawsuit against this Paragraph IV filer.

AGAMREE®

On June 19, 2023, we entered into a License and Collaboration Agreement (AGAMREE® License Agreement) and an Investment Agreement (Investment Agreement) with Santhera Pharmaceuticals Holding, Inc. (Santhera). Under the AGAMREE® License Agreement, we contracted to obtain an exclusive North America license, manufacturing and supply agreement for Santhera's investigational product candidate, AGAMREE® (vamorolone), a novel corticosteroid for the treatment of DMD. Under the Investment Agreement, we agreed to make a strategic investment into Santhera.

Both transactions closed on July 18, 2023. Under the AGAMREE® License Agreement, upon closing we made a $75 million payment to Santhera in return for the exclusive North American license for AGAMREE®. In addition to the rights to commercialize the product in North America, the AGAMREE® License Agreement provides us with the right of first negotiation for AGAMREE® in Japan should Santhera pursue partnership opportunities in that territory. Additionally, we will hold the North American rights to any future approved indications for AGAMREE®. Finally, under our AGAMREE® License Agreement with Santhera, we have agreed to purchase commercial supply of AGAMREE® from Santhera at agreed upon prices.

Concurrent with the closing of the AGAMREE® License Agreement, we made a strategic investment into Santhera in which we acquired 1,414,688 of Santhera's ordinary shares (representing approximately 11.26% of Santhera's outstanding ordinary shares immediately following the transaction) at an investment price of CHF 9.477 per share, with the approximately $15.7 million USD in equity investment proceeds to be used by Santhera for Phase IV studies of AGAMREE® in DMD and future development of additional indications for AGAMREE®. On February 24, 2025, the closing price of Santhera's common shares on the SIX Swiss Exchange was CHF 16.08 per share (approximately $17.90 USD based on then-current exchange rates).

On October 26, 2023, the U.S. FDA approved Santhera's NDA for AGAMREE® for use in treating DMD in patients aged two years and older. Shortly thereafter, as part of the previously described transaction, Santhera transferred the approved NDA to us. Additionally, following approval of the NDA for the drug, we became obligated to make a milestone payment of $36 million to Santhera, which we paid during the fourth quarter of 2023. We may also be obligated to pay future regulatory and commercial milestone payments to Santhera tied to calendar year sales of AGAMREE®, as well as commercial royalties.

On March 13, 2024, utilizing our FIRDAPSE® field-based force, we launched AGAMREE® for the treatment of DMD in the U.S. for patients aged two years or older. During the first quarter of 2024, in connection with our preparation for the commercial launch of AGAMREE®, we incurred substantial commercialization expenses, including sales, marketing, analytical infrastructure, patient services, patient advocacy, and other commercialization related expenses. Initially, we added approximately 10 additional members to our FIRDAPSE® team, and our commercial team marketed both products. However, in early 2025, we made a strategic decision to split our commercial field-based force into two units, one expressly focused on

the marketing of FIRDAPSE® and one expressly focused on the marketing of AGAMREE®. This change is expected to allow us to better focus on the market for each product. We expect to complete this division of our field-based force into two units early in the second quarter of 2025.

We are further supporting the distribution of AGAMREE® through our Catalyst Pathways® patient services program to ensure that patients have access to a dedicated, personalized support team that assists families through the AGAMREE® patient journey, from answering questions to coordinating financial assistance programs for eligible patients. Finally, we have donated and intend to continue to donate funds to one or more qualified, independent charitable financial foundations who assist U.S. DMD patients in financial need for paying the costs of care including medication, to the extent permitted by each such organization's guidelines.

DMD, the most common form of muscular dystrophy, is a rare and life-threatening neuromuscular disorder characterized by progressive muscle dysfunction, ultimately leading to loss of ambulation, respiratory failure, and fatality. Current standard treatment for DMD involves corticosteroids, which often come with significant side effects. It is estimated that between 11,000 and 13,000 patients in the U.S. are affected by DMD, with approximately 70% of patients currently receiving a corticosteroid treatment. Steroids are expected to remain the foundation of therapy for DMD patients and dosed concomitantly with other therapies.

AGAMREE®'s unique mode of action is based on differential effects on glucocorticoid and mineralocorticoid receptors and modifying further downstream activity. As such, it is considered a novel corticosteroid that we hope has the potential to demonstrate comparable efficacy to corticosteroids, with the potential for a better-tolerated side effect profile. This mechanism of action may allow vamorolone to emerge as an effective alternative to the current standard of care corticosteroids in children, adolescents, and adult patients with DMD. In that regard, we have launched our SUMMIT study to evaluate data about long-term patient safety and quality of life data from the use of our product, with the hope of offering a deeper understanding of the product's potential long-term benefits for patients.

On October 13, 2023, Santhera announced that the European Union's Committee for Medicinal Products for Human Use (CHMP) adopted a positive position in favor of AGAMREE® for the treatment of DMD patients aged four and older. In its recommendation for approval, CHMP acknowledged that there was a positive benefit-risk profile of AGAMREE® in such patient population, including certain safety benefits of AGAMREE® compared to standard of care corticosteroids in the treatment of DMD. Further, on December 18, 2023, the European Commission (EC) granted to Santhera marketing authorization for AGAMREE® for the treatment of DMD in patents ages four years and older and on January 12, 2024 Santhera announced that AGAMREE® had received approval by the Medicines and Healthcare products Regulatory Agency (MHRA) in the United Kingdom. Further, on January 15, 2024, Santhera announced that AGAMREE® was commercially launched in Germany. Finally, On January 16, 2025, the National Institute for Health and Care Excellence (NICE) issued positive Final Guidance that recommends AGAMREE® for use in the National Health Service (NHS) in England, Wales and Northern Ireland for the treatment of DMD in patients four years of age and older and on February 13, 2025, Santhera announced an agreement with the German National Association of Statutory Health Insurance Funds (GKV-SV) on the reimbursement for AGAMREE® (vamorolone) for the treatment of DMD. This milestone makes AGAMREE® the first product to receive an agreed federal price in Germany for the treatment of DMD in patients 4 years of age and older, independent of the underlying genetic mutation.

We are currently taking the first steps to seek to expand the number of diseases that can be treated with AGAMREE®. In furtherance of that objective, we are currently conducting a Phase 1 study in healthy adults comparing a single dose of vamorolone, prednisone, and deflazacort, and studying the immunosuppressive effect of multiple ascending doses of AGAMREE®, which study will attempt to define the immunosuppressive dose of vamorolone for future indications and for the use of our product in conjunction with gene and cell therapies that are approved to treat DMD and require a concurrent immunosuppressive regimen of a corticosteroid when administered. We expect to have the results of this study around the middle of this year and hope that the results of this study will provide important information for use in marketing our product to healthcare practitioners in an effort to help with the treatment of patients. We have also begun a long-term project to undertake long-term research and development efforts for the product.

Further, we have established a joint steering committee with Santhera that is overseeing the lifecycle management and development of AGAMREE®. There can be no assurance that we can develop our product for the treatment of diseases other than DMD.

In the U.S., AGAMREE® has New Chemical Entity exclusivity that expires in October 2028. AGAMREE® also has Orphan Drug Exclusivity expiring in October 2030. AGAMREE® is further protected by seven Orange Book listed patents expiring as early as May 28, 2029 and as late as July 16, 2040. The Company has also requested Patent Term Extension and will update the relevant expiration date in the Orange Book upon a final determination by the USPTO. The earliest a generic manufacturer could submit an ANDA is October 26, 2027. If we were to pursue a patent infringement action if any such ANDA challenges any of AGAMREE®'s Orange Book patents, then the automatic statutory 30-month stay would prevent FDA approval of such ANDA until April 26, 2031.

Finally, on July 23, 2024 we entered into a license, supply and commercialization agreement with KYE, which is already our sublicensee for FIRDAPSE® in Canada, granting KYE the exclusive Canadian commercial rights to market AGAMREE® in Canada for DMD and other indications. Under the agreement, KYE is responsible for obtaining regulatory approval of the product from Health Canada (of which there can be no assurance), and we will supply product to KYE. Further, KYE has advised us that they expect to file an application with Health Canada seeking approval to commercialize AGAMREE® in Canada during the first quarter of 2025.

There can be no assurance that any such application when and if filed will be approved, and even if such application is approved that KYE will be successful in commercializing AGAMREE® in Canada.

Business Development

We continue to advance our strategic initiatives and portfolio expansion efforts, focusing on broadening and diversifying our rare (orphan) neurology product portfolio with innovative therapies that address critical unmet medical needs and expanding the geographical footprint of our existing products. In that regard, we are currently exploring clinically differentiated and adequately de-risked opportunities, with a keen focus on orphan, rare disease products across therapeutic areas. These prospects include evaluating companies with existing commercial drug products or drugs in development, for potential partnerships, licensing, geographical expansion opportunities with our existing products, and/or asset acquisitions. We continue to employ a disciplined, comprehensive, and exhaustive approach to identifying and evaluating opportunities that we believe will add significant value to our company over the near, mid, and long term. However, other than the recent sublicense agreement described above between the Company and KYE for AGAMREE® in Canada, no definitive agreements have been entered into to-date, and there can be no assurance that any of the Company's business development initiatives will be successful.

Capital Resources

At December 31, 2024, we had cash and investments of approximately $517.6 million. Based on our current financial condition, including our profitability, cash flows generated from operations and forecasts of available cash, we believe that we have sufficient funds to support our operations for at least the next 12 months. There can be no assurance that we will continue to be successful in commercializing FIRDAPSE® and AGAMREE®, that our projections about the commercialization of FYCOMPA® after the expiration of its patents will be correct, or that we will continue to be profitable and cash flow positive. See “Management’s Discussion and Analysis of Financial Condition and Results of Operations - Liquidity and Capital Resources” below for further information on our liquidity and cash flow.

Basis of Presentation

Revenues

During the fiscal year ended December 31, 2024, we generated revenues from product sales of FIRDAPSE®, FYCOMPA®, and AGAMREE®. We expect these revenues to fluctuate in future periods based on our sales of FIRDAPSE®, FYCOMPA®, and AGAMREE®.

We received approval from Health Canada on July 31, 2020, for FIRDAPSE® for the symptomatic treatment of LEMS and as of December 31, 2020, our sub-licensee KYE launched FIRDAPSE® in Canada. During the fiscal year ended December 31, 2024, revenues generated under our collaboration agreement with KYE were immaterial. During the year ended December 31, 2024, we announced that we had entered into a collaboration agreement with KYE for the launch of AGAMREE® in Canada.

On September 24, 2024, we were informed by DyDo that it had received approval of its New Drug Application for the sale of FIRDAPSE® in Japan. Further, DyDo informed us that they had launched FIRDAPSE® in Japan in January 2025.

We expect revenues from both the KYE and DyDo agreements to be immaterial in 2025 as distribution ramps up in each jurisdiction and KYE begins the process of seeking approval to market AGAMREE® in Canada.

Cost of Sales

Cost of sales consists of third party manufacturing costs, freight, royalties, milestone payments, and indirect overhead costs associated with sales of our products. Cost of sales may also include period costs related to certain inventory manufacturing services, inventory adjustments charges, unabsorbed manufacturing and overhead costs and manufacturing variances.

Research and Development Expenses

Our research and development expenses consist of costs incurred for company-sponsored research and development activities, as well as support for selected investigator-sponsored research. The major components of research and development costs include acquired IPR&D, preclinical study costs, clinical manufacturing costs, clinical study and trial expenses, insurance coverage for clinical trials, consulting, and other third party costs, salaries and employee benefits, stock-based compensation expense, supplies and materials, and allocations of various overhead costs related to our product development efforts.

Prior to January 2023, all of our research and development resources had been devoted to the development of FIRDAPSE®, and until we acquire or license new products we currently expect that our future development costs will be attributable principally to the continued development of FIRDAPSE®, and AGAMREE®.

Our cost accruals for clinical studies and trials are based on estimates of the services received and efforts expended pursuant to contracts with numerous clinical study and trial sites and clinical research organizations (CROs). In the normal course of our business we contract with third parties to perform various clinical study and trial activities in the on-going development of potential products. The financial terms of these agreements are subject to negotiation and vary from contract to contract and may result in uneven payment flows. Payments under the contracts depend on factors such as the achievement of certain events or milestones, the successful enrollment of patients, the allocation of responsibilities among the parties to the agreement, and the completion of portions of the clinical study or trial or similar conditions. The objective of our accrual policy is to match the recording of expenses in our consolidated financial statements to the actual services received and efforts expended. As such, expense accruals related to preclinical and clinical studies or trials are recognized based on our estimate of the degree of completion of the event or events specified in the specific study or trial contract. We monitor service provider activities to the extent possible; however, if we underestimate activity levels associated with various studies or trials at a given point in time, we could be required to record significant additional research and development expenses in future periods. Preclinical and clinical study and trial activities require significant up-front expenditures. We anticipate paying significant portions of a study or trial's cost before they begin and incurring additional expenditures as the study or trial progresses and reaches certain milestones.

Selling, General and Administrative Expenses

During 2019, we began to commit funds to developing our commercialization program for FIRDAPSE® and we have continued to incur substantial commercialization expenses, including sales, marketing, patient services, patient advocacy and other commercialization related expenses as we have continued our sales program for FIRDAPSE®. We are also now incurring substantial commercialization expenses for FYCOMPA® and AGAMREE® as we continue commercialization of both products. We expect that such expenses for FYCOMPA® may begin to decline as a result of a potential decline in sales upon the expiration of patent protection and the commencement of generic competition during 2025.

Our general and administrative expenses consist primarily of salaries and personnel expenses for accounting, corporate, compliance, and administrative functions. Other costs include administrative facility costs, regulatory fees, insurance, and professional fees for legal including litigation cost, IT, accounting, and consulting services.

Amortization of Intangible Assets

Amortization of intangible assets consists of the amortization of the FYCOMPA® product rights, which are amortized using the straight-line method over its estimated useful life of 5 years, the RUZURGI® product rights, which are amortized using the straight-line method over its estimated useful life of 14.5 years, and the milestone payment made to Santhera relating to the approval of AGAMREE® in the U.S. in October 2023, which is amortized using the straight-line method over its estimated useful life of 10.5 years.

Income Taxes

Our effective income tax rate is the ratio of income tax expense over our income before income taxes. As of December 31 2024, 2023, and 2022, we had no federal net operating loss carry-forwards. Additionally, we had no state net operating loss carry-forwards in any such year.

Recently Issued Accounting Standards

For discussion of recently issued accounting standards, please see Note 2, "Basis of Presentation and Significant Accounting Policies,": in the consolidated financial statements included in this report.

Critical Accounting Policies and Estimates

Our discussion and analysis of our financial condition and results of operations are based on our consolidated financial statements, which have been prepared in accordance with U.S. Generally Accepted Accounting Principles (GAAP). The preparation of these consolidated financial statements requires us to make judgments, 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 reported revenue and expenses during the reporting periods. We continually evaluate our judgments, estimates and assumptions. We base our estimates on the terms of underlying agreements, our expected course of development, historical experience and other factors we believe are reasonable based on the circumstances, the results of which form our management's basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates.

The amounts of assets and liabilities reported in our consolidated balance sheets and the amounts reported in our consolidated statements of comprehensive income are affected by estimates and assumptions, which are used for, but not limited to, the accounting for revenue recognition, valuation of intangible assets, stock-based compensation and valuation allowance for deferred tax assets. The accounting policies described below are not intended to be a comprehensive list of all of our accounting policies but represent the accounting estimates which involve a significant level of estimation uncertainty and have had or are reasonably likely to have a material impact on our financial condition or results of operations. In many cases, the accounting treatment of a particular transaction is specifically dictated by U.S. GAAP. There are also areas in which our management's judgment in selecting any available alternative would not produce a materially different result. Our consolidated financial statements and the notes thereto included elsewhere in this report contain accounting policies and other disclosures as required by U.S. GAAP.

Revenue Recognition.

Revenue from product sales are recorded at the net sales price (transaction price), which includes estimates of variable consideration for which reserves are established. Components of variable consideration include trade discounts and allowances, product returns, provider chargebacks and discounts, government rebates, and other incentives, such as voluntary patient assistance, and other allowances that are offered within contracts with our customer, payors, and other indirect customers relating to the sale of our products. These reserves are based on the amounts earned, or to be claimed on the related sales, and are classified as reductions of accounts receivable (if the amount is payable to the customer) or a current liability (if the amount is payable to a party other than a customer). These estimates take into consideration a range of possible outcomes which are probability-weighted in accordance with the expected value method in Topic 606 for relevant factors such as current contractual and statutory requirements, specific known market events and trends, industry data, and forecasted customer buying and payment patterns. Overall, these reserves reflect our best estimates of the amount of consideration to which we are entitled based on the terms of the respective underlying contracts.

The amount of variable consideration which is included in the transaction price may be constrained, and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized under the contract will not occur in a future period. Our analyses also contemplated application of the constraint in accordance with the guidance, under which it determined a material reversal of revenue would not occur in a future period for the estimates as of December 31, 2024, 2023 and 2022 and, therefore, the transaction price was not reduced further during the years ended December 31, 2024, 2023 and 2022. Actual amounts of consideration ultimately received may differ from our estimates. If actual results in the future vary from our estimates, we will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known. Refer to Note 2, "Basis of Presentation and Significant Accounting Policies," in the consolidated financial statements included in this report for further details on revenue recognition.

Valuation of Intangible Assets.

We have acquired and continue to acquire significant intangible assets that we record at fair value at the acquisition date. Transactions involving the purchase or sale of intangible assets are usually based on a discounted cash flow analysis. The discounted cash flow model requires assumptions about the timing and amount of future net cash flows, risk, cost of capital and market participants. Each of these factors can significantly affect the value of the intangible asset. We engage independent valuation experts who review our critical assumptions and calculations for acquisitions of significant intangibles. We review intangible assets with finite lives for impairment whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. If indicators of impairment exist, an impairment test is performed to assess the recoverability of the affected assets by determining whether the carrying amount of such assets exceeds the undiscounted expected future cash flows. If the affected assets are deemed not recoverable, we would estimate the fair value of the assets and record an impairment loss. Where cash flows cannot be identified for an individual asset, the review is applied at the lowest group level for which cash flows are identifiable.

Stock-Based Compensation.

We recognize stock-based compensation for the fair value of all share-based payments, including grants of stock options and restricted stock units. For stock options, we use the Black-Scholes option valuation model to determine the fair value of stock options on the date of grant. This model derives the fair value of stock options based on certain assumptions related to expected stock price volatility, expected option life, risk-free interest rate and dividend yield. Expected volatility is based on reviews of historical volatility of our common stock. The estimated expected option life is based upon the simplified method. Under this method, the expected option life is presumed to be the mid-point between the vesting date and the end of the contractual term. We will continue to use the simplified method until we have sufficient historical exercise data to estimate the expected life of the options. The risk-free interest rate assumption is based upon the U.S. Treasury yield curve appropriate for the expected life of our stock option awards. For the years ended December 31, 2024 and 2023, the assumptions used were an estimated annual volatility of 54.1% to 61.5% and 68.0% to 71.0%, expected holding periods of 4.5 to 5.0 years and 4.5 to 5.2 years, and risk-free interest rates of 3.70% to 4.70% and 3.55% to 4.92%, respectively.

Results of Operations

Years Ended December 31, 2024 and 2023

Revenues.

For the fiscal year ended December 31, 2024, we recognized total revenues of approximately $491.7 million, which included approximately $489.3 million in net revenue from product sales primarily in the U.S., compared to approximately $398.2 million in total revenues, which included approximately $396.5 million in net revenues from product sales primarily in the U.S., for the fiscal year ended December 31, 2023.

FIRDAPSE® net sales were approximately $306.0 million for the fiscal year ended December 31, 2024, compared to approximately $258.4 million for the fiscal year ended December 31, 2023.

AGAMREE® net sales were approximately $46.0 million for the period between March 13, 2024 (date of commercial launch) and December 31, 2024.

FYCOMPA® net sales were approximately $137.3 million for the fiscal year ended December 31, 2024, compared to approximately $138.1 million for the period between January 24, 2023 (date of acquisition) and December 31, 2023. FYCOMPA® net sales for the fiscal year ended December 31, 2024 were affected by differences in variable consideration (gross-to-net) compared to the 2023 period when revenues were booked under Eisai's more favorable cost arrangements with distributors and government authorities.

The increase of approximately $92.8 million in net product revenues when comparing the fiscal year ended December 31, 2024 and 2023 was primarily due to the commercialization of AGAMREE®, and related product sales, and increases in FIRDAPSE® sales volumes of approximately 12.4% and net price increases. Net revenues from product sales of FIRDAPSE® increased by 18.4% from 2023 to 2024.

In the first quarter of each calendar year, like many companies in our industry, we are also impacted by the reset of patient insurance deductibles.

Beginning in May 2025, product revenue for FYCOMPA® will be affected by the expiration of FYCOMPA® patent protection, which we expect will decrease our net product revenue for this product.

For the fiscal year ended December 31, 2024, we recognized $2.4 million in license and other revenue, as compared to $1.7 million during the fiscal year ended December 31, 2023. For the fiscal year ended December 31, 2024, license and other revenue consisted primarily of a milestone payment of $2.1 million earned upon DyDo receiving product approval to commercialize FIRDAPSE® for the treatment of patients with LEMS in Japan. For the fiscal year ended December 31, 2023, such revenue consisted of the $1.4 million payment achieved as a result of DyDo submitting a Japan NDA for FIRDAPSE® to the PMDA on December 18, 2023.

Cost of Sales.

Cost of sales was approximately $68.8 million for the fiscal year ended December 31, 2024, compared to $52.0 million for the fiscal year ended December 31, 2023. Cost of sales in both periods consisted principally of royalty payments, which are based on net revenue as defined in the applicable license agreements. For FIRDAPSE®, royalties are payable on the terms set forth below in Liquidity and Capital Resources—*Contractual Obligations and Arrangements*, and increase by 3% when net sales (as defined in the applicable license agreement) exceed $100 million in any calendar year. Cost of sales for FYCOMPA® for the fiscal year ended December 31, 2024 consisted of product costs and excludes the amortization of the FYCOMPA® intangible assets. Cost of sales for AGAMREE® for the fiscal year ended December 31, 2024 consisted of royalties payable on the terms set forth below in Liquidity and Capital Resources—*Contractual Obligations and Arrangements*, product costs and excludes the amortization of the AGAMREE® intangible asset. Royalties on sales of AGAMREE® in future years may increase as a percentage if net sales exceed certain amounts over $100 million. See Note 9 of the Notes to Consolidated Financial Statements included elsewhere in this report.

Amortization of Intangible Assets.

Amortization of intangible assets was approximately $37.4 million for the fiscal year ended December 31, 2024 compared to $32.6 million for fiscal year ended December 31, 2023. Amortization of intangible assets consists of the amortization of the FYCOMPA® rights, which are amortized using the straight-line method over its estimated useful life of 5 years, the RUZURGI® rights, which are amortized using the straight-line method over its estimated useful life of 14.5 years and the AGAMREE® rights, which are amortized using the straight-line method over its estimated useful life of 10.5 years.

Each fiscal quarter, we review the value of our intangible assets to determine if they are impaired. If we determine one or more of our intangible assets are impaired during a future period we would record a charge in the amount of that impairment.

Research and Development Expenses.

Research and development expenses for the years ended December 31, 2024 and 2023 were approximately $12.6 million and $93.2 million, respectively, and represented approximately 4% and 30% of total operating costs and expenses, respectively. Research and development expenses for the years ended December 31, 2024 and 2023 were as follows (in thousands):

	For the year ended December 31,		Change	
	2024	2023	$	%
Salary and benefit expense	$ 3,905	$ 3,119	786	25.2
Employee stock-based compensation	1,779	1,481	298	20.1
Research and clinical trial expense	5,813	5,333	480	9.0
Acquired in-process research and development	—	81,513	(81,513)	(100.0)
Additional research and development expense	1,151	1,704	(553)	(32.5)
Total research and development expenses	$ 12,648	$ 93,150	$ (80,502)	(86.4)

Research and development expenses decreased approximately $80.5 million during year ended December 31, 2024 when compared to the same period in 2023. The decrease is primarily attributable to the $81.5 million IPR&D purchase consideration for the acquisition of the license in North America for AGAMREE® during the third quarter of 2023. During 2024, research and development expenses consisted of costs for company-sponsored research and development activities, support for selected investigator-sponsored research, and support for our commercial activities.

We expect that research and development activities may become more significant in the future if we seek to execute on the development of additional indications for FIRDAPSE® and AGAMREE® and on our portfolio expansion efforts.

Selling, General and Administrative Expenses.

Selling, general and administrative expenses for the years ended December 31, 2024 and 2023 were approximately $177.7 million and $133.7 million, respectively, and represented approximately 60% and 43% of total operating costs and expenses for the years ended December 31, 2024, and 2023, respectively. Selling, general and administrative expenses for the years ended December 31, 2024 and 2023 were as follows (in thousands):

	For the year ended December 31,		Change	
	2024	2023	$	%
Selling	$ 111,344	$ 86,689	24,655	28.4
General and administrative	45,924	34,252	11,672	34.1
Employee stock-based compensation	20,472	12,769	7,703	60.3
Total selling, general and administrative expenses	$ 177,740	$ 133,710	44,030	32.9

For the year ended December 31, 2024, selling, general and administrative expenses increased approximately $44.0 million when compared to the same period in 2023. This was primarily attributable to an approximately $18.1 million increase in employee compensation and stock-based compensation related to annual merit increases and an increase in headcount resulting from the acquisitions of FYCOMPA® and AGAMREE®, an approximately $24.7 million increase in commercialization expenses related to the launch of AGAMREE® and to the timing of our commitments to make contributions to 501(c)(3) organizations supporting LEMS patients of approximately $1.3 million. Further, an approximately $3.9 million increase in stock-based compensation expense is related to the retirement of two former executive officers, which was recorded during the first quarter of 2024 upon lapse of the applicable revocation periods under the respective separation agreements with these former executives.

We expect that selling, general, and administrative expenses will continue to be substantial in future periods as we continue to sell FIRDAPSE® and AGAMREE® and as we take other steps in an effort to continue to expand our business.

Stock-Based Compensation.

Total stock-based compensation for the years ended December 31, 2024 and 2023 was $22.3 million and $14.3 million, respectively. In 2024 and 2023, grants were principally for stock options and restricted stock units related to year-end bonus awards and grants to new employees. For 2024, stock-based compensation included stock option and restricted stock unit grants to our new Chief Executive and Chief Financial Officers, each of which began employment at the beginning of 2024.

Other Income, Net.

We reported other income, net in all periods, primarily relating to interest on our investment of our cash and cash equivalents of approximately $21.1 million and $7.7 million for the fiscal years ended December 31, 2024 and 2023, respectively. The increase in other income, net for the fiscal year ended December 31, 2024 when compared to the same period in 2023 was primarily due to increases in cash and cash equivalents partially a result of our underwritten public offering that we completed in January 2024 and an increase in the share price of our investment in Santhera, partially offset by lower interest rates.

Since Santhera's shares are traded on the SIX Swiss Exchange, they have a readily determinable fair value, and as a result the investment is measured quarterly, at fair value, with changes reported in other income, net.

The components of other income, net were as follows (in thousands):

	For the year ended December 31,	
	2024	2023
Interest income, net	$ 16,064	$ 4,675
Net gains recognized during the period on equity securities	5,075	3,024
Total other income, net	$ 21,139	$ 7,699

Income Taxes.

Our effective income tax rate was approximately 24.2% and 24.4% for fiscal years ended December 31, 2024 and 2023, respectively. Differences in our effective tax and the statutory federal income tax of 21% are driven by state income taxes and anticipated annual permanent differences offset by equity compensation deductions. Our effective tax rate is affected by many factors, including the number of stock options exercised in any period, and our effective tax rate is likely to fluctuate in future periods (and may be higher than it was in the 2024 fiscal year).

We had no material uncertain tax positions as of December 31, 2024 and 2023.

Net Income.

Our net income was approximately $163.9 million in the year ended December 31, 2024 ($1.38 per basic and $1.31 per diluted share) as compared to approximately $71.4 million in the year ended December 31, 2023 ($0.67 per basic and $0.63 per diluted share).

Years Ended December 31, 2023 and 2022

The information comparing results of operations for the year ended 2023 compared to 2022 was included in our Annual Report on Form 10-K for 2023 filed with the SEC on February 28, 2024.

Liquidity and Capital Resources

Since our inception, we have financed our operations primarily through revenues from product sales and multiple offerings of our securities. At December 31, 2024 we had cash and cash equivalents aggregating $517.6 million and working capital of $502.9 million. At December 31, 2023, we had cash and cash equivalents aggregating $137.6 million and working capital of $143.3 million. At December 31, 2024, substantially all of our cash and cash equivalents were deposited with two financial institutions, and such balances were in excess of federally insured limits. Further, as of such date, substantially all such funds were invested in money market accounts and U.S. Treasuries.

On September 8, 2023, we filed a shelf registration statement with the SEC to sell up to $500 million of common stock, preferred stock, warrants to purchase common stock, debt securities and units consisting of one or more of such securities (the 2023 Shelf Registration Statement). The 2023 Shelf Registration Statement (file no. 333-274427) became effective upon filing. On January 9, 2024, we completed a public offering of 10 million shares of our common stock under the 2023 Shelf Registration Statement, raising net proceeds of approximately $140.7 million.

Based on our current financial condition, including our profitability, cash flows generated from operations and forecasts of available cash, we believe that we have sufficient funds to support our operations for at least the next 12 months from the date of this report. There can be no assurance that we will remain profitable or that we will be able to obtain any additional funding that we may require in the future.

In the future, we may require additional working capital to support our operations depending on our future success with FIRDAPSE®, FYCOMPA® and AGAMREE® sales, or the products we may acquire and continue to develop and whether our results continue to be profitable and cash flow positive. There can be no assurance as to the amount of any such funding that will be required for these purposes or whether any such funding will be available to us if and when it is required.

In that regard, our future funding requirements will depend on many factors, including:

- the cost of diligence in seeking potential acquisitions and of the completion of such acquisitions, if any future acquisitions occur;
- future clinical trial results;
- the scope, rate of progress and cost of our clinical trials and other product development activities;
- the terms and timing of any collaborative, licensing and other arrangements that we may establish;
- the cost and timing of regulatory approvals;
- the cost and delays in product development as a result of any changes in regulatory oversight applicable to our products;
- the amount of net revenues that we report from sales of FIRDAPSE®, FYCOMPA® and AGAMREE®;
- the effect of competition and market developments;
- the cost of filing and potentially prosecuting, defending and enforcing any patent claims and other intellectual property rights; and
- the extent to which we acquire or invest in other products.

We may raise additional funds through public or private equity offerings, debt financings, corporate collaborations or other means. We also may seek governmental grants for a portion of the required funding for our clinical trials and preclinical trials. We may further seek to raise capital to fund additional product development efforts or product acquisitions, even if we have sufficient funds for our planned operations. Any sale by us of additional equity or convertible debt securities could result in dilution to our stockholders. There can be no assurance that any such required additional funding will be available to us at all or available on terms acceptable to us. Further, to the extent that we raise additional funds through collaborative arrangements, it may be necessary to relinquish some rights to our technologies or grant sublicenses on terms that are not favorable to us. If we are not able to secure additional funding when needed, we may have to delay, reduce the scope of or eliminate one or more research and development programs, which could have an adverse effect on our business.

Cash Flows.

Net cash provided by operating activities was $239.8 million and $143.6 million, respectively, for the years ended December 31, 2024 and 2023. During the year ended December 31, 2024, net cash provided by operating activities was primarily attributable to our net income of $163.9 million, increases of $1.8 million in accounts payable and $53.8 million in accrued expenses and other liabilities, $22.3 million in stock-based compensation and $37.8 million in amortization of intangible assets and depreciation. This was partially offset by increases of $12.0 million in accounts receivable, net, $3.9 million in inventory and $8.5 million prepaid expenses and other current assets, a decrease of $0.4 million in operating lease liability, $9.4 million in deferred taxes and $5.6 million in non-cash expenses. During the year ended December 31, 2023, net cash provided by operating activities was primarily attributable to our net income of $71.4 million, increases of $10.8 million in accounts payable and $5.8 million in accrued expenses and other liabilities, $81.5 million in acquired IPR&D, $14.3 million in stock-based compensation and $32.9 million in amortization of intangible assets and depreciation. This was partially offset by increases of $43.1 million in accounts receivable, net, $4.7 million in inventory and $5.8 million prepaid expenses and other current assets, a decrease of $0.3 million in operating lease liability, $17.8 million in deferred taxes and $1.3 million in non-cash expenses.

Net cash used in investing activities during the year ended December 31, 2024 was $0.6 million and consisted of purchases of property and equipment. Net cash used in investing activities during the year ended December 31, 2023 was $293.5 million and consisted primarily of payments in connection with asset acquisitions of $198.3 million, acquired IPR&D of $81.5 million and the purchase of equity securities of $13.5 million.

Net cash provided by financing activities during the year ended December 31, 2024 was $140.7 million, consisting primarily of proceeds from the issuance of common stock. Net cash used in financing activities during the year ended December 31, 2023 was $10.9 million, consisting primarily of payment of liabilities arising from asset acquisition of $12.7 million, partially offset by proceeds from the exercise of stock options of $2.8 million.

Contractual Obligations and Arrangements.

We have entered into the following contractual arrangements with respect to sales of FIRDAPSE®:

- *Payments due under our license agreement for FIRDAPSE®.* We currently pay the following royalties under our license agreement:

- Royalties to our licensor for seven years from the first commercial sale of FIRDAPSE® equal to 7% of net sales (as defined in the FIRDAPSE® License Agreement) in North America for any calendar year for sales up to $100 million, and 10% of net sales in North America in any calendar year in excess of $100 million; and
- Royalties to the third party licensor of the rights sublicensed to us from the first commercial sale of FIRDAPSE® equal to 7% of net sales (as defined in the FIRDAPSE® License Agreement between BioMarin (since transferred to SERB) and the third party licensor) in any calendar year for the duration of regulatory exclusivity within a territory and 3.5% for of net sales in any territory in which we do not enjoy regulatory exclusivity.

For the years ended December 31, 2024 and 2023, we recognized an aggregate of approximately $47.3 million and $39.5 million, respectively, of royalties payable under these license agreements, which is included in cost of sales in the accompanying consolidated statements of operations and comprehensive income.

Further, we will pay royalties to our licensor on net sales in Japan equal to a similar percentage to the royalties that we are currently paying for non-U.S. sales under our original FIRDAPSE® License Agreement for North America.

- *Payments due to Jacobus.* In connection with our July 2022 settlement with Jacobus, we agreed to pay the following consideration to Jacobus:
  - $30 million of cash, of which $10 million was paid at the closing of the settlement on July 11, 2022, $10 million was paid on the first anniversary of closing and $10 million was paid on the second anniversary of closing; and
  - An annual royalty on Catalyst's net sales (as defined in the License and Asset Purchase Agreement between Catalyst and Jacobus) of amifampridine products in the U.S. equal to: (a) for calendar years 2022 through 2025, 1.5% (with a minimum annual royalty of $3.0 million per year), and (b) for calendar years 2026 through the expiration of the last to expire of Catalyst's FIRDAPSE® patents in the U.S., 2.5% (with a minimum annual royalty of $5 million per year); *provided, however*, that the royalty rate may be reduced and the minimum annual royalty may be eliminated under certain circumstances.

For the years ended December 31, 2024 and 2023, we recognized an aggregate of approximately $4.4 million and $3.7 million, respectively, of royalties payable to Jacobus.

We have entered into the following contractual arrangements with respect to sales of FYCOMPA®:

- *Payments due under our asset purchase agreement for FYCOMPA®.* In connection with our asset purchase agreement with Eisai Co., Ltd. (Eisai), we agreed to pay the following consideration to Eisai:
  - We paid at closing a $160 million upfront cash payment, plus $1.6 million for reimbursement of certain prepayments.
  - Royalties commencing on loss of exclusivity for each calendar year during the royalty term equal to 12% on net sales greater than $10 million and less than $100 million, 17% on net sales of greater than $100 million and less than $125 million and 22% on net sales greater than $125 million prior to the date of generic entry. Royalties equal to 6% on net sales greater than $10 million and less than $100 million, 8.5% on net sales of greater than $100 million and less than $125 million and 11% on net sales greater than $125 million after the date of generic entry.
  - Concurrently with the acquisition, the parties entered into two related agreements: (i) a short-term TSA for commercial and manufacturing services (to which transition services ended on December 31, 2023) and (ii) a long-term Supply Agreement for the manufacturing of FYCOMPA®. Under the TSA, Eisai provided certain commercial and manufacturing services to the Company for a transition period following the closing of the acquisition. Further, under the Supply Agreement, Eisai will manufacture FYCOMPA® for the Company for a period of seven years (or such longer period as is set forth in the Supply Agreement) following the closing of the acquisition.

We have entered into the following contractual arrangements with respect to AGAMREE® (vamorolone):

- *Payments due under our license agreement for AGAMREE®.* In connection with our recent acquisition from Santhera:
  - At closing we paid a $75 million initial cash payment.
  - In the fourth quarter of 2023, following regulatory approval of Santhera's NDA for AGAMREE® by the FDA, we paid a regulatory milestone payment of $36 million. We are also obligated to pay additional regulatory

milestone payments upon regulatory approval by the FDA in the U.S. of an NDA for the product for the first, second, and third additional indications in the amounts of $50 million, $45 million, and $45 million, respectively.

- We are obligated to pay royalties and/or sales-based milestone payments if the applicable amount of net sales of all products in the territory in a single calendar year fall within the range of one or more of the net sales threshold levels set forth in the AGAMREE® License Agreement.
- Until January 1, 2026, we are obligated to purchase all of the requirements for product solely from Santhera, and Santhera is required to manufacture, supply, and sell product to us at an agreed upon supply price.
- Simultaneously with entering into the license agreement, we made a strategic equity investment into Santhera by acquiring 1,414,688 of Santhera's ordinary shares (representing approximately 11.26% of Santhera's outstanding ordinary shares immediately following the transaction) at an investment price of CHF 9.477 per share (corresponding to a mutually agreed volume-weighted average price prior to signing), with the approximately $15.7 million USD in equity investment proceeds, inclusive of the approximately $13.5 million USD fair value of the investment in Santhera and approximately $2.2 million USD of transaction costs included in acquired in-process research and development, to be used by Santhera for Phase IV studies in DMD and further development of additional indications for AGAMREE®.

For the year ended December 31, 2024, we recognized an aggregate of approximately $5.1 million of royalties payable under this license agreement, which is included in cost of sales in the accompanying consolidated statement of operations and comprehensive income. No royalties were recognized during the year ended December 31, 2023 under this agreement.

We also have entered into the following contractual arrangements:

- *Purchase commitment*. We have entered into a purchase commitment with a contract manufacturing organization for approximately $0.5 million per year. The agreement expires in December 2025.
- *Lease for office space*. We operate our business in leased office space in Coral Gables, Florida. We lease approximately 10,700 square feet of office space and we pay annual rent of approximately $0.5 million.

Off-Balance Sheet Arrangements.

We do not have any off-balance sheet arrangements as such term is defined in rules promulgated by the SEC.

Caution Concerning Forward-Looking Statements

This report contains "forward-looking statements", as that term is defined in the Private Securities Litigation Reform Act of 1995. These include statements regarding our expectations, beliefs, plans or objectives for future operations and anticipated results of operations. For this purpose, any statements contained herein that are not statements of historical fact may be deemed to be forward-looking statements. Without limiting the foregoing, "believes", "anticipates", "proposes", "plans", "expects", "intends", "may", and other similar expressions are intended to identify forward-looking statements. Such statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or other achievements to be materially different from any future results, performances or achievements expressed or implied by such forward-looking statements. Factors that might cause such differences include, but are not limited to, those discussed in the section entitled "Item 1A – Risk Factors."

The continued successful commercialization of FIRDAPSE® (amifampridine), FYCOMPA® (perampanel) CIII, and AGAMREE® (vamorolone) are highly uncertain. Factors that will affect our success include the uncertainty of:

- Whether we will be able to continue to successfully market and sell FIRDAPSE®, FYCOMPA®, and AGAMREE® while maintaining full compliance with applicable federal and state laws, rules and regulations;
- Whether we will be able to continue to attract and retain the qualified personnel necessary to run our business;
- Whether our estimates of the size of the market for FIRDAPSE® for the treatment of LEMS will prove to be accurate;
- Whether the daily dose of FIRDAPSE® taken by patients changes over time and affects our results of operations;
- Whether we will continue to be able to locate LEMS patients who are undiagnosed or are misdiagnosed with another disease;
- Whether patients will discontinue from the use of our products at rates that are higher than historically experienced or are higher than we project;

- Whether new FIRDAPSE®, FYCOMPA®, and AGAMREE® patients can be successfully titrated to stable therapy;
- Whether we can continue to market our products on a profitable and cash flow positive basis;
- Whether we will be able to demonstrate, to the satisfaction of the FDA and third party payors, whether AGAMREE® offers advantages compared to other corticosteroids or competitor's products;
- Whether DMD patients transitioning to current or future gene therapy treatments will delay initiating use of AGAMREE® while waiting for access to such gene therapy, or stop their AGAMREE® therapy during the course of their gene therapy treatment;
- Whether we will be able to continue to successfully commercialize FYCOMPA® after its patents expire starting in May 2025 and generic competition for FYCOMPA® enters the market;
- Whether any revenue or earnings guidance that we provide to the public market will turn out to be accurate;
- Whether payors will continue to provide coverage and reimburse for our products at the price that we charge for our products;
- The ability of our third party suppliers and contract manufacturers to continue to supply sufficient product to meet our customers' needs in a timely manner;
- The ability of our third party suppliers and contract manufacturers to maintain compliance with current Good Manufacturing Practices (cGMP);
- The ability of those third parties that distribute our products to maintain compliance with applicable law;
- Our ability to maintain compliance with applicable rules relating to our patient assistance programs for our products;
- Our ability to maintain compliance with the applicable rules that relate to our contributions to 501(c)(3) organizations that support patients in financial need;
- The scope of our intellectual property and the outcome of challenges to our intellectual property, and, conversely, whether any third party intellectual property presents unanticipated obstacles for FIRDAPSE®, FYCOMPA®, or AGAMREE®;
- Whether there will be a post-closing review by antitrust regulators of our previous acquisition transactions, and the outcome of any such reviews if they occur;
- Whether we will be able to acquire additional drug products under development, complete development required to commercialize such products, and thereafter, if such products are approved for commercialization, successfully market such products;
- Whether we will be successful in our litigation to enforce our patents against the Paragraph IV challengers who have filed ANDAs seeking to introduce generic versions of FIRDAPSE®;
- Whether our patents will be sufficient to prevent generic competition for FIRDAPSE® and AGAMREE® after our orphan drug exclusivity for each product expires;
- The impact on our profits and cash flow of adverse changes in reimbursement and coverage policies or regulations from government and private payors such as Medicare, Medicaid, insurance companies, health maintenance organizations and other plan administrators, or the impact of pricing pressures enacted by industry organizations, the federal government or the government of any state, including as a result of increased scrutiny over pharmaceutical pricing or otherwise;
- Changes in the healthcare industry and the effect of political pressure from and actions by the President, Congress and/or medical professionals seeking to reduce prescription drug costs, and changes to the healthcare industry occasioned by any future changes in laws relating to the pricing of drug products, including changes made in the Inflation Reduction Act of 2022, changes (if any) to be made by the current President and/or the current Congressional administrations, changes to the review and approval process at the FDA, or changes in the healthcare industry generally;
- Whether we and Santhera can successfully develop additional indications for AGAMREE® and obtain the ability to commercialize the product for these additional indications;

- The state of the economy generally and its impact on our business;
- The scope, rate of progress and expense of future clinical trials and studies, pre-clinical studies, proof-of-concept studies, and our other drug development activities, and whether any trials and studies we undertake will be successful;
- Our ability to complete any clinical trials and studies that we may undertake on a timely basis and within the budgets we establish for such trials and studies;
- Whether FIRDAPSE® can be successfully commercialized in Canada on a profitable basis through KYE, our collaboration partner in Canada;
- Whether AGAMREE® will be approved by Health Canada for commercialization in Canada and whether, if the product is approved, KYE can successfully commercialize it in Canada;
- Whether KYE will successfully file an application seeking to commercialize AGAMREE® in Canada during the first quarter of 2025 or at all;
- Now that FIRDAPSE® has been approved for commercialization in Japan and launched, whether DyDo will be successful in commercializing the product in Japan;
- The impact on sales of FIRDAPSE® in the U.S. if an amifampridine product is purchased in Canada for use in the U.S.;
- Whether our plans to expand the reach of FIRDAPSE® and AGAMREE® into other global regions will be successful;
- System failures or security or data breaches due to cyber-attacks, or cyber intrusions, including ransomware, phishing attacks and other malicious intrusions whether it occurs directly to us or indirectly through third parties; and
- Our ability to enhance our systems, processes and procedures to appropriately support the growing complexity and scale of our business.

Our current plans and objectives are based on assumptions relating to the continued commercialization of FIRDAPSE®, FYCOMPA®, and AGAMREE® and on our plans to seek to acquire or in-license additional products. Although we believe that our assumptions are reasonable, any of our assumptions could prove inaccurate. Considering the significant uncertainties inherent in the forward-looking statements we have made herein, which reflect our views only as of the date of this report, you should not place undue reliance upon such statements. We undertake no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk

Market risk represents the risk of changes in the value of market risk-sensitive instruments caused by fluctuations in interest rates, foreign exchange rates and commodity prices. Changes in these factors could cause fluctuations in our results of operations and cash flows.

Our exposure to interest rate risk is currently confined to our cash and cash equivalents that are from time to time invested in highly liquid money market funds and U.S. Treasuries. The primary objective of our investment activities is to preserve our capital to fund acquisitions and operations. We also seek to maximize income from our investments without assuming significant risk. We do not use derivative financial instruments in our investment portfolio. Our cash and investments policy emphasizes liquidity and preservation of principal over other portfolio considerations.

Item 8. Financial Statements and Supplementary Data

See the list of financial statements filed with this report under Item 15 below.

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

None.

Item 9A. Controls and Procedures

Disclosure Controls and Procedures

We have carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures. The term “disclosure controls and procedures”, as defined in Rules 13a-15(e) and 15(d)-15(e) under the Securities

Exchange Act of 1934 (the Exchange Act), means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is processed, summarized and reported, within the time periods specified in the Securities and Exchange Commission's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

Based on such evaluation, our principal executive officer and principal financial officer have concluded that as of December 31, 2024, our disclosure controls and procedures were effective to ensure that the information required to be disclosed by us in the reports filed or submitted by us under the Securities Exchange Act of 1934, as amended, was recorded, processed, summarized or reported within the time periods specified in the rules and regulations of the SEC, and include controls and procedures designed to ensure that information required to be disclosed by us in such reports was accumulated and communicated to management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosures.

Management's Annual Assessment of Internal Control Over Financial Reporting

Management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Exchange Act Rule 13a-15(f). Our internal control over financial reporting includes those policies and procedures that (i) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of our assets; (ii) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that our receipts and expenditures are being made only in accordance with authorizations of our management and directors; and (iii) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of our assets that could have a material effect on our consolidated financial statements.

Internal control over financial reporting is designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements prepared for external purposes in accordance with generally accepted accounting principles. Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Under the supervision and with the participation of our principal executive officer and our principal financial officer, management conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2024 based on the 2013 framework in Internal Control-Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission and in accordance with the interpretive guidance issued by the SEC in Release No. 34-55929. Based on that evaluation, management concluded that our internal control over financial reporting was effective as of December 31, 2024.

During the fourth quarter of 2024, there were no changes in our internal control over financial reporting, as defined in Rule 13a-15(f) under the Securities and Exchange Act of 1934 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Our independent registered public accounting firm, Grant Thornton LLP, has issued a report on our internal control over financial reporting, which is included in Item 15 of this Annual Report on Form 10-K.

Item 9B. Other Information

Not applicable.

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections

Not applicable.

PART III

Item 10. Directors and Executive Officers of the Registrant

The information required by this item will be contained in our definitive proxy statement, or Proxy Statement, to be filed with the SEC in connection with our 2025 Annual Meeting of Stockholders. Our Proxy Statement for the 2025 Annual Meeting of Stockholders is expected to be filed not later than 120 days after the end of our fiscal year ended December 31, 2024 and is incorporated into this report by this reference.

We have adopted a code of ethics that applies to our chief executive officer, chief financial officer, and to all of our other officers, directors, employees and agents. The code of ethics is attached to this Annual Report on Form 10-K as Exhibit 14.1 and is available on our website at www.catalystpharma.com. We intend to disclose future amendments to, or waivers from, certain provisions of our code of ethics on the above website within five business days following the date of such amendment or waiver.

Item 11. Executive Compensation

The information required by this item will be set forth in the Proxy Statement and is incorporated into this report by this reference.

Item 12. Security Ownership of Certain Beneficial Owners and Management

The information required by this item will be set forth in the Proxy Statement and is incorporated into this report by this reference.

Item 13. Certain Relationships and Related Transactions

The information required by this item will be set forth in the Proxy Statement and is incorporated into this report by this reference.

Item 14. Principal Accounting Fees and Services

The information required by this item will be set forth in the Proxy Statement and is incorporated into this report by this reference.

PART IV

Item 15. Exhibits and Financial Statement Schedules

Documents filed as part of this report:

The following financial statements of Catalyst Pharmaceuticals, Inc. and Reports of Grant Thornton LLP, independent registered public accounting firm, are included in this report:

Reports of Grant Thornton LLP, Independent Registered Public Accounting Firm.

Consolidated Balance Sheets as of December 31, 2024 and 2023.

Consolidated Statements of Operations and Comprehensive Income for the years ended December 31, 2024, 2023 and 2022.

Consolidated Statements of Changes in Stockholders' Equity for the years ended December 31, 2024, 2023 and 2022.

Consolidated Statements of Cash Flows for the years ended December 31, 2024, 2023 and 2022.

Notes to Consolidated Financial Statements.

List of financial statement schedules. All schedules are omitted because they are not applicable or the required information is shown in the financial statements or notes thereto.

List of exhibits required by Item 601 of Regulation S-K. See part (b) below.

Exhibits.

		Incorporated by Reference				
Exhibit Number	Description of Exhibit	Form	File Number	Date of Filing	Exhibit Number	Filed Herewith
2.1	Agreement and Plan of Merger, dated August 14, 2006, between the Company and Catalyst Pharmaceutical Partners, Inc., a Florida corporation	S-1	333-136039	9/1/2006	10.9	
2.2	Asset Purchase Agreement by and between Eisai Co., Ltd. and the Company, dated as of December 17, 2022	8-K	001-33057	12/22/2022	2.1	
3.1	Certificate of Incorporation	S-1	333-136039	7/25/2006	3.1	
3.2	Amendment to Certificate of Incorporation	S-1	333-136039	7/25/2006	3.2	
3.3	Amendment to Certificate of Incorporation	DEF 14A	001-33057	3/30/2015	Annex A	
3.4	Amendment to Certificate of Incorporation	8-K	001-33057	8/21/2020	3.1	
3.5	By-Laws	S-1	333-136039	9/1/2006	3.3	
3.6	Amendment to By-Laws	8-K	001-33057	11/27/2019	3.1	
4.1	Specimen Stock Certificate for Common Stock	S-1	333-136039	9/1/2006	4.1	
4.2	Description of the Company's Capital Stock	10-K	001-33057	3/16/2023	4.5	
10.1(a)+	Offer Letter between the Company and Richard J. Daly*	10-K	001-33057	2/28/2024	10.1(a)	
10.1(b)+	Offer Letter between the Company and Michael W. Kalb*	10-K	001-33057	2/28/2024	10.1(b)	

Exhibit Number	Description of Exhibit	Incorporated by Reference				Filed Herewith
		Form	File Number	Date of Filing	Exhibit Number	
10.1(c)+	Separation Agreement between the Company and Patrick J. McEnany*	10-K	001-33057	2/28/2024	10.1(c)	
10.1(d)+	Separation Agreement between the Company and Alicia Grande*	10-K	001-33057	2/28/2024	10.1(d)	
10.2(a)+	2018 Stock Incentive Plan	DEF 14A	001-33057	4/17/2018	Annex A	
10.2(b)+	Amendment No. 1 to 2018 Stock Incentive Plan	DEF 14A	001-33057	7/7/2020	Annex A	
10.2(c)+	Amendment No. 2 to 2018 Stock Incentive Plan	DEF 14A	001-33057	10/25/2021	Annex A	
10.2(d)+	Amendment No. 3 to 2018 Stock Incentive Plan	DEF 14A	001-33057	7/12/2023	Annex A	
10.2(e)+	Amendment No. 4 to 2018 Stock Incentive Plan	DEF 14A	001-33057	4/10/2024	Annex A	
10.3+	Severance and Change in Control Plan	10-K	001-33057	2/28/2024	10.4	
10.4(a)	Lease Agreement between the Company and 355 Alhambra Plaza, Ltd.	10-Q	001-33057	5/14/2007	10.1	
10.4(b)	First Amendment to Lease Agreement between the Company and CPT 355 Alhambra Circle, LLC	10-Q	001-33057	8/15/2011	10.1	
10.4(c)	Second Amendment to Lease Agreement between the Company and CPT 355 Alhambra Circle, LLC	8-K	001-33057	2/20/2014	10.1	
10.4(d)	Third Amendment to Lease Agreement between the Company and CPT 355 Alhambra Circle, LLC	8-K	001-33057	3/27/2015	10.1	
10.4(e)	Fourth Amendment to Lease Agreement between the Company and PRII 355 Alhambra Circle, LLC	8-K	001-33057	8/17/2018	10.1	
10.4(f)	Fifth Amendment to Lease Agreement between the Company and PRII 355 Alhambra Circle, LLC	8-K	001-33057	5/13/2020	10.1	
10.5	License Agreement, dated as of December 13, 2011, among New York University, the Feinstein Institute for Medical Research, and the Company	10-K	001-33057	3/30/2012	10.15	
10.6(a)	License Agreement, dated as of October 26, 2012, between the Company and BioMarin	8-K	001-33057	10/31/2012	10.2	
10.6(b)	Amendment No. 1 to License Agreement, dated as of April 8, 2014, between the Company and BioMarin	8-K	001-33057	4/17/2014	10.1	
10.6(c)	Settlement Agreement, dated effective as of July 26, 2018, by and among (i) Aceras BioMedical, LLC, in its capacity as Stockholder Representative for the Former stockholders of Huxley	10-Q	001-33057	8/17/2018	10.1	

Exhibit Number	Description of Exhibit	Incorporated by Reference				Filed Herewith
		Form	File Number	Date of Filing	Exhibit Number	
	Pharmaceuticals, Inc., (ii) BioMarin, and (iii) the Company					
10.6(d)	Second Amendment to License Agreement, dated May 29, 2019, between the Company and BioMarin	8-K	001-33057	5/30/2019	10.1	
10.7	Development, License and Commercialization Agreement, dated effective as of December 18, 2018, by and between Endo Ventures Limited and the Company	8-K	001-33057	12/26/2018	10.1	
10.8	License and Supply Agreement, dated as of August 14, 2020, by and between KYE Pharmaceuticals, Inc. and the Company	8-K	001-33057	8/20/2020	10.1	
10.9	License and Supply Agreement, dated as of June 28, 2021, by and between DyDo Pharma, Inc. and the Company	8-K	001-33057	6/28/2021	10.1	
10.10(a)	Settlement Agreement, dated July 11, 2022, by and between the Company and SERB SA, on the one hand, and Jacobus Pharmaceutical Company, Inc., PantherRx Specialty LLC, and Panther Specialty Holding Co., on the other hand	8-K	001-33057	7/12/2022	10.1	
10.10(b)	License and Asset Purchase Agreement, dated as of July 11, 2022, by and between Jacobus Pharmaceutical Company, Inc. and the Company	8-K	001-33057	7/12/2022	10.2	
10.11(a)	Transition Services Agreement between Eisai, Inc. and the Company	8-K	001-33057	12/22/2022	10.1	
10.11(b)	Amendment to Transition Services Agreement, dated as of July 31, 2023, made by and between the Company and Eisai	10-Q	001-33057	8/9/2023	10.1	
10.11(c)	Supply Agreement between Eisai Co., Ltd. and the Company	8-K	001-33057	12/22/2022	10.2	
10.12(a)	License and Collaboration Agreement, executed and delivered as of June 19, 2023, by and between Santhera, its wholly-owned subsidiary, Santhera Pharmaceuticals (Schweiz) AG and the Company	8-K	001-33057	6/23/2023	10.1	
10.12(b)	Investment Agreement, dated as of June 19, 2023, by and between Santhera and the Company	8-K	001-33057	6/23/2023	10.2	
10.13	License, Supply and Commercialization Agreement, made as of July 23, 2024, between the Company and KYE Pharmaceuticals, Inc.	10-Q	001-33057	8/7/2024	10.1	
19.1	Insider Trading Policy (certain identified information has been excluded from this					X

Exhibit Number	Description of Exhibit	Incorporated by Reference				Filed Herewith
		Form	File Number	Date of Filing	Exhibit Number	
	exhibit because it both (i) is not material and (ii) would be competitively harmful if publicly disclosed)					
21.1	Subsidiaries of the Registrant	10-K	001-33057	3/16/2020	21.1	
23.1	Consent of Grant Thornton LLP					X
31.1	Section 302 CEO Certification					X
31.2	Section 302 CFO Certification					X
32.1	Section 906 CEO Certification					X
32.2	Section 906 CFO Certification					X
97.1	Clawback Policy	10-K	001-33057	2/28/2024	97.1	
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 Inline XBRL document					
101.SCH	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents					
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101)					

***** Certain identified information has been excluded from these exhibits because it is both (i) not material, and (ii) would likely cause competitive harm to the Company if publicly disclosed.

+ Management contract or compensatory plan.

SIGNATURES

Pursuant to the requirements of Section 13 and 15(d) of the Securities Exchange Act of 1934, the Registrant has caused this Annual Report on Form 10-K to be signed by the undersigned, thereunto duly authorized, this 26th day of February, 2025.

CATALYST PHARMACEUTICALS, INC.

By: /s/ Richard J. Daly
Richard J. Daly,
President and CEO

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed by the following persons, in the capacities and on the dates indicated.

Signature	Title	Date
/s/ Richard J. Daly Richard J. Daly	President and Chief Executive Officer (Principal Executive Officer)	February 26, 2025
/s/ Michael W. Kalb Michael W. Kalb	Executive Vice President and Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	February 26, 2025
/s/ Patrick J. McEnany Patrick J. McEnany	Chairman of the Board of Directors	February 26, 2025
/s/ Charles B. O'Keeffe Charles B. O'Keeffe	Director	February 26, 2025
/s/ David S. Tierney, M.D. David S. Tierney, M.D.	Director	February 26, 2025
/s/ Donald A. Denkhaus Donald A. Denkhaus	Director	February 26, 2025
/s/ Molly Harper Molly Harper	Director	February 26, 2025
/s/ Tamar Thompson Tamar Thompson	Director	February 26, 2025

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

Board of Directors and Stockholders
Catalyst Pharmaceuticals, Inc.

Opinion on internal control over financial reporting

We have audited the internal control over financial reporting of Catalyst Pharmaceuticals, Inc. (a Delaware corporation) and subsidiary (the "Company") as of December 31, 2024, based on criteria established in the 2013 *Internal Control—Integrated Framework* issued by the Committee of Sponsoring Organizations of the Treadway Commission ("COSO"). In our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2024, based on criteria established in the 2013 *Internal Control—Integrated Framework* issued by COSO.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) ("PCAOB"), the consolidated financial statements of the Company as of and for the year ended December 31, 2024, and our report dated February 26, 2025 expressed an unqualified opinion on those financial statements.

Basis for opinion

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

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

Definition and limitations of internal control over financial reporting

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

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

/s/ GRANT THORNTON LLP

Miami, Florida
February 26, 2025

Opinion on the financial statements

We have audited the accompanying consolidated balance sheets of Catalyst Pharmaceuticals, Inc. (a Delaware corporation) and subsidiary (the "Company") as of December 31, 2024 and 2023, the related consolidated statements of comprehensive income, changes in stockholders' equity, and cash flows for each of the three years in the period ended December 31, 2024, and the related notes (collectively referred to as 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, 2024 and 2023, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2024, in conformity with accounting principles generally accepted in the United States of America.

Basis for opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's consolidated financial statements based on our audits. We are a public accounting firm registered with the 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 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 financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical audit matter

The critical audit matter communicated below is a matter arising from the current period audit of the 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 financial statements and (2) involved our especially challenging, subjective, or complex judgments. The communication of critical audit matters does not alter in any way our opinion on the 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.

Revenue adjustments for government rebates

As described further in Note 2 to the financial statements, the Company estimates reductions to its revenues for amounts due under various government rebate programs, including Medicaid and Managed Care, in the period in which the related sales occur. We identified the revenue adjustments for Medicaid and Managed Care as a critical audit matter.

The principal considerations for our determination that the revenue adjustments for Medicaid and Managed Care is a critical audit matter are that auditing the Company's reductions to revenue for Medicaid and Managed Care rebates are complex and involved significant judgment, particularly in assessing the reasonableness of estimated payor mix applied to sales during the year. This estimate relies heavily on historical data that is adjusted for changes in payor mix expectations over time.

Our audit procedures related to the revenue adjustments for Medicaid and Managed Care included the following, among others. We evaluated and tested the design and operating effectiveness of internal controls over the Company's estimates of the revenue adjustment for Medicaid and Managed Care rebates, including assumptions over payor mix. Our test of details procedures included, among others, performing a comparison of actual rebate claims received against the amounts recorded by management, performing a sensitivity analysis on the rebate amount and payor mix used in the estimates, analytically evaluating management's estimates and evaluating evidence contrary to the estimated amounts.

/s/ GRANT THORNTON LLP

We have served as the Company's auditor since 2006.

Miami, Florida
February 26, 2025

CATALYST PHARMACEUTICALS, INC.
CONSOLIDATED BALANCE SHEETS
(in thousands, except share data)

	December 31, 2024	December 31, 2023
ASSETS		
Current Assets:		
Cash and cash equivalents	$ 517,553	$ 137,636
Accounts receivable, net	65,476	53,514
Inventory	19,541	15,644
Prepaid expenses and other current assets	21,039	12,535
Total current assets	623,609	219,329
Operating lease right-of-use asset, net	2,230	2,508
Property and equipment, net	1,354	1,195
License and acquired intangibles, net	156,672	194,049
Deferred tax assets, net	45,982	36,544
Investment in equity securities	21,564	16,489
Total assets	$ 851,411	$ 470,114
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current Liabilities:		
Accounts payable	$ 16,593	$ 14,795
Accrued expenses and other liabilities	104,085	61,268
Total current liabilities	120,678	76,063
Operating lease liability, net of current portion	2,786	3,188
Other non-current liabilities	315	2,982
Total liabilities	123,779	82,233
Commitments and contingencies (Note 12)		
Stockholders' Equity:		
Preferred stock, $0.001 par value, 5,000,000 shares authorized: none issued and outstanding at December 31, 2024 and 2023	—	—
Common stock, $0.001 par value, 200,000,000 shares authorized; 120,879,099 shares and 107,121,549 shares issued and outstanding at December 31, 2024 and 2023, respectively	121	107
Additional paid-in capital	442,286	266,488
Retained earnings	285,161	121,272
Accumulated other comprehensive income (Note 4)	64	14
Total stockholders' equity	727,632	387,881
Total liabilities and stockholders' equity	$ 851,411	$ 470,114

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

CATALYST PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE INCOME
(in thousands, except share and per share data)

	Years Ended December 31,		
	2024	**2023**	**2022**
Revenues:			
Product revenue, net	$ 489,327	$ 396,502	$ 213,938
License and other revenue	2,407	1,702	265
Total revenues	491,734	398,204	214,203
Operating costs and expenses:			
Cost of sales (a)	68,845	51,967	34,393
Research and development	12,648	93,150	19,789
Selling, general and administrative (a)	177,740	133,710	57,085
Amortization of intangible assets	37,377	32,565	1,098
Total operating costs and expenses	296,610	311,392	112,365
Operating income	195,124	86,812	101,838
Other income, net	21,139	7,699	2,881
Net income before income taxes	216,263	94,511	104,719
Income tax provision	52,374	23,101	21,640
Net income	$ 163,889	$ 71,410	$ 83,079
Net income per share:			
Basic	$ 1.38	$ 0.67	$ 0.80
Diluted	$ 1.31	$ 0.63	$ 0.75
Weighted average shares outstanding:			
Basic	118,457,673	106,279,736	103,374,606
Diluted	124,943,603	113,753,154	111,375,631
Net income	$ 163,889	$ 71,410	$ 83,079
Other comprehensive income (Note 4):			
Unrealized gain (loss) on available-for-sale securities, net of tax of ($20), $4 and ($54), respectively	50	(10)	172
Comprehensive income	$ 163,939	$ 71,400	$ 83,251

(a) exclusive of amortization of intangible assets

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

CATALYST PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY
For the years ended December 31, 2024, 2023 and 2022
(in thousands)

	Preferred Stock	Common Stock Shares	Common Stock Amount	Additional Paid-in Capital	Retained Earnings (Accumulated Deficit)	Accumulated Other Comprehensive Gain (Loss)	Total
Balance at December 31, 2021	$ —	102,993	$ 103	$ 233,186	$ (26,310)	$ (148)	$ 206,831
Stock-based compensation	—	—	—	7,907	—	—	7,907
Exercise of stock options for common stock	—	3,172	2	9,567	—	—	9,569
Issuance of common stock upon vesting of restricted stock units, net	—	98	—	(230)	—	—	(230)
Repurchase of common stock	—	(1,000)	—	—	(6,907)	—	(6,907)
Other comprehensive gain (loss)	—	—	—	—	—	172	172
Net income	—	—	—	—	83,079	—	83,079
Balance at December 31, 2022	—	105,263	105	250,430	49,862	24	300,421
Stock-based compensation	—	—	—	14,250	—	—	14,250
Exercise of stock options for common stock	—	1,652	2	2,790	—	—	2,792
Issuance of common stock upon vesting of restricted stock units, net	—	207	—	(982)	—	—	(982)
Other comprehensive gain (loss)	—	—	—	—	—	(10)	(10)
Net income	—	—	—	—	71,410	—	71,410
Balance at December 31, 2023	—	107,122	107	266,488	121,272	14	387,881
Issuance of common stock, net	—	10,000	10	140,704	—	—	140,714
Stock-based compensation	—	—	—	22,251	—	—	22,251
Exercise of stock options for common stock	—	3,429	4	13,511	—	—	13,515
Issuance of common stock upon vesting of restricted stock units, net	—	328	—	(668)	—	—	(668)
Other comprehensive gain (loss)	—	—	—	—	—	50	50
Net income	—	—	—	—	163,889	—	163,889
Balance at December 31, 2024	$ —	120,879	$ 121	$ 442,286	$ 285,161	$ 64	$ 727,632

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

CATALYST PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)

	Years Ended December 31,		
	2024	**2023**	**2022**
Operating Activities:			
Net income	$ 163,889	$ 71,410	$ 83,079
Adjustments to reconcile net income to net cash provided by (used in) operating activities:			
Depreciation	397	316	141
Stock-based compensation	22,251	14,250	7,907
Amortization of intangible assets	37,377	32,565	1,098
Deferred taxes	(9,388)	(17,818)	4,937
Accretion of discount	(835)	1,320	17
Reduction in the carrying amount of right-of-use asset	278	262	247
Realized loss on sale of available-for-sale securities	—	—	762
Acquired research and development inventory expensed from asset acquisition	—	—	4,130
Acquired inventory samples expensed from asset acquisition	—	130	—
Acquired in-process research and development	—	81,513	—
Change in fair value of equity securities	(5,075)	(3,024)	—
(Increase) decrease in:			
Accounts receivable, net	(11,962)	(43,075)	(3,820)
Inventory	(3,897)	(4,739)	1,065
Prepaid expenses and other current assets	(8,504)	(5,792)	(807)
Increase (decrease) in:			
Accounts payable	1,798	10,820	1,207
Accrued expenses and other liabilities	53,848	5,800	16,391
Operating lease liability	(369)	(338)	(307)
Net cash provided by (used in) operating activities	239,808	143,600	116,047
Investing Activities:			
Purchases of property and equipment	(556)	(231)	(29)
Proceeds from sale of available-for-sale securities	—	—	19,238
Payments in connection with asset acquisitions	—	(198,293)	(10,000)
Acquisition of in-process research and development	—	(81,513)	—
Purchase of equity securities	—	(13,465)	—
Net cash provided by (used in) investing activities	(556)	(293,502)	9,209
Financing Activities:			
Payment of employee withholding tax related to stock-based compensation	(668)	(982)	(230)
Proceeds from exercise of stock options	13,515	2,792	9,569
Repurchase of common stock	—	—	(6,907)
Payment of liabilities arising from asset acquisition	(12,886)	(12,667)	(738)
Proceeds from issuance of common stock	141,000	—	—
Payment of fees in connection with issuance of common stock	(296)	—	—
Net cash provided by (used in) financing activities	140,665	(10,857)	1,694
Net increase (decrease) in cash and cash equivalents	379,917	(160,759)	126,950
Cash and cash equivalents – beginning of period	137,636	298,395	171,445
Cash and cash equivalents – end of period	$ 517,553	$ 137,636	$ 298,395
Supplemental disclosures of cash flow information:			
Cash paid for income taxes, net	$ 68,451	$ 50,458	$ 7,667
Cash paid for interest	$ 1,395	$ 705	$ —
Non-cash investing and financing activities:			
Liabilities arising from asset acquisition	$ —	$ 1,915	$ 27,699

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

1. Organization and Description of Business.

Catalyst Pharmaceuticals, Inc. and subsidiary (collectively, the Company) is a commercial-stage, patient-centric biopharmaceutical company focused on in-licensing, developing, and commercializing novel high-quality medicines for patients living with rare and difficult to treat diseases. The Company currently markets three drug products, FIRDAPSE® (amifampridine), FYCOMPA® (perampanel), and AGAMREE® (vamorolone). The Company is currently seeking to further expand its drug portfolio, with a focus on acquiring the rights to late-stage products to treat orphan, rare disease across therapeutic areas. With an unwavering patient focus embedded in everything it does, the Company is committed to providing innovative, best-in-class medications with the hope of making a meaningful impact on those affected by these conditions.

The Company's New Drug Application (NDA) for FIRDAPSE® (amifampridine) Tablets 10 mg for the treatment of adults with Lambert-Eaton myasthenic syndrome (LEMS) was approved in 2018 by the U.S. Food & Drug Administration (FDA), and FIRDAPSE® is commercially available in the U.S. as a treatment for adults with LEMS. Further, Canada's national healthcare regulatory agency, Health Canada, approved the use of FIRDAPSE® for the treatment of adult patients in Canada with LEMS in 2020 and FIRDAPSE® is commercially available in Canada for the treatment of patients with LEMS through a license and supply agreement with KYE Pharmaceuticals, Inc. (KYE). In the third quarter of 2022, the FDA approved the Company's supplemental New Drug Application approving an expansion of the FIRDAPSE® label to include pediatric patients (ages six and older). In the second quarter of 2024, the FDA approved the Company's supplemental New Drug Application increasing the indicated maximum daily dose of FIRDAPSE® (amifampridine) for adults and pediatric patients weighing more than 45 kg from 80 mg to 100 mg for the treatment of LEMS.

On December 17, 2022, the Company entered into an asset purchase agreement with Eisai Co., Ltd. (Eisai) for the acquisition of the U.S. rights to FYCOMPA® (perampanel) CIII, a prescription medication used alone or in combination with other medicines to treat focal onset seizures with or without secondarily generalized seizures in people with epilepsy aged four and older and with other medicines to treat primary generalized tonic-clonic seizures in people with epilepsy aged 12 and older. The Company closed the acquisition of the U.S. rights to FYCOMPA® on January 24, 2023 and is now marketing FYCOMPA® in the U.S.

In July 2023, the Company completed its acquisition from Santhera Pharmaceuticals Holdings (Santhera) of an exclusive license for North America for AGAMREE® (vamorolone), a treatment for patients suffering from Duchenne muscular dystrophy (DMD). The license is for exclusive commercial rights in the U.S., Canada, and Mexico, as well as the right of first negotiation in Japan should Santhera pursue partnership opportunities in that jurisdiction. Additionally, the Company holds the North American rights for any future approved indications of AGAMREE®. AGAMREE® previously received FDA Orphan Drug and Fast Track designations and on October 26, 2023, the FDA approved AGAMREE® oral suspension 40 mg/ml for the treatment of DMD in patients aged two years and older. On March 13, 2024, the Company commercially launched AGAMREE® in the U.S.

The Company has devoted substantially all its efforts since inception to selling its products, business planning, recruiting management and technical staff, acquiring operating assets, raising capital, and research and development. The Company has been able to fund its cash needs to date through profits generated from sales of its products and offerings of its securities. See Note 15 (Stockholders' Equity).

Capital Resources

Based on the Company's current financial condition, including its profitability, cash flows generated from operations and forecasts of available cash, the Company believes it has sufficient funds to support operations for at least the next 12 months.

1. Organization and Description of Business (continued).

The Company may raise funds in the future through public or private equity offerings, debt financings, corporate collaborations, governmental research grants or other means. The Company may also seek to raise new capital to fund additional business development activities, even if it has sufficient funds for its planned operations. Any sale by the Company of additional equity or convertible debt securities could result in dilution to the Company's current stockholders. There can be no assurance that any required additional funding will be available to the Company at all or available on terms acceptable to the Company.

On January 9, 2024, the Company completed a public offering of 10 million shares of its common stock, raising net proceeds of approximately $140.7 million. The proceeds of the offering will be used to acquire new products and for general corporate purposes.

2. Basis of Presentation and Significant Accounting Policies.

a. PRINCIPLES OF CONSOLIDATION. The consolidated financial statements include the Company's accounts and those of its wholly-owned subsidiary, Catalyst Pharmaceuticals Ireland, Ltd. (Catalyst Ireland). All intercompany accounts and transactions have been eliminated in consolidation. Catalyst Ireland was organized in 2017.

b. USE OF ESTIMATES. The preparation of financial statements in conformity with U.S. Generally Accepted Accounting Principles (GAAP) requires management to make estimates and assumptions that affect the amounts reported in the consolidated financial statements and accompanying notes. Actual results could differ from those estimates.

c. CASH AND CASH EQUIVALENTS. The Company primarily invests in high credit-quality instruments in order to obtain higher yields on its cash equivalents. The Company considers all highly liquid instruments, purchased with an original maturity of three months or less, to be cash equivalents. Cash equivalents consist mainly of money market funds and U.S. Treasuries. The Company has its cash and cash equivalents deposited with two financial institutions.

d. INVESTMENTS. At December 31, 2024 and 2023, investments consisted of U.S. Treasuries and an investment in equity securities. Such investments are not insured by the U.S. Federal Deposit Insurance Corporation.

U.S. Treasuries held at December 31, 2024 and 2023 were classified as available-for-sale securities. The Company classifies U.S. Treasuries with stated maturities of greater than three months and less than one year in short-term investments. U.S. Treasuries with stated maturities greater than one year are classified as non-current investments in its consolidated balance sheets.

The Company records available-for-sale securities at fair value with unrealized gains and losses reported in accumulated other comprehensive income (in stockholders' equity). Realized gains and losses are included in other income, net in the consolidated statements of operations and comprehensive income and are derived using the specific identification method for determining the cost of securities sold. Interest income is recognized when earned and is included in other income, net in the consolidated statements of operations and comprehensive income. The Company recognizes a charge when the declines in the fair value below the amortized cost basis of its available-for-sale securities are judged to be as a result of a credit loss. The Company considers various factors in determining whether to recognize an allowance for credit losses including whether the Company intends to sell the security or whether it is more likely than not that the Company would be required to sell the security before recovery of the amortized cost basis. If the unrealized loss of an available-for-sale debt security is determined to be a result of a credit loss the Company would recognize an allowance and the corresponding credit loss would be included in the consolidated statements of operations and comprehensive income. The Company has not recorded an allowance for credit loss on its available-for-sale securities. See Note 3 (Investments).

In July 2023, the Company made a strategic equity investment into Santhera by acquiring 1,414,688 of Santhera's ordinary shares (representing approximately 11.26% of Santhera's outstanding ordinary shares immediately following the transaction). The investment is denominated in Swiss Francs. The Company has determined that it does not have significant influence over the operations of Santhera and accordingly the investment in Santhera's ordinary shares is recorded under ASC 321, Equity Securities, with changes in fair value, inclusive of changes resulting from movements in foreign exchange rates, in other income, net in the consolidated statements of operations and comprehensive income.

2. Basis of Presentation and Significant Accounting Policies (continued).

e. ACCOUNTS RECEIVABLE, NET. Accounts receivable are recorded net of customer allowances for distribution fees, trade discounts, prompt payment discounts, chargebacks and expected credit losses. Allowances for distribution fees, trade discounts, prompt payment discounts and chargebacks are based on contractual terms. The Company estimates the allowance for expected credit losses based on existing contractual payment terms, actual payment patterns of its customers, current and future economic and market conditions and individual customer circumstances. The Company has not historically experienced any significant credit losses. All customer accounts are actively managed. At December 31, 2024 and 2023, the Company determined that an allowance for expected credit losses was not required. No amounts were written off during the periods presented.

f. INVENTORY. Inventories are stated at the lower of cost or net realizable value. Inventories consist of raw materials, work-in-process and finished goods. Costs to be capitalized as inventories primarily include third party manufacturing costs and other overhead costs. Cost is determined using a standard cost method, which approximates actual cost, and assumes a first-in, first out (FIFO) flow of goods. If information becomes available that suggests that inventories may not be realizable, the Company may be required to expense a portion or all of the previously capitalized inventories.

Products that have been approved by the FDA or other regulatory authorities, such as FIRDAPSE®, FYCOMPA® and AGAMREE® are also used in clinical programs to assess the safety and efficacy of the products for usage in treating diseases that have not been approved by the FDA or other regulatory authorities. The forms of FIRDAPSE®, FYCOMPA® and AGAMREE® utilized for both commercial and clinical programs are identical and, as a result, the inventories have an "alternative future use" as defined in authoritative guidance. Raw materials associated with clinical development programs are included in inventory and charged to research and development expense when the product enters the research and development process and no longer can be used for commercial purposes and, therefore, does not have an "alternative future use".

The Company evaluates for potential excess inventory by analyzing current and future product demand relative to the remaining product shelf life. The Company builds demand forecasts by considering factors such as, but not limited to, overall market potential, market share, market acceptance, and patient usage.

g. PREPAID EXPENSES AND OTHER CURRENT ASSETS. Prepaid expenses and other current assets consist primarily of prepaid manufacturing, prepaid tax, prepaid insurance, prepaid subscription fees, prepaid research fees, prepaid commercialization expenses, prepaid co-pay assistance program, amounts due from collaborative and license arrangements, and prepaid conference and travel expenses. Prepaid research fees consist of advances for the Company's product development activities, including contracts for pre-clinical studies, clinical trials and studies, regulatory affairs and consulting. Prepaid manufacturing costs consist of advances for the Company's drug manufacturing activities. Such advances are recorded as expense as the related goods are received or the related services are performed.

h. PROPERTY AND EQUIPMENT, NET. Property and equipment are recorded at cost less accumulated depreciation. Depreciation is calculated to amortize the depreciable assets over their useful lives using the straight-line method and commences when the asset is placed in service. Leasehold improvements are amortized on a straight-line basis over the term of the lease or the estimated life of the improvement, whichever is shorter. Useful lives generally range from three to five years for computer equipment and software, from five to seven years for furniture and equipment, and from five to ten years for leasehold improvements. Expenditures for repairs and maintenance are charged to expenses as incurred.

2. Basis of Presentation and Significant Accounting Policies (continued).

i. BUSINESS COMBINATIONS AND ASSET ACQUISITIONS. The Company evaluates acquisitions of assets and other similar transactions to assess whether or not the transaction should be accounted for as a business combination or asset acquisition by first applying a screen test to determine if substantially all of the fair value of the gross assets acquired is concentrated in a single identifiable asset or group of similar identifiable assets. If the screen test is met, the transaction is accounted for as an asset acquisition. If the screen test is not met, further determination is required as to whether or not the Company has acquired inputs and processes that have the ability to create outputs which would meet the requirements of a business. If determined to be an asset acquisition, the Company accounts for the transaction under ASC 805-50, which requires the acquiring entity in an asset acquisition to recognize assets acquired and liabilities assumed based on the cost to the acquiring entity on a relative fair value basis, which includes transaction costs in addition to consideration given. Goodwill is not recognized in an asset acquisition and any excess consideration transferred over the fair value of the net assets acquired is allocated to the identifiable assets based on relative fair values. Contingent consideration payments in asset acquisitions are recognized when the contingency is resolved and the consideration is paid or becomes payable.

See Notes 12 (Commitments and Contingencies) and 13 (Agreements) for further discussion of the Company's exclusive license agreement with Jacobus Pharmaceutical Company, Inc. (Jacobus), for the rights to develop and commercialize RUZURGI® in the U.S. and Mexico, which the Company accounted for as an asset acquisition under ASC 805-50. See Note 13 (Agreements) for further discussion on the Company's acquisitions of the U.S. rights to FYCOMPA® from Eisai, and on the exclusive license for North America acquired from Santhera for AGAMREE®, both of which the Company accounted for as asset acquisitions under ASC 805-50.

j. INTANGIBLE ASSETS, NET. Identifiable intangible assets with a finite life are comprised of licensed rights and other acquired intangible assets and are amortized on a straight-line basis over the respective estimated useful life.

The Company reviews intangible assets with finite lives for impairment whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. If indicators of impairment exist, an impairment test is performed to assess the recoverability of the affected assets by determining whether the carrying amount of such assets exceeds the undiscounted expected future cash flows. If the affected assets are deemed not recoverable, the Company would estimate the fair value of the assets and record an impairment loss.

k. FAIR VALUE OF FINANCIAL INSTRUMENTS. The Company's financial instruments consist of cash and cash equivalents, investments, accounts receivable, accounts payable, and certain components of accrued expenses and other liabilities. At December 31, 2024 and December 31, 2023, the fair value of these instruments approximated their carrying value as a result of their respective short-term duration.

l. FAIR VALUE MEASUREMENTS. Current Financial Accounting Standards Board (FASB) fair value guidance emphasizes that fair value is a market-based measurement, not an entity-specific measurement. Therefore, a fair value measurement should be determined based on the assumptions that market participants would use in pricing the asset or liability. As a basis for considering market participant assumptions in fair value measurements, current FASB guidance establishes a fair value hierarchy that distinguishes between market participant assumptions based on market data obtained from sources independent of the reporting entity (observable inputs that are classified within Levels 1 and 2 of the hierarchy) and the reporting entity's own assumptions that it believes market participants would use in pricing assets or liabilities (unobservable inputs classified within Level 3 of the hierarchy).

Level 1 inputs utilize quoted prices (unadjusted) in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date. Level 2 inputs are inputs other than quoted prices included in Level 1 that are observable for the asset or liability, either directly or indirectly. Level 2 inputs may include quoted prices for similar assets and liabilities in active markets, as well as inputs that are observable for the asset or liability (other than quoted prices), such as interest rates, foreign exchange rates, and yield curves that are observable at commonly quoted intervals. Level 3 inputs are unobservable inputs for the asset or liability, which are typically based on an entity's own assumptions, as there is little, if any, related market activity.

2. Basis of Presentation and Significant Accounting Policies (continued).

In instances where the determination of the fair value measurement is based on inputs from different levels of the fair value hierarchy, the level in the fair value hierarchy within which the entire fair value measurement falls is based on the lowest level input that is significant to the fair value measurement in its entirety. The Company's assessment of the significance of a particular input to the fair value measurement in its entirety requires judgment and considers factors specific to the asset or liability.

	Fair Value Measurements at Reporting Date Using (in thousands)			
	Balances as of December 31, 2024	Quoted Prices in Active Markets for Identical Assets/ Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Cash and cash equivalents:				
Money market funds	$ 109,947	$ 109,947	$ —	$ —
U.S. Treasuries	$ 329,457	$ 329,457	$ —	$ —
Investment in equity securities:				
Equity securities	$ 21,564	$ 21,564	$ —	$ —

	Balances as of December 31, 2023	Quoted Prices in Active Markets for Identical Assets/ Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Cash and cash equivalents:				
Money market funds	$ 18,256	$ 18,256	$ —	$ —
U.S. Treasuries	$ 94,523	$ 94,523	$ —	$ —
Investment in equity securities:				
Equity securities	$ 16,489	$ 16,489	$ —	$ —

m. OPERATING LEASES. The Company determines if an arrangement is a lease at inception. Operating leases are included in operating lease right-of-use (ROU) assets, net, other current liabilities, and operating lease liabilities on its consolidated balance sheets. Operating lease ROU assets and operating lease liabilities are recognized based on the present value of the future minimum lease payments over the lease term at commencement date. As the Company's lease does not provide an implicit rate, the Company uses its incremental borrowing rate based on the information available at commencement date in determining the present value of future payments. The operating lease ROU asset also includes any lease payments made and excludes lease incentives and initial direct costs incurred. The Company's lease term includes options to extend or terminate the lease, however, these options are not considered in the lease term as the Company is not reasonably certain that it will exercise these options. Lease expense for minimum lease payments is recognized on a straight-line basis over the lease term. The Company has a lease agreement with lease and non-lease components, which are accounted for separately.

n. SHARE REPURCHASES. In March 2021, the Company's Board of Directors approved a share repurchase program that authorizes the repurchase of up to $40 million of the Company's common stock. The share repurchase program currently expires in March 2025.

The Company accounts for share repurchases by charging the excess of the repurchase price over the repurchased common stock's par value entirely to retained earnings. All repurchased shares are retired and become authorized but unissued shares. The Company accrues for the shares purchased under the share repurchase plan based on the trade date. The Company may terminate or modify its share repurchase program at any time.

2. Basis of Presentation and Significant Accounting Policies (continued).

o. REVENUE RECOGNITION.

Product Revenues:

To determine revenue recognition for arrangements that are within the scope of Accounting Standards Codification (ASC) Topic 606 – Revenue from Contracts with Customers (Topic 606), the Company performs the following five steps: (i) identify the contract(s) with a customer, (ii) identify the 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 entity satisfies a performance obligation. The Company assesses the goods or services promised within each contract and determines those that are performance obligations by assessing 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. For a complete discussion of accounting for product revenue, see Product Revenue, Net below.

The Company also may generate revenues from payments received under collaborative and license agreements. Collaborative and license agreement payments may include nonrefundable fees at the inception of the agreements, contingent payments for specific achievements designated in the agreements, and/or net profit-sharing payments on sales of products resulting from the collaborative and license arrangements. For a complete discussion of accounting for collaborative and licensing arrangements, see Revenues from Collaboration and Licensing Arrangements below.

The Company recognizes revenue when its customers obtain title of the promised goods, in an amount that reflects the consideration to which the Company expects to be entitled in exchange for these goods. For FIRDAPSE® and AGAMREE®, subsequent to receiving FDA approvals, the Company entered into an arrangement with one distributor (the Customer), which is the exclusive distributor of FIRDAPSE® and AGAMREE® in the U.S. The Customer subsequently resells FIRDAPSE® and AGAMREE® to a small group of exclusive specialty pharmacies (SPs) whose dispensing activities for patients with specific payors may result in government-mandated or privately negotiated rebate obligations for the Company with respect to the purchase of FIRDAPSE® and AGAMREE®.

During 2023, the Company sold FYCOMPA® in the U.S. commercial market through a Transition Service Agreement with a U.S. subsidiary of Eisai to major wholesalers and specialty pharmaceutical distributors. These sales are often subject to contracts held with managed care organizations and government agencies. The distribution services under the Transition Services Agreement ended on December 31, 2023, and beginning on January 1, 2024, the Company commenced direct sales of FYCOMPA® in the U.S.

Product Revenue, Net: The Company recognizes revenue on product sales when its customers obtain control of the Company's products, which occur at a point in time (upon delivery or upon dispense to patient). Product revenue is recorded net of applicable reserves for variable consideration, including discounts and allowances. The Company's payment terms range between 15 and 60 days.

Shipping and handling costs for product shipments occur prior to the customer obtaining control of the goods and are recorded in cost of sales.

If taxes should be collected from the Customer relating to product sales and remitted to governmental authorities, they will be excluded from revenue. The Company expenses incremental costs of obtaining a contract when incurred if the expected amortization period of the asset that the Company would have recognized is one year or less. However, no such costs were incurred during the years ended December 31, 2024, 2023 and 2022.

During the years ended December 31, 2024, 2023 and 2022, substantially all of the Company's product revenues were from sales to customers in the U.S.

2. Basis of Presentation and Significant Accounting Policies (continued).

The following table summarizes the Company's net product revenue disaggregated by product (in thousands):

	For the Years Ended December 31,		
	2024	2023	2022
FIRDAPSE®	$ 306,035	$ 258,426	$ 213,938
FYCOMPA®+	137,251	138,076	—
AGAMREE®*	46,041	—	—
Total product revenue, net	$ 489,327	$ 396,502	$ 213,938

+ FYCOMPA® net product revenue for the year ended December 31, 2023 is for the period between January 24, 2023 (date of acquisition) and December 31, 2023.

* AGAMREE® net product revenue for the year ended December 31, 2024 is for the period between March 13, 2024 (date of commercial launch) and December 31, 2024.

Reserves for Variable Consideration: Revenue from product sales is recorded at the net sales price (transaction price), which includes estimates of variable consideration for which reserves are established. Components of variable consideration include trade discounts and allowances, prompt payment discounts, product returns, provider chargebacks and discounts, government rebates, payor rebates, and other incentives, such as voluntary patient assistance, and other allowances that are offered within contracts between the Company and its customers relating to the Company's sale of its products. These reserves, as detailed below, are based on the amounts earned, or to be claimed on the related sales, and are classified as reductions of accounts receivable (if the amount is payable to its customers) or a current liability (if the amount is payable to a party other than its customers).

These estimates take into consideration a range of possible outcomes which are probability-weighted in accordance with the expected value method in Topic 606 for relevant factors such as current contractual and statutory requirements, specific known market events and trends, industry data, and forecasted customer buying and payment patterns. Overall, these reserves reflect the Company's best estimates of the amount of consideration to which it is entitled based on the terms of the respective underlying contracts.

The amount of variable consideration which is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized under the contract will not occur in a future period. The Company's analyses also contemplates application of the constraint in accordance with the guidance, under which it determined a material reversal of revenue would not occur in a future period for the estimates detailed below as of December 31, 2024 and, therefore, the transaction price was not reduced further during the years ended December 31, 2024, 2023 and 2022. Actual amounts of consideration ultimately received may differ from the Company's estimates. If actual results in the future vary from the Company's estimates, the Company will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known.

Trade Discounts, Allowances and Wholesaler Fees: The Company provides its customers with a discount that is explicitly stated in its contract and is recorded as a reduction of revenue in the period the related product revenue is recognized. To the extent the services received are distinct from the sale of products to its customers, these payments are classified in selling, general and administrative expenses in the Company's consolidated statements of operations and comprehensive income. However, if the Company has determined such services received are not distinct from the Company's sale of products to its customers, these payments have been recorded as a reduction of revenue within the consolidated statements of operations and comprehensive income through December 31, 2024, 2023 and 2022, as well as a reduction to accounts receivable, net on the consolidated balance sheets.

Prompt Payment Discounts: The Company provides its customers with prompt payment discounts which may result in adjustments to the price that is invoiced for the product transferred, in the case that payments are made within a defined period. The prompt payment discount reserve is based on actual invoice sales and contractual discount rates. Reserves for prompt payment discounts are included in accounts receivable, net on the consolidated balance sheets.

2. Basis of Presentation and Significant Accounting Policies (continued).

Funded Co-pay Assistance Program: The Company contracts with a third party to manage the co-pay assistance program intended to provide financial assistance to qualified commercially-insured patients. The calculation of the accrual for co-pay assistance is based on an estimate of claims and the cost per claim that the Company expects to receive associated with its products, that have been recognized as revenue, but remains in the distribution channel at the end of each reporting period. These payments are considered payable to the third party vendor and the related reserve is recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included in accrued expenses and other liabilities in the consolidated balance sheets.

Product Returns: Consistent with industry practice, the Company offers its customers limited product return rights for damaged and expiring product, provided it is within a specified period around the product expiration date as set forth in the applicable individual distribution or master agreement. The Company estimates the amount of its product sales that may be returned by its customers and records this estimate as a reduction of revenue in the period in which the related product revenue is recognized. The Company currently estimates product return liabilities using available industry data and its own sales information, including its visibility into the inventory remaining in the distribution channel. Return payments related to the sale of products are considered payable to the third party vendor and the related reserve is recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included in accrued expenses and other liabilities in the consolidated balance sheets.

Provider Chargebacks and Discounts: Chargebacks for fees and discounts to providers represent the estimated obligations resulting from contractual commitments to sell products to qualified healthcare providers at prices lower than the list prices charged to the customer, who directly purchases the product from the Company. The customer charges the Company for the difference between what they paid for the product and the ultimate selling price to the qualified healthcare providers. The Company also participates in programs with government entities and other parties, including covered entities under the 340B Drug Pricing Program, whereby pricing on FYCOMPA® is extended below wholesaler list price to participating entities (the FYCOMPA® Participants). These entities purchase FYCOMPA® through wholesalers at the lower program price and the wholesalers then charge the Company the difference between their acquisition cost and the lower program price.

These reserves are established in the same period that the related revenue is recognized, resulting in a reduction of product revenue, net and accounts receivable, net. Chargeback amounts are generally determined at the time of resale to the qualified healthcare provider by the customer or at the time of a resale to a FYCOMPA® Participant by a wholesaler, and the Company generally issues credits for such amounts within a few weeks of the customer or wholesalers' notification to the Company of the resale. Reserves for chargebacks consist primarily of chargebacks that the customer or wholesalers have claimed, but for which the Company has not yet issued a credit, as well as an estimate of chargeback claims that the Company expects to receive associated with its products, that have been recognized as revenue but remains in the distribution channel at the end of each reporting period.

Government Rebates: The Company is subject to discount obligations under state Medicaid, Medicare and other government programs. These reserves are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue. For reserves related to the sale of its products, there is an establishment of a current liability, which is included in accrued expenses and other liabilities on the consolidated balance sheets. For Medicare, the Company also estimates the number of patients in the prescription drug coverage gap for whom the Company will owe an additional liability under the Medicare Part D program.

Additionally, the coverage gap program is sunsetting in 2024 and is being replaced with the Inflation Reduction Act (IRA) program. While most components of this program will begin 2025, the inflation penalty portion is effective as of 2024. Specifically, the program imposes manufacturer rebates on certain Part B and Part D drugs when prices rise faster than the rate of inflation. The Company has estimated this potential impact and has accounted for these inflation-related rebates as a reduction of product revenue in 2024 to the extent they apply to its drug portfolio. Similar to the coverage gap rebates, the associated reserve is accrued for as a current liability, which is included in accrued expenses and other liabilities on the consolidated balance sheets.

The Company's liability for these rebates consists of invoices received for claims from prior quarters that have not been paid or for which an invoice has not yet been received, estimates of claims for the current quarter, and estimated future claims that will be made for product that has been recognized as revenue, but which remains in the distribution channel inventories at the end of each reporting period.

2. Basis of Presentation and Significant Accounting Policies (continued).

Payor Rebates: The Company contracts with certain private payor organizations, primarily insurance companies and pharmacy benefit managers, for the payment of rebates with respect to utilization of its products. The Company estimates these rebates and records such estimates in the same period the related revenue is recognized, resulting in a reduction of product revenue, net and the establishment of a current liability, which is included in accrued expenses and other liabilities on the consolidated balances sheets.

Bridge and Patient Assistance Programs: The Company provides FIRDAPSE® and AGAMREE® free of charge to uninsured patients who satisfy pre-established criteria for either the Bridge Program or the Patient Assistance Program. Patients who meet the Bridge Program eligibility criteria and are transitioning from investigational product while they are waiting for a coverage determination, or later, for patients whose access is threatened by the complications arising from a change of insurer may receive a temporary supply of free FIRDAPSE® or AGAMREE® while the Company is determining the patient's third party insurance, prescription drug benefit or other third party coverage for FIRDAPSE® or AGAMREE®. The Patient Assistance Program provides FIRDAPSE® or AGAMREE® free of charge for longer periods of time for those who are uninsured or functionally uninsured with respect to FIRDAPSE® or AGAMREE® because they are unable to obtain coverage from their payor despite having health insurance, to the extent allowed by applicable law.

The Company provides FYCOMPA® free of charge to uninsured patients who satisfy pre-established criteria through a Patient Assistance Program. In addition, Catalyst provides programs to assist patients through the process for obtaining reimbursement approval for their FYCOMPA® prescriptions from their insurers. Catalyst also provides support for patients using FYCOMPA® through an Instant Savings Card Program.

The Company does not recognize any revenue related to these free products and the associated costs are classified in selling, general and administrative expenses in the Company's consolidated statements of operations and comprehensive income.

Revenues from Collaboration and Licensing Arrangements:

The Company analyzes license and collaboration arrangements pursuant to FASB ASC Topic 808, Collaborative Arrangement Guidance and Consideration (Topic 808), to assess whether such arrangements, or transactions between arrangement participants, involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards dependent on the commercial success of such activities or are more akin to a vendor-customer relationship. In making this evaluation, the Company considers whether the activities of the collaboration are considered to be distinct and deemed to be within the scope of the collaborative arrangement guidance or if they are more reflective of a vendor-customer relationship and, therefore, within the scope of Topic 606. This assessment is performed throughout the life of the arrangement based on changes in the responsibilities of all parties in the arrangement.

For elements of collaboration arrangements that are not accounted for pursuant to guidance in Topic 606, an appropriate recognition method is determined and applied consistently, generally by analogy to the revenue from contracts with customers guidance.

The Company evaluates the performance obligations promised in the contract that are based on goods and services that will be transferred to the customer and determines whether those obligations are both (i) capable of being distinct and (ii) distinct in the context of the contract. Goods or services that meet these criteria are considered distinct performance obligations. The Company estimates the transaction price based on the amount expected to be received for transferring the promised goods or services in the contract. The consideration may include fixed consideration or variable consideration. Revenue is included in product revenue, net in the Company's consolidated statements of operations and comprehensive income.

2. Basis of Presentation and Significant Accounting Policies (continued).

The agreements provide for milestone payments upon achievement of development and regulatory events. The Company accounts for milestone payments as variable consideration in accordance with Topic 606. At the inception of each arrangement that includes variable consideration, the Company evaluates the amount of potential transaction price and the likelihood that the transaction price will be received. The Company utilizes either the most likely amount method or expected value method to estimate the amount expected to be received based on which method best predicts the amount expected to be received. The amount of variable consideration that is included in the transaction price may be constrained and is included in the transaction price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period. Arrangements that include rights to additional goods or services that are exercisable at a customer's discretion are generally considered options. The Company assesses if these options provide a material right to the customer and, if so, these options are considered performance obligations. Revenue is included in license and other revenue in the Company's consolidated statements of operations and comprehensive income.

After contract inception, the transaction price is reassessed at every period end and updated for changes such as resolution of uncertain events. Any change in the overall transaction price is allocated to the performance obligations based on the same methodology used at contract inception.

The Company recognizes sales-based royalties or net profit-sharing when the latter of (a) the subsequent sale occurs, or (b) the performance obligation to which the sales-based royalty or net profit-sharing has been allocated has been satisfied. Revenue is included in license and other revenue in the Company's consolidated statements of operations and comprehensive income.

Payments to and from the collaborator are presented in the statements of operations based on the nature of the Company's business operations, the nature of the arrangement, including the contractual terms, and the nature of the payments.

See Note 11 (Collaborative and Licensing Arrangements), for further discussion on the Company's collaborative and licensing arrangements.

p. RESEARCH AND DEVELOPMENT. Costs incurred in connection with research and development activities are expensed as incurred. These costs consist of direct and indirect costs associated with specific projects, as well as fees paid to various entities that perform research-related services for the Company.

The Company records upfront and milestone payments made to third parties under licensing and collaboration arrangements that occur before a compound receives regulatory approval as acquired in-process research and development (IPR&D). IPR&D acquired as part of an asset acquisition with no alternative future use is expensed immediately to research and development. Milestone payments made after regulatory approval are capitalized as a developed asset and unless the asset is determined to have an indefinite life, the Company amortizes its definite-lived intangible assets using the straight-line method, which is considered the best estimate of economic benefit, over their estimated useful lives.

q. ADVERTISING EXPENSE. Advertising costs are expensed as incurred. The Company incurred approximately $10.0 million, $9.1 million and $3.3 million in advertising costs during the years ended December 31, 2024, 2023 and 2022, respectively, which are included in selling, general and administrative expenses in the Company's consolidated statements of operations and comprehensive income.

r. STOCK-BASED COMPENSATION. The Company recognizes expense in the consolidated statements of operations and comprehensive income for the grant date fair value of all stock-based payments to employees, directors and consultants, including grants of stock options and other share-based awards. For stock options, the Company uses the Black-Scholes option valuation model, the single-option award approach, and the straight-line attribution method. Using this approach, compensation cost is amortized on a straight-line basis over the vesting period of each respective stock option, generally one to three years. Forfeitures are recognized as a reduction of stock-based compensation expense as they occur.

2. Basis of Presentation and Significant Accounting Policies (continued).

s. CONCENTRATION OF RISK. The financial instruments that potentially subject the Company to concentration of credit risk are cash equivalents, investments and accounts receivable, net. The Company places its cash and cash equivalents with high-credit quality financial institutions. These amounts at times may exceed federally insured limits. The Company has not experienced any credit losses in these accounts.

The Company sells its products, FIRDAPSE® and AGAMREE®, in the U.S. through an exclusive distributor (its Customer) to SPs. Therefore, its distributor and SPs account for principally all of its trade receivables and net product revenues related to these products. The Company sells its product, FYCOMPA®, directly to major wholesalers and specialty pharmaceutical distributors and indirectly to managed care organizations and government agencies. The creditworthiness of its customers is continuously monitored, and the Company has internal policies regarding customer credit limits. The Company estimates an allowance for expected credit loss primarily based on the creditworthiness of its customers, historical payment patterns, aging of receivable balances and general economic conditions.

As of December 31, 2024, the Company had three FDA approved products, which makes it difficult to evaluate its current business, predict its future prospects, and forecast financial performance and growth. The Company had invested a significant portion of its efforts and financial resources in the development and commercialization of its lead product, FIRDAPSE®. The Company expects sales of FIRDAPSE®, FYCOMPA®, and AGAMREE® to constitute virtually all of the Company's product revenue for the foreseeable future.

The Company relies exclusively on third parties to formulate and manufacture its products and any future drug candidates. The commercialization of its products and any other drug candidates, if approved, could be stopped, delayed or made less profitable if those third parties fail to provide sufficient quantities of product or fail to do so at acceptable quality levels or prices. The Company does not intend to establish its own manufacturing facilities. The Company is using the same third party contractors to manufacture, supply, store and distribute drug supplies for clinical trials and for the commercialization of FIRDAPSE®. The Company relies on the same third party manufacturers for FYCOMPA® as utilized by Eisai prior to the Company's acquisition of the U.S. rights to the product in January 2023. It also relies on Santhera and its supplier as its sole source of supply for AGAMREE®. If the Company is unable to continue its relationships with one or more of these third party contractors, it could experience delays in the development or commercialization efforts as it locates and qualifies new manufacturers. The Company intends to rely on one or more third party contractors to manufacture the commercial supply of its drugs.

The following table illustrates the approximate percentage of the Company's total net product revenue attributed to the Company's largest customers for the periods presented:

	For the Years Ended December 31,		
	2024	2023	2022
Customer A	72.0%	65.2%	100.0%
Customer B*	—	34.8%	—
Total	72.0%	100.0%	100.0%

* During 2023, the Company sold FYCOMPA® through a Transition Service Agreement with a U.S. subsidiary of Eisai. Effective January 1, 2024, FYCOMPA® is being sold and distributed through a third party logistics (3PL) organization under the Company's contracts.

t. ROYALTIES. Royalties incurred in connection with the Company's license agreement for FIRDAPSE® and AGAMREE®, as disclosed in Note 13 (Agreements), are expensed to cost of sales as revenue from product sales is recognized.

Royalties incurred in connection with the Company's license agreement for RUZURGI®, as disclosed in Note 13 (Agreements), are expensed to cost of sales as revenue from product sales is recognized for any royalties in excess of the minimum annual royalty payment from July 11, 2022 (the Effective Date) through 2025. The minimum royalty payment that exists annually for calendar years from the Effective Date through 2025 of $3 million are included in the purchase price of the agreement.

2. Basis of Presentation and Significant Accounting Policies (continued).

u. INCOME TAXES. The Company utilizes the asset and liability method of accounting for income taxes. Under this method, deferred tax assets and liabilities are determined based on differences between the financial reporting and tax basis of assets and liabilities and are measured using enacted tax rates and laws that will be in effect when the differences are expected to reverse. A valuation allowance is provided when it is more likely than not that some portion or all of a deferred tax asset will not be realized.

The Company recognizes the financial statement benefit of a tax position only after determining that the relevant tax authority would more likely than not sustain the position following an audit. For tax positions meeting the more-likely-than-not threshold, the amount recognized in the financial statements is the largest benefit that has a greater than 50 percent likelihood of being realized upon ultimate settlement with the relevant tax authority. The Company is subject to income taxes in the U.S. federal jurisdiction and various state jurisdictions. Tax regulations within each jurisdiction are subject to the interpretation of the related tax laws and regulations and require significant judgment to apply. The Company is not subject to U.S. federal, state and local tax examinations by tax authorities for years before 2021. If the Company were to subsequently record an unrecognized tax benefit, associated penalties and tax related interest expense would be reported as a component of income tax expense.

v. COMPREHENSIVE INCOME. U.S. GAAP requires that all components of comprehensive income be reported in the financial statements in the period in which they are recognized. Comprehensive income is net income, plus certain other items that are recorded directly into stockholders' equity. The Company's comprehensive income is shown on the consolidated statements of operations and comprehensive income for the years ended December 31, 2024, 2023 and 2022, and is comprised of net unrealized gains (losses) on the Company's available-for-sale securities.

w. NET INCOME PER COMMON SHARE. Basic net income per share is computed by dividing net income for the period by the weighted average number of common shares outstanding during the period. With regard to common stock subject to vesting requirements, the calculation includes only the vested portion of such stock and units.

Diluted net income per common share is computed by dividing net income by the weighted average number of common shares outstanding, increased by the assumed conversion of other potentially dilutive securities during the period.

The following table reconciles basic and diluted weighted average common shares:

	For the Years Ended December 31,		
	2024	2023	2022
Basic weighted average common shares outstanding	118,457,673	106,279,736	103,374,606
Effect of dilutive securities	6,485,930	7,473,418	8,001,025
Diluted weighted average common shares outstanding	124,943,603	113,753,154	111,375,631

Outstanding common stock equivalents totaling approximately 6.7 million, 4.5 million and 1.0 million, were excluded from the calculation of diluted net income per common share for the years ended December 31, 2024, 2023 and 2022, respectively, as their effect would be anti-dilutive. Potentially dilutive options to purchase common stock as of December 31, 2024, 2023 and 2022 had exercise prices ranging from $1.13 to $14.23, $0.79 to $7.10 and $0.79 to $7.07, respectively.

2. Basis of Presentation and Significant Accounting Policies (continued).

x. SEGMENT INFORMATION. Management has determined that the Company operates in one reportable segment, which is the development and commercialization of drug products. The Company's chief operating decision maker (CODM) is its president and chief executive officer, who reviews financial information presented on a consolidated basis. The CODM uses consolidated operating margin (operating income divided by product revenue, net) and net income to assess financial performance and allocate resources. These financial metrics are used by the CODM to make key operating decisions, such as the determination of the rate at which the Company seeks to grow operating margin and the allocation of budget between cost of revenues, selling, research and development, and general and administrative expenses.

The following table illustrates information about significant segment expenses:

	For the Years Ended December 31,		
	2024	2023	2022
Research and development	$ 12,648	$ 93,150	$ 19,789
Selling	118,746	91,500	31,044
General and administrative (a)	58,994	42,210	26,041
Total	$ 190,388	$ 226,860	$ 76,874

(a) exclusive of amortization of intangible assets

y. RECLASSIFICATIONS. Certain prior year amounts in the consolidated financial statements have been reclassified to conform to the current year presentation.

z. RECENTLY ISSUED ACCOUNTING STANDARDS. In November 2023, the FASB issued ASU No. 2023-07, *Improvements to Reportable Segment Disclosures* (ASU 2023-07) which is intended to improve reportable segment disclosures primarily through enhanced disclosure of reportable segment expenses and requires that a public entity that has a single reportable segment provide all the disclosures required by ASU 2023-07 and all existing segment disclosures in Topic 280. The new guidance is required to be applied retrospectively to all prior periods presented in the financial statements and is effective for the Company for fiscal periods beginning after December 15, 2023 and interim periods within fiscal years beginning after December 15, 2024. The Company adopted ASU 2023-07 during the year ended December 31, 2024.

In December 2023, the FASB issued ASU No. 2023-09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures* which requires significant disclosures about income taxes, primarily focused on the disclosure of income taxes paid and the rate reconciliation table. The new guidance will be applied prospectively and is effective for the Company for fiscal periods beginning after December 15, 2024. The Company is evaluating the impact of the standard on the Company’s consolidated financial statements.

In November 2024, the FASB issued ASU 2024-03, *Income Statement: Reporting Comprehensive Income—Expense Disaggregation Disclosures (Subtopic 220-40)* which requires more detailed information about specified categories of expenses (purchases of inventory, employee compensation, depreciation, amortization, and depletion) included in certain expense captions presented on the face of the income statement, as well as disclosures about selling expenses. This ASU is effective for fiscal years beginning after December 15, 2026 and for interim periods within fiscal years beginning after December 15, 2027. Early adoption is permitted. The amendments may be applied either (1) prospectively to financial statements issued for reporting periods after the effective date of this ASU or (2) retrospectively to all prior periods presented in the financial statements. The Company is currently evaluating this guidance to determine the impact it may have on its consolidated financial statements disclosures.

3. Investments.

Available-for-sale investments by security type were as follows (in thousands):

	Estimated Fair Value	Gross Unrealized Gains	Gross Unrealized Losses	Amortized Cost
At December 31, 2024:				
U.S. Treasuries - Cash equivalents	$ 329,457	$ 84	$ —	$ 329,373
Total	$ 329,457	$ 84	$ —	$ 329,373
At December 31, 2023:				
U.S. Treasuries - Cash equivalents	$ 94,523	$ 18	$ —	$ 94,505
Total	$ 94,523	$ 18	$ —	$ 94,505

There were no realized gains or losses from available-for-sale securities for the years ended December 31, 2024 or 2023. There were realized losses from sale of available-for-sale securities of $762 thousand for the year ended December 31, 2022.

The estimated fair values of available-for-sale securities at December 31, 2024, by contractual maturity, are summarized as follows (in thousands):

	2024
Due in one year or less	$ 329,457

	For the Years Ended December 31,		
	2024	2023	2022
Equity securities:			
Net gains (losses) recognized during the period on equity securities	$ 5,075	$ 3,024	$ —
Unrealized net gains (losses) recognized during the period on equity securities still held at the reporting date	$ 5,075	$ 3,024	$ —

There were no sales of equity securities during the years ended December 31, 2024, 2023 and 2022.

4. Accumulated Other Comprehensive Income.

The following table summarizes the changes in accumulated other comprehensive income, net of tax from unrealized gains (losses) on available-for-sale securities, the Company's only component of accumulated other comprehensive income for the years ended December 31, 2024, 2023 and 2022.

The amount reclassified out of accumulated other comprehensive income, net of tax and into net income during the year ended December 31, 2022, was solely due to a realized loss from sale of available-for-sale securities. There were no reclassifications out of accumulated other comprehensive income during the years ended December 31, 2024 or 2023.

	Total Accumulated Other Comprehensive Income
Balance at December 31, 2022	$ 24
Other comprehensive gain (loss) before reclassifications	(10)
Net current period other comprehensive gain	(10)
Balance at December 31, 2023	$ 14
Other comprehensive gain (loss) before reclassifications	50
Net current period other comprehensive gain	50
Balance at December 31, 2024	$ 64

5. Inventory.

Inventory consists of the following (in thousands):

	December 31, 2024	December 31, 2023
Raw materials	$ 6,518	$ 1,910
Work-in-process	3,445	4,573
Finished goods	9,578	9,161
Total inventory	$ 19,541	$ 15,644

6. Prepaid Expenses and Other Current Assets.

Prepaid expenses and other current assets consist of the following (in thousands):

	December 31, 2024	December 31, 2023
Prepaid manufacturing costs	$ 206	$ 2,005
Prepaid tax	7,959	1,238
Prepaid insurance	1,660	1,332
Prepaid subscriptions fees	1,233	1,299
Prepaid research fees	1,135	1,500
Prepaid commercialization expenses	4,957	3,038
Due from collaborative and licensing arrangements	11	138
Prepaid conference and travel expenses	1,287	771
Prepaid co-pay assistance program	1,561	863
Other	1,030	351
Total prepaid expenses and other current assets	$ 21,039	$ 12,535

7. Operating Leases.

The Company has an operating lease agreement for its corporate office. The lease includes an option to extend the lease for up to 5 years and options to terminate the lease within 6 and 7.6 years. The Company has no obligations under finance leases.

The Company entered into an agreement in May 2020 that amended its lease for its office facilities. Under the amended lease, the Company's leased space increased from approximately 7,800 square feet of space to approximately 10,700 square feet of space. The amended lease commenced in March 2021 when construction of the asset was completed and space became available for use.

The components of lease expense were as follows (in thousands):

	For the Years Ended December 31,	
	2024	2023
Operating lease cost	$ 431	$ 431

Supplemental cash flow information related to lease was as follows (in thousands):

	For the Years Ended December 31,	
	2024	2023
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows	$ 522	$ 506
Right-of-use assets obtained in exchange for lease obligations:		
Operating lease	$ 89	$ 89

Supplemental balance sheet information related to lease was as follows (in thousands):

	December 31, 2024	December 31, 2023
Operating lease right-of-use assets, net	$ 2,230	$ 2,508
Other current liabilities	$ 402	$ 369
Operating lease liabilities, net of current portion	2,786	3,188
Total operating lease liabilities	$ 3,188	$ 3,557

As of December 31, 2024 and December 31, 2023, the weighted average remaining lease term was 6.3 years and 7.3 years, respectively. The weighted average discount rate used to determine the operating lease liabilities was 4.51% as of December 31, 2024 and 2023.

Remaining payments of lease liabilities as of December 31, 2024 were as follows (in thousands):

2025	$ 537
2026	553
2027	570
2028	587
2029	605
Thereafter	835
Total lease payments	3,687
Less: imputed interest	(499)
Total	$ 3,188

Rent expense was approximately $0.4 million for the years ended December 31, 2024, 2023 and 2022.

8. Property and Equipment, Net.

Property and equipment, net consists of the following (in thousands):

	December 31, 2024	December 31, 2023
Furniture and equipment	$ 1,050	$ 494
Leasehold improvements	991	991
Software	433	433
Less: Accumulated depreciation	(1,120)	(723)
Total property and equipment, net	$ 1,354	$ 1,195

9. License and Acquired Intangibles, Net.

The following table presents the Company's intangible assets at December 31, 2024 (in thousands):

	Gross Carrying Value	Accumulated Amortization	Net Carrying Value
Intangible assets:			
License and acquired intangibles for RUZURGI®	$ 33,569	$ 5,739	$ 27,830
License and acquired intangibles for FYCOMPA®	158,143	61,301	96,842
License and acquired intangibles for AGAMREE®	36,000	4,000	32,000
Total	$ 227,712	$ 71,040	$ 156,672

The following table presents the Company's intangible assets at December 31, 2023 (in thousands):

	Gross Carrying Value	Accumulated Amortization	Net Carrying Value
Intangible assets:			
License and acquired intangibles for RUZURGI®	$ 33,569	$ 3,418	$ 30,151
License and acquired intangibles for FYCOMPA®	158,143	29,673	128,470
License and acquired intangibles for AGAMREE®	36,000	572	35,428
Total	$ 227,712	$ 33,663	$ 194,049

The Company amortizes its definite-lived intangible assets using the straight-line method, which is considered the best estimate of economic benefit, over its estimated useful life. The estimated useful life used for this purpose for RUZURGI®, FYCOMPA® and AGAMREE® was approximately 14.5 years, 5 years and 10.5 years, respectively.

The Company recorded approximately $2.3 million, $2.3 million and $1.1 million in amortization expense related to the licensed and acquired intangibles for RUZURGI® during the years ended December 31, 2024, 2023 and 2022, respectively, within selling, general and administrative expenses in the consolidated statements of operations and comprehensive income. The Company recorded approximately $31.6 million, $29.7 million and $0 in amortization expense related to the licensed and acquired intangibles for FYCOMPA® during the years ended December 31, 2024, 2023 and 2022, respectively, within cost of sales in the consolidated statements of operations and comprehensive income. The Company recorded approximately $3.4 million, $0.6 million and $0 in amortization expense related to the licensed and acquired intangibles for AGAMREE® during the years ended December 31, 2024, 2023 and 2022, respectively, within cost of sales in the consolidated statements of operations and comprehensive income.

9. License and Acquired Intangibles, Net (continued).

The following table presents future amortization expense the Company expects for its intangible assets (in thousands):

2025	$ 37,378
2026	37,378
2027	37,378
2028	7,705
2029	5,750
Thereafter	31,083
Total	$ 156,672

At December 31, 2024 and December 31, 2023, the weighted average amortization period remaining for intangible assets was 5.4 years and 6.5 years, respectively.

There were no impairment charges recognized on definite-lived intangibles for the years ended December 31, 2024, 2023 or 2022.

10. Accrued Expenses and Other Liabilities.

Accrued expenses and other liabilities consist of the following as of December 31 (in thousands):

	2024	2023
Accrued preclinical and clinical trial expenses	$ 267	$ 1,015
Accrued professional fees	11,011	4,730
Accrued compensation and benefits	10,746	8,883
Accrued license fees	30,991	24,437
Accrued purchases	447	192
Operating lease liability	402	369
Accrued gross-to-net revenue liabilities*	44,939	6,877
Accrued income tax	894	729
Due to licensor	3,582	12,540
Accrued interest payable	389	1,031
Other	417	465
Current accrued expenses and other liabilities	104,085	61,268
Lease liability – non-current	2,786	3,188
Due to licensor – non-current	—	2,497
Other – non-current	315	485
Non-current accrued expenses and other liabilities	3,101	6,170
Total accrued expenses and other liabilities	$ 107,186	$ 67,438

* During 2023, the Company sold FYCOMPA® through a Transition Service Agreement with Eisai. Effective January 1, 2024, FYCOMPA® is being sold and distributed through a 3PL organization under the Company's contracts.

11. Collaborative and Licensing Arrangements.

Endo

In December 2018, the Company entered into a collaboration and license agreement (Collaboration) with Endo, Inc. (formerly, Endo International plc) (Endo), for the further development and commercialization of generic Sabril® (vigabatrin) tablets through Endo's U.S. Generic Pharmaceuticals segment, doing business as Par Pharmaceutical, Inc. (Par). Under the Collaboration, Endo assumed all development, manufacturing, clinical, regulatory, sales and marketing costs under the collaboration, while the Company was responsible for exercising commercially reasonable efforts to develop, or cause the development of, a final finished, stable dosage form of generic Sabril® tablets.

In July 2024, a termination and mutual release agreement was finalized between Endo and the Company that discontinued work on the collaboration for development and commercialization of vigabatrin. The end of the collaboration does not have a material impact on the Company's consolidated financial statements.

KYE Pharmaceuticals Inc.

In August 2020, the Company entered into a collaboration and license agreement with KYE, for the commercialization of FIRDAPSE® in Canada.

Under the agreement, Catalyst granted KYE an exclusive license to commercialize and market FIRDAPSE® in Canada. KYE assumes all selling and marketing costs under the collaboration, while the Company is responsible for supply of FIRDAPSE® based on the collaboration partner's purchase orders.

Under the terms of the agreement, the Company received an up-front payment, has received payment upon transfer of Marketing Authorization and delivery of commercial product, received payment for supply of FIRDAPSE®, and will receive milestone payments and a sharing of defined net profits upon commercialization from KYE consisting of a mid-double-digit percent of net sales of FIRDAPSE®. The Company has also agreed to the sharing of certain development expenses. Unless terminated earlier in accordance with its terms, the collaboration continues in effect until the date that is ten years following the commercial launch of the product in Canada.

The collaborative agreement included a nonrefundable upfront license fee that was recognized upon transfer of the license based on a determination that the right is provided as the intellectual property existed at the point in time in which the license was granted.

In July 2024, the Company entered into a license, supply and commercialization agreement with KYE, for the commercialization of AGAMREE® in Canada granting KYE the exclusive Canadian commercial rights to market AGAMREE® in Canada for DMD and other indications.

Under the agreement, KYE will be responsible for obtaining regulatory approval of the product from Health Canada and the Company will supply product to KYE. Further, the Company received an upfront payment from KYE and will be eligible to receive further reimbursement, sales milestones and sales royalties for AGAMREE®.

These agreements are in form identified as collaborative agreements and the Company has concluded for accounting purposes that they also represent contracts with a customer. This is because the Company grants to KYE a license and provides supply of FIRDAPSE® and AGAMREE® in exchange for consideration, which are outputs of the Company's ongoing activities. Accordingly, the Company has concluded that these collaborative arrangements will be accounted for pursuant to Topic 606.

Revenue from sales by KYE is recognized in the quarter in which the sales occurred.

Revenues from the arrangements with KYE for the years ended December 31, 2024, 2023 and 2022 were not material. Revenue is included in license and other revenue in the accompanying consolidated statements of operations and comprehensive income. Expenses incurred, net have been included in selling, general and administrative expenses in the accompanying consolidated statements of operations and comprehensive income.

11. Collaborative and Licensing Arrangements (continued).

DyDo Pharma, Inc.

On June 28, 2021, the Company entered into a license agreement with DyDo Pharma, Inc. (DyDo), for the development and commercialization of FIRDAPSE® in Japan.

Under the agreement, DyDo has joint rights to develop FIRDAPSE®, and exclusive rights to commercialize the product, in Japan. DyDo is responsible for funding all clinical, regulatory, marketing and commercialization activities in Japan, while the Company is responsible for clinical and commercial supply based on purchase orders, as well as providing support to DyDo in its efforts to obtain regulatory approval for the product from the Japanese regulatory authorities.

Under the terms of the agreement, the Company has earned an up-front payment and certain regulatory milestones and may earn sales-based milestones for FIRDAPSE®, as well as revenue on product supplied to DyDo.

The Company has concluded that this license agreement will be accounted for pursuant to Topic 606. The agreement included a nonrefundable upfront license fee that was recognized upon the effective date of the agreement as the intellectual property existed at the point in time in which the right to the license was granted. The Company determined the granting of the right to the license is distinct from the supply of FIRDAPSE® and represents a separate performance obligation in the agreement.

The agreement includes milestones that are considered a sales-based royalty in which the license is deemed to be the predominant item to which these milestones relate. Revenue will be recognized when the latter of (a) the subsequent sale occurs, or (b) the performance obligation to which the sales-based royalty has been allocated has been satisfied. Additionally, the agreement includes regulatory milestone payments which represent variable consideration, and due to uncertainty are fully constrained and only recognized when the uncertainty is subsequently resolved. For clinical and commercial supply of the product, the Company will recognize revenue when the Customer obtains control of the Company's product, which will occur at a point in time which is generally at time of shipment.

There were revenues of $3.1 million from the arrangement with DyDo for the year ended December 31, 2024, of which $1.0 million is included in product revenue, net in the accompanying consolidated statements of operations and comprehensive income and $2.1 million related to a milestone payment earned upon DyDo receiving regulatory approval to commercialize FIRDAPSE® for the treatment of patients with LEMS in Japan, which is included in license and other revenue in the accompanying consolidated statements of operations and comprehensive income. There were revenues of $1.9 million from the arrangement with DyDo for the year ended December 31, 2023, of which $0.5 million was included in product revenue, net in the accompanying consolidated statements of operations and comprehensive income and $1.4 million related to a regulatory filing milestone in Japan, which was included in licensing and other revenue in the accompanying consolidated statements of operations and comprehensive income. There were revenues of $0.5 million from the arrangement with DyDo for the year ended December 31, 2022, which was included in product revenue, net in the accompanying consolidated statements of operations and comprehensive income.

Finally, on September 24, 2024, DyDo advised the Company that the Ministry of Health, Labour and Welfare (MHLW) had approved DyDo's Japan NDA to commercialize FIRDAPSE® for the treatment of patients with LEMS. See Note 18 (Subsequent Events).

12. Commitments and Contingencies.

In May 2019, the FDA approved a NDA for RUZURGI®, Jacobus Pharmaceuticals' version of amifampridine (3,4-DAP), for the treatment of pediatric LEMS patients (ages 6 to under 17). In June 2019 the Company filed suit against the FDA and several related parties challenging this approval and related drug labeling. Jacobus later intervened in the case. The Company ultimately prevailed in its litigation in September 2021 when the U.S. Court of Appeals for the 11th Circuit determined that the FDA's approval of RUZURGI® violated the Company's rights to Orphan Drug Exclusivity.

On July 11, 2022, the Company settled certain of its disputes with Jacobus. In connection with the settlement, the Company licensed the rights to develop and commercialize RUZURGI® in the U.S. and Mexico (the Territory). Simultaneously, the Company purchased, among other intellectual property rights, Jacobus' U.S. patents related to RUZURGI®, its new drug applications in the U.S. for RUZURGI®, and certain RUZURGI® inventory previously manufactured by Jacobus. At the same time, the Company received a license from Jacobus for use of its know-how related to the manufacture of RUZURGI®. Further, the Company settled its patent case against Jacobus, which was dismissed without prejudice. Finally, Jacobus agreed that until the later of (i) the expiration of the royalty term or (ii) December 31, 2034, Jacobus and its affiliates, will not, directly or indirectly, research, develop, manufacture, commercialize, distribute, use or otherwise exploit any product competitive to FIRDAPSE® or RUZURGI® in the Territory, and Laura Jacobus, the sole shareholder of Jacobus, and two of Jacobus' other officers, also signed individual non-competition agreements containing the same terms.

In connection with the settlement with Jacobus, the Company paid the following consideration to Jacobus:

- $30 million of cash, of which $10 million was paid at the closing of the settlement, $10 million was paid on the first anniversary of the closing, and the remaining $10 million was paid on the second anniversary of the closing; and
- An annual royalty on the Company's net sales (as defined in the License and Asset Purchase Agreement between Catalyst and Jacobus) of amifampridine products in the U.S. equal to: (a) for calendar years 2022 through 2025, 1.5% (with a minimum annual royalty of $3.0 million per year), and (b) for calendar years 2026 through the expiration of the last to expire of the Company's FIRDAPSE® patents in the U.S., 2.5% (with a minimum annual royalty of $5 million per year); provided, however, that the royalty rate may be reduced and the minimum annual royalty may be eliminated under certain circumstances.

In January 2023, the Company received Paragraph IV Certification Notice Letters from three generic drug manufacturers (Teva Pharmaceuticals USA, Inc. and Teva Pharmaceuticals, Inc. (collectively Teva), Hetero USA, Inc. (Hetero), and Lupin Pharmaceuticals, Inc. (Lupin)) advising that they had each submitted an Abbreviated New Drug Application (ANDA) to the FDA seeking authorization from the FDA to manufacture, use or sell a generic version of FIRDAPSE® in the U.S. The notice letters each alleged that the six patents listed in the FDA Orange Book covering FIRDAPSE® are not valid, not enforceable, and/or will not be infringed by the commercial manufacture, use or sale of the proposed product described in these ANDA submissions. Under the FDCA, as amended by the Drug Price Competition and Patent Term Restoration Act of 1984, the Company had 45 days from receipt of the notice letters to commence patent infringement lawsuits against these generic drug manufacturers in a federal district court to trigger a stay precluding the FDA from approving any ANDA until May 2026 or entry of judgment holding the patents invalid, unenforceable, or not infringed, whichever occurs first. In that regard, after conducting the necessary due diligence, the Company filed lawsuits on March 1, 2023 in the U.S. District Court for the District of New Jersey against each of the three generic drug manufacturers who notified the Company of their ANDA submissions, thus triggering the stay.

In June 2024, Lupin converted five of its Paragraph IV Certifications in its ANDA to Paragraph III certifications acknowledging the validity and their ANDA's infringement of five of those patents, the latest ending in 2034. The Company subsequently dismissed all of its claims against Lupin related to those five patents but maintains its claims against Lupin for the remaining Paragraph IV certification for U.S. Patent No. 10,626,088 which is the patent expiring in 2037, so the litigation continues.

12. Commitments and Contingencies (continued).

Further, on January 8, 2025, the Company reached a settlement with Teva in which Teva agreed not to market a generic version of FIRDAPSE® in the U.S. any earlier than February 25, 2035, if approved by the FDA, unless certain limited circumstances customarily included in these types of agreements occur. In accordance with the settlement agreement, the parties terminated all ongoing patent litigation between the Company and Teva regarding FIRDAPSE® patents pending in the U.S. District Court for the District of New Jersey. See Note 18 (Subsequent Events).

The pending FIRDAPSE® patent litigation against the remaining defendants, Hetero (for all of FIRDAPSE®'s Orange Book-listed patents) and Lupin (only for FIRDAPSE® patent expiring in 2037), remains ongoing, and there can be no assurance as to whether the currently ongoing litigation with Hetero and Lupin will allow a generic version of FIRDAPSE® to be marketed in the U.S. prior to February 25, 2035.

Finally, in October 2023, the Company received a Paragraph IV Certification Notice Letter from a fourth generic drug manufacturer (Inventia Life Science Pty Ltd (Inventia)), and the Company filed a similar lawsuit against that manufacturer in November 2023. On July 30, 2024, the Company settled its patent litigation with Inventia for FIRDAPSE®. In that settlement, Inventia acknowledged both the validity of the Company's FIRDAPSE® patents and also the infringement by the ANDA filer's product of the Company's patents. As part of the settlement, Inventia also agreed not to commercialize its product until the earlier of all FIRDAPSE® patents expiration scheduled for February 2037, or the earlier entry into the market of another ANDA product meeting certain conditions.

The outcome of patent litigation with Paragraph IV challengers is always uncertain and there can be no assurance that the Company will prevail in these litigations.

On February 20, 2023, the Company received a Paragraph IV Certification Notice Letter from a company that appears to have filed the first ANDA for the oral suspension formulation for FYCOMPA®. The same company sent a similar letter to the Company later in February with a similar certification for the tablet formulation for FYCOMPA®. Similar to the actions with the FIRDAPSE® Paragraph IV Certifications described above, after due diligence the Company filed lawsuits on April 5, 2023, in the U.S. District Court for the District of New Jersey against the drug manufacturer who notified the Company of their ANDA submissions for both FYCOMPA® formulations, thus triggering the 30-month stay for each application. This lawsuit was settled in June 2024. As part of this settlement, this Paragraph IV filer agreed not to commercialize their proposed ANDA products for both the oral suspension formulation of FYCOMPA® and for FYCOMPA® tablets until at least December 15, 2025.

Additionally, from time to time the Company may become involved in legal proceedings arising in the ordinary course of business. Except as set forth above, the Company believes that there is no other litigation pending at this time that could have, individually or in the aggregate, a material adverse effect on its results of operations, financial condition, or cash flows.

13. Agreements.

a. LICENSE AGREEMENT FOR FIRDAPSE®. On October 26, 2012, the Company entered into a license agreement with BioMarin Pharmaceutical, Inc. (BioMarin) for the North American rights to FIRDAPSE®. Under the license agreement, the Company pays: (i) royalties to the licensor for seven years from the first commercial sale of FIRDAPSE® equal to 7% of net sales (as defined in the license agreement) in North America for any calendar year for sales up to $100 million, and 10% of net sales in North America in any calendar year in excess of $100 million; and (ii) royalties to the third party licensor of the rights sublicensed to the Company for seven years from the first commercial sale of FIRDAPSE® equal to 7% of net sales (as defined in the license agreement between BioMarin and the third party licensor) in any calendar year for the duration of any regulatory exclusivity within a territory and 3.5% for territories in any calendar year in territories without regulatory exclusivity.

On May 29, 2019, the Company and BioMarin entered into an amendment to the Company's license agreement for FIRDAPSE®. Under the amendment, the Company has expanded its commercial territory for FIRDAPSE®, which originally was comprised of North America, to include Japan. Additionally, the Company's commercial territory was expanded under the license agreement in December 2023 to include most of Asia, as well as Latin America, upon the acceptance by the Pharmaceuticals and Medical Devices Agency (PMDA) of a Japan MAA for FIRDAPSE® for LEMS. Under the amendment, the Company will pay royalties to its licensor on net sales in Japan of a similar percentage to the royalties that the Company is currently paying under its original license agreement for North America.

In January 2020, the Company was advised that BioMarin has transferred substantially all of its rights under the license agreement to SERB S.A. (SERB), and SERB is now the Company's licensor under the license agreement.

b. LICENSE AGREEMENT FOR RUZURGI®. On July 11, 2022 (the Effective Date), the Company entered into an exclusive license agreement with Jacobus Pharmaceutical Company, Inc. (Jacobus), for the rights to develop and commercialize RUZURGI® in the U.S. and Mexico.

Pursuant to the terms of the license agreement, the Company paid Jacobus a $10 million up-front payment on the Effective Date, $10 million on the first annual anniversary of the Effective Date (July 11, 2023), and $10 million on the second annual anniversary of the Effective Date (July 11, 2024). The Company is also obligated to pay tiered royalty payments on net sales (as defined in the license agreement) of all of the Company's amifampridine products in the U.S. that range from 1.25% to 2.5% based on whether there is a competing product or generic version of FIRDAPSE® being marketed or sold in the U.S.

A minimum royalty payment exists annually for calendar years from the Effective Date through 2025 of $3 million, provided that such minimum annual royalty payment shall be prorated in the first calendar year of the agreement. As these minimum payments are both probable and estimable, they are included in the purchase price of the agreement and any royalties in excess of this amount will be charged to cost of sales as revenue from product sales is recognized. A minimum royalty payment exists annually for calendar years from 2026 through the expiration of the royalty term (which ends when there is no valid claim under the Company's FIRDAPSE® patents in the U.S.) of $5 million unless a competing product or generic version of FIRDAPSE® is being marketed or sold in the U.S. If these minimum payments become probable in the future, the Company would recognize a contingent liability at that time with an offset to the value of the intangible asset acquired. Any royalties in excess of this amount will be charged to cost of sales as revenue from product sales is recognized. Royalties over the minimum, if any, will be paid based on the agreement terms on a quarterly basis.

Assets acquired as part of the license agreement include among other intellectual property rights, Jacobus' U.S. patents related to RUZURGI®, its new drug applications in the U.S. for RUZURGI®, its U.S. Trademark for RUZURGI®, the Orphan Drug Designation for RUZURGI® and a license from Jacobus for use of its know-how related to the manufacture of RUZURGI®.

Additionally, the Company also purchased from Jacobus approximately $4.1 million of RUZURGI® inventory previously manufactured by Jacobus, which was recorded as an expense in research and development expenses in the consolidated statements of operations and comprehensive income for 2022.

13. Agreements (continued).

Under business combination guidance, the screen test states that if substantially all of the fair value of the gross assets acquired is concentrated in a single identifiable asset or group of similar identifiable assets, the set is not considered a business and is accounted for as an asset acquisition. The Company has determined that the screen test was not met. However, the Company determined that the acquisition did not meet the definition of a business under ASC 805, Business Combination. The Company believes that the licensing agreement and other assets acquired from Jacobus are similar and considered them all to be intangible assets with the exception of the inventory acquired. As the screen test was not met, further determination was required to determine that the Company had not acquired inputs and processes that have the ability to create outputs, which would meet the requirements of a business, and therefore, determined that this was an asset acquisition. The Company accounted for the Jacobus license agreement as an asset acquisition under ASC 805-50, which requires the acquiring entity in an asset acquisition to recognize assets acquired and liabilities assumed based on the cost to the acquiring entity on a relative fair value basis, which includes consideration given.

The total purchase price was allocated to the acquired assets based on their relative fair values, as follows (in thousands):

License and acquired intangibles	$	33,569
Acquired research and development inventory expensed from asset acquisition		4,130
Total purchase price	$	37,699

The straight-line method is used to amortize the license and acquired intangibles, as disclosed in Note 9 (License and Acquired Intangibles, Net).

c. ACQUISITION OF U.S. RIGHTS FOR FYCOMPA®. On January 24, 2023, the Company acquired the U.S. Rights for FYCOMPA® (perampanel) CIII a commercial stage epilepsy asset, from Eisai. The aggregate consideration for the acquisition was $164.2 million in cash, including the reimbursement of certain liabilities and the payment of transaction costs.

Eisai was eligible to receive a contingent payment of $25 million if a certain regulatory milestone was met. As meeting the regulatory milestone was not probable, the Company did not recognize any amount related to the milestone payments in the purchase price. Additionally, after the loss of patent exclusivity for FYCOMPA®, the Company may be obligated to pay certain royalties to Eisai on net sales of FYCOMPA®. As the transaction is accounted for as an asset acquisition under U.S. GAAP, the Company will recognize the royalty payments in cost of sales as revenue from product sales is recognized.

Royalties commencing on loss of exclusivity for each calendar year during the royalty term equal to 12% on net sales greater than $10 million and less than $100 million, 17% on net sales of greater than $100 million and less than $125 million and 22% on net sales greater than $125 million prior to the date of generic entry. Royalties equal to 6% on net sales greater than $10 million and less than $100 million, 8.5% on net sales of greater than $100 million and less than $125 million and 11% on net sales greater than $125 million after the date of generic entry.

The following table summarizes the aggregate amount paid for the assets acquired by the Company in connection with the acquisition of FYCOMPA® (in thousands):

Base cash payment	$	160,000
Cash paid for pro-rated prepaid expenses		1,576
Reimbursement on base purchase price[(i)]		(3,238)
Transaction costs[(ii)]		5,870
Total purchase consideration	$	164,208

(i) Recorded in prepaid expenses and other current assets in the accompanying consolidated balance sheet as of the acquisition date and reimbursement was fully applied as of June 30, 2023.

(ii) As of December 31, 2024, the full $5.9 million has been paid in cash.

13. Agreements (continued).

The acquisition of FYCOMPA® has been accounted for as an asset acquisition in accordance with FASB ASC 805-50. The Company accounted for the acquisition of FYCOMPA® as an asset acquisition because substantially all of the fair value of the assets acquired is concentrated in a single asset, the FYCOMPA® product rights. The FYCOMPA® product rights consist of certain patents and trademarks, at-market contracts and regulatory approvals, marketing assets, and other records, and are considered a single asset as they are inextricably linked. ASC 805-10-55-5A includes a screen test, which provides that if substantially all of the fair value of the assets acquired is concentrated in a single identifiable asset or group of similar identifiable assets, the assets acquired are not considered to be a business. ASC 805 requires the acquiring entity in an asset acquisition to recognize assets acquired and liabilities assumed based on the cost to the acquiring entity on a relative fair value basis, which includes consideration given.

The total purchase price was allocated to the acquired assets based on their relative fair values, as follows (in thousands):

Inventory	$	4,100
Prepaid expenses and other current assets (samples)		130
Prepaid commercialization expenses		1,576
Property and equipment, net		433
License and acquired intangibles for FYCOMPA®		158,143
Accrued preclinical and clinical trial expenses		(174)
Total purchase consideration	$	164,208

The straight-line method is used to amortize the license and acquired intangibles, as disclosed in Note 9 (License and Acquired Intangibles, Net).

d. LICENSE AGREEMENT FOR AGAMREE® (VAMOROLONE). In July 2023, the Company completed its acquisition from Santhera of an exclusive license for North America for AGAMREE® (vamorolone), a treatment for patients suffering with DMD which was approved by the FDA on October 26, 2023. On March 13, 2024, the Company announced the U.S. commercial launch of AGAMREE® for the treatment of DMD in patients aged two years or older. The license is for exclusive commercial rights in the U.S., Canada, and Mexico, as well as the right of first negotiation in Japan should Santhera pursue partnership opportunities in those jurisdictions. Additionally, the Company will hold North American rights for any future approved indications of AGAMREE®. The Company made an all-cash initial payment of $75 million at the closing of the acquisition to acquire the license.

Under the license agreement, the Company pays: (i) royalties to the licensor until the later of expiration of product exclusivity or ten years from the first commercial sale of AGAMREE® equal to 5% of net sales (as defined in the license agreement) in North America for any calendar year for sales equal to or less than $100 million (prior to December 31, 2025 only), 7% of net sales for sales in excess of $100 million and up to $200 million, 9% of net sales for sales in excess of $200 million and up to $300 million, 11% of net sales for sales in excess of $300 million; and (ii) royalties to the third party licensor of the rights sublicensed to the Company until the later of expiration of product exclusivity or ten years from the first commercial sale of AGAMREE® equal to 7% of net sales (as defined in the license agreement) in North America for any single calendar year for sales equal to or less than $250 million, 8.5% of net sales for sales in excess of $250 million and up to $500 million, 10% of net sales for sales in excess of $500 million and up to $750 million, 12% of net sales for sales in excess of $750 million and up to $1 billion, 13% of net sales for sales in excess of $1 billion and up to $2 billion and 15% of net sales for sales in excess of $2 billion. Furthermore, the Company may be obligated to pay Santhera sales-based milestones of up to $105 million as well as up to 11% percent royalties for all additional indications and milestones of up to $50 million for the first three additional indications.

Simultaneously, the Company made a strategic equity investment into Santhera by acquiring 1,414,688 of Santhera's post reverse-split ordinary shares (representing approximately 11.26% of Santhera's outstanding ordinary shares immediately following the transaction), which are traded on the SIX Swiss Exchange, at an investment price of CHF 9.477 per share (corresponding to a mutually agreed volume-weighted average price prior to signing), with the funds invested into Santhera to be used by Santhera for Phase IV studies in DMD and further development of additional indications for AGAMREE®.

13. Agreements (continued).

The following table summarizes the aggregate amount paid for the assets acquired by the Company in connection with the acquisition of AGAMREE® and the strategic equity investment (in thousands):

Initial cash payment	$	75,000
Investment in Santhera		13,465
Transaction costs		6,513
Total purchase consideration	$	94,978

The transaction has been accounted for as an asset acquisition in accordance with ASC 805-50. The Company accounted for the transaction as an asset acquisition because substantially all of the fair value of the assets acquired is concentrated in a single asset, the rights to develop, commercialize and manufacture AGAMREE®. The AGAMREE® rights consist of certain licenses and regulatory approvals and are considered a single asset as they are inextricably linked. ASC 805-10-55-5A includes a screen test, which provides that if substantially all of the fair value of the assets acquired is concentrated in a single identifiable asset or group of similar identifiable assets, the assets acquired are not considered to be a business. Additionally, the Company did not acquire a substantive process. ASC 805 requires the acquiring entity in an asset acquisition to recognize assets acquired and liabilities assumed based on the cost to the acquiring entity on a relative fair value basis, which includes consideration given. Goodwill is not recognized in an asset acquisition and any excess consideration transferred over the fair value of the net assets acquired is allocated to the non-financial assets based on relative fair values.

The total purchase price was allocated to the acquired assets based on their relative fair values, as follows (in thousands):

License and acquired intangibles for AGAMREE® (vamorolone) (IPR&D)	$	81,513
Investment in Santhera[(i)]		13,465
Total purchase consideration	$	94,978

(i) The fair value of the investment in Santhera was determined based on the closing market price (CHF 8.25) of Santhera shares and the exchange rate (1.1537) of CHF to USD on the date the shares were transferred, July 19, 2023.

In accordance with FASB ASC 730-10-25, as AGAMREE® (vamorolone) had not achieved regulatory approval when acquired, the portion of the purchase price allocated to the IPR&D asset acquired (which includes all transaction costs related to the transactions with Santhera) was immediately expensed to research and development. Milestone payments made are either expensed as research and development or capitalized as a developed asset based on when regulatory approval is obtained. As the transaction is accounted for as an asset acquisition under U.S. GAAP, the Company will recognize all sales-based milestone and royalty payments in cost of sales as revenue from product sales is recognized.

Following the approval of the NDA for AGAMREE® on October 26, 2023, the Company became obligated to make a milestone payment of $36 million to Santhera. The $36 million payment was made during the fourth quarter of 2023. The Company capitalized the $36 million payment which is being amortized using the straight-line method over the product's estimated useful life of 10.5 years.

The strategic equity investment in Santhera is accounted for as an investment in equity securities, and is recognized as a non-current asset, as the Company does not intend to sell the shares within 12 months. Since Santhera shares have a readily determinable fair value, the investment will be measured quarterly at fair value with changes reported in earnings in other income, net in the accompanying consolidated statements of operations and comprehensive income.

13. Agreements (continued).

e. AGREEMENTS FOR DRUG MANUFACTURING, DEVELOPMENT, PRECLINICAL AND CLINICAL STUDIES. The Company has entered into agreements with contract manufacturers for the manufacture of commercial drug and drug and study placebo for the Company's trials and studies, with contract research organizations (CRO) to conduct and monitor the Company's trials and studies and with various entities for laboratories and other testing related to the Company's trials and studies. The contractual terms of the agreements vary, but most require certain advances as well as payments based on the achievement of milestones. Further, these agreements are cancellable at any time, but obligate the Company to reimburse the providers for any time or costs incurred through the date of termination.

14. Income Taxes.

The Company is subject to income taxes in the U.S. federal jurisdiction and various states jurisdictions.

The income tax expense for the years ended December 31, 2024, 2023 and 2022 consists of (in thousands):

	2024	2023	2022
Current - Federal	$ 52,876	$ 34,975	$ 12,858
Current - State	8,951	5,931	3,877
Deferred - Federal	(8,442)	(16,093)	4,739
Deferred - State	(1,011)	(1,712)	166
	$ 52,374	$ 23,101	$ 21,640

The reconciliation of income tax expense computed at the statutory federal income tax rate of 21% to amounts included in the statements of operations is as follows:

	2024	2023	2022
Statutory rate	21.0%	21.0%	21.0%
State tax	2.8%	3.1%	3.1%
Executive compensation limitation	4.1%	2.6%	3.6%
Tax credit	(0.1)%	—	(1.9)%
Stock compensation windfall	(4.1)%	(4.4)%	(5.6)%
Other	0.5%	2.1%	0.5%
	24.2%	24.4%	20.7%

14. Income Taxes (continued).

Deferred tax assets and liabilities reflect the net tax effects of net operating loss and tax credit carryovers and the temporary differences between the carrying amounts of assets and liabilities for financial reporting and the amounts used for income tax purposes. Significant components of the Company's deferred tax assets/(liabilities) as of December 31, 2024 and 2023 are as follows (in thousands):

	2024	2023
Deferred tax assets:		
Deferred compensation	$ 7,950	$ 6,473
Inventory	561	448
Intangible assets	29,167	24,847
Accrued expenses	4,863	788
Operating lease liability	762	854
Capitalized research	5,755	4,927
Total deferred tax assets	49,058	38,337
Deferred tax liabilities:		
Prepaid expenses	(1,096)	(1,023)
Right-of use asset	(668)	(759)
Other	(1,312)	(11)
Total deferred tax liabilities	(3,076)	(1,793)
Deferred tax assets, net	$ 45,982	$ 36,544

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. As of December 31, 2024, the Company determined that there is sufficient positive evidence to conclude that it is more likely than not that the above deferred taxes of approximately $46 million are realizable.

The Company has received several orphan drug designations by the FDA for products currently under development. The orphan drug designations allow the Company to claim increased federal tax credits for certain research and development activities.

An immaterial amount of interest and penalties were accrued through December 31, 2024 and 2023. The Company's policy is to recognize any related interest or penalties in income tax expense. The Company is not currently under income tax examinations by any tax authorities.

15. Stockholders' Equity.

Preferred Stock

The Company has 5,000,000 shares of authorized preferred stock, $0.001 par value per share. At December 31, 2024 and December 31, 2023, no shares of preferred stock were outstanding.

Common Stock

The Company has 200,000,000 shares of authorized common stock, par value $0.001 per share. At December 31, 2024 and 2023, 120,879,099 and 107,121,549 shares, respectively, of common stock were issued and outstanding. Each holder of common stock is entitled to one vote of each share of common stock held of record on all matters on which stockholders generally are entitled to vote.

Share Repurchases

In March 2021, the Company's Board of Directors approved a share repurchase program that authorizes the repurchase of up to $40 million of the Company's common stock, pursuant to a repurchase plan under Rule 10b-18 of the Securities Act. The share repurchase program commenced on March 22, 2021 and currently expires on March 22, 2025. No shares were repurchased during the years ended December 31, 2024 and 2023. During the year ended December 31, 2022, 1,000,000 shares were repurchased for an aggregate purchase price of approximately $6.9 million ($6.91 average price per share).

15. Stockholders' Equity (continued).

2023 Shelf Registration Statement

On September 8, 2023, the Company filed a shelf registration statement with the SEC to sell up to $500 million of common stock, preferred stock, warrants to purchase common stock, debt securities and units consisting of one or more of such securities (the 2023 Shelf Registration Statement). The 2023 Shelf Registration Statement (file no. 333-274427) became effective upon filing. On January 9, 2024, the Company completed a public offering of 10 million shares of its common stock, raising net proceeds of approximately $140.7 million under the Company's 2023 Shelf Registration Statement.

16. Stock Compensation.

For the years ended December 31, 2024, 2023 and 2022, the Company recorded stock-based compensation expense as follows (in thousands):

	2024	2023	2022
Research and development	$ 1,779	$ 1,481	$ 1,729
Selling, general and administrative	20,472	12,769	6,178
Total stock-based compensation	$ 22,251	$ 14,250	$ 7,907

The Company may issue stock options, restricted stock, stock appreciation rights and restricted stock units (collectively, the Awards) to employees, directors, and consultants of the Company under the 2014 and 2018 Stock Incentive Plans (the 2014 Plan and the 2018 Plan or collectively, the Plans). At December 31, 2024, no shares remain available for future issuance under the 2014 Plan. Under the 2018 Plan, 21,000,000 shares are reserved for issuance and as of December 31, 2024, 2,254,102 shares remain available for future issuance.

Stock Options

The Company has granted stock options to employees, officers, directors, and consultants generally at exercise prices equal to the market price of the common stock at grant date. Option awards generally vest over a period of 1 to 3 years of continuous service and have contractual terms of 7 years. Certain awards provide for accelerated vesting if there is a change in control. The Company issues new shares as shares are required to be delivered upon exercise of outstanding stock options.

During the years ended December 31, 2024, 2023 and 2022, options to purchase 3,429,184, 1,651,345 and 3,172,342 shares, respectively, of the Company's common stock were exercised with gross proceeds to the Company of approximately $13.5 million, $2.8 million and $9.6 million, respectively. During the years ended December 31, 2024, 2023 and 2022, no options to purchase shares of the Company's common stock were exercised on a "cashless" basis.

During the years ended December 31, 2024, 2023 and 2022 the Company recorded non-cash stock-based compensation expense related to stock options totaling approximately $16.8 million, $11.1 million and $6.3 million, respectively.

During the years ended December 31, 2024, 2023 and 2022, the Company granted seven-year options to purchase an aggregate of 2,476,946, 3,598,535 and 1,386,500 shares, respectively, of the Company's common stock to certain of the Company's officers, employees, directors, and consultants.

16. Stock Compensation (continued).

Stock option activity under the Company's Plans for the year ended December 31, 2024 is summarized as follows:

	Number of Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding at beginning of year	14,177,488	$ 7.73		
Granted	2,476,946	18.95		
Exercised or released	(3,429,184)	3.94		
Forfeited or cancelled	(137,337)	15.29		
Expired	(11,124)	16.47		
Outstanding at end of year	13,076,789	$ 10.76	4.26	$ 132,570
Exercisable at end of year	7,353,893	$ 6.53	2.93	$ 105,449

Other information pertaining to stock option activity during the years ended December 31, 2024, 2023 and 2022 was as follows:

	2024	2023	2022
Weighted–average fair value of granted stock options	$ 9.53	$ 8.66	$ 8.52
Total fair value of vested stock options (in thousands)	$ 12,860	$ 8,278	$ 6,096
Total intrinsic value of exercised stock options (in thousands)	$ 53,230	$ 22,265	$ 31,881

As of December 31, 2024, there was approximately $41.7 million of unrecognized compensation expense related to non-vested stock option awards granted under the Plans. That cost is expected to be recognized over a weighted average period of approximately 2.72 years.

The Company utilizes the Black-Scholes option-pricing model to determine the fair value of stock options on the date of grant. This model derives the fair value of stock options based on certain assumptions related to the expected stock price volatility, expected option life, risk-free interest rate and dividend yield. Expected volatility is based on reviews of historical volatility of the Company's common stock. The Company estimates the expected option life for options granted to employees and directors based upon the simplified method. Under this method, the expected life is presumed to be the mid-point between the vesting date and the end of the contractual term. The Company will continue to use the simplified method until it has sufficient historical exercise data to estimate the expected life of the options. The risk-free interest rate assumption is based upon the U.S. Treasury yield curve appropriate for the estimated life of the stock option awards. The expected dividend rate is zero. Forfeitures are recognized as a reduction of stock-based compensation expense as they occur.

Assumptions used during the years ended December 31, 2024, 2023 and 2022 were as follows:

	2024	2023	2022
Risk free interest rate	3.70% to 4.70%	3.55% to 4.92%	1.27% to 4.07%
Expected term	4.5 to 5.0 years	4.5 to 5.2 years	4.5 years
Expected volatility	54.1% to 61.5%	68.0% to 71.0%	68.4% to 69.5%
Expected dividend yield	—%	—%	—%
Expected forfeiture rate	—%	—%	—%

16. Stock Compensation (continued).

Restricted Stock Units

Under the 2018 Plan, participants may be granted restricted stock units, each of which represents a conditional right to receive shares of common stock in the future. The restricted stock units granted under this plan generally vest ratably over a three-year period. Upon vesting, the restricted stock units will convert into an equivalent number of shares of common stock. The amount of expense relating to the restricted stock units is based on the closing market price of the Company's common stock on the date of grant and is amortized on a straight-line basis over the requisite service period. Restricted stock unit activity for the year ended December 31, 2024 was as follows:

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Nonvested balance at beginning of year	854,459	$ 12.48
Granted	228,029	20.45
Vested	(364,861)	11.19
Forfeited	(8,667)	18.59
Nonvested balance at end of year	708,960	$ 15.63

During the years ended December 31, 2024, 2023 and 2022, the Company recorded non-cash stock-based compensation expense related to restricted stock units totaling $5.5 million, $3.2 million and $1.6 million, respectively.

17. Benefit Plan.

The Company maintains an employee savings plan pursuant to Section 401(k) of the Internal Revenue Code covering all eligible employees. Subject to certain dollar limits, eligible employees may contribute up to 15% of their pre-tax annual compensation to the plan. The Company has elected to make discretionary matching contributions of employee contributions up to 4% of an employee's gross salary. For the years ended December 31, 2024, 2023 and 2022, the Company's matching contributions were approximately $1.2 million, $0.7 million and $0.5 million, respectively.

18. Subsequent Events.

On January 8, 2025, the Company announced that it and its licensor, SERB have entered into a Settlement Agreement with Teva. This agreement resolves the patent litigation brought by the Company and SERB in response to Teva's ANDA seeking approval to market a generic version of FIRDAPSE® (amifampridine) 10 mg tablets prior to the expiration of the applicable patents.

Pursuant to the terms of the settlement agreement, Teva has agreed not to market its generic version of FIRDAPSE® in the U.S. any earlier than February 25, 2035, if approved by the U.S. Food and Drug Administration, unless certain limited circumstances customarily included in these types of settlements occur. In accordance with the Agreement, the Company/SERB and Teva have terminated all ongoing patent litigation between them that is currently pending in the U.S. District Court for the District of New Jersey. The pending FIRDAPSE® patent litigation against the remaining defendants, Hetero (for all of FIRDAPSE®'s Orange Book-listed patents) and Lupin (only for Catalyst's FIRDAPSE® patent expiring in 2037) is ongoing, and there can be no assurance whether such ongoing patent litigation will allow a generic version of FIRDAPSE® to be marketed in the U.S. prior to February 25, 2035.

On January 17, 2025, Charles B. O'Keeffe, an independent member of the Board of Directors (Board) of the Company, advised the Company of his decision to not stand for reelection at the 2025 annual meeting of the Company's stockholders. Mr. O'Keeffe will continue to serve as a member of the Board until the 2025 annual meeting, when his current term will expire. Mr. O'Keeffe has been a member of the Board since December 2004 and currently serves as the Board's Lead Independent Director. Mr. O'Keeffe's intention to retire from the Board was not the result of any disagreement with the Company on any matter relating to the Company's operations, policies, or practices.

On January 21, 2025, the Company reported that its sub-licensee in Japan, DyDo, has launched FIRDAPSE® Tablets 10 mg in Japan for the indication of improving muscle weakness in patients living with LEMS.

On February 13, 2025, Gregg Russo was promoted from VP, Head of Human Resources to Chief Human Resources Officer.

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Corporate Directory

BOARD OF DIRECTORS

Patrick J. McEnany
Non-executive Chairman of the Board and Co-Founder

Richard J. Daly
President and Chief Executive Officer
Catalyst Pharmaceuticals, Inc.

Donald A. Denkhaus
Chair, Audit Committee

Molly Harper
Managing Partner
Peacock Hall LLC

Charles B. O'Keeffe
Lead Independent Director
Professor, Pharmacology, Epidemiology and Community Health
Virginia Commonwealth University

Tamar Thompson
Chair, Corporate Governance and Nominating Committee
Vice President/Head of Global Corporate Affairs
Alexion Pharmaceuticals/AstraZeneca Rare Disease

David S. Tierney, MD
Chair, Compensation Committee
Chief Executive Officer
Aramis Biosciences

EXECUTIVE OFFICERS

Richard J. Daly
President and Chief Executive Officer

Steven R. Miller, PhD
Executive Vice President, Chief Operating Officer and Chief Scientific Officer

Michael W. Kalb, CPA
Executive Vice President, Treasurer and Chief Financial Officer

Gary Ingenito, M.D., Ph.D.
Chief Medical and Regulatory Officer

Jeffrey Del Carmen
Executive Vice President and Chief Commercial Officer

Brian Elsbernd, J.D.
Chief Compliance Officer and Chief Legal Officer

Preethi Sundaram, Ph.D.
Chief Strategy Officer

Gregg Russo
Chief Human Resources Officer

INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

Grant Thornton LLP
Miami, Florida

CORPORATE COUNSEL

Akerman LLP
Fort Lauderdale, Florida

ANNUAL MEETING

The annual meeting of stockholders will be held virtually on Tuesday, May 20, 2025, at 9:00 a.m, eastern time.

INVESTOR INFORMATION

Recent press releases and other Catalyst Pharmaceuticals information are available without charge on Catalyst's website at www.catalystpharma.com or by written request to:

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STOCK LISTING

Catalyst's common stock trades on the Nasdaq Capital Market under the symbol CPRX.

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