Catalyst pharmaceuticals

2023 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, 2023

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

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

Securities Registered Pursuant to Section 12(b) of the 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 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, 2023, 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 $1,341,304,702.

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date: 117,863,258 shares of common stock, $0.001 par value per share, were outstanding as of February 26, 2024.

Part III incorporates certain information by reference from the registrant's definitive proxy statement for the 2024 annual meeting of stockholders. The proxy statement with respect to the 2024 annual meeting of stockholders will be filed no later than 120 days after the close of the registrant's fiscal year ended December 31, 2023.

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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 the successful launch and commercialization of 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® and FYCOMPA® while maintaining full compliance with applicable federal and state laws, rules and regulations;
- 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 our supplemental New Drug Application (sNDA) seeking to increase the maximum daily dosage of FIRDAPSE® from 80 mg to 100 mg will be approved by the U.S. Food and Drug Administration (FDA);
- Whether the daily dose of FIRDAPSE® taken by patients changes over time and affects our results of operations;
- Whether we will be able to locate LEMS patients who are undiagnosed or are misdiagnosed with other diseases;
- Whether patients will discontinue from the use of FIRDAPSE® and FYCOMPA® at rates that are higher than historically experienced or are higher than we project;
- Whether new FIRDAPSE® patients and new FYCOMPA® patients can be successfully titrated to stable therapy;
- Whether we can continue to market FIRDAPSE® and FYCOMPA® on a profitable and cash flow positive basis;
- Whether we will successfully launch AGAMREE® in the first quarter of 2024 as we currently plan;
- Whether we will be able to successfully commercialize AGAMREE® in the territory;
- Whether we will be able to demonstrate, to the satisfaction of the FDA and third-party payors, whether AGAMREE® offers advantages compared to corticosteroids or competitor's products;
- Whether the acquisition of AGAMREE® will prove to be accretive to EBITDA and EPS in 2024 and beyond;
- Whether any revenue or earnings guidance that we provide to the public market will turn out to be accurate;
- Whether payors will 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 supply sufficient product to meet our customers' needs in future periods;
- 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 any 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 our patents will be sufficient to prevent generic competition for FIRDAPSE® and AGAMREE® after our orphan drug exclusivity for each product expires;
- 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® and FYCOMPA®;
- 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, or changes in the healthcare industry generally;
- Whether we and Santhera Pharmaceuticals 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 our clinical trials and studies, pre-clinical studies, proof-of-concept studies, and our other drug development activities, and whether our trials and studies 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, our collaboration partner in Canada;
- The impact on sales of FIRDAPSE® in the United States if an amifampridine product is purchased in Canada for use in the United States;
- Whether DyDo will be able to obtain approval to commercialize FIRDAPSE® in Japan; and
- Whether our plans to expand the reach of FIRDAPSE® and AGAMREE® into other global regions will be successful.

Our current plans and objectives are based on assumptions relating to the continued commercialization of FIRDAPSE® and FYCOMPA®, the commercialization of 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 utilize concerted diligence efforts in search of therapies that will improve the lives of those who suffer from rare or difficult to treat diseases. 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.

Our flagship U.S. commercial product is FIRDAPSE® (amifampridine) Tablets 10 mg approved for the treatment of Lambert-Eaton myasthenic syndrome, or LEMS, for adults and for children ages six and up. Further, on January 24, 2023, we closed our acquisition of FYCOMPA® and are now also marketing that product in the United States. FYCOMPA® (perampanel) CIII is a prescription medication used alone or 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. Finally, on July 18, 2023, we closed our acquisition of an exclusive license for North America for vamorolone, a novel corticosteroid treatment for patients suffering from Duchenne Muscular Dystrophy (DMD). On October 26, 2023, the FDA approved AGAMREE® (vamorolone) oral suspension 40 mg/ml for the treatment of DMD. We are currently planning the commercial launch of AGAMREE® in the United States during the first quarter of 2024.

FIRDAPSE®

On November 28, 2018, we received approval from the FDA for our new drug application, or 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 United States. Further, on September 29, 2022, the FDA approved our supplemental NDA (sNDA) to expand the indicated age range for FIRDAPSE® Tablets 10 mg to include pediatric patients, six years of age and older for the treatment of LEMS.

We sell FIRDAPSE® through a field force experienced in neurologic, central nervous system or rare disease products consisting at this time of approximately 35 field personnel, including sales (Regional Account Managers), thought leader liaisons, patient assistance and insurance navigation support (Patient Access Liaisons), and payor reimbursement (National Account Managers). 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, for the last few years we have contracted with an experienced inside sales agency that works to generate leads through telemarketing to targeted physicians. This inside sales agency allowed our sales efforts to not only reach the neuromuscular specialists who regularly treat LEMS patients, but also the roughly 9,000 neurology and neuromuscular healthcare providers that may be treating a LEMS patient who can benefit from FIRDAPSE®. However, effective January 1, 2024 we have terminated that arrangement. We also 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 with 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.

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 and the National Organization for Rare Disorders) 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. 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 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 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. A co-pay assistance program designed to keep out-of-pocket costs to not more than $10 per month (currently less than $2 per month) is available for all LEMS patients with commercial coverage who are prescribed FIRDAPSE®. Our FIRDAPSE® co-pay assistance program is not available to patients enrolled in state or federal healthcare programs, including Medicare, Medicaid, VA, DoD, or TRICARE. Separately, 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. Subject to compliance with regulatory requirements, our goal is that no LEMS patient is ever denied access to their medication for financial reasons.

In January 2023, we received Paragraph IV Certification Notice Letters from three generic drug manufacturers 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 United States. The notice letters each alleged that the six patents listed in the FDA Approved Drug Products with Therapeutic Equivalence Evaluations (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, as amended, 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 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. Further, in October 2023, we received a Paragraph IV Certification Notice Letter from a fourth generic drug manufacturer, and we filed a similar lawsuit against this manufacturer in November 2023 in the U.S. District Court for the District of New Jersey.

We intend to vigorously protect and defend our intellectual property for FIRDAPSE® and, although there can be no assurance, we believe that our patent estate will protect FIRDAPSE® from generic competition.

On August 4, 2023, we submitted an sNDA to increase the indicated maximum daily dosage of FIRDAPSE® tablets from 80 mg to 100 mg for the treatment of LEMS. On October 13, 2023, we announced that the FDA had accepted for review our sNDA and assigned a Prescription Drug User Fee Act (PDUFA) action date of June 4, 2024. There can be no assurance that the FDA will approve our sNDA.

We are advised by our sub-licensee for FIRDAPSE® in Japan, DyDo Pharma, Inc. (DyDo), that on December 18, 2023, based on a preliminary favorable interim data analysis after six months into the safety phase of its registration study to evaluate the efficacy and safety of FIRDAPSE® for the treatment of LEMS, they filed a Japan NDA with the Pharmaceuticals and Medical Devices Agency (PMDA) in Japan seeking approval to commercialize the product in Japan. The review period is expected to be approximately nine months from the submission date, and there can be no assurance that the NDA filing made by DyDo will be approved.

Further, upon acceptance of the Japan NDA by the PMDA, which occurred on December 18, 2023, our license for FIRDAPSE® automatically expanded to include other key markets in Asia and Latin America, and we are currently initiating plans to seek opportunities to expand FIRDAPSE®'s global footprint through strategic partnerships (with the current focus on the Asia Pacific and Latin American regions).

FYCOMPA®

On December 17, 2022, we entered into an agreement with 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 an additional cash payment of $25 million if a requested patent extension for FYCOMPA® until June 8, 2026 was approved by the U.S. Patent and Trademark Office (USPTO), which did not occur. Finally, we 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 with Eisai; a Transition Services Agreement (TSA) and a Supply Agreement. Under the TSA, a U.S. subsidiary of Eisai provided us with certain transitional services, and 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). The transition services provided under the TSA ended on December 31, 2023.

Initially, following the closing of the acquisition, we began to market FYCOMPA® in the U.S. through Eisai under the TSA as we built our FYCOMPA® marketing and sales team, and in May 2023, we took over the marketing program for FYCOMPA®. In that regard, we have hired approximately 35 sales and marketing personnel to support FYCOMPA®, most of whom previously worked in Eisai's U.S. sales division marketing FYCOMPA®. We have also hired seven medical science liaisons to help us educate the medical community who treat epilepsy about scientific literature regarding epilepsy and 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 $10 for their FYCOMPA® co-pay (with a maximum savings of $1,300 per year). The FYCOMPA® Instant Savings Card 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.

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 May 23, 2025, including patent term extension. Although the Company had requested that the USPTO reconsider this expiration date in favor of a June 8, 2026 expiration date, the request for reconsideration of the agency's patent term extension calculation was denied and the Company has exhausted its reasonable avenues for an extension of that patent term. The Company will update the Orange Book to reflect the May 23, 2025 expiration date at the appropriate time. 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. The '497 patent has been the subject of previous Paragraph IV certifications from three NDA filers.

On February 20, 2023, we 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 us later in February with a similar certification for the tablet formulation for FYCOMPA®, the fourth such certification for this formulation. Both of these letters were Paragraph IV Certifications of non-infringement, non-validity, and unenforceability of the '497 patent for FYCOMPA® but each application, like the previous Paragraph IV notices from ANDA filers, does not challenge the '571 patent. Accordingly, the FDA may not approve any ANDA prior to expiration of the '571 patent, including patent term extension. Similar to the actions with the FIRDAPSE® Paragraph IV Certifications described above, 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 for both FYCOMPA® formulations, thus triggering the 30 month stay for each application.

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®. Additionally, following approval of the NDA for the drug, on October 26, 2023, we became obligated to make a milestone payment of $36 million to Santhera, $26 million of which Santhera has advised us was used to make milestone payments that they owe to third parties. The $36 million payment was made 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. 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 Europe and Japan should Santhera pursue partnership opportunities in those territories. Additionally, we will hold the North American rights to any future approved indications for AGAMREE®.

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 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 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 of AGAMREE® in DMD and future development of additional indications for AGAMREE®. At February 26, 2024, the closing price of Santhera's common shares on the SIX Swiss Exchange was CHF 11.36 per share.

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 backbone 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 with dissociative properties in maintaining efficacy 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 the pivotal VISION-DMD study, vamorolone met the

primary endpoint Time to Stand (TTSTAND) velocity versus placebo (p=0.002) at 24 weeks of treatment and showed a good safety and tolerability profile. The most commonly reported adverse events versus placebo from the VISION-DMD study were cushingoid features, vomiting, and vitamin D deficiency. Adverse events were generally of mild to moderate severity.

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. 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. As part of the previously described transaction, Santhera has transferred the approved New Drug Application to us. 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. On January 15, 2024, Santhera announced that AGAMREE® was commercially launched in Germany.

We currently expect to launch AGAMREE® in the U.S. during the first quarter of 2024. We are incurring substantial commercialization expenses, including sales, marketing, analytical infrastructure, patient services, patient advocacy, and other commercialization related expenses, in preparation for the launch of the product in the U.S. We incurred a portion of such commercialization expenses during the fourth quarter of 2023 and we are incurring additional expenses during the first quarter of 2024. We anticipate minimal sales and marketing personnel expansion to market AGAMREE®, with approximately 10 additional commercial team members required, due to the synergy of this product within our existing neuromuscular franchise. We will support 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.

We have established a joint steering committee with Santhera that will oversee the lifecycle management and development of AGAMREE® for additional indications beyond DMD. Under our AGAMREE® License Agreement with Santhera, we have agreed to purchase commercial supply of AGAMREE® from Santhera at agreed upon rates.

To support the approval of AGAMREE®, a randomized, double-blind, placebo and prednisone-controlled multinational trial of vamorolone was carried out in 121 patients with DMD. The study included patients ages four to less than seven years of age at time of enrollment in the study who were corticosteroid naïve and ambulatory. The trial met the primary (time to stand velocity after 24 weeks for vamorolone, 6 mg/kg per day vs placebo) and several 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 several 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.

AGAMREE® has New Chemical Entity exclusivity that expires in October 2028. AGAMREE® also enjoys Orphan Drug Exclusivity expiring in October 2030. AGAMREE® is further protected by six 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 could file 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 the ANDA until April 26, 2031.

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 products to treat rare (orphan) central nervous system (CNS) and adjacent rare (orphan) diseases. 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, no additional definitive agreements have been entered into to date, and there can be no assurance that these initiatives will be successful.

Capital Resources

At December 31, 2023, we had cash and cash equivalents of approximately $137.6 million. Further, on January 9, 2024, we completed a public offering of 10 million shares of our common stock, raising net proceeds of approximately $140.1 million. The proceeds from this offering will be used to potentially acquire new products and for general corporate purposes.

Based on our current financial condition 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 FYCOMPA®, that our commercialization of AGAMREE® will be successful, or that we will continue to be profitable and cash flow positive. Further, there can be no assurance that if we need additional funding in the future, whether such funding will be available to us on acceptable terms. See Item 7. "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.

Recent Management Changes

On December 31, 2023, Patrick J. McEnany, our then-President and Chief Executive Officer, and Alicia Grande, our then-Vice President, Treasurer and Chief Financial Officer, both retired from their respective roles with the Company. On January 1, 2024, Richard J. Daly became our President and Chief Executive Officer, and Michael W. Kalb became our Executive Vice President and Chief Financial Officer. Mr. McEnany remains as the Chairman of our Board of Directors and Mr. Daly remains a member of our Board of Directors.

Our Strategy

Our goal is to acquire, develop and commercialize novel prescription drugs targeting rare (orphan) neuromuscular and neurological diseases and adjacent rare (orphan) disorders. 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 United States and through a sublicense to KYE Pharmaceuticals in Canada. We are working to expand awareness of the disease, including to physicians treating LEMS patients who have small-cell lung cancer, and helping health care providers and their patients understand the benefits of FIRDAPSE®. A cornerstone of our U.S. 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 FYCOMPA®. In the first quarter of 2023, we acquired the U.S. rights to the epilepsy drug FYCOMPA®. We are currently successfully distributing and marketing FYCOMPA®. We believe that having a second marketed product adds substantially to our business franchise.
- Successfully launch AGAMREE® in the United States. In the third quarter of 2023, we acquired an exclusive license from Santhera Pharmaceuticals. Under the AGAMREE® License Agreement, we obtained an exclusive North America license for AGAMREE® for the treatment of DMD and as a treatment for other diseases. We also made a strategic investment into Santhera, the funds of which will be used to further the development of the product. We believe that this transaction not only added a third product to our portfolio, but also leverages the sales infrastructure that we have developed to market FIRDAPSE®. We currently plan to launch this product before the end of the first quarter of 2024.
- Obtain approval for FIRDAPSE® in Japan and seek approval in other potential territories in Asia and Latin America. We are currently supporting our sub-licensee, DyDo, as they take the steps required to obtain approval to market FIRDAPSE® in Japan for the treatment of Japanese patients with LEMS. DyDo announced on December 18, 2023 that it filed and the Japanese Ministry of Health, Labor and Welfare had accepted its application for approval to market FIRDAPSE® in Japan. As a consequence of the acceptance of this filing, we have now gained territorial rights to commercialize FIRDAPSE® in several countries in Asia and Latin America and we have begun our activities to expand FIRDAPSE® into some of these countries.
- Seek to acquire additional products. We intend to continue our efforts to broaden and diversify our product portfolio through acquisitions of clinically differentiated and adequately de-risked opportunities, with a keen focus on products to treat rare (orphan) CNS, and adjacent rare (orphan) therapeutic categories. 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. To accomplish these priorities, we are continuing to employ a disciplined, comprehensive and exhaustive approach to identifying and evaluating assets. 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, mid and long-term.

FIRDAPSE® Product Overview

FIRDAPSE® is the registered trade name in the United States for amifampridine phosphate tablets and it is licensed to us by SERB, S.A. Amifampridine is the WHO (World Health Organization) registered INN (International Nonproprietary Name) and United States 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 United States (FIRDAPSE®), by the INN/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 100 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)*

Lambert-Eaton Myasthenic Syndrome (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 the 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 United States, approximately half of which are presently diagnosed and identified and the other approximately half 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 United States can be determined with better certainty (as is typical of rare diseases), and the actual number of patients in the United States with LEMS may be higher or lower than our estimate.

Some of the factors that affect the 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 FIRDAPSE® License Agreement) between us and BioMarin Pharmaceutical Inc. (BioMarin). Under the FIRDAPSE® 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 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 for seven years from the first commercial sale of FIRDAPSE® equal to 7% of net sales (as defined in the FIRDAPSE® 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 our FIRDAPSE® License Agreement. Under the amendment, we expanded our commercial territory for FIRDAPSE®, which originally was comprised of North America, to include Japan. Additionally, our territory automatically expanded to include most of Asia, as well as Latin America, upon the acceptance by the Japanese Ministry of Health, Labor and Welfare in Japan of an application to market our product in Japan, which occurred on December 18, 2023. 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 FIRDAPSE® License Agreement for North America.

In January 2020, we were advised that BioMarin had transferred substantially all of its rights under the FIRDAPSE® License Agreement to SERB SA.

We believe that we remain in compliance with our obligations under the FIRDAPSE® License Agreement.

License Agreement with Jacobus Pharmaceutical Company, Inc. (Jacobus)

In May 2019, the FDA approved an NDA for RUZURGI®, Jacobus' 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. Our complaint, which was filed in the federal district court for the Southern District of Florida, alleged that the FDA's approval of RUZURGI® violated multiple provisions of FDA regulations regarding labeling, resulting in misbranding in violation of the FDCA; violated our statutory rights to Orphan Drug Exclusivity and New Chemical Entity Exclusivity under the FDCA; and was in multiple other respects arbitrary, capricious, and contrary to law, in violation of the Administrative Procedure Act. Among other remedies, the suit sought an order vacating the FDA's approval of RUZURGI®.

On July 30, 2020, the Magistrate Judge considering this lawsuit filed a Report and Recommendation in which she recommended to the District Judge handling the case that the Court grant the FDA's and Jacobus' motions for summary judgment and deny our motion for summary judgment. On September 29, 2020, the District Judge adopted the Report and Recommendation of the Magistrate Judge, granted the FDA's and Jacobus' motions for summary judgment, and dismissed our case. We appealed the District Court's decision to the U.S. Court of Appeals for the 11th Circuit. The case was fully briefed in early 2021, and oral argument was held in March 2021.

On September 30, 2021, a three-judge panel of 11th Circuit judges issued a unanimous decision overturning the District Court's decision. The appellate court adopted our argument that the FDA's approval of RUZURGI® violated our rights to Orphan Drug Exclusivity and remanded the case to the District Court with orders to enter summary judgment in our favor. In November 2021, Jacobus filed a motion seeking rehearing of the case from the full 11th Circuit, which motion was denied in January 2022. Further, in January 2022, Jacobus filed motions with both the 11th Circuit and the U.S. Supreme Court seeking a stay of the 11th Circuit's ruling indicating that it would seek a review of the 11th Circuit's decision from the U.S. Supreme Court. Both stay motions were denied, and on January 28, 2022, the 11th Circuit issued a mandate directing the District Court to enter summary judgment in our favor. The District Court entered that order on January 31, 2022. On February 1, 2022, the FDA informed Jacobus that, consistent with the Court of Appeals for the Eleventh Circuit's September 30, 2021, decision in favor of Catalyst, the final approval of the RUZURGI® NDA was switched to a tentative approval until the 7-year orphan-drug exclusivity (ODE) for FIRDAPSE® has expired.

On July 11, 2022, we settled certain of our disputes with Jacobus. In connection with the settlement, we licensed the rights to develop and commercialize RUZURGI® in the United States and Mexico. Simultaneously, we purchased, among other intellectual property rights, Jacobus' U.S. patents related to RUZURGI®, its NDAs in the United States 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 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, 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 and $10 million was paid on the first anniversary of the closing of the settlement. We will be obligated to pay the remaining balance on the second anniversary of closing;
- An annual royalty on our net sales (as defined in the License and Asset Purchase Agreement between Catalyst and Jacobus) of amifampridine products in the United States 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 United States, 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; and
- If Catalyst were to receive a priority review voucher for FIRDAPSE® or RUZURGI® in the future, 50% of the consideration paid by a third-party to acquire that voucher will be paid to Jacobus.

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 were 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.

Required Post-Approval Studies

As part of the approval of our NDA for FIRDAPSE® for LEMS, the FDA required us to conduct 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. This study has been completed and submitted to the FDA. We have also established a pregnancy surveillance program to collect and analyze information for a minimum of ten (10) years on pregnancy complications and birth outcomes related to FIRDAPSE®. Further, the FDA required us to perform a second carcinogenicity study of amifampridine phosphate in mice, which has been completed and the FDA has advised us is acceptable. Finally, in connection with the recent approval of our sNDA for FIRDAPSE® for the treatment of children ages six through seventeen with LEMS, we are now required to complete 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 (DN) through investigator-sponsored compassionate use programs. Further, when we acquired the U.S. rights to RUZURGI®, 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. We will continue to supply this RUZURGI® research drug to these IND holders for as long as this research amifampridine drug product is available. We currently estimate that current stocks of this research drug will last for approximately 12 to 18 months. The active ingredient used to make this product is no longer available. As a result, we do not anticipate being able to continue to supply this drug once we have exhausted the existing research drug supplies.

Sales, Marketing and Distribution

Launch of FIRDAPSE® in January 2019

In January 2019, we launched FIRDAPSE® in the United States through a field force of approximately 20 personnel who are experienced in neurologic, central nervous system or rare diseases in sales, patient support and payer reimbursement. The sales representatives (Regional Account Managers) who were part of the field force targeted approximately 1,250 physicians who are either neuromuscular specialists or general neurologists with a known adult LEMS patient or specific training in neuromuscular diseases. We also utilized field force Patient Access Liaisons who work with the patients and provider offices to help navigate the insurance

landscape, as well as National Account Managers who work directly with the payors to ensure comprehensive coverage for FIRDAPSE® across the commercial and governmental plans in the United States. We also at that time had a field-based force of seven medical science liaisons who help educate the medical communities about LEMS and about our company's ongoing clinical trial activities. Further, we work closely with several rare disease advocacy organizations (including the National Organization for Rare Disorders (NORD) and the Myasthenia Gravis Foundation of America) to help increase awareness and the level of support for patients living with LEMS and other neuromuscular diseases, and to provide education for the physicians who treat these rare diseases and the patients they treat.

In early 2020, we expanded our field sales group by almost one hundred percent and established a partnership with an experienced inside sales agency generating leads through telemarketing to targeted physicians. Through this expansion of our sales team, we were working to expand our sales efforts beyond the neuromuscular specialists who regularly treat LEMS patients to reach roughly 9,000 neurology and neuromuscular healthcare providers that might be treating an adult LEMS patient who can benefit from FIRDAPSE®. However, we terminated the inside sales agency effective January 1, 2024. We also use non-personal promotion to reach the 20,000 neurologists who are potential LEMS treaters and the 16,000 oncologists who might treat a LEMS patient with small cell lung cancer. Finally, we make available a no-cost LEMS voltage gated calcium channel (VGCC) antibody testing program for physicians who suspect their patient may have LEMS and wish to reach a definitive diagnosis.

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 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 payor despite having health insurance.

We are continuing efforts on the challenging process to identify patients and their physicians who have been diagnosed with 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 who are prescribed FIRDAPSE® for LEMS, a co-pay assistance program is available, which is designed to keep out-of-pocket costs to $10 or less per month (currently less than $2 per month). Our FIRDAPSE® co-pay assistance program is not available to patients enrolling in state or federal healthcare programs, including Medicare, Medicaid, VA, DoD, or Tricare. Secondly, we are 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 LEMS patients.

sNDA to increase the maximum daily dose for FIRDAPSE®

On August 4, 2023, we submitted an sNDA to increase the indicated maximum daily dosage of FIRDAPSE® tablets from 80 mg to 100 mg for the treatment of LEMS. On October 13, 2023, we announced that the FDA had accepted for review our sNDA and assigned a Prescription Drug User Fee Act (PDUFA) action date of June 4, 2024. There can be no assurance that the FDA will approve our sNDA.

Canadian Market

Our New Drug Submission filing for FIRDAPSE® for the symptomatic treatment of LEMS was approved when Health Canada issued a Notice of Compliance, or NOC, on July 31, 2020. In August 2020, we entered into a license agreement with KYE Pharmaceuticals, or KYE, pursuant to which we licensed to KYE the Canadian rights for FIRDAPSE® for the treatment of LEMS.

On August 10, 2020, Health Canada issued a NOC to Medunik (Jacobus' licensee in Canada for RUZURGI®) for the treatment of LEMS. Shortly thereafter, we initiated a legal proceeding in Canada seeking judicial review of Health Canada's decision to issue the NOC for RUZURGI® as incorrect and unreasonable under Canadian law. Data protection, per Health Canada regulations, is supposed to prevent Health Canada from issuing an NOC to a drug that directly or indirectly references an innovative drug's data, for eight years from the date of the innovative drug's approval. The RUZURGI® Product Monograph clearly references pivotal nonclinical carcinogenicity and reproductive toxicity data for amifampridine phosphate developed by us. As such, we believe that our data was relied upon to establish the nonclinical safety profile of RUZURGI® needed to meet the standards of the Canadian Food and Drugs Act.

On June 3, 2021, we announced a positive decision in this proceeding that quashed the NOC previously issued for RUZURGI® and remanded the matter to the Minister of Health to redetermine its decision to grant marketing authorization to RUZURGI® despite FIRDAPSE®'s data protection rights. However, on June 28, 2021, we announced that Health Canada had re-issued an NOC for RUZURGI®, once again allowing the product to be marketed in Canada for patients with LEMS. As a result, in July 2021 we, along with our partner in Canada, KYE, filed a second suit against Health Canada to overturn this decision.

On March 11, 2022, we announced that we had received a favorable decision from the Canadian court setting aside, for the second time, the decision of Health Canada approving RUZURGI® for the treatment of LEMS patients. In its ruling, the court determined that the Minister of Health's approach to evaluating whether FIRDAPSE®'s data deserved protection based on FIRDAPSE®'s status as an innovative drug, which protects by regulation the use of such data as part of a submission seeking an NOC for eight years from approval of the innovative drug, was legally flawed and not supported by the evidence. The Minister of Health appealed that decision, and, in January 2023, the Canadian Appellate Court overturned the trial court's decision. Thereafter, the Minister of Health reissued an NOC for RUZURGI® in Canada and, as a result, RUZURGI® is once again approved for sale in Canada.

While there can be no assurance, we do not expect that the reissuance of the NOC for RUZURGI® in Canada will have a material adverse effect on our results of operations.

Japanese Market

In May 2019, we entered into an amendment to our license agreement for FIRDAPSE®. Under the amendment, we expanded our commercial territory for FIRDAPSE®, which originally was comprised of North America, to include Japan. We have also reached an agreement with Japanese regulatory authorities as to the scope of the clinical trial that will be required to be completed before an application can be submitted to Japanese regulatory authorities to commercialize FIRDAPSE® for the treatment of LEMS in Japan. Finally, we have been 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. Under the terms of the agreement, DyDo has the exclusive rights to commercialize the product in Japan. DyDo is responsible for funding all clinical, regulatory, marketing and commercialization activities in Japan. We are responsible for clinical and commercial supply, as well as providing support to DyDo in its efforts to obtain regulatory approval for the product from the Japanese regulatory authorities. Subject to the satisfaction of terms and conditions as set forth in the agreement, we have earned an upfront payment and are eligible to receive further development and sales milestones for FIRDAPSE®, as well as revenue on sales to DyDo of product that we supply to them.

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. On December 18, 2023, DyDo submitted a Japan NDA for FIRDAPSE® to the PMDA. There can be no assurance that the application will be approved.

Future Markets for FIRDAPSE®

Under the amendment to our FIRDAPSE® license agreement that added Japan to our territory, upon the acceptance of DyDo's NDA by the PMDA (Japanese regulatory agency), which occurred on December 18, 2023, our territory in which we have the right to seek to commercialize FIRDAPSE® has automatically expanded to include several countries in Asia and Latin America, and we have begun taking steps seeking to expand our FIRDAPSE® activities into some of these other countries.

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 United States 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 April 7, 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 Patent No. 11,060,128 (the '128 patent) to our licensor and thereby to us, and this second 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.

On December 24, 2021, January 3, 2022 and January 7, 2022, the USPTO allowed various continuing applications that matured into U.S. Patent Nos. 11,268,128, 11,274,332 and 11,274,331, respectively. These patents were timely listed in the Orange Book and cover, among other things, methods of treating LEMS in subjects who are fast metabolizers of amifampridine.

As part of our transaction with Jacobus Pharmaceuticals, Catalyst 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®. The other patent, 9,783,497 issued by the USPTO on October 10, 2017, is not considered listable in the Orange Book, but, to the extent that it is necessary, Catalyst intends to enforce that patent against infringement as it would any of the Orange Book patents.

On December 19, 2023 and January 6, 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 any additional patents will be issued that provide additional intellectual property protection for 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).

In January 2023, we received Paragraph IV Certification Notice Letters from three generic drug manufacturers advising us 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 United States. The notice letters each allege that our six patents listed in the FDA 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 FDCA, as amended by the Drug Price Competition and Patent Term Restoration Act of 1984, as amended, 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 ANDAs until May 2026 or entry of judgment holding the patents invalid, unenforceable, or not infringed, whichever occurs first, and 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. Further, in October 2023, we received a Paragraph IV Certification Notice Letter from a fourth generic drug manufacturer, and we filed a similar lawsuit against this manufacturer in November 2023 in the U.S. District Court for the District of New Jersey.

We intend to vigorously protect and defend our intellectual property for FIRDAPSE® and, although there can be no assurance, we believe that our patents will protect FIRDAPSE® from generic competition.

We have also in-licensed the FIRDAPSE® trademark from our licensor, SERB S.A., and the trademark was registered in the United States 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 approximately 50 million individuals worldwide, 80% of whom live in developing countries. An estimated 1.7% of U.S. adults have been diagnosed with the condition.

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 four 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® 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 Schedule III controlled substance under the Federal Controlled Substances Act (CSA) prior to its approval due to evidence of prolonged use creating a physical dependence in some patients and the potential for 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 3.47 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, approximately 50 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®.

Focal onset seizures, also known as focal aware seizures and formerly known as partial onset seizures, are the most common type of seizure in people with epilepsy. There are two types of focal onset seizures, though there is often not a clear distinction between them. Simple focal seizures, also known as auras, occur in one area on one side of the brain, but may spread from there. The person does not lose consciousness during a simple focal seizure. Doctors generally break focal seizures into four groups:

- **Motor:** A simple focal seizure with motor symptoms will affect muscle activity, causing jerking movements of a foot, the face, an arm or another part of the body. Physicians can diagnose which side of the brain is affected by observing which side of the body experiences symptoms, since the left brain controls the right side of the body and the right brain controls the left.
- **Sensory:** A simple focal seizure may cause sensory symptoms affecting the senses, such as: hearing problems, hallucinations and olfactory or other distortions.
- **Autonomic:** A simple focal seizure with autonomic symptoms affects the part of the brain responsible for involuntary functions. These seizures may cause changes in blood pressure, heart rhythm, or bowel or bladder function.
- **Psychic:** Some simple focal seizures strike parts of the brain that trigger emotions or memories of previous experiences, causing feelings of fear, anxiety, or déjà vu (the illusory feeling that something has been experienced before).

Complex focal seizures are often preceded by a simple focal seizure. Patients experiencing a complex focal seizure may stare blankly into space, or experience automatisms (non-purposeful, repetitive movements such as lip smacking, blinking, grunting, gulping or shouting).

Tonic-clonic seizures, formerly known as grand mal seizures, comprise two stages: a tonic phase and a clonic phase. These intense seizures can be frightening to experience or observe, as extreme muscle spasms may temporarily arrest breathing. The seizure may start with a simple or complex partial seizure known as an aura. The person may experience abnormal sensations such as a particular smell, vertigo, nausea, or anxiety. If the person is familiar with having seizures, they may recognize the warning signs of a seizure about to begin.

When the tonic-clonic seizure begins, the person loses consciousness and may fall. Strong tonic spasms of the muscles can force air out of the lungs, resulting in a cry or moan, even though the person is not aware of their surroundings. There may be saliva or foam coming from the mouth. If the person inadvertently bites their tongue or cheek, blood may be visible in the saliva. Stiffness of the chest muscles may impair breathing, the person's face may look bluish or gray, and he or she may make gasping or gurgling sounds. This is known as the "tonic" phase.

Jerking movements affect the face, arms and legs, becoming intense and rapid. After one to three minutes, the jerking movements slow down and the body relaxes, sometimes including the bowel or bladder. The person may let out a deep sigh and return to more normal breathing. This is known as the "clonic" phase.

After a tonic-clonic seizure, the person may remain unconscious for several minutes as the brain recovers from the seizure activity. He or she may appear to be sleeping or snoring. Gradually the person regains awareness and may feel confused, exhausted, physically sore, sad or embarrassed for a few hours. The person may not remember having a seizure and may have other memory loss. Occasionally, people may have abnormal or combative behavior after a tonic-clonic seizure while the brain is recovering.

Access to FYCOMPA®

Catalyst is supporting patients using FYCOMPA® through an Instant Savings Card Program. Through the program, eligible commercially insured patients could pay as little as $10 for their FYCOMPA® co-pay (with a maximum savings of $1,300 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.

Acquisition of FYCOMPA®

On December 17, 2022, we entered into an Asset Purchase Agreement (Eisai APA) with Eisai, pursuant to which we acquired the U.S. rights to FYCOMPA®. Pursuant to the Eisai APA 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 agreed to pay Eisai an additional cash payment of $25 million if the then pending request for reconsideration of the patent extension for FYCOMPA® was approved by the USPTO, which request was denied in June 2023 (see "Intellectual Property Protections for FYCOMPA®" below for additional information on the patent).

In conjunction with the Eisai APA, at the closing of our purchase on January 24, 2023 we entered into two additional agreements with Eisai:

- A TSA under which a U.S. subsidiary of Eisai is providing 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 is assisting 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.

The transition services provided under the TSA ended on December 31, 2023.

Clinical Trials Supporting the Approval of 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 (see Figure 1). 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 one to three AEDs experiencing at least three 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 four 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 May 23, 2025, including patent term extension. Although the Company had requested that the USPTO reconsider this expiration date in favor of a June 8, 2026 expiration date, the request for reconsideration of the agency's patent term extension calculation was denied and the Company has exhausted its reasonable avenues for an extension of that patent term. The Company will update the Orange Book to reflect the May 23, 2025 expiration date at the appropriate time. The second FYCOMPA® patent in the Orange Book is U.S. Patent No. 8,772,497 (the '497 patent). This patent claims the commercial crystalline form of perampanel for FYCOMPA® and expires on July 1, 2026. The '497 patent has been the subject of previous Paragraph IV certifications from three ANDA filers. U.S. Patent No. 8,304,548, which is not Orange Book listable, claims the commercial process used to produce perampanel for FYCOMPA® and has an expiration date of October 14, 2027.

On February 20, 2023, we 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 us later in February with a similar certification for the tablet formulation for FYCOMPA®, the fourth such certification for this formulation. Both of these letters were paragraph IV certifications of non-infringement, non-validity, and unenforceability of the '497 patent for FYCOMPA® but each application, like the previous Paragraph IV notices from ANDA filers, does not challenge the '571 patent. Similar to the actions with the FIRDAPSE® Paragraph IV Certifications described above, 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 for both FYCOMPA® formulations, thus triggering the 30 month stay for each application.

AGAMREE® Product Overview

AGAMREE® is a structurally unique steroidal anti-inflammatory drug to treat children and adolescents 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 or side effects.

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

Clinical Trials Supporting the Approval of AGAMREE®

To support the approval of AGAMREE®, a randomized, double-blind, placebo and prednisone-controlled multinational trial of vamorolone was carried out in 121 male patients with DMD. The study included patients 4 to less than 7 years of age at time of enrollment in the study who were corticosteroid naïve and ambulatory. The trial met the primary (time to stand velocity after 24 weeks for vamorolone, 6 mg/kg per day vs placebo) and several 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 several 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.

Access to AGAMREE®

We plan to support AGAMREE® through our Catalyst Pathway 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 the AGAMREE® License Agreement and the Investment Agreement with 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®. Additionally, following approval of the NDA for the drug, on October 26, 2023, we became obligated to make a milestone payment of $36 million to Santhera, $26 million of which Santhera has advised us was used to make milestone payments to third parties. The $36 million payment was made 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. 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 Europe and Japan should Santhera pursue partnership opportunities in those territories. Additionally, we will hold the North American rights to any future approved indications for AGAMREE®

Concurrently 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 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 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 of AGAMREE® in DMD and future development of additional indications for AGAMREE®. At February 26, 2024, the closing price of Santhera's common shares on the SIX Swiss Exchange was CHF 11.36 per share.

Intellectual Property Protections for AGAMREE®

AGAMREE® is protected by six Orange Book listed patents with expiration dates ranging from May 2029 to July 2040. The Orange Book listed patents 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-.kappa.B 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-κβ for treatment of disease	March 7, 2033
11,833,159	Non-hormonal steroid modulators of NF-κβ for treatment of disease	May 28, 2029

In addition, vamorolone is covered by pending and issued patents in Canada and Mexico.

AGAMREE® has New Chemical Entity exclusivity that expires in October 2028. AGAMREE® also enjoys Orphan Drug Exclusivity expiring in October 2030. 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 could file 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 the ANDA until April 26, 2031.

Duchenne Muscular Dystrophy

Duchenne Muscular Dystrophy, or DMD, is an x-linked 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 United States, 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 in the dystrophin gene on that chromosome is expected to cause DMD.

Generic Sabril®

In September 2015, we announced the launch of a program to develop our version of vigabatrin (CPP-109) as a generic version of Sabril®, which is marketed in the United States by Lundbeck. Lundbeck's exclusivity for Sabril® expired on April 26, 2018. Vigabatrin comes in two dosage forms – a powder sachet and a tablet. Par Pharmaceutical brought the first generic version of the powder sachet to market, and since then numerous additional generic versions of this product have been approved. Further, four generic versions of vigabatrin tablets have also been approved.

On December 18, 2018, we entered into a definitive agreement with Endo International plc's subsidiary, Endo Ventures Limited (Endo), for the further development and commercialization of generic Sabril® tablets through Endo's United States Generic Pharmaceuticals segment, Par Pharmaceutical (Par). Pursuant to the agreement, in December 2018, we received an up-front payment of $0.5 million.

During October 2023, we were informed by Endo that it is discontinuing work on the collaboration for development and commercialization of vigabatrin and that it wished to terminate the arrangement. The end of the collaboration did not have a material impact on our consolidated financial statements.

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 manufacture 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 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®.

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. 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 from compounding pharmacies for many years and likely remains available, even though we have obtained FDA approval of FIRDAPSE® and federal law, as discussed below, generally prohibits compounding "essentially copies" of commercially available drugs unless those drugs are on the FDA's published drug shortages list. Compounded amifampridine is likely to be substantially less expensive than FIRDAPSE®. The Food and Drug Administration Modernization Act of 1997 included a new section, which clarified the status of pharmacy compounding under Federal law. Under Section 503A, drug products that are lawfully compounded by a pharmacist or physician for an individual patient may be entitled to exemptions from three key provisions of the FDCA: (1) the adulteration provision of section 501(a)(2)(B) (concerning FDA's cGMP regulations); (2) the misbranding provision of section 502(f)(1) (concerning the labeling of drugs with adequate directions for use); and (3) the new drug provision of section 505 (concerning the approval of drugs under NDAs or ANDAs).

To qualify for these statutory exemptions, a compounded drug product must satisfy several legal requirements. One of these requirements restricts the universe of bulk drug substances that a compounder may use. Specifically, every bulk drug substance used in compounding: (1) must comply with an applicable and current USP or NF drug monograph, if one exists, as well as the current USP chapters on pharmacy compounding; (2) if such a monograph does not exist, the bulk drug substance must be a component of an FDA-approved drug; or (3) if a monograph does not exist and the bulk drug substance is not a component of an FDA-approved drug, it must appear on a list of bulk drug substances that may be used in compounding (i.e., the "Section 503A bulk substances list 1"). While the advertising provisions in Section 503A were ruled unconstitutional in part in the United States by the Supreme Court in 2002, the FDA, since 2013, has aggressively regulated and exercised oversight over the practice of pharmacy compounding following the compounding incident at the New England Compounding Center in Massachusetts that sickened hundreds and killed over 60 individuals.

In 2013, Congress removed the unconstitutional advertising provisions in Section 503A when it passed the Drug Quality and Security Act of 2013 (DQSA), Title I (The Compounding Quality Act), rendering Section 503A enforceable in all states. The DQSA also created "outsourcing facilities" under Section 503B of the FDCA, which are drug compounders that voluntarily register with FDA and may produce compounded formulations for office use (at least one of which must be sterile), but must comply with FDA's cGMP

regulations and other requirements set forth in Section 503B. Section 503B outsourcing facilities may also only compound from bulk substances if the product is on FDA's drug shortage list, or the substance is on FDA's Section 503B interim and final 2013 list of bulk substances that may be used in compounding (i.e., the Section 503B bulk substances list 1). The active pharmaceutical ingredient in FIRDAPSE® is not currently included in the FDA's interim or final Section 503B list of bulk substances that may be used in compounding, nor has it been nominated for its inclusion.

While the FDA has been enforcing Section 503A since its re-enactment, compounders may still compound "near copies" (but not "essentially copies") of approved drug products, under Section 503A, so long as the prescriber makes a change to the compounded formulation that produces for that patient a significant difference between the commercially available drug and the compounded version. Section 503A compounders may also copy commercially available products if they do not do so in "regular or inordinate amounts." In January 2018, FDA published a Final Guidance document titled, "Compounded Drug Products That Are Essentially Copies of a Commercially Available Drug Product Under Section 503A of the Federal Food, Drug, and Cosmetic Act." This Final Guidance sets forth FDA's enforcement policy concerning those compounders that make essentially copies of commercially available drug products. FDA has defined the term "regular or inordinate" in the Final Guidance to mean: "a drug product that is essentially a copy of a commercially available drug product is compounded regularly or in inordinate amounts if it is compounded more frequently than needed to address unanticipated, emergency circumstances, or in more than the small quantities needed to address unanticipated, emergency circumstances." FDA has further stated it will not take enforcement action, considering all the facts and circumstances, against a compounder that compounds less than four "essentially copies" of a commercially available drug product in a calendar month.

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.

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 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. Glucocorticoids are also used, with such drugs being seen as delaying the development of scoliosis and reducing the need for surgery. In September 2016, the FDA granted accelerated approval of Eteplirsen, an "exon skipping" drug that targets a section of DNA called exon 51. Alaluren is an orally administered drug being developed for the treatment of genetic defects caused by what are known as "nonsense mutation", a condition suffered by 10-15% of DMD patients and is currently approved in the United Kingdom and European Union. In December 2019, Vyondys 53, an "exon skipping" drug that targets a section of DNA called exon 53, and in August 2020, Viltespo, also an "exon skipping" drug that targets a section of DNA called exon 53, was also approved. Exon 53 skipping may help up to 8% of DMD patients. In addition, there are many companies who have announced plans for pre-clinical candidates and clinical development for the treatment of DMD, including gene transfer or gene editing therapies. The status of clinical development is unknown for many of these compounds, but as other companies continue to pursue development and approval of products for the treatment of DMD, their products may or may not prove to be safer and/or more efficacious than AGAMREE®.

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 cover and 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, including the availability of funding from the federal government.

Business Development

Following our recent 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 clinically differentiated and adequately de-risked opportunities, with a keen focus on products to treat rare (orphan) CNS, and adjacent rare (orphan) therapeutic categories. 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. To accomplish these priorities, we are continuing to employ a disciplined, comprehensive, and exhaustive approach to identifying and evaluating assets. 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, mid 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, 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 United States, 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, sale, 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 United States.

In the United States, 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 United States 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 United States 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 current good manufacturing practice (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.

United States 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 individuals with the disease or condition to be studied 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.

United States Review and Approval Process

FDA approval of an NDA is required before marketing of the product may begin in the United States. 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 2024 this fee is $4,048,695), 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 2024 is $416,734 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

Drugs and other substances are subject to scheduling as a controlled substance under the Controlled Substances Act (CSA) depending on the drug's potential for abuse. The CSA and regulations promulgated by the Drug Enforcement Administration (DEA) impose certain requirements on controlled substances that are pharmaceutical products approved by FDA related to manufacturing, import, export, distribution and dispensing of such substances. These include registration, security, recordkeeping and reporting requirements. The CSA and regulations classify 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 classified in Schedule II, III, IV or V, depending on the potential for abuse and psychological or physical dependence. Schedule II substances are defined as having 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 United States, and (iii) abuse may lead to moderate or low physical dependence or high psychological dependence.

Manufacturing, distributing and dispensing of Schedule III drugs are subject to specific regulatory requirements including limits on amounts that may be imported. In addition to federal regulations, the states have established separate regulations and requirements for controlled substances relating to manufacturing, storage, distribution and physician prescription procedures, including in some cases 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 and comply with the regulatory requirements. The DEA and the relevant state agencies periodically inspect 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 Abbreviated New Drug Application (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 notification 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 2024, the application fees are $252,453 per ANDA application. In addition, there are facility fees based on the location of the finished dosage form facility and active pharmaceutical ingredient facility, and an 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 United States, 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 United States 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 United States, governmental payors such as Medicare and Medicaid, managed care organizations, private commercial health insurers and pharmacy benefit managers (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 United States 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 (Affordable Care Act) and the Inflation Reduction Act of 2022 (IRA).

We anticipate that in the United States, 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 prices of newly launched drugs, 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 recently 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 United States

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 PBMs. 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, 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 United States 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 United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making available in the United States a drug for this type of disease or condition will be recovered from sales in the United States for that drug. In the United States, 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) was recently enacted, which 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. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it. 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. Additionally, a violation of the federal anti-kickback statute can serve as a basis for liability under the federal civil false claims act.

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.

Other federal statutes pertaining to healthcare fraud and abuse include the Civil Monetary Penalties Law statute, which prohibits, among other things, the offer or payment of remuneration to a Medicaid or Medicare beneficiary that the offeror or payor knows or should know is likely to influence the beneficiary to order or receive a reimbursable item or service from a particular provider, practitioner, or supplier, and the additional federal criminal health care fraud statute created by the Health Insurance Portability and Accountability Act of 1996 (HIPAA), which prohibit, among other things, knowingly and willfully executing or attempting to execute a scheme to defraud any healthcare benefit program or obtain by means of false or fraudulent pretenses, representations or promises any money or property owned by or under the control of any healthcare benefit program in connection with the delivery of or payment for healthcare benefits, items or services. In addition, HIPAA, as amended by the Health Information Technology for Economic and

Clinical Health Act of 2009 (HITECH), and their respective implementing regulations impose obligations on certain healthcare providers, health plans and healthcare clearinghouses, known as covered entities, as well as their business associates and their subcontractors 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. HITECH increased the civil and criminal penalties that may be imposed against covered entities, business associates, their covered subcontractors and possibly other persons, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorney's fees and costs associated with pursuing federal civil actions. Pursuant to the Physician Payment Sunshine Act, the Centers for Medicare & Medicaid Services (CMS) issued a final rule that requires certain manufacturers of approved prescription drugs that are reimbursable under Medicare, Medicaid, or the Children's Health Insurance Program, with certain exceptions, to collect and report information on payments or transfers of value to physicians, defined to include doctors, dentists, optometrists, podiatrists and chiropractors, certain other health care professionals such as physician assistants, and 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 or to report certain pricing information, including price increases and the price of newly launched drugs. 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, several states, including California, Connecticut, Nevada, and Massachusetts require pharmaceutical companies to implement compliance programs and/or marketing codes. Several additional states are considering similar proposals. Certain states and local jurisdictions also require the registration of pharmaceutical sales representatives. Compliance with these laws is difficult and time consuming, and companies that do not comply with these state laws face civil penalties. Additionally, we may also be subject to state laws governing the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts. For example, the California Consumer Privacy Act of 2018 (CCPA) imposes obligations on businesses to which it applies, including, but not limited to, providing specific disclosures in privacy notices and affording California residents certain rights related to their personal data, although it exempts some data processed in the context of clinical trials. In addition, the California Privacy Rights Act of 2020 (CPRA), which went into effect on January 1, 2023, imposes additional obligations on companies covered by the legislation and significantly modifies the CCPA, including by expanding consumers' rights with respect to certain sensitive personal information. The CPRA also creates a new state agency that is vested with authority to implement and enforce the CCPA and CPRA. Virginia's Consumer Data Protection Act, which took effect on January 1, 2023, requires businesses subject to the legislation to conduct data protection assessments in certain circumstances and requires opt-in consent from consumers to acquire and process their sensitive personal information, which includes information revealing a consumer's physical and mental health diagnosis and genetic and biometric information that can identify a consumer. In addition, Colorado enacted the Colorado Privacy Act, and Connecticut enacted the Connecticut Data Privacy Act, each of which took effect on July 1, 2023, and Utah enacted the Consumer Privacy Act, which became effective on December 31, 2023, and each of these laws may increase the complexity, variation in requirements, restrictions and potential legal risks, and could require increased compliance costs and changes in business practices and policies. Other states have also enacted, proposed, or are considering proposing, data privacy laws, which could further complicate compliance efforts, increase our potential liability and adversely affect our business.

Prescription drug advertising is subject to federal, state and foreign regulations. In the United States, 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 United States 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 new "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 United States 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 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 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. The rebate amount for a drug has been capped at 100% of the AMP; however, effective January 1, 2024, this cap is 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 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.

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 suppliers for drugs covered under Medicare Part B. 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 seniors and people with disabilities. Medicare Part D enrollees once had a gap in their coverage (between the initial coverage limit and the point at which catastrophic coverage begins) where Medicare did not cover their prescription drug costs, known as the coverage gap. However, beginning in 2019, Medicare Part D enrollees paid 25% of brand drug costs after they reach the initial coverage limit—the same percentage they were responsible for before they reached that limit—thereby closing the coverage gap. Most of the cost of closing the coverage gap is being borne by innovator companies and the government through subsidies. Each manufacturer of a drug approved under an NDA is required to enter into a Medicare Part D coverage gap discount agreement and provide a 70% discount on those drugs dispensed to Medicare enrollees in the coverage gap, in order for its drugs to be reimbursed by Medicare Part D. Beginning in 2025, the IRA eliminates 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 maximum, and 20% once the out-of-pocket maximum has been reached. Although these discounts represent a lower percentage of enrollees' costs than the current discounts required below the out-of-pocket maximum (that is, in the coverage gap phase of Part D coverage), the new manufacturer contribution required above the out-of-pocket maximum could be considerable for very high-cost patients and the total contributions by manufacturers to a Part D enrollee's drug expenses may exceed those currently provided.

The IRA also allows HHS to directly negotiate the selling price of a statutorily specified number of drugs and biologics each year that CMS reimburses under Medicare Part B and Part D, although only high-expenditure single-source drugs that have been approved for at least 7 years (11 years for single-source biologics) are eligible to be selected by CMS for negotiation, with the negotiated price taking effect two years after the selection year. Negotiations for Medicare Part D products begin in 2024 with the negotiated price taking effect in 2026, and negotiations for Medicare Part B products will begin in 2026 with the negotiated price taking effect in 2028. A drug or biological product that has an orphan drug designation for only one rare disease or condition will be excluded from the IRA's price negotiations requirements, but will lose that exclusion if it receives designations for more than one rare disease or condition, or if is approved for an indication that is not within that single designated rare disease or condition, unless such additional designation or such disqualifying approvals are withdrawn by the time CMS evaluates the drug for selection for negotiation. In August 2023, HHS announced the ten Medicare Part D drugs and biologics that it selected for negotiations. HHS will announce the negotiated

maximum fair prices by September 1, 2024, and this price cap, which cannot exceed a statutory ceiling price, will come into effect on January 1, 2026, and will represent a significant discount from average prices to wholesalers and direct purchasers. The IRA also imposes rebates on Medicare Part D and Part B drugs whose prices have increased at a rate greater than the rate of inflation. The IRA also extends enhanced subsidies for individuals purchasing health insurance coverage in ACA marketplaces through plan year 2025. The IRA permits the Secretary of HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years. Manufacturers that fail to comply with the IRA may be subject to various penalties, some significant, including civil monetary penalties. These provisions may be subject to legal challenges. For example, the provisions related to the negotiation of selling prices of high-expenditure single-source drugs and biologics have been challenged in multiple lawsuits brought by pharmaceutical manufacturers. Thus, while it is unclear how the IRA will be implemented, it will likely have a significant impact on the pharmaceutical industry and our business.

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) of the General Services Administration. The law also requires manufacturers to offer deeply discounted FSS contract pricing for purchases of their covered drugs by the VA, DoD, the Coast Guard, and the Public Health Service (including the Indian Health Service) in order for federal funding to be available for reimbursement or purchase of the manufacturer's drugs under certain federal programs. 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 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.

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 or biologics approved biologics license applications (BLAs), 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 or BLAs. 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.

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.

Employee Profile

As of February 28, 2024, we had approximately 167 employees, approximately 105 of whom are in our commercial organization, approximately 30 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 for all employees 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.

Diversity, Equity and Inclusion

Our goal is a diverse and inclusive workforce – not because it is the right thing to do but because we believe that such a workforce is key to our long-term success. Approximately 57% of our employees are female. At the leadership level (employees at manager and above) approximately 58% are female, and one of seven members of our C-suite is female.

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. We hold regular in-person meetings with our sales staff, which serve to bring together and energize our staff. In addition, we are always looking for new and different ways to further engage our staff as a team and individually.

Available Information

We make available free of charge on or through our Internet 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 payors 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.
- Our business may require additional capital.
- 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 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 product development programs.
- Our employees, sales agents and consultants may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

Risks Related to Government Regulation

- 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.
- 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.
- 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 United States 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® 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.
- Changes to the ODA or successful legal challenges to the FDA's interpretation of the ODA 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.

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.

- Whether we will be successful in our litigation to enforce our patents against Paragraph IV challengers who have filed relating to FIRDAPSE® and FYCOMPA®.
- 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 Lambert-Eaton Myasthenic Syndrome (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 Duchenne Muscular Dystrophy (DMD). We invest a significant amount of effort and financial resources in the commercialization of these drug products in the U.S. 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 current good manufacturing practice (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 payors' 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 payors 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 payors, 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 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 payor 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 outside of the neuromuscular disease space 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 rare disease therapeutic categories outside of neuromuscular diseases. To accomplish these new 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 and 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 several of our products are small, we must achieve significant market share and obtain relatively high per-patient prices for those 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, 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, and, in the case of FIRDAPSE®, high per patient prices could drive physicians to seek out compounding pharmacies to provide compounded amifampridine to fill their prescriptions rather than FIRDAPSE®, thereby lowering the FIRDAPSE® market share or penetration in the market. 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® for diseases with small patient populations. Further, even if we obtain significant market share for FIRDAPSE®, 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 United States;
- 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 currently have the ability to independently conduct pre-clinical studies or clinical studies and trials, and we typically 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 current good manufacturing practices (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 sales 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 sales 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. In the United States, the principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services (CMS). CMS decides whether and to what extent our products will be covered and reimbursed under Medicare and private payors tend to follow CMS to a substantial degree. 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 United States 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. There have been and continue to be a number of initiatives at the United States federal and state levels that seek to reduce healthcare costs. For example, in December 2020, CMS issued a final rule implementing significant manufacturer price reporting changes under the Medicaid Drug Rebate Program, including an alternative rebate calculation for line extensions that is tied to the price increases of the original drug, and Best Price reporting related to certain value-based purchasing arrangements. Additionally, under the American Rescue Plan Act of 2021, effective January 1, 2024, the statutory cap on Medicaid Drug Rebate Program rebates that manufacturers pay to state Medicaid programs on a unit of drug is eliminated. Elimination of this cap may, in some cases, require pharmaceutical manufacturers to pay more in rebates than they receive on the sale of products. Additionally, the Budget Control Act which, subject to certain temporary suspension periods, imposed 2% reductions in Medicare payments to providers per fiscal year starting April 1, 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2031, unless additional Congressional action is taken. Healthcare reforms that have been adopted, and that may be adopted in the future, could result in further reductions in coverage and levels of reimbursement for pharmaceutical products, increases in rebates payable under U.S. government rebate programs and additional downward pressure on pharmaceutical product prices. These 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 United States. 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 United States 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. Several healthcare reform proposals recently culminated in the enactment of the IRA, which will eliminate, 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 maximum, and 20% once the out-of-pocket maximum has been reached. The IRA also allows HHS to directly negotiate the selling price of a statutorily specified number of drugs and biologics each year that CMS reimburses under Medicare Part B and Part D. Only high-expenditure single-source drugs that have been approved for at least 7 years (11 years for single-source biologics) are eligible to be selected by CMS for negotiation, with the negotiated price taking effect two years after the selection year. Negotiations for Medicare Part D products take place in 2024 with the negotiated price taking effect in 2026, and negotiations for Medicare Part B products will begin in 2026 with the negotiated price taking effect in 2028. In August 2023, HHS announced the ten Medicare Part D drugs and biologics that it selected for negotiations. HHS will announce the negotiated maximum fair prices by September 1, 2024, and this price cap, which cannot exceed a statutory ceiling price, will go into effect on January 1, 2026. A drug or biological product that has an orphan drug designation for only one rare disease or condition will be excluded from the IRA's price negotiation requirements, but will lose that exclusion if it receives designations for more than one rare disease or condition, or if is approved for an indication that is not within that single designated rare disease or condition, unless such additional designation or such disqualifying approvals are withdrawn by the time CMS evaluates the drug for selection for negotiation. The IRA also imposes rebates on Medicare Part D and Part B drugs whose prices have increased at a rate greater than the rate of inflation. Manufacturers that fail to comply with the IRA may be subject to various penalties, including significant civil monetary penalties. The IRA also extends enhanced subsidies for individuals purchasing health insurance coverage in ACA marketplaces through plan year 2025. These provisions may be subject to legal challenges. For example, the provisions related to the negotiation of selling prices of high-expenditure single-source drugs and biologics have been challenged in multiple lawsuits brought by pharmaceutical manufacturers. Thus, while it is unclear how the IRA will be implemented, it will likely have a significant impact on the biopharmaceutical industry and the pricing of our products and product candidates. 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.

At the state level in the United States, legislatures are increasingly enacting laws and implementing regulations designed to control pharmaceutical and biologic product pricing, including price constraints, restrictions on certain product access, reporting on price increases and the introduction of high-cost drugs. In some states, laws have been enacted to encourage importation of lower cost drugs from other countries and bulk purchasing. For example, the FDA released a final rule in September 2020 providing guidance for states to build and submit importation plans for drugs from Canada, and FDA authorized the first such plan in Florida in January 2024. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our drug products that we successfully commercialize or put pressure on our product pricing. Legally mandated price controls on payment amounts by third-party payors or other restrictions on coverage or access could have a material adverse effect on our business, operations and financial condition.

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 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 United States 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, DEA, and state regulations for controlled substances, as well as any applicable corresponding manufacturing regulations outside of the United States. 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, DEA, state and other regulatory agencies. If any of our third-party suppliers or contract manufacturers fail to comply with cGMP or other applicable manufacturing regulations and requirements related to registration, security, recordkeeping and reporting of controlled substances, our ability to develop, commercialize, manufacture and distribute 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 United States 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® or any other drug candidates we may acquire or license and affect the prices we may obtain.

In the United States, 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® 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 United States 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 the conditions of use reflected in our label. 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.

If we fail to obtain or subsequently maintain orphan drug exclusivity or regulatory exclusivity for FIRDAPSE® 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 United States, 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 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.

Changes to the ODA or successful legal challenges to the FDA's interpretation of the ODA 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 United States 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 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 newly announced 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 ODA has been the subject of recent scrutiny from the press, from some members of Congress and from some in the medical community. Furthermore, the FDA's interpretations of the ODA 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 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.

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 governmental 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.

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.

Risks associated with our pending Paragraph IV litigation

As noted throughout this report, we are presently litigating several Paragraph IV challenges relating to two of our products, FIRDAPSE® and FYCOMPA®. If we are not successful in our litigation to enforce our patents against these Paragraph IV challengers, it could have a material adverse effect on our business and financial condition.

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 (IT) 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 IT 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 IT systems, such measures may not prevent such events. Significant disruptions of our IT 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 liability 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, such as the COVID-19 pandemic, 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 such as the COVID-19 pandemic 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.

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® and FYCOMPA®;
- 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 FIRDAPSE® License Agreement), 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 United States, 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 26, 2024, we had 117,863,258 shares of our common stock outstanding, of which 7,296,124 shares were held by our executive officers and directors. This includes the 10 million shares of our common stock that we sold in a public offering that closed on January 9, 2024. We also had outstanding: (i) stock options to purchase an aggregate of 14,414,654 shares at exercise prices ranging from $2.11 to $19.02 (9,025,987 of which are currently exercisable); and (ii) restricted stock units for 622,816 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.

We may fail to meet our publicly announced guidance or other expectations about our business and future operating results, which would cause our stock price to decline.

We release earnings guidance from time to time in our quarterly and annual earnings releases, or otherwise, regarding our future performance that represents our management's estimates as of the date of release. This guidance includes forward-looking statements based on projections prepared by our management. Projections are based upon a number of assumptions and estimates that, while presented with numerical specificity, are inherently subject to significant business, economic and competitive uncertainties and contingencies on our business, many of which are beyond our control and are based upon specific assumptions with respect to future business decisions, some of which will change. For example, in light of our acquisition of an exclusive license for North America for vamorolone in July 2023, you are cautioned not to place undue reliance on any guidance issued prior to such acquisition.

The principal reason that we release guidance is to provide a basis for our management to discuss our business outlook with analysts and investors. Furthermore, analysts and investors may develop and publish their own projections of our business, which may form a consensus about our future performance. Our actual business results may vary significantly from such guidance or that consensus due to a number of factors, many of which are outside of our control. Furthermore, if we make downward revisions of our previously announced guidance, if we withdraw our previously announced guidance, or if our publicly announced guidance of future operating results fails to meet expectations of securities analysts, investors or other interested parties, the price of our common stock would likely decline.

Guidance is necessarily speculative in nature, and it can be expected that some or all of the assumptions underlying the guidance furnished by us will not materialize or will vary significantly from actual results. Accordingly, our guidance is only an estimate of what management believes is realizable as of the date of release. Actual results may vary from our guidance, and the variations may be material. In light of the foregoing, investors are urged not to rely upon our guidance in making an investment decision regarding our common stock. Any failure to successfully implement our operating strategy or the occurrence of any of the events or circumstances set forth under the header "Item 1A – Risk Factors" from our filings with the SEC could result in the actual operating results being different from our guidance, and the differences may be adverse and material.

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 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 Chief Legal and Compliance Officer (CLCO), our Chief Operating Officer (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 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 is approximately $0.5 million.

Item 3. Legal Proceedings

Paragraph IV Patent Litigation

In January 2023, we received Paragraph IV Certification Notice Letters from three generic drug manufacturers 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 United States. The notice letters each alleged that the six patents listed in the FDA 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 FDCA, as amended by the Drug Price Competition and Patent Term Restoration Act of 1984, as amended, 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 ANDAs 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, 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. Further, in October 2023, we received a Paragraph IV Certification Notice Letter from a fourth generic drug manufacturer, and we filed a similar lawsuit against the manufacturer in November 2023.

We intend to vigorously protect and defend our intellectual property for FIRDAPSE® and, although there can be no assurance, we believe that our patent estate will protect FIRDAPSE® from generic competition.

On February 20, 2023, we 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 us later in February with a similar certification for the tablet formulation for FYCOMPA®, the fourth such certification for this formulation. Both of these letters were Paragraph IV certifications of non-infringement, non-validity, and unenforceability to the '497 patent for FYCOMPA® but each application, like the previous Paragraph IV notices from ANDA filers, for FYCOMPA® tablets does not challenge the '571 patent. Accordingly, the FDA may not approved any ANDA prior to expiration of the '571 patent, including patent term extension. Similar to the actions with the FIRDAPSE® Paragraph IV Certifications described above, 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 for both FYCOMPA® formulations, thus triggering the 30 month stay for each application.

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

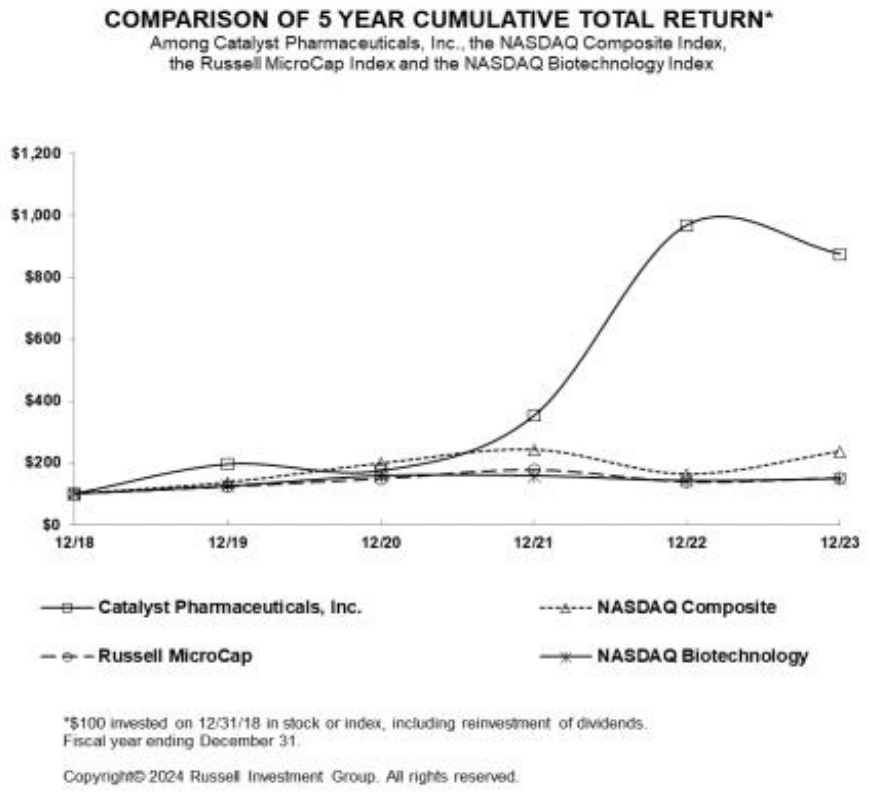
Not applicable.

PART II

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

Performance Graph

The graph below matches Catalyst Pharmaceuticals, Inc.'s cumulative 5-Year total shareholder return on common stock with the cumulative total returns of the NASDAQ Composite index, the Russell MicroCap index, and the NASDAQ Biotechnology index. The graph tracks the performance of a $100 investment in our common stock and in each index (with the reinvestment of all dividends) from 12/31/2018 to 12/31/2023.



	12/18	12/19	12/20	12/21	12/22	12/23
Catalyst Pharmaceuticals, Inc.	**100.00**	**195.31**	**173.96**	**352.60**	**968.75**	**875.52**
NASDAQ Composite	**100.00**	**136.69**	**198.10**	**242.03**	**163.28**	**236.17**
Russell MicroCap	**100.00**	**122.43**	**148.10**	**176.73**	**137.93**	**150.80**
NASDAQ Biotechnology	**100.00**	**125.11**	**158.17**	**158.20**	**142.19**	**148.72**

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

Market Information

Our common stock trades on the Nasdaq Capital Market under the symbol "CPRX." The closing sale price for the common stock on February 26, 2024 was $14.18. As of February 26, 2024, there were 17 holders of record of our common stock, which includes custodians who hold our securities for the benefit of others.

Dividend Policy

We have never declared or paid any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings to support operations and finance the growth and development of our business. We do not intend to pay cash dividends on our common stock for the foreseeable future. Any future determination related to our dividend policy will be made at the discretion of our Board of Directors.

Securities Authorized for Issuance under Equity Compensation Plans

The following table presents information as of December 31, 2023 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]	14,177,488	$ 7.73	1,801,949[2]
Equity compensation plans not approved by security holders	—	—	—
Total	**14,177,488**	**$ 7.73**	**1,801,949**

[1] Includes our 2014 Stock Incentive Plan and our 2018 Stock Incentive Plan

[2] Remaining shares are only under our 2018 Stock Incentive Plan

Sales of Unregistered Securities

None.

Issuer Purchases of Equity Securities

In March 2021, our Board of Directors approved a share repurchase program that authorizes the repurchase of up to $40 million of our common stock, pursuant to a repurchase program under Rule 10b-18 of the Securities Act (the "Share Repurchase Program"). The Share Repurchase Program commenced on March 22, 2021.

At present, we are not purchasing shares under our share repurchase program, but rather we are retaining cash for use in our business development activities.

During the fiscal year ended December 31, 2023, we did not repurchase any of our common stock. Approximately $21 million remains available under the Share Repurchase Program as of December 31, 2023.

Item 6. Selected Financial Data

Not applicable.

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

The following discussion and analysis of our financial condition and results of operations should be read in conjunction our consolidated financial statements and related notes appearing elsewhere in this report. In addition to historical information, this discussion and analysis contains forward-looking statements that involve risks, uncertainties, and assumptions. Our actual results may differ materially from those anticipated in these forward-looking statements as a result of certain factors, including but not limited to those set forth under the caption "Risk Factors" in Item 1A of this report.

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 2023 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 utilize concerted diligence efforts in search of therapies that will improve the lives of those who suffer from rare or difficult to treat diseases. 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.

Our flagship U.S. commercial product is FIRDAPSE® (amifampridine) Tablets 10 mg approved for the treatment of Lambert-Eaton myasthenic syndrome, or LEMS, for adults and for children ages six and up. Further, on January 24, 2023, we closed our acquisition of FYCOMPA® and are now also marketing that product in the United States. FYCOMPA® (perampanel) CIII is a prescription medication used alone or 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. Finally, on July 18, 2023, we closed our acquisition of an exclusive license for North America for vamorolone, a novel corticosteroid treatment for patients suffering from Duchenne Muscular Dystrophy (DMD). On October 26, 2023, the FDA approved AGAMREE® (vamorolone) oral suspension 40 mg/ml for the treatment of DMD. We are currently planning the commercial launch of AGAMREE® in the United States during the first quarter of 2024.

FIRDAPSE®

On November 28, 2018, we received approval from the FDA for our new drug application, or 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 United States. Further, on September 29, 2022, the FDA approved our supplemental NDA (sNDA) to expand the indicated age range for FIRDAPSE® Tablets 10 mg to include pediatric patients, six years of age and older for the treatment of LEMS.

We sell FIRDAPSE® through a field force experienced in neurologic, central nervous system or rare disease products consisting at this time of approximately 35 field personnel, including sales (Regional Account Managers), thought leader liaisons, patient assistance and insurance navigation support (Patient Access Liaisons), and payor reimbursement (National Account Managers). 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, for the last few years we have contracted with an experienced inside sales agency that works to generate leads through telemarketing to targeted physicians. This inside sales agency allowed our sales efforts to not only reach the neuromuscular specialists who regularly treat LEMS patients, but also the roughly 9,000 neurology and neuromuscular healthcare providers that may be treating a LEMS patient who can benefit from FIRDAPSE®. However, effective January 1, 2024 we have terminated that arrangement. We also 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 with 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.

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 and the National Organization for Rare Disorders) 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. 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 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 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. A co-pay assistance program designed to keep out-of-pocket costs to not more than $10 per month (currently less than $2 per month) is available for all LEMS patients with commercial coverage who are prescribed FIRDAPSE®. Our FIRDAPSE® co-pay assistance program is not available to patients enrolled in state or federal healthcare programs, including Medicare, Medicaid, VA, DoD, or TRICARE. Separately, 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. Subject to compliance with regulatory requirements, our goal is that no LEMS patient is ever denied access to their medication for financial reasons.

In January 2023, we received Paragraph IV Certification Notice Letters from three generic drug manufacturers 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 United States. The notice letters each alleged that the six patents listed in the FDA Approved Drug Products with Therapeutic Equivalence Evaluations (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, as amended, 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 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. Further, in October 2023, we received a Paragraph IV Certification Notice Letter from a fourth generic drug manufacturer, and we filed a similar lawsuit against this manufacturer in November 2023 in the U.S. District Court for the District of New Jersey.

We intend to vigorously protect and defend our intellectual property for FIRDAPSE® and, although there can be no assurance, we believe that our patent estate will protect FIRDAPSE® from generic competition.

On August 4, 2023, we submitted an sNDA to increase the indicated maximum daily dosage of FIRDAPSE® tablets from 80 mg to 100 mg for the treatment of LEMS. On October 13, 2023, we announced that the FDA had accepted for review our sNDA and assigned a Prescription Drug User Fee Act (PDUFA) action date of June 4, 2024. There can be no assurance that the FDA will approve our sNDA.

We are advised by our sub-licensee for FIRDAPSE® in Japan, DyDo Pharma, Inc. (DyDo), that on December 18, 2023, based on a preliminary favorable interim data analysis after six months into the safety phase of its registration study to evaluate the efficacy and safety of FIRDAPSE® for the treatment of LEMS, they filed a Japan NDA with the Pharmaceuticals and Medical Devices Agency (PMDA) in Japan seeking approval to commercialize the product in Japan. The review period is expected to be approximately nine months from the submission date, and there can be no assurance that the NDA filing made by DyDo will be approved.

Further, upon acceptance of the Japan NDA by the PMDA, which occurred on December 18, 2023, our license for FIRDAPSE® automatically expanded to include other key markets in Asia and Latin America, and we are currently initiating plans to seek opportunities to expand FIRDAPSE®'s global footprint through strategic partnerships (with the current focus on the Asia Pacific and Latin American regions).

FYCOMPA®

On December 17, 2022, we entered into an agreement with 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 an additional cash payment of $25 million if a requested patent extension for FYCOMPA® until June 8, 2026 was approved by the U.S. Patent and Trademark Office (USPTO), which did not occur. Finally, we 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 with Eisai; a TSA and a Supply Agreement. Under the TSA, a U.S. subsidiary of Eisai provided us with certain transitional services, and 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). As of December 31, 2023, the transition services under the TSA have been completed.

Initially, following the closing of the acquisition, we began to market FYCOMPA® in the U.S. through Eisai under the TSA as we built our FYCOMPA® marketing and sales team, and in May 2023, we took over the marketing program for FYCOMPA®. In that regard, we have hired approximately 35 sales and marketing personnel to support FYCOMPA®, most of whom previously worked in Eisai's U.S. sales division marketing FYCOMPA®. We have also hired seven medical science liaisons to help us educate the medical community who treat epilepsy about scientific literature regarding epilepsy and 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 $10 for their FYCOMPA® co-pay (with a maximum savings of $1,300 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® is primarily from two patents listed in the Orange Book. The first, U.S. patent no. 6,949,571 (the '571 patent) will expire May 23, 2025, including patent term extension. Although the Company had requested that the USPTO reconsider this expiration date in favor of a June 8, 2026 expiration date, the request for reconsideration of the agency's patent term extension calculation was denied and the Company has exhausted its reasonable avenues for an extension of that patent term. The Company will update the Orange Book to reflect the May 23, 2025 expiration date at the appropriate time. 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. The '497 patent has been the subject of previous Paragraph IV certifications from three NDA filers.

On February 20, 2023, we 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 us later in February with a similar certification for the tablet formulation for FYCOMPA®, the fourth such certification for this formulation. Both of these letters were paragraph IV certifications of non-infringement, non-validity, and unenforceability of the '497 patent for FYCOMPA® but each application, like the previous Paragraph IV notices from ANDA filers, does not challenge the '571 patent. Accordingly, the FDA may not approve any ANDA prior to expiration of the '571 patent, including patent term extension. Similar to the actions with the FIRDAPSE® Paragraph IV Certifications described above, 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 for both FYCOMPA® formulations, thus triggering the 30 month stay for each application.

AGAMREE®

On June 19, 2023, we entered into the AGAMREE® License Agreement and the Investment Agreement with 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®. Additionally, following approval of the NDA for the drug, on October 26, 2023, we became obligated to make a milestone payment of $36 million to Santhera, $26 million of which Santhera has advised us was used to make milestone payments that they owe to third parties. The $36 million payment was made 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. 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 Europe and Japan should Santhera pursue partnership opportunities in those territories. Additionally, we will hold the North American rights to any future approved indications for AGAMREE®.

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 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 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 of AGAMREE® in DMD and future development of additional indications for AGAMREE®. At February 26, 2024, the closing price of Santhera's common shares on the SIX Swiss Exchange was CHF 11.36 per share.

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 backbone 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 with dissociative properties in maintaining efficacy 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 the pivotal VISION-DMD study, vamorolone met the primary endpoint Time to Stand (TTSTAND) velocity versus placebo (p=0.002) at 24 weeks of treatment and showed a good safety and tolerability profile. The most commonly reported adverse events versus placebo from the VISION-DMD study were cushingoid features, vomiting, and vitamin D deficiency. Adverse events were generally of mild to moderate severity.

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. 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. As part of the previously described transaction, Santhera has transferred the approved New Drug Application to us. 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. On January 15, 2024, Santhera announced that AGAMREE® was commercially launched in Germany.

We currently expect to launch AGAMREE® in the U.S. during the first quarter of 2024. We are incurring substantial commercialization expenses, including sales, marketing, analytical infrastructure, patient services, patient advocacy, and other commercialization related expenses, in preparation for the launch of the product in the U.S. We incurred a portion of such commercialization expenses during the fourth quarter of 2023 and we are incurring additional expenses during the first quarter of 2024. We anticipate minimal sales and marketing personnel expansion to market AGAMREE®, with approximately 10 additional commercial team members required, due to the synergy of this product within our existing neuromuscular franchise. We will support 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.

We have established a joint steering committee with Santhera that will oversee the lifecycle management and development of AGAMREE® for additional indications beyond DMD. Under our AGAMREE® License Agreement with Santhera, we have agreed to purchase commercial supply of AGAMREE® from Santhera at agreed upon rates.

To support the approval of AGAMREE®, a randomized, double-blind, placebo and prednisone-controlled multinational trial of vamorolone was carried out in 121 patients with DMD. The study included patients ages four to less than seven years of age at time of enrollment in the study who were corticosteroid naïve and ambulatory. The trial met the primary (time to stand velocity after 24 weeks for vamorolone, 6 mg/kg per day vs placebo) and several 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 several 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.

AGAMREE® has New Chemical Entity exclusivity that expires in October 2028. AGAMREE® also enjoys Orphan Drug Exclusivity expiring in October 2030. AGAMREE® is further protected by six 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 could file 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 the ANDA until April 26, 2031.

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 products to treat rare (orphan) central nervous system (CNS) and adjacent rare (orphan) diseases. 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, no additional definitive agreements have been entered into to date, and there can be no assurance that these initiatives will be successful.

Capital Resources

At December 31, 2023, we had cash and cash equivalents of approximately $137.6 million. Further, on January 9, 2024, we completed a public offering of 10 million shares of our common stock, raising net proceeds of approximately $140.1 million. The proceeds from this offering will be used to potentially acquire new products and for general corporate purposes.

Based on our current financial condition 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 FYCOMPA®, that our commercialization of AGAMREE® will be successful, or that we will continue to be profitable and cash flow positive. Further, there can be no assurance that if we need additional funding in the future, whether such funding will be available to us on acceptable terms.

Basis of Presentation

Revenues.

During the fiscal year ended December 31, 2023, we generated revenues from product sales of FIRDAPSE® primarily in the U.S. and FYCOMPA® in the U.S. We expect these revenues to fluctuate in future periods based on our sales of FIRDAPSE® and FYCOMPA®. 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 Pharmaceuticals launched FIRDAPSE® in Canada. During the fiscal year ended December 31, 2023, revenues generated under our collaboration agreement with KYE Pharmaceuticals were immaterial. We expect our revenues from the KYE collaboration agreement to fluctuate in future periods based on our collaborator's ability to sell FIRDAPSE® in Canada.

For the fiscal year ended December 31, 2023, we did not generate revenues under our collaborative agreement with Endo. We do not expect to generate revenue in future periods as a result of Endo informing us in the fourth quarter of 2023 that they are discontinuing work on the collaboration for development and commercialization of vigabatrin and that they wish to terminate the arrangement.

For the fiscal year ended December 31, 2023, we generated $1.9 million in revenues from our collaborative agreement with DyDo. We expect our revenue from the DyDo license agreement to fluctuate in future periods based on DyDo's ability to meet various regulatory milestones set forth in such agreement.

Cost of Sales.

Cost of sales consists of third-party manufacturing costs, freight, royalties, 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 have been devoted to the development of FIRDAPSE®, CPP-109 (our version of vigabatrin), and formerly CPP-115, 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®, FYCOMPA® 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 actively committed 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 substantial commercialization expenses for AGAMREE®, as we prepare for the planned launch of AGAMREE® during the first quarter of 2024.

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, and the RUZURGI® product rights, which are amortized using the straight-line method over its estimated useful life of 14.5 years. We also capitalized the $36 million of milestone payment paid to Santhera during the fourth quarter of 2023 which is being amortized over the product's estimated useful life of 10.5 years.

Stock-Based Compensation.

We recognize expense for the fair value of all stock-based awards to employees, directors, and consultants in accordance with accounting principles generally accepted in the U.S. (U.S. GAAP). For stock options, we use the Black-Scholes option valuation model in calculating the fair value of the awards.

Income Taxes.

Our effective income tax rate is the ratio of income tax expense over our income before income taxes.

As of December 31, 2023, 2022 and 2021, we had no federal net operating loss carry-forwards. Additionally, we had state net operating loss carry-forwards of approximately $0, $0 and $28 million, respectively, available to reduce future Florida taxable income for the years ended December 31, 2023, 2022 and 2021.

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. 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, leases, preclinical study and clinical trial expenses, 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, 2023, 2022 and 2021 and, therefore, the transaction price was not reduced further during the years ended December 31, 2023, 2022 and 2021. 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, 2023 and 2022, the assumptions used were an estimated annual volatility of 68.0% to 71.0% and 68.4% to

69.5%, expected holding periods of 4.5 to 5.2 years and 4.5 years, and risk-free interest rates of 3.55% to 4.92% and 1.27% to 4.07%, respectively.

Results of Operations

Years Ended December 31, 2023 and 2022

Revenues.

For the fiscal year ended December 31, 2023, we recognized total revenues of $398.2 million which included $396.5 million in net revenue from product sales primarily in the U.S. compared to $214.2 million in total revenues, which included $213.9 million in net revenues from product sales for the fiscal year ended December 31, 2022. FIRDAPSE® net sales were approximately $258.4 million for the fiscal year ended December 31, 2023 and FYCOMPA® net sales were approximately $138.1 million for the period between January 24, 2023 (date of acquisition) and December 31, 2023. All net revenue from product sales during the fiscal year ended December 31, 2022 were from sales of FIRDAPSE®.

The increase of approximately $182.6 million in net product revenues when comparing the fiscal year ended December 31, 2023 and 2022 was primarily due to the acquisition of FYCOMPA® during January 2023, and related product sales, and increases in FIRDAPSE® sales volumes of approximately 12% (which included patients who were transferred to FIRDAPSE® in the first and second quarter of 2022 when RUZURGI® was removed from the market) and net price increases. Net revenues from product sales of FIRDAPSE® increased by 20.8% from 2022 to 2023.

Product revenue for FYCOMPA® in 2024 will be affected by differences in variable consideration (gross-to-net) compared to 2023, when revenues were booked under Eisai's cost arrangements with distributors and government authorities. Starting on January 1, 2024, all such costs are tied to arrangements between us and those distributors and government agencies, which costs are likely to be higher than Eisai's costs, thereby increasing the gross-to-net deductions for FYCOMPA® and correspondingly decreasing FYCOMPA® net product revenue.

For the fiscal year ended December 31, 2023, we also recognized $1.7 million in license and other revenue, as compared to $0.3 million during the fiscal year ended December 31, 2022. The increase was primarily due to the milestone 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 $52.0 million for the fiscal year ended December 31, 2023, compared to $34.4 million for the fiscal year ended December 31, 2022. 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 2023 consisted of product costs and excludes the amortization of the FYCOMPA® intangible assets. 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 $32.6 million for the fiscal year ended December 31, 2023 compared to $1.1 million for fiscal year ended December 31, 2022. 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, and the RUZURGI® rights, which are amortized using the straight-line method over its estimated useful life of 14.5 years. We also capitalized a $36 million milestone payment paid to Santhera during the fourth quarter of 2023 which is being amortized over the product's estimated useful life of 10.5 years.

Research and Development Expenses.

Research and development expenses for the years ended December 31, 2023 and 2022 were approximately $93.2 million and $19.8 million, respectively, and represented approximately 30% and 18% of total operating costs and expenses, respectively. Research and development expenses for the years ended December 31, 2023 and 2022 were as follows (in thousands):

	For the year ended December 31,		Change	
	2023	2022	$	%
Research and development expenses	$ 10,156	$ 18,060	(7,904)	(43.8)
Acquired in-process research and development	81,513	—	81,513	—
Employee stock-based compensation	1,481	1,729	(248)	(14.3)
Total research and development expenses	$ 93,150	$ 19,789	73,361	370.7

Research and development expenses increased approximately $73.4 million during year ended December 31, 2023 when compared to the same period in 2022. The increase 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. This was partially offset by decreases in costs relating to closing out sites for both our MuSK-MG clinical trial and our previously operated expanded access program.

We expect that research and development expenses will continue to be significant in 2024 and beyond as we execute on our strategic initiative and portfolio expansion efforts, with a keen focus on products to treat rare and difficult to treat diseases.

Selling, General and Administrative Expenses.

Selling, general and administrative expenses for the years ended December 31, 2023 and 2022 were approximately $133.7 million and $57.1 million, respectively, and represented approximately 43% and 51% of total operating costs and expenses for the years ended December 31, 2023, and 2022, respectively. Selling, general and administrative expenses for the years ended December 31, 2023 and 2022 were as follows (in thousands):

	For the year ended December 31,		Change	
	2023	2022	$	%
Selling	$ 86,689	$ 29,469	57,220	194.2
General and administrative	34,252	21,438	12,814	59.8
Employee stock-based compensation	12,769	6,178	6,591	106.7
Total selling, general and administrative expenses	$ 133,710	$ 57,085	76,625	134.2

For the year ended December 31, 2023, selling, general and administrative expenses increased approximately $76.6 million when compared to the same period in 2022. The increase was primarily attributable to the direct fees payable under the TSA associated with net product sales of FYCOMPA® of approximately $15.7 million, increases of approximately $22.5 million in selling (commercialization) expenses due to the acquisition of FYCOMPA®, which consist primarily of commercial systems implementation costs, hiring of the sales force and supporting personnel and an approximately $22.2 million increase in employee compensation and stock based compensation related to the increase in headcount resulting from the acquisitions of FYCOMPA® and AGAMREE®, and annual merit increases. SG&A excludes the amortization of the RUZURGI® intangible asset. See Note 9 of the Notes to Consolidated Financial Statements included elsewhere in this report.

We expect that selling, general and administrative expenses will continue to be substantial in future periods as we continue our efforts to increase our revenues from FIRDAPSE®, continue our efforts to market FYCOMPA®, take steps to prepare for the commercial launch of AGAMREE® in 2024, and take steps to continue to expand our business.

Stock-Based Compensation.

Total stock-based compensation for the years ended December 31, 2023 and 2022 was $14.3 million and $7.9 million, respectively. In 2023 and 2022, grants were principally for stock options relating to year-end bonus awards and grants to new employees.

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 $7.7 million and $2.9 million for the fiscal years ended December 31, 2023 and 2022, respectively. The increase in other income, net for the fiscal year ended December 31, 2023 of approximately $4.8 million when compared to the same period in 2022 is primarily due to higher yields on investments offset by $2.0 million of accretion expense related to payments arising from our acquisition of RUZURGI®.

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,	
	2023	2022
Interest income (expense), net	$ 4,675	$ 3,550
Dividend income	—	93
Realized gains (losses) from the sale of available-for-sale securities	—	(762)
Net gains (losses) recognized during the period on equity securities	3,024	—
Total other income, net	$ 7,699	$ 2,881

Income Taxes.

Our effective income tax rate was 24.4% and 20.7% for fiscal years ended December 31, 2023 and 2022, respectively. Differences in our effective tax and the statutory federal income tax rate 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 in future periods than it was in the 2023 fiscal year).

We had no uncertain tax positions as of December 31, 2023 and 2022.

Net Income.

Our net income was approximately $71.4 million in the year ended December 31, 2023 ($0.67 per basic and $0.63 per diluted share) as compared to $83.1 million in the year ended December 31, 2022 ($0.80 per basic and $0.75 per diluted share).

Years Ended December 31, 2022 and 2021

The information comparing results of operations for the year ended 2022 compared to 2021 was included in our Annual Report on Form 10-K for 2022 filed with the SEC on March 15, 2023.

Liquidity and Capital Resources

Since our inception, we have financed our operations primarily through multiple offerings of our securities and revenues from product sales. At December 31, 2023 we had cash and cash equivalents aggregating $137.6 million and working capital of $143.3 million. At December 31, 2022, we had cash and cash equivalents aggregating $298.4 million and working capital of $263.2 million. At December 31, 2023, substantially all of our cash and cash equivalents were deposited with one financial institution, 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.

Based on forecasts of available cash, we believe that we have sufficient resources to support our currently anticipated 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 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.

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 $140.1 million.

Cash Flows.

Net cash provided by operating activities was $143.6 million and $116.0 million, respectively, for the years ended December 31, 2023 and 2022. 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 and $45.8 million in non-cash expenses. 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 and $17.8 million in deferred taxes. During the year ended December 31, 2022, net cash provided by operating activities was primarily attributable to our net income of $83.1 million, a decrease of $1.1 million in inventory, increases of $1.2 million in accounts payable and $16.4 million in accrued expenses and other liabilities, $4.9 million in deferred taxes, $4.1 million in acquired research and development inventory expensed from asset acquisition and of $10.2 million of non-cash expenses. This was partially offset by increases of $3.8 million in accounts receivable, net and $0.8 million in prepaid expenses and other current assets and a decrease of $0.3 million in operating lease liability.

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 investing activities during the year ended December 31, 2022 was $9.2 million and consisted primarily of proceeds from the sale of available-for-sale securities of $19.2 million, offset partially by payments in connection with asset acquisitions of $10.0 million.

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. Net cash provided by financing activities during the year ended December 31, 2022 was $1.7 million, consisting primarily of proceeds from the exercise of stock options, partially offset by repurchases of common stock.

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 and the third-party licensor) in any calendar year for the duration of regulatory exclusivity within a territory and 3.5% for territories in any calendar year in territories without regulatory exclusivity.

For the year ended December 31, 2023, we recognized an aggregate of approximately $39.5 million 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, if DyDo is successful in obtaining the right to commercialize FIRDAPSE® in Japan, 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 the remaining $10 million will be paid on the second anniversary of closing;
  - 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 United States 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 United States, 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; and
  - If Catalyst were to receive a priority review voucher for FIRDAPSE® or RUZURGI® in the future, 50% of the consideration paid by a third-party to acquire that voucher will be paid to Jacobus.

  For the year ended December 31, 2023, we recognized an aggregate of approximately $3.7 million 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 paid at closing a $160 million upfront cash payment, plus $1.6 million for reimbursement of certain prepayments. Eisai was also eligible to receive a contingent payment of $25 million if a patent term extension for FYCOMPA® was approved until June 8, 2026 by the USPTO, which request for reconsideration of the patent term extension was denied by the USPTO in June 2023;
  - 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 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 sales-based milestone payments if the applicable amount of net sales of all products in the territory in a single calendar year reach one of 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, we 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 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®.

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 2024.
- *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 the successful launch and commercialization of 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® and FYCOMPA® while maintaining full compliance with applicable federal and state laws, rules and regulations;
- 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 our supplemental New Drug Application (sNDA) seeking to increase the maximum daily dosage of FIRDAPSE® from 80 mg to 100 mg will be approved by the U.S. Food and Drug Administration (FDA);
- Whether the daily dose of FIRDAPSE® taken by patients changes over time and affects our results of operations;
- Whether we will be able to locate LEMS patients who are undiagnosed or are misdiagnosed with other diseases;
- Whether patients will discontinue from the use of FIRDAPSE® and FYCOMPA® at rates that are higher than historically experienced or are higher than we project;
- Whether new FIRDAPSE® patients and new FYCOMPA® patients can be successfully titrated to stable therapy;
- Whether we can continue to market FIRDAPSE® and FYCOMPA® on a profitable and cash flow positive basis;
- Whether we will successfully launch AGAMREE® in the first quarter of 2024 as we currently plan;

- Whether we will be able to successfully commercialize AGAMREE® in the territory;
- Whether we will be able to demonstrate, to the satisfaction of the FDA and third-party payors, whether AGAMREE® offers advantages compared to corticosteroids or competitor's products;
- Whether the acquisition of AGAMREE® will prove to be accretive to EBITDA and EPS in 2024 and beyond;
- Whether any revenue or earnings guidance that we provide to the public market will turn out to be accurate;
- Whether payors will 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 supply sufficient product to meet our customers' needs in future periods;
- 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 any 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 our patents will be sufficient to prevent generic competition for FIRDAPSE® and AGAMREE® after our orphan drug exclusivity for each product expires;
- 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® and FYCOMPA®;
- 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, or changes in the healthcare industry generally;
- Whether we and Santhera Pharmaceuticals 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 our clinical trials and studies, pre-clinical studies, proof-of-concept studies, and our other drug development activities, and whether our trials and studies 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, our collaboration partner in Canada;
- The impact on sales of FIRDAPSE® in the United States if an amifampridine product is purchased in Canada for use in the United States;
- Whether DyDo will be able to obtain approval to commercialize FIRDAPSE® in Japan; and
- Whether our plans to expand the reach of FIRDAPSE® and AGAMREE® into other global regions will be successful.

Our current plans and objectives are based on assumptions relating to the continued commercialization of FIRDAPSE® and FYCOMPA®, the commercialization of 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 short-term investments 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 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, 2023, 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, 2023 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, 2023.

During the fourth quarter of 2023, 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.

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 2024 Annual Meeting of Stockholders. Our Proxy Statement for the 2024 Annual Meeting of Stockholders is expected to be filed not later than 120 days after the end of our fiscal year ended December 31, 2023 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 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, 2023 and 2022.

Consolidated Statements of Operations and Comprehensive Income for the years ended December 31, 2023, 2022 and 2021.

Consolidated Statements of Changes in Stockholders' Equity for the years ended December 31, 2023, 2022 and 2021.

Consolidated Statements of Cash Flows for the years ended December 31, 2023, 2022 and 2021.

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 *					X
10.1(b)+	Offer Letter between the Company and Michael W. Kalb*					X
10.1(c)+	Separation Agreement between the Company and Patrick J. McEnany*					X
10.1(d)+	Separation Agreement between the Company and Alicia Grande *					X
10.2(a)+	2014 Stock Incentive Plan	DEF 14A	001-33057	3/19/2014	Annex A	
10.2(b)+	Amendment No. 1 to 2014 Stock Incentive Plan	DEF 14A	001-33057	4/29/2016	Annex A	
10.2(c)+	Amendment No. 2 to 2014 Stock Incentive Plan	DEF 14A	001-33057	4/14/2017	Annex A	
10.3(a)+	2018 Stock Incentive Plan	DEF 14A	001-33057	4/17/2018	Annex A	
10.3(b)+	Amendment No. 1 to 2018 Stock Incentive Plan	DEF 14A	001-33057	7/7/2020	Annex A	
10.3(c)+	Amendment No. 2 to 2018 Stock Incentive Plan	DEF 14A	001-33057	10/25/2021	Annex A	

Exhibit Number	Description of Exhibit	Incorporated by Reference				Filed Herewith
		Form	File Number	Date of Filing	Exhibit Number	
10.3(d)+	Amendment No. 3 to 2018 Stock Incentive Plan	DEF 14A	001-33057	7/12/2023	Annex A	
10.4+	Severance and Change in Control Plan					X
10.5(a)	Lease Agreement between the Company and 355 Alhambra Plaza, Ltd.	10-Q	001-33057	5/14/2007	10.1	
10.5(b)	First Amendment to Lease Agreement between the Company and CPT 355 Alhambra Circle, LLC	10-Q	001-33057	8/15/2011	10.1	
10.5(c)	Second Amendment to Lease Agreement between the Company and CPT 355 Alhambra Circle, LLC	8-K	001-33057	2/20/2014	10.1	
10.5(d)	Third Amendment to Lease Agreement between the Company and CPT 355 Alhambra Circle, LLC	8-K	001-33057	3/27/2015	10.1	
10.5(e)	Fourth Amendment to Lease Agreement between the Company and PRII 355 Alhambra Circle, LLC	8-K	001-33057	8/17/2018	10.1	
10.5(f)	Fifth Amendment to Lease Agreement between the Company and PRII 355 Alhambra Circle, LLC	8-K	001-33057	5/13/2020	10.1	
10.6	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	
10.7(a)	License Agreement, dated as of October 26, 2012, between the Company and BioMarin	8-K	001-33057	10/31/2012	10.2	
10.7(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	
10.7(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 Pharmaceuticals, Inc., (ii) BioMarin, and (iii) the Company	10-Q	001-33057	8/17/2018	10.1	
10.7(d)	Second Amendment to License Agreement, dated May 29, 2019, between the Company and BioMarin	8-K	001-33057	5/30/2019	10.1	
10.8	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	
10.9	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	
10.10	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	
10.11(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	
10.11(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	
10.12(a)	Transition Services Agreement between Eisai, Inc. and the Company	8-K	001-33057	12/22/2022	10.1	
10.12(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	
10.12(c)	Supply Agreement between Eisai Co., Ltd. and the Company	8-K	001-33057	12/22/2022	10.2	

Exhibit Number	Description of Exhibit	Incorporated by Reference				Filed Herewith
		Form	File Number	Date of Filing	Exhibit Number	
10.13(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	
10.13(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	
21.1	Subsidiaries of the Registrant	10-K	001-33057	3/16/2020	21.1	
23.1	Consent of Independent Registered Public Accounting Firm					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					X
101.INS	XBRL Instance Document					
101.SCH	XBRL Taxonomy Extension Schema					
101.CAL	XBRL Taxonomy Extension Calculation Linkbase					
101.DEF	XBRL Taxonomy Extension Definition Linkbase					
101.LAB	XBRL Taxonomy Extension Label Linkbase					
101.PRE	XBRL Taxonomy Extension Presentation Linkbase					
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 28th day of February, 2024.

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 28, 2024
/s/ Michael W. Kalb Michael W. Kalb	Executive Vice President and Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	February 28, 2024
/s/ Patrick J. McEnany Patrick J. McEnany	Chairman of the Board of Directors	February 28, 2024
/s/ Charles B. O'Keeffe Charles B. O'Keeffe	Director	February 28, 2024
/s/ David S. Tierney, M.D. David S. Tierney, M.D.	Director	February 28, 2024
/s/ Donald A. Denkhaus Donald A. Denkhaus	Director	February 28, 2024
/s/ Molly Harper Molly Harper	Director	February 28, 2024
/s/ Tamar Thompson Tamar Thompson	Director	February 28, 2024

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Years ended December 31, 2023, 2022 and 2021

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, 2023, 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, 2023, 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, 2023, and our report dated February 28, 2024 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 28, 2024

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, 2023 and 2022, the related consolidated statements of comprehensive income, changes in shareholders' equity, and cash flows for each of the three years in the period ended December 31, 2023, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2023 and 2022, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2023, in conformity with accounting principles generally accepted in the United States of America.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2023, based on criteria established in the 2013 *Internal Control—Integrated Framework* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO), and our report dated February 28, 2024 expressed an unqualified opinion.

Basis for opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.
We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

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, analytically evaluating management's estimates, evaluating evidence contrary to the estimated amounts, performing a sensitivity analysis on the rebate amount and payor mix used in the estimates, and by performing a comparison of actual rebate claims received against the amounts recorded by management.

GRANT THORNTON LLP

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

Miami, Florida
February 28, 2024

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

	December 31, 2023	December 31, 2022
ASSETS		
Current Assets:		
Cash and cash equivalents	$ 137,636	$ 298,395
Accounts receivable, net	53,514	10,439
Inventory	15,644	6,805
Prepaid expenses and other current assets	12,535	5,167
Total current assets	219,329	320,806
Operating lease right-of-use asset	2,508	2,770
Property and equipment, net	1,195	847
License and acquired intangibles, net	194,049	32,471
Deferred tax assets, net	36,544	18,736
Investment in equity securities	16,489	—
Total assets	$ 470,114	$ 375,630
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current Liabilities:		
Accounts payable	$ 14,795	$ 3,975
Accrued expenses and other liabilities	61,268	53,613
Total current liabilities	76,063	57,588
Operating lease liability, net of current portion	3,188	3,557
Other non-current liabilities	2,982	14,064
Total liabilities	82,233	75,209
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, 2023 and 2022	—	—
Common stock, $0.001 par value, 200,000,000 shares authorized; 107,121,549 shares and 105,263,031 shares issued and outstanding at December 31, 2023 and 2022, respectively	107	105
Additional paid-in capital	266,488	250,430
Retained earnings	121,272	49,862
Accumulated other comprehensive income (Note 4)	14	24
Total stockholders' equity	387,881	300,421
Total liabilities and stockholders' equity	$ 470,114	$ 375,630

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 data)

	Year Ended December 31,		
	2023	**2022**	**2021**
Revenues:			
Product revenue, net	$ 396,502	$ 213,938	$ 137,997
License and other revenue	1,702	265	2,836
Total revenues	398,204	214,203	140,833
Operating costs and expenses:			
Cost of sales (a)	51,967	34,393	21,884
Research and development	93,150	19,789	16,936
Selling, general and administrative (a)	133,710	57,085	49,628
Amortization of intangible assets	32,565	1,098	—
Total operating costs and expenses	311,392	112,365	88,448
Operating income	86,812	101,838	52,385
Other income, net	7,699	2,881	282
Net income before income taxes	94,511	104,719	52,667
Income tax provision	23,101	21,640	13,185
Net income	$ 71,410	$ 83,079	$ 39,482
Net income per share:			
Basic	$ 0.67	$ 0.80	$ 0.38
Diluted	$ 0.63	$ 0.75	$ 0.37
Weighted average shares outstanding:			
Basic	106,279,736	103,374,606	103,379,349
Diluted	113,753,154	111,375,631	107,795,585
Net income	$ 71,410	$ 83,079	$ 39,482
Other comprehensive income (Note 4):			
Unrealized gain (loss) on available-for-sale securities, net of tax of $4, ($54) and $46, respectively	(10)	172	(179)
Comprehensive income	$ 71,400	$ 83,251	$ 39,303

(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, 2023, 2022 and 2021
(in thousands)

	Preferred Stock	Common Stock		Additional Paid-in Capital	Retained Earnings (Accumulated Deficit)	Accumulated Other Comprehensive Gain (Loss)	Total
		Shares	Amount				
Balance at December 31, 2020	$ —	103,782	$ 104	$223,168	$ (53,705)	$ 31	$169,598
Stock-based compensation	—	—	—	6,073	—	—	6,073
Exercise of stock options for common stock	—	1,328	1	4,098	—	—	4,099
Issuance of common stock upon vesting of restricted stock units, net	—	91	—	(153)	—	—	(153)
Repurchase of common stock	—	(2,208)	(2)	—	(12,087)	—	(12,089)
Other comprehensive gain (loss)	—	—	—	—	—	(179)	(179)
Net income	—	—	—	—	39,482	—	39,482
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

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

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

	Year Ended December 31,		
	2023	2022	2021
Operating Activities:			
Net income	$ 71,410	$ 83,079	$ 39,482
Adjustments to reconcile net income to net cash provided by (used in) operating activities:			
Depreciation	316	141	192
Stock-based compensation	14,250	7,907	6,073
Amortization of intangible assets	32,565	1,098	—
Deferred taxes	(17,818)	4,937	9,316
Accretion of discount	1,320	17	(5)
Reduction in the carrying amount of right-of-use asset	262	247	292
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	(3,024)	—	—
(Increase) decrease in:			
Accounts receivable, net	(43,075)	(3,820)	(632)
Inventory	(4,739)	1,065	(3,219)
Prepaid expenses and other current assets	(5,792)	(807)	3,977
Increase (decrease) in:			
Accounts payable	10,820	1,207	(1,488)
Accrued expenses and other liabilities	5,800	16,391	5,520
Operating lease liability	(338)	(307)	864
Net cash provided by (used in) operating activities	143,600	116,047	60,372
Investing Activities:			
Purchases of property and equipment	(231)	(29)	(1,021)
Purchases of investments	—	—	(10,000)
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	(293,502)	9,209	(11,021)
Financing Activities:			
Payment of employee withholding tax related to stock-based compensation	(982)	(230)	(153)
Proceeds from exercise of stock options	2,792	9,569	4,099
Repurchase of common stock	—	(6,907)	(12,089)
Payment of liabilities arising from asset acquisition	(12,667)	(738)	—
Net cash provided by (used in) financing activities	(10,857)	1,694	(8,143)
Net increase (decrease) in cash and cash equivalents	(160,759)	126,950	41,208
Cash and cash equivalents – beginning of period	298,395	171,445	130,237
Cash and cash equivalents – end of period	$ 137,636	$ 298,395	$ 171,445
Supplemental disclosures of cash flow information:			
Cash paid for income taxes	$ 50,458	$ 7,667	$ 3,000
Cash paid for interest	$ 705	$ —	$ —
Non-cash investing and financing activities:			
Operating lease liabilities arising from obtaining right-of-use assets	$ —	$ —	$ 3,309
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 utilizes concerted diligence efforts in search of therapies that will improve the lives of those who suffer from rare or difficult to treat diseases. With an unwavering patient focus embedded in everything the Company does, it 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 United States 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. 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).

On December 17, 2022, the Company entered into an asset purchase agreement with Eisai Co., Ltd. (Eisai) for the acquisition of the United States 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 FYCOMPA® on January 24, 2023 and the Company began marketing FYCOMPA® in the United States.

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 with 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 Europe and Japan should Santhera pursue partnership opportunities in those jurisdictions. Additionally, the Company will hold North American rights for any future approved indications of AGAMREE®. AGAMREE® has previously received FDA Orphan Drug and Fast Track designations. On October 26, 2023, AGAMREE® was approved by the FDA for commercialization in the U.S.

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

Capital Resources

Based on forecasts of available cash, the Company believes that it has sufficient resources to support the currently anticipated operations for at least the next 12 months from the date of this report.

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. Further, to the extent that the Company raises additional funds through collaborative arrangements, it may be necessary to relinquish some rights to the Company's drug candidates or grant sublicenses on terms that are not favorable to the Company. If the Company is not able to secure additional funding when needed, the Company may have to delay, reduce the scope of, or eliminate one or more research and development programs, which could have an adverse effect on the Company's business. Subsequent to year-end, on January 9, 2024, the Company completed a public offering of 10 million shares of its common stock, raising net proceeds of approximately $140.1 million. The proceeds of the offering will be used to potentially acquire new products and for general corporate purposes. See Note 18 (Subsequent Events).

Risks and Uncertainties

The Company is subject to risks and adversities that could affect its business in unforeseen ways.

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. 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 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 substantially all its cash and cash equivalents deposited with one financial institution. These amounts exceed federally insured limits.

d. INVESTMENTS. The Company invests in high credit-quality instruments in order to obtain higher yields on its cash available for investments. At December 31, 2023, investments consisted of U.S. Treasuries and an investment in equity securities. At December 31, 2022, investments consisted of U.S. Treasuries. Such investments are not insured by the Federal Deposit Insurance Corporation.

The U.S. Treasuries held at December 31, 2023 and December 31, 2022 are 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.

There are no short-term investments as of December 31, 2023 and December 31, 2022.

The Company records available-for-sale securities at fair value with unrealized gains and losses reported in accumulated other comprehensive income (loss) (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 post reverse-split ordinary shares (representing approximately 11.26% of Santhera's outstanding ordinary shares 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 statement of operations and comprehensive income.

e. ACCOUNTS RECEIVABLE, NET. Accounts receivable is recorded net of customer allowance 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. At December 31, 2023 and December 31, 2022, the Company determined that an allowance for expected credit losses was not required. No accounts 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.

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

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 is identical and, as a result, the inventory has 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 consists 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.

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 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 is met, the transaction is accounted for as an asset acquisition. If the screen 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 on the Company's exclusive license agreement with Jacobus Pharmaceutical Company, Inc. (Jacobus), for the rights to develop and commercialize RUZURGI® in the United States 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 Co., Ltd, 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 accrued expenses and other liabilities. At December 31, 2023 and December 31, 2022, the fair value of these instruments approximated their carrying value.

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

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.

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, 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	$ —	$ —

	Balances as of December 31, 2022	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	$ 168,853	$ 168,853	$ —	$ —
U.S. Treasuries	$ 105,442	$ 105,442	$ —	$ —

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, 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.

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

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.

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 customer for FIRDAPSE® and its customers for FYCOMPA® 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®, subsequent to receiving FDA approval, the Company entered into an arrangement with one distributor (the Customer), which is the exclusive distributor of FIRDAPSE® in the United States. The Customer subsequently resells FIRDAPSE® 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®. During 2023, the Company sold FYCOMPA®, through a Transition Service Agreement with Eisai to major wholesalers, specialty pharmaceutical distributors, managed care organizations, and government agencies. The transition services under the Transition Services Agreement ended on December 31, 2023.

Product Revenue, Net: The Company recognizes revenue on product sales when the customer obtains control of the Company's product, which occurs 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 30 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, 2023, 2022 and 2021.

During the years ended December 31, 2023, 2022 and 2021, substantially all of the Company's product revenues were from sales to customers in the United States.

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

	For the Years Ended December 31,		
	2023	2022	2021
FIRDAPSE®	$ 258,426	$ 213,938	$ 137,997
FYCOMPA®	138,076	—	—
Total product revenue, net	$ 396,502	$ 213,938	$ 137,997

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

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, 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, 2023 and, therefore, the transaction price was not reduced further during the years ended December 31, 2023, 2022 and 2021. 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. In addition, the Company receives sales order management, transactional data and distribution services from the Customer and Eisai. 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, 2023, 2022 and 2021, 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.

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 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 FIRDAPSE® 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.

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

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.

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 FIRDAPSE®, 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.

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.

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® 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® while the Company is determining the patient's third-party insurance, prescription drug benefit or other third-party coverage for FIRDAPSE®. The Patient Assistance Program provides FIRDAPSE® or FYCOMPA® free of charge for longer periods of time for those who are uninsured or functionally uninsured with respect to FIRDAPSE® or FYCOMPA® 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.

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

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.

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.

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 later 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.

Payments to and from the collaborator are presented in the statement 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.

Refer to 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.

q. ADVERTISING EXPENSE. Advertising costs are expensed as incurred. The Company incurred approximately $9.1 million, $3.3 million and $2.9 million in advertising costs during the years ended December 31, 2023, 2022 and 2021, respectively, which are included in selling, general and administrative expenses in the Company's consolidated statements of operations and comprehensive income.

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

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.

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 product, FIRDAPSE®, in the United States 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 this product. The Company sells its product, FYCOMPA® through a Transition Service Agreement with Eisai to major wholesalers, specialty pharmaceutical distributors, managed care organizations, and government agencies. Therefore, Eisai accounts for principally all of its trade receivables and net product revenues related to this product. 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 credit worthiness of its customers, historical payment patterns, aging of receivable balances and general economic conditions.

As of December 31, 2023, 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 FIRDAPSE® and the recently acquired products 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 FIRDAPSE®, FYCOMPA®, AGAMREE® and any future drug candidates. The commercialization of FIRDAPSE®, FYCOMPA®, AGAMREE® 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®. It also relies on Eisai as its sole source of supply for FYCOMPA® and on Santhera 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.

t. ROYALTIES. Royalties incurred in connection with the Company's license agreement for FIRDAPSE®, 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.

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.

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

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 2020. 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, 2023, 2022 and 2021, 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,		
	2023	2022	2021
Basic weighted average common shares outstanding	106,279,736	103,374,606	103,379,349
Effect of dilutive securities	7,473,418	8,001,025	4,416,236
Diluted weighted average common shares outstanding	113,753,154	111,375,631	107,795,585

Outstanding common stock equivalents totaling approximately 4.5 million, 1.0 million and 4.3 million, were excluded from the calculation of diluted net income per common share for the years ended December 31, 2023, 2022 and 2021, respectively, as their effect would be anti-dilutive. Potentially dilutive options to purchase common stock as of December 31, 2023, 2022 and 2021 had exercise prices ranging from $0.79 to $7.10, $0.79 to $7.07 and $0.79 to $4.64, respectively.

x. SEGMENT INFORMATION. Management has determined that the Company operates in one reportable segment, which is the development and commercialization of drug products.

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. The Company did not adopt any accounting standards during the year ended December 31, 2023.

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 has one reportable segment and is evaluating the impact of the standard on the Company's consolidated financial statements.

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

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.

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, 2023:				
U.S. Treasuries - Cash equivalents	$ 94,523	$ 18	$ —	$ 94,505
Total	$ 94,523	$ 18	$ —	$ 94,505
At December 31, 2022:				
U.S. Treasuries - Cash equivalents	$ 105,442	$ 32	$ —	$ 105,410
Total	$ 105,442	$ 32	$ —	$ 105,410

There were no realized gains or losses from available-for-sale securities for the years ended December 31, 2023 or 2021. 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, 2023, by contractual maturity, are summarized as follows (in thousands):

	2023
Due in one year or less	$ 94,523

	For the Years Ended December 31,		
	2023	2022	2021
Equity securities:			
Net gains (losses) recognized during the period on equity securities	$ 3,024	$ —	$ —
Unrealized net gains (losses) recognized during the period on equity securities still held at the reporting date	$ 3,024	$ —	$ —

There were no sales of equity securities during the years ended December 31, 2023, 2022 and 2021. Unrealized net gains (losses) recognized during the periods on equity securities are included in other income, net in the consolidated statements of operations.

4. Accumulated Other Comprehensive Income (loss).

The following table summarizes the changes in accumulated other comprehensive income (loss), net of tax from unrealized gains (losses) on available-for-sale securities, the Company's only component of accumulated other comprehensive income (loss) for the years ended December 31, 2023, 2022 and 2021.

The amount reclassified out of accumulated other comprehensive income (loss), 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 (loss) during the years ended December 31, 2023 or 2021.

4. Accumulated Other Comprehensive Income (loss) (continued).

	Total Accumulated Other Comprehensive Income (Loss)
Balance at December 31, 2021	$ (148)
Other comprehensive gain (loss) before reclassifications	(590)
Amount reclassified from accumulated other comprehensive income (loss)	762
Net current period other comprehensive gain	172
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

5. Inventory.

Inventory consists of the following (in thousands):

	December 31, 2023	December 31, 2022
Raw materials	$ 1,910	$ —
Work-in-process	4,573	5,543
Finished goods	9,161	1,262
Total inventory	$ 15,644	$ 6,805

6. Prepaid Expenses and Other Current Assets.

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

	December 31, 2023	December 31, 2022
Prepaid manufacturing costs	$ 2,005	$ 1,147
Prepaid tax	1,238	44
Prepaid insurance	1,332	1,224
Prepaid subscriptions fees	1,299	808
Prepaid research fees	1,500	178
Prepaid commercialization expenses	3,038	592
Due from collaborative and licensing arrangements	138	354
Prepaid conference and travel expenses	771	234
Prepaid co-pay assistance program	863	97
Other	351	489
Total prepaid expenses and other current assets	$ 12,535	$ 5,167

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. There are 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.

7. Operating Leases (continued).

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

	For the Years Ended December 31,	
	2023	2022
Operating lease cost	$ 431	$ 431

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

	For the Years Ended December 31,	
	2023	2022
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows	$ 506	$ 492
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, 2023	December 31, 2022
Operating lease right-of-use assets	$ 2,508	$ 2,770
Other current liabilities	$ 369	$ 337
Operating lease liabilities, net of current portion	3,188	3,557
Total operating lease liabilities	$ 3,557	$ 3,894

As of December 31, 2023 and December 31, 2022, the weighted average remaining lease term was 7.3 years and 8.3 years, respectively. The weighted average discount rate used to determine the operating lease liabilities was 4.51% as of December 31, 2023 and 2022.

Remaining payments of lease liabilities as of December 31, 2023 were as follows (in thousands):

2024	$ 522
2025	537
2026	553
2027	570
2028	587
Thereafter	1,440
Total lease payments	4,209
Less: imputed interest	(652)
Total	$ 3,557

Rent expense was $0.4 million for the years ended December 31, 2023, 2022 and 2021.

8. Property and Equipment, Net.

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

	December 31, 2023	December 31, 2022
Furniture and equipment	$ 494	$ 273
Leasehold improvements	991	980
Software	433	—
Less: Accumulated depreciation	(723)	(406)
Total property and equipment, net	$ 1,195	$ 847

9. License and Acquired Intangibles, Net.

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 following table presents the Company's intangible assets at December 31, 2022 (in thousands):

	Gross Carrying Value	Accumulated Amortization	Net Carrying Value
Intangible assets:			
License and acquired intangibles for RUZURGI®	$ 33,569	$ 1,098	$ 32,471
Total	$ 33,569	$ 1,098	$ 32,471

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 in amortization expense related to the licensed and acquired intangibles for RUZURGI® during the year ended December 31, 2023, within selling, general and administrative expenses in the consolidated statements of operations and comprehensive income. The Company recorded approximately $29.7 million in amortization expense related to the licensed and acquired intangibles for FYCOMPA® during the year ended December 31, 2023, within cost of sales in the consolidated statement of operations and comprehensive income. The Company recorded approximately $0.6 million in amortization expense related to the licensed and acquired intangibles for AGAMREE® during the year ended December 31, 2023, within cost of sales in the consolidated statement of operations and comprehensive income. The Company recorded approximately $1.1 million in amortization expense related to the licensed and acquired intangibles for RUZURGI® during the year ended December 31, 2022, within selling, general and administrative expenses in the consolidated statements of operations and comprehensive income. No amortization expense was recorded during the year ended December 31, 2021. Amortization of the FYCOMPA®, RUZURGI® and AGAMREE® intangible assets are reported together as amortization of intangible assets 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):

2024	$ 37,378
2025	37,378
2026	37,378
2027	37,378
2028	7,705
Thereafter	36,832
Total	$ 194,049

At December 31, 2023 and December 31, 2022, the weighted average amortization period remaining for intangible assets was 6.5 years and 14.0 years, respectively.

If all or a portion of the intangible assets are deemed not recoverable, the Company would estimate the fair value of the assets and record an impairment loss. There were no impairment charges recognized on definite-lived intangibles for the years ended December 31, 2023, 2022 or 2021.

10. Accrued Expenses and Other Liabilities.

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

	2023	2022
Accrued preclinical and clinical trial expenses	$ 1,015	$ 479
Accrued professional fees	4,730	1,619
Accrued compensation and benefits	8,883	5,132
Accrued license fees	24,437	20,444
Accrued purchases	192	154
Operating lease liability	369	337
Accrued variable consideration	6,877	3,381
Accrued income tax	729	8,702
Due to licensor	12,540	13,127
Accrued interest payable	1,031	—
Other	465	238
Current accrued expenses and other liabilities	61,268	53,613
Lease liability – non-current	3,188	3,557
Due to licensor – non-current	2,497	14,064
Other – non-current	485	—
Non-current accrued expenses and other liabilities	6,170	17,621
Total accrued expenses and other liabilities	$ 67,438	$ 71,234

11. Collaborative and Licensing Arrangements.

Endo

In December 2018, the Company entered into a collaboration and license agreement (Collaboration) with 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 (Par). Under the Collaboration, Endo assumes all development, manufacturing, clinical, regulatory, sales and marketing costs under the collaboration, while the Company is responsible for exercising commercially reasonable efforts to develop, or cause the development of, a final finished, stable dosage form of generic Sabril® tablets.

11. Collaborative and Licensing Arrangements (continued).

In October 2023, Endo informed the Company that it is discontinuing work on the Collaboration for development and commercialization of vigabatrin and that it wished to terminate the arrangement. As the Company proceeds with the termination process, the Company does not expect the end of the collaboration to 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 Pharmaceuticals Inc. (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 will receive an up-front payment, 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.

This agreement is in form identified as a collaborative agreement and the Company has concluded for accounting purposes that it also represents a contract with a customer. This is because the Company grants to KYE a license and provides supply of FIRDAPSE® in exchange for consideration, which are outputs of the Company's ongoing activities. Accordingly, the Company has concluded that this collaborative arrangement will be accounted for pursuant to Topic 606.

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 exists at the point in time in which the license is granted.

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

Revenues from the arrangement with KYE for the years ended December 31, 2023, 2022 and 2021 were not material. Revenue is included in product revenue, net and 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.

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 may earn further development and sales 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 exists at the point in time in which the right to the license is 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.

11. Collaborative and Licensing Arrangements (continued).

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 later 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 was $1.9 million in revenue from the arrangement with DyDo for the year ended December 31, 2023, of which $0.5 million is 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 is included in licensing and other revenue in the accompanying consolidated statements of operations and comprehensive income. There was $0.5 million in revenue from the arrangement with DyDo for the year ended December 31, 2022, which is included in product revenue, net in the accompanying consolidated statements of operations and comprehensive income. Revenues from the arrangement with DyDo for the year ended December 31, 2021 were approximately $2.9 million, which primarily consisted of a $2.7 million nonrefundable upfront license fee, which is included in licensing and other revenue in the accompanying consolidated statements of operations and comprehensive income.

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's complaint, which was filed in the federal district court for the Southern District of Florida, alleged that the FDA's approval of RUZURGI® violated multiple provisions of FDA regulations regarding labeling, resulting in misbranding in violation of the Federal Food, Drug, and Cosmetic Act (FDCA); violated the Company's statutory rights to Orphan Drug Exclusivity and New Chemical Entity Exclusivity under the FDCA; and was in multiple other respects arbitrary, capricious, and contrary to law, in violation of the Administrative Procedure Act. Among other remedies, the suit sought an order vacating the FDA's approval of RUZURGI®.

On July 30, 2020, the Magistrate Judge considering this lawsuit filed a Report and Recommendation in which she recommended to the District Judge handling the case that she grant the FDA's and Jacobus' motions for summary judgment and deny the Company's motion for summary judgment. On September 29, 2020, the District Judge adopted the Report and Recommendation of the Magistrate Judge, granted the FDA's and Jacobus' motions for summary judgment, and dismissed the Company's case. The Company appealed the District Court's decision to the U.S. Court of Appeals for the 11th Circuit. The case was fully briefed in early 2021, and oral argument was held in March 2021.

On September 30, 2021, a three-judge panel of 11th Circuit judges issued a unanimous decision overturning the District Court's decision. The appellate court adopted the Company's argument that the FDA's approval of RUZURGI® violated the Company's rights to Orphan Drug Exclusivity and remanded the case to the District Court with orders to enter summary judgment in the Company's favor. In November 2021, Jacobus filed a motion seeking rehearing of the case from the full 11th Circuit, which motion was denied in January 2022. Further, in January 2022, Jacobus filed motions with both the 11th Circuit and the U.S. Supreme Court seeking a stay of the 11th Circuit's ruling indicating that it would seek a review of the 11th Circuit's decision from the U.S. Supreme Court. Both stay motions were denied, and on January 28, 2022, the 11th Circuit issued a mandate directing the District Court to enter summary judgment in the Company's favor. The District Court entered that order on January 31, 2022. On February 1, 2022, the FDA informed Jacobus that, consistent with the Court of Appeals for the Eleventh Circuit's September 30, 2021, decision in favor of Catalyst, the final approval of the RUZURGI® NDA was switched to a tentative approval until the 7-year orphan-drug exclusivity (ODE) for FIRDAPSE® has expired.

12. Commitments and Contingencies (continued).

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 United States 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 United States 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 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 the remaining $10 million will be paid on the second anniversary of closing;
- An annual royalty on our net sales (as defined in the License and Asset Purchase Agreement between Catalyst and Jacobus) of amifampridine products in the United States 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 United States, 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; and
- If the Company were to receive a priority review voucher for FIRDAPSE® or RUZURGI® in the future, 50% of the consideration paid by a third-party to acquire that voucher will be paid to Jacobus.

The Company's New Drug Submission filing for FIRDAPSE® for the symptomatic treatment of LEMS was approved when Health Canada issued a Notice of Compliance, or NOC, on July 31, 2020. In August 2020, the Company entered into a license agreement with KYE Pharmaceuticals, or KYE, pursuant to which the Company licensed to KYE the Canadian rights for FIRDAPSE® for the treatment of LEMS. On August 10, 2020, Health Canada issued a NOC to Medunik (Jacobus' licensee in Canada for RUZURGI®) for the treatment of LEMS. Shortly thereafter, the Company initiated a legal proceeding in Canada seeking judicial review of Health Canada's decision to issue the NOC for RUZURGI® as incorrect and unreasonable under Canadian law due to Medunik's use of Catalyst's protected data in its application. After two decisions by the trial judge to quash the RUZURGI® approval and remand the matter back to Health Canada, the Canadian Federal Appellate Court overturned the trial judge's decision. The Minister subsequently reapproved RUZURGI®'s NOC for Canada in 2023. The Company does not expect the approval of RUZURGI® in Canada to have a material impact on the Company's consolidated financial statements.

In January 2023, the Company received Paragraph IV Certification Notice Letters from three generic drug manufacturers 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 United States. 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, as amended, 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. Further, in October 2023, the Company received a Paragraph IV Certification Notice Letter from a fourth generic drug manufacturer, and the Company filed a similar lawsuit against the manufacturer in November 2023. The Company intends to vigorously protect and defend its intellectual property for FIRDAPSE® and, although there can be no assurance, the Company believes that its patent estate will protect FIRDAPSE® from generic competition for the life of the patents.

12. Commitments and Contingencies (continued).

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®, the fourth such certification for this formulation. Both of these letters were paragraph IV certifications of non-infringement, non-validity, and unenforceability to the '497 patent for FYCOMPA® but each application, like the previous Paragraph IV notices from ANDA filers, for FYCOMPA® tablets does not challenge the '571 patent. 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.

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 will be expanded under the license agreement 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 our 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.

On December 18, 2023, DyDo filed a Japan NDA with the PMDA, which was accepted for filing upon its submission. As a result, the Company's territory automatically expanded on that date to include most of Asia, as well as Latin 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., and SERB S.A. 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 United States and Mexico.

Pursuant to the terms of the license agreement, the Company paid Jacobus a $10 million up-front payment on the Effective Date and also paid an additional $10 million on the first annual anniversary of the Effective Date (July 11, 2023). The Company is obligated to pay an additional $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 products in the United States 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 United States.

13. Agreements (continued).

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 United States) of $5 million unless a competing product or generic version of FIRDAPSE® is being marketed or sold in the United States. 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 United States for RUZURGI®, its 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 were recorded as an expense in research and development expenses in the consolidated statement of operations and comprehensive income for 2022.

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 Co., Ltd. (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.

13. Agreements (continued).

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, 2023, the full $5.9 million has been paid in cash.

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® products 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. The license is for exclusive commercial rights in the U.S., Canada, and Mexico, as well as the right of first negotiation in Europe and 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.

13. Agreements (continued).

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 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®. The Company may also be obligated under certain circumstances to make milestone payments and to pay royalties to Santhera.

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 amortized using the straight-line method over the product's estimated useful life of 10.5 years.

13. Agreements (continued).

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 on selling 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 statement of operations and comprehensive income.

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, 2023, 2022, and 2021 consists of (in thousands):

	2023	2022	2021
Current - Federal	$ 34,975	$ 12,858	$ 2,455
Current - State	5,931	3,877	1,414
Deferred - Federal	(16,093)	4,739	8,620
Deferred - State	(1,712)	166	696
	$ 23,101	$ 21,640	$ 13,185

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:

	2023	2022	2021
Statutory rate	21.0%	21.0%	21.0%
State tax	3.1%	3.1%	3.4%
Executive compensation limitation	2.6%	3.6%	1.1%
Tax credit	—	(1.9)%	(0.6)%
Stock compensation windfall	(4.4)%	(5.6)%	(0.6)%
Other	2.1%	0.5%	0.7%
	24.4%	20.7%	25.0%

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, 2023 and 2022 are as follows (in thousands):

	2023	2022
Deferred tax assets:		
Start-up costs	$ —	$ 9,771
Deferred compensation	6,473	4,706
Inventory	448	296
Intangible assets	24,847	52
Accrued expenses	788	—
Operating lease liability	854	953
Capitalized research	4,927	4,255
Total deferred tax assets	38,337	20,033
Deferred tax liabilities:		
Prepaid expenses	(1,023)	(481)
Right-of use asset	(759)	(860)
Other	(11)	44
Total deferred tax liabilities	(1,793)	(1,297)
Deferred tax assets, net	$ 36,544	$ 18,736

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. As of December 31, 2023, 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 $37 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, 2023 and 2022. 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, 2023 and 2022. No shares of preferred stock were outstanding at December 31, 2023 and 2022.

Common Stock

The Company has 200,000,000 shares of authorized common stock, par value $0.001 per share. At December 31, 2023 and 2022, 107,121,549 and 105,263,031 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. See Note 18 (Subsequent Events).

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. No shares were repurchased during the year ended December 31, 2023. During the years ended December 31, 2022 and 2021, 1,000,000 and 2,208,292 shares, respectively, were repurchased for an aggregate purchase price of approximately $6.9 million and $12.1 million, respectively, ($6.91 and $5.47 average price per share).

15. Stockholders' Equity (continued).

2020 Shelf Registration Statement

On July 23, 2020, the Company filed a shelf registration statement with the SEC to sell up to $200 million of common stock, preferred stock, warrants to purchase common stock, debt securities and units consisting of one or more of such securities (the "2020 Shelf Registration Statement"). The 2020 Shelf Registration Statement (file no. 333-240052) was declared effective by the SEC on July 31, 2020. The Company's 2020 Shelf Registration Statement expired on July 31, 2023.

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 $140.1 million under the Company's 2023 Shelf Registration Statement. See Note 18 (Subsequent Events).

16. Stock Compensation.

For the years ended December 31, 2023, 2022 and 2021, the Company recorded stock-based compensation expense as follows (in thousands):

	2023	2022	2021
Research and development	$ 1,481	$ 1,729	$ 1,611
Selling, general and administrative	12,769	6,178	4,462
Total stock-based compensation	$ 14,250	$ 7,907	$ 6,073

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, 2023, no shares remain available for future issuance under the 2014 Plan. Under the 2018 Plan, 18,000,000 shares are reserved for issuance and as of December 31, 2023, 1,801,949 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, 2023, 2022 and 2021, options to purchase 1,651,345, 3,172,342 and 1,328,936 shares, respectively, of the Company's common stock were exercised with gross proceeds to the Company of approximately $2.8 million, $9.6 million and $4.1 million, respectively. During the years ended December 31, 2023, 2022 and 2021, no options to purchase shares of the Company's common stock were exercised on a "cashless" basis.

During the years ended December 31, 2023, 2022 and 2021 the Company recorded non-cash stock-based compensation expense related to stock options totaling approximately $11.1 million, $6.3 million and $5.5 million, respectively.

During the years ended December 31, 2023, 2022 and 2021, the Company granted seven-year options to purchase an aggregate of 3,598,535, 1,386,500 and 2,330,000 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, 2023 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	12,309,108	$ 4.93		
Granted	3,598,535	14.62		
Exercised or released	(1,651,345)	1.69		
Forfeited or cancelled	(78,810)	11.83		
Expired	—	—		
Outstanding at end of year	14,177,488	$ 7.73	4.05	$ 130,966
Exercisable at end of year	8,891,904	$ 4.45	2.78	$ 110,488

Other information pertaining to stock option activity during the years ended December 31, 2023, 2022 and 2021 was as follows:

	2023	2022	2021
Weighted–average fair value of granted stock options	$ 8.66	$ 8.52	$ 3.24
Total fair value of vested stock options (in thousands)	$ 8,278	$ 6,096	$ 6,421
Total intrinsic value of exercised stock options (in thousands)	$ 22,265	$ 31,881	$ 3,623

As of December 31, 2023, there was approximately $36.3 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 3.11 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 were as follows:

	2023	2022	2021
Risk free interest rate	3.55% to 4.92%	1.27% to 4.07%	0.34% to 1.18%
Expected term	4.5 to 5.2 years	4.5 years	4.5 to 4.8 years
Expected volatility	68.0% to 71.0%	68.4% to 69.5%	68.6% to 72.8%
Expected dividend yield	—%	—%	—%
Expected forfeiture rate	—%	—%	—%

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, 2023 was as follows:

16. Stock Compensation (continued).

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Nonvested balance at beginning of year	752,500	$ 11.46
Granted	370,117	13.67
Vested	(268,158)	11.25
Forfeited	—	—
Nonvested balance at end of year	854,459	$ 12.48

During the year ended December 31, 2023, 2022 and 2021, the Company recorded non-cash stock-based compensation expense related to restricted stock units totaling $3.2 million, $1.6 million and $0.5 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, 2023, 2022, and 2021, the Company's matching contributions were approximately $0.7 million, $0.5 million and $0.5 million, respectively.

18. Subsequent Events.

On January 9, 2024, the Company completed a public offering of 10 million shares of its authorized but unissued common stock, raising net proceeds of approximately $140.1 million. The proceeds of the offering will be used to potentially acquire new products and for general corporate purposes.

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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
Chairman and Chief Financial Officer
The Kitchen, LLC

Molly Harper
Chief Business Officer
Synlogic, Inc.

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

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 21, 2024 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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