



## 2022 ANNUAL REPORT





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**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**  
Washington, D.C. 20549

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**FORM 10-K**

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[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, 2022

OR

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

Commission File No. 001-33057

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**CATALYST PHARMACEUTICALS, INC.**

(Exact name of registrant as specified in its charter)

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Delaware  
(State of jurisdiction of  
incorporation or organization)

355 Alhambra Circle, Suite 801  
Coral Gables, Florida  
(Address of principal executive offices)

76-0837053  
(IRS Employer  
Identification No.)

33134  
(Zip Code)

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

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

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

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

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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	<input type="checkbox"/>	Accelerated filer	<input checked="" type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input type="checkbox"/>
		Emerging Growth Company	<input type="checkbox"/>

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, 2022, 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 \$665,590,948.

Indicate the number of shares outstanding of each of the issuer’s classes of common stock, as of the latest practicable date: 105,654,395 shares of common stock, \$0.001 par value per share, were outstanding as of March 13, 2023.

Part III incorporates certain information by reference from the registrant’s definitive proxy statement for the 2023 annual meeting of stockholders. The proxy statement with respect to the 2023 annual meeting of stockholders will be filed no later than 120 days after the close of the registrant’s fiscal year ended December 31, 2022.

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## **EXHIBITS FILED WITH FORM 10-K**

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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 “Item 1A – Risk Factors” and those discussed in the section entitled “Item 7 – Management’s Discussion and Analysis of Financial Condition and Results of Operations – Caution Concerning Forward-Looking Statements.”

The continued successful commercialization of FIRDAPSE® and FYCOMPA® are highly uncertain. Factors that will affect our success include the uncertainty of:

- The impact of the COVID-19 pandemic on our business or on the economy generally;
- Whether we will be able to continue to successfully market FIRDAPSE® and now successfully market 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 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 the daily dose of FIRDAPSE® taken by patients changes over time and affects our results of operations;
- Whether new FIRDAPSE® patients and FYCOMPA® patients can be successfully titrated to stable therapy;
- Whether we can continue to market FIRDAPSE® and now market FYCOMPA® on a profitable and cash flow positive basis;
- Whether we can successfully integrate the team that we are hiring to market FYCOMPA® into our current business structure;
- Whether the acquisition of FYCOMPA® will prove to be accretive to EBITDA and EPS in 2023;
- Whether any revenue or earnings guidance that we provide to the public market will turn out to be accurate;
- Whether payors will reimburse for our products at the price that we charge for our products;
- 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 FIRDAPSE® and FYCOMPA®;
- Our ability to maintain compliance with the applicable rules that relate to our contributions to 501(c)(3) organizations that support LEMS patients;
- 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® or FYCOMPA®;
- Our ability to obtain a favorable decision on our pending request for reconsideration for an extension of the expiration date of patent protection for one of our patents listed in the Orange Book for FYCOMPA®;

- 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 the research and 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® after our orphan drug exclusivity for FIRDAPSE® expires;
- Whether we will be successful in our litigation to enforce our patents against the Paragraph IV challengers who have filed relating to FIRDAPSE®;
- The impact on our profits and cash flow of adverse changes in reimbursement and coverage policies 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;
- The state of the economy generally and its impact on our business;
- The potential impact of future healthcare reform in the United States, including the Inflation Reduction Act of 2022, and measures being taken worldwide designed to reduce healthcare costs and limit the overall level of government expenditures, including the impact of pricing actions and reduced reimbursement for our product;
- 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 our collaboration partner in Japan, DyDo, will successfully complete the clinical trial in Japan that will be required to seek approval to commercialize FIRDAPSE® in Japan;
- Whether DyDo will be able to obtain approval to commercialize FIRDAPSE® in Japan; and
- Whether our version of vigabatrin tablets will ever be approved by the FDA and successfully marketed by Endo, whether we will earn milestone payments or royalties on sales of our version of generic vigabatrin tablets, and whether Endo's bankruptcy filing will impact these issues.

Our current plans and objectives are based on assumptions relating to the continued commercialization of FIRDAPSE® and FYCOMPA® 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 diseases and diseases that are difficult to treat. With exceptional patient focus, we are committed to developing a robust pipeline of cutting-edge, best-in-class medicines for treating rare and difficult to treat diseases. We are dedicated to making a meaningful impact on the lives of those suffering from rare and difficult to treat diseases, and we believe in putting patients first in everything we do.



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. On December 17, 2022, we entered into an agreement with Eisai Inc. (“Eisai”) for the acquisition of the United States rights to FYCOMPA®(perampanel) CIII, 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. We closed that acquisition on January 24, 2023 and we are now marketing FYCOMPA® in the United States.

### ***Impact of the COVID-19 pandemic on our business***

The COVID-19 pandemic affected our business operations in numerous ways. At various times during the pandemic, we had to make modifications to our normal operations, including allowing our employees to work remotely. Further, during the pandemic, national, state and local governments in affected regions implemented varying safety precautions, such as quarantines, border closures, increased border controls, travel restrictions, shelter-in-place orders and shutdowns, business closures, cancellations of public gatherings, mask mandates, and other measures. While most of these measures have since been relaxed or removed, a resurgence in cases as a result of one or more new variants could lead to some or all of these precautions being put back into place. At present, our operations have returned to mostly being in-person, with some contact with physicians by our commercial sales force still being done remotely. However, there can be no assurance that the COVID-19 pandemic will not in the future disrupt once again our normal business operations.

### ***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 Lambert-Eaton myasthenic syndrome, or 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 27 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 six medical science liaisons who are helping educate the medical communities and patients about LEMS and our programs supporting patients and access to FIRDAPSE®.

Additionally, we have contracted with an experienced inside sales agency that works to generate leads through telemarketing to targeted physicians. This inside sales agency allows 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®. 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 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 product and services to potential patients and their healthcare providers. We also work with several rare disease advocacy organizations (including Global Genes 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 \$10.00 per month (currently less than \$2.00 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. However, we are donating, and committing to continue to donate, money to qualified, independent charitable foundations dedicated to providing assistance to any U.S. LEMS patients in financial need. 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 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 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 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 commence patent infringement lawsuits against these generic drug manufacturers in a federal district court to trigger a stay precluding FDA from approving any ANDA 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.

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 for the life of our patents.

### ***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 4 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.

Pursuant to the Asset Purchase Agreement, which closed on January 24, 2023, 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® is approved by the U.S. Patent and Trademark Office (USPTO). 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 and a Supply Agreement. Under the Transition Services Agreement, a U.S. subsidiary of Eisai is providing us with certain transitional services, and under the Supply Agreement, Eisai has 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). Following the closing of the acquisition, we are currently marketing FYCOMPA® in the U.S. through Eisai under the Transition Services Agreement as we build our FYCOMPA® marketing and sales team, and we expect to take over the marketing program in May 2023. In that regard, we currently expect to hire approximately 34 sales and marketing personnel to support FYCOMPA®, many of whom previously worked in Eisai’s U.S. sales division marketing FYCOMPA®. We also are planning on hiring up to six medical science liaisons to help us educate the medical community who treat epilepsy and the patients who have epilepsy about their disease and the benefits of 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). Eligible cash-paying patients receive up to \$60 towards each prescription, up to a maximum of \$720 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 will expire no earlier than May 23, 2025, the current expiration date of U.S. patent no. 6,949,571 including the USPTO’s patent term extension calculation. A request for reconsideration of the agency’s patent term extension calculation is currently pending. If successful, we would be entitled to patent term extension that would extend U.S. patent no. 6,949,571 until June 8, 2026. There can be no assurance that our request for reconsideration will be granted by the U.S. Patent and Trademark Office.

### ***Business Development***

We are continuing our efforts to broaden and diversify our product portfolio through acquisitions of early and/or late-stage products or companies or technology platforms in rare disease and CNS therapeutic categories. To accomplish these priorities, we are continuing

to employ a disciplined approach to evaluating assets, and we believe that this strategic expansion will better position our company long term to build out a broader more diversified portfolio of drug candidates (which should add greater value to our company over the near and long-term). In that regard, we are currently exploring several additional potential opportunities to acquire companies with commercial drug products and/or drug products in development or to in-license or acquire commercialized drug products or drug products in development. However, 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.

## Capital Resources

At December 31, 2022, we had cash and investments of approximately \$298 million. Subsequent to the end of 2022, on January 24, 2023 we used \$162 million of our available cash and cash equivalents to fund our acquisition of FYCOMPA® and to reimburse Eisai for certain prepaid expenses. 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®, that our commercialization of FYCOMPA® 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.

## Our Strategy

Our goal is to develop and commercialize novel prescription drugs targeting rare (orphan) neuromuscular and neurological diseases and 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 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 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.
- Begin to commercialize FYCOMPA®. We recently acquired the rights to the epilepsy drug FYCOMPA®. We are currently marketing FYCOMPA® through Eisai under a Transition Services Agreement and expect to begin marketing the product through our own sales organization in May 2023. We believe that having a second marketed product that we expect to be accretive to EBITDA and EPS in 2023 adds substantially to our business franchise.
- Seek approval for FIRDAPSE® in Japan and potentially in other territories in Asia. We are currently supporting our sublicensee, DyDo Pharma, as they take the steps required to seek approval to market FIRDAPSE® in Japan for the treatment of Japanese patients with LEMS. Further, the territory in which we have the right to seek to commercialize FIRDAPSE® will automatically expand to include numerous countries in Asia and Latin America upon acceptance by the Japanese Ministry of Health, Labor and Welfare in Japan of an application to market our product in Japan, and we plan to seek to expand our FIRDAPSE® activities into other countries in Asia once our licensed territory is expanded to include these new countries.
- Seek to acquire additional products. We intend to continue our efforts to broaden and diversify our product portfolio through acquisitions of early and/or late-stage products or companies or technology platforms in rare disease and CNS therapeutic categories. To accomplish these priorities, we are continuing to employ a disciplined approach to evaluating assets and we believe that this strategic expansion will better position our company to build out a broader more diversified portfolio of drug candidates, which should add greater value to our company over the near and long-term.

## FIRDAPSE® Product Overview

FIRDAPSE® is Catalyst’s registered trade name in the United States for amifampridine phosphate tablets. 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 80 mg per day. Among the 1,279 patients or healthy subjects

assessed in the literature, the most frequently reported adverse events (AEs) were perioral and peripheral paresthesias (unusual sensations like pins and needles), and gastrointestinal disorders (abdominal pain, nausea, diarrhea, and epigastralgia (pain around the upper part of the stomach)). These events were typically mild or moderate in severity, and transient, seldom requiring dose reduction or withdrawal from treatment.

### *Lambert-Eaton Myasthenic Syndrome (LEMS)*

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 approximately 3,000 LEMS patients in the United States, approximately 1,500 of which are presently diagnosed and identified and approximately 1,500 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 License Agreement) between us and BioMarin Pharmaceutical Inc. (BioMarin). Under the License Agreement, we make the following royalty payments on our net sales of FIRDAPSE®:

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



On May 29, 2019, we entered into an amendment to our 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 will be automatically expanded to include most of Asia, as well as Central and South America, upon the acceptance by the Japanese Ministry of Health, Labor and Welfare in Japan of an application to market our product in Japan. Under the amendment, we will pay royalties on net sales in Japan of a similar percentage to the royalties that we are currently paying under our original License Agreement for North America.

In January 2020, we were advised that BioMarin had sold certain rights under the License Agreement to SERB SA.

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

#### License Agreement with Jacobus

In May 2019, the FDA approved an NDA for RUZURGI®, Jacobus Pharmaceuticals' version of amifampridine (3,4-DAP), for the treatment of pediatric LEMS patients (ages 6 to under 17). In June 2019 we filed suit against the FDA and several related parties challenging this approval and related drug labeling. Jacobus later intervened in the case. 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 11<sup>th</sup> 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 11<sup>th</sup> 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 11<sup>th</sup> Circuit, which motion was denied in January 2022. Further, in January 2022, Jacobus filed motions with both the 11<sup>th</sup> Circuit and the U.S. Supreme Court seeking a stay of the 11<sup>th</sup> Circuit's ruling indicating that it would seek a review of the 11<sup>th</sup> Circuit's decision from the U.S. Supreme Court. Both stay motions were denied, and on January 28, 2022, the 11<sup>th</sup> 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 has been dismissed without prejudice. Finally, Jacobus agreed that until the later of (i) the expiration of the royalty term or (ii) December 31, 2034, Jacobus and its affiliates, will not, directly or indirectly, research, develop, manufacture, commercialize, distribute, use or otherwise exploit any product competitive to FIRDAPSE® or RUZURGI® in the 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 the balance of which will be paid over the next two years, on the first and 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.

Royalties will be trued up at the end of the year to the extent that royalties on net sales are below the minimum royalty.

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

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

On September 29, 2022, the FDA approved our sNDA to expand the indicated age range for FIRDAPSE® for the treatment of LEMS to include pediatric patients, six years of age and older.

## *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 we has been completed and the FDA has advised us is acceptable. Finally, in connection with the recent approval of our sNDA for FIRDASE® 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 CMS 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.

## *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 six medical science liaisons who help educate the medical communities and patients about LEMS and about our company's ongoing clinical trial activities. Further, we work closely with several rare disease advocacy organizations (including Global Genes, 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 are 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®. 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. We also 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 during transitioning from investigational product while they are waiting for a coverage determination or, later on, for patients whose access is threatened by the bureaucratic complications arising from a change of insurer. The “Catalyst Pathways”® program is also the access point for our Patient Assistance Program, which provides longer-term free medication for those who are uninsured or functionally uninsured with respect to FIRDAPSE® because they may be unable to obtain coverage from their payer despite having health insurance.

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

#### Access to FIRDAPSE®

In order to help patients afford their medication, we, like other pharmaceutical companies who are marketing drugs for ultra-orphan conditions, have developed an array of financial assistance programs that are available to reduce patient co-pays and deductibles to a nominal affordable amount. For eligible patients with commercial coverage, a co-pay assistance program designed to keep out-of-pocket costs to \$10 or less per month (currently less than \$2.00 per month) is available for all LEMS patients prescribed FIRDAPSE®. We are also donating, and committing to continue to donate, money to qualified, independent charitable foundations dedicated to providing assistance to LEMS patients in financial need. Our goal is to ensure that no LEMS patient is ever denied access to their medication for financial reasons.

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

#### 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® in spite of 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. We anticipate completion of that study in late 2023 and, assuming the study is successful, the filing of an application to market the product in Japan in the second quarter of 2024. There can be no assurance that the study will be successful or that the application will ever be filed or approved.

## Future Markets for FIRDAPSE®

Under the amendment to our license agreement that added Japan to our territory, our territory in which we have the right to seek to commercialize FIRDAPSE® will automatically expand to include numerous countries in Asia and South and Central America upon acceptance by the Japanese Ministry of Health, Labor and Welfare in Japan of an application to market our product in Japan, and we plan to expand our FIRDAPSE® activities into other countries in Asia once our licensed territory is expanded to include these new 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, the USPTO allowed continuing application, 11,268,128. On January 3, 2022, the USPTO allowed related continuing application 11,274,332. A further related continuing application, 11,274,331 was allowed on January 7, 2022. All three patents were issued in March 2022 as Patent Nos. 11,268,128, 11,274,332, and 11,274,331, respectively, and extend the coverage of our patents to include fast metabolizers of amifampridine. These patents are now listed in the Orange Book for FIRDAPSE®.

As part of our transaction with Jacobus Pharmaceuticals, 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 PTO on October 10, 2017, is not considered listable under 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.

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 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, we 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 FDA from approving any ANDA 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.

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 for the life of our patents.

We have also in-licensed the FIRDAPSE® trademark, 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 more than 50 million individuals globally, 80% of whom live in developing countries. An estimated 1.7% of U.S. adults have been diagnosed with the condition. From prominent historical figures, such as Julius Caesar and Vladimir Lenin, to friends or family members, most people probably know someone affected by epilepsy.

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

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

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

## Epilepsy

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

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

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). Eligible cash-paying patients receive up to \$60 towards each prescription, up to a maximum of \$720 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 with Eisai, pursuant to which we acquired the U.S. rights to FYCOMPA®. Pursuant to the Asset Purchase Agreement entered into with Eisai for FYCOMPA®, we purchased Eisai’s regulatory approvals and documentation, product records, intellectual property, inventory, and other matters relating to the U.S. rights for FYCOMPA®, in exchange for an up-front cash payment of \$160 million; and royalty payments on net sales post-expiration of the patents for FYCOMPA®, which royalty payments will be reduced upon generic equivalents to FYCOMPA® entering the market. Finally, we have agreed to pay Eisai an additional cash payment of \$25 million if a patent extension for FYCOMPA® is approved by the USPTO (see “Intellectual Property Protections for FYCOMPA® below for additional information on the patent).

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

- A Transition Services Agreement under which a U.S. subsidiary of Eisai 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.

These additional agreements became effective upon the closing of the transaction with Eisai on January 24, 2023.

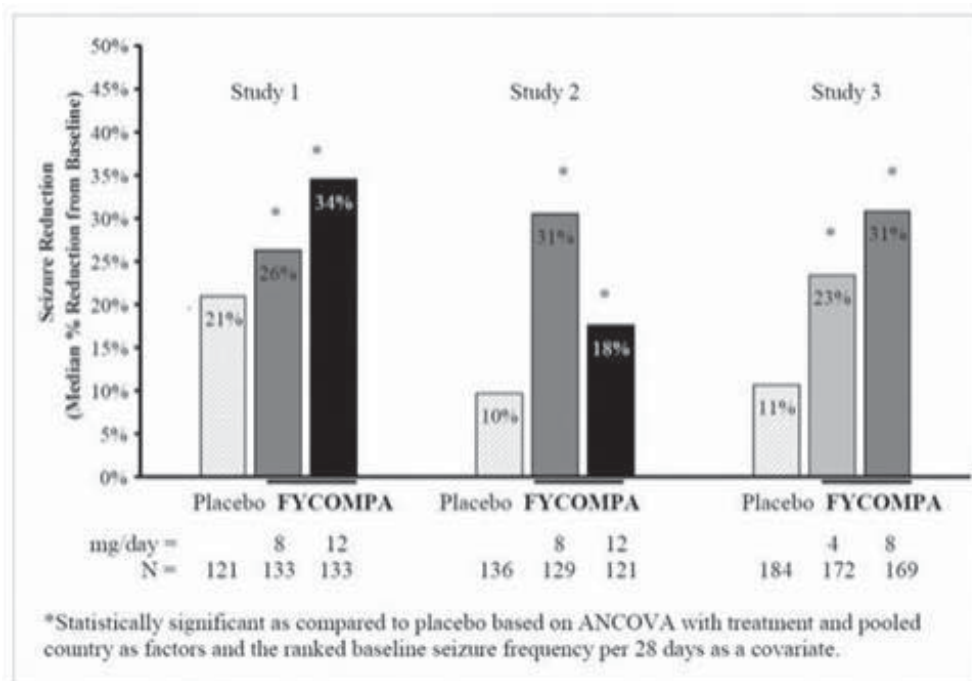
#### *Clinical Trials Supporting FYCOMPA®*

##### Partial Onset Seizures

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

Each study evaluated placebo and multiple FYCOMPA® dosages (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.



Tables 1 and 2 present an analysis combining data from all 3 studies, grouping patients based upon whether or not concomitant enzyme-inducing AEDs (carbamazepine, oxcarbazepine, or phenytoin) were used. The analysis revealed a substantially reduced effect in the presence of inducers.

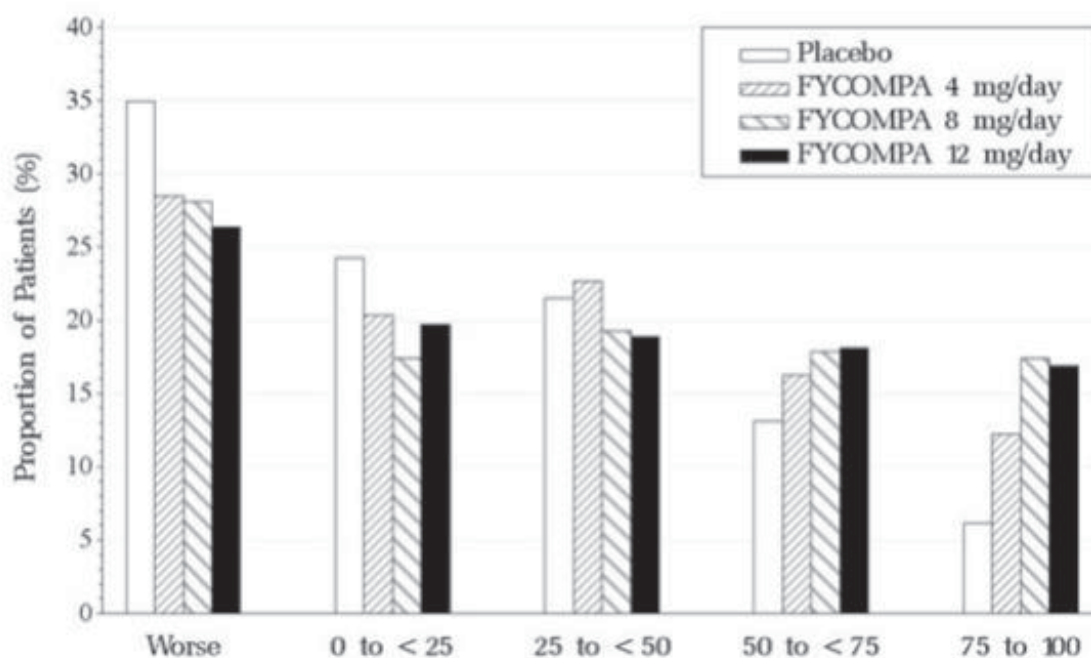
**Table 1. Median Percent Reduction for Combined Studies (Study 1, 2, and 3) Based on the Presence or Absence of Concomitant Enzyme-Inducing AEDs (carbamazepine, oxcarbazepine, or phenytoin)**

	Without Enzyme-Inducing AEDs		With Enzyme-Inducing AEDs	
	Placebo (%)	FYCOMPA® (%)	Placebo (%)	FYCOMPA® (%)
2 mg/day	16	23	14	16
4 mg/day	16	22	14	33
8 mg/day	19	45	12	24
12 mg/day	19	54	9	22

**Table 2. Responder Rate for Combined Studies (Study 1, 2, and 3) Based on the Presence or Absence of Concomitant Enzyme-Inducing AEDs (carbamazepine, oxcarbazepine, or phenytoin)**

	Without Enzyme-Inducing AEDs		With Enzyme-Inducing AEDs	
	Placebo (%)	FYCOMPA® (%)	Placebo (%)	FYCOMPA® (%)
2 mg/day	19	26	18	20
4 mg/day	19	35	18	26
8 mg/day	17	45	19	52
12 mg/day	15	54	21	33

Figure 2 shows the proportion of patients with different percent reductions during the maintenance phase over baseline across all three trials. Patients in whom the seizure frequency increased are shown at left as “worse.” Patients in whom the seizure frequency decreased are shown in the remaining four categories.



The percentages of patients with a 50% or greater reduction in seizure frequency were 19%, 29%, 35%, 35% for placebo, 4, 8, and 12 mg, respectively.

#### Primary Generalized Tonic-Clonic Seizures

The efficacy of FYCOMPA® as adjunctive therapy in patients 12 years of age and older with idiopathic generalized epilepsy experiencing primary generalized tonic-clonic seizures was established in one multicenter, randomized, double-blind, placebo-controlled study (Study 4), conducted at 78 sites in 16 countries. Eligible patients on a stable dose of 1 to 3 AEDs experiencing at least 3 primary generalized tonic-clonic seizures during the 8-week baseline period were randomized to either FYCOMPA® or placebo. Efficacy was analyzed in 162 patients (FYCOMPA® N=81, placebo N=81) who received medication and at least one post-treatment seizure assessment. Patients were titrated over 4 weeks up to a dose of 8 mg per day or the highest tolerated dose and treated for an additional 13 weeks on the last dose level achieved at the end of the titration period. The total treatment period was 17 weeks. Study drug was given once per day.

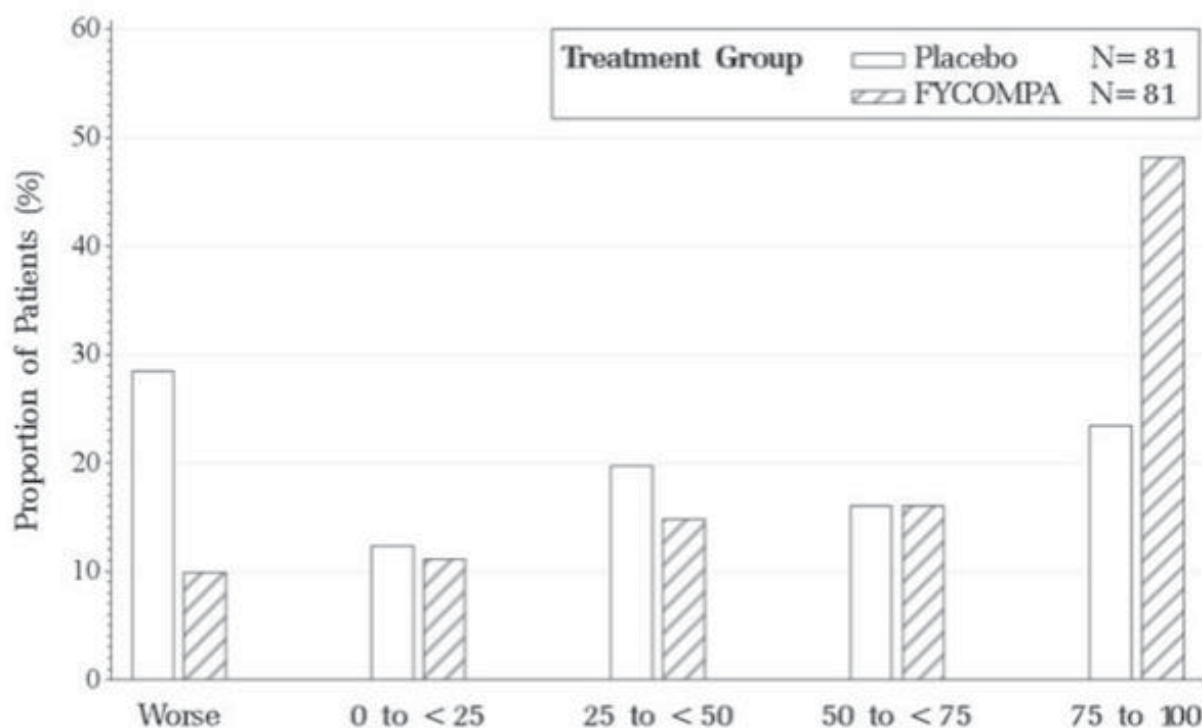
The primary endpoint was the percent change from baseline in primary generalized tonic-clonic seizure frequency per 28 days during the treatment period as compared to the baseline period. The criterion for statistical significance was  $p < 0.05$ . Table 3 shows the results of this study. A statistically significant decrease in seizure rate was observed with FYCOMPA® compared to placebo.

**Table 3. Median Percent Reduction from Baseline in Primary Generalized Tonic-Clonic Seizure Frequency in Study 4**

	Placebo (N=81)	FYCOMPA® 8mg (N=81)
Percent Reduction During Treatment	38	76 <sup>a</sup>
A P-value compared to placebo: <0.0001. Statistically significant as compared to placebo based on ANCOVA with treatment and pooled country as factors and the ranked baseline seizure frequency per 28 days as a variable.		

Figure 3 shows the proportion of patients with different percent reductions during the maintenance phase over baseline in primary generalized tonic-clonic seizure frequency. Patients in whom the seizure frequency increased are shown at left as “worse.” Patients in whom the seizure frequency decreased are shown in the remaining four categories.





#### Intellectual Property Protections for FYCOMPA®

FYCOMPA® (all strengths and formulations) has patent exclusivity until at least May 23, 2025 pursuant to U.S. Patent No. 6,949,571 (the ‘571 patent), entitled “1,2-Dihydropyridine Compounds, Process for Preparation of the Same and Use Thereof.” This patent was issued on September 27, 2005 and covers the approved drug substance (perampanel).

Our predecessor in interest, Eisai, applied for a Patent Term Extension for the ‘571 patent through June 8, 2026, which was rejected by the U.S. Patent and Trademark Office in favor of a Patent Term Extension calculation resulting in the patent terminating on May 23, 2025. Our predecessor in interest filed a request for reconsideration, arguing that FYCOMPA® is entitled to patent exclusivity through June 8, 2026 due to a lengthy delay in scheduling FYCOMPA® as a controlled substance such that Eisai was not able to market FYCOMPA® commercially after approval. The U.S. Patent and Trademark Office has not yet ruled on Eisai, now Catalyst’s, request for reconsideration. Should the patent extension be granted, the term of the patent would be extended through June 8, 2026. There can be no assurance that the USPTO will grant the extension. Because we are disputing the U.S. Patent and Trademark Office’s decision with respect to Patent Term Extension, we have not updated the Orange Book to reflect the extended expiration date of the ‘571 patent and have instead requested that the Orange Book reflect the expiration date of the patent resulting from a series of Interim Patent Term Extension Requests. Catalyst will continue to file Interim Patent Term Extension requests until the matter is resolved, at which time the expiration date of the ‘571 patent will be updated in the Orange Book.

FYCOMPA® is also the subject of the following additional intellectual property:

- U.S. Patent No. 8,772,497 claims the commercial crystalline form of perampanel for FYCOMPA®. This patent expires on July 1, 2026 and is Orange Book listed.
- U.S. Patent No. 8,304,548 claims the commercial process used to produce perampanel for FYCOMPA®, and has an expiration date of October 24, 2027.
- There are three patents related to non-commercial forms of perampanel:
  - o U.S. Patent No. 9,045,426 with an expiration date of July 5, 2025.
  - o U.S. Patent No. 7,803,818 with an expiration date of December 20, 2026.
  - o U.S. Patent No. 7,718,807 with an expiration date of April 27, 2027.

## ***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 \$500,000. We will be entitled to receive a milestone payment of \$2.0 million on the commercial launch of the product. Further, we will receive a sharing of defined net profits upon commercialization and certain expenses for development.

There can be no assurance that our collaboration with Endo for the development of generic Sabril® (vigabatrin) tablets will be successful and that if an ANDA is approved for vigabatrin tablets in the future, that it will be profitable to us.

## **Manufacturing and Supply**

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

## ***FIRDAPSE®***

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

## ***FYCOMPA®***

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

## ***Manufacturing Changes***

Any significant change that we make for any of our drug products must be approved by the FDA in an sNDA. If the manufacturing plan and data are insufficient, any sNDA we submit will not be approved. Before an sNDA can be approved, our manufacturers must also demonstrate compliance with FDA's cGMP regulations and policies. Further, even if we receive approval of any sNDAs for our drug product(s), if our manufacturers do not follow cGMPs in the manufacture of our products, it may delay product launches or shipments and adversely affect our business.

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

## **Competition**

The pharmaceutical industry is intensely competitive, and any product candidate developed or licensed by us would likely compete with currently marketed and potentially new drugs and therapies even though they are not indicated for these conditions. 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 may remain available, even though we have obtained FDA approval of FIRDAPSE®. 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). 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 list of bulk substances that may be used in compounding (i.e., the Section 503B bulk substances list 1).

While the FDA has been aggressively 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. 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.



Sabril® is marketed by Lundbeck in the United States for infantile spasms and for refractory complex partial seizures. Four generic versions of Sabril® tablets has been approved to date in the United States, as have numerous generic versions of the powder form. We have entered into a definitive agreement with Endo/Par for the further development and commercialization of generic Sabril® tablets.

*Factors affecting competition generally*

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

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

**Business Development**

Following our recent acquisition of the U.S. rights to FYCOMPA®, we are continuing to work to broaden and diversify our product portfolio through acquisitions of early and/or late-stage products or companies or technology platforms in rare disease therapeutic categories outside of neuromuscular diseases. To accomplish these priorities, we are continuing to employ a disciplined approach to evaluating assets, and we believe that this strategic expansion will better position our company long term to build out a broader more diversified portfolio of drug candidates (which should add greater value to our company over the near and long-term). In that regard, we are currently exploring several additional potential opportunities to acquire companies with commercial drug products and/or drug products in development or to in-license or acquire commercialized drug products or drug products in development. However, 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, 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 patients under the supervision of one or more qualified investigators in accordance with federal regulations and GCP.

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

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

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

may be able to use the data from these clinical trials to meet all or part of any Phase 4 clinical trial requirement. Failure to promptly conduct Phase 4 clinical trials where necessary could result in withdrawal of approval for products approved under accelerated approval regulations.

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

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

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

### *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 2023 this fee is \$3,242,026), 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 2023 is \$393,333 per prescription drug product. User fees typically increase annually. The approval process is lengthy and difficult, and the FDA may refuse to approve an NDA if the applicable regulatory criteria are not satisfied or may require additional clinical or other data and information. Even if such data and information is submitted, the FDA may ultimately decide that the NDA does not satisfy the criteria for approval. The FDA may also refer applications for novel drug products or drug products which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee. The FDA reviews an NDA to determine, among other things, whether a product is safe and effective for its intended use. Before approving an NDA, the FDA will inspect the facility or facilities where the product is manufactured to determine whether its manufacturing is cGMP-compliant to assure and preserve the product's identity, strength, quality, purity and stability.

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

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

### *Post-approval Requirements*

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

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

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

### *Controlled Substance Regulations*

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

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

### *The Hatch-Waxman Amendments*

#### Orange Book Listing

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent with claims covering the applicant's product or approved methods of using the product. Upon approval of a drug, each of the patents listed in the application for the drug are then published in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential generic competitors in support of approval of an 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 certification is filed. If there is no listed patent in the Orange Book, there cannot be a Paragraph IV certification, and, thus, no ANDA can be filed before the expiration of the exclusivity period.

### *Section 505(b)(2) NDAs*

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

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

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

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

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

A section 505(b)(2) NDA applicant must send notice of the Paragraph IV certification to the owner of the referenced NDA for the previously approved product and relevant patent holders within 20 days after the Section 505(b)(2) NDA has been accepted for filing by the FDA. If the relevant patent holder elects to initiate litigation, the Section 505(b)(2) applicant may invest a significant amount of time and expense in the development of its product, only to be subject to significant delay and patent litigation before its product may be commercialized. Alternatively, if the NDA applicant or relevant patent holder does not file a patent infringement lawsuit within the specified 45 day period, the FDA may approve the Section 505(b)(2) application at any time.

## ANDAs

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

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

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

There are also user fees for ANDA applicants, sponsors, and manufacturers. For fiscal year 2023, the application fees are \$240,582 per ANDA application and the facility fees are \$213,134 per domestic finished dosage form facility, \$228,134 per foreign finished dosage form facility, \$37,544 per domestic active pharmaceutical ingredient facility, and \$52,544 per foreign active pharmaceutical ingredient facility. In addition, there is a new annual program fee based on the size of the generic drug applicant. These user fees typically increase each fiscal year.

### *Other Regulatory Requirements*

In addition to regulation by the FDA and certain state regulatory agencies, we are also subject to a variety of foreign regulations governing clinical trials and the marketing of other products. Outside of the 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 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 (the “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 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 pharmacy benefit management (PBM) plans. Decisions regarding the extent of coverage and the amount of reimbursement are expected to be made on a plan-by-plan, and in some cases, on a patient-by-patient basis. Particularly given the rarity of LEMS, 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. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties, and exclusion from participation in federal healthcare programs. Although there are a number of statutory exemptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor.

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

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

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

Prescription drug advertising is subject to federal, state and foreign regulations. In the 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. To date, the rebate amount for a drug has been capped at 100% of the AMP; however, effective January 1, 2024, this cap will be 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 Inflation Reduction Act of 2022 (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 will also allow HHS to negotiate the selling price of certain drugs and biologics that CMS reimburses under Medicare Part B and Part D, although only high-expenditure single-source drugs (excluding drugs and biologics that are designated and approved for only one rare disease or condition) that have been approved for at least 7 years (11 years for biologics) can be selected by CMS for negotiation, with the negotiated price taking effect two years after the selection year. The negotiated prices, which will first become effective in 2026, will be capped at a statutory ceiling price. Beginning in October 2022 for Medicare Part D and January 2023 for Medicare Part B, the IRA will also penalize drug manufacturers that increase prices of Medicare Part D and Part B drugs at a rate greater than the rate of inflation.

#### *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 Department of Veterans Affairs, the Department of Defense (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), federal law requires manufacturers to pay refunds on utilization of their covered drugs sold to Tricare beneficiaries through retail pharmacies in DoD's Tricare network. These refunds are generally the difference between the Non-FAMP and the FCP and are due on a quarterly basis. Absent an agreement from the manufacturer to provide such refunds, DoD will designate the manufacturer's products as Tier 3 (non-formulary) and require that beneficiaries obtain prior authorization in order for the products to be dispensed at a Tricare retail network pharmacy. However, refunds are due whether or not the manufacturer has entered into such an agreement.

### *Branded Pharmaceutical Fee*

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

### **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 March 15, 2023, we had approximately 82 employees, approximately 34 of whom are in our commercial organization, approximately 25 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.

Following the closing of the acquisition of FYCOMPA®, we are currently marketing FYCOMPA® in the U.S. through Eisai under the Transition Services Agreement as we build our FYCOMPA® marketing and sales team. We expect to take over the marketing program for FYCOMPA® in May 2023 and, in that regard, we currently expect to hire approximately 34 sales and marketing personnel for marketing FYCOMPA®, many of whom previously worked in Eisai's U.S. sales division marketing FYCOMPA®. We also are planning to hire up to six medical science liaisons who help us educate the medical community who treat epilepsy and the patients who have epilepsy about their disease and the benefits of FYCOMPA®. Finally, we also expect to add additional product support personnel to our R&D organization and additional persons to our G&A organization to support our FYCOMPA® commercial activities.

### *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 56% of our employees are female. At the leadership level (employees at manager and above) approximately 69% are female, and two of seven members of our C-suite are 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. In October 2021, we resumed holding in-person meeting with our sales staff for the first time since the beginning of the COVID-19 pandemic. This meeting, as with the meetings prior to the pandemic, serve to bring together and energize our staff. We continue to hold such in-person meetings as the course of the COVID-19 pandemic allows. In addition, we are always looking for new and different ways to engage our staff further as a team and individually.

## *Health, Wellness and Safety*

We are committed to the health and safety of our employees.

In March 2020, in light of worsening conditions as a result of the COVID-19 pandemic, we implemented a number of safety related initiatives among our employees, including a travel ban and a work from home policy for all employees. This included our customer-facing employees, who began working remotely and utilizing telephone and web-based technologies to provide support to patients and their healthcare providers. At present, our operations have returned to mostly being in-person, with some contact with doctors by our commercial sales force still being done remotely. Notwithstanding, the COVID-19 pandemic, including the emergence of new COVID-19 variants, including the omicron variant and subvariants, could affect the health and availability of our workforce, and we may return to a work from home policy if it is in the best interests of the health and welfare of our employees.

## **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](http://www.catalystpharma.com). The content on our website is not, nor should it be deemed to be, incorporated by reference into this report.

## **Item I.A. Risk Factors**

### **Risk Factors Summary**

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

### ***Risks related to the marketing of approved products***

- Our success depends on the successful commercialization of our products. To the extent that our drug products are not commercially successful, our business, financial condition and results of operations will be materially harmed.
- Our drug products may fail to receive the degree of market acceptance by physicians, patients, third-party payers or others in the medical community necessary for commercial success, which would negatively impact our business.
- Our strategy of seeking to acquire or in-license innovative technical platforms or earlier stage drug development programs outside of the neuromuscular disease space may not be successful.
- Our business may require additional capital.
- The ongoing COVID-19 pandemic and the worldwide attempts to contain it could harm our business and results of operations and financial condition and we could be adversely impacted by it.
- Because the target patient population for FIRDAPSE® is 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 Orphan Drug Act or successful legal challenges to the FDA's interpretation of the Orphan Drug Act may affect our ability to obtain or subsequently maintain orphan drug exclusivity or may affect the scope orphan drug exclusivity for our products.
- Our operations and relationships with healthcare providers, healthcare organizations, customers and third-party payors are subject to applicable anti-bribery, anti-kickback, fraud and abuse, transparency and other healthcare laws and regulations, which could expose us to, among other things, enforcement actions, criminal sanctions, civil penalties, contractual damages, reputational harm, administrative burdens and diminished profits and future earnings.

#### ***Risks Related to our Intellectual Property***

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

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

#### **Risk Factors**

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

## Risks related to Our Business

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

We received approval for FIRDAPSE® for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS) from the FDA in November 2018, and 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. We invest a significant amount of effort and financial resources in the commercialization of these drug products in the U.S., and, in the case of FIRDAPSE®, Canada. 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 payers’ formularies and the associated tiers;
- effectively competing with other approved or used medicines and future compounds in development;
- continued demonstration of safety and efficacy of our drug products in comparison to competing products; and
- obtaining, maintaining, enforcing, and defending intellectual property rights and claims.

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

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

- the efficacy and potential advantages compared to alternative treatments, including the convenience and ease, or duration of administration;
- the prevalence and severity of any side effects;
- the acceptability of the price of our drug products relative to other treatments;
- the content of the approved product labels and our ability to make compelling product claims;
- the effectiveness and adequacy of our and our collaboration partner’s sales and marketing efforts;
- the patients’ out-of-pocket costs in relation to alternative treatments;
- the breadth and cost of distribution support;
- the effectiveness of our patient assistance and support programs;
- the availability of third-party payer coverage and adequate reimbursement; and
- any restrictions on the use of our drug products together with other medications.

***Our business is subject to substantial competition.***

The biotechnology and pharmaceutical industries are highly competitive. Many of our competitors have substantially greater financial and other resources, larger research and development staffs and more experience developing products, obtaining FDA and other regulatory approvals of products and manufacturing and marketing products than we have. We compete against pharmaceutical companies that are developing or currently marketing therapies that will compete with us. In addition, we compete against biotechnology companies, universities, government agencies, and other research institutions in the development of pharmaceutical products. Our business could be negatively impacted if our competitors’ present or future offerings are more effective, safer or less expensive than ours, or more readily accepted by regulators, healthcare providers or third-party payors. Further, we may also compete with respect to manufacturing efficiency and marketing capabilities.

For all of these reasons, we may not be able to compete successfully.

***Our strategy of seeking to acquire or in-license innovative technical platforms or earlier stage drug development programs 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.

***The ongoing COVID-19 pandemic and the worldwide attempts to contain it could harm our business and results of operations and financial condition and we could be adversely impacted by it.***

The COVID-19 pandemic has had an impact on our business operations, and we continue to monitor applicable government modifications. We had to make modifications to our normal operations at various points in time during the pandemic, including requiring our employees to work remotely. At present, our operations have returned mostly to being in-person, with some contact with doctors by our commercial sales force still being done remotely. Notwithstanding, the COVID-19 pandemic, including the emergence of new COVID-19 variants, including the delta and omicron variants, has in the past and may in the future affect the health and availability of our workforce as well as those of third parties whom we are relying upon to take similar measures. As a result, we have previously and may in the future experience disruptions to our business operations due to the COVID-19 pandemic, and our business could be materially adversely affected by such disruptions, directly or indirectly. National, state and local governments in affected regions have implemented and may continue to implement varying safety precautions, such as quarantines, border closures, increased border controls, travel restrictions, shelter-in-place orders and shutdowns, business closures, cancellations of public gatherings and other measures. Organizations and individuals may continue to take additional steps to avoid infection, including limiting travel and staying home from work. These measures may continue to disrupt normal business operations both inside and outside of affected areas and have had significant impacts on healthcare and businesses worldwide.

***We cannot assess the impact on our business of the public concerns expressed by a vocal group of neuromuscular physicians and patients about the pricing of our product.***

We are also aware that the vocal group of neuromuscular physicians and a number of LEMS patients who have raised these issues in the past are continuing to raise concerns with the pricing of our product and with the appropriateness of the provisions in the Orphan Drug Act that grant us exclusivity for FIRDAPSE®. A few of these patients continue to say negative things about us to the media, to other patients, to the FDA, and to politicians. We cannot assess the impact of these activities on our business.

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

FIRDAPSE® targets a disease 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 FIRDAPSE® 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 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 goods sold and result in lost sales. If our suppliers were to be unable to supply us with adequate supply of our drugs, it could have a material adverse effect on our ability to successfully commercialize our drug candidates.

***We could be impacted by the viability of our suppliers.***

We source our products 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 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. 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. 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. Healthcare reform proposals recently culminated in the enactment of the Inflation Reduction Act (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 will also allow the Department of Health and Human Services (HHS) to negotiate the selling price of certain drugs and biologics that Centers for Medicare & Medicaid Services (CMS) reimburses under Medicare Part B and Part D (excluding drugs and biologics that are designated and approved for only one rare disease or condition), although only high-expenditure single-source drugs that have been approved for at least 7 years (11 years for biologics) can be selected by CMS for negotiation, with the negotiated price taking effect two years after the selection year. The negotiated prices, which will first become effective in 2026, will

be capped at a statutory ceiling price. Beginning in October 2022 for Medicare Part D and January 2023 for Medicare Part B, the IRA will also penalize drug manufacturers that increase prices of Medicare Part D and Part B drugs at a rate greater than the rate of inflation. It is unclear to what extent other statutory, regulatory, and administrative initiatives will be enacted and implemented in the future and to what extent these or any future legislation or regulations will have on our business, including market acceptance, and sales, of our products and product candidates.

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

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

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

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

We are exposed to the risk of fraud or other misconduct by our employees, sales agents or consultants. Misconduct could include failures to comply with FDA regulations, provide accurate information to the FDA, comply with manufacturing standards, comply with federal and state healthcare fraud and abuse laws and regulations, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing, and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs, and other business arrangements. Misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter such misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

## **Risks Related to Government Regulation**

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

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

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

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

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

We rely, and intend to continue to rely, on third-party suppliers and contract manufacturers to provide us with materials for our clinical trials and commercial-scale production of our products. These suppliers and manufacturers must continuously adhere to cGMP as well as any applicable corresponding manufacturing regulations outside of the 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 and other regulatory agencies. If any of our third-party suppliers or contract manufacturers fail to comply with cGMP or other applicable manufacturing regulations, our ability to develop and commercialize our products could suffer significant interruptions and delays.

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

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

If any of our contract manufacturers fail to achieve and maintain appropriate manufacturing standards, patients using our products could be injured or die, resulting in product liability claims. Even absent patient injury, we may be subject to product recalls, product seizures or withdrawals, delays or failures in testing or delivery, cost overruns, or other problems that could seriously harm our business or profitability.

***Our drug products are subject to continuing regulatory review. If we fail to comply with continuing 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 our label in the interest of patient adherence and tolerability. We cannot predict whether other legislative changes will be adopted or how such changes would affect the pharmaceutical industry generally and specifically the commercialization of FIRDAPSE® and any other products we develop.

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

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

There can be no assurance that the designation and/or exclusivity provisions currently in the law may not be changed in the future and the impact of any such changes (if made) on us. For example, the 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. 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 11<sup>th</sup> 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 11<sup>th</sup> Circuit's decision.

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

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

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

- the Federal health care program Anti-Kickback Statute, which prohibits individuals and entities from, among other things, knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under a federal and state healthcare program such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- the federal criminal and civil false claims and civil monetary penalties laws, including the federal False Claims Act, which can be imposed through civil whistleblower or qui tam actions against individuals or entities, prohibits, among other things, knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent, knowingly making, using or causing to be made or used, a false record or statement material to a false or fraudulent claim, or from knowingly making a false statement to avoid, decrease or conceal an obligation to pay



money to the federal government. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws. Moreover, the government may assert that a claim including items and services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal False Claims Act;

- HIPAA, which imposes criminal and civil liability, prohibits, among other things, knowingly and willfully executing, or attempting to execute a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services; similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- HIPAA, as amended by HITECH, which impose obligations on certain healthcare providers, health plans, and healthcare clearinghouses, known as covered entities, as well as their business associates that perform certain services involving the storage, use or disclosure of individually identifiable health information, including mandatory contractual terms, with respect to safeguarding the privacy, security, and transmission of individually identifiable health information, and require notification to affected individuals and regulatory authorities of certain breaches of security of individually identifiable health information;
- the federal legislation commonly referred to as the Physician Payments Sunshine Act, enacted as part of the ACA, and its implementing regulations, which requires certain manufacturers of covered drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children's Health Insurance Program, with certain exceptions, to report annually to CMS information related to certain payments and other transfers of value to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), physician assistants, certain types of advanced care practice nurses and teaching hospitals, as well as ownership and investment interests held by the physicians described above and their immediate family members, with the information made publicly available on a searchable website;
- the U.S. Foreign Corrupt Practices Act of 1977, as amended, which prohibits, among other things, U.S. companies and their employees and agents from authorizing, promising, offering, or providing, directly or indirectly, corrupt or improper payments or anything else of value to foreign government officials, employees of public international organizations and foreign government owned or affiliated entities, candidates for foreign political office, and foreign political parties or officials thereof;
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, that may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; and
- certain state and local laws that, among other things, require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government; require drug and therapeutic biologics manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures; require manufacturers to report price increases that exceed a statutory threshold, as well as information on the reasons for the price increase; require manufacturers to report the introduction into the market of costly drugs; require the registration of pharmaceutical sales representatives; and govern the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

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

Efforts to ensure that our current and future business arrangements with third parties comply with applicable healthcare laws and regulations could involve substantial costs. It is possible that 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 (the "USPTO") or become involved in opposition, derivation, reexamination, inter partes review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. The costs of defending our patents or enforcing our proprietary rights in post-issuance administrative proceedings and litigation can be substantial and the outcome can be uncertain. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the U.S. and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Generally, issued patents are granted a term of 20 years from the earliest claimed non-provisional filing date. In certain instances, patent term can be adjusted to recapture a portion of delay by the USPTO in examining the patent application (patent term adjustment) or extended to account for term effectively lost as a result of the FDA regulatory review period (patent term extension), or both. The scope of patent protection may also be limited. Without patent protection for our current or future product candidates, we may be open to competition from generic versions of such products. Given the amount of time required for the development, testing, and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

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

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

Also, there may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our products or product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our products or product candidates may infringe.

In addition, third parties may obtain patent rights in the future and claim that use of our technologies infringes upon rights. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our products or product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such products or product candidates unless we obtained a license under the applicable patents, or until such patents expire. Similarly, if any third-party patent were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patent may be able to block our ability to develop and commercialize the applicable products or product candidates unless we obtained a license or until such patent expires. In either case, such a license may not be available on commercially reasonable terms or at all. In addition, we may be subject to claims that we are infringing other intellectual property rights, such as trademarks or copyrights, or misappropriating the trade secrets of others, and to the extent that our employees, consultants or contractors use intellectual property or proprietary information owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

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

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

Competitors may infringe or otherwise violate our patents, the patents of our licensors or our other intellectual property rights. To counter infringement or unauthorized use, we may be required to file legal claims, which can be expensive and time consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing. The initiation of a claim against a third party may also cause the third party to bring counter claims against us such as claims asserting that our patents are invalid or unenforceable. In patent litigation in the U.S., defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, enablement, written description, or patentable subject matter. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant material information from the USPTO, or made a materially misleading statement, during prosecution.

Third parties may also raise similar validity claims before the USPTO in post-grant proceedings, such as ex parte reexaminations, inter partes review, or post-grant review, or oppositions or similar proceedings outside the U.S., in parallel with litigation or even outside the context of litigation. The outcome following legal assertions of invalidity and unenforceability is unpredictable. We cannot be certain that there is no invalidating prior art of which we and the patent examiner were unaware during prosecution. For the patents and patent applications that we have licensed, we may have limited or no right to participate in the defense of any licensed patents against challenge by a third party. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of any future patent protection on our products, or current or future product candidates. Such a loss of patent protection could harm our business.

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

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

### **General Risk Factors**

#### ***Our business may require additional capital.***

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

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

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

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

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

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



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

If we are unable to prevent such security breaches or privacy violations or implement satisfactory remedial measures, our operations could be disrupted, and we may suffer negative impact to our reputation, financial loss and be subject to regulatory fines and penalties. In addition, breaches and other unauthorized data access can be difficult to detect, and any delay in identifying them may lead to increased harm of the type described above. Moreover, the reliance on remote working technologies by our employees and third-party partners due to COVID-19 and related public health safety measures and the prevalent use of mobile devices that access confidential and personal information increases the risk of data security breaches, which could lead to the loss of confidential information, personal information, trade secrets or other intellectual property. As cyber threats continue to evolve, we may be required to expend significant additional resources to continue to modify or enhance our protective measures or to investigate and remediate any information security vulnerabilities. While we have implemented security measures to protect our data security and information technology systems, such measures may not prevent such events. Significant disruptions of our information technology systems or breaches of data security could have a material adverse effect on our business, financial condition and results of operations.

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

We are highly dependent on our executive officers and key employees, and on our Board of Directors. The loss of the services of one or more of these individuals could significantly impede the achievement of our scientific and business objectives. Other than an employment agreement with Patrick J. McEnany, our Chairman, President and Chief Executive Officer with respect to his services, 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®;
- changes in reimbursement levels;
- changes in financial estimates by securities analysts;
- actual or unanticipated variations in operating results;
- changes in laws regarding FDA approval;
- expiration or termination of licenses (particularly our License Agreement for FIRDAPSE®), research contracts, or other collaboration agreements;
- conditions or trends in the regulatory climate and the biotechnology and pharmaceutical industries;
- intellectual property, product liability or other litigation against us;
- changes in the market valuations of similar companies;
- changes in pharmaceutical company regulations or reimbursements for pharmaceutical products as a result of healthcare reform or other legislation;
- changes in economic conditions; and
- sales of shares of our common stock, particularly sales by our officers, directors and significant stockholders, or the perception that such sales may occur.

In addition, equity markets in general, and the market for emerging pharmaceutical and life sciences companies in particular, have experienced substantial price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of companies traded in those markets. Further, changes in economic conditions in the 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 March 15, 2023, we had 105,654,395 shares of our common stock outstanding, of which 6,301,895 shares were held by our executive officers and directors. We also had outstanding: (i) stock options to purchase an aggregate of 12,348,580 shares at exercise prices ranging from \$0.79 to \$21.05 per share (8,723,519 of which are currently exercisable); and (ii) restricted stock units for 594,337 shares of common stock (none of which are currently vested). Sales of shares, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock.

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

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

**Item 1B. Unresolved Staff Comments**

None.

**Item 2. Properties**

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

**Item 3. Legal Proceedings**

*Paragraph IV Patent Litigation*

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 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 Federal Food, Drug, and Cosmetic Act, 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 commence patent infringement lawsuits against these generic drug manufacturers in a federal district court to trigger a stay precluding FDA from approving any ANDA 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. Federal District Court for the District of New Jersey against each of the three generic drug manufacturers who notified us of their ANDA filings.

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 for the life of our patents.

#### *Canadian Litigation*

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 available for sale in Canada.

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

#### *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**

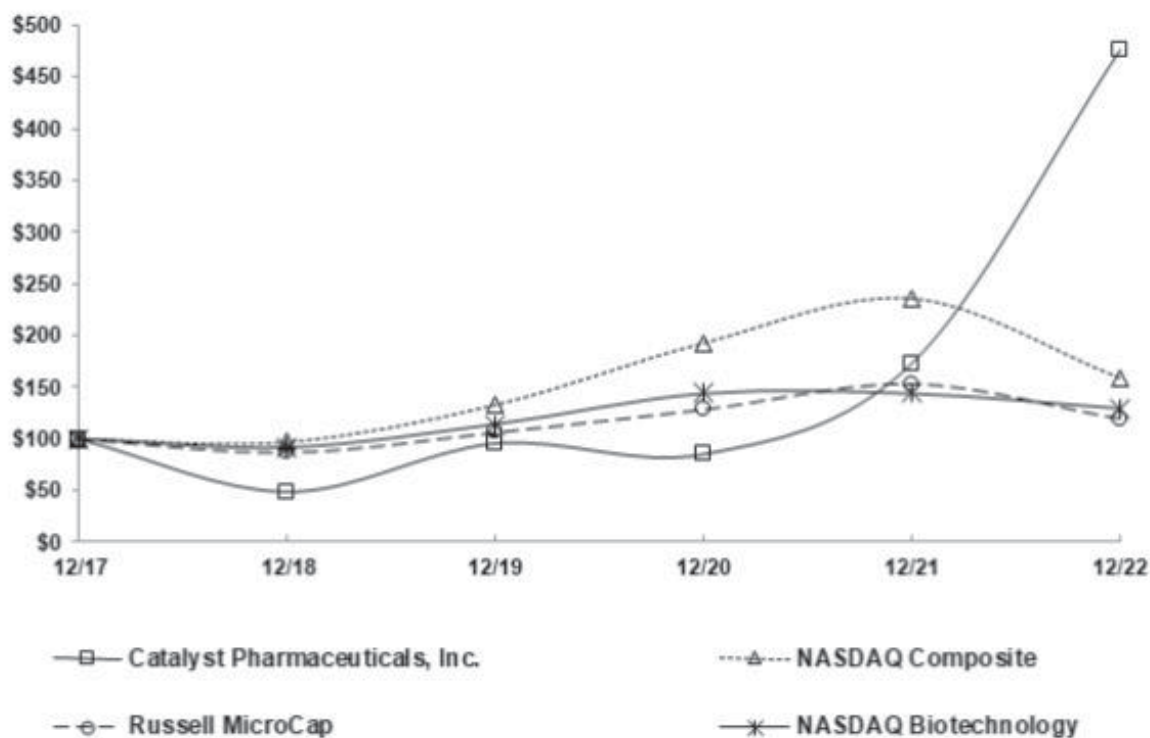
#### **Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities**

##### **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 12/31/2017 to 12/31/2022.

## COMPARISON OF 5 YEAR CUMULATIVE TOTAL RETURN\*

Among Catalyst Pharmaceuticals, Inc., the NASDAQ Composite Index, the Russell MicroCap Index and the NASDAQ Biotechnology Index



\*\$100 invested on 12/31/17 in stock or index, including reinvestment of dividends.  
Fiscal year ending December 31.

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Catalyst Pharmaceuticals, Inc.  
NASDAQ Composite  
Russell MicroCap  
NASDAQ Biotechnology

	12/17	12/18	12/19	12/20	12/21	12/22
Catalyst Pharmaceuticals, Inc.	100.00	49.10	95.91	85.42	173.15	475.70
NASDAQ Composite	100.00	97.16	132.81	192.47	235.15	158.65
Russell MicroCap	100.00	86.92	106.42	128.72	153.61	119.88
NASDAQ Biotechnology	100.00	91.14	114.02	144.15	144.18	129.59

*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 March 13, 2023 was \$14.63. As of March 13, 2023, there were 32 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, finance the growth and development of our business, and repurchase up to \$21 million of our common stock. 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, 2022 with respect to compensation plans under which shares of our common stock may be issued.

Plan Category	Equity Compensation Plan Information		
	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)	12,309,108	\$ 4.93	2,691,791(2)
Equity compensation plans not approved by security holders	—	—	—
<b>Total</b>	<b>12,309,108</b>	<b>\$ 4.93</b>	<b>2,691,791</b>

(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.

The following table presents information regarding repurchases by us of our common stock under the Share Repurchase Program during the three months ended December 31, 2022:

Period	Total Number of Shares Purchased	Average Price Paid Per Share	Total Number of Shares Purchased as Part of Publicly Announced Program	Dollar Value of Shares that May Yet Be Purchased (in thousands)
October 1 – October 31, 2022	—	\$ —	—	\$ 21,003
November 1 – November 30, 2022	—	\$ —	—	\$ 21,003
December 1 – December 31, 2022	—	\$ —	—	\$ 21,003
<b>Total</b>	<b>—</b>	<b>—</b>	<b>—</b>	<b>—</b>



## 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 2022 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 diseases and diseases that are difficult to treat. With exceptional patient focus, we are committed to developing a robust pipeline of cutting-edge, best-in-class medicines for treating rare and difficult to treat diseases. We are dedicated to making a meaningful impact on the lives of those suffering from rare and difficult to treat diseases, and we believe in putting patients first in everything we do.

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. On December 17, 2022, we entered into an agreement with Eisai Inc. ("Eisai") for the acquisition of the United States rights to FYCOMPA®(perampanel) CIII, 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. We closed that acquisition on January 24, 2023 and we are now marketing FYCOMPA® in the United States.

### Impact of the COVID-19 pandemic on our business

The COVID-19 pandemic affected our business operations in numerous ways. At various times during the pandemic, we had to make modifications to our normal operations, including allowing our employees to work remotely. Further, during the pandemic, national, state and local governments in affected regions implemented varying safety precautions, such as quarantines, border closures, increased border controls, travel restrictions, shelter-in-place orders and shutdowns, business closures, cancellations of public gatherings, mask mandates, and other measures. While most of these measures have since been relaxed or removed, a resurgence in cases as a result of one or more new variants could lead to some or all of these precautions being put back into place. At present, our operations have returned to mostly being in-person, with some contact with physicians by our commercial sales force still being done remotely. However, there can be no assurance that the COVID-19 pandemic will not in the future disrupt once again our normal business operations.

## **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 Lambert-Eaton myasthenic syndrome, or 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 27 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 six medical science liaisons who are helping educate the medical communities and patients about LEMS and our programs supporting patients and access to FIRDAPSE®.

Additionally, we have contracted with an experienced inside sales agency that works to generate leads through telemarketing to targeted physicians. This inside sales agency allows 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®. 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 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 product and services to potential patients and their healthcare providers. We also work with several rare disease advocacy organizations (including Global Genes 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 LEMS patients 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 \$10.00 per month (currently less than \$2.00 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. However, we are donating, and committing to continue to donate, money to qualified, independent charitable foundations dedicated to providing assistance to any U.S. LEMS patients in financial need. 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 three 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 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 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 commence patent infringement lawsuits against these generic drug manufacturers in a federal district court to trigger a stay precluding FDA from approving any ANDA 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.

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 for the life of our patents.

## **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 4 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.

Pursuant to the Asset Purchase Agreement, which closed on January 24, 2023, 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® is approved by the U.S. Patent and Trademark Office (USPTO). 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 and a Supply Agreement. Under the Transition Services Agreement, a U.S. subsidiary of Eisai is providing us with certain transitional services, and under the Supply Agreement, Eisai has 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). Following the closing of the acquisition, we are currently marketing FYCOMPA® in the U.S. through Eisai under the Transition Services Agreement as we build our FYCOMPA® marketing and sales team, and we expect to take over the marketing program in May 2023. In that regard, we currently expect to hire approximately 34 sales and marketing personnel to support FYCOMPA®, many of whom previously worked in Eisai’s U.S. sales division marketing FYCOMPA®. We also are planning on hiring up to six medical science liaisons to help us educate the medical community who treat epilepsy and the patients who have epilepsy about their disease and the benefits of 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). Eligible cash-paying patients receive up to \$60 towards each prescription, up to a maximum of \$720 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 will expire no earlier than May 23, 2025, the current expiration date of U.S. patent no. 6,949,571 including the USPTO’s patent term extension calculation. A request for reconsideration of the agency’s patent term extension calculation is currently pending. If successful, we would be entitled to patent term extension that would extend U.S. patent no. 6,949,571 until June 8, 2026. There can be no assurance that our request for reconsideration will be granted by the U.S. Patent and Trademark Office.

## ***Business Development***

We are continuing our efforts to broaden and diversify our product portfolio through acquisitions of early and/or late-stage products or companies or technology platforms in rare disease and CNS therapeutic categories. To accomplish these priorities, we are continuing to employ a disciplined approach to evaluating assets, and we believe that this strategic expansion will better position our company long term to build out a broader more diversified portfolio of drug candidates (which should add greater value to our company over the near and long-term). In that regard, we are currently exploring several additional potential opportunities to acquire companies with commercial drug products and/or drug products in development or to in-license or acquire commercialized drug products or drug products in development. However, 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.

## **Capital Resources**

At December 31, 2022, we had cash and investments of approximately \$298 million. Subsequent to the end of 2022, on January 24, 2023 we used \$162 million of our available cash and cash equivalents to fund our acquisition of FYCOMPA® and to reimburse Eisai for certain prepaid expenses. 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®, that our commercialization of FYCOMPA® 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.

## **Basis of Presentation**

### *Revenues.*

During the fiscal year ended December 31, 2022, we continued to generate revenues from product sales of FIRDAPSE® in the U.S. We expect these revenues to fluctuate in future periods based on our sales of FIRDAPSE®. We received approval from Health Canada on July 31, 2020, for FIRDAPSE® for the symptomatic treatment of LEMS and as of December 31, 2020, we had launched FIRDAPSE® in Canada. During the fiscal year ended December 31, 2022, 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, 2022, we did not generate revenues under our collaborative agreement with Endo. We expect our revenues from the Endo collaborative agreement to fluctuate in future periods based on our collaborator’s ability to meet various regulatory milestones set forth in such agreement.

For the fiscal year ended December 31, 2022, we generated revenues of approximately \$0.5 million from our agreement with DyDo Pharma. 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 FIRDAPSE®. 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 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. To date, 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®.

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®.

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, information technology, accounting, and consulting services.

### *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 (benefit) over our income before income taxes.

We incurred operating losses from inception through the three-month period ended March 31, 2019. As of December 31, 2022, 2021 and 2020, respectively, we had federal net operating loss carry-forwards of approximately \$0, \$0 and \$3 million. Additionally, we had state net operating loss carry-forwards of approximately \$0, \$28 million and \$42 million, respectively, available to reduce future Florida taxable income for the years ended December 31, 2022, 2021 and 2020.

In the third quarter of 2020, we determined that there was sufficient positive evidence to conclude that it is more likely than not that our additional deferred taxes of approximately \$33 million are realizable. As a result, we reduced the valuation allowance accordingly.

### *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, 2022 and 2021 and, therefore, the transaction price was not reduced further during the years ended December 31, 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, 2022 and 2021, the assumptions used were an estimated annual volatility of 69.2% and 70.0%, expected holding periods of primarily four and a half years, and risk-free interest rates of 1.27% to 4.07% and 0.34% to 1.18%, respectively.

#### *Valuation Allowance for Deferred Tax Assets.*

We assess the need for a valuation allowance against our deferred tax asset each quarter through the review of all available positive and negative evidence. Deferred tax assets are reduced by a tax valuation allowance when, in the opinion of management, it is more likely than not that some portion of the deferred tax assets will not be realized. Management’s analysis depends on historical and projected taxable income. Projected taxable income includes significant assumptions related to revenue, commercial expenses and research and development activities. In the third quarter of 2020, we determined that there was sufficient positive evidence to conclude that it is more likely than not that our additional deferred taxes are realizable. As a result, we reduced the valuation allowance accordingly.

## **Results of Operations**

### ***Years Ended December 31, 2022 and 2021***

#### ***Revenues.***

For the year ended December 31, 2022, we recognized \$213.9 million in net revenue from product sales of FIRDAPSE® primarily in the U.S. compared to \$138.0 million for the year ended December 31, 2021. The increase of approximately \$75.9 million was due to increases in sales volumes of approximately 49% (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. For the year ended December 31, 2022, we also recognized \$0.3 million in license and other revenue, as compared to \$2.8 million during the year ended December 31, 2021. The decrease was primarily due to our license agreement with DyDo Pharma for the commercialization of FIRDAPSE® in Japan that was signed in 2021.

#### *Cost of Sales.*

Cost of sales was approximately \$34.4 million for the year ended December 31, 2022, compared to \$21.9 million for the year ended December 31, 2021. Cost of sales in both periods consisted principally of royalty payments, which are based on net revenue as defined in the applicable license agreement. 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.

#### *Research and Development Expenses.*

Research and development expenses for the years ended December 31, 2022 and 2021 were approximately \$19.8 million and \$16.9 million, respectively, and represented approximately 18% and 19% of total operating costs and expenses, respectively. Research and development expenses for the years ended December 31, 2022 and 2021 were as follows (in thousands):

	For the year ended December 31,		Change	
	2022	2021	\$	%
Research and development expenses	\$ 18,060	\$ 15,325	2,735	17.8
Employee stock-based compensation	1,729	1,611	118	7.3
Total research and development expenses	<u>\$ 19,789</u>	<u>\$ 16,936</u>	<u>2,853</u>	<u>16.8</u>

Research and development expenses increased approximately \$2.9 million during year ended December 31, 2022 when compared to the same period in 2021. The increase is primarily due to the acquisition of RUZURGI® inventory previously manufactured by Jacobus on July 11, 2022 of approximately \$4.1 million, which was expensed in full in the third quarter of 2022 and is not a recurring expense. This was partially offset by an overall decrease in research and development activity. Further, for the year ended December 31, 2022, research and development expenses included costs relating to closing out sites for both the MuSK-MG clinical trial and our previously operated expanded access program. Research and development costs in the 2021 period included expenses relating to medical and regulatory affairs, our previously operated expanded access program, and our efforts to develop a long-acting formulation of amifampridine phosphate (which efforts have been discontinued).

We expect that research and development expenses will continue to be substantial in 2023 and beyond as we execute on our strategic initiative to acquire or in-license innovative technology platforms and/or earlier stage programs in rare disease categories outside of neuromuscular diseases.

#### *Selling, General and Administrative Expenses.*

Selling, general and administrative expenses for the years ended December 31, 2022 and 2021 were approximately \$58.2 million and \$49.6 million, respectively, and represented approximately 52% and 56% of total operating costs and expenses for the years ended December 31, 2022, and 2021, respectively. Selling, general and administrative expenses for the years ended December 31, 2022 and 2021 were as follows (in thousands):

	For the year ended December 31,		Change	
	2022	2021	\$	%
Selling	\$ 29,469	\$ 26,151	3,318	12.7
General and administrative	22,536	19,015	3,521	18.5
Employee stock-based compensation	6,178	4,462	1,716	38.5
Total selling, general and administrative expenses	<u>\$ 58,183</u>	<u>\$ 49,628</u>	<u>8,555</u>	<u>17.2</u>

For the year ended December 31, 2022, selling, general and administrative expenses increased approximately \$8.6 million when compared to the same period in 2021. The increase for the year ended December 31, 2022 was primarily attributable to the timing of our commitments to make contributions to 501(c)(3) organizations supporting LEMS patients of approximately \$1.8 million, increases of sales commissions due to higher sales volume of approximately \$2.1 million, approximately \$3.1 million increase in employee compensation related to annual merit increases, approximately \$1.1 million increase in amortization expense related to intangibles acquired in connection with the acquisition of RUZURGI® and an increase in stock-based compensation expense due to an increase in the average share price.

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®, begin our efforts to market FYCOMPA®, and take steps to continue to expand our business.

#### *Stock-Based Compensation.*

Total stock-based compensation for the years ended December 31, 2022 and 2021 was \$7.9 million and \$6.1 million, respectively. In 2022 and 2021, 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 our investment of our cash and cash equivalents and investments of \$2.9 million and \$0.3 million for the years ended December 31, 2022 and 2021, respectively, which includes realized losses from the sale of available-for-sale securities of \$0.8 million and \$0 million, respectively. The increase in other income, net for the year ended December 31, 2022 of approximately \$2.6 million when compared to the same period in 2021 is primarily due to higher yields on investments as well as higher invested balances. Other income, net, consists primarily of interest and dividend income.

#### *Income Taxes.*

As of December 31, 2022 and 2021, respectively, we had state net operating loss carryforwards of approximately \$0 million and \$28 million, respectively, available to reduce future Florida taxable income. We had no uncertain tax positions as of December 31, 2022 and December 31, 2021.

Our effective income tax rate was 20.7% and 25.0%, respectively, for fiscal year 2022 and fiscal year 2021. The difference in the effective rates between periods is driven by the stock compensation windfall tax benefit due to an increase in the number of awards exercised in the current period. Differences in the effective tax and the statutory federal income tax rate of 21% are driven by state income taxes and anticipated annual permanent differences, and offset by the orphan drug credit claimed. The 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 2022).

#### *Net Income.*

Our net income was approximately \$83.1 million in the year ended December 31, 2022 (\$0.80 per basic and \$0.75 per diluted share) as compared to \$39.5 million in the year ended December 31, 2021 (\$0.38 per basic and \$0.37 per diluted share).

#### ***Years Ended December 31, 2021 and 2020***

The information comparing results of operations for the year ended 2021 compared to 2020 was included in our Annual Report on Form 10-K for 2021.

#### **Liquidity and Capital Resources**

Since our inception, we have financed our operations primarily through multiple offerings of our securities and, since January 2019, from revenues from product sales of FIRDAPSE®. At December 31, 2022 we had cash and cash equivalents aggregating \$298.4 million and working capital of \$263.2 million. At December 31, 2021, we had cash and cash equivalents and investments aggregating \$191.3 million and working capital of \$183.0 million. At December 31, 2022, 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, short-term interest bearing obligations 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® 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 when it is required.

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

- the scope, rate of progress and cost of our clinical trials and other product development activities;
- the cost of diligence in seeking a potential acquisition and of the completion of such acquisition, if an acquisition so occurs;
- future clinical trial results;
- 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 level of revenues that we report from sales of FIRDAPSE® and FYCOMPA®;
- 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 July 23, 2020, we 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. As of the date of this report, no offerings have been completed under the 2020 Shelf Registration Statement.

#### *Cash Flows.*

Net cash provided by operating activities was \$116.0 million and \$60.4 million, respectively, for the years ended December 31, 2022 and 2021. 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 deposits and a decrease of \$0.3 million in operating lease liability. During the year ended December 31, 2021, net cash provided by operating activities was primarily attributable to our net income of \$39.5 million, a decrease of \$4.0 million in prepaid expenses and other current assets and deposits, increases of \$5.5 million in accrued expenses and other liabilities, \$0.9 million in operating lease liability, \$9.3 million in deferred taxes and of \$6.6 million of non-cash expenses. This was partially offset by increases of \$0.6 million in accounts receivable, net and \$3.2 million in inventory and a decrease of \$1.5 million in accounts payable.

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 payment in connection with license agreement of \$10.0 million. Net cash used in investing activities was \$11.0 million for the year ended December 31, 2021, consisting primarily of purchases of investments.

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 options to purchase shares of common stock, partially offset by repurchases of common stock. Net cash used in financing activities during the year ended December 31, 2021 was \$8.1 million, consisting primarily of repurchases of common stock, partially offset by proceeds from the exercise of options to purchase shares 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 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 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, 2022, we recognized an aggregate of approximately \$32.1 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 under our original license agreement for North America.

- *Payments due to Jacobus.* In connection with its recent settlement with Jacobus, Catalyst has 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 the balance of which will be paid over the next two years, on the first and 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.

Royalties will be trued up at the end of the year to the extent that royalties on net sales are below the minimum royalty.

For the year ended December 31, 2022, we recognized an aggregate of approximately \$1.6 million of royalties payable to Jacobus.

We also have entered into the following contractual arrangements:

- *Employment agreements.* We have entered into an employment agreement with our Chief Executive Officer that required us to make base salary payments of approximately \$0.7 million in 2022. The agreement expires in November 2024.
- *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 2023.
- *Lease for office space.* We operate our business in leased office space in Coral Gables, Florida. We entered into an agreement in May 2020 that amended our lease for office facilities. Under the amended lease, our leased space increased from approximately 7,800 square feet of office space to approximately 10,700 square feet of office space. We moved into the new space around March 1, 2021 when the space became available for use. 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® and FYCOMPA® are highly uncertain. Factors that will affect our success include the uncertainty of:

- The impact of the COVID-19 pandemic on our business or on the economy generally;
- Whether we will be able to continue to successfully market FIRDAPSE® and now successfully market 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 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 the daily dose of FIRDAPSE® taken by patients changes over time and affects our results of operations;
- Whether new FIRDAPSE® patients and FYCOMPA® patients can be successfully titrated to stable therapy;
- Whether we can continue to market FIRDAPSE® and now market FYCOMPA® on a profitable and cash flow positive basis;
- Whether we can successfully integrate the team that we are hiring to market FYCOMPA® into our current business structure;
- Whether the acquisition of FYCOMPA® will prove to be accretive to EBITDA and EPS in 2023;
- Whether any revenue or earnings guidance that we provide to the public market will turn out to be accurate;
- Whether payors will reimburse for our products at the price that we charge for our products;
- 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 FIRDAPSE® and FYCOMPA®;
- Our ability to maintain compliance with the applicable rules that relate to our contributions to 501(c)(3) organizations that support LEMS patients;
- 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® or FYCOMPA®;
- Our ability to obtain a favorable decision on our pending request for reconsideration for an extension of the expiration date of patent protection for one of our patents listed in the Orange Book for FYCOMPA®;
- 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 the research and 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® after our orphan drug exclusivity for FIRDAPSE® expires;
- Whether we will be successful in our litigation to enforce our patents against the Paragraph IV challengers who have filed relating to FIRDAPSE®;

- The impact on our profits and cash flow of adverse changes in reimbursement and coverage policies 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;
- The state of the economy generally and its impact on our business;
- The potential impact of future healthcare reform in the United States, including the Inflation Reduction Act of 2022, and measures being taken worldwide designed to reduce healthcare costs and limit the overall level of government expenditures, including the impact of pricing actions and reduced reimbursement for our product;
- 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 our collaboration partner in Japan, DyDo, will successfully complete the clinical trial in Japan that will be required to seek approval to commercialize FIRDAPSE® in Japan;
- Whether DyDo will be able to obtain approval to commercialize FIRDAPSE® in Japan; and
- Whether our version of vigabatrin tablets will ever be approved by the FDA and successfully marketed by Endo, whether we will earn milestone payments or royalties on sales of our version of generic vigabatrin tablets, and whether Endo's bankruptcy filing will impact these issues.

Our current plans and objectives are based on assumptions relating to the continued commercialization of FIRDAPSE® and FYCOMPA® 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, 2022, 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, 2022 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, 2022.

During the fourth quarter of 2022, 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 2023 Annual Meeting of Stockholders. Our Proxy Statement for the 2023 Annual Meeting of Stockholders is expected to be filed not later than 120 days after the end of our fiscal year ended December 31, 2022 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](http://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, 2022 and 2021.

Consolidated Statements of Operations and Comprehensive Income for the years ended December 31, 2022, 2021 and 2020.

Consolidated Statements of Changes in Stockholders' Equity for the years ended December 31, 2022, 2021 and 2020.

Consolidated Statements of Cash Flows for the years ended December 31, 2022, 2021 and 2020.

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.

Exhibit Number	Description of Exhibit	Incorporated by Reference				Filed Herewith
		Form	File Number	Date of Filing	Exhibit Number	
2.1	<u>Agreement and Plan of Merger, dated August 14, 2006, between the Company and Catalyst Pharmaceutical Partners, Inc., a Florida corporation</u>	S-1	333-136039	9/1/2006	10.9	
2.2	<u>Asset Purchase Agreement by and between Eisai Co., Ltd. and the Company, dated as of December 17, 2022</u>	8-K	001-33057	12/22/2022	2.1	
3.1	<u>Certificate of Incorporation</u>	S-1	333-136039	7/25/2006	3.1	
3.2	<u>Amendment to Certificate of Incorporation</u>	S-1	333-136039	7/25/2006	3.2	
3.3	<u>Amendment to Certificate of Incorporation</u>	DEF 14A	001-33057	3/30/2015	Annex A	
3.4	<u>Amendment to Certificate of Incorporation</u>	8-K	001-33057	8/21/2020	3.1	
3.5	<u>By-Laws</u>	S-1	333-136039	9/1/2006	3.3	

Exhibit Number	Description of Exhibit	Incorporated by Reference				Filed Herewith
		Form	File Number	Date of Filing	Exhibit Number	
3.6	<u>Amendment to By-Laws</u>	8-K	001-33057	11/27/2019	3.1	
4.1	<u>Specimen Stock Certificate for Common Stock</u>	S-1	333-136039	9/1/2006	4.1	
4.2	<u>Description of the Company's Capital Stock</u>	10-K	001-33057	3/16/2022	4.5	
10.1(a)+	<u>Employment Agreement between the Company and Patrick J. McEnany</u>	10-Q	001-33057	12/15/2006	10.1	
10.1(b)+	<u>First Amendment to Employment Agreement between the Company and Patrick J. McEnany</u>	8-K	001-33057	12/23/2008	10.1	
10.1(c)+	<u>Second Amendment to Employment Agreement between the Company and Patrick J. McEnany</u>	10-Q	001-33057	11/12/2009	10.1	
10.1(d)+	<u>Third Amendment to Employment Agreement between the Company and Patrick J. McEnany</u>	8-K	001-33057	9/15/2011	10.1	
10.1(e)+	<u>Fourth Amendment to Employment Agreement between the Company and Patrick J. McEnany</u>	8-K	001-33057	8/29/2013	10.1	
10.1(f)+	<u>Fifth Amendment to Employment Agreement between the Company and Patrick J. McEnany</u>	8-K	001-33057	6/24/2016	10.1	
10.1(g)+	<u>Sixth Amendment to Employment Agreement between the Company and Patrick J. McEnany</u>	8-K	001-33057	5/31/2018	10.1	
10.1(h)+	<u>Seventh Amendment to Employment Agreement between the Company and Patrick J. McEnany</u>	8-K	001-33057	9/11/2020	10.1	
10.1(i)+	<u>Eighth Amendment to Employment Agreement between the Company and Patrick J. McEnany</u>	8-K	001-33057	9/9/2022	10.1	
10.2(a)+	<u>2014 Stock Incentive Plan</u>	DEF 14A	001-33057	3/19/2014	Annex A	
10.2(b)+	<u>Amendment No. 1 to 2014 Stock Incentive Plan</u>	DEF 14A	001-33057	4/29/2016	Annex A	
10.2(c)+	<u>Amendment No. 2 to 2014 Stock Incentive Plan</u>	DEF 14A	001-33057	4/14/2017	Annex A	
10.3(a)+	<u>2018 Stock Incentive Plan</u>	DEF 14A	001-33057	4/17/2018	Annex A	
10.3(b)+	<u>Amendment No. 1 to 2018 Stock Incentive Plan</u>	DEF 14A	001-33057	7/7/2020	Annex A	
10.3(c)+	<u>Amendment No. 2 to 2018 Stock Incentive Plan</u>	DEF 14A	001-33057	10/25/2021	Annex A	
10.4(a)	<u>Lease Agreement between the Company and 355 Alhambra Plaza, Ltd.</u>	10-Q	001-33057	5/14/2007	10.1	
10.4(b)	<u>First Amendment to Lease Agreement between the Company and CPT 355 Alhambra Circle, LLC</u>	10-Q	001-33057	8/15/2011	10.1	
10.4(c)	<u>Second Amendment to Lease Agreement between the Company and CPT 355 Alhambra Circle, LLC</u>	8-K	001-33057	2/20/2014	10.1	
10.4(d)	<u>Third Amendment to Lease Agreement between the Company and CPT 355 Alhambra Circle, LLC</u>	8-K	001-33057	3/27/2015	10.1	
10.4(e)	<u>Fourth Amendment to Lease Agreement between the Company and PRII 355 Alhambra Circle, LLC</u>	8-K	001-33057	8/17/2018	10.1	
10.4(f)	<u>Fifth Amendment to Lease Agreement between the Company and PRII 355 Alhambra Circle, LLC</u>	8-K	001-33057	5/13/2020	10.1	
10.5	<u>License Agreement, dated as of December 13, 2011, among New York University, the Feinstein Institute for Medical Research, and the Company</u>	10-K	001-33057	3/30/2012	10.15	
10.6(a)	<u>License Agreement, dated as of October 26, 2012, between the Company and BioMarin</u>	8-K	001-33057	10/31/2012	10.2	



Exhibit Number	Description of Exhibit	Incorporated by Reference				Filed Herewith
		Form	File Number	Date of Filing	Exhibit Number	
10.6(b)	<u>Amendment No. 1 to License Agreement, dated as of April 8, 2014, between the Company and BioMarin</u>	8-K	001-33057	4/17/2014	10.1	
10.6(c)	<u>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</u>	10-Q	001-33057	8/17/2018	10.1	
10.6(d)	<u>Second Amendment to License Agreement, dated May 29, 2019, between the Company and BioMarin</u>	8-K	001-33057	5/30/2019	10.1	
10.7	<u>Development, License and Commercialization Agreement, dated effective as of December 18, 2018, by and between Endo Ventures Limited and the Company</u>	8-K	001-33057	12/26/2018	10.1	
10.8	<u>License and Supply Agreement, dated as of August 14, 2020, by and between KYE Pharmaceuticals, Inc. and the Company</u>	8-K	001-33057	8/20/2020	10.1	
10.9	<u>License and Supply Agreement, dated as of June 28, 2021, by and between DyDo Pharma, Inc. and the Company</u>	8-K	001-33057	6/28/2021	10.1	
10.10(a)	<u>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</u>	8-K	001-33057	7/12/2022	10.1	
10.10(b)	<u>License and Asset Purchase Agreement, dated as of July 11, 2022, by and between Jacobus Pharmaceutical Company, Inc. and the Company</u>	8-K	001-33057	7/12/2022	10.2	
10.11(a)	<u>Transition Services Agreement between Eisai, Inc. and the Company</u>	8-K	001-33057	12/22/2022	10.1	
10.11(b)	<u>Supply Agreement between Eisai Co., Ltd. and the Company</u>	8-K	001-33057	12/22/2022	10.2	
21.1	<u>Subsidiaries of the Registrant</u>	10-K	001-33057	3/16/2020	21.1	
23.1	<u>Consent of Independent Registered Public Accounting Firm</u>					X
31.1	<u>Section 302 CEO Certification</u>					X
31.2	<u>Section 302 CFO Certification</u>					X
32.1	<u>Section 906 CEO Certification</u>					X
32.2	<u>Section 906 CFO Certification</u>					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)					

## 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 15<sup>th</sup> day of March, 2023.

### CATALYST PHARMACEUTICALS, INC.

By: /s/ Patrick J. McEnany

Patrick J. McEnany, Chairman,  
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
<u>/s/ Patrick J. McEnany</u> Patrick J. McEnany	Chairman of the Board of Directors, President and Chief Executive Officer (Principal Executive Officer)	March 15, 2023
<u>/s/ Alicia Grande</u> Alicia Grande	Vice President, Treasurer, Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	March 15, 2023
<u>/s/ Charles B. O'Keeffe</u> Charles B. O'Keeffe	Director	March 15, 2023
<u>/s/ Philip H. Coelho</u> Philip H. Coelho	Director	March 15, 2023
<u>/s/ David S. Tierney, M.D.</u> David S. Tierney, M.D.	Director	March 15, 2023
<u>/s/ Donald A. Denkhaus</u> Donald A. Denkhaus	Director	March 15, 2023
<u>/s/ Richard Daly</u> Richard Daly	Director	March 15, 2023
<u>/s/ Molly Harper</u> Molly Harper	Director	March 15, 2023

## INDEX TO FINANCIAL STATEMENTS

**Years ended December 31, 2022, 2021 and 2020**

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## 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, 2022, 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, 2022, 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, 2022, and our report dated March 15, 2023 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  
March 15, 2023

## REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

Board of Directors and Stockholders  
Catalyst Pharmaceuticals, Inc.

### 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, 2022 and 2021, the related consolidated statements of operations and comprehensive income, changes in stockholders’ equity, and cash flows for each of the three years in the period ended December 31, 2022, 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, 2022 and 2021, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2022, 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, 2022, 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 March 15, 2023 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 matters

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.

#### *Accounting for the asset acquisition*

As described further in Notes 12 and 13 to the financial statements, during the year ended December 31, 2022, the Company completed the acquisition of research and development inventory and related intangible assets for total consideration of approximately \$37.7 million. The transaction was accounted for as an asset acquisition, and as such, the total consideration was allocated to the acquired assets based upon their relative fair values. We identified the accounting for the asset acquisition as a critical audit matter.

The principal consideration for our determination that the accounting for the asset acquisition is a critical audit matter is that the interpretation and application of the relevant accounting literature required significant auditor judgment. Specifically, the accounting for the transaction as an asset acquisition versus a business combination, and the accounting for the particular terms of the contingent consideration.

Our audit procedures related to the accounting for the asset acquisition included the following, among others. We obtained an understanding of the internal controls and processes in place over management’s process that related to the recording of the asset acquisition. We evaluated the Company’s accounting memoranda and other documentation, including application of the relevant accounting guidance. We compared the underlying terms of the License and Asset Purchase Agreement (dated July 11, 2022) to the Company’s accounting memoranda; and with the assistance of our internal subject matter experts, independently interpreted and applied the accounting literature to the transaction, considering alternative accounting treatments and evaluating the relative merits of the possible alternatives.

/s/ GRANT THORNTON LLP

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

Miami, Florida  
March 15, 2023



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

	<u>December 31, 2022</u>	<u>December 31, 2021</u>
<b>ASSETS</b>		
Current Assets:		
Cash and cash equivalents	\$ 298,395	\$ 171,445
Short-term investments	—	19,821
Accounts receivable, net	10,439	6,619
Inventory	6,805	7,870
Prepaid expenses and other current assets	5,158	4,351
Total current assets	320,797	210,106
Operating lease right-of-use asset	2,770	3,017
Property and equipment, net	847	959
License and acquired intangibles, net	32,471	—
Deferred tax assets, net	18,736	23,697
Deposits	9	9
Total assets	<u>\$ 375,630</u>	<u>\$ 237,788</u>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current Liabilities:		
Accounts payable	\$ 3,975	\$ 2,768
Accrued expenses and other liabilities	53,613	24,295
Total current liabilities	57,588	27,063
Operating lease liability, net of current portion	3,557	3,894
Other non-current liabilities	14,064	—
Total liabilities	75,209	30,957
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, 2022 and 2021	—	—
Common stock, \$0.001 par value, 200,000,000 shares authorized; 105,263,031 shares and 102,992,913 shares issued and outstanding at December 31, 2022 and 2021, respectively	105	103
Additional paid-in capital	250,430	233,186
Retained earnings (accumulated deficit)	49,862	(26,310)
Accumulated other comprehensive income (loss)	24	(148)
Total stockholders' equity	300,421	206,831
Total liabilities and stockholders' equity	<u>\$ 375,630</u>	<u>\$ 237,788</u>

**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,		
	2022	2021	2020
Revenues:			
Product revenue, net	\$ 213,938	\$ 137,997	\$ 118,790
License and other revenue	265	2,836	283
Total revenues	<u>214,203</u>	<u>140,833</u>	<u>119,073</u>
Operating costs and expenses:			
Cost of sales	34,393	21,884	17,039
Research and development	19,789	16,936	16,497
Selling, general and administrative	58,183	49,628	44,234
Total operating costs and expenses	<u>112,365</u>	<u>88,448</u>	<u>77,770</u>
Operating income	101,838	52,385	41,303
Other income, net	2,881	282	587
Net income before income taxes	104,719	52,667	41,890
Income tax provision (benefit)	21,640	13,185	(33,093)
Net income	<u>\$ 83,079</u>	<u>\$ 39,482</u>	<u>\$ 74,983</u>
Net income per share:			
Basic	<u>\$ 0.80</u>	<u>\$ 0.38</u>	<u>\$ 0.72</u>
Diluted	<u>\$ 0.75</u>	<u>\$ 0.37</u>	<u>\$ 0.71</u>
Weighted average shares outstanding:			
Basic	<u>103,374,606</u>	<u>103,379,349</u>	<u>103,512,913</u>
Diluted	<u>111,375,631</u>	<u>107,795,585</u>	<u>106,242,273</u>
Net income	\$ 83,079	\$ 39,482	\$ 74,983
Other comprehensive income:			
Unrealized gain (loss) on available-for-sale securities, net of tax of (\$54), \$46 and \$0, respectively	172	(179)	22
Comprehensive income	<u>\$ 83,251</u>	<u>\$ 39,303</u>	<u>\$ 75,005</u>

**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, 2022, 2021 and 2020**  
*(in thousands)*

	Preferred Stock	Common Stock	Additional Paid-in Capital	Retained Earnings (Accumulated Deficit)	Accumulated Other Comprehensive Gain (Loss)	Total
	Shares	Amount				
<b>Balance at December 31, 2019</b>	\$ —	103,397	\$ 104	\$216,205	\$ (128,688)	\$ 87,630
Issuance of stock options for services	—	—	—	5,694	—	5,694
Exercise of stock options for common stock	—	282	—	758	—	758
Amortization of restricted stock for services	—	—	—	567	—	567
Issuance of common stock upon vesting of restricted stock units, net	—	103	—	(56)	—	(56)
Other comprehensive gain (loss)	—	—	—	—	22	22
Net income	—	—	—	—	74,983	74,983
<b>Balance at December 31, 2020</b>	—	103,782	104	223,168	(53,705)	169,598
Issuance of stock options for services	—	—	—	5,550	—	5,550
Exercise of stock options for common stock	—	1,328	1	4,098	—	4,099
Amortization of restricted stock for services	—	—	—	523	—	523
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
<b>Balance at December 31, 2021</b>	—	102,993	103	233,186	(26,310)	206,831
Issuance of stock options for services	—	—	—	6,346	—	6,346
Exercise of stock options for common stock	—	3,172	2	9,567	—	9,569
Amortization of restricted stock for services	—	—	—	1,561	—	1,561
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
<b>Balance at December 31, 2022</b>	<u>\$ —</u>	<u>105,263</u>	<u>\$ 105</u>	<u>\$250,430</u>	<u>\$ 49,862</u>	<u>\$ 300,421</u>

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

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

	<b>Year Ended December 31,</b>		
	<b>2022</b>	<b>2021</b>	<b>2020</b>
<b>Operating Activities:</b>			
Net income	\$ 83,079	\$ 39,482	\$ 74,983
Adjustments to reconcile net income to net cash provided by (used in) operating activities:			
Depreciation	141	192	92
Stock-based compensation	7,907	6,073	6,261
Amortization of intangible assets	1,098	—	—
Deferred taxes	4,937	9,316	(32,971)
Change in accrued interest and accretion of discount on investments	17	(5)	(12)
Reduction in the carrying amount of right-of-use asset	247	292	793
Realized loss on sale of available-for-sale securities	762	—	—
Acquired research and development inventory expensed from asset acquisition	4,130	—	—
(Increase) decrease in:			
Accounts receivable, net	(3,820)	(632)	4,549
Inventory	1,065	(3,219)	(2,694)
Prepaid expenses and other current assets and deposits	(807)	3,977	(3,977)
Increase (decrease) in:			
Accounts payable	1,207	(1,488)	138
Accrued expenses and other liabilities	16,391	5,520	(1,209)
Operating lease liability	(307)	864	(919)
Net cash provided by (used in) operating activities	<u>116,047</u>	<u>60,372</u>	<u>45,034</u>
<b>Investing Activities:</b>			
Purchases of property and equipment	(29)	(1,021)	(11)
Purchases of investments	—	(10,000)	(10,000)
Proceeds from maturities and sale of available-for-sale securities	19,238	—	5,000
Payment in connection with license agreement	(10,000)	—	—
Net cash provided by (used in) investing activities	<u>9,209</u>	<u>(11,021)</u>	<u>(5,011)</u>
<b>Financing Activities:</b>			
Payment of employee withholding tax related to stock-based compensation	(230)	(153)	(56)
Proceeds from exercise of stock options	9,569	4,099	758
Repurchase of common stock	(6,907)	(12,089)	—
Payment of liabilities arising from asset acquisition	(738)	—	—
Net cash provided by (used in) financing activities	<u>1,694</u>	<u>(8,143)</u>	<u>702</u>
Net increase in cash and cash equivalents	126,950	41,208	40,725
Cash and cash equivalents – beginning of period	<u>171,445</u>	<u>130,237</u>	<u>89,512</u>
Cash and cash equivalents – end of period	<u>\$298,395</u>	<u>\$171,445</u>	<u>\$130,237</u>
Supplemental disclosures of cash flow information:			
Cash paid for income taxes	\$ 7,667	\$ 3,000	\$ 2,785
Non-cash investing and financing activities:			
Operating lease liabilities arising from obtaining right-of-use assets	\$ —	\$ 3,309	\$ —
Liabilities arising from asset acquisition	\$ 27,699	\$ —	\$ —

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

**CATALYST PHARMACEUTICALS, INC.**  
**NOTES TO CONSOLIDATED FINANCIAL STATEMENTS**

**1. Organization and Description of Business.**

Catalyst Pharmaceuticals, Inc. and subsidiary (collectively, the “Company”) is a commercial-stage biopharmaceutical company focused on in-licensing, developing, and commercializing novel medicines for patients living with rare diseases. With exceptional patient focus, Catalyst is committed to developing a robust pipeline of cutting-edge, best-in-class medicines for rare and difficult to treat diseases.

Catalyst’s New Drug Application 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. Finally, in the third quarter of 2022, the FDA approved the Company’s sNDA approving an expansion of the FIRDAPSE® label to include pediatric patients (ages six and older).

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 product. The Company incurred operating losses in each period from inception and started reporting operating income during the year ended December 31, 2019. The Company has been able to fund its cash needs to date through offerings of its securities and from revenues from sales of its product. See Note 15 (Stockholders’ Equity).

*Capital Resources*

While there can be no assurance, based on currently available information, the Company estimates that it has sufficient resources to support its operations for at least the next 12 months from the issuance 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 drug development efforts, 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.

*Risks and Uncertainties*

There are numerous aspects of the coronavirus (COVID-19) pandemic that have adversely affected the Company’s business since the beginning of the pandemic. The Company closely monitors the impact of the pandemic on all aspects of its business and takes steps, wherever possible, to lessen those impacts. However, the Company is unable to predict the impact that the coronavirus pandemic will have on its business in future periods.

**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.



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

- 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, 2022, investments consisted of U.S. Treasuries. At December 31, 2021, investments consisted of short-term bond funds and U.S. Treasuries. Such investments are not insured by the Federal Deposit Insurance Corporation.

The U.S. Treasuries held at 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 non-current investments as of December 31, 2022 and December 31, 2021.

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 (expense), 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 (expense), 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).

- 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 and individual customer circumstances. At December 31, 2022 and December 31, 2021, 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. The Company began capitalizing inventories post FDA approval of FIRDAPSE® on November 28, 2018 as the related costs were expected to be recoverable through the commercialization of the product. Costs incurred prior to the FDA approval of FIRDAPSE® were recorded as research and development expenses in prior years' consolidated statements of operations and comprehensive income. If information becomes available that suggests that inventories may not be realizable, the Company may be required to expense a portion or all of the previously capitalized inventories.

Products that have been approved by the FDA or other regulatory authorities, such as FIRDAPSE®, 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 form of FIRDAPSE® 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, do 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, 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.

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

- 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, 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.

Refer to 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.

- 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, 2022 and 2021, the fair value of these instruments approximated their carrying value.
- 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.

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

Fair Value Measurements at Reporting Date Using (in thousands)				
	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)
<i>Cash and cash equivalents:</i>				
Money market funds	\$ 168,853	\$ 168,853	\$ —	\$ —
U.S. Treasuries	\$ 105,442	\$ 105,442	\$ —	\$ —
	Balances as of December 31, 2021	Quoted Prices in Active Markets for Identical Assets/Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
<i>Cash and cash equivalents:</i>				
Money market funds	\$ 10,990	\$ 10,990	\$ —	\$ —
U.S. Treasuries	\$ 140,995	\$ 140,995	\$ —	\$ —
<i>Short-term investments:</i>				
Short-term bond funds	\$ 19,821	\$ 19,821	\$ —	\$ —

**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.

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 (accumulated deficit). 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:*

The Company recognizes revenue when its customer obtains 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. 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®.

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

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.

**Product Revenue, Net:** The Company sells FIRDAPSE® to the Customer (its exclusive distributor) who subsequently resells FIRDAPSE® to both a small group of SPs who have exclusive contracts with the Company to distribute the Company’s products to patients and potentially to medical centers or hospitals on an emergency basis. In addition to the distribution agreement with its Customer, the Company enters into arrangements with health care providers and payors that provide for government-mandated and/or privately negotiated rebates, chargebacks, and discounts with respect to the purchase of the Company’s products.

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, 2022, 2021 and 2020.

During the years ended December 31, 2022, 2021 and 2020, principally all of the Company’s sales of FIRDAPSE® in the United States were to its Customer.

**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 Customer, payors, and other indirect 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 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 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, 2022 and, therefore, the transaction price was not reduced further during the years ended December 31, 2022, 2021 and 2020. 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.

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

**Trade Discounts and Allowances:** The Company provides its Customer 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. To the extent the services received are distinct from the sale of FIRDAPSE® to the Customer, these payments are classified in selling, general and administrative expenses in the Company's consolidated statement 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 the Customer, these payments have been recorded as a reduction of revenue within the consolidated statements of operations and comprehensive income through December 31, 2022, 2021 and 2020, as well as a reduction to accounts receivable, net on the consolidated balance sheets.

**Prompt Payment Discounts:** The Company provides its Customer 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 FIRDAPSE® that has 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 current liabilities in the consolidated balance sheets.

**Product Returns:** Consistent with industry practice, the Company offers the SPs and its distributor 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 agreement. The Company estimates the amount of its product sales that may be returned by its Customer 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. 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 current liabilities in the consolidated balance sheets. The Company has an insignificant amount of returns to date and believes that returns of its products will continue to be minimal.

**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. 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, and the Company generally issues credits for such amounts within a few weeks of the Customer's notification to the Company of the resale. Reserves for chargebacks consist primarily of chargebacks that the Customer has claimed, but for which the Company has not yet issued a credit.

**Government Rebates:** The Company is subject to discount obligations under state Medicaid programs and Medicare. These reserves are 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 current 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.



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

**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® free of charge for longer periods of time for those who are uninsured or functionally uninsured with respect to FIRDAPSE® because they are unable to obtain coverage from their payor despite having health insurance, to the extent allowed by applicable law. The Company does not recognize any revenue related to these free products and the associated costs are classified in selling, general and administrative expenses in the Company's consolidated statements of operations and comprehensive income.

### ***Revenues from Collaboration and Licensing Arrangements:***

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

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

The Company evaluates the performance obligations promised in the contract that are based on goods and services that will be transferred to the customer and determines whether those obligations are both (i) capable of being distinct and (ii) distinct in the context of the contract. Goods or services that meet these criteria are considered distinct performance obligations. The Company estimates the transaction price based on the amount expected to be received for transferring the promised goods or services in the contract. The consideration may include fixed consideration or variable consideration.

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.

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

- q. **ADVERTISING EXPENSE.** In connection with the FDA approval and commercial launch of FIRDAPSE® in 2019, the Company began to incur advertising costs. Advertising costs are expensed as incurred. The company incurred \$3.3 million, \$2.9 million and \$2.5 million in advertising costs during the years ended December 31, 2022, 2021 and 2020, respectively, which are included in selling, general and administrative expenses in the Company's consolidated statement of operations and comprehensive income.
- r. **STOCK-BASED COMPENSATION.** The Company recognizes expense in the consolidated statements of operations 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 (i.e., money market funds), 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 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. The creditworthiness of its Customer 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 Customer, historical payment patterns, aging of receivable balances and general economic conditions.

As of December 31, 2022, the Company had a single product, which made 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 the lead product, FIRDAPSE®. The Company expects FIRDAPSE® and the recently acquired product FYCOMPA® to constitute virtually all of the Company's product revenue for the foreseeable future. See Note 18 (Subsequent Events).

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

The Company recognizes the financial statement benefit of a tax position only after determining that the relevant tax authority would more likely than not sustain the position following an audit. For tax positions meeting the more-likely-than-not threshold, the amount recognized in the financial statements is the largest benefit that has a greater than 50 percent likelihood of being realized upon ultimate settlement with the relevant tax authority. The Company is subject to income taxes in the U.S. federal jurisdiction and various state jurisdictions. Tax regulations within each jurisdiction are subject to the interpretation of the related tax laws and regulations and require significant judgment to apply. The Company is not subject to U.S. federal, state and local tax examinations by tax authorities for years before 2018. 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.

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

- 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, 2022, 2021 and 2020, 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,		
	2022	2021	2020
Basic weighted average common shares outstanding	103,374,606	103,379,349	103,512,913
Effect of dilutive securities	8,001,025	4,416,236	2,729,360
Diluted weighted average common shares outstanding	<u>111,375,631</u>	<u>107,795,585</u>	<u>106,242,273</u>

Outstanding common stock equivalents totaling approximately 1.0 million, 4.3 million and 7.1 million, were excluded from the calculation of diluted net income per common share for the years ended December 31, 2022, 2021 and 2020, respectively, as their effect would be anti-dilutive. Potentially dilutive options to purchase common stock as of December 31, 2022, 2021 and 2020 had exercise prices ranging from \$0.79 to \$7.07, \$0.79 to \$4.64 and \$0.79 to \$3.95, 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, 2022.

## 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
<b>At December 31, 2022:</b>				
U.S. Treasuries - Cash equivalents	\$ 105,442	\$ 32	\$ —	\$ 105,410
Total	<u>\$ 105,442</u>	<u>\$ 32</u>	<u>\$ —</u>	<u>\$ 105,410</u>
<b>At December 31, 2021:</b>				
U.S. Treasuries - Cash equivalents	\$ 140,995	\$ 2	\$ —	\$ 140,993
Short-term bond funds	19,821	—	(196)	20,017
Total	<u>\$ 160,816</u>	<u>\$ 2</u>	<u>\$ (196)</u>	<u>\$ 161,010</u>

There were realized losses from sale of available-for-sale securities of \$762 thousand for the year ended December 31, 2022. There were no realized gains or losses from available-for-sale securities for the years ended December 31, 2021 or 2020.

The estimated fair values of available-for-sale securities at December 31, 2022, by contractual maturity, are summarized as follows (in thousands):

	2022
Due in one year or less	<u>\$ 105,442</u>

#### 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, 2022, 2021 and 2020.

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, 2021 or 2020.

	<b>Total Accumulated Other Comprehensive Income (Loss)</b>
<b>Balance at December 31, 2021</b>	<b>\$ (148)</b>
Other comprehensive gain (loss) before reclassifications	(590)
Amount reclassified from accumulated other comprehensive income	762
Net current period other comprehensive gain (loss)	172
<b>Balance at December 31, 2022</b>	<b>\$ 24</b>

#### 5. Inventory.

Inventory consists of the following (in thousands):

	<b>December 31, 2022</b>	<b>December 31, 2021</b>
Raw materials	\$ —	\$ 1,769
Work-in-process	5,543	5,172
Finished goods	1,262	929
Total inventory	<u>\$ 6,805</u>	<u>\$ 7,870</u>

#### 6. Prepaid Expenses and Other Current Assets.

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

	<b>December 31, 2022</b>	<b>December 31, 2021</b>
Prepaid manufacturing costs	\$ 1,147	\$ 307
Prepaid tax	44	564
Prepaid insurance	1,224	1,213
Prepaid subscriptions fees	1,202	909
Prepaid research fees	178	452
Prepaid commercialization expenses	198	195
Due from collaborative and licensing arrangements	354	105
Prepaid conference and travel expenses	234	279
Other	577	327
Total prepaid expenses and other current assets	<u>\$ 5,158</u>	<u>\$ 4,351</u>

## 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. Consequently, the Company recorded the effects of the amended lease during Q1 2021.

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

	For the Years Ended December 31,	
	2022	2021
Operating lease cost	\$ 431	\$ 379

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

	For the Years Ended December 31,	
	2022	2021
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows	\$ 492	\$ 109
Right-of-use assets obtained in exchange for lease obligations:		
Operating lease	\$ 89	\$ 80

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

	December 31, 2022	December 31, 2021
Operating lease right-of-use assets	\$ 2,770	\$ 3,017
Other current liabilities	\$ 337	\$ 308
Operating lease liabilities, net of current portion	3,557	3,894
Total operating lease liabilities	\$ 3,894	\$ 4,202

As of December 31, 2022 and December 31, 2021, the weighted average remaining lease term was 8.3 years and 9.3 years, respectively. The weighted average discount rate used to determine the operating lease liabilities was 4.51% as of December 31, 2022 and December 31, 2021.

Remaining payments of lease liabilities as of December 31, 2022 were as follows (in thousands):

2023	\$ 506
2024	522
2025	537
2026	553
2027	570
Thereafter	2,027
Total lease payments	4,715
Less: imputed interest	(821)
Total	\$3,894

Rent expense was \$0.4 million, \$0.4 million and \$0.3 million for the years ended December 31, 2022, 2021 and 2020.



## 8. Property and Equipment, Net.

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

	<u>December 31, 2022</u>	<u>December 31, 2021</u>
Computer equipment	\$ 51	\$ 51
Furniture and equipment	222	203
Leasehold improvements	980	980
Less: Accumulated depreciation	(406)	(275)
Total property and equipment, net	<u>\$ 847</u>	<u>\$ 959</u>

## 9. License and Acquired Intangibles, Net.

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

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

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 of approximately 14.5 years. Amortization of these assets during each of the next five years is estimated to be approximately \$2.3 million per year. 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.

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 year ended December 31, 2022. There were no definite-lived intangible assets at December 31, 2021.

## 10. Accrued Expenses and Other Liabilities.

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

	<u>2022</u>	<u>2021</u>
Accrued preclinical and clinical trial expenses	\$ 479	\$ 659
Accrued professional fees	1,619	2,391
Accrued compensation and benefits	5,132	4,035
Accrued license fees	20,444	12,819
Accrued purchases	154	2,045
Operating lease liability	337	308
Accrued variable consideration	3,381	1,716
Accrued income tax	8,702	79
Due to licensor	13,127	—
Other	238	243
Current accrued expenses and other liabilities	<u>53,613</u>	<u>24,295</u>
Lease liability – non-current	3,557	3,894
Due to licensor – non-current	14,064	—
Non-current accrued expenses and other liabilities	<u>17,621</u>	<u>3,894</u>
Total accrued expenses and other liabilities	<u>\$71,234</u>	<u>\$28,189</u>

## 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.

Under the terms of the Collaboration, the Company has received an up-front payment, and will receive a milestone payment, and a sharing of defined net profits upon commercialization from Endo consisting of a mid-double digit percent of net sales of generic Sabril®. The Company has also agreed to a 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.

The Company evaluated the license agreement with Endo to determine whether it is a collaborative arrangement for purposes of Topic 808. As the Company shares in the significant risks and rewards, the Company has concluded that this is a collaborative arrangement. As developing a final finished dosage form of a generic product in exchange for consideration is not an output of the Company's ongoing activities, Endo does not represent a contract with a customer. However, Topic 808 does not provide guidance on the recognition of consideration exchanged or accounting for the obligations that may arise between the parties. The Company concluded that ASC Topic 730, *Research and Development*, should be applied by analogy to payments between the parties during the development activities and Topic 606 for the milestone payment and sharing of defined net profits upon commercialization.

The collaborative agreement included a nonrefundable upfront license fee that was recognized upon receipt following execution of the collaborative arrangement for vigabatrin tablets.

The collaborative agreement provides for a \$2.0 million milestone payment on the commercial launch of the product by Endo/Par. As of December 31, 2022, 2021 and 2020, no milestone payments have been earned.

There were no revenues from this collaborative arrangement for the years ended December 31, 2022, 2021 or 2020. Total expenses incurred, net, in connection with the collaborative agreement for the years ended December 31, 2022, 2021 and 2020 were approximately \$0, \$45,000 and \$4,200, respectively. These expenses have been included in research and development expenses in the accompanying consolidated statements of operations and comprehensive income.

### *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®, 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 a 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.

Under the arrangement, the Company will receive profit-sharing reports within nine days after quarter end from KYE. Revenue from sales of FIRDAPSE® by KYE will be recognized in the quarter in which the sales occurred.

## 11. Collaborative and Licensing Arrangements (continued).

Revenues from the arrangement with KYE for the years ended December 31, 2022, 2021 and 2020 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.

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 \$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. As of December 31, 2022, no milestone payments have been earned.

## 12. Commitments and Contingencies.

In May 2019, the FDA approved a New Drug Application (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 11<sup>th</sup> Circuit. The case was fully briefed in early 2021, and oral argument was held in March 2021.

## 12. Commitments and Contingencies (continued).

On September 30, 2021, a three-judge panel of 11<sup>th</sup> 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 11<sup>th</sup> Circuit, which motion was denied in January 2022. Further, in January 2022, Jacobus filed motions with both the 11<sup>th</sup> Circuit and the U.S. Supreme Court seeking a stay of the 11<sup>th</sup> Circuit's ruling indicating that it would seek a review of the 11<sup>th</sup> Circuit's decision from the U.S. Supreme Court. Both stay motions were denied, and on January 28, 2022, the 11<sup>th</sup> 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.

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 the patent case, which has been dismissed without prejudice. Finally, Jacobus agreed that until the later of (i) the expiration of the royalty term or (ii) December 31, 2034, Jacobus and its affiliates, will not, directly or indirectly, research, develop, manufacture, commercialize, distribute, use or otherwise exploit any product competitive to FIRDAPSE® or RUZURGI® in the 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 and the balance of which will be paid over the next two years, on the first and 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.

Royalties will be trued up at the end of the year to the extent that royalties on net sales are below the minimum royalty.

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. 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 the Company. As such, the Company believes that its 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, the Company 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® in spite of FIRDAPSE®'s data protection rights. However, on June 28, 2021, the Company 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 the Company, along with its partner in Canada, KYE, filed a second suit against Health Canada to overturn this decision.

## 12. Commitments and Contingencies (continued).

On March 11, 2022, the Company announced that the Company 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 available for sale in Canada.

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 has an option to further expand its territory under the license agreement to include most of Asia, as well as Central and South America, upon the achievement of certain milestones in Japan. 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.

In January 2020, the Company was advised that BioMarin has transferred certain rights under the license agreement to SERB S.A.

- 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 will pay an additional \$10 million on the first annual anniversary of the Effective Date (July 11, 2023), another \$10 million on the second annual anniversary of the Effective Date (July 11, 2024) and 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. 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. As these minimum payments are not probable and estimable, they 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®.



### 13. Agreements (continued).

Additionally, the Company also purchased from Jacobus approximately \$4.1 million of RUZURGI® inventory previously manufactured by Jacobus, which have been recorded as an expense in research and development expenses in the accompanying consolidated statement of operations and comprehensive income for the year ended December 31, 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. 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.

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	<u>\$ 37,699</u>

The straight-line method is used to amortize the license and acquired intangibles, as disclosed in Note 9 (License and Acquired Intangibles, Net).

- c. **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 (benefit) for the years ended December 31, 2022, 2021, and 2020 consists of (in thousands):

	<u>2022</u>	<u>2021</u>	<u>2020</u>
Current - Federal	\$12,858	\$ 2,455	\$ —
Current - State	3,877	1,414	(122)
Deferred - Federal	4,739	8,620	(29,378)
Deferred - State	166	696	(3,593)
	<u>\$21,640</u>	<u>\$ 13,185</u>	<u>\$(33,093)</u>

#### 14. Income Taxes (continued).

The reconciliation of income tax expense (benefit) computed at the statutory federal income tax rate of 21% to amounts included in the statements of operations is as follows:

	<u>2022</u>	<u>2021</u>	<u>2020</u>
Statutory rate	21.0%	21.0%	21.0%
State tax	3.1%	3.4%	2.2%
Valuation allowance	—	—	(99.4)%
Executive compensation limitation	3.6%	1.1%	—
Tax credit	(1.9)%	(0.6)%	(2.4)%
Stock compensation windfall	(5.6)%	(0.6)%	—
Other	0.5%	0.7%	(0.4)%
	<u>20.7%</u>	<u>25.0%</u>	<u>(79.0)%</u>

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, 2022 and 2021 are as follows (in thousands):

	<u>2022</u>	<u>2021</u>
Deferred tax assets:		
Net operating loss	\$ —	\$ 1,218
Start-up costs	9,771	10,403
Tax credits	—	8,516
Deferred compensation	4,706	3,959
Inventory	296	163
Operating lease liability	953	1,003
Capitalized research	4,255	—
Other	96	—
Total deferred tax assets	<u>20,077</u>	<u>25,262</u>
Deferred tax liabilities:		
Prepaid expenses	(481)	(455)
Right-of use asset	(860)	(936)
Other	—	(174)
Total deferred tax liabilities	<u>(1,341)</u>	<u>(1,565)</u>
Deferred tax assets, net	<u>\$18,736</u>	<u>\$23,697</u>

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. As of December 31, 2022, 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 \$20 million are realizable.

At December 31, 2022 and 2021, respectively, the Company had state net operating loss carryforwards of approximately \$0 million and \$28 million, respectively, available to reduce future Florida taxable income.

During 2020, the Company completed an analysis to determine whether, as a result of prior ownership changes, the utilization of certain net operating loss and orphan drug tax credit carryforwards would be subject to annual limitations under Sections 382 and 383 of the Internal Revenue Code and similar state provisions. In this analysis, the Company determined that the total net operating loss and orphan drug tax credit carryforwards were fully utilizable.

Beginning in 2010, 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, 2022. While an immaterial amount of interest and no penalties were accrued through December 31, 2021. 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, 2022 and 2021. No shares of preferred stock were outstanding at December 31, 2022 and 2021.

### ***Common Stock***

The Company has 200,000,000 shares of authorized common stock, par value \$0.001 per share. At December 31, 2022 and 2021, 105,263,031 and 102,992,913 shares, respectively, of common stock were issued and outstanding. Each holder of common stock is entitled to one vote of each share of common stock held of record on all matters on which stockholders generally are entitled to vote.

### ***Share Repurchases***

In March 2021, the Company's Board of Directors approved a share repurchase program that authorizes the repurchase of up to \$40 million of the Company's common stock, pursuant to a repurchase plan under Rule 10b-18 of the Securities Act. The share repurchase program commenced on March 22, 2021 and, 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).

### ***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. As of the date of this report, no offerings have been completed under the Company's 2020 Shelf Registration Statement.

### ***Stockholder Rights Plan***

On September 20, 2011, the Board of Directors approved the Company's adoption of a Stockholder Rights Plan. Under the Stockholders' Rights Plan, a dividend of one preferred share purchase right (a Right) was declared for each share of common stock of the Company that was outstanding on October 7, 2011. Each Right entitled the holder to purchase from the Company one one-hundredth of a share of Series A Junior Preferred Stock at a purchase price of \$7.80, subject to adjustment.

The Rights traded automatically with the common stock and were not exercisable until a person or group had become an "acquiring person" by acquiring 17.5% or more of the Company's outstanding common stock, or a person or group commenced, or publicly announced a tender offer that would result in such a person or group owning 17.5% or more of the Company's outstanding common stock. Upon announcement that any person or group had become an acquiring person, each Right would entitle all rightholders (other than the acquiring person) to purchase, for the exercise price of \$7.80, a number of shares of the Company's common stock having a market value equal to twice the exercise price. Rightholders would also be entitled to purchase common stock of the acquiring person having a value of twice the exercise price if, after a person had become an acquiring person, the Company were to enter into certain mergers or other transactions. If any person becomes an acquiring person, the Board of Directors may, at its option and subject to certain limitations, exchange one share of common stock for each Right.

The Rights had certain anti-takeover effects, in that they would cause substantial dilution to a person or group that attempts to acquire a significant interest in the Company on terms not approved by the Board of Directors. In the event that the Board of Directors determines a transaction to be in the best interests of the Company and its stockholders, the Board of Directors may redeem the Rights for \$0.001 per share at any time prior to a person or group becoming an acquiring person.

On September 19, 2016, the Board of Directors unanimously approved, and on the same date the Company entered into Amendment No. 1 to the Stockholders Rights Plan (the "Amendment"). Under the terms of the Amendment, the outside expiration date of the rights plan was extended to September 20, 2019. Additionally, as part of the Amendment, the Board adopted a Certificate of Designation, Preferences and Rights of Series A Junior Participating Preferred Stock of the Company to increase the number of shares of Series A Junior Participating Preferred Stock of the Company available for issuance under the Rights Plan from 500,000 shares to 1.5 million shares.

On August 28, 2019, the Board of Directors unanimously adopted Amendment No. 2 to the Stockholders' Rights Plan further extending the outside expiration date of the rights plan to September 20, 2022.

## 15. Stockholders' Equity (continued).

On November 12, 2021, the Board of Directors terminated the Rights Plan. Despite the termination of the Rights Plan, the Board of Directors reserves the right to take all necessary actions it deems appropriate in the future to protect the interests of all of the Company's stockholders.

## 16. Stock Compensation.

For the years ended December 31, 2022, 2021 and 2020, the Company recorded stock-based compensation expense as follows (in thousands):

	2022	2021	2020
Research and development	\$1,729	\$1,611	\$1,585
Selling, general and administrative	6,178	4,462	4,676
Total stock-based compensation	<u>\$7,907</u>	<u>\$6,073</u>	<u>\$6,261</u>

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, 2022, no shares remain available for future issuance under the 2014 Plan. Under the 2018 Plan, 15,000,000 shares are reserved for issuance and as of December 31, 2022, 2,691,791 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, 2022, 2021 and 2020, options to purchase 3,172,342, 1,328,936 and 281,762 shares, respectively, of the Company's common stock were exercised with gross proceeds to the Company of approximately \$9.6 million, \$4.1 million and \$0.8 million, respectively. During the years ended December 31, 2022, 2021 and 2020, no options to purchase shares of the Company's common stock were exercised on a "cashless" basis.

During the years ended December 31, 2022, 2021 and 2020 the Company recorded non-cash stock-based compensation expense related to stock options totaling approximately \$6.3 million, \$5.5 million and \$5.7 million, respectively.

During the years ended December 31, 2022, 2021 and 2020, the Company granted seven-year options to purchase an aggregate of 1,386,500, 2,330,000 and 2,715,000 shares, respectively, of the Company's common stock to certain of the Company's officers, employees, directors, and consultants.

Stock option activity under the Company's Plans for the year ended December 31, 2022 is summarized as follows:

	Number of Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding at beginning of year	14,207,728	\$ 3.55		
Granted	1,386,500	14.82		
Exercised or released	(3,172,342)	3.02		
Forfeited or cancelled	(112,778)	5.73		
Expired	—	—		
Outstanding at end of year	<u>12,309,108</u>	<u>\$ 4.93</u>	<u>3.85</u>	<u>\$ 168,222</u>
Exercisable at end of year	<u>8,534,665</u>	<u>\$ 3.29</u>	<u>3.00</u>	<u>\$ 130,686</u>

## 16. Stock Compensation (continued).

Other information pertaining to stock option activity during the years ended December 31, 2022, 2021 and 2020 was as follows:

	2022	2021	2020
Weighted-average fair value of granted stock options	\$ 8.52	\$ 3.24	\$ 2.33
Total fair value of vested stock options (in thousands)	\$ 6,096	\$6,421	\$5,312
Total intrinsic value of exercised stock options (in thousands)	\$31,881	\$3,623	\$ 325

As of December 31, 2022, there was approximately \$16.7 million of unrecognized compensation expense related to non-vested stock option awards granted under the Plans. That cost is expected to be recognized over a weighted average period of approximately 2.53 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:

	2022	2021	2020
Risk free interest rate	1.27% to 4.07%	0.34% to 1.18%	0.24% to 1.64%
Expected term	4.5 years	4.5 – 4.8 years	4.5 years
Expected volatility	68.4% to 69.5%	68.6% to 72.8%	80.5% to 83.7%
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, 2022 was as follows;

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Nonvested balance at beginning of year	122,839	\$ 4.65
Granted	742,500	11.55
Vested	(112,839)	4.65
Forfeited	—	—
Nonvested balance at end of year	752,500	\$ 11.46

During the year ended December 31, 2022, 2021 and 2020, the Company recorded non-cash stock-based compensation expense related to restricted stock units totaling \$1.6 million, \$0.5 million and \$0.6 million, respectively.



## **17. Benefit Plan.**

The Company maintains an employee savings plan pursuant to Section 401(k) of the Internal Revenue Code covering all eligible employees. Subject to certain dollar limits, eligible employees may contribute up to 15% of their pre-tax annual compensation to the plan. The Company has elected to make discretionary matching contributions of employee contributions up to 4% of an employee's gross salary. For the years ended December 31, 2022, 2021, and 2020, the Company's matching contributions were approximately \$0.5 million each year.

## **18. Subsequent Events.**

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").

Under the terms of the purchase agreement, the Company made an upfront cash payment of \$160 million plus \$1.6 million for reimbursement of certain prepayments. Eisai is also eligible to receive a contingent payment of \$25 million if certain regulatory milestones are met and tiered royalty payments based on certain annual net sales milestones.

Concurrently with the acquisition, the parties entered into two related agreements: (i) a short-term Transition Services Agreement for commercial and manufacturing services and (ii) a long-term Supply Agreement for the manufacturing of FYCOMPA®. Under the Transition Services Agreement, Eisai will provide 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.

Given that the acquisition was recently closed, the Company's analysis of the accounting for the transaction is in process and is incomplete as of the filing date of this report.

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## Corporate Directory

### BOARD OF DIRECTORS

**Patrick J. McEnany**

Chairman of the Board, President,  
Chief Executive Officer and  
Co-Founder  
Catalyst Pharmaceuticals, Inc.

**Philip H. Coelho**

Chair, Nominating and Corporate  
Governance Committee  
Chairman and Chief Executive Officer  
PHC Medical

**Richard Daly**

President  
CARsgen Therapeutics Corporation

**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**

Organization Head – Global Corporate  
Affairs  
Alexion Pharmaceuticals/AstraZeneca  
Rare Disease

**David S. Tierney, MD**

Chair, Compensation Committee  
Chief Executive Officer  
Aramis Biosciences

### MANAGEMENT TEAM

**Patrick J. McEnany**

Chairman of the Board, President,  
Chief Executive Officer and  
Co-Founder

**Steven R. Miller, PhD**

Chief Operating Officer  
and Chief Scientific Officer

**Alicia Grande, CPA, CMA**

Vice President, Treasurer and Chief  
Financial Officer

**Gary Ingenito, M.D., Ph.D.**

Chief Medical and Regulatory Officer

**Jeffrey Del Carmen**

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, August  
22, 2023, 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](http://www.catalystpharma.com)  
or by written request to:

Catalyst Pharmaceuticals, Inc.  
355 Alhambra Circle, Suite 801  
Coral Gables, FL 33134  
(305) 420-3200  
(305) 569-0233 fax  
Email: [info@catalystpharma.com](mailto:info@catalystpharma.com)

### STOCK LISTING

Catalyst's common stock trades on the  
Nasdaq Capital Market under the  
symbol CPRX.

### TRANSFER AGENT

Continental Stock Transfer  
One State Street Plaza, 30<sup>th</sup> Floor  
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