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

FORM 10-K

[Mark One]

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

For the Fiscal Year Ended December 31, 2021

OR

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

Commission File No. 001-33057

CATALYST PHARMACEUTICALS, INC.
(Exact name of registrant as specified in its charter)

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

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

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

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

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

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

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

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

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

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

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

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

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

As of June 30, 2021, 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 $551,581,521.

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

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date: 102,744,913 shares of common stock, $0.001 par value per share, were outstanding as of March 14, 2022.

Part III incorporates certain information by reference from the registrant's definitive proxy statement for the 2022 annual meeting of stockholders. The proxy statement with respect to the 2022 annual meeting of stockholders will be filed no later than 120 days after the close of the registrant's fiscal year ended December 31, 2021.

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EXHIBITS FILED WITH FORM 10-K

PART I

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

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

Forward-Looking Statements

This Annual Report on Form 10-K contains "forward-looking statements", as that term is defined in the Private Securities Litigation Reform Act of 1995. These include statements regarding our expectations, beliefs, plans or objectives for future operations and anticipated results of operations. For this purpose, any statements contained herein that are not statements of historical fact may be deemed to be forward-looking statements. Without limiting the foregoing, "believes", "anticipates", "proposes", "plans", "expects", "intends", "may", and other similar expressions are intended to identify forward-looking statements. Such statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or other achievements to be materially different from any future results, performances or achievements expressed or implied by such forward-looking statements. Factors that might cause such differences include, but are not limited to, those discussed in the section entitled "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® is 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® 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 turn out 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 our drug at rates that are higher than historically experienced or are higher than we project;
- Whether the daily dose taken by patients changes over time and affects our results of operations;
- Whether FIRDAPSE® patients can be successfully titrated to stable therapy;
- Whether we can continue to market FIRDAPSE® on a profitable and cash flow positive basis;
- 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 product at the price that we charge for the product;
- The ability of our third-party suppliers and contract manufacturers to maintain compliance with current Good Manufacturing Practices (cGMP);
- The ability of our distributor and the specialty pharmacies that distribute our product to maintain compliance with applicable law;
- Our ability to maintain compliance with applicable rules relating to our patient assistance programs and our contributions to 501(c)(3) organizations that support LEMS patients;

- The scope of our intellectual property and the outcome of any future challenges or opposition to our intellectual property, and, conversely, whether any third-party intellectual property presents unanticipated obstacles for FIRDAPSE®;
- Whether our lawsuits against Jacobus Pharmaceutical Company (Jacobus) and the specialty pharmacy distributing its product for patent infringement will be successful;
- Whether Jacobus will seek U.S. Supreme Court review of the decision of the U.S. Court of Appeals for the 11th Circuit granting summary judgment in our favor in our case against the FDA, thereby overturning the FDA's approval of Ruzurgi® , whether the U.S. Supreme Court will agree to hear the case, or whether if the U.S. Supreme Court hears the case, they will overturn the decision of the 11th Circuit;
- Whether the United States Congress will pass, and the President will sign, legislation revising the Orphan Drug Act that effectively overturns the decision of the U.S. Court of Appeals for the 11th Circuit, and whether any such legislation, if passed and signed into law, will retroactively affect the outcome of the 11th Circuit decision and allow the FDA to reinstate the approval of Ruzurgi® before the expiration of FIRDAPSE®'s orphan drug exclusivity;
- The impact on FIRDAPSE® 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 organization, 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;
- The state of the economy generally and its impact on our business;
- Changes to the healthcare industry occasioned by any future changes in laws relating to the pricing of drug products, or changes in the healthcare industry generally;
- 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 COVID-19 will further affect the timing and costs of our currently ongoing and contemplated clinical trials;
- Whether FIRDAPSE® can be successfully commercialized in Canada on a profitable basis;
- Whether our suit with KYE Pharmaceuticals to overturn the approval of Ruzurgi® in Canada will be successful;
- 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 Pharma (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;
- Whether our efforts to grow our business beyond FIRDAPSE® through acquisitions of companies or in-licensing of product opportunities will be successful;
- Whether we will have sufficient capital to finance any such acquisitions;
- Whether our version of vigabatrin tablets will ever be approved by the FDA;
- Even if our version of vigabatrin tablets is approved for commercialization, whether Endo Ventures/Par Pharmaceutical (our collaborator in this venture) will be successful in marketing the product; and
- Whether we will earn milestone payments on the first commercial sale of vigabatrin tablets and royalties on sales of generic vigabatrin tablets.

Our current plans and objectives are based on assumptions relating to the continued commercialization of FIRDAPSE® 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. In light of 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 biopharmaceutical company focused on in-licensing, developing and commercializing novel medicines for patients living with rare diseases. With exceptional patient focus, we are committed to developing a robust pipeline of cutting-edge, best-in-class medicines for rare diseases. We historically focused our efforts on developing products that treat diseases in the neuromuscular and neurological space, but in 2021 we made a strategic decision 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.

We are currently exploring several potential opportunities to acquire companies with drug products in development or to in-license or acquire drug products in development. However, no definitive agreements have been entered into to date. Further, during the third quarter of 2021 we hired Dr. Preethi Sundaram, who serves as our Chief Strategy Officer. In that position, Dr. Sundaram is leading our efforts to acquire R&D assets, from early stage through late-stage clinical programs and technologies to treat rare diseases, and once such drug candidates are acquired, Dr. Sundaram will help oversee the development of those assets.

We are dedicated to making a meaningful impact on the lives of those suffering from rare diseases, and we believe in putting patients first in everything we do.

Impact of the COVID-19 pandemic on our business

The COVID-19 pandemic has affected our business operations in numerous ways, and we continue to monitor applicable government modifications. We had to make modifications to our normal operations because of the COVID-19 pandemic, including allowing our employees to work remotely. 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. Notwithstanding, the COVID-19 pandemic, including the emergence of new COVID-19 variants, including the delta and omicron variants, could 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 such, we have experienced in the past, and may experience in the future, disruptions to our business operations because of 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 believe that because many healthcare providers who treat LEMS patients have delayed seeing new patients because of the pandemic, there has been a delay in the diagnosis of new LEMS patients and their initiating therapy, which has slowed our efforts to locate new patients who could benefit from our therapy. However, we believe that when conditions allow healthcare providers to resume seeing new patients in person on a regular basis, the impact of this aspect of the COVID-19 pandemic on our business will lessen.

One area where we have not been impacted by the pandemic is in our supply chain. To date, we have been able to avoid material disruptions in the production of FIRDAPSE® and, based upon current estimates, we have sufficient inventory to meet current and foreseeable patient needs for at least the next 12 months.

FIRDAPSE®

On November 28, 2018, we received approval from the FDA for FIRDAPSE® Tablets 10 mg for the treatment of adult patients (ages 17 and above) with Lambert-Eaton, Myasthenic Syndrome ("LEMS"). In January 2019, we launched FIRDAPSE® in the United States. We sell our product through a field force experienced in neurologic, central nervous system or rare disease products consisting at this time of approximately 30 field personnel, including sales (Regional Account Managers), patient assistance and insurance navigation support (Patient Access Liaisons), and payor reimbursement (National Account Managers). We also have a field-based force of five medical science liaisons who are helping educate the medical communities and patients about LEMS and our programs supporting patients and access to FIRDAPSE®.

Further, 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 an adult LEMS patient who can benefit from FIRDAPSE®. Additionally, we recently began non-personal promotion to oncologists that may treat adult LEMS patients. We also are continuing to make available at no-cost a LEMS voltage gated calcium channel (VGCC) 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 in order 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, the National Organization for Rare Disorders (NORD), and the Myasthenia Gravis Foundation of America) 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 adult 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. For eligible patients with commercial coverage, a co-pay assistance program designed to keep out-of-pocket costs to not more than $10.00 per month (currently $0.00 per month) is available for all LEMS patients who are prescribed FIRDAPSE®. We are also 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 May 2019, the FDA approved a New Drug Application (NDA) for Ruzurgi®, another version of amifampridine (3,4-DAP), for the treatment of pediatric LEMS patients (ages 6 to under 17). While the NDA for Ruzurgi® only covers pediatric patients, we believe that Ruzurgi® has been regularly prescribed off-label to adult LEMS patients. We also believe that the FDA's approval of Ruzurgi® violated our statutory rights and was in multiple other respects arbitrary, capricious and contrary to law. As a result, in June 2019 we filed suit against the FDA and several related parties challenging this approval and related drug labeling, and Jacobus Pharmaceuticals (Jacobus) intervened in our 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 Federal Food, Drug, and Cosmetic Act (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 setting aside the FDA's approval of Ruzurgi®.

On July 30, 2020, the Magistrate Judge considering our lawsuit against the FDA 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 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. Circuit Court of Appeals for the 11th Circuit. By early 2021, the case was fully briefed, and oral argument was held in March 2021.

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

There can be no assurance as to whether Jacobus will seek U.S. Supreme Court review of the 11th Circuit's decision, whether the U.S. Supreme Court will agree to hear the case, or whether, if the U.S. Supreme Court agrees to hear the case, Jacobus' appeal to overturn the decision of the 11th Circuit will be successful. Similarly, there can be no assurance as to whether the U.S. Congress will pass, and the President will sign, legislation revising the Orphan Drug Act that effectively overturns the decision of the U.S. Court of Appeals for the 11th Circuit, and whether any such legislation, if passed and signed into law, will retroactively affect the outcome of the 11th Circuit decision and allow the FDA to reinstate the approval of Ruzurgi® before the expiration of FIRDAPSE®'s orphan drug exclusivity.

We are actively working with parents and physicians of pediatric LEMS patients to make sure that such patients will be able to obtain FIRDAPSE® through appropriate legal and regulatory means. In addition, we are working to file an application with the FDA seeking approval for use of FIRDAPSE® by pediatric LEMS patients, though any effort to obtain such authorization is not guaranteed. We anticipate submitting the sNDA before the end of the first quarter. For the adult LEMS patients who have been taking Ruzurgi® off-label (who are believed to be a large majority of the patients currently taking Ruzurgi®), we are working with prescribers to transition such patients to FIRDAPSE® as needed.

We have been developing a long-acting formulation of amifampridine phosphate. While a number of formulations have been prepared, after discussions with researchers and an advisory board made up of both patients and physicians, we recently concluded that we would be unable to develop a long-acting formulation that was both beneficial to patients and commercially viable, and as a result we have made the determination not to proceed with development of this product.

On August 10, 2020, we announced the top-line results from our Phase 3 clinical trial (MSK-002) evaluating FIRDAPSE® for the treatment of adults with MuSK-MG. Unfortunately, the MSK-002 trial did not achieve statistical significance on its primary endpoint or its secondary endpoint. Following our receipt of these results, we analyzed the data and proposed a plan to FDA to perform an additional study evaluating FIRDAPSE® for the treatment of MuSK-MG. In response, the FDA provided written comments that were unfavorable towards our proposed revised study design and further questioned the ability of the initial MuSK-MG pilot study to be supportive. These remarks make it unlikely that a single study of similar design to MSK-002 would be sufficient for potential approval of the MuSK-MG indication. We also held an expert panel with key opinion leaders (KOLs) to discuss options and review the likelihood of success for the MuSK-MG indication for FIRDAPSE® under these circumstances. After receiving the input of the FDA and the KOLs, we concluded that the approval of FIRDAPSE® as a first line therapy for MuSK-MG is unlikely, and therefore we have decided not to further pursue this indication.

We previously announced our intent to conduct a proof-of-concept study evaluating FIRDAPSE® as a treatment for Hereditary Neuropathy with Liability to Pressure Palsies (HNPP). The FDA requested that a new, patient-centric endpoint be researched and used for our proposed study, without assurance that such endpoint would be acceptable for approval. Based upon the uncertainty of such an endpoint, we have decided not to conduct this study as a company-sponsored study, though there is a possibility that this study will move forward as investigator-initiated study that we will support.

There can be no assurance that any future clinical trials of FIRDAPSE® that we undertake will be successful. Further, there can be no assurance that we will ever be granted the right to commercialize FIRDAPSE® for any additional indications.

Our NDS filing for FIRDAPSE® for the symptomatic treatment of LEMS was approved by Health Canada on July 31, 2020. In August 2020, we entered into a license agreement with KYE Pharmaceuticals (KYE), pursuant to which we licensed the Canadian rights for FIRDAPSE® for the treatment of LEMS to KYE. Pursuant to the license agreement, KYE was obligated to pay us an up-front payment based on approval, a milestone upon attainment of marketing authorization and product supply, milestones based on achievements of sales and regulatory milestones, and a sharing of defined net sales following commercialization.

On August 10, 2020, Health Canada issued a Notice of Compliance (NOC) to Medunik for Ruzurgi® for the treatment of LEMS. 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 a 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 KYE, filed a second suit against Health Canada to overturn this decision. That case was fully briefed in late 2021, with oral argument held in early December.

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. As a result, the matter has, once again, been remanded to the Minister of Health to redetermine its decision in light of the court's ruling. There can be no assurance as to the outcome of this proceeding.

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. Additionally, we have an option to further expand our 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, we will pay royalties to our licensor 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.

We have 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. We also 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. (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 will have joint rights to develop FIRDAPSE®, and exclusive rights to commercialize the product, in Japan. DyDo will be responsible for funding all clinical, regulatory, marketing and commercialization activities in Japan. We will be 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 product supplied to DyDo.

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 sometime in 2023. There can be no assurance that this trial will be successful or that DyDo will be granted the right to commercialize FIRDAPSE® in Japan.

All of our patent rights for FIRDAPSE® are derived from our license agreement. 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, 17/503,190. On January 3, 2022, the USPTO allowed related continuing application 17/503,148. A further related continuing application, 17/503,092 was allowed on January 7, 2022. All three patents were issued in March 2022. The claims in each of these applications have either already been listed in the Orange Book for FIRDAPSE® or are in the process of being listed.

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 which provide additional intellectual property protection for our drug product.

In that regard, in October 2020, we filed lawsuits against Jacobus and the specialty pharmacy marketing Ruzurgi®, PantherRx Rare LLC (PantherRx), for infringement of the '893 patent. The suits have now been consolidated in a single action in the U.S. District Court for New Jersey. Further, in August 2021, the lawsuits were amended to include alleged infringement of the '128 patent. The lawsuits arise from Jacobus' and PantherRx's sales and marketing of Ruzurgi® (amifampridine) Tablets, 10 mg. The lawsuits allege that the Ruzurgi® product infringes the '893 patent and the '128 patent when administered in accordance with its product labeling. The lawsuit seeks damages and injunctive relief to prevent further marketing of Ruzurgi® in violation of our patent rights. The lawsuit is in the discovery stage and there can be no assurance as to the results of these proceedings.

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, there is a possibility we may be prevented from pursuing product development, manufacturing or commercialization of our products that utilize such technologies until the underlying patent dispute is resolved. 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.

Generic Sabril®

In December 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. If and when the product is launched, 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 we are obligated to share the costs of certain development expenses. 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 abbreviated new drug application (ANDA) is approved for vigabatrin tablets in the future, that it will be profitable to us.

Capital Resources

At December 31, 2021, we had cash and investments of approximately $191.3 million. 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® or will continue to be profitable. Further, there can be no assurance that if we need additional funding in the future, whether such funding will be available to us. 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 supporting its commercialization in Canada. We are working to expand awareness of the disease, including to physicians treating LEMS patients with small-cell lung cancer, and helping health care providers and their patients understand the benefits of FIRDAPSE®. A cornerstone of our U.S. strategy is our continuing development of Catalyst Pathways®, our personalized treatment support program, and our development of the patient assistance programs that are required to further our goal that no LEMS patient be denied access to FIRDAPSE® for financial reasons within existing legal restrictions.
- Seek approval for FIRDAPSE® in Japan. We are currently supporting our sub-licensee, DyDo, as they begin taking necessary steps to seek approval for FIRDAPSE® in Japan for the treatment of patients with LEMS.
- Seek to acquire additional products. We recently made a strategic decision 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, no products have been acquired to date.

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 Agreement for FIRDAPSE®

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, we have an option to further expand our 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, 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.

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.

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 was recently 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®. Finally, the FDA required us to perform a second carcinogenicity study of amifampridine phosphate in mice, which we have completed and submitted to the FDA.

Expanded access program

We operate an expanded access program (EAP) that is currently making FIRDAPSE® available to a limited number of patients diagnosed with CMS or Downbeat Nystagmus (DN). It is anticipated that the EAP may be used to make FIRDAPSE® available to pediatric LEMS patients now that the Ruzurgi® approval has been vacated, subject to applicable legal and regulatory requirements.

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 have a field-based force of five 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, MuSK antibody positive myasthenia gravis, and other neuromuscular diseases that may be treatable with FIRDAPSE®, 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 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 for enrolled patients. 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 drug 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.

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

Access to FIRDAPSE®

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

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

FDA approval of Ruzurgi® for pediatric LEMS patients (ages 6 to under 17)

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). We believe that the vast majority of Ruzurgi® sales are to adult LEMS patients who are being prescribed the drug off label. If Jacobus is able to successfully continue to sell Ruzurgi® off-label to additional adult LEMS patients, it could have a material adverse effect on our business, financial condition and results of operations.

We believe that the FDA's approval of Ruzurgi® violated our statutory rights and was in multiple other respects arbitrary, capricious and contrary to law. As a result, 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 Federal Food, Drug, and Cosmetic Act (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 seeks 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 our motion for summary judgment. On September 29, 2020, the District Judge adopted the Report and Recommendation of the Magistrate Judge, granted the FDA's and Jacobus' motions for summary judgment, and dismissed our case. We appealed the District Court's decision to the U.S. Court of Appeals for the 11th Circuit. The case was fully briefed in early 2021, and oral argument was held in March 2021.

On September 30, 2021, a three-judge panel of 11th Circuit judges issued a unanimous decision overturning the District Court's decision. The appellate court adopted our argument that the FDA's approval of Ruzurgi® violated our rights to Orphan Drug Exclusivity and remanded the case to the District Court with orders to enter summary judgment in our favor. In November 2021, Jacobus filed a motion

seeking rehearing of the case from the full 11th Circuit, which motion was denied in January 2022. Further, in January 2022, Jacobus filed motions with both the 11th Circuit and the U.S. Supreme Court seeking a stay of the 11th Circuit's ruling indicating that it would seek a review of the 11th Circuit's decision from the U.S. Supreme Court. Both stay motions were denied, and on January 28, 2022, the 11th Circuit issued a mandate directing the District Court to enter summary judgment in our favor. The District Court entered that order on January 31, 2022. On February 1, 2022, the FDA informed Jacobus that, consistent with the Court of Appeals for the Eleventh Circuit's September 30, 2021, decision in favor of Catalyst, the final approval of the Ruzurgi® NDA was switched to a tentative approval until the 7-year orphan-drug exclusivity (ODE) for Firdapse® has expired.

There can be no assurance as to whether Jacobus will seek U.S. Supreme Court review of the 11th Circuit's decision, whether the U.S. Supreme Court will agree to hear the case, or whether, if the U.S. Supreme Court agrees to hear the case, Jacobus' appeal to overturn the decision of the 11th Circuit will be successful. Similarly, there can be no assurance as to whether the U.S. Congress will pass, and the President will sign, legislation revising the Orphan Drug Act that effectively overturns the decision of the U.S. Court of Appeals for the 11th Circuit, and whether any such legislation, if passed and signed into law, will retroactively affect the outcome of the 11th Circuit decision and allow the FDA to reinstate the approval of Ruzurgi® before the expiration of FIRDAPSE®'s orphan drug exclusivity.

Third-Party Reimbursement

Sales of drug 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 to be provided for FIRDAPSE® 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 product.

There can be no assurance, however, as to whether payors will continue to cover our product, 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.

Our efforts to develop FIRDAPSE® as a treatment for additional neuromuscular indications

Over the past few years, we have studied FIRDAPSE® as a potential treatment for multiple neuromuscular indications other than LEMS, and the results of recent studies are summarized below. Based on the results of these activities, in 2021 we have made a strategic decision not to proceed forward to further study FIRDAPSE® as a potential treatment for additional indications.

MuSK-MG studies

In February 2016, we initiated an investigator-sponsored, randomized, double-blind, placebo-controlled, crossover proof-of-concept clinical trial evaluating the safety, tolerability and potential efficacy of FIRDAPSE® as a symptomatic treatment for patients with MuSK-MG. Seven patients participated in this proof-of-concept trial. On March 15, 2017, we reported top-line results from this trial. Both of the co-primary efficacy endpoints of change from baseline (CFB) in total Quantitative Myasthenia Gravis (QMG) score (p=0.0003) and CFB in total Myasthenia Gravis Activities of Daily Living (MG-ADL) score (p=0.0006) were statistically and clinically significant in this trial. Several secondary efficacy measures also achieved statistical significance. Amifampridine phosphate was well tolerated in this population of patients. The results of this study were published in SAGE Open Medicine and can be accessed at https://journals.sagepub.com/doi/pdf/10.1177/2050312118819013. Subsequently, we engaged in a Phase 3 clinical trial (MSK-002) evaluating FIRDAPSE® for the treatment of adults with MuSK-MG. Our trial was a multi-site, international (United States, Italy and Serbia), double-blind, placebo-controlled, clinical trial being conducted under a Special Protocol Assessment (SPA) with the FDA. The trial enrolled more than 60 MuSK antibody positive patients. It also enrolled more than 10 generalized myasthenia gravis patients who were assessed with the same clinical endpoints. However, achieving statistical significance in this subgroup of patients was not required.

On August 10, 2020, we announced the top-line results from our Phase 3 clinical trial (MSK-002) evaluating FIRDAPSE® for the treatment of adults with MuSK-MG. Unfortunately, the MSK-002 trial did not achieve statistical significance on its primary endpoint or its secondary endpoint, even though clinical improvement was observed by patients and investigators during the initial dose-titration period of the trial and in the company's previous proof-of-concept trial. However, we have recently concluded a detailed analysis of the data from this trial in an effort to understand why the MuSK-MG Phase 3 trial did not meet statistical significance on its endpoints.

Following our receipt of these results, we analyzed the data and proposed a plan to the FDA to perform an additional study evaluating FIRDAPSE® for MuSK-MG. In response, the FDA provided written comments that were unfavorable towards our proposed revised study design and further questioned the ability of the initial MuSK-MG pilot study to be supportive. These remarks make it unlikely that a single study design similar to MSK-002 would be sufficient for potential approval of the MuSK-MG indication. We also held an appropriate expert panel to discuss options and review the likelihood for success for a MuSK-MG indication for FIRDAPSE®. Based on the input from the FDA and advisors, we have concluded that the approval of FIRDAPSE® as a first-line therapy for MuSK-MG is unlikely and therefore we have decided not to continue to pursue this indication.

Proof-of-concept clinical trial evaluating FIRDAPSE® for the treatment of SMA Type 3

Our exploratory study, SMA-001 (A Randomized Placebo Controlled Crossover Study to Evaluate the Safety and Efficacy of Amifampridine Phosphate in Ambulatory Patients with Spinal Muscular Atrophy (SMA) Type 3, met the primary endpoint of a statistically significant difference for the Hammersmith Functional Motor Scale Expanded (HFMSE). Clinically, however, the effect was modest. The secondary endpoints were not statistically significant, although several individual quality of life measures demonstrated a positive nominally statistically significant change. Key opinion leaders with whom we have spoken believed that FIRDAPSE® needed to show a large clinically significant change if there was the possibility to affect disease progression through retrograde signaling from enhanced neuromuscular junction function. After considering all of these factors, we have concluded that the modest results exhibited in this study are unlikely to result in a sufficient modification of disease progression, and, particularly in light of the fact that there are now three approved disease modifying medications for SMA Type 3, we have decided not to pursue the SMA Type 3 indication further.

Study to evaluate FIRDAPSE® as a treatment for HNPP

We previously announced our intent to conduct a proof-of-concept study evaluating FIRDAPSE® as a treatment for Hereditary Neuropathy with Liability to Pressure Palsies (HNPP). The FDA requested that a new, patient centric endpoint be researched and used for our proposed study, without assurance that such endpoint would be acceptable for approval. Based upon the uncertainty of such an endpoint, we have decided not to conduct this study as a company sponsored study, though there is a possibility that this study will move forward as investigator-initiated study that we will support.

Long-acting version of amifampridine phosphate

We were previously developing a long-acting formulation of amifampridine phosphate. While a number of formulations were prepared, after discussions with researchers and an advisory board made up of both patients and physicians, we concluded that we would be unable to develop a long-acting formulation that is both beneficial to patients and commercially viable, and as a result we made the determination not to proceed with development of this product.

Intellectual property and regulatory exclusivity protections for FIRDAPSE®

All of our patent rights for FIRDAPSE® are derived from our license agreement. 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, 17/503,190. On January 3, 2022, the USPTO allowed related continuing application 17/503,148. A further related continuing application, 17/503,092 was allowed on January 7, 2022. All three patents were issued in March 2022. The claims in each of these applications have either already been listed in the Orange Book for FIRDAPSE® or are in the process of being listed.

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 which provide additional intellectual property protection for our drug product.

In that regard, in October 2020, we filed lawsuits against Jacobus and the specialty pharmacy marketing Ruzurgi®, PantherRx Rare LLC (PantherRx), for infringement of the ‘893 patent. The suits have now been consolidated in a single action in the U.S. District Court for New Jersey. In August 2021, the lawsuits were amended to include alleged infringement of the ‘128 patent. The lawsuits arise from

Jacobus' and PantherRx's sales and marketing of Ruzurgi® (amifampridine) Tablets, 10 mg. The lawsuits allege that the Ruzurgi® product infringes the '893 patent and the '128 patent when administered in accordance with its product labeling. The lawsuit seeks damages and injunctive relief to prevent further marketing of Ruzurgi® in violation of our patent rights. The lawsuit is in the discovery stage and there can be no assurance as to the results of these proceedings.

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 abbreviated new drug application (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 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 will be permitted to submit ANDA filings to the FDA as early as the "NCE-1" date (November 28, 2022). In the event of such filings, and after appropriate investigation, we intend to vigorously enforce our patent rights.

We have 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."

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. Pursuant to the agreement, in December 2018, we received an up-front payment of $0.5 million. 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.

Sabril® is marketed by Lundbeck in the United States for infantile spasms and for refractory complex partial seizures. Lundbeck's sales of Sabril® (tablets and sachets) were approximately $129.0 million in 2019 and $118.3 million in 2020. Four generic versions of Sabril® tablets have 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.

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 abbreviated new drug application (ANDA) is approved for vigabatrin tablets in the future, that it will be profitable to us.

Plans to Acquire or In-License Additional Products

In 2021, we made a strategic decision 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.

We are currently exploring several potential opportunities to acquire companies with drug products in development or to in-license or acquire drug products in development. However, no definitive agreements have been entered into to date. Further, during the third quarter of 2021 we hired Dr. Preethi Sundaram, who serves as our Chief Strategy Officer. In that position, Dr. Sundaram is leading our efforts to acquire R&D assets, from early stage through late-stage clinical programs and technologies to treat rare diseases, and once such drug candidates are acquired, Dr. Sundaram will help oversee the development of those assets.

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.

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.

Any significant change that we make for FIRDAPSE® must be approved by the FDA in a supplemental new drug application (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 current Good Manufacturing Practices (cGMPs) regulations and policies. Further, even if we receive approval of any sNDAs for FIRDAPSE®, 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.

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.

For some years, Ruzurgi® for the treatment of pediatric patients with LEMS was often proscribed to adult LEMS patients, and at a lower price than FIRDAPSE®. Now that Ruzurgi® is no longer on the market, it is no longer competitive to FIRDAPSE®. However, if Ruzurgi® were to become available in the future, it would likely be competitive to FIRDAPSE®.

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 new drug or abbreviated new drug applications).

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 Federal Food, Drug, and Cosmetic Act, 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.

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.

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 Federal Food, Drug, and Cosmetic Act (FFDCA) 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, or GLP, regulations;
- submission of an investigational new drug application, or 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, or 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, or 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 2022 this fee is $3,117,218), 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 2022 is $369,413 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, or 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.

Special Protocol Assessments

A SPA is a process in which sponsors may request to meet with the FDA to reach agreement on the design and size of certain clinical trials, clinical studies, or animal trials to determine if they adequately address scientific and regulatory requirements. As part of this process, sponsors submit specific questions about protocol design and scientific and regulatory requirements. After the FDA completes the review of a SPA request, the FDA may issue a SPA Letter, including an assessment of the protocol, agreement or non-agreement with the proposed protocol, and answers to the sponsor's relevant questions.

A SPA agreement indicates concurrence by the FDA with the adequacy and acceptability of specific critical elements of overall protocol design (e.g., entry criteria, dose selection, endpoints, and planned analyses). These elements are critical to ensuring that the trial conducted under the protocol has the potential to support a future submitted application's ability to meet regulatory requirements for approval. Feedback on these issues provides the greatest benefit to sponsors in planning late-phase development strategy. However, a SPA agreement does not indicate FDA concurrence on every protocol detail. Further, the FDA may rescind a SPA if the director of the FDA reviewing division determines that a substantial scientific issue essential to determining the safety or efficacy of the drug was identified after the trial began. Thus, a SPA is not binding on the FDA if, for example, the Agency identifies a safety concern related to the product or its pharmacological class, if the FDA or the scientific community recognizes a paradigm shift in disease diagnosis or management, if the relevant data or assumptions provided by the sponsor in the SPA submission are found to be false or misstated, or if the sponsor fails to follow the protocol that was agreed upon with the FDA. The FDA retains significant latitude and discretion in interpreting the terms of a SPA agreement and the data and results from the applicable clinical trial.

Because a SPA provides for the evaluation of protocols for trials that have not been initiated, the conduct and results of the subsequent trial are not part of the evaluation. Therefore, the existence of an SPA agreement does not guarantee that the FDA will accept an NDA, or that the trial results will be adequate to support approval. Those issues are addressed during the review of a submitted application; however, it is hoped that trial quality will be improved by the SPA process.

Post-approval requirements and consideration

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, or 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, or 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 NDA supplement before the change can be implemented. An NDA supplement 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.

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, or 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) New Drug Applications

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 for an already approved product, the applicant is required to certify to the FDA concerning any patents listed for the approved product in the Orange Book to the same extent that an ANDA applicant would. A Section 505(b)(2) NDA may be eligible for three years of marketing exclusivity to the same extent that a Section 505(b)(1) NDA is.

Abbreviated new drug applications

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 2022, the application fees are $225,712 per ANDA application and the facility fees are $195,012 per domestic finished dosage form facility, $210,012 per foreign finished dosage form facility, $42,557 per domestic active pharmaceutical ingredient facility, and $57,557 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”). In fact, there continue to be efforts in Congress to revise the Affordable Care Act and replace it with another law. As a result, there is great uncertainty as to what changes will be made to United States healthcare laws and there can be no assurance how changes to those laws may affect our business.

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:

- controls on government-funded reimbursement for drugs;
- mandatory rebates or additional charges to manufacturers for their products to be covered on Medicare Part D formularies;
- controls on healthcare providers;
- controls on pricing of drug products, including the possible reference of the pricing of United States drugs to non-United States drug pricing for the same product;
- 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 drug 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 Senator Bernie Sanders 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 drug products generally cannot be determined with any certainty at this time.

Orphan Drug Exclusivity and Pediatric Exclusivity Designation

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, and a recent proposed change to the ODA would limit the availability of the benefits of the act for drugs that treat more than 200,000 individuals in the United States. There can be no assurance that the exclusivity granted in 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.

Pediatric exclusivity is another type of non-patent exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the five-year and three-year non-patent and seven-year orphan exclusivities. This six-month exclusivity may be granted if an NDA sponsor submits pediatric data that fairly responds to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied. If the FDA determines that information relating to the use of the new drug in the pediatric population may produce health benefits in the population, the clinical study is deemed to fairly respond to the FDA's request and the reports of FDA-requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or patent protection covering the product are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot approve another application relying on the NDA sponsor's data.

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.

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. 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 abbreviated new drug applications, 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 beneficiaries 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 beneficiaries pay 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 beneficiaries in the coverage gap, in order for its drugs to be reimbursed by Medicare Part D.

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 14, 2022, we had approximately 76 employees, approximately 32 of whom are in our commercial organization, approximately 23 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 work with our commercial organization. None of our employees are covered by a collective bargaining agreement. We believe our relationship with our employees and consultants is good.

Compensation and Benefits

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

Diversity, Equity and Inclusion

Our goal is a diverse and inclusive workforce – not because it is the right thing to do but because we believe that such a workforce is key to our long-term success. Approximately 58% of our employees are female. At the leadership level (employees at manager and above) approximately 64% 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 held an 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 plan to hold further such meetings as the course of the COVID-19 pandemic allows.

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 delta and omicron variants, 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. The content on our website is not, nor should it be deemed to be, incorporated by reference into this report.

Item 1A. Risk Factors

Risk Factors Summary

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

Risks related to the commercialization of FIRDAPSE®

- We depend substantially on the commercial success of FIRDAPSE®.
- Our success depends on our ability to continue to successfully commercialize FIRDAPSE®. We are primarily a single product company with only limited commercial experience, which makes it difficult to evaluate our current business, predict our future prospects and forecast our financial performance and growth.
- If we are unable to continue to successfully commercialize FIRDAPSE®, our business, results of operations and financial condition may be materially adversely affected.
- Our business is subject to substantial competition.
- Our strategy of seeking to acquire or in-license innovative technical platforms or earlier stage drug development programs in the rare disease space may not be successful.
- Our business may require additional capital.
- The obligations incident to being a public company place significant demands on our management.
- We are highly dependent on our small number of key personnel and advisors.

- 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.
- We face a risk of product liability claims and may not be able to obtain adequate insurance.
- Business or economic disruptions or global health concerns could seriously harm our development efforts and increase our costs and expenses.

Risks Related to the Development of Additional Drug Products

- Our efforts may fail.
- 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 FIRDAPSE® in all areas in which we are licensed to supply 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.
- 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.
- FIRDAPSE® is subject to ongoing 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 commercialize FIRDAPSE® or 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

- We are dependent on our relationships and license agreements, and we rely upon the patent rights granted to us pursuant to the license agreements.
- 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

We depend substantially on the commercial success of FIRDAPSE®.

Until we launched FIRDAPSE® for the treatment of LEMS in January 2019, we focused all of our efforts over the prior six years on obtaining regulatory approval for FIRDAPSE® for the treatment of LEMS, on evaluating FIRDAPSE® for the treatment of other neuromuscular diseases including CMS, MuSK-MG and SMA Type 3, on raising capital, and on recruiting personnel. On November 28, 2018, the FDA approved our first product, FIRDAPSE® for the treatment of adults with LEMS, which became commercially available in January 2019. While we reported net income in each year since 2019, we have a prior history of operating losses in all prior fiscal years of our existence. In addition, we have recently concluded that we will no longer go forward with the evaluation of FIRDAPSE® for the treatment of indications other than LEMS. As a result of this and other factors, there can be no assurance that we will remain cash flow positive and profitable.

Our success depends on our ability to continue to successfully commercialize FIRDAPSE®. We are primarily a single product company with only limited commercial experience, which makes it difficult to evaluate our current business, predict our future prospects, and forecast our financial performance and growth.

We have invested a significant portion of our efforts and financial resources to date into the development and commercialization of our lead product, FIRDAPSE®. Our success depends on our ability to effectively continue to commercialize FIRDAPSE®, and we expect that the vast majority of our product revenues in the foreseeable future will be from sales of FIRDAPSE®. Continued commercialization of FIRDAPSE® is subject to many risks. Until we launched FIRDAPSE®, we had never launched or commercialized a product, and there is no guarantee that we will be able to continue to be profitable and cash flow positive based on our sales of FIRDAPSE®. There are numerous examples of unsuccessful product launches and failures to meet high expectations of market growth potential, including by pharmaceutical companies with more resources and experience than we have. The long term commercial success of FIRDAPSE® depends on the extent to which patients and physicians accept and adopt FIRDAPSE®. For example, if the expected patient population is smaller than we estimate or if physicians are unwilling to prescribe or patients are unwilling to take FIRDAPSE®, or if patients discontinue from use of the medication at rates that are higher than we expect, or if payers decide not to reimburse for our product, the commercial potential of FIRDAPSE® will be limited. Thus, despite our success to date, significant uncertainty remains regarding the ultimate commercial potential of FIRDAPSE®.

Moreover, our ability to effectively continue to generate significant product revenue from FIRDAPSE® will depend on our ability to, among other things:

- educate patients and physicians successfully about efficacy expectations, side effects expectations, and how to successfully dose and titrate the medication to optimal patient benefit in order to minimize discontinuation due to perceived lack of efficacy or side effects;
- educate LEMS patients who also suffer from small cell lung cancer, and the physicians who treat them, as to the benefits to such patients of treatment for their LEMS using FIRDAPSE® (in addition to the treatments they are receiving for their cancer);
- achieve and maintain compliance with regulatory requirements, including those related to our required post-approval studies, promotion and advertising requirements;
- increase awareness for and achieve market acceptance of FIRDAPSE® through our sales and marketing activities and other arrangements established for the promotion of FIRDAPSE®;
- train, deploy, support, and retain a qualified field sales and marketing force;
- secure continued formulary approvals for FIRDAPSE® with a substantial number of targeted payors;
- ensure that our third-party manufacturers manufacture FIRDAPSE® in sufficient quantities, in compliance with requirements of the FDA and at acceptable quality and pricing levels, in order to meet commercial demand;
- ensure that our third-party manufacturers develop, validate and maintain commercially viable manufacturing processes that are compliant with current Good Manufacturing Practice (cGMP) regulations;
- implement and maintain agreements with wholesalers, distributors and group purchasing organizations on commercially reasonable terms;
- ensure that our entire supply chain efficiently and consistently delivers FIRDAPSE® to our customers;
- provide co-pay assistance to help qualified patients with out-of-pocket costs associated with their FIRDAPSE® prescription, and/or other programs to ensure patient access to our products, educate physicians and patients about the benefits, administration and use of FIRDAPSE®, and obtain acceptance of FIRDAPSE® as safe and effective by patients and the medical community;
- receive adequate levels of coverage and reimbursement for FIRDAPSE® from commercial health plans and governmental health programs;
- generate positive experience with our Catalyst Pathways® program in helping patients obtain access to FIRDAPSE® at an acceptable patient out-of-pocket cost;
- maintain quality relationships with patient advocacy groups;
- influence the nature of publicity related to our product relative to the publicity related to our competitors' products; and
- obtain regulatory approvals for additional indications for the use of FIRDAPSE® in treating other rare neuromuscular diseases.

Any disruption in our ability to generate product revenue from the sale of FIRDAPSE® will have a material and adverse impact on our results of operations.

If we are unable to continue to successfully commercialize FIRDAPSE®, our business, results of operations and financial condition may be materially adversely affected.

Our strategy is to continue to successfully commercialize FIRDAPSE® in the United States. There are risks involved both with maintaining our own sales and marketing capabilities, and with entering into arrangements with third parties to perform these services. For example, any efforts to maintain a direct sales and marketing organization are subject to numerous risks, including:

- the expense and time required to recruit, retain, and motivate members of the sales force;

- our inability to recruit, retain or motivate adequate numbers of effective marketing personnel and partner marketing agencies;
- the inability to provide adequate training to sales and marketing personnel;
- the expense and time required to monitor regulatory compliance;
- the inability of sales personnel to obtain access to physicians or convince adequate numbers of physicians to prescribe any product; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

Similarly, as we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenue or the profitability associated with any product revenue may be lower than if we were to market and sell any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our products or may be unable to do so on terms that are favorable to us. We may have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. Moreover, we may be negatively impacted by other factors outside of our control relating to such third parties, including, but not limited to, their inability to comply with regulatory requirements. If we do not establish sales, marketing and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our products.

Finally, because we are using a very small group of exclusive specialty pharmacies to distribute our product, if the organizations that we work with to deliver our drug do not perform in a lawful manner or have issues unrelated to our business, our business could be adversely affected.

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 drug 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.

Even with the FDA approval of FIRDAPSE®, the bulk active pharmaceutical ingredient in the drug (i.e., amifampridine) may be used by compounding pharmacies pursuant to Section 503A of the Federal Food, Drug, and Cosmetic Act because the ingredient is a component of an FDA-approved drug product, and pharmacies may lawfully compound for individually identified patients under Section 503A using components of approved drug products. In addition, drugs that are not approved by FDA for the treatment of LEMS may nonetheless be prescribed by physicians for the treatment of LEMS.

For all of these reasons, we may not be able to continue 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 have made a strategic decision 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 including those 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.

The in-licensing and acquisition of drug products is an area characterized by intense competition, and a number of companies (both more established and early-stage biotechnology companies) are also pursuing strategies to in-license or acquire product candidates or technologies that we may consider attractive. We believe that other companies may be particularly active in pursuing opportunities to in-license or acquire the same or similar products which we may seek to acquire. More established companies may have a competitive advantage over us due to their size, cash resources and greater research, preclinical or clinical development or commercialization capabilities, while earlier stage companies may be more aggressive or have a higher risk tolerance. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to in-license or acquire the rights to the relevant product candidate or technology on terms that would allow us to make an appropriate return on our investment. Moreover, we may devote resources to potential acquisitions or in-licensing opportunities that are never completed, or we may fail to realize the anticipated benefits of such efforts or we may incorrectly judge the value of an acquired or in-licensed product candidate or technology.

If we are unable to successfully obtain rights to suitable product candidates or technologies, our business, financial condition and prospects for growth could suffer. In addition, acquisitions and in-licensing arrangements for product candidates and technologies are inherently risky, and ultimately, if we do not complete an announced acquisition or license transaction or integrate an acquired or licensed product candidate or technology successfully and in a timely manner, we may not realize the benefits of the acquisition or license to the extent anticipated and the perception of the effectiveness of our management team and our company may suffer in the marketplace. In addition, even if we are able to successfully identify, negotiate and execute one or more transactions to acquire or in-license new product candidates or technologies, our expenses and short-term costs may increase materially and adversely affect our liquidity.

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.

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.

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.

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.

During 2020 and 2021, we believe that the COVID-19 pandemic impacted new patient starts in the United States, as some physicians were reluctant to diagnose LEMS via telemedicine. To the extent that the COVID-19 pandemic continues, we may continue to see an impact on the number of naïve patients who begin to take FIRDAPSE®. There can be no assurance as to how these matters will affect our business or results of operations.

We cannot assess the impact on our business of the public concerns expressed by a U.S. Senator and a vocal group of neuromuscular physicians and patients with LEMS 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.

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 drug products. Patients have received substantial damage awards in some jurisdictions against pharmaceutical companies based on claims for injuries allegedly caused by the use of drug 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.

Risks Related to the Development of Drug Products

Our efforts to develop additional drug products may fail.

Our efforts to develop additional products that we may acquire or in-license (or to develop additional indications for FIRDAPSE®) are subject to risks of failure. For example:

- Our drug candidates may be found to be ineffective or unsafe for one or more additional indications, or fail to receive necessary regulatory approvals;
- Our drug candidates may not be economical to market or take substantially longer to obtain necessary approvals for additional indications than anticipated; or
- Competitors may develop and market equivalent or superior products, including next generation products that act with the same mechanism of action as our drug candidates.

As a result, our drug development activities may not result in any safe, effective and commercially viable additional indications, and we may not be able to commercialize our products successfully. For example, for several years, we evaluated FIRDAPSE® for the treatment of CMS, MuSK-MG and SMA Type 3. However, FIRDAPSE® failed to meet the primary endpoints in a Phase 3 trial for CMS, and we are no longer pursing this indication. Further, and even though we achieved statistical significance in the primary endpoint in our proof-of-concept trial for SMA Type 3, the lack of robust results in this trial has caused us to decide to no longer pursue this indication. Finally, our Phase 3 clinical trial (MSK-002) evaluating FIRDAPSE® for the treatment of adults with MuSK-MG did not achieve statistical significance on its primary endpoint or its secondary endpoint, even though clinical improvement was observed by patients and investigators during the initial dose-titration period of the trial and in the company's previous proof-of-concept trial, and we are no longer pursuing this indication.

Further, the efforts of our collaboration partner in Japan, DyDo Pharma, to commercialize FIRDAPSE® in Japan requires successful completion of a small Phase 3 trial in Japan evaluating FIRDAPSE® for the treatment of LEMS that is currently ongoing. While we expect that trial to be successful based on our prior trials evaluating our product for the treatment of LEMS, there can be no assurance that DyDo Pharma's trial will be successful.

Our failure to develop safe, effective, and/or commercially viable products would have a material adverse effect on our business, prospects, results of operations and financial condition.

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, including FIRDAPSE® and any other products we are able to market in the future, 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 drug 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. Former President Trump frequently discussed his intention to reduce drug prices, as has President Biden. The Trump Administration solicited public comment on a variety of regulatory proposals to reduce drug prices, and the Centers for Medicare and Medicaid Services (Center) published an interim final rule that establishes a Most Favored Nation (MFN) Model for Medicare Part B drug payment. This regulation would substantially change the drug reimbursement landscape as it bases Medicare Part B payment for 50 selected drugs on prices in foreign countries instead of average sales price, or ASP. The MFN drug payment amount was expected to be lower than the current ASP-based payment limit because United States drug prices are generally the highest in the world. While the MFN Model payment methodology was scheduled to begin on January 1, 2021, by the end of December 2020, three federal courts had granted orders preventing implementation of the MFN Model rule. On August 6, 2021, the Center published a proposed rule rescinding the November 2020 MFN Model interim final rule, and on December 27, 2021, CMS published a final rule withdrawing the MFN Model effective February 28, 2022. In its release, the Center stated that it will consider stakeholder feedback as it explores options to incorporate value into payments for Medicare Part B drugs, improving access to evidence-based care, and reducing drug spending for consumers throughout the health care system. Further, since President Biden took office, there have been continuing efforts in Congress and through the administration to reduce drug prices.

For example, on November 20, 2020, the United States Department of Health and Human Services (HHS) finalized a regulation removing safe harbor protection under the Federal Anti-Kickback Statute for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law or unless it is passed through to the dispensing pharmacy and reflected in the price to the patient. The implementation of the rule has been delayed by the Biden administration to January 1, 2023 in response to ongoing litigation. In addition, effective January 1, 2024, a provision capping the rebate amount under the Medicaid Drug Rebate program at 100% of AMP will be eliminated, which means that a manufacturer could pay a rebate amount on a unit of the drug that is greater than the price the manufacturer receives for the drug. Further, effective January 1, 2023, a final rule issued by CMS will change the way copay assistance program prices are treated in best price for purposes of the Medicaid Drug Rebate Program. This change could result in manufacturers eliminating their patient assistance programs, which would make many innovator drugs more expensive for patients. This final rule is subject to ongoing litigation, but it is not clear when a decision will be made or how the court will rule.

On September 9, 2021, the Biden administration published a wide-ranging list of policy proposals, most of which would need to be carried out by Congress, to reduce drug prices and drug payment. The HHS plan includes, among other reform measures, proposals to lower prescription drug prices, including by allowing Medicare to negotiate prices and disincentivizing price increases, and to support market changes that strengthen supply chains, promote biosimilars and generic drugs, and increase price transparency. Many similar proposals, including the plans to give Medicare Part D authority to negotiate drug prices, require drug manufacturers to pay rebates on drugs whose prices increase greater than the rate of inflation, and cap out-of-pocket costs, have already been included in policy statements and legislation currently being considered by Congress. It is unclear to what extent these and other statutory, regulatory, and administrative initiatives will be enacted and implemented, and to what extent these or any future legislation or regulations by the Biden administration will have an effect 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 drug 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 in the future to manufacture and commercialize FIRDAPSE® or other products we may develop in the future in all areas in which we are licensed to supply it.

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.

Additionally, future clinical trials for FIRDAPSE® or any other drug candidate we may acquire may not be successfully completed or may take longer than anticipated because of any number of factors, including potential delays in the start of the trial, an inability to recruit clinical trial participants at the expected rate, failure to demonstrate safety and efficacy, unforeseen safety issues, or unforeseen governmental or regulatory delays. Further, our drug candidate may not be found to be safe and effective in particular indications and

may not be approved by regulatory authorities for the proposed indication. Further, regulatory authorities and IRBs that must approve and monitor the safety of each clinical study may suspend a clinical study at any time if the patients participating in such study are deemed to be exposed to any unacceptable health risk. We may also choose to suspend human clinical studies and trials if we become aware of any such risks. We might encounter problems in our clinical trials, including our expanded access program, such as seizures, weakness or other side effects that will cause us, regulatory authorities, or IRBs to delay or suspend such trial or study. Moreover, FDA will consider the data, including safety data, from patients enrolled in any expanded access program we may implement in the evaluation of any NDA or sNDA we may submit.

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 candidates, including FIRDAPSE®. 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.

FIRDAPSE® is subject to ongoing 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 Federal Food, Drug, and Cosmetic Act, 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.

The Medicare Prescription Drug Improvement and Modernization Act of 2003, or MMA, changed the way Medicare covers and pays for drug products. The legislation expanded Medicare coverage for outpatient drug purchases by those covered by Medicare under a new Part D and introduced a reimbursement methodology based on average sales prices for Medicare Part B physician-administered drugs. In addition, this legislation authorized Medicare Part D prescription drug plans to use formularies whereby they can limit the number of drugs that will be covered in any therapeutic class. As a result of this legislation and the expansion of federal coverage of drug products, there is additional pressure to contain and reduce costs. While the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates, and any reduction in reimbursement that results from the MMA may result in a similar reduction in payments from private payors. These cost reduction initiatives and other provisions of the MMA could decrease the coverage and reimbursement that we receive for any approved products and could seriously harm our business. Manufacturers’ contributions to this area, including donut hole coverage (as described below) or potential excise taxes, are increasing and are subject to additional changes in the future.

In 2010, former President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (together, the “Health Care Reform Law”), a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry, and impose additional health policy reforms. The Health Care Reform Law, among other things, revised the definition of Average Manufacturer Price used by the Medicaid Drug Rebate Program for reporting purposes, imposed a significant annual fee on companies that manufacture or import branded prescription drug products and established an annual non-deductible fee on entities that sell branded prescription drugs or biologics to specified government programs in the United States. The Health Care Reform Law also expanded the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance and included a discount (now 70%, on brand name drugs for Medicare Part D participants in the coverage gap, or “donut hole.” The Health Care Reform Law increased the Medicaid rebates for line extensions or reformulated drugs, which could substantially increase our Medicaid rebate rate (in effect limiting reimbursement for these patients).

Beginning in January 2017, former President Trump signed two Executive Orders and other directives designed to delay the implementation of certain provisions of the Health Care Reform Law or otherwise circumvent some of the requirements for health insurance mandated by the Health Care Reform Law. These actions include directing applicable federal agencies to waive, defer, grant exemptions from, or delay the implementation of any provision of the Health Care Reform Law that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. On October 13, 2017, an Executive Order was signed terminating the cost sharing subsidies that reimburse insurers under the Health Care Reform Law. Several state Attorneys Generals filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. Further, on June 14, 2018 the United States Court of Appeals for the Federal Circuit ruled that the federal government was not required to pay more than $12.0 billion in Health Care Reform Law risk corridor payments to third-party payors. The effects of this gap in reimbursement on third-party payors, the viability of the Health Care Reform Law marketplace, providers, and our business, are not yet known. On December 18, 2019, the United States Court of Appeals for the Fifth Circuit ruled that the Health Care Reform Law's individual mandate is unconstitutional but sent the matter back down to a district court to determine whether that provision can be removed from the rest of the Health Care Reform Law. On March 2, 2020, the U.S. Supreme Court agreed to review the Fifth Circuit's ruling, and oral argument was heard on November 10, 2020. On June 17, 2021, the U.S. Supreme Court dismissed the challenge to the Health Care Reform Law in a 7-2 decision.

Additionally, in response to controversies regarding pricing of drug products, there has been a recent push to propose legislation, both on state and federal levels, that would require greater disclosure as to the reasoning behind drug prices and, in some cases, could give state or federal-level commissions the right to impose cost controls on certain drugs. These and other new provisions are likely to continue the pressure on pharmaceutical pricing, may require us to modify our business practices with healthcare practitioners, and may also increase our regulatory burdens and operating costs. In that regard, President Biden and members of Congress in both parties have expressed concerns about high drug prices. However, whether and to what extent any such positions will result in changes of the law, and how any such changes could impact our business, cannot be determined at this time.

Legislative and regulatory proposals also have been made to expand post-approval requirements, restrict sales and promotional activities for drug 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 whether the exclusivity provisions in the Orphan Drug Act may be changed in the future and the impact of such changes, if made on us. For example, if the United States Congress were to pass, and the President were to sign, legislation revising the Orphan Drug Act that effectively overturns the decision of the U.S. Court of Appeals for the 11th Circuit, such legislation might retroactively affect the outcome of the 11th Circuit decision and allow the FDA to reinstate the approval of Ruzurgi® before the expiration of Firdapse®'s orphan drug exclusivity.

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 laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug and therapeutic biologics manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures and pricing information, state and local laws that require the registration of pharmaceutical sales representatives, and state laws governing 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

We are dependent on our relationships and license agreements, and we rely upon the patent rights granted to us pursuant to the license agreements.

All of our patent rights for FIRDAPSE® are derived from our license agreement. In August 2020, the United States Patent and Trademark Office (USPTO) allowed U.S. 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, 17/503,190. On January 3, 2022, the USPTO allowed related continuing application 17/503,148. A further related continuing application, 17/503,092 was allowed on January 7, 2022. All three patents were issued in March 2022. The claims in each of these applications have either already been listed in the Orange Book for FIRDAPSE® or are in the process of being listed.

We may lose our rights to these patents and patent applications if we breach our obligations under the License Agreement, including, without limitation, our financial obligations to the licensor. If we violate or fail to perform any term or covenant of the License Agreement, the licensor may terminate the License Agreement upon satisfaction of any applicable notice requirements and expiration of any applicable cure periods. Additionally, any termination of the License Agreement, whether by us or by the licensor, will not relieve us of our obligation to pay any license fees owing at the time of such termination. If we fail to retain our rights under the License Agreement, we would not be able to commercialize FIRDAPSE®, and our business, results of operations, financial condition and prospects would be materially adversely affected.

Our commercial success will depend in large part on our ability to use patents and regulatory exclusivity to exclude others from competing with our products. The patent position of emerging pharmaceutical companies like us can be highly uncertain and involve complex legal and technical issues. Until the ’893 patent and the ‘128 patent are interpreted by a court, and unless and until our other pending applications are granted, we will not know the breadth of protection that they will afford us. Our pending applications, if granted, may not contain claims sufficiently broad to prevent others from practicing our technologies or marketing competing products. Third parties may intentionally attempt to design around any FIRDAPSE® patents that ultimately grant so as to compete with us without infringing our patents. Although granted patents enjoy a presumption of validity, there is a risk that the ’893 patent, the ‘128 patent and any patents resulting from our ongoing prosecution efforts may be invalidated or rendered unenforceable if challenged by others.

As a result of the foregoing factors, we cannot be certain how much protection from competition patent rights will provide us.

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

Further, 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 one or more patents or violate other proprietary rights of any third parties, we may be prevented from pursuing product development, manufacturing or commercializing any of our products using 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 claims of infringement against us may have on our business, financial condition, results of operations, or prospects.

If a third-party claims that we infringe its patents, any of the following may occur:

- we may be preliminarily enjoined from making, using, selling, or offering to sell our allegedly infringing product by a court of competent jurisdiction in advance of any formal infringement determination;
- we may be required to pay substantial financial damages if a court formally decides that our technologies infringe the third party’s patent(s). Damages can be tripled if the infringement is deemed willful;
- we may be required to discontinue or significantly delay developing, marketing, selling and licensing the allegedly infringing product(s) absent a license from the patent holder, which may not be available on commercially acceptable terms or at all, or which may require us to pay substantial royalties or grant cross-licenses to our patents; and
- we may need to redesign our product so that it does not infringe the third party’s patent rights, which may not be possible or could require substantial funds or time and require additional studies.

In addition, our employees, consultants, contractors and others may knowingly or unknowingly use the proprietary information of others in their work for us or disclose our proprietary information to others. If our employees, consultants, contractors or others disclose our data to others or use data belonging to others in connection with our business, it could lead to disputes over the ownership of inventions derived from that information or expose us to potential damages or other penalties.

The occurrence of any of these events could have a material adverse effect on our business, financial condition, results of operations or prospects.

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

There is substantial history of litigation and other proceedings regarding patent and intellectual property rights in the pharmaceutical industry. We may be forced to defend claims of infringement brought by our competitors and others, and we may institute litigation against third parties who we believe are infringing our intellectual property rights. The outcome of intellectual property litigation is subject to substantial uncertainties and may, for example, turn on the interpretation of claim language by the court, which may not be to our advantage, or on the testimony of experts as to technical facts upon which experts may reasonably disagree.

Under our License Agreements, we have the right to bring legal action against any alleged infringers of the patents we license. In that regard, in October 2020, we filed lawsuits against Jacobus and the specialty pharmacy marketing Ruzurgi®, PantherRx Rare LLC (PantherRx), for infringement of the ‘893 patent. The suits have now been consolidated in a single action in the U.S. District Court for New Jersey. In August 2021, the lawsuits were amended to include alleged infringement of the ‘128 patent. The lawsuits arise from Jacobus’ and PantherRx’s sales and marketing of Ruzurgi® (amifampridine) Tablets, 10 mg. The lawsuits allege that the Ruzurgi® product infringes the ‘893 patent and the ‘128 patent when administered in accordance with its product labeling. The lawsuit seeks damages and injunctive relief to prevent further marketing of Ruzurgi® in violation of our patent rights. The lawsuit is in the discovery stage and there can be no assurance as to the results of these proceedings.

Our involvement in intellectual property litigation could result in significant expense to us. Some of our competitors have considerable resources available to them and a strong economic incentive to undertake substantial efforts to stop or delay us from commercializing products. Moreover, regardless of the outcome, intellectual property litigation against or by us could significantly disrupt our development and commercialization efforts, divert our management’s attention and quickly consume our financial resources.

In addition, if third parties have filed patent applications or have issued patents claiming technology that is also claimed by us in any of our pending applications, we may be required to participate in interference or derivation proceedings with the third party at the United States Patent Office. We may also need to participate in proceedings outside the United States, such as an opposition at the European Patent Office, to determine whether or not a patent issued by the EPO was properly granted. Even if we are successful in these proceedings, we may incur substantial costs, and the time and attention of our management and scientific personnel will be diverted from product development or other more productive matters.

General Risk Factors Relating to our Common Stock

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 of FIRDAPSE®;
- 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;
- 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 drug 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.

On September 20, 2011, the board of directors approved the adoption of a stockholder rights plan (Rights Plan), which was amended on September 19, 2016 and further amended on August 28, 2019. The Rights Plan was implemented through our entry into a rights agreement with Continental Stock Transfer & Trust Company, as rights agent, and the declaration of a non-taxable dividend distribution of one preferred stock purchase right (each, a Right) for each outstanding share of our common stock. The dividend had been paid on October 7, 2011 to holders of record as of that date. Each right was attached to and traded with the associated share of common stock. Under the Rights Plan, the rights would have become exercisable only if a person acquired beneficial ownership of 17.5% or more of our common stock (or, in the case of a person who beneficially owned 17.5% or more of our common stock on the date the rights plan was adopted, such person acquires beneficial ownership of any additional shares of our common stock) or after the date of the Rights Agreement, commenced a tender offer that, if consummated, would have resulted in beneficial ownership by a person of 17.5% or more of our common stock.

On November 12, 2021, our 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.

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 14, 2022, we had 102,744,913 shares of our common stock outstanding, of which 7,753,498 shares were held by our officers and directors. We also had outstanding: (i) stock options to purchase an aggregate of 14,455,728 shares at exercise prices ranging from $0.79 to $7.07 per share (9,726,842 of which are currently exercisable); and (ii) restricted stock units for 597,339 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 leased office space in Coral Gables, Florida. During 2020, we leased approximately 7,800 square feet of space, for which we paid annual rent of approximately $0.3 million. During May 2020 we amended our office lease to increase our leased space to approximately 10,700 square feet. The amended lease commenced in March 2021 when construction of the asset was completed and the space became available for use. Our current annual rent in the new space is approximately $0.5 million.

Item 3. Legal Proceedings

Ruzurgi®

In May 2019, the FDA approved a New Drug Application (NDA) for Ruzurgi®, another version of amifampridine (3,4-DAP), for the treatment of pediatric LEMS patients (ages 6 to under 17). While the NDA for Ruzurgi® only covers pediatric patients, we believe that Ruzurgi® has been regularly prescribed off-label to adult LEMS patients. We also believe that the FDA's approval of Ruzurgi® violated our statutory rights and was in multiple other respects arbitrary, capricious and contrary to law. As a result, in June 2019 we filed suit against the FDA challenging this approval and related drug labeling, and Jacobus Pharmaceuticals (Jacobus) intervened in our 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 Federal Food, Drug, and Cosmetic Act (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 setting aside the FDA's approval of Ruzurgi®.

On July 30, 2020, the Magistrate Judge considering our lawsuit against the FDA 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 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. Circuit Court of Appeals for the 11th Circuit. By early 2021, the case was fully briefed, and oral argument was held in March 2021.

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

There can be no assurance as to whether Jacobus will seek U.S. Supreme Court review of the 11th Circuit's decision, whether the U.S. Supreme Court will agree to hear the case, or whether, if the U.S. Supreme Court agrees to hear the case, Jacobus' appeal to overturn the decision of the 11th Circuit will be successful. Similarly, there can be no assurance as to whether the U.S. Congress will pass, and the President will sign, legislation effectively overturning the 11th Circuit's decision, and whether or not such legislation, if passed, would have any retroactive effect that would allow the FDA to reinstate Ruzurgi®.

On August 10, 2020, Health Canada issued a Notice of Compliance (NOC) to Medunik for Ruzurgi® for the treatment of LEMS. 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 a 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 early July 2021 we, along with our partner KYE, filed a second suit against Health Canada to overturn their most recent decision. That case was fully briefed in late 2021, with oral argument held in early December.

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. As a result, the matter has, once again, been remanded to the Minister of Health to redetermine its decision in light of the court's ruling. There can be no assurance as to the outcome of this proceeding.

Patent Litigation

All of our patent rights for FIRDAPSE® are derived from our license agreement. 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, 17/503,190. On January 3, 2022, the USPTO allowed related continuing application 17/503,148. A further related continuing application, 17/503,092 was allowed on January 7, 2022. All three patents were issued in March 2022. The claims in each of these applications have either already been listed in the Orange Book for FIRDAPSE® or are in the process of being listed.

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 which provide additional intellectual property protection for our drug product.

In that regard, in October 2020, we filed lawsuits against Jacobus and the specialty pharmacy marketing Ruzurgi®, PantherRx Rare LLC (PantherRx), for infringement of the ‘893 patent. The suits have been consolidated in a single action in the U.S. District Court for New Jersey. In August 2021, the lawsuits were amended to include alleged infringement of the ‘128 patent. The lawsuits arise from Jacobus’ and PantherRx’s sales and marketing of Ruzurgi® (amifampridine) Tablets, 10 mg. The lawsuits allege that the Ruzurgi® product infringes the ‘893 patent and the ‘128 patent when administered in accordance with its product labeling. The lawsuit seeks damages and injunctive relief to prevent further marketing of Ruzurgi® in violation of our patent rights. The lawsuit is in the discovery stage and there can be no assurance as to the results of these proceedings.

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

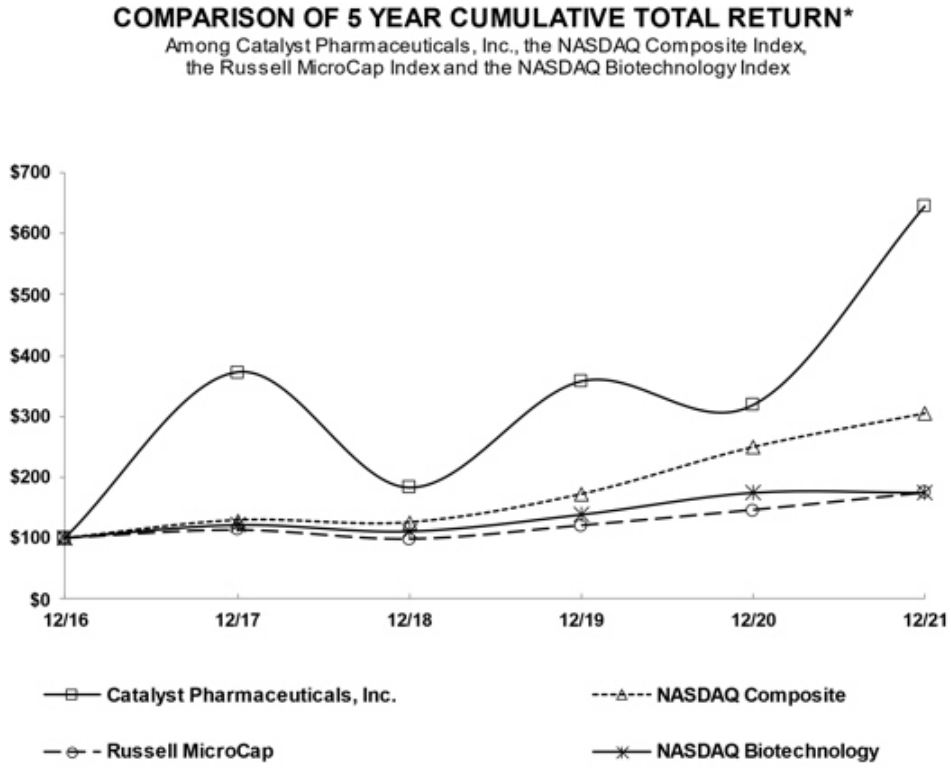
Not applicable.

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 12/31/2016 to 12/31/2021.



	12/16	12/17	12/18	12/19	12/20	12/21
Catalyst Pharmaceuticals, Inc.	**100.00**	**372.38**	**182.86**	**357.14**	**318.10**	**644.76**
NASDAQ Composite	**100.00**	**129.64**	**125.96**	**172.17**	**249.51**	**304.85**
Russell MicroCap	**100.00**	**113.17**	**98.36**	**120.43**	**145.67**	**173.84**
NASDAQ Biotechnology	**100.00**	**121.63**	**110.85**	**138.69**	**175.33**	**175.37**

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 14, 2022 was $7.63. As of March 14, 2022, there were 31 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 $40 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, 2021 with respect to compensation plans under which shares of our common stock may be issued.

	Equity Compensation Plan Information		
Plan Category	Number of securities to be issued upon exercise of outstanding options, warrants, and rights	Weighted-average exercise price of outstanding options, warrants, and rights	Number of securities remaining available for equity compensation plans
Equity compensation plans approved by security holders (1)	14,207,728	$ 3.55	4,708,013(2)
Equity compensation plans not approved by security holders	—	—	—
Total	**14,207,728**	**$ 3.55**	**4,708,013**

(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.

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, 2021:

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, 2021	58,546	$ 5.99	58,546	$ 30,637
November 1 – November 30, 2021	6,654	$ 7.01	6,654	$ 30,590
December 1 – December 31, 2021	393,346	$ 6.81	393,346	$ 27,912
Total	458,546		458,546	

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 2021 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 biopharmaceutical company focused on in-licensing, developing and commercializing novel medicines for patients living with rare diseases. With exceptional patient focus, we are committed to developing a robust pipeline of cutting-edge, best-in-class medicines for rare diseases. We historically focused our efforts on developing products that treat diseases in the neuromuscular and neurological space, but in 2021 we made a strategic decision 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.

We are currently exploring several potential opportunities to acquire companies with drug products in development or to in-license or acquire drug products in development. However, no definitive agreements have been entered into to date. Further, during the third quarter of 2021 we hired Dr. Preethi Sundaram, who serves as our Chief Strategy Officer. In that position, Dr. Sundaram is leading our efforts to acquire R&D assets, from early stage through late-stage clinical programs and technologies to treat rare diseases, and once such drug candidates are acquired, Dr. Sundaram will help oversee the development of those assets.

We are dedicated to making a meaningful impact on the lives of those suffering from rare diseases, and we believe in putting patients first in everything we do.

Impact of the COVID-19 pandemic on our business

The COVID-19 pandemic has affected our business operations in numerous ways, and we continue to monitor applicable government modifications. We had to make modifications to our normal operations because of the COVID-19 pandemic, including allowing our employees to work remotely. 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. Notwithstanding, the COVID-19 pandemic, including the emergence of new COVID-19 variants, including the delta and omicron variants, could 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 such, we have experienced in the past, and may experience in the future, disruptions to our business operations because of 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 believe that because many healthcare providers who treat LEMS patients have delayed seeing new patients because of the pandemic, there has been a delay in the diagnosis of new LEMS patients and their initiating therapy, which has slowed our efforts to locate new patients who could benefit from our therapy. However, we believe that when conditions allow healthcare providers to resume seeing new patients in person on a regular basis, the impact of this aspect of the COVID-19 pandemic on our business will lessen.

One area where we have not been impacted by the pandemic is in our supply chain. To date, we have been able to avoid material disruptions in the production of FIRDAPSE® and, based upon current estimates, we have sufficient inventory to meet current and foreseeable patient needs for at least the next 12 months.

FIRDAPSE®

On November 28, 2018, we received approval from the FDA for FIRDAPSE® Tablets 10 mg for the treatment of adult patients (ages 17 and above) with Lambert-Eaton, Myasthenic Syndrome (“LEMS”). In January 2019, we launched FIRDAPSE® in the United States. We sell our product through a field force experienced in neurologic, central nervous system or rare disease products consisting at this time of approximately 30 field personnel, including sales (Regional Account Managers), patient assistance and insurance navigation support (Patient Access Liaisons), and payor reimbursement (National Account Managers). We also have a field-based force of five medical science liaisons who are helping educate the medical communities and patients about LEMS and our programs supporting patients and access to FIRDAPSE®.

Further, 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 an adult LEMS patient who can benefit from FIRDAPSE®. Additionally, we recently began non-personal promotion to oncologists that may treat adult LEMS patients. We also are continuing to make available at no-cost a LEMS voltage gated calcium channel (VGCC) 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 in order 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, the National Organization for Rare Disorders (NORD), and the Myasthenia Gravis Foundation of America) 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 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 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 adult 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. For eligible patients with commercial coverage, a co-pay assistance program designed to keep out-of-pocket costs to not more than $10.00 per month (currently $0.00 per month) is available for all LEMS patients who are prescribed FIRDAPSE®. We are also 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 May 2019, the FDA approved a New Drug Application (NDA) for Ruzurgi®, another version of amifampridine (3,4-DAP), for the treatment of pediatric LEMS patients (ages 6 to under 17). While the NDA for Ruzurgi® only covers pediatric patients, we believe that Ruzurgi® has been regularly prescribed off-label to adult LEMS patients. We also believe that the FDA's approval of Ruzurgi® violated our statutory rights and was in multiple other respects arbitrary, capricious and contrary to law. As a result, in June 2019 we filed suit against the FDA and several related parties challenging this approval and related drug labeling, and Jacobus Pharmaceuticals (Jacobus) intervened in our 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 Federal Food, Drug, and Cosmetic Act (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 setting aside the FDA's approval of Ruzurgi®.

On July 30, 2020, the Magistrate Judge considering our lawsuit against the FDA 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 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. Circuit Court of Appeals for the 11th Circuit. By early 2021, the case was fully briefed, and oral argument was held in March 2021.

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

There can be no assurance as to whether Jacobus will seek U.S. Supreme Court review of the 11th Circuit's decision, whether the U.S. Supreme Court will agree to hear the case, or whether, if the U.S. Supreme Court agrees to hear the case, Jacobus' appeal to overturn the decision of the 11th Circuit will be successful. Similarly, there can be no assurance as to whether the U.S. Congress will pass, and the President will sign, legislation revising the Orphan Drug Act that effectively overturns the U.S. Court of Appeals for the 11th Circuit, and whether any such legislation, if passed and signed into law, will retroactively affect the outcome of the 11th Circuit decision and allow the FDA to reinstate the approval of Ruzurgi® before the expiration of FIRDAPSE®'s orphan drug exclusivity.

We are actively working with parents and physicians of pediatric LEMS patients to make sure that such patients will be able to obtain FIRDAPSE® through appropriate legal and regulatory means. In addition, we are working to file an application with the FDA seeking approval for use of FIRDAPSE® by pediatric LEMS patients, though any effort to obtain such authorization is not guaranteed. We anticipate submitting the sNDA before the end of the first quarter. For the adult LEMS patients who have been taking Ruzurgi® off-label (who are believed to be a large majority of the patients currently taking Ruzurgi®), we are working with prescribers to transition such patients to FIRDAPSE® as needed.

We have been developing a long-acting formulation of amifampridine phosphate. While a number of formulations have been prepared, after discussions with researchers and an advisory board made up of both patients and physicians, we recently concluded that we would be unable to develop a long-acting formulation that was both beneficial to patients and commercially viable, and as a result we have made the determination not to proceed with development of this product.

On August 10, 2020, we announced the top-line results from our Phase 3 clinical trial (MSK-002) evaluating FIRDAPSE® for the treatment of adults with MuSK-MG. Unfortunately, the MSK-002 trial did not achieve statistical significance on its primary endpoint or its secondary endpoint. Following our receipt of these results, we analyzed the data and proposed a plan to FDA to perform an additional study evaluating FIRDAPSE® for the treatment of MuSK-MG. In response, the FDA provided written comments that were unfavorable towards our proposed revised study design and further questioned the ability of the initial MuSK-MG pilot study to be supportive. These remarks make it unlikely that a single study of similar design to MSK-002 would be sufficient for potential approval of the MuSK-MG indication. We also held an expert panel with key opinion leaders (KOLs) to discuss options and review the likelihood of success for the MuSK-MG indication for FIRDAPSE® under these circumstances. After receiving the input of the FDA and the KOLs, we concluded that the approval of FIRDAPSE® as a first line therapy for MuSK-MG is unlikely, and therefore we have decided not to further pursue this indication.

We previously announced our intent to conduct a proof-of-concept study evaluating FIRDAPSE® as a treatment for Hereditary Neuropathy with Liability to Pressure Palsies (HNPP). The FDA requested that a new, patient centric endpoint be researched and used for our proposed study, without assurance that such endpoint would be acceptable for approval. Based upon the uncertainty of such an endpoint, we have decided not to conduct this study as a company sponsored study, though there is a possibility that this study will move forward as investigator-initiated study that we will support.

There can be no assurance that any future clinical trials of FIRDAPSE® that we undertake will be successful. Further, there can be no assurance that we will ever be granted the right to commercialize FIRDAPSE® for any additional indications.

Our NDS filing for FIRDAPSE® for the symptomatic treatment of LEMS was approved by Health Canada on July 31, 2020. In August 2020, we entered into a license agreement with KYE Pharmaceuticals (KYE), pursuant to which we licensed the Canadian rights for FIRDAPSE® for the treatment of LEMS to KYE. Pursuant to the license agreement, KYE was obligated to pay us an up-front payment based on approval, a milestone upon attainment of marketing authorization and product supply, milestones based on achievements of sales and regulatory milestones, and a sharing of defined net sales following commercialization.

On August 10, 2020, Health Canada issued a Notice of Compliance (NOC) to Medunik for Ruzurgi® for the treatment of LEMS. 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 a 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 KYE, filed a second suit against Health Canada to overturn this decision. That case was fully briefed in late 2021, with oral argument held in early December.

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. As a result, the matter has, once again, been remanded to the Minister of Health to redetermine its decision in light of the court's ruling. There can be no assurance as to the outcome of this proceeding.

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. Additionally, we have an option to further expand our 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, we will pay royalties to our licensor 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.

We have 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. We also 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. (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 will have joint rights to develop FIRDAPSE®, and exclusive rights to commercialize the product, in Japan. DyDo will be responsible for funding all clinical, regulatory, marketing and commercialization activities in Japan. We will be 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 product supplied to DyDo.

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 sometime in 2023. There can be no assurance that this trial will be successful or that DyDo will be granted the right to commercialize FIRDAPSE® in Japan.

All of our patent rights for FIRDAPSE® are derived from our license agreement. 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, 17/503,190. On January 3, 2022, the USPTO allowed related continuing application 17/503,148. A further related continuing application, 17/503,092 was allowed on January 7, 2022. All three patents were issued in March 2022. The claims in each of these applications have either already been listed in the Orange Book for FIRDAPSE® or are in the process of being listed.

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 which provide additional intellectual property protection for our drug product.

In that regard, in October 2020, we filed lawsuits against Jacobus and the specialty pharmacy marketing Ruzurgi®, PantherRx Rare LLC (PantherRx), for infringement of the ‘893 patent. The suits have now been consolidated in a single action in the U.S. District Court for New Jersey. Further, in August 2021, the lawsuits were amended to include alleged infringement of the ‘128 patent. The lawsuits arise from Jacobus’ and PantherRx’s sales and marketing of Ruzurgi® (amifampridine) Tablets, 10 mg. The lawsuits allege that the Ruzurgi® product infringes the ‘893 patent and the ‘128 patent when administered in accordance with its product labeling. The lawsuit seeks damages and injunctive relief to prevent further marketing of Ruzurgi® in violation of our patent rights. The lawsuit is in the discovery stage and there can be no assurance as to the results of these proceedings.

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, there is a possibility we may be prevented from pursuing product development, manufacturing or commercialization of our products that utilize such technologies until the underlying patent dispute is resolved. 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.

Generic Sabril®

In December 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. If and when the product is launched, 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 we are obligated to share the costs of certain development expenses. 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 abbreviated new drug application (ANDA) is approved for vigabatrin tablets in the future, that it will be profitable to us.

Capital Resources

At December 31, 2021, we had cash and cash equivalents and investments of approximately $191.3 million. 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® or will continue to be profitable. Further, there can be no assurance that if we need additional funding in the future, whether such funding will be available to us. 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, 2021, 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, 2021, 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, 2021, 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, 2021, we generated revenues of approximately $2.9 million from our collaborative 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, 2021 and 2020, respectively, we had federal net operating loss carry-forwards of approximately $0 million and $3 million. Additionally, we had state net operating loss carry-forwards of approximately $28 million and $42 million, respectively, available to reduce future Florida taxable income for the years ended December 31, 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 $33.0 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, 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, 2021 and 2020 and, therefore, the transaction price was not reduced further during the years ended December 31, 2021 and 2020. 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.

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, 2021 and 2020, the assumptions used were an estimated annual volatility of 70.0% and 81.4%, expected holding periods of primarily four and a half years, and risk-free interest rates of 0.34% to 1.18% and 0.24% to 1.64%, 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, 2021 and 2020

Revenues.

For the year ended December 31, 2021, we recognized $138.0 million in net revenue from product sales of FIRDAPSE® primarily in the U.S. compared to $118.8 million for the year ended December 31, 2020. The increase of approximately $19.2 million was due to increases in sales volumes of approximately 10.4% and net price increases. For the year ended December 31, 2021, we also recognized $2.8 million in license and other revenue, as compared to $0.3 million during the year ended December 31, 2020. The increase 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 $21.9 million for the year ended December 31, 2021, compared to $17.0 million for the year ended December 31, 2020. 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, 2021 and 2020 were approximately $16.9 million and $16.5 million, respectively, and represented approximately 19% and 21% of total operating costs and expenses, respectively. Research and development expenses for the years ended December 31, 2021 and 2020 were as follows (in thousands):

	For the year ended December 31,		Change	
	2021	2020	$	%
Research and development expenses	$ 15,325	$ 14,912	413	2.8
Employee stock-based compensation	1,611	1,585	26	1.6
Total research and development expenses	$ 16,936	$ 16,497	439	2.7

Research and development expenses stayed relatively consistent for the 2021 fiscal year, when compared to the same period in 2020. For the 2020 fiscal year, research and development expenses included costs related to our MuSK-MG clinical trial and our SMA type 3 proof-of-concept trial, both of which were completed in the second half of 2020. For the fiscal year ended December 31, 2021, research and development expenses included costs relating to closing out sites for both the MuSK-MG clinical trial and SMA type 3 proof-of-concept trial. Research and development costs in both the 2020 and 2021 periods also included expenses relating to medical and regulatory affairs, our expanded access programs, and our efforts to develop a long-acting formulation of amifampridine phosphate.

We expect that research and development expenses will continue to be substantial in 2022 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, 2021 and 2020 were approximately $49.6 million and $44.2 million, respectively, and represented approximately 56% and 57% of total operating costs and expenses for the years ended December 31, 2021, and 2020, respectively. Selling, general and administrative expenses for the years ended December 31, 2021 and 2020 were as follows (in thousands):

	For the year ended December 31,		Change	
	2021	2020	$	%
Selling	$ 26,151	$ 23,567	2,584	11.0
General and administrative	19,015	15,991	3,024	18.9
Employee stock-based compensation	4,462	4,676	(214)	(4.6)
Total selling, general and administrative expenses	$ 49,628	$ 44,234	5,394	12.2

For the year ended December 31, 2021, selling, general and administrative expenses increased approximately $5.4 million, compared to the same period in 2020, primarily attributable to the timing of our commitments to make contributions to 501(c)(3) organizations supporting LEMS patients of approximately $1 million and increases of legal fees of approximately $1.9 million. The increase was also due to increased costs due to the expansion of our operations and headcount required to support our ongoing efforts to commercialize FIRDAPSE®.

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® and take steps to expand our business.

Stock-Based Compensation.

Total stock-based compensation for the years ended December 31, 2021 and 2020 was $6.1 million and $6.3 million, respectively. In 2021 and 2020, 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. The decrease in other income, net for the year ended December 31, 2021 of approximately $0.3 million when compared to the same period in 2020 is primarily due to lower yields on investments, despite higher invested balances. Other income, net, consists primarily of interest and dividend income.

Income Taxes.

As of December 31, 2021 and 2020, respectively, we had federal net operating loss carryforwards of approximately $0 million and $3 million. The federal net operating loss carryforwards were utilized in 2020. Additionally, we had state net operating loss carryforwards of approximately $28 million and $42 million, respectively, available to reduce future Florida taxable income. We had no uncertain tax positions as of December 31, 2021 and December 31, 2020.

For the year ending December 31, 2020 we recorded a net valuation release of $41.6 million ($0.40 per basic share and $0.39 per diluted share) on the basis of management's determination that it is more likely than not that the amount of our deferred tax assets will be realized.

Our effective income tax rate was 25% and (79%), respectively, for fiscal year 2021 and fiscal year 2020. The difference in the effective rates between periods is driven by the release of the valuation allowance against deferred taxes in the third quarter of 2020. 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.

Net Income.

Our net income was approximately $39.5 million in the year ended December 31, 2021 ($0.38 per basic and $0.37 per diluted share) as compared to $75.0 million in the year ended December 31, 2020 ($0.72 per basic and $0.71 per diluted share).

Years Ended December 31, 2020 and 2019

The information comparing results of operations for the year ended 2020 compared to 2019 was included in our Annual Report on Form 10-K for 2020.

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, 2021 we had cash and cash equivalents and investments aggregating $191.3 million and working capital of $183.0 million. At December 31, 2020, we had cash and cash equivalents and investments aggregating $140.3 million and working capital of $136.5 million. At December 31, 2021, 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®;
- 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 $60.4 million and $45.0 million, respectively, for the years ended December 31, 2021 and 2020. 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 and non-current assets, 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. During the year ended December 31, 2020, net cash provided by operating activities was primarily attributable to our net income of $75.0 million, a decrease of $4.5 million in accounts receivable, net, an increase of $0.1 million in accounts payable and $7.1 million net of non-cash expenses. This was partially offset by increases of $4.0 million in prepaid expenses and other current and non-current assets and $2.7 million in inventory and decreases of $1.2 million in accrued expenses and other liabilities, $0.9 million in operating lease liability and $33.0 million in non-cash deferred taxes.

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 used in investing activities was $5.0 million for the year ended December 31, 2020, consisting primarily of purchases of investments of $10.0 million, partially offset by proceeds from sales/maturities of investments of $5.0 million.

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. Net cash provided by financing activities during the year ended December 31, 2020 was $0.7 million, consisting primarily of proceeds from the exercise of options to purchase shares of common stock.

Contractual Obligations and Arrangements.

We have entered into the following contractual arrangements:

- *Payments under our license agreement*. We have agreed to 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, 2021, we recognized an aggregate of approximately $19.5 million of royalties, which is included in cost of sales in the accompanying consolidated statement of operations and comprehensive income.

- *Employment agreements*. We have entered into an employment agreement with our Chief Executive Officer that required us to make base salary payments of approximately $0.6 million in 2021. The agreement expires in November 2022.
- *Purchase commitment*. We have entered into a purchase commitment with our 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 currently have no debt or finance leases. We have an operating lease for our office facilities. 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® is 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® 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 turn out 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 our drug at rates that are higher than historically experienced or are higher than we project;
- Whether the daily dose taken by patients changes over time and affects our results of operations;
- Whether FIRDAPSE® patients can be successfully titrated to stable therapy;
- Whether we can continue to market FIRDAPSE® on a profitable and cash flow positive basis;

- 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 product at the price that we charge for the product;
- The ability of our third-party suppliers and contract manufacturers to maintain compliance with current Good Manufacturing Practices (cGMP);
- The ability of our distributor and the specialty pharmacies that distribute our product to maintain compliance with applicable law;
- Our ability to maintain compliance with applicable rules relating to our patient assistance programs and our contributions to 501(c)(3) organizations that support LEMS patients;
- The scope of our intellectual property and the outcome of any future challenges or opposition to our intellectual property, and, conversely, whether any third-party intellectual property presents unanticipated obstacles for FIRDAPSE®;
- Whether our lawsuits against Jacobus Pharmaceutical Company (Jacobus) and the specialty pharmacy distributing its product for patent infringement will be successful;
- Whether Jacobus will seek U.S. Supreme Court review of the decision of the U.S. Court of Appeals for the 11th Circuit granting summary judgment in our favor in our case against the FDA, thereby overturning the FDA's approval of Ruzurgi®, whether the U.S. Supreme Court will agree to hear the case, or whether if the U.S. Supreme Court hears the case, they will overturn the decision of the 11th Circuit;
- Whether the United States Congress will pass, and the President will sign, legislation revising the Orphan Drug Act that effectively overturns the decision of the U.S. Court of Appeals for the 11th Circuit, and whether any such legislation, if passed and signed into law, will retroactively affect the outcome of the 11th Circuit decision and allow the FDA to reinstate the approval of Ruzurgi® before the expiration of Firdapse®'s orphan drug exclusivity;
- The impact on FIRDAPSE® 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 organization, 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;
- The state of the economy generally and its impact on our business;
- Changes to the healthcare industry occasioned by any future changes in laws relating to the pricing of drug products, or changes in the healthcare industry generally;
- 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 COVID-19 will further affect the timing and costs of our currently ongoing and contemplated clinical trials;
- Whether FIRDAPSE® can be successfully commercialized in Canada on a profitable basis;
- Whether our suit with KYE Pharmaceuticals to overturn the approval of Ruzurgi® in Canada will be successful;
- 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;

- Whether our efforts to grow our business beyond FIRDAPSE® through acquisitions of companies or in-licensing of product opportunities will be successful;
- Whether we will have sufficient capital to finance any such acquisitions;
- Whether our version of vigabatrin tablets will ever be approved by the FDA;
- Even if our version of vigabatrin tablets is approved for commercialization, whether Endo Ventures/Par Pharmaceutical (our collaborator in this venture) will be successful in marketing the product; and
- Whether we will earn milestone payments on the first commercial sale of vigabatrin tablets and royalties on sales of generic vigabatrin tablets.

Our current plans and objectives are based on assumptions relating to the continued commercialization of FIRDAPSE®. Although we believe that our assumptions are reasonable, any of our assumptions could prove inaccurate. In light of 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, U.S. Treasuries and short-term bond funds. 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, 2021, 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, 2021 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, 2021.

During the fourth quarter of 2021, 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 2022 Annual Meeting of Stockholders. Our Proxy Statement for the 2022 Annual Meeting of Stockholders is expected to be filed not later than 120 days after the end of our fiscal year ended December 31, 2021 and is incorporated into this report by this reference.

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

Item 11. Executive Compensation

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

Item 12. Security Ownership of Certain Beneficial Owners and Management

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

Item 13. Certain Relationships and Related Transactions

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

Item 14. Principal Accounting Fees and Services

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

PART IV

Item 15. Exhibits and Financial Statement Schedules

Documents filed as part of this report.

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

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

Consolidated Balance Sheets as of December 31, 2021 and 2020.

Consolidated Statements of Operations and Comprehensive Income for the years ended December 31, 2021, 2020 and 2019.

Consolidated Statements of Changes in Stockholders' Equity for the years ended December 31, 2021, 2020 and 2019.

Consolidated Statements of Cash Flows for the years ended December 31, 2021, 2020 and 2019.

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	Agreement and Plan of Merger, dated August 14, 2006, between the Company and Catalyst Pharmaceutical Partners, Inc., a Florida corporation	S-1	333-136039	9/1/2006	10.9	
3.1	Certificate of Incorporation	S-1	333-136039	7/25/2006	3.1	
3.2	Amendment to Certificate of Incorporation	S-1	333-136039	7/25/2006	3.2	
3.3	Amendment to Certificate of Incorporation	DEF 14A	001-33057	3/30/2015	Annex A	
3.4	Amendment to Certificate of Incorporation	8-K	001-33057	8/21/2020	3.1	
3.5	By-Laws	S-1	333-136039	9/1/2006	3.3	
3.6	Amendment to By-Laws	8-K	001-33057	11/27/2019	3.1	
4.1	Specimen Stock Certificate for Common Stock	S-1	333-136039	9/1/2006	4.1	
4.2	Rights Agreement between the Company and Continental Stock Transfer and Trust Company	8-K	001-33057	9/23/2011	4.1	
4.3	Amendment to Rights Agreement	8-K	001-33057	9/19/2016	4.1	
4.4	Second Amendment to Rights Agreement	8-K	001-33057	8/30/2019	4.1	
4.5	Description of the Company's Capital Stock					X
10.1(a)+	Employment Agreement between the Company and Patrick J. McEnany	10-Q	001-33057	12/15/2006	10.1	
10.1(b)+	First Amendment to Employment Agreement between the Company and Patrick J. McEnany	8-K	001-33057	12/23/2008	10.1	
10.1(c)+	Second Amendment to Employment Agreement between the Company and Patrick J. McEnany	10-Q	001-33057	11/12/2009	10.1	
10.1(d)+	Third Amendment to Employment Agreement between the Company and Patrick J. McEnany	8-K	001-33057	9/15/2011	10.1	

Exhibit Number	Description of Exhibit	Incorporated by Reference				Filed Herewith
		Form	File Number	Date of Filing	Exhibit Number	
10.1(e)+	Fourth Amendment to Employment Agreement between the Company and Patrick J. McEnany	8-K	001-33057	8/29/2013	10.1	
10.1(f)+	Fifth Amendment to Employment Agreement between the Company and Patrick J. McEnany	8-K	001-33057	6/24/2016	10.1	
10.1(g)+	Sixth Amendment to Employment Agreement between the Company and Patrick J. McEnany	8-K	001-33057	5/31/2018	10.1	
10.1(h)+	Seventh Amendment to Employment Agreement between the Company and Patrick J. McEnany	8-K	001-33057	9/11/2020	10.1	
10.2(a)+	2014 Stock Incentive Plan	DEF 14A	001-33057	3/19/2014	Annex A	
10.2(b)+	Amendment No. 1 to 2014 Stock Incentive Plan	DEF 14A	001-33057	4/29/2016	Annex A	
10.2(c)+	Amendment No. 2 to 2014 Stock Incentive Plan	DEF 14A	001-33057	4/14/2017	Annex A	
10.3(a)+	2018 Stock Incentive Plan	DEF 14A	001-33057	4/17/2018	Annex A	
10.3(b)+	Amendment No. 1 to 2018 Stock Incentive Plan	DEF 14A	001-33057	7/7/2020	Annex A	
10.3(c)+	Amendment No. 2 to 2018 Stock Incentive Plan	DEF 14A	001-33057	10/25/2021	Annex A	
10.4(a)	Lease Agreement between the Company and 355 Alhambra Plaza, Ltd.	10-Q	001-33057	5/14/2007	10.1	
10.4(b)	First Amendment to Lease Agreement between the Company and CPT 355 Alhambra Circle, LLC	10-Q	001-33057	8/15/2011	10.1	
10.4(c)	Second Amendment to Lease Agreement between the Company and CPT 355 Alhambra Circle, LLC	8-K	001-33057	2/20/2014	10.1	
10.4(d)	Third Amendment to Lease Agreement between the Company and CPT 355 Alhambra Circle, LLC	8-K	001-33057	3/27/2015	10.1	
10.4(e)	Fourth Amendment to Lease Agreement between the Company and PRII 355 Alhambra Circle, LLC	8-K	001-33057	8/17/2018	10.1	
10.4(f)	Fifth Amendment to Lease Agreement between the Company and PRII 355 Alhambra Circle, LLC	8-K	001-33057	5/13/2020	10.1	
10.5	License Agreement, dated as of December 13, 2011, among New York University, the Feinstein Institute for Medical Research, and the Company	10-K	001-33057	3/30/2012	10.15	
10.6(a)	License Agreement, dated as of October 26, 2012, between the Company and BioMarin	8-K	001-33057	10/31/2012	10.2	
10.6(b)	Amendment No. 1 to License Agreement, dated as of April 8, 2014, between the Company and BioMarin	8-K	001-33057	4/17/2014	10.1	
10.6(c)	Settlement Agreement, dated effective as of July 26, 2018, by and among (i) Aceras BioMedical, LLC, in its capacity as Stockholder Representative for the Former stockholders of Huxley Pharmaceuticals, Inc., (ii) BioMarin, and (iii) the Company	10-Q	001-33057	8/17/2018	10.1	
10.6(d)	Second Amendment to License Agreement, dated May 29, 2019, between the Company and BioMarin	8-K	001-33057	5/30/2019	10.1	
10.7	Development, License and Commercialization Agreement, dated effective as of December 18, 2018, by and between Endo Ventures Limited and the Company	8-K	001-33057	12/26/2018	10.1	
10.8	License and Supply Agreement, dated as of August 14, 2020, by and between KYE Pharmaceuticals, Inc. and the Company	8-K	001-33057	8/20/2020	10.1	
10.9	License and Supply Agreement, dated as of June 28, 2021, by and between DyDo Pharma, Inc. and the Company	8-K	001-33057	6/28/2021	10.1	
21.1	Subsidiaries of the registrant	10-K	001-33057	3/16/2020	21.1	
23.1	Consent of Independent Registered Public Accounting Firm					X
31.1	Section 302 CEO Certification					X
31.2	Section 302 CFO Certification					X

Exhibit Number	Description of Exhibit	Incorporated by Reference				Filed Herewith
		Form	File Number	Date of Filing	Exhibit Number	
32.1	Section 906 CEO Certification					X
32.2	Section 906 CFO Certification					X
101.INS	Inline XBRL Instance Document					
101.SCH	Inline XBRL Taxonomy Extension Schema					
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase					
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase					
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase					
101.PRE	Inline 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 16th day of March, 2022.

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
/s/ Patrick J. McEnany Patrick J. McEnany	Chairman of the Board of Directors, President and Chief Executive Officer (Principal Executive Officer)	March 16, 2022
/s/ Alicia Grande Alicia Grande	Vice President, Treasurer, Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	March 16, 2022
/s/ Charles B. O'Keeffe Charles B. O'Keeffe	Director	March 16, 2022
/s/ Philip H. Coelho Philip H. Coelho	Director	March 16, 2022
/s/ David S. Tierney, M.D. David S. Tierney, M.D.	Director	March 16, 2022
/s/ Donald A. Denkhaus Donald A. Denkhaus	Director	March 16, 2022
/s/ Richard Daly Richard Daly	Director	March 16, 2022
/s/ Molly Harper Molly Harper	Director	March 16, 2022

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Years ended December 31, 2021, 2020 and 2019

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, 2021, 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, 2021, 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, 2021, and our report dated March 16, 2022 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 16, 2022

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, 2021 and 2020, 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, 2021, 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, 2021 and 2020, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2021, 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, 2021, 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 16, 2022, 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

Critical audit matters are matters arising from the current period audit of the financial statements that were communicated or required to be communicated to the audit committee and that: (1) relate to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective, or complex judgments. We determined that there are no critical audit matters.

/s/ GRANT THORNTON LLP

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

Miami, Florida
March 16, 2022

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

	December 31, 2021	December 31, 2020
ASSETS		
Current Assets:		
Cash and cash equivalents	$ 171,445	$ 130,237
Short-term investments	19,821	10,041
Accounts receivable, net	6,619	5,987
Inventory	7,870	4,651
Prepaid expenses and other current assets	4,351	8,328
Total current assets	210,106	159,244
Operating lease right-of-use asset	3,017	—
Property and equipment, net	959	130
Deferred tax assets, net	23,697	32,971
Deposits	9	9
Total assets	$ 237,788	$ 192,354
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current Liabilities:		
Accounts payable	$ 2,768	$ 4,256
Accrued expenses and other liabilities	24,295	18,500
Total current liabilities	27,063	22,756
Operating lease liability, net of current portion	3,894	—
Total liabilities	30,957	22,756
Commitments and contingencies (Note 10)		
Stockholders' equity:		
Preferred stock, $0.001 par value, 5,000,000 shares authorized: none issued and outstanding at December 31, 2021 and 2020	—	—
Common stock, $0.001 par value, 200,000,000 shares authorized; 102,992,913 shares and 103,781,641 shares issued and outstanding at December 31, 2021 and 2020, respectively	103	104
Additional paid-in capital	233,186	223,168
Accumulated deficit	(26,310)	(53,705)
Accumulated other comprehensive income (loss)	(148)	31
Total stockholders' equity	206,831	169,598
Total liabilities and stockholders' equity	$ 237,788	$ 192,354

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, 2021	2020	2019
Revenues:			
Product revenue, net	$ 137,997	$ 118,790	$ 102,306
License and other revenue	2,836	283	—
Total revenues	140,833	119,073	102,306
Operating costs and expenses:			
Cost of sales	21,884	17,039	14,759
Research and development	16,936	16,497	18,843
Selling, general and administrative	49,628	44,234	36,881
Total operating costs and expenses	88,448	77,770	70,483
Operating income	52,385	41,303	31,823
Other income, net	282	587	1,586
Net income before income taxes	52,667	41,890	33,409
Income tax provision (benefit)	13,185	(33,093)	1,534
Net income	$ 39,482	$ 74,983	$ 31,875
Net income per share:			
Basic	$ 0.38	$ 0.72	$ 0.31
Diluted	$ 0.37	$ 0.71	$ 0.30
Weighted average shares outstanding:			

Basic	103,379,349	103,512,913	102,944,316
Diluted	107,795,585	106,242,273	106,020,936
Net income	$ 39,482	$ 74,983	$ 31,875
Other comprehensive income:			
Unrealized gain (loss) on available-for-sale securities, net of tax of $46, $0 and $0, respectively	(179)	22	30
Comprehensive income	$ 39,303	$ 75,005	$ 31,905

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

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CATALYST PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY
For the years ended December 31, 2021, 2020 and 2019
(in thousands)

	Preferred Stock	Common Stock		Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Gain (Loss)	Total
		Shares	Amount				
Balance at December 31, 2018	$ —	102,739	$ 103	$211,265	$ (160,563)	$ (20)	$ 50,785
Issuance of stock options for services	—	—	—	3,780	—	—	3,780
Exercise of stock options for common stock	—	658	1	1,115	—	—	1,116
Amortization of restricted stock for services	—	—	—	45	—	—	45
Other comprehensive gain (loss)	—	—	—	—	—	29	29
Net income	—	—	—	—	31,875	—	31,875
Balance at December 31, 2019	—	103,397	104	216,205	(128,688)	9	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
Balance at December 31, 2020	—	103,782	104	223,168	(53,705)	31	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
Balance at December 31, 2021	$ —	102,993	$ 103	$233,186	$ (26,310)	$ (148)	$206,831

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

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

	Year Ended December 31,		
	2021	**2020**	**2019**
Operating Activities:			
Net income	$ 39,482	$ 74,983	$ 31,875
Adjustments to reconcile net income to net cash provided by (used in) operating activities:			
Depreciation	192	92	55
Stock-based compensation	6,073	6,261	3,825
Deferred taxes	9,316	(32,971)	—
Change in accrued interest and accretion of discount on investments	(5)	(12)	(291)
Reduction in the carrying amount of right-of-use asset	292	793	244
(Increase) decrease in:			
Accounts receivable, net	(632)	4,549	(10,537)
Inventory	(3,219)	(2,694)	(1,901)
Prepaid expenses and other current assets and deposits	3,977	(3,977)	(2,701)
Increase (decrease) in:			
Accounts payable	(1,488)	138	1,780
Accrued expenses and other liabilities	5,520	(1,209)	12,540
Operating lease liability	864	(919)	(277)
Net cash provided by (used in) operating activities	60,372	45,034	34,612
Investing Activities:			
Purchases of property and equipment	(1,021)	(11)	(19)
Purchases of investments	(10,000)	(10,000)	(34,725)
Proceeds from maturities and sales of investments	—	5,000	71,969
Net cash provided by (used in) investing activities	(11,021)	(5,011)	37,225
Financing Activities:			
Payment of employee withholding tax related to stock-based compensation	(153)	(56)	—
Proceeds from exercise of stock options	4,099	758	1,116
Repurchase of common stock	(12,089)	—	—
Net cash provided by (used in) financing activities	(8,143)	702	1,116
Net increase in cash and cash equivalents	41,208	40,725	72,953
Cash and cash equivalents – beginning of period	130,237	89,512	16,559
Cash and cash equivalents – end of period	$171,445	$ 130,237	$ 89,512
Supplemental disclosures of cash flow information:			
Cash paid for income taxes	$ 3,000	$ 2,785	$ —
Non-cash investing and financing activities:			
Operating lease liabilities arising from obtaining right-of-use assets	$ 3,309	$ —	$ —

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 diseases. The Company (f/k/a Catalyst Pharmaceutical Partners, Inc.) was incorporated in Delaware in July 2006. It is the successor by merger to Catalyst Pharmaceutical Partners, Inc., a Florida corporation, which commenced operations in January 2002.

On November 28, 2018, the U.S. Food and Drug Administration, or FDA, granted approval of FIRDAPSE® for the treatment of adults with Lambert-Eaton Myasthenic Syndrome ("LEMS") (ages 17 and above). On January 15, 2019, the Company launched its first product, FIRDAPSE®, in the United States for the treatment of adults with LEMS.

On August 6, 2020, the Company announced that Canada's national healthcare regulatory agency, Health Canada, had approved FIRDAPSE® for the treatment of patients in Canada with LEMS. On October 28, 2020, the Company launched FIRDAPSE® in Canada for the treatment of patients with LEMS through a license and supply agreement with KYE Pharmaceuticals.

Since inception, the Company has devoted substantially all of 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 13 (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 accounting principles generally accepted in the U.S. (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.

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

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

d. INVESTMENTS. The Company invests in high credit-quality instruments in order to obtain higher yields on its cash available for investments. At December 31, 2021 and 2020, investments consisted of short-term bond funds and U.S. Treasuries. Such investments are not insured by the Federal Deposit Insurance Corporation.

The short-term bond funds and U.S. Treasuries held at December 31, 2021 are classified as available-for-sale securities. The short-term bond funds are classified as current assets, which reflects management's intention to use the proceeds from the sale of these investments to fund the Company's operations, as necessary. 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, 2021 and 2020.

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

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, 2021 and 2020, 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, does not have an "alternative future use".

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

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.

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 to five 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. **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, 2021 and 2020, the fair value of these instruments approximated their carrying value.

j. **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, 2021	Quoted Prices in Active Markets for Identical Assets/Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Cash and cash equivalents:				
Money market funds	$ 10,990	$ 10,990	$ —	$ —
U.S. Treasuries	$ 140,995	$ 140,995	$ —	$ —
Short-term investments:				
Short-term bond funds	$ 19,821	$ 19,821	$ —	$ —

	Balances as of December 31, 2020	Quoted Prices in Active Markets for Identical Assets/Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Cash and cash equivalents:				
Money market funds	$ 15,674	$ 15,674	$ —	$ —
U.S. Treasuries	$ 104,994	$ —	$ 104,994	$ —
Short-term investments:				
Short-term bond funds	$ 10,041	$ 10,041	$ —	$ —

k. **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.

l. **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 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.

m. **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. The Company had no contracts with customers until the FDA approved FIRDAPSE® in November 2018. 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, 2021, 2020 and 2019.

During the years ended December 31, 2021, 2020 and 2019, 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

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

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, 2021 and, therefore, the transaction price was not reduced further during the years ended December 31, 2021, 2020 and 2019. Actual amounts of consideration ultimately received may differ from the Company's estimates. If actual results in the future vary from the Company's estimates, the Company will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known.

Trade Discounts 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 statement of operations and comprehensive income through December 31, 2021, 2020 and 2019, 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 pay 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.

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

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

n. **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.

o. **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 $2.9 million, $2.5 million and $3.3 million in advertising costs during the years ended December 31, 2021, 2020 and 2019, respectively, which are included in selling, general and administrative expenses in the Company's consolidated statement of operations and comprehensive income.

p. **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.

q. **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.

The Company currently has a single product with limited commercial sales experience, which makes it difficult to evaluate its current business, predict its future prospects and forecast financial performance and growth. The Company has invested a significant portion of its efforts and financial resources in the development and commercialization of the lead product, FIRDAPSE®, and expects FIRDAPSE® to constitute virtually all of product revenue for the foreseeable future. The Company's success depends on its ability to effectively commercialize FIRDAPSE®.

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.

r. **ROYALTIES.** Royalties incurred in connection with the Company's license agreement, as disclosed in Note 11 (Agreements), are expensed to cost of sales as revenue from product sales is recognized.

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

s. **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.

t. **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, 2021, 2020 and 2019, and is comprised of net unrealized gains (losses) on the Company's available-for-sale securities.

u. **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,		
	2021	2020	2019
Basic weighted average common shares outstanding	103,379,349	103,512,913	102,944,316
Effect of dilutive securities	4,416,236	2,729,360	3,076,620
Diluted weighted average common shares outstanding	107,795,585	106,242,273	106,020,936

Outstanding common stock equivalents totaling approximately 4.3 million, 7.1 million and 4.6 million, were excluded from the calculation of diluted net income per common share for the years ended December 31, 2021, 2020 and 2019, respectively, as their effect would be anti-dilutive. Potentially dilutive options to purchase common stock as of December 31, 2021, 2020 and 2019 had exercise prices ranging from $0.79 to $4.64, $0.79 to $3.95 and $0.79 to $4.20, respectively.

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

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

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

x. RECENTLY ISSUED ACCOUNTING STANDARDS.

In December 2019, the FASB issued ASU 2019-12, *Income Taxes: Simplifying the Accounting for Income Taxes*, a new standard intended to simplify the accounting for income taxes by eliminating certain exceptions related to the approach for intraperiod tax allocation, the methodology for calculating income taxes in an interim period and the recognition of deferred tax liabilities for outside basis differences. The new standard also simplifies aspects of the accounting for franchise taxes and enacted changes in tax laws or rates and clarifies the accounting for transactions that result in a step-up in the tax basis of goodwill. The standard is effective for annual periods beginning after December 15, 2020 and interim periods within, with early adoption permitted. Adoption of the standard requires certain changes to be made prospectively, with some changes to be made retrospectively. The Company adopted the new standard on January 1, 2021. The adoption of this standard did not have a material impact on the Company's consolidated financial statements.

3. Investments.

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

	Estimated Fair Value	Gross Unrealized Gains	Gross Unrealized Losses	Amortized Cost
At December 31, 2021:				
U.S. Treasuries - Cash equivalents	$140,995	$ 2	$ —	$140,993
Short-term bond funds	19,821	—	(196)	20,017
Total	$160,816	$ 2	$ (196)	$161,010
At December 31, 2020:				
U.S. Treasuries - Cash equivalents	$104,994	$ 2	$ —	$104,992
Short-term bond funds	10,041	29	—	10,012
Total	$115,035	$ 31	$ —	$115,004

There were no realized gains or losses from available-for-sale securities for the years ended December 31, 2021, 2020 or 2019.

The estimated fair values of available-for-sale securities at December 31, 2021, by contractual maturity, are summarized as follows (in thousands):

	2021
Due in one year or less	$ 160,816

4. Inventory.

Inventory consists of the following (in thousands):

	December 31, 2021	December 31, 2020
Raw materials	$ 1,769	$ —
Work-in-process	5,172	3,555
Finished goods	929	1,096
Total inventory	$ 7,870	$ 4,651

5. Prepaid Expenses and Other Current Assets.

Prepaid expenses and other current assets consist of the following as of December 31 (in thousands):

	2021	2020
Prepaid manufacturing costs	$ 307	$3,328
Prepaid tax	564	1,368
Prepaid insurance	1,213	1,285
Prepaid subscriptions fees	909	729
Prepaid research fees	452	453
Prepaid commercialization expenses	195	199
Due from collaborative and licensing arrangements	105	437
Prepaid conference and travel expenses	279	83
Other	327	446
Total prepaid expenses and other current assets	$4,351	$8,328

6. 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 Year Ended December 31, 2021
Operating lease cost	$ 379

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

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

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

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

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

6. Operating Leases (continued).

Remaining payments of lease liabilities as of December 31, 2021 were as follows (in thousands):

2022	$ 492
2023	506
2024	522
2025	537
2026	553
Thereafter	2,597
Total lease payments	5,207
Less: imputed interest	(1,005)
Total	$ 4,202

Rent expense was $0.4 million, $0.3 million and $0.3 million for the years ended December 31, 2021, 2020 and 2019.

7. Property and Equipment, Net.

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

	December 31, 2021	December 31, 2020
Computer equipment	$ 51	$ 51
Furniture and equipment	203	242
Leasehold improvements	980	177
Less: Accumulated depreciation	(275)	(340)
Total property and equipment, net	$ 959	$ 130

8. Accrued Expenses and Other Liabilities.

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

	2021	2020
Accrued preclinical and clinical trial expenses	$ 659	$ 585
Accrued professional fees	2,391	1,884
Accrued compensation and benefits	4,035	3,991
Accrued license fees	12,819	10,373
Accrued purchases	2,045	258
Accrued contributions	—	310
Operating lease liability	308	29
Accrued variable consideration	1,716	964
Accrued income tax	79	—
Other	243	106
Current accrued expenses and other liabilities	24,295	18,500
Lease liability – non-current	3,894	—
Non-current accrued expenses and other liabilities	3,894	—
Total accrued expenses and other liabilities	$ 28,189	$ 18,500

9. 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 Par. As of December 31, 2021, 2020 and 2019, no milestone payments have been earned.

There were no revenues from this collaborative arrangement for the years ended December 31, 2021, 2020 or 2019. Total expenses incurred, net, in connection with the collaborative agreement for the years ended December 31, 2021, 2020 and 2019 were approximately $45,000, $4,200 and $65,000, 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.

9. Collaborative and Licensing Arrangements (continued).

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.

Revenues from the arrangement with KYE for the year ended December 31, 2021 were not material. Revenue is included in product revenue, net and license and other revenue in the accompanying consolidated statements of operations and comprehensive income. Expenses incurred, net have been included in selling, general and administrative expenses in the accompanying consolidated statements of operations and comprehensive income.

DyDo Pharma, Inc.

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

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

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

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

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.

Revenue from the arrangement with DyDo for the year ended December 31, 2021, was approximately $2.9 million, relating to the $2.7 million nonrefundable upfront license fee included in license and other revenue in the accompanying consolidated statements of operations and comprehensive income and the sale of FIRDAPSE® of $0.2 million included in product revenue, net within the accompanying consolidated statements of operations and comprehensive income. As of December 31, 2021, no milestone payments have been earned.

10. Commitments and Contingencies.

In May 2019, the FDA approved a New Drug Application (NDA) for Ruzurgi®, another version of amifampridine (3,4-DAP), for the treatment of pediatric LEMS patients (ages 6 to under 17). While the NDA for Ruzurgi® only covers pediatric patients, the Company believes that Ruzurgi® is regularly being prescribed off-label to adult LEMS patients. The Company also believes that the FDA's approval of Ruzurgi® violated the Company's statutory rights and was in multiple other respects arbitrary, capricious and contrary to law. As a result, in June 2019 the Company filed suit against the FDA and several related parties challenging this approval and related drug labeling, and Jacobus Pharmaceuticals (Jacobus) 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 setting aside the FDA's approval of Ruzurgi®.

10. Commitments and Contingencies (continued).

On July 30, 2020, the Magistrate Judge considering the Company's lawsuit against the FDA 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 case. The Company appealed the District Court's decision to the U.S. Circuit Court of Appeals for the 11th Circuit. By early 2021, the case was fully briefed, and oral argument was held in March 2021.

On September 30, 2021, a three-judge panel of 11th Circuit judges issued a unanimous decision overturning the District Court's decision. The appellate court adopted the Company's argument that the FDA's approval of Ruzurgi® violated the Company's rights to Orphan Drug Exclusivity and remanded the case to the District Court with orders to enter summary judgment in the Company's favor. In November 2021, Jacobus filed a motion seeking rehearing of the case from the full 11th Circuit, which motion was denied in January 2022. Further, in January 2022, Jacobus filed motions with both the 11th Circuit and the U.S. Supreme Court seeking a stay of the 11th Circuit's ruling indicating that it would seek a review of the 11th Circuit's decision from the U.S. Supreme Court. Both stay motions were denied, and on January 28, 2022, the 11th Circuit issued a mandate directing the District Court to enter summary judgment in the Company's favor. The District Court entered that order on January 31, 2022. On February 1, 2022, the FDA informed Jacobus that, consistent with the Court of Appeals for the 11th 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. See Note 16.

There can be no assurance as to whether Jacobus will seek U.S. Supreme Court review of the 11th Circuit's decision, whether the U.S. Supreme Court will agree to hear the case, or whether, if the U.S. Supreme Court agrees to hear the case, Jacobus' appeal to overturn the decision of the 11th Circuit will be successful. Similarly, there can be no assurance as to whether the U.S. Congress will pass, and the President will sign, legislation effectively overturning the 11th Circuit's decision, and whether or not such legislation, if passed, would have any retroactive effect allowing the FDA to reinstate the approval of Ruzurgi®.

On August 10, 2020, Health Canada issued a Notice of Compliance (NOC) to Medunik for Ruzurgi® for the treatment of LEMS. 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 a 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 a NOC for Ruzurgi®, once again allowing the product to be marketed in Canada for patients with LEMS. As a result, in early July 2021, the Company, along with its partner KYE, filed a second suit against Health Canada to overturn their most recent decision. That case was fully briefed in late 2021, with oral argument held in early December, and the Company is currently awaiting a decision from the court. There can be no assurance as to the outcome of this proceeding. See Note 16.

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.

11. 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.

11. Agreements (continued).

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. 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.

12. 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, 2021, 2020, and 2019 consists of (in thousands):

	2021	2020	2019
Current	$ 3,869	$ (122)	$ 1,534
Deferred	9,316	(32,971)	—
	$ 13,185	$ (33,093)	$ 1,534

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:

	2021	2020	2019
Statutory rate	21.0%	21.0%	21.0%
State tax	3.4%	2.2%	6.5%
Valuation allowance	—	(99.4)%	(20.9)%
Executive compensation limitation	1.1%	—	0.1%
Tax credit	(0.6)%	(2.4)%	(2.5)%
Other	0.1%	(0.4)%	0.4%
	25.0%	(79.0)%	4.6%

12. Income Taxes (continued).

Deferred tax assets and liabilities reflect the net tax effects of net operating loss and tax credit carryovers and the temporary differences between the carrying amounts of assets and liabilities for financial reporting and the amounts used for income tax purposes. Significant components of the Company's deferred tax assets/(liabilities) as of December 31, 2021 and 2020 are as follows (in thousands):

	2021	2020
Deferred tax assets:		
Net operating loss	$ 1,218	$ 2,320
Start-up costs	10,403	11,203
Tax credits	8,516	15,616
Deferred compensation	3,959	3,889
Inventory	163	212
Operating lease liability	1,003	—
Other	—	130
Total deferred tax assets	25,262	33,370
Deferred tax liabilities:		
Prepaid expenses	(455)	(399)
Right-of use asset	(936)	—
Other	(174)	—
Total deferred tax liabilities	(1,565)	(399)
Deferred tax assets, net	$ 23,697	$ 32,971

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. As of December 31, 2021, 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 $24 million are realizable. The Company released the full valuation allowance for deferred tax assets including net operating loss and tax credit carryover as of December 31, 2020.

At December 31, 2021 and 2020 respectively, the Company had federal net operating loss carryforwards of approximately $0 million and $3 million to reduce future taxable income. The federal net operating loss carryforwards were utilized in 2020. Additionally, at December 31, 2021 and 2020, respectively, the Company had state net operating loss carryforwards of approximately $28 million and $42 million available to reduce future Florida taxable income. The state net operating loss carryforwards will expire at various dates beginning in 2034.

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 are fully utilizable. Thus, the deferred tax assets were adjusted accordingly.

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. The orphan drug credit carryforwards will expire at various dates beginning in 2035.

An immaterial amount of interest and no penalties were accrued through December 31, 2021. No interest or penalties were accrued through December 31, 2020. 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.

13. Stockholders' Equity.

Preferred Stock

The Company has 5,000,000 shares of authorized preferred stock, $0.001 par value per share, at December 31, 2021 and 2020. No shares of preferred stock were outstanding at December 31, 2021 and 2020.

13. Stockholders' Equity (continued).

Common Stock

The Company has 200,000,000 shares of authorized common stock, par value $0.001 per share. At December 31, 2021 and 2020, 102,992,913 and 103,781,641 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 year ended December 31, 2021, 2,208,292 shares were repurchased for an aggregate purchase price of approximately $12.1 million ($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.

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.

14. Stock Compensation.

For the years ended December 31, 2021, 2020 and 2019, the Company recorded stock-based compensation expense as follows (in thousands):

	2021	2020	2019
Research and development	$ 1,611	$ 1,585	$ 1,138
Selling, general and administrative	4,462	4,676	2,687
Total stock-based compensation	$ 6,073	$ 6,261	$ 3,825

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, 2021, 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, 2021, 4,708,013 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, 2021, 2020, and 2019, options to purchase 1,328,936, 281,762 and 654,332 shares, respectively, of the Company's common stock were exercised with gross proceeds to the Company of approximately $4.1 million, $0.8 million, and $1.1 million, respectively. During the years ended December 31, 2021 and 2020, no options to purchase shares of the Company's common stock were exercised on a "cashless" basis. During the year ended December 31, 2019, options to purchase 6,666 shares of the Company's common stock were exercised on a "cashless" basis, resulting in the issuance of an aggregate of 3,444 shares of the Company's common stock.

During the years ended December 31, 2021, 2020, and 2019 the Company recorded non-cash stock-based compensation expense related to stock options totaling approximately $5.5 million, $5.7 million, and $3.8 million, respectively.

During the years ended December 31, 2021, 2020, and 2019, the Company granted seven-year options to purchase an aggregate of 2,330,000, 2,715,000 and 2,183,500 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, 2021 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	13,393,669	$ 3.10		
Granted	2,330,000	5.89		
Exercised or released	(1,328,936)	3.09		
Forfeited or cancelled	(177,006)	3.92		
Expired	(9,999)	5.12		
Outstanding at end of year	14,207,728	$ 3.55	4.15	$ 46,168
Exercisable at end of year	9,473,196	$ 2.87	3.21	$ 36,958

14. Stock Compensation (continued).

Other information pertaining to stock option activity during the years ended December 31, 2021, 2020, and 2019 was as follows:

	2021	2020	2019
Weighted–average fair value of granted stock options	$ 3.24	$ 2.33	$ 2.69
Total fair value of vested stock options (in thousands)	$6,421	$5,312	$ 3,865
Total intrinsic value of exercised stock options (in thousands)	$3,623	$ 325	$ 1,900

As of December 31, 2021, there was approximately $11.6 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 2.43 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:

	2021	2020	2019
Risk free interest rate	0.34% to 1.18%	0.24% to 1.64%	1.51% to 2.53%
Expected term	4.5 – 4.8 years	4.5 years	4.5 years
Expected volatility	68.6% to 72.8%	80.5% to 83.7%	75.5%
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 during 2021 and 2020 was as follows:

	2021		2020	
	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Nonvested balance at beginning of year	235,671	$ 4.65	352,500	$ 4.64
Granted	—	—	30,000	4.70
Vested	(112,832)	4.65	(117,495)	4.64
Forfeited	—	—	(29,334)	4.64
Nonvested balance at end of year	122,839	$ 4.65	235,671	$ 4.65

14. Stock Compensation (continued).

During the year ended December 31, 2019, 352,500 restricted stock units were granted and outstanding and there were no vested or forfeited shares.

During the year ended December 31, 2021, 2020 and 2019, the Company recorded non-cash stock-based compensation expense related to restricted stock units totaling $0.5 million, $0.6 million and $0.1 million, respectively.

15. 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, 2021, 2020, and 2019, the Company's matching contributions were approximately $0.5 million, $0.5 million and $0.3 million, respectively.

16. Subsequent Events.

Subsequent to year end, in January 2022, Jacobus filed motions with both the 11th Circuit and the U.S. Supreme Court seeking a stay of the 11th Circuit's ruling indicating that it would seek a review of the 11th Circuit's decision from the U.S. Supreme Court. Both stay motions were denied, and on January 28, 2022, the 11th Circuit issued a mandate directing the District Court to enter summary judgment in the Company's favor. The District Court entered that order on January 31, 2022. On February 1, 2022, the FDA informed Jacobus that, consistent with the Court of Appeals for the 11th 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. See Note 10.

Subsequent to year end, on March 11, 2022, the Company announced that it 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. As a result, the matter has, once again, been remanded to the Minister of Health to redetermine its decision in light of the court's ruling. See Note 10.

Exhibit 4.5

Description of the Registrant's Securities Registered Pursuant to Section 12 of the Securities Exchange Act of 1934, as amended

The common stock, par value $0.001 per share ("Common Stock"), of Catalyst Pharmaceuticals, Inc. ("Catalyst," "we," or "our") is registered under Section 12 of the Securities Exchange Act of 1934, as amended. The following description sets forth certain general terms and provisions of our Common Stock. These descriptions are in all respects subject to and qualified in their entirety by, and should be read in conjunction with, the applicable provisions of our Certificate of Incorporation (our "Certificate of Incorporation") and our Bylaws (our "Bylaws"), each of which is incorporated herein by reference and copies of which are incorporated by reference as exhibits to our most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission, and the applicable provisions of General Corporation Law of the State of Delaware (the "DGCL").

Authorized Capital Stock

Our authorized capital currently consists of 200,000,000 shares of common stock, par value $0.001 per share, and 5,000,000 shares of preferred stock, par value $0.001 per share.

Common Stock

The following summary of the material features of our common stock does not purport to be complete and is subject to, and qualified in its entirety by the provisions of our Certificate of Incorporation, our Bylaws and other applicable law. See "Where You Can Find Additional Information".

Each holder of common stock is entitled to one vote for each share held of record on all matters presented to our stockholders, including the election of directors. In the event of our liquidation, dissolution, or winding-up, the holders of common stock are entitled to share ratably and equally in our assets, if any, that remain after paying all debts and liabilities and the liquidation preferences of any outstanding preferred stock. The common stock has no preemptive or cumulative rights and no redemption or conversion provisions.

Holders of our common stock are entitled to receive dividends if, as, and when declared by our board of directors out of funds legally available therefor, subject to the dividend and liquidation rights of any preferred stock that may be issued and outstanding, all subject to any dividend restrictions in our credit facilities. No dividend or other distribution (including redemptions and repurchases of shares of capital stock) may be made, if after giving effect to such distribution, we would not be able to pay our debts as they come due in the usual course of business, or if our total assets would be less than the sum of our total liabilities plus the amount that would be needed at the time of a liquidation to satisfy the preferential rights of any holders of preferred stock.

Preferred Stock

Our Certificate of Incorporation, as amended, authorizes our board of directors to establish one or more series of preferred stock. Unless required by law or by any stock exchange on which our common stock is listed, the authorized shares of preferred stock will be available for issuance at the discretion of our board of directors without further action by our stockholders. Our board of directors is able to determine, with respect to any series of preferred stock, the terms and rights of that series, including:

- the designation of the series;
- the number of shares of the series;
- whether dividends, if any, will be cumulative or non-cumulative and the dividend rate, if any, of the series;
- the dates at which dividends, if any, will be payable;
- the redemption rights and price or prices, if any, for shares of the series;
- the terms and amounts of any sinking fund provided for the purchase or redemption of shares of the series;
- the amounts payable on shares of the series in the event of any voluntary or involuntary liquidation, dissolution or winding-up of the affairs of our company;
- whether the shares of the series will be convertible into shares of any other class or series, or any other security, of our company or any other entity, and, if so, the specification of the other class or series or other security, the conversion price or prices or rate or rates and provisions for any adjustments to such prices or rates, the date or dates as of which the shares will be convertible, and all other terms and conditions upon which the conversion may be made;

- the ranking of such series with respect to dividends and amounts payable on our liquidation, dissolution or winding-up, which may include provisions that such series will rank senior to our common stock with respect to dividends and those distributions;
- restrictions on the issuance of shares of the same series or any other class or series; or
- voting rights, if any, of the holders of the series.

The issuance of preferred stock could adversely affect, among other things, the voting power of holders of common stock and the likelihood that stockholders will receive dividend payments and payments upon our liquidation, dissolution or winding up. The issuance of preferred stock could also have the effect of delaying, deferring or preventing a change in control of us.

A prospectus supplement relating to any series of preferred stock being offered will include specific terms related to the offering. They will include, where applicable:

- the title and stated value of the series of preferred stock and the number of shares constituting that series;
- the number of shares of the series of preferred stock offered, the liquidation preference per share and the offering price of the shares of preferred stock;
- the dividend rate(s), period(s) and/or payment date(s) or the method(s) of calculation for those values relating to the shares of preferred stock of the series;
- the date from which dividends on shares of preferred stock of the series shall cumulate, if applicable;
- our right, if any, to defer payment of dividends and the maximum length of any such deferral period;
- the procedures for any auction and remarketing, if any, for shares of preferred stock of the series;
- the provision for redemption or repurchase, if applicable, of shares of preferred stock of the series;
- any listing of the series of shares of preferred stock on any securities exchange;
- the terms and conditions, if applicable, upon which shares of preferred stock of the series will be convertible into shares of preferred stock of another series or common stock, including the conversion price, or manner of calculating the conversion price;
- whether the preferred stock will be exchangeable into debt securities, and, if applicable, the exchange period, the exchange price, or how it will be calculated, and under what circumstances it may be adjusted;
- voting rights, if any, of the preferred stock;
- restrictions on transfer, sale or other assignment, if any;
- whether interests in shares of preferred stock of the series will be represented by global securities;
- any other specific terms, preferences, rights, limitations or restrictions of the series of shares of preferred stock;
- a discussion of any material United States federal income tax consequences of owning or disposing of the shares of preferred stock of the series;
- the relative ranking and preferences of shares of preferred stock of the series as to dividend rights and rights upon liquidation, dissolution or winding up of our affairs; and
- any limitations on issuance of any series of shares of preferred stock ranking senior to or on a parity with the series of shares of preferred stock as to dividend rights and rights upon liquidation, dissolution or winding up of our affairs.

Provisions of the Certificate and Bylaws

A number of provisions of our certificate of incorporation and bylaws concern matters of corporate governance and the rights of stockholders. Certain of these provisions, as well as the ability of our board of directors to issue shares of preferred stock and to set the voting rights, preferences and other terms thereof, may be deemed to have an anti-takeover effect and may discourage takeover attempts not first approved by the board of directors (including takeovers which certain stockholders may deem to be in their best interests). To the extent takeover attempts are discouraged, temporary fluctuations in the market price of the common stock, which may result from

actual or rumored takeover attempts, may be inhibited. These provisions, together with the ability of the board to issue preferred stock without further stockholder action, also could delay or frustrate the removal of incumbent directors or the assumption of control by stockholders, even if such removal or assumption would be beneficial to our stockholders. These provisions also could discourage or make more difficult a merger, tender offer or proxy contests, even if they could be favorable to the interests of stockholders and could potentially depress the market price of the common stock. The board of directors believes that these provisions are appropriate to protect our interest and the interests of our stockholders.

Meetings of Stockholders. The bylaws provide that a special meeting of stockholders may be called only by the board of directors unless otherwise required by law. The bylaws provide that only those matters set forth in the notice of the special meeting may be considered or acted upon at that special meeting, unless otherwise provided by law. In addition, the bylaws set forth certain advance notice and informational requirements and time limitations on any director nomination or any new business which a stockholder wishes to propose for consideration at an annual meeting of stockholders.

No Stockholder Action by Written Consent. The certificate provides that any action required or permitted to be taken by our stockholders at an annual or special meeting of stockholders must be effected at a duly called meeting and may not be taken or effected by a written consent of stockholders in lieu thereof.

Amendment of the Certificate. The certificate provides that an amendment thereof must first be approved by a majority of the board of directors and (with certain exceptions) thereafter approved by the holders of a majority of the total votes eligible to be cast by holders of voting stock with respect to such amendment or repeal; provided, however, that the affirmative vote of 80% of the total votes eligible to be cast by holders of voting stock, voting together as a single class, is required to amend provisions relating to the establishment of the board of directors and amendments to the certificate.

Amendments of Bylaws. The certificate provides that the board of directors or the stockholders may amend or repeal the bylaws. Such action by the board of directors requires the affirmative vote of a majority of the directors then in office. Such action by the stockholders requires the affirmative vote of the holders of at least two-thirds of the total votes eligible to be cast by holders of voting stock with respect to such amendment or repeal at an annual meeting of stockholders or a special meeting called for such purposes, unless the board of directors recommends that the stockholders approve such amendment or repeal at such meeting, in which case such amendment or repeal shall only require the affirmative vote of a majority of the total votes eligible to be cast by holders of voting stock with respect to such amendment or repeal.

Certain Anti-Takeover Matters

We are subject to the provisions of Section 203 of the Delaware General Corporation Law, or Delaware law, regulating corporate takeovers. In general, these provisions prohibit a Delaware corporation from engaging in any business combination with any interested stockholders for a period of three years following the date that the stockholder became an interested stockholder, unless:

- either the business combination or the transaction that resulted in the stockholder becoming an interested stockholder is approved by our board of directors before the date the interested stockholder attained that status;
- upon consummation of the transaction that resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding (but not the outstanding voting stock owned by the interested stockholder) those shares owned (i) by persons who are directors and also officers and (ii) employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or
- on or after that date, the business combination is approved by our board of directors and authorized at a meeting of stockholders, and not by written consent, by at least two-thirds of the outstanding voting stock that is not owned by the interested stockholder.

Section 203 defines “business combination” to include the following:

- any merger or consolidation involving the corporation and the interested stockholder;
- any sale, transfer, pledge or other disposition of 10% or more of the assets of the corporation involving the interested stockholder;
- subject to certain exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;
- any transaction involving the corporation that has the effect of increasing the proportionate share of the stock of any class or series of the corporation beneficially owned by the interested stockholder; or

- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits provided by or through the corporation.

In general, Section 203 of the DGCL defines an interested stockholder as any entity or person beneficially owning 15% or more of the outstanding voting stock of the corporation and any entity or person affiliated with or controlling or controlled by any of these entities or persons.

A Delaware corporation may opt out of this provision either with an express provision in its original certificate of incorporation or in an amendment to its certificate of incorporation or bylaws approved by its stockholders. However, we have not opted out of this provision. The statute could prohibit or delay mergers or other takeover or change in control attempts and, accordingly, may discourage attempts to acquire us.

Limitation of Liability and Indemnification Matters

Our certificate of incorporation limits the liability for monetary damages for breach of fiduciary duty by members of our Board of Directors, except for liability that cannot be eliminated under Delaware law. Under Delaware law, our directors have a fiduciary duty to us which is not eliminated by this provision in our certificate of incorporation. In addition, each of our directors is subject to liability under Delaware law for breach of their duty of loyalty for acts or omissions which are found by a court of competent jurisdiction to be not in good faith or which involve intentional misconduct or knowing violations of law for actions leading to improper personal benefit to the director and for payments of dividends or approval of stock repurchases or redemptions that are prohibited by Delaware law. This provision does not affect our directors' responsibilities under any other laws, such as federal securities laws.

Delaware law provides that the directors of a company will not be personally liable for monetary damages for breach of their fiduciary duty as directors, except for liability for any of the following:

- any breach of a director's duty of loyalty to us or our stockholders;
- acts or omissions not in good faith or which involve intentional misconduct or a knowing violation of law;
- unlawful payment of dividends or unlawful stock repurchases or redemptions; or
- any transaction from which the director derived an improper personal benefit.

Delaware law provides that the indemnification permitted thereunder shall not be deemed exclusive of any other rights to which our directors and officers may be entitled to under our bylaws, any agreement, a vote of stockholders or otherwise. Our certificate of incorporation and bylaws eliminate the personal liability of directors to the maximum extent permitted by Delaware law. In addition, our certificate of incorporation and bylaws provide that we may fully indemnify any person who is or was a party to or is threatened to be made a party to any threatened, pending or completed action, suit of proceeding (whether civil, criminal, administrative or investigative) by reason of the fact that such person is or was one of our directors, officers, employees or other agents, against expenses (including attorneys' fees), judgments, fines and amounts paid in settlement actually and reasonably incurred by such person in connection with such action, suit or proceeding.

Listing

Our common stock is listed on the Nasdaq Capital Market and trades under the symbol "CPRX".

Transfer Agent and Registrar

Our transfer agent and registrar for our common stock is Continental Stock Transfer & Trust Company. They are located at One State Street Plaza, 30th Floor, New York, New York 10004. They can be reached via telephone at (212) 509-4000.

Exhibit 23.1

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We have issued our reports dated March 16, 2022, with respect to the consolidated financial statements and internal control over financial reporting included in the Annual Report of Catalyst Pharmaceuticals, Inc. on Form 10-K for the year ended December 31, 2021. We consent to the incorporation by reference of said reports in the Registration Statements of Catalyst Pharmaceuticals, Inc. on Form S-3 (File No. 333-240052) and Forms S-8 (File No. 333-226008 and File No. 333-198119).

/s/ GRANT THORNTON LLP

Miami, Florida
March 16, 2022

Exhibit 31.1

Certification of Principal Executive Officer

I, Patrick J. McEnany, certify that:

1. I have reviewed this annual report on Form 10-K of Catalyst Pharmaceuticals, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, 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;

c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting.

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's Board of Directors (or persons performing the equivalent functions):

a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 16, 2022

/s/ Patrick J. McEnany
Patrick J. McEnany
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

Certification of Principal Financial Officer

I, Alicia Grande, certify that:

1. I have reviewed this annual report on Form 10-K of Catalyst Pharmaceuticals, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, 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;

c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting.

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's Board of Directors (or persons performing the equivalent functions):

a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 16, 2022

/s/ Alicia Grande
Alicia Grande
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

Certification Required by 18 U.S.C. Section 1350
(as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002)

I, Patrick J. McEnany, as Principal Executive Officer of Catalyst Pharmaceuticals, Inc. (the "Company"), certify, pursuant to 18 U.S.C. Section 1350 (as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002), that to my knowledge:

1. the accompanying Annual Report on Form 10-K of the Company for the year ended December 31, 2021 (the "Report"), filed with the U.S. Securities and Exchange Commission, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

2. the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 16, 2022

/s/ Patrick J. McEnany
Patrick J. McEnany
Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

Certification Required by 18 U.S.C. Section 1350
(as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002)

I, Alicia Grande, as Principal Financial Officer of Catalyst Pharmaceuticals, Inc. (the "Company"), certify, pursuant to 18 U.S.C. Section 1350 (as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002), that to my knowledge:

1. the accompanying Annual Report on Form 10-K of the Company for the year ended December 31, 2021 (the "Report"), filed with the U.S. Securities and Exchange Commission, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

2. the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: March 16, 2022

/s/ Alicia Grande
Alicia Grande
Chief Financial Officer
(Principal Financial Officer)