Catalyst pharmaceuticals

2019 ANNUAL REPORT

AN EXAMPLE OF OUR NEW AD DIRECTED AT PHYSICIANS.

FIRDAPSE®
(amifampridine) Tablets 10 mg

What moves them.

Help your adult patients move forward with FIRDAPSE® (amifampridine), the only FDA-approved, evidence-based therapy for the treatment of LEMS in adults.

FIRDAPSE has been proven in clinical trials to significantly improve muscle strength and patient perception of physical well-being.[1]

INDICATIONS AND USAGE:

FIRDAPSE is a potassium channel blocker indicated for the treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults.

SELECTED IMPORTANT SAFETY INFORMATION

FIRDAPSE can cause seizures. Do not use FIRDAPSE in patients with a history of seizures, or with a hypersensitivity to amifampridine or another aminopyridine.

Please see Brief Summary of full Prescribing Information on the next page.

Reference: 1. Full Prescribing Information for FIRDAPSE (amifampridine). Catalyst Pharma.; 2018.

Catalyst pharmaceuticals

Printed in USA. 18LEM0116 February 2019

Visit www.FIRDAPSEHCP.com to learn more.

Dear Shareholders,

I am pleased to report that 2019 was a very positive and transformational year for Catalyst and, most importantly, for the patients that we seek to help. The year was purpose-driven, as we transformed to a fully integrated commercial-stage biopharmaceutical company with the successful launch of Firdapse® for adult patients suffering from Lambert-Eaton Myasthenic Syndrome (LEMS). We are gratified with the positive response that we have received from the LEMS community of patients and healthcare providers since our launch of Firdapse® in January 2019. We believe that it was extremely important to go through the rigors required to get Firdapse® approved by the FDA, so that all adult LEMS patients, and not just a select few who participated in an early access program, would have affordable access to an FDA approved therapy to treat their rare disease. Of all that we accomplished in 2019 and during the first half of 2020, I am proud that because of the efforts of the Catalyst team, Firdapse® has become the market leader and standard of care for the treatment of adult LEMS patients.

I am most excited by the growth and progress that our company has made across all functional areas of our business, including the financial results that we reported for our first year as a commercial company. When we launched Firdapse® (amifampridine) 10 mg tablets in the U.S. for adult LEMS patients, we identified key objectives for the year based upon-patient enrollments in *Catalyst Pathways*, physician adoption rates for Firdapse®, and high patient satisfaction ratings. I am happy to say that we exceeded our lofty goals. As we move forward, however, there is much more work to be done. We ended 2019 with approximately 500 patients that had been prescribed Firdapse®, but we believe that there are still many more LEMS patients that need access to an affordable, safe, and effective therapy to treat their disease. As part of our efforts to expand our reach to additional physicians that treat LEMS patients, early this year we nearly doubled the size of our highly motivated field sales force and contracted with a rare-disease experienced inside marketing team. We believe that we are now well positioned for growth within the Firdapse® franchise, as we work to expand the number of LEMS patients that will have access and prepare for another potential indication next year.

While we are gratified with the success of the commercial launch of Firdapse® thus far, it is important that we recognize the impact of the challenges that we have recently faced due to the COVID-19 pandemic. As the virus was spreading, our primary objective was the safety for our employees, patients, and healthcare providers. We quickly addressed that on March 16th by implementing a travel ban for all of our employees. Our team was very efficient in adapting to electronic and digital communications to interact with most of our constituents. Virtual meetings with physicians, patients, and other Catalyst team members became a common occurrence. As of this date, I am pleased to see that the country is beginning to slowly open up and that physicians are starting to again see patients and sales representatives face-to-face. Additionally, we have not experienced any disruptions in our supply chain for Firdapse®, nor do we anticipate any, as we continue to build additional safety stock. Our entire supply chain is North American based, with all manufacturing being conducted in the U.S. As we have previously expressed, we did expect to see and have seen some disruption associated with delayed diagnosis and new patient enrollments in *Catalyst Pathways* as a result of the pandemic keeping patients from seeing their physicians. We believe that we are well prepared for any other challenges that may arise from this outbreak, and we remain confident that we have the proper measures in place to support the LEMS community during this difficult time.

We continue to advance our neuromuscular programs, with two critical readouts expected in the third and fourth quarters of this year, one of which has the potential to make this an exciting year for patients hoping for new treatment options. That program is our pivotal Phase 3 trial evaluating Firdapse® for the treatment of patients with anti-MuSK antibody positive Myasthenia Gravis (MuSK-MG). We have completed the active portion of our registrational clinical trial, and we expect to report top-line results in the third quarter of 2020. Assuming positive results, we plan to meet with the FDA as soon as practical to discuss our path forward to seeking an approval for this indication, and we hope to submit a supplemental NDA for Firdapse® in the treatment of MuSK-MG late this year or early next year. MuSK-MG is a rare neuromuscular condition that affects approximately 5,000 patients in the U.S. The patient population is fairly well-defined, and this condition represents an unmet medical need, as there is no approved therapy for these patients. Since off-label therapeutic options are lacking in many respects for the treatment of this disease, we hope that Firdapse® will become the standard of care for treating patients with this rare disease.

Additionally, we expect to report top-line results from our SMA Type 3 proof-of-concept study before the end of 2020, and we plan to start two additional investigator-sponsored proof-of-concept studies evaluating Firdapse® as a potential therapy for two other neuromuscular conditions. Lastly, our program to develop a long-acting formulation of Firdapse® continues to make progress and we hope to have more to report about this program later in the year.

We also are looking to expand both our global footprint for Firdapse® and our portfolio of drug candidates. Earlier this year we submitted our New Drug Submission (NDS) to Health Canada for Firdapse® to treat LEMS patients, and we expect a decision in the second half of 2020. We are currently in negotiations with potential marketing partners for the Canadian territory. In addition, we have a meeting scheduled for later this month with Japanese regulators to finalize a path forward for registering Firdapse® in Japan, and discussions are currently underway with a number of potential marketing partners in Japan. And lastly, we have made it a strategic priority to acquire or in-license one or more additional products to broaden our product offerings and leverage our sales and marketing team.

From day one, Catalyst has been a company with a patient-centric focus, and with that perspective we are learning more everyday about the needs and challenges that patients and their caregivers face in dealing with their debilitating diseases. We believe that with the insight that we gain from what we are learning, we are well equipped to further our mission to better the lives of patients suffering from rare neuromuscular diseases.

Thank you to our shareholders for your continued support of Catalyst throughout our commercial launch of Firdapse® and as we navigate the unprecedented challenges of the COVID-19 pandemic. I am confident in our ability to face these challenges and continue to build the Firdapse® franchise, as well as to develop our clinical pipeline in order to help patients suffering from other rare neuromuscular and neurological diseases. I would also like to thank our employees for their commitment to our mission and strategic vision.

I look forward to updating you on our progress.

Regards,

Patrick J. McEnany
Chairman and CEO
July 6, 2020

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, 2019

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 1250 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.

Common Stock, par value $0.001 per share	**Nasdaq Capital Market**
(Title of each class)	**(Name of exchange on which registered)**

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 if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§229.405 of this chapter) is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ☐

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 ☐

As of June 30, 2019, 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 $367,949,157.

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: 103,408,699 shares of common stock, $0.001 par value per share, were outstanding as of March 12, 2020.

Part III incorporates certain information by reference from the registrant's definitive proxy statement for the 2020 annual meeting of stockholders. The proxy statement with respect to the 2020 annual meeting of stockholders will be filed no later than 120 days after the close of the registrant's fiscal year ended December 31, 2019.

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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® and the development of additional indications for Firdapse® is highly uncertain. Factors that will affect our success include the uncertainty of:

- Whether we will be able to continue 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;
- If the average daily dose taken by patients changes over time, it could affect 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 guidance that we provide to the public market will turn out to be accurate;
- Whether payors will continue to 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®;

- The effect on our business and future results of operations arising from the approval by the Food and Drug Administration (FDA) of Ruzurgi® for the treatment of pediatric LEMS patients (ages 6 to under 17);
- Whether our suit against the United States FDA seeking to vacate the FDA's approval of Ruzurgi® will be successful;
- Whether we can continue to compete successfully if the approval of Ruzurgi® is not overturned and Ruzurgi® continues to be prescribed for off-label use in adult LEMS patients;
- Whether, because of the lower price of Ruzurgi®, payers will require that patients try off-label Ruzurgi® first before they approve Firdapse® as a treatment for adult LEMS patients;
- The impact on Firdapse® of adverse changes in potential reimbursement and coverage policies from government and private payors such as Medicare, Medicaid, insurance companies, health maintenance organizations and other plan administrators, or the impact of pricing pressures enacted by industry organizations, the federal government or the government of any state, including as a result of increased scrutiny over pharmaceutical pricing or otherwise;
- The impact on our business and results of operations of public statements by politicians and a vocal group of LEMS patients and doctors who object to our pricing of Firdapse®;
- Changes in the healthcare industry and the effect of political pressure from President Trump, Congress and/or medical professionals seeking to reduce prescription drug costs;
- The impact of the recent outbreak of a novel strain of coronavirus ("COVID-19") on our business or on the economy generally;
- The state of the economy generally and its impact on our business;
- Changes to the healthcare industry occasioned by any future repeal and replacement of the Affordable Care Act, 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 our trials and studies on a timely basis and within the budgets we establish for such trials and studies;
- Whether the recent coronavirus outbreak will affect the timing of the completion of our currently ongoing clinical trials;
- Whether the trials that we are currently undertaking to evaluate Firdapse® for the treatment of Anti-MuSK antibody positive myasthenia gravis (MuSK-MG), and Spinal Muscular Atrophy (SMA) Type 3, or any other trials that we may undertake in the future, will be successful;
- Whether if our MuSK-MG Phase 3 clinical trial is successful, the FDA will permit us to submit a supplemental new drug application (sNDA) for MuSK-MG without a second Phase 3 trial, and whether any such application will be accepted for filing (and even if accepted, whether such application will be approved);
- Whether Firdapse® will ever be approved for the treatment of MuSK-MG, SMA Type 3, or any other neuromuscular disease;
- Whether our New Drug Submissions (NDS) filing in Canada to commercialize Firdapse® in that jurisdiction will be approved and, even if approved for sale in Canada, whether we can successfully commercialize the product in Canada on a profitable basis;
- Whether we will be able to obtain approval to commercialize Firdapse® in Japan and what clinical trials will be required in Japan in order to obtain such marketing approval;
- Whether we can successfully develop, obtain approval of and successfully market a sustained release version of Firdapse®;

- Whether our efforts to grow our business beyond Firdapse® through acquisitions of companies or in-licensing of product opportunities in the neuromuscular or neurology therapeutic areas will be successful;
- Whether we will have sufficient capital to finance any such acquisitions;
- Whether our version of generic vigabatrin tablets will ever be approved by the FDA;
- Even if vigabatrin tablets are 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 the development of additional indications for 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 1. Business

Overview

We are a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare, debilitating, chronic neuromuscular and neurological diseases. 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.

Firdapse®

In October 2012, we licensed the North American rights to Firdapse®, a proprietary form of amifampridine phosphate, or chemically known as 3,4-diaminopyridine phosphate. When we acquired the rights to the product, it had already been granted orphan drug designation by the FDA for the treatment of patients with LEMS, a rare and sometimes fatal autoimmune disease characterized by muscle weakness. Additionally, in August 2013, we were granted "breakthrough therapy designation" by the FDA for Firdapse® for the treatment of LEMS. Further, the FDA has granted Orphan Drug Designation for Firdapse® for the treatment of Myasthenia Gravis (MG).

On November 28, 2018, we received approval from the FDA for Firdapse® 10 mg tablets for the treatment of adults with LEMS (age 17 and above). In January 2019, we launched Firdapse® in the United States, selling through a field force experienced in neurologic, central nervous system or rare disease products consisting at the time of approximately 20 field personnel, including sales (Regional Account Managers), patient assistance and insurance navigation support (Patient Access Liaisons), and payer reimbursement (National Account Managers) personnel. We also have a field-based force of six medical science liaisons who are helping educate the medical communities and patients about LEMS and about our ongoing clinical trial activities evaluating Firdapse® for other ultra-orphan, neuromuscular diseases. Finally, we are working 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, Anti-MuSK antibody positive myasthenia gravis, or MuSK-MG, and Spinal Muscular Atrophy (SMA) Type 3, 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 a rare-disease experienced inside sales agency. Through this recent expansion of our sales team, we hope 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 may be treating an adult LEMS patient who can benefit from Firdapse®. We also recently launched our no-cost LEMS voltage gated calcium channel (VGCC) antibody testing program (using a commercially available test approved by the FDA) for use by physicians who suspect their patient may have LEMS and wish to reach a definitive diagnosis.

We are supporting the distribution of Firdapse® through "Catalyst Pathways™", our personalized treatment support program. "Catalyst Pathways™" is a single source for personalized treatment support, education and guidance through the challenging dosing and titration regimen to an effective therapeutic dose. It also includes distributing the drug through a very small group of exclusive specialty pharmacies (primarily AnovoRx), which is consistent with the way that most pharmaceutical products for ultra-orphan diseases are distributed and dispensed to patients. 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 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 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 Firdapse® 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). Based on publicly available information, we believe that Jacobus Pharmaceuticals is offering Ruzurgi® at a list price of $80 for each 10 mg tablet, and Jacobus' drug is approved up to a maximum daily dose of 100 mg. Based on this price, we believe that the cost for a 60 mg dosing regimen would be $175,200 annually and the cost to support a patient requiring a daily dose of 100 mg would be $292,000 annually. Both prices are lower than the list price for an equivalent amount of Firdapse®. In addition, while the NDA for Ruzurgi® only covers pediatric patients, we believe that Ruzurgi® is being prescribed off label to some number of adult LEMS patients. If Jacobus is able to successfully 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. Our complaint, which was filed in the federal district court for the Southern District of Florida, alleges 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 setting aside the FDA's approval of Ruzurgi®.

We recently filed a motion for summary judgement in our case, and the FDA has filed cross motions for summary judgement. Further, Jacobus has intervened in our case and filed a cross motion for summary judgement. Based on currently available information, we expect a decision in the case sometime in mid-year 2020. There can be no assurance as to the outcome of this lawsuit, as to the timing of any decision, or the likelihood of an appeal if our suit is successful.

We are currently conducting a Phase 3 clinical trial evaluating Firdapse® for the treatment of adults with MuSK-MG under a Special Protocol Assessment (SPA) with the FDA. The trial is a multi-site, international (United States, Italy and Serbia), double-blind, placebo-controlled, clinical trial. This trial has enrolled more than 60 MuSK antibody positive patients. The trial has 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 is not required and only summary statistics will be provided. While there can be no assurance, based on currently available information we expect to report top-line results from this trial in the first half of 2020, although the recent coronavirus outbreak may cause delays in our trial and in our ability to meet this timetable. If the trial is successful, we plan to file a supplemental new drug application (sNDA) with the FDA seeking approval of Firdapse® for this indication. Details of this trial are available on www.clinicaltrials.gov (NCT03304054).

We are currently conducting a proof-of-concept clinical study evaluating Firdapse® as a symptomatic treatment for patients with Spinal Muscular Atrophy (SMA) Type 3, ambulatory. The study is designed as a randomized (1:1), double-blind, 2-period, 2-treatment, crossover, outpatient proof-of-concept study to evaluate the safety, tolerability and potential efficacy of amifampridine in ambulatory patients diagnosed with SMA Type 3. The study is planned to include approximately 12 patients, and we currently expect to report top-line results from this study in the first half of 2020, although the recent coronavirus outbreak may cause delays in our trial and in our ability to meet this timetable. Details of this trial are available on www.clinicaltrials.gov (NCT03781479).

We also plan to begin studies in 2020 evaluating Firdapse® as a treatment for Kennedy's Disease and Hereditary Neuropathy with liability to Pressure Palsies (HNPP). However, our plans for these studies have not yet been finalized and we do not yet know what form they will take or what timelines they will be on.

There can be no assurance that our clinical programs evaluating Firdapse® for the treatment of MuSK-MG, SMA Type 3, or any trials we may undertake in the future to evaluate Firdapse® for the treatment of other rare neuromuscular diseases, will be successful. Further, there can be no assurance that we will ever be granted the right to commercialize Firdapse® for any of these additional indications.

We are also currently in the early stages of developing a long-acting formulation of amifampridine. We have retained a contractor who is currently assisting us in developing the formulation of the product. We currently anticipate that

initial formulation candidates and their drug release and absorption properties should be determined in 2020. There can be no assurance we will be able to successfully develop a long-acting formulation of amifampridine and that such formulation will ever be approved by the FDA for commercialization.

In October 2019, we submitted an NDS in Canada seeking approval of Firdapse® for the treatment of LEMS. Our application has been accepted for review and we have been granted a priority review. There can be no assurance that our application will be approved.

On May 29, 2019, we entered into an amendment to our License Agreement for Firdapse®. Under the amendment, we have 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.

We are currently in discussions with Japanese regulatory authorities to determine the type of clinical trial that will be required before we will be granted the right to file an application to commercialize Firdapse® in Japan. There can be no assurance that we will successfully obtain the right to commercialize Firdapse® in Japan.

All of our patent rights for Firdapse® are derived from the License Agreement. Under the License Agreement, we had rights to two pending patent families and certain trademarks for Firdapse®. One of the licensed applications, U.S. App. No. 10/467,082, is abandoned as are its children (U.S. App. No. 14/085,017 and 14/818,848) such that we are no longer pursuing patent protection out of this family of applications. The second licensed patent application (U.S. 14/128,672) claims methods of administering Firdapse®. We recently received a "Final" office action from the United States Patent and Trademark Office and we are in the process of responding to that office action. There can be no assurance that this licensed application will be granted or the protection from competition that it will provide to us if it is granted.

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 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.

Broad-based business or economic disruptions could adversely affect our ongoing or planned research and development activities. For example, in December 2019 an outbreak of a novel strain of coronavirus (COVID-19) originated in Wuhan, China and has since spread to a number of other countries, including the United States. To date, this outbreak has already resulted in extended shutdowns of certain businesses and curtailment of travel and large gatherings around the world. While we do not source Firdapse® or its active pharmaceutical ingredient from China, global health concerns, such as coronavirus, could also result in social, economic, and labor instability in the countries in which we, or the third parties with whom we engage, operate. Further, this outbreak could affect the timing of our clinical trials. 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 likely that these global health concerns such as this one could disproportionately impact the hospitals and clinical sites in which we conduct any of our clinical trials, which could slow our clinical trials or adversely affect our business.

Generic Sabril®

In September 2015, we announced the initiation of a project to develop generic versions of Sabril® (vigabatrin). Sabril® is marketed by Lundbeck Inc. in the United States in two dosage forms (powder sachets and tablets) for the treatment of infantile spasms and refractory complex partial seizures. Par Pharmaceutical brought the first generic version of the powder sachet to market, and, to date, several generic versions of the powder sachets have been approved. However, at this time, there is only one approved generic version of the tablets.

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 $500,000. We will be entitled to receive a milestone payment of $2.0 million on the commercial launch of the product. Further, we will receive a sharing of defined net profits upon commercialization and are obligated to share the cost 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, 2019, we had cash and investments of approximately $94.5 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 and cash flow positive. Further, there can be no assurance that if we need additional funding in the future, whether such funding will be available to us. 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:

- Commercialize Firdapse® for the treatment of LEMS and improve disease awareness. We are currently commercializing Firdapse® in the United States. A cornerstone of our strategy is our continuing development of Catalyst Pathways™, our personalized treatment support program, and our development of the patient assistance programs that are required to further our goal that no LEMS patient be denied access to Firdapse® for financial reasons within existing legal restrictions.
- Pursue approval of Firdapse® for MuSK-MG, SMA Type 3 and other neuromuscular indications. We are currently conducting clinical trials evaluating Firdapse® for the treatment of MuSK-MG and SMA Type 3. If our clinical trials are successful, we hope to add these additional indications to our labeling for Firdapse®. We also intend to begin evaluating Firdapse® in 2020 as a potential treatment for Kennedy's Disease and HNPP.
- Seek to develop a sustained release formulation for Firdapse®. We are currently developing a Firdapse® sustained release formulation that we hope will provide meaningful patient benefits for patients with LEMS, MuSK-MG and other neuromuscular indications. There can be no assurance that we will be successful in these efforts.
- Seek approval for Firdapse® in Canada and Japan. We are currently taking steps to seek approval for Firdapse® in Canada and Japan.
- Seek to acquire additional products. While our current focus is in evaluating Firdapse® for other neuromuscular indications, we have recently expanded our efforts to seek acquisitions or product in licensing opportunities in the neuromuscular or neurology therapeutic areas. This expansion includes

the hiring of a dedicated resource to oversee this program and to bring further focus and formality to these efforts. However, no agreements have been entered into to date and future product acquisitions would be subject to the availability of funding, if required. Further, while we expect that, between our current cash and investment balances and our expectation as to the availability to us of non dilutive financing, we will have the resources for one or more acquisitions or in-licensing opportunities, there can be no assurance we will have the financing required to take advantage of any such opportunities.

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 Form 10-K.

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, or 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 a commercially available antibody test that positively identifies patients with 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 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®.

Firdapse® is the only FDA approved, evidence-based therapy for the treatment of LEMS in adults.

Anti-MuSK Antibody Positive Myasthenia Gravis (MuSK-MG)

Myasthenia Gravis, or MG, is a chronic autoimmune neuromuscular disorder that is characterized by fluctuating weakness of the voluntary muscle groups. The prevalence of MG in the United States is estimated to be about 20/100,000 population (equating to an estimate of approximately 64,000 patients in the United States). However, according to the Myasthenia Gravis Foundation of America, MG is probably under diagnosed and the prevalence may be higher. For example, patients with MuSK-MG may have focal or regional weakness and muscle atrophy that are more suggestive of motor neuron or muscle membrane (myopathy) disease. MG occurs in all races, both genders, and at any age. MG is not thought to be directly inherited (although it occasionally occurs in more than one member of the same family), nor is it contagious.

The voluntary muscles of the entire body are controlled by nerve impulses that arise in the brain. These nerve impulses travel down the nerves to the place where the nerves meet the muscle fibers. Nerve fibers do not actually connect with muscle fibers. There is a space between the nerve ending and muscle fiber; this space is called the neuromuscular junction. When the nerve impulse originating in the brain arrives at the nerve ending, it releases a chemical called acetylcholine. Acetylcholine travels across the space to the muscle fiber side of the neuromuscular junction where it attaches to many receptor sites. The muscle contracts when enough of the receptor sites have been activated by the acetylcholine. In MG, there can be as much as an 80% reduction in the number of these receptor sites. The reduction in the number of receptor sites is caused by an antibody that destroys or blocks the receptor site. Antibodies are proteins that play an important role in the immune system. They are normally directed at foreign proteins called antigens that attack the body. Such foreign proteins include bacteria and viruses. Antibodies help the body to protect itself from these foreign proteins. For reasons not well understood, the immune system of the person with MG makes antibodies against the receptor sites of the neuromuscular junction. Abnormal antibodies can be measured in the blood of many people with MG. The antibodies destroy the receptor sites more rapidly than the body can replace them. Muscle weakness occurs when acetylcholine cannot activate enough receptor sites at the neuromuscular junction.

About 15% of MG patients test negative for the acetylcholine receptor antibody. These patients have seronegative (SN) MG. Approximately 40-50% of these patients with SNMG test positive for antibodies against muscle-specific receptor tyrosine kinase (MuSK), a surface membrane component essential in the development of the neuromuscular junction. These patients are identified as having MuSK-MG. Anti-MuSK antibodies identify a clinically distinguishable, more severe form of MG. The disease is characterized by a prominent weakness of the neck, oro-bulbar and sometimes respiratory musculature. Although many patients with MuSK-MG are presently treated with standard MG treatments such as anticholinesterase inhibitors or immunosuppressants, such patients do not generally respond adequately to these treatments.

Based on currently available information, we estimate that there are between 3,000 and 4,800 MuSK-MG patients in the United States. There is currently no drug therapy approved by the FDA for the treatment of MuSK-MG.

Spinal Muscular Atrophy

Spinal Muscular Atrophy (SMA) is a group of genetic disorders to the Survival Motor Neuron (SMN) protein that affects the health of motor neurons and the function of the neuromuscular junction. The pathogenesis may, in part, progress due to the lack of retrograde signaling from dysfunctional neuromuscular junctions leading to motor neuron degeneration and ultimately motor neuron death. As a group of genetic disorders of the SMN protein, the condition varies in severity and rate of degenerative progression and the disease has been classified into Types (SMA Types 1 through 4), based primarily on clinical symptoms of the disease. The overall incidence of SMA is believed to be 1 in 6,000 to 10,000 live births, with over half of the cases diagnosed as SMA Type 1. Due to the poor prognosis of SMA Type 1 patients, the actual prevalence is lower, since well over half of the SMA patients are Type 1 and have a very short life span.

SMA Type 3 (sometimes called Kugelberg-Welander disease) includes clinically heterogeneous patients. They typically reach all major motor milestones in childhood and independent walking by adulthood. However, during infancy they typically have proximal muscular weakness. Some might need wheelchair assistance in childhood, whereas others might continue to walk and live productive adult lives with minor muscular weakness. Patients who lose ambulation often develop scoliosis and other medical problems related to poor mobility and muscle tone, such as obesity and osteoporosis. Two subgroups of severity have been suggested based on the probability of being able to walk by age 10 and on the subsequent probability of losing the ability to walk by age 40. Significant differences in losing the ability to walk have been observed in relation to those with an onset of weakness before (SMA 3a) and after (SMA 3b) age 3.

Due to the heterogeneity of the disease and the variations in life expectancy, prevalence is difficult to determine and not well defined for the different types of SMA. Current estimates place the prevalence of SMA 3 at about 3,500 to 4,200 patients in the United States. While the incidence of SMA Type 3 is about 13% of the total disease incidence, the poor prognosis for SMA Types 1 and 2 (particularly Type 1) leads to a different distribution for the prevalence, with about 35% to 42% of the 10,000 SMA patients in the United States being SMA Type 3.

There are presently two FDA approved treatments for SMA: (Nusinersen (Spinraza®) and Onasemnogene abeparvovec (Zolgensma®)), as well as other treatments under development. We believe that Firdapse® as a treatment for SMA Type 3 has the potential to provide symptomatic relief and may also slow the progression of this disease and improve patients' quality of life, although there can be no assurance that our current study will show these effects. We also believe that Firdapse® may be an effective adjunct therapy with other medications to treat this disease if symptomatic relief can be demonstrated.

Kennedy's Disease

Kennedy's disease, also referred to as spinal and bulbar muscular atrophy, is an inherited motor neuron disease that almost exclusively affects males. It is one of a group of disorders called lower motor neuron disorders (which involve disruptions in the transmission of nerve cell signals in the brain to nerve cells in the brain stem and spinal cord). Onset of the disease is usually between the ages of 20 and 40, although it has been diagnosed in men from their teens to their 70s. Early symptoms include tremor of the outstretched hands, muscle cramps with exertion, and fasciculations (fleeting muscle twitches visible under the skin). Eventually, individuals develop limb weakness which usually begins in the pelvic or shoulder regions. Weakness of the facial and tongue muscles may occur later in the course of the disease and often leads to dysphagia (difficulty in swallowing), dysarthria (slurring of speech), and recurrent aspiration pneumonia. Some individuals develop gynecomastia (excessive enlargement of male breasts) and low sperm count or infertility. Still others develop non-insulin-dependent diabetes mellitus. Kennedy's disease is an x-linked recessive disease, which means the patient's mother carries the defective gene on one of her X chromosomes. Daughters of patients with Kennedy's disease are also carriers and have a 1 in 2 chance of having a son affected with the disease.

Kennedy's disease is slowly progressive. Individuals tend to remain ambulatory until late in the disease, although some may be wheelchair-bound during later stages. The life span of individuals with Kennedy's disease is usually normal.

Currently there is no known cure for Kennedy's disease and there are no FDA-approved treatments. Treatment is symptomatic and supportive. Physical therapy and rehabilitation to slow muscle weakness and atrophy may prove

helpful. Based on currently available data, we estimate the prevalence of Kennedy's disease in the United States to be approximately 600 patients.

Hereditary Neuropathy with Liability to Pressure Palsies

Hereditary Neuropathy with liability to Pressure Palsies (HNPP) is a disorder that affects peripheral nerves. These nerves connect the brain and spinal cord to muscles and sensory cells that detect touch, pain, and temperature. In people with this disorder, the peripheral nerves are unusually sensitive to pressure, such as the pressure that occurs when carrying heavy grocery bags, leaning on an elbow, or sitting without changing position, particularly with crossed legs. These activities would not normally cause sensation problems in people without the disorder.

Hereditary neuropathy with liability to pressure palsies is characterized by recurrent episodes of numbness, tingling, and loss of muscle function (palsy) in the region associated with the affected nerve, usually an arm, hand, leg, or foot. An episode can last from several minutes to several months, but recovery is usually complete. Repeated incidents, however, can cause permanent muscle weakness or loss of sensation. This disorder is also associated with pain in the limbs, especially the hands.

A pressure palsy episode results from pressure on a single nerve, and any peripheral nerve can be affected. Although episodes often recur, they can affect different nerves. The most common problem sites involve nerves in the wrists, elbows, and knees. The fingers, shoulders, hands, feet, and scalp can also be affected. Many people with this disorder experience carpal tunnel syndrome, which occurs when a nerve in the wrist (the median nerve) is involved. Carpal tunnel syndrome is characterized by numbness, tingling, and weakness in the hand and fingers. An episode in the hand may affect fine motor activities such as writing, opening jars, and fastening buttons. An episode of nerve compression in the knee can lead to a condition called foot drop, which makes walking, climbing stairs, or driving difficult or impossible.

The symptoms of HNPP usually begin during adolescence or early adulthood but may develop anytime from childhood to late adulthood. Symptoms vary in severity; many people never realize they have the disorder, while some people experience prolonged disability. HNPP does not affect life expectancy. Based on currently available data, we estimate the prevalence of HNPP in the United States to be between 2,300 and 5,200 patients.

There is currently no standard medical treatment for HNPP. Management generally involves strategies to avoid or modify positions (such as leaning on the elbows) and activities that cause symptoms, and using splints or pads on the wrists or arms to avoid pressure on the nerves. An ankle-foot orthosis may be needed permanently for those with a residual foot drop. Management of pain may include over-the-counter pain medicines and/or prescription drugs used for peripheral neuropathy.

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"). We believe that we remain in compliance with the License Agreement.

BioMarin previously held the worldwide rights to Firdapse® and sold the product in the European Union (EU). In January 2020, BioMarin advised us that they have transferred certain of their rights under the License Agreement to SERB SA.

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.

On May 29, 2019, we entered into an amendment to our License Agreement. Under the amendment, we have 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.

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.

Post-Approval Required Studies

As part of its approval of our NDA for Firdapse® for LEMS, the FDA is requiring 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. We have submitted the final protocol for this trial and we expect to complete this trial on a timely basis. The FDA has also asked us to perform a carcinogenicity study of amifampridine phosphate in a second species of mice and to establish a pregnancy surveillance program to collect and analyze information for a minimum of ten (10) years on pregnancy complications and birth outcomes in women exposed to Firdapse®.

Expanded Access Program

We continue to operate an expanded access program (EAP) that is currently making Firdapse® available to certain patients diagnosed with Congenital Myasthenic Syndromes (CMS) or Downbeat Nystagmus (DN). While we are no longer seeking an indication for Firdapse® for the treatment of CMS and have closed enrollment in the EAP to new CMS and DN patients, we will continue to provide Firdapse to current EAP patients who are already enrolled in our EAP who wish to remain on therapy.

Prior to the approval of our NDA for Firdapse® for LEMS, adult LEMS patients were also eligible to participate in our EAP program. We have since migrated the adult LEMS patients previously in our EAP to Catalyst Pathways™ or to commercial Firdapse®.

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. Additionally, we have a field-based force of six medical science liaisons who help educate the medical communities and patients about LEMS and about our company's ongoing clinical trial activities. Further, we work closely with several rare disease advocacy organizations (including Global Genes, the National Organization for Rare Disorders (NORD), and the Myasthenia Gravis Foundation of America) to help increase awareness and the level of support for patients living with LEMS, 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 a rare-disease experienced inside sales agency. Through this recent expansion of our sales team, we hope to expand our sales efforts beyond the neuromuscular specialists who regularly treat LEMS patients in order 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 recently launched our no-cost LEMS voltage gated calcium channel (VGCC) antibody testing program (using a commercially available test approved by the FDA) for physicians who suspect their patient may have LEMS and wish to reach a definitive diagnosis.

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

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

We are continuing efforts on the 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.

Pricing of and access to Firdapse®

At launch, we established pricing for Firdapse® at an annual list price of $375,000 for a typical LEMS patient who remains 100% persistent and compliant with therapy for an entire year. We also do not foresee any need to raise prices more than annually, we expect that price increases will be in line with inflation and/or cost of living increases. We believe that the pricing of our product is in line with the pricing of other products that provide significant clinical benefits in treating an ultra-orphan disease of similar severity and in order to properly compensate companies for the costs associated with developing, manufacturing, and marketing an orphan drug in compliance with regulatory requirements. To date, our drug has been widely covered and reimbursed by private and public payors for the indicated small population of adult LEMS patients, as part of their mission to assure that rare disease patients receive timely treatment for proven medicines. Furthermore, forecasted rebates, discounts, patient commercial co-pay support, Medicare coverage gap subsidies, statutory Medicaid discounts and other governmental discounts may result in the future in our net sale price being 10-20% lower than our annual list price for the product.

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 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. Subject to compliance

with regulatory requirements, our goal is to ensure that no LEMS patient is ever denied access to Firdapse® for financial reasons.

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

In May 2019, the FDA approved an NDA for Ruzurgi®, another version of amifampridine (3,4-DAP), for the treatment of pediatric LEMS patients (ages 6 to under 17). Based on publicly available information, we believe that Jacobus Pharmaceuticals is offering Ruzurgi® at a list price of $80 for each 10 mg tablet, and Jacobus' drug is approved up to a maximum daily dose of 100 mg. As such, the cost for a 60 mg dosing regimen would be $175,200 annually and the cost to support a patient requiring a daily dose of 100 mg would be $292,000 annually. Both are prices lower than the list price for an equivalent amount of Firdapse®. In addition, while the NDA for Ruzurgi® only covers pediatric patients, we believe that Ruzurgi® is being prescribed off label to some number of adult LEMS patients. If Jacobus is able to successfully 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. Our complaint, which was filed in the federal district court for the Southern District of Florida, alleges 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 setting aside the FDA's approval of Ruzurgi®.

We recently filed a motion for summary judgement in our case, and the FDA has filed a cross motion for summary judgement. Further, Jacobus has intervened in our case and has filed a cross motion summary judgement. Based on currently available information, we expect a decision in the case sometime in mid-year 2020. There can be no assurance as to the outcome of this lawsuit, as to the timing of any decision, or as to the likelihood of an appeal if our suit is successful.

Third-Party Reimbursement

Sales of pharmaceutical products depend in significant part on the availability of coverage and adequate reimbursement by third party payors, such as state and federal governments, including Medicare and Medicaid, managed care providers, private commercial insurance plans and pharmacy benefit management (PBM) plans. Decisions regarding the extent of coverage and the amount of reimbursement 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 and MuSK-MG, 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. Further, there can be no assurance as to whether payors will in the future require that patients try Ruzurgi® before they are treated with Firdapse® (because of its lower cost). In that regard, we have advised payors that we will provide free medication to support titration and confirm patient therapeutic benefit. Further, we are providing patients with access to therapy at no charge while those patients are awaiting coverage decisions.

Our efforts to develop Firdapse® as a treatment of additional indications

We are currently evaluating Firdapse® for the treatment of two additional neuromuscular indications, MuSK-MG, and SMA Type 3, and recently completed a Phase 3 clinical trial evaluating Firdapse® for the treatment of CMS. The current status of our clinical trials evaluating Firdapse® for these additional indications is as follows:

Previously completed MuSK-MG proof-of-concept study

In February 2016, we announced the initiation of 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. We provided study drug, placebo, and financial support for this study.

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.

Ongoing phase 3 clinical trial evaluating Firdapse® for the treatment of MuSK-MG

We are currently conducting a Phase 3 clinical trial evaluating Firdapse® for the treatment of adults with MuSK-MG under a SPA with the FDA. The trial is a multi-site, international (United States, Italy and Serbia), double-blind, placebo-controlled, clinical trial. This trial has enrolled more than 60 MuSK antibody positive patients. The trial has 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 is not required and only summary statistics will be provided. The trial employs a primary endpoint of Myasthenia Gravis Activities of Daily Living (MG-ADL) and a secondary endpoint of Quantitative Myasthenia Gravis Score (QMG).

While there can be no assurance, based on currently available information we expect to report top-line results from this trial in the first half of 2020, although the recent coronavirus outbreak may cause delays in our trial and in our ability to meet this timetable. If the trial is successful, we plan to file a supplemental new drug application (sNDA) with the FDA seeking approval of Firdapse® for this indication. Details of this trial are available on www.clinicaltrials.gov (NCT03304054).

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

We are currently conducting a proof-of-concept clinical study in Italy and Serbia evaluating Firdapse® as a symptomatic treatment for patients with SMA Type 3. The study is designed as a randomized (1:1), double-blind, 2-period, 2-treatment, crossover, outpatient proof-of-concept study to evaluate the safety, tolerability and potential efficacy of amifampridine in ambulatory patients diagnosed with SMA Type 3. The study is planned to include approximately 12 patients, and we currently expect to report top-line results from this study in the first half of 2020, although the recent coronavirus outbreak may cause delays in our trial and in our ability to meet this timetable. Details of this trial are available on www.clinicaltrials.gov (NCT03781479).

Other potential indications

We are continuing to identify additional neuromuscular diseases for which Firdapse® may be an effective treatment, and we hope to commence additional clinical studies and trials to evaluate Firdapse® for the treatment of those diseases. Two diseases we intend to study in 2020 are Kennedy's Disease and Hereditary Neuropathy with liability to Pressure Palsies (HNPP). There can be no assurance that any studies or trials we undertake will be successful.

Our unsuccessful efforts to evaluate Firdapse® for the treatment of CMS

We recently completed a Phase 3 clinical trial evaluating Firdapse® for the treatment of genetically confirmed CMS patients. Our trial was the first ever double-blind, placebo-controlled, clinical trial conducted in genetically confirmed CMS patients. In the trial, 20 subjects were enrolled and 16 randomized, in a 2 period, 2 treatment crossover study designed to evaluate the efficacy and safety of amifampridine phosphate in patients (aged 2 years and above) diagnosed with certain genetic subtypes of CMS. While individual patient improvements were observed, the trial did not meet its primary endpoint of subject global impression (SGI) or its secondary endpoint of muscle function measure (MFM) across all tested subtypes. Due to the rarity of CMS, this trial took almost four years to recruit. Previously, the FDA had granted Orphan Drug Designation for Firdapse® for the treatment of CMS.

Following the completion of our clinical trial, we submitted an extensive briefing package to the FDA in order to determine whether there was a potential path forward to seek approval of amifampridine phosphate for the symptomatic treatment of some subsets of CMS. After reviewing the briefing package, the FDA advised us that the results of the study do not support the use of Firdapse® as a treatment of any types of CMS and that amifampridine does not appear to have a clinically meaningful benefit in the CMS population. They further stated that controlled clinical data demonstrating efficacy would need to be provided to support review of any indication for CMS. As a result, we have determined that we will not proceed with seeking an indication for Firdapse® for the treatment of CMS. However, we will continue to provide Firdapse® to CMS patients who are already enrolled in our expanded access program and who wish to remain on therapy.

Intellectual property and regulatory exclusivity protections for Firdapse®

All of our patent rights for Firdapse® are derived from the License Agreement. Under the License Agreement, we had rights to two pending patent families and certain trademarks for Firdapse®. One of the licensed applications, U.S. App. No. 10/467,082, is abandoned as are its children (U.S. App. No. 14/085,017 and 14/818,848) such that we are no longer pursuing patent protection out of this family of applications. The second licensed patent application (U.S. 14/128,672) claims methods of administering Firdapse®. We recently received a "Final" office action from the United States Patent and Trademark Office and we are in the process of responding to that office action. There can be no assurance that this licensed application will be granted or the protection from competition that it will provide to us if it is granted.

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 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. As a result, 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.

In light of the FDA's decision to approve of Ruzurgi®, the scope and protections afforded by the exclusivities that we received upon the approval of our product remain unclear, and our suit against the FDA seeking to set aside the approval of Ruzurgi® is our attempt to undo what we believe was a violation of our statutory right to exclude others from the market. There can be no assurance that we will be successful in those efforts.

We are in the early stages of developing a sustained release formulation of Firdapse®. If we are successful, we plan to seek to add additional patent protections for that sustained release product, to the extent available. There can be no assurance that we will be successful in those efforts.

We have licensed the Firdapse® trademark from BioMarin, 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 several additional generic versions of this product have been approved. However, there is only one approved generic version of the tablets at this time.

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 $500,000. We will be entitled to receive a milestone payment of $2.0 million on the commercial launch of the product. Further, we will receive a sharing of defined net profits upon commercialization and we are obligated to share the cost 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.

Our failed efforts to develop a next generation GABA aminotransferase inhibitor

For several years, we sought to develop a next generation GABA aminotransferase inhibitor. In 2009, we entered into a license agreement with Northwestern University (Northwestern) under which we acquired worldwide rights to commercialize new GABA aminotransferase inhibitors and derivatives of vigabatrin which had been discovered and patented by Northwestern. Under the terms of the license agreement, Northwestern granted us an exclusive worldwide license to United States composition of matter patents related to the new class of inhibitors and a patent application relating to derivatives of vigabatrin. This included United States patent number 6,794,413 covering the composition of matter for this product, which we designated as CPP-115.

During 2018, we became aware that certain patents granted to Northwestern in 2018 (which patents have been licensed by Northwestern to an unaffiliated pharmaceutical company for a new GABA aminotransferase inhibitor) were derived from CPP-115. As a result, in October 2018, we notified Northwestern that we were terminating the license agreement and seeking damages for Northwestern's breach of the license agreement. Further, on the same date, we filed a claim for damages in arbitration against Northwestern for Northwestern's breaches of the license agreement.

On May 21, 2019, we entered into a settlement agreement with Northwestern that resolved all pending disputes between the parties with no admission of liability by either party, released all claims of liability or wrongdoing between us and Northwestern, and dismissed the pending arbitration. Under the settlement agreement we received a $100,000 payment on May 21, 2019, which is reported as income in other income, net in the consolidated statement of operations. We are also entitled to receive certain contingent compensation that will be reported when and if received.

There can be no assurance that we will receive any future contingent compensation relating to this settlement.

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 our drug products and drug substances are 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 are using contractors to manufacture and supply our products, we rely 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 a drug 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. Both suppliers are located in United States.

Any significant change that we make for Firdapse® must be approved by the FDA in a 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.

In May 2019, we became aware that the FDA had approved an NDA for Jacobus Pharmaceuticals for Ruzurgi®, their version of amifampridine (3,4-DAP) for the treatment of pediatric LEMS patients (ages 6 to under 17). While we have filed suit against the FDA to vacate their approval of Ruzurgi®, there can be no assurance as to the outcome of this lawsuit or as to the impact of the approval of Ruzurgi® on our business, financial condition or results of operations.

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 act: (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 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, the 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.

In early February 2019, Senator Bernie Sanders issued a public statement asking then-FDA Commissioner Scott Gottlieb to allow pharmacies and manufacturers who were previously making 3,4-DAP to be permitted to resume providing it. Senator Sanders continues to tweet about the approval and high price of Firdapse®. We cannot assess the impact of his statements on our business.

Generic Sabril®

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 $195 million in 2018 and $123 million in 2019. One generic version of Sabril® tablets has been approved to date in the United States, as have numerous generic version of the powder form. We have entered into a definitive agreement with Endo/Par for the further development and commercialization of generic Sabril® tablets.

Factors affecting competition generally

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

- our ability to complete clinical development and obtain regulatory approvals for our drug candidates;
- the demonstrated efficacy, safety and reliability of our drug candidates;
- the timing and scope of regulatory approvals;

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

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. 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, or FDCA, and implementing regulations, as well as other federal and state statutes. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local, and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable United States requirements at any time during the product development process, approval process, or after approval, may subject an applicant to administrative or judicial sanctions. These sanctions could include the FDA's refusal to approve pending applications, license suspension or revocation, withdrawal of an approval, a clinical hold, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties or criminal prosecution. Any agency or judicial enforcement action could have a material adverse effect on us. The process required by the FDA before a drug may be marketed in the United States generally involves the following:

- completion of pre-clinical laboratory tests, animal studies and formulation studies according to the FDA's good laboratory practice, 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 accepting it for filing. The FDA may request additional information rather than accept an NDA for filing. In this event, the NDA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The submission of an NDA is also subject to the payment of a substantial application fee (for FDA fiscal year 2020 this fee is $2,942,965), 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 2020 is $325,424 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 a 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 a 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 that contains no active moiety that has 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 may not be a Paragraph IV certification, and, thus, no ANDA may 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 be approved only 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 2020, the application fees are $176,237 per ANDA application and the facility fees are $195,662 per domestic finished dosage form facility, $210,602 per foreign finished dosage form facility, $44,400 per domestic active pharmaceutical ingredient facility, and $59,400 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 (though they decreased slightly for the 2020 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 repeal the Affordable Care Act and replace it with another law, and President Trump has stated that he supports repeal of all or portions of the Affordable Care Act. 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 pharmaceutical 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 pharmaceutical products generally, and particularly the pricing of orphan drugs, has recently received scrutiny from the press, from members of Congress in both parties, and from President Trump. Some members of the medical community and Senator and Presidential candidate 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 pharmaceutical 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. 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.

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 pharmaceutical 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 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 and to report gifts and payments to individual physicians in these states. 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.

Our Employees

As of March 12, 2020, we had 76 employees. We also utilize the services of several consultants. None of our employees are covered by a collective bargaining agreement. We believe our relationship with our employees and consultants is good.

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 Form 10-K.

Item 1A. 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 the commercialization of Firdapse®

We depend substantially on the commercial success of Firdapse®.

Until we launched Firdapse® for the treatment of LEMS in January 2018, we focused all of our efforts 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 of $31.9 million for fiscal 2019, we have a history of operating losses, with a net loss of $34.0 million in fiscal 2018 and net losses in all prior fiscal years of our existence. There can be no assurance that we will remain profitable.

Our business may in the future require additional capital.

We may need to raise additional capital in the future in order to fund our business or make acquisitions. 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.

Our success depends on our ability to continue to successfully commercialize Firdapse®. We are currently 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 into the development and commercialization of our lead product, Firdapse®, which was approved by the FDA as a treatment for adults with LEMS on November 28, 2018. Our success depends on our ability to continue to profitably commercialize Firdapse®, and we expect that the vast majority of our product revenues in the foreseeable future will be from sales of Firdapse®. Successful 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 based on our sales of Firdapse® to date. 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 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, significant uncertainty remains regarding the ultimate commercial potential of Firdapse®.

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

- compete successfully with Jacobus Pharmaceuticals' version of amifampridine;
- successfully maintain our exclusivity for adult LEMS patients based on the results of our lawsuit against the FDA;
- 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 discontinuations due to perceived lack of efficacy or side effects;
- achieve and maintain compliance with regulatory requirements, including 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®;
- continue to 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, or 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.

We have limited experience as a company in marketing or distributing pharmaceutical products. If we are unable to expand our marketing capabilities and effectively commercialize Firdapse®, our business, results of operations and financial condition may be materially adversely affected.

Our strategy is to continue to build our sales, marketing and distribution capabilities to successfully continue to commercialize Firdapse® in the United States. While we have established our commercial team and launched our product, we have limited experience commercializing pharmaceutical products as an organization. In order to continue

to successfully market Firdapse®, we must continue to build our sales, marketing, managerial, compliance, and related capabilities or make arrangements with third parties to perform these services. If we are unable to maintain adequate sales, marketing, and distribution capabilities, whether independently or with third parties, we may not be able to appropriately commercialize Firdapse® and may not remain profitable.

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;
- the 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 our Distributor is selling our product to a very small group of exclusive specialty pharmacies who 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 pharmaceutical products. Our business could be negatively impacted if our competitors' present or future offerings are more effective, safer or less expensive than ours, or more readily accepted by regulators, healthcare providers or third-party payors. Further, we may also compete with respect to manufacturing efficiency and marketing capabilities.

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, such as a related aminopyridine drug, dalfampridine (Ampyra®), may nonetheless be prescribed by physicians for the treatment of LEMS.

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

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

Our business exposes us to potential liability risks that may arise from the clinical testing, manufacture, and/or sale of our pharmaceutical products. Patients have received substantial damage awards in some jurisdictions against pharmaceutical companies based on claims for injuries allegedly caused by the use of pharmaceutical products used in clinical trials or after FDA approval. Liability claims may be expensive to defend and may result in large judgments against us. We currently carry liability insurance that we believe to be adequate. Our insurance may not reimburse us for certain claims or the coverage may not be sufficient to cover claims made against us. We cannot predict all of the possible harms or side effects that may result from the use of our current drug candidates, or any potential future products we may acquire and use in clinical trials or after FDA approval and, therefore, the amount of insurance coverage we currently hold may not be adequate to cover all liabilities we might incur. If we are sued for any injury allegedly caused by our products, our liability could exceed our ability to pay the liability. Whether or not we are ultimately successful in any adverse litigation, such litigation could consume substantial amounts of our financial and managerial resources, all of which could have a material adverse effect on our business, financial condition, results of operations, prospects and/or 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. For example, in December 2019 an outbreak of a novel strain of coronavirus originated in Wuhan, China and has since spread to a number of other countries, including the United States. To date, this outbreak has already resulted in extended shutdowns of certain businesses and curtailment of travel and large gatherings around the world. While we do not source Firdapse® or its active pharmaceutical ingredient from China, global health concerns, such as coronavirus, could also result in social, economic, and labor instability in the countries in which we or the third parties with whom we engage operate. Further, this outbreak could affect the timing of our clinical trials. 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 likely that these global health concerns such as this one could disproportionately impact the hospitals and clinical sites in which we conduct any of our clinical trials, which could slow our clinical trials or adversely effect 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 Sarbanes-Oxley, 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 Sarbanes-Oxley regarding our management's assessment as to the effectiveness of our internal control over financial reporting. Further, under Section 404(b) of Sarbanes-Oxley, 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 officers and key employees and on our Board of Directors. The loss of the services of any 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 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.

Risks Related to the Development of Additional Indications for Firdapse®

Our efforts may fail.

Development of additional indications for Firdapse® is subject to risks of failure. For example:

- Firdapse® may be found to be ineffective or unsafe for one or more additional indications, or fail to receive necessary regulatory approvals;
- Firdapse® 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 Firdapse®.

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. However, Firdapse® failed to meet the primary endpoints in a Phase 3 trial for CMS, and we are no longer pursuing the evaluation of Firdapse® for CMS.

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 Firdapse® for additional indications 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;
- the novel coronavirus may result in a suspension or termination of one or more of our clinical trials;
- 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 Firdapse® for additional indications 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.

If we rely on a sole source of supply to manufacture our products we could be impacted by the viability of our supplier.

We attempt to source our products from more than one supplier. We also seek to enter into contracts with any supplier of our products to contractually obligate them to meet our requirements. However, if we are reliant on a single supplier and that supplier cannot or will not meet our requirements (for whatever reason), our business could be adversely impacted.

We may not be able to sufficiently scale-up manufacturing of our drug candidates.

We may not be able to successfully increase in a sufficient manner the manufacturing capacity for our drug candidates, whether in collaboration with third-party manufacturers or on our own, in a timely or cost-effective manner or at all. If a contract manufacturer makes improvements in the manufacturing process for our drug candidates, we may not own, or may have to share, the intellectual property rights to those improvements.

Significant scale-up of manufacturing may require additional validation studies, which are costly and which the FDA must review and approve. In addition, quality issues may arise during those scale-up activities because of the inherent properties of a drug candidate itself or of a drug candidate in combination with other components added during the manufacturing and packaging process, or during shipping and storage of the finished product or active pharmaceutical ingredients. If we are unable to successfully scale-up manufacture of any of our drug candidates in sufficient quality and quantity, the development of that drug candidate and regulatory approval or commercial launch for any resulting drug products may be delayed or there may be a shortage in supply, which could significantly harm our business.

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

To manage future growth, we will 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 Firdapse® will continue to depend substantially on the extent to which the cost of Firdapse® 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 Firdapse®. 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.

Our ability to continue to commercialize Firdapse® or any other product candidate will depend in large part on the extent to which coverage and reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will cover and establish reimbursement levels. The healthcare industry is acutely focused on cost containment,

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

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

In addition, increasingly, third-party payors are requiring higher levels of evidence of the benefits and clinical outcomes of new technologies and are challenging the prices charged. We cannot be sure that coverage will be available for any product candidate that we commercialize and, if available, that the reimbursement rates will be adequate. Further, the net reimbursement for drug products may be subject to additional reductions if there are changes to laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. An inability to promptly obtain coverage and adequate payment rates from both government funded and private payors for any of our product candidates for which we obtain marketing approval could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

The pricing of pharmaceutical products, in general, and of specialty drugs, in particular, has been a topic of concern in the United States Congress, where hearings have been held on the topic, and several bills have been introduced proposing a variety of actions to restrain the prices of drugs. The President of the United States has frequently discussed his intention to reduce drug prices. The Administration has solicited public comment on a variety of regulatory proposals to reduce drug prices, and has also issued several proposed regulations with that objective, such as a proposal to conduct a pilot test that involves tying reimbursement of separately paid drugs under Medicare Part B to an index of average prices of the drug in certain foreign countries, and a proposal to require drug companies to disclose the list price of a drug in direct-to-consumer television advertisements. It is possible that at least some of these legislative proposals will be enacted and some of the proposed regulations will be finalized. We cannot predict how any such laws or regulations, or new laws or regulations that have yet to be proposed, will affect the pricing of our product, of orphan drugs generally, or of pharmaceutical products generally.

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 our pricing of our drug product.

In February 2019, we received a letter from Senator Bernie Sanders asking us to justify our pricing decision for Firdapse®. In the letter, Senator Sanders accuses us of "fleecing" Americans and "immoral exploitation" because of our decision regarding the pricing of Firdapse®. We responded to Senator Sanders, who issued a public statement in response asking then-FDA Commissioner Scott Gottlieb to allow pharmacies and manufacturers who were previously making this drug to be permitted to resume providing it.

There can be no assurance as to how these matters will affect our business or results of operations.

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 populations for Firdapse® and our other drug candidates are small, we must achieve significant market share and obtain relatively high per-patient prices for our products to achieve meaningful gross margins.

Firdapse® targets diseases with small patient populations. 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 anticipate we may sell Firdapse® will need to be 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.

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

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

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

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

Risks Related to Government Regulation

The regulatory approval process is lengthy, and we may not be able to obtain all of the regulatory approvals required to manufacture and commercialize Firdapse® for additional indications.

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 candidate 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.

The FDA and other regulatory authorities generally approve products for particular indications. Firdapse® may not be approved for any or all of the indications that we request, which would limit the indications for which we can promote it and adversely impact our ability to generate revenues. We may also be required to conduct costly, post-marketing follow-up studies if FDA or other regulatory authorities request additional information.

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 candidate 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 our drug candidates 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 our expanded access program in the evaluation of any NDA or sNDA we may submit for Firdapse®.

In other countries where Firdapse®, or any other product we develop 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. For example, there are two products approved by FDA to treat SMA, and other treatments are under development. 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 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 is 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 candidate we develop, 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 candidate 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 pharmaceutical 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, 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, which could increase the amount of Medicaid drug rebates to states and extended the rebate program to beneficiaries enrolled in Medicaid managed care organizations. The Health Care Reform Law also 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).

Since January 2017, President Trump has 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 but has not yet scheduled arguments in the case, and a decision may not be made until June 2021.

Additionally, in response to controversies regarding pricing of pharmaceutical 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 Trump 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 and restrict sales and promotional activities for pharmaceutical products. 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®.

If we fail to obtain or subsequently maintain orphan drug exclusivity or regulatory exclusivity for Firdapse® and our other orphan drug candidates, 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 and 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 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 disease. 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 disease or 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.

Even though our MuSK-MG trial is being conducted under a Special Protocol Assessment (SPA) agreed to with the FDA, we cannot guarantee that the design of, or data collected from, that trial or any of our clinical trials will be sufficient to support filing or approval of an NDA.

In the context of a Phase 3 clinical trial, the purpose of a SPA is to reach agreement with the FDA on the protocol design and analysis that will form the primary basis of an efficacy claim: in other words, if the agreed-upon clinical trial protocol is followed, the clinical trial endpoints are achieved, and there is a favorable risk-benefit profile, the data may serve as the primary basis for an efficacy claim in support of an NDA. However, 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 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 FDA. In addition, a SPA may be modified with the written agreement of the FDA and the trial sponsor. 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. Moreover, even if a clinical trial is conducted pursuant to a SPA, that does not mean that the NDA will meet the standard for approval.

Further, there can be no assurance that the FDA, even if our current Phase 3 trial evaluating Firdapse® as a treatment of MuSK-MG, is successful, will not require a second adequate and well controlled clinical trial to approve Firdapse® for MuSK-MG, even if the clinical trial we are currently undertaking is successful.

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 product 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) 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; effective January 1, 2022, transfers of value to physician assistants, nurse practitioners or clinical nurse specialists, certified registered nurse anesthetists, and certified nurse-midwives must also be reported;
- 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 the License Agreement. Under the License Agreement, we had rights to two pending patent families and certain trademarks for Firdapse®. One of the licensed applications, U.S. App. No. 10/467,082, is abandoned as are its children (U.S. App. No. 14/085,017 and 14/818,848) such that we are no longer pursuing patent protection out of this family of applications. The second licensed patent application (U.S. 14/128,672) claims methods of administering Firdapse®. We recently received a "Final" office action from the United States Patent and Trademark Office and we are in the process of responding to that office action. There can be no assurance that this licensed application will be granted or the protection from competition that it will provide to us if it is granted.

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 our licensed applications are granted and the

resulting patents are interpreted by a court, either because we have sought to enforce them against a competitor or because a competitor has preemptively challenged them, we will not know the breadth of protection that they will afford us. Our patents, 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 any patent resulting from our ongoing 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. However, we are responsible for all costs relating to such potential litigation. We have the right to any proceeds received as a result of such litigation, but, even if we are successful in such litigation, there is no assurance we would be awarded any monetary damages.

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.

Risks Related 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;
- expiration or termination of licenses (particularly our License Agreement for Firdapse®), research contracts, or other collaboration agreements;
- conditions or trends in the regulatory climate and the biotechnology and pharmaceutical industries;
- intellectual property, product liability or other litigation against us;
- changes in the market valuations of similar companies;
- changes in pharmaceutical company regulations or reimbursements for pharmaceutical products as a result of healthcare reform or other legislation;
- changes in economic conditions; and
- sales of shares of our common stock, particularly sales by our officers, directors and significant stockholders, or the perception that such sales may occur.

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

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

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

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

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. Further, at the 2017 annual meeting of stockholders, the stockholders ratified the Rights Plan. We plan to again seek ratification of the most-recent extension of the Rights Plan at our 2020 annual meeting of stockholders.

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 was paid on October 7, 2011 to holders of record as of that date. Each right is attached to and trades with the associated share of common stock. The rights will become exercisable only if a person acquires 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, commences a tender offer that, if consummated, would result in beneficial ownership by a person of 17.5% or more of our common stock. The rights will expire on September 20, 2022, unless the rights are earlier redeemed or exchanged.

The intent of the Rights Plan is to protect our stockholders' interests by encouraging anyone seeking control of our company to negotiate with our Board of Directors. However, our Rights Plan could make it more difficult for a third party to acquire us without the consent of our Board of Directors, even if doing so may be beneficial to our stockholders. This plan may discourage, delay or prevent a tender offer or takeover attempt, including offers or attempts that could result in a premium over the market price of our common stock. This plan could reduce the price that stockholders might be willing to pay for shares of our common stock in the future. Furthermore, the anti-takeover provisions of our Rights Plan may entrench management and make it more difficult to replace management even if the stockholders consider it beneficial to do so.

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 12, 2020, we had 103,408,699 shares of our common stock outstanding, of which 7,129,164 shares were held by our officers and directors. We also had outstanding: (i) stock options to purchase an aggregate of 12,201,672 shares at exercise prices ranging from $0.79 to $4.64 per share (6,674,652 of which are currently exercisable); and (ii) restricted stock units for 352,500 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. We currently lease approximately 7,800 square feet of space for which we pay annual rent of approximately $330,000.

Item 3. Legal Proceedings

Northwestern

During 2018, we became aware that certain patents granted to Northwestern in 2018 (which patents have been licensed by Northwestern to an unaffiliated pharmaceutical company for a new GABA aminotransferase inhibitor) were derived from CPP-115. On October 26, 2018, we notified Northwestern that we were terminating the license agreement and seeking damages for Northwestern's breach of the license agreement. Further, on the same date, we filed a claim for damages in arbitration against Northwestern for Northwestern's breaches of the license agreement.

On May 21, 2019, we entered into a settlement agreement with Northwestern that resolved all pending disputes between the parties with no admission of liability by either party, released all claims of liability or wrongdoing between us and Northwestern, and dismissed the pending arbitration. Under the settlement agreement we received a $100,000 payment on May 21, 2019, which is reported as income in other income, net in the consolidated statement of operations. We are also entitled to receive certain contingent compensation that will be reported when and if received.

Ruzurgi®

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. Our complaint, which was filed in the federal district court for the Southern District of Florida, alleges 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®.

We recently filed a motion for summary judgement in our case, and the FDA has filed a cross motion for summary judgement. Further, Jacobus has intervened in our case and filed its own cross-motion for summary judgement. Based on currently available information, we expect that there will be a decision in the case sometime mid-year 2020. There can be no assurance as to the outcome of this lawsuit, the timing of any decision, or the likelihood of an appeal if our suit is successful.

Other Litigation

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

Item 4. Mine Safety Disclosure

Not applicable.

PART II

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

Market Information

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

The closing sale price for the common stock on March 13, 2020 was $3.41. As of March 12, 2020, there were 34 holders of record of our common stock, which includes custodians who hold our securities for the benefit of others. We estimate that there are approximately 11,500 beneficial holders of our common stock.

Dividend Policy

We have never declared or paid any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings to support operations and finance the growth and development of our business and 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.

Performance Graph

Not applicable.

Item 6. Selected Financial Data

The selected statement of operations data for the years ended December 31, 2019 and 2018, and the balance sheet data as of December 31, 2019 and 2018, have been derived from our audited consolidated financial statements included elsewhere in this Form 10-K. The selected statement of operations data for the years ended December 31, 2017, 2016 and 2015 and the selected balance sheet data at December 31, 2017, 2016 and 2015 have been derived from financial statements that are not included in this Form 10-K. Historical results are not necessarily indicative of future results. This selected financial data should be read in conjunction with "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our consolidated financial statements and related notes included elsewhere in this Form 10-K.

	Year Ended December 31,				
Statement of Operations Data:	**2019**	**2018**	**2017**	**2016**	**2015**
Revenues:					
Product revenue, net	$102,306,337	$ —	$ —	$ —	$ —
Revenues from collaborative arrangement	—	500,000	—	—	—
Total revenues	102,306,337	500,000	—	—	—
Operating costs and expenses:					
Cost of sales	14,759,139	—	—	—	—
Research and development	18,842,752	19,919,204	11,375,237	11,369,941	11,801,342
Selling, general and administrative	36,881,187	15,875,961	7,304,399	7,910,260	8,597,010
Total operating cost and expenses	70,483,078	35,795,165	18,679,636	19,280,201	20,398,352
Operating income (loss)	31,823,259	(35,295,165)	(18,679,636)	(19,280,201)	(20,398,352)
Other income, net	1,585,774	1,291,651	454,163	321,612	100,389
Change in fair value of warrants liability	—	—	(186,904)	886,137	65,005
Net income (loss) before income taxes	33,409,033	(34,003,514)	(18,412,377)	(18,072,452)	(20,232,958)
Provision for income taxes	1,533,696	—	—	—	—
Net income (loss)	$ 31,875,337	$ (34,003,514)	$ (18,412,377)	$ (18,072,452)	$ (20,232,958)
Net income (loss) per share – basic	$ 0.31	$ (0.33)	$ (0.21)	$ (0.22)	$ (0.25)
Net income (loss) per share – diluted	$ 0.30	$ (0.33)	$ (0.21)	$ (0.22)	$ (0.25)
Weighted average shares outstanding – basic	102,944,316	102,633,884	85,802,487	82,875,281	80,858,393
Weighted average shares outstanding – diluted	106,020,936	102,633,884	85,802,487	82,875,281	80,858,393

	As of December 31,				
Balance Sheet Data:	**2019**	**2018**	**2017**	**2016**	**2015**
Cash and cash equivalents, certificates of deposit and investments	$ 94,518,760	$ 58,489,856	$ 84,013,413	$ 40,405,817	$ 58,396,395
Working capital	87,264,881	45,676,052	80,920,995	39,359,226	56,460,530
Total assets	112,376,230	60,449,962	85,387,430	41,706,853	60,101,570
Warrants liability, at fair value	—	—	—	122,226	1,008,363
Total liabilities	24,746,274	9,666,153	4,423,618	2,397,923	4,625,259
Stockholders' equity	87,629,956	50,783,809	80,963,812	39,308,930	55,476,311

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 with "Selected Financial Data" and our consolidated financial statements and related notes appearing elsewhere in this Form 10-K. 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 Form 10-K.

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 2019 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 two fiscal years presented in the accompanying consolidated statements of operations.

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 biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare, debilitating, chronic neuromuscular and neurological diseases. 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.

Firdapse®

In October 2012, we licensed the North American rights to Firdapse®, a proprietary form of amifampridine phosphate, or chemically known as 3,4-diaminopyridine phosphate. When we acquired the rights to the product, it had already been granted orphan drug designation by the Food and Drug Administration (FDA) for the treatment of patients with LEMS, a rare and sometimes fatal autoimmune disease characterized by muscle weakness. Additionally, in August 2013, we were granted "breakthrough therapy designation" by the FDA for Firdapse® for the treatment of LEMS. Further, the FDA has granted Orphan Drug Designation for Firdapse® for the treatment of Myasthenia Gravis (MG).

On November 28, 2018, we received approval from the FDA for Firdapse® 10 mg tablets for the treatment of adults with LEMS (age 17 and above). In January 2019, we launched Firdapse® in the United States, selling through a field force experienced in neurologic, central nervous system or rare disease products consisting at the time of approximately 20 field personnel, including sales (Regional Account Managers), patient assistance and insurance navigation support (Patient Access Liaisons), and payer reimbursement (National Account Managers) personnel. We also have a field-based force of six medical science liaisons who are helping educate the medical communities and

patients about LEMS and about our ongoing clinical trial activities evaluating Firdapse® for other ultra-orphan, neuromuscular diseases. Finally, we are working 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, Anti-MuSK antibody positive myasthenia gravis, or MuSK-MG, and Spinal Muscular Atrophy (SMA) Type 3, 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 a rare-disease experienced inside sales agency. Through this recent expansion of our sales team, we hope 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 may be treating an adult LEMS patient who can benefit from Firdapse®. We also recently launched our no-cost LEMS voltage gated calcium channel (VGCC) antibody testing program (using a commercially available test approved by the FDA) for use by physicians who suspect their patient may have LEMS and wish to reach a definitive diagnosis.

We are supporting the distribution of Firdapse® through "Catalyst Pathways™", our personalized treatment support program. "Catalyst Pathways™" is a single source for personalized treatment support, education and guidance through the challenging dosing and titration regimen to an effective therapeutic dose. It also includes distributing the drug through a very small group of exclusive specialty pharmacies (primarily AnovoRx), which is consistent with the way that most pharmaceutical products for ultra-orphan diseases are distributed and dispensed to patients. 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 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 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 Firdapse® 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). Based on publicly available information, we believe that Jacobus Pharmaceuticals is offering Ruzurgi® at a list price of $80 for each 10 mg tablet, and the Jacobus' drug is approved up to a maximum daily dose of 100 mg. Based on this price, we believe that the cost for a 60 mg dosing regimen would be $175,200 annually and the cost to support a patient requiring a daily dose of 100 mg would be $292,000 annually. Both are prices lower than the list price for an equivalent amount of Firdapse®. In addition, while the NDA for Ruzurgi® only covers pediatric patients, we believe that Ruzurgi® is being prescribed off label to some number of adult LEMS patients. If Jacobus is able to successfully 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. Our complaint, which was filed in the federal district court for the Southern District of Florida, alleges 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 setting aside the FDA's approval of Ruzurgi®.

We recently filed a motion for summary judgement in our case, and the FDA has filed a cross motion for summary judgement. Further, Jacobus has intervened in our case and has filed a cross motion for summary judgement. Based on currently available information, we expect a decision in the case sometime in mid-year 2020. There can be no

assurance as to the outcome of this lawsuit, as to the timing of any decision, or the likelihood of an appeal if our suit is successful .

We are currently conducting a Phase 3 clinical trial evaluating Firdapse® for the treatment of adults with MuSK-MG under a Special Protocol Assessment (SPA) with the FDA. The trial is a multi-site, international (United States, Italy and Serbia), double-blind, placebo-controlled, clinical trial. This trial enrolled more than 60 MuSK antibody positive patients. The trial also enrolled 10 generalized myasthenia gravis patients who were assessed with the same clinical endpoints. However, achieving statistical significance in this subgroup of patients is not required and only summary statistics will be provided. While there can be no assurance, based on currently available information we expect to report top-line results from this trial in the first half of 2020, although the recent coronavirus outbreak may cause delays in our trial and in our ability to meet this deadline. If the trial is successful, we plan to file a supplemental new drug application (sNDA) with the FDA. Details of this trial are available on www.clinicaltrials.gov (NCT03304054).

We are currently conducting a proof-of-concept clinical study evaluating Firdapse® as a symptomatic treatment for patients with Spinal Muscular Atrophy (SMA) Type 3, ambulatory. The study is designed as a randomized (1:1), double-blind, 2-period, 2-treatment, crossover, outpatient proof-of-concept study to evaluate the safety, tolerability and potential efficacy of amifampridine in ambulatory patients diagnosed with SMA Type 3. The study is planned to include approximately 12 patients, and we currently expect to report top-line results from this study in the first half of 2020, although the recent coronavirus outbreak may cause delays in our trial and in our ability to meet this deadline. Details of this trial are available on www.clinicaltrials.gov (NCT03781479).

We also plan to begin studies in 2020 evaluating Firdapse® as a treatment for Kennedy's Disease and Hereditary Neuropathy with liability to Pressure Palsies (HNPP). However, our plans for these studies have not yet been finalized and we do not yet know what form they will take or what timelines they will be on.

There can be no assurance that our clinical programs evaluating Firdapse® for the treatment of MuSK-MG, SMA Type 3, or any trials we may undertake in the future to evaluate Firdapse® for the treatment of other rare neuromuscular diseases, will be successful. Further, there can be no assurance that we will ever be granted the right to commercialize Firdapse® for any of these additional indications.

We are also currently in the early stages of developing a long-acting formulation of amifampridine. We have retained a contractor who is currently assisting us in developing the formulation of the product. We currently anticipate that initial formulation candidates and their drug release and absorption properties should be determined in 2020. There can be no assurance we will be able to successfully develop a long-acting formulation of amifampridine and that such formulation will ever be approved by the FDA for commercialization.

In October 2019, we submitted an NDS in Canada seeking approval of Firdapse® for the treatment of LEMS. Our application has been accepted for review and we have been granted a priority review. There can be no assurance that our application will be approved.

On May 29, 2019, we entered into an amendment to our license agreement for Firdapse®. Under the amendment, we have 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.

We are currently in discussions with Japanese regulatory authorities to determine the type of clinical trial that will be required before we will be granted the right to file an application to commercialize Firdapse® in Japan. There can be no assurance that we will successfully obtain the right to commercialize Firdapse® in Japan.

All of our patent rights for Firdapse® are derived from our license agreement. Under the License Agreement, we licensed two pending patents and certain trademarks for Firdapse®. One of the licensed applications, U.S. App. No. 10/467,082 is abandoned as are its children (U.S. App. No. 14/085,017 and 14/818,848) such that we are no longer pursuing patent protection out of this family of applications. The second licensed patent application claims methods

of administering Firdapse®. We recently received an office action from the United States Patent and Trademark Office responding to our second application, and we are in the process of responding to that office action. There can be no assurance that our pending patent will be granted or as to the protection from competition that it will provide us if it is granted.

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 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.

Broad-based business or economic disruptions could adversely affect our ongoing or planned research and development activities. For example, in December 2019 an outbreak of a novel strain of coronavirus (COVID-19) originated in Wuhan, China and has since spread to a number of other countries, including the United States. To date, this outbreak has already resulted in extended shutdowns of businesses and curtailment of travel and large gatherings around the world. While we do not source Firdapse® or its active pharmaceutical ingredient from China, global health concerns, such as coronavirus, could also result in social, economic, and labor instability in the countries in which we or the third parties with whom we engage operate. Further, this outbreak could affect the timing of our clinical trials. 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 likely that these global health concerns could disproportionately impact the hospitals and clinical sites in which we conduct our clinical trials, which could slow our clinical trials or adversely effect our business.

Generic Sabril®

In September 2015, we announced the initiation of a project to develop generic versions of Sabril® (vigabatrin). Sabril® is marketed by Lundbeck Inc. in the United States in two dosage forms (powder sachets and tablets) for the treatment of infantile spasms and refractory complex partial seizures. Par Pharmaceutical brought the first generic version of the powder sachet to market, and, to date, several generic versions of the powder sachets have been approved. However, at this time, there is only one approved generic version of the tablets.

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 $500,000. We will be entitled to receive a milestone payment of $2.0 million on the commercial launch of the product. Further, we will receive a sharing of defined net profits upon commercialization and 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, 2019, we had cash and investments of approximately $94.5 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 and cash flow positive. Further, there can be no assurance that if we need additional funding in the future, whether such funding will be available to us. See "Liquidity and Capital Resources" below for further information on our liquidity and cash flow.

Basis of Presentation

Revenues.

Prior to the launch of Firdapse® in January 2019 we did not generate revenues for product sales. In 2019 we generated revenues from product sales of Firdapse®. We expect these revenues to fluctuate in future periods based on our sales of Firdapse®. In 2018, we generated revenues of $500,000 from up-front license fees received under a collaborative agreement with Endo. We expect our revenues from the collaborative agreement to fluctuate in future periods based on our collaborators' abilities 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. Prior to FDA approval in November 2018, the cost of manufacturing Firdapse® was expensed, including our build-up of anticipated launch product. This will cause the cost of sales to appear artificially low for product manufactured prior to approval, until we deplete such product and additional product is manufactured.

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 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 have continued to incur commercialization expenses, inclusive of sales, marketing and other commercialization related expenses as we have begun our sales program for Firdapse®. We had no product sales or selling expenses in 2018.

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, cost for preparation for commercialization, and professional fees for legal, 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.

At December 31, 2019 and 2018, respectively, we had net operating loss carryforwards and other credits of approximately $41.5 million and $79.0 million available to reduce future taxable income, if any. We have evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. As of December 31, 2019, and December 31, 2018, based on our long history of operating losses, we have concluded that it is more likely than not that the benefit of our deferred tax assets will not be realized. Accordingly, we have provided a full valuation allowance for deferred tax assets including NOL and tax credit carryover as of December 31, 2019 and December 31, 2018. The valuation allowance decreased by $6.8 million and increased by $9.2 million during 2019 and 2018, respectively.

As required by ASC 740, *Income Taxes*, we recognize 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.

We are currently conducting a study of the availability for use of our net operating loss carryforwards and other credits under Section 382 of the Internal Revenue Code, and the results of this study could impact the amounts of net operating losses and other credits that we have available for use in future periods, and the timing of their use.

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.

Non-GAAP Financial Measures.

We prepare our consolidated financial statements and notes thereto which accompany this report in accordance with U.S. GAAP. To supplement our financial results presented on a U.S. GAAP basis, we may use non-GAAP financial measures in our reports filed with the Commission and/or our communications with investors. Non-GAAP measures are provided as additional information and not as an alternative to our consolidated financial statements presented in accordance with GAAP. Our non-GAAP financial measures are intended to enhance an overall understanding of our current financial performance. We believe that the non-GAAP financial measures we present provide investors and prospective investors with an alternative method for assessing our operating results in a manner that we believe is focused on the performance of ongoing operations and provide a more consistent basis for comparison between periods.

The non-GAAP financial measure that we present excludes from the calculation of net income the expense associated with stock-based compensation. Further, we often report non-GAAP net income (loss) per share, which is calculated by dividing non-GAAP net income (loss) by the weighted average common shares outstanding.

Any non-GAAP financial measures that we report should not be considered in isolation or as a substitute for comparable U.S. GAAP accounting, and investors should read them in conjunction with our financial statements and notes thereto prepared in accordance with U.S. GAAP. Finally, the non-GAAP measures of net income (loss) we may use may be different from, and not directly comparable to, similarly titled measures used by other companies.

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 accounting policies described below are not intended to be a comprehensive list of all of our accounting policies. In many cases, the accounting treatment of a particular transaction is specifically dictated by 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 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 distributor (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, 2019 and, therefore, the transaction price was not reduced further during the year ended December 31, 2019. 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.

Leases.

Operating lease right-of-use (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 our leases do not provide an implicit rate, we used our 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 terms do not include options to extend or terminate the lease as it 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 lease agreements with lease and non-lease components, which are generally accounted for separately. Refer to Note 2, "Basis of Presentation and Significant Accounting Policies," in the consolidated financial statements included in this report for further details on leases.

Preclinical Study and Clinical Trial Expenses.

Research and development expenditures are charged to operations as incurred. Our expenses related to preclinical and clinical trials are based on actual and estimated costs of the services received and efforts expended pursuant to contracts with multiple research institutions and any CRO that conducts and manages our clinical trials. The financial terms of these agreements are subject to negotiation and will vary from contract to contract and may result in uneven payment flows. Generally, these agreements will set forth the scope of the work to be performed at a fixed fee or unit price. Payments under these contracts will depend on factors such as the successful enrollment of patients or the completion of clinical trial milestones. Expenses related to clinical trials generally are accrued based on contracted amounts applied to the level of patient enrollment and activity according to the protocol. If timelines or contracts are modified based upon changes in the clinical trial protocol or scope of work to be performed, we would be required to modify estimates accordingly on a prospective basis.

Stock-Based Compensation.

We recognize stock-based compensation for the fair value of all stock-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 options awards. For the years ended December 31, 2019 and 2018, the assumptions used were an estimated annual volatility of 75.5% and 82%, expected holding periods of four and a half years and zero to seven years, and risk-free interest rates of 1.51% to 2.53% and 2.09% to 2.88%, respectively.

Results of Operations

Years Ended December 31, 2019 and 2018

Revenues.

For the year ended December 31, 2019, we recognized $102.3 million in net revenue from product sales of Firdapse®. We had no revenues from product sales for the year ended December 31, 2018. We had no revenues from our collaborative arrangement in 2019. We had revenues in 2018 in the amount of $500,000 relating to the up-front payment from Endo in connection with the collaboration agreement for vigabatrin tablets.

Cost of Sales.

Cost of sales was $14.8 million for the year ended December 31, 2019, compared to $0 for the year ended December 31, 2018. The increase in cost of sales was entirely attributable to the commercial launch of Firdapse® in January 2019. Cost of sales includes royalty payments which are based on net revenue as defined in the applicable license agreement. Further, cost of sales may be artificially low until we fully utilize product manufactured and recorded as expense prior to approval.

Research and Development Expenses.

Research and development expenses for the years ended December 31, 2019 and 2018 were approximately $18.8 million and $19.9 million, respectively, and represented approximately 27% and 56% of total operating costs and expenses for the years ended December 31, 2019, and 2018 respectively. Research and development expenses for the years ended December 31, 2019 and 2018 were as follows:

	For the year ended December 31,		Change	
	2019	2018	$	%
Research and development expenses	$ 17,705,156	$ 18,839,974	(1,134,818)	(6.0)%
Employee stock-based compensation	1,137,596	1,079,230	58,366	5.4%
Total research and development expenses	$ 18,842,752	$ 19,919,204	(1,076,452)	(5.4)%

Research and development expenses for the fiscal year ended December 31, 2019 primarily consisted of expenses for medical and regulatory affairs and quality assurance programs, as well as expenses from our ongoing clinical trials and studies evaluating Firdapse® for the treatment of other ultra-orphan neuromuscular diseases and our Expanded Access Program. Research and development expenses in the comparable period in 2018 primarily consisted of consulting expenses and milestones as we submitted and the FDA approved an NDA for Firdapse® for the treatment of adults (age 17 and up) with LEMS, as well as expenses from our clinical trials and studies and our Expanded Access Program.

We expect that research and development expenses will continue to be substantial in 2020 as we continue our clinical program evaluating Firdapse® for the treatment of MuSK-MG, continue our proof-of-concept trial for SMA Type 3, continue our Expanded Access Program, take steps to develop a sustained release formulation of Firdapse®, begin to evaluate Firdapse® as a treatment for other neuromuscular diseases, and potentially prepare an sNDA for Firdapse® for the treatment of MuSK-MG.

Selling, General and Administrative Expenses.

Selling, general and administrative expenses for the years ended December 31, 2019 and 2018 were approximately $36.9 million and $15.9 million, respectively, and represented approximately 52% and 44% of total operating costs and expenses for the years ended December 31, 2019, and 2018 respectively. Selling, general and administrative expenses for the years ended December 31, 2019 and 2018 were as follows:

	For the year ended December 31,		Change	
	2019	2018	$	%
Selling	$ 19,947,916	$ —	19,947,916	100%
General and administrative	14,246,052	13,404,547	841,505	6.3%
Employee stock-based compensation	2,687,219	2,471,414	215,805	8.7%
Total selling, general and administrative expenses	$ 36,881,187	$ 15,875,961	21,005,226	132.3%

For the year ended December 31, 2019, selling, general and administrative expenses increased approximately $21.0 million, compared to the same period in 2018, primarily attributable to the following:

- increases in selling (commercialization) expenses, which consist primarily of commercial systems implementation costs, hiring of the sales force and supporting personnel, product launch costs, and costs of our market access and market research efforts (pre-commercial expenses incurred in 2018 in the amount of $6,897,483 before our launch of commercial Firdapse® were included in general and administrative expenses); and
- increases in general and administrative costs attributable to the growth of our organization as we have grown from an R&D company to a commercial stage pharmaceutical company;
- contributions to 501(c)(3) organizations supporting LEMS patients, including the accrual in the fourth quarter of 2019 of approximately $1.5 million in contributions attributable to 2020 activities; and
- increases in employee stock-based compensation which is non cash and relates to the expense of stock options awarded to certain employees, officers and directors.

Stock-Based Compensation.

Total stock-based compensation expense for the years ended December 31, 2019 and 2018 was $3,824,815 and $3,550,644, respectively. We regularly grant non-cash stock-based compensation to employees and directors as part of their compensation packages. The increase in stock-based compensation for the year ended December 31, 2019, when compared to the same period in 2018, is primarily due to the expense of grants to new employees hired in connection with the launch of Firdapse®.

Other Income, Net.

We reported other income, net in all periods primarily relating to our investment of funds received from offerings of our securities and product sales. The increase in other income, net for the year ended December 31, 2019 when compared to the same period in 2018 is primarily due to higher invested balances and higher yields on investments. Other income, net, consists of interest income, dividend income, and realized gain (loss) on trading securities. For the year ended December 31, 2019, other income, net also includes $100,000 received as part of a settlement agreement between us and Northwestern.

Income Taxes.

Our effective income tax rate was 4.6% and 0.0% for the year ended December 31, 2019 and 2018, respectively. Differences in the effective tax and the statutory federal income tax rate of 21% is driven by state income taxes and anticipated annual permanent differences, including orphan drug credit expense limitations and other items.

We had no uncertain tax positions as of December 31, 2019 and December 31, 2018. We have a full valuation allowance for our deferred tax assets at December 31, 2019 and December 31, 2018.

Net Income (Loss).

Our net income was $31,875,337 in the year ended December 31, 2019 ($0.31 and $0.30, respectively, per basic and diluted share) as compared to a net loss of ($34,003,514) in the year ended December 31, 2018 ($0.33 per basic and diluted share).

Non-GAAP Net Income.

Our non-GAAP net income, which excludes for 2019 a $3,824,815 expense associated with stock-based compensation was $35,700,152 ($0.35 and $0.34, respectively, per basic and diluted share). Our non-GAAP net loss for the year ended December 31, 2018 was $30,452,870 ($0.30 per basic and diluted share), which excludes non-cash stock compensation of $3,550,644.

Liquidity and Capital Resources

Since our inception, we have financed our operations primarily through multiple public and private offerings of our securities. However, during January 2019, we launched our initial product, Firdapse®, and began to receive revenues from product sales. At December 31, 2019, we had cash and cash equivalents and investments aggregating $94.5 million and working capital of $87.3 million. At December 31, 2018, we had cash and cash equivalents and investments aggregating $58.5 million and working capital of $45.7 million. At December 31, 2019, 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 short-term interest-bearing obligations and U.S. Treasuries.

We incurred operating losses through the quarter ended March 31, 2019 and reported operating income for the first time during the three and six month periods ended June 30, 2019. We expect to continue to spend substantial dollars on our current and future drug development programs.

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 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;
- 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 plan to raise additional funds that we may require in the future 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 12, 2017, we filed a shelf registration statement with the SEC to sell up to $150 million of common stock, preferred stock, warrants to purchase common stock, debt securities and units consisting of one or more of such securities (the "2017 Shelf Registration Statement"). The 2017 Shelf Registration Statement (file no. 333-219259) was declared effective by the SEC on July 26, 2017. We have completed one offering under the 2017 Shelf Registration Statement, raising net proceeds of approximately $53.8 million from the sale of 16,428,572 shares of our common stock on November 28, 2017.

As of the date of this Form 10-K, $92.5 million of our 2017 Shelf Registration Statement remains available for future sales.

On December 23, 2016, we filed a shelf registration statement with the SEC to sell up to $33.8 million of common stock (the "2016 Shelf Registration Statement"). This shelf registration statement was declared effective by the SEC on January 9, 2017. We have made no sales under the 2016 Shelf Registration Statement. This shelf registration statement has now expired.

Cash Flows.

Net cash provided by (used in) operating activities was $34,611,473 and ($26,147,545), respectively, for the years ended December 31, 2019 and 2018. During the year ended December 31, 2019, net cash provided by operating activities was primarily attributable to our net income of $31,875,337, increases of $1,780,080 in accounts payable, $12,540,197 in accrued expenses and other liabilities, and of $3,831,736 of non-cash expenses. This was partially offset by increases of $10,536,997 in accounts receivable, net, $1,900,780 in inventory, and $2,701,293 in prepaid expenses and other current and non-current assets and a decrease of $276,807 in operating lease liability. During the year ended December 31, 2018, net cash used in operating activities was primarily attributable to our net loss of $34,003,514 and increases of $476,037 in prepaid expenses and other current assets and $56,012 in inventory, which was partially offset by increases of $391,792 in accounts payable and $4,850,743 in accrued expenses and other liabilities. The loss included an additional $3,145,483 of non-cash expenses.

Net cash provided by investing activities was $37,224,595 for the year ended December 31, 2019, consisting primarily of proceeds from sales and maturities of investments of $71,969,365, partially offset by purchases of investments of $34,725,401. Net cash used in investing activities was $15,082,872 for the year ended December 31, 2018, consisting primarily of purchases of investments of $36,790,854, partially offset by proceeds from sales and maturities of investments of $21,800,000.

Net cash provided by financing activities during the years ended December 31, 2019 and 2018 was $1,116,242 and $293,115, respectively, consisting primarily of proceeds from the exercise of options to purchase common stock.

Contractual Obligations and Arrangements.

We have entered into the following contractual arrangements:

- *Payments to BioMarin and others under our license agreement with BioMarin*. Under our license agreement, we have agreed to pay (i) royalties to BioMarin 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 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 year ended December 31, 2019, we recognized approximately $13.6 million of royalties, which is included in cost of sales in the accompanying consolidated statement of operations.
- *Purchase commitments*. We have entered into purchase commitments with our contract manufacturing organizations aggregating to approximately $950,000 per year. The agreements expire on various dates through 2024.
- *Employment agreements*. We have entered into an employment agreement with our Chief Executive Officer that requires us to make base salary payments of approximately $546,000 in 2019. The agreement expires in November 2020.
- *Lease for office space*. We operate our business in leased office space in Coral Gables, Florida. We currently lease approximately 7,800 square feet of office space for which we pay annual rent of approximately $330,000.

Off-Balance Sheet Arrangements.

We currently have no debt or finance leases. We have operating leases 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 Annual Report on Form 10-K contains “forward-looking statements”, as that term is defined in the Private Securities Litigation Reform Act of 1995. These include statements regarding our expectations, beliefs, plans or

objectives for future operations and anticipated results of operations. For this purpose, any statements contained herein that are not statements of historical fact may be deemed to be forward-looking statements. Without limiting the foregoing, "believes", "anticipates", "proposes", "plans", "expects", "intends", "may", and other similar expressions are intended to identify forward-looking statements. Such statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or other achievements to be materially different from any future results, performances or achievements expressed or implied by such forward-looking statements. Factors that might cause such differences include, but are not limited to, those discussed in the section entitled "Item 1A – Risk Factors" and those discussed in the section entitled "Item 7 – Management's Discussion and Analysis of Financial Condition and Results of Operations – Caution Concerning Forward-Looking Statements."

The continued successful commercialization of Firdapse® and the development of additional indications for Firdapse® is highly uncertain. Factors that will affect our success include the uncertainty of:

- Whether we will be able to continue 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;
- If the average daily dose taken by patients changes over time, it could affect 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 guidance that we provide to the public market will turn out to be accurate;
- Whether payors will continue to 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®;
- The effect on our business and future results of operations arising from the approval by the FDA of Ruzurgi® for the treatment of pediatric LEMS patients (ages 6 to under 17);
- Whether our suit against the United States FDA seeking to vacate the FDA's approval of Ruzurgi® will be successful;
- Whether we can continue to compete successfully if the approval of Ruzurgi® is not overturned and Ruzurgi® continues to be prescribed for off-label use in adult LEMS patients;
- Whether, because of the lower price of Ruzurgi®, payers will require that patients try off-label Ruzurgi® first before they approve Firdapse® as a treatment for adult LEMS patients;
- The impact on Firdapse® of adverse changes in potential 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;

- The impact on our business and results of operations of public statements by politicians and a vocal group of LEMS patients and doctors who object to our pricing of Firdapse®;
- Changes in the healthcare industry and the effect of political pressure from President Trump, Congress and/or medical professionals seeking to reduce prescription drug costs;
- The impact of the recent outbreak of a novel strain of coronavirus on our business or on the economy generally;
- The state of the economy generally and its impact on our business;
- Changes to the healthcare industry occasioned by any future repeal and replacement of the Affordable Care Act, 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 our trials and studies on a timely basis and within the budgets we establish for such trials and studies;
- Whether the recent coronavirus outbreak will affect the timing of our currently on going clinical trials;
- Whether the trials that we are currently undertaking to evaluate Firdapse® for the treatment of Anti-MuSK antibody positive myasthenia gravis (MuSK-MG), and Spinal Muscular Atrophy (SMA) Type 3, or any other trials that we may undertake in the future, will be successful;
- Whether if our MuSK-MG Phase 3 clinical trial is successful, the FDA will permit us to submit a supplemental new drug application (sNDA) for MuSK-MG without a second Phase 3 trial, and whether any such application will be accepted for filing (and even if accepted, whether such application will be approved);
- Whether Firdapse® will ever be approved for the treatment of MuSK-MG, SMA Type 3, or any other neuromuscular disease;
- Whether our NDS filing in Canada to commercialize Firdapse® in that jurisdiction will be approved and, even if approved for sale in Canada, whether we can successfully commercialize the product in Canada on a profitable basis;
- Whether we will be able to obtain approval to commercialize Firdapse® in Japan and what clinical trials will be required in Japan in order to obtain such marketing approval;
- Whether we can successful develop, obtain approval of and successfully market a sustained release version of Firdapse®;
- Whether our efforts to grow our business beyond Firdapse® through acquisitions of companies or in-licensing of product opportunities in the neuromuscular or neurology therapeutic areas will be successful;
- Whether we will have sufficient capital to finance any such acquisitions;
- Whether our version of generic vigabatrin tablets will ever be approved by the FDA;
- Even if vigabatrin tablets are 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 commercialization of Firdapse® and the development of additional indications for 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

Not applicable.

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, 2019, 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, 2019 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, 2019.

During the fourth quarter of 2019, 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 2020 Annual Meeting of Stockholders. Our Proxy Statement for the 2020 Annual Meeting of Stockholders is expected to be filed not later than 120 days after the end of our fiscal year ended December 31, 2019 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

(a) Documents filed as part of this report.

1. 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, 2019 and 2018
- Consolidated Statements of Operations and Comprehensive Income (Loss) for the years ended December 31, 2019 and 2018
- Consolidated Statements of Changes in Stockholders' Equity for the years ended December 31, 2019 and 2018
- Consolidated Statements of Cash Flows for the years ended December 31, 2019 and 2018
- Notes to Consolidated Financial Statements

2. 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.

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

(b) Exhibits.

Exhibit No.	Description of Exhibit
2.1	Agreement and Plan of Merger, dated August 14, 2006, between the Company and Catalyst Pharmaceutical Partners, Inc., a Florida corporation
3.1	Certificate of Incorporation
3.2	Amendment to Certificate of Incorporation
3.3	Amendment to Certificate of Incorporation
3.4	Amendment to Certificate of Incorporation
3.5	By-laws
4.1	Specimen stock certificate for common stock
4.2	Rights Agreement between the Company and Continental Stock Transfer and Trust Company
4.3	Amendment to Rights Agreement
4.4	Second Amendment to Rights Agreement
4.5	Description of the Company's Capital Stock*
10.1 +	Employment Agreement between the Company and Patrick J. McEnany
10.2 +	First Amendment to Employment Agreement between the Company and Patrick J. McEnany

Exhibit No.	Description of Exhibit
10.3 +	Second Amendment to Employment Agreement between the Company and Patrick J. McEnany
10.4 +	Third Amendment to Employment Agreement between the Company and Patrick J. McEnany
10.5+	Fourth Amendment to Employment Agreement between the Company and Patrick J. McEnany
10.6+	Fifth Amendment to Employment Agreement between the Company and Patrick J. McEnany
10.7+	Sixth Amendment to Employment Agreement between the Company and Patrick J. McEnany
10.8+	2014 Stock Incentive Plan
10.9+	Amendment No. 1 to 2014 Stock Incentive Plan
10.10+	Amendment No. 2 to 2014 Stock Incentive Plan
10.11+	2018 Stock Incentive Plan
10.12	Lease Agreement between the Company and 355 Alhambra Plaza, Ltd.
10.13	First Amendment to Lease Agreement between the Company and 355 Alhambra Plaza, Ltd.
10.14	Second Amendment to Lease, dated as of February 4, 2014, between the Company and 355 Alhambra Circle LLC
10.15	Third Amendment to Lease, dated effective as of March 16, 2015, between the Company and 355 Alhambra Circle LLC
10.16	Fourth Amendment to Lease, dated effective as of August 13, 2018 among the Company and PRII 355 Alhambra Circle, LLC
10.17	License Agreement, dated as of October 26, 2012, between the Company and BioMarin
10.18	Amendment No. 1 to License Agreement, dated April 8, 2014, between the Company and BioMarin
10.19	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
10.20	Second Amendment to License Agreement, dated May 29, 2019 between the Company and BioMarin
10.21	Development, License and Commercialization Agreement, dated effective as of December 18, 2018, by and between Endo Ventures Limited and the Company
21.1	Subsidiaries of the registrant*
23.1	Consent of Independent Registered Public Accounting Firm*
31.1	Section 302 CEO Certification*
31.2	Section 302 CFO Certification*

Exhibit No.	Description of Exhibit
32.1	Section 906 CEO Certification*
32.2	Section 906 CFO Certification*
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema
101.CAL	XBRL Taxonomy Extension Calculation Linkbase
101.DEF	XBRL Taxonomy Extension Definition Linkbase
101.LAB	XBRL Taxonomy Extension Label Linkbase
101.PRE	XBRL Taxonomy Extension Presentation Linkbase

+ Management contract or compensatory plan
* Filed herewith

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, 2020.

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, 2020
/s/ Alicia Grande Alicia Grande	Vice President, Treasurer, Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	March 16, 2020
/s/ Charles B. O'Keeffe Charles B. O'Keeffe	Director	March 16, 2020
/s/ Philip H. Coelho Philip H. Coelho	Director	March 16, 2020
/s/ David S. Tierney, M.D. David S. Tierney, M.D.	Director	March 16, 2020
/s/ Donald A. Denkhaus Donald A. Denkhaus	Director	March 16, 2020
/s/ Richard Daly Richard Daly	Director	March 16, 2020

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Years ended December 31, 2019 and 2018

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, 2019, 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, 2019, 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, 2019, and our report dated March 16, 2020 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, 2020

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, 2019 and 2018, the related consolidated statements of operations and comprehensive income (loss), changes in stockholders' equity, and cash flows for each of the two years in the period ended December 31, 2019, 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, 2019 and 2018, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2019, 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, 2019, 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, 2020, 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 supporting 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.

/s/ GRANT THORNTON LLP

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

Miami, Florida
March 16, 2020

CATALYST PHARMACEUTICALS, INC.
CONSOLIDATED BALANCE SHEETS

	December 31, 2019	December 31, 2018
ASSETS		
Current Assets:		
Cash and cash equivalents	$ 89,511,710	$ 16,559,400
Short-term investments	5,007,050	36,922,213
Accounts receivable, net	10,536,997	—
Inventory	1,956,792	56,012
Prepaid expenses and other current assets	4,351,074	1,649,781
Total current assets	111,363,623	55,187,406
Investments	—	5,008,243
Operating lease right-of-use asset	793,252	—
Property and equipment, net	210,467	245,425
Deposits	8,888	8,888
Total assets	$ 112,376,230	$ 60,449,962
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current Liabilities:		
Accounts payable	$ 4,117,447	$ 2,337,367
Accrued expenses and other liabilities	19,981,295	7,173,987
Total current liabilities	24,098,742	9,511,354
Accrued expenses and other liabilities, non-current	—	154,799
Operating lease liability, net of current portion	647,532	—
Total liabilities	24,746,274	9,666,153
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, $0.001 par value, 5,000,000 shares authorized: none issued and outstanding at December 31, 2019 and 2018	—	—
Common stock, $0.001 par value, 150,000,000 shares authorized; 103,397,033 shares and 102,739,257 shares issued and outstanding at December 31, 2019 and 2018, respectively	103,397	102,739
Additional paid-in capital	216,205,678	211,265,279
Accumulated deficit	(128,688,624)	(160,563,961)
Accumulated other comprehensive income (loss)	9,505	(20,248)
Total stockholders' equity	87,629,956	50,783,809
Total liabilities and stockholders' equity	$ 112,376,230	$ 60,449,962

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

CATALYST PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE INCOME (LOSS)

	Year Ended December 31,	
	2019	**2018**
Revenues:		
Product revenue, net	$102,306,337	$ —
Revenues from collaborative arrangement	—	500,000
Total revenues	102,306,337	500,000
Operating costs and expenses:		
Cost of sales	14,759,139	—
Research and development	18,842,752	19,919,204
Selling, general and administrative	36,881,187	15,875,961
Total operating costs and expenses	70,483,078	35,795,165
Operating income (loss)	31,823,259	(35,295,165)
Other income, net	1,585,774	1,291,651
Net income (loss) before income taxes	33,409,033	(34,003,514)
Provision for income taxes	1,533,696	—
Net income (loss)	$ 31,875,337	$ (34,003,514)
Net income (loss) per share:		
Basic	$ 0.31	$ (0.33)
Diluted	$ 0.30	$ (0.33)
Weighted average shares outstanding:		
Basic	102,944,316	102,633,884
Diluted	106,020,936	102,633,884
Net income (loss)	$ 31,875,337	$ (34,003,514)
Other comprehensive income (loss):		
Unrealized gain (loss) on available-for-sale securities	29,753	(20,248)
Comprehensive income (loss)	$ 31,905,090	$ (34,023,762)

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

CATALYST PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY

For the years ended December 31, 2019 and 2018

	Preferred Stock	Common Stock	Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Gain (Loss)	Total
Balance at December 31, 2017	$ —	$102,549	$207,421,710	$ (126,560,447)	$ —	$ 80,963,812
Issuance of common stock, net	—	3	10,546	—	—	10,549
Issuance of stock options for services	—	—	3,535,647	—	—	3,535,647
Exercise of stock options for common stock	—	187	297,376	—	—	297,563
Other comprehensive gain (loss)	—	—	—	—	(20,248)	(20,248)
Net income (loss)	—	—	—	(34,003,514)	—	(34,003,514)
Balance at December 31, 2018	—	102,739	211,265,279	(160,563,961)	(20,248)	50,783,809
Issuance of stock options for services	—	—	3,780,086	—	—	3,780,086
Exercise of stock options for common stock	—	658	1,115,584	—	—	1,116,242
Amortization of restricted stock for services	—	—	44,729	—		44,729
Other comprehensive gain (loss)	—	—	—	—	29,753	29,753
Net income (loss)	—	—	—	31,875,337	—	31,875,337
Balance at December 31, 2019	$ —	$103,397	$216,205,678	$ (128,688,624)	$ 9,505	$ 87,629,956

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

CATALYST PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS

	Year Ended December 31,	
	2019	**2018**
Operating Activities:		
Net income (loss)	$ 31,875,337	$(34,003,514)
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities:		
Depreciation	54,327	37,978
Amortization of right-of-use asset	243,399	—
Stock-based compensation	3,824,815	3,550,644
Change in accrued interest and accretion of discount on investments	(290,805)	(443,139)
(Increase) decrease in:		
Accounts receivable, net	(10,536,997)	—
Inventory	(1,900,780)	(56,012)
Prepaid expenses and other current assets and deposits	(2,701,293)	(476,037)
Increase (decrease) in:		
Accounts payable	1,780,080	391,792
Accrued expenses and other liabilities	12,540,197	4,850,743
Operating lease liability	(276,807)	—
Net cash provided by (used in) operating activities	34,611,473	(26,147,545)
Investing Activities:		
Purchases of property and equipment	(19,369)	(92,018)
Purchases of investments	(34,725,401)	(36,790,854)
Proceeds from maturities and sales of investments	71,969,365	21,800,000
Net cash provided by (used in) investing activities	37,224,595	(15,082,872)
Financing Activities:		
Payment of employee withholding tax related to stock-based compensation	—	(4,448)
Proceeds from exercise of stock options	1,116,242	297,563
Net cash provided by (used in) financing activities	1,116,242	293,115
Net increase (decrease) in cash and cash equivalents	72,952,310	(40,937,302)
Cash and cash equivalents – beginning of period	16,559,400	57,496,702
Cash and cash equivalents – end of period	$ 89,511,710	$ 16,559,400
Non-cash investing and financing activities:		
Unrealized gain (loss) on available-for-sale securities	$ 29,753	$ (20,248)

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 biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare debilitating, chronic neuromuscular and neurological diseases, including Lambert-Eaton Myasthenic Syndrome (LEMS), Anti-MuSK antibody positive myasthenia gravis (MuSK-MG), and Spinal Muscular Atrophy (SMA) Type 3. 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 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.

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 several public and private offerings of its securities and from revenues from its product sales. See Note 11 (Stockholders' Equity).

Capital Resources

While there can be no assurance, based on currently available information, the Company estimates that it currently has sufficient resources to support its operations for at least the next 12 months from the issuance date of this Form 10-K.

The Company may raise required 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 product 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.

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.

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

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

cash and cash equivalents deposited with one financial institution. These amounts at times may exceed federally insured limits.

d. INVESTMENTS. The Company invests in high credit-quality funds in order to obtain higher yields on its cash and investments pending the use of those funds in its business. At December 31, 2019, investments consisted of U.S. Treasuries. At December 31, 2018, investments consisted of a short-term bond fund and U.S. Treasuries. Such investments are not insured by the Federal Deposit Insurance Corporation.

Short-Term Bond Fund

The short-term bond fund was classified as a trading security. Trading securities are recorded at fair value based on the closing market price of the security. For trading securities, the Company recognizes realized gains and losses and unrealized gains and losses to earnings. At December 31, 2019, there were no investments classified as trading securities. At December 31, 2018, the only investment classified as trading securities was the short-term bond fund. Realized gains on trading securities were $80,045 for the year ended December 31, 2019. There were no sales of trading securities for the year ended December 31, 2018. Unrealized gain (loss) on trading securities was $0 and ($29,430), respectively, for the years ended December 31, 2019, and 2018 and is included in other income, net in the accompanying consolidated statements of operations.

U.S. Treasuries

U.S. Treasuries are classified as available-for-sale securities. The Company classifies available-for-sale securities with stated maturities of greater than three months and less than one year as short-term investments. Available-for-sale securities with stated maturities greater than one year are classified as non-current investments in the accompanying consolidated balance sheets. The Company records available-for-sale securities at fair value with unrealized gains and losses in accumulated other comprehensive income (loss) in stockholders' equity. Realized gains and losses are included in other income, net in the consolidated statements of operations and comprehensive income (loss) 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 (loss). 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 other-than-temporary. The Company considers various factors in determining whether to recognize an other-than-temporary charge, 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. The Company has not recorded any other-than-temporary impairment charges on its available-for-sale securities. See Note 3 (Investments).

e. ACCOUNTS RECEIVABLE, NET. Accounts receivable are recorded net of customer allowance for distribution fees, trade discounts, prompt payment discounts, chargebacks and doubtful accounts. Allowances for distribution fees, trade discounts, prompt payment discounts and chargebacks are based on contractual terms. The Company estimates the allowance for doubtful accounts based on existing contractual payment terms, actual payment patterns of its customer and individual customer circumstances. At December 31, 2019, the Company determined that an allowance for doubtful accounts 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 with cost determined under the first-in-first-out (FIFO) cost method. Inventories consist of raw materials and supplies, work in process and finished goods. Costs to be capitalized as inventories primarily include third party manufacturing costs and other overhead costs. The Company began capitalizing inventories post FDA approval of Firdapse® on November 28, 2018 as the related costs were expected to be recoverable

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

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 (loss). 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. As of December 31, 2019 inventory consisted mainly of raw materials, work-in-process and finished goods. As of December 31, 2018, inventory consisted mainly of packaging and labeling costs.

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".

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 research fees, prepaid insurance, prepaid commercialization expenses, prepaid subscription fees and prepaid manufacturing. 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. 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 four to seven 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, 2019 and 2018, 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).

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

Level 1 inputs utilize quoted prices (unadjusted) in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date. Level 2 inputs are inputs other than quoted prices included in Level 1 that are observable for the asset or liability, either directly or indirectly. Level 2 inputs may include quoted prices for similar assets and liabilities in active markets, as well as inputs that are observable for the asset or liability (other than quoted prices), such as interest rates, foreign exchange rates, and yield curves that are observable at commonly quoted intervals. Level 3 inputs are unobservable inputs for the asset or liability, which are typically based on an entity's own assumptions, as there is little, if any, related market activity. In instances where the determination of the fair value measurement is based on inputs from different levels of the fair value hierarchy, the level in the fair value hierarchy within which the entire fair value measurement falls is based on the lowest level input that is significant to the fair value measurement in its entirety. The Company's assessment of the significance of a particular input to the fair value measurement in its entirety requires judgment, and considers factors specific to the asset or liability.

		Fair Value Measurements at Reporting Date Using		
	Balances as of December 31, 2019	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	$ 23,963,617	$ 23,963,617	$ —	$ —
U.S. Treasuries	$ 59,932,200	$	$ 59,932,200	$
Short-term investments:				
U.S. Treasuries	$ 5,007,050	$ —	$ 5,007,050	$ —

	Balances as of December 31, 2018	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	$14,462,087	$ 14,462,087	$ —	$ —
Short-term investments:				
Short-term bond fund	$26,541,349	$ 26,541,349	$ —	$ —
U.S. Treasuries	$10,380,864	$ —	$ 10,380,864	$ —
Investments:				
U.S. Treasuries	$ 5,008,243	$ —	$ 5,008,243	$ —

k. OPERATING LEASES. Effective January 1, 2019, the Company determined 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 leases do 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 terms do not include options to extend or terminate the lease as it 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 lease agreements with lease and

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

non-lease components, which are generally accounted for separately. Refer to Note 2.v. for discussion on adoption method.

l. REVENUE RECOGNITION. Prior to the January 2019 launch of Firdapse®, the Company did not generate any product revenue. Therefore, on January 1, 2019, the Company adopted Accounting Standards Codification ("ASC") Topic 606 – Revenue from Contracts with Customers ("Topic 606"). This standard applies to all contracts with customers, except for contracts that are within the scope of other standards, such as leases, collaborative arrangements and financial instruments. Under Topic 606, an entity recognizes revenue when its customer obtains control of the promised goods or services, in an amount that reflects the consideration which the entity expects to be entitled in exchange for these goods or services. 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"), who 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®.

To determine revenue recognition for arrangements that are within the scope of 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 only applies the five-step model to arrangements that meet the definition of a contract under Topic 606, including when it is probable that the Company will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, once the contract is determined to be within the scope of Topic 606, the Company assesses the goods or services promised within each contract and determines those that are performance obligations and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied. For a complete discussion of accounting for product revenue, see Product Revenue, Net below.

The Company also may generate revenues from payments received under a collaborative agreement. Collaborative agreement payments may include nonrefundable fees at the inception of the agreements, milestone and event-based payments for specific achievements designated in the collaborative agreements, and/or royalties on sales of products resulting from a collaborative arrangement. For a complete discussion of accounting for collaborative arrangements, see Revenues from Collaborative Arrangement 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). 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.

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

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 year ended December 31, 2019.

As of December 31, 2019, all of the Company's sales are to its Customer.

Reserves for Variable Consideration: 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 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 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 detailed below as of December 31, 2019 and, therefore, the transaction price was not reduced further during the year ended December 31, 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, 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 (loss). However, if the Company has determined such services received to date 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 (loss) through December 31, 2019, as well as a reduction to 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 Customer 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.

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

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, as well as reductions to accounts receivable, net on the consolidated balance sheets. 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. 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 principally 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.

Bridge and Patient Assistance Programs: The Company provides free Firdapse® 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 free Firdapse® 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 payer 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.

Revenues from Collaborative Arrangement: The Company has entered into a collaboration agreement for the further development and commercialization of generic Sabril® (vigabatrin) tablets. Pursuant to the terms of this agreement, collaborators could be required to make various payments to the Company, including upfront license fees, milestone payments based on achievement of regulatory approvals, and royalties on sales of products resulting from the collaborative agreement.

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

Nonrefundable upfront license fees are recognized upon receipt as persuasive evidence of an arrangement exists, the price to the collaborator is fixed or determinable and collectability is reasonably assured.

The collaborative agreement provides for a milestone payment upon achievement of development and regulatory events. The Company accounts for milestone payments in accordance with the provisions of Accounting Standards Update (ASU) No. 2010-17, Revenue *Recognition – Milestone Method ("Milestone Method of Accounting")*. The Company recognizes consideration that is contingent upon the achievement of a milestone in its entirety as revenue in the period in which the milestone is achieved only if the milestone is substantive in its entirety. A milestone is considered substantive when it meets all of the following criteria:

1. The consideration is commensurate with either the entity's performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the entity's performance to achieve the milestone;
2. The consideration relates solely to past performance; and
3. The consideration is reasonable relative to all of the deliverables and payment terms within the arrangement.

A milestone is defined as an event (i) that can only be achieved based in whole or in part on either the entity's performance or on the occurrence of a specific outcome resulting from the entity's performance, (ii) for which there is substantive uncertainty at the date the arrangement is entered into that the event will be achieved, and (iii) that would result in additional payments being due to the vendor.

The Company believes that achievement of the milestone will be substantive and there will be no substantive uncertainty once the milestone is achieved.

Since the Company will receive royalty reports 60 days after quarter end, royalty revenue from sales of collaboration products by our collaborators will be recognized in the quarter following the quarter in which the corresponding sales occurred. As of December 31, 2019 and 2018, there was no royalty revenue from sales of the collaborative product.

Refer to Note 7 (Collaborative Arrangement), for further discussion on the Company's collaborative arrangement.

m. 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.

n. STOCK-BASED COMPENSATION. The Company recognizes expense in the consolidated statements of operations for the fair value of all stock-based payments to employees, directors, scientific advisors 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 five years. Forfeitures are recognized as a reduction of stock-based compensation expense as they occur.

o. 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.

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

The Company sells its product in the United States through an exclusive distributor (its Customer) to specialty pharmacies. Therefore, its distributor and specialty pharmacies account for 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 doubtful accounts 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.

p. ROYALTIES. Royalties incurred in connection with the Company's license agreement, as disclosed in Note 9 (Agreements), are expensed to cost of sales as revenue from product sales is recognized.

q. 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 2016. 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.

On December 22, 2017, the U.S. government enacted comprehensive tax legislation commonly referred to as the Tax Cuts and Jobs Act (the "Tax Act"). The Tax Act makes broad and complex changes to the U.S. tax code, including, but not limited to: (1) reducing the U.S. federal corporate tax rate from 35 to 21 percent; (2) requiring companies to pay a one-time transition tax on certain repatriated earnings of foreign subsidiaries; (3) generally eliminating U.S. federal income taxes on dividends from foreign subsidiaries; (4) requiring a current inclusion in U.S. federal taxable income earnings of controlled foreign corporations; (5) eliminating the corporate alternative minimum tax (AMT) and changing how existing AMT credits can be realized; (6) creating the base erosion anti-abuse tax (BEAT), a new minimum tax;

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

(7) creating a new limitation on deductible interest expense, and (8) changing rules related to uses and limitations of net operating loss carryforwards created in tax years beginning after December 31, 2017.

r. COMPREHENSIVE INCOME (LOSS). U.S. GAAP requires that all components of comprehensive income (loss) be reported in the financial statements in the period in which they are recognized. Comprehensive income (loss) is net income (loss), plus certain other items that are recorded directly into stockholders' equity. The Company's comprehensive income (loss) is shown on the consolidated statements of operations and comprehensive income (loss) for the years ended December 31, 2019 and 2018, and is comprised of net unrealized gains (losses) on the Company's available-for-sale securities.

s. NET INCOME (LOSS) PER COMMON SHARE. Basic net income (loss) per share is computed by dividing net income (loss) 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 (loss) per common share is computed by dividing net income (loss) 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,	
	2019	2018
Basic weighted average common shares outstanding	102,944,316	102,633,884
Effect of dilutive securities:		
Common stock issuable upon the exercise of stock options	3,076,620	—
Diluted weighted average common shares outstanding	106,020,936	102,633,884

Outstanding common stock equivalents totaling approximately 4.6 million, were excluded from the calculation of diluted net income (loss) per common share for the year ended December 31, 2019 as their effect would be anti-dilutive. For the year ended December 31, 2018, approximately 10.5 million shares of outstanding stock options were excluded from the calculation of diluted net loss per common share because a net loss was reported in this period and therefore their effect was anti-dilutive. Potentially dilutive options to purchase common stock as of December 31, 2019 had exercise prices ranging from $0.79 to $4.20. Potentially dilutive options to purchase common stock as of December 31, 2018 had exercise prices ranging from $0.79 to $4.64.

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

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

v. RECENTLY ISSUED ACCOUNTING STANDARDS. In February 2016, the FASB issued ASU No. 2016-02, Leases (Topic 842), which requires an entity to recognize assets and liabilities arising from a lease for both financing and operating leases. ASU No. 2016-02 also requires new qualitative and quantitative disclosures to help investors and other financial statement users better understand the amount, timing, and uncertainty of cash flows arising from leases. ASU 2016-02 is effective for fiscal years beginning after December 15, 2018. The Company adopted the standard as of January 1, 2019, using the modified retrospective approach in which prior comparative periods are not adjusted. The Company elected

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

the package of practical expedients permitted under the transition guidance within the new standard, which among other things, allows the Company to carry forward historical lease classification. The Company has operating leases for its office facilities, which expire on November 30, 2022. As of January 1, 2019, the Company recognized an additional right-of-use asset and corresponding operating lease liability related to its facility lease on the consolidated balance sheet. No cumulative effect adjustment was recognized as the amount was not material. The standard did not materially impact the Company's consolidated statement of operations or cash flows. See Note 5 (Operating Leases) for the financial position impact and additional disclosures.

In June 2018, the FASB issued ASU No. 2018-07, Compensation—Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting that largely aligns the accounting for share-based payment awards issued to employees and nonemployees. Under ASU No. 2018-07, most of the guidance on such payments to nonemployees would be aligned with the requirements for share-based payments granted to employees. ASU 2018-07 is effective for all entities for annual reporting periods beginning after December 15, 2018, including interim reporting periods within each annual reporting period, with early adoption permitted. The Company adopted this standard as of January 1, 2019. The adoption of this standard did not have a material impact on the Company's consolidated financial statements.

In June 2016, the FASB issued ASU No. 2016-13, Financial Instruments — Credit Losses (Topic 326), Measurement of Credit Losses on Financial Instruments. The standard amends the impairment model by requiring entities to use a forward-looking approach based on expected losses to estimate credit losses for most financial assets and certain other instruments that aren't measured at fair value through net income. For available-for-sale debt securities, entities will be required to recognize an allowance for credit losses rather than a reduction in carrying value of the asset. Entities will no longer be permitted to consider the length of time that fair value has been less than amortized cost when evaluating when credit losses should be recognized. The Company adopted the new standard on January 1, 2020. 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:

	Estimated Fair Value	Gross Unrealized Gains	Gross Unrealized Losses	Amortized Cost
At December 31, 2019:				
U.S. Treasuries - Cash equivalents	$59,932,200	$ 2,042	$ —	$ 59,930,158
U.S. Treasuries - ST	5,007,050	7,463	—	4,999,587
Total	$64,939,250	$ 9,505	$ —	$ 64,929,745
At December 31, 2018:				
U.S. Treasuries - ST	$10,380,864	$ —	$ (1,835)	$ 10,382,699
U.S. Treasuries - LT	5,008,243	—	(18,413)	5,026,656
Total	$15,389,107	$ —	$ (20,248)	$ 15,409,355

There were no realized gains or losses from available-for-sale securities for the years ended December 31, 2019 or 2018.

The Company did not hold any securities in an unrealized loss position for more than 12 months as of December 31, 2019.

3. Investments (continued).

The estimated fair values of available-for-sale securities at December 31, 2019, by contractual maturity, are summarized as follows:

	2019
Due in one year or less	$ 64,939,250

4. Prepaid Expenses and Other Current Assets.

Prepaid expenses and other current assets consist of the following as of December 31:

	2019	2018
Prepaid manufacturing costs	$1,526,013	$ —
Prepaid insurance	1,263,129	800,261
Prepaid subscriptions fees	501,251	170,552
Prepaid research fees	481,057	358,209
Prepaid commercialization expenses	62,959	17,030
Other	516,665	303,729
Total prepaid expenses and other current assets	$4,351,074	$1,649,781

5. Operating Leases.

The Company has operating lease agreements for its corporate office. The leases include options to extend the leases for up to 1 year and options to terminate the lease within 1 year. There are no obligations under finance leases.

The components of lease expense were as follows:

	For the Year Ended December 31, 2019
Operating lease cost	$ 296,316

Supplemental cash flow information related to leases was as follows:

	December 31, 2019
Cash paid for amounts included in the measurement of lease liabilities:	
Operating cash flows	$ 329,725
Right-of-use assets obtained in exchange for lease obligations:	
Operating leases	$ 21,220

5. Operating Leases (continued).

Supplemental balance sheet information related to leases was as follows:

	December 31, 2019
Operating lease right-of-use assets	$ 793,252
Other current liabilities	$ 300,518
Operating lease liabilities, net of current portion	647,532
Total operating lease liabilities	$ 948,050
Weighted average remaining lease term	2.9 years
Weighted average discount rate	4.81 %

Remaining payments of lease liabilities as of December 31, 2019 were as follows:

2020	$ 339,605
2021	349,788
2022	329,662
Total lease payments	1,019,055
Less imputed interest	(71,005)
Total	$ 948,050

Rent expense was $242,155 for the year ended December 31, 2018.

6. Accrued Expenses and Other Liabilities.

Accrued expenses and other liabilities consist of the following as of December 31:

	2019	2018
Accrued preclinical and clinical trial expenses	$ 1,183,513	$ 821,633
Accrued professional fees	1,241,526	1,311,061
Accrued compensation and benefits	3,064,645	1,941,449
Accrued license fees	8,751,991	3,000,000
Accrued purchases	1,313,310	—
Accrued contributions	1,535,000	—
Operating lease liability	300,518	—
Accrued variable consideration	884,764	—
Deferred rent and lease incentive	—	33,408
Income tax payable	1,533,696	—
Other	172,332	66,436
Current accrued expenses and other liabilities	19,981,295	7,173,987
Lease liability – non-current	647,532	—
Deferred rent and lease incentive—non-current	—	154,799
Non-current accrued expenses and other liabilities	647,532	154,799
Total accrued expenses and other liabilities	$ 20,628,827	$ 7,328,786

7. Collaborative Arrangement.

In December 2018, the Company entered into a collaboration and license agreement (Collaboration) with Endo Ventures Limited (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.

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 collaborative agreement provides for a $2.0 million milestone payment on the commercial launch of the product by Par. As of December 31, 2019 and 2018, no milestone payments have been earned.

Revenues from collaborative arrangement were $0 and $500,000 (for upfront license fees) for the years ended December 31, 2019 and 2018, respectively. Total expenses incurred, net, in connection with the collaboration agreement for the years ended December 31, 2019 and December 31, 2018 were $65,061 and $0, and have been included in research and development expenses in the accompanying consolidated statements of operations.

8. Commitments and Contingencies.

In 2018, the Company became aware that certain patents granted to Northwestern University (which patents have been licensed by Northwestern to a third party) for a new GABA aminotransferase inhibitor were developed from CPP-115, which had previously been licensed to the Company by Northwestern. As a result, on October 26, 2018, the Company terminated the license agreement for CPP-115 and commenced an arbitration proceeding against Northwestern seeking damages for alleged breaches of the license agreement. Shortly thereafter, Northwestern filed counterclaims against the Company in the arbitration action seeking damages for alleged breaches by the Company of the license agreement. On May 21, 2019, the Company entered into a settlement agreement with Northwestern that resolved all pending disputes between the parties with no admission of liability by either party, released all claims of liability or wrongdoing between the Company and Northwestern, and dismissed the pending arbitration. Under the settlement agreement, the Company received a $100,000 payment on May 21, 2019, which is reported as income in other income, net in the consolidated statement of operations. The Company is also entitled to receive certain contingent compensation that will be reported when and if received.

In May 2019, the FDA approved an NDA for Jacobus Pharmaceuticals for Ruzurgi®, their version of amifampridine (3,4-DAP), for the treatment of pediatric LEMS patients (ages 6 to under 17). The Company believes that Jacobus is offering Ruzurgi® at a lower price than the Company is offering Firdapse®. In addition, while the NDA for Ruzurgi® only covers pediatric patients, the Company believes Ruzurgi® is being prescribed off label to adult LEMS patients. If Jacobus is able to successfully sell Ruzurgi® off-label to adult LEMS patients, it could have a material adverse effect on the Company's business, financial condition and results of operations.

The Company believes that the FDA's approval of Ruzurgi® violated its 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. The Company's complaint, which was filed in the federal district court for the Southern District of Florida, alleges 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 its 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®. Jacobus has intervened in the case. Each party has filed a cross motion for summary judgement. There can be no assurance as to the outcome of this lawsuit.

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.

9. Agreements.

a. LICENSE AGREEMENT WITH BIOMARIN (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.

On May 29, 2019, the Company entered into an amendment to its 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

9. Agreements (continued).

pay royalties 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.

Subsequent to year-end, during 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, most of 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.

10. Income Taxes.

Due to the Company's historical operating losses and the inability to recognize any income tax benefit, there was no provision for income taxes in the preceding period presented in these financial statements. Commencing in 2019, the Company has recorded income taxes on their pretax income using an effective tax rate.

The income tax expense for the years ended December 31, 2019 and 2018 consists of:

	2019	2018
Current	$ 1,533,696	$ —
Deferred	—	—
	$ 1,533,696	$ —

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:

	2019	2018
Statutory rate	21.0%	21.0%
State tax	6.5%	4.2%
Valuation allowance	(20.9)%	(25.9)%
Federal rate change	0.0%	0.0%
Tax credit	(2.5)%	1.4%
Other	0.5%	(0.7)%
	4.6%	0.0%

10. 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 as of December 31, 2019 and 2018 are as follows:

	2019	2018
Net operating loss	$ 10,645,128	$ 19,867,591
Start-up costs	12,894,926	13,861,147
Tax credits	14,320,860	12,625,275
Deferred compensation	3,183,767	1,919,434
Inventory	229,050	—
Other	355,023	109,779
Gross deferred tax asset	41,628,754	48,383,226
Valuation allowance	(41,628,754)	(48,383,226)
Net deferred tax assets	$ —	$ —

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. As of December 31, 2019, and December 31, 2018, based on the Company's history of operating losses, the Company has concluded that it is more likely than not that the benefit of its deferred tax assets will not be realized. Accordingly, the Company has provided a full valuation allowance for deferred tax assets including NOL and tax credit carryover as of December 31, 2019 and December 31, 2018. The valuation allowance decreased by $6.8 million and increased by $9.2 million during 2019 and 2018, respectively.

At December 31, 2019 and 2018, respectively, the Company had net operating loss carryforwards and other credits of approximately $41.5 million and $79.0 million available to reduce future taxable income, if any. The net operating loss carryforwards will expire at various dates beginning in 2024 and ending in 2037, the amount of net operating loss generated in 2018 will have an infinite life, but will be limited to utilization per year of 80% of taxable income.

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 tax credit carryforwards will expire at various dates beginning in 2032 and ending in 2040.

Utilization of certain of the Company's NOL and tax credit carryforwards in the United States is subject to annual limitation due to the ownership change limitations provided by Sections 382 and 383 of the Internal Revenue Code and similar state provisions. Such an annual limitation may result in the expiration of certain NOLs and tax credits before future utilization.

The Company is currently conducting a study of the availability for use of its net operating loss carryforwards and other credits under Section 382 of the Internal Revenue Code. The results of this study could impact the amounts of net operating losses and other credits that the Company has available for use in future periods and the timing of their use.

No interest or penalties were accrued through December 31, 2019. The Company's policy is to recognize any related interest or penalties in income tax expense. The Company is not subject to U.S. federal, state and local tax examinations by tax authorities for any years before 2016. However, authorities can examine net operating loss carryforwards incurred prior to 2016 to the extent utilized in subsequent periods with open statutes. The Company is not currently under income tax examinations by any tax authorities.

11. Stockholders' Equity.

Preferred Stock

The Company has 5,000,000 shares of authorized preferred stock, $0.001 par value per share, at December 31, 2019 and 2018. No shares of preferred stock were outstanding at December 31, 2019 and 2018.

Common Stock

The Company has 150,000,000 shares of authorized common stock, par value $0.001 per share. At December 31, 2019 and 2018, 103,397,033 and 102,739,257 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.

2016 Shelf Registration Statement

On December 23, 2016, the Company filed a shelf Registration Statement on Form S-3 (the 2016 Shelf Registration Statement) with the SEC to sell up to approximately $33.8 million of common stock. The 2016 Shelf Registration Statement (file No. 333-215315) was declared effective by the SEC on January 9, 2017. No sales have been conducted to date under the 2016 Shelf Registration Statement.

On January 9, 2020, the 2016 Shelf Registration Statement expired.

2017 Shelf Registration Statement

On July 12, 2017, the Company filed a universal shelf Registration Statement on Form S-3 (the 2017 Shelf Registration Statement) with the SEC to sell up to $150 million of common stock, preferred stock, warrants to purchase common stock, or debt securities (including debt securities that may be convertible or exchangeable for common stock or other securities), which securities may be offered separately or together in units or multiple series. The 2017 Shelf Registration Statement (file No. 333-219259) was declared effective by the SEC on July 26, 2017.

On November 28, 2017, the Company filed a prospectus supplement and offered for sale 16,428,572 shares of its common stock at a price of $3.50 per share in an underwritten public offering under the 2017 Shelf Registration. The Company received gross proceeds in the public offering of approximately $57.5 million before underwriting commission and incurred expenses of approximately $3.7 million.

At December 31, 2019, there is approximately $92.5 million available for future sale under the 2017 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 entitles 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 trade automatically with the common stock and will not be exercisable until a person or group has become an "acquiring person" by acquiring 17.5% or more of the Company's outstanding common stock, or a person or group commences, or publicly announces a tender offer that will 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 has become an acquiring person, each Right will 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

11. Stockholders' Equity (continued).

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 have 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 has been extended from September 20, 2016 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 extending the outside expiration date of the rights plan to September 20, 2022.

12. Stock Compensation.

For the years ended December 31, 2019 and 2018, the Company recorded stock-based compensation expense as follows:

	2019	2018
Research and development	$1,137,596	$1,079,230
Selling, general and administrative	2,687,219	2,471,414
Total stock-based compensation	$3,824,815	$3,550,644

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, 2019, no shares remain available for future issuance under the 2014 Plan. Under the 2018 Plan, 7,500,000 shares were reserved for issuance and as of December 31, 2019, 1,596,271 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 5 years of continuous service and have contractual terms from 5 to 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, 2019 and 2018, options to purchase 654,332 and 186,665 shares of the Company's common stock were exercised with gross proceeds to the Company of $1,116,242 and $297,563, respectively. Further, 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, respectively. During the year ended December 31, 2018, no options to purchase shares of the Company's common stock were exercised on a "cashless" basis.

12. Stock Compensation (continued).

During the years ended December 31, 2019 and 2018 the Company recorded non-cash stock-based compensation expense related to stock options totaling $3,780,086 and $3,535,647, respectively.

During the years ended December 31, 2019 and 2018, the Company granted seven-year options to purchase an aggregate of 2,183,500 and 5,882,500 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, 2019 is summarized as follows:

	Number of Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
Outstanding at beginning of year	10,532,500	$ 2.54		
Granted	2,183,500	4.54		
Exercised or released	(660,998)	1.72		
Forfeited or cancelled	(511,668)	2.73		
Expired	(65,000)	3.90		
Outstanding at end of year	11,478,334	$ 2.95	4.89	$ 11,590,899
Exercisable at end of year	5,844,819	$ 2.41	3.87	$ 8,084,671

Other information pertaining to stock option activity during the years ended December 31, 2019 and 2018 was as follows:

	2019	2018
Weighted–average fair value of granted stock options	$ 2.69	$ 1.93
Total fair value of vested stock options	$3,864,995	$2,193,294
Total intrinsic value of exercised stock options	$1,899,862	$ 274,864

The following table summarizes information about the Company's options outstanding at December 31, 2019:

	Options Outstanding			Options Exercisable		
Range of Exercise Prices	Number Outstanding	Weighted Average Remaining Contractual Life (Years)	Weighted Average Exercise Price	Number Exercisable	Weighted Average Remaining Contractual Life (Years)	Weighted Average Exercise Price
$0.79 to $2.17	2,139,000	3.83	$ 1.05	1,757,332	3.73	$ 1.01
$2.18 to $2.43	2,350,000	5.97	$ 2.25	983,324	5.97	$ 2.24
$2.44 to $3.38	2,345,334	3.07	$ 2.88	1,919,996	2.46	$ 2.86
$3.39 to $4.07	2,592,500	5.17	$ 3.82	1,019,167	5.11	$ 3.85
$4.08 to $6.63	2,051,500	6.54	$ 4.74	165,000	2.48	$ 4.18
	11,478,334	4.89	$ 2.95	5,844,819	3.87	$ 2.41

As of December 31, 2019, there was approximately $9.9 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 2.33 years.

12. Stock Compensation (continued).

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 estimated expected option life is based upon estimated employee exercise patterns and considers whether and the extent to which the options are in-the-money. 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 options 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:

	December 31, 2019	December 31, 2018
Risk free interest rate	1.51% to 2.53%	2.09% to 2.88%
Expected term	4.5 years	0 to 7 years
Expected volatility	75.5%	82%
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 2019 was as follows:

	2019	
	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Nonvested balance at beginning of year	—	$ —
Granted	352,500	4.64
Vested	—	—
Forfeited	—	—
Nonvested balance at end of year	352,500	$ 4.64

No restricted stock units were granted or outstanding during 2018.

During the year ended December 31, 2019, the Company recorded non-cash stock-based compensation expense related to restricted stock units totaling $44,729. No stock-based compensation related to restricted stocks was recorded during 2018.

Common Stock

No shares of common stock were granted during the year ended December 31, 2019. During the year ended December 31, 2018, the Company granted 3,094 net shares of common stock to an employee as compensation. The

12. Stock Compensation (continued).

Company recorded stock-based compensation related to common stock issued to an employee totaling approximately $15,000, during the year ended December 31, 2018.

13. 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, 2019 and 2018, the Company's matching contributions were approximately $268,000 and $123,000, respectively.

14. Quarterly Financial Information (unaudited).

The following table presents unaudited supplemental quarterly financial information for the years ended December 31, 2019 and 2018:

	Quarter Ended			
	March 31, 2019	June 30, 2019	September 30, 2019	December 31, 2019
Revenues	$ 12,448,438	$ 28,837,900	$ 30,897,444	$ 30,122,555
Income (loss) from operations	$ (987,769)	$ 10,959,189	$ 13,845,152	$ 8,006,687
Net income (loss)	$ (644,503)	$ 10,959,948	$ 13,630,179	$ 7,929,713
Net income (loss) per share – basic	$ (0.01)	$ 0.11	$ 0.13	$ 0.08
Net income (loss) per share – diluted	$ (0.01)	$ 0.10	$ 0.13	$ 0.07

	Quarter Ended			
	March 31, 2018	June 30, 2018	September 30, 2018	December 31, 2018
Revenues	$ —	$ —	$ —	$ 500,000
Loss from operations	$ (5,933,440)	$ (6,335,855)	$ (8,182,603)	$ (14,843,267)
Net loss	$ (5,699,892)	$ (5,965,140)	$ (7,838,873)	$ (14,499,609)
Net loss per share – basic and diluted	$ (0.06)	$ (0.06)	$ (0.08)	$ (0.14)

Quarterly basic and diluted net income (loss) per common share were computed independently for each quarter and do not necessarily total to the full year basic and diluted net income (loss) per common share.

Corporate Directory

BOARD OF DIRECTORS

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

Philip H. Coelho
Chair, Nominating and Corporate Governance Committee
Chief Technology Officer
ThermoGenesis Corp.

Richard J. Daly
Chief Operating Officer
BeyondSpring Pharma

Donald A. Denkhaus
Chair, Audit Committee
Chairman and Chief Financial Officer
The Kitchen, LLC

Charles B. O'Keeffe
Lead Independent Director
Professor, Pharmacology, Epidemiology and Community Health
Virginia Commonwealth University

David S. Tierney, MD
Chair, Compensation Committee
Chief Executive Officer
Pharma2B

EXECUTIVE OFFICERS

Patrick J. McEnany
Chairman of the Board, President, Chief Executive Officer and Co-Founder

Steven R. Miller, PhD
Chief Operating Officer and Chief Scientific Officer

Alicia Grande, CPA, CMA
Vice President, Treasurer and Chief Financial Officer

Gary Ingenito, M.D., Ph.D.
Chief Medical and Regulatory Officer

Jeffrey Del Carmen
Chief Commercial Officer

Brian Elsbernd, J.D.
Chief Compliance Officer and Chief Legal Officer

INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

Grant Thornton LLP
Miami, Florida

CORPORATE COUNSEL

Akerman LLP
Miami, Florida

ANNUAL MEETING

The annual meeting of stockholders will be held on Thursday, August 20, 2020 at 9:00 a.m., local time, at the Hyatt Regency Coral Gables, located at:

50 Alhambra Plaza
Coral Gables, Florida 33134

INVESTOR INFORMATION

Recent press releases and other Catalyst Pharmaceuticals information are available without charge on Catalyst's website at www.catalystpharma.com or by written request to:

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STOCK LISTING

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

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