CATALYST PHARMACEUTICALS, INC.
Form 424B5
November 28, 2017
Table of Contents

PROSPECTUS SUPPLEMENT
(To Prospectus dated July 26, 2017)

Filed Pursuant to Rule 424(b)(5)
Registration No. 333-219259

14,285,715 Shares

Common Stock

$3.50 Per Share

Catalyst Pharmaceuticals, Inc. is offering 14,285,715 shares of its common stock.

The last reported sale price of our common stock on November 27, 2017 was $3.61.

Trading symbol: Nasdaq Capital Market - CPRX

This investment involves risks. See Risk Factors on page S-7 of this prospectus supplement and on page 7 of the accompanying prospectus.

	Per Share	Total
Public Offering Price	$ 3.50	$ 50,000,002
Underwriting discount [(1)]	$ 0.21	$ 3,000,000
Proceeds, before expenses, to us	$ 3.29	$ 47,000,002

(1) We have also agreed to reimburse the underwriters for certain of their expenses. See Underwriting on page S-35 of this prospectus supplement for more information about these arrangements.

We have granted the underwriters an option, for a period of 30 days from the date of this prospectus supplement, to purchase up to an additional 2,142,857 shares of common stock to cover over-allotments, if any. If the underwriters exercise the option in full, the total underwriting discount payable by us will be $3,450,000 and the proceeds to us, before expenses, will be $54,050,002.

Neither the Securities and Exchange Commission (SEC) nor any state securities commission or other regulatory body has approved or disapproved these securities, or determined if this prospectus supplement or the accompanying prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

Piper Jaffray **SunTrust Robinson Humphrey**

H.C. Wainwright & Co. **Roth Capital Partners**

The date of this prospectus supplement is November 28, 2017.

Table of Contents

TABLE OF CONTENTS

This document is in two parts. The first part is this prospectus supplement, which describes the terms of the offering of the securities offered hereby and also adds to and updates the information contained in the accompanying prospectus and the documents incorporated by reference into this prospectus supplement and the accompanying prospectus. The second part is the accompanying prospectus, which provides more general information. To the extent that there is any conflict between the information contained in this prospectus supplement, on the one hand, and the information contained in the accompanying prospectus or any document incorporated by reference herein or therein, on the other hand, you should rely on the information in this prospectus supplement.

You should rely only on the information contained in this prospectus supplement, contained in the accompanying prospectus or incorporated herein or therein by reference. We have not authorized anyone to

provide you with information that is different. We are offering to sell, and seeking offers to buy, the securities offered hereby only in jurisdictions where offers and sales are permitted. The information contained, or incorporated by reference, in this prospectus supplement and contained, or incorporated by reference, in the accompanying prospectus is accurate only as of the respective dates thereof, regardless of the time of delivery of this prospectus supplement and the accompanying prospectus, or of any sale of our common stock. It is important for you to read and consider all information contained in this prospectus supplement and the accompanying prospectus, including the documents we have referred you to in the section entitled Where You Can Find Additional Information below.

Table of Contents

SUMMARY

This summary highlights information contained elsewhere in this prospectus supplement; it does not contain all of the information you should consider before investing. You should carefully read the entire prospectus supplement before making an investment decision.

This prospectus supplement includes trademarks, service marks or trade names owned by us or other companies. All trademarks, service marks or trade names included in this prospectus supplement are the property of their respective owners.

Throughout this prospectus supplement, the terms we , us , our and company refer to Catalyst Pharmaceuticals, Inc.

Overview

We are a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare, debilitating, chronic neuromuscular and neurological diseases. We currently have three drug candidates in development.

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, from BioMarin Pharmaceutical Inc. (BioMarin). In August 2013, we were granted breakthrough therapy designation by the U.S. Food & Drug Administration (FDA) for Firdapse® for the treatment of patients with Lambert-Eaton Myasthenic Syndrome, or LEMS, a rare and sometimes fatal autoimmune disease characterized by muscle weakness. Further, the FDA has previously granted Orphan Drug Designation for Firdapse® for the treatment of patients with LEMS, Congenital Myasthenic Syndromes, or CMS, and Myasthenia Gravis (MG).

The chemical entity, amifampridine (3,4-diaminopyridine, or 3,4-DAP), has never been approved by the FDA for any indication. Because amifampridine phosphate (Firdapse®) has been granted three separate Orphan Drug designations for the treatment of LEMS, CMS and MG by the FDA, the product is also eligible to receive seven years of marketing exclusivity upon approval of amifampridine for any or all of these indications. Further, if we are the first pharmaceutical company to obtain approval for marketing an amifampridine product, of which there can be no assurance, we will be eligible to receive five years of marketing exclusivity with respect to the use of this product for any indication, running concurrently with the seven years of orphan marketing exclusivity described above (if both exclusivities are granted).

We previously sponsored a multi-center, randomized, placebo-controlled Phase 3 trial evaluating Firdapse® for the treatment of LEMS. This Phase 3 trial, which involved 38 subjects, was designed as a randomized withdrawal trial in which all patients were treated with Firdapse® during a 7 to 91-day run-in-period followed by treatment with either Firdapse® or placebo over a two-week randomization period. The co-primary endpoints for this Phase 3 trial were the comparison of changes in patients randomized to continue Firdapse® versus those who transitioned to placebo that occurred in both the Quantitative Myasthenia Gravis Score (QMG), which measures muscle strength, and subject global impression score (SGI), on which the subjects rate their global impression of the effects of a study treatment during the two-week randomization period. In September 2014, we reported positive top-line results from this Phase 3 trial.

During 2014, we established an expanded access program (EAP) to make Firdapse® available to any patients diagnosed with LEMS, CMS, or Downbeat Nystagmus in the United States, who meet the inclusion and exclusion criteria, with Firdapse® being provided to patients for free until sometime after new drug application (NDA) approval, should we receive such approval (of which there can be no assurance). We continue to inform neuromuscular physicians on the availability of the Firdapse® EAP and also to work with various rare disease advocacy organizations to inform patients and other physicians about the program.

Table of Contents

On December 17, 2015, we announced completion of the submission of an NDA for Firdapse® for the treatment of LEMS and CMS. However, on February 17, 2016, we announced that we had received a refusal-to-file (RTF) letter from the FDA regarding our NDA submission. In early April 2016, we met with the FDA to obtain greater clarity regarding what will be required by the FDA to accept the Firdapse® NDA for filing. Following the receipt of the formal minutes of that meeting, on April 26, 2016, we issued a press release reporting that the FDA has advised us that in addition to the results of our previously submitted multi-center, randomized, placebo-controlled Phase 3 trial, we will need to submit positive results from a second adequate and well-controlled study in patients with LEMS. Additionally, there was a requirement for us to perform several abuse liability studies for Firdapse®.

In October 2016, we announced that we had reached an agreement with the FDA under a Special Protocol Assessment (SPA) for the protocol design, clinical endpoints, and statistical analysis approach to be taken in our second Phase 3 study evaluating Firdapse® for the symptomatic treatment of LEMS. A SPA is a process by which sponsors ask the FDA to evaluate the protocol of a proposed clinical trial to determine whether it adequately addresses scientific and regulatory requirements for the purpose identified by the sponsor. A SPA agreement indicates FDA concurrence with the adequacy and acceptability of specific critical elements of protocol design, endpoints and analysis. Additionally, it provides a binding agreement with FDA s review division that critical design elements of a pivotal trial adequately address the scientific and regulatory objectives in support of a regulatory submission for drug approval. However, even if a clinical trial is conducted pursuant to a SPA, that does not mean that the NDA will meet the standard for approval. Moreover, the FDA may rescind a SPA agreement when the division director determines that a substantial scientific issue essential to determining the safety or efficacy of the product has been identified after the trial has begun.

We recently completed our second Phase 3 trial evaluating Firdapse® for the treatment of LEMS (designated as LMS-003) at sites in Miami, Florida and Los Angeles, California. This double-blind, placebo-controlled withdrawal trial had the same co-primary endpoints as our first Phase 3 trial evaluating Firdapse® for the treatment of LEMS. Further, the FDA allowed us to enroll patients from our expanded access program as study subjects in this second trial. Details of the Phase 3 clinical trial are available on www.clinicaltrials.gov (NCT02970162). Enrollment in this trial, which included 26 subjects, was completed in October 2017.

On November 27, 2017, we reported positive top-line results from this trial. This trial had two prospectively defined co-primary endpoints. The first of these, quantitative myasthenia gravis score (QMG), achieved a statistically significant p-value of 0.0004, and the second, subject global impression (SGI), achieved a statistically significant p-value of 0.0003. More importantly, a clinically significant difference of 6.4 points was observed between the Firdapse and placebo groups for the QMG endpoint. Firdapse was well tolerated and showed a similar safety profile to that seen in earlier studies. All p-values reported are based on the entire intent to treat (ITT) population of patients that enrolled in this trial.

The prospectively defined secondary endpoint for the physician s clinical global impression of improvement (CGI-I) achieved statistical significance (p-value 0.0020). Further, the exploratory endpoints had the following results: (i) the triple timed up and go (3TUG) endpoint achieved statistical significance (p-value 0.0112), (ii) the evaluation of the QMG-Limb domains endpoint achieved statistical significance (p-value 0.0010), and (iii) the most bothersome symptom (MBS) endpoint was not statistically significant, but showed a positive trend (p-value 0.0572).

We were also required to conduct three pre-clinical abuse liability studies under the FDA guidance for Assessment of Abuse Potential of Drugs that was finalized in January 2017 (Self-Administration, Physical Dependence and Drug Discrimination). All three studies have now been completed, and top-line results indicate that amifampridine phosphate does not exhibit abuse potential in these assessment models.

Table of Contents

Based on the positive top-line results of the LMS-003 trial, we expect to resubmit an NDA for Firdapse® during the first quarter of 2018. There can be no assurance whether this trial, along with the results of our first Phase 3 trial, will be sufficient for the FDA to accept for filing any NDA that we might resubmit in the future for Firdapse®, or whether Firdapse® will ever be approved for commercialization.

Our original NDA submission for Firdapse® included data and information (including data from a currently ongoing investigator treatment IND) providing evidence supporting the benefits of Firdapse® for treating certain types of CMS, and requested that CMS be included in our initial label for Firdapse®. To provide additional support for our submission of an NDA for Firdapse® for the treatment of CMS, in October 2015 we initiated a small blinded clinical trial at four academic centers of up to 10 subjects in the pediatric CMS population, ages 2 to 17. However, after considering comments from the FDA, we determined to enroll both adult and pediatric subjects with CMS in this trial and to expand the number of subjects to be evaluated in the trial to an aggregate of approximately 20 subjects. We are currently conducting this study at six sites around the United States, and we are currently working to add several additional sites outside the United States. Details of this trial are available on www.clinicaltrials.gov (NCT02562066).

Based on currently available information, we expect to report top line results from this trial in the first half of 2018 and, if the results of the study are successful, we hope to add the CMS indication to our labeling for Firdapse®. We also intend to include in our initial filing for LEMS those limited types of CMS that are generally considered mechanistically similar to LEMS, subject to confirming at any pre-NDA meeting that we may be granted that inclusion will not slow down the FDA s review of a resubmitted NDA for Firdapse® for LEMS.

There can be no assurance that any trial we perform for Firdapse® for the treatment of CMS will be successful or whether any NDA or NDA supplement that we may submit for Firdapse® for the treatment of CMS will be filed by the FDA for review and approved.

In February 2016, we announced the initiation of an investigator-sponsored, randomized, double-blind, placebo-controlled, crossover Phase 2/3 clinical trial evaluating the safety, tolerability and potential efficacy of Firdapse® as a symptomatic treatment for patients with MuSK antibody positive myasthenia gravis (MuSK-MG).MuSK-MG is a particularly severe form of myasthenia gravis that affects about 3,000 to 4,800 patients in the U.S., for which there are no approved effective therapies (and therefore it is an unmet medical need). 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.

On August 30, 2017, we announced that we had reached an agreement with the FDA on a SPA for the protocol design, clinical endpoints, and statistical analysis approach to be taken in our proposed Phase 3 registration trial evaluating the safety and efficacy of amifampridine phosphate treatment in patients with MuSK-MG. The protocol that the FDA has reviewed is for a multi-site, international (U.S. and Italy), double-blind, placebo-controlled, clinical trial that is targeted to enroll approximately 60 subjects diagnosed with MuSK-MG. The trial will employ a primary endpoint of Myasthenia Gravis Activities of Daily Living (MG-ADL) and a secondary endpoint of Quantitative Myasthenia Gravis Score (QMG). At the FDA s request, the trial will also enroll up to 10 generalized myasthenia gravis patients who will be assessed with the same clinical endpoints, but achieving statistical significance in this subgroup of patients is not required and only summary statistics will be provided. Catalyst anticipates that enrollment

in this trial will commence in the first quarter of 2018, and that it will take about 12 months to complete the enrollment for the trial. Details of this trial are available on www.clinicaltrials.gov (NCT03304054).

Table of Contents

On November 21, 2017, we announced the initiation of a company-sponsored, adequate and well-controlled, proof-of-concept clinical trial evaluating safety, tolerability and efficacy of Firdapse® as a symptomatic treatment for patients with Spinal Muscular Atrophy (SMA) Type 3. The study will be conducted by a team of researchers led by Lorenzo Maggi, MD, and Giovanni Baranello, MD, of the Fondazione Istituto Neurologico Carlo Besta in Milan, Italy, a major referral center for SMA patients. 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 Catalyst anticipates reporting top-line results from the study in the first half of 2019.

There can be no assurance that any trial that we initiate to evaluate Firdapse® for MuSK-MG or SMA will be successful, or whether we have sufficient resources available to fund such trials. Further, there can also be no assurance that the FDA will ever approve Firdapse® for these indications.

Finally, we may seek to evaluate Firdapse® for the treatment of other treatment-refractory types of MG or other rare, similar neuromuscular diseases, although we have not yet begun to develop clinical programs for these indications and all such programs are subject to the availability of funding. There can be no assurance that Firdapse® will be an effective treatment for other treatment-refractory types of MG or for any other rare, similar neuromuscular diseases.

Prior to the receipt of the RTF letter, we had actively been taking steps to prepare for the commercialization of Firdapse® in the United States. However, in light of the receipt of the RTF letter, in the first quarter of 2016 we put most of our commercialization activities on hold in order to conserve cash. During the fourth quarter of 2017, we restarted the development of our commercialization plans for Firdapse®. We are also continuing to work with several rare disease advocacy organizations to help increase awareness of LEMS, CMS and MuSK-MG and to provide awareness and outreach support for the physicians who treat these rare diseases and the patients they treat.

CPP-115

We are developing CPP-115, a GABA aminotransferase inhibitor that, based on our preclinical studies to date, we believe is a more potent form of vigabatrin, and may have fewer side effects (e.g., visual field defects) than those associated with vigabatrin. We are hoping to develop CPP-115 for the treatment of refractory infantile spasms and possibly for the treatment of adult refractory patients with Tourette s Disorder. CPP-115 has been granted Orphan Drug Designation by the FDA for the treatment of infantile spasms and Orphan Medicinal Product Designation in the European Union, or E.U., for West syndrome (a form of infantile spasms).

We are currently refining our development plans for this product. Once the refinement of our development plans is completed, and subject to the then availability of funding, we plan to take the steps to complete the work required to make our drug candidate Phase 2 ready. We are also working with one or more potential investigators who have expressed an interest in evaluating our product for particular indications (particularly infantile spasms).

We are also continuing our efforts to seek a partner to work with us in furthering the development of CPP-115. However, no agreements have been entered into to date.

There can be no assurance that we will ever successfully commercialize CPP-115.

Table of Contents

Generic Sabril®

During September 2015, we announced the initiation of a project to develop generic versions of Sabril® (vigabatrin) in both dosage forms: tablets and powder sachets. Sabril® is marketed by Lundbeck Inc. in the United States in both dosage forms for the treatment of infantile spasms and complex partial seizures. There can be no assurance that we will be successful in these efforts or that any abbreviated new drug applications (ANDAs) that we submit for vigabatrin will be accepted for review or approved.

We are also continuing our efforts to seek a partner to work with us in furthering the development of generic Sabril®. However, no agreements have been entered into to date.

There can be no assurance that we will ever successfully commercialize a generic version of Sabril®.

Company Information

Our principal executive offices are located at 355 Alhambra Circle, Suite 1250, Coral Gables, Florida 33134, and our telephone number at that address is (305) 420-3200. Our website is located at www.catalystpharma.com. We do not incorporate by reference into this prospectus supplement or the accompanying prospectus the information on, or accessible through, our website, and you should not consider it as part of this prospectus supplement or the accompanying prospectus.

Table of Contents

THE OFFERING

Common Stock Offered	14,285,715 shares
Underwriter s Overallotment Option	We have granted the underwriters an option to purchase up to an additional 2,142,857 shares to cover over-allotments, if any
Common Stock to be Outstanding after this Offering	100,331,641 shares
Use of Proceeds	We intend to use the net proceeds from the sale of the securities: (i) to fund clinical studies of Firdapse® for the treatment of MuSK-MG and SMA, (ii) to fund our pre-commercialization activities for Firdapse®, and (iii) for general corporate purposes. See Use of Proceeds on page S-31 for more information.
Risk Factors	See Risk Factors beginning on page S-7 of this prospectus supplement and on page 7 of the accompanying prospectus for a discussion of factors you should consider carefully before deciding to invest in our common stock.
NASDAQ Capital Market Symbol	CPRX

The number of shares of our common stock to be outstanding after this offering as shown above is based on 86,045,926 shares outstanding as of November 27, 2017 and excludes:

- 5,285,000 shares of our common stock subject to outstanding options under our 2006 Stock Incentive Plan and our 2014 Stock Incentive Plan, having a weighted average exercise price of $1.93 per share; and
- 3,628,334 shares of our common stock that have been reserved for issuance under our 2014 Stock Incentive Plan.

Unless otherwise indicated, all information in this prospectus supplement assumes no exercise by the underwriters of their overallotment option.

Table of Contents

RISK FACTORS

Before you make a decision to invest in our common stock, you should consider carefully the risks described below, together with other information in this prospectus supplement, the accompanying prospectus and the information incorporated by reference herein and therein. If any of the following events actually occur, our business, operating results, prospects or financial condition could be materially and adversely affected. This could cause the trading price of our common stock to decline and you may lose all or part of your investment. The risks described below are not the only ones that we face. Additional risks not presently known to us or that we currently deem immaterial may also significantly impair our business operations and could result in a complete loss of your investment.

Risks Related to our Business

We are a development stage company. Our limited operating history makes it difficult to evaluate our future performance.

We are a development stage company and, as such, we have a limited operating history upon which you can evaluate our current business and our prospects. The likelihood of our future success must be viewed in light of the problems, expenses, difficulties, delays and complications often encountered in the operation of a business without revenues, especially in the pharmaceutical industry, where failures of companies are common. We are subject to the risks inherent in the ownership and operation of a development stage company, including availability of capital, regulatory setbacks and delays, fluctuations in expenses, competition and government regulation. If we fail to address these risks and uncertainties our business, results of operations, financial condition and prospects would be adversely affected.

We have no products currently available and we have never had any products available for commercial sale.

We have had no revenues from product sales to date, currently have no products available for commercial sale, and have never had any products available for commercial sale. We expect to incur losses at least until we are in a position to commercialize Firdapse®, which may never occur. Our net loss was $18.1 million and $20.2 million for the years ended December 31, 2016 and December 31, 2015, respectively, and $13.0 million and $13.9 million for the nine months ended September 30, 2017 and September 30, 2016, respectively. We may never obtain approval of an NDA for any of our drug candidates and we may never achieve profitability.

Our business will require additional capital.

Our business will require additional capital to meet our product development objectives. Based on currently available information, we estimate that we have sufficient working capital to support our operations through at least the next 12 months without the proceeds of this offering. The expectations described above are based on current information available to us. If the cost of our ongoing activities are greater than we expect, our assumptions may not prove to be accurate. There can be no assurance as to the exact amount of the funding we will require or as to whether any such required funding will be available to us when it is required.

We plan to 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 grants will be available, and, if available, that we will qualify to receive any such grants. We may also seek to raise additional capital to fund additional product development efforts, even if we have sufficient funds for our planned operations.

Table of Contents

Any sale by us of additional equity or debt securities convertible into additional equity could result in dilution to our stockholders. There can be no assurance that any required additional funding will be available to us at all or available on terms acceptable to us. 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.

If we are not the first to obtain approval for Firdapse® for the treatment of LEMS, we may not be able to bring it to market in the United States.

Another pharmaceutical company, Jacobus Pharmaceutical, has completed its own clinical trial studying their own formulation of amifampridine (3,4-DAP) for the treatment of LEMS. Jacobus Pharmaceutical is a privately held company and there is little public information available about their development plans. While there can be no assurance, we believe that Firdapse® is further along in development and as a result we expect that we will be in a position to obtain the first approval of an NDA for 3,4-DAP. Under the Orphan Drug Act of 1983, the first pharmaceutical product to obtain approval for an orphan designated indication receives the orphan exclusivity under the statute. If Jacobus Pharmaceutical receives approval of an NDA for its formulation of amifampridine for the treatment of LEMS before we are able to receive approval of Firdapse® for the same indication, we would be barred from marketing Firdapse® in the United States during the seven-year orphan exclusivity period, which would have a severe adverse effect on our results of operations. In addition, if Jacobus Pharmaceutical were to receive five-year new chemical entity exclusivity for amifampridine for any indication prior to approval of Firdapse®, we would be barred from marketing Firdapse® for any indication in the United States during this five-year exclusivity period.

The development of CPP-115 is at an early stage.

Our development of CPP-115 is at an early stage, and it is going to be several years before we are in a position to submit an NDA for CPP-115, assuming our future clinical trials of this product are successful. At the present time, there can be no assurance that we will ever submit an NDA for CPP-115 or successfully commercialize CPP-115.

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 our drug candidates. In addition, we compete against biotechnology companies, universities, government agencies, and other research institutions in the development of pharmaceutical products. While we believe that our drug candidates will offer advantages over many of the currently available competing therapies, 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, if we are permitted to commence commercial sales of our drug candidates, we may also compete with respect to manufacturing efficiency and marketing capabilities.

Table of Contents

For example, amifampridine, the active ingredient in Firdapse®, despite not being FDA approved, has been available from compounding pharmacies and from Jacobus Pharmaceutical under compassionate use INDs for many years. Amifampridine from these sources can be expected to be substantially less expensive than Firdapse®. The FDA Pharmacy Compounding Advisory Committee, however, has previously issued a list of drugs that were nominated without adequate clinical support (i.e., FDA s Bulks List 3), and amifampridine was included on that list. However, that does not necessarily prevent pharmacists from compounding amifampridine, and we know of no enforcement action that FDA has taken concerning compounders that compound formulations using substances on List 3. 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. Finally, if FDA approves Firdapse®, the ingredients in the drug may be used by compounding pharmacies pursuant to Section 503A of the Federal Food, Drug, and Cosmetic Act because pharmacies that compound for individually identified patients under Section 503A may compound using components of approved drug products.

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 with an aggregate annual coverage limit of $15,000,000 per claim and $15,000,000 in the aggregate, with a deductible of $10,000 per occurrence. Our insurance may not reimburse us for certain claims or the coverage may not be sufficient to cover claims made against us. We cannot predict all of the possible harms or side effects that may result from the use of our current drug candidates, or any potential future products we may acquire and use in clinical trials or after FDA approval and, therefore, the amount of insurance coverage we currently hold may not be adequate to cover all liabilities we might incur. If we are sued for any injury allegedly caused by our products, our liability could exceed our ability to pay the liability. Whether or not we are ultimately successful in any adverse litigation, such litigation could consume substantial amounts of our financial and managerial resources, all of which could have a material adverse effect on our business, financial condition, results of operations, prospects and stock price.

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 financial statements, which could result in a decrease in the value of our common stock.

Table of Contents

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

We are highly dependent on our officers and employees, on our Board of Directors and on our scientific advisors. 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, and the consulting agreements we have with several of our scientific advisors, we have no employment or retention agreements with our officers, directors or scientific advisors. If we lose the services of any of our existing officers, directors or scientific advisors, or if we were unable to recruit qualified replacements on a timely basis for persons who leave our employ, our efforts to develop our drug candidates might be significantly delayed. We do not carry key-man insurance on any of our personnel.

We have relationships with our scientific advisors and collaborators at academic and other institutions. Such individuals are employed by entities other than us and may have commitments to, or consulting advisory contracts with, such entities that may limit their availability to us. Although each scientific advisor and collaborator has agreed not to perform services for another person or entity that would create an appearance of a conflict of interest, conflicts may arise from the work in which other scientific advisors and/or collaborators are involved.

Risks Related to the Development of Our Drug Candidates

Our drug development efforts may fail.

Development of our pharmaceutical drug candidates is subject to risks of failure. For example:

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

As a result, our drug development activities may not result in any safe, effective and commercially viable products, and we may not be able to commercialize our products successfully. For example, for several years, we evaluated CPP-109 (our formulation of vigabatrin) for the treatment of cocaine addiction. However, CPP-109 failed to meet the primary and two key secondary endpoints in a Phase 2b trial for cocaine addiction, and we are no longer pursuing the evaluation of CPP-109 for addiction. Further, our lead compound, Firdapse®, is for very rare conditions for which there is no FDA-approved treatment. As such, the clinical development plan we pursued after consulting with FDA, including the clinical endpoints, protocol design, and statistical analysis plan, may not allow the FDA to ultimately conclude that our NDA for Firdapse® meets the safety and efficacy standards for approval. For example, in 2015, we submitted an NDA for Firdapse® for the treatment of LEMS and CMS. However, we received a refusal-to-file (RTF) letter from the FDA regarding our NDA submission. FDA advised us that, in addition to the results of our previously submitted multi-center, randomized, placebo-controlled Phase 3 trial, we will need to submit positive results from a second adequate and well-controlled study in patients with LEMS and several abuse liability studies for Firdapse®. 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.

Table of Contents

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

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

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

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

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

We do not currently have the ability to independently conduct pre-clinical studies or clinical studies and trials for our drug candidates, 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 of our drug candidates. Our reliance on third parties for development activities reduces our control over these activities. These third parties may not complete activities on schedule, or may not conduct our pre-clinical studies and our clinical studies and trials in accordance with regulatory requirements or our study design. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may be adversely affected, and our efforts to obtain regulatory approvals for and commercialize our drug candidates may be delayed.

Table of Contents

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 our drug candidates if these requirements are not met.

We will need to develop marketing, distribution and production capabilities or relationships to be successful.

In order to generate sales of any products we may develop, we must either acquire or develop an internal marketing force with technical expertise and with supporting documentation capabilities, or make arrangements with third parties to perform these services for us. The acquisition and development of a marketing and distribution infrastructure requires substantial resources and compete for available resources with our drug development efforts. To the extent that we enter into marketing and distribution arrangements with third parties, our revenues will depend on the efforts of others. If we fail to enter into such agreements, or if we fail to develop our own marketing and distribution channels, we would experience delays in product sales and incur increased costs.

We have no in-house manufacturing capacity and, to the extent we are successful in completing the development of our drug candidates, we will be obligated to rely on contract manufacturers. We cannot be sure that we will successfully manufacture any product we may develop, 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 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 potential 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 drug candidates, it could have a material adverse effect on our ability to commercialize our drug candidates.

Table of Contents

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

We intend to attempt to source our products from more than one supplier. We also intend 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.

If our NDA for Firdapse® is approved, we will need to manufacture our product in larger quantities than we have in the past to launch the product and meet customer requirements. With respect to our other products, to date they have only been manufactured in small quantities for pre-clinical studies and clinical trials, and, in order to conduct large trials and commercialize these products, we will need to manufacture our products in larger quantities than we have in the past.

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.

If we are successful in obtaining approval to commercialize Firdapse® or any of our other drug candidates, we will need to significantly expand our operations, which could put significant strain on our management and our operational and financial resources. We currently have 19 employees and conduct many of our activities through outsourcing arrangements. 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.

Table of Contents

Pressure on drug product third-party payor coverage, reimbursement and pricing may impair our ability to be reimbursed for any of our drug candidates which we commercialize in the future at prices or on terms sufficient to provide a viable financial outcome.

The commercial success of Firdapse® will 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 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 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 candidate profitably. These payors may not view our products, if any, 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, if any, 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.

Table of Contents

The pricing of pharmaceutical products, in general, and specialty drugs, in particular, has been a topic of concern in the U.S. Congress, where hearings on the topic have been held. It has also been a topic raised by President Trump, most recently in a meeting with pharmaceutical industry participants. There can be no assurance as to how this scrutiny on pricing of pharmaceutical products will impact future pricing of orphan drugs or pharmaceutical products generally or our products in particular.

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

There is a vocal group of neuromuscular physicians who have raised public concerns in a letter to the editor of a medical journal and some LEMS patients and neuromuscular physicians who have raised public concerns in interviews quoted in articles published in the press. Their overarching concern appears to be that LEMS patients may not be able to get amifampridine treatment because of the concern that it would be priced too high as an orphan drug if we are the first pharmaceutical company to receive an FDA approval for an amifampridine product, thereby giving us the seven-year orphan drug exclusivity and the five-year new chemical entity exclusivity for our product. Articles about their concerns have been published in several national publications and some in the press have sought to tie their expectations about the anticipated pricing of Firdapse® to stories about perceived abusive price increases of drug products by other pharmaceutical companies. This vocal group has also questioned the appropriateness of the provisions of the Orphan Drug Act that would grant us exclusivity if our product were to be the first amifampridine product approved by the FDA, and whether this exclusivity should be eliminated from the law. We have responded to their concerns in a letter to the editor to the same medical journal. However, there can be no assurance as to the ultimate impact of the activities of this vocal group on us or our products.

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® and our other orphan drug candidates target 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. 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®, if approved, because the potential target populations are very small, we may never achieve 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.

Table of Contents

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

We are exposed to the risk of employee or consultant fraud or other misconduct. Misconduct by our employees or consultants could include intentional 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. Employee and consultant 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

We have not received regulatory approval in the United States or any foreign jurisdiction for the commercial sale of any of our drug candidates. The regulatory approval process is lengthy, and we may not be able to obtain all of the regulatory approvals required to manufacture and commercialize our drug candidates.

We do not currently have any products that have been approved for commercialization. We will not be able to commercialize our products until we have obtained the requisite regulatory approvals from applicable governmental authorities. To obtain regulatory approval of a drug candidate, we must demonstrate to the satisfaction of the applicable regulatory agency that such drug candidate is safe and effective for its intended uses. The type and magnitude of the testing required for regulatory approval varies depending on the drug candidate and the disease or condition for which it is being developed. In addition, in the U.S. we must show that the facilities used to manufacture our drug 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 candidates are safe and effective, in which event we would not receive the regulatory approvals required to market them.

Table of Contents

The FDA and other regulatory authorities generally approve products for particular indications. Our drug candidates 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 requests additional information.

The FDA and other regulatory bodies must approve trade names for products. The FDA typically conducts a thorough review of a proposed trade name, including an evaluation of potential confusion with other trade names. We have previously submitted a request for FDA approval of the trade name Firdapse®, which request was conditionally approved in 2014; however, the approval of other drugs since that time may affect the applicability of that conditional approval.

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

In September 2014, we announced positive results from our first Phase 3 clinical trial for Firdapse®. In October 2016, we announced that we had reached an agreement with the FDA under a SPA for the protocol design, clinical endpoints, and statistical analysis approach to be taken in our second Phase 3 study evaluating Firdapse® for the symptomatic treatment of LEMS. We recently announced positive top-line results for our second Phase 3 trial of Firdapse®. Even with a successful second Phase 3 trial of Firdapse®, we may nevertheless fail to meet the safety and efficacy standards required by the FDA to obtain regulatory approval. In addition, while we believe our single proposed Phase 3 registration trial for Firdapse® in MuSK-MG, if successful, along with the Phase 2/3 investigator-sponsored trial, will be sufficient to support an NDA for this indication, there can be no assurance that the FDA will find these trials sufficient for filing or approval of this indication.

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 candidates may not be found to be safe and effective, 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 we may submit for Firdapse®.

In other countries where Firdapse®, CPP-115 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.

Table of Contents

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. 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. 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 considerable more time than other trials, sometimes years, depending on factors such as type, complexity, novelty and intended use of a product candidate. As a result of the uncertainties described above, there can be no assurance that we will meet timelines that we establish for any of our clinical trials.

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

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

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

- reliance on the third party for regulatory compliance and quality assurance;
- reliance on the continued financial viability of the third parties;
- limitations on supply availability resulting from capacity and scheduling constraints of the third parties;
- impact on our reputation in the marketplace if manufacturers of our products, once commercialized, 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.

Table of Contents

If any of our contract manufacturers fail to achieve and maintain appropriate manufacturing standards, patients using our drug candidates 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.

Even if we obtain regulatory approvals, our drug candidates will be subject to ongoing regulatory review. If we fail to comply with continuing U.S. and applicable foreign regulations, we could lose those approvals, and our business would be severely harmed.

Even if we receive regulatory approval of any drugs we are developing or may develop, we will be subject to continuing regulatory review, 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.

As a condition of approval for some of our products, the FDA might require a Risk Evaluation and Mitigation Strategy (REMS) to help ensure that the benefits of the drug outweigh the potential risks. REMS can include medication guides, communication plans for healthcare professionals, and other Elements To Assure Safe Use (ETASU). ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring, and the use of patient registries. For example, approved versions of vigabatrin, the active moiety in our CPP-109 product (which operates by the same mechanism of action as our CPP-115 product) were approved with an FDA-mandated REMS program due to the risks of visual field damage and are only available through a special restricted distribution program approved by the FDA. Accordingly, our abbreviated new drug application (ANDA) for vigabatrin, if approved, will be subject to either the same REMS, or a comparable REMS that will need to be reviewed and approved by the FDA. If any of our products were to be approved with a REMS, the potential market and profitability of the drug could be materially affected.

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.

Table of Contents

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 U.S., there have been a number of court cases, legislative and regulatory changes and other potential changes relating to the healthcare system that restrict or regulate post-approval activities, which may affect our ability to profitably sell Firdapse® 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 U.S. 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 50% discount 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).

Table of Contents

Both President Trump and the Republican leadership in Congress have expressed their intention to eliminate the Health Care Reform Law and replace it with a still unknown new law. While proposals have been introduced in Congress, it is still unknown what form any such modifications or any law passed to replace the Health Care Reform Law would take, and how or any such new law may affect our business in the future.

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 U.S. Congress of the FDA s approval process may subject us to more stringent product labeling and post-marketing testing and other requirements. Delays in feedback from the FDA may affect our ability to quickly update or adjust our label in the interest of patient adherence and tolerability. We cannot predict whether other legislative changes will be adopted or how such changes would affect the pharmaceutical industry generally and specifically the commercialization of Firdapse®.

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 U.S., orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and user-fee waivers. The company that first obtains FDA approval for a designated orphan drug for a given rare disease receives marketing exclusivity for use of that drug for the stated 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.

In the EU, the EMA s Committee for Orphan Medicinal Products, or COMP, grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the EU Community and for which no satisfactory method of diagnosis, prevention, or treatment has been authorized (or the product would be a significant benefit to those affected). Additionally, designation is granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the EU would be sufficient to justify the necessary investment in developing the medicinal product. An EU orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and 10 years of market exclusivity is granted following medicinal product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Orphan drug designation must be requested before submitting an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

Table of Contents

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

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

Breakthrough Therapy Designation may not actually lead to a faster review process.

Under the Prescription Drug User Fee Act, the FDA has a goal of responding to NDAs for new molecular entities within 10 months of the date that the FDA files the NDA for standard review, but this timeframe is also often extended. We have in the past and we may in the future, seek approval of our drug candidates under programs designed to accelerate the FDA s review and approval of NDAs. For example, there is a category of drugs referred to as breakthrough therapies, which are defined as drugs which are intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. In our case, Firdapse® has been granted breakthrough therapy designation for the treatment of LEMS. In the future, we may request breakthrough designation or fast track designation from the FDA for our other drug candidates or for treatment of other diseases, but we cannot assure that we will obtain such designations. Moreover, even if we obtain breakthrough designation, under the Prescription Drug User Fee Act, the FDA has a goal of responding to NDAs for new molecular entities within 10 months of the date that the FDA files the NDA for standard review, but this timeframe is also often extended. Further, these designations do not guarantee FDA approval of our NDAs, that the development program or review timeline will ultimately be shorter than if we had not obtained the designations, or that the FDA will not request additional information, including requesting additional clinical studies (although potentially a post-marketing requirement), during its review. Any request for additional information or clinical data could delay the FDA s timely review of our NDA.

Table of Contents

Even though our second Phase 3 study of Firdapse® for the treatment of LEMS was 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.

Risks Related to Our Intellectual Property

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

All of our patent rights for Firdapse® are derived from our license agreement with BioMarin. Pursuant to this license agreement, we have licensed rights under BioMarin s Firdapse® patent applications in the United States, which expire in 2022 and 2034. 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 BioMarin. If we violate or fail to perform any term or covenant of the license agreement, BioMarin 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 BioMarin, 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.

Most of our patent rights for CPP-115 are derived from our license agreement with Northwestern University. Pursuant to this license agreement, we have exclusive worldwide rights to two patents in the United States. These were filed and obtained by Northwestern relating to compositions of matter for a class of molecules, including CPP-115. Both patents expire in 2023. Additionally, we have licensed rights from Northwestern to know how for derivatives of vigabatrin that are unrelated to CPP-115. These rights are subject to the right of Northwestern, under limited circumstances, to practice the covered inventions for or on its own behalf for research. 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, including milestone payments, to Northwestern. If we violate or fail to perform any term or covenant of the license agreement, Northwestern 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 Northwestern, 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 CPP-115, and our business, results of operations, financial condition and prospects would be materially adversely affected.

Table of Contents

If we obtain approval to market Firdapse® or CPP-115, our commercial success will depend in large part on our ability to use patents, especially those licensed to us by BioMarin and Northwestern, respectively, 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 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 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 our patents or design around our patents so as to compete with us without infringing our patents. Moreover, the issuance of a patent is not conclusive as to its validity or enforceability, and so our patents 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.

While we are not currently aware of any third-party patents which we may infringe, there can be no assurance that we do not or will not infringe on patents held by third parties or that third parties will not claim that we have infringed on their patents. In the event that our 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. There may be patents held by others of which we are unaware that contain claims that our products or operations infringe. In addition, given the complexities and uncertainties of patent laws, there may be patents of which we are aware that we may ultimately be held to infringe, particularly if the claims of the patent are determined to be broader than we believe them to be. Adding to this uncertainty, in the U.S., patent applications filed in recent years are confidential for 18 months, while older applications are not publicly available until the patent issues. As a result, avoiding patent infringement may be difficult.

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

- we may be required to pay substantial financial damages if a court decides that our technologies infringe a competitor s patent, which can be tripled if the infringement is deemed willful, or be required to discontinue or significantly delay development, marketing, selling and licensing of the affected products and intellectual property rights;

- a court may prohibit us from selling or licensing our product without 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

Table of Contents

we may have to redesign our product so that it does not infringe others patent rights, which may not be possible or could require substantial funds or time and require additional studies.

In addition, employees, consultants, contractors and others may use the proprietary information of others in their work for us or disclose our proprietary information to others. As an example, we do not currently have written agreements regarding confidentiality with several principal members of our Scientific Advisory Board. 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 others 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 file patent applications or issue patents claiming technology that is also claimed by us in pending applications, we may be required to participate in interference proceedings with the U.S. Patent Office or in other proceedings outside the U.S., including oppositions, to determine priority of invention or patentability. 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.

Table of Contents

Risks Related to Our Common Stock and this Offering

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;
- 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 licenses from BioMarin and Northwestern), 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 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.

Table of Contents

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. Further, at the 2016 annual meeting of stockholders, the stockholders approved the Rights Plan.

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, 2019, 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.

Table of Contents

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 November 27, 2017, we had 86,045,926 shares of our common stock outstanding, of which 6,886,070 shares were held by our officers and directors. We also had outstanding: (i) stock options to purchase an aggregate of 5,285,000 shares at exercise prices ranging from $0.47 to $4.64 per share (2,726,664 of which are currently exercisable). Sales of restricted shares or shares underlying stock options, 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.

Management will have broad discretion to use the proceeds from this offering, and we may not use the proceeds effectively.

We have not designated any portion of the net proceeds from this offering to be used for any particular purpose. Accordingly, our management will have broad discretion as to the application of the net proceeds from this offering, and could spend the proceeds in ways that do not necessarily improve our operating results or enhance the value of our common stock.

You will experience immediate dilution in the book value per share of the common stock you purchase.

Since the price per share of our common stock being offered is substantially higher than the net tangible book value per share of our common stock, you will suffer substantial dilution with respect to the net tangible book value of the common stock you purchase in this offering. Based on the public offering price of $3.50 per share and our net tangible book value as of September 30, 2017, if you purchase shares of common stock in this offering, you will suffer immediate and substantial dilution of $2.71 per share with respect to the net tangible book value of the common stock. See the section titled Dilution in this prospectus supplement for a more detailed discussion of the dilution you will incur if you purchase common stock in this offering.

Table of Contents

CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS

This prospectus supplement 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 forward-looking statements. Such statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by such forward looking statements. The forward-looking statements made in this prospectus are based on current expectations that involve numerous risks and uncertainties.

The successful development and commercialization of our current drug candidates is highly uncertain. We cannot reasonably estimate or know the nature, timing, or estimated expenses of the efforts necessary to complete the development of, or the period in which material net cash inflows are expected to commence due to the numerous risks and uncertainties associated with developing such products, including the uncertainty of:

- our estimates regarding anticipated capital requirements and our need for additional funding;
- the risk that another pharmaceutical company will receive an approval for its formulation of 3,4-diaminopyridine (3,4-DAP) for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS), Congenital Myasthenic Syndromes (CMS), or any other indication, before we do;
- whether the clinical studies or trials that are required to be completed before the FDA will accept an NDA submission for Firdapse® for the treatment of either LEMS or CMS will be successful;
- what additional supporting information, including any additional clinical studies or trials, will be required before the FDA will accept our NDA submission for Firdapse® for the treatment of either LEMS or CMS (or any other condition or disease);
- whether any NDA that we may submit for Firdapse® will be accepted for filing by the FDA, and if accepted, whether it will be granted a priority review;
- whether, even if the FDA accepts an NDA submission for Firdapse®, such product will be determined to be safe and effective and approved for commercialization for any of the submitted indications;
- whether the receipt of breakthrough therapy designation for Firdapse® for LEMS will result in an expedited review of Firdapse® by the FDA or affect the likelihood that the product will be found to be safe and effective;

whether as part of the FDA review of any NDA that we may submit for filing for Firdapse®, the tradename Firdapse®, which is the tradename used for the same product in Europe, will be approved for use for the product in the United States;

Table of Contents

whether, assuming Firdapse® is approved for commercialization, we will be able to develop or contract with a sales and marketing organization that can successfully market Firdapse® while maintaining full compliance with applicable federal and state laws, rules and regulations;

whether any future trial that we undertake evaluating Firdapse® for the treatment of MuSK-MG or SMA will be successful and whether we can obtain the funding required to conduct such trials;

whether CPP-115 will be determined to be safe for humans;

whether CPP-115 will be determined to be effective for the treatment of infantile spasms, or possibly Tourette s Disorder;

whether we can successfully design and complete a bioequivalence study of our version of vigabatrin compared to Sabril® that is acceptable to the FDA;

whether any ANDA that we submit for a generic version of Sabril® will be accepted by the FDA for review and approved (and the timing of any such approval);

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;

our ability to complete our trials and studies on a timely basis and within the budgets we establish for such trials and studies and whether our trials and studies will be successful;

the ability of our third-party suppliers and contract manufacturers to maintain compliance with cGMP;

whether our estimates of the size of the market for our drug candidates will turn out to be accurate;

the pricing of our products that we may be able to achieve if we are granted the ability to commercialize our drug candidates; and

changes in 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 in the healthcare industry generally.

Our current plans and objectives are based on assumptions relating to the development of our current drug candidates. 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 prospectus supplement, 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.

Table of Contents

USE OF PROCEEDS

We expect that the net proceeds from this offering will be approximately $46.6 million, after deducting underwriting fees and estimated offering expenses payable by us, or approximately $53.6 million if the underwriters exercise their over-allotment option in full. We intend to use the net proceeds from the sale of the securities: (i) to fund clinical studies of Firdapse® for the treatment of MuSK-MG and SMA, (ii) to fund our pre-commercialization activities for Firdapse®, and (iii) for general corporate purposes.

As of the date of this prospectus supplement, we cannot specify with certainty the particular uses of all of the proceeds from this offering. As a result, our management will retain broad discretion in the allocation and use of the net proceeds from this offering. Pending the application of the net proceeds for these purposes, we expect to invest the proceeds in short-term, interest-bearing instruments or other investment-grade securities.

Table of Contents

DILUTION

The net tangible book value of our common stock as of September 30, 2017 was approximately $31.7 million, or $0.37 per share. Net tangible book value per share of our common stock is equal to our net tangible assets (tangible assets less total liabilities) divided by the number of shares of our common stock issued and outstanding as of September 30, 2017.

Dilution in net tangible book value per share represents the difference between the public offering price per share of our common stock and the adjusted net tangible book value per share of our common stock after giving effect to this offering. After giving effect to the sale of 14,285,715 shares of our common stock in this offering at the public offering price of $3.50 per share, and after deducting the underwriter s fees and estimated offering expenses payable by us, our adjusted net tangible book value per share of our common stock at September 30, 2017, would have been approximately $78.3 million, or $0.79 per share. This represents an immediate increase in net tangible book value per share of our common stock of approximately $0.42 per share to existing stockholders and an immediate dilution of approximately $2.71 per share to purchasers in this offering. The following table illustrates this per-share dilution:

Public offering price per share		$ 3.50
Net tangible book value per share as of September 30, 2017,	$ 0.37	
Increase per share attributable to this offering	$ 0.42	
As adjusted net tangible book value per share as of September 30, 2017		$ 0.79
Dilution per share to new investors		$ 2.71

The foregoing discussion and table do not take into account further dilution to new investors that could occur upon the exercise of the underwriters over-allotment option to purchase additional shares within 30 days of the date of this prospectus supplement. If the underwriters exercise in full their over-allotment option to purchase additional shares, our net tangible book value as of September 30, 2017, after giving effect to this offering, would have been approximately $85.4 million, or approximately $0.84 per share, representing an immediate dilution of $2.66 per share to new investors purchasing shares of common stock in this offering.

The above table is based on 85,234,979 shares outstanding as of September 30, 2017 and excludes, as of that date:

- 6,085,000 shares of our common stock subject to outstanding options under our 2006 Stock Incentive Plan and our 2014 Stock Incentive Plan having a weighted average exercise price of $1.74 per share;
- 26,667 restricted stock units subject to vesting; and
- 3,628,334 shares of our common stock that have been reserved for issuance in connection with our 2014 Stock Incentive Plan.

Subsequent to September 30, 2017, options to purchase an aggregate of 700,000 shares of our common stock were exercised at an exercise price of $0.47 per share, resulting in gross proceeds of approximately $329,000, and 26,667 restricted stock units vested and an equal number of shares of common stock were distributed. Additionally, options to purchase an aggregate of 100,000 shares of our common stock were exercised on a cashless basis at an exercise price of $0.47 per share, resulting in a distribution of an aggregate of 84,280 shares.

Table of Contents

MARKET PRICE OF OUR COMMON STOCK AND DIVIDEND POLICY

Our common stock trades on The NASDAQ Capital Market under the symbol CPRX. The following table sets forth the high and low closing sales prices per share of our common stock as reported on The NASDAQ Capital Market for the periods indicated.

	High	Low
Year Ended December 31, 2015		
First Quarter	$ 4.93	$ 2.74
Second Quarter	$ 4.73	$ 3.16
Third Quarter	$ 5.74	$ 3.00
Fourth Quarter	$ 3.50	$ 2.33
Year Ended December 31, 2016		
First Quarter	$ 2.36	$ 1.01
Second Quarter	$ 1.25	$ 0.56
Third Quarter	$ 1.25	$ 0.72
Fourth Quarter	$ 1.46	$ 0.96
Year Ending December 31, 2017		
First Quarter	$ 2.01	$ 1.09
Second Quarter	$ 2.84	$ 1.64
Third Quarter	$ 3.14	$ 2.40
Fourth Quarter (through November 27, 2017)	$ 3.61	$ 2.51

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.

Table of Contents

DESCRIPTION OF SECURITIES

The following description of our capital stock is intended as a summary only and is qualified in its entirety by reference to our amended and restated certificate of incorporation, as amended, and to our by-laws.

Our authorized capital stock consists of:

- 150,000,000 shares of common stock, par value $0.001 per share; and
- 5,000,000 shares of preferred stock, par value $0.001 per share.

As of November 27, 2017 we had outstanding:

- 86,045,926 shares of our common stock;
- 5,285,000 shares of our common stock subject to outstanding options under our 2006 Stock Incentive Plan and our 2014 Stock Incentive Plan, having a weighted average exercise price of $1.93 per share; and
- 3,628,334 shares of our common stock that have been reserved for issuance in connection with our 2014 Stock Incentive Plan.

Common Stock

The material terms of our common stock are described under the caption General Description of our Capital Stock starting on page 33 of the accompanying prospectus.

Table of Contents

UNDERWRITING

We are offering the shares of common stock described in this prospectus supplement through the several underwriters identified in the table below. We have entered into a firm commitment underwriting agreement with Piper Jaffray & Co., as representative of the several underwriters. Subject to the terms and conditions of the underwriting agreement, we have agreed to sell to the underwriters, and the underwriters have agreed to purchase from us, the number of shares of common stock listed opposite their names below. The underwriters are committed to purchase and pay for all of the shares of common stock if any are purchased.

Underwriter	Number of Shares
Piper Jaffray & Co.	8,857,143
SunTrust Robinson Humphrey, Inc.	3,857,143
H.C. Wainwright & Co.	857,143
Roth Capital Partners, LLC	714,286
Total	14,285,715

The underwriters have advised us that they propose to offer the common stock directly to the public at the offering price set forth on the cover page of this prospectus supplement. The underwriters propose to offer the shares to certain dealers at the same price less a concession of not more than $0.126 per share. After the offering, these figures may be changed by the underwriters.

We have granted the underwriters an option to buy up to 2,142,857 additional shares of common stock from us to cover over allotments, if any. The underwriters may exercise this option at any time and from time to time during the 30 day period from the date of this prospectus supplement. If any additional shares of common stock are purchased, the underwriters will offer the additional shares on the same terms as those on which the shares are being offered.

The underwriting fee is equal to the public offering price per share of common stock less the amount paid by the underwriters to us per share of common stock. The following table shows the per share and total underwriting discount to be paid to the underwriters in this offering assuming both no exercise and full exercise of the underwriters option to purchase additional shares:

	With no Over-Allotment	With Over-Allotment
Per Share	$ 0.21	$ 0.21
Total	$ 3,000,000	$ 3,450,000

We estimate that the total fees and expenses payable by us, excluding underwriting discount, will be approximately $425,000, which includes approximately $125,000 that we have agreed to reimburse the underwriters for the fees incurred by it in connection with the offering.

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act, or to contribute to payments that the underwriters may be required to make in respect of those liabilities.

We and each of our directors and executive officers are subject to lock up agreements that prohibit us and them from offering for sale, pledging, assigning, encumbering, announcing the intention to sell, selling, contracting to sell, granting any option, right or warrant to purchase, or otherwise transferring or disposing of, any shares of our common

stock or any securities convertible into or exercisable or exchangeable for shares of our common stock for a period of at least 90 days following the date of this prospectus supplement without the prior written consent of Piper Jaffray. The lock up agreements do not prohibit our directors and executive officers from transferring shares of our common stock for bona fide estate or tax planning purposes, subject to certain requirements, including that the transferee be subject to the same lock up terms.

Table of Contents

The lock up provisions do not prohibit us from issuing shares upon the exercise or conversion of securities outstanding on the date of this prospectus supplement. The lock up provisions do not prevent us from selling shares to the underwriters pursuant to the underwriting agreement, or from granting options to acquire securities under our existing stock option plans or issuing shares upon the exercise or conversion of securities outstanding on the date of this prospectus supplement.

Our shares are quoted on the Nasdaq Capital Market under the symbol CPRX.

To facilitate the offering, the underwriters may engage in transactions that stabilize, maintain or otherwise affect the price of our common stock during and after the offering. Specifically, the underwriters may over allot or otherwise create a short position in the common stock for its own account by selling more shares of common stock than we have sold to it. Short sales involve the sale by the underwriters of a greater number of shares than the underwriters are required to purchase in the offering. The underwriters may close out any short position by either exercising its option to purchase additional shares or purchasing shares in the open market.

In addition, the underwriters may stabilize or maintain the price of the common stock by bidding for or purchasing shares of common stock in the open market and may impose penalty bids. If penalty bids are imposed, selling concessions allowed to syndicate members or other broker dealers participating in the offering are reclaimed if shares of common stock previously distributed in the offering are repurchased, whether in connection with stabilization transactions or otherwise. The effect of these transactions may be to stabilize or maintain the market price of the common stock at a level above that which might otherwise prevail in the open market. The imposition of a penalty bid may also affect the price of the common stock to the extent that it discourages resales of the common stock. The magnitude or effect of any stabilization or other transactions is uncertain. These transactions may be effected on the Nasdaq Capital Market or otherwise and, if commenced, may be discontinued at any time. The underwriters may also engage in passive market making transactions in our common stock. Passive market making consists of displaying bids on the Nasdaq Capital Market limited by the prices of independent market makers and effecting purchases limited by those prices in response to order flow. Rule 103 of Regulation M promulgated by the Commission limits the amount of net purchases that each passive market maker may make and the displayed size of each bid. Passive market making may stabilize the market price of the common stock at a level above that which might otherwise prevail in the open market and, if commenced, may be discontinued at any time.

This prospectus supplement and the accompanying prospectus in electronic format may be made available on the web sites maintained by the underwriters and the underwriters may distribute prospectuses and prospectus supplements electronically.

From time to time in the ordinary course of its businesses, the underwriters and certain of their affiliates have engaged, and may in the future engage, in commercial banking or investment banking transactions with us and our affiliates.

Table of Contents

Selling Restrictions

European Economic Area

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive (each, a Relevant Member State) an offer to the public of any shares of our common stock may not be made in that Relevant Member State, except that an offer to the public in that Relevant Member State of any shares of our common stock may be made at any time under the following exemptions under the Prospectus Directive, if they have been implemented in that Relevant Member State:

(a) to any legal entity which is a qualified investor as defined in the Prospectus Directive;

(b) to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150, natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the representatives for any such offer; or

(c) in any other circumstances falling within Article 3(2) of the Prospectus Directive, provided that no such offer of shares of our common stock shall result in a requirement for the publication by us or any underwriter of a prospectus pursuant to Article 3 of the Prospectus Directive.

For the purposes of this provision, the expression an offer to the public in relation to any shares of our common stock in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and any shares of our common stock to be offered so as to enable an investor to decide to purchase any shares of our common stock, as the same may be varied in that Member State by any measure implementing the Prospectus Directive in that Member State, the expression Prospectus Directive means Directive 2003/71/EC (and amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member State), and includes any relevant implementing measure in the Relevant Member State, and the expression 2010 PD Amending Directive means Directive 2010/73/EU.

United Kingdom

This prospectus supplement is only being distributed to, and is only directed at, persons in the United Kingdom that are qualified investors within the meaning of Article 2(1)(e) of the Prospectus Directive that are also (i) investment professionals falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005 (the Order) or (ii) high net worth entities, and other persons to whom it may lawfully be communicated, falling within Article 49(2)(a) to (d) of the Order (each such person being referred to as a relevant person). This prospectus supplement and its contents are confidential and should not be distributed, published or reproduced (in whole or in part) or disclosed by recipients to any other person in the United Kingdom. Any person in the United Kingdom that is not a relevant person should not act or rely on this document or any of its contents.

Table of Contents

Canada

The shares of our common stock may be sold only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions and Ongoing Registrant Obligations. Any resale of the shares of our common stock must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if the prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser s province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser s province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 (or, in the case of securities issued or guaranteed by the government of a non-Canadian jurisdiction, section 3A.4) of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriter is not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

Hong Kong

The common shares may not be offered or sold in Hong Kong by means of any document other than (i) in circumstances which do not constitute an offer to the public within the meaning of the Companies Ordinance (Cap. 32, Laws of Hong Kong), or (ii) to professional investors within the meaning of the Securities and Futures Ordinance (Cap. 571, Laws of Hong Kong) and any rules made thereunder, or (iii) in other circumstances which do not result in the document being a prospectus within the meaning of the Companies Ordinance (Cap. 32, Laws of Hong Kong) and no advertisement, invitation or document relating to the shares may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or elsewhere), which is directed at, or the contents of which are likely to be accessed or read by, the public in Hong Kong (except if permitted to do so under the laws of Hong Kong) other than with respect to common shares which are or are intended to be disposed of only to persons outside Hong Kong or only to professional investors within the meaning of the Securities and Futures Ordinance (Cap. 571, Laws of Hong Kong) and any rules made thereunder.

Singapore

This prospectus supplement has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus supplement and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the common shares may not be circulated or distributed, nor may the common shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor under Section 274 of the Securities and Futures Act, Chapter 289 of Singapore (the SFA), (ii) to a relevant person pursuant to Section 275(1), or any person pursuant to Section 275(1A), and in accordance with the conditions specified in Section 275 of the SFA or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA, in each case subject to compliance with conditions set forth in the SFA.

Table of Contents

Where the common shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is:

(a) a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or

(b) a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary of the trust is an individual who is an accredited investor,

shares, debentures and units of shares and debentures of that corporation or the beneficiaries rights and interest (howsoever described) in that trust shall not be transferred within six months after that corporation or that trust has acquired the common shares pursuant to an offer made under Section 275 of the SFA except:

(a) to an institutional investor (for corporations, under Section 274 of the SFA) or to a relevant person defined in Section 275(2) of the SFA, or to any person pursuant to an offer that is made on terms that such shares, debentures and units of shares and debentures of that corporation or such rights and interest in that trust are acquired at a consideration of not less than $200,000 (or its equivalent in a foreign currency) for each transaction, whether such amount is to be paid for in cash or by exchange of securities or other assets, and further for corporations, in accordance with the conditions specified in Section 275 of the SFA;

(b) where no consideration is or will be given for the transfer; or

(c) where the transfer is by operation of law.

Switzerland

The common shares may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange (the SIX) or on any other stock exchange or regulated trading facility in Switzerland. This document has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this document nor any other offering or marketing material relating to the common shares or the offering may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this document nor any other offering or marketing material relating to the offering, or the common shares have been or will be filed with or approved by any Swiss regulatory authority. In particular, this document will not be filed with, and the offer of common shares will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA, and the offer of common shares has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes (CISA). Accordingly, no public distribution, offering or advertising, as defined in CISA, its implementing ordinances and notices, and no distribution to any non-qualified investor, as defined in CISA, its implementing ordinances and notices, shall be undertaken in or from Switzerland, and the investor protection afforded to acquirers of interests in collective investment schemes under CISA does not extend to acquirers

of common shares.

Table of Contents

United Arab Emirates

This offering has not been approved or licensed by the Central Bank of the United Arab Emirates (the UAE), Securities and Commodities Authority of the UAE and/or any other relevant licensing authority in the UAE including any licensing authority incorporated under the laws and regulations of any of the free zones established and operating in the territory of the UAE, in particular the Dubai Financial Services Authority (DFSA), a regulatory authority of the Dubai International Financial Centre (DIFC). The offering does not constitute a public offer of securities in the UAE, DIFC and/or any other free zone in accordance with the Commercial Companies Law, Federal Law No 8 of 1984 (as amended), DFSA Offered Securities Rules and NASDAQ Dubai Listing Rules, accordingly, or otherwise. The common shares may not be offered to the public in the UAE and/or any of the free zones.

The common shares may be offered and issued only to a limited number of investors in the UAE or any of its free zones who qualify as sophisticated investors under the relevant laws and regulations of the UAE or the free zone concerned.

France

This prospectus supplement (including any amendment, supplement or replacement thereto) is not being distributed in the context of a public offering in France within the meaning of Article L. 411-1 of the French Monetary and Financial Code (Code monétaire et financier).

This prospectus supplement has not been and will not be submitted to the French Autorité des marchés financiers (the AMF) for approval in France and accordingly may not and will not be distributed to the public in France.

Pursuant to Article 211-3 of the AMF General Regulation, French residents are hereby informed that:

(a) the transaction does not require a prospectus to be submitted for approval to the AMF;

(b) persons or entities referred to in Point 2°, Section II of Article L.411-2 of the Monetary and Financial Code may take part in the transaction solely for their own account, as provided in Articles D. 411-1, D. 734-1, D. 744-1, D. 754-1 and D. 764-1 of the Monetary and Financial Code; and

(c) the financial instruments thus acquired cannot be distributed directly or indirectly to the public otherwise than in accordance with Articles L. 411-1, L. 411-2, L. 412-1 and L. 621-8 to L. 621-8-3 of the Monetary and Financial Code.

This prospectus supplement is not to be further distributed or reproduced (in whole or in part) in France by the recipients of this prospectus supplement. This prospectus supplement has been distributed on the understanding that such recipients will only participate in the issue or sale of our common stock for their own account and undertake not to transfer, directly or indirectly, our common stock to the public in France, other than in compliance with all applicable laws and regulations and in particular with Articles L. 411-1 and L. 411-2 of the French Monetary and Financial Code.

Table of Contents

LEGAL MATTERS

The validity of the shares of common stock that we are offering hereby will be passed upon by Akerman LLP, Fort Lauderdale, Florida. Goodwin Procter LLP, New York, New York, is acting as counsel for the underwriters in connection with this offering.

EXPERTS

The audited financial statements and management s assessment of the effectiveness of internal control over financial reporting incorporated by reference in this prospectus supplement and the accompanying prospectus have been so incorporated by reference in reliance upon the reports of Grant Thornton LLP, independent registered public accountants, upon the authority of said firm as experts in accounting and auditing.

WHERE YOU CAN FIND ADDITIONAL INFORMATION

We file annual, quarterly and special reports, proxy statements and other information with the SEC. Our SEC filings are available to the public over the SEC s website at http://www.sec.gov. You may also read and copy any document we file at the SEC s Public Reference Room at 100 F Street, N.E., Washington, D.C. 20549. Please call the SEC at (800) SEC 0330 for further information on the operating rules and procedures for the public reference room.

This prospectus supplement and the accompanying prospectus do not contain all of the information included in the registration statement. We have omitted certain parts of the registration statement in accordance with the rules and regulations of the SEC. For further information, we refer you to the registration statement, including its exhibits and schedules. Statements contained in this prospectus supplement and the accompanying prospectus about the provisions or contents of any contract, agreement or any other document referred to are not necessarily complete. Please refer to the actual exhibit for a more complete description of the matters involved.

INCORPORATION OF INFORMATION BY REFERENCE

The SEC allows us to incorporate by reference information into this prospectus supplement, which means that we can disclose important information to you by referring you to another document filed separately with the SEC. The information incorporated by reference is deemed to be a part of this prospectus supplement, except for any information superseded by information in any amendment to this prospectus supplement.

The following documents filed with the SEC are incorporated by reference in this prospectus supplement:

- our Annual Report on Form 10-K for the year ended December 31, 2016, filed with the SEC on March 15, 2017;
- our Quarterly Report on Form 10-Q for the quarter ended March 31, 2017, filed with the SEC on May 10, 2017;

Table of Contents

our Quarterly Report on Form 10-Q for the quarter ended June 30, 2017, filed with the SEC on August 9, 2017;

our Quarterly Report on Form 10-Q for the quarter ended September 30, 2017, filed with the SEC on November 8, 2017;

our Current Reports on Form 8-K (or amendments thereto) filed with the SEC on March 15, 2017, March 15, 2017, May 10, 2017, May 26, 2017, July 12, 2017, August 9, 2017, August 30, 2017, October 31, 2017, November 8, 2017, November 21, 2017 and November 27, 2017; and

our description of our common stock contained in our Registration Statement on Form 8-A, filed with the SEC on September 29, 2006, along with Amendment No. 1 thereto, filed with the SEC on October 18, 2006.

We also incorporate by reference into this prospectus supplement all documents (other than current reports furnished under Item 2.02 or Item 7.01 of Form 8-K and exhibits filed on such form that are related to such items) that are filed by us with the SEC pursuant to Sections 13(a), 13(c), 14 or 15(d) of the Exchange Act after the date of this prospectus supplement but prior to the termination of the offering. These documents include periodic reports, such as Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q and Current Reports on Form 8-K, as well as proxy statements.

You may obtain a copy of any of these documents at no cost by requesting them from us or by writing or calling: Catalyst Pharmaceuticals, Inc., 355 Alhambra Circle, Suite 1250, Coral Gables, Florida, 33134, Attn: Investor Relations, or by calling (305) 420-3200. Copies of each of these filings are also available for no cost on our website, www.catalystpharma.com, or on the SEC s web site, www.sec.gov.

Table of Contents

Prospectus

$150,000,000

Common Stock

Preferred Stock

Warrants to Purchase Common Stock

Debt Securities

Units

We may offer and sell from time to time common stock, preferred stock, warrants to purchase common stock, and debt securities (including debt securities that may be convertible or exchangeable for common stock or other securities). The common stock, preferred stock, warrants to purchase common stock and debt securities may be offered separately or together, in units or multiple series, in amounts, at prices and on terms that will be set forth in one or more prospectus supplements to this prospectus.

The prospectus provides a general description of the securities that we may offer. Each time securities are offered and sold pursuant to this prospectus, a supplement to this prospectus that contains specific information about the offering will be provided. We may also authorize one or more free writing prospectuses to be provided to you in connection with these offerings. The prospectus supplement and any related free writing prospectus may add, update or change information contained in this prospectus. You should carefully read this prospectus, the applicable prospectus supplement and any related free writing prospectus, as well as the documents incorporated by reference, before you invest in shares of our common stock. This prospectus may not be used to sell securities unless accompanied by a prospectus supplement.

Our common stock is listed on The NASDAQ Capital Market under the symbol CPRX. On July 20, 2017, the last reported sale price on The NASDAQ Capital Market was $3.05 per share. There is no market for any preferred stock, warrants to purchase common stock, or debt securities we may sell pursuant to this prospectus.

Our business and investing in our securities involves significant risks. You should carefully read and consider the *Risk Factors* beginning on page 7 of this prospectus before investing.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed upon the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense.

The date of this prospectus is July 26, 2017

Table of Contents

TABLE OF CONTENTS

ABOUT THIS PROSPECTUS

This prospectus is part of a shelf registration statement on Form S-3 that we filed with the Securities and Exchange Commission (SEC), using the shelf registration process. By using a shelf registration statement, we may, from time to time, sell our securities in one or more offerings up to a total dollar amount of $150,000,000.

Each time that we offer and sell securities, we will provide a prospectus supplement to this prospectus that contains specific information about the securities being offered and sold and the specific terms of that offering. The prospectus supplement may also add, update or change information contained in this prospectus with respect to that offering. If there is any inconsistency between the information in this prospectus and the applicable prospectus supplement, you should rely on the prospectus supplement. Before purchasing any securities, you should carefully read both this prospectus and the applicable prospectus supplement, together with the additional information described under the headings Where You Can Find Additional Information and Incorporation of Information by Reference.

You should rely only on the information contained or incorporated by reference in this prospectus and any related prospectus supplement. We have not authorized anyone to provide you with different information. If anyone provides you with different or inconsistent information, you should not rely on it. We will not make an offer to sell these securities in any jurisdiction where the offer or sale is not permitted. You should assume that the information contained in this prospectus and the applicable prospectus supplement is accurate as of the date on its respective front cover, and that any information incorporated by reference is accurate only as of the date given in the document incorporated by reference, regardless of the time of delivery of this prospectus, any applicable prospectus supplement or any sale of our securities. Our business, financial condition, results of operations and prospects may have changed since that date.

Table of Contents

SUMMARY

This summary highlights information contained elsewhere in this prospectus; it does not contain all of the information you should consider before investing. You should carefully read the entire prospectus, including our filings with the U.S. Securities and Exchange Commission that are incorporated by reference into this prospectus, before making an investment decision.

This prospectus includes trademarks, service marks or trade names owned by us or other companies. All trademarks, service marks or trade names included in this prospectus are the property of their respective owners.

Throughout this prospectus, the terms we , us , our and company refer to Catalyst Pharmaceuticals, Inc.

Our Business

We are a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare debilitating diseases. We currently have three drug candidates in development:

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, from BioMarin Pharmaceutical Inc. (BioMarin). In August 2013, we were granted breakthrough therapy designation by the U.S. Food & Drug Administration (FDA) for Firdapse® for the treatment of patients with Lambert-Eaton Myasthenic Syndrome, or LEMS, a rare and sometimes fatal autoimmune disease characterized by muscle weakness. Further, the FDA has granted Orphan Drug Designation for Firdapse® for the treatment of patients with LEMS, Congenital Myasthenic Syndromes, or CMS, and Myasthenia Gravis (MG).

The chemical entity, amifampridine (3,4-diaminopyridine or 3,4-DAP), has never been approved by the FDA for any indication. Because Firdapse® has been granted Orphan Drug designation for the treatment of LEMS, CMS and MG by the FDA, the product is eligible to receive seven years of marketing exclusivity for either or all of these indications. Further, if we are the first pharmaceutical company to obtain approval for an amifampridine product, of which there can be no assurance, we will be eligible to receive five years of marketing exclusivity with respect to the use of this product for any indication, running concurrently with the seven years of orphan marketing exclusivity described above (if both exclusivities are granted).

We previously sponsored a multi-center, randomized, placebo-controlled Phase 3 trial evaluating Firdapse® for the treatment of LEMS. This Phase 3 trial, which involved 38 subjects, was designed as a randomized withdrawal trial in which all patients were treated with Firdapse® during a 7 to 91-day run-in-period followed by treatment with either Firdapse® or placebo over a two-week randomization period. The co-primary endpoints for this Phase 3 trial were the comparison of changes in patients randomized to continue Firdapse® versus those who transitioned to placebo that occurred in both the Quantitative Myasthenia Gravis Score (QMG), which measures muscle strength, and subject global impression score (SGI), on which the subjects rate their global impression of the effects of a study treatment during the two-week randomization period. In September 2014, we reported positive top-line results from this Phase 3 trial.

During 2014, we established an expanded access program (EAP) to make Firdapse® available to any patients diagnosed with LEMS, CMS, or Downbeat Nystagmus in the United States, who meet the inclusion and exclusion

criteria, with Firdapse® being provided to patients for free until sometime after New Drug Application (NDA) approval, should we receive such approval (of which there can be no assurance). We continue to inform neuromuscular physicians on the availability of the Firdapse® EAP and also to work with various rare disease advocacy organizations to inform patients and physicians about the program.

Table of Contents

On December 17, 2015, we announced completion of the submission of an NDA for Firdapse® for the treatment of LEMS and CMS. However, on February 17, 2016, we announced that we had received a refusal to file letter from the FDA regarding our NDA submission. In early April 2016, we met with the FDA to obtain greater clarity regarding what will be required by the FDA to accept the Firdapse® NDA for filing. Following the receipt of the formal minutes of that meeting, on April 26, 2016, we issued a press release reporting that the FDA has advised us that in addition to the results of the Company s previously submitted multi-center, randomized, placebo-controlled Phase 3 trial, we will need to submit positive results from a second adequate and well-controlled study in patients with LEMS. Additionally, there is a requirement for several more short-term toxicology studies, which are currently in process.

In October 2016, we announced that we had reached an agreement with the FDA under a Special Protocol Assessment (SPA) for the protocol design, clinical endpoints, and statistical analysis approach to be taken in our second Phase 3 study evaluating Firdapse® (amifampridine phosphate) for the symptomatic treatment of LEMS. A SPA is a process by which sponsors ask the FDA to evaluate the protocol of a proposed clinical trial to determine whether it adequately addresses scientific and regulatory requirements for the purpose identified by the sponsor. A SPA agreement indicates FDA concurrence with the adequacy and acceptability of specific critical elements of protocol design, endpoints and analysis. Additionally, it provides a binding agreement with FDA s review division that a pivotal trial design, conduct, and planned analysis adequately addresses the scientific and regulatory objectives in support of a regulatory submission for drug approval. However, the FDA may rescind a SPA agreement when the division director determines that a substantial scientific issue essential to determining the safety or efficacy of the product has been identified after the trial has begun.

We are presently conducting our second Phase 3 trial evaluating Firdapse® for the treatment of LEMS (designated as LMS-003) at sites in Miami, Florida and Los Angeles, California. This double-blind, placebo-controlled withdrawal trial will include approximately 28 subjects, and will have the same co-primary endpoints as our first Phase 3 trial evaluating Firdapse® for the treatment of LEMS. Further, the FDA is allowing us to enroll patients from our expanded access program as study subjects in this second trial. Details of the Phase 3 clinical trial are available on www.clinicaltrials.gov (NCT02970162).

We initiated this trial in December 2016, and we expect to report top-line results from this trial during the second half of 2017. Assuming the results of this trial are successful, and our anticipated timeline for the completion of this trial is met, we expect to resubmit an NDA for Firdapse® for the treatment of LEMS in the second half of 2017. There can be no assurance as to the timing or requirements of this trial, whether this trial, along with the results of our first Phase 3 trial, will be sufficient for the FDA to accept for filing any NDA that we might resubmit in the future for Firdapse® , or whether Firdapse® will ever be approved for commercialization.

Our original NDA submission for Firdapse® included data and information (including data from a currently ongoing investigator treatment IND) providing evidence supporting the benefits of Firdapse® for treating certain types of CMS, and requested that CMS be included in our initial label for Firdapse®. To provide additional support for our submission of an NDA for Firdapse® for the treatment of CMS, in October 2015 we initiated a small blinded clinical trial at four academic centers of up to 10 subjects in the pediatric CMS population, ages 2 to 17. However, after considering comments from the FDA, we determined to enroll both adult and pediatric subjects with CMS in this trial and to expand the number of subjects to be evaluated in the trial to an aggregate of approximately 20 subjects. We also added a fifth trial site for this study, and we expect to add one or more additional sites in the future. Details of this trial are available on www.clinicaltrials.gov (NCT02562066).

2

Table of Contents

Based on currently available information, we expect to report top line results from this study in the first half of 2018 and if the results of the study are successful, we hope to add the CMS indication to our labeling for Firdapse® (as a supplement to that resubmission). We also may include in our initial NDA filing for LEMS those limited types of CMS that are generally considered mechanistically similar to LEMS. There can be no assurance that any trial we perform for Firdapse® for the treatment of CMS will be successful or whether any NDA that we may submit for Firdapse® for the treatment of CMS will be filed by the FDA for review and approved.

In February 2016, we announced the initiation of an investigator-sponsored, randomized, double-blind, placebo-controlled, crossover Phase 2/3 clinical trial evaluating the safety, tolerability and potential efficacy of Firdapse® as a symptomatic treatment for patients with MuSK-MG. MuSK- MG, an ultra-rare sub-population of MG patients, is a debilitating neuromuscular disease, and there are currently no FDA approved therapies for this specific form of 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.

We are currently discussing with the FDA conducting a registration trial evaluating Firdapse® for the treatment of patients with MuSK- MG. There can be no assurance that future clinical trials that we initiate to evaluate Firdapse® for this indication will be successful, or whether we can obtain the resources available to fund any such registration trial. Further, there can also be no assurance that the FDA will ever approve Firdapse® for this indication.

Finally, we may seek to evaluate Firdapse® for the treatment of other treatment-refractory types of MG or other rare, similar neuromuscular diseases, although we have not yet begun to develop clinical programs for these indications and all such programs are subject to the availability of funding. There can be no assurance that Firdapse® will be an effective treatment for other treatment-refractory types of MG or for any other rare, similar neuromuscular diseases.

Prior to the receipt of the refusal to file letter, we had been actively taking steps to prepare for the commercialization of Firdapse® in the United States. In light of the determination that we will have to complete a second adequate and well controlled study evaluating Firdapse® for the treatment of LEMS, we have placed most of these commercialization activities on hold in order to conserve cash. We currently expect to recommence our commercialization plans for Firdapse® during the second half of 2017. Notwithstanding, we are continuing to work with several rare disease advocacy organizations to help increase awareness of LEMS and CMS and to provide awareness and outreach support for the physicians who treat these rare diseases and the patients they treat.

Table of Contents

Under our License Agreement with BioMarin, we have agreed to make the following royalty payments on commercial sales of Firdapse®: (i) royalty payments to BioMarin for seven years from the first commercial sale equal to: (a) 7% of net sales (as defined in the license agreement) in North America in any calendar year for sales up to $100 million, and (b) 10% of net sales in North America in any calendar year in excess of $100 million; and (ii) royalty payments to a third-party licensor of the rights sublicensed to us for seven years from the first commercial sale equal to 7% of net sales (as defined in the license agreement between BioMarin and the third party licensor) in North America in any calendar year. We have also agreed to make certain milestone payments to such third-party licensor and to the former stockholders of Huxley Pharmaceuticals, Inc. (Huxley) that BioMarin is obligated to make. With respect to Firdapse®, the milestones aggregate up to $2.6 million upon acceptance of an NDA for Firdapse® by the FDA for the treatment of LEMS, and up to $7.2 million upon the unconditional approval by the FDA of an NDA for Firdapse® for the treatment of LEMS.

CPP-115

We are developing CPP-115, a GABA aminotransferase inhibitor that, based on our preclinical studies to date, we believe is a more potent form of vigabatrin, and may have fewer side effects (e.g., visual field defects) than those associated with vigabatrin. We are hoping to develop CPP-115 for the treatment of refractory infantile spasms and possibly for the treatment of adult refractory patients with Tourette s Disorder. CPP-115 has been granted Orphan Drug Designation by the FDA for the treatment of infantile spasms and Orphan Medicinal Product Designation in the European Union, or E.U., for West syndrome (a form of infantile spasms).

We are currently refining our development plans for this product. Once the refinement of our development plans is completed, and subject to the then availability of funding, we plan to take the steps to complete the work required to make our drug candidate Phase 2 ready. We are also working with one or more potential investigators who have expressed an interest in evaluating our product for particular indications (particularly infantile spasms). Further, we continue to seek a partner to work with us in furthering the development of CPP-115, although no agreements have been entered into to date.

There can be no assurance that we will ever successfully commercialize CPP-115.

Generic Sabril®

During September 2015, we announced the initiation of a project to develop a generic version of Sabril® (vigabatrin). Sabril® is marketed by Lundbeck Inc. in the United States for the treatment of infantile spasms and complex partial seizures. There can be no assurance that we will be successful in these efforts or that any abbreviated new drug application (ANDA) that we submit for vigabatrin will be accepted for review or approved. Further, while there can be no assurance, we are hopeful that any ANDA submission we make for vigabatrin will be among the first ANDAs submitted for this product.

We are also continuing our efforts to seek a partner to work with us in furthering the development of generic Sabril®. However, no agreements have been entered into to date.

4

Table of Contents

Risks Associated with Product Development

The successful development of our current drug candidates or any other drug candidate we may acquire, develop or license in the future is highly uncertain. We cannot reasonably estimate or know the nature, timing, or estimated expenses of the efforts necessary to complete the development of, or the period in which material net cash inflows are expected to commence due to the numerous risks and uncertainties associated with developing such products, including the uncertainty of:

- our estimates regarding anticipated capital requirements and our need for additional funding;
- the risk that another pharmaceutical company will receive an approval for its formulation of 3,4-diaminopyridine (3,4-DAP) for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS), Congenital Myasthenic Syndromes (CMS), or any other indication, before we do;
- whether the clinical studies or trials that are required to be completed before the FDA will accept an NDA submission for Firdapse® for the treatment of either LEMS will be successful;
- what additional supporting information, including any additional clinical studies or trials, will be required before the FDA will accept our NDA submission for Firdapse® for the treatment of either LEMS or CMS (or any other condition or disease);
- whether any NDA that we may submit for Firdapse® will be accepted for filing by the FDA, and if accepted, whether it will be granted a priority review;
- whether, even if the FDA accepts an NDA submission for Firdapse®, such product will be determined to be safe and effective and approved for commercialization for any of the submitted indications;
- whether the receipt of breakthrough therapy designation for Firdapse® for LEMS will result in an expedited review of Firdapse® by the FDA or affect the likelihood that the product will be found to be safe and effective;
- whether as part of the FDA review of any NDA that we may submit for filing for Firdapse®, the tradename Firdapse®, which is the tradename used for the same product in Europe, will be approved for use for the product in the United States;
- whether, assuming Firdapse® is approved for commercialization, we will be able to develop or contract with a sales and marketing organization that can successfully market Firdapse® while maintaining full compliance with applicable federal and state laws, rules and regulations;

whether any future trial that we undertake evaluating Firdapse® for the treatment of MuSK-MG will be successful and whether we can obtain the funding required to conduct such trial;

whether CPP-115 will be determined to be safe for humans;

whether CPP-115 will be determined to be effective for the treatment of infantile spasms, Tourette s Disorder, or any other indication;

whether we can successfully design and complete a bioequivalence study of our version of vigabatrin compared to Sabril® that is acceptable to the FDA;

Table of Contents

whether any ANDA that we submit for a generic version of Sabril® will be accepted by the FDA for review and approved (and the timing of any such approval);

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;

our ability to complete our trials and studies on a timely basis and within the budgets we establish for such trials and studies and whether our trials and studies will be successful;

the ability of our third-party suppliers and contract manufacturers to maintain compliance with current Good Manufacturing Practices (cGMP);

whether our estimates of the size of the market for our drug candidates will turn out to be accurate;

the pricing of our products that we may be able to achieve if we are granted the ability to commercialize our drug candidates; and

changes in 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 in the healthcare industry generally.

Company Information

Our principal executive offices are located at 355 Alhambra Circle, Suite 1250, Coral Gables, Florida 33134, and our telephone number at that address is (305) 420-3200.

Table of Contents

RISK FACTORS

An investment in our securities involves a high degree of risk. You should carefully consider the risks described below as well as the other information in this prospectus before deciding to invest in or maintain your investment in our company. You should also carefully review the Risk Factors contained in the applicable prospectus supplement and in our most recent Annual Report on Form 10-K and any updates in subsequent Quarterly Reports on Form 10-Q. The risks described below are not intended to be an all-inclusive list of the potential risks relating to an investment in our securities. Any of the risk factors described below could significantly and adversely affect our business, prospects, financial condition and results of operations. Additional risks and uncertainties not currently known or that are currently considered to be immaterial may also materially and adversely affect our business. As a result, the trading price or value of our common stock could be materially adversely affected and you may lose all or part of your investment.

Risks Related to our Business

We are a development stage company. Our limited operating history makes it difficult to evaluate our future performance.

We are a development stage company and, as such, we have a limited operating history upon which you can evaluate our current business and our prospects. The likelihood of our future success must be viewed in light of the problems, expenses, difficulties, delays and complications often encountered in the operation of a business without revenues, especially in the pharmaceutical industry, where failures of companies are common. We are subject to the risks inherent in the ownership and operation of a development stage company, including availability of capital, regulatory setbacks and delays, fluctuations in expenses, competition and government regulation. If we fail to address these risks and uncertainties our business, results of operations, financial condition and prospects would be adversely affected.

We have no products currently available and we have never had any products available for commercial sale.

We have had no revenues from product sales to date, currently have no products available for commercial sale, and have never had any products available for commercial sale. We expect to incur losses at least until we are in a position to commercialize Firdapse®, which may never occur. Our net loss was $18.1 million and $20.2 million for the years ended December 31, 2016 and December 31, 2015, respectively, and $5.0 million and $5.4 million for the three months ended March 31, 2017 and March 31, 2016, respectively. We may never obtain approval of an NDA for any of our drug candidates and we may never achieve profitability.

Our business will require additional capital.

Our business will require additional capital to meet our product development objectives. Based on currently available information, we estimate that we have sufficient working capital to support our operations through at least the next 12 months. The expectations described above are based on current information available to us. If the cost of our ongoing activities are greater than we expect, our assumptions may not prove to be accurate. There can be no assurance as to the exact amount of the funding we will require or as to whether any such required funding will be available to us when it is required.

We plan to 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 grants will be available, and, if available, that we will qualify to receive any such grants. We may also seek to raise additional capital to fund additional product development efforts, even if

we have sufficient funds for our planned operations.

Table of Contents

Any sale by us of additional equity or debt securities convertible into additional equity could result in dilution to our stockholders. There can be no assurance that any required additional funding will be available to us at all or available on terms acceptable to us. 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.

If we are not the first to obtain approval for Firdapse® for the treatment of LEMS, we may not be able to bring it to market in the United States.

Another pharmaceutical company, Jacobus Pharmaceutical, has completed its own clinical trial studying their own formulation of amifampridine (3,4-DAP) for the treatment of LEMS. Jacobus Pharmaceutical is a privately held company and there is little public information available about their development plans. While there can be no assurance, we believe that Firdapse® is further along in development and as a result we expect that we will be in a position to obtain the first approval of an NDA for 3,4-DAP. Under the Orphan Drug Act of 1983, the first pharmaceutical product to obtain approval for an indication receives the orphan exclusivity under the statute. If Jacobus Pharmaceutical receives approval of an NDA for its formulation of amifampridine for the treatment of LEMS before we are able to receive approval of Firdapse® for the same indication, we would be barred from marketing Firdapse® in the United States during the seven-year orphan exclusivity period, which would have a severe adverse effect on our results of operations. In addition, if Jacobus Pharmaceutical were to receive five-year new chemical entity exclusivity for amifampridine for any indication prior to approval of Firdapse®, we would be barred from marketing Firdapse® in the United States during this five-year exclusivity period.

The development of CPP-115 is at an early stage.

Our development of CPP-115 is at an early stage, and it is going to be several years before we are in a position to submit an NDA for CPP-115, assuming our future clinical trials of this product are successful. At the present time, there can be no assurance that we will ever submit an NDA for CPP-115 or successfully commercialize CPP-115.

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 our drug candidates. In addition, we compete against biotechnology companies, universities, government agencies, and other research institutions in the development of pharmaceutical products. While we believe that our drug candidates will offer advantages over many of the currently available competing therapies, 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, if we are permitted to commence commercial sales of our drug candidates, we may also compete with respect to manufacturing efficiency and marketing capabilities.

Table of Contents

For example, amifampridine, the active ingredient in Firdapse®, despite not being FDA approved, has been available from compounding pharmacies and from Jacobus Pharmaceutical under compassionate use INDs for many years. Amifampridine from these sources can be expected to be substantially less expensive than Firdapse®. The FDA Pharmacy Compounding Advisory Committee, however, has previously issued a list of drugs which cannot be compounded, and amifampridine was included on that list. 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 with an aggregate annual coverage limit of $15,000,000 per claim and $15,000,000 in the aggregate, with a deductible of $10,000 per occurrence. Our insurance may not reimburse us for certain claims or the coverage may not be sufficient to cover claims made against us. We cannot predict all of the possible harms or side effects that may result from the use of our current drug candidates, or any potential future products we may acquire and use in clinical trials or after FDA approval and, therefore, the amount of insurance coverage we currently hold may not be adequate to cover all liabilities we might incur. If we are sued for any injury allegedly caused by our products, our liability could exceed our ability to pay the liability. Whether or not we are ultimately successful in any adverse litigation, such litigation could consume substantial amounts of our financial and managerial resources, all of which could have a material adverse effect on our business, financial condition, results of operations, prospects and stock price.

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 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 employees, on our Board of Directors and on our scientific advisors. 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, and the consulting agreements we have with several of our scientific advisors, we have no employment or retention agreements with our officers, directors or scientific advisors. If we lose the services of any of our existing officers, directors or scientific advisors, 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.

Table of Contents

We have relationships with our scientific advisors and collaborators at academic and other institutions. Such individuals are employed by entities other than us and may have commitments to, or consulting advisory contracts with, such entities that may limit their availability to us. Although each scientific advisor and collaborator has agreed not to perform services for another person or entity that would create an appearance of a conflict of interest, conflicts may arise from the work in which other scientific advisors and/or collaborators are involved.

Risks Related to the Development of Our Drug Candidates

Our drug development efforts may fail.

Development of our pharmaceutical drug candidates is subject to risks of failure. For example:

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

As a result, our drug development activities may not result in any safe, effective and commercially viable products, and we may not be able to commercialize our products successfully. For example, for several years, we evaluated CPP-109 (our formulation of vigabatrin) for the treatment of cocaine addiction. However, CPP-109 failed to meet the primary and two key secondary endpoints in a Phase 2b trial for cocaine addiction, and we are no longer pursuing the evaluation of CPP-109 for addiction. Further, our lead compound, Firdapse®, is for very rare conditions for which there is no FDA-approved treatment. As such, the clinical development plan we pursued after consulting with FDA including the clinical endpoints, protocol design, and statistical analysis plan, may not allow the FDA to ultimately conclude that our Phase 3 trial of Firdapse® is adequate to establish the clinical benefit of the drug.

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

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

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

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

Table of Contents

conditions may be imposed upon us by the FDA regarding the scope or design of our clinical trials, or we may be required to resubmit our clinical trial protocols to IRBs for review due to changes in the regulatory environment;

the number of subjects required for our clinical trials may be larger, patient enrollment may take longer, or patients may drop out of our clinical trials at a higher rate than we anticipate;

we may have to suspend or terminate one or more of our clinical trials if we, regulators, or IRBs determine that the participants are being subjected to unreasonable health risks;

our third-party contractors, clinical investigators or contractual collaborators may fail to comply with regulatory requirements or fail to meet their contractual obligations to us in a timely manner;

the FDA may not accept clinical data from trials that are conducted at clinical sites in countries where the standard of care is potentially different from the United States;

our tests may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional testing; and

the costs of our pre-clinical and/or clinical trials may be greater than we anticipate.

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

We do not currently have the ability to independently conduct pre-clinical studies or clinical studies and trials for our drug candidates, 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 of our drug candidates. Our reliance on third parties for development activities reduces our control over these activities. These third parties may not complete activities on schedule, or may not conduct our pre-clinical studies and our clinical studies and trials in accordance with regulatory requirements or our study design. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may be adversely affected, and our efforts to obtain regulatory approvals for and commercialize our drug candidates may be delayed.

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

Although we also rely on third parties to manage the data from our studies and trials, we are responsible for confirming that each of our studies and trials is conducted in accordance with its general investigational plan and protocol. Moreover, the FDA and foreign regulatory agencies will require us to comply with applicable regulations and standards, including Good Laboratory Practice (GLP) and Good Clinical Practice (GCP), for conducting, recording and reporting the results of such studies and trials to assure that the data and the results are credible and accurate and that the human study and trial participants are adequately protected. Our reliance on third-parties does

not relieve us of these obligations and requirements, and we may fail to obtain regulatory approval for our drug candidates if these requirements are not met.

Table of Contents

We will need to develop marketing, distribution and production capabilities or relationships to be successful.

In order to generate sales of any products we may develop, we must either acquire or develop an internal marketing force with technical expertise and with supporting documentation capabilities, or make arrangements with third parties to perform these services for us. The acquisition and development of a marketing and distribution infrastructure requires substantial resources and compete for available resources with our drug development efforts. To the extent that we enter into marketing and distribution arrangements with third parties, our revenues will depend on the efforts of others. If we fail to enter into such agreements, or if we fail to develop our own marketing and distribution channels, we would experience delays in product sales and incur increased costs.

We have no in-house manufacturing capacity and, to the extent we are successful in completing the development of our drug candidates, we will be obligated to rely on contract manufacturers. We cannot be sure that we will successfully manufacture any product we may develop, 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 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 potential 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 drug candidates, it could have a material adverse effect on our ability to 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 intend to attempt to source our products from more than one supplier. We also intend 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.

If our NDA for Firdapse® is approved, we will need to manufacture our product in larger quantities than we have in the past to launch the product and meet customer requirements. With respect to our other products, to date they have only been manufactured in small quantities for pre-clinical studies and clinical trials, and, in order to conduct large trials and commercialize these products, we will need to manufacture our products in larger quantities than we have in

the past.

Table of Contents

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.

If we are successful in obtaining approval to commercialize Firdapse® or any of our other drug candidates, we will need to significantly expand our operations, which could put significant strain on our management and our operational and financial resources. We currently have 19 employees and conduct many of our activities through outsourcing arrangements. 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 for any of our drug candidates which we commercialize in the future at prices or on terms sufficient to provide a viable financial outcome.

The commercial success of Firdapse® will 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 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.

Table of Contents

Our ability 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 candidate profitably. These payors may not view our products, if any, 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, if any, 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 specialty drugs, in particular, has been a topic of concern in the U.S. Congress, where hearings on the topic have been held. It has also been a topic raised by President Trump, most recently in a meeting with pharmaceutical industry participants. There can be no assurance as to how this scrutiny on pricing of pharmaceutical products will impact future pricing of orphan drugs or pharmaceutical products generally or our products in particular.

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

There is a vocal group of neuromuscular physicians who have raised public concerns in a letter to the editor of a medical journal and some LEMS patients and neuromuscular physicians who have raised public concerns in interviews quoted in articles published in the press. Their overarching concern appears to be that LEMS patients may not be able to get amifampridine treatment because of the concern that it would be priced too high as an orphan drug if we are the first pharmaceutical company to receive an FDA approval for an amifampridine product, thereby giving us the seven-year orphan drug exclusivity and the five-year new chemical entity exclusivity for our product. Articles about their concerns have been published in several national publications and some in the press have sought to tie their expectations about the anticipated pricing of Firdapse® to stories about perceived abusive price increases of drug

products by other pharmaceutical companies. This vocal group has also questioned the appropriateness of the provisions of the Orphan Drug Act that would grant us exclusivity if our product were to be the first amifampridine product approved by the FDA, and whether this exclusivity should be eliminated from the law. We have responded to their concerns in a letter to the editor to the same medical journal. However, there can be no assurance as to the ultimate impact of the activities of this vocal group on us or our products.

Table of Contents

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® and our other orphan drug candidates target 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. 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®, if approved, because the potential target populations are very small, we may never achieve 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.

Table of Contents

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

We are exposed to the risk of employee or consultant fraud or other misconduct. Misconduct by our employees or consultants could include intentional 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. Employee and consultant 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

We have not received regulatory approval in the United States or any foreign jurisdiction for the commercial sale of any of our drug candidates. The regulatory approval process is lengthy, and we may not be able to obtain all of the regulatory approvals required to manufacture and commercialize our drug candidates.

We do not currently have any products that have been approved for commercialization. We will not be able to commercialize our products until we have obtained the requisite regulatory approvals from applicable governmental authorities. To obtain regulatory approval of a drug candidate, we must demonstrate to the satisfaction of the applicable regulatory agency that such drug candidate is safe and effective for its intended uses. The type and magnitude of the testing required for regulatory approval varies depending on the drug candidate and the disease or condition for which it is being developed. In addition, in the U.S. we must show that the facilities used to manufacture our drug 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 candidates are safe and effective, in which event we would not receive the regulatory approvals required to market them.

The FDA and other regulatory authorities generally approve products for particular indications. Our drug candidates 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 requests additional information.

The FDA and other regulatory bodies must approve trade names for products. The FDA typically conducts a thorough review of a proposed trade name, including an evaluation of potential confusion with other trade names. We have previously submitted a request for FDA approval of the trade name Firdapse®, which request has been conditionally approved.

16

Table of Contents

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

In September 2014, we announced positive results from our first Phase 3 clinical trial for Firdapse®. In October 2016, we announced that we had reached an agreement with the FDA under a SPA for the protocol design, clinical endpoints, and statistical analysis approach to be taken in our ongoing second Phase 3 study evaluating Firdapse® for the symptomatic treatment of LEMS. Even if our second Phase 3 trial of Firdapse® is successful, we may nevertheless fail to meet the safety and efficacy standards required by the FDA to obtain regulatory approval.

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 candidates may not be found to be safe and effective, 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, such as problems associated with Visual Field Defects (VFDs) or other side effects that will cause us, regulatory authorities, or IRBs to delay or suspend such trial or study.

In other countries where Firdapse®, CPP-115 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. 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. 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 considerable 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.

17

Table of Contents

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

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

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

- reliance on the third party for regulatory compliance and quality assurance;
- reliance on the continued financial viability of the third parties;
- limitations on supply availability resulting from capacity and scheduling constraints of the third parties;
- impact on our reputation in the marketplace if manufacturers of our products, once commercialized, 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 drug candidates 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.

Even if we obtain regulatory approvals, our drug candidates will be subject to ongoing regulatory review. If we fail to comply with continuing U.S. and applicable foreign regulations, we could lose those approvals, and our business

would be severely harmed.

Even if we receive regulatory approval of any drugs we are developing or may develop, we will be subject to continuing regulatory review, 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.

Table of Contents

As a condition of approval for some of our products, the FDA might require a Risk Evaluation and Mitigation Strategy (REMS) to help ensure that the benefits of the drug outweigh the potential risks. REMS can include medication guides, communication plans for 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. For example, approved versions of vigabatrin, the active moiety in our CPP-109 product (which operates by the same mechanism of action as our CPP-115 product) were approved with an FDA-mandated REMS program due to the risks of visual field damage and are only available through a special restricted distribution program approved by the FDA. Accordingly, our abbreviated new drug application (ANDA) for vigabatrin, if approved, will be subject to either the same REMS, or a comparable REMS that will need to be reviewed and approved by the FDA. If any of our products were to be approved with a REMS, the potential market and profitability of the drug could be materially affected.

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

Table of Contents

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 U.S. 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 50% discount 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).

Both President Trump and the Republican leadership in Congress have expressed their intention to eliminate the Health Care Reform Law and replace it with a still unknown new law. While proposals have been introduced in Congress, it is still unknown what form any such modifications or any law passed to replace the Health Care Reform Law would take, and how or any such new law may affect our business in the future.

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 U.S. Congress of the FDA s approval process may subject us to more stringent product labeling and post-marketing testing and other requirements. Delays in feedback from the FDA may affect our ability to quickly update or adjust our label in the

interest of patient adherence and tolerability. We cannot predict whether other legislative changes will be adopted or how such changes would affect the pharmaceutical industry generally and specifically the commercialization of Firdapse®.

Table of Contents

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 U.S., orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and user-fee waivers. The company that first obtains FDA approval for a designated orphan drug for a given rare disease receives marketing exclusivity for use of that drug for the stated 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.

In the EU, the EMA s Committee for Orphan Medicinal Products, or COMP, grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the EU Community and for which no satisfactory method of diagnosis, prevention, or treatment has been authorized (or the product would be a significant benefit to those affected). Additionally, designation is granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the EU would be sufficient to justify the necessary investment in developing the medicinal product. An EU orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and 10 years of market exclusivity is granted following medicinal product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Orphan drug designation must be requested before submitting an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

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

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

21

Table of Contents

Breakthrough Therapy Designation may not actually lead to a faster review process.

Under the Prescription Drug User Fee Act, the FDA has a goal of responding to NDAs for new molecular entities within 10 months of the date that the FDA files the NDA for standard review, but this timeframe is also often extended. We have in the past and we may in the future, seek approval of our drug candidates under programs designed to accelerate the FDA s review and approval of NDAs. For example, there is a category of drugs referred to as breakthrough therapies, which are defined as drugs intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. In our case, Firdapse® has been granted breakthrough therapy designation for the treatment of LEMS. In the future, we may request breakthrough designation or fast track designation from the FDA for our other drug candidates or for treatment of other diseases, but we cannot assure that we will obtain such designations.

Moreover, even if we obtain breakthrough designation or fast track designation from the FDA, the designations do not guarantee FDA approval of our NDA, that the development program or review timeline will ultimately be shorter than if we had not obtained the designations, or that the FDA will not request additional information, including requesting additional clinical studies (although potentially a post-marketing requirement), during its review. Any request for additional information or clinical data could delay the FDA s timely review of our NDA.

Even though our second Phase 3 study is being conducted under a Special Protocol Assessment, or 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.

Risks Related to Our Intellectual Property

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

All of our patent rights for Firdapse® are derived from our license agreement with BioMarin. Pursuant to this license agreement, we have licensed rights under BioMarin s Firdapse® patent applications in the United States, which expire in 2022 and 2034. 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 BioMarin. If we violate or fail to perform any term or covenant of the license agreement, BioMarin 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 BioMarin, 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.

Table of Contents

Most of our patent rights for CPP-115 are derived from our license agreement with Northwestern University. Pursuant to this license agreement, we have exclusive worldwide rights to two patents in the United States. These were filed and obtained by Northwestern relating to compositions of matter for a class of molecules, including CPP-115. Both patents expire in 2023. Additionally, we have licensed rights from Northwestern to a pending patent for derivatives of vigabatrin that are unrelated to CPP-115. These rights are subject to the right of Northwestern, under limited circumstances, to practice the covered inventions for or on its own behalf for research. 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, including milestone payments, to Northwestern. If we violate or fail to perform any term or covenant of the license agreement, Northwestern 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 Northwestern, 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 CPP-115, and our business, results of operations, financial condition and prospects would be materially adversely affected.

If we obtain approval to market Firdapse® or CPP-115, our commercial success will depend in large part on our ability to use patents, especially those licensed to us by BioMarin and Northwestern, respectively, 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 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 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 our patents or design around our patents so as to compete with us without infringing our patents. Moreover, the issuance of a patent is not conclusive as to its validity or enforceability, and so our patents 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.

While we are not currently aware of any third-party patents which we may infringe, there can be no assurance that we do not or will not infringe on patents held by third parties or that third parties will not claim that we have infringed on their patents. In the event that our 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. There may be patents held by others of which we are unaware that contain claims that our products or operations infringe. In addition, given the complexities and uncertainties of patent laws, there may be patents of which we are aware that we may ultimately be held to infringe, particularly if the claims of the patent are determined to be broader than we believe them to be. Adding to this uncertainty, in the U.S., patent applications filed in recent years are confidential for 18 months, while older applications are not publicly available until the patent issues. As a result, avoiding patent infringement may be difficult.

Table of Contents

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

- we may be required to pay substantial financial damages if a court decides that our technologies infringe a competitor s patent, which can be tripled if the infringement is deemed willful, or be required to discontinue or significantly delay development, marketing, selling and licensing of the affected products and intellectual property rights;

- a court may prohibit us from selling or licensing our product without 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 have to redesign our product so that it does not infringe others patent rights, which may not be possible or could require substantial funds or time and require additional studies.

In addition, employees, consultants, contractors and others may use the proprietary information of others in their work for us or disclose our proprietary information to others. As an example, we do not currently have written agreements regarding confidentiality with several principal members of our Scientific Advisory Board. 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 others 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.

24

Table of Contents

In addition, if third parties file patent applications or issue patents claiming technology that is also claimed by us in pending applications, we may be required to participate in interference proceedings with the U.S. Patent Office or in other proceedings outside the U.S., including oppositions, to determine priority of invention or patentability. 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 and this Offering

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;
- 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 licenses from BioMarin and Northwestern), 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 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.

Table of Contents

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. Further, at the 2016 annual meeting of stockholders, the stockholders approved the Rights Plan.

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, 2019, unless the rights are earlier redeemed or exchanged.

Table of Contents

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 July 20, 2017, we had 84,554,979 shares of our common stock outstanding, of which 6,105,123 shares were held by our officers and directors. We also had outstanding: (i) common stock purchase warrants to purchase 675,000 additional shares of our common stock at an exercise price of $2.08 per share, (ii) stock options to purchase an aggregate of 6,090,000 shares at exercise prices ranging from $0.47 to $4.64 per share (3,064,996 of which are currently exercisable), and (iii) 26,667 restricted stock units that are subject to vesting. Sales of restricted shares or shares underlying stock options and common stock purchase warrants, 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.

There is no market for the other securities that may be offered under this prospectus

While our common stock is traded on the NASDAQ Capital Market under the symbol CPRX , there is no market for the other securities we may offer pursuant to this prospectus, and there is no assurance that such a market will develop if we were to issue such securities. Consequently, investors may not be able to resell any such securities purchased should they need or wish to do so.

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.

Table of Contents

CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS

This Registration Statement on Form S-3 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 i identify forward-looking statements. Such statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by such forward looking statements. The forward-looking statements made in this prospectus are based on current expectations that involve numerous risks and uncertainties.

The successful development and commercialization of our current drug candidates is highly uncertain. We cannot reasonably estimate or know the nature, timing, or estimated expenses of the efforts necessary to complete the development of, or the period in which material net cash inflows are expected to commence due to the numerous risks and uncertainties associated with developing such products, including the uncertainty of:

- our estimates regarding anticipated capital requirements and our need for additional funding;
- the risk that another pharmaceutical company will receive an approval for its formulation of 3,4-diaminopyridine (3,4-DAP) for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS), Congenital Myasthenic Syndromes (CMS), or any other indication, before we do;
- whether the clinical studies or trials that are required to be completed before the FDA will accept an NDA submission for Firdapse® for the treatment of either LEMS will be successful;
- what additional supporting information, including any additional clinical studies or trials, will be required before the FDA will accept our NDA submission for Firdapse® for the treatment of either LEMS or CMS (or any other condition or disease);
- whether any NDA that we may submit for Firdapse® will be accepted for filing by the FDA, and if accepted, whether it will be granted a priority review;
- whether, even if the FDA accepts an NDA submission for Firdapse®, such product will be determined to be safe and effective and approved for commercialization for any of the submitted indications;
- whether the receipt of breakthrough therapy designation for Firdapse® for LEMS will result in an expedited review of Firdapse® by the FDA or affect the likelihood that the product will be found to be safe and effective;

whether as part of the FDA review of any NDA that we may submit for filing for Firdapse®, the tradename Firdapse®, which is the tradename used for the same product in Europe, will be approved for use for the product in the United States;

whether, assuming Firdapse® is approved for commercialization, we will be able to develop or contract with a sales and marketing organization that can successfully market Firdapse® while maintaining full compliance with applicable federal and state laws, rules and regulations;

whether any future trial that we undertake evaluating Firdapse® for the treatment of MuSK-MG will be successful and whether we can obtain the funding required to conduct such trial;

whether CPP-115 will be determined to be safe for humans;

Table of Contents

whether CPP-115 will be determined to be effective for the treatment of infantile spasms, Tourette s Disorder, or any other indication;

whether we can successfully design and complete a bioequivalence study of our version of vigabatrin compared to Sabril® that is acceptable to the FDA;

whether any ANDA that we submit for a generic version of Sabril® will be accepted by the FDA for review and approved (and the timing of any such approval);

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;

our ability to complete our trials and studies on a timely basis and within the budgets we establish for such trials and studies and whether our trials and studies will be successful;

the ability of our third-party suppliers and contract manufacturers to maintain compliance with cGMP;

whether our estimates of the size of the market for our drug candidates will turn out to be accurate;

the pricing of our products that we may be able to achieve if we are granted the ability to commercialize our drug candidates; and

changes in 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 in the healthcare industry generally.

Our current plans and objectives are based on assumptions relating to the development of our current drug candidates. 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 prospectus, 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.

RATIO OF EARNINGS TO FIXED CHARGES

The following table sets forth our ratio of earnings to fixed charges on a historical basis for each of the periods indicated. You should read these ratios in conjunction with our financial statements, including the notes to those financial statements, incorporated by reference in this prospectus.

December 31, 2012

	December 31, 2013	Year Ended December 31, 2014	December 31, 2015	December 31, 2016	Three Months Ended March 31, 2017
Ratio of earnings to fixed charges					

As of the date of this prospectus, we have no shares of preferred stock outstanding that require us to accrue or pay dividends, and consequently, our ratio of earnings to preferred share dividends and our ratio of earnings to fixed charges are identical.

Table of Contents

USE OF PROCEEDS

Except as may otherwise be provided in a prospectus supplement, we will use the net proceeds from sales of securities to fund non-clinical studies and clinical studies with respect to our product candidates, for manufacturing and marketing purposes for any product candidate which we may commercialize, and for general working capital purposes. When particular securities are offered, the prospectus supplement relating to that offering will set forth our intended use of the net proceeds received from the sale of those securities.

Pending the application of the net proceeds for these purposes, we expect to invest the proceeds in short-term, interest-bearing instruments or other investment-grade securities.

Table of Contents

DETERMINATION OF OFFERING PRICE

Our common stock trades on the Nasdaq Capital Market under the symbol CPRX. The following table sets forth the high and low closing sales prices per share of our common stock as reported on the Nasdaq Capital Market for the period indicated.

	High	Low
Year Ended December 31, 2015		
First Quarter	$ 4.93	$ 2.74
Second Quarter	$ 4.73	$ 3.16
Third Quarter	$ 5.74	$ 3.00
Fourth Quarter	$ 3.50	$ 2.33
Year Ended December 31, 2016		
First Quarter	$ 2.36	$ 1.01
Second Quarter	$ 1.25	$ 0.56
Third Quarter	$ 1.25	$ 0.72
Fourth Quarter	$ 1.46	$ 0.96
Year Ending December 31, 2017		
First Quarter	$ 2.01	$ 1.09
Second Quarter	$ 2.84	$ 1.64
Third Quarter (Through July 20, 2017)	$ 3.12	$ 2.67

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.

There is no public market for any securities we may offer pursuant to this prospectus other than our common stock. The principal factors to be considered in determining the offering price for any securities we sell pursuant to this prospectus includes:

- The information set forth in this prospectus and otherwise available to the market;
- our history and prospects and the history and prospects for the industry in which we compete;
- our past and present financial performance;
- our prospects for future earnings and the present state of our development;
- the general condition of the securities market at the time of the offering;

the recent market prices of, and demand for, similar publicly traded securities of generally comparable companies; and

other factors we may deem relevant.

Table of Contents

DILUTION

Information on the potential dilutive effects of any offering of common stock we may make under this prospectus will be set forth in the relevant prospectus supplement for such offering.

PLAN OF DISTRIBUTION

We may sell the securities from time-to-time pursuant to underwritten public offerings, negotiated transactions, block trades or a combination of these methods. We may sell the securities: (1) through underwriters or dealers, (2) through agents, and/or (3) directly to one or more purchasers. If we sell shares of our common stock for less than the market value, we may only sell an amount equal to 20% of our outstanding common stock without stockholder approval. Further, if our public float (the market value of the common stock held by our non-affiliate stockholders) goes below $75 million, we will also be subject to a limitation that we may sell no more than one third (1/3) of our public float during any 12-month period.

We may distribute the securities from time to time in one or more transactions at:

a fixed price or prices, which may change;

market prices prevailing at the time of sale;

prices relating to the prevailing market prices;

varying prices determined at the time of sale; or

negotiated prices.

The applicable prospectus supplement with respect to a particular offering of securities will describe the terms of the offering of the securities, including:

the name or names of any underwriters, and if required, any dealers or agents;

the purchase price of the securities and the proceeds we will receive from the sale;

the terms of the securities that we sell, including any convertibility features of such securities;

any underwriting discounts and other items constituting underwriters compensation;

any discounts or concessions allowed or reallowed or paid to dealers; and

any securities exchange or market on which the securities may be listed.

We may solicit directly offers to purchase the securities being offered by this prospectus. We may also designate agents to solicit offers to purchase the securities from time to time. We will name in a prospectus supplement any agent involved in the offer or sale of our securities.

If we utilize a dealer in the sale of the securities offered by this prospectus, we will sell the securities to the dealer, as principal. The dealer may then resell the securities to the public at varying prices to be determined by the dealer at the time of resale. If we utilize an underwriter in the sale of the securities being offered by this prospectus, we will execute an underwriting agreement with the underwriter at the time of sale and we will provide the name of any underwriter in the prospectus supplement which the underwriter will use to make resales of the securities to the public. In connection with the sale of the securities, we, or the purchasers of the securities for whom the underwriter may act as agent, may compensate the underwriter in the form of underwriting discounts or commissions. The underwriter may sell the securities to or through dealers, and the underwriter may compensate those dealers in the form of discounts, concessions or commissions.

Table of Contents

With respect to underwritten public offerings, negotiated transactions and block trades, we will provide in the applicable prospectus supplement any compensation we pay to underwriters, dealers or agents in connection with the offering of the securities, and any discounts, concessions or commissions allowed by underwriters to participating dealers. Underwriters, dealers and agents participating in the distribution of the securities may be deemed to be underwriters within the meaning of the Securities Act of 1933, as amended, and any discounts and commissions received by them and any profit realized by them on resale of the securities may be deemed to be underwriting discounts and commissions. We may enter into agreements to indemnify underwriters, dealers and agents against civil liabilities, including liabilities under the Securities Act of 1933, as amended, or to contribute to payments they may be required to make in respect thereof.

To facilitate the offering of our securities, certain persons participating in the offering may engage in transactions that stabilize, maintain or otherwise affect the price of the securities. This may include over allotments or short sales of the securities, which involve the sale by persons participating in the offering of more securities than we sold to them. In these circumstances, these persons would cover such over allotments or short positions by making purchases in the open market or by exercising their over allotment option. In addition, these persons may stabilize or maintain the price of the securities by bidding for or purchasing securities in the open market or by imposing penalty bids, whereby selling concessions allowed to dealers participating in the offering may be reclaimed if securities sold by them are repurchased in connection with stabilization transactions. The effect of these transactions may be to stabilize or maintain the market price of the securities at a level above that which might otherwise prevail in the open market. These transactions may be discontinued at any time.

The underwriters, dealers and agents may engage in other transactions with us, or perform other services for us, in the ordinary course of their business.

GENERAL DESCRIPTION OF OUR CAPITAL STOCK

Our authorized capital currently consists of 150,000,000 shares of common stock, par value $0.001 per share, and 5,000,000 shares of preferred stock, par value $0.001 per share. As of the date of this prospectus, we had 84,554,979 shares of our common stock outstanding. There are no shares of preferred stock outstanding. As of the date of this prospectus, 1,500,000 shares of our preferred stock are reserved pursuant to our Stockholder Rights Plan. See Provisions of the Certificate and Bylaws Issuance of Rights .

We are a Delaware corporation, and were incorporated on July 24, 2006. We are the successor by merger to Catalyst Pharmaceutical Partners, Inc., a Florida corporation, which was incorporated in January 2002.

Common Stock

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

Table of Contents

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

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

Preferred Stock

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

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

security, the conversion price or prices or rate or rates and provisions for any adjustments to such prices or rates, the date or dates as of which the shares will be convertible, and all other terms and conditions upon which the conversion may be made;

the ranking of such series with respect to dividends and amounts payable on our liquidation, dissolution or winding-up, which may include provisions that such series will rank senior to our common stock with respect to dividends and those distributions;

restrictions on the issuance of shares of the same series or any other class or series; or

voting rights, if any, of the holders of the series.

Table of Contents

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

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

- the title and stated value of the series of preferred stock and the number of shares constituting that series;
- the number of shares of the series of preferred stock offered, the liquidation preference per share and the offering price of the shares of preferred stock;
- the dividend rate(s), period(s) and/or payment date(s) or the method(s) of calculation for those values relating to the shares of preferred stock of the series;
- the date from which dividends on shares of preferred stock of the series shall cumulate, if applicable;
- our right, if any, to defer payment of dividends and the maximum length of any such deferral period;
- the procedures for any auction and remarketing, if any, for shares of preferred stock of the series;
- the provision for redemption or repurchase, if applicable, of shares of preferred stock of the series;
- any listing of the series of shares of preferred stock on any securities exchange;
- the terms and conditions, if applicable, upon which shares of preferred stock of the series will be convertible into shares of preferred stock of another series or common stock, including the conversion price, or manner of calculating the conversion price;
- whether the preferred stock will be exchangeable into debt securities, and, if applicable, the exchange period, the exchange price, or how it will be calculated, and under what circumstances it may be adjusted;
- voting rights, if any, of the preferred stock;

restrictions on transfer, sale or other assignment, if any;

whether interests in shares of preferred stock of the series will be represented by global securities;

any other specific terms, preferences, rights, limitations or restrictions of the series of shares of preferred stock;

a discussion of any material United States federal income tax consequences of owning or disposing of the shares of preferred stock of the series;

the relative ranking and preferences of shares of preferred stock of the series as to dividend rights and rights upon liquidation, dissolution or winding up of our affairs; and

any limitations on issuance of any series of shares of preferred stock ranking senior to or on a parity with the series of shares of preferred stock as to dividend rights and rights upon liquidation, dissolution or winding up of our affairs.

Table of Contents

If we issue shares of preferred stock under this prospectus, the shares will be fully paid and nonassessable and will not have, or be subject to, any preemptive or similar rights.

Provisions of the Certificate and Bylaws

A number of provisions of our certificate of incorporation and bylaws concern matters of corporate governance and the rights of stockholders. Certain of these provisions, as well as the ability of our board of directors to issue shares of preferred stock and to set the voting rights, preferences and other terms thereof, may be deemed to have an anti-takeover effect and may discourage takeover attempts not first approved by the board of directors (including takeovers which certain stockholders may deem to be in their best interests). To the extent takeover attempts are discouraged, temporary fluctuations in the market price of the common stock, which may result from actual or rumored takeover attempts, may be inhibited. These provisions, together with the ability of the board to issue preferred stock without further stockholder action, also could delay or frustrate the removal of incumbent directors or the assumption of control by stockholders, even if such removal or assumption would be beneficial to our stockholders. These provisions also could discourage or make more difficult a merger, tender offer or proxy contests, even if they could be favorable to the interests of stockholders, and could potentially depress the market price of the common stock. The board of directors believes that these provisions are appropriate to protect our interest and the interests of our stockholders.

Issuance of Rights. On September 20, 2011, the Board of Directors approved the adoption of a stockholder rights plan, which was amended on September 19, 2016. Further, at the 2017 Annual meeting of Stockholders, the Company s stockholders approved the rights plan.

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, 2019, unless the rights are earlier redeemed or exchanged.

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

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

Table of Contents

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

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

Certain Anti-Takeover Matters

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

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

Section 203 defines business combination to include the following:

- any merger or consolidation involving the corporation and the interested stockholder;

any sale, transfer, pledge or other disposition of 10% or more of the assets of the corporation involving the interested stockholder;

subject to certain exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;

Table of Contents

any transaction involving the corporation that has the effect of increasing the proportionate share of the stock of any class or series of the corporation beneficially owned by the interested stockholder; or

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

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

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

Limitation of Liability and Indemnification Matters

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

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

any breach of a director s duty of loyalty to us or our stockholders;

acts or omissions not in good faith or which involve intentional misconduct or a knowing violation of law;

unlawful payment of dividends or unlawful stock repurchases or redemptions; or

any transaction from which the director derived an improper personal benefit.

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

fees), judgments, fines and amounts paid in settlement actually and reasonably incurred by such person in connection with such action, suit or proceeding.

Table of Contents

Listing

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

Transfer Agent and Registrar

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

DESCRIPTION OF DEBT SECURITIES

The following description, together with the additional information we may include in any applicable prospectus supplement and in any related free writing prospectuses, summarizes the material terms and provisions of the debt securities that we may offer under this prospectus. While the terms summarized below will apply generally to any debt securities that we may offer, we will describe the particular terms of any debt securities in more detail in the applicable prospectus supplement. The terms of any debt securities offered under a prospectus supplement may differ from the terms described below.

When describing any debt securities, references to issuer refers to Catalyst Pharmaceuticals, Inc.

We have filed as an exhibit to the registration statement, of which this prospectus is a part, the form of indenture pursuant to which the debt securities will be issued and will file as exhibits to the registration statement of which this prospectus is a part, or will incorporate by reference from reports that we file with the SEC, the form of debt security that describes the terms of the particular debt securities we are offering before the issuance of the related debt securities. We may issue debt securities from time to time in one or more distinct series. The debt securities may be senior debt securities or subordinated debt securities. Senior debt securities may be issued under a senior indenture and subordinated debt securities may be issued under a subordinated indenture. If we issue debt securities pursuant to an indenture, we will specify the trustee under such indenture in the applicable prospectus supplement. We will include in a supplement to this prospectus the specific terms of debt securities being offered, including the terms, if any, on which debt securities may be convertible into or exchangeable for common stock or other debt securities. The statements and descriptions in this prospectus or in any prospectus supplement regarding provisions of debt securities and any indentures are summaries of those provisions, do not purport to be complete and are subject to, and are qualified in their entirety by reference to, all of the provisions of the debt securities and the indentures (including any amendments or supplements we may enter into from time to time which are permitted under the debt securities or any indenture).

Unless otherwise specified in a prospectus supplement, the debt securities will be our direct unsecured obligations. Any debt securities designated as senior will rank equally with any of our other senior and unsubordinated debt. Any debt securities designated as subordinated will be subordinate and junior in right of payment to any senior indebtedness. There may be subordinated debt securities that are senior or junior to other series of subordinated debt securities.

The payment obligations of the issuer under any series of debt securities may be guaranteed by one or more of our direct or indirect subsidiaries (if in the future we have any subsidiaries). If a series of debt securities is so guaranteed, the guarantors will execute the applicable indenture, a supplemental indenture or a notation of guarantee as further evidence of their guarantee. The applicable prospectus supplement will describe the terms of any guarantee.

39

Table of Contents

The obligations of each guarantor under its guarantee may be limited to the maximum amount that will not result in such guarantee obligations constituting a fraudulent conveyance or fraudulent transfer under federal or state law, after giving effect to all other contingent and fixed liabilities of that subsidiary and any collections from or payments made by or on behalf of any other guarantor in respect to its obligations under its guarantee.

The applicable prospectus supplement will set forth the terms of the debt securities or any series thereof, including, if applicable:

- the title of the debt securities and whether the debt securities will be senior debt securities or subordinated debt securities;
- any limit upon the aggregate principal amount of the debt securities;
- the date or dates on which the principal amount of the debt securities will mature;
- if the debt securities bear interest, the rate or rates at which the debt securities bear interest, or the method for determining the interest rate, and the date or dates from which interest will accrue;
- if the debt securities bear interest, the dates on which interest will be payable, or the method for determining such dates, and the regular record dates for interest payments;
- any optional redemption provisions, which would allow us to redeem the debt securities in whole or in part;
- any sinking fund or other provisions that would obligate us to redeem, repay or purchase the debt securities;
- the denominations in which any registered securities will be issuable, if other than denominations of $1,000 and any integral multiple thereof;
- if other than the entire principal amount, the portion of the principal amount of debt securities which will be payable upon a declaration of acceleration of the maturity of the debt securities;
- the events of default and covenants relevant to the debt securities, including, the inapplicability of any event of default or covenant set forth in the indenture relating to the debt securities, or the applicability of any other events of defaults or covenants in addition to the events of default or covenants set forth in the indenture relating to the debt securities;

the name and location of the corporate trust office of the applicable trustee under the indenture for such debt securities;

if the debt securities are to be payable, at our election or the election of a holder of the debt securities, in a currency other than that in which the debt securities are denominated or stated to be payable, the terms and conditions upon which that election may be made, and the time and manner of determining the exchange rate between the currency in which the debt securities are denominated or stated to be payable and the currency in which the debt securities are to be so payable;

if the debt securities are issuable as indexed securities, the manner in which the amount of payments of principal, any premium and interest will be determined;

Table of Contents

if the debt securities do not bear interest, the dates on which we will furnish to the applicable trustee the names and addresses of the holders of the debt securities;

any provisions for the satisfaction and discharge or defeasance or covenant defeasance of the indenture under which the debt securities are issued;

the date as of which any bearer securities and any global security will be dated if other than the date of original issuance of the first debt security of a particular series to be issued;

whether and under what circumstances we will pay additional amounts to non-United States holders in respect of any tax assessment or government charge;

whether the debt securities will be issued in whole or in part in the form of a global security or securities and, in that case, any depositary and global exchange agent for the global security or securities, whether the global form shall be permanent or temporary;

if debt securities are to be issuable initially in the form of a temporary global security, the circumstances under which the temporary global security can be exchanged for definitive debt securities and whether the definitive debt securities will be registered securities and provisions relating to the payment of interest in respect of any portion of a global security payable in respect of an interest payment date prior to the exchange date;

the extent and manner to which payment on or in respect of debt securities will be subordinated to the prior payment of our other liabilities and obligations;

whether payment of any amount due under the debt securities will be guaranteed by one or more guarantors;

whether the debt securities will be secured or unsecured;

whether the debt securities will be convertible and the terms of any conversion provisions;

the forms of the debt securities;

a discussion of any material United States federal income tax consequences of owning and disposing of the debt securities; and

any other terms of the debt securities, which terms shall not be inconsistent with the requirements of the Trust Indenture Act of 1939, as amended.

This prospectus is part of a registration statement that provides that we may issue debt securities from time to time in one or more series under one or more indentures, in each case with the same or various maturities, at par or at a discount. Unless indicated in a prospectus supplement, we may issue additional debt securities of a particular series without the consent of the holders of the debt securities of such series outstanding at the time of the issuance. Any such additional debt securities, together with all other outstanding debt securities of that series, will constitute a single series of debt securities under the applicable indenture.

We intend to disclose any restrictive covenants for any issuance or series of debt securities in the applicable prospectus supplement.

Table of Contents

DESCRIPTION OF WARRANTS

We may issue warrants for the purchase of common stock in one or more series. We may issue warrants independently or together with common stock, preferred stock, or debt securities, and the warrants may be attached to or separate from these securities. While the terms summarized below will apply generally to any warrants that we may offer, we will describe the particular terms of any series of warrants in more detail in the applicable prospectus supplement. The terms of any warrants offered under a prospectus supplement may differ from the terms described below.

We will file as exhibits to the registration statement of which this prospectus is a part, or will incorporate by reference from reports that we file with the SEC, the form of warrant agreement that describes the terms of the particular series of warrants we are offering before the issuance of the related series of warrants. The following summaries of material provisions of the warrants and the warrant agreements are subject to, and qualified in their entirety by reference to, all the provisions of the warrant agreement and warrant certificate applicable to the particular series of warrants that we may offer under this prospectus. We urge you to read the applicable prospectus supplements related to the particular series of warrants that we may offer under this prospectus, as well as any related free writing prospectuses, and the complete warrant agreements and warrant certificates that contain the terms of the warrants.

General

We will describe in the applicable prospectus supplement the terms of the series of warrants being issued, including:

- the offering price and aggregate number of warrants offered;
- if applicable, the designation and terms of the securities with which the warrants are issued and the number of warrants issued with each security or each principal amount of such security;
- if applicable, the date on and after which the warrants and the related securities will be separately transferable;
- the number of shares of common stock exercisable upon the exercise of one warrant and the price at which these shares may be purchased upon such exercise;
- the effect of any merger, consolidation, sale or other disposition of our business on the warrant agreements and the warrants;
- the terms of any rights to redeem or call the warrants;
- any provisions for changes to or adjustments in the exercise price or number of securities issuable upon exercise of the warrants;

the dates on which the right to exercise the warrants will commence and expire;

the manner in which the warrant agreements and warrants may be modified;

a discussion of any material or special United States income tax consequences of holding or exercising the warrants;

Table of Contents

the terms of the securities issuable upon exercise of the warrants; and

any other specific terms, preferences, rights, limitations or restrictions on the warrants.

Before exercising their warrants, holders of warrants will not have any of the rights of holders of the securities upon such exercise, including the right to receive dividends, if any, or payments upon our liquidation, dissolution or winding up or to exercise voting rights, if any.

Exercise of Warrants

Each warrant will entitle the holder to purchase the securities that we specify in the applicable prospectus supplement at the exercise price that we describe in the applicable prospectus supplement. Unless we otherwise specify in the applicable prospectus supplement, holders of the warrants may exercise the warrants at any time up to the specified time on the expiration date that we set forth in the applicable prospectus supplement. After the close of business on the expiration date, unexercised warrants will become void.

Holders of the warrants may exercise the warrants by delivering the warrant certificate representing the warrants to be exercised together with specified information, and paying the required amount to the warrant agent in immediately available funds, as provided in the applicable prospectus supplement. We will set forth on the reverse side of the warrant certificate and in the applicable prospectus supplement the information that the holder of the warrant will be required to deliver to the warrant agent.

Upon receipt of the required payment and the warrant certificate properly completed and duly executed at the corporate trust office of the warrant agent or any other office indicated in the applicable prospectus supplement, we will issue and deliver the securities purchasable upon such exercise. If fewer than all of the warrants represented by the warrant certificate are exercised, then we will issue a new warrant certificate for the remaining amount of warrants. If we so indicate in the applicable prospectus supplement, holders of the warrants may surrender securities as all or part of the exercise price for warrants.

Governing Law

Unless we provide otherwise in the applicable prospectus supplement, the warrants and warrant agreements will be governed by and construed in accordance with the laws of the State of New York.

Enforceability of Rights by Holders of Warrants

Each warrant agent will act solely as our agent under the applicable warrant agreement and will not assume any obligation or relationship of agency or trust with any holder of any warrant. A single bank or trust company may act as warrant agent for more than one issue of warrants. A warrant agent will have no duty or responsibility in case of any default by us under the applicable warrant agreement or warrant, including any duty or responsibility to initiate any proceedings at law or otherwise, or to make any demand upon us. Any holder of a warrant may, without the consent of the related warrant agent or the holder of any other warrant, enforce by appropriate legal action its right to exercise, and receive the securities purchasable upon exercise of, its warrants.

Table of Contents

DESCRIPTION OF UNITS

We may issue, in one or more series, units consisting of common stock and/or warrants for the purchase of common stock in any combination. Each unit will be issued so that the holder of the unit is also the holder of each security included in the unit. Thus, the holder of a unit will have the rights and obligations of a holder of each included security. The units may be issued under unit agreements to be entered into between us and a unit agent, as detailed in the prospectus supplement relating to the units being offered. The prospectus supplement will describe:

- the designation and terms of the units and the securities comprising the units, including whether and under what circumstances the securities comprising the units may be held or transferred separately;
- a description of the terms of any unit agreement governing the units;
- a description of the provisions for the payment, settlement, transfer and exchange of the units; and
- whether the units, if issued as a separate security, will be issued in fully registered or global form.

While the terms summarized above will apply generally to any units that we may offer, we will describe the particular terms of any series of units in more detail in the applicable prospectus supplement. The terms of any units offered under a prospectus supplement may differ from the terms described above. We will file as exhibits to the registration statement of which this prospectus is a part, or will incorporate by reference from reports that we file with the SEC, any form of unit agreement, including any related agreements or certificates, that describes the terms of the particular series of units we are offering before the issuance of the related series of units. The material provisions of the units and any unit agreements are subject to, and qualified in their entirety by reference to, all the provisions of the unit agreement and related agreements and certificates applicable to the particular series of units that we may offer under this prospectus. We urge you to read the applicable prospectus supplements related to the particular series of units that we may offer under this prospectus, as well as any related free writing prospectuses, and the complete unit agreements and related agreements and certificates that contain the terms of the units.

LEGAL MATTERS

Akerman LLP, Miami, Florida, has rendered an opinion with respect to the validity of the securities covered by this prospectus. Certain partners and employees of that firm beneficially own shares or options to acquire shares of our common stock.

EXPERTS

The audited financial statements and management s assessment of the effectiveness of internal control over financial reporting incorporated by reference in this prospectus and elsewhere in the registration statement have been so incorporated by reference in reliance upon the reports of Grant Thornton LLP, independent registered public accountants, upon the authority of said firm as experts in accounting and auditing.

WHERE YOU CAN FIND ADDITIONAL INFORMATION

We file annual, quarterly and special reports, proxy statements and other information with the SEC. Our SEC filings are available to the public over the SEC s website at http://www.sec.gov. You may also read and copy any document we file at the SEC s Public Reference Room at 100 F Street, N.E., Washington, D.C. 20549. Please call the SEC at (800) SEC-0330 for further information on the operating rules and procedures for the public reference room.

Table of Contents

INCORPORATION OF INFORMATION BY REFERENCE

The SEC allows us to incorporate by reference into this prospectus the information we have filed with the SEC. The information we incorporate by reference into this prospectus is an important part of this prospectus. Any statement in a document we incorporate by reference into this prospectus will be considered to be modified or superseded to the extent a statement contained in this prospectus or any other subsequently filed document that is incorporated by reference into this prospectus modifies or supersedes that statement. The modified or superseded statement will not be considered to be a part of this prospectus, except as modified or superseded.

We incorporate by reference into this prospectus the information contained in the documents below, which is considered to be a part of this prospectus:

- our Annual Report on Form 10-K for the year ended December 31, 2016, filed with the SEC on March 15, 2017;
- our Quarterly Report on Form 10-Q for the quarter ended March 31, 2017, filed with the SEC on May 10, 2017;
- our Current Reports on Form 8-K (or amendments thereto) filed with the SEC on March 15, 2017, March 15, 2017, May 10, 2017, May 26, 2017 and July 12, 2017;
- our description of our common stock contained in our Registration Statement on Form 8-A, filed with the SEC on September 29, 2006, along with Amendment No. 1 thereto, filed with the SEC on October 18, 2006; and

We also incorporate by reference into this prospectus all documents (other than current reports furnished under Item 2.02 or Item 7.01 of Form 8-K and exhibits filed on such form that are related to such items) that are filed by us with the SEC pursuant to Sections 13(a), 13(c), 14 or 15(d) of the Exchange Act (i) after the date of the initial filing of the registration statement of which this prospectus forms a part and prior to effectiveness of the registration statement, or (ii) after the date of this prospectus but prior to the termination of the offering. These documents include periodic reports, such as Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q and Current Reports on Form 8-K, as well as proxy statements.

You may obtain a copy of any of these documents at no cost by requesting them from us or by writing or calling: Catalyst Pharmaceuticals, Inc., 355 Alhambra Circle, Suite 1250, Coral Gables, Florida, 33134, Attn: Investor Relations, or by calling (305) 420-3200. Copies of each of these filings are also available for no cost on our website, www.catalystpharma.com, or on the SEC s web site, www.sec.gov.

DISCLOSURE OF COMMISSION POSITION ON INDEMNIFICATION

FOR SECURITIES ACT LIABILITIES

Insofar as indemnification for liabilities arising under the Securities Act of 1933 may be permitted to directors, officers or persons controlling the registrant pursuant to the foregoing provisions, the registrant has been informed that

in the opinion of the Securities and Exchange Commission such indemnification is against public policy as expressed in the Act and is therefore unenforceable.

Table of Contents

14,285,715 Shares

Common Stock

PROSPECTUS SUPPLEMENT

Piper Jaffray **SunTrust Robinson Humphrey**

H.C. Wainwright & Co. **Roth Capital Partners**

November 28, 2017