

CATALYST PHARMACEUTICALS, INC.

Form 424B3

August 27, 2015

Table of Contents

**Filed Pursuant to Rule 424(b)(3)  
Registration No. 333-180617**

**Prospectus**

**968,750 Shares of Common Stock Issuable**

**Upon Exercise of Outstanding Warrants**

We are offering up to 968,750 shares of our common stock that are issuable for a purchase price of \$1.04 per share from time to time upon exercise of currently outstanding five-year warrants that we issued in May 2012 as part of a public offering of common stock and warrants. No securities are being offered pursuant to this prospectus other than the shares of our common stock that will be issued upon exercise of these currently outstanding warrants.

Our common stock is listed on the Nasdaq Capital Market under the symbol **CPRX** . On August 25, 2015, the last reported price per share of our common stock as reported on the Nasdaq Capital Market was \$3.25 per share.

**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 August 26, 2015**

**Table of Contents**

**TABLE OF CONTENTS**

<u>Summary</u>	1
<u>Risk Factors</u>	7
<u>Cautionary Statement Regarding Forward-Looking Statements</u>	26
<u>Use of Proceeds</u>	28
<u>Dilution</u>	29
<u>Plan of Distribution</u>	30
<u>Description of Securities we are Offering</u>	30
<u>Legal Matters</u>	35
<u>Experts</u>	35
<u>Where You Can Find Additional Information</u>	35
<u>Incorporation of Information by Reference</u>	35

**ABOUT THIS PROSPECTUS**

This prospectus is part of a registration statement that we filed with the Securities and Exchange Commission, or SEC. As permitted by the rules and regulations of the SEC, the registration statement filed by us includes additional information not contained in this prospectus. You may read the registration statement and the other reports we file with the SEC at the SEC's website or its offices described below under the heading "Where You Can Find Additional Information".

You should rely only on the information that is contained in this prospectus or that is incorporated by reference into this prospectus. We have not authorized anyone to provide you with information that is in addition to or different from that contained in, or incorporated by reference into, this prospectus. If anyone provides you with different or inconsistent information, you should not rely on it.

The shares of common stock offered by this prospectus are not being offered in any jurisdiction where the offer or sale of such common stock is not permitted. You should not assume that the information contained in, or incorporated by reference into, this prospectus is accurate as of any date other than the date of this prospectus or, in the case of the documents incorporated by reference, the date of such documents, regardless of the date of delivery of this prospectus or any sale of the common stock offered by this prospectus. Our business, financial condition, liquidity, results of operations and prospects may have changed since those dates.

**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 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, and, in March 2015, we were granted Orphan Drug Designation for Firdapse® for the treatment of patients with Congenital Myasthenic Syndromes, or CMS.

The chemical entity, 3,4-diaminopyridine (3,4-DAP), has never been approved by the FDA for any indication. If we are the first pharmaceutical company to obtain approval for an amifampridine-based product, we will be eligible to receive five years of marketing exclusivity with respect to the use of this product for any indication. Further, because Firdapse® for the treatment of LEMS has previously been granted Orphan Drug Designation by the FDA, the product is also eligible to receive seven years of marketing exclusivity for this indication, running concurrently with the five years of marketing exclusivity described above if both exclusivities are granted.

As part of our agreements with BioMarin, we took over the sponsorship of an ongoing Phase 3 clinical trial evaluating Firdapse® for the treatment of LEMS. The Phase 3 trial was designed as a double blind, randomized withdrawal trial in which all patients were initially treated with Firdapse® during a 91-day run-in period followed by treatment with either Firdapse® or placebo (randomly assigned, about 1:1) during a two-week randomization period. A total of 38 patients completed the three month run-in period and subsequent two week randomization period. In a trial of this design, the clinically significant findings, when present, are worsening of symptoms in the placebo group.

**Table of Contents**

On September 29, 2014, we reported top-line results from this trial. A summary of the results is as follows:

**Primary endpoints:**

The primary endpoint of change in quantitative myasthenia gravis score, or QMG, at day 14 reached statistical significance ( $p=0.0452$ ), with a worsening of 2.2 points observed in the placebo group and a worsening of 0.4 points observed in the treatment group.

The primary endpoint of change in subject global impression, or SGI, at day 14 was highly statistically significant ( $p=0.0028$ ), with a worsening of 2.6 points observed in the placebo group versus a worsening of 0.8 points observed in the treatment group.

**Secondary endpoints:**

The secondary endpoint for the physician's clinical global impression of improvement, or CGI-I, reached statistical significance ( $p=0.0267$ ), with a worsening at day 14 of 1.1 points between the placebo group and the treatment group.

The secondary endpoint of change in walking speed at day 14 showed a worsening of 9.7 feet per minute in the placebo group. The magnitude of the change relative to the variance in this test prevented the change from achieving statistical significance.

**Patient tolerance of Firdapse®:**

Firdapse® was generally safe and well tolerated. During the 91-day open label run-in period, treatment emergent adverse events occurred more frequently in treatment-naïve patients than in previously treated patients (approximately 10% of patients withdrew during this part of the study due to adverse events). During the placebo-controlled portion of the study, side effects occurring more frequently in the Firdapse® group were benign and consisted primarily of perioral and digital paresthesias and infections. No patients withdrew during this period.

All subjects who were randomized into the trial elected to continue with Firdapse® in the two year safety follow-up phase of the trial.

During 2014, we established an expanded access program (EAP) to make Firdapse® available to any patients diagnosed with LEMS, Congenital Myasthenic Syndromes (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 NDA approval. We are working with various rare disease advocacy organizations to inform physicians and patients as to the availability of the Firdapse® EAP.

During January 2015, we met with the FDA to discuss our anticipated submission of an NDA for Firdapse® for the treatment of LEMS. Based on our discussions with the FDA, we believe that our Phase 3 clinical program will provide acceptable support for submission of an NDA for Firdapse® for LEMS.

On July 22, 2015, we announced that we have initiated a rolling submission of an NDA for Firdapse® for the treatment of LEMS. Based on currently available information, we expect to complete the submission of our NDA during the fourth quarter of 2015, at which time we intend to request a priority review for our application. If our NDA is accepted for filing by the FDA and we are granted a priority review, of which there can be no assurance, we are hopeful that we will be able to obtain an approval from the FDA of our NDA for Firdapse® in third quarter of 2016 and commercially launch Firdapse® for the treatment of LEMS sometime shortly thereafter. There can be no assurance that we will obtain an approval of our NDA for Firdapse® for LEMS.

In addition to LEMS, we plan to evaluate Firdapse® for the treatment of other neuromuscular orphan indications, including certain forms of CMS and Myasthenia Gravis (MuSK antibody positive myasthenia gravis). We are currently completing the steps that we believe will be necessary to seek to include certain forms of CMS on our initial label for Firdapse®, assuming we are successful in obtaining approval of an NDA for Firdapse®. However, there can be no assurance that we will be successful in this effort. We are also currently in the process of developing a clinical program to evaluate Firdapse® for the treatment of a certain form of Myasthenia Gravis (MuSK antibody positive Myasthenia Gravis). There can be no assurance that Firdapse® will be determined to be effective in the treatment of MuSK antibody positive Myasthenia Gravis.

## Table of Contents

In anticipation of the commercialization of Firdapse<sup>®</sup>, we have been taking steps to prepare for the marketing of Firdapse<sup>®</sup> in the United States. We have recruited three rare disease clinical liaisons that are working with several rare disease advocacy organizations to help increase awareness of LEMS and CMS and to provide education for the physicians who treat these rare diseases and the patients they treat. We also intend to develop a sales force of 15-20 representatives experienced in selling drugs that treat rare disease. This sales force will market Firdapse<sup>®</sup> to the approximately 900 neuromuscular and oncology specialists who we believe most often diagnose and treat neuromuscular diseases, such as LEMS and CMS.

### 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, or VFDs, and reduction of somnolence) than those associated with vigabatrin. We are hoping to develop CPP-115 for the treatment of epilepsy (initially infantile spasms) and for the treatment of other selected neurological indications, such as complex partial seizures and 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 evaluating CPP-115 in a Phase 1(b) multi-dose safety and tolerance study. We recently revised the protocol for this study to add additional testing to assess whether surrogate markers of potential efficacy in treating Tourette's Disorder might be observed in study participants. These changes have delayed slightly our reporting of the top-line results from this study, and, based on currently available information, we now expect to report top-line results from this study during the fourth quarter of 2015. There can be no assurance that this trial will be successful.

### CPP-109

During June 2015, we announced encouraging results from an academic investigator proof-of-concept study evaluating the use of CPP-109 (our formulation of vigabatrin, another GABA aminotransferase inhibitor) for the treatment of Tourette's Disorder. The 8-week clinical trial was designed as an open label trial to evaluate the potential effect of GABA-aminotransferase inhibition as a mechanism for reducing tics in patients with treatment-refractory Tourette's Disorder. Vigabatrin was used as a research surrogate in this study to demonstrate the utility of GABA-aminotransferase (GABA-AT) blockade, with the expectation that upon successfully demonstrating the utility of this mechanism, further development activities would focus on the potentially safer, more potent GABA-AT inhibitor, CPP-115.

We believe that the top-line results from this study demonstrate an encouraging signal of activity in adult treatment-refractory patients with Tourette's Disorder. Further, we believe that CPP-109's mechanism of action validates the potential for CPP-115 to be a candidate for the treatment of Tourette's Disorder. However, there can be no assurance that CPP-115 will be effective for the treatment of treatment-refractory patients with Tourette's disorder.

## **Table of Contents**

### **Securities Class Action Lawsuit**

We have settled our previously disclosed securities class action lawsuit. For historic information about the securities class action lawsuit that was filed against us in late 2013, see Note 7 to the Notes to Financial Statements in our financial statements for the year ended December 31, 2014. In connection with the settlement, which became final on April 16, 2015, we paid \$3.5 million to settle this matter, all of which was paid by our insurance carrier. Under the settlement, the defendants, and various of their related persons and entities, received a full release of all claims that were or could have been brought in the action, as well as all claims that arise out of, are based upon, or relate to the allegations, transactions, facts, representations, omissions or other matters involved in the action related in any way to the purchase or acquisition of our securities by class members during the class period.

The settlement contains no admission of any liability or wrongdoing on the part of the defendants, each of whom continue to deny all of the allegations against each of them and believe that the claims were without merit. Because the full amount of the settlement payment was paid by our insurance carrier, the settlement did not have a material adverse effect on our financial position or results of operations. There were no opt outs from the settlement.

### **Our Strategy**

Pursue approval of Firdapse® for LEMS. In 2014, we reported positive results from our Phase 3 trial evaluating Firdapse® for the treatment of LEMS. We submitted the first module of our NDA to the FDA on July 22, 2015 and, based on currently available information, we expect to complete submission of our NDA during the fourth quarter of 2015. Although there can be no assurance, we are hopeful that we may obtain approval from the FDA of such NDA in the third quarter of 2016. If approved on this timeline, we would expect to commercially launch this product for the treatment of LEMS shortly after its approval.

Seek additional orphan drug indications for Firdapse®. We believe that Firdapse® may also be an effective treatment for other neuromuscular orphan indications such as certain forms of Congenital Myasthenic Syndromes and Myasthenia Gravis (MuSK antibody positive myasthenia gravis). We are currently completing the steps that we believe will be necessary to seek to include certain forms of CMS on our initial label for Firdapse®. We are also currently developing a clinical program to evaluate Firdapse® for the treatment of MuSK antibody positive myasthenia gravis.

Continue required clinical and pre-clinical work on CPP-115. We are currently conducting a Phase 1(b) multi-dose safety and tolerance study of CPP-115, and we hope to report the results of this study during the fourth quarter of 2015. We hope to develop CPP-115 for the treatment of epilepsy (initially infantile spasms) and for the treatment of other selected neurological indications. CPP-115 has been granted Orphan Drug Designation by the FDA for the treatment of infantile spasms and Orphan Medicinal Product Designation in the EU for West's syndrome (a form of infantile spasms), making the drug eligible for the seven-year and ten-year marketing exclusivities available from the FDA and the EU for these indications, respectively, if we are the first pharmaceutical company to obtain approval of an NDA and/or a Marketing Authorization Application, or MAA (the European Union equivalent of an NDA) for CPP-115.

Continue studies of CPP-109 and/or CPP-115 for Tourette s Disorder. We recently announced top-line results from an academic study evaluating the use of CPP-109 in the treatment of Tourette s Disorder. Based upon the results of this study, we may pursue CPP-115, which has a similar mechanism of action as CPP-109, for the treatment of Tourette s Disorder.

Seek additional funding or a partner for CPP-115. Once we have the results of our Phase 1(b) study, we plan to decide whether to seek a strategic partner to work with us in the development and future commercialization of CPP-115. However, no arrangements have been entered into to date.



**Table of Contents**

Seek to acquire additional products. We intend to seek to acquire other late stage orphan drug opportunities that might be complementary to Firdapse® and can be sold through any sales force we develop to market Firdapse®. However, no agreements have been entered into to date to acquire additional products and any such product acquisitions would be subject to the availability of funding.

**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) 529-2522.

**Table of Contents**

**The Offering**

**Securities being offered by us** 968,750 shares of common stock issuable upon the exercise of the warrants issued as a part of the securities sold in our May 2012 public offering. The warrants are exercisable until May 30, 2017 at an exercise price of \$1.04 per share.

**Common stock to be outstanding after this offering if all of the securities offered by us are exercised** 83,474,686 shares

**Use of proceeds** We intend to use the net proceeds of this offering for general corporate purposes. See *Use of Proceeds* for further information.

**Risk Factors** See *Risk Factors*, as well as other information included in this prospectus, for a discussion of factors you should read and consider carefully before investing in our securities.

**Trading Market** Our common stock is traded on the Nasdaq Capital Market under the symbol CPRX.

The number of shares of our common stock to be outstanding after this offering as shown above is based on 83,474,686 shares outstanding as of August 13, 2015 (including the 968,750 shares offered hereby) and excludes:

3,320,000 shares of our common stock subject to outstanding options under our 2014 Stock Incentive Plan and our 2006 Stock Incentive Plan having a weighted average exercise price of \$1.93 per share;

80,000 restricted stock units subject to vesting under our 2014 Stock Incentive Plan;

2,325,000 shares of our common stock that have been reserved for future issuance under our 2014 Stock Incentive Plan;

763,913 shares of our common stock that have been reserved for issuance upon exercise of outstanding warrants at an exercise price of \$1.30 per share; and

690,000 shares of our common stock that have been reserved for issuance upon exercise of outstanding warrants at an exercise price of \$2.08 per share.



**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. 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 securities 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 \$15.5 million and \$12.2 million for the years ended December 31, 2014 and December 31, 2013, respectively, and \$10.0 million for the six months ended June 30, 2015. 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 the end of 2016. 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, capital lease transactions, corporate collaborations, governmental research grants or cost sharing arrangements with appropriate agencies that operate under the umbrella of the National Institutes of Health and/or other means. However, there is no assurance that any such grants will be made 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 convertible debt securities 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<sup>®</sup> for the treatment of LEMS, we may not be able to bring it to market.***

In January of 2012, another pharmaceutical company, Jacobus Pharmaceutical, began its own Phase 2 trial studying their own formulation of amifampridine (3,4-DAP) for the treatment of LEMS (according to the information about this study on [clinicaltrials.gov](http://clinicaltrials.gov), enrollment in this trial has been discontinued as of April 2014). While there can be no assurance, we believe that Firdapse<sup>®</sup> 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 another pharmaceutical company is able to receive approval of an NDA for its formulation of amifampridine for the treatment of LEMS before we are able to receive approval of Firdapse<sup>®</sup> for the same indication, we would be barred from marketing Firdapse<sup>®</sup> 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 another company were to receive five-year new chemical entity exclusivity for amifampridine for any indication prior to approval of Firdapse<sup>®</sup>, we would be barred from marketing Firdapse<sup>®</sup> 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, if our future clinical trials of this product are successful. Further, our ability to develop CPP-115 will be dependent on our having the resources to conduct the studies and trials that would be required. There can be no assurance that we will ever submit an NDA for CPP-115 or 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.

For example, amifampridine, the active ingredient in Firdapse<sup>®</sup>, has been available from compounding pharmacies and from Jacobus Pharmaceutical under compassionate use INDs for many years, and will likely be available from these sources even if we are able to obtain FDA approval of Firdapse<sup>®</sup>.



## **Table of Contents**

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 should not 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 \$20,000,000 per claim and \$20,000,000 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, 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 advisers 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 advisers 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 market equivalent or superior products.

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 2(b) trial for cocaine addiction, and we are no longer pursuing the evaluation of CPP-109 for addiction. Further, our lead compound, Firdapse<sup>®</sup>, is for a very rare condition 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<sup>®</sup> is adequate to establish the clinical benefit of the drug. In addition, FDA has indicated that additional data from published studies, and data from a patient registry, would be useful in establishing the safety of Firdapse<sup>®</sup>, but we may not be able to obtain that data in a form that is satisfactory to the FDA. 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 reinspection 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 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 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 will require 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 obliged to rely on contract manufacturers. We cannot assure you 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) requirement 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.

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.

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

To date, our drug candidates have been manufactured in small quantities for pre-clinical studies and clinical trials. In order to conduct larger trials for a drug candidate and for commercialization of the resulting drug product if that drug candidate is approved for sale, we will need to manufacture in larger quantities. We may not be able to successfully increase the manufacturing capacity for any of 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.



**Table of Contents**

***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 16 employees and conduct much of our operations 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.***

Market acceptance and sales of Firdapse® or any other drug candidates we develop will depend in large part on third party payor coverage and reimbursement policies and may be affected by future healthcare reform measures in the U.S. and other jurisdictions where we may offer our products. The continuing efforts of governmental and third-party payors to contain, reduce or shift the costs of healthcare through various means, including an increased emphasis on managed care and attempts to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, may result in downward pressure on product pricing, reimbursement and utilization, which may adversely affect our product sales and results from operations. These pressures can arise from rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, drug coverage and reimbursement policies and pricing in general. Moreover, private health insurers and other third-party payors in the U.S. often follow the coverage and reimbursement policies of government payors, including the Medicare or Medicaid programs. In the U.S., third-party payors are shifting their cost containment measures to specialty products and high-cost drugs may be a target of such measures. All of these factors may significantly affect our income and ability to be reimbursed by third-party payors for Firdapse® and any other drug candidate we may develop in the future.



**Table of Contents**

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

***Our employees 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.

**Table of Contents**

**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 Current Good Manufacturing Processes (cGMP). 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 recently submitted a request for FDA approval of the trade name Firdapse<sup>®</sup>, which request has been conditionally approved.

*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.

We recently announced positive results from our Phase 3 clinical trial for Firdapse<sup>®</sup>. However, Firdapse<sup>®</sup> may nevertheless fail to meet the safety and efficacy standards required by the FDA to obtain regulatory approval.

## **Table of Contents**

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 Institutional Review Boards (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<sup>®</sup>, 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 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 expressed interest in testing 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.

***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;

**Table of Contents**

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.

***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.

***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 NDA 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 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. If any of our products were to be approved with a REMS, the potential market and profitability of the drug could be materially affected.



---

**Table of Contents**

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. 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. 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 that promote drugs or biologics for unapproved uses, based on the False Claims Act and other federal laws governing reimbursement for such products under the Medicare, Medicaid and other federally supported healthcare programs. Monetary penalties in such cases have often been substantial, and civil penalties can include costly mandatory compliance programs and exclusion from federal healthcare programs.

***Enacted and future legislation 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 legislative and regulatory changes and proposed 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 new 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 AMP 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 includes a provision to increase the Medicaid rebate for line extensions or reformulated drugs, which depending on how this provision is implemented could substantially increase our Medicaid rebate rate (in effect limiting reimbursement for these patients). 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.

## **Table of Contents**

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 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 if for a pediatric indication. 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.

## **Table of Contents**

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.

### ***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 may seek approval of our drug candidates under programs designed to accelerate the FDA's review and approval of NDAs. For example, the Food and Drug Administration Safety and Innovation Act, or FDASIA, which was enacted and signed into law in 2012, established a new 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 for our drug candidates, 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.

## **Risks Related to Our Intellectual Property**

### ***We are dependent on our relationship 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 us. 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 or any other matters 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;

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 anticipated 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. Such litigation could cause us to incur substantial costs and divert 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, our Board of Directors approved the adoption of a stockholder 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, 2016, unless the rights are earlier redeemed or exchanged.

The intent of the stockholder 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 stockholder 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 stockholder 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 acquirors 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 August 13, 2015, we had 82,505,936 shares of our common stock outstanding, of which 5,492,447 shares were held by our officers and directors. We also had outstanding: (i) common stock purchase warrants to purchase an aggregate of 2,422,663 additional shares of our common stock at exercise prices ranging from \$1.04 to \$2.08 per share (which includes the shares offered hereby), (ii) stock options to purchase an aggregate of 3,320,000 shares at exercise prices ranging from \$0.47 to \$4.64 per share (1,903,333 of which are currently exercisable), and (iii) 80,000 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.

***Our Board of Directors has the ability to issue blank check preferred stock.***

Our Certificate of Incorporation authorizes the issuance of up to 5,000,000 shares of blank check preferred stock, with such designation rights and preferences as may be determined from time to time by our Board of Directors. Our board of directors is empowered, without stockholder approval, to issue shares of preferred stock with dividend, liquidation, conversion, voting or other rights which could adversely affect the voting power or other rights of the holders of our common stock. In the event of such issuances, the preferred stock could be utilized, under certain circumstances, as a method of discouraging, delaying or preventing a change in control of our company, pursuant to our stockholder rights plan. Although we have no present intention to issue any shares of our preferred stock, there can be no assurance that we will not do so in the future.

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

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

***We may allocate the net proceeds from this offering in ways that you and other shareholders may not approve.***

We currently intend to use the net proceeds of this offering for general corporate purposes. See *Use of Proceeds*. However, because of the factors described above, we cannot at this time determine with specificity the particular uses 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 and could spend the proceeds in ways that do not necessarily improve our operating results or enhance the value of our common stock.

**Table of Contents**

*You will experience immediate and substantial dilution in the net tangible book value per share of the common stock you purchase.*

Since the price per share of our common stock being offered is higher than the net tangible book value per share of our common stock as of June 30, 2015, you will suffer immediate and substantial dilution in the net tangible book value of the common stock you purchase in this offering. See *Dilution* for a more detailed discussion of the dilution you will incur if you purchase common stock in this offering.

**CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS**

This prospectus 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 of our product 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 financing;

the scope, rate of progress and expense of our clinical trials and studies, preclinical 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;

whether our trials and studies will be successful;

the results of our clinical studies and trials, preclinical studies, proof-of-concept studies and our other development activities, and the number of such studies and trials that will be required for us to seek and obtain approval of NDAs for our drug candidates;

whether the third parties that assist us in our trials and studies perform as anticipated and within the budgets established for their activities;

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

whether any of our drug candidates will ever be approved for commercialization;

**Table of Contents**

the risk that another pharmaceutical company will receive an approval for its formulation of 3,4-DAP for the treatment of LEMS before we do;

what clinical trials and studies will be required before we can submit an NDA for Firdapse® for the treatment of CMS and whether any such clinical trials will be successful;

whether the FDA will accept for filing any NDA for Firdapse® that we may file, and even if such NDA filing is accepted, whether the FDA will grant a priority review of that NDA;

whether the receipt of breakthrough therapy designation for Firdapse® will expedite the development and review of Firdapse® by the FDA or the likelihood that the product will be found to be safe and effective;

even if one or more of our drug candidates is approved for commercialization, whether we will be able to successfully commercialize those products;

whether we will ever be able to achieve sustained profitability;

our estimates of the pricing of our drug candidates, if approved, and the size of the market for such drug candidates;

third-party payor reimbursement for any of our drug candidates that are commercialized;

the market adoption of any of our drug candidates approved for commercialization by physicians and patients;

our ability to obtain a sufficient commercial supply of our products;

our ability to successfully obtain additional indications for our drug candidates beyond those which may initially be approved;

the impact on sales of our products by others that are competitive to our products;

if one or more of our products are approved for commercialization, the costs, timing or estimated completion of any post-marketing studies that we are obligated to complete;

our expectations regarding licensing, acquisitions or strategic relationships;

changes in the laws and regulations affecting our business;

whether we can successfully protect any of our drug candidates under intellectual property laws;

the expense of filing, and potentially prosecuting, defending and enforcing any patent claims and other intellectual property rights we may have for our drug candidates;

our ability to develop a sales force to commercialize any products as to which we may obtain the right to commercialize;

our ability to attract and retain skilled employees;

**Table of Contents**

security breaches of our computer systems, or the computer systems of our contractors and/or vendors;

the impact of employee or consultant misconduct; and

changes in general economic conditions and interest rates.

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 made herein, which reflect our views only as of the date of this report, you should not place undue reliance upon such statements. We undertake no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

**USE OF PROCEEDS**

We estimate that the net proceeds of this offering will be approximately \$1.0 million. We currently expect to use the net proceeds of this offering for general corporate purposes.

Due to the factors set forth above, we cannot currently determine with absolute certainty how we will use 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. We will pay all of the costs associated with registering the securities covered by this prospectus.



**Table of Contents****DILUTION**

Purchasers of the securities offered by this prospectus will suffer immediate and substantial dilution in the net tangible book value per share of the common stock they purchase. Our net tangible book value as of June 30, 2015 was \$62,792,029, or approximately \$0.76 per share of our common stock. Net tangible book value per share represents the amount of total tangible assets less total liabilities, divided by the number of shares of our common stock outstanding as of June 30, 2015.

Dilution in net tangible book value per share represents the difference between the amount per share paid by purchasers in this offering and the net tangible book value per share of our common stock immediately after this offering. After giving effect to the sale of 968,750 shares of common stock in this offering at an exercise price of \$1.04 per share, our as adjusted net tangible book value as of June 30, 2015 would have been \$63,799,529, or approximately \$0.77 per share of our common stock. This represents an immediate increase in net tangible book value of \$0.01 per share of common stock to our already existing stockholders and an immediate dilution in net tangible book value of \$0.27 per share of common stock to purchasers in this offering. The following table illustrates this per share dilution:

Public offering price per share	\$ 1.04
Net tangible book value per share as of June 30, 2015	0.76
Increase per share attributable to this offering	0.01
As adjusted net tangible book value per share as of June 30, 2015	0.77
Dilution per share to investors participating in this offering	\$ 0.27

The above table is based on 82,096,803 shares outstanding as of June 30, 2015 and excludes:

The shares of common stock offered hereby that are issuable upon exercise of the warrants issued as part of our May 2012 public offering;

3,370,000 shares of our common stock subject to outstanding options under our 2014 Stock Incentive Plan and our 2006 Stock Incentive Plan having a weighted average exercise price of \$1.93 per share;

80,000 restricted stock units subject to vesting under our 2014 Stock Incentive Plan;

2,325,000 shares of our common stock that have been reserved for future issuance under our 2014 Stock Incentive Plan;

1,003,043 shares of our common stock that have been reserved for issuance upon exercise of outstanding warrants at an exercise price of \$1.30 per share; and

810,000 shares of our common stock that have been reserved for issuance upon exercise of outstanding warrants at an exercise price of \$2.08 per share.

To the extent that any outstanding options or warrants are exercised, new options are issued under our 2014 Stock Incentive Plan, or we otherwise issue additional shares of common stock in the future, at a price less than the public offering price, there will be further dilution to new investors.

**Table of Contents**

**PLAN OF DISTRIBUTION**

We will deliver shares of our common stock upon exercise of the common stock purchase warrants that we issued in May 2012. As of August 13, 2015, these warrants were exercisable for a total of 968,750 shares of our common stock, and no more of these warrants will be issued. We will not issue fractional shares upon exercise of these warrants. Each of these warrants contains instructions for exercise. In order to exercise any of these warrants, the holder must deliver to us or our transfer agent the information required in the warrants, along with payment for the exercise price of the shares to be purchased. We will then deliver shares of our common stock in the manner described below in the section titled *Description of Securities we are offering* *May 2012 Warrants* .

**DESCRIPTION OF SECURITIES WE ARE OFFERING**

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 August 13, 2015, we had 82,505,936 shares of our common stock outstanding. There are no shares of preferred stock outstanding.

We are a Delaware corporation, and were incorporated on July 24, 2006. On May 21, 2015, we filed an amendment to our Certificate of Incorporation changing our corporate name from Catalyst Pharmaceutical Partners, Inc. to Catalyst Pharmaceuticals, Inc. 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* .

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.

## Table of Contents

### **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. 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, 2016, 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.

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.

**Table of Contents**

**May 2012 Warrants**

The following summary description of the material features of the warrants we issued in May 2012 is necessarily general and is qualified in its entirety by reference to the form of warrant, a copy of which has been filed with the SEC as an exhibit to the registration statement of which this prospectus is a part. See *Where You Can Find More Information* .

Each warrant represents the right to purchase one share of common stock at an exercise price of \$1.04 per share. Each warrant may be exercised after the date of issuance through and including the date that is five years after the warrant is first exercisable.

*Exercise.* The warrants may be exercised on or prior to the expiration date at the offices of the company, with the delivery of a written notice in the form attached to the warrant completed and executed as indicated, accompanied by full payment of the exercise price for the number of warrants being exercised in the form discussed below. Within three trading days, certificates representing the shares of common stock purchased will be delivered to the warrant holder, or at the warrant holder's request, the warrant shares will be credited to the warrant holder's account with the Depository Trust Company. The warrants may be exercised in whole or in part.

*Payment.* The holder shall pay the exercise price in immediately available funds; provided, however, if at any time there is (i) no effective registration statement registering the relevant common stock and (ii) no effective registration statement registering the resale of or no current prospectus available for the resale of the relevant common stock by the holder, the holder may elect to satisfy its obligation to pay the exercise price through a cashless exercise .

*Fractional Shares.* No fractional shares will be issued upon an exercise of the warrants. If, upon exercise of the warrants, a holder would be entitled to receive a fractional interest in a share, we will, upon exercise, pay a cash adjustment in respect of such final fraction in an amount equal to such fraction multiplied by the exercise price.

*Limitations on Exercise.* The number of shares of our common stock that may be acquired by a holder upon any exercise of a warrant shall be limited so that the total number of shares of our common stock then beneficially owned by such holder does not exceed 9.99% of the total number of issued and outstanding shares of our common stock (including for such purpose the shares of common stock issuable upon such exercise). Our obligation to issue shares of common stock upon the exercise of a warrant shall be suspended until such time, if any, as shares of common stock may be issued in compliance with such limitation. This limitation on exercise will not apply to any holder who owned more than the percentage limitation of our shares set forth above prior to the issuance of the warrants in this offering.

*Adjustment.* The exercise price and the number of shares underlying the warrants are subject to appropriate adjustment in the event of stock splits, stock dividends on our common stock, stock combinations or similar events affecting our common stock. In addition, in the event we consummate any merger, consolidation, sale or other reorganization event in which our common stock is converted into or exchanged for securities, cash or other property, then following such event, the holders of the warrants will be entitled to receive upon exercise of such warrants the kind and amount of securities, cash or other property which the holders would have received had they exercised such warrants immediately prior to such reorganization event.

*Rights as Stockholders.* The warrant holders do not have the rights or privileges of holders of common stock and any voting rights until they exercise their warrants and receive shares of common stock. After the issuance of shares of common stock upon exercise of the warrants, each holder will be entitled to one vote for each share held of record in all matters to be voted on by stockholders.



**Table of Contents**

**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 participates do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or

on or after that date, the business combination is approved by our board of directors and authorized at a meeting of stockholders, and not by written consent, by at least two-thirds of the outstanding voting stock that is not owned by the interested stockholder.

Section 203 defines business combination to include the following:

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

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

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

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

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



In general, Section 203 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.

## **Table of Contents**

### **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 or proceeding (whether civil, criminal, administrative or investigative) by reason of the fact that such person is or was one of our directors, officers, employees or other agents, against expenses (including attorneys fees), judgments, fines and amounts paid in settlement actually and reasonably incurred by such person in connection with such action, suit or proceeding.

### **Listing**

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

### **Transfer Agent and Registrar**

Our transfer agent and registrar for our common stock is Continental Stock Transfer & Trust Company. They are located at 17 Battery Park, 8<sup>th</sup> Floor, New York, New York 10004. They can be reached via telephone at (212) 509-4000.



**Table of Contents**

**LEGAL MATTERS**

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

**EXPERTS**

The audited financial statements incorporated by reference in this 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.

**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, 2014, filed with the SEC on March 13, 2015;

our Quarterly Report on Form 10-Q for the quarter ended March 31, 2015, filed with the SEC on May 11, 2015;

our Quarterly Report on Form 10-Q for the quarter ended June 30, 2015, filed with the SEC on August 10, 2015;

our Current Reports on Form 8-K filed with the SEC on January 5, 2015, January 12, 2015, February 2, 2015, February 4, 2015, February 9, 2015, February 20, 2015, March 5, 2015, March 16, 2015, March 19, 2015, March 27, 2015 (as amended on August 7, 2015), April 14, 2015, May 12, 2015, May 22,

2015, May 28, 2015, June 24, 2015, June 30, 2015, July 22, 2015, August 10, 2015 and August 17, 2015;

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

**Table of Contents**

all documents subsequently filed by the Company pursuant to Sections 13(a), 13(c), 14 and 15(d) of the Exchange Act, from the date of filing of such documents.

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) 529-2522. Copies of each of these filings are also available for no cost on our website, [www.catalystpharma.com](http://www.catalystpharma.com), or on the SEC's web site, [www.sec.gov](http://www.sec.gov).