

CATALYST PHARMACEUTICALS, INC.

Form 424B3

January 10, 2017

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**Filed Pursuant to Rule 424(b)(3)  
Registration No. 333-215315**

## **Prospectus**

\$33,842,512

We may, from time to time in one or more offerings, offer and sell up to \$33,842,512, in the aggregate, of shares of our common stock. This continues the registration of the remaining unsold amount under our Registration Statement on Form S-3 (Registration No. 333-193699), which was declared effective by the Securities and Exchange Commission on March 19, 2014.

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

Our common stock is listed on The NASDAQ Capital Market under the symbol CPRX. On December 22, 2016, the last reported sale price on The NASDAQ Capital Market was \$1.09 per share.

**Our business and investing in shares of our common stock involves significant risks. You should carefully read and consider the Risk Factors beginning on page 6 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 January 9, 2017

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**ABOUT THIS PROSPECTUS**

This prospectus is part of a registration statement that we have filed with the Securities and Exchange Commission, or SEC, using the shelf registration process. Under this shelf registration statement, we may sell shares of our common stock. All such offerings will not exceed, in the aggregate, a total dollar amount of \$33,842,512. If our public float (the market value of the common stock held by our non-affiliate stockholders) goes below \$75 million, we will also be subject to a further limitation under which we can sell no more than one third (1/3) of our public float during any 12-month period. 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.

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### **SUMMARY**

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

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

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

### **Our Business**

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

#### **Firdapse®**

In October 2012, we licensed the North American rights to Firdapse®, a proprietary form of amifampridine phosphate, or chemically known as 3,4-diaminopyridine phosphate, from BioMarin Pharmaceutical Inc. (BioMarin). In August 2013, we were granted breakthrough therapy designation by the U.S. Food & Drug Administration (FDA) for Firdapse® for the treatment of patients with Lambert-Eaton Myasthenic Syndrome, or LEMS, a rare and sometimes fatal autoimmune disease characterized by muscle weakness. In March 2015, we were granted Orphan Drug Designation for Firdapse® for the treatment of patients with Congenital Myasthenic Syndromes, or CMS, and in August 2016, we were granted Orphan Drug Designation for Firdapse® for the treatment of patients with myasthenia gravis.

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

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



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

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

In October 2016, we announced that we had reached an agreement with the FDA under a Special Protocol Assessment (SPA) for the protocol design, clinical endpoints, and statistical analysis approach to be taken in our ongoing second Phase 3 study evaluating Firdapse® (amifampridine phosphate) for the symptomatic treatment of LEMS. A SPA is a process by which sponsors ask the FDA to evaluate the protocol of a proposed clinical trial to determine whether it adequately addresses scientific and regulatory requirements for the purpose identified by the sponsor. A SPA agreement indicates concurrence with the adequacy and acceptability of specific critical elements of protocol design, endpoints and analysis. Additionally, it provides a binding agreement with FDA's review division that a pivotal trial design, conduct, and planned analysis adequately addresses the scientific and regulatory objectives in support of a regulatory submission for drug approval. However, final marketing approval depends upon the results of efficacy, the safety profile, and an evaluation of the risk/benefit of treatment demonstrated in the Phase 3 clinical trial, among other requirements.

We intend to conduct our second Phase 3 trial evaluating Firdapse® for the treatment of LEMS (designated as LMS-003) at sites in Miami, Florida and Los Angeles, California. This double-blind, placebo-controlled withdrawal trial will include approximately 28 subjects, and will have the same co-primary endpoints as our first Phase 3 trial evaluating Firdapse® for the treatment of LEMS. Further, the FDA has agreed to allow us to enroll patients from our expanded access program as study subjects in this second trial. Finally, after further discussion with the FDA in connection with the SPA request, this second trial will be a parallel design and not a cross-over design. Final details of the Phase 3 clinical trial are available on [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

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

Our original NDA submission for Firdapse® included data and information (including data from a currently ongoing investigator treatment IND) providing evidence supporting the benefits of



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Firdapse<sup>®</sup> for treating certain types of CMS, and requested that CMS be included in our initial label for Firdapse<sup>®</sup>. To provide additional support for our submission of an NDA for Firdapse<sup>®</sup> for the treatment of CMS, in October 2015 we initiated a small blinded clinical trial at four academic centers of up to 10 subjects in the pediatric CMS population, ages 2 to 17. However, after discussions with the FDA, we have determined to enroll both adult and pediatric subjects with CMS in this trial and to expand the number of subjects to be evaluated in the trial to an aggregate of approximately 20 subjects. We have also added a fifth trial site for this study. Finally, we are currently discussing the primary and second endpoints to be used in this trial with the FDA.

Based on currently available information, we expect to report top line results from this study in the second half of 2017 and if the results of the study are successful, we hope to add the CMS indication to our label for Firdapse<sup>®</sup> (either as a part of our NDA resubmission for Firdapse<sup>®</sup> for LEMS or as a supplement to that resubmission). There can be no assurance that any trial we perform for Firdapse<sup>®</sup> for the treatment of CMS will be successful or whether any NDA that we may submit for Firdapse<sup>®</sup> for the treatment of CMS will be filed by the FDA for review and approved.

Firdapse<sup>®</sup> is also currently being evaluated as a treatment for MuSK-antibody positive myasthenia gravis. In February 2016, we announced the initiation of an investigator-sponsored, randomized, double-blind, placebo-controlled, crossover Phase 2/3 clinical trial evaluating the safety, tolerability and potential efficacy of Firdapse<sup>®</sup> as a symptomatic treatment for patients with MuSK-antibody positive myasthenia gravis (MuSK-MG). The study is planned to include up to 10 patients and we anticipate that the investigator will report top-line results from this study in the first half of 2017. We are providing study drug and financial support for this study.

If this study is successful, and subject to the availability of funding, we intend to initiate a registration quality trial in the United States evaluating Firdapse<sup>®</sup> for the treatment of patients with MuSK-MG, and we have submitted a SPA to the FDA with respect to this proposed trial. There can be no assurance that the currently ongoing investigator-sponsored trial will be successful, or, even if the current trial is successful, whether other future clinical trials that we initiate to evaluate Firdapse<sup>®</sup> for this indication will be successful, or whether we will receive a SPA for this trial. There can also be no assurance that the FDA will ever approve Firdapse<sup>®</sup> for this indication.

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

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

### **CPP-115.**

We are developing CPP-115, a GABA aminotransferase inhibitor that, based on our preclinical studies to date, we believe is a more potent form of vigabatrin, and may have fewer side effects (e.g., visual field defects) than those associated with vigabatrin. We are hoping to develop CPP-115 for the





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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).

Subject to the availability of funding or our entering into an agreement with a partner who will fund these development activities, of which there can be no assurance, we plan to move forward and perform the required toxicology studies and dose ranging studies evaluating the safety of CPP-115 that will be required in order to make this drug Phase 2 ready.

### CPP-109.

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

### Risks Associated with Product Development

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

the risk that another pharmaceutical company will receive an approval for its formulation of 3,4-diaminopyridine (3,4-DAP) for the treatment of LEMS or CMS, or any other indication, before we do;

what additional supporting information will be required before the FDA will file an NDA submission for Firdapse® for the treatment of either LEMS or CMS (or any other condition or disease);

whether any NDA that we may submit for Firdapse®, if accepted for filing by the FDA, will be granted a priority review;

the scope and timing of the clinical studies or trials that will be required before the FDA will accept an NDA submission for Firdapse® for the treatment of either LEMS or CMS;

whether, even if the FDA accepts an NDA submission for Firdapse<sup>®</sup>, such product will be determined to be safe and effective and approved for commercialization;

whether the receipt of breakthrough therapy designation for Firdapse<sup>®</sup> for LEMS will expedite the review of Firdapse<sup>®</sup> by the FDA or affect the likelihood that the product will be found to be safe and effective;

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

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

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

whether any such bioequivalence study, the design of which is acceptable to the FDA, will be successful;

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

the scope, rate of progress and expense of our clinical trials and studies, pre-clinical studies, proof-of-concept studies, and our other drug development activities;

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

whether our trials and studies will be successful;

**Company Information**

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

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**RISK FACTORS**

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

**Risks Related to our Business**

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

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

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

We have had no revenues from product sales to date, currently have no products available for commercial sale, and have never had any products available for commercial sale. We expect to incur losses at least until we are in a position to commercialize Firdapse®, which may never occur. Our net loss was \$13.9 million and \$14.4 million for the nine months ended September 30, 2016 and September 30, 2015, respectively and \$20.2 million and \$15.5 million for the years ended December 31, 2015 and December 31, 2014, respectively. We may never obtain approval of an NDA for any of our drug candidates and we may never achieve profitability.

*Our business will require additional capital.*

Our business will require additional capital to meet our product development objectives. Based on currently available information, we estimate that we have sufficient working capital to support our operations through at least the next year. 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



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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.

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® for the treatment of LEMS, we may not be able to bring it to market.***

Another pharmaceutical company, Jacobus Pharmaceutical, has completed its own Phase 2 trial studying their own formulation of amifampridine (3,4-DAP) for the treatment of LEMS. While there can be no assurance, we believe that Firdapse® is further along in development and as a result we expect that we will be in a position to obtain the first approval of an NDA for 3,4-DAP. Under the Orphan Drug Act of 1983, the first pharmaceutical product to obtain approval for an indication receives the orphan exclusivity under the statute. If Jacobus Pharmaceutical files an NDA and receives an approval of an NDA for its formulation of amifampridine for the treatment of LEMS before we are able to receive approval of Firdapse® for the same indication, we would be barred from marketing Firdapse® in the United States during the seven-year orphan exclusivity period, which would have a severe adverse effect on our results of operations. In addition, if Jacobus Pharmaceutical were to receive five-year new chemical entity exclusivity for amifampridine for any indication prior to approval of Firdapse®, we would be barred from marketing Firdapse® in the United States during this five-year exclusivity period.

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

Our development of CPP-115 is at an early stage, and it is going to be several years before we are in a position to submit an NDA for CPP-115, 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.

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***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>, despite not being FDA approved, has been available from compounding pharmacies and from Jacobus Pharmaceutical under compassionate use INDs for many years. Amifampridine from these sources can be expected to be substantially less expensive than Firdapse<sup>®</sup>. 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<sup>®</sup>), may nonetheless be prescribed by physicians for the treatment of LEMS.

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

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

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

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

As a public reporting company, we are required to comply with the Sarbanes-Oxley Act of 2002 and the related rules and regulations of the SEC, including periodic reports, disclosures and more complex





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

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

We are highly dependent on our officers and employees, on our Board of Directors and on our scientific advisors. The loss of the services of any of these individuals could significantly impede the achievement of our scientific and business objectives. Other than an employment agreement with Patrick J. McEnany, our Chairman, President and Chief Executive Officer with respect to his services, and the consulting agreements we have with several of our scientific advisors, we have no employment or retention agreements with our officers, directors or scientific advisors. If we lose the services of any of our existing officers, directors or scientific advisors, or if we were unable to recruit qualified replacements on a timely basis for persons who leave our employ, our efforts to develop our drug candidates might be significantly delayed. We do not carry key-man insurance on any of our personnel.

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

## **Risks Related to the Development of Our Drug Candidates**

### ***Our drug development efforts may fail.***

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

our drug candidates may be found to be ineffective or unsafe, or fail to receive necessary regulatory approvals;

our drug candidates may not be economical to market or take substantially longer to obtain necessary regulatory approvals than anticipated; or

competitors may 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 2b 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

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that our Phase 3 trial of Firdapse® 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®, 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;

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.

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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.

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

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

We have no in-house manufacturing capacity and, to the extent we are successful in completing the development of our drug candidates, we will be obligated to rely on contract manufacturers. We cannot 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) requirements enforced by the FDA, and similar requirements of other countries. The failure by a manufacturer to comply with these requirements could affect its ability to provide us with product. Although we intend to rely on third-party contract manufacturers, we are ultimately responsible for ensuring that our products are manufactured in accordance with cGMP.

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.

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

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

We may not be able to successfully increase in a sufficient manner the manufacturing capacity for our drug candidates, whether in collaboration with third-party manufacturers or on our own, in a timely or cost-effective manner or at all. If a contract manufacturer makes improvements in the manufacturing process for our drug candidates, we may not own, or may have to share, the intellectual property rights to those improvements. Significant scale-up of manufacturing may require additional validation studies, which are costly and which the FDA must review and approve. In addition, quality issues may arise during those scale-up activities because of the inherent properties of a drug candidate itself or of a drug candidate in combination with other components added during the manufacturing and packaging process, or during shipping and storage of the finished product or active pharmaceutical ingredients. If we are unable to successfully scale-up manufacture of any of our drug candidates in sufficient quality and quantity, the development of that drug candidate and regulatory approval or commercial launch for any resulting drug products may be delayed or there may be a shortage in supply, which could significantly harm our business.

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

If we are successful in obtaining approval to commercialize Firdapse® or any of our other drug candidates, we will need to significantly expand our operations, which could put significant strain on our management and our operational and financial resources. We currently have 18 employees and conduct many of our activities through outsourcing arrangements. To manage future growth, we will need to hire, train, and manage additional employees. Concurrent with expanding our operational and marketing capabilities, we will also need to increase our product development activities. We may not be able to support, financially or otherwise, future growth, or hire, train, motivate, and manage the required personnel. Our failure to manage growth effectively could limit our ability to achieve our goals.

Our success in managing our growth will depend in part on the ability of our executive officers to continue to implement and improve our operational, management, information and financial control systems and to expand, train and manage our employee base, and particularly to expand, train and manage a specially-trained sales force to market our products. We may not be able to attract and retain personnel on acceptable terms given the intense competition for such personnel among biotechnology, pharmaceutical and healthcare companies, universities and non-profit research institutions. Our inability to manage growth effectively could cause our operating costs to grow at a faster pace than we currently anticipate, and could have a material adverse effect on our business, financial condition, results of operations and prospects.

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

The commercial success of Firdapse® will depend substantially on the extent to which the cost of Firdapse® will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities (such as Medicare and Medicaid), private health coverage insurers and other third party payors. If reimbursement is not available, or is available only to limited levels, we may not be able to successfully commercialize Firdapse®. Even if coverage is provided, the approved reimbursement amount may not be high enough to establish and maintain pricing sufficient to realize a meaningful return on our investment.

Our ability to commercialize Firdapse® or any other product candidate will depend in large part on the extent to which coverage and reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and third party payors, such as private health insurers and health maintenance organizations, decide which medications they will cover and establish reimbursement levels. The healthcare industry is acutely focused on cost containment, both in the United States and elsewhere. Government authorities and third party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications, which could affect our ability to sell our product candidate profitably. These payors may not view our products, if any, as cost-effective, and coverage and reimbursement may not be available to our customers, or may not be sufficient to allow our products, if any, to be marketed on a competitive basis. Cost-control initiatives could cause us to decrease the price we might establish for products, which could result in lower than anticipated product revenues. If the prices for our products, if any, decrease or if governmental and other third party payors do not provide adequate coverage or reimbursement, our prospects for revenue and profitability will suffer.

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

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

The pricing of pharmaceutical products, in general, and specialty drugs, in particular, has been a topic of concern in the U.S. Congress, where hearings on the topic have been held, and has been a topic of speeches given by political figures, including President-Elect Donald Trump. There can be no assurance as to how this scrutiny on pricing of pharmaceutical products will impact future pricing of our products or orphan drugs or pharmaceutical products generally.





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***We cannot assess the impact on our business of the recent activities of a vocal group of neuromuscular physicians and some patients with LEMS.***

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

***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 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. Furthermore, because the potential target populations are very small, even if we do obtain significant market share for Firdapse®, we may never achieve profitability. Additionally, patients who discontinue therapy or do not fill prescriptions are not easily replaced by new patients, given the limited patient population.

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

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



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

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

## **Risks Related to Government Regulation**

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

We do not currently have any products that have been approved for commercialization. We will not be able to commercialize our products until we have obtained the requisite regulatory approvals from applicable governmental authorities. To obtain regulatory approval of a drug candidate, we must demonstrate to the satisfaction of the applicable regulatory agency that such drug candidate is safe and effective for its intended uses. The type and magnitude of the testing required for regulatory approval varies depending on the drug candidate and the disease or condition for which it is being developed. In addition, in the U.S. we must show that the facilities used to manufacture our drug candidate are in compliance with cGMP requirements. We will also have to meet similar regulations in any foreign country where we may seek to commercialize our drug candidates. In general, these requirements mandate that manufacturers follow elaborate design, testing, control, documentation and other quality assurance procedures throughout the entire manufacturing process. The process of obtaining regulatory approvals typically takes several years and requires the expenditure of substantial capital and other resources. Despite the time, expense and resources invested by us in the approval process, we may not be able to demonstrate that our drug candidates are safe and effective, in which event we would not receive the regulatory approvals required to market them.

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

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

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

Additionally, future clinical trials for our drug candidates may not be successfully completed or may take longer than anticipated because of any number of factors, including potential delays in the start of the trial, an inability to recruit clinical trial participants at the expected rate, failure to demonstrate safety and efficacy, unforeseen safety issues, or unforeseen governmental or regulatory delays. Further, our drug candidates may not be found to be safe and effective, and may not be approved by regulatory authorities for the proposed indication. Further, regulatory authorities and IRBs that must approve and monitor the safety of each clinical study may suspend a clinical study at any time if the patients participating in such study are deemed to be exposed to any unacceptable health risk. We may also choose to suspend human clinical studies and trials if we become aware of any such risks. We might encounter problems in our clinical trials, such as problems associated with Visual Field Defects (VFDs) or other side effects that will cause us, regulatory authorities, or IRBs to delay or suspend such trial or study.

In other countries where Firdapse<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

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

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

reliance on the third party for regulatory compliance and quality assurance;

reliance on the continued financial viability of the third parties;

limitations on supply availability resulting from capacity and scheduling constraints of the third parties;

impact on our reputation in the marketplace if manufacturers of our products, once commercialized, fail to meet the demands of our customers;

the possible breach of the manufacturing agreement by the third party because of factors beyond our control; and

the possible termination or nonrenewal of the agreement by the third party, based on its own business priorities, at a time that is costly or inconvenient for us.

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

***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

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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. Accordingly, our ANDA for vigabatrin, if approved, will be subject to either the same REMS, or a comparable REMS that will need to be reviewed and approved by the FDA. If any of our products were to be approved with a REMS, the potential market and profitability of the drug could be materially affected.

Our product promotion and advertising is also subject to regulatory requirements and continuing regulatory review. In particular, the marketing claims we will be permitted to make in labeling or advertising regarding our marketed products will be limited by the terms and conditions of the FDA-approved labeling. 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 reimbursement methodology based on



average sales prices for Medicare Part B physician-administered

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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). Additionally, in response to controversies regarding pricing of pharmaceutical products, there has been a recent push to propose legislation, both on state and federal levels, that would require greater disclosure as to the reasoning behind drug prices and, in some cases, could give state or federal-level commissions the right to impose cost controls on certain drugs. These and other new provisions are likely to continue the pressure on pharmaceutical pricing, may require us to modify our business practices with healthcare practitioners, and may also increase our regulatory burdens and operating costs. Finally, both President-Elect Donald Trump and certain members of the incoming Congress have expressed their intention to modify or eliminate the Health Care Reform Law. It is unknown what form any such modifications or any law proposed to replace the Health Care Reform Law would take, and how or whether it may affect our business in the future.

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®.

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***If we fail to obtain or subsequently maintain orphan drug exclusivity or regulatory exclusivity for Firdapse® and our other orphan drug candidates, our competitors may sell products to treat the same conditions at greatly reduced prices, and our revenues would be significantly adversely affected.***

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

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 have in the past and we may in the future, seek approval of our drug candidates under programs

designed to accelerate the FDA's review and approval of NDAs. For example,

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

**Risks Related to Our Intellectual Property**

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

All of our patent rights for Firdapse® are derived from our license agreement with BioMarin. Pursuant to this license agreement, we have licensed rights under BioMarin's Firdapse® patent applications in the United States, which expire in 2022 and 2034. We may lose our rights to these patents and patent applications if we breach our obligations under the license agreement, including, without limitation, our financial obligations to BioMarin. If we violate or fail to perform any term or covenant of the license agreement, BioMarin may terminate the license agreement upon satisfaction of any applicable notice requirements and expiration of any applicable cure periods. Additionally, any termination of the license agreement, whether by us or by BioMarin, will not relieve us of our obligation to pay any license fees owing at the time of such termination. If we fail to retain our rights under the license agreement, we would not be able to commercialize Firdapse®, and our business, results of operations, financial condition and prospects would be materially adversely affected.

Most of our patent rights for CPP-115 are derived from our license agreement with Northwestern University. Pursuant to this license agreement, we have exclusive worldwide rights to two patents in the United States. These were filed and obtained by Northwestern relating to compositions of matter for a class of molecules, including CPP-115. Both patents expire in 2023. Additionally, we have licensed rights from Northwestern to 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



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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

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.



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### ***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.

## **Risks Related to Our Common Stock**

### ***The trading price of the shares of our common stock has been and could in the future be highly volatile.***

The market price of our common stock has fluctuated in the past and is likely to fluctuate in the future. Market prices for biopharmaceutical companies have historically been particularly volatile. Some of the factors that may cause the market price of our common stock to fluctuate include:

developments concerning our clinical studies and trials and our pre-clinical studies;

status of regulatory requirements for approval of our drug candidates;

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;

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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.

***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;

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requirements that special meetings of our stockholders may only be called by the Board of Directors; and

advance notice procedures our stockholders must comply with in order to nominate candidates for election to our Board of Directors or to place stockholders' proposals on the agenda for consideration at meetings of stockholders.

On September 20, 2011, the Board of Directors approved the adoption of a stockholder rights plan, which was amended on September 19, 2016. The rights plan was implemented through our entry into a rights agreement with Continental Stock Transfer & Trust Company, as rights agent, and the declaration of a non-taxable dividend distribution of one preferred stock purchase right (each, a Right) for each outstanding share of our common stock. The dividend was paid on October 7, 2011 to holders of record as of that date. Each right is attached to and trades with the associated share of common stock. The rights will become exercisable only if a person acquires beneficial ownership of 17.5% or more of our common stock (or, in the case of a person who beneficially owned 17.5% or more of our common stock on the date the rights plan was adopted, such person acquires beneficial ownership of any additional shares of our common stock) or after the date of the Rights Agreement, commences a tender offer that, if consummated, would result in beneficial ownership by a person of 17.5% or more of our common stock. The rights will expire on September 20, 2019, unless the rights are earlier redeemed or exchanged.

The intent of the 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.

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 December 22, 2016, we had 82,972,316 shares of our common stock outstanding, of which 6,022,623 shares were held by our officers and directors. We also had outstanding: (i) common stock purchase warrants to purchase an aggregate of 2,407,663 additional shares of our common stock at exercise prices ranging from \$1.04 to \$2.08 per share, (ii) stock options to purchase an aggregate of 4,660,000 shares at exercise prices ranging from \$0.47 to \$4.64 per share (2,016,662 of which are currently exercisable), and 26,667 restricted stock units that are subject to vesting. Sales of restricted shares or shares underlying stock options and common stock purchase warrants, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock.



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***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.

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**CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS**

This Registration Statement on Form S-3 contains forward-looking statements, as that term is defined in the Private Securities Litigation Reform Act of 1995. These include statements regarding our expectations, beliefs, plans or objectives for future operations and anticipated results of operations. For this purpose, any statements contained herein that are not statements of historical fact may be deemed to be forward-looking statements. Without limiting the foregoing, believes, anticipates, proposes, plans, expects, intends, may, and other similar expressions are intended to identify forward-looking statements. Such statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or 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 report are based on current expectations that involve numerous risks and uncertainties.

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

our estimates regarding anticipated capital requirements and our need for additional financing;

the risk that another pharmaceutical company will receive an approval for its formulation of 3,4-diaminopyridine (3,4-DAP) for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS), Congenital Myasthenic Syndromes (CMS), or any other indication, before we do;

what additional supporting information will be required before the U.S. Food and Drug Administration (FDA) will file a new drug application (NDA) submission for Firdapse® for the treatment of either LEMS or CMS;

whether any NDA that we may submit for Firdapse®, if accepted for filing by the FDA, will be granted a priority review;

whether any additional clinical studies or trials will be required before the FDA will accept an NDA submission for Firdapse® for the treatment of either LEMS or CMS (or any other condition or disease);

whether the clinical studies or trials that are required before the FDA will accept an NDA submission for Firdapse® for the treatment of either LEMS or CMS will be successful;

whether, even if the FDA accepts an NDA submission for Firdapse®, such product will be determined to be safe and effective and approved for commercialization;



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

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

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

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

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

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

whether any such bioequivalence study, the design of which is acceptable to the FDA, will be successful;

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

the scope, rate of progress and expense of our clinical trials and studies, pre-clinical studies, proof-of-concept studies, and our other drug development activities;

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

whether our trials and studies will be successful;

the results of our clinical studies and trials, pre-clinical 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 new drug applications, or 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 Practices (cGMP);

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

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

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

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

what pricing we will be able to achieve with respect to our drug products and the impact of public scrutiny on the pricing of drug products generally and our products and orphan drugs in particular;

changes in the laws and regulations and regulatory guidance affecting our business;

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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;

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

our expectations regarding licensing, acquisitions or strategic relationships;

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 attract and retain skilled employees;

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

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

**USE OF PROCEEDS**

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

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



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

	<b>High</b>	<b>Low</b>
<b>Year Ended December 31, 2014</b>		
First Quarter	\$ 2.39	\$ 1.78
Second Quarter	\$ 2.61	\$ 1.73
Third Quarter	\$ 3.42	\$ 2.17
Fourth Quarter	\$ 3.03	\$ 2.37
<b>Year Ended December 31, 2015</b>		
First Quarter	\$ 4.93	\$ 2.74
Second Quarter	\$ 4.73	\$ 3.16
Third Quarter	\$ 5.74	\$ 3.00
Fourth Quarter	\$ 3.50	\$ 2.33
<b>Year Ending December 31, 2016</b>		
First Quarter	\$ 2.36	\$ 1.01
Second Quarter	\$ 1.25	\$ 0.56
Third Quarter	\$ 1.25	\$ 0.72
Fourth Quarter (through December 22, 2016)	\$ 1.46	\$ 0.96

We have never declared or paid any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings to support operations and finance the growth and development of our business and do not intend to pay cash dividends on our common stock for the foreseeable future. Any future determination related to our dividend policy will be made at the discretion of our board of directors.

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**GENERAL DESCRIPTION OF OUR COMMON STOCK**

Our authorized capital currently consists of 150,000,000 shares of common stock, par value \$0.001 per share, and 5,000,000 shares of preferred stock, par value \$0.001 per share. As of the date of this prospectus, we had 82,972,316 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. 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.

**Provisions of the Certificate and Bylaws**

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

**Issuance of Rights.** On September 20, 2011, the Board of Directors approved the adoption of a stockholder rights plan, which was amended on September 19, 2016. The rights plan was implemented





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through our entry into a rights agreement with Continental Stock Transfer & Trust Company, as rights agent, and the declaration of a non-taxable dividend distribution of one preferred stock purchase right (each, a Right) for each outstanding share of our common stock. The dividend was paid on October 7, 2011 to holders of record as of that date. Each right is attached to and trades with the associated share of common stock. The rights will become exercisable only if a person acquires beneficial ownership of 17.5% or more of our common stock (or, in the case of a person who beneficially owned 17.5% or more of our common stock on the date the rights plan was adopted, such person acquires beneficial ownership of any additional shares of our common stock) or after the date of the Rights Agreement, commences a tender offer that, if consummated, would result in beneficial ownership by a person of 17.5% or more of our common stock. The rights will expire on September 20, 2019, unless the rights are earlier redeemed or exchanged.

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

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

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

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

## **Certain Anti-Takeover Matters**

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

either the business combination or the transaction that resulted in the stockholder becoming an interested stockholder is approved by our board of directors before the date the interested stockholder attained that status;

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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.

## **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.

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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.

## **PLAN OF DISTRIBUTION**

We may sell the shares of our common stock from time-to-time pursuant to underwritten public offerings, negotiated transactions, block trades or a combination of these methods. We may sell the shares: (1) through underwriters or dealers, (2) through agents, and/or (3) directly to one or more purchasers. If our public float (the market value of the common stock held by our non-affiliate stockholders) goes below \$75 million, we will also be subject to a limitation that we may sell no more than one third (1/3) of our public float during any 12-month period. We may distribute the shares of common stock from time to time in one or more transactions at:

a fixed price or prices, which may change;

market prices prevailing at the time of sale;

prices relating to the prevailing market prices;

varying prices determined at the time of sale; or

negotiated prices.

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

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

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

any underwriting discounts and other items constituting underwriters' compensation;

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

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

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

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

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

To facilitate the offering of shares of our common stock, certain persons participating in the offering may engage in transactions that stabilize, maintain or otherwise affect the price of the shares. This may include over-allotments or short sales of the shares, which involve the sale by persons participating in the offering of more shares than we sold to them. In these circumstances, these persons would cover such over-allotments or short positions by making purchases in the open market or by exercising their over-allotment option. In addition, these persons may stabilize or maintain the price of the shares by bidding for or purchasing shares in the open market or by imposing penalty bids, whereby

selling concessions allowed to dealers participating in the offering may be reclaimed if shares sold by them are repurchased in connection with stabilization transactions. The effect of these transactions may be to stabilize or maintain the market price of the securities at a level above that which might otherwise prevail in the open market. These transactions may be discontinued at any time.

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



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**LEGAL MATTERS**

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

**EXPERTS**

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

**WHERE YOU CAN FIND ADDITIONAL INFORMATION**

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

**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, 2015, filed with the SEC on March 15, 2016;

our Quarterly Report on Form 10-Q for the quarter ended March 31, 2016, filed with the SEC on May 10, 2016;

our Quarterly Report on Form 10-Q for the quarter ended June 30, 2016, filed with the SEC on August 9, 2016;

our Quarterly Report on Form 10-Q for the quarter ended September 30, 2016, filed with the SEC on November 9, 2016;

our Current Reports on Form 8-K (or amendments thereto) filed with the SEC on February 8, 2016, February 17, 2016, March 16, 2016, April 26, 2016, May 10, 2016, May 17, 2016, June 7, 2016, June 10, 2016, June 13, 2016, June 24, 2016, August 9, 2016, September 2, 2016, September 19, 2016, September 20, 2016, October 3, 2016, October 31, 2016, November 9, 2016, December 8, 2016, and December 15, 2016;

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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

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) 420-3200. 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).