

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.

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® for treating certain types of CMS, and requested that CMS be included in our initial label for Firdapse®. To provide additional support for our submission of an NDA for Firdapse® for the treatment of CMS, in October 2015 we initiated a small blinded clinical trial at four academic centers of up to 10 subjects in the pediatric CMS population, ages 2 to 17. However, after 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® (either as a part of our NDA resubmission for Firdapse® for LEMS or as a supplement to that resubmission). There can be no assurance that any trial we perform for Firdapse® for the treatment of CMS will be successful or whether any NDA that we may submit for Firdapse® for the treatment of CMS will be filed by the FDA for review and approved.

Firdapse® 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® 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® 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® 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® for this indication.

Finally, we may seek to evaluate Firdapse® 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® 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® in the United States. In light of the determination that we will have to complete another adequate and well controlled study evaluating Firdapse® 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[®], 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 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[®], despite not being FDA approved, has been available from compounding pharmacies and from Jacobus Pharmaceutical under compassionate use INDs for many years. Amifampridine from these sources can be expected to be substantially less expensive than Firdapse[®]. The FDA Pharmacy Compounding Advisory Committee, however, has previously issued a list of drugs which should not be compounded, and amifampridine was included on that list. In addition, drugs that are not approved by FDA for the treatment of LEMS, such as a related aminopyridine drug, dalfampridine (Ampyra[®]), may nonetheless be prescribed by physicians for the treatment of LEMS.

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

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

Our business exposes us to potential liability risks that may arise from the clinical testing, manufacture, and/or sale of our pharmaceutical products. Patients have received substantial damage awards in some jurisdictions against pharmaceutical companies based on claims for injuries allegedly caused by the use of pharmaceutical products used in clinical trials or after FDA approval. Liability claims may be expensive to defend and may result in large judgments against us. We currently carry liability insurance with an aggregate annual coverage limit of \$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[®], 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[®], 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[®]. In October 2016, we announced that we had reached an agreement with the FDA under a SPA for the protocol design, clinical endpoints, and statistical analysis approach to be taken in our ongoing second Phase 3 study evaluating Firdapse[®] for the symptomatic treatment of LEMS. However, Firdapse[®] may nevertheless fail to meet the safety and efficacy standards required by the FDA to obtain regulatory approval.

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

In other countries where Firdapse[®], CPP-115 or any other product we develop or license may be marketed, we will also be subject to regulatory requirements governing human clinical studies, trials and marketing approval for drugs. The requirements governing the conduct of clinical studies, trials, product licensing, pricing and reimbursement varies widely from country to country.

We may face significant delays in our clinical studies and trials due to an inability to recruit patients for our clinical studies and trials or to retain patients in the clinical studies and trials we may perform.

We may encounter difficulties in our current and future clinical studies and trials recruiting patients, particularly since the conditions we are studying are rare 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.