CATALYST PHARMACEUTICAL PARTNERS, INC.
Form 10-K
March 19, 2014
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

[Mark One]

x **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the Fiscal Year Ended December 31, 2013

OR

¨ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

Commission File No. 001-33057

CATALYST PHARMACEUTICAL PARTNERS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**76-0837053**
(State of jurisdiction of incorporation or organization)	**(IRS Employer Identification No.)**
355 Alhambra Circle, Suite 1500 Coral Gables, Florida	**33134**
(Address of principal executive offices)	**(Zip Code)**

Registrant s telephone number, including area code: (305) 529-2522

Securities Registered Pursuant to Section 12(b) of the Act.

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

Securities registered pursuant to Section 12(g) of the Act.: None

Indicate by check mark if registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ¨ No x

Indicate by check mark if registrant is not required to file reports pursuant to Rule 13 or Section 15(d) of the Act. Yes ¨ No x

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such report(s), and (2) has been subject to such filing requirements for the past 90 days. Yes x No ¨

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate web site, if any, every Interactive Data File required to be submitted and posted pursuant to rule 405 of Regulation S-T ((§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes x No ¨

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer or a smaller reporting company. See the definitions of large accelerated filer , accelerated filer and smaller reporting company in Rule 12b-2 of the Exchange Act (Check one):

Large accelerated filer ¨	Accelerated filer ¨
Non-accelerated filer ¨ (Do not check if a smaller reporting company)	Smaller reporting company x

As of June 30, 2013, the last business day of the Registrant s most recently completed second quarter, the aggregate market value of all voting, and non-voting common equity held by non-affiliates was $32,240,770.

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ¨ No x

Indicate the number of shares outstanding of each of the issuer s classes of common stock, as of the latest practicable date: 54,145,633 shares of common stock, $0.001 par value per share, were outstanding as of March 14, 2014.

Part III incorporates certain information by reference from the registrant s definitive proxy statement for the 2014 annual meeting of stockholders. The proxy statement with respect to the 2014 annual meeting of stockholders will be filed no later than 120 days after the close of the registrant s fiscal year ended December 31, 2013.

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PART I

You are urged to read this Annual Report on Form 10-K (Form 10-K) in its entirety. This Form 10-K contains forward-looking statements that involve risks and uncertainties. Our actual results may differ significantly from the projected results discussed in these forward-looking statements. Factors that may cause such a difference include, but are not limited to, those discussed below and in Item 1A, Risk Factors.

We, our, ours, us, Catalyst, or the Company, when used herein, refers to Catalyst Pharmaceutical Partners, Inc., a Delaware corporation.

Forward-Looking Statements

This Annual Report on Form 10-K contains forward-looking statements , as that term is defined in the Private Securities Litigation Reform Act of 1995. These include statements regarding our expectations, beliefs, plans or objectives for future operations and anticipated results of operations. For this purpose, any statements contained herein that are not statements of historical fact may be deemed to be forward-looking statements. Without limiting the foregoing, believes , anticipates , proposes , plans , expects , intends , may , and other similar expressions are i identify forward-looking statements. Such statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or other achievements to be materially different from any future results, performances or achievements expressed or implied by such forward-looking statements. Factors that might cause such differences include, but are not limited to, those discussed in the section entitled Item 1A Risk Factors and those discussed in the section entitled Item 7 Management s Discussion and Analysis of Financial Condition and Results of Operations Caution Concerning Forward-Looking Statements.

The successful development of our current drug candidates, Firdapse , CPP-115 and CPP-109, 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 scope, rate of progress and expense of our clinical trials and studies, pre-clinical studies, proof-of-concept studies, and our other product 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;
- the ability of our third-party suppliers and contract manufacturers to maintain compliance with current Good Manufacturing Processes (cGMP);
- 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 product 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 expense of filing, and potentially prosecuting, defending and enforcing any patent claims and other intellectual property rights;

the risk that another pharmaceutical company will receive an approval for its formulation of amifampridine for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS) before us;

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whether others develop and commercialize products competitive to our products;

whether others obtain exclusive patent or marketing rights that make it difficult or impossible for us to commercialize our product candidates, even if we obtain regulatory approvals for our product candidates;

changes in the laws and regulations affecting our business;

the impact of the class action lawsuit filed against us;

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 employee or consultant misconduct; and

changes in general economic conditions and interest rates.

Our current plans and objectives are based on assumptions relating to the development of our current product 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.

Item 1. Business

Overview

We are a development-stage specialty pharmaceutical company focused on the development and commercialization of novel prescription drugs targeting rare (orphan) neuromuscular and neurological diseases. We currently have three pharmaceutical products 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). As part of our agreements with BioMarin, we have taken over the sponsorship of an ongoing Phase 3 clinical trial evaluating Firdapse for the treatment of Lambert-Eaton Myasthenic Syndrome, or LEMS, a rare and sometimes fatal autoimmune disease characterized by muscle weakness. We also hope to evaluate Firdapse for the treatment of other neuromuscular orphan indications such as certain forms of Congenital Myasthenic Syndrome and Myasthenia Gravis. In August 2013, we were granted breakthrough therapy designation by the U.S. Food & Drug Administration (FDA)

for Firdapse for the treatment of LEMS.

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

The Phase 3 trial is designed as a randomized double-blind, placebo-controlled discontinuation study followed by an open-label extension period in approximately 36-patients across 24 sites in the United States, Canada, South America and Europe. Based on currently available information, we expect that we will complete enrollment in the trial before the end of the first quarter of 2014 and that we will report top-line results from the double-blind portion of this Phase 3 trial during the third quarter of 2014 (and, if

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the trial results are successful, we expect to submit to the FDA, on a rolling basis, all of the modules required to complete a new drug application (NDA) by the middle of 2015).

The following discusses other aspects of our development program for Firdapse :

Amifampridine is a voltage-gated potassium channel blocker. The Firdapse tablets currently being used in our Phase 3 pivotal trial are the same product approved for marketing in Europe and have been shown to be more stable than the free base form, 3,4-DAP. This enhanced stability is expected to provide LEMS patients with the assurance that their drug has the correct potency and purity in every dose.

We believe that another pharmaceutical company, Jacobus Pharmaceutical, is conducting a Phase 2 trial with a different formulation of amifampridine (3,4-DAP) for the treatment of LEMS. While there can be no assurance, based on currently available information, we believe that our development program for amifampridine is further along in development than the other company s development program.

We believe that the LEMS patient community deserves the benefits of having an approved product to treat their disease that has met the FDA s stringent burden of proof in safety and efficacy and is widely available for use by physicians treating LEMS patients. To date, no version of amifampridine has been approved by the FDA for use in the treatment of LEMS. To obtain approval to market a drug in the U.S., a significant number of pre-clinical and clinical safety and efficacy studies must be completed. This includes studies which evaluate the efficacy of the product, including in most cases at least one adequate and well controlled pivotal registration trial that meets the requirements established by the FDA. It also includes studies that evaluate the drug s long-term toxicity, acute toxicity, reproductive toxicity, carcinogenicity, mutagenicity, cardiac safety, renal safety, pharmacokinetics, absorption, distribution, metabolism, and elimination. Particularly with respect to products containing amifampridine, there is a wide metabolic variability within the patient population, which must be characterized in order to provide physicians with information about what to expect in the patients they treat and, more importantly, with instructions on how to safely prescribe the drug to their patients. The FDA typically expects that the registration clinical trial supporting approval of a product will be done with batches of the to-be commercialized form of the drug, which has to be manufactured under current good manufacturing practices (cGMP), using a validated manufacturing process suitable for commercialization, tested with validated analytical methods, and tested for shelf life stability. Our development plan for Firdapse has been designed to meet all of these requirements and was discussed with the FDA at our recent Type B meeting.

Based on currently available information, we expect to make a cumulative investment in the development and commercialization of Firdapse of between $40 million and $50 million, consisting of: (i) approximately $25 million that we currently anticipate will be spent conducting the clinical, non-clinical and safety evaluations, and manufacturing the three validation batches, that will be required for us to obtain an NDA for Firdapse for the treatment of LEMS, (ii) approximately $10 million in milestone payments that we will be obligated to pay under our license agreement with BioMarin (a portion of which will be due when an NDA for Firdapse for the treatment of LEMS is accepted by the FDA and a portion of which will be due upon the final approval of an NDA for Firdapse for the treatment of LEMS), and (iii) $5 million to $15 million that we expect to spend in connection with post-marketing studies of Firdapse and to develop the

infrastructure required to commercialize Firdapse (including our efforts to develop the patient advocacy programs and patient assistance program discussed below). This is a significant investment of capital and years of research and development by us, and is in addition to the millions of dollars that have already been spent in the development of this product by BioMarin, by the other former licensors of the product, and by the innovator of the product (the pharmaceutical unit (AGEPS) of the Paris Public Hospital Authority).

While pricing for Firdapse has not been established, we recognize the importance of access to affordable medicines. We expect to work with insurers to develop appropriate plans for broad patient access in the U.S. market.

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We are already working on the development of a patient assistance program that will insure that all LEMS patients who need the drug will have access to an FDA approved drug, regardless of their economic circumstances.

We intend to develop patient advocacy and solutions programs that will allow for disease awareness and for patient and physician education.

CPP-115

We are in the early stages of developing CPP-115, a GABA aminotransferase inhibitor that, based on our pre-clinical studies to date, we believe is a more potent form of vigabatrin, but may have fewer side effects (e.g., visual field defects, or VFDs) than those associated with vigabatrin. We are hoping to develop CPP-115 for the treatment of epilepsy (initially infantile spasms) and for the treatment of other selected neurological indications. 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 s syndrome (a form of infantile spasms). We expect to begin a multi-dose safety and tolerance study of CPP-115 during the first half of 2014.

CPP-109

For several years, we evaluated CPP-109 (our formulation of vigabatrin, another GABA aminotransferase inhibitor) for the treatment of cocaine addiction. However, in November 2012, we reported that CPP-109 failed to meet the primary and two key secondary endpoints in a Phase 2(b) trial for cocaine addiction. As a result, we are no longer focusing our efforts on evaluating CPP-109 for addiction. Further, on November 8, 2013, effective October 1, 2013, we terminated our license agreement with Brookhaven National Laboratories under which we had previously licensed nine patents relating to the use of vigabatrin as a treatment of a wide variety of substance addictions.

An academic investigator proof-of-concept study evaluating the use of CPP-109 for the treatment of Tourette Syndrome is currently ongoing and, if the results of that study show evidence of reduced number of tics, we will likely seek to develop CPP-109 or CPP-115 (which has the same mechanism of action as CPP-109) for this indication. We do not control this proof-of-concept study and therefore have no control over its timing. However, based on currently available information, we expect to have top-line results for this academic investigator proof-of-concept study during 2014.

Capital Resources

Based on our current financial condition and forecasts of available cash, we believe that we have sufficient funding to support our planned operations through at least the end of 2014. However, we will require additional funding to support our planned operations beyond the end of 2014. There can be no assurance that we will obtain additional funding or that we will ever be in a position to commercialize any of our product candidates. See Item 7. Management s Discussion and Analysis of Financial Condition and Results of Operations Liquidity and Capital Resources below for further information on our liquidity and cash flow.

Our Strategy

Our goal is to develop and commercialize novel prescription drugs targeting rare (orphan) neuromuscular and neurological diseases and disorders. Specifically we intend to:

Pursue Firdapse for LEMS. Enrollment in the Phase 3 clinical trial evaluating Firdapse for the treatment of LEMS is expected to be completed by the end of the first quarter of 2014 and we expect to report top-line results from the double-blind portion of this clinical trial during the third quarter of 2014. Assuming success in the Phase 3 trial, we expect to complete the filing of an NDA for Firdapse by the middle of 2015 and, although there can be no assurance, we anticipate that under those circumstances we may obtain approval from the FDA of such NDA by the end of the first quarter of 2016. If approved on this timeline, we would hope to commercially launch this product for the treatment of LEMS during the second quarter of 2016.

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Seek additional orphan drug indications for Firdapse . We believe that Firdapse may also be an effective treatment for other neuromuscular orphan indications such as certain forms of Congenital Myasthenic Syndrome and Myasthenia Gravis. Subject to the availability of funding, we hope to pursue the necessary clinical and non-clinical trials and studies to support applications to the FDA for approval to market Firdapse for these additional indications.

Continue required clinical and pre-clinical work on CPP-115. We expect to commence a Phase 1(b) multi-dose safety and tolerance study of CPP-115 during the first half of 2014. Subject to the availability of funding, if the Phase 1(b) study is successful, we hope to commence a Phase 2 trial evaluating CPP-115 for the treatment of epilepsy in 2015. We hope to develop CPP-115 for the treatment of epilepsy (initially infantile spasms) and for the treatment of other selected neurological indications. CPP-115 has been granted Orphan Drug Designation by the FDA for the treatment of infantile spasms and Orphan Medicinal Product Designation in the EU for West s syndrome (a form of infantile spasms), making the drug eligible for the seven-year and ten-year marketing exclusivities available from the FDA and the EU for these indications, respectively, if we are the first pharmaceutical company to obtain approval of an NDA and/or a Marketing Authorization Application, or MAA (the European Union equivalent of an NDA) for CPP-115.

Seek additional funding for CPP-115. We continue to seek a strategic partner to work with us in the development and future commercialization of CPP-115. We also continue to seek government and/or other grants to help fund the development of CPP-115. However, no arrangements have been entered into to date.

Firdapse

Product overview

Firdapse is Catalyst s and BioMarin s (depending on market region) registered trade name for Amifampridine phosphate tablets. Amifampridine is the WHO (World Health Organization) registered INN (International Nonproprietary Name) and United States Adopted Name (USAN) for the chemical entity, 3,4-diaminopyridine, or 3,4-DAP. Firdapse is the phosphate salt of amifampridine, hence the name amifampridine phosphate. The name of this drug is sometimes abbreviated as 3,4-DAP, or 3,4-DAPP or simply DAPP. We will refer to our drug by its trade name (Firdapse), by the INN/USAN (amifampridine phosphate), or both, throughout this report.

Clinical efficacy data for the symptomatic treatment of patients with LEMS with amifampridine base are derived from five published randomized, double-blind, placebo-controlled studies and one double-blind study with an active comparator in patients with LEMS. The data from the randomized controlled studies demonstrate statistically significant improvements across a number of independent measures of neurological function, including Quantitative Myasthenia Gravis (QMG) score and compound muscle action potential (CMAP), which have been demonstrated to be clinically relevant in patients with LEMS. Results of these trials demonstrate that amifampridine is more effective for the symptomatic treatment of LEMS compared with placebo or active investigational comparator (pyridostigmine). Additionally, supportive data from multiple published uncontrolled investigations and case reports demonstrate the long-term benefits of treatment with amifampridine in patients with LEMS. These data also show that removal of patients from drug can lead to a recurrence of underlying symptoms, but with reintroduction of amifampridine improvement of muscle function is regained. As such, amifampridine has been recommended as first-line symptomatic treatment for LEMS by the European Federation of Neurological Societies (EFNS) (Skeie, 2006; Skeie, 2010; Lindquist, 2011). In December 2009, amifampridine phosphate received marketing approval by the European Commission as Firdapse for the symptomatic treatment of patients with LEMS.

Safety data from clinical data published over the last 30 years in patients with LEMS or other neurological disorders treated with amifampridine show that amifampridine is well tolerated at doses £80 mg per day. Among the 1,279 patients or healthy subjects

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assessed in the literature, the most frequently reported adverse events (AEs) were perioral and peripheral paresthesias (unusual sensations like pins and needles), and gastrointestinal disorders (abdominal pain, nausea, diarrhea, epigastralgia (pain around the upper part of the stomach). These events were typically mild or moderate in severity, and transient, seldom requiring dose reduction or withdrawal from treatment.

Lambert-Eaton Myasthenic Syndrome

Lambert-Eaton Myasthenic Syndrome, or LEMS, is a rare autoimmune disorder characterized primarily by muscle weakness of the limbs. The disease is caused by an autoimmune reaction where antibodies are formed against voltage gated calcium channels on nerve endings, which damages the channels. These calcium channels are responsible for the transport of charged calcium atoms that activate the biochemical machinery responsible releasing acetylcholine. Acetylcholine is the neurotransmitter responsible for causing muscles to contract and the failure to release this neurotransmitter results in the muscle weakness in LEMS patients. Additionally, LEMS is often associated with an underlying malignancy, most commonly small-cell lung cancer, and in some individuals, LEMS is the first symptom of such malignancy.

Orphanet, the British Medical Journal and other publically available sources, estimate that the prevalence of LEMS in the U.S. and E.U. is approximately 1 per 100,000 in population. Based on current population statistics for the United States, Mexico and Canada and other available data, we currently estimate that there are about 4,650 LEMS patients in North America (3,150 in the U.S., 350 in Canada and 1,150 in Mexico).

LEMS generally affects the extremities, especially the legs. As LEMS most affects the parts of limbs closest to the trunk, difficulties with climbing stairs or rising from a sitting position are commonly noted. Physical exercise and high temperatures tend to worsen the symptoms. Other symptoms occasionally seen include weakness of the muscles of the mouth, throat, and eyes. Individuals affected with LEMS also may have a disruption of the autonomic nervous system, including dry mouth, constipation, blurred vision, impaired sweating, and/or hypotension.

LEMS is treated by treating the symptoms or the underlying autoimmune attack on voltage gated calcium channels. Treatments include steroids, azathioprine and intravenous immunoglobulin, which work by suppressing the immune system; and pyridostigmine and amifampridine, which enhance neuromuscular transmission. Plasma exchange has also been used in an attempt to remove antibodies from the body. Firdapse is a symptomatic treatment and does not alter the underlying autoimmune condition. As a voltage gated potassium blocker, Firdapse prevents charged potassium particles from leaving the nerve cells, which prolongs the period of depolarization. This allows more charged calcium atoms to enter the nerves, which enables the nerves to release acetylcholine. This causes muscles to contract and to restore lost muscle strength in LEMS patients.

Strategic collaboration with BioMarin for Firdapse

On October 26, 2012, we entered into a strategic collaboration with BioMarin for Firdapse . The key components of the collaboration included our licensing of the exclusive North American rights to Firdapse pursuant to a License Agreement, dated October 26, 2012, between us and BioMarin (the BioMarin License Agreement), and BioMarin making a $5.0 million investment in our common stock to advance the development of Firdapse in the United States pursuant to a Convertible Promissory Note and Note Purchase Agreement, dated as of October 26, 2012, between us and BioMarin (the Investment Agreement).

Under the BioMarin License Agreement, we licensed the North American rights to Firdapse , and, as part of the license, we have taken over the Phase 3 clinical trial that BioMarin had previously begun in the United States evaluating Firdapse for the treatment of LEMS. We are obligated to use our diligent efforts to seek to obtain

regulatory approval for and to commercialize Firdapse in the United States. We are further obligated to use diligent efforts to complete the double-blind treatment phase of the Phase 3 trial by October 26, 2014, and BioMarin has the right to terminate the BioMarin License Agreement if such treatment phase has not been completed in such period (unless we are using diligent efforts to pursue the completion of such treatment phase and have spent at least $5.0 million in connection with the conduct of the Phase III Trial during such period). Based on current expectations, we expect to satisfy the required milestones.

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Under the BioMarin License Agreement, we have agreed to make: (i) certain royalty payments to BioMarin based on our net sales in North America; (ii) certain royalty payments to a third-party licensor of the rights sublicensed to us based on our net sales in North America; and (iii) certain milestone payments to such third-party licensor and to the former stockholders of Huxley Pharmaceuticals, Inc. (Huxley) that BioMarin is obligated to make (which milestone payments are due, in part, upon acceptance by the FDA of a filing of an NDA for Firdapse for the treatment of LEMS, and, in part, on the unconditional approval by the FDA of an NDA for Firdapse for the treatment of LEMS).

Under the Investment Agreement, BioMarin delivered $5.0 million to us on October 26, 2012. This amount was initially treated as a loan, but, pursuant to the terms of the Investment Agreement, was automatically converted, at a conversion price of $0.75 per share, into 6,666,667 shares of our authorized but unissued common stock on December 10, 2012.

Firdapse was approved by European Medicines Agency for the treatment of LEMS in December 2009, and BioMarin sells the product in the European Union (EU). BioMarin is also currently performing or will in the future perform certain post-marketing studies of Firdapse that they are required to conduct to support their continued approval of Firdapse in the EU. We have agreed to pay one-half of the costs of these studies. We have also shared the costs of a cardiac safety study and we are sharing the costs of reproductive toxicity studies that are required for approval of Firdapse by the FDA.

The Phase 3 clinical trial

In June 2011, BioMarin commenced a Phase 3 clinical trial in the United States studying Firdapse for the treatment of LEMS, which trial has been transferred to us pursuant to the License Agreement. The trial is designed as a randomized, double-blind, placebo-controlled, discontinuation trial in approximately 36 LEMS patients. After patients have been treated with amifampridine phosphate for at least 91 days, they are randomly assigned to either continue on amifampridine phosphate or be discontinued to placebo over a 2-week period. They are then returned to open label amifampridine phosphate treatment for a two-year follow-up period.

The primary endpoint is a comparison of changes in patients randomized to continue amifampridine phosphate versus those who transition to placebo that occur in both the QMG score, which measures muscle strength, and subject global impression score, on which the subject rates their global impression of the effects of a study treatment during a 14-day double-blind efficacy evaluation period. The secondary endpoints are change in the investigator s assessment of worsening of disease symptoms and changes in walking speed (Timed 25-foot walking test) during the two-week, double-blind testing period. Further details regarding the trial and its design can be found on www.clinicaltrials.gov (NCT01377922).

Based on currently available information, we expect to complete enrollment in our Phase 3 trial in the first quarter of 2014 and to report top-line results from this trial during the third quarter of 2014. If the results are successful, we expect to submit all required modules of an NDA for Firdapse for the FDAs review by the middle of 2015.

We have hired a clinical research organization (CRO) to manage this Phase 3 trial for us, as well as independent subcontractors to assist in other aspects of trial management. We have also arranged to purchase sufficient supplies of Firdapse through BioMarin s supplier for the Phase 3 trial.

Breakthrough Therapy Designation

Firdapse for LEMS has been granted Breakthrough Therapy Designation by the FDA. Breakthrough Therapy Designation was enacted as part of the 2012 Food and Drug Administration Safety and Innovation Act (FDASIA).

FDASIA defines breakthrough therapy as a drug that is 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.

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A breakthrough therapy designation conveys all of the fast track program features, as well as more intensive FDA guidance on an efficient drug development program. The FDA also has an organizational commitment to involve senior management in such guidance. The Breakthrough Therapy Designation is a distinct status from both accelerated approval and priority review, which can also be granted to the same drug if relevant criteria are met.

Recent Type B Meeting with the FDA on Firdapse Development Program

In January 2014, we reported on the successful completion of a Type B meeting that we had with the FDA about Firdapse tablets. It was our first meeting with FDA since we became the sponsor of the IND for Firdapse. We provided FDA with an update on our development program for Firdapse and confirmed with FDA the clinical, non-clinical, and chemistry and manufacturing controls requirements that FDA will require to approve an NDA for Firdapse .

We provided a briefing package to the FDA that described all completed, in-progress, and planned pre-clinical studies, clinical studies, and drug manufacturing activities. This package included summaries of 54 pre-clinical studies, six clinical studies, and information related to drug manufacturing (the clinical supplies and the to-be marketed commercial product). The FDA concurred that our completed, in-progress, and planned development activities represented a nearly complete package of information that would be needed for a complete NDA. They also confirmed that they will allow us to submit the required NDA modules as completed in anticipation of receiving a priority review of our NDA for Firdapse .

We also agreed that as part of our NDA filing package for Firdapse , we will submit data from additional *in vitro* pre-clinical studies. Based on the discussions at this meeting and based on past communications and meetings with the FDA about Firdapse , all of these studies and remaining development activities constitute information needed to file a complete NDA and seek approval for Firdapse . We do not anticipate that these additional studies will impact our NDA filing timeline or materially add to our forecast of the aggregate development costs for Firdapse .

The Company and FDA also discussed the acceptability of the primary and secondary endpoints specified in the protocol for the ongoing Phase 3 trial. FDA requested a slight modification in the analyses to be conducted for the endpoints, which we believe will not require any changes in the data being collected or the number of patients needed to complete enrollment.

Data Monitoring Committee

As part of the Phase 3 trial, a data monitoring committee (DMC) has been established to oversee the trial. The DMC is a group of experts responsible for the independent review of accumulated clinical safety and efficacy data obtained in our clinical trial, in order to safeguard the interests and safety of participants and future patients. The DMC, which meets regularly during the pendency of the trial, considers study-specific data, as well as relevant background knowledge about the disease, test agent or patient population under study.

In March 2013 and October 2013, we announced that the DMC had met and recommended that we continue the trial as planned based on the DMC s review of safety and clinical data from the trial.

Orphan drug designation

Amifampridine phosphate for LEMS has been granted Orphan Drug Designation by the FDA for the treatment of LEMS, making the drug eligible to be granted seven-years marketing exclusivity for this indication if we are the first pharmaceutical company to obtain approval of an NDA for a product containing amifampridine as the active moiety

for the treatment of LEMS.

Intellectual property protections for Firdapse

Under the BioMarin License Agreement, we licensed two pending patents and certain trademarks for Firdapse . One of the licensed patents is a pending composition of matter patent that, if issued, will protect Firdapse until February 2027, which includes five years of patent term extension that is expected under the Patent Term Restoration Act. This application was initially rejected

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following an appeal to the Patent Trial and Appeal Board. The application was recently refiled with new claims and is awaiting examination. There can be no assurance that this patent will be issued. We may also pursue other patents in order to seek to protect the exclusivity of the drug, dosage forms and methods of administration.

No drug product containing amifampridine for any indication has been approved by the FDA. Therefore, our version of amifampridine, if we are the first to obtain approval of the product in the U.S., will be eligible for five-years new chemical entity exclusivity, which provides a five-year period of marketing exclusivity for all indications.

We have licensed the FIRDAPSE trademark from BioMarin. A trademark application for FIRDAPSE was allowed but did not register due to the inability to show use of the mark in interstate commerce. The application was refiled and FIRDAPSE should be registrable once we can show use of the mark in interstate commerce, which is expected to occur in 2014. In January 2014, the FDA provisionally approved Firdapse as a proprietary name for amifampridine. This provisional approval by the FDA does not stop the FDA from rejecting the name FIRDAPSE at a later date.

CPP-115

Product Overview

In August 2009, we licensed the exclusive worldwide rights to commercialize certain composition of matter patents relating to a new class of novel GABA aminotransferase inhibitors and derivatives of vigabatrin. We intend to develop these compounds for a broad range of neurological illnesses that could benefit from the inhibition of GABA aminotransferase. CPP-115 is our lead compound from this group of composition of matter patents.

The development efforts of CPP-115 were led by Dr. Richard B. Silverman, the John Evans Professor of Chemistry at Northwestern University (Northwestern). Dr. Silverman, who holds 52 patents, is the inventor of pregabalin, also known as Lyrica®, which is marketed by Pfizer. His goal in inventing the compound that became CPP-115 was to mimic the mechanism of action of vigabatrin, while making it both more potent and specific.

CPP-115 works by the same mechanism of action as vigabatrin; that is, the inhibition of GABA aminotransferase, which leads to increased brain GABA levels that reduce epileptogenesis. Due to these similarities, we believe that these two drugs will share a number of biochemical features related to absorption, metabolism, and elimination, and our non-clinical studies of CPP-115 to date support our expectations. In addition, non-clinical data of CPP-115 indicate that there may be a significant reduction, and possibly elimination, of VFDs from the use of CPP-115 compared to vigabatrin. However, there can be no assurance that this will ultimately prove to be the case.

Further, based on animal testing to date, CPP-115 has been shown to be at least 200 times more potent than vigabatrin in both in-vitro and animal model studies. The increased potency could enable the development of dosage forms potentially administrable by other routes of administration compared with the marketed oral, immediate release formulation of vigabatrin, Sabril®. Further, based on non-clinical testing completed to date, CPP-115 appears to have superior specificity to GABA aminotransferase and we believe, will have a better side effect profile (e.g. less visual field defects) compared with Sabril®.

CPP-115 has been granted Orphan Drug Designation in the U.S. for the treatment of infantile spasms. CPP-115 has also been granted Orphan Medicinal Product Designation in the EU to treat West Syndrome (a form of infantile spasms).

Mechanism of action for CPP-115

We believe that our drug candidate, CPP-115, will be an effective treatment for epilepsy because it increases endogenous GABA levels in the brain through the inhibition of GABA-aminotransferase (GABA-AT). GABA-AT is responsible for the eventual breakdown of GABA and helps to balance its inhibitory effects.

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CPP-115 is a GABA analog that is readily absorbed and promptly available to the nervous system, producing effects that last for many hours after a single dose. Due to the fact that this drug is not receptor active , its administration does not appear to affect the baseline levels of dopamine, nor those variations in dopamine levels caused by normal stimuli.

Epilepsy

Epilepsy is a brain disorder in which clusters of nerve cells, or neurons, in the brain sometimes signal abnormally. In epilepsy, the normal pattern of neuronal activity becomes disturbed, causing strange sensations, emotions, and behavior or sometimes convulsions, muscle spasms, and loss of consciousness. Epilepsy is a disorder with many possible causes. Anything that disturbs the normal pattern of neuron activity from illness to brain damage to abnormal brain development can lead to seizures. Epilepsy may develop because of an abnormality in brain wiring, an imbalance of nerve signaling chemicals called neurotransmitters, imbalance of sensitivity to neurotransmitters, or some combination of these factors. We intend to focus our development efforts for CPP-115 on its use as a treatment for infantile spasms (West Syndrome) and adult complex partial seizures.

An infantile spasm is a specific type of seizure seen in an epilepsy syndrome of infancy and childhood. The onset of infantile spasms is usually in the first year of life, typically between 4-8 months. The seizures primarily consist of a sudden bending forward of the body with stiffening of the arms and legs; some children arch their backs as they extend their arms and legs. Spasms tend to occur upon awakening or after feeding, and often occur in clusters of up to 100 spasms at a time. Infants may have dozens of clusters and several hundred spasms per day. Infantile spasms usually stop by age five, but may be replaced by other seizure types.

In complex partial seizures, consciousness is altered. Patients may exhibit automatisms (automatic repetitive behavior) such as walking in a circle, sitting and standing, or smacking their lips together. Often accompanying these symptoms are the presence of unusual thoughts, such as the feeling of déjà vu, uncontrollable laughing, fear, visual hallucinations, and experiencing unusual unpleasant odors. These symptoms are thought to be caused by abnormal discharges in the temporal lobe.

According to the Epilepsy Foundation, there are about 2.5 million epilepsy patients in the United States, with approximately 180,000 new cases diagnosed in the U.S. each year. Worldwide, 50 million people are estimated to have epilepsy. The incidence of epilepsy appears to depend somewhat on the age of the individual. The risk of epilepsy from birth through age 20 is approximately 1%. Within this group, incidence is highest during the first year of life and increases somewhat at the onset of puberty. From age 20 to 55 it decreases again, but increases after age 55.

Anti-epileptic drugs work through a variety of mechanisms, including inhibition of sodium ion channels and the enhancement of GABA mechanisms. Although the different types of epilepsy vary greatly, in general, available medications can only control seizures in about two-thirds of patients. CPP-115, like vigabatrin, is a GABA-AT inhibitor, and we are developing it initially for infantile spasms (West Syndrome) and refractory complex partial seizures. Based on the historic use of vigabatrin in treating epilepsy, we believe that CPP-115 may ultimately work best as an adjunct therapy to existing drugs.

Vigabatrin has been marketed for decades in over 30 countries by Lundbeck Inc. (Lundbeck) and Sanofi-Aventis and their predecessors and licensees under the brand names Sabril®, Sabrilex® and Sabrilan® (hereinafter referred to as Sabril®) as an adjunct (add-on) treatment for adult epilepsy and as a primary treatment for the management of infantile spasms. The composition of matter patents for Sabril® in the U.S. expired more than ten years ago. On August 21, 2009, the FDA approved two NDAs for Sabril® for the treatment of infantile spasms and as an adjunctive therapy for adult patients with refractory complex partial seizures who have failed treatments with several other anti-epileptic

drugs. The NDAs are for different formulations of Sabril® and both NDAs are held by Lundbeck and Sanofi-Aventis. Due to the risks of visual field damage associated with vigabatrin, Sabril® was approved under an FDA-mandated Risk Evaluation and Mitigation Strategy (REMS) program and is only available through a special restricted distribution program approved by the FDA.

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In chronic use for the treatment of epilepsy, vigabatrin has been generally well tolerated with lower than average neurological side effects compared to other approved epilepsy therapies. The most common side effects reported have been drowsiness and fatigue. However, one clearly established adverse side effect is the development, of peripheral visual field defects, or VFDs. VFDs occur in approximately 33% of users when cumulative dosage levels of vigabatrin approach approximately 1,500 grams. These VFDs are manifest as a constriction of the peripheral field of vision (i.e., tunnel vision).

Our completed clinical and non-clinical studies of CPP-115 to date

On November 1, 2010, we announced key results for our initial series of safety and efficacy evaluations of CPP-115 in a number of animal and in-vitro laboratory studies. These results included superior visual safety of CPP-115, compared to vigabatrin, pharmacokinetic data supporting oral administration of CPP-115, pharmacologic target specificity, metabolic profile, and an absence of genotoxic, cardiovascular, respiratory, and liver enzyme side effects. It was also shown to be effective in multiple animal models for epilepsy and cocaine addiction.

On May 22, 2012, we reported positive results from a Phase 1(a) double-blind, placebo-controlled clinical trial evaluating the safety, tolerability and pharmacokinetic profile of CPP-115. The study evaluated single ascending doses ranging from 5 mg to 500 mg (a dose greater than ten times the predicted effective dose of 15-30 mg/day derived from animal data) of CPP-115 solution administered orally to 55 healthy volunteers. CPP-115 was found to be well tolerated with no side effects, rapidly absorbed and eliminated, and exhibited linear, dose depended pharmacokinetics.

Upcoming Phase 1(b) Clinical Trial of CPP-115

We expect to commence during the first half of 2014 a dose ranging study and a Phase 1(b) multiple ascending dose study of CPP-115. After the Phase 1(b) study, we expect to undertake the non-clinical studies of CPP-115 that will be required to support a Phase 2 study of CPP-115 evaluating its efficacy as a treatment for infantile spasms and/or Tourette s Disorder, assuming our Phase 1(b) study is successful. The Phase 1(b) study will be a randomized, double-blind, placebo-controlled, safety, tolerability and pharmacokinetic study of multiple ascending oral doses of CPP-115 in healthy volunteers. The primary objective will be to evaluate the safety and tolerability of multiple ascending oral doses of CPP-115. Secondary objectives will be to determine the pharmacokinetic profile of CPP-115 and to determine the effects of CPP-115 on brain GABA levels as measured by GABA-MRS (GABA-Magnetic Resonance Spectroscopy) following administration of multiple oral daily doses. The dose ranging study will be conducted using GABA-MRS in order to establish the correct doses for the Phase 1(b) study.

Clinical and Pre-Clinical Studies of CPP-115 Undertaken by Others

The primary focus of our product development efforts is on our clinical trials and pre-clinical studies. However, we have in the past supported and will continue in the future to support pre-clinical studies and clinical trials and studies by academic investigators (including members of our scientific advisory committee and academic institutions with which they are affiliated) of the use of our product candidates that we believe might further the understanding or increase the value of our product candidates.

In some cases, in the past, we have provided unrestricted sponsorship funds for such studies and we may do so again in the future. In other cases, we have provided, and may in the future provide, alternative assistance to the investigator, most typically providing drug substance or dosage form as well as matching placebo. We expect to continue supporting investigator studies in the future to the extent that they meet criteria acceptable to us. Such criteria include research on the use of CPP-115 to treat various forms of epilepsy and/or other neurological disorders, to assist

investigators in designing their studies so that such studies are most appropriately conducted and, to the extent possible, to make sure that these investigator studies potentially complement, and do not adversely impact, our activities.

An animal study reporting positive pre-clinical efficacy in a rat multiple hit model in which the use of CPP-115 was evaluated for the treatment of infantile spasms was published during November 2013 online as an early view in the journal, *Epilepsia*. The study was authored by Stephen W. Briggs, Tomonori Ono, MD, PhD, Solomon L. Moshe, MD and Aristea S. Galanopoulou, MD, PhD of the Saul R. Korey Department of Neurology, Dominick P. Purpura Department of Neuroscience, Laboratory of

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Developmental Epilepsy, The Comprehensive Epilepsy Center (CEC) at Montefiore Medical Center / Albert Einstein College of Medicine of Yeshiva University, Bronx, New York. The study concluded that (i) CPP-115 suppresses spasms in the multiple-hit model of infantile spasm, with onset of effect as early as the day after the first dose; (ii) the therapeutic doses of CPP-115 were well tolerated in developing rat pups; and (iii) CPP-115 showed efficacy for a longer duration at lower doses that were better tolerated than the previously tested therapeutic vigabatrin doses.

CPP-115 has also been submitted to the Anticonvulsant Screening Program (ASP) of the National Institute of Neurological Disorders and Stroke (NINDS), one of the institutes within the National Institutes of Health (NIH). To date, CPP-115 has been tested in about 20 animal models of epilepsy, including maximal electric shock (MES) in both rats and mice, corneal kindling in mice, minimal clonic seizure (6 Hz) model in mice, and subcutaneous picrotoxin (scPIC). CPP-115 was also evaluated for potential efficacy in neuroprotection and neuropathic pain models. CPP-115 has shown significant potential in a variety of epilepsy models. Due to change in focus and budgetary constraints, the ASP has suspended further testing of a variety of potential anticonvulsant drugs, including CPP-115. Samples of CPP-115 remain on file at NIH, and we will provide additional material to the NIH upon request for future testing, should it be resumed. There can be no assurance that the ASP will conduct any further testing of CPP-115.

Northwestern University License Agreement

On August 27, 2009, we entered into a license agreement with Northwestern University (Northwestern), under which we acquired worldwide rights to commercialize new GABA aminotransferase inhibitors and derivatives of vigabatrin which have been discovered and patented by Northwestern. Under the terms of the license agreement, Northwestern granted us an exclusive worldwide license to certain composition of matter patents related to the new class of inhibitors and a patent application relating to derivatives of vigabatrin. We have designated the lead compound to be developed under this license as CPP-115.

We believe that these licensed compounds are the only known GABA aminotransferase inhibitors in existence or in development other than vigabatrin. We also believe, based on our non-clinical testing to date of CPP-115, that the newly licensed compounds are significantly more potent than vigabatrin with less visual side effects than vigabatrin. We plan to seek to develop these compounds for the treatment of several indications, including epilepsy (specifically, complex partial seizures and infantile spasms). However, these compounds are at an early stage of development and there can be no assurance as to whether these new compounds will ever be determined to be safe and effective.

Under our license agreement with Northwestern, we will be responsible for continued research and development of any resulting product candidates. We have the right to terminate the agreement in whole or in part after August 27, 2012, upon written notice. As of December 31, 2013, we have paid Northwestern upfront payments, milestone fees and maintenance and patent fees aggregating $246,590, and we are obligated to pay certain additional fees and milestone payments in future years relating to our clinical development activities under this license or payable upon passage of time. The next milestone payment of $150,000 is due on the earlier of successful completion of the Phase 2 clinical trial for CPP-115 or August 27, 2015. We are also obligated to pay Northwestern royalties on any products resulting from the license agreement. We also have the right to enter into sub-license agreements, and if we do, a royalty on any sub-license fees will be payable to Northwestern.

We have filed applications seeking to protect methods of using CPP-115 in the U.S., Europe and Canada. Prosecution of this patent is ongoing. There can be no assurance that the claims of this patent will be allowed, or if allowed, that such claims will provide adequate patent protection for CPP-115.

CPP-109

CPP-109 for Addiction

For several years, we evaluated CPP-109 (our formulation of vigabatrin, another GABA aminotransferase inhibitor) for the treatment of cocaine addiction. However, in November 2012, we reported that CPP-109 failed to meet the primary and two key secondary endpoints in a Phase 2(b) trial for cocaine addiction. As a result, we are no longer focusing our efforts on evaluating CPP-109 for addiction.

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Brookhaven License Agreement

In 2002, we entered into an exclusive, worldwide license from Brookhaven National Laboratory (Brookhaven) to nine patents relating to the use of vigabatrin for a range of indications, including the treatment of a wide variety of substance addictions, with expiration dates for the issued patents between 2018 and 2023, with the principal patents expiring in 2018. Following our decision not to focus our efforts on evaluating CPP-109 for addiction, on November 8, 2013, effective October 1, 2013, we terminated our license agreement with Brookhaven.

CPP-109 and CPP-115 for the treatment of Tourette Syndrome and related license agreement

We, as a co-inventor, with scientists at New York University and the Feinstein Institute for Medical Research, have filed a patent application under the Patent Cooperation Treaty with the U.S. Patent and Trademark Office for the use of GABA aminotransferase inhibitors, including CPP-109 and CPP-115, in the treatment of Tourette Syndrome. We have also entered into a license agreement with NYU and the Feinstein Institute granting us worldwide rights with respect to such patent. We expect that this application, which is a class patent and covers all GABA-AT inhibitors, including CPP-109 and CPP-115, will likely undergo national stage filing in July 2014.

Tourette Syndrome is a psychiatric disorder which usually has its onset in children or adolescents. Tourette Syndrome is generally defined by multiple motor and vocal tics lasting for more than one year. The first symptoms are usually involuntary movements (tics) of the face, arms, limbs, or trunks, and are frequent, repetitive and rapid. The most common first symptom is a facial tic (for example, eye blinking) and is replaced or added to by other tics of the neck, trunk, and limbs. There can also be verbal tics that occur with the movements, including vocalizations such as grunting, throat clearing, shouting, and barking.

Tourette Syndrome is generally treated by a combination of therapy and psychiatric medication. Tics can be treated with medications such as clonidine (Catapres®), haloperidol (Haldol®), pimazide (Orap®), or fluphenazine (Prolixin®). Medications used to treat Obsessive Compulsive Disorder can also be used, such as clomipramine (Anafranil®), fluoxetine (Prozac®) and sertraline (Zoloft®), as well as stimulants used to treat ADHD, a disorder commonly comorbid with Tourette Syndrome, such as methylphenidate (Ritalin®), pemoline (Cylert®) and dextroamphetamine (Dexadrine®).

We have provided CPP-109 and financial support for a small Phase 1 proof-of-concept study being undertaken at Mt. Sinai School of Medicine in New York to evaluate the use of CPP-109 in treating patients with refractory Tourette Syndrome. This is a 6-10 patient, open-label study. Since this is an academic investigator proof-of-concept study, we do not control the study and therefore we have no control over its timing. However, based on currently available information, we expect to receive the results from this proof-of-concept study during 2014. If the trial results show evidence of reduced numbers of tics, we hope to develop CPP-109 and/or CPP-115 for this indication.

Intellectual Property Rights

Licensing and Patents

Protection of our intellectual property and proprietary technology is a strategic priority for our business. We rely on a combination of patent, trademark, copyright and trade secret laws along with institutional know-how and continuing technological advancement, to develop and maintain our competitive position. Our ability to protect and use our intellectual property rights in the future development and commercialization of our products, operate without infringing the proprietary rights of others, and prevent others from infringing our proprietary rights, is crucial to our future success. See Item 1A., Risk Factors Risks Related to Our Intellectual Property.

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Manufacturing and Supply

Firdapse

We have sufficient stock of Firdapse in hand to complete our ongoing Phase 3 clinical trial of Firdapse . We have entered into agreements with a supplier of the active pharmaceutical ingredient (API) contained in Firdapse for future requirements and we have identified the third-party contract manufacturer who we expect will manufacture Firdapse tablets for us if Firdapse is approved for commercialization.

Any NDA that we file for Firdapse will require a manufacturing plan. If the manufacturing plan and data are insufficient, any NDA we may file will not be approved. Before an NDA can be approved, our manufacturer must also demonstrate compliance with FDA s good manufacturing practices (cGMP) regulations and policies. Further, even if we receive approval of an NDA for Firdapse , if our manufacturer does not follow cGMP in the manufacture of our products, it may delay product launches or shipments or adversely affect our business.

Since we intend to contract with a third party to manufacture our products, if the FDA approves an NDA for Firdapse , our contract manufacturer will be required to comply with all applicable environmental laws and regulations that affect the manufacturing process. As a result, we do not believe that we will have any significant exposure to environmental issues.

CPP-115

We have entered into a contract to manufacture the API sufficient to meet the needs of our ongoing and planned pre-clinical and clinical studies of CPP-115. While we believe that we have ordered and obtained sufficient API for our upcoming studies, there can be no assurance of this.

We have no plans at this time to build or acquire the manufacturing capability needed to prepare either the CPP-115 API or CPP-115 product on a commercial scale. We expect at this time that these materials will be prepared by a contractor with suitable capabilities for these tasks and that we will enter into appropriate supply agreements with these contractors at appropriate times in the development and commercialization of this product. There are no plans at this time to enter into such agreements. Further, the contractors selected would have to be inspected by the FDA and found to be in substantial compliance with federal regulations in order for an NDA for CPP-115 to be approved, and there can be no assurance that the contractors we select in the future would pass such an inspection.

CPP-109

Consistent with our discontinuation of our efforts to further evaluate CPP-109 for addiction, we have shut down our supply activities as well. However, we have retained sufficient CPP-109 to allow for the completion of the Tourette Syndrome proof-of-concept study described above.

Sales and Marketing

We have not obtained regulatory approval for any of our product candidates and thus have not yet established a commercial organization or distribution capabilities. Due to the rare nature of LEMS and the lack of an FDA approved, effective treatment, patients suffering from LEMS, together with their physicians, often have a high degree of organization and are well informed, which may simplify the identification of a target population for Firdapse if it is approved. We believe that, if approved for commercial sale, it will be possible to commercialize Firdapse with a relatively small specialty sales and marketing force that calls on the physicians, foundations and other

patient-advocacy groups focused on LEMS. Our current expectation is to commercialize Firdapse ourselves in the United States, and we plan to recruit a sales and marketing force and take other steps to establish the necessary commercial infrastructure at such time as we believe that Firdapse is approaching regulatory approval. However, we may also consider entering into arrangements with other pharmaceutical or biotechnology companies for the marketing and sale of Firdapse in Canada or Mexico, where we have also licensed the product.

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Competition

The pharmaceutical industry is intensely competitive, and any product candidate developed or licensed by us would likely compete with existing drugs and therapies. There are many pharmaceutical companies, biotechnology companies, public and private universities, government agencies and research organizations that compete with us in developing various approaches to the treatment of orphan diseases. Many of these organizations have substantially greater financial, technical, marketing and manufacturing resources than we have. Several of them have developed or are developing therapies that are used for the treatment of the same conditions that we are targeting. In addition, many of these competitors have substantially greater commercial infrastructure than we have.

Firdapse for LEMS

Treatments for LEMS include steroids, azathioprine and intravenous immunoglobulin, which work by suppressing the immune system, and pyridostigmine and Firdapse , which enhance neuromuscular transmission. Plasma exchange has also been used in an attempt to remove antibodies from the body. Neither Firdapse , nor any of the current treatments for LEMS, operate by treating the underlying disease. Firdapse allows the nerves to better transmit electrical impulses to the muscles through its mechanism as a voltage-gated potassium channel blocker. One other aminopyridine, dalfampridine (4-AP), has been approved in the U.S. for marketing by another pharmaceutical company and is sold under the trade name Ampyra®. However, it is indicated to improve walking in patients with Multiple Sclerosis. Clinical testing regarding the role of dalfampridine in LEMS has suggested that it is less effective with a higher incidence of side effects when compared to amifampridine. Further, one other product, guanidine HCl tablets, was approved for use in the treatment of LEMS many years ago. However, it has significant side effects and, in our view, it is not currently viewed as an effective treatment for the disease. Notwithstanding, drugs may be prescribed by physicians for the treatment of LEMS whether or not they are considered effective.

In January of 2012, another pharmaceutical company, Jacobus Pharmaceutical, began its own Phase 2 trial studying their own formulation of amifampridine for the treatment of LEMS. While there can be no assurance we believe that Firdapse is further along in development than this other company s version of amifampridine. Under the Orphan Drug Act of 1983, the first pharmaceutical product to get approval for an indication receives the orphan exclusivity under the statute. If this other pharmaceutical company is able to receive approval of an NDA for its formulation of amifampridine for the treatment of LEMS before we are able to receive approval of Firdapse 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 this other company 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. Further, we are aware that Jacobus Pharmaceutical has been making 3,4-DAP available to LEMS patients under compassionate use INDs for a number of years and we believe that approximately 200 LEMS patients have received the drug in this manner. Even if we are the first to obtain an approval for this product, we may not be able to stop Jacobus from continuing to supply patients under compassionate use INDs.

Finally, we are aware that amifampridine, the active ingredient in Firdapse , has been available from compounding pharmacies for many years and will likely remain available even if we are able to obtain FDA approval of Firdapse . Compounded amifampridine is likely to be substantially less expensive than Firdapse .

The Food and Drug Administration Modernization Act of 1997 included a new section, which clarified the status of pharmacy compounding under Federal law. Under section 503A, drug products that are compounded by a pharmacist or physician on a customized basis for an individual patient may be entitled to exemptions from three key provisions of the act: (1) the adulteration provision of section 501(a)(2)(B) (concerning the good manufacturing practice

requirements); (2) the misbranding provision of section 502(f)(1) (concerning the labeling of drugs with adequate directions for use); and (3) the new drug provision of section 505 (concerning the approval of drugs under new drug or abbreviated new drug applications).

To qualify for these statutory exemptions, a compounded drug product must satisfy several requirements. One of these requirements restricted the universe of bulk drug substances that a compounder may use; i.e. that every bulk drug substance used in compounding: (1) must comply with an applicable and current USP or NF monograph, if one exists, as well as the current USP chapter on pharmacy compounding; (2) if such a monograph does not exist, the bulk drug substance must be a component of an

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FDA-approved drug; or (3) if a monograph does not exist and the bulk drug substance is not a component of an FDA-approved drug, it must appear on a list of bulk drug substances that may be used in compounding (i.e., the bulk drugs list). While Section 503 was ruled unconstitutional by the Supreme Court in 2002, the FDA has continued to oversee the practice of compounding under a compliance policy guide utilizing its discretion under the principles described above, and these principles were codified into a new section 503A passed by Congress as part of the Drug Quality and Security Act in 2013.

The FDA s Pharmacy Compounding Advisory Committee at its meeting on May 6-7, 1999 voted 7-4 against inclusion of 3,4-DAP on the bulk drugs list, largely based on the safety concerns and the commitment of Jacobus Pharmaceuticals to make the drug available under compassionate use INDs, while pursuing FDA approval. Therefore, the individual or firm that compounds a drug product containing 3,4-DAP may be subject to a warning letter, seizure of product, injunction, and/or criminal prosecution for violations of the Federal Food, Drug, and Cosmetic Act (FDCA).

We intend to take all steps available to us to try to enforce our marketing proprietary rights if we are the first company to obtain an approval for this product. However, we cannot determine with certainty what impact the above factors will have on the market for our product and whether we will be able to prevent distribution of 3,4-DAP by others even if we are able to obtain marketing exclusivity.

CPP-115 for Epilepsy

The market for epilepsy treatments is highly competitive. Large pharmaceutical companies, including Pfizer (Neurontin®, Lyrica®, Dilantin®, Zarontin®), J&J (Topamax®), UCB (Keppra®), Abbott (Depakote®), GSK (Lamictal®), Roche (Klonopin®), and Novartis (Trileptal®) sell, or are developing, epilepsy therapies. However, as stated earlier, approximately one-third of all epilepsy patients are refractory to treatment with any currently available epilepsy treatments. It is difficult to determine sales of products specifically for epilepsy as many of these products are used in other indications such as neuropathic pain, migraine, dementia, and bipolar disorders.

Factors to Consider Affecting Competition Generally

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

- our ability to complete clinical development and obtain regulatory approvals for our product candidates;
- the efficacy, safety and reliability of our product candidates;
- the timing and scope of regulatory approvals;
- product acceptance by physicians and other health care providers and patients;
- protection of our proprietary rights and the level of generic competition;

the speed at which we develop product candidates;

our ability to supply commercial quantities of a product to the market;

our ability to obtain reimbursement from private or public insurance entities for product use in approved indications;

our ability to recruit and retain skilled employees; and

the availability of capital resources to fund development and commercialization activities, including the availability of funding from the federal government.

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Regulatory Matters

Government Regulation and Product Approval

Government authorities in the United States, at the federal, state and local level, and other countries extensively regulate, among other things, the research, development, testing, manufacture, labeling, record-keeping, promotion, storage, advertising, distribution, marketing and export and import of products such as those we are developing. Our drugs must be approved by the FDA through the NDA process before they may be legally marketed in the United States.

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

- completion of pre-clinical laboratory tests, animal studies and formulation studies according to the FDA s good laboratory practice, or GLP, regulations;

- submission of an investigational new drug application, or IND, which must become effective before human clinical trials may begin and which must include approval by an institutional review board, or IRB, at each clinical site before the trials are initiated;

- performance of adequate and well-controlled human clinical trials to establish the safety and efficacy of the proposed drug for its intended use conducted in compliance with federal regulations and good clinical practice, or GCP, an international standard meant to protect the rights and health of patients and to define the roles of clinical trial sponsors, administrators, and monitors;

- submission to, and acceptance by, the FDA of an NDA;

- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the drug is produced to assess compliance with current good manufacturing practice, or cGMP, regulations to assure that the facilities, methods and controls are adequate to preserve the drug s identity, strength, quality and purity;

potential FDA audit of the non-clinical and clinical trial sites that generated the data in support of the NDA; and

FDA review and approval of the NDA.

United States Drug Development Process

Once a pharmaceutical candidate is identified for development it enters the pre-clinical testing stage. Pre-clinical tests include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies. Prior to beginning human clinical trials, an IND sponsor must submit an IND to the FDA. The IND sponsor must submit the results of the pre-clinical tests, together with manufacturing information and analytical data, to the FDA as part of the IND. Some pre-clinical or non-clinical testing may continue even after the IND is submitted. In addition to including the results of the pre-clinical studies, the IND will also include a protocol detailing, among other things, the objectives of the clinical trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated, if the trial lends itself to an efficacy evaluation. The IND automatically becomes effective

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30 days after receipt by the FDA, unless the FDA, within the 30 day time period, raises concerns or questions about the conduct of the trial. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. The FDA may, at any time, impose a clinical hold on ongoing clinical trials. If the FDA imposes a clinical hold, clinical trials cannot commence or recommence without FDA authorization and then only under terms authorized by the FDA.

Clinical trials involve the administration of the investigation new drug to healthy volunteers or patients under the supervision of one or more qualified investigators in accordance with federal regulations and GCP.

Clinical trials must be conducted under protocols detailing the objectives of the trial and the safety and effectiveness criteria to be evaluated. Each protocol must be submitted to the FDA as part of the IND. Further, an Institutional Review Board (IRB) at each institution participating in the clinical trial must review and approve each protocol before any clinical trial commences at that institution. All research subjects must provide informed consent, and informed consent information must be submitted to the IRB for approval prior to initiation of the trial. Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if adverse events or other certain types of other changes occur.

Human clinical trials are typically conducted in three phases. A fourth, or post-approval, phase may include additional clinical studies. These phases generally include the following, and may be sequential, or may overlap or be combined:

Phase 1 clinical trials involve the initial introduction of the drug into human subjects. These studies are designed to determine the safety of usually single doses of the compound and determine any dose limiting intolerance, as well as evidence of the metabolism and pharmacokinetics of the drug in humans.

Phase 2 clinical trials usually involve studies in a limited patient population to evaluate the safety and efficacy of the drug for specific, targeted indications, to determine dosage tolerance and optimal dosage, and to identify possible adverse effects and safety risks.

In Phase 3, if a compound is found to be potentially effective and to have an acceptable safety profile in Phase 2 (or occasionally Phase 1) studies, the Phase 3 studies will be conducted to further confirm clinical efficacy, optimal dosage and safety within an expanded population which may involve geographically diverse clinical trial sites. Generally, but not always, two adequate and well-controlled Phase 3 clinical trials are required by the FDA for approval of an NDA.

Phase 4 clinical trials are studies required of or agreed to by a sponsor that are conducted after the FDA has approved a product for marketing. These studies are used to gain additional experience from the treatment of patients in the intended therapeutic indication and to document a clinical benefit in the case of drugs approved under accelerated approval regulations. If the FDA approves a product while a company has ongoing clinical trials that were not necessary for approval, a company may be able to use the data from these clinical trials to meet all or part of any Phase 4 clinical trial requirement. Failure to promptly conduct Phase 4 clinical trials where necessary could result in withdrawal of approval for products approved under accelerated approval regulations.

While Phase 1, Phase 2, and Phase 3 tests are generally required for approval of an NDA, certain drugs may not require one or more steps in the process depending on other testing and the situation involved. Additionally, the FDA, an IRB, or the sponsor may stop testing at any time if results show patients being exposed to unnecessary health risks or overly dangerous side effects.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the drug and finalize a process for manufacturing the product in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the drug candidate and, among other requirements, the manufacturer must develop methods for testing the identity, strength, quality and purity of the final drug. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the drug candidate does not undergo unacceptable deterioration over its shelf life.

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United States Review and Approval Process

FDA approval of an NDA is required before marketing of the product may begin in the United States. The NDA must include the results of product development, pre-clinical studies and clinical studies, together with other detailed information, including information on the chemistry, manufacture and composition of the product. The FDA has 60 days from its receipt of the NDA to review the application to ensure that it is sufficiently complete for substantive review before accepting it for filing. The FDA may request additional information rather than accept an NDA for filing. In this event, the NDA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The submission of an NDA is also subject to the payment of a substantial application fee (currently exceeding $2,169,000), although a waiver of such fee may be obtained under certain limited circumstances, including when the drug that is subject of the application has received Orphan Drug Designation for the indication sought. Further, the sponsor of an approved NDA is subject to annual product and establishment user fees, currently exceeding $104,000 per product and $554,000 per establishment. The approval process is lengthy and difficult and the FDA may refuse to approve an NDA if the applicable regulatory criteria are not satisfied or may require additional clinical or other data and information. Even if such data and information is submitted, the FDA may ultimately decide that the NDA does not satisfy the criteria for approval. The FDA may also refer applications for novel drug products or drug products which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee. The FDA reviews an NDA to determine, among other things, whether a product is safe and effective for its intended use. Before approving an NDA, the FDA will inspect the facility or facilities where the product is manufactured to determine whether its manufacturing is cGMP compliant to assure and preserve the product s identity, strength, quality, purity and stability.

If the FDA s evaluation of the NDA submission or manufacturing facilities is not favorable, the FDA will issue a complete response letter. The complete response letter outlines the deficiencies in the submission and often requires additional testing or information in order for the FDA to reconsider the application. Even after submitting this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. With limited exceptions, the FDA may withhold approval of a NDA regardless of prior advice it may have provided or commitments it may have made to the sponsor.

Post-Approval Requirements and Consideration

Once an NDA is approved, a product will be subject to certain post-approval requirements. For instance, the FDA closely regulates the post-approval marketing and promotion of drugs, including standards and regulations for direct-to-consumer advertising, off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the internet. As a condition of NDA approval, the FDA may also require a risk evaluation and mitigation strategy, or REMS, to help ensure that the benefits of the drug outweigh the potential risks. REMS can include medication guides, communication plans for the healthcare professionals, and elements to assure safe use, or ETASU. ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring, and the use of patient registries. The requirement for a REMS can materially affect the potential market and profitability of the drug.

Drugs may be marketed only for the approved indications and in accordance with the provisions of the approved labeling. Changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, require submission and FDA approval of a new NDA or NDA supplement before the change can be implemented. An NDA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing

NDA supplements as it does in reviewing NDAs.

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Adverse event reporting and submission of periodic reports is required following FDA approval of an NDA. The FDA also may require post-marketing testing, known as Phase 4 testing, and surveillance to monitor the effects of an approved product or place conditions on an approval that could restrict the distribution or use of the product. In addition, quality control as well as drug manufacture, packaging, and labeling procedures must continue to conform to cGMPs after approval. Drug manufacturers and certain of their subcontractors are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA during which the agency inspects manufacturing facilities to assess compliance with cGMPs. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality control to maintain compliance with cGMPs. Regulatory authorities may withdraw product approvals or request product recalls if a company fails to comply with regulatory standards, if it encounters problems following initial marketing, or if previously unrecognized problems are subsequently discovered.

Other Regulatory Requirements

In addition to regulation by the FDA and certain state regulatory agencies, we are also subject to a variety of foreign regulations governing clinical trials and the marketing of other products. Outside of the United States, our ability to market a product depends upon receiving a marketing authorization from the appropriate regulatory agencies. The requirements governing the conduct of clinical trials, marketing authorization, pricing and reimbursement vary widely from country to country. In any country, however, we will only be permitted to commercialize our products if the appropriate regulatory agency is satisfied that we have presented adequate evidence of safety, quality and efficacy. Whether or not FDA approval has been obtained, approval of a product by the comparable regulatory authorities of foreign countries must be obtained prior to the commencement of marketing of the product in those countries. The regulatory approval and oversight process in other countries includes all of the risks associated with regulation by the FDA and certain state regulatory agencies as described above.

Under the European Union regulatory system, applications for drug approval may be submitted either in a centralized or decentralized manner. Under the centralized procedure, a single application to the European Medicines Agency leads to an approval granted by the European Commission which permits marketing of the product throughout the European Union. The decentralized procedure provides for mutual recognition of nationally approved decisions and is used for products that do not comply with requirements for the centralized procedure. Under the decentralized procedure, the holders of national marketing authorization in one of the countries within the European Union may submit further applications to other countries within the European Union, who will be requested to recognize the original authorization based on an assessment report provided by the country in which marketing authorization is held.

Pharmaceutical Pricing and Reimbursement

In both US and foreign markets, our ability to commercialize our products successfully, and to attract commercialization partners for our products, depends in significant part on the availability of adequate financial coverage and reimbursement from third-party payors, including, in the United States, governmental payors such as Medicare and Medicaid, managed care organizations, and private health insurers. Third party payors are increasingly challenging the prices charged for medicines and examining their cost effectiveness, in addition to their safety and efficacy. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the cost effectiveness of our products. Even with the availability of such studies, our products may be considered less safe, less effective or less cost-effective than alternative products, and third party payors may not provide coverage and reimbursement for our product candidates, in whole or in part.

Political, economic and regulatory influences are subjecting the health care industry in the United States to fundamental changes. There have been, and we expect there will continue to be, legislative and regulatory proposals to

change the healthcare system in ways that could significantly affect our business, including the Patient Protection and Affordable Care Act of 2010. We anticipate that in the US, Congress, state legislatures, and private sector entities will continue to consider and may adopt healthcare policies intended to curb rising healthcare costs. These cost containment measures include:

controls on government-funded reimbursement for drugs;

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controls on healthcare providers;

challenges to the pricing of drugs or limits or prohibitions on reimbursement for specific products through other means;

reform of drug importation laws; and

expansion of use of managed-care systems in which healthcare providers contract to provide comprehensive healthcare for a fixed cost per person.

We are unable to predict what additional legislation, regulations or policies, if any, relating to the healthcare industry or third-party coverage and reimbursement may be enacted in the future or what effect such legislation, regulations or policies would have on our business. Any cost containment measures, including those listed above, or other healthcare system reforms that are adopted may have a material adverse effect on our business prospects.

Orphan Drug Designation

Some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983 (ODA), the FDA may grant Orphan Drug Designation to drugs intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making available in the United States a drug for this type of disease or condition will be recovered from sales in the United States for that drug. In the United States, Orphan Drug Designation must be requested before submitting an application for marketing approval. An Orphan Drug Designation does not shorten the duration of the regulatory review and approval process. The grant of an Orphan Drug Designation request does not alter the standard regulatory requirements and process for obtaining marketing approval. Safety and efficacy of a compound must be established through adequate and well-controlled studies. If a product which has been granted Orphan Drug Designation subsequently receives the first FDA approval for the indication for which it has such designation, the product is entitled to an orphan drug exclusivity period, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven (7) years, except in limited circumstances, such as where an alternative product demonstrates clinical superiority to the product with orphan exclusivity. In addition, holders of exclusivity for orphan drugs are expected to assure the availability of sufficient quantities of their orphan drugs to meet the needs of patients. Failure to do so could result in the withdrawal of marketing exclusivity for the drug. An additional six (6) months of exclusivity may be granted to the sponsor of an NDA, if the sponsor conducted certain pediatric studies of such product. This process is initiated by the FDA as a written request for pediatric studies that applies to the sponsor s product. If the sponsor conducts qualifying studies and the studies are accepted by the FDA within the statutory timeframe, then an additional six months of pediatric exclusivity will attach to any other regulatory exclusivity or patient protection applicable to any drug product containing the same active moiety as the drug studied and for which the party submitting the studies holds the NDA.

The European Orphan Drug Regulation is considered for drugs intended to diagnose, prevent or treat a life-threatening or very serious condition afflicting five or fewer per 10,000 people in the EU, including compounds that for serious and chronic conditions would likely not be marketed without incentives due to low market return on the sponsor s development investment. The medicinal product considered should be of significant benefit to those affected by the condition. Benefits of being granted Orphan Medicinal Product Designation are significant, including eight years of data exclusivity, two years of marketing exclusivity and a potential one-year extension of both. The EU Community

and Member States may not accept or grant for ten years a new marketing authorization or application for another drug for the same therapeutic indication as the orphan drug, although the ten year period can be reduced to six years if, after the end of the fifth year, available evidence establishes that the product is sufficiently profitable not to justify maintenance of the marketing exclusivity. A supplementary protection certificate may extend the protection six months beyond patent expiration if that is later than the orphan drug exclusivity period. To apply for the supplementary protection, a pediatric investigation plan, or PIP, must be included in the market application. In Europe all drugs now seeking marketing authorization need to have a PIP

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agreed with the European Medicines Agency (EMA) before it can be approved, even if it is a drug being developed specifically for a pediatric indication. If a product is developed solely for use in the pediatric population, then a Pediatric Use Marketing Authorization, or PUMA, may provide eight years of data exclusivity and ten years of marketing exclusivity.

Breakthrough Therapy Designation

Breakthrough therapy designation is intended to expedite the development and review of drugs for serious or life-threatening conditions. The criteria for breakthrough therapy designation require preliminary clinical evidence that demonstrates the drug may have substantial improvement on at least one clinically significant endpoint over available therapy. A breakthrough therapy designation conveys all of the fast track program features, as well as more intensive FDA guidance on an efficient drug development program. The FDA also has an organizational commitment to involve senior management in such guidance. Section 902 of the Food and Drug Administration Safety and Innovation Act (FDASIA) provides that actions taken to expedite development may include the following actions, as appropriate:

- holding meetings with the sponsor and review team throughout the development of the drug;
- providing timely advice to, and interactive communication with, the sponsor regarding the development of the drug to ensure that the development program to gather the pre-clinical and clinical data necessary for approval is as efficient as possible;
- taking steps to ensure that the design of the clinical trials is as efficient as practicable, when scientifically appropriate, such as by minimizing the number of patients exposed to a potentially less efficacious treatment;
- assigning a cross-disciplinary project lead for the FDA review team to facilitate an efficient review of the development program and to serve as a scientific liaison between the cross-discipline members of the review team (i.e., clinical, pharmacology-toxicology, chemistry, manufacturing and control (CMC), compliance) for coordinated internal interactions and communications with the sponsor through the review division s Regulatory Health Project Manager; and
- involving senior managers and experienced review staff, as appropriate, in a collaborative, cross-disciplinary review.

Priority Review

Under FDA policies, a drug candidate is eligible for priority review, or review within a six to eight-month time frame from the time a complete NDA is submitted, if the drug candidate is intended for the treatment, diagnosis or prevention of a serious or life-threatening condition, demonstrates the potential to address an unmet medical need, or provides a significant improvement compared to marketed drugs.

Disclosure of Clinical Trial Information

Sponsors of clinical trials of FDA-regulated products, including drugs, are required to register and disclose certain clinical trial information. Information related to the product, patient population, phase of investigation, study sites and investigators, and other aspects of the clinical trial is then made public as part of the registration. Sponsors are also obligated to discuss the results of their clinical trials after completion. Disclosure of results of these trials can be delayed until the new product or new indication being studied has been approved. Competitors may use this publicly-available information to gain knowledge regarding the progress of development programs.

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Anti-Kickback, False Claims Laws & The Prescription Drug Marketing Act

In addition to FDA restrictions on marketing of pharmaceutical products, other state and federal laws have been applied to restrict certain marketing practices in the pharmaceutical industry in recent years. These laws include anti-kickback statutes and false claims statutes. The federal healthcare program anti-kickback statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers on the other. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties and exclusion from participation in federal healthcare programs. Although there are a number of statutory exemptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor.

Federal false claims laws prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. Recently, several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly inflating drug prices they report to pricing services, which in turn were used by the government to set Medicare and Medicaid reimbursement rates, and for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws. The majority of states also have statutes or regulations similar to the federal anti-kickback law and false claims laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payer.

As part of the sales and marketing process, pharmaceutical companies frequently provide samples of approved drugs to physicians. The Prescription Drug Marketing Act, or the PDMA, imposes requirements and limitations upon the provision of drug samples to physicians, as well as prohibits states from licensing distributors of prescription drugs unless the state licensing program meets certain federal guidelines that include minimum standards for storage, handling, and record keeping. In addition, the PDMA sets forth civil and criminal penalties for violations.

Our Employees

As of March 14, 2014 we had seven employees. We also utilize the services of consultants including our Chief Medical Officer and several members of our Scientific Advisory Board. None of our employees are covered by a collective bargaining agreement. We believe our relationship with our employees and consultants is good.

Our Scientific Advisory Board

We rely on prominent scientists and physicians to advise us on the development of our drug candidates. All of our advisors are employed by organizations other than ours and may have commitments to or consulting or advisory agreements with other entities that may limit their availability to us. Our Scientific Advisory Board currently consists of the following members:

Jonathan Brodie, PhD, MD, is the chairman of our Scientific Advisory Board and the Marvin Stern Professor of Psychiatry at New York University School of Medicine. Dr. Brodie completed his bachelor of science degree in chemistry as a Ford Foundation Scholar and his PhD in Physiological Chemistry (Organic Chemistry minor) at the University of Wisconsin-Madison. He was an NIH postdoctoral Fellow in Biochemistry at Scripps Clinic and Research Foundation and a tenured associate professor of Biochemistry at the School of Medicine at SUNY at Buffalo. He then received his MD degree at New York University School of Medicine and joined the faculty after completing his residency in psychiatry at NYU/Bellevue Medical Center. He is a member of the Promotions and Tenure Committee of the School of Medicine and co-chairman of the Executive Advisory Committee of the General Clinical Research Center and the Protocol Review Committee of the Center for Advanced Brain Imaging (CABI) of Nathan Kline Institute. He also served as Interim Chairman of the Department of Psychiatry of the NYU School of Psychiatry at the NYU School of Medicine. For 15 years, he was the NYU Director of the Brookhaven National Laboratory/NYUSoM collaboration investigating the use of positron

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emitters and PET in neuroscience and psychiatry. In addition, Dr. Brodie serves as a psychopharmacology preceptor to psychiatry residents. As a clinician, he treats patients in general issues of adult psychiatry including anxiety and depression.

Robert D. Fechtner, MD, is Professor of Ophthalmology and Director, of the Glaucoma Division, at the Institute of Ophthalmology and Visual Science, Rutgers, the State University of New Jersey. Dr. Fechtner received his bachelor of science degree in biomedical science and his medical degree from the University of Michigan. He completed his residency at Albert Einstein College of Medicine in New York. A fellowship in glaucoma followed at the University of California, San Diego, under a National Research Service Award from the National Institutes of Health. Dr. Fechtner is the Executive Vice President of the World Glaucoma Association and has published more than 100 scientific articles and book chapters.

Eugene Laska, PhD, is a professor in the Department of Psychiatry at New York University and the Director of the Statistical Sciences unit at the Nathan S. Kline Institute for Psychiatric Research. Dr. Laska was for 20 years the Director of the WHO Collaborating Center for Research and Training in Mental Health Program Management and has served as a consultant to many pharmaceutical companies both large and small with regard to biostatistics and clinical trial design.

Richard B. Silverman, Ph.D. is the John Evans Professor of Chemistry at Northwestern University. He is the inventor of Pfizer s $4.5 billion/year Lyrica® (pregabalin), marketed worldwide for the treatment of epilepsy, neuropathic pain, fibromyalgia, pain from spinal cord injury, and (in Europe) for generalized anxiety disorder. He has received numerous awards, most recently the 2014 Excellence in Medicinal Chemistry Prize of the Israel Chemistry Society, 2013 Bristol-Myers Squibb-Edward E. Smissman Award of the American Chemical Society, 2012 Sato Memorial International Award of the Pharmaceutical Society of Japan, 2011 Fellow of the American Chemical Society, 2011 E.B. Hershberg Award for Important Discoveries in Medicinally Active Substances from the American Chemical Society, 2009 Perkin Medal, from the Society of Chemical Industry, and, in 2009, he was inducted into the American Chemical Society Medicinal Chemistry Hall of Fame. Dr. Silverman holds 52 patents, has published over 320 peer-reviewed articles and has written four books over his 37-year career in academia.

We plan to add additional members to our Scientific Advisory Board in the future who will be able to advise on the development and commercialization of Firdapse for LEMS or other neuromuscular diseases.

Available Information

We make available free of charge on or through our Internet website our Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and all amendments to those reports as soon as reasonably practicable after such material is electronically filed with or furnished to the Securities and Exchange Commission (SEC). Our Internet address is www.catalystpharma.com. The content on our website is not, nor should it be deemed to be, incorporated by reference into this Form 10-K.

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Item 1A. Risk Factors

Our business involves a high degree of risk. You should carefully consider the risks and uncertainties described below, and all of the other information contained in this Form 10-K in assessing the risks relating to ownership of our common stock. The risks described below could cause our business, results of operations, financial condition and prospects to materially suffer and the market price of our stock to decline.

Risks Related to 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 $12.2 million for the year ended December 31, 2013, and as of December 31, 2013 we had a deficit accumulated during the development stage of $54.3 million. We may never obtain approval of an NDA for any of our product 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 planned operations through at least the end of 2014. The expectations described above are based on current information available to us. If the cost of our ongoing studies 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 the National Institute of Neurological Disorders and Stroke (NINDS) or other appropriate agencies that operate under the umbrella of the National Institutes of Health and/or other means. However, there is no assurance that any such grants will be made available, and if available, that we will qualify to receive any such grants. We may also seek to raise additional capital to fund additional product development efforts, even if we have sufficient funds for our planned operations.

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.

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

In January of 2012, another pharmaceutical company, Jacobus Pharmaceutical, began its own Phase 2 trial studying their own formulation of amifampridine (3,4-DAP) for the treatment of LEMS. While there can be no assurance, based on currently available information, we believe that our development program for Firdapse is further along in development than this other company s development program. 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 this other pharmaceutical company is able to receive approval of an NDA for its formulation of amifampridine for the treatment of LEMS before we are able to receive approval of Firdapse 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 this other company 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 file 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 file an NDA for CPP-115 or commercialize CPP-115.

Our business is subject to substantial competition.

The biotechnology and pharmaceutical industries are highly competitive. Many of our competitors have substantially greater financial and other resources, larger research and development staffs and more experience developing products, obtaining FDA and other regulatory approvals of products and manufacturing and marketing products than we have. We compete against pharmaceutical companies that are developing or currently marketing therapies that will compete with our product 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 product 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 product candidates, we may also compete with respect to manufacturing efficiency and marketing capabilities.

For example, amifampridine, the active ingredient in Firdapse , has been available from compounding pharmacies and from Jacobus Pharmaceutical under compassionate use INDs for many years, and will likely be available from these sources even if we are able to obtain FDA approval of Firdapse . Amifampridine from these sources is likely 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. Further, drugs that are not approved by FDA for the treatment of LEMS, such as 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 product candidates, or any

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potential future products we may acquire and use in clinical trials or after FDA approval and, therefore, the amount of insurance coverage we currently hold may not be adequate to cover all liabilities we might incur. If we are sued for any injury allegedly caused by our products, our liability could exceed our ability to pay the liability. Whether or not we are ultimately successful in any adverse litigation, such litigation could consume substantial amounts of our financial and managerial resources, all of which could have a material adverse effect on our business, financial condition, results of operations, prospects and stock price.

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

As a public reporting company, we are required to comply with the Sarbanes-Oxley Act of 2002 and the related rules and regulations of the SEC, including periodic reports, disclosures and more complex accounting rules. As directed by Section 404 of Sarbanes-Oxley, the SEC adopted rules requiring public companies to include a report of management on a company s internal control over financial reporting in their Annual Report on Form 10-K. Based on current rules, we are required to annually report under Section 404(a) of Sarbanes-Oxley regarding our management s assessment as to the effectiveness of our internal control over financial reporting. If we are unable to conclude that we have effective internal control over our financial reporting, investors could lose confidence in the reliability of our financial statements, which could result in a decrease in the value of our common stock.

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

We are highly dependent on our officers, on our Board of Directors and on our scientific advisors. The loss of the services of any of these individuals could significantly impede the achievement of our scientific and business objectives. Other than an employment agreement with Patrick J. McEnany, our Chairman, President and Chief Executive Officer with respect to his services, and the consulting agreements we have with our chief medical officer and 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 product candidates might be significantly delayed. We do not carry key-man insurance on any of our personnel.

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

Risks Related to the Development of Our Drug Candidates

Our product development efforts may fail.

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

- Our product candidates may be found to be ineffective or unsafe, or fail to receive necessary regulatory approvals;

Our product 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 product development activities may not result in any safe, effective and commercially viable products, and we may not be able to commercialize our products successfully. For example, for several years, we evaluated CPP-109 (our formulation of vigabatrin) for the treatment of cocaine addiction. However, CPP-109 failed to meet the primary and two key secondary endpoints in a Phase 2(b) trial for cocaine addiction, and we are no longer pursuing the evaluation of CPP-109 for addiction. Further, our lead compound, Firdapse , is for a very rare condition for which there is no FDA-approved, effective treatment. As such, the clinical

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development plan we are pursuing after consulting with FDA including the clinical endpoints, protocol design, and statistical analysis plan, may not allow the FDA to conclude 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 product development efforts.

We will only obtain regulatory approval to commercialize our product 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 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 product 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 product 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;
- 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 product 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 governmental and third-party contract research organizations, medical institutions and clinical investigators (including academic clinical investigators), to conduct studies and trials of our drug candidates. Our reliance on third parties for development activities reduces our control over these activities. These third parties may not complete activities on schedule, or may not conduct our pre-clinical studies and our clinical studies and trials in accordance with regulatory requirements or our study design. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may be adversely affected, and our efforts to obtain regulatory approvals for and commercialize our drug candidates may be delayed.

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

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Although we intend to rely on third parties to manage the data from these 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 product 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 will require substantial resources and compete for available resources with our drug development efforts. To the extent that we enter into marketing and distribution arrangements with third parties, our revenues will depend on the efforts of others. If we fail to enter into such agreements, or if we fail to develop our own marketing and distribution channels, we would experience delays in product sales and incur increased costs.

We have no in-house manufacturing capacity and, to the extent we are successful in completing the development of our product candidates, we will be obliged to rely on contract manufacturers. We cannot assure you that we will successfully manufacture any product we may develop, either independently or under manufacturing arrangements, if any, with third party manufacturers. Moreover, if any manufacturer should cease doing business with us or experience delays, shortages of supply or excessive demands on their capacity, we may not be able to obtain adequate quantities of product in a timely manner, or at all. Manufacturers, and in certain situations their suppliers, are required to comply with current NDA commitments and good manufacturing practices requirement enforced by the FDA, and similar requirements of other countries. The failure by a manufacturer to comply with these requirements could affect its ability to provide us with product.

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 product candidates, it could have a material adverse effect on our ability to commercialize our product candidates.

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

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

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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 product 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 seven employees and conduct much of our operations through outsourcing arrangements. To manage future growth, we will need to hire, train, and manage additional employees. Concurrent with expanding our operational and marketing capabilities, we will also need to increase our product development activities. We may not be able to support, financially or otherwise, future growth, or hire, train, motivate, and manage the required personnel. Our failure to manage growth effectively could limit our ability to achieve our goals.

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

Our commercial success depends on reimbursement from third-party and governmental insurers.

Sales of pharmaceutical products in the United States depend largely on reimbursement of patients costs by private insurers, government health care programs including Medicare and Medicaid, and other organizations. These third-party payors control healthcare costs by limiting both coverage and the level of reimbursement for healthcare products. In particular, the rising costs of pharmaceutical products are a subject of considerable attention and debate. Third-party payors are increasingly altering reimbursement levels and challenging the price and cost-effectiveness of pharmaceutical products. The reimbursement status of newly approved pharmaceutical products in particular is generally uncertain. The levels at which government authorities and private health insurers reimburse physicians or patients for the price they pay for any products we may develop could affect the extent to which we are able to commercialize our products successfully.

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

Our internal computer systems and those of our contract research organization and other key vendors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such system failure, accident or security breach to date, 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 product candidates could be delayed.

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

We are exposed to the risk of employee or consultant fraud or other misconduct. Misconduct by our employees or consultants could include intentional failures to comply with FDA regulations, provide accurate information to the FDA, comply with manufacturing standards, comply with federal and state healthcare fraud and abuse laws and regulations, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing and other abusive practices.

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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 product 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 product 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 product candidate, we must demonstrate to the satisfaction of the applicable regulatory agency that such product candidate is safe and effective for its intended uses. The type and magnitude of the testing required for regulatory approval varies depending on the product 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 product candidate are in compliance with Current Good Manufacturing Processes (cGMP). We will also have to meet similar regulations in any foreign country where we may seek to commercialize our product 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 product 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 product candidates may not be approved for any or all of the indications that we request, which would limit the indications for which we can promote it and adversely impact our ability to generate revenues. We may also be required to conduct costly, post-marketing follow-up studies if FDA requests additional information.

The FDA and other regulatory bodies must approve trade names for products. The FDA typically conducts a thorough review of a proposed trade name, including an evaluation of potential confusion with other trade names. We have recently submitted a request for FDA approval of the trade name Firdapse , 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 product candidates, we may have to conduct, at our own expense, pre-clinical tests in animals in order to support the safety of our product candidates. Pre-clinical testing is expensive, difficult to design and implement, can take several years to complete and is uncertain as to outcome. Our pre-clinical tests may produce negative or inconclusive results, and on the basis of such results, we may decide, or

regulators may require us, to halt ongoing clinical trials or conduct additional pre-clinical testing.

We are in the process of conducting a Phase 3 clinical trial for Firdapse and are currently planning to commence (during the first half of 2014) a Phase 1(b) clinical trial for CPP-115. Even if the results of our clinical trials are promising, Firdapse and CPP-115 may subsequently fail to meet the safety and efficacy standards required to obtain regulatory approvals. Future clinical trials for Firdapse or CPP-115 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.

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Any clinical trials we might develop and implement, may not be completed in a timely manner or at all. Our product 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 for the indications we are studying for our product candidates. Further, unrelated third parties and investigators in the academic community have expressed interest in testing our product candidates. If these third-party tests are unsuccessful, or if they show significant health risk to the test subjects, our development efforts may also be adversely affected.

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

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

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

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

reliance on the continued financial viability of the third parties;

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

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

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

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

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

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

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

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

Even if we receive regulatory approval of any drugs we are developing or may develop, we will be subject to continuing regulatory review, including the review of clinical results which are reported after our drug candidates become commercially available approved drugs. As greater numbers of patients use a drug following its approval, side effects and other problems may be observed after approval that were not seen or anticipated during preapproval clinical studies and trials. In addition, the manufacturer, and the manufacturing facilities we use to make any approved drugs, will also be subject to periodic review and inspection by the FDA. The subsequent discovery of previously unknown problems with the drug, manufacturer or facility may result in restrictions on the drug, manufacturer or facility, including withdrawal of the drug from the market. If we fail to comply with applicable continuing regulatory requirements, we may be subject to fines, suspension or withdrawal of regulatory approval, product recalls and seizures, operating restrictions and criminal prosecutions.

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

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

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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.

Risks Related to Our Intellectual Property

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

All of our patent rights for Firdapse are derived from our license agreement with BioMarin. Pursuant to this license agreement, we have licensed rights under BioMarin s Firdapse patent in the United States, which expire in 2022. 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.

All 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.

A patent to protect CPP-115 in all anticipated non-U.S. markets throughout the world was filed in March 2011 under the Patent Cooperation Treaty (PCT). Prosecution of this patent is ongoing, but it cannot be assured that the claims of this patent will be allowed, or, even if allowed, whether such claims will be allowed in a form that will provide adequate protection for CPP-115 outside the United States.

If we obtain approval to market Firdapse , CPP-115 or CPP-109, our commercial success will depend in large part on our ability to use patents, especially those licensed to us by BioMarin and Northwestern, respectively, to exclude others from competing with us. The patent position of emerging pharmaceutical companies like us can be highly uncertain and involve complex legal and technical issues. Until our licensed patents are interpreted by a court, either because we have sought to enforce them against a competitor or because a competitor has preemptively challenged them, we will not know the breadth of protection that they will afford us. Our patents may not contain claims

sufficiently broad to prevent others from practicing our technologies or marketing competing products. Third parties may intentionally attempt to design around our patents or design around our patents so as to compete with us without infringing our patents. Moreover, the issuance of a patent is not conclusive as to its validity or enforceability, and so our patents may be invalidated or rendered unenforceable if challenged by others.

As a result of the foregoing factors, we cannot be certain how much protection from competition patent rights will provide us.

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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.

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.

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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 could 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 early-stage pharmaceutical companies have historically been particularly volatile. Some of the factors that may cause the market price of our common stock to fluctuate include:

- developments concerning our clinical studies and trials and our pre-clinical studies;
- announcements of product development successes and failures by us or our competitors;
- new products introduced or announced by us or our competitors;
- adverse changes in the abilities of our third party manufacturers to provide drug or product in a timely manner or to meet FDA requirements;
- changes in reimbursement levels;
- changes in financial estimates by securities analysts;
- actual or anticipated variations in operating results;
- expiration or termination of licenses (particularly our licenses from BioMarin and Northwestern), research contracts or other collaboration agreements;
- conditions or trends in the regulatory climate and the biotechnology and pharmaceutical industries;
- intellectual property, product liability or other litigation against us;

changes in the market valuations of similar companies;

changes in pharmaceutical company regulations or reimbursements as a result of healthcare reform or other legislation;

changes in economic conditions; and

sales of shares of our common stock, particularly sales by our officers, directors and significant stockholders, or the perception that such sales may occur.

In addition, equity markets in general, and the market for emerging pharmaceutical and life sciences companies in particular, have experienced substantial price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of companies traded in those markets. Further, changes in economic conditions in the United States, Europe or globally could impact our ability to grow profitably. Adverse economic changes are outside our control and may result in material adverse

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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;
- requirements that special meetings of our stockholders may only be called by the Board of Directors; and
- advance notice procedures our stockholders must comply with in order to nominate candidates for election to our Board of Directors or to place stockholders proposals on the agenda for consideration at meetings of stockholders.

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

The intent of the stockholder rights plan is to protect our stockholders interests by encouraging anyone seeking control of our company to negotiate with our board of directors. However, our stockholder rights plan could make it more difficult for a third party to acquire us without the consent of our board of directors, even if doing so may be beneficial to our stockholders. This plan may discourage, delay or prevent a tender offer or takeover attempt, including offers or attempts that could result in a premium over the market price of our common stock. This plan could reduce the price

that stockholders might be willing to pay for shares of our common stock in the future. Furthermore, the anti-takeover provisions of our stockholder rights plan may entrench management and make it more difficult to replace management even if the stockholders consider it beneficial to do so.

In addition, Section 203 of the Delaware General Corporation Law generally prohibits us from engaging in a business combination with any person who owns 15% or more of our common stock for a period of three years from the date such person acquired such common stock, unless board or stockholder approval is obtained. These provisions could make it difficult for a third party to acquire us, or for members of our Board of Directors to be replaced, even if doing so would be beneficial to our stockholders.

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.

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Future sales of our common stock may cause our stock price to decline.

As of March 14, 2014, we had 54,145,633 shares of our common stock outstanding, of which 5,750,609 shares were held by our officers and directors. We also had outstanding: (i) common stock purchase warrants to purchase an aggregate of 4,835,924 additional shares of our common stock at exercise prices ranging from $1.04 to $2.08 per share, and (ii) stock options to purchase an aggregate of 3,401,906 shares at exercise prices ranging from $0.47 to $6.00 per share (2,968,572 of which are currently exercisable). Sales of restricted shares or shares underlying stock options and common stock purchase warrants, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock.

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

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

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

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

Item 1B. Unresolved Staff Comments

Not applicable.

Item 2. Properties

We currently operate our business in leased office space in Coral Gables, Florida. We pay annual rent on our office space of approximately $96,000.

Item 3. Legal Proceedings

In October 2013 and November 2013, three securities class action lawsuits were filed against us and certain of our executive officers and directors seeking unspecified damages in the U.S. District Court for the Southern District of Florida. The complaints, which were substantially identical, purported to state a claim for violation of federal securities laws on behalf of a class of those who purchased our common stock between October 31, 2012 and October 18, 2013. Two of the cases were voluntarily dismissed by the plaintiffs and the Court granted our motion to dismiss the third case on January 3, 2014. However, the Court granted leave to the plaintiffs to file an amended

complaint within 20 days.

On January 23, 2014, the plaintiffs filed an amended complaint against us and one of our executive officers seeking unspecified damages. The amended complaint purports to state a claim for alleged misrepresentations regarding the development of Firdapse on behalf of a class of those who purchased our common stock between August 27, 2013 and October 18, 2013. We have filed a motion to dismiss the amended complaint, which has not yet been ruled on by the Court. We believe that the amended lawsuit, which is at a very early stage, is without merit, and we intend to vigorously defend this lawsuit. While there can be no assurance, we do not expect this lawsuit to have a material adverse effect on us.

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Item 4. Mine Safety Disclosure

Not applicable.

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PART II

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

Market Information

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

	High	Low
Year Ended December 31, 2012		
First Quarter	$ 1.34	$ 1.05
Second Quarter	$ 1.11	$ 0.53
Third Quarter	$ 1.99	$ 0.53
Fourth Quarter	$ 1.71	$ 0.39
Year Ended December 31, 2013		
First Quarter	$ 0.59	$ 0.43
Second Quarter	$ 1.07	$ 0.45
Third Quarter	$ 3.23	$ 0.87
Fourth Quarter	$ 3.39	$ 1.32
Year Ended December 31, 2014		
First Quarter (through March 14, 2014)	$ 2.33	$ 1.78

The closing sale price for the common stock on March 14, 2014 was $2.17. As of March 14, 2014, there were 46 holders of record of our common stock, which includes custodians who hold our securities for the benefit of others. We estimate that there are approximately 9,300 beneficial holders of our common stock.

Dividend Policy

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

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Performance Graph

The following graph compares the cumulative total shareholder return on our common stock since December 31, 2008 to three indices: the Russell Microcap Index, the NASDAQ Composite Index, and the NASDAQ Biotechnology Index. The graph assumes an initial investment of $100 on December 31, 2008. The comparisons in this graph are required by the SEC and are not intended to forecast or be indicative of possible future performance of our common stock.

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Item 6. Selected Financial Data

The selected statement of operations data for the years ended December 31, 2013, 2012, 2011 and for the cumulative period from inception (January 4, 2002) through December 31, 2013, and the balance sheet data as of December 31, 2013 and 2012, have been derived from our audited financial statements included elsewhere in this Form 10-K. The selected statement of operations data for the years ended December 31, 2010 and 2009 and the selected balance sheet data at December 31, 2011, 2010 and 2009 have been derived from financial statements that are not included in this Form 10-K. Historical results are not necessarily indicative of future results. This selected financial data should be read in conjunction with Management s Discussion and Analysis of Financial Condition and Results of Operations and our financial statements and related notes included elsewhere in this Form 10-K.

	Year Ended December 31,					Cumulative period from inception (January 4, 2002) through December 31, 2013
	2013	2012	2011	2010	2009	
Statement of Operations Data:						
Revenues government grant	$	$	$	$ 488,958	$	$ 488,958
Operating costs and expenses:						
Research and development	8,096,774	2,659,597	3,383,965	2,306,781	5,097,440	36,400,079
General and administrative	2,214,884	2,561,543	2,698,174	2,206,358	2,177,954	18,882,175
Total operating cost and expenses	10,311,658	5,221,140	6,082,139	4,513,139	7,275,394	55,282,254
Loss from operations	(10,311,658)	(5,221,140)	(6,082,139)	(4,024,181)	(7,275,394)	(54,793,296)
Interest income	47,421	14,976	10,985	17,858	33,466	1,540,186
Change in fair value of warrants liability	(1,890,359)	1,129,778	(319,908)			(1,080,489)
Loss before income taxes	(12,154,596)	(4,076,386)	(6,391,062)	(4,006,323)	(7,241,928)	(54,333,599)
Provision for income taxes						
Net loss	$ (12,154,596)	$ (4,076,386)	$ (6,391,062)	$ (4,006,323)	$ (7,241,928)	$ (54,333,599)
Net loss per share basic and diluted	$ (0.27)	$ (0.14)	$ (0.29)	$ (0.22)	$ (0.48)	

Weighted average shares outstanding basic and diluted	45,452,447	30,033,108	21,728,292	18,580,223	15,066,799

	As of December 31,				
	2013	2012	2011	2010	2009
Balance Sheet Data:					
Cash and cash equivalents, certificates of deposit and short-term investments	$ 23,710,596	$ 15,417,208	$ 6,029,067	$ 5,475,158	$ 7,779,277
Working capital	23,180,429	15,080,013	5,394,382	5,476,443	7,593,272
Total assets	25,369,554	16,789,245	6,249,257	5,831,488	7,966,382
Warrants liability	1,819,562	498,587	1,645,240		
Total liabilities	3,978,302	2,167,130	2,488,559	313,709	348,522
Stockholders equity	21,391,252	14,622,115	3,760,698	5,517,779	7,617,860

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Item 7. Management s Discussion and Analysis of Financial Condition and Results of Operations

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with Selected Financial Data and our financial statements and related notes appearing elsewhere in this Form 10-K. In addition to historical information, this discussion and analysis contains forward-looking statements that involve risks, uncertainties, and assumptions. Our actual results may differ materially from those anticipated in these forward-looking statements as a result of certain factors, including but not limited to those set forth under the caption Risk Factors in Item 1A of this Form 10-K.

Introduction

Management s Discussion and Analysis of Financial Condition and Results of Operations (MD&A) is intended to provide an understanding of our financial condition, changes in financial condition and results of operations. The discussion and analysis is organized as follows:

Overview. This section provides a general description of our business and information about our business that we believe is important in understanding our financial condition and results of operations.

Basis of Presentation. This section provides information about key accounting estimates and policies that we followed in preparing our financial statements for the 2013 fiscal year.

Critical Accounting Policies and Estimates. This section discusses those accounting policies that are both considered important to our financial condition and results of operations, and require significant judgment and estimates on the part of management in their application. All of our significant accounting policies, including the critical accounting policies, are also summarized in the notes to our accompanying financial statements.

Results of Operations. This section provides an analysis of our results of operations for all three fiscal years presented in the accompanying statements of operations.

Liquidity and Capital Resources. This section provides an analysis of our cash flows, capital resources, off-balance sheet arrangements and our outstanding commitments, if any.

Caution Concerning Forward-Looking Statements. This section discusses how certain forward-looking statements made throughout this MD&A and in other sections of this report are based on management s present expectations about future events and are inherently susceptible to uncertainty and changes in circumstance.

Overview

We are a development-stage specialty pharmaceutical company focused on the development and commercialization of novel prescription drugs targeting rare (orphan) neuromuscular and neurological diseases. We currently have three pharmaceutical products 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). As part of our agreements with BioMarin, we have taken over the sponsorship of an ongoing Phase 3 clinical trial evaluating Firdapse for the treatment of Lambert-Eaton Myasthenic Syndrome, or LEMS, a rare and sometimes fatal autoimmune disease characterized by muscle weakness. We also hope to evaluate Firdapse for the treatment of other neuromuscular orphan indications such as certain forms of Congenital Myasthenic Syndrome and Myasthenia Gravis. In August 2013, we were granted breakthrough therapy designation by the U.S. Food & Drug Administration (FDA) for Firdapse for the treatment of LEMS.

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

The Phase 3 trial is designed as a randomized double-blind, placebo-controlled discontinuation study followed by an open-label extension period in approximately 36-patients across 24 sites in the United States, Canada, South America and Europe. Based on currently available information, we expect that we will complete enrollment in the trial before the end of the first quarter of 2014 and that we will report top-line results from the double-blind portion of this Phase 3 trial during the third quarter of 2014 (and, if the trial results are successful, we expect to submit to the FDA, on a rolling basis, all of the modules required to complete an NDA) by the middle of 2015.

CPP-115

We are in the early stages of developing CPP-115, a GABA aminotransferase inhibitor that, based on our pre-clinical studies to date, we believe is a more potent form of vigabatrin, but may have fewer side effects (e.g., visual field defects, or VFDs) than those associated with vigabatrin. We are hoping to develop CPP-115 for the treatment of epilepsy (initially infantile spasms) and for the treatment of other selected neurological indications. 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 s syndrome (a form of infantile spasms). We expect to begin a multi-dose safety and tolerance study of CPP-115 during the first half of 2014.

CPP-109

For several years, we evaluated CPP-109 (our formulation of vigabatrin, another GABA aminotransferase inhibitor) for the treatment of cocaine addiction. However, in November 2012, we reported that CPP-109 failed to meet the primary and two key secondary endpoints in a Phase 2(b) trial for cocaine addiction. As a result, we are no longer focusing our efforts on evaluating CPP-109 for addiction. Further, on November 8, 2013, effective October 1, 2013, we terminated our license agreement with Brookhaven National Laboratories under which we had previously licensed nine patents relating to the use of vigabatrin as a treatment of a wide variety of substance addictions.

An academic investigator proof-of-concept study evaluating the use of CPP-109 for the treatment of Tourette Syndrome is currently ongoing and, if the results of that study show evidence of reduced number of tics, we will likely seek to develop CPP-109 or CPP-115 (which has the same mechanism of action as CPP-109) for this indication. We do not control this proof-of-concept study and therefore have no control over its timing. However, based on currently available information, we expect to have top-line results for this academic investigator proof-of-concept study during 2014.

Capital Resources

Based on our current financial condition and forecasts of available cash, we believe that we have sufficient funding to support our planned operations through at least the end of 2014. However, we will require additional funding to support our planned operations beyond the end of 2014. There can be no assurance that we will obtain additional funding or that we will ever be in a position to commercialize any of our product candidates. See Liquidity and Capital Resources below for further information on our liquidity and cash flow.

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Basis of presentation

Revenues

We are a development stage company and have no revenues from product sales to date. We will not have revenues from product sales until such time as we receive approval of our product candidates, successfully commercialize our products or enter into a licensing agreement which may include up-front licensing fees, of which there can be no assurance.

Research and development expenses

Our research and development expenses consist of costs incurred for company-sponsored research and development activities. The major components of research and development costs include pre-clinical study costs, clinical manufacturing costs, clinical study and trial expenses, insurance coverage for clinical trials, consulting, scientific advisors and other third-party costs, salaries and employee benefits, stock-based compensation expense, supplies and materials and allocations of various overhead costs related to our product development efforts. To date, all of our research and development resources have been devoted to the development of CPP-109, CPP-115 and Firdapse , and we expect this to continue for the foreseeable future. Costs incurred in connection with research and development activities are expensed as incurred.

Our cost accruals for clinical studies and trials are based on estimates of the services received and efforts expended pursuant to contracts with numerous clinical study and trial sites and clinical research organizations. In the normal course of business we contract with third parties to perform various clinical study and trial activities in the on-going development of potential products. The financial terms of these agreements are subject to negotiation and vary from contract to contract and may result in uneven payment flows. Payments under the contracts depend on factors such as the achievement of certain events or milestones, the successful enrollment of patients, the allocation of responsibilities among the parties to the agreement, and the completion of portions of the clinical study or trial or similar conditions. The objective of our accrual policy is to match the recording of expenses in our financial statements to the actual services received and efforts expended. As such, expense accruals related to pre-clinical and clinical studies or trials are recognized based on our estimate of the degree of completion of the event or events specified in the specific study or trial contract. We monitor service provider activities to the extent possible; however, if we underestimate activity levels associated with various studies or trials at a given point in time, we could be required to record significant additional research and development expenses in future periods. Pre-clinical and clinical study and trial activities require significant up front expenditures. We anticipate paying significant portions of a study or trial s cost before such begins, and incurring additional expenditures as the study or trial progresses and reaches certain milestones.

Selling and marketing expenses

We do not currently have any selling or marketing expenses. We expect we will begin to incur costs tied to our future sales and marketing efforts during the 2014 fiscal year as we move closer to the potential commercialization of Firdapse . Our plan is to put in place over the next year the personnel that will help us develop both a sales force and a patient advocacy and assistance program so that we are in a position to commence our selling efforts immediately if we are successful in obtaining an approval of any NDA that we may file for Firdapse , of which there can be no assurance.

General and administrative expenses

Our general and administrative expenses consist primarily of salaries and personnel expenses for accounting, corporate and administrative functions. Other costs include administrative facility costs, regulatory fees, and professional fees for legal, information technology, accounting and consulting services.

Stock-based compensation

We recognize expense for the fair value of all stock-based awards to employees, directors, scientific advisors and consultants in accordance with U.S. generally accepted accounting principles. For stock options we use the Black-Scholes option valuation model in calculating the fair value of the awards.

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Warrants Liability

We issued warrants to purchase shares of our common stock as part of the equity financing completed in October 2011. In accordance with U.S. generally accepted accounting principles, we have recorded the fair value of the warrants as a liability in the accompanying balance sheets at December 31, 2013 and 2012 using a Black-Scholes option-pricing model. We will remeasure the fair value of the warrants liability at each reporting date until the warrants are exercised or have expired. Changes in the fair value of the warrants liability are reported in the statements of operations as income or expense. The fair value of the warrants liability is subject to significant fluctuation based on changes in the inputs to the Black-Scholes option-pricing model, including our common stock price, expected volatility, expected life, the risk-free interest rate and dividend yield. The market price for our common stock has been and may continue to be volatile. Consequently, future fluctuations in the price of our common stock may cause significant increases or decreases in the fair value of the warrants.

Income taxes

We have incurred operating losses since inception. As of December 31, 2013 and 2012, we had net operating loss carryforwards of approximately 30,675,000 and $22,997,000, respectively. Our net deferred tax asset has a 100% valuation allowance as of December 31, 2013 and 2012, as we believe it is more likely than not that the deferred tax asset will not be realized. The net operating loss carry-forwards will expire at various dates beginning 2024 through 2033. If an ownership change, as defined under Internal Revenue Code 382, occurs, the use of these carry-forwards may be subject to limitations.

As required by ASC 740, *Income Taxes*, we recognize the financial statement benefit of a tax position only after determining that the relevant tax authority would more likely than not sustain the position following an audit. For tax positions meeting the more-likely-than-not threshold, the amount recognized in the financial statements is the largest benefit that has a greater than 50 percent likelihood of being realized upon ultimate settlement with the relevant tax authority.

Recent Accounting Pronouncements

There are no recent accounting pronouncements which we anticipate will have a significant impact on our financial statements.

Non-GAAP Financial Measures

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

The non-GAAP financial measures that we often present exclude from the calculation of net loss the expense (or the income) associated with the change in fair value of the liability-classified warrants.

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

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Critical Accounting Policies and Estimates

Our discussion and analysis of our financial condition and results of operations are based on our financial statements, which have been prepared in accordance with accounting principles generally accepted in the U.S. The preparation of these financial statements requires us to make judgments, estimates, and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenue and expenses during the reporting periods. We continually evaluate our judgments, estimates and assumptions. We base our estimates on the terms of underlying agreements, our expected course of development, historical experience and other factors we believe are reasonable based on the circumstances, the results of which form our management s basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates.

The list below is not intended to be a comprehensive list of all of our accounting policies. In many cases, the accounting treatment of a particular transaction is specifically dictated by generally accepted accounting principles, or GAAP. There are also areas in which our management s judgment in selecting any available alternative would not produce a materially different result. Our financial statements and the notes thereto included elsewhere in this report contain accounting policies and other disclosures as required by GAAP.

Pre-clinical study and clinical trial expenses

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

Warrants Liability

We have issued warrants to purchase our common stock that may require us to purchase unexercised warrants for a cash amount equal to their fair value following the announcement of specified events defined as Fundamental Transactions (Fundamental Transactions) involving us, which is deemed to occur if we are acquired in an all cash transaction or by a company that is not listed on a national securities exchange, or when the common stock is no longer listed on a national securities exchange. The cash settlement provisions require use of the Black-Scholes model in calculating the cash payment value in the event of a Fundamental Transaction. As a consequence of these provisions, these warrants are classified as a liability on our balance sheets. The cash settlement value at the time of any future Fundamental Transaction will depend upon the value of the following inputs at that time: the price per share of our common stock, the volatility of our common stock, the expected term of the warrants, the risk-free interest rate based on U.S. Treasury security yields, and our dividend yield. The fair value of these warrants is determined using a Black-Scholes model. The valuation of warrants is subjective and is affected by changes in inputs to the valuation model including the price per share of our common stock, the historical volatility of our common stock price, risk-free rates based on U.S. Treasury security yields, the expected term of the warrants and our dividend yield. Changes in these assumptions can materially affect the fair value estimate. We could ultimately incur amounts to settle the warrants at a cash settlement value that is significantly different than the carrying value of the liability on

our financial statements. We will continue to classify the fair value of these warrants as a liability until the warrants are exercised, expire, or are amended in a way that would no longer require these warrants to be classified as a liability. Changes in the fair value of the common stock warrants liability are recognized as a component of other income (expense) in the statement of operations.

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Stock-based compensation

We recognize stock-based compensation for the fair value of all share-based payments, including grants of stock options and restricted stock units. For stock options, we use the Black-Scholes option valuation model to determine the fair value of stock options on the date of grant. This model derives the fair value of stock options based on certain assumptions related to expected stock price volatility, expected option life, risk-free interest rate and dividend yield. Expected volatility is based on reviews of historical volatility of our common stock. The estimated expected option life is based upon the simplified method. Under this method, the expected option life is presumed to be the mid-point between the vesting date and the end of the contractual term. We will continue to use the simplified method until we have sufficient historical exercise data to estimate the expected life of the options. The risk-free interest rate assumption is based upon the U.S. Treasury yield curve appropriate for the estimated expected life of our stock options awards. For the years ended December 31, 2013, 2012 and 2011, the assumptions used were an estimated annual volatility of 137%, 120%, and 130%, average expected holding periods of three years, three to five years and three to five years, and risk-free interest rates of 0.45% to 0.53%, 0.28% to 0.66% and 0.29% to 1.55%, respectively.

Results of Operations

Years Ended December 31, 2013 and 2012

Revenues

We had no revenues for the year ended December 31, 2013 or 2012.

Research and Development Expenses

Year	Amount	Change from Prior Year	Percentage of Total Operating Costs and Expenses
2013	$ 8,096,774	204.4%	78.5%
2012	$ 2,659,597	(21.4%)	50.9%

Our expenses, excluding stock based compensation, for research and development for the year ended December 31, 2013 increased substantially compared to amounts expended in the same period in 2012. During 2013, we continued our Phase 3 trial of Firdapse and performed pre-clinical testing on Firdapse and on CPP-115. During the first months of 2013, BioMarin completed the transfer of the management and oversight of the currently ongoing Phase 3 trial for Firdapse for the treatment of LEMS to us. In connection with such transfer, we retained a CRO and hired additional personnel to provide day-to-day oversight of the Phase 3 trial, including identifying and contracting with an additional 15 clinical sites throughout the United States, Europe and South America. Such efforts increased the number of total clinical sites for our Phase 3 trial from 7, upon transfer of the Phase 3 trial to us, to 22 at the end of 2013. Expenses in the comparable period in 2012 included expenses related to our Phase 1(a) clinical trial safety study for CPP-115 and our NIDA/VA Phase 2(b) clinical trial evaluating CPP-109 for use in the treatment of cocaine addiction, which was completed during 2012. In addition, since we licensed Firdapse in October 2012, the comparable period includes only approximately two months of expenses for the development of Firdapse. We expect that research and development expenses will increase substantially in 2014 as we continue the research and development activities described above and in Part I of this report.

In our research and development activities for 2013 and 2012, we recorded stock-based compensation relating to the value of stock options granted to certain employees. The amount of stock-based compensation recorded in 2013 and 2012 relating to our research and development activities was $84,728 and $100,221, respectively. The weighted-average grant-date fair value of the stock options granted in 2013 and 2012 was $0.48 and $0.32, respectively.

Selling and Marketing Expenses

We had no selling and marketing expenses during 2013 and 2012. We expect we will begin to incur costs tied to our future sales and marketing efforts during the 2014 fiscal year as we move closer to the potential commercialization of Firdapse . Our plan is to put in place over the next year the personnel that will help us develop both a sales force and a patient advocacy and assistance program so that we are in a position to commence our selling efforts immediately if we are successful in obtaining an approval of any NDA that we may file for Firdapse , of which there can be no assurance.

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General and Administrative Expenses

Year	Amount	Change from Prior Year	Percentage of Total Operating Costs and Expenses
2013	$ 2,214,884	(13.5%)	21.5%
2012	$ 2,561,543	(5.1%)	49.1%

General and administrative expenses include, among other expenses, office expenses, legal, accounting and consulting fees and travel expenses for our administrative employees, consultants and members of our Board. Included in general and administrative expenses in the years 2013 and 2012, was stock-based compensation of $91,127 and $239,818, respectively. The decrease in general and administrative expenses for the year ended December 31, 2013 when compared to the same period in 2012 is primarily due to decreases in director compensation, travel expenses and stock-based compensation expense partially offset by increases in professional fees. We expect general and administrative costs, other than costs associated with the sales and marketing efforts described above, to remain relatively stable in future periods as we continue the monitoring and oversight of our research and development activities. However, general and administrative costs in total will increase in 2014 and future periods based on our anticipated efforts to prepare for the potential future commercialization of Firdapse .

Stock-Based Compensation

We issued stock options to several of our employees, directors, and consultants in 2013 and 2012. Total stock-based compensation expense for the years ended December 31, 2013 and 2012 was $175,855 and $340,039, respectively.

Change in fair value of warrants liability

In connection with our October 2011 equity offering, we issued warrants to purchase an aggregate of 1,523,370 shares of common stock. The fair value of the warrants is recorded in the liability section of the balance sheet and was estimated at $1.8 million and $0.5 million at December 31, 2013 and 2012, respectively. The fair value of the warrants liability is determined at the end of each reporting period, with the resulting gains or losses recorded as the change in fair value of warrant liability in the statements of operations. For the years ended December 31, 2013 and 2012, we recognized a loss of $1,890,359 and a gain of $1,129,778, respectively, due to the change in the fair value of the warrants liability. The loss during 2013 was principally a result of the increase in our stock price between December 31, 2012 and December 31, 2013, and the gain during 2012 was principally a result of the decrease of our stock price between December 31, 2011 and December 31, 2012. We believe that future changes in the fair value of the warrants liability will be due primarily to future fluctuations in the value of our common stock.

Interest Income

We reported interest income in all periods relating to our investment of funds received from offerings of our securities. The increased in interest income for the year ended December 31, 2013 as compared to the year ended December 31, 2012 was due to higher average investment balances from the proceeds of our offerings, partially offset by lower interest rates. These proceeds were used to fund our product-development activities and our operations. Substantially all such funds were invested in short-term interest bearing obligations and short-term bond funds.

Income taxes

We have incurred net operating losses since inception. Consequently, we have applied a 100% valuation allowance against our deferred tax asset as we believe that it is more likely than not that the deferred tax asset will not be realized.

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Net Loss

Our net loss was $12,154,596 in the year ended December 31, 2013 ($0.27 per basic and diluted share) as compared to $4,076,386 in the year ended December 31, 2012 ($0.14 per basic and diluted share).

Non-GAAP Net Loss

Our non-GAAP net loss, which excludes for 2013 a $1,890,359 loss associated with the change in the fair value of liability-classified warrants and excludes for 2012 a $1,129,778 gain associated with the change in the fair value of liability-classified warrants, was $10,264,237 for 2013, compared to a non-GAAP net loss of $5,206,164 for 2012.

Years Ended December 31, 2012 and 2011

Revenues

We had no revenues for the year ended December 31, 2012 or 2011.

Research and Development Expenses

Year	Amount	Change from Prior Year	Percentage of Total Operating Costs and Expenses
2012	$ 2,659,597	(21.4%)	50.9%
2011	$ 3,383,965	46.7%	55.6%

Our expenses, excluding stock-based compensation, for research and development for the year ended December 31, 2012 decreased compared to amounts expended in the same period in 2011. During 2012, we concluded our Phase 2(b) trial evaluating CPP-109 for the treatment of cocaine addiction that was initiated in the fourth quarter of 2010, performed pre-clinical testing for CPP-115, concluded our Phase 1(a) trial for CPP-115 and began expending resources on the Phase 3 clinical trial evaluating Firdapse for the treatment of LEMS.

In our research and development activities for 2012 and 2011, we recorded stock-based compensation relating to the value of stock options granted to certain employees and non-employees. The amount of stock-based compensation recorded in 2012 and 2011 relating to our research and development activities was $100,221 and $111,283, respectively. The weighted-average grant-date fair value of the stock options granted in 2012 and 2011 was $0.32 and $0.79, respectively.

Selling and Marketing Expenses

We had no selling and marketing expenses during 2012 and 2011.

General and Administrative Expenses

Year	Amount	Change from Prior Year	Percentage of Total Operating Costs and

			Expenses
2012	$ 2,561,543	(5.1%)	49.1%
2011	$ 2,698,174	22.3%	44.4%

General and administrative expenses include, among other expenses, office expenses, legal, accounting and consulting fees and travel expenses for our administrative employees, consultants and members of our Board. Included in general and administrative expenses in the years 2012 and 2011 was stock-based compensation of $239,818 and $305,452, respectively. The decrease in general and administrative expenses for the year ended December 31, 2012 when compared to the same period in 2011 is primarily due to decreases in payroll expense, as we accrued severance related to a separation during 2011, director compensation, travel expenses and stock-based compensation expense partially offset by increases in professional fees.

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Stock-Based Compensation

We issued stock options to several of our employees, directors, and consultants in 2012 and 2011. Total stock-based compensation expense for the years ended December 31, 2012 and 2011 was $340,039 and $416,735, respectively.

Change in fair value of warrants liability

In connection with the October 2011 equity offering, we issued warrants to purchase an aggregate of 1,523,370 shares of common stock. The fair value of the warrants is recorded in the liability section of the balance sheet and was estimated at $0.5 million and $1.6 million at December 31, 2012 and 2011, respectively. The fair value of the warrants liability is determined at the end of each reporting period with the resulting gains or losses recorded as the change in fair value of warrant liability in the statements of operations. For the years ended December 31, 2012 and 2011, we recognized a gain of $1,129,778 and a loss of $319,908, respectively, due to the change in the fair value of the warrants liability. The gain during 2012 was principally a result of the decrease in our stock price between December 31, 2011 and December 31, 2012, and the loss during 2011 was principally a result of the increase of our stock price between the closing date of the equity offering and December 31, 2011. We believe that future changes in the fair value of the warrants liability will be due primarily to future fluctuations in the value of our common stock.

Interest Income

We reported interest income in all periods relating to our investment of funds received from offerings of our securities. The increased in interest income for the year ended December 31, 2012 as compared to the year ended December 31, 2011 was due to higher average investment balances from the proceeds of our offerings, partially offset by lower interest rates. These proceeds were used to fund our product-development activities and our operations. Substantially all such funds were invested in short-term interest bearing obligations and short-term bond funds.

Income taxes

We have incurred net operating losses since inception. Consequently, we have applied a 100% valuation allowance against our deferred tax asset as we believe that it is more likely than not that the deferred tax asset will not be realized.

Net Loss

Our net loss was $4,076,386 in the year ended December 31, 2012 ($0.14 per basic and diluted share) as compared to $6,391,062 in the year ended December 31, 2011 ($0.29 per basic and diluted share).

Non-GAAP Net Loss

Our non-GAAP net loss, which excludes for 2012 a $1,129,778 gain associated with the change in the fair value of liability-classified warrants and excludes for 2011 a $319,908 loss associated with the change in the fair value of liability-classified warrants, was $5,206,164 for 2012, compared to a non-GAAP net loss of $6,071,154 for 2011.

Liquidity and Capital Resources

Our historical capital resource requirements have been the funding of working capital and pre-clinical and clinical testing of our product candidates. We have historically funded all of our requirements from equity issuances, government grants, and an investment by a strategic purchaser.

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Since our inception, we have financed our operations primarily with the net proceeds of three private placements, an initial public offering (IPO), a secondary public offering and seven registered direct offerings under our shelf registration statements. At December 31, 2013, we had cash and cash equivalents, certificates of deposit and short-term investments aggregating $23,710,596 and working capital of $23,180,429, as compared to cash and cash equivalents, certificates of deposit and short-term investments aggregating $15,417,208 and working capital of $15,080,013 at December 31, 2012. At December 31, 2013 substantially all of our cash and cash equivalents were deposited with one financial institution and our short-term investments were invested in certificates of deposit and a high-quality short-term bond fund. Throughout 2013, we had cash balances at certain financial institutions in excess of federally insured limits.

We have to date incurred operating losses, and we expect these losses to increase substantially in the future as we expand our drug development programs and prepare for the commercialization of our drug candidates. We anticipate using current cash on hand to finance these activities. It will likely take several years to obtain the necessary regulatory approvals to commercialize one or more of our product candidates in the United States.

We currently believe that we have the cash resources to support our planned operations through at least the end of 2014. These expectations are based on current information available to us. If our costs are greater than we expect, our assumptions may not prove to be accurate.

At the present time, we will require additional funding for future studies or trials and to pay future milestone payments that we may be obligated to make. We will also require additional working capital to support our planned operations beyond the end of 2014. There can be no assurance as to the amount of any such funding that will be required for these purposes or whether any such funding will be available to us when it is required.

In that regard, our future funding requirements will depend on many factors, including:

- the scope, rate of progress and cost of our clinical trials and other product development activities;
- future clinical trial results;
- the terms and timing of any collaborative, licensing and other arrangements that we may establish;
- the cost and timing of regulatory approvals;
- the cost and delays in product development as a result of any changes in regulatory oversight applicable to our products;
- the cost and timing of establishing sales, marketing and distribution capabilities;
- the effect of competition and market developments;

the cost of filing and potentially prosecuting, defending and enforcing any patent claims and other intellectual property rights; and

the extent to which we acquire or invest in other products.

We hope to raise additional funds to support our product development activities and working capital requirements through public or private equity offerings, corporate collaborations or other means. We also intend to seek governmental grants for a portion of the required funding for our clinical trials and pre-clinical trials. We may also seek to raise 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 such required additional funding will be available to us at all or available on terms acceptable to us. Further, to the extent that we raise additional funds through collaborative arrangements, it may be necessary to relinquish some rights to our technologies or grant sublicenses on terms that are not favorable to us. If we are not able to secure additional funding when needed, we may have to delay, reduce the scope of or eliminate one or more research and development programs, which could have an adverse effect on our business.

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On January 31, 2014, we filed a shelf registration statement with the SEC to sell up to $100 million of common stock. This shelf registration statement has not yet been declared effective by the SEC. If this registration statement is declared effective, we will be able to sell up to $100 million of our common stock. However, if our public float (the market value of our common stock held by non-affiliates) falls below $75 million (as of March 14, 2014, it was $105.0 million, based on 48,395,024 shares of common stock held by non-affiliates at a price of $2.17 per share, which was the last reported price of our common stock on The NASDAQ Stock Market on March 14, 2014), 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 in any 12-month period. Further, the number of shares we can sell at any one time may be limited to 20% of our outstanding common stock under applicable NASDAQ marketplace rules.

On December 3, 2010 we filed a shelf registration statement with the SEC to sell up to $30 million of common stock. This shelf registration was declared effective by the SEC on December 15, 2010. On September 5, 2013, we filed a registration statement on Form S-3MEF to register an additional $2.6 million of securities under the December 3, 2010 registration statement. We completed four registered direct public offerings to institutional investors under this shelf registration statement:

On March 8, 2011, we raised net proceeds of approximately $2.2 million from the sale of 2,259,943 shares of our common stock;

On October 28, 2011, we raised net proceeds of approximately $3.2 million from the sale of 3,046,740 shares of our common stock and five-year warrants to purchase 1,523,370 shares of our common stock at an exercise price of $1.30 per share;

On August 28, 2012, we raised net proceeds of approximately $5.5 million from the sale of 4,000,000 shares of our common stock and five-year warrants to purchase 1,200,000 shares of our common stock at an exercise price of $2.08 per share; and

On September 5, 2013, we raised net proceeds of approximately $14.1 million from the sale of 8,800,000 shares of our common stock.

Contractual obligations and arrangements

As of December 31, 2013, we had the following contractual obligations. Further, we may owe in the future certain milestone or royalty payment obligations (as described below). Since we are not currently able to determine when or if these milestones will be achieved, or when or if the events triggering payment of the obligations will occur, they are not included in the following table.

	Payments Due by Period				
	Total	Less than 1 year	1-3 years	4-5 years	After 5 years
Operating lease obligations	$ 279,616	$ 68,534	$ 143,254	$ 67,828	$
License obligations	150,000		150,000		

Total	$ 429,616	$ 68,534	$ 293,254	$ 67,828	$

We have entered into the following contractual arrangements:

Payments to BioMarin and others under our license agreement. We have agreed: (i) to pay BioMarin certain royalty payments based on our net sales in North America; (ii) to pay to a third-party licensor of the rights sublicensed to us certain royalty payments based on our net sales in North America, and (iii) to pay certain milestone payments that BioMarin is obligated to make (approximately $2.6 million of which will be due upon acceptance by the FDA of a filing of an NDA for Firdapse for the treatment of LEMS, and approximately $7.2 million of which will be due on the unconditional approval by the FDA of an NDA for Firdapse for the treatment of LEMS). We have also agreed to share in the cost of certain post-marketing studies that are being conducted by BioMarin.

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Payments for Firdapse development. Based on current available information, we estimate that the total product development costs for Firdapse , excluding third-party milestone payments, will be approximately $25 million. At December 31, 2013, we had paid approximately $6.3 million of this amount and had prepaid research fees of approximately $1.3 million, accounts payable of approximately $692,000 and accrued liabilities of approximately $1,087,000 in the accompanying balance sheet in connection with related agreements. Under our license agreement with BioMarin, we are obligated to spend at least $5 million in connection with the Phase 3 clinical trial of Firdapse during the two years following the date of the license agreement (October 26, 2012). As of December 31, 2013, we had disbursed approximately $4.1 million in connection with the Phase 3 clinical trial, and expect to spend the remaining $0.9 million during the first half of 2014.

Payments to Northwestern under our license agreement. Under our license agreement with Northwestern, we have paid to date $246,590, have accrued $65,000 and owe certain milestone payments in future years if we do not cancel the license agreement. The next milestone payment of $150,000 is due on the earlier of August 27, 2015 or the successful completion of the first Phase 2 clinical trial of CPP-115.

Payments for drug development, pre-clinical and clinical studies and trials for the development of CPP-115. We estimate that we will pay various consultants, drug manufacturers and other vendors approximately $1.5 million in connection with our currently ongoing drug development work, including pre-clinical and clinical studies and trials, consulting and data analysis. At December 31, 2013, we had paid approximately $1.4 million of this amount in connection with these agreements.

Employment agreement. We have entered into an employment agreement with our Chief Executive Officer that requires us to make base salary payments of approximately $425,000 per annum.

Leases for office space. We have entered into lease agreements for our office space, which we recently amended to lease additional space. The lease, as amended, requires annual lease payments of approximately $96,000 per annum.

Previous Dispute with Brookhaven

We previously had a license agreement with Brookhaven under which we licensed several patents relating to the use of vigabatrin for the treatment of addiction and obsessive compulsive disorders. Under the license agreement, we were obligated, among other obligations, to reimburse Brookhaven for certain patent related expenses, beginning on the filing of an NDA for CPP-109 (which did not occur). In that regard, Brookhaven had previously advised us that they believed we owed them approximately $1.3 million in patent related expenses as of December 31, 2012. We, on the other hand, believed that if we became obligated to reimburse patent related expenses under the license agreement, that we would only be liable to Brookhaven for approximately $166,000.

On November 8, 2013, effective October 1, 2013, we entered into a termination agreement with Brookhaven under which our license agreement with Brookhaven was cancelled and we exchanged mutual general releases with Brookhaven. As part of the general releases contained in the termination agreement, Brookhaven expressly released us from any future obligation under the license agreement to reimburse them for any patent related expenses.

Off-Balance Sheet Arrangements

We currently have no debt. Capital lease obligations as of December 31, 2013 and 2012 were not material. We have operating leases for our office facilities. We do not have any off-balance sheet arrangements as such term is defined in rules promulgated by the SEC.

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Cash Flows

Net cash used in operating activities was $9,875,674 and $5,140,366, respectively, for the years ended December 31, 2013 and 2012. During the year ended December 31, 2013, net cash used in operating activities was primarily attributable to our net loss of $12,154,596, an increase of $299,972 in prepaid expenses and deposits, and a decrease of $514,874 in accounts payable, offset by an increase of $1,005,071 in accrued expenses and other liabilities, and a loss of $1,890,359 of non-cash expense for the change in fair value of warrants liability. The loss included an additional $198,338 of non-cash expenses. Such additional non-cash expenses include depreciation and stock-based compensation expense.

Net cash used in investing activities was $7,496,801 and $14,059,651, respectively, for 2013 and 2012. For 2013, such funds were used primarily for purchases of short-term investments and certificates of deposit along with $9,432 of capital expenditures relating to computer software and equipment.

Net cash provided by financing activities was $18,178,494 and $14,580,889, respectively, for 2013 and 2012. During 2013 and 2012, net cash from financing activities consisted of the net proceeds from the sale of shares of common stock and warrants to purchase shares of common stock in underwritten and registered direct public offerings under our registration statements. Such funds have been used to fund our research and development costs and our general and administrative costs.

Caution Concerning Forward-Looking Statements

Some of the statements in this Form 10-K are forward-looking statements , as that term is defined in the Private Securities Litigation Reform Act of 1995. These include statements regarding our expectations, beliefs, plans or objectives for future operations and anticipated results of operations. For this purpose, any statements contained herein that are not statements of historical fact may be deemed to be forward-looking statements. Without limiting the foregoing, believes , anticipates , proposes , plans , expects , intends , may , and other similar expressions are i identify forward-looking statements. Such statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by such forward-looking statements. The forward-looking statements made in this Form 10-K are based on current expectations that involve numerous risks and uncertainties.

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

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

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

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 product 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 expense of filing, and potentially prosecuting, defending and enforcing any patent claims and other intellectual property rights;

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the risk that another pharmaceutical company will receive an approval for its formulation of amifampridine for the treatment of LEMS before us;

whether others develop and commercialize products competitive to our products;

whether others obtain exclusive patent or marketing rights that make it difficult or impossible for us to commercialize our product candidates, even if we obtain regulatory approvals for our product candidates;

changes in the laws and regulations affecting our business;

the impact of the class action lawsuit filed against us;

our ability to attract and retain skilled employees;

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

the impact of employee or consultant misconduct; and

changes in general economic conditions and interest rates.

Our current plans and objectives are based on assumptions relating to the development of our current product candidates. Although we believe that our assumptions are reasonable, any of our assumptions could prove inaccurate. In light of the significant uncertainties inherent in the forward-looking statements made herein, which reflect our views only as of the date of this prospectus, you should not place undue reliance upon such statements. We undertake no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk

Market risk represents the risk of changes in the value of market risk-sensitive instruments caused by fluctuations in interest rates, foreign exchange rates and commodity prices. Changes in these factors could cause fluctuations in our results of operations and cash flows.

Our exposure to interest rate risk is currently confined to our cash, certificates of deposit and short-term investments that are from time to time invested in highly liquid money market funds, short-term certificates of deposit and short-term, high-quality bond funds. The primary objective of our investment activities is to preserve our capital to fund operations. We also seek to maximize income from our investments without assuming significant risk. We do not use derivative financial instruments in our investment portfolio. Our cash and investments policy emphasizes liquidity and preservation of principal over other portfolio considerations.

Item 8. Financial Statements and Supplementary Data

See the list of financial statements filed with this report under Item 15 below.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure

Not applicable.

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Item 9A. Controls and Procedures

Disclosure Controls and Procedures

We have carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures. The term disclosure controls and procedures , as defined in Rules 13a-15(e) and 15(d)-15(e) under the Securities Exchange Act of 1934 (the Exchange Act), means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is processed, summarized and reported, within the time periods specified in the Securities and Exchange Commission s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

Based on such evaluation, our principal executive officer and principal financial officer have concluded that as of December 31, 2013, our disclosure controls and procedures were effective.

Management s Annual Assessment of Internal Control Over Financial Reporting

Management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Exchange Act Rule 13a-15(f). Our internal control over financial reporting includes those policies and procedures that (i) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of our assets; (ii) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that our receipts and expenditures are being made only in accordance with authorizations of our management and directors; and (iii) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of our assets that could have a material effect on our financial statements.

Internal control over financial reporting is designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements prepared for external purposes in accordance with generally accepted accounting principles. Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Under the supervision and with the participation of our principal executive officer and our principal financial officer, management conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2013 based on the 1992 framework in Internal Control Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission and in accordance with the interpretive guidance issued by the SEC in Release No. 34-55929. Based on that evaluation, management concluded that our internal control over financial reporting was effective as of December 31, 2013.

There have been no changes in our internal control or in other factors that could have a material effect, or are reasonably likely to have a material effect on the internal control subsequent to the date of the evaluation in connection with the preparation of this Form 10-K.

Item 9B. Other Information

Not applicable.

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PART III

Item 10. Directors and Executive Officers of the Registrant

The information required by this item will be contained in our definitive proxy statement, or Proxy Statement, to be filed with the SEC in connection with our 2014 Annual Meeting of Stockholders. Our Proxy Statement for the 2014 Annual Meeting of Stockholders is expected to be filed not later than 120 days after the end of our fiscal year ended December 31, 2013 and is incorporated into this report by this reference.

We have adopted a code of ethics that applies to our chief executive officer, chief financial officer, and to all of our other officers, directors, employees and agents. The code of ethics is available on our website at www.catalystpharma.com. We intend to disclose future amendments to, or waivers from, certain provisions of our code of ethics on the above website within five business days following the date of such amendment or waiver.

Item 11. Executive Compensation

The information required by this item will be set forth in the Proxy Statement and is incorporated into this report by this reference.

Item 12. Security Ownership of Certain Beneficial Owners and Management

The information required by this item will be set forth in the Proxy Statement and is incorporated into this report by this reference.

Item 13. Certain Relationships and Related Transactions

The information required by this item will be set forth in the Proxy Statement and is incorporated into this report by this reference.

Item 14. Principal Accounting Fees and Services

The information required by this item will be set forth in the Proxy Statement and is incorporated into this report by this reference.

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PART IV

Item 15. Exhibits and Financial Statement Schedules

(a) Documents filed as part of this report.

1. The following financial statements of Catalyst Pharmaceutical Partners, Inc. and Report of Grant Thornton LLP, independent registered public accounting firm, are included in this report:

Report of Grant Thornton LLP, Independent Registered Public Accounting Firm.

Balance Sheets as of December 31, 2013 and 2012.

Statements of Operations for the years ended December 31, 2013, 2012 and 2011 and the period from inception (January 4, 2002) through December 31, 2013.

Statement of Stockholders Equity for the period from inception (January 4, 2002) through December 31, 2013.

Statements of Cash Flows for the years ended December 31, 2013, 2012 and 2011 and the period from inception (January 4, 2002) through December 31, 2013.

Notes to Financial Statements.

2. List of financial statement schedules. All schedules are omitted because they are not applicable or the required information is shown in the financial statements or notes thereto.

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

(b) Exhibits.

Exhibit No.	Description of Exhibit
2.1	Agreement and Plan of Merger, dated August 14, 2006, between the Company and Catalyst Pharmaceutical Partners, Inc., a Florida corporation(1)
3.1	Certificate of Incorporation(1)

3.2	Amendment to Certificate of Incorporation(1)
3.3	By-laws(1)
4.1	Specimen stock certificate for common stock(1)
4.2	Rights Agreement between the Company and Continental Stock Transfer and Trust Company(11)
4.3	Form of Warrant to Purchase Common Stock issued in our October 2011 offering; (12)
4.4	Form of Warrant to Purchase Common Stock issued in our May 2012 offering (15)
4.5	Form of Warrant to Purchase Common Stock issued in our August 2012 offering (16)
10.1 +	Employment Agreement between the Company and Patrick J. McEnany(2)
10.2 +	Amendment to Employment Agreement between the Company and Patrick J. McEnany(4)

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Exhibit No.	Description of Exhibit
10.3 +	Amendment to Employment Agreement between the Company and Patrick J. McEnany(6)
10.4 +	Amendment to Employment Agreement between the Company and Patrick J. McEnany(10)
10.5+	Amendment to Employment Agreement between the Company and Patrick J. McEnany (18)
10.6 +	Stock Option Agreement between the Company and Patrick J. McEnany(1)
10.7 +	Stock Option Agreement between the Company and Hubert Huckel(1)
10.8+	Agreement between the Company and Charles Gorodetzky(1)
10.9+	2006 Stock Incentive Plan(1)
10.10+	Amendment No. 1 to 2006 Stock Incentive Plan (8)
10.11+	Amendment No. 2 to 2006 Stock Incentive Plan (14)
10.12	License Agreement between the Company and Northwestern University(5)
10.13	Agreement between the Company and the Division of Pharmacotherapies and Medical Consequences of Drug Abuse, National Institute on Drug Abuse(7)
10.14	Lease Agreement between the Company and 355 Alhambra Plaza, Ltd.(3)
10.15	First Amendment to Lease Agreement between the Company and 355 Alhambra Plaza, Ltd. (9)
10.16	License Agreement among the Company, New York University, and The Feinstein Institute for Medical Research (13)
10.17	Convertible Promissory Note and Note Purchase Agreement, dated as of October 26, 2012, between the Company and BioMarin Pharmaceutical, Inc. (17)
10.18	License Agreement, dated as of October 26, 2012, between the Company and BioMarin Pharmaceutical, Inc. (17)
10.19	Termination Agreement, dated effective October 1, 2013, between the Company and Brookhaven Science Associates, LLC (19)
10.20	Second Amendment to Lease, dated as of February 4, 2014, between the Company and 355 Alhambra Circle LLC (20)
23.1	Consent of Independent Registered Public Accounting Firm*
31.1	Section 302 CEO Certification*
31.2	Section 302 CFO Certification*
32.1	Section 906 CEO Certification*
32.2	Section 906 CFO Certification*
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema

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Exhibit No.	Description of Exhibit
101.CAL	XBRL Taxonomy Extension Calculation Linkbase
101.DEF	XBRL Taxonomy Extension Definition Linkbase
101.LAB	XBRL Taxonomy Extension Label Linkbase
101.PRE	XBRL Taxonomy Extension Presentation Linkbase

(1) Filed by reference to the Company s Registration Statement on Form S-1 (File No. 333-136039)
(2) Filed by reference to the Company s Quarterly Report on Form 10-Q for the period ended September 30, 2006
(3) Filed by reference to the Company s Quarterly Report on Form 10-Q for the period ended March 31, 2007
(4) Filed by reference to the Company s Current Report on Form 8-K dated December 23, 2008
(5) Filed by reference to the Company s Current Report on Form 8-K dated September 2, 2009
(6) Filed by reference to the Company s Quarterly Report on Form 10-Q for the period ended September 30, 2009
(7) Filed by reference to the Company s Registration Statement on Form S-3 (File No. 333-170945)
(8) Filed by reference to the Company s 2011 Annual Meeting Proxy Statement on Schedule 14A dated April 11, 2011
(9) Filed by reference to the Company s Quarterly Report on Form 10-Q for the period ended June 30, 2011
(10) Filed by reference to the Company s Current Report on Form 8-K dated September 14, 2011
(11) Filed by reference to the Company s Current Report on Form 8-K dated September 20, 2011
(12) Filed by reference to the Company s Current Report on Form 8-K dated October 28, 2011
(13) Filed by reference to the Company s Annual Report on Form 10-K for the period ended December 31, 2011.
(14) Filed by reference to the Company s 2012 Annual Meeting Proxy Statement on Schedule 14A dated April 17, 2012
(15) Filed by reference to the Company s Registration Statement on Form S-1 (File No. 333-180617)
(16) Filed by reference to the Company s Current Report on Form 8-K dated August 28, 2012
(17) Filed by reference to the Company s Current Report on Form 8-K dated October 26, 2012
(18) Filed by reference to the Company s Current Report on Form 8-K dated August 28, 2013
(19) Filed by reference to the Company s Quarterly Report on Form 10-Q for the period ended September 30, 2013
(20) Filed by reference to the Company s Current Report on Form 8-K dated February 20, 2014
* Filed herewith
+ Management contract or compensatory plan

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SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the Registrant has caused this Annual Report on Form 10-K to be signed by the undersigned, thereunto duly authorized, this 18th day of March, 2014.

CATALYST PHARMACEUTICAL PARTNERS, IN

By: /s/ Patrick J. McEnany
Patrick J. McEnany, Chairman,
President and CEO

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed by the following persons, in the capacities and on the dates indicated.

Signature	Title	Date
/s/ Patrick J. McEnany Patrick J. McEnany	Chairman of the Board of Directors, President and Chief Executive Officer (Principal Executive Officer)	March 18, 2014
/s/ Alicia Grande Alicia Grande	Vice President, Treasurer, Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	March 18, 2014
/s/ Hubert E. Huckel, M.D. Hubert E. Huckel, M.D.	Director	March 18, 2014
/s/ Charles B. O Keeffe Charles B. O Keeffe	Director	March 18, 2014
/s/ Philip H. Coelho Philip H. Coelho	Director	March 18, 2014
/s/ David S. Tierney, M.D. David S. Tierney, M.D.	Director	March 18, 2014

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Years ended December 31, 2013, 2012, and 2011

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REPORT OF INDEPENDENT REGISTERED

PUBLIC ACCOUNTING FIRM

Board of Directors and Stockholders

Catalyst Pharmaceutical Partners, Inc.

We have audited the accompanying balance sheets of Catalyst Pharmaceutical Partners, Inc. (a Development Stage Company) (the Company) as of December 31, 2013 and 2012, and the related statements of operations, stockholders equity, and cash flows for each of the three years in the period ended December 31, 2013 and the period from January 4, 2002 (date of inception) through December 31, 2013. These financial statements are the responsibility of the Company s management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. We were not engaged to perform an audit of the Company s internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company s internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of Catalyst Pharmaceutical Partners, Inc. (a Development Stage Company) as of December 31, 2013 and 2012, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2013 and the period from January 4, 2002 (date of inception) through December 31, 2013 in conformity with accounting principles generally accepted in the United States of America.

/s/ Grant Thornton LLP
Miami, Florida

March 18, 2014

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CATALYST PHARMACEUTICAL PARTNERS, INC.

(a development stage company)

BALANCE SHEETS

	December 31, 2013	December 31, 2012
ASSETS		
Current Assets:		
Cash and cash equivalents	$ 2,215,958	$ 1,409,939
Certificates of deposit	4,011,576	6,502,825
Short-term investments	17,483,062	7,504,444
Prepaid expenses	1,609,442	1,309,470
Total current assets	25,320,038	16,726,678
Property and equipment, net	40,628	53,679
Deposits	8,888	8,888
Total assets	$ 25,369,554	$ 16,789,245
LIABILITIES AND STOCKHOLDERS EQUITY		
Current Liabilities:		
Accounts payable	$ 850,789	$ 1,365,663
Accrued expenses and other liabilities	1,288,820	281,002
Total current liabilities	2,139,609	1,646,665
Accrued expenses and other liabilities, non-current	19,131	21,878
Warrants liability, at fair value	1,819,562	498,587
Total liabilities	3,978,302	2,167,130
Commitments and contingencies		
Stockholders equity:		
Preferred stock, $0.001 par value, 5,000,000 shares authorized: none issued and outstanding at December 31, 2013 and 2012		
Common stock, $0.001 par value, 100,000,000 shares authorized; 54,132,937 shares and 41,420,687 shares issued and outstanding at December 31, 2013 and 2012, respectively	54,133	41,421
Additional paid-in capital	75,670,718	56,759,697
Deficit accumulated during the development stage	(54,333,599)	(42,179,003)
Total stockholders equity	21,391,252	14,622,115
Total liabilities and stockholders equity	$ 25,369,554	$ 16,789,245

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

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CATALYST PHARMACEUTICAL PARTNERS, INC.

(a development stage company)

STATEMENTS OF OPERATIONS

	Year Ended December 31,			Cumulative period from January 4, 2002 (date of inception) through December 31, 2013
	2013	2012	2011	
Revenues government grant	$	$	$	$ 488,958
Operating costs and expenses:				
Research and development	8,096,774	2,659,597	3,383,965	36,400,079
General and administrative	2,214,884	2,561,543	2,698,174	18,882,175
Total operating costs and expenses	10,311,658	5,221,140	6,082,139	55,282,254
Loss from operations	(10,311,658)	(5,221,140)	(6,082,139)	(54,793,296)
Interest income	47,421	14,976	10,985	1,540,186
Change in fair value of warrants liability	(1,890,359)	1,129,778	(319,908)	(1,080,489)
Loss before income taxes	(12,154,596)	(4,076,386)	(6,391,062)	(54,333,599)
Provision for income taxes				
Net loss	$ (12,154,596)	$ (4,076,386)	$ (6,391,062)	$ (54,333,599)
Net loss per share basic and diluted	$ (0.27)	$ (0.14)	$ (0.29)	
Weighted average shares outstanding basic and diluted	45,452,447	30,033,108	21,728,292	

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

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CATALYST PHARMACEUTICAL PARTNERS, INC.

(a development stage company)

STATEMENT OF STOCKHOLDERS EQUITY

for the period from January 4, 2002 (date of inception) through December 31, 2013

	Preferred Stock Series A	Preferred Stock Series B	Common Stock	Additional Paid-In Capital	Deficit Accumulated During the Development Stage	Total
Balance at January 4, 2002 (date of inception)	$	$	$ 21,888	$ 78,112	$	$ 100,000
Issuance of common stock, net			7,296	117,704		125,000
Issuance of stock options for services				75,833		75,833
Net loss					(255,945)	(255,945)
Balance at December 31, 2002			29,184	271,649	(255,945)	44,888
Issuance of preferred stock, net	700			669,757		670,457
Issuance of stock options for services				75,833		75,833
Net loss					(428,615)	(428,615)
Balance at December 31, 2003	700		29,184	1,017,239	(684,560)	362,563
Issuance of stock options for services				294,833		294,833
Net loss					(539,820)	(539,820)
Balance at December 31, 2004	700		29,184	1,312,072	(1,224,380)	117,576
Issuance of common stock, net			39,545	1,006,971		1,046,516
Issuance of common stock and stock options for services			146	1,087,604		1,087,750
Net loss					(1,805,380)	(1,805,380)
Balance at December 31, 2005	700		68,875	3,406,647	(3,029,760)	446,462
Change in par value	(630)		(61,988)	62,618		

Issuance of preferred stock Series B , net		8		3,225,132	3,225,140
Issuance of common stock (IPO), net			3,350	17,634,670	17,638,020
Conversion of preferred stock Series A into common stock, upon closing of IPO	(70)		1,022	(952)	
Conversion of preferred stock Series B into common stock, upon closing of IPO		(8)	1,116	(1,108)	
Issuance of common stock and stock options for services			142	1,266,323	1,266,465
Net loss				(2,729,454)	(2,729,454)
Balance at December 31, 2006			12,517	25,593,330	(5,759,214) 19,846,633
Issuance of common stock and stock options for services			11	579,676	579,687
Amortization of restricted stock for services				35,930	35,930
Net loss				(4,139,493)	(4,139,493)
Balance at December 31, 2007			12,528	26,208,936	(9,898,707) 16,322,757
Issuance of common stock, net			1,488	4,086,412	4,087,900
Issuance of stock options for services				583,836	583,836
Issuance of restricted stock units for services, net			44	130,275	130,319
Net loss				(10,564,597)	(10,564,597)
Balance at December 31, 2008			14,060	31,009,459	(20,463,304) 10,560,215
Issuance of common stock, net			3,973	3,694,162	3,698,135
Issuance of stock options for services				581,286	581,286
Issuance of restricted stock units for services, net			5	20,147	20,152
Net loss				(7,241,928)	(7,241,928)
Balance at December 31, 2009 (carried forward)			18,038	35,305,054	(27,705,232) 7,617,860

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

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CATALYST PHARMACEUTICAL PARTNERS, INC.

(a development stage company)

STATEMENT OF STOCKHOLDERS EQUITY

for the period from January 4, 2002 (date of inception) through December 31, 2013

	Preferred Stock Series A	Preferred Stock Series B	Common Stock	Additional Paid-In Capital	Deficit Accumulated During the Development Stage	Total
Balance at December 31, 2009 (brought forward)	$	$	$ 18,038	$ 35,305,054	$ (27,705,232)	$ 7,617,860
Issuance of common stock, net			1,352	1,454,801		1,456,153
Issuance of stock options for services				450,089		450,089
Issuance of restricted stock units for services, net			5	(5)		
Net loss					(4,006,323)	(4,006,323)
Balance at December 31, 2010			19,395	37,209,939	(31,711,555)	5,517,779
Issuance of stock options for services				416,735		416,735
Issuance of common stock and warrants, net			5,306	4,211,940		4,217,246
Net loss					(6,391,062)	(6,391,062)
Balance at December 31, 2011			24,701	41,838,614	(38,102,617)	3,760,698
Issuance of common stock, net			53	33,071		33,124
Issuance of stock options for services				340,039		340,039
Issuance of common stock and warrants, net			10,000	9,554,640		9,564,640
Issuance of common stock upon note conversion			6,667	4,993,333		5,000,000
Net loss					(4,076,386)	(4,076,386)
Balance at December 31, 2012			41,421	56,759,697	(42,179,003)	14,622,115
Issuance of common stock, net			8,850	14,086,344		14,095,194
				175,855		175,855

Issuance of stock options for services						
Exercise of warrants for common stock			3,862	4,648,822		4,652,684
Net loss					(12,154,596)	(12,154,596)
Balance at December 31, 2013	$	$	$ 54,133	$ 75,670,718	$ (54,333,599)	$ 21,391,252

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

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CATALYST PHARMACEUTICAL PARTNERS, INC.

(a development stage company)

STATEMENTS OF CASH FLOWS

	Year Ended December 31,			Cumulative period from January 4, 2002 (date of inception) through December 31, 2013
	2013	2012	2011	
Operating Activities:				
Net loss	$ (12,154,596)	$ (4,076,386)	$ (6,391,062)	$ (54,333,599)
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation and amortization	22,483	10,889	42,835	187,361
Stock-based compensation	175,855	340,039	416,735	6,138,055
Change in fair value of warrants liability	1,890,359	(1,129,778)	319,908	1,080,489
(Increase) decrease in:				
Government grant receivable			134,025	
Prepaid expenses and deposits	(299,972)	(1,110,354)	(31,272)	(1,618,330)
Increase (decrease) in:				
Accounts payable	(514,874)	1,101,729	158,001	850,789
Accrued expenses and other liabilities	1,005,071	(276,505)	365,781	1,244,599
Net cash used in operating activities	(9,875,674)	(5,140,366)	(4,985,049)	(46,450,636)
Investing Activities:				
Capital expenditures	(9,432)	(52,382)	(3,620)	(164,640)
Purchase of short-term investments	(9,978,618)	(7,504,444)		(17,483,062)
Proceeds (purchase) of certificates of deposit	2,491,249	(6,502,825)		(4,011,576)
Net cash used in investing activities	(7,496,801)	(14,059,651)	(3,620)	(21,659,278)
Financing Activities:				
Proceeds from issuance of common stock and warrants, net	14,071,694	9,564,640	5,542,578	57,210,636
Proceeds from issuance of preferred stock, net				3,895,597
Proceeds from issuance of convertible promissory note		5,000,000		5,000,000
Proceeds from exercise of warrants	4,083,300	16,249		4,099,549
Proceeds from exercise of options	23,500			23,500

Payment of employee withholding tax related to restricted stock units					(3,410)
Net cash provided by financing activities	18,178,494	14,580,889	5,542,578	70,225,872	
Net increase (decrease) in cash and cash equivalents	806,019	(4,619,128)	553,909	2,115,958	
Cash and cash equivalents beginning of period	1,409,939	6,029,067	5,475,158	100,000	
Cash and cash equivalents end of period	$ 2,215,958	$ 1,409,939	$ 6,029,067	$ 2,215,958	
Non-cash investing and financing activities:					
Non-cash incentive received from lessor	$	$	$	$ 52,320	
Exercise of liability classified warrants for common stock	$ 569,384	$ 16,875	$	$ 586,259	
Conversion of note to common stock	$	$ 5,000,000	$	$ 5,000,000	

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

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CATALYST PHARMACEUTICAL PARTNERS, INC.

(a development stage company)

NOTES TO FINANCIAL STATEMENTS

1. Organization and Description of Business

Catalyst Pharmaceutical Partners, Inc. (the Company) is a development-stage specialty pharmaceutical company focused on the development and commercialization of prescription drugs targeting rare (orphan) neurological diseases and disorders, including Lambert-Eaton Myasthenic Syndrome (LEMS) and infantile spasms. The Company was incorporated in Delaware in July 2006. It is the successor by merger to Catalyst Pharmaceutical Partners, Inc., a Florida corporation, which commenced operations in January 2002.

The Company has incurred operating losses in each period from inception through December 31, 2013. The Company has been able to fund its cash needs to date through public and private offerings of its common stock and warrants and through government grants. See Note 11.

Capital Resources

On January 31, 2014, the Company filed a shelf Registration Statement on Form S-3 (the 2014 Shelf Registration Statement) with the SEC to sell up to $100 million of common stock. The 2014 shelf Registration Statement has not yet been declared effective. See Note 15.

While there can be no assurance, based on available information, the Company currently believes that it has sufficient resources to support its planned operations through at least the end of 2014. The Company will require additional capital to support its planned operations in periods after the end of 2014.

The Company may raise in the future required funds through public or private equity offerings, debt financings, corporate collaborations, governmental research grants or other means. The Company may also seek to raise new capital to fund additional product development efforts, even if it has sufficient funds for its planned operations. Any sale by the Company of additional equity or convertible debt securities could result in dilution to the Company s current stockholders. There can be no assurance that any required additional funding will be available to the Company at all or available on terms acceptable to the Company. Further, to the extent that the Company raises funds through collaborative arrangements, it may be necessary to relinquish some rights to the Company s technologies or grant sublicenses on terms that are not favorable to the Company. If the Company is not able to secure additional funding when needed, the Company may have to delay, reduce the scope of, or eliminate one or more research and development programs, which could have an adverse effect on the Company s business.

2. Basis of Presentation and Significant Accounting Policies

a. **DEVELOPMENT STAGE COMPANY.** Since inception, the Company has devoted substantially all of its efforts to business planning, research and development, recruiting management and technical staff, acquiring operating assets and raising capital. Accordingly, the Company is considered to be in the development stage

and the Company s financial statements are presented in that manner in accordance with U.S. generally accepted accounting principles. The Company s primary focus is on the development and commercialization of its drug candidates.

b. **USE OF ESTIMATES.** The preparation of financial statements in conformity with U.S. generally accepted accounting principles requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. Actual results could differ from those estimates.

c. **CASH AND CASH EQUIVALENTS.** The Company considers all highly liquid instruments, purchased with an original maturity of three months or less to be cash equivalents. Cash equivalents consist mainly of U.S. Treasury bills, certificates of deposit and money market funds. The Company has substantially all of its cash and cash equivalents deposited with one financial institution.

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

d. CERTIFICATES OF DEPOSIT. The certificates of deposit were issued by a banking institution and are recorded at cost plus accrued interest. The original maturity was greater than three months but did not exceed one year. Interest income is recorded in the statement of operations as it is earned. Carrying value at December 31, 2013 and 2012 approximates fair value.

e. SHORT-TERM INVESTMENTS. The Company invests in short-term investments in high credit-quality funds in order to obtain higher yields on its cash available for investments. As of December 31, 2013 and 2012 short-term investments consisted of a short-term bond fund. Such investments are not insured by the Federal Deposit Insurance Corporation. Short-term investments at December 31, 2013 and 2012 were considered trading securities. Trading securities are recorded at fair value based on the closing market price of the security. For trading securities, the Company charges realized gains and losses and unrealized gains and losses to earnings. Unrealized and realized losses on trading securities for the years ended December 31, 2013 and 2012 were nominal.

f. PREPAID EXPENSES. Prepaid expenses consist primarily of prepaid research fees, prepaid insurance and prepaid subscription fees. Prepaid research fees consist of advances for the Company s drug development activities, including drug manufacturing, contracts for pre-clinical studies, clinical trials and studies, regulatory affairs and consulting. Such advances are recorded as expense as the related goods are received or the related services are performed.

g. PROPERTY AND EQUIPMENT. Property and equipment are recorded at cost. Depreciation is calculated to amortize the depreciable assets over their useful lives using the straight-line method and commences when the asset is placed in service. Leasehold improvements are amortized on a straight-line basis over the term of the lease or the estimated life of the improvement, whichever is shorter. Useful lives generally range from three years for computer equipment to three to six years for furniture and equipment and leasehold improvements. Expenditures for repairs and maintenance are charged to expenses as incurred.

h. OPERATING LEASES. The Company recognizes lease expense on a straight-line basis over the initial lease term. For leases that contain rent holidays, escalation clauses or tenant improvement allowances, the Company recognizes rent expense on a straight-line basis and records the difference between the rent expense and rental amount payable as deferred rent. As of December 31, 2013 and 2012, the Company had $21,877 and $22,643, respectively, of deferred rent in accrued expenses and other liabilities.

i. FAIR VALUE OF FINANCIAL INSTRUMENTS. The Company s financial instruments consist of cash and cash equivalents, certificates of deposit, short-term investments, accounts payable and accrued expenses and other liabilities, and warrants liability. At December 31, 2013, the fair value of these instruments approximated their carrying value.

j.

FAIR VALUE MEASUREMENTS. Current Financial Accounting Standards Board (FASB) fair value guidance emphasizes that fair value is a market-based measurement, not an entity-specific measurement. Therefore, a fair value measurement should be determined based on the assumptions that market participants would use in pricing the asset or liability. As a basis for considering market participant assumptions in fair value measurements, current FASB guidance establishes a fair value hierarchy that distinguishes between market participant assumptions based on market data obtained from sources independent of the reporting entity (observable inputs that are classified within Levels 1 and 2 of the hierarchy) and the reporting entity s own assumptions that it believes market participants would use in pricing assets or liabilities (unobservable inputs classified within Level 3 of the hierarchy).

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

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

		Fair Value Measurements at Reporting Date Using		
	Balances as of December 31, 2013	Quoted Prices in Active Markets for Identical Assets/Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Money market funds	$ 25,693	$ 25,693	$	$
Certificates of deposit	$ 4,011,576	$	$ 4,011,576	$
Short-term investments	$ 17,483,062	$ 17,483,062	$	$
Warrants liability	$ 1,819,562	$	$	$ 1,819,562

		Fair Value Measurements at Reporting Date Using		
	Balances as of December 31, 2012	Quoted Prices in Active Markets for Identical Assets/Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Certificates of deposit	$ 6,502,825	$	$ 6,502,825	$
Short-term investments	$ 7,504,444	$ 7,504,444	$	$
Warrants liability	$ 498,587	$	$	$ 498,587

k. WARRANTS LIABILITY. In October 2011, the Company issued 1,523,370 warrants (the 2011 warrants) to purchase shares of the Company s common stock in connection with a registered direct offering under the 2010 Shelf Registration Statement. The Company accounted for these warrants as a liability measured at fair value due to a provision included in the warrants agreement that provides the warrants holders with an option to require the Company (or its successor) to purchase their warrants for cash in an amount equal to their Black-Scholes Option Pricing Model (the Black-Scholes Model) value, in the event that certain fundamental transactions, as defined, occur. The fair value of the warrants liability is estimated using the Black-Scholes Model which requires inputs such as the expected term of the warrants, share price volatility and risk-free interest rate. These assumptions are reviewed on a quarterly basis and changes in the estimated fair value of the outstanding warrants are recognized each reporting period in the Change in fair value of warrants liability line in the statement of operations. As of December 31, 2013, 1,254,870 of the 2011 warrants remained outstanding.

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

l. RESEARCH AND DEVELOPMENT. Costs incurred in connection with research and development activities are expensed as incurred. These costs consist of direct and indirect costs associated with specific projects as well as fees paid to various entities that perform research related services for the Company.

m. STOCK-BASED COMPENSATION. The Company recognizes expense in the statement of operations for the fair value of all stock-based payments to employees, directors and consultants, including grants of stock options and other share-based awards. For stock options, the Company uses the Black-Scholes option valuation model, the single-option award approach and straight-line attribution method. Using this approach, compensation cost is amortized on a straight-line basis over the vesting period of each respective stock option, generally three to five years. The Company estimates forfeitures and adjusts this estimate periodically based on actual forfeitures.

For the years ended December 31, 2013, 2012 and 2011, the Company recorded stock-based compensation expense as follows:

	2013	2012	2011
Research and development	$ 84,728	$ 100,221	$ 111,283
General and administrative	91,127	239,818	305,452
Total stock-based compensation	$ 175,855	$ 340,039	$ 416,735

n. CONCENTRATION OF CREDIT RISK. The financial instruments that potentially subject the Company to concentration of credit risk are cash equivalents (i.e. money market funds), short-term investments and certificates of deposit. The Company places its cash equivalents with high-credit quality financial institutions. These amounts at times may exceed federally insured limits. The Company has not experienced any credit losses in these accounts.

o. INCOME TAXES. The Company utilizes the asset and liability method of accounting for income taxes. Under this method, deferred tax assets and liabilities are determined based on differences between financial reporting and tax bases of assets and liabilities and are measured using enacted tax rates and laws that will be in effect when the differences are expected to reverse. A valuation allowance is provided when it is more likely than not that some portion or all of a deferred tax asset will not be realized.

The Company recognizes the financial statement benefit of a tax position only after determining that the relevant tax authority would more likely than not sustain the position following an audit. For tax positions meeting the more-likely-than-not threshold, the amount recognized in the financial statements is the largest benefit that has a greater than 50 percent likelihood of being realized upon ultimate settlement with the relevant tax authority.

The Company is subject to income taxes in the U.S. federal jurisdiction and various state jurisdictions. Tax regulations within each jurisdiction are subject to the interpretation of the related tax laws and regulations and require significant judgment to apply. The Company is not subject to U.S. federal, state and local tax examinations by tax authorities for

years before 2010. If the Company were to subsequently record an unrecognized tax benefit, associated penalties and tax related interest expense would be reported as a component of income tax expense.

p. **COMPREHENSIVE INCOME (LOSS).** U.S. generally accepted accounting principles require that all components of comprehensive income (loss) be reported in the financial statements in the period in which they are recognized. Comprehensive income (loss) is net income (loss), plus certain other items that are recorded directly into stockholders equity. The Company has reported comprehensive income (loss) in the statement of stockholders equity as net income (loss).

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

q. NET INCOME (LOSS) PER SHARE. Basic income (loss) per share is computed by dividing net income (loss) for the period by the weighted average number of common shares outstanding during the period. Diluted income (loss) per share is computed by dividing net income (loss) for the period by the weighted average number of common shares outstanding during the period, plus the dilutive effect of common stock equivalents, such as convertible preferred stock, stock options and restricted stock units. For all periods presented, all common stock equivalents were excluded because their inclusion would have been anti-dilutive. The potential shares, which are excluded from the determination of basic and diluted net loss per share as their effect is anti-dilutive, are as follows, for the years ended December 31, 2013, 2012 and 2011:

	2013	2012	2011
Options to purchase common stock	3,428,906	3,650,535	3,723,108
Warrants to purchase common stock	4,848,620	8,710,870	1,523,370
Potential equivalent common stock excluded	8,277,526	12,361,405	5,246,478

Potentially dilutive options to purchase common stock as of December 31, 2013, 2012 and 2011 have exercise prices ranging from $0.47 to $6.00. Potentially dilutive warrants to purchase common stock as of December 31, 2013, 2012 and 2011 have exercise prices ranging from $1.04 to $2.08.

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

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

t. RECENTLY ISSUED ACCOUNTING STANDARDS. There are no recent accounting pronouncements which the Company anticipates will have a significant impact on the Company s financial statements.

3. Warrants Liability, at Fair Value

2011 Warrants

The Company allocated approximately $1.3 million of proceeds from its October 2011 registered direct offering to the fair value of common stock purchase warrants issued in connection with the offering that are classified as a liability (the 2011 warrants). The 2011 warrants are classified as a liability because of provisions in such warrants that allow for the net cash settlement of such warrants in the event of certain fundamental transactions (as defined in the warrant agreement). The valuation of the 2011 warrants is determined using the Black-Scholes Model. This model uses inputs such as the underlying price of the shares issued when the warrant is exercised, volatility, risk free interest rate and expected life of the instrument. The Company has determined that the 2011 warrants liability should be classified

within Level 3 of the fair value hierarchy by evaluating each input for the Black-Scholes Model against the fair value hierarchy criteria and using the lowest level of input as the basis for the fair value classification. There are six inputs: closing price of the Company s common stock on the day of evaluation; the exercise price of the warrants; the remaining term of the warrants; the volatility of the Company s common stock; annual rate of dividends; and the risk free rate of return. Of those inputs, the exercise price of the warrants and the remaining term are readily observable in the warrants agreement. The annual rate of dividends is based on the Company s historical practice of not granting dividends. The closing price of the Company s common stock would fall under Level 1 of the fair value hierarchy as it is a quoted price in an active market. The risk free rate of return is a Level 2 input, while the historical volatility is a Level 3 input in accordance with the fair value accounting guidance. Since the lowest level input is a Level 3, the Company determined the 2011 warrants liability is most appropriately classified within Level 3 of the fair value hierarchy. This liability is subject to fair value mark-to-market adjustment each reporting period. The calculated value of the 2011 warrants liability was determined using the Black-Scholes option-pricing model with the following assumptions:

	December 31, 2013	December 31, 2012
Risk free interest rate	0.94%	0.60%
Expected term	3.34 years	4.34 years
Expected volatility	108%	136%
Expected dividend yield	0%	0%
Expected forfeiture rate	0%	0%

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3. Warrants Liability, at Fair Value (continued)

The following table rolls forward the fair value of the Company s warrants liability activity for the years ended December 31, 2013, 2012 and 2011:

	2013	2012	2011
Fair value, beginning of period	$ 498,587	$ 1,645,240	$
Issuance of warrants			1,325,332
Exercise of warrants	(569,384)	(16,875)	
Change in fair value	1,890,359	(1,129,778)	319,908
Fair value, end of period	$ 1,819,562	$ 498,587	$ 1,645,240

During 2013, 256,000 of the 2011 warrants were exercised, with proceeds to the Company of $332,800. During 2012, 12,500 of the 2011 warrants were exercised, with proceeds to the Company of $16,249. The Company recognizes the change in the fair value of the warrants liability as a non-operating income or loss in the accompanying statements of operations. See Note 15.

4. Prepaid Expenses

Prepaid expenses consist of the following as of December 31:

	2013	2012
Prepaid research fees	$ 1,334,149	$ 1,138,185
Prepaid insurance	219,651	143,520
Prepaid subscriptions fees	24,643	12,369
Prepaid rent	7,848	683
Other	23,151	14,713
Total prepaid expenses	$ 1,609,442	$ 1,309,470

5. Property and Equipment

Property and equipment, net consists of the following as of December 31:

	2013	2012
Computer equipment	$ 81,551	$ 74,191
Furniture and equipment	51,523	49,451

	133,074	123,642
Less: Accumulated depreciation	(92,446)	(69,963)
Total property and equipment, net	$ 40,628	$ 53,679

Depreciation expense was $22,483, $10,889 and $42,835, respectively, for the years ended December 31, 2013, 2012 and 2011.

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6. Accrued Expenses and Other Liabilities

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

	2013	2012
Accrued pre-clinical and clinical trial expenses	$ 1,083,749	$ 197,572
Accrued professional fees	117,240	51,050
Accrued compensation and benefits	14,539	5,949
Accrued license fees	65,000	15,000
Deferred rent	2,746	765
Other	5,546	10,666
Current accrued expenses and other liabilities	1,288,820	281,002
Deferred rent non-current	19,131	21,878
Non-current accrued expenses and other liabilities	19,131	21,878
Total accrued expenses and other liabilities	$ 1,307,951	$ 302,880

7. Commitments and Contingencies

The Company has contracted with drug manufacturers and other vendors, including the clinical research organization (CRO) overseeing the clinical trial of one of the Company s drug candidates, to assist in the execution of the Company s pre-clinical and clinical trials, analysis, and the preparation of material necessary for the future filings of new drug applications (NDA s) with the U.S. Food and Drug Administration (FDA). The contracts are cancelable at any time, but obligate the Company to reimburse the providers for any time or costs incurred through the date of termination.

The Company has executed a noncancellable operating lease agreement for its corporate office. The lease has free and escalating rent payment provisions. The Company recognizes rent expense under such lease on a straight-line basis over the term of the lease. As of December 31, 2013, future minimum lease payments under the operating lease agreement are as follows:

2014	$ 68,534
2015	70,576
2016	72,678
2017	67,828
	$ 279,616

During June 2011, in connection with the renewal of the corporate office lease, the Company entered into the first amendment to the lease. The amendment extends the original lease term for five years and relocates the Company into another space within the same building. The corporate office lease is cancellable upon the payment of an early termination penalty during 2015. The relocation occurred in November 2011. The lease provides for fixed increases in

minimum annual rent payments, as well as rent free periods. The total amount of rental payments due over the lease term is being charged to rent expense on the straight-line method over the term of the lease. The differences between rent expense recorded and the amount paid is credited or charged to accrued expenses and other liabilities in the accompanying balance sheets. Rent expense was $69,930, $65,310 and $61,653, respectively, for the years ended December 31, 2013, 2012 and 2011. The Company s leases expire on various dates through November 2017. Subsequent to year end, during February 2014, the Company entered into the second amendment of the lease for an additional contiguous space under substantially the same terms.

Securities Class Action Lawsuit

In October 2013 and November 2013, three securities class action lawsuits were filed against the Company and certain of its executive officers and directors seeking unspecified damages in the U.S. District Court for the Southern District of Florida (the Court). The complaints, which were substantially identical, purported to state a claim for violation of federal securities laws on behalf of a class of those who purchased the Company s common stock between October 31, 2012 and October 18, 2013. Two of the cases were voluntarily dismissed by the plaintiffs and the Court granted the Company s motion to dismiss the third case on January 3, 2014. However, the Court granted leave to the plaintiffs to file an amended complaint within 20 days.

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7. Commitments and Contingencies (continued)

On January 23, 2014, the plaintiffs filed an amended complaint against the Company and one of its executive officers seeking unspecified damages. The amended complaint purports to state a claim for alleged misrepresentations regarding the development of Firdapse on behalf of a class of those who purchased the Company s common stock between August 27, 2013 and October 18, 2013. The Company has filed a motion to dismiss the amended complaint, which has not yet been ruled upon by the Court. The Company believes that the amended lawsuit, which is at a very early stage, is without merit, and the Company intends to vigorously defend this lawsuit. While there can be no assurance, the Company does not expect this lawsuit to have a material adverse effect on the Company, and no amounts have been accrued with respect to this potential contingent liability in the accompanying December 31, 2013 balance sheet.

Obligations under capital leases are not significant.

For commitments related to the Company s license agreements with BioMarin (defined below), Brookhaven (defined below), and Northwestern (defined below), see Note 8.

8. Agreements

a. **LICENSE AGREEMENT WITH BROOKHAVEN.** The Company had a license agreement with Brookhaven Science Associates, LLC, as operator of Brookhaven National Laboratory under contract with the United States Department of Energy (Brookhaven), whereby the Company has obtained an exclusive license for several patents and patent applications in the U.S. and outside the U.S. relating to the use of vigabatrin as a treatment for cocaine and other addictions and obsessive-compulsive disorders. This license agreement ran concurrently with the term of the last to expire of the licensed patents, the last of which currently expires in 2023. The Company paid a fee to obtain the license in the amount of $50,000. Under the license agreement, the Company agreed to pay Brookhaven certain milestones and to reimburse them for certain patent related expenses.

On November 8, 2013, effective October 1, 2013, the Company and Brookhaven entered into a termination agreement cancelling the license agreement. As part of that agreement, the Company and Brookhaven entered into mutual releases, including a release from any further obligation for the Company to reimburse Brookhaven for any of Brookhaven s patent related expenses.

b. **LICENSE AGREEMENT WITH NORTHWESTERN UNIVERSITY.** On August 27, 2009, the Company entered into a license agreement with Northwestern University (Northwestern), under which it acquired worldwide rights to commercialize new GABA aminotransferase inhibitors and derivatives of vigabatrin that have been discovered by Northwestern. Under the terms of the license agreement, Northwestern granted the Company an exclusive worldwide license to certain composition of matter patents related to the new class of inhibitors and a patent application relating to derivatives of vigabatrin. The Company has identified and designated the lead compound under this license as CPP-115.

Under the license agreement with Northwestern, the Company will be responsible for continued research and development of any resulting product candidates. As of December 31, 2013, the Company had paid Northwestern

$246,590 in connection with the license and had accrued license fees of $65,000 and $15,000 as of December 31, 2013 and 2012, respectively, in the accompanying balance sheets for expenses, maintenance fees and milestones. In addition, the Company is obligated to pay certain milestone payments in future years relating to clinical development activities with respect to CPP-115, and royalties on any products resulting from the license agreement. The next milestone payment of $150,000 is due on the earlier of successful completion of the first Phase 2 clinical trial for CPP-115 or August 27, 2015.

c. **LICENSE AGREEMENT WITH NEW YORK UNIVERSITY AND THE FEINSTEIN INSTITUTE FOR MEDICAL RESEARCH.** On December 13, 2011, the Company entered into a license agreement with New York University (NYU) and the Feinstein Institute for Medical Research (FIMR) under which it acquired worldwide rights to commercialize GABA aminotransferase inhibitors in the treatment for Tourette s Syndrome. The Company is obligated to pay certain milestone payments in future years relating to clinical development activities and royalties on any products resulting from the license agreement.

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8. Agreements (continued)

d. LICENSE AGREEMENT WITH BIOMARIN. On October 26, 2012, the Company entered into a strategic collaboration with BioMarin for Firdapse . The key components of the collaboration include: (i) the Company licensed the exclusive North American rights to Firdapse pursuant to a License Agreement, dated as of October 26, 2012 (the License Agreement) between the Company and BioMarin, and (ii) BioMarin made a $5,000,000 investment in the Company pursuant to the terms of a Convertible Promissory Note and Note Purchase Agreement, dated as of October 26, 2012 (the Investment Agreement). The Investment Agreement provides that the Company will use the $5 million solely for the purpose of developing Firdapse . Initially, the $5,000,000 investment from BioMarin was treated as a loan to the Company. However, on December 10, 2012, the loan automatically converted, at a conversion rate of $0.75 per share, into 6,666,667 shares of the Company s authorized but unissued common stock.

As part of the License Agreement, the Company has taken over a Phase 3 clinical trial previously being conducted by BioMarin and is obligated to use its diligent efforts to seek to obtain regulatory approval for and to commercialize Firdapse in the United States. The Company is obligated to use diligent efforts to complete the double-blind treatment phase of the Phase 3 trial within 24 months of entering into the License Agreement, and BioMarin has the right to terminate the License Agreement if such treatment phase has not been completed in such 24-month period (unless the Company is using diligent effort to pursue the completion of such treatment phase and has spent at least $5 million in connection with the conduct of the Phase 3 clinical trial during such 24 month period). As of December 31, 2013, the Company had disbursed approximately $4.1 million in connection with expenses related to the Phase 3 trial, and the Company anticipates that the remaining $0.9 million will be expended during 2014.

As part of the License Agreement, the Company has agreed to (i) pay BioMarin certain royalty payments based on net sales in North America; (ii) to pay to a third-party licensor of the rights sublicensed certain royalty payments based on net sales in North America, and (iii) to pay certain milestone payments that BioMarin is obligated to make (approximately $2.6 million of which will be due upon acceptance by the FDA of a filing of an NDA for Firdapse for the treatment of LEMS, and approximately $7.2 million of which will be due on the unconditional approval by the FDA of an NDA for Firdapse for the treatment of LEMS). The Company has also agreed to share in the cost of certain post-marketing studies that are being conducted by BioMarin.

e. AGREEMENTS WITH CONTRACT MANUFACTURERS, CONTRACT RESEARCH ORGANIZATIONS AND FOR LABORATORIES AND OTHER RELATED TRIAL TESTS. The Company has entered into agreements with contract manufacturers for the manufacture of drug and study placebo for the Company s trials and studies; with contract research organizations (CROs) to conduct and monitor the Company s trials and studies; and with various entities for laboratories and other testing related to the Company s trials and studies. The contractual terms of the agreements vary, but most require certain advances as well as payments based on the achievement of milestones. Further, these agreements are cancellable at any time, but obligate the Company to reimburse the providers for any time or costs incurred through the date of termination.

9. Related Party Transactions

The Company has entered into consulting agreements with one of the Company s officers and members of the Company s Scientific Advisory Board. During the years ended December 31, 2013, 2012 and 2011, the Company paid

approximately $10,000, $42,000 and $93,000, respectively, in consulting fees to related parties.

The Company has an employment agreement with Patrick J. McEnany, its Chairman, President and Chief Executive Officer. Under this agreement, Mr. McEnany will receive an annual base salary of approximately $425,000 in 2014, and may earn bonus compensation based on performance. This agreement expires in November 2016.

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10. Income Taxes

As of December 31, 2013 and 2012, the Company had deferred tax assets of approximately $19,387,000 and $15,591,000, respectively, of which approximately $17,685,000 and $13,956,000 represent United States federal and state net operating loss carryforwards and start-up costs. The remaining temporary differences represent non-deductible stock option and equity expense. The related deferred tax asset has a 100% valuation allowance as of December 31, 2013 and 2012, as the Company believes it is more likely than not that the deferred tax asset will not be realized. The change in valuation allowance was approximately $3,796,000, $2,151,000 and $2,012,000 in 2013, 2012 and 2011, respectively. There are no other significant temporary differences. The net operating loss carry-forwards of approximately $30,675,000 as of December 31, 2013 will expire at various dates beginning in 2024 and ending in 2033. If an ownership change, as defined under Internal Revenue Code Section 382, occurs, the use of these carry-forwards may be subject to limitation. The effective tax rate of 0% in all periods presented differs from the statutory rate of 35% due to the valuation allowance and because the Company had no taxable income.

11. Stockholders Equity

Preferred Stock

The Company has 5,000,000 shares of authorized preferred stock, $0.001 par value per share at December 31, 2013 and 2012. No shares of preferred stock were outstanding at December 31, 2013 and 2012.

Common Stock

The Company has 100,000,000 shares of authorized common stock with a par value of $0.001 per share. At December 31, 2013 and 2012, 54,132,937 and 41,420,687 shares, respectively, of common stock were issued and outstanding. Each holder of common stock is entitled to one vote of each share of common stock held of record on all matters on which stockholders generally are entitled to vote.

2010 Shelf Registration Statement

On December 3, 2010, the Company filed a shelf Registration Statement on Form S-3 (the 2010 Shelf Registration Statement) with the SEC to sell up to $30 million of common stock and common stock purchase warrants. This registration statement (file No. 333-170945) was declared effective by the SEC on December 15, 2010. The Company has to date conducted the following sales of its securities under the 2010 Shelf Registration Statement:

(a) On March 8, 2011, the Company filed a prospectus supplement and offered for sale to institutional investors 2,259,943 shares of its common stock at a price of $1.12 per share and received gross proceeds of approximately $2.5 million, before underwriting commission and incurred expenses of approximately $300,000.

(b) On October 28, 2011, the Company filed a prospectus supplement and offered for sale to institutional investors 3,046,740 shares of its common stock together with common stock purchase warrants to purchase 1,523,370 shares of the Company s common stock at a price of $1.15 per share and corresponding warrant and received gross proceeds of approximately $3.5 million, before underwriting commission and other expenses totaling approximately $335,000. The warrants issued in this offering, which expire on April 28,

2017 and have an exercise price of $1.30 per share, have been accounted for as a liability. See Note 3.

(c) On August 28, 2012, the Company filed a prospectus supplement and offered for sale to institutional investors 4,000,000 shares of its common stock together with common stock purchase warrants to purchase 1,200,000 shares of the Company s common stock at a price of $1.50 per share and corresponding warrant and received gross proceeds of approximately $6.0 million, before underwriting commission and other expenses totaling approximately $440,000. These warrants, which will expire on August 28, 2017 and have an exercise price of $2.08 per share, have been accounted for as equity instruments, since they do not contain features (such as cash settlement or anti-dilution features) that would preclude the Company from accounting for these warrants as equity.

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11. Stockholders Equity (continued)

(d) On September 5, 2013, the Company filed a prospectus supplement and offered for sale to institutional investors 8,800,000 shares of its common stock at a price of $1.72 per share and received gross proceeds of approximately $15.1 million before underwriting commissions and incurred expenses of approximately $1,064,000.

The Company has no further availability under the 2010 Shelf Registration Statement.

2012 Form S-1 Registration Statement

On May 24, 2012, the Company sold 6,000,000 shares of its common stock together with common stock purchase warrants to purchase 6,000,000 shares of the Company s common stock, at a price of $0.80 per share and corresponding warrant. These securities were issued pursuant to a Form S-1 registration statement that became effective on May 23, 2012 (file no. 333-180617). The Company received gross proceeds of approximately $4.8 million from this offering, before underwriting commission and other expenses totaling approximately $795,000. The May 2012 warrants, which expire five years from their date of issuance and have an exercise price of $1.04 per share, have been accounted for as equity instruments, since they do not contain features (such as net cash settlement or anti-dilution features) that would preclude the Company from accounting for these warrants as equity.

BioMarin convertible promissory note automatic conversion into common stock shares

On October 26, 2012, the Company entered into a note purchase agreement with BioMarin, pursuant to which the Company issued BioMarin a convertible promissory note in the principal amount of $5 million. (See Note 8). The $5 million note automatically converted into 6,666,667 shares of the Company s common stock (at a price of $0.75 per share) on December 10, 2012.

Nasdaq Listing

The Company s common stock currently trades on the Nasdaq Capital Market. On December 24, 2012, the Company received a staff deficiency letter from The Nasdaq Stock Market (Nasdaq) notifying the Company that it was not in compliance with the minimum bid price requirement set forth in Nasdaq Listing Rule 5550(a)(2) for continued listing on the Nasdaq Capital Market. The Nasdaq Listing Rules (the Rules) require listed securities to maintain a minimum bid price of $1.00 per share and, based on the then closing bid prices for the last 30 consecutive business days, the Company no longer met that requirement. Under the Rules, the Company had a grace period of 180 days, or until June 24, 2013, to regain compliance. On June 25, 2013, the Company received a letter from the Listing Qualifications Staff of the Nasdaq indicating that the Company had been granted an additional 180-day grace period (until December 23, 2013) to regain compliance with the minimum bid price requirement. On August 1, 2013, the Company received notice from the Nasdaq confirming that as a result of the Company s common stock closing with a bid price of at least $1.00 for at least ten consecutive trading days, the Company had regained compliance with the $1.00 minimum bid price requirement for continued listing on The Nasdaq Capital Market.

Stockholder Rights Plan

On September 20, 2011, the Board of Directors approved the Company s adoption of a Stockholder Rights Plan. Under the Plan, a dividend of one preferred share purchase right (a Right) was declared for each share of common stock of the Company that was outstanding on October 7, 2011. Each Right entitles the holder to purchase from the Company one one-hundredth of a share of Series A Junior Preferred Stock at a purchase price of $7.80, subject to adjustment.

The Rights will trade automatically with the common stock and will not be exercisable until a person or group has become an acquiring person by acquiring 17.5% or more of the Company s outstanding common stock, or a person or group commences, or publicly announces a tender offer that will result in such a person or group owning 17.5% or more of the Company s outstanding common stock. Upon announcement that any person or group has become an acquiring person, each Right will entitle all rightholders (other than the acquiring person) to purchase, for the exercise price of $7.80, a number of shares of the Company s common stock having a market value equal to twice the exercise price. Rightholders would also be entitled to purchase common stock of the acquiring person having a value of twice the exercise price if, after a person had become an acquiring person, the Company were to enter into certain mergers or other transactions. If any person becomes an acquiring person, the Board of Directors may, at its option and subject to certain limitations, exchange one share of common stock for each Right.

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11. Stockholders Equity (continued)

The Rights have certain anti-takeover effects, in that they would cause substantial dilution to a person or group that attempts to acquire a significant interest in the Company on terms not approved by the Board of Directors. In the event that the Board of Directors determines a transaction to be in the best interests of the Company and its stockholders, the Board of Directors may redeem the Rights for $0.001 per share at any time prior to a person or group becoming an acquiring person. The Rights will expire on September 20, 2016, unless earlier redeemed or exchanged.

12. Stock Compensation Plans

The Company issues options, restricted stock, stock appreciation rights and restricted stock units (collectively, the Awards) to employees, directors, consultants and scientific advisors of the Company under the 2006 Stock Incentive Plan (the Plan) (see Note 2). Prior to July 2006, the Company granted options pursuant to written agreements to purchase an aggregate of 2,352,254 shares of common stock. Under the Plan, 3,688,828 shares of the Company s common stock were reserved for issuance. At December 31, 2013, 217,604 of these shares remained available for future issuance under the Plan.

Stock Options

The Company has granted stock options to employees, officers, directors, scientific advisors and consultants generally at exercise prices equal to the market price of the common stock at grant date. Share awards generally vest over a period of 2 to 4 years of continuous service and have contractual terms from 5 to 10 years. Certain awards provide for accelerated vesting if there is a change in control. The Company issues new shares as shares are required to be delivered upon exercise of outstanding stock options.

During the year ended December 31, 2013, options to purchase 50,000 shares of the Company s common stock were exercised with proceeds of $23,500. During the year ended December 31, 2012, options to purchase 195,000 shares of the Company s common stock were exercised on a cashless basis, resulting in the issuance of an aggregate of 40,100 shares of the Company s common stock.

During the years ended December 31, 2013, 2012 and 2011 the Company recorded non-cash stock-based compensation expense related to stock options totaling $175,855, $340,039 and $416,735, respectively.

During the years ended December 31, 2013, 2012 and 2011, the Company granted five-year options to purchase an aggregate of 115,000 shares, 975,000 shares and 625,000 shares, respectively, of the Company s common stock to certain of the Company s officers, employees, directors and consultants.

Stock option activity under the Company s written stock option agreements and the Plan for the year ended December 31, 2013 is summarized as follows:

	Number of Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual	Aggregate Intrinsic Value

			Term (in years)	
Outstanding at beginning of year	3,650,535	$ 0.89		
Granted	115,000	0.60		
Exercised	(50,000)	0.47		
Forfeited or cancelled	(170,333)	1.20		
Expired	(116,296)	3.04		
Outstanding at end of year	3,428,906	$ 0.80	2.26	$ 3,999,388
Exercisable at end of year	3,088,905	$ 0.83	2.06	$ 3,511,386

Other information pertaining to stock option activity during the years ended December 31, 2013, 2012 and 2011 was as follows:

	2013	2012	2011
Weighted average fair value of granted stock options	$ 0.48	$ 0.32	$ 0.79
Total fair value of vested stock options	$ 166,633	$ 348,815	$ 438,139
Total intrinsic value of exercised stock options	$ 17,975	$ 45,050	$

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12. Stock Compensation Plans (continued)

The following table summarizes information about the Company s options outstanding at December 31, 2013:

Range of Exercise Prices	Options Outstanding Number Outstanding	Options Outstanding Weighted Average Remaining Contractual Life (Years)	Options Outstanding Weighted Average Exercise Price	Options Exercisable Number Exercisable	Options Exercisable Weighted Average Remaining Contractual Life (Years)	Options Exercisable Weighted Average Exercise Price
$0.47	1,000,000	3.96	$ 0.47	699,999	3.95	$ 0.47
$0.69	729,610	1.17	$ 0.69	729,610	1.17	$ 0.69
$0.85- $0.90	805,000	0.98	$ 0.90	765,000	0.80	$ 0.90
$1.07- $1.09	860,000	2.48	$ 1.08	860,000	2.48	$ 1.08
$2.55	27,000	0.05	$ 2.55	27,000	0.05	$ 2.55
$6.00	7,296	0.67	$ 6.00	7,296	0.67	$ 6.00
	3,428,906	2.26	$ 0.80	3,088,905	2.06	$ 0.83

As of December 31, 2013, there was approximately $111,000 of unrecognized compensation expense related to non-vested stock option awards granted under the Plan. That cost is expected to be recognized over a weighted average period of approximately 1.45 years.

The Company utilizes the Black-Scholes option-pricing model to determine the fair value of stock options on the date of grant. This model derives the fair value of stock options based on certain assumptions related to the expected stock price volatility, expected option life, risk-free interest rate and dividend yield. Expected volatility is based on reviews of historical volatility of the Company s common stock. The estimated expected option life is based upon estimated employee exercise patterns and considers whether and the extent to which the options are in-the-money. The Company estimates the expected option life for options granted to employees and directors based upon the simplified method. Under this method, the expected life is presumed to be the mid-point between the vesting date and the end of the contractual term. The Company will continue to use the simplified method until it has sufficient historical exercise data to estimate the expected life of the options. The risk-free interest rate assumption is based upon the U.S. Treasury yield curve appropriate for the estimated life of the stock options awards. The expected dividend rate is zero. Stock based compensation expense also includes an estimate, which the Company makes at grant date, of the number of awards that are expected to be forfeited. The Company revises this estimate in subsequent periods if actual forfeitures differ from those estimates.

Assumptions used during the years were as follows:

	Year ended December 31,		
	2013	2012	2011
Risk free interest rate	0.45% to 0.53%	0.28% to 0.66%	0.29% to 1.55%

Expected term	3 years	3 to 5 years	3 to 5 years
Expected volatility	137%	120%	130%
Expected dividend yield	%	%	%
Expected forfeiture rate	%	%	%

13. Benefit Plan

The Company maintains an employee savings plan pursuant to Section 401(k) of the Internal Revenue Code covering all eligible employees. Subject to certain dollar limits, eligible employees may contribute up to 15% of their pre-tax annual compensation to the plan. The Company has elected to make discretionary matching contributions of employee contributions up to 4% of an employee s gross salary. For the years ended December 31, 2013, 2012 and 2011, the Company s matching contributions were approximately $30,000, $28,000 and $34,000, respectively.

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14. Quarterly Financial Information (unaudited)

The following table presents unaudited supplemental quarterly financial information for the years ended December 31, 2013 and 2012:

	Quarter Ended			
	March 31, 2013	June 30, 2013	September 30, 2013	December 31, 2013
Revenues	$	$	$	$
Loss from operations	(1,705,430)	(2,653,529)	(3,245,776)	(2,706,923)
Change in fair value of warrants liability	(45,326)	(498,587)	(2,676,601)	1,330,155
Net loss	(1,744,289)	(3,143,590)	(5,912,059)	(1,354,658)
Loss per share basic and diluted	$ (0.04)	$ (0.08)	$ (0.13)	$ (0.03)

	March 31, 2012	June 30, 2012	September 30, 2012	December 31, 2012
Revenues	$	$	$	$
Loss from operations	(1,364,710)	(1,067,364)	(1,283,713)	(1,505,353)
Change in fair value of warrants liability	274,207	776,919	(1,340,566)	1,419,218
Net loss	(1,089,186)	(289,080)	(2,621,535)	(76,585)
Loss per share basic and diluted	$ (0.04)	$ (0.01)	$ (0.08)	$ (0.00)

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

15. Subsequent Event

Subsequent to year end, on January 31, 2014, the Company filed a shelf Registration Statement on Form S-3 (the 2014 Shelf Registration Statement) with the SEC to sell up to $100 million of common stock. This shelf registration statement (file No. 333-193699) has not yet been declared effective by the SEC. If this registration statement is declared effective, the Company will be able to sell up to $100 million of its common stock. However, if the Company s public float (the market value of the Company s common stock held by non-affiliates) falls below $75 million, the Company will also be subject to a further limitation under which the Company can sell no more than one third of the Company s public float in any 12-month period. Further, the number of shares the Company can sell at any time may be limited to 20% of the Company s common stock under applicable Nasdaq Marketplace Rules.

Subsequent to year end, during February 2014, 12,696 of the 2011 warrants were exercised, with proceeds to the Company of $16,505. In addition, on February 27, 2014, the Company s Board of Directors approved the adoption of the Catalyst Pharmaceutical Partners, Inc. 2014 Stock Incentive Plan (the 2014 Plan). The 2014 Plan will not become effective until it is approved by the Company s stockholders. The Company expects to submit the 2014 Plan to its stockholders for approval at the Company s 2014 Annual Meeting of Stockholders.