

CATALYST PHARMACEUTICAL PARTNERS, INC.

Form 10-K

March 13, 2015

[Table of Contents](#)

**UNITED STATES**

**SECURITIES AND EXCHANGE COMMISSION**

**Washington, D.C. 20549**

**FORM 10-K**

[Mark One]

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

**For the Fiscal Year Ended December 31, 2014**

**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

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

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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  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  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 <input type="checkbox"/>   | Accelerated filer <input checked="" type="checkbox"/> |
| Non-accelerated filer <input type="checkbox"/> (Do not check if a smaller reporting company) | Smaller reporting company <input type="checkbox"/>    |

As of June 30, 2014, 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 \$155,035,298.

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

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date: 81,444,849 shares of common stock, \$0.001 par value per share, were outstanding as of March 10, 2015.

Part III incorporates certain information by reference from the registrant's definitive proxy statement for the 2015 annual meeting of stockholders. The proxy statement with respect to the 2015 annual meeting of stockholders will be filed no later than 120 days after the close of the registrant's fiscal year ended December 31, 2014.

**Table of Contents**

**Table of Contents**

|   | <b>Page</b> |
|---|-------------|
| <b><u>PART I</u></b>  | 1           |
| <u>Item 1. Business</u>   | 3           |
| <u>Item 1A. Risk Factors</u>  | 29          |
| <u>Item 1B. Unresolved Staff Comments</u>   | 45          |
| <u>Item 2. Properties</u>   | 45          |
| <u>Item 3. Legal Proceedings</u>  | 45          |
| <u>Item 4. Mine Safety Disclosure</u>   | 46          |
| <b><u>PART II</u></b>   | 47          |
| <u>Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities</u> | 47          |
| <u>Item 6. Selected Financial Data</u>  | 49          |
| <u>Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations</u>                        | 50          |
| <u>Item 7A. Quantitative and Qualitative Disclosures about Market Risk</u>  | 65          |
| <u>Item 8. Financial Statements and Supplementary Data</u>  | 65          |
| <u>Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure</u>                         | 65          |
| <u>Item 9A. Controls and Procedures</u>   | 65          |
| <u>Item 9B. Other Information</u>   | 66          |
| <b><u>PART III</u></b>  | 67          |
| <u>Item 10. Directors and Executive Officers of the Registrant</u>  | 67          |
| <u>Item 11. Executive Compensation</u>  | 67          |
| <u>Item 12. Security Ownership of Certain Beneficial Owners and Management</u>  | 67          |
| <u>Item 13. Certain Relationships and Related Transactions</u>  | 67          |
| <u>Item 14. Principal Accounting Fees and Services</u>  | 67          |
| <b><u>PART IV</u></b>   | 68          |
| <u>Item 15. Exhibits and Financial Statement Schedules</u>  | 68          |
| <u>Financial Statements</u>   | F-1         |
| <b>EXHIBITS FILED WITH FORM 10-K</b>  |             |
| <u>Ex. 23.1 Consent of Independent Registered Public Accounting Firm</u>  |             |
| <u>EX 31.1 Section 302 Certification of CEO</u>   |             |

[EX 31.2 Section 302 Certification of CFO](#)

[EX 32.1 Section 906 Certification of CEO](#)

[EX 32.2 Section 906 Certification of CFO](#)

**Table of Contents**

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

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

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

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

whether our trials and studies will be successful;

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

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

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

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

**Table of Contents**

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

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

whether we will ever be able to achieve sustained profitability;

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

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

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

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

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

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

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

our expectations regarding licensing, acquisitions or strategic relationships;

changes in the laws and regulations affecting our business;

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

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



whether the settlement that we have reached of the claims brought against us in the class action lawsuit will be approved;

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

our ability to attract and retain skilled employees;

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

the impact of employee or consultant misconduct; and

changes in general economic conditions and interest rates.

Our current plans and objectives are based on assumptions relating to the development of our current drug candidates. Although we believe that our assumptions are reasonable, any of our assumptions could prove inaccurate. In light of the significant uncertainties inherent in the forward-looking statements 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.

## Table of Contents

### **Item 1. Business Overview**

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

#### *Firdapse*

In October 2012, we licensed the North American rights to Firdapse, a proprietary form of amifampridine phosphate, or chemically known as 3,4-diaminopyridine phosphate, from BioMarin Pharmaceutical Inc. (BioMarin). As part of our agreements with BioMarin, we took over the sponsorship of an ongoing Phase 3 clinical trial evaluating Firdapse for the treatment of 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 Syndromes (CMS) and Myasthenia Gravis (MuSK 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 and, in March 2015, we were granted orphan drug designation for Firdapse for the treatment of patients with CMS.

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

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

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

#### Primary endpoints:

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

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

Secondary endpoints:

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

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

**Table of Contents**

Patient tolerance of Firdapse :

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

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

During 2014, we established an expanded access program (EAP) to make Firdapse available to any patients diagnosed with LEMS, Congenital Myasthenic Syndrome (CMS) or Downbeat Nystagmus in the United States who meet the inclusion and exclusion criteria, with Firdapse being provided to patients for free until sometime after NDA approval. We are working with various rare disease advocacy organizations to inform physicians and patients as to the availability of the Firdapse EAP.

In January 2015, we met with the FDA to discuss our anticipated submission of an NDA for Firdapse for the treatment of LEMS. Based on our discussions with the FDA, we believe that our Phase 3 clinical program will provide acceptable support for submission of an NDA for Firdapse for LEMS. We currently expect to submit an NDA for Firdapse during the third quarter of 2015. Although there can be no assurance, we anticipate that under those circumstances we may obtain approval from the FDA of such NDA in the first half of 2016. If approved on this timeline, we would hope to commercially launch Firdapse for the treatment of LEMS shortly after its approval.

In anticipation of the commercialization of Firdapse, we have recently begun to prepare for the marketing of Firdapse in the United States. This has included the appointment of a Chief Commercial Officer, the hiring of a Vice President of Patient Advocacy and Reimbursement and the recent hiring of several rare disease clinical liaisons. We are currently working with several rare disease advocacy organizations to help increase awareness of LEMS and CMS, and to provide education for the physicians who treat these rare diseases and the patients they treat. We anticipate developing a sales force of 15-20 representatives experienced in selling drugs that treat rare diseases. This sales force will market Firdapse to the approximately 900 neuromuscular and oncology specialists who we believe most often diagnose and treat neuromuscular diseases such as LEMS and CMS.

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

Amifampridine is a voltage-gated potassium channel blocker. The Firdapse tablets used in our Phase 3 pivotal trial was the same product approved for marketing in Europe and has 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, has conducted 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 continue to believe that our development program

for amifampridine phosphate is further along in development than this 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 approval requirements, including a demonstration of 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 preclinical and clinical safety and efficacy studies must be completed. This includes studies that evaluate the efficacy of the product, including in most cases two adequate and well-controlled pivotal registration trials that meet the requirements established by the FDA, although a single adequate and well-controlled pivotal registration trial may be sufficient in some cases. It also includes studies that evaluate the drug's long-term toxicity, acute toxicity, reproductive toxicity,

## **Table of Contents**

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. Our development plan for Firdapse has been designed to meet all of these requirements.

We expect to ultimately make a cumulative investment in the development and commercialization of Firdapse of more than \$50 million, consisting of: (i) approximately \$25 million that has been spent or we currently anticipate will be spent to conduct the clinical, non-clinical and safety evaluations, and manufacturing the three exhibit batches, that will be required for us to submit 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 for filing 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) the more than \$15 million that we expect to spend to conduct post-marketing studies of Firdapse and to develop the infrastructure required to commercialize Firdapse, including our efforts to improve diagnosis of the disease through education and to develop patient advocacy programs and patient assistance programs. 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.

We expect to develop a patient assistance program to assist patients who cannot afford their medication.

### ***CPP-115***

We are currently 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 such as complex partial seizures and Tourette Syndrome. CPP-115 has been granted Orphan Drug Designation by the FDA for the treatment of infantile spasms and Orphan Medicinal Product Designation in the European Union, or E.U., for West syndrome (a form of infantile spasms). We are currently evaluating CPP-115 in a Phase 1(b) multi-dose safety and tolerance study. We expect to report the results of this study during the second quarter of 2015.

### ***CPP-109***

An academic investigator proof-of-concept study evaluating the use of CPP-109 (our formulation of vigabatrin, another GABA aminotransferase inhibitor) for the treatment of Tourette Syndrome is currently ongoing and, if the results of this study show evidence of reduced number of tics, we will likely seek to develop CPP-115 (which has the same mechanism of action as CPP-109) for this indication. Although we have provided drug and financial support, we

do not control this study and therefore have no control over the timing of its completion. However, based on currently available information, we expect to have top-line results for this study during the second quarter of 2015.

### **Capital Resources**

Based on our current financial condition and forecasts of available cash, we believe that we have sufficient funds to support our operations through the end of 2016. However, we will require additional funding to support our operations beyond the end of 2016. 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 drug 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.

## Table of Contents

### **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 approval of Firdapse for LEMS. In 2014, we reported positive results from our Phase 3 trial evaluating Firdapse for the treatment of LEMS. We expect to submit an NDA for Firdapse in the 2015 third quarter. Although there can be no assurance, we anticipate that under those circumstances we may obtain approval from the FDA of such NDA in the first half of 2016. If approved on this timeline, we would expect to commercially launch this product for the treatment of LEMS shortly after its approval.

Seek additional orphan drug indications for Firdapse. We believe that Firdapse may also be an effective treatment for other neuromuscular orphan indications such as certain forms of Congenital Myasthenic Syndromes and Myasthenia Gravis (MuSK myasthenia gravis). Subject to the availability of funding, we plan 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 are currently conducting a Phase 1(b) multi-dose safety and tolerance study of CPP-115, and we hope to report the results of this study during the second quarter of 2015. We hope to develop CPP-115 for the treatment of epilepsy (initially infantile spasms) and for the treatment of other selected neurological indications. CPP-115 has been granted Orphan Drug Designation by the FDA for the treatment of infantile spasms and Orphan Medicinal Product Designation in the EU for West's syndrome (a form of infantile spasms), making the drug eligible for the seven-year and ten-year marketing exclusivities available from the FDA and the EU for these indications, respectively, if we are the first pharmaceutical company to obtain approval of an NDA and/or a Marketing Authorization Application, or MAA (the European Union equivalent of an NDA) for CPP-115.

Continue studies of CPP-109 and/or CPP-115 for Tourette Syndrome. We are currently supporting an academic study evaluating the use of CPP-109 in the treatment of Tourette Syndrome. Based upon the results of this study, we may pursue CPP-109, or CPP-115, which has a similar mechanism of action as CPP-109, for the treatment of Tourette Syndrome.

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

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

### **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 contains 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 DAP. We will refer to our drug by its trade name (Firdapse), by the INN/USAN (amifampridine phosphate), or both, throughout this report.

## Table of Contents

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 (now known as the European Academy of Neurology). In December 2009, amifampridine phosphate received marketing approval by the European Commission as Firdapse<sup>®</sup> 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 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 for releasing acetylcholine. Acetylcholine is the neurotransmitter responsible for causing muscles to contract and the failure to release enough of this neurotransmitter results in muscle weakness in LEMS patients. Additionally, LEMS is often associated with an underlying malignancy, most commonly small-cell lung cancer (SCLC), and in some individuals, LEMS is the first symptom of such malignancy.

Based on currently available information, we estimate that there are approximately 3,000 LEMS patients in the United States.

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<sup>®</sup> is a symptomatic treatment and does not alter the underlying autoimmune condition. As a voltage gated potassium blocker, Firdapse<sup>®</sup> 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 and causes muscles to contract and to restore lost muscle strength in LEMS patients.

## **Table of Contents**

### *Congenital Myasthenic Syndromes*

Congenital myasthenic syndromes, or CMS, is a rare neuromuscular disease comprising a spectrum of genetic defects and is characterized by fatigable weakness of skeletal muscles with onset at or shortly after birth or early childhood; in rare cases symptoms may not manifest themselves until later in childhood. The severity and course of the disease are variable, ranging from minor symptoms to progressive disabling weakness; symptoms may be mild, but sudden severe exacerbations of weakness or even sudden episodes of respiratory insufficiency also occur.

Most patients with CMS respond to pharmacologic intervention, including esterase inhibitors, amifampridine (i.e. 3,4-DAP), ephedrine, fluoxetine or quinidine, and albuterol, alone or in combinations. The particular therapy is dictated by the diagnosed CMS type, as drugs beneficial in treating one type of CMS can be detrimental to patients with another type of CMS.

Congenital myasthenic syndrome(s) is rare, estimated at one-tenth that of myasthenia gravis, which in itself is rare. Based on currently available information, we estimate that there are between 1,000 and 1,500 CMS patients in the United States.

### *MuSK Myasthenia Gravis*

The Company is currently considering conducting one or more clinical trials to evaluate whether Firdapse is effective to treat patients with a type of myasthenia gravis caused by antibodies to the muscle-specific receptor tyrosine kinase. We believe that this subtype of patients with myasthenia gravis represents 5-8% of the patients diagnosed with myasthenia gravis. There can be no assurance that Firdapse will be effective in treating this subtype of myasthenia gravis.

### *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.

Under the BioMarin License Agreement, we licensed the North American rights to Firdapse, and, as part of the license, we took over the Phase 3 clinical trial that BioMarin had previously begun in the United States and Europe 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 were further obligated to use diligent efforts to complete the double-blind treatment phase of the Phase 3 trial by October 26, 2014, and BioMarin had the right to terminate the BioMarin License Agreement if such treatment phase has not been completed in such period (unless we were using diligent efforts to pursue the completion of such treatment phase and had spent at least \$5.0 million in connection with the conduct of the Phase III Trial during such period). We completed the Phase 3 treatment phase during September 2014 and believe that we remain in compliance with the license agreement.

Under the BioMarin License Agreement, we have agreed to make the following royalty payments:

(i) royalty payments to BioMarin for seven years from the first commercial sale equal to: (a) 7% of net sales (as defined in the license agreement) in North America in any calendar year for sales up to \$100 million, and (b) 10% of net sales in North America in any calendar year in excess of \$100 million; and



## **Table of Contents**

(ii) royalty payments to a third party licensor of the rights sublicensed to us for seven years from the first commercial sale equal to 7% of net sales (as defined in the license agreement between BioMarin and the third party licensor) in North America in any calendar year.

Under the BioMarin License Agreement, we have also agreed to make certain milestone payments to such third party licensor and to the former stockholders of Huxley Pharmaceuticals, Inc. (Huxley) that BioMarin is obligated to make. With respect to Firdapse<sup>®</sup>, the milestones aggregate approximately \$2.6 million upon filing by the FDA of an NDA for Firdapse<sup>®</sup> for the treatment of LEMS, and approximately \$7.2 million upon the unconditional approval by the FDA of an NDA for Firdapse<sup>®</sup> for the treatment of LEMS.

Firdapse<sup>®</sup> was approved by European Medicines Agency for the treatment of LEMS in December 2009, and BioMarin sells the product in the EU. BioMarin is also currently performing or will in the future perform certain post-marketing studies of Firdapse<sup>®</sup> that they are required to conduct to support their continued approval of Firdapse<sup>®</sup> 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 reproductive toxicity studies that have been completed and are required for approval of Firdapse<sup>®</sup> by the FDA.

On April 15, 2014, effective as of April 8, 2014, we and BioMarin entered into Amendment No. 1 to the License Agreement, amending in certain respects the BioMarin License Agreement, dated October 26, 2012. The amendment related to purchases of additional product by us from BioMarin, the sharing of data between the parties with respect to clinical trials and studies undertaken by each party, and the payment terms for certain joint studies.

### *The Phase 3 clinical trial*

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

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

#### Primary endpoints:

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

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

#### Secondary endpoints:

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

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

Patient tolerance of Firdapse :

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

## **Table of Contents**

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

Further details regarding the trial and its design can be found on [www.clinicaltrials.gov](http://www.clinicaltrials.gov) (NCT01377922).

The results of the Phase 3 trial were reported in October 2014 at the 139<sup>th</sup> Annual Meeting of the American Neurological Association (ANA) and at a symposium sponsored by us at the annual meeting of the American Association of Neuromuscular and Electrodiagnostic Medicine (AANEM).

### *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.

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 Pre-NDA Meeting with the FDA on Firdapse*

In January 2015, we met with the FDA to discuss our anticipated submission of an NDA for Firdapse for the treatment of LEMS. Based on our discussions with the FDA and our review of the minutes of the meeting that we have received from the FDA, we believe that our Phase 3 clinical program will provide acceptable support for submission of an NDA for Firdapse for LEMS. Further, at the meeting, we discussed potential paths forward for approval of certain types of CMS as an additional indication for Firdapse .

### *Orphan Drug Designation*

Amifampridine phosphate for LEMS has been granted Orphan Drug Designation by the FDA for the treatment of both LEMS and CMS, making the drug eligible to be granted seven-year marketing exclusivity for these indications 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 and/or CMS.

### *Pre-commercialization efforts*

In anticipation of the commercial launch of Firdapse , we have recently begun to prepare for the marketing of Firdapse in the United States. This has included the appointment of a Chief Commercial Officer, the hiring of a Vice President of Patient Advocacy and Reimbursement and the recent hiring of several rare disease clinical liaisons. We are currently working with several rare disease advocacy organizations to help increase awareness of LEMS and CMS and to provide education for the physicians who treat these rare diseases and the patients they treat. We anticipate developing a sales force of 15-20 representatives experienced in selling drugs that treat rare diseases. This sales force will market Firdapse to the approximately 900 neuromuscular and oncology specialists who we believe most often diagnose and treat neuromuscular diseases such as LEMS and CMS.



Further, as part of our commitment to the patient community, we expect to hire several account managers who will work with our rare disease clinical science liaisons to educate payors and facilitate coverage and we expect to have a comprehensive set of patient financial support services, including a patient assistance program. We also expect to distribute our product through a limited network of specialty pharmacies with a single dedicated call center responsible for interfacing with patients, physicians and payors.

## **Table of Contents**

We have not yet established the pricing for our product. However, the independent market research that we have obtained to date indicates that we will be able to obtain typical orphan disease pricing for our product and that our product will be widely reimbursed by private and public payors. There can be no assurance, however, as to the pricing of our product that we may be able to obtain or as to whether payors will object to paying for our product.

### *Expanded Access Program*

During 2014, we established an expanded access program (EAP) to make Firdapse available to all patients diagnosed with LEMS, Congenital Myasthenic Syndromes (CMS) or Downbeat Nystagmus in the United States who meet the inclusion and exclusion criteria, with Firdapse being provided to patients for free until sometime after NDA approval. We are working with various rare disease advocacy organizations to inform physicians and patients as to the availability of the Firdapse EAP.

### *Third-Party Reimbursement*

Sales of pharmaceutical products depend in significant part on the availability of coverage and adequate reimbursement by third party payors, such as state and federal governments, including Medicare and Medicaid, managed care providers and private insurance plans. Decisions regarding the extent of coverage and the amount of reimbursement to be provided for Firdapse is expected to be made on a plan-by-plan, and in some cases, a patient-by-patient basis. Particularly given the rarity of LEMS, we anticipate that securing coverage and appropriate reimbursement from third-party payors will require targeted education. To that end, we expect to hire a dedicated group of field-based payer account managers and reimbursement experts focused on ensuring that clinically qualified patients have affordable access to our product.

### *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 following an appeal to the Patent Trial and Appeal Board. The application was refiled with new claims. The new claims were the subject of an office action in which the claims were rejected. A response to the rejection was filed and is awaiting further examination. There can be no assurance that this patent will be issued. The second patent claims methods of administering Firdapse. No examination has occurred on this patent application and none is expected until 2016. 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-year 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 shipment. The application was refiled and a Statement of Use submitted and accepted by the Trademark Office. We expect that FIRDAPSE should become registered in due course during 2015. In January 2014, the FDA provisionally approved Firdapse as a proprietary name for amifampridine phosphate tablets. This provisional approval by the FDA does not stop the agency from rejecting the name FIRDAPSE at a later date.



## **Table of Contents**

### **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 59 patents, is the inventor of pregabalin, also known as Lyrica<sup>®</sup>, 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 pre-clinical studies of CPP-115 to date support our expectations. However, based upon our pre-clinical studies of CPP-115 to date, we expect that there will be a significant reduction, and possibly elimination, of visual field defects (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<sup>®</sup>. 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 VFDs) compared with Sabril<sup>®</sup>.

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.

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.

## Table of Contents

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 and Sanofi-Aventis and their predecessors and licensees under the brand names Sabril®, Sabrillex® 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 many 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 Inc. (Lundbeck). 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.

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 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 dependent pharmacokinetics.

## **Table of Contents**

### *Phase 1(b) Clinical Trial of CPP-115*

We are currently evaluating CPP-115 in a Phase 1(b) multiple ascending dose study. The Phase 1(b) study is a randomized, double-blind, placebo-controlled, safety, tolerability and pharmacokinetic study of multiple oral doses of CPP-115 in healthy volunteers. The primary objective is to evaluate the safety and tolerability of multiple oral doses of CPP-115. Secondary objectives are 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. We expect to report the top-line results from this study during the second quarter of 2015. We also expect to undertake, subject to the availability of funding, 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.

### *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 drug candidates that we believe might further the understanding or increase the value of our drug 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 in the January 2014 issue of the journal, *Epilepsia*. The study was authored by Stephen W. Briggs, Tomonori Ono, MD, PhD, Solomon L. Moshe, MD and Aristeia S. Galanopoulou, MD, PhD of the Saul R. Korey Department of Neurology, Dominick P. Purpura Department of Neuroscience, Laboratory of 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 had been discovered and patented by Northwestern. Under the terms of the license agreement, Northwestern granted us an exclusive worldwide license to United States 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.

## **Table of Contents**

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 these 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 drug 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, 2014, we have paid Northwestern upfront payments, milestone fees and maintenance and patent fees aggregating \$251,590, 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.

In November 2014, the U.S. Patent & Trademark Office (US PTO) issued a Notice of Allowance on the method of use patent for CPP-115 for neurological and psychological uses. This patent will expire in 2032, subject to potential extensions allowed under the patent term restoration act. Patents for the same coverage remain pending in the European Patent Office, Japan and Canada. 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 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 patent applications in the United States, European and Canadian Patent Offices for the use of GABA aminotransferase inhibitors, including CPP-109 and CPP-115, in the treatment of Tourette Syndrome. We also have entered into a license agreement with NYU and the Feinstein Institute granting us worldwide rights with respect to such patent. No office actions have been received to date and none are expected until late 2016.

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®).

An academic investigator proof-of-concept study evaluating the use of CPP-109 (our formulation of vigabatrin, another GABA aminotransferase inhibitor) for the treatment of Tourette Syndrome is currently ongoing and, if the results of this 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. Although we have provided drug and financial support, we do not control this study and therefore have no control over the timing of its completion. However, based on currently available information, we expect to have top-line results for this study before the end of the second quarter of 2015.

## **Table of Contents**

### **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.

### **Manufacturing and Supply**

#### *Firdapse*

We have entered into agreements with a supplier of the active pharmaceutical ingredient (API) contained in Firdapse for future requirements and we have contracted with a third-party contract manufacturer who we expect will manufacture Firdapse tablets for us if Firdapse is approved for commercialization.

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

Since we 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 planned 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.

## **Table of Contents**

### **Sales and Marketing**

We have not obtained regulatory approval for any of our drug candidates and thus we have only recently begun to establish a commercial organization. We believe that, if approved by the FDA, it will be possible to commercialize Firdapse with a focused 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. In furtherance of those objectives, in January 2015 we appointed a Chief Commercial Officer to help plan our commercialization activities for Firdapse and to help us establish the required commercial infrastructure to market Firdapse. 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.

### **Competition**

The pharmaceutical industry is intensely competitive, and any product candidate developed or licensed by us would likely compete with currently marketed and potentially new drugs and therapies even though they are not indicated for these conditions. 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.

#### *Firdapse for LEMS*

LEMS is currently treated with drugs and therapies including steroids, azathioprine, other immunosuppressants and intravenous immunoglobulin, which work by suppressing the immune system, and pyridostigmine. Plasma exchange has also been used in an attempt to remove antibodies from the body. Further, one other product, guanidine HCl tablets, was approved many years ago (during a period when drugs were not required to be reviewed by the FDA for both safety and effectiveness) for use in the treatment of LEMS. However, this drug has significant side effects and is not currently viewed as an effective treatment for LEMS. 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 its 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 for any indication. Further, we are aware that Jacobus Pharmaceutical has been making 3,4-DAP available to LEMS patients under compassionate use Investigational New Drug applications (INDs) for a number of years and we believe that approximately 200 LEMS patients receive the drug. 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 (i.e. 3,4-DAP), has been available from compounding pharmacies for many years and may remain available even if we are able to obtain FDA approval of Firdapse . Compounded amifampridine, if it is available, 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

## **Table of Contents**

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 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 aggressively 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, since 3,4-DAP does not meet the requirements codified in Section 503A described above, 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 FD&C Act.

We intend to take all available steps to try to enforce our marketing proprietary rights if we are the first company to obtain an approval for this product. We cannot determine with certainty what impact these factors will have on the market for our product. However, while there can be no assurance, we expect that despite these factors, we will be able to successfully market our product.

### *CPP-115 for Epilepsy*

The market for epilepsy treatments is highly competitive. Large pharmaceutical companies, including Pfizer (Neurontin<sup>®</sup>, Lyrica<sup>®</sup>, Dilantin<sup>®</sup>, Zarontin<sup>®</sup>), J&J (Topamax<sup>®</sup>), UCB (Keppra<sup>®</sup>), Abbott (Depakote<sup>®</sup>), GSK (Lamictal<sup>®</sup>), Roche (Klonopin<sup>®</sup>), and Novartis (Trileptal<sup>®</sup>) 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 drug candidates;

the efficacy, safety and reliability of our drug candidates;



the timing and scope of regulatory approvals;

product acceptance by physicians and other health care providers;

protection of our proprietary rights and the level of generic competition;

the speed at which we develop drug candidates;

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

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

## **Table of Contents**

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.

## **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 nonclinical and clinical trial sites that generated the data in support of the NDA;  
and

FDA review and approval of the NDA.

## **Table of Contents**

### *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 nonclinical 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 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 investigational 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.

## **Table of Contents**

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.

### *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 \$2,335,000), 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 \$114,000 per product and \$569,000 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.

## Table of Contents

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.

### *The Hatch-Waxman Amendments*

#### Orange Book Listing

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent whose claims cover the applicant's product. Upon approval of a drug, each of the patents listed in the application for the drug is then published in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential generic competitors in support of approval of an abbreviated new drug application, or ANDA. An ANDA provides for marketing of a drug product that has the same active ingredients in the same strengths and dosage form as the listed drug and has been shown through bioequivalence testing to be therapeutically equivalent to the listed drug. Other than the requirement for bioequivalence testing, ANDA applicants are not required to conduct, or submit results of, pre-clinical or clinical tests to prove the safety or effectiveness of their drug product. Drugs approved in this way are commonly referred to as generic equivalents to the listed drug, and can often be substituted by pharmacists under prescriptions written for the original listed drug.

The ANDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA's Orange Book. Specifically, the applicant must certify that: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or (iv) the listed patent is invalid or will not be infringed by the new product. The ANDA applicant may also elect to submit a section viii statement certifying that its proposed ANDA label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent. If the applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

A certification that the new product will not infringe the already approved product's listed patents, or that such patents are invalid, is called a Paragraph IV certification. If the ANDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days of the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit, or a decision in the infringement case that is favorable to the ANDA applicant.

The ANDA application also will not be approved until any applicable non-patent exclusivity listed in the Orange Book for the referenced product has expired.





## Table of Contents

### Exclusivity

Upon NDA approval of a new chemical entity or NCE, which is a drug that contains no active moiety that has been approved by FDA in any other NDA, that drug receives five years of marketing exclusivity during which FDA cannot receive any ANDA seeking approval of a generic version of that drug. A drug may obtain a three-year period of exclusivity for a particular condition of approval, or change to a marketed product, such as a new formulation for the previously approved product, if one or more new clinical studies (other than bioavailability or bioequivalence studies) was essential to the approval of the application and was conducted/sponsored by the applicant. During this period of exclusivity, FDA cannot approve an ANDA for a generic drug that includes the change.

An ANDA may be submitted one year before NCE exclusivity expires if a Paragraph IV certification is filed. If there is no listed patent in the Orange Book, there may not be a Paragraph IV certification, and, thus, no ANDA may be filed before the expiration of the exclusivity period.

### *Section 505(b)(2) New Drug Applications*

Most drug products obtain FDA marketing approval pursuant to an NDA or an ANDA. A third alternative is a special type of NDA, commonly referred to as a Section 505(b)(2), or 505(b)(2), NDA, which enables the applicant to rely, in part, on FDA's previous approval of a similar product, or published literature, in support of its application.

505(b)(2) NDAs often provide an alternate path to FDA approval for new or improved formulations or new uses of previously approved products. Section 505(b)(2) permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by, or for, the applicant and for which the applicant has not obtained a right of reference. If the Section 505(b)(2) applicant can establish that reliance on FDA's previous approval is scientifically appropriate, it may eliminate the need to conduct certain preclinical or clinical studies of the new product. The FDA may also require companies to perform additional studies or measurements to support the change from the approved product. The FDA may then approve the new product candidate for all, or some, of the label indications for which the referenced product has been approved, as well as for any new indication sought by the Section 505(b)(2) applicant.

To the extent that the Section 505(b)(2) applicant is relying on studies conducted for an already approved product, the applicant is required to certify to the FDA concerning any patents listed for the approved product in the Orange Book to the same extent that an ANDA applicant would. A Section 505(b)(2) NDA may be eligible for three years of marketing exclusivity to the same extent that a Section 505(b)(1) NDA is.

### *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.

## **Table of Contents**

### *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 drug 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;

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 Exclusivity and Pediatric Exclusivity 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.

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**Table of Contents**

Pediatric exclusivity is another type of non-patent exclusivity in the U.S. and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the five-year and three-year non-patent and seven-year orphan exclusivities. This six-month exclusivity may be granted if an NDA or Biologic License Application sponsor submits pediatric data that fairly responds to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied. If the FDA determines that information relating to the use of the new drug in the pediatric population may produce health benefits in the population, the clinical study is deemed to fairly respond to the FDA's request and the reports of FDA-requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or patent protection covering the product are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot approve another application relying on the NDA sponsor's data.

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 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 (see below for more details on fast track designation), 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 (FDAISA) 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 nonclinical 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.

## **Table of Contents**

### *Fast Track Designation and Accelerated Approval*

FDA is required to facilitate the development, and expedite the review, of drugs that are intended for the treatment of a serious or life-threatening disease or condition for which there is no effective treatment and which demonstrate the potential to address unmet medical needs for the condition. Under the fast track program, the sponsor of a new drug candidate may request that FDA designate the drug candidate for a specific indication as a fast track drug concurrent with, or after, the filing of the IND for the drug candidate. FDA must determine if the drug candidate qualifies for fast track designation within 60 days of receipt of the sponsor's request.

Under the fast track program and FDA's accelerated approval regulations, FDA may approve a drug for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments.

In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions, or survives. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A drug candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, will allow FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by FDA.

In addition to other benefits such as the ability to use surrogate endpoints and engage in more frequent interactions with FDA, FDA may initiate review of sections of a fast track drug's NDA before the application is complete. This rolling review is available if the applicant provides, and FDA approves, a schedule for the submission of the remaining information and the applicant pays applicable user fees. However, FDA's time period goal for reviewing an application does not begin until the last section of the NDA is submitted. Additionally, the fast track designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

### *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.





## **Table of Contents**

### *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 10, 2015 we had twelve 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 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 has been 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

## **Table of Contents**

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 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 statistical consultant to many pharmaceutical companies (including us) 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 Fellow of the National Academy of Inventors (2014), Fellow of the American Academy of Arts & Sciences (2014), iCON Innovator Award of the iBIO Institute (2014), Northwestern University Trustee Medal for Faculty Innovation and Entrepreneurship (2014), Medicinal Chemistry Prize of the Israel Chemistry Society (2014), Fellow of the Royal Society of Chemistry (UK, 2013), Centenary Prize of the Royal Society of Chemistry (2013), Bristol-Myers Squibb-Edward E. Smisman Award of the American Chemical Society (2013), Sato Memorial International Award of the Pharmaceutical Society of Japan (2012), Fellow of the American Chemical Society (2011), E.B. Hershberg Award for Important Discoveries in Medicinally Active Substances from the American Chemical Society (2011), Perkin Medal from the Society of Chemical Industry (2009), Medicinal Chemistry Hall of Fame of the American Chemical Society (2009). Dr. Silverman holds 59 patents, has published over 335 peer-reviewed articles and has written five books over his 38-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 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](http://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.



## **Table of Contents**

### **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 \$15.5 million and \$12.2 million for the years ended December 31, 2014 and December 31, 2013, respectively. We may never obtain approval of an NDA for any of our drug candidates and we may never achieve profitability.

#### ***Our business will require additional capital.***

Our business will require additional capital to meet our product development objectives. Based on currently available information, we estimate that we have sufficient working capital to support our operations through the end of 2016. The expectations described above are based on current information available to us. If the cost of our ongoing activities are greater than we expect, our assumptions may not prove to be accurate. There can be no assurance as to the exact amount of the funding we will require or as to whether any such required funding will be available to us when it is required.

We plan to raise additional funds in the future through public or private equity offerings, debt financings, capital lease transactions, corporate collaborations, governmental research grants or cost sharing arrangements with appropriate agencies that operate under the umbrella of the National Institutes of Health and/or other means. However, there is no assurance that any such grants will be made available, and if available, that we will qualify to receive any such grants. We may also seek to raise additional capital to fund additional product development efforts, even if we have sufficient funds for our planned operations.

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.

## **Table of Contents**

***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 (according to the information about this study on [clinicaltrials.gov](http://clinicaltrials.gov), enrollment in this trial has been discontinued as of April 2014). While there can be no assurance, we believe that Firdapse is further along in development and as a result we expect that we will be in a position to obtain the first approval of an NDA for 3,4-DAP. Under the Orphan Drug Act of 1983, the first pharmaceutical product to obtain approval for an indication receives the orphan exclusivity under the statute. If another pharmaceutical company is able to receive approval of an NDA for its formulation of amifampridine for the treatment of LEMS before we are able to receive approval of Firdapse 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 another 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 submit an NDA for CPP-115, if our future clinical trials of this product are successful. Further, our ability to develop CPP-115 will be dependent on our having the resources to conduct the studies and trials that would be required. There can be no assurance that we will ever submit an NDA for CPP-115 or commercialize CPP-115.

***Our business is subject to substantial competition.***

The biotechnology and pharmaceutical industries are highly competitive. Many of our competitors have substantially greater financial and other resources, larger research and development staffs and more experience developing products, obtaining FDA and other regulatory approvals of products and manufacturing and marketing products than we have. We compete against pharmaceutical companies that are developing or currently marketing therapies that will compete with our drug candidates. In addition, we compete against biotechnology companies, universities, government agencies, and other research institutions in the development of pharmaceutical products. While we believe that our drug candidates will offer advantages over many of the currently available competing therapies, our business could be negatively impacted if our competitors' present or future offerings are more effective, safer or less expensive than ours, or more readily accepted by regulators, healthcare providers or third-party payors. Further, if we are permitted to commence commercial sales of our drug candidates, we may also compete with respect to manufacturing efficiency and marketing capabilities.

For example, amifampridine, the active ingredient in Firdapse, 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 can be expected to be substantially less expensive than Firdapse. The FDA Pharmacy Compounding Advisory Committee, however, has previously issued a list of drugs which should not be compounded, and amifampridine was included on that list. In addition, drugs that are not approved by FDA for the treatment of LEMS, such as a related aminopyridine drug, dalfampridine (Ampyra®), may nonetheless be prescribed by physicians for the treatment of LEMS.

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

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



Our business exposes us to potential liability risks that may arise from the clinical testing, manufacture, and/or sale of our pharmaceutical products. Patients have received substantial damage awards in some jurisdictions against pharmaceutical companies based on claims for injuries allegedly caused by the use of pharmaceutical products used in clinical trials or after FDA approval. Liability claims may be expensive to defend and may result in large judgments against us. We currently carry liability insurance with an aggregate annual coverage limit of \$15,000,000 per claim and \$15,000,000 in the aggregate, with a deductible of \$10,000 per occurrence. Our insurance may not

## **Table of Contents**

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

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

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

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

We are highly dependent on our officers, on our Board of Directors and on our scientific advisors. The loss of the services of any of these individuals could significantly impede the achievement of our scientific and business objectives. Other than an employment agreement with Patrick J. McEnany, our Chairman, President and Chief Executive Officer with respect to his services, and the consulting agreement 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 drug 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 drug development efforts may fail.***

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

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

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

competitors may market equivalent or superior products.

## **Table of Contents**

As a result, our drug development activities may not result in any safe, effective and commercially viable products, and we may not be able to commercialize our products successfully. For example, for several years, we evaluated CPP-109 (our formulation of vigabatrin) for the treatment of cocaine addiction. However, CPP-109 failed to meet the primary and two key secondary endpoints in a Phase 2(b) trial for cocaine addiction, and we are no longer pursuing the evaluation of CPP-109 for addiction. Further, our lead compound, Firdapse , is for a very rare condition for which there is no FDA-approved treatment. As such, the clinical development plan we pursued after consulting with FDA including the clinical endpoints, protocol design, and statistical analysis plan, may not allow the FDA to ultimately conclude that our Phase 3 trial of Firdapse is adequate to establish the clinical benefit of the drug. In addition, FDA has indicated that additional data from published studies, and data from a patient registry, would be useful in establishing the safety of Firdapse , but we may not be able to obtain that data in a form that is satisfactory to the FDA. Our failure to develop safe, effective, and/or commercially viable products would have a material adverse effect on our business, prospects, results of operations and financial condition.

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

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

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

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

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

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

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

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

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

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

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

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

## **Table of Contents**

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

Although we rely on third parties to manage the data from our studies and trials, we are responsible for confirming that each of our studies and trials is conducted in accordance with its general investigational plan and protocol. Moreover, the FDA and foreign regulatory agencies will require us to comply with applicable regulations and standards, including Good Laboratory Practice (GLP) and Good Clinical Practice (GCP), for conducting, recording and reporting the results of such studies and trials to assure that the data and the results are credible and accurate and that the human study and trial participants are adequately protected. Our reliance on third parties does not relieve us of these obligations and requirements, and we may fail to obtain regulatory approval for our drug candidates if these requirements are not met.

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

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

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

Any manufacturing problem, natural disaster affecting manufacturing facilities, or the loss of a contract manufacturer could be disruptive to our operations and result in lost sales. Additionally, we will be reliant on third parties to supply the raw materials needed to manufacture our potential products. Any reliance on suppliers may involve several risks, including a potential inability to obtain critical materials and reduced control over production costs, delivery schedules, reliability and quality. Any unanticipated disruption to future contract manufacture caused by problems at suppliers could delay shipment of products, increase our cost of goods sold and result in lost sales. If our suppliers were to be unable to supply us with adequate supply of our drug candidates, it could have a material adverse effect on our ability to commercialize our drug candidates.

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

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

increase the manufacturing capacity for any of our drug candidates, whether in collaboration with third-party manufacturers or on our own, in a timely or cost-effective manner or at all. If a contract manufacturer makes improvements in the manufacturing process for our drug candidates, we may not own, or may have to share, the intellectual property rights to those improvements. Significant scale-up of manufacturing may require additional

## **Table of Contents**

validation studies, which are costly and which the FDA must review and approve. In addition, quality issues may arise during those scale-up activities because of the inherent properties of a drug candidate itself or of a drug candidate in combination with other components added during the manufacturing and packaging process, or during shipping and storage of the finished product or active pharmaceutical ingredients. If we are unable to successfully scale-up manufacture of any of our drug candidates in sufficient quality and quantity, the development of that drug candidate and regulatory approval or commercial launch for any resulting drug products may be delayed or there may be a shortage in supply, which could significantly harm our business.

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

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

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

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

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

***Because the target patient population for Firdapse and our other drug candidates are small, we must achieve significant market share and obtain relatively high per-patient prices for our products to achieve meaningful gross***



*margins.*

Firdapse and our clinical development of our other orphan drug candidates target diseases with small patient populations. A key component of the successful commercialization of a drug product for these indications includes identification of patients and a targeted prescriber base for the drug product. Due to small patient populations, we believe that we would need to have significant market penetration to achieve meaningful revenues and identifying

## **Table of Contents**

patients and targeting the prescriber base are key to achieving significant market penetration. In addition, the per-patient prices at which we anticipate we may sell Firdapse will need to be relatively high in order for us to generate an appropriate return for the investment in these product development programs and achieve meaningful gross margins. There can be no assurance that we will be successful in achieving a sufficient degree of market penetration and/or obtaining or maintaining high per-patient prices for Firdapse for diseases with small patient populations. Further, even if we obtain significant market share for Firdapse, if approved, because the potential target populations are very small, we may never achieve profitability despite obtaining such significant market share. Furthermore, because the potential target populations are very small, even if we do obtain significant market share for Firdapse, we may never achieve profitability. Additionally, patients who discontinue therapy or do not fill prescriptions are not easily replaced by new patients, given the limited patient population.

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

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

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

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

## **Risks Related to Government Regulation**

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

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

## **Table of Contents**

manufacture our drug candidate are in compliance with Current Good Manufacturing Processes (cGMP). We will also have to meet similar regulations in any foreign country where we may seek to commercialize our drug candidates. In general, these requirements mandate that manufacturers follow elaborate design, testing, control, documentation and other quality assurance procedures throughout the entire manufacturing process. The process of obtaining regulatory approvals typically takes several years and requires the expenditure of substantial capital and other resources. Despite the time, expense and resources invested by us in the approval process, we may not be able to demonstrate that our drug candidates are safe and effective, in which event we would not receive the regulatory approvals required to market them.

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

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

***If our pre-clinical studies or our clinical studies and trials are unsuccessful or significantly delayed, our ability to commercialize our products will be impaired.***

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

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

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

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

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

We may encounter difficulties in our current and future clinical studies and trials recruiting patients, particularly since the conditions we are studying are rare conditions. We compete for study and trial subjects with others conducting clinical trials testing other treatments for the indications we are studying for our drug candidates. Further, unrelated third parties and investigators in the academic community have expressed interest in testing our drug candidates. If these third-party tests are unsuccessful, or if they show significant health risk to the test subjects, our development efforts may also be adversely affected.

**Table of Contents**

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

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

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

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

reliance on the continued financial viability of the third parties;

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

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

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

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

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

***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.

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**Table of Contents**

***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 agency, the FDA could withdraw our approvals. Moreover, the Department of Justice can bring civil or criminal actions against companies that promote drugs or biologics for unapproved uses, based on the False Claims Act and other federal laws governing reimbursement for such products under the Medicare, Medicaid and other federally supported healthcare programs. Monetary penalties in such cases have often been substantial, and civil penalties can include costly mandatory compliance programs and exclusion from federal healthcare programs.

***Enacted and future legislation may increase the difficulty and cost for us to commercialize Firdapse or any other drug candidate we develop and affect the prices we may obtain.***

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



The Medicare Prescription Drug Improvement and Modernization Act of 2003, or MMA, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for outpatient drug purchases by those covered by Medicare under a new Part D and introduced a new reimbursement methodology based on average sales prices for Medicare Part B physician-administered drugs. In addition, this legislation authorized Medicare Part D prescription drug plans to use formularies whereby they can limit the number of drugs that will be covered in any therapeutic class. As a result of this legislation and the expansion of federal coverage of drug products, there is additional pressure to contain and reduce costs. While the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates, and any reduction in reimbursement that results from the MMA may result in a similar reduction in payments from private payors. These cost reduction initiatives and other provisions of the

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**Table of Contents**

MMA could decrease the coverage and reimbursement that we receive for any approved products, and could seriously harm our business. Manufacturers' contributions to this area, including donut hole coverage (as described below) or potential excise taxes, are increasing and are subject to additional changes in the future.

In 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (together, the Health Care Reform Law), a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The Health Care Reform Law, among other things, revised the definition of AMP for reporting purposes, which could increase the amount of Medicaid drug rebates to states and extended the rebate program to beneficiaries enrolled in Medicaid managed care organizations. The Health Care Reform Law also imposed a significant annual fee on companies that manufacture or import branded prescription drug products and established an annual non-deductible fee on entities that sell branded prescription drugs or biologics to specified government programs in the U.S. The Health Care Reform Law also expanded the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance and included a 50% discount on brand name drugs for Medicare Part D participants in the coverage gap, or donut hole. The Health Care Reform Law includes a provision to increase the Medicaid rebate for line extensions or reformulated drugs, which depending on how this provision is implemented could substantially increase our Medicaid rebate rate (in effect limiting reimbursement for these patients). These and other new provisions are likely to continue the pressure on pharmaceutical pricing, may require us to modify our business practices with healthcare practitioners, and may also increase our regulatory burdens and operating costs.

Legislative and regulatory proposals also have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may subject us to more stringent product labeling and post-marketing testing and other requirements. Delays in feedback from the FDA may affect our ability to quickly update or adjust our label in the interest of patient adherence and tolerability. We cannot predict whether other legislative changes will be adopted or how such changes would affect the pharmaceutical industry generally and specifically the commercialization of Firdapse.

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

In the U.S., orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. The company that first obtains FDA approval for a designated orphan drug for a given rare disease receives marketing exclusivity for use of that drug for the stated condition for a period of seven years, with an additional six months if for a pediatric indication. Orphan drug exclusive marketing rights may be lost if the FDA later determines that the request for designation was materially defective, a subsequent product is deemed clinically superior, or if the manufacturer is unable to deliver sufficient quantity of the drug.

In the EU, the EMA's Committee for Orphan Medicinal Products, or COMP, grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the EU Community and for which no satisfactory method of diagnosis, prevention, or treatment has been authorized (or the product would be a significant benefit to those affected). Additionally, designation is granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when,

without incentives, it is unlikely that sales of the drug in the EU would be sufficient to justify the necessary investment in developing the medicinal product. An EU orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and 10 years of market exclusivity is granted following medicinal product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Orphan drug designation must be requested before submitting an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

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**Table of Contents**

Because the extent and scope of patent protection for some of our drug products may be particularly limited, orphan drug designation is especially important for our products that are eligible for orphan drug designation. For eligible drugs, we plan to rely on the orphan exclusivity period to maintain a competitive position. However, if we do not obtain orphan drug exclusivity for our drug candidates or we cannot maintain orphan exclusivity for our drug candidates, our competitors may then sell the same drug to treat the same condition and our revenues will be reduced. Also, without strong patent protection, competitors may sell a generic version upon the expiration of orphan exclusivity, if our patent position is not upheld.

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

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

Under the Prescription Drug User Fee Act, the FDA has a goal of responding to NDAs for new molecular entities within 10 months of the date that the FDA files the NDA for standard review, but this timeframe is also often extended. We have in the past and we may in the future, seek approval of our drug candidates under programs designed to accelerate the FDA's review and approval of NDAs. For example, the Food and Drug Administration Safety and Innovation Act, or FDASIA, which was enacted and signed into law in 2012, established a new category of drugs referred to as breakthrough therapies, which are defined as drugs intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. In our case, Firdapse has been granted breakthrough therapy designation for the treatment of LEMS. In the future, we may request breakthrough designation or fast track designation from the FDA for our other drug candidates or for treatment of other diseases, but we cannot assure that we will obtain such designations. Moreover, even if we obtain breakthrough designation or fast track designation from the FDA, the designations do not guarantee FDA approval of our NDA, that the development program or review timeline will ultimately be shorter than if we had not obtained the designations, or that the FDA will not request additional information, including requesting additional clinical studies (although potentially a post-marketing requirement), during its review. Any request for additional information or clinical data could delay the FDA's timely review of our NDA.

**Risks Related to Our Intellectual Property**

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

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

the time of such termination. If we fail to retain our rights under the license agreement, we would not be able to commercialize Firdapse , and our business, results of operations, financial condition and prospects would be materially adversely affected.

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

## **Table of Contents**

derivatives of vigabatrin that are unrelated to CPP-115. These rights are subject to the right of Northwestern, under limited circumstances, to practice the covered inventions for or on its own behalf for research. We may lose our rights to these patents and patent applications if we breach our obligations under the license agreement, including, without limitation, our financial obligations, including milestone payments, to Northwestern. If we violate or fail to perform any term or covenant of the license agreement, Northwestern may terminate the license agreement upon satisfaction of any applicable notice requirements and expiration of any applicable cure periods. Additionally, any termination of the license agreement, whether by us or by Northwestern, will not relieve us of our obligation to pay any license fees owing at the time of such termination. If we fail to retain our rights under the license agreement, we would not be able to commercialize CPP-115, and our business, results of operations, financial condition and prospects would be materially adversely affected.

If we obtain approval to market Firdapse or CPP-115, our commercial success will depend in large part on our ability to use patents, especially those licensed to us by BioMarin and Northwestern, respectively, to exclude others from competing with us. The patent position of emerging pharmaceutical companies like us can be highly uncertain and involve complex legal and technical issues. Until our licensed patents are interpreted by a court, either because we have sought to enforce them against a competitor or because a competitor has preemptively challenged them, we will not know the breadth of protection that they will afford us. Our patents may not contain claims sufficiently broad to prevent others from practicing our technologies or marketing competing products. Third parties may intentionally attempt to design around our patents or design around our patents so as to compete with us without infringing our patents. Moreover, the issuance of a patent is not conclusive as to its validity or enforceability, and so our patents may be invalidated or rendered unenforceable if challenged by others.

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

***Our success will depend significantly on our ability to operate without infringing the patents and other proprietary rights of third parties.***

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

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

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

a court may prohibit us from selling or licensing our product without a license from the patent holder, which may not be available on commercially acceptable terms or at all, or which may require us to pay substantial royalties or grant cross-licenses to our patents; and

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

## **Table of Contents**

Board. If our employees, consultants, contractors or others disclose our data to others or use data belonging to others in connection with our business, it could lead to disputes over the ownership of inventions derived from that information or expose us to potential damages or other penalties.

The occurrence of any of these events could have a material adverse effect on our business, financial condition, results of operations or prospects.

### ***We may incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights.***

There is substantial history of litigation and other proceedings regarding patent and intellectual property rights in the pharmaceutical industry. We may be forced to defend claims of infringement brought by our competitors and others, and we may institute litigation against others who we believe are infringing our intellectual property rights. The outcome of intellectual property litigation is subject to substantial uncertainties and may, for example, turn on the interpretation of claim language by the court, which may not be to our advantage, or on the testimony of experts as to technical facts upon which experts may reasonably disagree.

Under our license agreements, we have the right to bring legal action against any alleged infringers of the patents we license. However, we are responsible for all costs relating to such potential litigation. We have the right to any proceeds received as a result of such litigation, but, even if we are successful in such litigation, there is no assurance we would be awarded any monetary damages.

Our involvement in intellectual property litigation could result in significant expense to us. Some of our competitors have considerable resources available to them and a strong economic incentive to undertake substantial efforts to stop or delay us from commercializing products. Moreover, regardless of the outcome, intellectual property litigation against or by us could significantly disrupt our development and commercialization efforts, divert our management's attention and quickly consume our financial resources.

In addition, if third parties file patent applications or issue patents claiming technology that is also claimed by us in pending applications, we may be required to participate in interference proceedings with the U.S. Patent Office or in other proceedings outside the U.S., including oppositions, to determine priority of invention or patentability. Even if we are successful in these proceedings, we may incur substantial costs, and the time and attention of our management and scientific personnel will be diverted from product development or other more productive matters.

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

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

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

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

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;

**Table of Contents**

expiration or termination of licenses (particularly our licenses from BioMarin and Northwestern), research contracts or other collaboration agreements;

conditions or trends in the regulatory climate and the biotechnology and pharmaceutical industries;

intellectual property, product liability or other litigation against us;

changes in the market valuations of similar companies;

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

changes in economic conditions; and

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

In addition, equity markets in general, and the market for emerging pharmaceutical and life sciences companies in particular, have experienced substantial price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of companies traded in those markets. Further, changes in economic conditions in the United States, Europe or globally could impact our ability to grow profitably. Adverse economic changes are outside our control and may result in material adverse impacts on our business or financial results. These broad market and industry factors may materially affect the market price of our shares, regardless of our own development and operating performance. In the past, following periods of volatility in the market price of a company's securities, securities class-action litigation has often been instituted against that company. Such litigation could cause us to incur substantial costs and divert management's attention and resources, which could have a material adverse effect on our business, financial condition and results of operations.

***Delaware law and our certificate of incorporation and by-laws contain provisions that could delay and discourage takeover attempts that stockholders may consider favorable.***

Certain provisions of our certificate of incorporation and by-laws, and applicable provisions of Delaware corporate law, may make it more difficult for or prevent a third party from acquiring control of us or changing our Board of Directors and management. These provisions include:

the ability of our Board of Directors to issue preferred stock with voting or other rights or preferences;

limitations on the ability of stockholders to amend our charter documents, including stockholder supermajority voting requirements;

the inability of stockholders to act by written consent or to call special meetings;

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

## **Table of Contents**

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.

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

As of March 10, 2015, we had 81,444,849 shares of our common stock outstanding, of which 5,488,492 shares were held by our officers and directors. We also had outstanding: (i) common stock purchase warrants to purchase an aggregate of 3,433,750 additional shares of our common stock at exercise prices ranging from \$1.04 to \$2.08 per share, (ii) stock options to purchase an aggregate of 3,170,000 shares at exercise prices ranging from \$0.47 to \$3.35 per share (1,883,333 of which are currently exercisable), and 80,000 restricted stock units that are subject to vesting. Sales of restricted shares or shares underlying stock options and common stock purchase warrants, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock.

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

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

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

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

## **Table of Contents**

### **Item 1B. Unresolved Staff Comments**

None.

### **Item 2. Properties**

We currently operate our business in leased office space in Coral Gables, Florida. We currently lease approximately 2,600 square feet of space for which we pay annual rent 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 Court). These 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 the Company's motion to dismiss on 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 shares of our common stock between August 27, 2013 and October 18, 2013. In February 2014, we filed a motion to dismiss the amended complaint, which was granted in part and denied in part by the Court. Subsequently, on September 29, 2014, the Court certified a class consisting of all persons or entities that purchased shares of our common stock during the period from August 27, 2013, through October 18, 2013 (the Class Period), and who did not sell such securities prior to October 18, 2013 (excluding: defendants; any entities affiliated with us, our present and former officers and directors or any subsidiary or affiliate thereof; members of such excluded persons' immediate families and their legal representatives, heirs, successors or assigns; and any entity in which any excluded person has or had a controlling interest).

Following a mediation in mid-October 2014 conducted by an independent mediator, we entered into a memorandum of understanding (MOU) with the lead plaintiffs in the class action lawsuit to settle the lawsuit. The settlement was then reduced to a formal stipulation of settlement between the parties to the lawsuit, which was filed with the Court on November 21, 2014. The settlement was preliminarily approved by the Court on December 3, 2014, and a final hearing to determine the fairness of the settlement is currently scheduled for March 16, 2015.

In connection with the settlement, we will pay \$3.5 million in return for a dismissal and release of all claims against the defendants. The settlement amount has been placed in escrow by our insurance carrier, subject to final Court approval of the settlement. Under the proposed settlement, the defendants, and various of their related persons and entities, will receive a full release of all claims that were or could have been brought in the action, as well as all claims that arise out of, are based upon, or relate to the allegations, transactions, facts, representations, omissions or other matters involved in the action related in any way to the purchase or acquisition of our securities by class members during the class period.

The proposed settlement contains no admission of any liability or wrongdoing on the part of the defendants, each of whom continues to deny all of the allegations against each of them and believes that the claims are without merit. Because the full amount of the proposed settlement payment is to be paid by our insurance carrier, the settlement is

not expected to have a material adverse effect on our financial position or results of operations. There can be no assurance that the proposed settlement will be approved by the Court.

From time to time we may become involved in legal proceedings arising in the ordinary course of business. We believe that there is no litigation pending at this time that could have, individually or in the aggregate, a material adverse effect on our results of operations, financial condition or cash flows.

**Table of Contents**

**Item 4. Mine Safety Disclosure**

Not applicable.

46



**Table of Contents****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.

|  | <b>High</b> | <b>Low</b> |
|--|-------------|------------|
| <b>Year Ended December 31, 2013</b>    |             |            |
| First Quarter                          | \$ 0.59     | \$ 0.43    |
| Second Quarter                         | \$ 1.07     | \$ 0.45    |
| Third Quarter                          | \$ 3.23     | \$ 0.87    |
| Fourth Quarter                         | \$ 3.39     | \$ 1.32    |
| <b>Year Ended December 31, 2014</b>    |             |            |
| First Quarter                          | \$ 2.39     | \$ 1.78    |
| Second Quarter                         | \$ 2.61     | \$ 1.73    |
| Third Quarter                          | \$ 3.42     | \$ 2.17    |
| Fourth Quarter                         | \$ 3.03     | \$ 2.37    |
| <b>Year ended December 31, 2015</b>    |             |            |
| First Quarter (through March 10, 2015) | \$ 4.23     | \$ 2.74    |

The closing sale price for the common stock on March 10, 2015 was \$4.13. As of March 10, 2015, there were 42 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 10,000 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.

**Table of Contents****Performance Graph**

The graph below matches Catalyst Pharmaceutical Partners, Inc.'s cumulative 5-Year total shareholder return on common stock with the cumulative total returns of the NASDAQ Composite index, the Russell MicroCap index, and the NASDAQ Biotechnology index. The graph tracks the performance of a \$100 investment in our common stock and in each index (with the reinvestment of all dividends) from 12/31/2009 to 12/31/2014.

|   | 12/09         | 12/10         | 12/11         | 12/12         | 12/13         | 12/14         |
|---|---------------|---------------|---------------|---------------|---------------|---------------|
| <b>Catalyst Pharmaceutical Partners, Inc.</b> | <b>100.00</b> | <b>157.14</b> | <b>204.76</b> | <b>69.05</b>  | <b>309.52</b> | <b>471.43</b> |
| <b>NASDAQ Composite</b>                       | <b>100.00</b> | <b>117.61</b> | <b>118.70</b> | <b>139.00</b> | <b>196.83</b> | <b>223.74</b> |
| <b>Russell MicroCap</b>                       | <b>100.00</b> | <b>128.89</b> | <b>116.93</b> | <b>140.02</b> | <b>203.90</b> | <b>211.34</b> |
| <b>NASDAQ Biotechnology</b>                   | <b>100.00</b> | <b>106.73</b> | <b>122.40</b> | <b>166.72</b> | <b>286.55</b> | <b>379.71</b> |

*The stock price performance included in this graph is not necessarily indicative of future stock price performance.*

**Table of Contents****Item 6. Selected Financial Data**

The selected statement of operations data for the years ended December 31, 2014, 2013, 2012, and the balance sheet data as of December 31, 2014 and 2013, 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, 2011 and 2010 and the selected balance sheet data at December 31, 2012, 2011 and 2010 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.

|   | <b>Year Ended December 31,</b> |                 |                |                |                |
|---|--------------------------------|-----------------|----------------|----------------|----------------|
|   | <b>2014</b>                    | <b>2013</b>     | <b>2012</b>    | <b>2011</b>    | <b>2010</b>    |
| <b>Statement of Operations Data:</b>                  |                                |                 |                |                |                |
| Revenues government grant                             | \$                             | \$              | \$             | \$             | \$ 488,958     |
| Operating costs and expenses:                         |                                |                 |                |                |                |
| Research and development                              | 10,117,774                     | 8,096,774       | 2,659,597      | 3,383,965      | 2,306,781      |
| General and administrative                            | 4,473,654                      | 2,214,884       | 2,561,543      | 2,698,174      | 2,206,358      |
| Total operating cost and expenses                     | 14,591,428                     | 10,311,658      | 5,221,140      | 6,082,139      | 4,513,139      |
| Loss from operations                                  | (14,591,428)                   | (10,311,658)    | (5,221,140)    | (6,082,139)    | (4,024,181)    |
| Other income, net                                     | 76,233                         | 47,421          | 14,976         | 10,985         | 17,858         |
| Change in fair value of warrants liability            | (993,866)                      | (1,890,359)     | 1,129,778      | (319,908)      |                |
| Loss before income taxes                              | (15,509,061)                   | (12,154,596)    | (4,076,386)    | (6,391,062)    | (4,006,323)    |
| Provision for income taxes                            |                                |                 |                |                |                |
| Net loss  | \$ (15,509,061)                | \$ (12,154,596) | \$ (4,076,386) | \$ (6,391,062) | \$ (4,006,323) |
| Net loss per share basic and diluted                  | \$ (0.24)                      | \$ (0.27)       | \$ (0.14)      | \$ (0.29)      | \$ (0.22)      |
| Weighted average shares outstanding basic and diluted | 64,142,534                     | 45,452,447      | 30,033,108     | 21,728,292     | 18,580,223     |

|   | <b>As of December 31,</b> |               |               |              |              |
|---|---------------------------|---------------|---------------|--------------|--------------|
|   | <b>2014</b>               | <b>2013</b>   | <b>2012</b>   | <b>2011</b>  | <b>2010</b>  |
| <b>Balance Sheet Data:</b>  |                           |               |               |              |              |
| Cash and cash equivalents, certificates of deposit and short-term investments | \$ 39,275,123             | \$ 23,710,596 | \$ 15,417,208 | \$ 6,029,067 | \$ 5,475,158 |
| Working capital   | 37,972,795                | 23,180,429    | 15,080,013    | 5,394,382    | 5,476,443    |
| Total assets  | 43,908,086                | 25,369,554    | 16,789,245    | 6,249,257    | 5,831,488    |
| Warrants liability  | 2,794,891                 | 1,819,562     | 498,587       | 1,645,240    |              |
| Total liabilities   | 8,665,756                 | 3,978,302     | 2,167,130     | 2,488,559    | 313,709      |
| Stockholders' equity  | 35,242,330                | 21,391,252    | 14,622,115    | 3,760,698    | 5,517,779    |



## **Table of Contents**

### **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 2014 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 biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare debilitating diseases. We currently have three drug candidates in development:

*Firdapse*

In October 2012, we licensed the North American rights to Firdapse<sup>®</sup>, 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 took 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 Syndromes and Myasthenia Gravis (MuSK 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 and, in March 2015, we were granted orphan drug designation for Firdapse for the treatment of patients with CMS.

## **Table of Contents**

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

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

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

### Primary endpoints:

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

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

### Secondary endpoints:

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

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

### Patient tolerance of Firdapse :

Firdapse was generally safe and well tolerated. During the 91-day open label run-in period, treatment emergent adverse events occurred more frequently in treatment-naïve patients than in previously

treated patients (10% of treatment naïve patients withdrew during this part of the study). During the placebo-controlled portion of the study, side effects occurring more frequently in the Firdapse group were benign and consisted primarily of perioral and digital paresthesia and infections. No patients withdrew during this period.

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

During 2014, we established an expanded access program (EAP) to make Firdapse available to any patients diagnosed with LEMS, Congenital Myasthenic Syndrome (CMS) or Downbeat Nystagmus in the United States who meet the inclusion and exclusion criteria, with Firdapse being provided to patients for free until sometime after NDA approval. We are working with various rare disease advocacy organizations to inform physicians and patients as to the availability of the Firdapse EAP.

In January 2015, we met with the FDA to discuss our anticipated submission of an NDA for Firdapse for the treatment of LEMS. Based on our discussions with the FDA, we believe that our Phase 3 clinical program will provide acceptable support for submission of an NDA for Firdapse for LEMS. We currently expect to submit an NDA for Firdapse during the third quarter of 2015. Although there can be no assurance, we anticipate that under those circumstances we may obtain approval from the FDA of such NDA in the first half of 2016. If approved on this timeline, we would hope to commercially launch Firdapse for the treatment of LEMS shortly after its approval.



## **Table of Contents**

In anticipation of the commercialization of Firdapse, we have recently begun to prepare for the marketing of Firdapse in the United States. This has included the appointment of a Chief Commercial Officer, the hiring of a Vice President of Patient Advocacy and Reimbursement and the recent hiring of several rare disease clinical liaisons. We are currently working with several rare disease advocacy organizations to help increase awareness of LEMS and CMS and to provide education for the physicians who treat these rare diseases and the patients they treat. We anticipate developing a sales force of 15-20 representatives experienced in selling drugs that treat rare diseases. This sales force will market Firdapse to the approximately 900 neuromuscular and oncology specialists who we believe most often diagnosis and treat neuromuscular diseases such as LEMS and CMS.

### *CPP-115*

We are currently 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 such as complex partial seizures and Tourette Syndrome. CPP-115 has been granted Orphan Drug Designation by the FDA for the treatment of infantile spasms and Orphan Medicinal Product Designation in the European Union, or E.U., for West syndrome (a form of infantile spasms). We are currently evaluating CPP-115 in a Phase 1(b) multi-dose safety and tolerance study. We expect to report the results of this study during the second quarter of 2015.

### *CPP-109*

An academic investigator proof-of-concept study evaluating the use of CPP-109 (our formulation of vigabatrin, another GABA aminotransferase inhibitor) for the treatment of Tourette Syndrome is currently ongoing and, if the results of this study show evidence of reduced number of tics, we will likely seek to develop CPP-115 (which has the same mechanism of action as CPP-109) for this indication. Although we have provided drug and financial support, we do not control this study and therefore have no control over the timing of its completion. However, based on currently available information, we expect to have top-line results for this study during the second quarter of 2015.

### *Capital Resources*

Based on our current financial condition and forecasts of available cash, we believe that we have sufficient funding to support our operations through the end of 2016. However, we will require additional funding to support our operations beyond the end of 2016. 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.

### **Basis of presentation**

#### *Revenues government grant*

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.

## **Table of Contents**

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 (CROs). 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 expenses. During 2014, we have begun to incur costs relating to our future sales and marketing efforts, as we move closer to the potential commercialization of Firdapse<sup>®</sup>. 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<sup>®</sup>, 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.

### *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, 2014 and 2013 using a Black-Scholes option-pricing model. We 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 term, 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, 2014 and 2013, we had net operating loss carryforwards of approximately \$40,604,000 and \$30,675,000, respectively. Our net deferred tax asset has a 100% valuation allowance as of December 31, 2014 and 2013, 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 2025 through 2034. If an ownership change, as defined under Internal Revenue Code 382, occurs, the use of these carry-forwards may be subject to limitations.

## **Table of Contents**

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*

In June 2014, the FASB issued ASU No. 2014-10, Development Stage Entities (Topic 915): *Elimination of Certain Financial Reporting Requirements, Including an Amendment to Variable Interest Entities Guidance in Topic 810, Consolidation*. The amendments in this ASU include: i) eliminating the requirement to present inception-to-date information on the statements of income, cash flows, and shareholders' equity, ii) eliminating the need to label the financial statements as those of a development stage entity, iii) eliminating the need to disclose a description of the development stage activities in which the entity is engaged, and iv) eliminating the requirement to disclose in the first year in which the entity is no longer a development stage entity that in prior years it had been in the development stage. The amendments in ASU No. 2014-10 are effective for public companies for annual and interim reporting periods beginning after December 15, 2014. Early adoption is permitted. The Company has early adopted ASU No. 2014-10, beginning with the interim period ended June 30, 2014.

In August 2014, the FASB issued ASU No. 2014-15, Presentation of Financial Statements - Going Concern (Subtopic 205-40): *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern*. The amendments in this ASU, require management to assess a company's ability to continue as a going concern and to provide related disclosures in certain circumstances. The guidance will be effective for the annual period ending after December 15, 2016 and subsequent interim and annual periods thereafter. The Company is currently evaluating the impact of this accounting standard update on its 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 investor. 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 ongoing operations and provide 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. Further, we often report non-GAAP net loss per share, which is calculated by dividing non-GAAP net loss by the weighted average common shares outstanding.

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.



## **Table of Contents**

### **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 accounting policies described below are 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 the Company, 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, the 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 the 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 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 warrant 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 the 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 income or loss in the changes in fair value of warrants liability line in the statement of operations.



**Table of Contents***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, 2014, 2013 and 2012, the assumptions used were an estimated annual volatility of 115%, 137% and 120% average expected holding periods of three to seven years, three years and three to five years, and risk-free interest rates of 1.18% to 2.03%, 0.45% to 0.53% and 0.28% to 0.66%, respectively.

**Results of Operations***Years Ended December 31, 2014 and 2013**Revenues*

We had no revenues for the year ended December 31, 2014 or 2013.

*Research and Development Expenses*

| Year | Amount        | Change from Prior Year | Percentage of Total Operating Costs and Expenses |
|------|---------------|------------------------|--|
| 2014 | \$ 10,117,774 | 25.0%                  | 69.3%  |
| 2013 | \$ 8,096,774  | 204.4%                 | 78.5%  |

Our expenses, excluding stock-based compensation, for research and development for the year ended December 31, 2014 increased substantially compared to amounts expended in the 2013 fiscal year. Research and development expenses in 2014 consisted mainly of costs related to our Phase 3 trial of Firdapse, cost relating to pre-clinical and clinical testing on both Firdapse and CPP-115, costs related to the launch and operation of the Firdapse Expanded Access Program, costs relating to the manufacturing of Firdapse, and our share of the costs of the joint studies being conducted with BioMarin. During 2013, our research and development expenses primarily related to our Phase 3 trial of Firdapse and our continuing pre-clinical and clinical testing on Firdapse and CPP-115.

We expect that research and development expenses will increase in fiscal 2015 compared to fiscal 2014 as we continue our currently planned research and development activities, including the completion of all testing required to submit an NDA for Firdapse, costs relating to our currently anticipated submission of an NDA for Firdapse in the third quarter of 2015, costs relating to the operation of the Firdapse Expanded Access Program and costs related to the Phase 1b trial for CPP-115.

In our research and development activities for 2014 and 2013, we recorded stock-based compensation relating to the value of stock options granted to certain employees. The amount of stock-based compensation recorded in 2014 and

2013 relating to our research and development activities was \$133,862 and \$84,728, respectively. The weighted-average grant-date fair value of the stock options granted in 2014 and 2013 was \$2.45 and \$0.48, respectively.

**Table of Contents***Selling and Marketing Expenses*

We had no selling expenses during 2014 and 2013. We have begun to incur pre-commercialization costs, tied to our preparation for future sales and marketing efforts, as we move closer to the potential commercialization of Firdapse . These costs are for personnel, and their related activities, to develop both a sales force and a patient advocacy and assistance program so that we are in a position to commence our selling efforts at such time as we are successful in obtaining an approval of any NDA that we may file for Firdapse , of which there can be no assurance. Pre-commercialization costs have been included in general and administrative expenses.

*General and Administrative Expenses*

| Year | Amount       | Change from Prior Year | Percentage of Total Operating Costs and Expenses |
|------|--------------|------------------------|--|
| 2014 | \$ 4,473,654 | 102.2%                 | 30.7%  |
| 2013 | \$ 2,214,884 | (13.5%)                | 21.5%  |

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 2014 and 2013, was stock-based compensation of \$664,107 and \$91,127, respectively.

The increase in general and administrative expenses for the year ended December 31, 2014 when compared to the same period in 2013 was primarily due to increases in payroll (based on both increased headcount and increased salaries and benefits, including stock-based compensation), substantially increased consulting and marketing expenses, relating to our pre-commercialization efforts for Firdapse during 2014, and an increase in professional fees and investor relations expenses. We expect that general and administrative costs will increase in future periods as we expand our operations and headcount in preparation for the potential future commercialization of Firdapse .

*Stock-Based Compensation*

We issued stock options and other share based payments to several of our employees, directors, and consultants in 2014 and 2013. Total stock-based compensation expense for the years ended December 31, 2014 and 2013 was \$777,969 and \$175,855, respectively. The Company regularly grants non-cash stock based compensation to directors and employees as part of their compensation packages. No such grant was done during 2013; consequently, the 2013 expense represents mostly amortization expense for prior year grants and a few grants to new employees. The 2014 increase in expense from the prior year is mostly due to additional expense related to the August 2014 annual grant to employees and directors.

*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 \$2.8 million and \$1.8 million at December 31, 2014 and 2013, 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, 2014 and 2013, we recognized losses of \$993,866 and \$1,890,359 respectively, in connection with the change in the fair value of the

warrants liability. The losses during 2014 and 2013 were principally a result of the increase in our stock price. 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.

*Other Income, net*

We reported other income, net in all periods relating to our investment of funds received from offerings of our securities. Other income, net consists of interest income, dividend income and unrealized and realized gain (loss) on trading securities. The increase in other income, net for the year ended December 31, 2014 as compared to the year ended December 31, 2013 was principally 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.

**Table of Contents***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 \$15,509,061 in the year ended December 31, 2014 (\$0.24 per basic and diluted share), as compared to \$12,154,596 in the year ended December 31, 2013 (\$0.27 per basic and diluted share).

*Non-GAAP Net Loss*

Our non-GAAP net loss per share, which excludes for 2014 a \$993,866 loss associated with the change in the fair value of liability-classified warrants and excludes for 2013 a \$1,890,359 loss associated with the change in the fair value of liability-classified warrants, was \$14,515,195 (or \$0.23 per basic and diluted share) for 2014, compared to a non-GAAP net loss of \$10,264,237 (or \$0.23 per basic and diluted share) for 2013.

***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 .

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.

**Table of Contents***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.

*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.

*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 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 \$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.

*Other Income, net*

We reported other income in all periods relating to our investment of funds received from offerings of our securities. Other income consists of interest income, dividend income and unrealized and realized gain (loss) on trading securities. The increased in other 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 registered direct

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.



## **Table of Contents**

### *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 per share, 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 (or \$0.23 per basic and diluted share) for 2013, compared to a non-GAAP net loss per share of \$5,206,164 (or \$0.17 per basic and diluted share) for 2012.

## **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.

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 nine registered direct public offerings under our effective shelf registration statements. At December 31, 2014, we had cash and cash equivalents, certificates of deposit and short-term investments aggregating \$39,275,123 and working capital of \$37,972,795, as compared to cash and cash equivalents, certificates of deposit and short-term investments aggregating \$23,710,596 and working capital of \$23,180,429 at December 31, 2013. At December 31, 2014 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 2014, 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 be some time before we 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 operations through the end of 2016. 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 operations beyond 2016. 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;

**Table of Contents**

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 plan 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.

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 was declared effective on March 19, 2014. We have completed two offerings under this shelf registration statement:

On April 3, 2014, we raised net proceeds of approximately \$26.7 million from the sale of 13,023,750 shares of our common stock; and

On February 4, 2015, we raised net proceeds of approximately \$34.7 million from the sale of 11,500,000 shares of our common stock.

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.

On May 24, 2012, we sold 6,000,000 shares of our 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. 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 expire five years from their date of issuance and have an exercise price of \$1.04 per share.

**Table of Contents***Contractual obligations and arrangements*

As of December 31, 2014, 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.

|                             | Total             | Payments Due by Period |                   |           |               |
|-----------------------------|-------------------|------------------------|-------------------|-----------|---------------|
|                             |                   | Less than 1 year       | 1-3 years         | 4-5 years | After 5 years |
| Operating lease obligations | \$ 310,988        | \$ 103,902             | \$ 207,086        | \$        | \$            |
| License obligations         | 150,000           | 150,000                |                   |           |               |
| <b>Total</b>                | <b>\$ 460,988</b> | <b>\$ 253,902</b>      | <b>\$ 207,086</b> | <b>\$</b> | <b>\$</b>     |

We have entered into the following contractual arrangements:

*Payments to BioMarin and others under our license agreement.* We have agreed: (i) to pay BioMarin royalties for seven years from the first commercial sale of Firdapse equal to 7% of net sales (as defined in our license agreement) in North America for any calendar year for sales up to \$100 million, and 10% of net sales in North America in any calendar year in excess of \$100 million; (ii) to pay to the third-party licensor of the rights sublicensed to us royalty payments for seven years from the first commercial sale of Firdapse equal to 7% of net sales (as defined in the license agreement between BioMarin and the third-party licensor) in any calendar year; and (iii) to pay certain milestone payments that BioMarin is obligated to pay (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.

*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, 2014, we had paid approximately \$13.9 million of this amount and had prepaid research fees of approximately \$566,000, accounts payable of approximately \$1,441,000 and accrued liabilities of approximately \$352,000 in the accompanying balance sheet in connection with related agreements. Under our license agreement with BioMarin, we were obligated to complete our Phase III trial of Firdapse during the two years following the date of the license agreement (October 26, 2012), which condition was satisfied during the third quarter of 2014.

*Payments to Northwestern University under our license agreement.* Under our license agreement with Northwestern, we have paid to date \$251,590, had accrued liabilities of \$115,000, at December 31, 2014 in the accompanying balance sheet, 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 trial of CPP-115.

*Employment agreements.* We have entered into an employment agreement with our Chief Executive Officer that requires us to make base salary payments of approximately \$453,000 in 2015. The agreement expires in November 2016.

*Lease for office space.* We currently operate our business in leased office space in Coral Gables, Florida. We currently lease approximately 2,600 square feet of office space for which we pay annual rent of approximately \$96,000.

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**Table of Contents**

*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 or capital leases. 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.

*Cash Flows*

Net cash used in operating activities was \$12,941,783 and \$9,875,674, respectively, for the years ended December 31, 2014 and 2013. During the year ended December 31, 2014, net cash used in operating activities was primarily attributable to our net loss of \$15,509,061, and an increase of \$2,943,256 in prepaid expenses and other current assets and deposits, which were partially offset with increases of \$963,421 in accounts payable, and \$2,748,704 in accrued expenses and liabilities, and a loss of \$993,866 of non-cash expense for the change in fair value of warrants liability. The loss included an additional \$804,543 of non-cash expenses. Such additional non-cash expenses consist of depreciation and stock-based compensation expense.

Net cash used in investing activities was \$8,741,030 and \$7,496,801, respectively, for 2014 and 2013. For 2014 and 2013 such funds were used primarily for purchases of short-term investments and capital expenditures relating to computer equipment and furniture and equipment, partially offset by proceeds from certificates of deposit.

Net cash provided by financing activities was \$28,563,633 and \$18,178,494, respectively, for 2014 and 2013. During 2014 and 2013, net cash from financing activities consisted mainly of the net proceeds from the sale of shares of common stock in underwritten and registered direct public offerings under our registration statements, as well as proceeds from exercise of warrants. 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 intended to identify forward-looking statements. Such statements involve known and unknown risks, uncertainties and other

factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by such forward-looking statements. The forward-looking statements made in this Form 10-K are based on current expectations that involve numerous risks and uncertainties.



**Table of Contents**

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

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

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

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

whether our trials and studies will be successful;

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

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

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

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

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

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

whether we will ever be able to achieve sustained profitability;

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

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

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

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

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

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

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

our expectations regarding licensing, acquisitions or strategic relationships;

changes in the laws and regulations affecting our business;

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

**Table of Contents**

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

whether the settlement that we have reached of the claims brought against us in the class action lawsuit will be approved;

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

our ability to attract and retain skilled employees;

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 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 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.

**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

## **Table of Contents**

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, 2014, our disclosure controls and procedures were effective to ensure that the information required to be disclosed by us in the reports filed or submitted by us under the Securities Exchange Act of 1934, as amended, was recorded, processed, summarized or reported within the time periods specified in the rules and regulations of the SEC, and include controls and procedures designed to ensure that information required to be disclosed by us in such reports was accumulated and communicated to management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosures.

### *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, 2014 based on the 2013 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, 2014.

During the fourth quarter of 2014, there were no changes in our internal control over financial reporting, as defined in Rule 13a-15(f) under the Securities and Exchange Act of 1934 that have materially affected, or are reasonably likely to materially affect, the Company's internal control over financial reporting.

Our independent registered public accounting firm, Grant Thornton LLP, has issued a report on our internal control over financial reporting, which is included in Item 15 of this Annual Report on Form 10-K.

**Item 9B. Other Information**

Not applicable.

**Table of Contents**

**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 2015 Annual Meeting of Stockholders. Our Proxy Statement for the 2015 Annual Meeting of Stockholders is expected to be filed not later than 120 days after the end of our fiscal year ended December 31, 2014 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](http://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.

**Table of Contents**

**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:

Reports of Grant Thornton LLP, Independent Registered Public Accounting Firm

Balance Sheets as of December 31, 2014 and 2013

Statements of Operations for the years ended December 31, 2014, 2013 and 2012

Statement of Stockholders' Equity for the period December 31, 2011 until December 31, 2014

Statements of Cash Flows for the years ended December 31, 2014, 2013 and 2012.

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.

| <b>Exhibit No.</b> | <b>Description of Exhibit</b>   |
|--------------------|---|
| 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(10) |
| 4.3    | Form of Warrant to Purchase Common Stock issued in our October 2011 offering; (11)        |
| 4.4    | Form of Warrant to Purchase Common Stock issued in our May 2012 offering (14)             |
| 4.5    | Form of Warrant to Purchase Common Stock issued in our August 2012 offering (15)          |
| 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)           |
| 10.3 + | Amendment to Employment Agreement between the Company and Patrick J. McEnany(6)           |

**Table of Contents**

| <b>Exhibit No.</b> | <b>Description of Exhibit</b>   |
|--------------------|---|
| 10.4 +             | Amendment to Employment Agreement between the Company and Patrick J. McEnany(9)   |
| 10.5+              | Amendment to Employment Agreement between the Company and Patrick J. McEnany (17)   |
| 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 (7)  |
| 10.11+             | Amendment No. 2 to 2006 Stock Incentive Plan (13)   |
| 10.12+             | 2014 Stock Incentive Plan (20)  |
| 10.13              | License Agreement between the Company and Northwestern University(5)  |
| 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. (8)   |
| 10.16              | License Agreement among the Company, New York University, and The Feinstein Institute for Medical Research (12)                                   |
| 10.17              | Convertible Promissory Note and Note Purchase Agreement, dated as of October 26, 2012, between the Company and BioMarin Pharmaceutical, Inc. (16) |
| 10.18              | License Agreement, dated as of October 26, 2012, between the Company and BioMarin Pharmaceutical, Inc. (16)                                       |
| 10.19              | Amendment No, 1 to License Agreement, dated April 8, 2014, between the Company and BioMarin Pharmaceutical, Inc. (21)                             |
| 10.20              | Termination Agreement, dated effective October 1, 2013, between the Company and Brookhaven Science Associates, LLC (18)                           |
| 10.21              | Second Amendment to Lease, dated as of February 4, 2014, between the Company and 355 Alhambra Circle LLC (19)                                     |
| 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*  |

**Table of Contents**

| <b>Exhibit No.</b> | <b>Description of Exhibit</b>                 |
|--------------------|---|
| 101.INS            | XBRL Instance Document                        |
| 101.SCH            | XBRL Taxonomy Extension Schema                |
| 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 Form 10-Q for the period ended September 30, 2006
- (3) Filed by reference to the Company s Form 10-Q for the period ended June 30, 2007
- (4) Filed by reference to the Company s Form 8-K dated December 23, 2008
- (5) Filed by reference to the Company s Form 8-K dated September 2, 2009
- (6) Filed by reference to the Company s Form 10-Q for the period ended September 30, 2009
- (7) Filed by reference to the Company s 2011 Annual Meeting Proxy Statement dated April 11, 2011
- (8) Filed by reference to the Company s Form 10-Q for the period ended June 30, 2011
- (9) Filed by reference to the Company s Form 8-K dated September 14, 2011
- (10) Filed by reference to the Company s Form 8-K dated September 20, 2011
- (11) Filed by reference to the Company s Form 8-K dated October 28, 2011
- (12) Filed by reference to the Company s Annual Report on Form 10-K for the period ended December 31, 2011.
- (13) Filed by reference to the Company s 2012 Annual Meeting Proxy Statement dated April 17, 2012
- (14) Filed by reference to the Company s Registration Statement on Form S-1 (File No. 333-180617)
- (15) Filed by reference to the Company s Form 8-K dated August 28, 2012
- (16) Filed by reference to the Company s Form 8-K dated October 26, 2012
- (17) Filed by reference to the Company s Form 8-K dated August 28, 2013
- (18) Filed by reference to the Company s Form 10-Q for the period ended September 30, 2013
- (19) Filed by reference to the Company s Form 8-K dated February 20, 2014
- (20) Filed by reference to the Company s Form 8-K dated March 4, 2014
- (21) Filed by reference to the Company s Form 8-K dated April 17, 2014

\* Filed herewith

+ Management contract or compensatory plan

**Table of Contents****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 13 day of March, 2015.

**CATALYST PHARMACEUTICAL PARTNERS, INC.**

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.

| <b>Signature</b>                                     | <b>Title</b>   | <b>Date</b>    |
|--|--|----------------|
| /s/ Patrick J. McEnany<br>Patrick J. McEnany         | Chairman of the Board of Directors,<br>President and Chief Executive Officer<br>(Principal Executive Officer)              | March 13, 2015 |
| /s/ Alicia Grande<br>Alicia Grande                   | Vice President, Treasurer,<br>Chief Financial Officer<br>(Principal Financial Officer and<br>Principal Accounting Officer) | March 13, 2015 |
| /s/ Charles B. O Keeffe<br>Charles B. O Keeffe       | Director   | March 13, 2015 |
| /s/ Philip H. Coelho<br>Philip H. Coelho             | Director   | March 13, 2015 |
| /s/ David S. Tierney, M.D.<br>David S. Tierney, M.D. | Director   | March 13, 2015 |
| /s/ Donald A. Denkhous<br>Donald A. Denkhous         | Director   | March 13, 2015 |
| /s/ Richard Daly<br>Richard Daly                     | Director   | March 13, 2015 |



**Table of Contents**

**INDEX TO FINANCIAL STATEMENTS**

**Years ended December 31, 2014, 2013, and 2012**

|  |            |
|--|------------|
| <b><u>Reports of independent registered public accounting firm</u></b> | <b>F-2</b> |
| <b><u>Balance sheets</u></b>   | <b>F-4</b> |
| <b><u>Statements of operations</u></b>                                 | <b>F-5</b> |
| <b><u>Statement of stockholders' equity</u></b>                        | <b>F-6</b> |
| <b><u>Statements of cash flows</u></b>                                 | <b>F-7</b> |
| <b><u>Notes to financial statements</u></b>                            | <b>F-8</b> |

F-1

**Table of Contents**

**REPORT OF INDEPENDENT REGISTERED**

**PUBLIC ACCOUNTING FIRM**

Board of Directors and Stockholders

Catalyst Pharmaceutical Partners, Inc.

We have audited the internal control over financial reporting of Catalyst Pharmaceutical Partners, Inc. (the Company) as of December 31, 2014, based on criteria established in the 2013 Internal Control Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting, included in the accompanying Management's Annual Assessment of Internal Control Over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit.

We conducted our audit 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 effective internal control over financial reporting was maintained in all material respects. Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

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.

In our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2014, based on criteria established in the 2013 Internal Control Integrated Framework issued by COSO.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), the financial statements of the Company as of and for the year ended December 31, 2014, and our report dated March 13, 2015 expressed an unqualified opinion on those financial statements.

/s/ Grant Thornton LLP

Miami, Florida

March 13, 2015

F-2



**Table of Contents**

**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. (the Company) as of December 31, 2014 and 2013, and the related statements of operations, stockholders' equity, and cash flows for each of the three years in the period ended December 31, 2014. 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. 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. as of December 31, 2014 and 2013, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2014 in conformity with accounting principles generally accepted in the United States of America.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), the Company's internal control over financial reporting as of December 31, 2014, based on criteria established in the 2013 Internal Control - Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO), and our report dated March 13, 2015 expressed an unqualified opinion thereon.

/s/ Grant Thornton LLP

Miami, Florida

March 13, 2015

**Table of Contents****CATALYST PHARMACEUTICAL PARTNERS, INC.****BALANCE SHEETS**

|  | <b>December 31,<br/>2014</b> | <b>December 31,<br/>2013</b> |
|--|------------------------------|------------------------------|
| <b>ASSETS</b>  |                              |                              |
| Current Assets:  |                              |                              |
| Cash and cash equivalents  | \$ 9,096,778                 | \$ 2,215,958                 |
| Certificates of deposit  | 3,715,383                    | 4,011,576                    |
| Short-term investments   | 26,462,962                   | 17,483,062                   |
| Prepaid expenses and other current assets  | 4,552,698                    | 1,609,442                    |
| <b>Total current assets</b>  | <b>43,827,821</b>            | <b>25,320,038</b>            |
| Property and equipment, net  | 71,377                       | 40,628                       |
| Deposits   | 8,888                        | 8,888                        |
| <b>Total assets</b>  | <b>\$ 43,908,086</b>         | <b>\$ 25,369,554</b>         |
| <b>LIABILITIES AND STOCKHOLDERS EQUITY</b>   |                              |                              |
| Current Liabilities:   |                              |                              |
| Accounts payable   | \$ 1,814,210                 | \$ 850,789                   |
| Accrued expenses and other liabilities   | 4,040,816                    | 1,288,820                    |
| <b>Total current liabilities</b>   | <b>5,855,026</b>             | <b>2,139,609</b>             |
| Accrued expenses and other liabilities, non-current  | 15,839                       | 19,131                       |
| Warrants liability, at fair value  | 2,794,891                    | 1,819,562                    |
| <b>Total liabilities</b>   | <b>8,665,756</b>             | <b>3,978,302</b>             |
| Commitments and contingencies  |                              |                              |
| Stockholders equity:   |                              |                              |
| Preferred stock, \$0.001 par value, 5,000,000 shares authorized: none issued and outstanding at December 31, 2014 and 2013   |                              |                              |
| Common stock, \$0.001 par value, 100,000,000 shares authorized; 69,119,092 shares and 54,132,937 shares issued and outstanding at December 31, 2014 and 2013, respectively | 69,119                       | 54,133                       |
| Additional paid-in capital   | 105,015,871                  | 75,670,718                   |
| Accumulated deficit  | (69,842,660)                 | (54,333,599)                 |
| <b>Total stockholders equity</b>   | <b>35,242,330</b>            | <b>21,391,252</b>            |
| <b>Total liabilities and stockholders equity</b>   | <b>\$ 43,908,086</b>         | <b>\$ 25,369,554</b>         |

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



Table of Contents

## CATALYST PHARMACEUTICAL PARTNERS, INC.

## STATEMENTS OF OPERATIONS

|   | Year Ended December 31, |                 |                 |
|---|-------------------------|-----------------|-----------------|
|   | 2014                    | 2013            | 2012            |
| Revenues  | \$                      | \$              | \$              |
| Operating costs and expenses:                           |                         |                 |                 |
| Research and development                                | 10,117,774              | 8,096,774       | 2,659,597       |
| General and administrative                              | 4,473,654               | 2,214,884       | 2,561,543       |
| Total operating costs and expenses                      | 14,591,428              | 10,311,658      | 5,221,140       |
| Loss from operations                                    | (14,591,428)            | (10,311,658)    | (5,221,140)     |
| Other income, net                                       | 76,233                  | 47,421          | 14,976          |
| Change in fair value of warrants liability              | (993,866)               | (1,890,359)     | 1,129,778       |
| Loss before income taxes                                | (15,509,061)            | (12,154,596)    | (4,076,386)     |
| Provision for income taxes                              |                         |                 |                 |
| Net loss  | \$ (15,509,061)         | \$ (12,154,596) | \$ (4,076,386)  |
| Net loss per share   basic and diluted                  | \$       (0.24)         | \$       (0.27) | \$       (0.14) |
| Weighted average shares outstanding   basic and diluted | 64,142,534              | 45,452,447      | 30,033,108      |

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

Table of Contents

## CATALYST PHARMACEUTICAL PARTNERS, INC.

## STATEMENT OF STOCKHOLDERS EQUITY

for the years ended December 31, 2014, 2013 and 2012

|  | Preferred<br>Stock | Common<br>Stock | Additional<br>Paid-In<br>Capital | Accumulated<br>Deficit | Total         |
|--|--------------------|-----------------|----------------------------------|------------------------|---------------|
| <b>Balance at December 31, 2011</b>              | \$                 | \$ 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,<br>net    |                    | 10,000          | 9,554,640                        |                        | 9,564,640     |
| Issuance of common stock upon note<br>conversion |                    | 6,667           | 4,993,333                        |                        | 5,000,000     |
| Net loss   |                    |                 |                                  | (4,076,386)            | (4,076,386)   |
| <b>Balance at December 31, 2012</b>              |                    | 41,421          | 56,759,697                       | (42,179,003)           | 14,622,115    |
| Issuance of common stock, net                    |                    | 8,850           | 14,086,344                       |                        | 14,095,194    |
| Issuance of stock options for services           |                    |                 | 175,855                          |                        | 175,855       |
| Exercise of warrants for common stock            |                    | 3,862           | 4,648,822                        |                        | 4,652,684     |
| Net loss   |                    |                 |                                  | (12,154,596)           | (12,154,596)  |
| <b>Balance at December 31, 2013</b>              |                    | 54,133          | 75,670,718                       | (54,333,599)           | 21,391,252    |
| Issuance of common stock, net                    |                    | 13,024          | 26,712,106                       |                        | 26,725,130    |
| Issuance of stock options for services           |                    |                 | 767,838                          |                        | 767,838       |
| Amortization of restricted stock for<br>services |                    |                 | 10,131                           |                        | 10,131        |
| Exercise of warrants for common stock            |                    | 1,262           | 1,333,778                        |                        | 1,335,040     |
| Exercise of stock options for common<br>stock    |                    | 700             | 521,300                          |                        | 522,000       |
| Net loss   |                    |                 |                                  | (15,509,061)           | (15,509,061)  |
| <b>Balance at December 31, 2014</b>              | \$                 | \$ 69,119       | \$ 105,015,871                   | \$ (69,842,660)        | \$ 35,242,330 |

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

Table of Contents

## CATALYST PHARMACEUTICAL PARTNERS, INC.

## STATEMENTS OF CASH FLOWS

|   | Year Ended December 31, |                 |                |
|---|-------------------------|-----------------|----------------|
|   | 2014                    | 2013            | 2012           |
| <b>Operating Activities:</b>  |                         |                 |                |
| Net loss  | \$ (15,509,061)         | \$ (12,154,596) | \$ (4,076,386) |
| Adjustments to reconcile net loss to net cash used in operating activities: |                         |                 |                |
| Depreciation  | 26,574                  | 22,483          | 10,889         |
| Stock-based compensation  | 777,969                 | 175,855         | 340,039        |
| Change in fair value of warrants liability                                  | 993,866                 | 1,890,359       | (1,129,778)    |
| (Increase) decrease in:   |                         |                 |                |
| Prepaid expenses and other current assets and deposits                      | (2,943,256)             | (299,972)       | (1,110,354)    |
| Increase (decrease) in:   |                         |                 |                |
| Accounts payable  | 963,421                 | (514,874)       | 1,101,729      |
| Accrued expenses and other liabilities                                      | 2,748,704               | 1,005,071       | (276,505)      |
| Net cash used in operating activities                                       | (12,941,783)            | (9,875,674)     | (5,140,366)    |
| <b>Investing Activities:</b>  |                         |                 |                |
| Capital expenditures  | (57,323)                | (9,432)         | (52,382)       |
| Purchase of short-term investments  | (8,979,900)             | (9,978,618)     | (7,504,444)    |
| Proceeds (purchase) of certificates of deposit                              | 296,193                 | 2,491,249       | (6,502,825)    |
| Net cash used in investing activities                                       | (8,741,030)             | (7,496,801)     | (14,059,651)   |
| <b>Financing Activities:</b>  |                         |                 |                |
| Proceeds from issuance of common stock and warrants, net                    | 26,725,130              | 14,071,694      | 9,564,640      |
| Proceeds from issuance of convertible promissory note                       |                         |                 | 5,000,000      |
| Proceeds from exercise of warrants  | 1,316,503               | 4,083,300       | 16,249         |
| Proceeds from exercise of options   | 522,000                 | 23,500          |                |
| Net cash provided by financing activities                                   | 28,563,633              | 18,178,494      | 14,580,889     |
| Net increase (decrease) in cash and cash equivalents                        | 6,880,820               | 806,019         | (4,619,128)    |
| Cash and cash equivalents beginning of period                               | 2,215,958               | 1,409,939       | 6,029,067      |
| Cash and cash equivalents end of period                                     | \$ 9,096,778            | \$ 2,215,958    | \$ 1,409,939   |
| <b>Non-cash investing and financing activities:</b>                         |                         |                 |                |
| Exercise of liability classified warrants for common stock                  | \$ 18,537               | \$ 569,384      | \$ 16,875      |
| Conversion of note to common stock  | \$                      | \$              | \$ 5,000,000   |

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



**Table of Contents**

**CATALYST PHARMACEUTICAL PARTNERS, INC.**

**NOTES TO FINANCIAL STATEMENTS**

**1. Organization and Description of Business**

Catalyst Pharmaceutical Partners, Inc. (the Company) is a development-stage biopharmaceutical 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.

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. The Company's primary focus is on the development and commercialization of its drug candidates. The Company has incurred operating losses in each period from inception through December 31, 2014. The Company has been able to fund its cash needs to date through several public and private offerings of its common stock and warrants, through government grants, and through an investment by a strategic purchaser. 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 U.S. Securities and Exchange Commission (SEC) to sell up to \$100 million of common stock. This registration statement (file No. 333-193699) was declared effective by the SEC on March 19, 2014. On April 3, 2014, the Company sold 13,023,750 shares of its common stock in an underwritten public offering under the 2014 Shelf Registration Statement, raising net proceeds of approximately \$26.7 million. Subsequent to year end, on February 4, 2015, the Company sold 11,500,000 shares of its common stock in an underwritten public offering under the 2014 Shelf Registration Statement, raising net proceeds of approximately \$34.7 million. (See Note 11). While there can be no assurance, based on currently available information, the Company estimates that it currently has sufficient working capital to support its operations through the end of 2016. The Company will require additional capital to support its operations in periods after 2016.

The Company may raise required funds in the future 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 such 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 additional funds through collaborative arrangements, it may be necessary to relinquish some rights to the Company's drug candidates 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. 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.
  
- b. 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 money market funds. The Company has substantially all of its cash and cash equivalents deposited with one financial institution.

**Table of Contents**

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

- c. 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, 2014 and 2013 approximates fair value.
- d. 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, 2014 and 2013 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, 2014 and 2013 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 recognizes realized gains and losses and unrealized gains and losses to earnings. Unrealized and realized losses on trading securities for the years ended December 31, 2014 and 2013 were nominal and are included in other income, net in the accompanying statements of operations.
- e. PREPAID EXPENSES AND OTHER CURRENT ASSETS.** Prepaid expenses and other current assets consist primarily of insurance recoverable, prepaid research fees, prepaid insurance and prepaid subscription fees. Insurance recoverable relates to the securities class action lawsuit proposed settlement to be paid by the Company's insurance carrier. Prepaid research fees consist of advances for the Company's product 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.
- f. 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. Useful lives generally range from three years for computer equipment to three to six years for furniture and equipment. Expenditures for repairs and maintenance are charged to expenses as incurred.
- g. 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, 2014 and 2013, the Company had \$19,997 and \$21,877, respectively, of deferred rent in accrued expenses and other liabilities.
- h. 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, 2014 and 2013, the fair value of these

instruments approximated their carrying value.

- i. 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).

F-9

**Table of Contents****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.

|                         | <b>Fair Value Measurements at Reporting Date Using</b> |  |   |   |
|-------------------------|--|--|---|---|
|                         | Balances as of December 31, 2014                       | 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      | \$ 7,053,310   | \$ 7,053,310   | \$  | \$  |
| Certificates of deposit | \$ 3,715,383   | \$   | \$ 3,715,383                                  | \$  |
| Short-term investments  | \$ 26,462,962  | \$ 26,462,962  | \$  | \$  |
| Warrants liability      | \$ 2,794,891   | \$   | \$  | \$ 2,794,891                              |

|                         | <b>Fair Value Measurements at Reporting Date Using</b> |  |   |   |
|-------------------------|--|--|---|---|
|                         | 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 |

- j. 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 statements of operations. As of December 31, 2014 and 2013, 1,242,174 and 1,254,870, respectively, of the 2011 warrants remained outstanding.
- k. 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.

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

- 1. STOCK-BASED COMPENSATION.** The Company recognizes expense in the statement of operations for the fair value of all stock-based payments to employees, directors, scientific advisors 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 the 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 seven years. The Company estimates forfeitures and adjusts this estimate periodically based on actual forfeitures.

For the years ended December 31, 2014, 2013 and 2012, the Company recorded stock-based compensation expense as follows:

|                                | 2014       | 2013       | 2012       |
|--------------------------------|------------|------------|------------|
| Research and development       | \$ 133,862 | \$ 84,728  | \$ 100,221 |
| General and administrative     | 644,107    | 91,127     | 239,818    |
| Total stock-based compensation | \$ 777,969 | \$ 175,855 | \$ 340,039 |

- m. 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.

- n. 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.

- o. 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. For all periods presented, the Company's net loss equals comprehensive loss, since the Company has no items which are considered other comprehensive income (loss).

F-11

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

- p. 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, 2014, 2013 and 2012:

|  | 2014      | 2013      | 2012       |
|--|-----------|-----------|------------|
| Options to purchase common stock           | 3,884,610 | 3,428,906 | 3,650,535  |
| Warrants to purchase common stock          | 3,585,924 | 4,848,620 | 8,710,870  |
| Unvested restricted stock                  | 80,000    |           |            |
| Potential equivalent common stock excluded | 7,550,534 | 8,277,526 | 12,361,405 |

Potentially dilutive options to purchase common stock as of December 31, 2014 have exercise prices ranging from \$0.47 to \$3.12. Potentially dilutive options to purchase common stock as of December 31, 2013 and 2012 have exercise prices ranging from \$0.47 to \$6.00. Potentially dilutive warrants to purchase common stock as of December 31, 2014, 2013 and 2012 have exercise prices ranging from \$1.04 to \$2.08.

- q. SEGMENT INFORMATION.** Management has determined that the Company operates in one reportable segment, which is the development and commercialization of pharmaceutical products.
- r. RECLASSIFICATIONS.** Certain prior year amounts in the financial statements have been reclassified to conform to the current year presentation.
- s. RECENTLY ISSUED ACCOUNTING STANDARDS.** In June 2014, the FASB issued ASU No. 2014-10, Development Stage Entities (Topic 915): *Elimination of Certain Financial Reporting Requirements, Including an Amendment to Variable Interest Entities Guidance in Topic 810, Consolidation*. The amendments in this ASU include: i) eliminating the requirement to present inception-to-date information on the statements of income, cash flows, and shareholders' equity, ii) eliminating the need to label the financial statements as those of a development stage entity, iii) eliminating the need to disclose a description of the development stage activities in which the entity is engaged, and iv) eliminating the requirement to disclose in the first year in which the entity is no longer a development stage entity that in prior years it had been in the development stage. The amendments in ASU No. 2014-10 are effective for public companies for annual and interim reporting periods beginning after December 15, 2014. Early adoption is permitted. The Company has early adopted ASU No. 2014-10, beginning with the interim period ended June 30, 2014.



In August 2014, the FASB issued ASU No. 2014-15, Presentation of Financial Statements – Going Concern (Subtopic 205-40): *Disclosure of Uncertainties about an Entity’s Ability to Continue as a Going Concern*. The amendments in this ASU, require management to assess a company’s ability to continue as a going concern and to provide related disclosures in certain circumstances. The guidance will be effective for the annual period ending after December 15, 2016 and subsequent interim and annual periods thereafter. The Company is currently evaluating the impact of this accounting standard update on its financial statements.

**Table of Contents****3. Warrants Liability, at Fair Value**

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:

|                          | <b>December 31, 2014</b> | <b>December 31, 2013</b> |
|--------------------------|--------------------------|--------------------------|
| Risk free interest rate  | 0.81%                    | 0.94%                    |
| Expected term            | 2.34 years               | 3.34 years               |
| Expected volatility      | 112%                     | 108%                     |
| Expected dividend yield  | 0%                       | 0%                       |
| Expected forfeiture rate | 0%                       | 0%                       |

The following table rolls forward the fair value of the Company's warrants liability activity for the years ended December 31, 2014, 2013 and 2012:

|                                 | <b>2014</b>  | <b>2013</b>  | <b>2012</b>  |
|---------------------------------|--------------|--------------|--------------|
| Fair value, beginning of period | \$ 1,819,562 | \$ 498,587   | \$ 1,645,240 |
| Issuance of warrants            |              |              |              |
| Exercise of warrants            | (18,537)     | (569,384)    | (16,875)     |
| Change in fair value            | 993,866      | 1,890,359    | (1,129,778)  |
| Fair value, end of period       | \$ 2,794,891 | \$ 1,819,562 | \$ 498,587   |

During 2014, 12,696 of the 2011 warrants were exercised, with proceeds to the Company of \$16,504. 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.

F-13

**Table of Contents****4. Prepaid Expenses and Other Current Assets**

Prepaid expenses and other current assets consist of the following as of December 31:

|                                    | <b>2014</b>      | <b>2013</b>      |
|------------------------------------|------------------|------------------|
| Insurance recoverable (see Note 7) | \$ 3,500,000     | \$               |
| Prepaid research fees              | 571,428          | 1,334,149        |
| Prepaid insurance                  | 385,496          | 219,651          |
| Prepaid subscriptions fees         | 30,495           | 24,643           |
| Prepaid offering costs             | 20,029           |                  |
| Prepaid rent                       | 10,870           | 7,848            |
| Other                              | 34,380           | 23,151           |
| <br>Total prepaid expenses         | <br>\$ 4,552,698 | <br>\$ 1,609,442 |

**5. Property and Equipment**

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

|                                       | <b>2014</b>   | <b>2013</b>   |
|---------------------------------------|---------------|---------------|
| Computer equipment                    | \$ 95,754     | \$ 81,551     |
| Furniture and equipment               | 88,816        | 51,523        |
|                                       | 184,570       | 133,074       |
| Less: Accumulated depreciation        | (113,193)     | (92,446)      |
| <br>Total property and equipment, net | <br>\$ 71,377 | <br>\$ 40,628 |

Depreciation expense was \$26,574, \$22,483 and \$10,889, respectively, for the years ended December 31, 2014, 2013 and 2012.

**6. Accrued Expenses and Other Liabilities**

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

|  | <b>2014</b>  | <b>2013</b> |
|--|--------------|-------------|
| Accrued settlement liability (see Note 7)        | \$ 3,500,000 | \$          |
| Accrued pre-clinical and clinical trial expenses | 333,928      | 1,083,749   |
| Accrued professional fees                        | 43,973       | 117,240     |
| Accrued compensation and benefits                | 31,956       | 14,539      |
| Accrued license fees                             | 115,000      | 65,000      |

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|  |              |              |
|--|--------------|--------------|
| Deferred rent                                      | 4,158        | 2,746        |
| Other  | 11,801       | 5,546        |
| Current accrued expenses and other liabilities     | 4,040,816    | 1,288,820    |
| Deferred rent non-current                          | 15,839       | 19,131       |
| Non-current accrued expenses and other liabilities | 15,839       | 19,131       |
| Total accrued expenses and other liabilities       | \$ 4,056,655 | \$ 1,307,951 |

The accrued settlement liability of \$3,500,000 as of December 31, 2014 is related to the securities class action lawsuit proposed settlement, as disclosed with more particularity in Note 7. The proposed settlement amount is expected to be paid for and covered by the Company's insurance carrier; therefore, there is a corresponding insurance recoverable recorded in Prepaid Expenses and Other Current Assets in the accompanying balance sheet as of December 31, 2014.

**Table of Contents****7. Commitments and Contingencies**

The Company has contracted with drug manufacturers and other vendors, including clinical research organizations (CRO) overseeing the clinical trials 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 submission 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, 2014, future minimum lease payments under the operating lease agreement are as follows:

|      |            |
|------|------------|
| 2015 | \$ 103,902 |
| 2016 | 107,010    |
| 2017 | 100,076    |
|      | \$ 310,988 |

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 relocated the Company into another space within the same building. During February 2014, the Company entered into the second amendment of the lease for an additional contiguous space under substantially the same terms. The corporate office lease is cancellable upon the payment of an early termination penalty during 2015. 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 \$90,163, \$69,930 and \$65,310, respectively, for the years ended December 31, 2014, 2013 and 2012. The Company's office lease expires in November 2017.

***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). These 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 on 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 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 shares of the Company's common stock between August 27, 2013 and October 18, 2013. In February 2014, the Company filed a motion to dismiss the amended complaint, which was granted in part and denied in part by the Court. Subsequently, on September 29, 2014, the Court certified a class consisting of all persons or entities that purchased shares of the Company's common stock

during the period from August 27, 2013, through October 18, 2013 (the Class Period), and who did not sell such securities prior to October 18, 2013 (excluding: defendants; any entities affiliated with the Company, the present and former officers and directors of the Company or any subsidiary or affiliate thereof; members of such excluded persons immediate families and their legal representatives, heirs, successors or assigns; and any entity in which any excluded person has or had a controlling interest).

F-15

## Table of Contents

### 7. Commitments and Contingencies (continued)

Following a mediation in mid-October conducted by an independent mediator, the Company entered into a memorandum of understanding (MOU) with the lead plaintiffs in the class action lawsuit to settle the lawsuit. The settlement was then reduced to a formal stipulation of settlement between the parties to the lawsuit, which was filed with the Court on November 21, 2014. The settlement was preliminarily approved by the Court in December 3, 2014, and a final hearing to determine the fairness of the settlement has been scheduled for March 16, 2015.

In connection with the settlement, the Company will pay \$3.5 million in return for a dismissal and release of all claims against the defendants. The settlement amount has been placed in escrow by the Company's insurance carrier, subject to final Court approval of the settlement. Under the proposed settlement, the defendants, and various of their related persons and entities, will receive a full release of all claims that were or could have been brought in the action, as well as all claims that arise out of, are based upon, or relate to the allegations, transactions, facts, representations, omissions or other matters involved in the action related in any way to the purchase or acquisition of the Company's securities by class members during the class period.

The proposed settlement contains no admission of any liability or wrongdoing on the part of the defendants, each of whom continues to deny all of the allegations against each of them and believes that the claims are without merit. Because the full amount of the proposed settlement payment is expected to be paid by the Company's insurance carrier, the settlement is not expected to have a material adverse effect on the Company's financial position or results of operations. There can be no assurance that the settlement will be approved by the Court.

Obligations under capital leases are not significant.

For commitments related to the Company's license agreements with BioMarin (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 had 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.

F-16

**Table of Contents****8. Agreements (continued)**

Under the license agreement with Northwestern, the Company is responsible for continued research and development of any resulting product candidates. As of December 31, 2014, the Company had paid Northwestern \$251,590 in connection with the license and had accrued license fees of \$115,000 and \$65,000 as of December 31, 2014 and 2013, 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 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.
- d. LICENSE AGREEMENT WITH BIOMARIN.** On October 26, 2012, the Company entered into a strategic collaboration with BioMarin Pharmaceutical, Inc. (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 .

As part of the License Agreement, the Company took over a Phase 3 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 was 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 had the right to terminate the License Agreement if such treatment phase had not been completed in such 24-month period (unless the Company was using diligent effort to pursue the completion of such treatment phase and had spent at least \$5 million in connection with the conduct of the Phase 3 Trial during such 24 month period, which condition has been satisfied during the third quarter of 2014. ). On September 29, 2014, the Company announced positive top-line results from its Phase 3 Trial of Firdapse for the symptomatic treatment of LEMS. Both co-primary endpoints, quantitative myasthenia gravis score (QMG) and subject global impression (SGI) demonstrated statistical significance, as did a secondary endpoint for the physician s clinical global impression of improvement (CGI-I).

As part of the License Agreement, the Company agreed: (i) to pay BioMarin royalties for seven years from the first commercial sale of Firdapse equal to 7% of net sales (as defined in our license agreement) in North America for any calendar year for sales up to \$100 million, and 10% of net sales in North America in any calendar year in excess of \$100 million; (ii) to pay to the third-party licensor of the rights sublicensed to us royalty payments for seven years from the first commercial sale of Firdapse equal to 7% of net sales (as defined in the license agreement between BioMarin and the third-party licensor) in any calendar year; and (iii) to pay certain milestone payments that BioMarin

is obligated to pay (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 also agreed to share in the cost of certain post-marketing studies being conducted by BioMarin, and, as of December 31, 2014, the Company had paid BioMarin \$3.1 million related to expenses in connection with Firdapse studies and trials.

F-17

## **Table of Contents**

### **8. Agreements (continued)**

On April 15, 2014, effective as of April 8, 2014, the Company and BioMarin entered into Amendment No. 1 to the License Agreement, amending in certain respects the License Agreement, dated October 26, 2012, between the Company and BioMarin. The amendment related to purchases of additional product by the Company from BioMarin, the sharing of data between the parties with respect to clinical trials and studies undertaken by each party and the payment terms for certain joint studies.

- e. **AGREEMENTS FOR DRUG DEVELOPMENT, PRE-CLINICAL AND CLINICAL STUDIES.** 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 (CRO) 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, 2014, 2013 and 2012, the Company paid approximately \$10,000, \$10,000 and \$42,000, respectively, in consulting fees to related parties.

The Company has an employment agreement with its Chief Executive Officer. Under this agreement, the CEO will receive an annual base salary of approximately \$453,000 in 2015, and may earn bonus compensation of up to 50% of his salary based on performance. This agreement expires in November 2016.

### **10. Income Taxes**

As of December 31, 2014 and 2013, the Company had deferred tax assets of approximately \$24,895,000 and \$19,387,000, respectively, of which approximately \$22,898,000 and \$17,685,000 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, 2014 and 2013, 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 \$5,508,000, \$3,796,000 and \$2,151,000 in 2014, 2013 and 2012, respectively. There are no other significant temporary differences. The net operating loss carry-forwards of approximately \$40,604,000 as of December 31, 2014 will expire at various dates beginning in 2025 and ending in 2034. 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, 2014 and 2013. No shares of preferred stock were outstanding at December 31, 2014 and 2013.

***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, 2014 and 2013, 69,119,092 and 54,132,937 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.

**Table of Contents**

**11. Stockholders Equity (continued)**

***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.
- (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.

F-19

**Table of Contents**

**11. Stockholders Equity (continued)**

***2014 Shelf Registration Statement***

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 shares of common stock. This registration statement (file No. 333-193699) was declared effective by the SEC on March 19, 2014. The Company has to date conducted the following sales of its securities under the 2014 Shelf Registration Statement:

- (a) On April 3, 2014, the Company filed a prospectus supplement and offered for sale 13,023,750 shares of its common stock at a price of \$2.21 per share in an underwritten public offering. The Company received gross proceeds in the public offering of approximately \$28.8 million before underwriting commission and incurred expenses of approximately \$2.1 million.
- (b) Subsequent to year end, on February 4, 2015, the Company filed a prospectus supplement and offered for sale 11,500,000 shares of its common stock at a price of \$3.25 per share in an underwritten public offering. The Company received gross proceeds in the public offering of approximately \$37.4 million before underwriting commission and incurred expenses of approximately \$2.7 million. (See Note 15).

Following the February 2015 offering, there is approximately \$33.8 million available for future sale under the 2014 Shelf Registration Statement. If the Company's public float (the market value of its common stock held by non-affiliate stockholders) falls below \$75 million, the Company will be subject to a further limitation under which it can sell no more than one-third (1/3) of its public float during any 12-month period. Further, the number of shares that the Company can sell at any one time may be limited under certain circumstances to 20% of the outstanding common stock under applicable NASDAQ marketplace rules.

***Warrant Exercises***

During the years ended December 31, 2014 and 2013, the Company issued an aggregate of 1,262,696 and 3,862,250 shares of its authorized but unissued common stock upon the exercise of previously issued common stock purchase warrants, raising gross proceeds of \$1,316,503 and \$4,083,300, respectively.

***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.

***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.





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**Table of Contents****11. Stockholders Equity (continued)**

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.

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 and 2014 Stock Incentive Plans (the 2006 Plan and the 2014 Plan or collectively, the Plans). Prior to July 2006, the Company granted options pursuant to written agreements to purchase an aggregate of 2,352,254 shares of common stock. At December 31, 2014, no shares remain available for future issuance under the 2006 Plan. 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 became effective upon stockholder approval of the 2014 Plan at the Company's 2014 Annual Meeting of Stockholders held on May 15, 2014. Under the Plan, 4,000,000 shares were reserved for issuance under the 2014 Plan and as of December 31, 2014, 2,640,000 shares remain available for future issuance under the 2014 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. Option 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, 2014, options to purchase 580,000 shares of the Company's common stock were exercised with proceeds of \$522,000. Further, during the year ended December 31, 2014, options to purchase 185,000 shares of the Company's common stock were exercised on a cashless basis, resulting in the issuance of an aggregate of 119,709 shares of the Company's common stock.

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 years ended December 31, 2014, 2013 and 2012 the Company recorded non-cash stock-based compensation expense related to stock options totaling \$767,838, \$175,855 and \$340,039, respectively.

**Table of Contents****12. Stock Compensation Plans (continued)**

During the year ended December 31, 2014, the Company granted five and seven-year options to purchase an aggregate of 1,305,000 shares of the Company's common stock to certain of the Company's officers, employees, directors, and consultants. During the years ended December 31, 2013 and 2012, the Company granted five-year options to purchase an aggregate of 115,000 shares and 975,000 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 Plans for the year ended December 31, 2014 is summarized as follows:

|                                  | Number of<br>Options | Weighted<br>Average<br>Exercise<br>Price | Weighted<br>Average<br>Remaining<br>Contractual<br>Term (in years) | Aggregate<br>Intrinsic<br>Value |
|----------------------------------|----------------------|--|--|---------------------------------|
| Outstanding at beginning of year | 3,428,906            | \$ 0.80                                  |  |                                 |
| Granted                          | 1,305,000            | 3.08                                     |  |                                 |
| Exercised                        | (765,000)            | 0.90                                     |  |                                 |
| Forfeited or cancelled           | (50,000)             | 2.71                                     |  |                                 |
| Expired                          | (34,296)             | 3.28                                     |  |                                 |
| Outstanding at end of year       | 3,884,610            | \$ 1.50                                  | 3.29   | \$ 5,897,040                    |
| Exercisable at end of year       | 2,687,943            | \$ 0.86                                  | 1.91   | \$ 5,703,106                    |

Other information pertaining to stock option activity during the years ended December 31, 2014, 2013 and 2012 was as follows:

|  | 2014         | 2013       | 2012       |
|--|--------------|------------|------------|
| Weighted average fair value of granted stock options | \$ 2.41      | \$ 0.48    | \$ 0.32    |
| Total fair value of vested stock options             | \$ 409,476   | \$ 166,633 | \$ 348,815 |
| Total intrinsic value of exercised stock options     | \$ 1,339,100 | \$ 17,975  | \$ 40,050  |

The following table summarizes information about the Company's options outstanding at December 31, 2014:

| Range of<br>Exercise<br>Prices | Options Outstanding   |   |  | Options Exercisable   |   |  |
|--------------------------------|-----------------------|---|--|-----------------------|---|--|
|                                | Number<br>Outstanding | Weighted<br>Average<br>Remaining<br>Contractual<br>Life | Weighted<br>Average<br>Exercise<br>Price | Number<br>Exercisable | Weighted<br>Average<br>Remaining<br>Contractual<br>Life | Weighted<br>Average<br>Exercise<br>Price |

|                |           | (Years) |         |           | (Years) |         |
|----------------|-----------|---------|---------|-----------|---------|---------|
| \$0.47         | 1,000,000 | 2.97    | \$ 0.47 | 950,000   | 2.95    | \$ 0.47 |
| \$0.69         | 729,610   | 0.17    | \$ 0.69 | 729,610   | 0.17    | \$ 0.69 |
| \$0.85         | 40,000    | 3.39    | \$ 0.85 | 13,333    | 3.39    | \$ 0.85 |
| \$1.07- \$1.09 | 860,000   | 1.48    | \$ 1.08 | 860,000   | 1.48    | \$ 1.08 |
| \$2.34- \$2.35 | 30,000    | 4.39    | \$ 2.35 | 10,000    | 4.24    | \$ 2.34 |
| \$3.03- \$3.12 | 1,225,000 | 6.67    | \$ 3.12 | 125,000   | 6.66    | \$ 3.12 |
|                | 3,884,610 | 3.29    | \$ 1.50 | 2,687,943 | 1.91    | \$ 0.86 |

As of December 31, 2014, there was approximately \$2,400,000 of unrecognized compensation expense related to non-vested stock option awards granted under the Plans. That cost is expected to be recognized over a weighted average period of approximately 2.5 years.

**Table of Contents****12. Stock Compensation Plans (continued)**

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, |                |                |
|--------------------------|-------------------------|----------------|----------------|
|                          | 2014                    | 2013           | 2012           |
| Risk free interest rate  | 1.18% to 2.03%          | 0.45% to 0.53% | 0.28% to 0.66% |
| Expected term            | 3 to 7 years            | 3 years        | 3 to 5 years   |
| Expected volatility      | 115%                    | 137%           | 120%           |
| Expected dividend yield  | %                       | %              | %              |
| Expected forfeiture rate | %                       | %              | %              |

**Restricted Stock Units**

Under the 2014 Plan, participants may be granted restricted stock units, each of which represents a conditional right to receive shares of common stock in the future. The restricted stock units granted under this plan generally vest ratably over a three to four-year period. Upon vesting, the restricted stock units will convert into an equivalent number of shares of common stock. The amount of expense relating to the restricted stock units is based on the closing market price of the Company's common stock on the date of grant and is amortized on a straight-line basis over the requisite service period. There was no restricted stock unit activity during 2013 or 2012. Restricted stock unit activity during 2014 was as follows:

|  | 2014                             |  |
|--|----------------------------------|--|
|  | Number of Restricted Stock Units | Weighted Average Grant Date Fair Value |
| Nonvested balance at beginning of year |                                  |  |
| Granted                                | 80,000                           | \$ 2.83                                |
| Vested                                 |                                  |  |

Forfeited

|                                  |        |    |      |
|----------------------------------|--------|----|------|
| Nonvested balance at end of year | 80,000 | \$ | 2.83 |
|----------------------------------|--------|----|------|

During the years ended December 31, 2014, 2013 and 2012, the Company recorded non-cash stock-based compensation expense related to restricted stock units totaling \$10,131, \$0 and \$0, respectively.

F-23

**Table of Contents****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, 2014, 2013 and 2012, the Company's matching contributions were approximately \$44,000, \$30,000 and \$28,000, respectively.

**14. Quarterly Financial Information (unaudited)**

The following table presents unaudited supplemental quarterly financial information for the years ended December 31, 2014 and 2013:

|  | Quarter Ended     |                  |                       |                      |
|--|-------------------|------------------|-----------------------|----------------------|
|  | March 31,<br>2014 | June 30,<br>2014 | September 30,<br>2014 | December 31,<br>2014 |
| Revenues                                   | \$                | \$               | \$                    | \$                   |
| Loss from operations                       | (3,508,365)       | (2,990,173)      | (4,109,029)           | (3,983,861)          |
| Change in fair value of warrants liability | (335,514)         | (223,591)        | (906,787)             | 472,026              |
| Net loss                                   | (3,811,119)       | (3,198,020)      | (5,009,892)           | (3,490,030)          |
| Loss per share basic and diluted           | \$ (0.07)         | \$ (0.05)        | \$ (0.07)             | \$ (0.05)            |

|  | Quarter Ended     |                  |                       |                      |
|--|-------------------|------------------|-----------------------|----------------------|
|  | March 31,<br>2013 | June 30,<br>2013 | September 30,<br>2013 | December 31,<br>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)            |

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 February 4, 2015, the Company filed a prospectus supplement and offered for sale 11,500,000 shares of its common stock at a price of \$3.25 per share in an underwritten public offering. The Company received gross proceeds in the public offering of approximately \$37.4 million before underwriting commission and incurred expenses of approximately \$2.7 million. (See Note 11).

Subsequent to year-end, the Company issued an aggregate of 152,174 shares of its authorized but unissued common stock upon the exercise of previously issued common stock purchase warrants that were issued in October 2011, raising gross proceeds of approximately \$198,000. Additionally, subsequent to year end, stock options to purchase



829,608 shares of the Company's common stock were exercised on a cashless basis, resulting in the issuance of an aggregate of 673,583 shares of the Company's common stock.

F-24