CATALYST PHARMACEUTICAL PARTNERS, INC.
Form 424B5
April 02, 2014
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The information in this prospectus supplement and the accompanying prospectus is not complete and may be changed. This prospectus supplement and the accompanying prospectus are not an offer to sell these securities, nor are they soliciting offers to buy these securities, in any jurisdiction where the offer or sale is not permitted.

Subject to completion, dated April 2, 2014

PROSPECTUS SUPPLEMENT
(To Prospectus dated March 19, 2014)

Filed Pursuant to Rule 424(b)(5)
Registration No. 333-193699

Shares

CATALYST PHARMACEUTICAL

PARTNERS, INC.

Common Stock

$ Per Share

Catalyst Pharmaceutical Partners, Inc. is offering shares of its common stock.

Trading symbol: Nasdaq Capital Market - CPRX

The last reported sale price of our common stock on April 1, 2014 was $2.32 per share.

This investment involves risks. See Risk Factors beginning on page 25 of our Annual Report on Form 10-K for the year ended December 31, 2013, page S-4 of this prospectus supplement and on page 3 of the accompanying prospectus.

	Per Share	Total
Public Offering Price	$	$
Underwriting Discount[(1)]	$	$
Proceeds, before expenses, to us	$	$

(1) We have also agreed to reimburse the underwriters for certain of its expenses. See Underwriting on page S-22 of this prospectus supplement for more information about these arrangements.

We have granted the underwriters an option, for a period of 30 days from the date of this prospectus supplement, to purchase up to an additional shares of common stock to cover over-allotments, if any. If the underwriters exercise the option in full, the total underwriting discount payable by us will be $, and the total proceeds to us, before expenses, will be $.

We expect to deliver the shares against payment on or about , 2014.

Neither the Securities and Exchange Commission nor any state securities commission or other regulatory body has approved or disapproved of these securities, or determined if this prospectus supplement or the accompanying prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

Piper Jaffray

Sole Book-Running Manager

The date of this prospectus supplement is April , 2014

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This document is in two parts. The first part is this prospectus supplement, which describes the terms of the offering of the securities offered hereby and also adds to and updates the information contained in the accompanying prospectus and the documents incorporated by reference into this prospectus supplement and the accompanying prospectus. The second part is the accompanying prospectus, which provides more general information. To the extent that there is any conflict between the information contained in this prospectus supplement, on the one hand, and the information contained in the accompanying prospectus or any document incorporated by reference herein or therein, on the other hand, you should rely on the information in this prospectus supplement.

You should rely only on the information contained in this prospectus supplement, contained in the accompanying prospectus or incorporated herein or therein by reference. We have not authorized anyone to provide you with information that is different. We are offering to sell, and seeking offers to buy, the securities offered hereby only in jurisdictions where offers and sales are permitted. The information contained, or incorporated by reference, in this prospectus supplement and contained, or incorporated by reference, in the accompanying prospectus is accurate only as of the respective dates thereof, regardless of the time of delivery of this prospectus supplement and the

accompanying prospectus, or of any sale of our common stock. It is important for you to read and consider all information contained in this prospectus supplement and the accompanying prospectus, including the documents we have referred you to in the section entitled Where You Can Find Additional Information below.

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

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SUMMARY

This summary highlights information contained else where in this prospectus supplement and in the accompanying prospectus and in the documents we incorporate by reference. After you read this summary, you should read and consider carefully the more detailed information and financial statements and related notes that we include in and/or incorporate by reference into this prospectus supplement and the accompanying prospectus, especially the section entitled Risk Factors. If you invest in the shares, you are assuming a high degree of risk. it does not contain all of the information you should consider before investing. You should carefully read the entire prospectus before making an investment decision. Throughout this prospectus, the terms we , us , our and company refer to Catalyst Pharmaceutical Partners, Inc.

Our Business

We are a development-stage specialty pharmaceutical company focused on the development and commercialization of novel prescription drugs targeting rare (orphan) neuromuscular and neurological diseases and disorders. We have three pharmaceutical products in development:

Firdapse

In October 2012, we licensed the North American rights to Firdapse , a proprietary form of amifampridine phosphate, or chemically known as 3,4-diaminopyridine phosphate, from BioMarin Pharmaceutical Inc. (BioMarin). As part of our agreements with BioMarin, we have taken over the sponsorship of an ongoing Phase 3 clinical trial evaluating Firdapse for the treatment of Lambert-Eaton Myasthenic Syndrome, or LEMS, a rare and sometimes fatal autoimmune disease characterized by muscle weakness. We also hope to evaluate Firdapse for the treatment of other neuromuscular orphan indications such as certain forms of Congenital Myasthenic Syndrome and Myasthenia Gravis. In August 2013, we were granted breakthrough therapy designation by the U.S. Food & Drug Administration (FDA) for Firdapse for the treatment of LEMS.

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

The Phase 3 trial is designed as a randomized, double-blind, placebo-controlled discontinuation study followed by an open-label extension period in approximately 36-patients across 22 sites in the United States, Canada, South America and Europe. After enrolled patients have been treated with Firdapse for at least 91 days, they are randomly assigned to either continue on Firdapse or be discontinued to placebo over a 2-week period. Following the randomization phase of the trial, patients then receive open label Firdapse treatment for a two-year follow-up period, to obtain additional long term safety and efficacy data.

The co-primary endpoints of the Phase 3 trial are comparisons of changes in patients randomized to continue Firdapse versus those who transition to placebo that occur in both the QMG score, which measures muscle strength, and subject global impression score, on which the subject rates their global impression of the effects of a study treatment during a 14-day double-blind efficacy evaluation period. The secondary endpoints are change in the investigator s assessment of worsening of disease symptoms and changes in walking speed (Timed 25-foot walking test) during the two-week, double-blind testing period. Further details regarding the Phase 3 trial and its design can be found on

www.clinicaltrials.gov (NCT01377922).

We recently reported that, based on enrollment and randomization success metrics achieved to date, we believe that we have enrolled a sufficient number of LEMS patients to ensure that 36 patients will be randomized into the double-blind, placebo-controlled discontinuation portion of the trial. Based on currently available information, we expect that we will report top-line results from the double-blind portion of this Phase 3 trial during the third quarter of 2014 (and, if the trial results are successful, we expect to submit to the FDA, on a rolling basis, all of the modules required to complete a new drug application (NDA) by the middle of 2015).

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CPP-115

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

CPP-109

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

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

Recently Filed Securities Class Action Lawsuit

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

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

Company Information

Our principal executive offices are located at 355 Alhambra Circle, Suite 1500, Coral Gables, Florida 33134, and our telephone number at that address is (305) 529-2522. Our website is located on the world wide web at

http://www.catalystpharma.com. We do not incorporate by reference into this prospectus supplement or the accompanying prospectus the information on, or accessible through, our website, and you should not consider it as part of this prospectus supplement or the accompanying prospectus.

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THE OFFERING

Common stock offered	Shares
Underwriters Overallotment Option	We have granted the underwriters an option to purchase additional Shares to cover over-allotments, if any.
Common stock to be outstanding after this offering	Shares
Use of proceeds	We intend to use the net proceeds from the sale of the securities: (i) to fund our product development efforts for Firdapse and CPP-115, and (ii) to fund our pre-commercialization activities for Firdapse, and (iii) for general corporate purposes. See Use of Proceeds on page S-20 for additional information.
Risk Factors	See Risk Factors beginning on page 25 of our Annual Report on Form 10-K for the year ended December 31, 2013, on page S-4 of this prospectus supplement and in the documents incorporated by reference in this prospectus supplement and the accompanying prospectus for a discussion of factors you should consider carefully before deciding to invest in our common stock.
NASDAQ Capital Market symbol	CPRX

The number of shares of our common stock to be outstanding after this offering as shown above is based on 54,145,633 shares outstanding as of April 2, 2014 and excludes:

- 2,697,296 shares of our common stock subject to outstanding options under our 2006 Stock Incentive Plan having a weighted average exercise price of $0.82 per share;
- 729,610 shares of our common stock subject to outstanding options outside of our 2006 Stock Incentive Plan having a weighted average exercise price of $0.69 per share;
- 192,604 shares of our common stock that have been reserved for issuance in connection with our 2006 Stock Incentive Plan;
- 1,242,174 shares of our common stock that have been reserved for issuance upon exercise of outstanding warrants at an exercise price of $1.30 per share;

2,393,750 shares of our common stock that have been reserved for issuance upon exercise of outstanding warrants at an exercise price of $1.04 per share; and

1,200,000 shares of our common stock that have been reserved for issuance upon exercise of outstanding warrants at an exercise price of $2.08 per share.

Unless otherwise indicated, all information in this prospectus supplement assumes no exercise by the underwriters of their overallotment option.

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RISK FACTORS

Before you make a decision to invest in our common stock, you should consider carefully the risks described below, together with other information in this prospectus supplement, the accompanying prospectus and the information incorporated by reference herein and therein. If any of the following events actually occur, our business, operating results, prospects or financial condition could be materially and adversely affected. This could cause the trading price of our common stock to decline and you may lose all or part of your investment. The risks described below are not the only ones that we face. Additional risks not presently known to us or that we currently deem immaterial may also significantly impair our business operations and could result in a complete loss of your investment.

See Risk Factors beginning on page 25 of our Annual Report on Form 10-K for the year ended December 31, 2013 and on page 3 of the accompanying prospectus, which are incorporated herein by reference.

Risks Related to this Offering

Management will have broad discretion to use the proceeds from this offering, and we may not use the proceeds effectively.

We have not designated any portion of the net proceeds from this offering to be used for any particular purpose. Accordingly, our management will have broad discretion as to the application of the net proceeds from this offering, and could spend the proceeds in ways that do not necessarily improve our operating results or enhance the value of our common stock.

You will experience immediate dilution in the book value per share of the common stock you purchase.

Because the price per share of our common stock being offered is substantially higher than the book value per share of our common stock, you will suffer substantial dilution in the net tangible book value of the common stock you purchase in this offering. After giving effect to the sale by us of shares of common stock in this offering, based on a public offering price of $ per share in this offering and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us and based on a net tangible book value of our common stock of $21.4 million, or $0.40 per share, as of December 31, 2013, if you purchase shares of common stock in this offering, you will suffer immediate and substantial dilution of $ per share in the net tangible book value of the common stock. See Dilution on page S 21 for a more detailed discussion of the dilution you will incur in connection with this offering.

Risks Related to Our Business

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

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

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

We have had no revenues from product sales to date, currently have no products available for commercial sale, and have never had any products available for commercial sale. We expect to incur losses at least until we are in a position to commercialize Firdapse , which may never occur. Our net loss was $12.2 million for the year ended December 31, 2013, and as of December 31, 2013 we had a deficit accumulated during the development stage of $54.3 million. We may never obtain approval of an NDA for any of our product candidates and we may never achieve profitability.

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Our business will require additional capital.

Our business will require additional capital to meet our product development objectives. Based on currently available information, we estimate that we have sufficient working capital to support our planned operations through at least the end of 2014. The expectations described above are based on current information available to us. If the cost of our ongoing studies are greater than we expect, our assumptions may not prove to be accurate. There can be no assurance as to the exact amount of the funding we will require or as to whether any such required funding will be available to us when it is required.

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

Any sale by us of additional equity or convertible debt securities could result in dilution to our stockholders. There can be no assurance that any required additional funding will be available to us at all or available on terms acceptable to us. Further, to the extent that we raise funds through collaborative arrangements, it may be necessary to relinquish some rights to our technologies or grant sublicenses on terms that are not favorable to us. If we are not able to secure funding when needed, we may have to delay, reduce the scope of or eliminate one or more research and development programs, which could have an adverse effect on our business.

If we are not the first to get approval for Firdapse for the treatment of LEMS, we may not be able to bring it to market; Even if we obtain marketing exclusivity, it may not prevent others from competing with us.

In January of 2012, another pharmaceutical company, Jacobus Pharmaceutical, began its own Phase 2 trial studying their own formulation of amifampridine (3,4-DAP) for the treatment of LEMS. While there can be no assurance, based on currently available information, we believe that Firdapse is further along in development than this other company s development program. Under the Orphan Drug Act of 1983, the first pharmaceutical product to 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 3,4-DAP for any indication prior to approval of Firdapse , we would be barred from marketing Firdapse in the United States during this five-year exclusivity period.

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

Our development of CPP-115 is at an early stage, and it is going to be several years before we are in a position to file an NDA for CPP-115, if our future clinical trials of this product are successful. Further, our ability to develop CPP-115 will be dependent on our having the resources to conduct the studies and trials that would be required. There can be no assurance that we will ever file an NDA for CPP-115 or commercialize CPP-115.

Our business is subject to substantial competition.

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

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For example, amifampridine, the active ingredient in Firdapse , has been available from compounding pharmacies and from Jacobus Pharmaceutical under compassionate use INDs for many years and will likely be available from these sources even if we are able to obtain FDA approval of Firdapse . Amifampridine from these sources is likely to be substantially less expensive than Firdapse . The FDA Pharmacy Compounding Advisory Committee, however, has previously issued a list of drugs which should not be compounded, and amifampridine was included on that list. Further, drugs that are not approved by FDA for the treatment of LEMS, such as dalfampridine (Ampyra®), may nonetheless be prescribed by physicians for the treatment of LEMS.

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

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

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

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

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

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

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

We are highly dependent on our officers, on our Board of Directors and on our scientific advisors. The loss of the services of any of these individuals could significantly impede the achievement of our scientific and business objectives. Other than an employment agreement with Patrick J. McEnany, our Chairman, President and Chief

Executive Officer with respect to his services, and the consulting agreements that we have with our Chief Medical Officer and with several of our scientific advisors, we have no employment or retention agreements with our officers, directors or scientific advisors. If we lose the services of any of our existing officers, directors or scientific advisors, or if we were unable to recruit qualified replacements on a timely basis for persons who leave our employ, our efforts to develop our product candidates might be significantly delayed. We do not carry key-man insurance on any of our personnel.

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

Risks Related to the Development of Our Drug Candidates

Our product development efforts may fail.

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

- Our product candidates may be found to be ineffective or unsafe, or fail to receive necessary regulatory approvals;
- Our product candidates may not be economical to market or take substantially longer to obtain necessary regulatory approvals than anticipated; or
- Competitors may market equivalent or superior products.

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

Failure can occur at any stage of our product development efforts.

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

Any manufacturing problem, natural disaster affecting manufacturing facilities, or the loss of a contract manufacturer could be disruptive to our operations and result in lost sales. Additionally, we will be reliant on third parties to supply the raw materials needed to manufacture our potential products. Any reliance on suppliers may involve several risks,

including a potential inability to obtain critical materials and reduced control over production costs, delivery schedules, reliability and quality. Any unanticipated disruption to future contract manufacture caused by problems at suppliers could delay shipment of products, increase our cost of goods sold and result in lost sales. If our suppliers were to be unable to supply us with adequate supply of our product candidates, it could have a material adverse effect on our ability to commercialize our product candidates.

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

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

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

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

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

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

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

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

Our internal computer systems and those of our contract research organization and other key vendors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our programs. For example, the loss of clinical trial data from completed or ongoing clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development of our product candidates could be delayed.

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

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

Risks Related to Government Regulation

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

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

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

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

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

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

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We are in the process of conducting a Phase 3 clinical trial for Firdapse and we are currently planning to commence (during the first half of 2014) a Phase 1(b) clinical trial for CPP-115. Even if the results of our clinical trials are promising, Firdapse or CPP-115 may subsequently fail to meet the safety and efficacy standards required to obtain regulatory approvals. Future clinical trials for Firdapse or CPP-115 may not be successfully completed or may take longer than anticipated because of any number of factors, including potential delays in the start of the trial, an inability to recruit clinical trial participants at the expected rate, failure to demonstrate safety and efficacy, unforeseen safety issues, or unforeseen governmental or regulatory delays.

Any clinical trials we might develop and implement, may not be completed in a timely manner or at all. Our product candidates may not be found to be safe and effective, and may not be approved by regulatory authorities for the proposed indication. Further, regulatory authorities and IRBs that must approve and monitor the safety of each clinical study may suspend a clinical study at any time if the patients participating in such study are deemed to be exposed to any unacceptable health risk. We may also choose to suspend human clinical studies and trials if we become aware of any such risks. We might encounter problems in our clinical trials, including 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 product candidates. Further, unrelated third parties and investigators in the academic community have expressed interest in testing our product candidates. If these third-party tests are unsuccessful, or if they show significant health risk to the test subjects, our development efforts may also be adversely affected.

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

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

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reliance on the continued financial viability of the third parties;

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

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

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

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

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

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

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

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

Even if we receive regulatory approval of any drugs we are developing or may develop, we will be subject to continuing regulatory review, including the review of clinical results which are reported after 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 may 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.

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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.

Risks Related to Our Intellectual Property

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

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

All of our patent rights for CPP-115 are derived from our license agreement with Northwestern University. Pursuant to this license agreement, we have exclusive worldwide rights to two patents in the United States. These were filed and obtained by Northwestern relating to compositions of matter for a class of molecules, including CPP-115. Both patents expire in 2023. Additionally, we have licensed rights from Northwestern to a pending patent for derivatives of vigabatrin that are unrelated to CPP-115. These rights are subject to the right of Northwestern, under limited circumstances, to practice the covered inventions for or on its own behalf for research. We may lose our rights to these patents and patent applications if we breach our obligations under the license agreement, including, without limitation, our financial obligations, including milestone payments, to Northwestern. If we violate or fail to perform any term or covenant of the license agreement, Northwestern may terminate the license agreement upon satisfaction of any applicable notice requirements and expiration of any applicable cure periods. Additionally, any termination of the license agreement, whether by us or by Northwestern, will not relieve us of our obligation to pay any license fees owing at the time of such termination. If we fail to retain our rights under the license agreement, we would not be able to commercialize CPP-115, and our business, results of operations, financial condition and prospects would be materially adversely affected.

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

If we obtain approval to market Firdapse , CPP-115 or CPP-109, our commercial success will depend 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.

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

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

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

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

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

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

- we may have to redesign our product so that it does not infringe others patent rights, which may not be possible or could require substantial funds or time and require additional studies.

In addition, employees, consultants, contractors and others may use the proprietary information of others in their work for us or disclose our proprietary information to others. As an example, we do not currently have written agreements regarding confidentiality or any other matters with several principal members of our Scientific Advisory Board. If our employees, consultants, contractors or others disclose our data to others or use data belonging to others in connection with our business, it could lead to disputes over the ownership of inventions derived from that information or expose us to potential damages or other penalties.

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

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.

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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 could be highly volatile.

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

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

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

intellectual property, product liability or other litigation against us;

changes in the market valuations of similar companies;

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

changes in economic conditions; and

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

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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 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.

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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 April [*], 2014 we had 54,145,633 shares of our common stock outstanding, of which 5,750,609 shares were held by our officers and directors. We also had outstanding: (i) common stock purchase warrants to purchase an aggregate of 4,835,924 additional shares of our common stock at exercise prices ranging from $1.04 to $2.08 per share, and (ii) stock options to purchase an aggregate of 3,426,906 shares at exercise prices ranging from $0.47 to $6.00 per share (3,086,905 of which are currently exercisable). Sales of restricted shares or shares underlying stock options and common stock purchase warrants, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock.

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

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

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

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

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CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS

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

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

- the scope, rate of progress and expense of our clinical trials and studies, pre-clinical studies, proof-of-concept studies, and our other product development activities;
- our ability to complete our trials and studies on a timely basis and within the budgets we establish for such trials and studies;
- whether historic metrics of patients enrolled in our Phase 3 trial who complete the run-in phase of the trial and are randomized into the double-blind, placebo-controlled portion of the trial will continue to apply, such that at least 36 patients will be randomized into the double-blind, placebo-controlled portion of the trial from the patients already enrolled in the trial;
- the accuracy of our expectations as to the anticipated timing of the receipt of top-line results from the double-blind, placebo-controlled portion of the trial;
- whether the receipt of breakthrough therapy designation will expedite the development and review of Firdapse by the FDA or the likelihood that the product will be found to be safe and effective;
- the ability of our third-party suppliers and contract manufacturers to maintain compliance with current Good Manufacturing Practices (cGMP);
- whether our trials and studies will be successful;

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

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

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

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

whether others develop and commercialize products competitive to our products;

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

changes in the laws and regulations affecting our business;

the impact of the class action lawsuit filed against us;

our ability to attract and retain skilled employees;

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

the impact of employee or consultant misconduct; and

changes in general economic conditions and interest rates.

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

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USE OF PROCEEDS

We estimate that the net proceeds from the sale of the shares of common stock we are offering will be approximately $ million, after deducting underwriting fees and estimated offering expenses payable by us, or approximately $ million if the underwriters exercise their over-allotment option in full. We expect to use the net proceeds from this offering: (i) to fund our continuing product development efforts for Firdapse and CPP-115, (ii) to fund pre-commercialization activities for Firdapse, and (iii) for general corporate purposes.

As of the date of this prospectus supplement, we cannot specify with certainty the particular uses of the proceeds from this offering. As a result, our management will retain broad discretion in the allocation and use of the net proceeds from this offering. Pending the application of the net proceeds for these purposes, we expect to invest the proceeds in short-term, interest-bearing instruments or other investment-grade securities.

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DILUTION

The net tangible book value of our common stock as of December 31, 2013 was approximately $21.4 million, or $0.40 per share. Net tangible book value per share of our common stock is equal to our net tangible assets (tangible assets less total liabilities) divided by the number of shares of our common stock issued and outstanding as of December 31, 2013.

Dilution in net tangible book value per share represents the difference between the public offering price per share of our common stock and the adjusted net tangible book value per share of our common stock after giving effect to this offering. After giving effect to the sale of shares of our common stock in this offering at the public offering price of $ per share, and after deducting the underwriters fees and estimated offering expenses payable by us, our adjusted net tangible book value per share of our common stock at December 31, 2013, would have been approximately $ million, or $ per share. This represents an immediate increase in net tangible book value per share of our common stock of approximately $ per share to existing stockholders and an immediate dilution of approximately $ per share to purchasers in this offering. The following table illustrates this per-share dilution:

Public offering price per share	$
Net tangible book value per share as of December 31, 2013,	$ 0.40
Increase per share attributable to this offering	$
As adjusted net tangible book value per share as of December 31, 2013	$
Dilution per share to new investors	$

The above table is based on 54,132,937 shares outstanding as of December 31, 2013 and excludes, as of that date:

- 2,699,296 shares of our common stock subject to outstanding options under our 2006 Stock Incentive Plan having a weighted average exercise price of $0.83 per share;
- 729,610 shares of our common stock subject to outstanding options outside of our 2006 Stock Incentive Plan having a weighted average exercise price of $0.69 per share;
- 217,604 shares of our common stock that have been reserved for issuance in connection with our 2006 Stock Incentive Plan;
- 1,254,870 shares of our common stock that have been reserved for issuance upon exercise of outstanding warrants at an exercise price of $1.30 per share;

2,393,750 shares of our common stock that have been reserved for issuance upon exercise of outstanding warrants at an exercise price of $1.04 per share; and

1,200,000 shares of our common stock that have been reserved for issuance upon exercise of outstanding warrants at an exercise price of $2.08 per share.

Subsequent to December 31, 2013, common stock purchase warrants to purchase an aggregate of 12,696 shares of our common stock at an exercise price of $1.30 per share, resulting in gross proceeds of approximately $16,500 and stock options to purchase 25,000 shares of our common stock at an exercise price of $2.30 per share were granted.

To the extent that any outstanding options or warrants are exercised, new options are issued under our 2006 Stock Incentive Plan, or we otherwise issue additional shares of common stock in the future, at a price less than the public offering price, there will be further dilution to new investors.

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UNDERWRITING

We are offering the shares of common stock described in this prospectus supplement through Piper Jaffray & Co. as the sole book running manager. We have entered into a firm commitment underwriting agreement with Piper Jaffray, as representative of the several underwriters. Subject to the terms and conditions of the underwriting agreement, we have agreed to sell to the underwriters, and the underwriters have agreed to purchase from us, the number of shares of common stock listed opposite their names below. The underwriters are committed to purchase and pay for all the shares of common stock if any are purchased.

Underwriter	Number of Shares
Piper Jaffray & Co.	
Total	

The underwriters have advised us that they propose to offer the common stock directly to the public at the offering price set forth on the cover page of this prospectus supplement. The underwriters propose to offer the shares to certain dealers at the same price less a concession of not more than $ per share. After the offering, these figures may be changed by the underwriters.

We have granted the underwriters an option to buy up to additional shares of common stock from us to cover over allotments, if any. The underwriters may exercise this option at any time and from time to time during the 30 day period from the date of this prospectus supplement. If any additional shares of common stock are purchased, the underwriters will offer the additional shares on the same terms as those on which the shares are being offered.

The underwriting fee is equal to the public offering price per share of common stock less the amount paid by the underwriters to us per share of common stock. The following table shows the per share and total underwriting discount to be paid by the underwriters in this offering assuming both no exercise and full exercise of the underwriters option to purchase additional shares:

	With no Over-Allotment	With Over-Allotment
Per Share	$	$
Total	$	$

We estimate that the total fees and expenses payable by us, excluding underwriting discount, will be approximately $, which includes $ that we have agreed to reimburse the underwriters for the fees incurred by it in connection with the offering.

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act, or to contribute to payments that the underwriters may be required to make in respect of those liabilities.

We and each of our directors and executive officers are subject to lock up agreements that prohibit us and them from offering for sale, pledging, assigning, encumbering, announcing the intention to sell, selling, contracting to sell, granting any option, right or warrant to purchase, or otherwise transferring or disposing of, any shares of our common stock or any securities convertible into or exercisable or exchangeable for shares of our common stock for a period of

at least 90 days following the date of this prospectus supplement without the prior written consent of Piper Jaffray. The lock up agreements do not prohibit our directors and executive officers from transferring shares of our common stock for bona fide estate or tax planning purposes, subject to certain requirements, including that the transferee be subject to the same lock up terms.

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The lock up provisions do not prohibit us from issuing shares upon the exercise or conversion of securities outstanding on the date of this prospectus supplement. The lock up provisions do not prevent us from selling shares to the underwriters pursuant to the underwriting agreement, or from granting options to acquire securities under our existing stock option plans or issuing shares upon the exercise or conversion of securities outstanding on the date of this prospectus supplement.

The 90 day lock up period in all of the lock up agreements is subject to extension if (i) during the last 17 days of the lock up period we issue an earnings release or material news or a material event relating to us occurs or (ii) prior to the expiration of the lock up period, we announce that we will release earnings results during the 16 day period beginning on the last day of the lock up period, in which case the restrictions imposed in these lock up agreements shall continue to apply until the expiration of the 18 day period beginning on the issuance of the earnings release or the occurrence of the material news or material event, unless Piper Jaffray waives the extension in writing.

Our shares are quoted on the Nasdaq Capital Market under the symbol CPRX.

To facilitate the offering, the underwriters may engage in transactions that stabilize, maintain or otherwise affect the price of our common stock during and after the offering. Specifically, the underwriters may over allot or otherwise create a short position in the common stock for its own account by selling more shares of common stock than we have sold to it. Short sales involve the sale by the underwriters of a greater number of shares than the underwriters are required to purchase in the offering. The underwriters may close out any short position by either exercising its option to purchase additional shares or purchasing shares in the open market.

In addition, the underwriters may stabilize or maintain the price of the common stock by bidding for or purchasing shares of common stock in the open market and may impose penalty bids. If penalty bids are imposed, selling concessions allowed to syndicate members or other broker dealers participating in the offering are reclaimed if shares of common stock previously distributed in the offering are repurchased, whether in connection with stabilization transactions or otherwise. The effect of these transactions may be to stabilize or maintain the market price of the common stock at a level above that which might otherwise prevail in the open market. The imposition of a penalty bid may also affect the price of the common stock to the extent that it discourages resales of the common stock. The magnitude or effect of any stabilization or other transactions is uncertain. These transactions may be effected on the Nasdaq Capital Market or otherwise and, if commenced, may be discontinued at any time. The underwriters may also engage in passive market making transactions in our common stock. Passive market making consists of displaying bids on the Nasdaq Capital Market is limited by the prices of independent market makers and effecting purchases limited by those prices in response to order flow. Rule 103 of Regulation M promulgated by the Commission limits the amount of net purchases that each passive market maker may make and the displayed size of each bid. Passive market making may stabilize the market price of the common stock at a level above that which might otherwise prevail in the open market and, if commenced, may be discontinued at any time.

This prospectus supplement and the accompanying prospectus in electronic format may be made available on the web sites maintained by the underwriters and the underwriters may distribute prospectuses and prospectus supplements electronically.

From time to time in the ordinary course of its businesses, the underwriters and certain of their affiliates have engaged, and may in the future engage, in commercial banking or investment banking transactions with us and our affiliates.

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LEGAL MATTERS

The validity of the shares of common stock that we are offering hereby will be passed upon by Akerman LLP, Miami, Florida. Goodwin Procter LLP, New York, New York, is acting as counsel for the underwriters in connection with this offering.

EXPERTS

The audited financial statements incorporated by reference in this prospectus supplement and the accompanying prospectus have been so incorporated by reference in reliance upon the report of Grant Thornton LLP, independent registered public accountants, upon the authority of said firm as experts in accounting and auditing in giving said report, which is also incorporated by reference in this prospectus supplement and the accompanying prospectus.

WHERE YOU CAN FIND ADDITIONAL INFORMATION

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

This prospectus supplement and the accompanying prospectus do not contain all of the information included in the registration statement. We have omitted certain parts of the registration statement in accordance with the rules and regulations of the SEC. For further information, we refer you to the registration statement, including its exhibits and schedules. Statements contained in this prospectus supplement and any accompanying prospectus supplement about the provisions or contents of any contract, agreement or any other document referred to are not necessarily complete. Please refer to the actual exhibit for a more complete description of the matters involved.

INCORPORATION BY REFERENCE

The SEC allows us to incorporate by reference information into this prospectus supplement, which means that we can disclose important information to you by referring you to another document filed separately with the SEC. The information incorporated by reference is deemed to be a part of this prospectus supplement, except for any information superseded by information in any amendment to this prospectus supplement.

The following documents filed with the SEC are incorporated by reference in this prospectus supplement:

- our Annual Report on Form 10-K for the year ended December 31, 2013, filed with the SEC on March 18, 2014;

- our Current Reports on Form 8-K (or amendments thereto) filed with the SEC on January 8, 2014, February 20, 2014, March 4, 2014, March 20, 2014, March 25, 2014, March 28, 2014 and April 1, 2014;

our description of our common stock contained in our Registration Statement on Form 8-A, filed with the SEC on September 29, 2006, along with Amendment No. 1 thereto, filed with the SEC on October 18, 2006; and

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

You may obtain a copy of any of these documents at no cost by requesting them from us or by writing or calling: Catalyst Pharmaceutical Partners, Inc., 355 Alhambra Circle, Suite 1500, Coral Gables, Florida, 33134, Attn: Investor Relations, or by calling (305) 529-2522. Copies of each of these filings are also available for no cost on our website, www.catalystpharma.com , or on the SEC s web site, www.sec.gov .

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Prospectus

$100,000,000

We may, from time to time in one or more offerings, offer and sell up to $100,000,000, in the aggregate, of shares of our common stock.

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

Our common stock is listed on The NASDAQ Capital Market under the symbol CPRX. On March 14, 2014, the last reported sale price on The NASDAQ Capital Market was $2.17 per share. As of March 14, 2014, the aggregate market value of our outstanding common stock held by non-affiliates was $105.0 million, based on 48,395,024 shares of outstanding common stock held by non-affiliates and a price of $2.17 per share, which was the last reported sale price of our common stock on The NASDAQ Capital Market on March 14, 2014. During the prior 12 calendar months that ends on and includes the date of this Prospectus, we offered $15.1 million of securities pursuant to General Instruction I.B.6. of Form S-3.

Our business and investing in shares of our common stock involves significant risks. You should carefully read and consider the *Risk Factors* beginning on page 3 of this prospectus before investing.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed upon the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense.

The date of this prospectus is March 19, 2014

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ABOUT THIS PROSPECTUS

This prospectus is part of a registration statement that we have filed with the Securities and Exchange Commission, or SEC, using the shelf registration process. Under this shelf registration statement, we may sell shares of our common stock. All such offerings will not exceed, in the aggregate, a total dollar amount of $100,000,000. If our public float (the market value of the common stock held by our non-affiliate stockholders) falls below $75 million, we will also be subject to a further limitation under which we can sell no more than one third (1/3) of our public float during any 12-month period. Further, the number of shares that we can sell at any one time may be limited to 20% of our outstanding common stock under applicable NASDAQ Marketplace Rules. Finally, as permitted by the rules and regulations of the SEC, the registration statement filed by us includes additional information not contained in this prospectus. You may read the registration statement and the other reports we file with the SEC at the SEC s website or its offices described below under the heading Where You Can Find Additional Information .

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

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

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SUMMARY

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

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

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

Our Business

We are a development-stage specialty pharmaceutical company focused on the development and commercialization of novel prescription drugs targeting rare (orphan) neuromuscular and neurological diseases and disorders. We have three pharmaceutical products in development:

Firdapse

In October 2012, we licensed the North American rights to Firdapse , a proprietary form of amifampridine phosphate, or chemically known as 3,4-diaminopyridine phosphate, from BioMarin Pharmaceutical Inc. (BioMarin). As part of our agreements with BioMarin, we have taken over the sponsorship of an ongoing Phase 3 clinical trial evaluating Firdapse for the treatment of Lambert-Eaton Myasthenic Syndrome, or LEMS, a rare and sometimes fatal autoimmune disease characterized by muscle weakness. We also hope to evaluate Firdapse for the treatment of other neuromuscular orphan indications such as certain forms of Congenital Myasthenic Syndrome and Myasthenia Gravis. In August 2013, we were granted breakthrough therapy designation by the U.S. Food & Drug Administration (FDA) for Firdapse for the treatment of LEMS.

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

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

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CPP-115

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

CPP-109

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

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

Recently Filed Securities Class Action Lawsuit

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

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

Company Information

Our principal executive offices are located at 355 Alhambra Circle, Suite 1500, Coral Gables, Florida 33134, and our telephone number at that address is (305) 529-2522.

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RISK FACTORS

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

Risks Related to Our Business

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

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

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

We have had no revenues from product sales to date, currently have no products available for commercial sale, and have never had any products available for commercial sale. We expect to incur losses at least until we are in a position to commercialize Firdapse , which may never occur. Our net loss was $12.2 million for the year ended December 31, 2013, and as of December 31, 2013 we had a deficit accumulated during the development stage of $54.3 million. We may never obtain approval of an NDA for any of our product candidates and we may never achieve profitability.

Our business will require additional capital.

Our business will require additional capital to meet our product development objectives. Based on currently available information, we estimate that we have sufficient working capital to support our planned operations through at least the end of 2014. The expectations described above are based on current information available to us. If the cost of our ongoing studies are greater than we expect, our assumptions may not prove to be accurate. There can be no assurance as to the exact amount of the funding we will require or as to whether any such required funding will be available to us when it is required.

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

Any sale by us of additional equity or convertible debt securities could result in dilution to our stockholders. There can be no assurance that any required additional funding will be available to us at all or available on terms acceptable to us. Further, to the extent that we raise funds through collaborative arrangements, it may be necessary to relinquish some rights to our technologies or grant sublicenses on terms that are not favorable to us. If we are not able to secure funding when needed, we may have to delay, reduce the scope of or eliminate one or more research and development programs, which could have an adverse effect on our business.

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

In January of 2012, another pharmaceutical company, Jacobus Pharmaceutical, began its own Phase 2 trial studying their own formulation of amifampridine (3,4-DAP) for the treatment of LEMS. While there can be no assurance, based on currently available information, we believe that Firdapse is further along in development than this other company s development program. Under the Orphan Drug Act of 1983, the first pharmaceutical product to 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 3,4-DAP for any indication prior to approval of Firdapse , we would be barred from marketing Firdapse in the United States during this five-year exclusivity period.

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

Our development of CPP-115 is at an early stage, and it is going to be several years before we are in a position to file an NDA for CPP-115, if our future clinical trials of this product are successful. Further, our ability to develop CPP-115 will be dependent on our having the resources to conduct the studies and trials that would be required. There can be no assurance that we will ever file an NDA for CPP-115 or commercialize CPP-115.

Our business is subject to substantial competition.

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

manufacturing efficiency and marketing capabilities.

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For example, amifampridine, the active ingredient in Firdapse , has been available from compounding pharmacies and from Jacobus Pharmaceutical under compassionate use INDs for many years and will likely be available from these sources even if we are able to obtain FDA approval of Firdapse . Amifampridine from these sources is likely to be substantially less expensive than Firdapse . The FDA Pharmacy Compounding Advisory Committee, however, has previously issued a list of drugs which should not be compounded, and amifampridine was included on that list. Further, drugs that are not approved by FDA for the treatment of LEMS, such as dalfampridine (Ampyra®), may nonetheless be prescribed by physicians for the treatment of LEMS.

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

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

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

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

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

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

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

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

Risks Related to the Development of Our Drug Candidates

Our product development efforts may fail.

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

- Our product candidates may be found to be ineffective or unsafe, or fail to receive necessary regulatory approvals;
- Our product candidates may not be economical to market or take substantially longer to obtain necessary regulatory approvals than anticipated; or
- Competitors may market equivalent or superior products.

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

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Failure can occur at any stage of our product development efforts.

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

- regulators or Institutional Review Boards (IRBs) may not authorize us to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- conditions may be imposed upon us by the FDA regarding the scope or design of our clinical trials, or we may be required to resubmit our clinical trial protocols to IRBs for reinspection due to changes in the regulatory environment;
- the number of subjects required for our clinical trials may be larger, patient enrollment may take longer, or patients may drop out of our clinical trials at a higher rate than we anticipate;
- we may have to suspend or terminate one or more of our clinical trials if we, regulators, or IRBs determine that the participants are being subjected to unreasonable health risks;
- our third-party contractors, clinical investigators or contractual collaborators may fail to comply with regulatory requirements or fail to meet their contractual obligations to us in a timely manner;
- our tests may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional testing; and
- the costs of our pre-clinical and/or clinical trials may be greater than we anticipate.

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

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

efforts to obtain regulatory approvals for and commercialize our drug candidates may be delayed.

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

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

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

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

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

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

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

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

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

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

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

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

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

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

Our internal computer systems and those of our contract research organization and other key vendors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our programs. For example, the loss of clinical trial data from completed or ongoing clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development of our product candidates could be delayed.

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

We are exposed to the risk of employee or consultant fraud or other misconduct. Misconduct by our employees or consultants could include intentional failures to comply with FDA regulations, provide accurate information to the FDA, comply with manufacturing standards, comply with federal and state healthcare fraud and abuse laws and regulations, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing and other abusive practices. 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 lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

Risks Related to Government Regulation

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

We do not currently have any products that have been approved for commercialization. We will not be able to commercialize our products until we have obtained the requisite regulatory approvals from applicable governmental authorities. To obtain regulatory approval of a product candidate, we must demonstrate to the satisfaction of the applicable regulatory agency that such product candidate is safe and effective for its intended uses. The type and magnitude of the testing required for regulatory approval varies depending on the product candidate and the disease or condition for which it is being developed. In addition, in the U.S. we must show that the facilities used to manufacture our product candidate are in compliance with Current Good Manufacturing Processes (cGMP). We will also have to meet similar regulations in any foreign country where we may seek to commercialize our product candidates. In general, these requirements mandate that manufacturers follow elaborate design, testing, control, documentation and other quality assurance procedures throughout the entire manufacturing process. The process of obtaining regulatory

approvals typically takes several years and requires the expenditure of substantial capital and other resources. Despite the time, expense and resources invested by us in the approval process, we may not be able to demonstrate that our product candidates are safe and effective, in which event we would not receive the regulatory approvals required to market them.

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

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

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

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

We are in the process of conducting a Phase 3 clinical trial for Firdapse and we are currently planning to commence (during the first half of 2014) a Phase 1(b) clinical trial for CPP-115. Even if the results of our clinical trials are promising, Firdapse or CPP-115 may subsequently fail to meet the safety and efficacy standards required to obtain regulatory approvals. Future clinical trials for Firdapse or CPP-115 may not be successfully completed or may take longer than anticipated because of any number of factors, including potential delays in the start of the trial, an inability to recruit clinical trial participants at the expected rate, failure to demonstrate safety and efficacy, unforeseen safety issues, or unforeseen governmental or regulatory delays.

Any clinical trials we might develop and implement, may not be completed in a timely manner or at all. Our product candidates may not be found to be safe and effective, and may not be approved by regulatory authorities for the proposed indication. Further, regulatory authorities and IRBs that must approve and monitor the safety of each clinical study may suspend a clinical study at any time if the patients participating in such study are deemed to be exposed to any unacceptable health risk. We may also choose to suspend human clinical studies and trials if we become aware of any such risks. We might encounter problems in our clinical trials, including 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.

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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 product candidates. Further, unrelated third parties and investigators in the academic community have expressed interest in testing our product candidates. If these third-party tests are unsuccessful, or if they show significant health risk to the test subjects, our development efforts may also be adversely affected.

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

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

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

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

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

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

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

As a condition of NDA approval for some of our products, the FDA may 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.

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Risks Related to Our Intellectual Property

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

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

All of our patent rights for CPP-115 are derived from our license agreement with Northwestern University. Pursuant to this license agreement, we have exclusive worldwide rights to two patents in the United States. These were filed and obtained by Northwestern relating to compositions of matter for a class of molecules, including CPP-115. Both patents expire in 2023. Additionally, we have licensed rights from Northwestern to a pending patent for derivatives of vigabatrin that are unrelated to CPP-115. These rights are subject to the right of Northwestern, under limited circumstances, to practice the covered inventions for or on its own behalf for research. We may lose our rights to these patents and patent applications if we breach our obligations under the license agreement, including, without limitation, our financial obligations, including milestone payments, to Northwestern. If we violate or fail to perform any term or covenant of the license agreement, Northwestern may terminate the license agreement upon satisfaction of any applicable notice requirements and expiration of any applicable cure periods. Additionally, any termination of the license agreement, whether by us or by Northwestern, will not relieve us of our obligation to pay any license fees owing at the time of such termination. If we fail to retain our rights under the license agreement, we would not be able to commercialize CPP-115, and our business, results of operations, financial condition and prospects would be materially adversely affected.

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

If we obtain approval to market Firdapse , CPP-115 or CPP-109, our commercial success will depend 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.

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

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

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

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

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

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

- we may have to redesign our product so that it does not infringe others patent rights, which may not be possible or could require substantial funds or time and require additional studies.

In addition, employees, consultants, contractors and others may use the proprietary information of others in their work for us or disclose our proprietary information to others. As an example, we do not currently have written agreements regarding confidentiality or any other matters with several principal members of our Scientific Advisory Board. If our employees, consultants, contractors or others disclose our data to others or use data belonging to others in connection with our business, it could lead to disputes over the ownership of inventions derived from that information or expose us to potential damages or other penalties.

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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 could be highly volatile.

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

- developments concerning our clinical studies and trials and our pre-clinical studies;
- announcements of product development successes and failures by us or our competitors;
- new products introduced or announced by us or our competitors;

adverse changes in the abilities of our third party manufacturers to provide drug or product in a timely manner or to meet FDA requirements;

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changes in reimbursement levels;

changes in financial estimates by securities analysts;

actual or anticipated variations in operating results;

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

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

intellectual property, product liability or other litigation against us;

changes in the market valuations of similar companies;

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

changes in economic conditions; and

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

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

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

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

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

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

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

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

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

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

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

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

Any delay or prevention of a change of control transaction or changes in our Board of Directors or management could deter potential acquirors or prevent the completion of a transaction in which our stockholders could receive a substantial premium over the then current market price for their shares.

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

As of March 18, 2014 we had 54,145,633 shares of our common stock outstanding, of which 5,750,609 shares were held by our officers and directors. We also had outstanding: (i) common stock purchase warrants to purchase an aggregate of 4,835,924 additional shares of our common stock at exercise prices ranging from $1.04 to $2.08 per share, and (ii) stock options to purchase an aggregate of 3,401,906 shares at exercise prices ranging from $0.47 to $6.00 per share (3,086,905 of which are currently exercisable). Sales of restricted shares or shares underlying stock options and common stock purchase warrants, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock.

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

Our Certificate of Incorporation authorizes the issuance of up to 5,000,000 shares of blank check preferred stock, with such designation rights and preferences as may be determined from time to time by our Board of Directors. Our board of directors is empowered, without stockholder approval, to issue shares of preferred stock with dividend, liquidation, conversion, voting or other rights which could adversely affect the voting power or other rights of the holders of our common stock. In the event of such issuances, the preferred stock could be utilized, under certain circumstances, as a method of discouraging, delaying or preventing a change in control of our company, pursuant to our stockholder rights plan. Although we have no present intention to issue any additional 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.

CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS

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

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

- the scope, rate of progress and expense of our clinical trials and studies, pre-clinical studies, proof-of-concept studies, and our other product development activities;
- our ability to complete our trials and studies on a timely basis and within the budgets we establish for such trials and studies;
- the ability of our third-party suppliers and contract manufacturers to maintain compliance with current Good Manufacturing Processes (cGMP);

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whether our trials and studies will be successful;

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

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

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

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

whether others develop and commercialize products competitive to our products;

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

changes in the laws and regulations affecting our business;

the impact of the class action lawsuit filed against us;

our ability to attract and retain skilled employees;

security breaches of our computer systems, or 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.

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USE OF PROCEEDS

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

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

PRICE RANGE OF COMMON STOCK AND DIVIDEND POLICY

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

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

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

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GENERAL DESCRIPTION OF OUR COMMON STOCK

Our authorized capital currently consists of 100,000,000 shares of common stock, par value $0.001 per share, and 5,000,000 shares of preferred stock, par value $0.001 per share. As of the date of this prospectus, we had 54,145,633 shares of our common stock outstanding. There are no shares of preferred stock outstanding.

We are a Delaware corporation, and were incorporated on July 24, 2006. We are the successor by merger to Catalyst Pharmaceutical Partners, Inc., a Florida corporation, which was incorporated in January 2002.

Common Stock

The following summary of the material features of our common stock does not purport to be complete and is subject to, and qualified in its entirety by the provisions of our Certificate of Incorporation, our Bylaws and other applicable law. See *Where You Can Find Additional Information* .

Each holder of common stock is entitled to one vote for each share held of record on all matters presented to our stockholders, including the election of directors. In the event of our liquidation, dissolution, or winding-up, the holders of common stock are entitled to share ratably and equally in our assets, if any, that remain after paying all debts and liabilities and the liquidation preferences of any outstanding preferred stock. The common stock has no preemptive or cumulative rights and no redemption or conversion provisions.

Holders of our common stock are entitled to receive dividends if, as, and when declared by our board of directors out of funds legally available therefor, subject to the dividend and liquidation rights of any preferred stock that may be issued and outstanding, all subject to any dividend restrictions in our credit facilities. No dividend or other distribution (including redemptions and repurchases of shares of capital stock) may be made, if after giving effect to such distribution, we would not be able to pay our debts as they come due in the usual course of business, or if our total assets would be less than the sum of our total liabilities plus the amount that would be needed at the time of a liquidation to satisfy the preferential rights of any holders of preferred stock.

Provisions of the Certificate and Bylaws

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

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

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

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

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

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

Certain Anti-Takeover Matters

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

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

upon consummation of the transaction that resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding (but not the outstanding voting stock owned by the interested stockholder) those shares owned (i) by persons who are directors and also officers and (ii) employee stock plans in which employee participates do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or

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

Section 203 defines business combination to include the following:

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

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

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

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

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

In general, Section 203 defines an interested stockholder as any entity or person beneficially owning 15% or more of the outstanding voting stock of the corporation and any entity or person affiliated with or controlling or controlled by any of these entities or persons.

A Delaware corporation may opt out of this provision either with an express provision in its original certificate of incorporation or in an amendment to its certificate of incorporation or bylaws approved by its stockholders. However, we have not opted out of this provision. The statute could prohibit or delay mergers or other takeover or change in control attempts and, accordingly, may discourage attempts to acquire us.

Limitation of Liability and Indemnification Matters

Our certificate of incorporation limits the liability for monetary damages for breach of fiduciary duty by members of our Board of Directors, except for liability that cannot be eliminated under Delaware law. Under Delaware law, our directors have a fiduciary duty to us which is not eliminated by this provision in our certificate of incorporation. In addition, each of our directors is subject to liability under Delaware law for breach of their duty of loyalty for acts or omissions which are found by a court of competent jurisdiction to be not in good faith or which involve intentional misconduct or knowing violations of law for actions leading to improper personal benefit to the director and for payments of dividends or approval of stock repurchases or redemptions that are prohibited by Delaware law. This provision does not affect our directors responsibilities under any other laws, such as federal securities laws.

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Delaware law provides that the directors of a company will not be personally liable for monetary damages for breach of their fiduciary duty as directors, except for liability for any of the following:

any breach of a director s duty of loyalty to us or our stockholders;

acts or omissions not in good faith or which involve intentional misconduct or a knowing violation of law;

unlawful payment of dividends or unlawful stock repurchases or redemptions; or

any transaction from which the director derived an improper personal benefit.

Delaware law provides that the indemnification permitted thereunder shall not be deemed exclusive of any other rights to which our directors and officers may be entitled to under our bylaws, any agreement, a vote of stockholders or otherwise. Our certificate of incorporation and bylaws eliminate the personal liability of directors to the maximum extent permitted by Delaware law. In addition, our certificate of incorporation and bylaws provide that we may fully indemnify any person who is or was a party to or is threatened to be made a party to any threatened, pending or completed action, suit of proceeding (whether civil, criminal, administrative or investigative) by reason of the fact that such person is or was one of our directors, officers, employees or other agents, against expenses (including attorneys fees), judgments, fines and amounts paid in settlement actually and reasonably incurred by such person in connection with such action, suit or proceeding.

Listing

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

Transfer Agent and Registrar

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

PLAN OF DISTRIBUTION

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

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a fixed price or prices, which may change;

market prices prevailing at the time of sale;

prices relating to the prevailing market prices;

varying prices determined at the time of sale; or

negotiated prices.

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

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

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

any underwriting discounts and other items constituting underwriters compensation;

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

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

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

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

With respect to underwritten public offerings, negotiated transactions and block trades, we will provide in the applicable prospectus supplement any compensation we pay to underwriters, dealers or agents in connection with the offering of the shares of common stock, and any discounts, concessions or commissions allowed by underwriters to

participating dealers. Underwriters, dealers and agents participating in the distribution of the shares of common stock may be deemed to be underwriters within the meaning of the Securities Act of 1933, as amended, and any discounts and commissions received by them and any profit realized by them on resale of the shares may be deemed to be underwriting discounts and commissions. We may enter into agreements to indemnify underwriters, dealers and agents against civil liabilities, including liabilities under the Securities Act of 1933, as amended, or to contribute to payments they may be required to make in respect thereof.

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To facilitate the offering of shares of our common stock, certain persons participating in the offering may engage in transactions that stabilize, maintain or otherwise affect the price of the shares. This may include over allotments or short sales of the shares, which involve the sale by persons participating in the offering of more shares than we sold to them. In these circumstances, these persons would cover such over allotments or short positions by making purchases in the open market or by exercising their over allotment option. In addition, these persons may stabilize or maintain the price of the shares by bidding for or purchasing shares in the open market or by imposing penalty bids, whereby selling concessions allowed to dealers participating in the offering may be reclaimed if shares sold by them are repurchased in connection with stabilization transactions. The effect of these transactions may be to stabilize or maintain the market price of the securities at a level above that which might otherwise prevail in the open market. These transactions may be discontinued at any time.

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

LEGAL MATTERS

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

EXPERTS

The audited financial statements incorporated by reference in this prospectus have been so incorporated by reference in reliance upon the report of Grant Thornton, LLP, independent registered public accountants, upon the authority of said firm as experts in accounting and auditing.

WHERE YOU CAN FIND ADDITIONAL INFORMATION

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

INCORPORATION OF INFORMATION BY REFERENCE

The SEC allows us to incorporate by reference into this prospectus the information we have filed with the SEC. The information we incorporate by reference into this prospectus is an important part of this prospectus. Any statement in a document we incorporate by reference into this prospectus will be considered to be modified or superseded to the extent a statement contained in this prospectus or any other subsequently filed document that is incorporated by reference into this prospectus modifies or supersedes that statement. The modified or superseded statement will not be considered to be a part of this prospectus, except as modified or superseded.

We incorporate by reference into this prospectus the information contained in the documents below, which is considered to be a part of this prospectus:

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our Annual Report on Form 10-K for the year ended December 31, 2013, filed with the SEC on March 18, 2014;

our Current Reports on Form 8-K (or amendments thereto) filed with the SEC on January 8, 2014, February 20, 2014 and March 4, 2014;

our description of our common stock contained in our Registration Statement on Form 8-A, filed with the SEC on September 29, 2006, along with Amendment No. 1 thereto, filed with the SEC on October 18, 2006; and

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

You may obtain a copy of any of these documents at no cost by requesting them from us or by writing or calling: Catalyst Pharmaceutical Partners, Inc., 355 Alhambra Circle, Suite 1500, Coral Gables, Florida, 33134, Attn: Investor Relations, or by calling (305) 529-2522. Copies of each of these filings are also available for no cost on our website, www.catalystpharma.com, or on the SEC s web site, www.sec.gov.

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Shares

CATALYST PHARMACEUTICAL

PARTNERS, INC.

COMMON STOCK

PROSPECTUS SUPPLEMENT

Piper Jaffray & Co.

Sole Book-Running Manager

April , 2014