

CATALYST PHARMACEUTICAL PARTNERS, INC.

Form 10-Q

November 13, 2013

[Table of Contents](#)

**UNITED STATES**

**SECURITIES AND EXCHANGE COMMISSION**

**Washington, D.C. 20549**

**FORM 10-Q**

[Mark One]

- QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE  
SECURITIES EXCHANGE ACT OF 1934  
For the Quarterly Period Ended September 30, 2013**

**OR**

- TRANSITION REPORT UNDER SECTION 13 OR 15(d) OF THE  
SECURITIES EXCHANGE ACT OF 1934  
Commission File No. 001-33057**

**CATALYST PHARMACEUTICAL PARTNERS, INC.**

**(Exact name of registrant as specified in its charter)**

<b>Delaware</b> <b>(State or other jurisdiction of</b>	<b>76-0837053</b> <b>(IRS Employer</b>
<b>incorporation or organization)</b>	<b>Identification No.)</b>
<b>355 Alhambra Circle</b>	
<b>Suite 1500</b>	
<b>Coral Gables, Florida</b>	<b>33134</b>
<b>(Address of principal executive offices)</b>	<b>(Zip Code)</b>
<b>Registrant's telephone number, including area code: <u>(305) 529-2522</u></b>	

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

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

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

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

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

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date 54,132,937 shares of common stock, \$0.001 par value per share, were outstanding as of November 8, 2013.

**Table of Contents**

**CATALYST PHARMACEUTICAL PARTNERS, INC.**

**INDEX**

**PART I. FINANCIAL INFORMATION**

Item 1.	<b>CONDENSED FINANCIAL STATEMENTS</b>	
	<u>Condensed balance sheets at September 30, 2013 (unaudited) and December 31, 2012</u>	3
	<u>Condensed statements of operations for the three and nine months ended September 30, 2013 and 2012 and from January 4, 2002 (date of inception) through September 30, 2013 (unaudited)</u>	4
	<u>Condensed statement of stockholders' equity for the nine months ended September 30, 2013 (unaudited)</u>	5
	<u>Condensed statements of cash flows for the nine months ended September 30, 2013 and 2012 and from January 4, 2002 (date of inception) through September 30, 2013 (unaudited)</u>	6
	<u>Notes to unaudited condensed financial statements</u>	7
Item 2.	<b><u>MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS</u></b>	18
Item 3.	<b><u>QUANTITATIVE AND QUALITATIVE DISCLOSURE ABOUT MARKET RISK</u></b>	29
Item 4.	<b><u>CONTROLS AND PROCEDURES</u></b>	29

**PART II. OTHER INFORMATION**

Item 1.	<b><u>LEGAL PROCEEDINGS</u></b>	30
Item 1A.	<b><u>RISK FACTORS</u></b>	30
Item 2.	<b><u>UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS</u></b>	30
Item 3.	<b><u>DEFAULTS UPON SENIOR SECURITIES</u></b>	30
Item 4.	<b><u>MINE SAFETY DISCLOSURE</u></b>	30
Item 5.	<b><u>OTHER INFORMATION</u></b>	30
Item 6.	<b><u>EXHIBITS</u></b>	30

Table of Contents**CATALYST PHARMACEUTICAL PARTNERS, INC.****(a development stage company)****CONDENSED BALANCE SHEETS**

	<b>September 30, 2013</b>	<b>December 31, 2012</b>
	(unaudited)	
<b>ASSETS</b>		
Current Assets:		
Cash and cash equivalents	\$ 12,962,966	\$ 1,409,939
Certificates of deposit	4,010,160	6,502,825
Short-term investments	10,677,928	7,504,444
Prepaid expenses	988,701	1,309,470
<b>Total current assets</b>	<b>28,639,755</b>	<b>16,726,678</b>
Property and equipment, net	46,334	53,679
Deposits	8,888	8,888
<b>Total assets</b>	<b>\$ 28,694,977</b>	<b>\$ 16,789,245</b>
<b>LIABILITIES AND STOCKHOLDERS EQUITY</b>		
Current Liabilities:		
Accounts payable	\$ 1,473,341	\$ 1,365,663
Accrued expenses and other liabilities	1,538,320	281,002
<b>Total current liabilities</b>	<b>3,011,661</b>	<b>1,646,665</b>
Accrued expenses and other liabilities, non-current	20,040	21,878
Warrants liability, at fair value	3,544,201	498,587
<b>Total liabilities</b>	<b>6,575,902</b>	<b>2,167,130</b>
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, \$0.001 par value, 5,000,000 shares authorized: none issued and outstanding		
Common stock, \$0.001 par value, 100,000,000 shares authorized; 53,986,937 shares and 41,420,687 shares issued and outstanding at September 30, 2013 and December 31, 2012, respectively	53,987	41,421
Additional paid-in capital	75,044,029	56,759,697
Deficit accumulated during the development stage	(52,978,941)	(42,179,003)
<b>Total stockholders' equity</b>	<b>22,119,075</b>	<b>14,622,115</b>

Total liabilities and stockholders' equity	\$ 28,694,977	\$ 16,789,245
--	---------------	---------------

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

Table of Contents**CATALYST PHARMACEUTICAL PARTNERS, INC.****(a development stage company)****CONDENSED STATEMENTS OF OPERATIONS (unaudited)**

	<b>For the Three Months Ended September 30,</b>		<b>For the Nine Months Ended September 30,</b>		<b>Cumulative Period from January 4, 2002 (date of inception) to September 30, 2013</b>
	<b>2013</b>	<b>2012</b>	<b>2013</b>	<b>2012</b>	
Revenues - government grant	\$	\$	\$	\$	\$ 488,958
Operating costs and expenses:					
Research and development	2,804,352	654,837	6,028,691	1,914,905	34,331,996
General and administrative	441,424	628,876	1,576,044	1,800,882	18,243,335
Total operating costs and expenses	3,245,776	1,283,713	7,604,735	3,715,787	52,575,331
Loss from operations	(3,245,776)	(1,283,713)	(7,604,735)	(3,715,787)	(52,086,373)
Interest income	10,318	2,744	25,311	5,426	1,518,076
Change in fair value of warrants liability	(2,676,601)	(1,340,566)	(3,220,514)	(289,440)	(2,410,644)
Loss before income taxes	(5,912,059)	(2,621,535)	(10,799,938)	(3,999,801)	(52,978,941)
Provision for income taxes					
Net loss	\$ (5,912,059)	\$ (2,621,535)	\$ (10,799,938)	\$ (3,999,801)	\$ (52,978,941)
Loss per share basic and diluted	\$ (0.13)	\$ (0.08)	\$ (0.25)	\$ (0.14)	
Weighted average shares outstanding basic and diluted	44,686,310	32,132,824	42,529,432	27,913,800	

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



Table of Contents**CATALYST PHARMACEUTICAL PARTNERS, INC.****(a development stage company)****CONDENSED STATEMENT OF STOCKHOLDERS EQUITY (unaudited)****For the nine months ended September 30, 2013**

	<b>Preferred Stock</b>	<b>Common Stock</b>	<b>Additional Paid-in Capital</b>	<b>Deficit Accumulated During the Development Stage</b>	<b>Total</b>
<b>Balance at December 31, 2012</b>	\$	\$ 41,421	\$ 56,759,697	\$ (42,179,003)	\$ 14,622,115
Issuance of stock options for services			133,305		133,305
Issuance of common stock, net		8,850	14,086,344		14,095,194
Exercise of warrants for common stock		3,716	4,064,683		4,068,399
Net loss				(10,799,938)	(10,799,938)
<b>Balance at September 30, 2013</b>	\$	\$ 53,987	\$ 75,044,029	\$ (52,978,941)	\$ 22,119,075

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

**Table of Contents****CATALYST PHARMACEUTICAL PARTNERS, INC.****(a development stage company)****CONDENSED STATEMENTS OF CASH FLOWS (unaudited)**

	<b>For the Nine Months Ended, September 30,</b>		<b>Cumulative Period from January 4, 2002 (date of inception) through September 30, 2013</b>
	<b>2013</b>	<b>2012</b>	
<b>Operating Activities:</b>			
Net loss	\$ (10,799,938)	\$ (3,999,801)	\$ (52,978,941)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	16,777	8,236	181,655
Stock-based compensation	133,305	135,765	6,095,505
Change in fair value of warrants liability	3,220,514	289,440	2,410,644
(Increase) decrease in:			
Prepaid expenses and deposits	320,769	87,484	(997,589)
Increase (decrease) in:			
Accounts payable	107,678	91,975	1,473,341
Accrued expenses and other liabilities	1,255,480	(159,679)	1,495,007
Net cash used in operating activities	(5,745,415)	(3,546,580)	(42,320,378)
<b>Investing Activities:</b>			
Capital expenditures	(9,432)	(6,882)	(164,640)
Purchase of short term investments	(3,173,484)		(10,677,928)
Proceeds from (purchase of) certificates of deposit	2,492,665		(4,010,160)
Net cash used in investing activities	(690,251)	(6,882)	(14,852,728)
<b>Financing Activities:</b>			
Proceeds from issuance of common stock and warrants, net	14,071,694	9,498,255	57,210,636
Proceeds from issuance of preferred stock, net			3,895,597
Proceeds from issuance of convertible promissory note			5,000,000
Proceeds from exercise of warrants	3,893,499		3,909,749
Proceeds from exercise of options	23,500		23,500
Payment of employee withholding tax related to restricted stock units			(3,410)
Net cash provided by financing activities	17,988,693	9,498,255	70,036,072
Net (decrease) increase in cash	11,553,027	5,944,793	12,862,966

Cash and cash equivalents at beginning of period	1,409,939	6,029,067	100,000
Cash and cash equivalents at end of period	\$ 12,962,966	\$ 11,973,860	\$ 12,962,966

**Supplemental disclosures of non-cash investing and financing activity**

Non-cash incentive received from lessor	\$	\$	\$ 52,320
Exercise of liability classified warrants for common stock	\$ 174,900	\$	\$ 191,775
Conversion of note for common stock	\$	\$	\$ 5,000,000

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

**Table of Contents**

**CATALYST PHARMACEUTICAL PARTNERS, INC.**

**(a development stage company)**

**NOTES TO UNAUDITED CONDENSED FINANCIAL STATEMENTS**

**1. Organization and Description of Business.**

Catalyst Pharmaceutical Partners, Inc. (the Company) is a development-stage specialty pharmaceutical company focused on the development and commercialization of novel prescription drugs targeting rare (orphan) neuromuscular and neurological diseases, including Lambert-Eaton Myasthenic Syndrome (LEMS) and infantile spasms.

The Company has incurred operating losses in each period from inception through September 30, 2013. The Company has been able to fund its cash needs to date through several public and private offerings of its common stock and warrants, through government grants, and through an investment by a strategic purchaser. See Note 9.

***Capital Resources***

On December 3, 2010, the Company filed a Shelf Registration Statement on Form S-3 (the 2010 Shelf Registration Statement) with the SEC to sell up to \$30 million of common stock and common stock purchase warrants. This registration statement (file No. 333-170945) was declared effective by the SEC on December 15, 2010. The Company has conducted four registered direct offerings under the 2010 Shelf Registration Statement. See Note 9.

The number of shares that the Company could sell in the future and the amount of the gross proceeds that the Company could raise (in the aggregate) under the 2010 Shelf Registration Statement was limited to 20% of the number of shares of outstanding common stock and 33% of the Company's public float, respectively, pursuant to applicable NASDAQ marketplace and SEC rules. As of September 30, 2013, there is no further availability under the 2010 Shelf Registration Statement.

While there can be no assurance, based on currently available information, the Company estimates that it has sufficient working capital to support its operations through the end of 2014. The Company will require additional capital to support the Company's operations in periods after 2014.

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

**2. Basis of Presentation and Significant Accounting Policies.**

- a. **DEVELOPMENT STAGE COMPANY.** Since inception, the Company has devoted substantially all of its efforts to business planning, research and development, recruiting management and technical staff, acquiring operating assets and raising capital. Accordingly, the Company is considered to be in the development stage and the Company's financial statements are presented in accordance with U.S. generally accepted accounting principles applicable to a development stage company. The Company's primary focus is on the development and commercialization of its drug candidates.

**Table of Contents**

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

- b. INTERIM FINANCIAL STATEMENTS.** The accompanying unaudited interim financial statements have been prepared pursuant to the rules and regulations of the Securities and Exchange Commission (SEC) for reporting of interim financial information. Pursuant to such rules and regulations, certain information and footnote disclosures normally included in financial statements prepared in accordance with accounting principles generally accepted in the U.S. have been condensed or omitted.

In the opinion of management, the accompanying unaudited interim financial statements of the Company contain all adjustments (consisting of only normal recurring adjustments) necessary to present fairly the financial position of the Company as of the dates and for the periods presented. Accordingly, these statements should be read in conjunction with the financial statements and notes thereto for the year ended December 31, 2012 included in the 2012 Annual Report on Form 10-K filed by the Company with the SEC. The results of operations for the three and nine months ended September 30, 2013 are not necessarily indicative of the results to be expected for any future period or for the full 2013 fiscal year.

- c. USE OF ESTIMATES.** The preparation of financial statements in conformity with U.S. generally accepted accounting principles requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. Actual results could differ from those estimates.
- d. CASH AND CASH EQUIVALENTS.** The Company considers all highly liquid instruments purchased with an original maturity of three months or less to be cash equivalents. Cash equivalents consist mainly of money market funds. The Company has substantially all of its cash and cash equivalents deposited with one financial institution.
- e. CERTIFICATES OF DEPOSIT.** The certificates of deposit are issued by a banking institution and are recorded at cost plus accrued interest. The original maturity is greater than three months but does not exceed one year. Interest income is recorded in the statement of operations as it is earned. Carrying value at September 30, 2013 and December 31, 2012 approximates fair value.
- f. SHORT-TERM INVESTMENTS.** The Company invests in short-term investments in high credit-quality funds in order to obtain higher yields on its cash available for investments. As of September 30, 2013 and December 31, 2012 short-term investments consisted of a short-term bond fund. Such investments are not insured by the Federal Deposit Insurance Corporation. Short-term investments at September 30, 2013 and December 31, 2012 were considered trading securities. Trading securities are recorded at fair value based on the closing market price of the security. For trading securities, the Company recognizes realized gains and losses and unrealized gains and losses to earnings. Unrealized losses for the three and nine months ended September 30, 2013 were nominal.
- g. PREPAID EXPENSES.** Prepaid expenses consist primarily of prepaid research fees, prepaid insurance and prepaid subscription fees. Prepaid research fees consists of advances for the Company's product development

activities, including drug manufacturing, contracts for pre-clinical studies, clinical trials, regulatory affairs and consulting. Such advances are recorded as expense as the related goods are received or the related services are performed.

- h. FAIR VALUE OF FINANCIAL INSTRUMENTS.** The Company's financial instruments consist of cash and cash equivalents, certificates of deposit, short-term investments, accounts payables, accrued expenses and other liabilities, and warrants liability. At September 30, 2013 and December 31, 2012, the fair value of these instruments approximated their carrying value.

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

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

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

	<b>Fair Value Measurements at Reporting Date Using</b>			
	Balances as of September 30, 2013	Quoted Prices in Active Markets for Identical Assets/Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Money market funds	\$ 7,812,683	\$ 7,812,683	\$	\$
Certificates of deposit	\$ 4,010,160	\$	\$ 4,010,160	\$
Short-term investments	\$ 10,677,928	\$ 10,677,928	\$	\$
Warrants liability	\$ 3,544,201	\$	\$	\$ 3,544,201

	<b>Fair Value Measurements at Reporting Date Using</b>			
	Balances as of December 31, 2012	Quoted Prices in Active Markets for Identical Assets/Liabilities	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)

		(Level 1)		
Certificates of deposit	\$ 6,502,825	\$	\$ 6,502,825	\$
Short-term investments	\$ 7,504,444	\$	7,504,444	\$
Warrants liability	\$ 498,587	\$	\$	\$ 498,587

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

- j. WARRANTS LIABILITY.** In October 2011, the Company issued 1,523,370 warrants to purchase shares of the Company's common stock in connection with a registered direct offering under the 2010 Shelf Registration Statement. The Company accounted for these warrants as a liability measured at fair value due to a provision included in the warrants agreement that provides the warrants holders with an option to require the Company (or its successor) to purchase their warrants for cash in an amount equal to their Black-Scholes Option Pricing Model (the Black-Scholes Model) value, in the event that certain fundamental transactions, as defined, occur. The fair value of the warrants liability is estimated using the Black-Scholes Model which requires inputs such as the expected term of the warrants, share price volatility and risk-free interest rate. These assumptions are reviewed on a quarterly basis and changes in the estimated fair value of the outstanding warrants are recognized each reporting period in the Change in fair value of warrants liability line in the statement of operations. As of September 30, 2013, 1,400,870 of the 2011 warrants remained outstanding.
- k. STOCK-BASED COMPENSATION.** The Company recognizes expense in the statement of operations for the fair value of all share-based payments to employees, directors, consultants and scientific advisors, including grants of stock options and other share-based awards. For stock options, the Company uses the Black-Scholes option valuation model, the single-option award approach, and the straight-line attribution method. Using this approach, compensation cost is amortized on a straight-line basis over the vesting period of each respective stock option, generally three to five years. The Company estimates forfeitures and adjusts this estimate periodically based on actual forfeitures.

As of September 30, 2013, there were outstanding stock options to purchase 3,488,906 shares of common stock, of which stock options to purchase 2,805,574 shares of common stock were exercisable as of September 30, 2013.

For the three and nine month periods ended September 30, 2013 and 2012, the Company recorded stock-based compensation expense as follows:

	Three months ended September 30,		Nine months ended September 30,	
	2013	2012	2013	2012
Research and development	\$ 23,133	\$ 18,503	\$ 63,508	\$ 55,108
General and administrative	23,532	27,082	69,797	80,657
Total stock-based compensation	\$ 46,665	\$ 45,585	\$ 133,305	\$ 135,765

- l. COMPREHENSIVE INCOME (LOSS).** U.S. generally accepted accounting principles require that all components of comprehensive income (loss) be reported in the financial statements in the period in which they are recognized. Comprehensive income (loss) is net income (loss), plus certain other items that are recorded directly into stockholders' equity. For all periods presented, the Company's net loss equals comprehensive loss, since the Company has no items which are considered other comprehensive income

(loss).

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

- m. NET LOSS PER SHARE.** Basic loss per share is computed by dividing net loss for the period by the weighted average number of common shares outstanding during the period. The calculation of basic and diluted net loss per share is the same for all periods presented, as the effect of potential common stock equivalents is anti-dilutive due to the Company's net loss position for all periods presented. The potential shares, which are excluded from the determination of basic and diluted net loss per share as their effect is anti-dilutive, are as follows:

	<b>September 30,</b>	
	<b>2013</b>	<b>2012</b>
Options to purchase common stock	3,488,906	2,742,202
Warrants to purchase common stock	4,994,620	8,723,370
Potential equivalent common stock excluded	8,483,526	11,465,572

Potentially dilutive options to purchase common stock as of September 30, 2013 and 2012 have exercise prices ranging from \$0.47 to \$6.00 and \$0.62 to \$6.00, respectively. Potentially dilutive warrants to purchase common stock as of September 30, 2013 and 2012 have exercise prices ranging from \$1.04 to \$2.08.

- n. RECENTLY ISSUED ACCOUNTING STANDARDS.** There are no recent accounting pronouncements which we anticipate will have a significant impact on the Company's financial statements.

**3. Warrants Liability, at Fair Value.***2011 Warrants*

The Company allocated approximately \$1.3 million of proceeds from its October 2011 registered direct offering to the fair value of common stock purchase warrants issued in connection with the offering that are classified as a liability (the 2011 warrants). The 2011 warrants are classified as a liability because of provisions in such warrants that allow for the net cash settlement of such warrants in the event of certain fundamental transactions (as defined in the warrant agreement). The valuation of the 2011 warrants is determined using the Black-Scholes Model. This model uses inputs such as the underlying price of the shares issued when the warrant is exercised, volatility, risk free interest rate and expected life of the instrument. The Company has determined that the 2011 warrants liability should be classified within Level 3 of the fair value hierarchy by evaluating each input for the Black-Scholes Model against the fair value hierarchy criteria and using the lowest level of input as the basis for the fair value classification. There are six inputs: closing price of the Company's common stock on the day of evaluation; the exercise price of the warrants; the remaining term of the warrants; the volatility of the Company's common stock; annual rate of dividends; and the risk free rate of return. Of those inputs, the exercise price of the warrants and the remaining term are readily observable in the warrants agreement. The annual rate of dividends is based on the Company's historical practice of not granting dividends. The closing price of the Company's common stock would fall under Level 1 of the fair value hierarchy as it is a quoted price in an active market. The risk free rate of return is a Level 2 input, while the historical volatility is a Level 3 input in accordance with the fair value accounting guidance. Since the lowest level input is a Level 3, the Company determined the warrants liability is most appropriately classified within Level 3 of the fair value hierarchy.

This liability is subject to fair value mark-to-market adjustment each reporting period.

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

The calculated value of the 2011 warrants liability was determined using the Black-Scholes Model with the following assumptions:

	<b>September 30, 2013</b>	<b>December 31, 2012</b>
Risk free interest rate	0.85%	0.60%
Expected term	3.59 years	4.34 years
Expected volatility	115%	136%
Expected dividend yield	0%	0%
Expected forfeiture rate	0%	0%

The following table rolls forward the fair value of the Company's warrants liability activity for the three and nine month periods ended September 30, 2013 and 2012:

	<b>Three months ended September 30,</b>		<b>Nine months ended September 30,</b>	
	<b>2013</b>	<b>2012</b>	<b>2013</b>	<b>2012</b>
Fair value, beginning of period	\$ 1,042,500	\$ 594,114	\$ 498,587	\$ 1,645,240
Issuance of warrants				
Exercise of warrants	(174,900)		(174,900)	
Change in fair value	2,676,601	1,340,566	3,220,514	289,440
Fair value, end of period	\$ 3,544,201	\$ 1,934,680	\$ 3,544,201	\$ 1,934,680

During the three month period ended September 30, 2013, 110,000 of the 2011 warrants were exercised, with proceeds to the company of \$143,000. The Company recognizes the change in the fair value of the warrants liability as a non-operating income or loss in the accompanying statements of operations.

**4. Prepaid Expenses.**

Prepaid expenses consist of the following:

	<b>September 30, 2013</b>	<b>December 31, 2012</b>
Prepaid research fees	\$ 842,429	\$ 1,138,185
Prepaid insurance	88,043	143,520
Prepaid subscription fees	27,215	12,369
Prepaid rent	6,535	683
Other	24,479	14,713
Total prepaid expenses	\$ 988,701	\$ 1,309,470

**5. Property and Equipment.**

Property and equipment, net consists of the following:

	<b>September 30, 2013</b>	<b>December 31, 2012</b>
Computer equipment	\$ 81,551	\$ 74,191
Furniture and equipment	51,523	49,451
	133,074	123,642
Less: Accumulated depreciation	(86,740)	(69,963)
<b>Total property and equipment, net</b>	<b>\$ 46,334</b>	<b>\$ 53,679</b>

Depreciation expense was \$5,707 and \$2,725 and \$16,777 and \$8,236, respectively, for the three and nine month periods ended September 30, 2013 and 2012.

**Table of Contents****6. Accrued Expenses and Other Liabilities.**

Accrued expenses and other liabilities consist of the following:

	September 30, 2013	December 31, 2012
Accrued pre-clinical and clinical trial expenses	\$ 1,287,384	\$ 197,572
Accrued professional fees	115,814	51,050
Accrued compensation and benefits	78,697	5,949
Accrued license fees	51,250	15,000
Deferred rent	2,245	765
Other	2,930	10,666
Current accrued expenses and other liabilities	1,538,320	281,002
Deferred rent- non-current	20,040	21,878
Non-current accrued expenses and other liabilities	20,040	21,878
Total accrued expenses and other liabilities	\$ 1,558,360	\$ 302,880

**7. Commitments.**

- a. **LICENSE AGREEMENT WITH BROOKHAVEN.** The Company has entered into a license agreement with Brookhaven Science Associates, LLC, as operator of Brookhaven National Laboratory under contract with the United States Department of Energy (Brookhaven), whereby the Company has obtained an exclusive license for several patents and patent applications in the U.S. and outside the U.S. relating to the use of vigabatrin as a treatment for cocaine, other addictions and obsessive-compulsive disorders. This license agreement runs concurrently with the term of the last to expire of the licensed patents, the last of which currently expires in 2023. The Company paid a fee to obtain the license of \$50,000. Under the license agreement, the Company has agreed to pay Brookhaven a fee of \$100,000 in the year of new drug application (NDA) approval of CPP-109, \$250,000 in each of the second and third years following approval and \$500,000 per year thereafter until the license agreement expires. The Company is also obligated to reimburse Brookhaven for certain of their patent related expenses. The Company believes that as of September 30, 2013 and December 31, 2012, it had a contingent liability of approximately \$166,000 related to this obligation. Of these costs, approximately \$69,000 will become payable in six equal monthly installments at the time the Company submits an NDA to the U.S. Food and Drug Administration (FDA), and the remaining \$97,000 will be due commencing within 60 days of obtaining FDA regulatory approval to sell any product covered by the license agreement. The Company also has the right to enter into sub-license agreements, and if it does, a royalty of 20% of any sub-license fees will be payable to Brookhaven.

Brookhaven has formally advised the Company that they believe that the amount potentially due from the Company to Brookhaven for reimbursement of patent related expenses is approximately \$1.3 million. The Company has advised Brookhaven that it disputes their determination of patent-related expenses due under the license agreement. There can be no assurance as to the outcome of this matter. In any event, no patent-related expenses are due to Brookhaven under the license agreement until the submission by the Company of an NDA for CPP-109. As the Company has not yet filed an NDA for CPP-109, no amounts relating to this matter are accrued in the accompanying September 30, 2013 and December 31, 2012 condensed balance sheets. See Note 12.

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

---

**Table of Contents****7. Commitments (continued).**

Under the license agreement with Northwestern, the Company will be responsible for continued research and development of any resulting product candidates. As of September 30, 2013, the Company has paid \$246,590 in connection with the license and has accrued license fees of \$51,250 in the accompanying September 30, 2013 condensed balance sheet for expenses, maintenance fees and milestones. In addition, the Company is obligated to pay certain milestone payments in future years relating to clinical development activities with respect to CPP-115, and royalties on any products resulting from the license agreement. The next milestone payment of \$150,000 is due on the earlier of successful completion of the first Phase 2 clinical trial of CPP-115 or August 27, 2015.

- c. LICENSE AGREEMENT WITH NEW YORK UNIVERSITY AND THE FEINSTEIN INSTITUTE FOR MEDICAL RESEARCH.** On December 13, 2011, the Company entered into a license agreement with New York University (NYU) and the Feinstein Institute for Medical Research (FIMR) under which it acquired worldwide rights to commercialize GABA aminotransferase inhibitors in the treatment for Tourette Syndrome. The Company is obligated to pay certain milestone payments in future years relating to clinical development activities and royalties on any products resulting from the license agreement.
- d. LICENSE AGREEMENT WITH BIOMARIN.** On October 26, 2012, the Company entered into a strategic collaboration with BioMarin Pharmaceutical, Inc. (BioMarin) for Firdapse . The key components of the collaboration include: (i) the Company licensed the exclusive North American rights to Firdapse pursuant to a License Agreement, dated as of October 26, 2012 (the License Agreement) between the Company and BioMarin, and (ii) BioMarin made a \$5,000,000 investment in the Company pursuant to the terms of a Convertible Promissory Note and Note Purchase Agreement, dated as of October 26, 2012 (the Investment Agreement). The Investment Agreement provides that the Company will use the \$5 million solely for the purpose of developing Firdapse .

Initially, the \$5,000,000 investment from BioMarin was treated as a loan to the Company. However, on December 10, 2012, the loan automatically converted, at a conversion rate of \$0.75 per share, into 6,666,667 shares of the Company's authorized but unissued common stock.

As part of the License Agreement, the Company has taken over a Phase 3 Trial previously being conducted by BioMarin and is obligated to use its diligent efforts to seek to obtain regulatory approval for and to commercialize Firdapse in the United States. The Company is obligated to use diligent efforts to complete the double-blind treatment phase of the Phase 3 trial within 24 months of entering into the License Agreement, and BioMarin has the right to terminate the License Agreement if such treatment phase has not been completed in such 24-month period (unless the Company is using diligent effort to pursue the completion of such treatment phase and has spent at least \$5 million in connection with the conduct of the Phase 3 Trial during such 24 month period). As of September 30, 2013, the Company had disbursed approximately \$3.3 million in connection with expenses related to the Phase 3 trial, and the Company anticipates that the remaining \$1.7 million will be expended during 2013.

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

FDA of an NDA for Firdapse for the treatment of LEMS). The Company has also agreed to share in the cost of certain post-marketing studies that are being conducted by BioMarin if such studies are required as a condition for approval of the product by the FDA. However, no such payments will be due until an NDA has been filed for Firdapse .

**Table of Contents**

**7. Commitments (continued).**

- e. **AGREEMENTS FOR DRUG DEVELOPMENT, PRE-CLINICAL AND CLINICAL STUDIES.** The Company has entered into agreements with contract manufacturers for the manufacture of drug and study placebo for the Company's trials and studies, with contract research organizations (CRO) to conduct and monitor the Company's trials and studies and with various entities for laboratories and other testing related to the Company's trials and studies. The contractual terms of the agreements vary, but most require certain advances as well as payments based on the achievement of milestones. Further, these agreements are cancellable at any time, but obligate the Company to reimburse the providers for any time or costs incurred through the date of termination.

**8. Income Taxes.**

The Company is subject to income taxes in the U.S. federal jurisdiction and various states jurisdictions. Tax regulations within each jurisdiction are subject to the interpretation of the related tax laws and regulations and require significant judgment to apply. The Company is not subject to U.S. federal, state and local tax examinations by tax authorities for any years before 2010. If the Company were to subsequently record an unrecognized tax benefit, associated penalties and tax related interest expense would be reported as a component of income tax expense.

**9. Stockholders Equity.**

***2010 Shelf Registration Statement***

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

- (a) On March 8, 2011, the Company filed a prospectus supplement and offered for sale to institutional investors 2,259,943 shares of its common stock at a price of \$1.12 per share and received gross proceeds of approximately \$2.5 million before underwriting commission and incurred expenses of approximately \$300,000.
- (b) On October 28, 2011, the Company filed a prospectus supplement and offered for sale to institutional investors 3,046,740 shares of its common stock together with common stock purchase warrants to purchase 1,523,370 shares of the Company's common stock at a price of \$1.15 per share and corresponding warrant and received gross proceeds of approximately \$3.5 million before underwriting commission and other expenses totaling approximately \$335,000. The warrants issued in this registered direct offering, which expire on April 28, 2017 and have an exercise price of \$1.30 per share, have been accounted for as a liability. See Note 3.

(c)

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

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

**Table of Contents**

**9. Stockholders Equity (continued).**

The number of shares that the Company can sell and the amount of the gross proceeds that the Company can raise (in the aggregate) at any one time under its currently outstanding Shelf Registration Statement is limited to 20% of the number of shares of outstanding common stock and 33% of the Company's public float, respectively, pursuant to applicable NASDAQ marketplace and SEC rules.

***2012 Form S-1 Registration Statement***

On May 24, 2012, the Company sold 6,000,000 shares of its common stock together with common stock purchase warrants to purchase 6,000,000 shares of the Company's common stock, at a price of \$0.80 per share and corresponding warrant. These securities were issued pursuant to a Form S-1 registration statement that became effective on May 23, 2012 (file no. 333-180167). The Company received gross proceeds of approximately \$4.8 million from this offering, before underwriting commission and other expenses totaling approximately \$795,000. The May 2012 warrants, which expire five years from this offering, before underwriting commission and other expenses totaling approximately \$795,000. The May 2012 warrants, which expire five years from their date of issuance and have an exercise price of \$1.04 per share, have been accounted for as equity instruments, since they do not contain features (such as net cash settlement or anti-dilution features) that would preclude the Company from accounting for these warrants as equity.

***Warrant Exercises***

During the three month period ended September 30, 2013, the Company issued an aggregate of 3,716,250 shares of its authorized but unissued common stock upon the exercise of previously issued common stock purchase warrants, raising gross proceeds of approximately \$3.9 million.

***Nasdaq Listing***

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

**10. Stock Compensation.**

***Stock Options***

During the nine month period ended September 30, 2013, the Company granted five-year options to purchase an aggregate of 115,000 shares of the Company's common stock to certain employees. No options were granted during the three month period ended September 30, 2013 or the three and nine month periods ended September 30, 2012. The Company recorded stock-based compensation related to stock options totaling \$46,665 and \$45,585, respectively, during the three month period ended September 30, 2013 and 2012 and \$133,305 and \$135,765, respectively, during the nine month period ended September 30, 2013 and 2012. No options vested during the three and nine month periods ended September 30, 2013 and 2012.

**Table of Contents**

**10. Stock Compensation (continued).**

During the nine month period ended September 30, 2013, options to purchase 50,000 shares of the Company's common stock were exercised with proceeds of \$23,500. During the nine month period ended September 30, 2012, options to purchase 195,000 shares of the Company's common stock were exercised on a cashless basis, resulting in the issuance of an aggregate of 40,100 shares of the Company's common stock.

As of September 30, 2013, there was approximately \$153,000 of unrecognized compensation expense related to non-vested stock compensation awards granted under the 2006 Stock Incentive Plan. The cost is expected to be recognized over a weighted average period of approximately 1.48 years.

**11. Related Party Transactions.**

The Company has consulting arrangements with its Chief Medical Officer and with several members of its Scientific Advisory Board. During the three and nine month periods ended September 30, 2013 and 2012, the Company paid approximately \$2,500 and \$11,000, and \$7,500 and \$32,000 respectively, in consulting fees to related parties.

**12. Subsequent Events.**

In October 2013, a securities class action lawsuit was filed against the Company and certain of its executive officers and directors seeking unspecified damages in the U.S. District Court for the Southern District of Florida. The complaint purports to state a claim for violation of federal securities laws on behalf of a class of those who purchased the Company's common stock between October 31, 2012 and October 18, 2013. The lawsuit is at a very early stage and the Company intends to vigorously defend this lawsuit. While there can be no assurance, the Company does not expect this lawsuit to have a material adverse effect on the Company.

On November 8, 2013, effective October 1, 2013, the Company terminated its license agreement with Brookhaven. Under the termination agreement, the Company was released from any future obligation to reimburse Brookhaven for Brookhaven's patent-related expenses. See Note 7.

## Table of Contents

### ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

#### **Introduction**

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

*Overview.* This section provides a general description of our business, trends in our industry, as well as a discussion regarding recent developments in our business.

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

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

*Results of Operations.* This section provides an analysis of our results of operations for the three and nine month periods ended September 30, 2013 as compared to the same periods ended September 30, 2012.

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

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

#### **Overview**

We are a development-stage specialty pharmaceutical company focused on the development and commercialization of novel prescription drugs targeting rare (orphan) neuromuscular and neurological diseases 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 Congenital Myasthenic Syndrome and Myasthenia Gravis. We have recently been granted breakthrough therapy designation by the U.S. Food & Drug Administration (FDA) for Firdapse™ for the treatment of LEMS.

Neither the chemical entity 3,4-diaminopyridine (3,4-DAP) nor its phosphate salt has ever been approved by the FDA for any indication. If we are the first pharmaceutical company to obtain approval for 3,4-diaminopyridine or its phosphate salt, 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, if we are the first company to obtain approval of 3,4-diaminopyridine or its phosphate salt, it would make us eligible to receive seven-years of marketing exclusivity for this indication.

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 19 sites in the United States and Europe. We also expect to add at least six

**Table of Contents**

additional sites in the United States (U.S.), Canada, South America and Europe in the near future. Based on currently available information, we expect that we will complete enrollment in our trial around the end of the year and that we will report top-line results from the double-blind portion of this Phase 3 trial during the second quarter of 2014 (and, if the trial results are successful, we expect to submit a new drug application (NDA) to the FDA in the first half of 2015).

Further, an interim safety analysis was recently performed by the independent Data Monitoring Committee that is part of the routine oversight of the Phase 3 trial. Following this meeting, the committee recommended that we continue the trial as planned. This recommendation is based on the committee's review of unblinded safety and clinical data available from the on-going Phase 3 trial.

Additionally, we also recently commented on the following matters relating to our development of Firdapse :

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

We have previously reported that another pharmaceutical company is conducting a Phase 2 trial with a different formulation of amifampridine (3,4-DAP) for the treatment of LEMS. While there can be no assurance, we continue to believe that our development program for the product is further along in development than this other company.

We believe that the LEMS patient community deserves the benefits of having an approved product to treat their disease that has met the FDA's stringent burden of proof in safety and efficacy and is widely available for use by physicians treating LEMS patients. To date, no version of 3,4-DAP or its phosphate salt has been approved by the FDA for use in the treatment of LEMS. To obtain approval to market a drug in the U.S., a significant number of preclinical and clinical safety and efficacy studies must be completed. This includes studies which evaluate the efficacy of the product, including in most cases at least one double-blind, placebo-controlled pivotal registration trial that meets the requirements established by the FDA. It also includes studies that evaluate the drug's long-term toxicity, acute toxicity, reproductive toxicity, carcinogenicity, cardiac safety, renal safety, pharmacokinetics, absorption, distribution, metabolism, and elimination. Particularly with respect to products containing 3,4-DAP or its phosphate salts, there is a wide metabolic variability within the patient population, which must be characterized in order to provide physicians with information about what to expect in the patients they treat and, more importantly, with instructions on how to safely prescribe the drug to their patients. The FDA typically expects that the registrational clinical trial supporting approval of a product will be done with batches of the to-be commercialized form of the drug, which has to be manufactured under current good manufacturing practices (cGMP), using a validated manufacturing process suitable for commercialization, tested with validated analytical methods, and tested for shelf life stability. Our development plan for Firdapse has been designed to meet all of these requirements and has been previously discussed with the FDA.

Based on currently available information, we expect to make a cumulative investment in the development and commercialization of Firdapse of between \$40 million and \$50 million, consisting of: (i) \$20 million to \$25 million that we currently anticipate will be spent conducting the clinical, non-clinical and safety evaluations, and manufacturing the three validation batches required to be submitted, that will be required for us to obtain an NDA for Firdapse for the treatment of LEMS, (ii) approximately \$10 million in milestone payments that we will be obligated to pay under our license agreement with BioMarin (a portion of which will be due when an NDA for Firdapse for the treatment of LEMS is accepted by the FDA and a portion of which will be due upon the final approval of an NDA for Firdapse for the treatment of LEMS), and (iii) \$5 million to \$15 million that we expect to spend in connection with future post-marketing studies of Firdapse and to develop the infrastructure required to commercialize Firdapse (including our efforts to develop the patient advocacy programs and patient assistance program discussed below). This is a significant investment of capital and years of research and development by us, and is additional to the millions of dollars that have already been spent in the development of this product by BioMarin, by the other former licensors of the product, and by the innovator of the product (the pharmaceutical unit (AGEPS) of the Paris Public Hospital Authority).

## Table of Contents

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

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

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

### CPP-115

We are in the early stages of developing CPP-115, a GABA aminotransferase inhibitor that, based on our pre-clinical studies to date, we believe is a more potent form of vigabatrin, but may have fewer side effects (e.g., visual field defects, or VFDs) than those associated with vigabatrin. We are hoping to develop CPP-115 for the treatment of epilepsy (initially infantile spasms) and for the treatment of other selected neurological indications. CPP-115 has been granted Orphan Drug Designation by the FDA for the treatment of infantile spasms and Orphan Medicinal Product Designation in the European Union, or E.U., for West's syndrome (a form of infantile spasms). We plan 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 recently 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 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. Based on currently available information, we expect to have top line results for this academic investigator proof-of-concept study during the first half of 2014.

### Recently Filed Securities Class Action Lawsuit

In October 2013, a securities class action lawsuit was 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 complaint purports 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. In general, the complaint alleges, among other things, that during the period between October 31, 2012 and October 18, 2013, we made alleged misrepresentations regarding the development of Firdapse. 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.



## **Table of Contents**

### **Risks Associated with Product Development**

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 studies and trials, pre-clinical studies, and other product development activities;

the results of our pre-clinical studies and clinical studies and trials, and the number of such studies and trials (and the scope of such studies and trials) that will be required for us to seek and obtain approval of our product candidates; and

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

For further information regarding the risks associated with our business, see the **Risk Factors** section of our Annual Report on Form 10-K for the fiscal year ended December 31, 2012.

### **Available Capital Resources**

Based on an analysis of our current financial condition and forecasts of available cash, we believe that we have sufficient resources to support our operations through 2014. However, we will require additional funding to support our operations beyond 2014. There can be no assurance that we will obtain required additional funding or ever be able to commercialize any of our product candidates. See *Liquidity and Capital Resources* below.

### **Basis of presentation**

#### *Revenues*

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

#### *Research and development expenses*

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

future. Costs incurred in connection with research and development activities are expensed as incurred.

Our cost accruals for clinical studies and trials are based on estimates of the services received and efforts expended pursuant to contracts with numerous clinical study and trial sites and clinical research organizations. In the normal course of business we contract with third parties to perform various clinical study and trial activities in the on-going development of potential products. The financial terms of these agreements are subject to negotiation and vary from contract to contract and may result in uneven payment flows. Payments under the contracts depend on factors such as the achievement of certain events or milestones, the successful enrollment of subjects, the allocation of responsibilities among the parties to the agreements, and the completion of portions of the clinical study or trial or similar conditions. The objective of our accrual policy is to match the recording of expenses in our financial statements to the actual services received and efforts expended. As such, expense accruals related to pre-clinical and clinical studies or trials are recognized based on our estimate of the degree of completion of the event or events specified in the specific study or trial contract. We monitor service provider activities to the extent possible; however, if we underestimate activity levels associated with various

## **Table of Contents**

studies or trials at a given point in time, we could be required to record significant additional research and development expenses in future periods. Pre-clinical and clinical study and trial activities require significant up front expenditures. We anticipate paying significant portions of a study or trial's cost before such study or trial begins, and incurring additional expenditures as the study or trial progresses and reaches certain milestones.

### *Selling and marketing expenses*

We do not currently have any selling or marketing expenses, as we have not yet received approval for the commercialization of any of our product candidates. We expect we will begin to incur such costs upon our filing of an NDA, so that we can have a sales force in place to commence our selling efforts immediately upon receiving approval of such NDA, of which there can be no assurance.

### *General and administrative expenses*

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

### *Stock-based compensation*

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

### *Warrants Liability*

We issued warrants to purchase shares of our common stock as part of the equity financing that we completed in October 2011. In accordance with U.S. generally accepted accounting principles, we have recorded the fair value of the warrants as a liability in the accompanying balance sheets at September 30, 2013 and December 31, 2012 using a Black-Scholes option-pricing model. We will remeasure the fair value of the warrants liability at each reporting date until the warrants are exercised or have expired. Changes in the fair value of the warrants liability are reported in the statements of operations as income or expense. The fair value of the warrants liability is subject to significant fluctuation based on changes in the inputs to the Black-Scholes option-pricing model, including our common stock price, expected volatility, expected life, the risk-free interest rate and dividend yield. The market price for our common stock has been and may continue to be volatile. Consequently, future fluctuations in the price of our common stock may cause significant increases or decreases in the fair value of the warrants.

### *Income taxes*

We have incurred operating losses since inception. Our net deferred tax asset has a 100% valuation allowance as of September 30, 2013 and December 31, 2012, as we believe it is more likely than not that the deferred tax asset will not be realized. If an ownership change, as defined under Internal Revenue Code Section 382, occurs, the use of any of our carry-forward tax losses may be subject to limitation.

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

authority.

*Recently Issued Accounting Standards*

For discussion of recently issued accounting standards, please see Note 2, Basis of Presentation and Significant Accounting Policies, in the interim financial statements included in this report.

**Critical Accounting Policies and Estimates**

This discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements

## **Table of Contents**

requires us to make judgments, estimates, and assumptions that affect the reported amounts of assets, liabilities, revenues, expenses and disclosures of contingent assets and liabilities. For a full discussion of our accounting policies please refer to Note 2 on the Financial Statements included in our 2012 Annual Report on Form 10-K filed with the SEC. Our most critical accounting policies and estimates include: accounting for development stage, research and development expenses and stock-based compensation, measurement of fair value, fair value of warrants liability, income taxes and reserves. We continually evaluate our judgments, estimates and assumptions. We base our estimates on the terms of underlying agreements, our expected course of development, historical experience and other factors that we believe are reasonable based on the circumstances, the results of which form our management's basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates. There have been no material changes to our critical accounting policies and estimates from the information provided in Part II, Item 7, Management's Discussion and Analysis of Financial Condition and Results of Operations included in our 2012 Annual Report on Form 10-K.

## **Results of Operations**

### *Revenues.*

We had no revenues for the three and nine month periods ended September 30, 2013 and 2012.

### *Research and Development Expenses.*

Research and development expenses for the three months periods ended September 30, 2013 and 2012 were \$2,804,352 and \$654,837, respectively, including stock-based compensation expense in each of the three month periods of \$23,133 and \$18,503, respectively. Research and development expenses for the nine months periods ended September 30, 2013 and 2012 were \$6,028,691 and \$1,914,905 respectively, including stock-based compensation expense in each of the nine month periods of \$63,508 and \$55,108, respectively.

Research and development expenses, in the aggregate, represented approximately 86% and 51%, respectively, of total operating costs and expenses for the three month periods ended September 30, 2013 and 2012. Research and development expenses, in the aggregate, represented approximately 79% and 52%, respectively, of total operating costs and expenses for the nine month periods ended September 30, 2013 and 2012. The stock-based compensation is non-cash and relates to the expense of stock options awards to certain employees.

Expenses for research and development for the nine month period ended September 30, 2013 increased compared to amounts expended in the same period in 2012 as we increased our product development activities and clinical trial activities related to the currently ongoing phase 3 trial of Firdapse<sup>®</sup> for the treatment of LEMS. Since we licensed Firdapse<sup>®</sup> in October 2012, no expenses for the development of this product were included in the comparable 2012 nine month period. Expenses for the comparable period in 2012 included expenses related to the NIDA/VA Phase 2(b) clinical trial evaluating CPP-109 for use in the treatment of cocaine addiction and expenses related to our Phase 1(a) human clinical safety study for CPP-115, which were completed during 2012.

As a result of our ongoing and projected studies and trials required for us to submit an NDA for Firdapse<sup>®</sup>, we expect that costs related to research and development activities will continue to be substantial during the balance of 2013 and during 2014.

### *Selling and Marketing Expenses.*

We had no selling and marketing expenses during the three and nine month periods ended September 30, 2013 and 2012. We anticipate that we will begin to incur sales and marketing expenses when we file NDAs for our product candidates, in order to develop a sales organization to market products we may develop upon the receipt of required approvals.

*General and Administrative Expenses.*

General and administrative expenses for the three months ended September 30, 2013 and 2012 were \$441,424 and \$628,876, respectively, including stock-based compensation expense in each of the three month periods of \$23,532 and \$27,082, respectively. General and administrative expenses for the nine months ended September 30, 2013 and 2012 were \$1,576,044 and \$1,800,882, respectively, including stock-based compensation expense in each of the nine month periods of \$69,797 and \$80,657, respectively.

**Table of Contents**

General and administrative expenses represented 14% and 49%, respectively, of total operating costs and expenses for the three month periods ended September 30, 2013 and 2012. General and administrative expenses represented 21% and 48%, respectively, of total operating costs and expenses for the nine month periods ended September 30, 2013 and 2012.

The decrease in general and administrative expenses for the nine months ended September 30, 2013 as compared to those of the same period in 2012, is due primarily to decreases in professional expenses and travel expenses, as well as a reduction in payroll expenses (and related benefits) from period to period.

*Stock-Based Compensation.*

Total stock-based compensation for the three month periods ended September 30, 2013 and 2012 was \$46,665 and \$45,585, respectively. Total stock-based compensation for the nine month periods ended September 30, 2013 and 2012 was \$133,305 and \$135,765, respectively. Stock-based compensation was comparable to those of the same periods in 2012.

*Change in fair value of warrants liability.*

In connection with our October 2011 equity offering, we issued warrants to purchase an aggregate of 1,523,370 shares of common stock. The fair value of these warrants is recorded in the liability section of the balance sheet and was estimated at \$3,544,201 and \$498,587 at September 30, 2013 and December 31, 2012, respectively. The fair value of the warrants liability is determined at the end of each reporting period with the resulting gains or losses recorded as the change in fair value of warrants liability in the statements of operations.

For the three and nine months ended September 30, 2013, we recognized losses of \$2,676,601 and \$3,220,514, respectively, due to the change in the fair value of the warrants liability. The losses during the three and nine months ended September 30, 2013 were principally a result of the increase of our stock price between June 30, 2013 and September 30, 2013 and December 31, 2012 and September 30, 2013, respectively.

For the three and nine months ended September 30, 2012, we recognized losses of \$1,340,566 and \$289,440, respectively, due to the change in the fair value of the warrants liability. The losses during the three and nine months ended September 30, 2012 were principally a result of the increase of our stock price between June 30, 2012 and September 30, 2012 and December 31, 2011 and September 30, 2012, respectively.

We believe that future changes in the fair value of the warrants liability will be due primarily to future fluctuations in the market price of our common stock.

*Interest Income.*

We reported interest income in all periods relating to our investment of funds received primarily from offerings of our common stock and warrants. Total interest income for the three month periods ended September 30, 2013 and 2012 was \$10,318 and \$2,744, respectively. Total interest income for the nine month periods ended September 30, 2013 and 2012 was \$25,311 and \$5,426, respectively. The increase in interest income in the three and nine month periods ended September 30, 2013 when compared to the same periods in 2012 is due to higher average investment balances in the 2013 periods. Substantially all of our investment funds were invested in short-term interest bearing obligations.

*Income taxes.*

We have incurred net operating losses since inception. For the three and nine month periods ended September 30, 2013 and 2012, we have applied a 100% valuation allowance against our deferred tax asset as we believe that it is more likely than not that the deferred tax asset will not be realized.

**Table of Contents**

**Liquidity and Capital Resources**

Since our inception, we have financed our operations primarily through equity issuances, government grants, and an investment by a strategic purchaser. As of September 30, 2013, there is no further availability under the 2010 Shelf Registration Statement. At September 30, 2013, we had cash and cash equivalents, certificates of deposit and short-term investments aggregating \$27.7 million and working capital of \$25.6 million. At December 31, 2012, we had cash and cash equivalents, certificates of deposit and short term investments aggregating \$15.4 million and working capital of \$15.1 million. At September 30, 2013, substantially all of our cash and cash equivalents were deposited with one financial institution, and such balances were in excess of federally insured limits throughout the quarter.

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

While there can be no assurance, based on currently available information, we believe that we have the cash resources to support our operations through 2014. If our costs are greater than we expect, our assumptions may not prove to be accurate.

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

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

the scope, rate of progress and cost of our clinical trials and other product development activities;

future clinical trial results;

the terms and timing of any collaborative, licensing and other arrangements that we may establish;

the cost and timing of regulatory approvals;

the cost and delays in product development as a result of any changes in regulatory oversight applicable to our products;

the cost and timing of establishing sales, marketing and distribution capabilities;

the effect of competition and market developments;

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

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

We hope to raise additional funds to support our product development activities and working capital requirements through public or private equity offerings, corporate collaborations or other means. We also intend to seek governmental grants for a portion of the required funding for our clinical trials and pre-clinical trials. We may also seek to raise capital to fund additional product development efforts, even if we have sufficient funds for our planned operations. Any sale by us of additional equity or convertible debt securities could result in dilution to our stockholders. There can be no assurance that any such required additional funding will be available to us at all or available on terms acceptable to us. Further, to the extent that we raise additional funds through collaborative arrangements, it may be necessary to relinquish some rights to our drug candidates or grant sublicenses on terms that are not favorable to us. If we are not able to secure additional funding when needed, we may have to delay, reduce the scope of or eliminate one or more research and development programs, which could have an adverse effect on our business.

---

**Table of Contents***Cash Flows*

Net cash used in operating activities was \$5,745,415 and \$3,546,580, respectively, for the nine month periods ended September 30, 2013 and 2012. During the nine months ended September 30, 2013, net cash used in operating activities was primarily attributable to our net loss of \$10,799,938, partially offset by increases of \$1,255,480 in accrued expenses and other liabilities and \$107,678 in accounts payable, a decrease of \$320,769 in prepaid expenses and other assets, \$3,220,514 of non-cash change in fair value of warrants liability and \$150,082 of other non-cash expenses. During the nine months ended September 30, 2012, net cash used in operating activities was primarily attributable to our net loss of \$3,999,801 and a decrease of \$159,679 in accrued expenses and other liabilities. This was offset in part by \$144,001 of non-cash expenses and \$289,440 of change in the fair value of warrants liability, a \$87,484 decrease in prepaid expenses and deposits and a \$91,975 increase in accounts payable. Other non-cash expenses include depreciation and stock-based compensation expense.

Net cash used in investing activities during the nine months period ended September 30, 2013 was \$690,251 consisting primarily of purchases of short term investments of \$3,173,484 offset by redemptions of investments of \$2,492,665, and purchases of furniture and computer equipment of \$9,432. Net cash used in investing activities during the nine month period ended September 30, 2012 was \$6,882 for the purchase of furniture and equipment.

Cash provided by financing activities during the nine month period ended September 30, 2013 was \$17,988,693, consisting of \$14,071,694 from the net proceeds from the sale of common stock under the 2010 shelf registration statement, \$3,893,499 from proceeds of warrant exercises and \$23,500 from proceeds from the exercise of stock options. Cash provided by financing activities during the nine month period ended September 30, 2012 was \$9,498,255, consisting of \$3,938,303 from net proceeds from the sale of common stock and warrants through a secondary public offering and \$5,559,952 from the net proceeds from the sale of common stock and warrants under the 2010 shelf registration statement.

*Contractual Obligations*

We have entered into the following contractual arrangements:

*Payments to BioMarin and others under our license agreement.* We have agreed: (i) to pay BioMarin certain royalty payments based on our net sales in North America; (ii) to pay to a third-party licensor of the rights sublicensed to us certain royalty payments based on our net sales in North America, and (iii) to pay certain milestone payments that BioMarin is obligated to make (approximately \$2.6 million of which will be due upon acceptance by the FDA of a filing of an NDA for Firdapse for the treatment of LEMS, and approximately \$7.2 million of which will be due on the unconditional approval by the FDA of an NDA for Firdapse for the treatment of LEMS). We have also agreed to share in the cost of certain post-marketing studies that are being conducted by BioMarin if such studies are required as a condition for approval of the product by the FDA. However, no such payments will be due until the FDA has accepted for filing an NDA for Firdapse .

*Payments for Firdapse development.* Based on currently available information, we estimate that we will spend between \$20 million and \$25 million conducting the clinical, non-clinical and safety evaluations, and manufacturing the three validation batches that will be required to be submitted, in order for us to obtain an NDA for Firdapse for the treatment of LEMS. Further, if we are successful in obtaining an NDA for Firdapse , we will also incur substantial costs in the future associated with required post-marketing studies of Firdapse and in

connection with the commercialization of the product.

At September 30, we had paid approximately \$3.3 million of this amount and had prepaid research fees of \$842,429, accounts payable of approximately \$1,395,880 and accrued liabilities of approximately \$1,287,384 in the accompanying condensed balance sheet in connection with related agreements. Under our license agreement with BioMarin, we are obligated to spend at least \$5 million in connection with the Phase 3 trial of Firdapse during the two years following the date of the license agreement (October 26, 2012). We currently expect that we will spend more than \$5 million on the Phase 3 trial during 2013.

## **Table of Contents**

*Payments to Northwestern University under our license agreement.* Under our license agreement with Northwestern, we have paid to date \$246,590, had accrued liabilities of \$51,250, at September 30, 2013 in the accompanying condensed balance sheet, and owe certain milestone payments in future years if we do not cancel the license agreement. The next milestone payment of \$150,000 is due on the earlier of August 27, 2015 or the successful completion of the first Phase 2 trial of CPP-115.

*Employment agreements.* We have entered into an employment agreement with our Chief Executive Officer that requires us to make base salary payments of approximately \$406,000 per annum in 2013. The agreement expires in November 2016.

*Leases for office space.* We have entered into a lease agreement for our office space that requires payments of approximately \$6,000 per month.

### *Off-Balance Sheet Arrangements*

We currently have no debt. Capital lease obligations as of September 30, 2013 and December 31, 2012 were not material. We have an operating lease for our corporate office facility. We do not have any off-balance sheet arrangements as such term is defined in rules promulgated by the SEC.

## **Caution Concerning Forward-Looking Statements**

This Quarterly Report on Form 10-Q 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 used to identify forward-looking statements. Such statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by such forward looking statements. The forward-looking statements made in this report are based on current expectations that involve numerous risks and uncertainties.

The successful development 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 pre-clinical studies, proof-of-concept studies and clinical studies and trials and other product development activities;

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

whether our studies and trials will be successful;

the results of our pre-clinical studies and clinical studies and trials, and the number and scope of such studies and trials that will be required for us to seek and obtain approval of NDAs for our product candidates;

whether the third parties we retain to assist us in our trials and studies perform as contracted for 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 3,4-DAP for the treatment of LEMS first;

**Table of Contents**

whether others develop and commercialize products competitive to our products;

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; and

changes in general economic conditions and interest rates.

For further information regarding the risks associated with our business, see the **Risk Factors** section of our Annual Report on Form 10-K for the fiscal year ended December 31, 2012.

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.

**Table of Contents**

**ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURE ABOUT MARKET RISK**

As a smaller reporting company as defined by Item 10 of Regulation S-K we are not required to provide the information required by this section.

**ITEM 4. CONTROLS AND PROCEDURES**

- a.** We have carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rules 13a-15(c) and 15d-15(e) of the Securities Exchange Act of 1934, as amended (the Exchange Act)). Based on such evaluation, our principal executive officer and principal financial officer have concluded that as of September 30, 2013, our disclosure controls and procedures were effective to ensure that the information required to be disclosed by us in the reports filed or submitted by us under the Exchange Act, was recorded, processed, summarized or reported within the time periods specified in the rules and regulations of the SEC, and include controls and procedures designed to ensure that information required to be disclosed by us in such reports was accumulated and communicated to management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosures.
- b.** During the three months ended September 30, 2013, there were no changes in our internal controls or in other factors that could have a material effect, or are reasonably likely to have a material effect, on our internal control over financial reporting.

**Table of Contents**

**PART II. OTHER INFORMATION**

**ITEM 1. LEGAL PROCEEDINGS**

In October 2013, a securities class action lawsuit was filed against the Company and certain of the Company's executive officers and directors seeking unspecified damages in the U.S. District Court for the Southern District of Florida (Case No. 1:13-cv-23878-UU). The complaint purports to state a claim for alleged violations of Section 10(b) and 20(a) of the Securities Exchange Act of 1934 and Rule 10b-5 thereunder based on alleged misrepresentations regarding the development of Firdapse. The case seeks damages on behalf of a class of those who purchased shares of the Company's common stock between October 31, 2012 and October 18, 2013. The Company believes this lawsuit, which is at a very early stage, to be without merit, and the Company intends to vigorously defend this lawsuit. While there can be no assurance, the Company does not expect that this lawsuit will have a material adverse effect on the Company.

The Company is not a party to any other legal proceedings.

**ITEM 1A. RISK FACTORS**

There are many factors that affect our business, our financial condition, and the results of our operations. In addition to the information set forth in this quarterly report, you should carefully read and consider Item 1A. Risk Factors in Part I, and Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations in Part II, of our 2012 Annual Report on Form 10-K filed with the SEC, which contain a description of significant factors that might cause our actual results of operations in future periods to differ materially from those currently expected or desired.

**ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS**

None

**ITEM 3. DEFAULTS UPON SENIOR SECURITIES**

None

**ITEM 4. MINE SAFETY DISCLOSURE**

Not applicable

**ITEM 5. OTHER INFORMATION**

None

**ITEM 6. EXHIBITS**

10.1 Termination Agreement between Brookhaven Science Associates, LLC and Catalyst Pharmaceutical Partners, Inc.

Edgar Filing: CATALYST PHARMACEUTICAL PARTNERS, INC. - Form 10-Q

31.1	Certification of Principal Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Certification of Principal Financial Officer under Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Certification of Principal Executive Officer under Section 906 of the Sarbanes-Oxley Act of 2002
32.2	Certification of Principal Financial Officer under Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema
101.CAL	XBRL Taxonomy Extension Calculation Linkbase
101.DEF	XBRL Taxonomy Extension Definition Linkbase
101.LAB	XBRL Taxonomy Extension Label Linkbase
101.PRE	XBRL Taxonomy Extension Presentation Linkbase

**Table of Contents**

**SIGNATURES**

Pursuant to the Securities Exchange Act of 1934, the Registrant has caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

**Catalyst Pharmaceutical Partners, Inc.**

By: /s/ Alicia Grande  
Alicia Grande  
Vice President, Treasurer and Chief Financial Officer

Date: November 13, 2013

---

**Table of Contents****Exhibit Index**

Exhibit	
Number	Description
10.1	Termination Agreement between Brookhaven Science Associates, LLC and Catalyst Pharmaceutical Partners, Inc.
31.1	Certification of Principal Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Certification of Principal Financial Officer under Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Certification of Principal Executive Officer under Section 906 of the Sarbanes-Oxley Act of 2002
32.2	Certification of Principal Financial Officer under Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema
101.CAL	XBRL Taxonomy Extension Calculation Linkbase
101.DEF	XBRL Taxonomy Extension Definition Linkbase
101.LAB	XBRL Taxonomy Extension Label Linkbase
101.PRE	XBRL Taxonomy Extension Presentation Linkbase