CATALYST PHARMACEUTICALS, INC.
Form 10-Q
November 09, 2016
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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, 2016

OR

TRANSITION REPORT UNDER SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

Commission File No. 001-33057

CATALYST PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**76-0837053**
(State or other jurisdiction of	**(IRS Employer**
incorporation or organization)	**Identification No.)**
355 Alhambra Circle	
Suite 1250	
Coral Gables, Florida	**33134**
(Address of principal executive offices)	**(Zip Code)**

Registrant s telephone number, including area code: (305) 420-3200

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	Accelerated Filer
Non-Accelerated Filer (Do not check if a smaller reporting company)	Smaller reporting company

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 82,870,649 shares of common stock, $0.001 par value per share, were outstanding as of November 4, 2016.

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CATALYST PHARMACEUTICALS, INC.

BALANCE SHEETS

	September 30, 2016 (unaudited)	December 31, 2015
ASSETS		
Current Assets:		
Cash and cash equivalents	$ 17,638,100	$ 28,235,016
Certificates of deposit	568,031	3,717,229
Short-term investments	26,538,304	26,444,150
Prepaid expenses and other current assets	310,855	1,504,738
Total current assets	45,055,290	59,901,133
Property and equipment, net	249,038	191,549
Deposits	8,888	8,888
Total assets	$ 45,313,216	$ 60,101,570
LIABILITIES AND STOCKHOLDERS EQUITY		
Current Liabilities:		
Accounts payable	$ 1,055,324	$ 1,794,127
Accrued expenses and other liabilities	940,271	1,646,476
Total current liabilities	1,995,595	3,440,603
Accrued expenses and other liabilities, non-current	185,095	176,293
Warrants liability, at fair value	229,172	1,008,363
Total liabilities	2,409,862	4,625,259
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, 150,000,000 shares authorized; 82,870,649 shares and 82,850,619 shares issued and outstanding at September 30, 2016 and December 31, 2015, respectively	82,871	82,851
Additional paid-in capital	146,805,233	145,469,078
Accumulated deficit	(103,984,750)	(90,075,618)
Total stockholders equity	42,903,354	55,476,311
Total liabilities and stockholders equity	$ 45,313,216	$ 60,101,570

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

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CATALYST PHARMACEUTICALS, INC.

STATEMENTS OF OPERATIONS (unaudited)

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2016	2015	2016	2015
Operating costs and expenses:				
Research and development	$ 2,493,999	$ 3,042,671	$ 8,549,287	$ 7,969,731
General and administrative	1,420,015	1,974,757	6,416,715	6,236,942
Total operating costs and expenses	3,914,014	5,017,428	14,966,002	14,206,673
Loss from operations	(3,914,014)	(5,017,428)	(14,966,002)	(14,206,673)
Other income, net	66,981	46,659	277,679	113,464
Change in fair value of warrants liability	(106,948)	521,731	779,191	(324,591)
Loss before income taxes	(3,953,981)	(4,449,038)	(13,909,132)	(14,417,800)
Provision for income taxes				
Net loss	$ (3,953,981)	$ (4,449,038)	$ (13,909,132)	$ (14,417,800)
Net loss per share basic and diluted	$ (0.05)	$ (0.05)	$ (0.17)	$ (0.18)
Weighted average shares outstanding basic and diluted	82,870,649	82,470,139	82,867,140	80,205,864

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

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CATALYST PHARMACEUTICALS, INC.

STATEMENT OF STOCKHOLDERS EQUITY (unaudited)

For the nine months ended September 30, 2016

	Preferred Stock	Common Stock	Additional Paid-in Capital	Accumulated Deficit	Total
Balance at December 31, 2015	$	$ 82,851	$ 145,469,078	$ (90,075,618)	$ 55,476,311
Issuance of stock options for services			1,290,943		1,290,943
Amortization of restricted stock for services			56,497		56,497
Exercise of stock options for common stock		20	(11,285)		(11,265)
Net loss				(13,909,132)	(13,909,132)
Balance at September 30, 2016	$	$ 82,871	$ 146,805,233	$ (103,984,750)	$ 42,903,354

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

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CATALYST PHARMACEUTICALS, INC.

STATEMENTS OF CASH FLOWS (unaudited)

	For the Nine Months Ended, September 30,	
	2016	**2015**
Operating Activities:		
Net loss	$ (13,909,132)	$ (14,417,800)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	31,442	28,382
Stock-based compensation	1,347,440	1,009,116
Change in fair value of warrants liability	(779,191)	324,591
Decrease (increase) in:		
Prepaid expenses and other current assets and deposits	1,193,883	(341,220)
(Decrease) increase in:		
Accounts payable	(738,803)	(997,189)
Accrued expenses and other liabilities	(697,403)	1,010,221
Net cash used in operating activities	(13,551,764)	(13,383,899)
Investing Activities:		
Capital expenditures	(88,931)	(23,465)
Purchase of short-term investments	(94,154)	(32,071)
Proceeds (purchase) of certificates of deposit	3,149,198	(1,440)
Net cash provided by (used in) investing activities	2,966,113	(56,976)
Financing Activities:		
Proceeds from issuance of common stock, net		34,873,869
Payment of employee withholding tax related to stock-based compensation	(11,265)	
Proceeds from exercise of warrants		1,895,738
Proceeds from exercise of stock options		324,949
Net cash (used in) provided by financing activities	(11,265)	37,094,556
Net (decrease) increase in cash and cash equivalents	(10,596,916)	23,653,681
Cash and cash equivalents - beginning of period	28,235,016	9,096,778
Cash and cash equivalents - end of period	$ 17,638,100	$ 32,750,459
Supplemental disclosures of non-cash investing and financing activity		
Exercise of liability classified warrants for common stock	$	$ 1,721,523
Non-cash incentive received from lessor	$	$ 131,175

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

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CATALYST PHARMACEUTICALS, INC.

NOTES TO UNAUDITED FINANCIAL STATEMENTS

1. Organization and Description of Business.

Catalyst Pharmaceuticals, Inc. (the Company) is a development-stage biopharmaceutical company focused on developing and commercializing innovating therapies for people with rare debilitating diseases, including Lambert-Eaton Myasthenic Syndrome (LEMS), Congenital Myasthenic Syndromes (CMS), infantile spasms and Tourette s Disorder.

Since inception, the Company has devoted substantially all of its efforts to business planning, research and development, recruiting management and technical staff, acquiring operating assets and raising capital. The Company s primary focus is on the development and commercialization of its drug candidates. The Company has incurred operating losses in each period from inception through September 30, 2016. 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 January 31, 2014, the Company filed a Shelf Registration Statement on Form S-3 (the 2014 Shelf Registration Statement) with the U.S. Securities Exchange Commission (SEC) to sell up to $100 million of common stock. This registration statement (file No. 333-193699) was declared effective by the SEC on March 19, 2014. The Company has conducted two registered direct offerings under the 2014 Shelf Registration Statement. See Note 9.

While there can be no assurance, based on currently available information, the Company estimates that it has sufficient resources to support its operations for at least the next year.

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. **INTERIM FINANCIAL STATEMENTS.** The accompanying unaudited interim financial statements have been prepared in accordance with U.S. generally accepted accounting principles (U.S. GAAP), and pursuant to the rules and regulations of the SEC for reporting of interim financial information.

Pursuant to such rules and regulations, certain information and note disclosures normally included in financial statements prepared in accordance with U.S. GAAP have been omitted. The balance sheet as of December 31, 2015 included in this Form 10-Q was derived from the audited financial statements and does not include all disclosures required by U.S. GAAP.

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

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, 2015 included in the 2015 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, 2016 are not necessarily indicative of the results to be expected for any future period or for the full 2016 fiscal year.

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

c. **CASH AND CASH EQUIVALENTS.** The Company considers all highly liquid instruments purchased with an original maturity of three months or less to be cash equivalents. Cash equivalents consist mainly of money market funds. The Company has substantially all of its cash and cash equivalents deposited with one financial institution. These amounts at times may exceed federally insured limits.

d. **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, 2016 and December 31, 2015 approximates fair value.

e. **SHORT-TERM INVESTMENTS.** The Company invests in short-term investments in high credit-quality funds in order to obtain higher yields on its cash available for investments. As of September 30, 2016 and December 31, 2015, 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, 2016 and December 31, 2015 are 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 gain (loss) for the three and nine months ended September 30, 2016 were $0 and $88,291, respectively. Unrealized gain (loss) for the three and nine months ended September 30, 2015 were $29,430 and $29,430 respectively. Unrealized gain (loss) for each period is included in other income, net in the accompanying statements of operations.

f. **PREPAID EXPENSES AND OTHER CURRENT ASSETS**. Prepaid expenses and other current assets consist primarily of prepaid research fees, prepaid pre-commercialization expenses, prepaid insurance and prepaid subscription fees. Prepaid research fees consist of advances for the Company s product development activities, including drug manufacturing, contracts for preclinical studies, clinical

trials and studies, regulatory affairs and consulting. Such advances are recorded as expense as the related goods are received or the related services are performed.

g. **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, 2016 and December 31, 2015, the fair value of these instruments approximated their carrying value.

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

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

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

	Fair Value Measurements at Reporting Date Using			
	Balances as of September 30, 2016	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	$ 17,329,687	$ 17,329,687	$	$
Certificates of deposit	$ 568,031	$	$ 568,031	$
Short-term investments	$ 26,538,304	$ 26,538,304	$	$
Warrants liability	$ 229,172	$	$	$ 229,172

	Fair Value Measurements at Reporting Date Using			
	Balances as of December 31,	Quoted Prices in Active Markets	Significant Other Observable	Significant Unobservable Inputs

	2015	for Identical Assets/Liabilities (Level 1)	Inputs (Level 2)	(Level 3)
Money market funds	$ 25,157,601	$ 25,157,601	$	$
Certificates of deposit	$ 3,717,229	$	$ 3,717,229	$
Short-term investments	$ 26,444,150	$ 26,444,150	$	$
Warrants liability	$ 1,008,363	$	$	$ 1,008,363

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

i. WARRANTS LIABILITY. In October 2011, the Company issued 1,523,370 warrants (the 2011 warrants) to purchase shares of the Company s common stock in connection with a registered direct offering under the 2010 Shelf Registration Statement. The Company accounted for these warrants as a liability measured at fair value due to a provision included in the warrants agreement that provides the warrants holders with an option to require the Company (or its successor) to purchase their warrants for cash in an amount equal to their Black-Scholes Option Pricing Model (the Black-Scholes Model) value, in the event that certain fundamental transactions, as defined, occur. The fair value of the warrants liability is estimated using the Black-Scholes Model which requires inputs such as the expected term of the warrants, share price volatility and risk-free interest rate. These assumptions are reviewed on a quarterly basis and changes in the estimated fair value of the outstanding warrants are recognized each reporting period in the Change in fair value of warrants liability line in the statement of operations. As of both September 30, 2016 and December 31, 2015, 763,913 of the 2011 warrants remained outstanding. The 2011 warrants expire in May 2017.

j. STOCK-BASED COMPENSATION. The Company recognizes expense in the statement of operations for the fair value of all stock-based payments to employees, directors, scientific advisors and consultants, including grants of stock options and other share-based awards. For stock options, the Company uses the Black-Scholes option valuation model, the single-option award approach, and the straight-line attribution method. Using this approach, compensation cost is amortized on a straight-line basis over the vesting period of each respective stock option, generally one to three years. The Company estimates forfeitures and adjusts this estimate periodically based on actual forfeitures. As of September 30, 2016, there were outstanding stock options to purchase 5,150,000 shares of common stock, of which stock options to purchase 2,451,662 shares of common stock were exercisable as of September 30, 2016.

For the three and nine month periods ended September 30, 2016 and 2015, the Company recorded stock-based compensation expense as follows:

	Three months ended September 30,		Nine months ended September 30,	
	2016	2015	2016	2015
Research and development	$ 185,122	$ 113,612	$ 443,297	$ 253,756
General and administrative	347,080	217,158	904,143	755,360
Total stock-based compensation	$ 532,202	$ 330,770	$ 1,347,440	$ 1,009,116

k. COMPREHENSIVE INCOME (LOSS). U.S. GAAP 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).

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

l. 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:

	September 30,	
	2016	**2015**
Options to purchase common stock	5,150,000	3,240,000
Warrants to purchase common stock	2,407,663	2,407,663
Unvested restricted stock	53,334	80,000
Potential equivalent common stock excluded	7,610,997	5,727,663

Potentially dilutive options to purchase common stock as of both September 30, 2016 and 2015 have exercise prices ranging from $0.47 to $4.64. Potentially dilutive warrants to purchase common stock as of both September 30, 2016 and 2015 have exercise prices ranging from $1.04 to $2.08 and expire in periods between May 2017 and August 2017.

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

In February 2016, the FASB issued ASU No. 2016-02, *Leases* (*Topic 842)*, which requires an entity to recognize assets and liabilities arising from a lease for both financing and operating leases. The ASU will also require new qualitative and quantitative disclosures to help investors and other financial statement users better understand the amount, timing, and uncertainty of cash flows arising from leases. ASU 2016-02 is effective for fiscal years beginning after December 15, 2018, with early adoption permitted. The Company is currently evaluating the impact this accounting standard will have on its financial statements.

In March, 2016, the FASB issued ASU No. 2016-09, *Compensation Stock Compensation* (Topic 718): *Improvements to Employee Share-Based Payment Accounting*, which simplifies several aspects of the accounting for employee share-based payment transactions for both public and nonpublic entities, including the accounting for income taxes, forfeitures, and statutory tax withholding requirements, as well as classification in the statement of cash flows. For public companies, the changes are effective for reporting periods (annual and interim) beginning after December 15, 2016. Early adoption is permitted. However, if early adoption is elected in an interim period, any adjustments should be reflected as of the beginning of the annual period that includes that interim period. The Company is currently evaluating the effect this standard will have on its 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.

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3. Warrants Liability, at Fair Value (continued).

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

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

	September 30, 2016	December 31, 2015
Risk free interest rate	0.59%	0.79%
Expected term	0.59 years	1.34 years
Expected volatility	112%	68%
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, 2016 and 2015:

	Three months ended September 30,		Nine months ended September 30,	
	2016	2015	2016	2015
Fair value, beginning of period	$ 122,224	$ 2,979,038	$ 1,008,363	$ 2,794,891
Issuance of warrants				
Exercise of warrants		(1,059,348)		(1,721,523)
Change in fair value	106,948	(521,731)	(779,191)	324,591
Fair value, end of period	$ 229,172	$ 1,397,959	$ 229,172	$ 1,397,959

During the three and nine months ended September 30, 2016 none of the 2011 warrants were exercised. During the three and nine months ended September 30, 2015, 239,130 and 478,261 of the 2011 warrants were exercised, with proceeds to the Company of $310,869 and $691,739, respectively. 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 and Other Current Assets.

Prepaid expenses and other current assets consist of the following:

	September 30, 2016	December 31, 2015
Prepaid research fees	$ 112,971	$ 915,194
Prepaid insurance	78,323	436,726
Prepaid pre-commercialization fees	26,855	90,248
Prepaid subscription fees	36,893	26,602
Prepaid rent	19,660	1,252
Other	36,153	34,716
Total prepaid expenses and other current assets	$ 310,855	$ 1,504,738

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5. Property and Equipment.

Property and equipment, net consists of the following:

	September 30, 2016	December 31, 2015
Computer equipment	$ 27,915	$ 27,915
Furniture and equipment	169,931	102,533
Leasehold improvements	152,708	131,175
	350,554	261,623
Less: Accumulated depreciation	(101,516)	(70,074)
Total property and equipment, net	$ 249,038	$ 191,549

Depreciation expense was $9,482 and $31,442, respectively, for the three and nine month periods ended September 30, 2016 and $11,259 and $28,382 for the three and nine month periods ended September 30, 2015, respectively.

6. Accrued Expenses and Other Liabilities.

Accrued expenses and other liabilities consist of the following:

	September 30, 2016	December 31, 2015
Accrued preclinical and clinical trial expenses	$ 287,793	$ 332,905
Accrued professional fees	37,853	330,490
Accrued compensation and benefits	445,325	894,846
Accrued license fees	126,250	52,500
Deferred rent and lease incentive	18,093	18,093
Other	24,957	17,642
Current accrued expenses and other liabilities	940,271	1,646,476
Deferred rent and lease incentive - non-current	185,095	176,293
Non-current accrued expenses and other liabilities	185,095	176,293
Total accrued expenses and other liabilities	$ 1,125,366	$ 1,822,769

Accrued compensation and benefits as of September 30, 2016 consists principally of accrued severance in connection with the reduction in workforce that occurred during May 2016.

7. Commitments and Contingencies.

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

Under the license agreement with Northwestern, the Company is responsible for continued research and development of any resulting product candidates. As of September 30, 2016, the Company has paid $411,590 in connection with the license and has accrued license fees of $126,250 in the accompanying September 30, 2016 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 $300,000 is due on the earlier of successful completion of the first Phase 3 clinical trial for CPP-115 or August 27, 2018.

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7. Commitments and Contingencies (continued).

b. LICENSE AGREEMENT WITH NEW YORK UNIVERSITY AND THE FEINSTEIN INSTITUTE FOR MEDICAL RESEARCH. On December 13, 2011, the Company entered into a license agreement with New York University (NYU) and the Feinstein Institute for Medical Research (FIMR) under which it acquired worldwide rights to commercialize GABA aminotransferase inhibitors in the treatment for Tourette s Disorder. 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.

c. LICENSE AGREEMENT WITH BIOMARIN. On October 26, 2012, the Company entered into a strategic collaboration with BioMarin Pharmaceutical, Inc. (BioMarin) for Firdapse® under which: (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 to further the development of Firdapse®.

As part of the License Agreement, the Company agreed: (i) to pay BioMarin royalties for seven years from the first commercial sale of Firdapse® equal to 7% of net sales (as defined in the license agreement) in North America for any calendar year for sales up to $100 million, and 10% of net sales in North America in any calendar year in excess of $100 million; (ii) to pay to the third-party licensor of the rights sublicensed to the Company royalty payments for seven years from the first commercial sale of Firdapse® equal to 7% of net sales (as defined in the license agreement between BioMarin and the third-party licensor) in any calendar year; and (iii) to pay certain milestone payments that BioMarin is obligated to pay (approximately $2.6 million of which will be due upon acceptance by the FDA of a filing of an NDA for Firdapse® for the treatment of LEMS, and approximately $7.2 million of which will be due on the unconditional approval by the FDA of an NDA for Firdapse® for the treatment of LEMS). The Company also agreed to share in the cost of certain post-marketing studies being conducted by BioMarin, and, as of September 30, 2016, the Company had paid BioMarin $3.8 million related to expenses in connection with Firdapse® studies and trials.

d. 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 2013. 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.

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9. Stockholders Equity.

2014 Shelf Registration Statement

On January 31, 2014, the Company filed a Shelf Registration Statement on Form S-3 (the 2014 Shelf Registration Statement) with the SEC to sell up to $100 million of common stock. This registration statement (file No. 333-193699) was declared effective by the SEC on March 19, 2014. The Company has to date conducted the following sales under the 2014 Shelf Registration Statement:

(a) On April 3, 2014, the Company filed a prospectus supplement and offered for sale 13,023,750 shares of its common stock at a price of $2.21 per share in an underwritten public offering. The Company received gross proceeds in the public offering of approximately $28.8 million before underwriting commission and incurred expenses of approximately $2.1 million.

(b) On February 4, 2015, the Company filed a prospectus supplement and offered for sale 11,500,000 shares of its common stock at a price of $3.25 per share in an underwritten public offering. The Company received gross proceeds in the public offering of approximately $37.4 million before underwriting commission and incurred expenses of approximately $2.5 million.

Warrant Exercises

No warrants were exercised during the three and nine months ended September 30, 2016. During the three and nine months ended September 30, 2015, the Company issued an aggregate of 374,130 and 1,178,261 shares of its authorized but unissued common stock upon the exercise of previously issued common stock purchase warrants, raising gross proceeds of $591,668 and $1,895,738, respectively.

Nasdaq Listing

The Company s common stock currently trades on the Nasdaq Capital Market. On June 9, 2016, 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. However, on September 30, 2016, the Company received notice from Nasdaq confirming that the Company had regained compliance as a result of the Company s common stock having closed with a bid price of at least $1.00 for at least ten consecutive trading days.

Shareholder Rights Plan

On September 19, 2016, the Board of Directors of the Company unanimously approved, and on the same date the Company entered into Amendment No. 1 to the Rights Agreement (the Amendment), which amends that certain Rights Agreement, dated as of September 20, 2011, between the Company and Continental Stock Transfer and Trust Company, as rights agent (the Rights Agreement). Under the terms of the Amendment, the outside expiration date of the rights granted under the Rights Agreement has been extended from September 20, 2016 to September 20, 2019. Additionally, as part of the Amendment, the Board adopted a Certificate of Designation, Preferences and Rights of Series A Junior Participating Preferred Stock of the Company to increase the number of shares of Series A Junior Participating Preferred Stock of the Company available for issuance under the Rights Agreement from 500,000 shares

to 1.5 million shares.

10. Stock Compensation.

Stock Options

During the three and nine month periods ended September 30, 2016, the Company granted seven-year options to purchase an aggregate of 15,000 and 1,260,000 shares of the Company s common stock to employees and directors. The option awards vest over a period of 1 to 3 years of continuous service. The Company recorded stock-based compensation related to stock options totaling $513,231 and $1,290,943 respectively, during the three and nine month periods ended September 30, 2016. During the three and nine month periods ended September 30, 2016, respectively, 256,668 and 488,333 options vested.

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10. Stock Compensation (continued).

During the three and nine month periods ended September 30, 2015, the Company granted seven-year options to purchase an aggregate of 150,000 and 565,000 shares of the Company s common stock to employees and directors, respectively. The options vest over a period of 2 to 3 years. The Company recorded stock-based compensation related to stock options totaling $311,748 and $952,670 respectively, during the three and nine month periods ended September 30, 2015. During the three and nine month periods ended September 30, 2015, respectively, 366,664 and 461,664 options vested.

No options were exercised during the three months ended September 30, 2016. During the nine months ended September 30, 2016, options to purchase 50,000 shares of the Company s common stock were exercised on a cashless basis, resulting in the issuance of an aggregate 20,030 shares of the Company s common stock.

During the three and nine month periods ended September 30, 2015, options to purchase 205,000 shares of the Company s common stock were exercised with proceeds of $324,949. Further, during the three and nine months ended September 30, 2015, options to purchase 50,000 and 879,608 shares of the Company s common stock were exercised on a cashless basis, resulting in the issuance of an aggregate of 19,136 and 692,719 shares of the Company s common stock.

As of September 30, 2016, there was approximately $2,847,000 of unrecognized compensation expense related to non-vested stock option awards granted under the 2006 and 2014 Stock Incentive Plans. The cost is expected to be recognized over a weighted average period of approximately 1.72 years.

Restricted Stock Units

No restricted stock units were granted during the three and nine months ended September 30, 2016 and 2015. The Company recorded stock-based compensation related to restricted stock units totaling $18,971 and $56,497, respectively, during the three and nine month periods ended September 30, 2016. The Company recorded stock-based compensation related to restricted stock units totaling $19,022 and $56,446 during the three and nine month periods ended September 30, 2015. As of September 30, 2016, there was approximately $84,000 of total restricted stock unit compensation expense related to non-vested awards not yet recognized, which is expected to be recognized over a weighted average period of 1.12 years.

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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 and information about our business that we believe is important in understanding our financial condition and results of operations.

Basis of Presentation. This section provides information about key accounting estimates and policies that we followed in preparing our financial statements for the third quarter of fiscal 2016.

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 months ended September 30, 2016 as compared to the same periods ended September 30, 2015.

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

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

Overview

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

Firdapse®

In October 2012, we licensed the North American rights to Firdapse®, a proprietary form of amifampridine phosphate, or chemically known as 3,4-diaminopyridine phosphate, from BioMarin Pharmaceutical Inc. (BioMarin).

In August 2013, we were granted breakthrough therapy designation by the U.S. Food & Drug Administration (FDA) for Firdapse® for the treatment of patients with Lambert-Eaton Myasthenic Syndrome, or LEMS, a rare and sometimes fatal autoimmune disease characterized by muscle weakness. In March 2015, we were granted Orphan Drug Designation for Firdapse® for the treatment of patients with Congenital Myasthenic Syndromes, or CMS, and in August 2016, we were granted Orphan Drug Designation for Firdapse® for the treatment of patients with myasthenia gravis.

The chemical entity, amifampridine (3,4-diaminopyridine or 3,4-DAP), has never been approved by the FDA for any indication. Because Firdapse® has been granted Orphan Drug designation for the treatment of LEMS, CMS and myasthenia gravis by the FDA, the product is also eligible to receive seven years of marketing exclusivity for any or all of these indications. Further, if we are the first pharmaceutical company to obtain approval for an amifampridine product, of which there can be no assurance, we will be eligible to receive five years of marketing exclusivity with respect to the use of this product for any indication, running concurrently with the seven years of orphan marketing exclusivity described above (if both exclusivities are granted).

We previously sponsored a multi-center, randomized, placebo-controlled Phase 3 trial evaluating Firdapse® for the treatment of LEMS. The Phase 3 trial, which involved 38 subjects, was designed as a

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randomized withdrawal trial in which all patients were treated with Firdapse® during a 7 to 91-day run-in-period followed by treatment with either Firdapse® or placebo over a two-week randomization period. The co-primary endpoints for this Phase 3 trial were the comparison of changes in patients randomized to continue Firdapse® versus those who transitioned to placebo that occurred in both the Quantitative Myasthenia Gravis Score (QMG), which measures muscle strength, and subject global impression score (SGI), on which the subjects rate their global impression of the effects of a study treatment during the two-week randomization period. In September 2014, we reported positive top-line results from this Phase 3 trial.

During 2014, we established an expanded access program (EAP) to make Firdapse® available to any patients diagnosed with LEMS, CMS, or Downbeat Nystagmus in the United States, who meet the inclusion and exclusion criteria, with Firdapse® being provided to patients for free until sometime after new drug application (NDA) approval, should we receive such approval (of which there can be no assurance). We are informing neuromuscular physicians on the availability of the Firdapse® EAP and working with various rare disease advocacy organizations to inform patients and physicians about the program.

On December 17, 2015, we announced completion of the submission of an NDA for Firdapse® for the treatment of LEMS and CMS. However, on February 17, 2016, we announced that we had received a refusal to file letter from the FDA regarding our NDA submission. In early April 2016, we met with the FDA to obtain greater clarity regarding what will be required by the FDA to accept the Firdapse® NDA for filing. Following the receipt of the formal minutes of that meeting, on April 26, 2016, we issued a press release reporting that the FDA has stated that in addition to the results of the Company s previously submitted multi-center, randomized, placebo-controlled Phase 3 trial, we will need to submit positive results from a second adequate and well-controlled study in patients with LEMS. Additionally, there is a requirement for several more short-term toxicology studies, which are currently in process.

In October 2016, we announced that we had reached an agreement with the FDA under a Special Protocol Assessment (SPA) for the protocol design, clinical endpoints, and statistical analysis approach to be taken in our upcoming second Phase 3 study evaluating Firdapse® (amifampridine phosphate) for the symptomatic treatment of LEMS. A SPA is a process by which sponsors ask the FDA to evaluate the protocol of a proposed clinical trial to determine whether it adequately addresses scientific and regulatory requirements for the purpose identified by the sponsor. A SPA agreement indicates concurrence with the adequacy and acceptability of specific critical elements of protocol design, endpoints and analysis. Additionally, it provides a binding agreement with FDA s review division that a pivotal trial design, conduct, and planned analysis adequately addresses the scientific and regulatory objectives in support of a regulatory submission for drug approval. However, final marketing approval depends upon the results of efficacy, the safety profile, and an evaluation of the risk/benefit of treatment demonstrated in the Phase 3 clinical trial, among other requirements.

We intend to conduct our second Phase 3 trial evaluating Firdapse® for the treatment of LEMS (designated as LMS-003) at two clinical trial sites, one on the east coast of the United States and one on the west coast of the United States. This double-blind, placebo-controlled withdrawal trial will include approximately 28 subjects, so that the trial is adequately powered, and will have the same co-primary endpoints as our first Phase 3 trial evaluating Firdapse® for the treatment of LEMS. Further, the FDA has agreed to allow us to enroll patients from our expanded access program as study subjects in this second trial. Finally, after further discussion with the FDA in connection with the SPA request, this second trial will be a parallel design and not a cross-over design. Final details of the Phase 3 clinical trial will be available at the launch of the study on www.clinicaltrials.gov.

As previously reported, we expect to initiate this trial before the end of this year and to report top-line results from this second trial during the second half of 2017. Assuming the results of this second trial are successful, and our anticipated timeline for this trial is met, we expect to resubmit an NDA for Firdapse® for the treatment of LEMS in

the second half of 2017. There can be no assurance as to the timing or requirements of this confirmatory study, whether this additional study will be sufficient for the FDA to accept for filing any NDA that we might resubmit in the future for Firdapse®, or whether Firdapse® will ever be approved for commercialization.

Our original NDA submission for Firdapse® included data and information (including data from a currently ongoing investigator treatment IND) providing evidence supporting the benefits of Firdapse® for treating certain types of CMS, and requested that CMS be included in our initial label for Firdapse®. To provide additional support for our submission of an NDA for Firdapse® for the treatment of CMS, in October 2015 we

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initiated a small blinded clinical trial at four academic centers of up to 10 subjects in the pediatric CMS population, ages 2 to 17. However, after discussions with the FDA, we have determined to enroll both adult and pediatric subjects with CMS in this trial and to expand the number of subjects to be evaluated in the trial to an aggregate of approximately 20 subjects. We have also added a fifth trial site for this study. Finally, we are currently discussing the primary and second endpoints to be used in this trial with the FDA.

Based on currently available information, we expect to report top line results from this study in the second half of 2017 and if the results of the study are successful, we hope to add the CMS indication to our label for Firdapse® (either as a part of our NDA resubmission for Firdapse® for LEMS or as a supplement to that resubmission). There can be no assurance that any trial we perform for Firdapse® for the treatment of CMS will be successful or whether any NDA that we may submit for Firdapse® for the treatment of CMS will be filed by the FDA for review and approved.

Firdapse® is also currently being evaluated as a treatment for MuSK-antibody positive myasthenia gravis. In February 2016, we announced the initiation of an investigator-sponsored, randomized, double-blind, placebo-controlled, crossover Phase 2/3 clinical trial evaluating the safety, tolerability and potential efficacy of Firdapse® as a symptomatic treatment for patients with MuSK-antibody positive myasthenia gravis (MuSK-MG). The study is planned to include up to 10 patients and we anticipate that the investigator will report top-line results from this study in the first half of 2017. We are providing study drug and financial support for this study.

If this study is successful, and subject to the availability of funding, we intend to initiate a registration quality trial in the Unites States evaluating Firdapse® for the treatment of patients with MuSK-MG, and we have submitted a SPA to the FDA with respect to this proposed trial. There can be no assurance that the currently ongoing investigator-sponsored trial will be successful, or, even if the current trial is successful, whether other future clinical trials that we initiate to evaluate Firdapse® for this indication will be successful, or whether we will receive a SPA for this trial. There can also be no assurance that the FDA will ever approve Firdapse® for this indication.

Finally, we may seek to evaluate Firdapse® for the treatment of other treatment-refractory types of myasthenia gravis or other rare, similar neuromuscular diseases, although we have not yet begun to develop clinical programs for these indications and all such programs are subject to the availability of funding. There can be no assurance that Firdapse® will be an effective treatment for other treatment-refractory types of myasthenia gravis or for any other rare, similar neuromuscular diseases.

Prior to the receipt of the refusal-to-file letter, we had been actively taking steps to prepare for the commercialization of Firdapse® in the United States. In light of the determination that we will have to complete another adequate and well controlled study evaluating Firdapse® for the treatment of LEMS, we have placed most of these activities on hold in order to conserve cash. Notwithstanding, we are continuing to work with several rare disease advocacy organizations to help increase awareness of LEMS and CMS and to provide awareness and outreach support for the physicians who treat these rare diseases and the patients they treat.

CPP-115.

We are developing CPP-115, a GABA aminotransferase inhibitor that, based on our preclinical studies to date, we believe is a more potent form of vigabatrin, and may have fewer side effects (e.g., visual field defects) than those associated with vigabatrin. We are hoping to develop CPP-115 for the treatment of epilepsy (initially infantile spasms) and for the treatment of other selected neurological indications such as complex partial seizures and Tourette s Disorder. CPP-115 has been granted Orphan Drug Designation by the FDA for the treatment of infantile spasms and

Orphan Medicinal Product Designation in the European Union, or E.U., for West syndrome (a form of infantile spasms).

Subject to the availability of funding or our entering into an agreement with a partner who will fund these development activities, of which there can be no assurance, we plan to move forward and perform the required toxicology studies and dose ranging studies evaluating the safety of CPP-115 that will be required in order to make this drug Phase 2 ready.

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CPP-109.

During September 2015, we announced the initiation of a project to develop a generic version of Sabril® (vigabatrin). Sabril® is marketed by Lundbeck Inc. in the United States for the treatment of infantile spasms and complex partial seizures. There can be no assurance that we will be successful in these efforts or that any abbreviated new drug application (ANDA) that we submit for vigabatrin will be accepted for review or approved. Further, while there can be no assurance, we are hopeful that any ANDA submission we make for vigabatrin will be one of the first ANDAs submitted for this product.

Risks Associated with Product Development

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

- the risk that another pharmaceutical company will receive an approval for its formulation of 3,4-diaminopyridine (3,4-DAP) for the treatment of LEMS or CMS, or any other indication, before we do;
- what additional supporting information will be required before the FDA will file an NDA submission for Firdapse® for the treatment of either LEMS or CMS (or any other condition or disease);
- whether any NDA that we may submit for Firdapse®, if accepted for filing by the FDA, will be granted a priority review;
- the scope and timing of the clinical studies or trials that will be required before the FDA will accept an NDA submission for Firdapse® for the treatment of either LEMS or CMS;
- whether, even if the FDA accepts an NDA submission for Firdapse®, such product will be determined to be safe and effective and approved for commercialization;
- whether the receipt of breakthrough therapy designation for Firdapse® for LEMS will expedite the review of Firdapse® by the FDA or affect the likelihood that the product will be found to be safe and effective;
- whether CPP-115 will be determined to be safe for humans;
- whether CPP-115 will be determined to be effective for the treatment of infantile spasms, post-traumatic stress disorder, Tourette s Disorder or any other indication;

whether we can successfully design and complete a bioequivalence study of our version of vigabatrin compared to Sabril® that is acceptable to the FDA;

whether any such bioequivalence study, the design of which is acceptable to the FDA, will be successful;

whether any ANDA that we submit for a generic version of Sabril® will be accepted by the FDA for review and approved (and the timing of any such approval);

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

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

whether our trials and studies will be successful.

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Available Capital Resources

Based on forecasts of available cash, we currently believe that we have sufficient resources to fund our operations for at least the next year. However, until we finalize the details and logistics of our required confirmatory study evaluating Firdapse® for the treatment of LEMS, it will be difficult for us to provide more details regarding our capital resources. In that regard, we have taken steps to allocate our resources and conserve cash based on our current plan. Notwithstanding, and while there can be no assurance, we continue to believe that our currently available resources will be sufficient to complete the development of Firdapse® and get us to an accepted NDA submission for Firdapse® without the need for additional financing.

If we require additional funding, there can be no assurance that we will obtain the required additional funding or that we will ever be in a position to commercialize any of our drug candidates. See Liquidity and Capital Resources below for further information on our liquidity and cash flow.

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 drug 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 preclinical 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 drug 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 (CROs). In the normal course of business, we contract with third parties to perform various clinical study and trial activities in the on-going development of potential products. The financial terms of these agreements are subject to negotiation and vary from contract to contract and may result in uneven payment flows. Payments under the contracts depend on factors such as the achievement of certain events or milestones, the successful enrollment of patients, the allocation of responsibilities among the parties to the 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 preclinical and clinical studies or trials are recognized based on our estimate of the degree of completion of the event or events specified in the specific study or trial contract. We monitor service provider activities to the extent possible; however, if we underestimate activity levels associated with various studies or trials at a given point in time, we could be required to record significant additional research and development expenses in future periods. Preclinical 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. We had been incurring costs tied to our future sales and marketing efforts for Firdapse®. However, during the first quarter of 2016, following the receipt of the refusal to file letter, we put most of these activities on hold in order to conserve cash. Pre-commercialization expenses to date have been included in general and administrative expenses.

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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. GAAP. For stock options we use the Black-Scholes option valuation model in calculating the fair value of the awards.

Warrants Liability.

We issued warrants to purchase shares of our common stock as part of an equity financing that we completed in October 2011. In accordance with U.S. GAAP, we have recorded the fair value of these warrants as a liability in the accompanying balance sheets at September 30, 2016 and December 31, 2015 using a Black-Scholes option-pricing model. We will re-measure the fair value of this warrants liability at each reporting date until the warrants are exercised or expire in May 2017. 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 these warrants.

Income taxes.

We have incurred operating losses since inception. Our net deferred tax asset has a 100% valuation allowance as of September 30, 2016 and December 31, 2015, 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 would recognize the financial statement benefit of a tax position only after determining that the relevant tax authority would more likely than not sustain the position following an audit. For tax positions meeting the more-likely-than-not threshold, the amount recognized in the financial statements is the largest benefit that has a greater than 50 percent likelihood of being realized upon ultimate settlement with the relevant tax authority.

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.

Non-GAAP Financial Measures.

We prepare our financial statements and footnotes thereto which accompany this report in accordance with U.S. GAAP. In order to supplement our financial results presented on a GAAP basis, we may use non-GAAP financial measures in our reports filed with the SEC and/or in our communications with investors. Non-GAAP measures are

provided as additional information and not as an alternative to our financial statements presented in accordance with GAAP. Our non-GAAP financial measures are intended to enhance an overall understanding of our current financial performance. We believe that the non-GAAP financial measures that we present provide investors and prospective investors with an alternative method for assessing our operating results in a manner that we believe is focused on the performance of ongoing operations and provide a more consistent basis for comparison between periods.

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

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

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Critical Accounting Policies and Estimates

Our discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with U.S. GAAP. The preparation of these financial statements 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 2015 Annual Report on Form 10-K filed with the SEC. Our most critical accounting policies and estimates include: accounting for 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 2015 Annual Report on Form 10-K.

Results of Operations

Revenues.

We had no revenues for the three and nine month periods ended September 30, 2016 and 2015.

Research and Development Expenses.

Research and development expenses for the three and nine month periods ended September 30, 2016 were $2,493,999 and $8,549,287, respectively, including stock-based compensation expense in the three and nine month periods of $185,122 and $443,297, respectively. Research and development expenses for the three and nine month periods ended September 30, 2015 were $3,042,671 and $7,969,731 respectively, including stock-based compensation expense in the three and nine month periods of $113,612 and $253,756 respectively. Research and development expenses, in the aggregate, represented approximately 64% and 57% of total operating costs and expenses for the three and nine month periods ended September 30, 2016 and 61% and 56% for the three and nine month periods ended September 30, 2015, respectively. 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 months ended September 30, 2016, excluding stock-based compensation, increased compared to amounts expended in the same period in 2015, due primarily to consulting fees paid on regulatory matters, activities related to the Firdapse® expanded access program, including manufacturing of related drug, and increased activities in on-going studies and trials. We expect that costs related to research and development activities will continue to be substantial throughout the balance of 2016 and into 2017.

Selling and Marketing Expenses.

We had no selling expenses during the three and nine month periods ended September 30, 2016 and 2015. We have been incurring pre-commercialization costs for Firdapse®, but in the first quarter of 2016 (following the receipt of the refusal to file letter), we put most of these activities on hold in order to conserve cash. These costs are principally for personnel and their related activities. Pre-commercialization costs incurred in 2016 and 2015 are included in general and administrative expenses.

General and Administrative Expenses.

General and administrative expenses for the three and nine months ended September 30, 2016 were $1,420,015 and $6,416,715, respectively, including stock-based compensation expense in the three and nine month periods ending September 30, 2016 of $347,080 and $904,143, respectively. General and administrative expenses for the three and nine months ended September 30, 2015 were $1,974,757 and $6,236,942, respectively, including stock-based compensation expense in the three and nine month periods ending September 30, 2015 of $217,158 and $755,360, respectively. General and administrative expenses represented 36% and 43% of total operating costs and expenses for the three and nine months ended September 30, 2016 and 39% and 44% for the three and nine months

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ended September 30, 2015, respectively. The increase in general and administrative expenses for the nine months ended September 30, 2016 when compared to the same period in 2015 is primarily due to increases in pre-commercialization expenses, payroll and benefits expenses during the first half of 2016, including approximately $600,000 for severance costs related to the reduction-in-force that occurred in May 2016, partly offset by our initiatives to conserve cash. We expect general and administrative expenses during the remainder of 2016 to be consistent with general and administrative expenses during the third quarter of 2016.

Stock-Based Compensation.

Total stock-based compensation for the three and nine month periods ended September 30, 2016 were $532,202 and $1,347,440, respectively, and for the three and nine month periods ended September 30, 2015 were $330,770 and $1,009,116, respectively. The increase in stock-based compensation for the nine month periods ended September 30, 2016 when compared to the same period in 2015, is primarily due to additional headcount.

Change in fair value of warrants liability.

In connection with our October 2011 equity offering, we issued warrants to purchase an aggregate of 1,523,370 shares of common stock. The fair value of the portion of these warrants which remain outstanding is recorded in the liability section of the balance sheet, and was estimated at $229,172 and $1,008,363 at September 30, 2016 and December 31, 2015, 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, 2016, we recognized a loss of $106,948 and a gain of $779,191, respectively, due to the change in the fair value of the warrants liability. The loss during the three months ended September 30, 2016 was principally a result of the increase of our stock price between June 30, 2016 and September 30, 2016. The gain during the nine months ended September 30, 2016 was principally a result of the decrease of our stock price between December 31, 2015 and September 30, 2016. For the three and nine months ended September 30, 2015, we recognized a gain of $521,731 and a loss of $324,591, respectively, due to the change in the fair value of the warrants liability. The gain during the three months ended September 30, 2015 was principally a result of the decrease of our stock price between June 30, 2015 and September 30, 2015 and the loss during the nine months ended September 30, 2015 was principally a result of the increase of our stock price between December 31, 2014 and September 30, 2015. We believe that future changes in the fair value of the warrants liability will be due primarily to fluctuations in the value of our common stock and the timing of warrant exercises.

Other Income, Net.

We reported other income, net in all periods relating to our investment of funds received from offerings of our securities. The increase in other income, net for the three and nine months ended September 30, 2016 when compared to the same period in 2015 is due to higher average investment balances from the proceeds of our offerings and slightly better yields on investments. Other income, net, consists of interest income, dividend income and unrealized and realized gain (loss) on trading securities. These proceeds are used to fund our drug development activities and our operations. Substantially all such funds were invested in short-term interest bearing obligations and short-term bond funds.

Income taxes.

We have incurred net operating losses since inception. For the three and nine month periods ended September 30, 2016 and 2015, we have applied a 100% valuation allowance against our deferred tax asset as we believe that it is more likely than not that the deferred tax asset will not be realized.

Net Loss.

Our net loss was $3,953,981 and $13,909,132, respectively, for the three and nine months ended September 30, 2016 ($0.05 and $0.17, respectively, per basic and diluted share) as compared to a net loss of $4,449,038 and $14,417,800, respectively, for the three and nine months ended September 30, 2015 ($0.05 and $0.18, respectively, per basic and diluted share).

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Non-GAAP Net Loss.

Our non-GAAP net loss, which excludes for the three and nine months ended September 30, 2016 a loss of $106,948 and a gain of $779,191, respectively, associated with the change in the fair value of liability classified warrants, was $3,847,033 and $14,688,323 for the three and nine months ended September 30, 2016 ($0.05 and $0.18, respectively, per basic and diluted share). Our non-GAAP net loss, which excludes for the three and nine months ended September 30, 2015 a gain of $521,731 and a loss of $324,591 associated with the change in the fair value of liability classified warrants, was $4,970,769 and $14,093,209 for the three and nine months ended September 30, 2015 ($0.06 and $0.18, respectively, per basic and diluted share).

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. At September 30, 2016, we had cash and cash equivalents, certificates of deposit and short-term investments aggregating $44.7 million and working capital of $43.1 million. At December 31, 2015, we had cash and cash equivalents, certificates of deposit and short term investments aggregating $58.4 million and working capital of $56.5 million. At September 30, 2016, substantially all of our cash and cash equivalents were deposited with one financial institution, and such balances were in excess of federally insured limits.

We have to date incurred operating losses, and we expect these losses to be substantial in the future as we continue to execute our drug development programs and prepare for the future commercialization of our drug candidates. We anticipate using current cash on hand to finance these activities. Based on currently available information, it is likely that we will not be in a position to commercialize any of our products for approximately the next two years.

Based on forecasts of available cash, we currently believe that we have sufficient resources to fund our operations for at least the next year. However, until we finalize the details and logistics of our required confirmatory study evaluating Firdapse® for the treatment of LEMS, it will be difficult for us to provide more details regarding our capital resources. In that regard, we have taken steps to allocate our resources and conserve cash based on our current plan. Notwithstanding, and while there can be no assurance, we continue to believe that our currently available resources will be sufficient to complete the development of Firdapse® and get us to an accepted NDA submission for Firdapse® without the need for additional financing.

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 drug 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.

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

On January 31, 2014, we filed a shelf registration statement with the SEC to sell up to $100 million of common stock. This shelf registration statement was declared effective on March 19, 2014. We have completed two offerings under this shelf registration statement:

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

- On February 4, 2015, we raised net proceeds of approximately $34.9 million from the sale of 11,500,000 shares of our common stock.

Nasdaq Listing

Our common stock currently trades on the Nasdaq Capital Market. On June 9, 2016, we received a staff deficiency letter from The Nasdaq Stock Market (Nasdaq) notifying us that we were 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, we no longer met that requirement. However, on September 30, 2016, we received notice from Nasdaq confirming that we had regained compliance as a result of our common stock having closed with a bid price of at least $1.00 for at least ten consecutive trading days.

Shareholder Rights Plan

On September 19, 2016, our Board of Directors unanimously approved, and on the same date we entered into Amendment No. 1 to the Rights Agreement (the Amendment), which amends that certain Rights Agreement, dated as of September 20, 2011, between us and Continental Stock Transfer and Trust Company, as rights agent (the Rights Agreement). Under the terms of the Amendment, the outside expiration date of the rights granted under the Rights Agreement has been extended from September 20, 2016 to September 20, 2019. Additionally, as part of the Amendment, the Board adopted a Certificate of Designation, Preferences and Rights of our Series A Junior Participating Preferred Stock to increase the number of shares of Series A Junior Participating Preferred Stock available for issuance under the Rights Agreement from 500,000 shares to 1.5 million shares.

Cash Flows

Net cash used in operating activities was $13,551,764 and $13,383,899, respectively, for the nine month periods ended September 30, 2016 and 2015. During the nine months ended September 30, 2016, net cash used in operating activities was primarily attributable to our net loss of $13,909,132, decreases of $738,803 in accounts payable and $697,403 in accrued expenses and other liabilities and $779,191 of non-cash change in fair value of warrants liability. This was partially offset by a $1,193,883 decrease in prepaid expenses and other current assets and deposits and $1,378,882 of other non-cash expenses. During the nine months ended September 30, 2015, net cash used in operating activities was primarily attributable to our net loss of $14,417,800, a decrease in accounts payable of $997,189 and an increase in prepaid expenses and other current assets and deposits of $341,220. This was partially offset by an increase of $1,010,221 in accrued expenses and other liabilities, a $324,591 non-cash change in fair value of warrants liability and $1,037,498 of other non-cash expenses. Other non-cash expenses include depreciation and stock-based compensation expense.

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Net cash provided by investing activities during the nine month period ended September 30, 2016 was $2,966,113, consisting primarily of proceeds of certificates of deposit. Net cash used in investing activities during the nine-month period ended September 30, 2015 was $56,976, consisting primarily of capital expenditures of $23,465 and purchases of short-term investments of $32,071.

Net cash used in financing activities during the nine month period ended September 30, 2016 was $11,265, for payment of employee withholding tax related to stock-based compensation. Net cash provided by financing activities during the nine-month period ended September 30, 2015 was $37,094,556, consisting of $34,873,869 from the net proceeds from the sale of common stock under the 2014 Shelf Registration Statement, $1,895,738 of proceeds from the exercise of warrants to purchase common stock and $324,949 of proceeds from the exercise of options to purchase common stock.

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 royalties for seven years from the first commercial sale of Firdapse® equal to 7% of net sales (as defined in our license agreement) in North America for any calendar year for sales up to $100 million, and 10% of net sales in North America in any calendar year in excess of $100 million; (ii) to pay to the third-party licensor of the rights sublicensed to us royalty payments for seven years from the first commercial sale of Firdapse® equal to 7% of net sales (as defined in the license agreement between BioMarin and the third-party licensor) in any calendar year; and (iii) to pay certain milestone payments that BioMarin is obligated to pay (approximately $2.6 million of which will be due upon acceptance by the FDA of a filing of an NDA for Firdapse® for the treatment of LEMS, and approximately $7.2 million of which will be due on the unconditional approval by the FDA of an NDA for Firdapse® for the treatment of LEMS). We have also agreed to share in the cost of certain post-marketing studies conducted by BioMarin.

Payments to Northwestern University under our license agreement. Under our license agreement with Northwestern, we have paid to date $411,590, had accrued liabilities of $126,250 at September 30, 2016 in the accompanying balance sheet, and owe certain milestone payments in future years if we do not cancel the license agreement. The next milestone payment of $300,000 is due on the earlier of successful completion of the first Phase 3 clinical trial of CPP-115 or August 27, 2018.

Employment agreements. We have entered into an employment agreement with our Chief Executive Officer that requires us to make base salary payments of approximately $471,000 in 2016. The agreement expires in November 2018.

Lease for office space. We operate our business in leased office space in Coral Gables, Florida. We currently lease approximately 5,200 square feet of office space for which we pay annual rent of approximately $200,000.

Off-Balance Sheet Arrangements.

We currently have no debt or capital leases. We have operating leases for our office facilities. We do not have any off-balance sheet arrangements as such term is defined in rules promulgated by the SEC.

Caution Concerning Forward-Looking Statements

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

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

- our estimates regarding anticipated capital requirements and our need for additional financing;
- the risk that another pharmaceutical company will receive an approval for its formulation of 3,4-diaminopyridine (3,4-DAP) for the treatment of LEMS, CMS, or any other indication, before we do;
- what additional supporting information will be required before the FDA will file an NDA submission for Firdapse® for the treatment of either LEMS or CMS;
- whether any NDA that we may submit for Firdapse®, if accepted for filing by the FDA, will be granted a priority review;
- the scope and timing of the clinical studies or trials that will be required before the FDA will accept an NDA submission for Firdapse® for the treatment of either LEMS or CMS;
- whether, even if the FDA accepts an NDA submission for Firdapse®, such product will be determined to be safe and effective and approved for commercialization;
- whether the receipt of breakthrough therapy designation for Firdapse® for LEMS will expedite the review of Firdapse® by the FDA or affect the likelihood that the product will be found to be safe and effective;
- whether as part of the FDA review of any NDA that we may submit for filing for Firdapse®, the tradename Firdapse®, which is the tradename used for the same product in Europe, will be approved for use for the product in the United States;
- whether, assuming Firdapse® is approved for commercialization, we will be able to develop a sales and marketing organization that can successfully market Firdapse® while maintaining full compliance with applicable federal and state laws, rules and regulations;
- whether CPP-115 will be determined to be safe for humans;

whether CPP-115 will be determined to be effective for the treatment of infantile spasms, post-traumatic stress disorder, Tourette s Disorder or any other indication;

whether we can successfully design and complete a bioequivalence study of our version of vigabatrin compared to Sabril® that is acceptable to the FDA;

whether any such bioequivalence study, the design of which is acceptable to the FDA, will be successful;

whether any ANDA that we submit for a generic version of Sabril® will be accepted by the FDA for review and approved (and the timing of any such approval);

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

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

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whether our trials and studies will be successful;

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

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

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

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

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

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

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

what pricing we will be able to achieve with respect to our drug products and the impact of public scrutiny on the pricing of drug products generally and our products and orphan drugs in particular;

changes in the laws and regulations and regulatory guidance affecting our business;

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

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

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

our expectations regarding licensing, acquisitions or strategic relationships;

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

the expense of filing, and potentially prosecuting, defending and enforcing any patent claims and other intellectual property rights we may have for our drug candidates;

our ability to attract and retain skilled employees;

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

the impact of potential employee, vendor or consultant misconduct; and

changes in general economic conditions and interest rates.

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

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ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURE ABOUT MARKET RISK

Our market risks during the three and nine months ended September 30, 2016 have not materially changed from those discussed in Item 7A of our Annual Report on Form 10-K for the year ended December 31, 2015.

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(e) 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, 2016, 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, 2016, 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.

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PART II. OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

See Note 7 to the Financial Statements in our financial statements for the year ended December 31, 2015 for information about the spring 2015 settlement of the securities class action litigation that was filed against us in 2013.

The Company is not a party to any 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 2015 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

3.1	Certificate of Designation, Preferences and Rights for Series A Junior Participating Preferred Stock (included as Exhibit A to Exhibit 4.1).

4.1	Amendment No 1 to Rights Agreement, dated as of September 19, 2016, between the Company and Continental Stock Transfer & Trust Company, as rights agent (incorporated by reference to the Company s Form 8-K dated September 19, 2016).
10.1	Amendment No 5 to Employment Agreement between the Company and Patrick J. McEnany, dated June 23, 2016 (incorporated by reference to the Company s Form 8-K dated June 23, 2016)
10.2	Amendment No 1 to the Company s 2014 Stock Incentive Plan (incorporated by reference to Annex A of the Company s 2016 Annual Meeting Proxy Statement dated April 29, 2016)
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

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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

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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 Pharmaceuticals, Inc.

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

Date: November 9, 2016

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Exhibit Index

Exhibit Number	Description
31.1	Certification of Principal Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002
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