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**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**  
Washington, D.C. 20549

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**FORM 10-Q**

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[Mark One]

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

For the Quarterly Period Ended June 30, 2017

OR

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

Commission File No. 001-33057

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**CATALYST PHARMACEUTICALS, INC.**

(Exact name of registrant as specified in its charter)

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**Delaware**  
(State or other jurisdiction of  
incorporation or organization)

**76-0837053**  
(IRS Employer  
Identification No.)

**355 Alhambra Circle**  
**Suite 1250**  
**Coral Gables, Florida**  
(Address of principal executive offices)

**33134**  
(Zip Code)

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

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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, a smaller reporting company or an emerging growth company. See definitions of "accelerated filer", "large accelerated filer", "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act (Check one):

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

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards pursuant to Section 13(a) of the Exchange Act.

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 84,554,979 shares of common stock, \$0.001 par value per share, were outstanding as of August 4, 2017.

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CATALYST PHARMACEUTICALS, INC.

INDEX

**PART I. FINANCIAL INFORMATION**

Item 1.	FINANCIAL STATEMENTS	
	<a href="#">Balance sheets at June 30, 2017 (unaudited) and December 31, 2016</a>	3
	<a href="#">Statements of operations for the three and six months ended June 30, 2017 and 2016 (unaudited)</a>	4
	<a href="#">Statement of stockholders' equity for the six months ended June 30, 2017 (unaudited)</a>	5
	<a href="#">Statements of cash flows for the six months ended June 30, 2017 and 2016 (unaudited)</a>	6
	<a href="#">Notes to unaudited financial statements</a>	7
Item 2.	<a href="#">MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS</a>	17
Item 3.	<a href="#">QUANTITATIVE AND QUALITATIVE DISCLOSURE ABOUT MARKET RISK</a>	30
Item 4.	<a href="#">CONTROLS AND PROCEDURES</a>	30

**PART II. OTHER INFORMATION**

Item 1.	<a href="#">LEGAL PROCEEDINGS</a>	31
Item 1A.	<a href="#">RISK FACTORS</a>	31
Item 2.	<a href="#">UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS</a>	31
Item 3.	<a href="#">DEFAULTS UPON SENIOR SECURITIES</a>	31
Item 4.	<a href="#">MINE SAFETY DISCLOSURE</a>	31
Item 5.	<a href="#">OTHER INFORMATION</a>	31
Item 6.	<a href="#">EXHIBITS</a>	31

**CATALYST PHARMACEUTICALS, INC.**  
**BALANCE SHEETS**

	<u>June 30,</u> <u>2017</u>	<u>December 31,</u> <u>2016</u>
<b>ASSETS</b>		
Current Assets:		
Cash and cash equivalents	\$ 8,583,727	\$ 13,893,064
Short-term investments	26,547,663	26,512,753
Prepaid expenses and other current assets	621,558	1,047,944
Total current assets	35,752,948	41,453,761
Property and equipment, net	218,289	244,204
Deposits	8,888	8,888
Total assets	<u>\$ 35,980,125</u>	<u>\$ 41,706,853</u>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current Liabilities:		
Accounts payable	\$ 692,345	\$ 933,176
Accrued expenses and other liabilities	1,148,080	1,161,359
Total current liabilities	1,840,425	2,094,535
Accrued expenses and other liabilities, non-current	170,519	181,162
Warrants liability, at fair value	—	122,226
Total liabilities	2,010,944	2,397,923
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, \$0.001 par value, 5,000,000 shares authorized: none issued and outstanding at June 30, 2017 and December 31, 2016	—	—
Common stock, \$0.001 par value, 150,000,000 shares authorized; 84,554,979 shares and 82,972,316 shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively	84,555	82,972
Additional paid-in capital	150,879,726	147,374,028
Accumulated deficit	(116,995,100)	(108,148,070)
Total stockholders' equity	33,969,181	39,308,930
Total liabilities and stockholders' equity	<u>\$ 35,980,125</u>	<u>\$ 41,706,853</u>

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

**CATALYST PHARMACEUTICALS, INC.**  
**STATEMENTS OF OPERATIONS (unaudited)**

	For the Three Months Ended		For the Six Months Ended	
	June 30,		June 30,	
	2017	2016	2017	2016
Operating costs and expenses:				
Research and development	\$ 2,451,751	\$ 2,508,897	\$ 5,265,680	\$ 6,055,288
General and administrative	1,729,520	2,305,555	3,595,462	4,996,700
Total operating costs and expenses	4,181,271	4,814,452	8,861,142	11,051,988
Loss from operations	(4,181,271)	(4,814,452)	(8,861,142)	(11,051,988)
Other income, net	91,039	92,755	201,016	210,698
Change in fair value of warrants liability	210,331	152,783	(186,904)	886,139
Loss before income taxes	(3,879,901)	(4,568,914)	(8,847,030)	(9,955,151)
Provision for income taxes	—	—	—	—
Net loss	\$ (3,879,901)	\$ (4,568,914)	\$ (8,847,030)	\$ (9,955,151)
Net loss per share – basic and diluted	\$ (0.05)	\$ (0.06)	\$ (0.11)	\$ (0.12)
Weighted average shares outstanding – basic and diluted	83,905,827	82,870,649	83,441,650	82,865,366

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

**CATALYST PHARMACEUTICALS, INC.**  
**STATEMENT OF STOCKHOLDERS' EQUITY (unaudited)**  
**For the six months ended June 30, 2017**

	<u>Preferred Stock</u>	<u>Common Stock</u>	<u>Additional Paid-in Capital</u>	<u>Accumulated Deficit</u>	<u>Total</u>
<b>Balance at December 31, 2016</b>	\$ —	\$82,972	\$147,374,028	\$(108,148,070)	\$39,308,930
Issuance of stock options for services	—	—	1,355,290	—	1,355,290
Amortization of restricted stock for services	—	—	37,423	—	37,423
Exercise of warrants for common stock	—	1,583	2,112,985	—	2,114,568
Net loss	—	—	—	(8,847,030)	(8,847,030)
<b>Balance at June 30, 2017</b>	<u>\$ —</u>	<u>\$84,555</u>	<u>\$150,879,726</u>	<u>\$(116,995,100)</u>	<u>\$33,969,181</u>

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

**CATALYST PHARMACEUTICALS, INC.**  
**STATEMENTS OF CASH FLOWS (unaudited)**

	For the Six Months Ended	
	June 30,	
	2017	2016
<b>Operating Activities:</b>		
Net loss	\$ (8,847,030)	\$ (9,955,151)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	25,915	21,960
Stock-based compensation	1,392,713	815,238
Change in fair value of warrants liability	186,904	(886,139)
(Increase) decrease in:		
Prepaid expenses and other current assets and deposits	426,386	853,761
Increase (decrease) in:		
Accounts payable	(240,831)	(1,086,835)
Accrued expenses and other liabilities	(23,921)	(36,572)
Net cash used in operating activities	(7,079,864)	(10,273,738)
<b>Investing Activities:</b>		
Capital expenditures	—	(88,929)
Purchase of short-term investments	(34,910)	(93,263)
Proceeds (purchase) of certificates of deposit	—	949,283
Net cash provided by (used in) investing activities	(34,910)	767,091
<b>Financing Activities:</b>		
Payment of employee withholding tax related to stock-based compensation	—	(11,265)
Proceeds from exercise of warrants	1,805,437	—
Net cash provided by (used in) financing activities	1,805,437	(11,265)
Net increase (decrease) in cash and cash equivalents	(5,309,337)	(9,517,912)
Cash and cash equivalents - beginning of period	13,893,064	28,235,016
Cash and cash equivalents - end of period	\$ 8,583,727	\$ 18,717,104
<b>Supplemental disclosures of non-cash investing and financing activity</b>		
Exercise of liability classified warrants for common stock	\$ 309,130	\$ —

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

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) and infantile spasms.

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 June 30, 2017. 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**

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 12 months.

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 Securities and Exchange Commission (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, 2016 included in this Form 10-Q was derived from the audited financial statements and does not include all disclosures required by U.S. GAAP.

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, 2016 included in the 2016 Annual Report on Form 10-K filed by the Company with the SEC. The results of operations for the three and six months ended June 30, 2017 are not necessarily indicative of the results to be expected for any future period or for the full 2017 fiscal year.

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

- 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. **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 June 30, 2017, and December 31, 2016, 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 June 30, 2017 and December 31, 2016 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 for the three and six months ended June 30, 2017 were \$0 and \$29,430, respectively. Unrealized gain for the three and six months ended June 30, 2016 were \$29,430 and \$88,291, respectively, and are included in other income, net in the accompanying statements of operations.
- e. **PREPAID EXPENSES AND OTHER CURRENT ASSETS.** Prepaid expenses and other current assets consist primarily of 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 pre-clinical studies, clinical trials and studies, regulatory affairs and consulting. Such advances are recorded as expense as the related goods are received or the related services are performed.
- f. **FAIR VALUE OF FINANCIAL INSTRUMENTS.** The Company's financial instruments consist of cash and cash equivalents, short-term investments, accounts payables, accrued expenses and other liabilities, and warrants liability. At June 30, 2017 and December 31, 2016, the fair value of these instruments approximated their carrying value.
- g. **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).

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

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

	<b>Fair Value Measurements at Reporting Date Using</b>			
	Balances as of June 30, 2017	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	\$ 8,454,851	\$ 8,454,851	\$ —	\$ —
Short-term investments	\$26,547,663	\$ 26,547,663	\$ —	\$ —

	<b>Fair Value Measurements at Reporting Date Using</b>			
	Balances as of December 31, 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	\$13,395,759	\$ 13,395,759	\$ —	\$ —
Short-term investments	\$26,512,753	\$ 26,512,753	\$ —	\$ —
Warrants liability	\$ 122,226	\$ —	\$ —	\$ 122,226

- h. 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. 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 June 30, 2017, none of the 2011 warrants remained outstanding and at December 31, 2016, 763,913 of the 2011 warrants remained outstanding.

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

- i. 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. Forfeitures are recognized as a reduction of share-based compensation expense as they occur.

As of June 30, 2017, there were outstanding stock options to purchase 6,090,000 shares of common stock, of which stock options to purchase 3,244,996 shares of common stock were exercisable as of June 30, 2017.

For the three and six-month periods ended June 30, 2017 and 2016, the Company recorded stock-based compensation expense as follows:

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
Research and development	\$223,552	\$164,392	\$ 429,904	\$258,175
General and administrative	415,017	192,877	962,809	557,063
Total stock-based compensation	<u>\$638,569</u>	<u>\$357,269</u>	<u>\$1,392,713</u>	<u>\$815,238</u>

- j. 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).
- k. 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:

	June 30,	
	2017	2016
Options to purchase common stock	6,090,000	5,148,333
Warrants to purchase common stock	675,000	2,407,663
Unvested restricted stock	26,667	53,334
Potential equivalent common stock excluded	<u>6,791,667</u>	<u>7,609,330</u>

Potentially dilutive options to purchase common stock as of both June 30, 2017 and 2016 have exercise prices ranging from \$0.47 to \$4.64. Potentially dilutive warrants to purchase common stock as of June 30, 2016 have exercise prices ranging from \$1.04 to \$2.08. Potentially dilutive warrants to purchase common stock as of June 30, 2017 have an exercise price of \$2.08 and expire in August 2017.

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

- I. RECENTLY ISSUED ACCOUNTING STANDARDS.** 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.

On March 30, 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. The Company adopted this standard in the first quarter of 2017. The adoption of this standard did not have a material impact on the Company's financial statements.

In May 2017, the FASB issued ASU No. 2017-09, *Compensation – Stock Compensation (Topic 718): Scope of Modification Accounting* to clarify when to account for a change to the terms or conditions of a share-based payment award as a modification. Under this new guidance, modification accounting is required if the fair value, vesting conditions, or classification of the award changes as a result of the change in terms or conditions. ASU 2017-09 is effective for all entities for annual reporting periods beginning after December 15, 2017, including interim reporting periods within each annual reporting period, applied prospectively on or after the effective date. The Company is currently evaluating the impact this accounting standard will have on its financial statements, however, does not expect the adoption of this standard to have a material impact on the Company's financial statements.

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

*2011 Warrants*

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

The closing price of the Company's common stock would fall under Level 1 of the fair value hierarchy as it is a quoted price in an active market. The risk-free rate of return is a Level 2 input, while the historical volatility is a Level 3 input in accordance with the fair value accounting guidance. Since the lowest level input is a Level 3, the Company determined the 2011 warrants liability is most appropriately classified within Level 3 of the fair value hierarchy. This liability is subject to a fair value mark-to-market adjustment each reporting period.

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

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

	<b>December 31, 2016</b>
Risk free interest rate	0.85%
Expected term	0.33 years
Expected volatility	100%
Expected dividend yield	0%
Expected forfeiture rate	0%

The following table rolls forward the fair value of the Company's warrants liability activity for the three and six-month periods ended June 30, 2017 and 2016:

	<b>Three months ended</b>		<b>Six months ended</b>	
	<b>June 30,</b>		<b>June 30,</b>	
	<b>2017</b>	<b>2016</b>	<b>2017</b>	<b>2016</b>
Fair value, beginning of period	\$ 519,461	\$ 275,007	\$ 122,226	\$ 1,008,363
Issuance of warrants	—	—	—	—
Exercise of warrants	(309,130)	—	(309,130)	—
Change in fair value	(210,331)	(152,783)	186,904	(886,139)
Fair value, end of period	<u>\$ —</u>	<u>\$ 122,224</u>	<u>\$ —</u>	<u>\$ 122,224</u>

During both the three and six months ended June 30, 2017, 613,913 of the 2011 warrants were exercised, with proceeds of \$798,087 to the Company. During the three and six months ended June 30, 2016, none of the 2011 warrants were exercised. On May 2, 2017, the 150,000 remaining outstanding and unexercised 2011 warrants expired.

**4. Prepaid Expenses and Other Current Assets.**

Prepaid expenses and other current assets consist of the following:

	<b>June 30, 2017</b>	<b>December 31, 2016</b>
Prepaid research fees	\$ 218,997	\$ 334,565
Prepaid insurance	275,563	598,909
Prepaid pre-commercialization fees	—	35,500
Prepaid subscription fees	73,536	22,770
Prepaid rent	19,906	19,756
Other	33,556	36,444
Total prepaid expenses and other current assets	<u>\$ 621,558</u>	<u>\$ 1,047,944</u>

**5. Property and Equipment, net.**

Property and equipment, net consists of the following:

	<b>June 30, 2017</b>	<b>December 31, 2016</b>
Computer equipment	\$ 27,915	\$ 27,915
Furniture and equipment	177,061	177,061
Leasehold improvements	152,708	152,708
	357,684	357,684
Less: Accumulated depreciation	(139,395)	(113,480)
Total property and equipment, net	<u>\$ 218,289</u>	<u>\$ 244,204</u>

**5. Property and Equipment, net (continued).**

Depreciation expense was \$12,957 and \$25,915, respectively, for the three and six-month periods ended June 30, 2017 and \$8,762 and \$21,960 for the three and six-month periods ended June 30, 2016, respectively.

**6. Accrued Expenses and Other Liabilities.**

Accrued expenses and other liabilities consist of the following:

	<u>June 30, 2017</u>	<u>December 31, 2016</u>
Accrued pre-clinical and clinical trial expenses	\$ 606,554	\$ 623,855
Accrued professional fees	130,234	102,673
Accrued compensation and benefits	182,586	264,237
Accrued license fees	205,000	152,500
Deferred rent and lease incentive	20,867	18,094
Other	2,839	—
Current accrued expenses and other liabilities	<u>1,148,080</u>	<u>1,161,359</u>
Deferred rent and lease incentive - non-current	170,519	181,162
Non-current accrued expenses and other liabilities	<u>170,519</u>	<u>181,162</u>
Total accrued expenses and other liabilities	<u>\$ 1,318,599</u>	<u>\$ 1,342,521</u>

**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 June 30, 2017, the Company has paid \$411,590 in connection with the license and has accrued license fees of \$205,000 in the accompanying June 30, 2017 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, if the Company does 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 for CPP-115 or August 27, 2018.

- 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.

**7. Commitments and Contingencies (continued).**

- 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 has agreed to pay: (i) royalties to BioMarin 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; and (ii) royalties to the third-party licensor of the rights sublicensed to the Company 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.

Additionally, the Company has agreed to pay certain milestone payments that BioMarin is obligated to pay to both the third-party licensor and to the former stockholders of Huxley Pharmaceuticals (“Huxley”) under an earlier stock purchase agreement between BioMarin and the former Huxley stockholders. These milestones aggregate (i) up to approximately \$2.6 million due upon acceptance by the U.S. Food & Drug Administration (FDA) of a filing of a new drug application (NDA) for Firdapse® for the treatment of LEMS or CMS, and (ii) up to approximately \$7.2 million due on the unconditional approval by the FDA of an NDA for Firdapse® for the treatment of LEMS; *provided, however* that the total milestone payments that the Company will be obligated to pay if it meets milestone (i) and/or milestone (ii) above will be reduced to an aggregate of \$150,000 and \$3.0 million, respectively, if either of these respective milestones are satisfied after April 20, 2018 (the date on which BioMarin’s obligations to pay milestone payments to the former stockholders of Huxley expire).

The Company also agreed to share in the cost of certain post-marketing studies being conducted by BioMarin, and, as of June 30, 2017, 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.

**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 and expired on March 19, 2017. The Company 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.

***2016 Shelf Registration Statement***

On December 23, 2016, the Company filed a shelf Registration Statement on Form S-3 (the 2016 Shelf Registration Statement) with the SEC to sell up to approximately \$33.8 million of common stock. The 2016 Shelf Registration Statement (file No. 333-215315) was declared effective by the SEC on January 9, 2017. No sales have been conducted to date under the 2016 Shelf Registration Statement.

***2017 Shelf Registration Statement***

Subsequent to quarter-end, on July 12, 2017, the Company filed a universal shelf Registration Statement on Form S-3 (the 2017 Shelf Registration Statement) with the SEC to sell up to \$150 million of common stock, preferred stock, warrants to purchase common stock, or debt securities (including debt securities that may be convertible or exchangeable for common stock or other securities), which securities may be offered separately or together in units or multiple series. The 2017 Shelf Registration Statement (file No. 333-219259) was declared effective by the SEC on July 26, 2017. No sales have been conducted to date under the 2017 Shelf Registration Statement. See Note 11.

***Warrant Exercises***

During both the three and six months ended June 30, 2017, the Company issued an aggregate of 1,582,663 shares of its authorized but unissued common stock upon the exercise of previously issued common stock purchase warrants, with net proceeds to the Company of \$1,805,437. No warrants were exercised during the three and six months ended June 30, 2016.

**10. Stock Compensation.**

***Stock Options***

During the three and six-month periods ended June 30, 2017, the Company granted seven-year options to purchase an aggregate of 15,000 and 1,535,000 shares, respectively, of the Company's common stock to employees and directors. The Company recorded stock-based compensation related to stock options totaling \$619,754 and \$1,355,290 respectively, during the three and six-month periods ended June 30, 2017. During the three and six-month periods ended June 30, 2017, respectively, 621,667 and 876,667 options vested.

During the three and six-month periods ended June 30, 2016, the Company granted seven-year options to purchase an aggregate of 1,090,000 and 1,245,000 shares, respectively, of the Company's common stock to employees and directors. The Company recorded stock-based compensation related to stock options totaling \$338,506 and \$777,712 respectively, during the three and six-month periods ended June 30, 2016. During the three and six-month periods ended June 30, 2016, respectively, 175,000 and 231,665 options vested.

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

No options were exercised during the three and six months ended June 30, 2017.

No options were exercised during the three months ended June 30, 2016. During the six months ended June 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.

As of June 30, 2017, there was approximately \$2,292,000 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 1.58 years.

*Restricted Stock Units*

No restricted stock units were granted during the three and six months ended June 30, 2017 and 2016. The Company recorded stock-based compensation related to restricted stock units totaling \$18,815 and \$37,423, respectively, during the three and six-month periods ended June 30, 2017. The Company recorded stock-based compensation related to restricted stock units totaling \$18,763 and \$37,526, respectively, during the three and six-month periods ended June 30, 2016. As of June 30, 2017, there was approximately \$28,000 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 0.37 years.

**11. Subsequent Events.**

Subsequent to quarter-end, on July 12, 2017, the Company filed a universal shelf Registration Statement on Form S-3 (the 2017 Shelf Registration Statement) with the SEC to sell up to \$150 million of common stock, preferred stock, warrants to purchase common stock, or debt securities (including debt securities that may be convertible or exchangeable for common stock or other securities), which securities may be offered separately or together in units or multiple series. The 2017 Shelf Registration Statement (file No. 333-219259) was declared effective by the SEC on July 26, 2017. No sales have been conducted to date under the 2017 Shelf Registration Statement. See Note 9.

## 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 second quarter of fiscal 2017.
- *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 six months ended June 30, 2017 as compared to the same periods ended June 30, 2016.
- *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. Further, the FDA granted Orphan Drug Designation for Firdapse® for the treatment of patients with LEMS, Congenital Myasthenic Syndromes, or CMS and Myasthenia Gravis (MG).

The chemical entity, amifampridine (3,4-diaminopyridine, or 3,4-DAP), has never been approved by the FDA for any indication. Because amifampridine phosphate (Firdapse®) has been granted Orphan Drug designation for the treatment of LEMS, CMS and MG by the FDA, the product is also eligible to receive seven years of marketing exclusivity for either 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. This Phase 3 trial, which involved 38 subjects, was designed as a 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 continue to inform neuromuscular physicians on the availability of the Firdapse® EAP and also to work with various rare disease advocacy organizations to inform patients and other 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” (RTF) 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 advised us that in addition to the results of our 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 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 FDA 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, the FDA may rescind a SPA agreement when the division director determines that a substantial scientific issue essential to determining the safety or efficacy of the product has been identified after the trial has begun.

We are conducting our second Phase 3 trial evaluating Firdapse® for the treatment of LEMS (designated as LMS-003) at sites in Miami, Florida and Los Angeles, California. This double-blind, placebo-controlled withdrawal trial will include approximately 28 subjects, and will have the same co-primary endpoints as our first Phase 3 trial evaluating Firdapse® for the treatment of LEMS. Further, the FDA is allowing us to enroll patients from our expanded access program as study subjects in this second trial. Details of the Phase 3 clinical trial are available on [www.clinicaltrials.gov](http://www.clinicaltrials.gov) (NCT02970162).

We initiated this trial in December 2016, and we expect to report top-line results from this trial during the second half of 2017. Assuming the results of this trial are successful, and our anticipated timeline for the completion of this trial is met, we expect to resubmit an NDA for Firdapse® for the treatment of LEMS before the end of 2017. There can be no assurance as to the timing or requirements of this trial, whether this trial, along with the results of our first Phase 3 trial, 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

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 considering comments from the FDA, we 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 are currently conducting this study at five sites around the United States, and we are currently adding several additional sites outside the United States. Details of this trial are available on [www.clinicaltrials.gov](http://www.clinicaltrials.gov) (NCT02562066).

Based on currently available information, we expect to report top line results from this study in the first half of 2018 and if the results of the study are successful, we hope to add the CMS indication to our labeling for Firdapse®. We also may include in our initial filing for LEMS those limited types of CMS that are generally considered mechanistically similar to LEMS. 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.

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-MG. MuSK-MG, an ultra-rare sub-population of MG patients, is a debilitating neuromuscular disease, and there are currently no FDA approved therapies for this specific form of MG. Seven patients participated in this proof-of-concept trial. We provided study drug, placebo and financial support for this study.

On March 15, 2017, we reported top-line results from this trial. Both of the co-primary efficacy endpoints of change from baseline (CFB) in total Quantitative Myasthenia Gravis (QMG) score ( $p=0.0003$ ) and CFB in total Myasthenia Gravis Activities of Daily Living (MG-ADL) score ( $p=0.0006$ ) were statistically and clinically significant in this trial. Several secondary efficacy measures also achieved statistical significance. Amifampridine phosphate was well tolerated in this population of patients.

We are currently discussing with the FDA a registration trial evaluating Firdapse® for the treatment of patients with MuSK-MG. There can be no assurance that future clinical trials that we initiate to evaluate Firdapse® for this indication will be successful, or whether we can obtain the resources available to fund any such registration trial. Further, 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 MG 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 MG or for any other rare, similar neuromuscular diseases.

Prior to the receipt of the RTF 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 a second adequate and well controlled study evaluating Firdapse® for the treatment of LEMS, in the first quarter of 2016 we placed most of these commercialization activities on hold in order to conserve cash. We currently expect to recommence our commercialization plans for Firdapse® during the second half of 2017 as we move closer to submitting an NDA for Firdapse®. 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 refractory infantile spasms and possibly for the treatment of adult refractory patients with 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).

## Table of Contents

We are currently refining our development plans for this product. Once the refinement of our development plans is completed, and subject to the then availability of funding, we plan to take the steps to complete the work required to make our drug candidate Phase 2 ready. We are also working with one or more potential investigators who have expressed an interest in evaluating our product for particular indications (particularly infantile spasms).

We are also continuing our efforts to seek a partner to work with us in furthering the development of CPP-115. However, no agreements have been entered into to date.

There can be no assurance that we will ever successfully commercialize CPP-115.

### • Generic Sabril®

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.

We are continuing our efforts to seek a partner to work with us in furthering the development of generic Sabril®. However, no agreements have been entered into to date.

There can be no assurance that we will ever successfully commercialize a generic version of Sabril®.

## 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:

- Our estimates regarding anticipated capital requirements and our need for additional funding;
- the risk that another pharmaceutical company will receive an approval for its formulation of 3,4-diaminopyridine (3,4-DAP) for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS), Congenital Myasthenic Syndromes (CMS), or any other indication, before we do;
- whether the clinical studies or trials that are required to be completed before the FDA will accept an NDA submission for Firdapse® for the treatment of either LEMS or CMS will be successful;
- what additional supporting information, including any additional clinical studies or trials, will be required before the FDA will accept our 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® will be accepted for filing by the FDA, and if accepted, whether it will be granted a priority review;
- 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 for any of the submitted indications;
- whether the receipt of breakthrough therapy designation for Firdapse® for LEMS will result in an expedited review of Firdapse® by the FDA or affect the likelihood that the product will be found to be safe and effective;

## Table of Contents

- whether as part of the FDA review of any NDA that we may submit for filing for Firdapse<sup>®</sup>, the tradename Firdapse<sup>®</sup>, 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<sup>®</sup> is approved for commercialization, we will be able to develop or contract with a sales and marketing organization that can successfully market Firdapse<sup>®</sup> while maintaining full compliance with applicable federal and state laws, rules and regulations;
- whether any future trial that we undertake evaluating Firdapse<sup>®</sup> for the treatment of MuSK-MG will be successful and whether we can obtain the funding required for such trial;
- 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, 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<sup>®</sup> that is acceptable to the FDA;
- whether any ANDA that we submit for a generic version of Sabril<sup>®</sup> 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;
- the ability of our third-party suppliers and contract manufacturers to maintain compliance with current Good Manufacturing Practices (cGMP);
- whether our estimates of the size of the market for our drug candidates will turn out to be accurate;
- the pricing of our products that we may be able to achieve if we are granted the ability to commercialize our drug candidates; and
- changes in the healthcare industry occasioned by any future repeal and replacement of the Affordable Care Act, in laws relating to the pricing of drug products, or in the healthcare industry generally.

## 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 12 months. However, we will require additional funding to support our operations beyond that time. There can be no assurance that we will obtain the 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.

## Table of Contents

### *Research and development expenses.*

Our research and development expenses consist of costs incurred for company-sponsored research and development activities, as well as 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 product development efforts. To date, all of our research and development resources have been devoted to the development of CPP-109 (our version of vigabatrin), CPP-115 and Firdapse®, and we expect this to continue for the foreseeable future.

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 our business we contract with third parties to perform various clinical study and trial activities in the on-going development of potential products. The financial terms of these agreements are subject to negotiation and vary from contract to contract and may result in uneven payment flows. Payments under the contracts depend on factors such as the achievement of certain events or milestones, the successful enrollment of patients, the allocation of responsibilities among the parties to the agreement, and the completion of portions of the clinical study or trial or similar conditions. The objective of our accrual policy is to match the recording of expenses in our financial statements to the actual services received and efforts expended. As such, expense accruals related to 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 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 RTF letter, we put most of these activities on hold in order to conserve cash. We currently expect to recommence developing our commercialization plans for Firdapse® during the second half of 2017. Pre-commercialization costs are included in general and administrative expenses.

### *General and administrative expenses.*

Our general and administrative expenses consist primarily of salaries and personnel expenses for accounting, corporate, compliance and administrative functions. Other costs include administrative facility costs, regulatory fees, insurance, pre-commercialization costs, 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 sheet at December 31, 2016 using a Black-Scholes option-pricing model. We have remeasured the fair value of this warrants liability at each reporting date until the warrants were exercised or until they expired on May 2, 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 was subject to significant fluctuation based on changes in the inputs to the Black-Scholes option-pricing model, including our common stock price, expected volatility, expected term, the risk-free interest rate and dividend yield. The remaining unexercised 2011 liability warrants expired on May 2, 2017.

## [Table of Contents](#)

### *Income taxes.*

We have incurred operating losses since inception. Our net deferred tax asset has a 100% valuation allowance as of June 30, 2017 and December 31, 2016, 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 (GAAP). To supplement our financial results presented on a GAAP basis, we may use non-GAAP financial measures in our reports filed with the Commission and/or 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. Further, we often report non-GAAP net loss per share, which is calculated by dividing non-GAAP net loss by the weighted average common shares outstanding.

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

### **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 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 2016 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 2016 Annual Report on Form 10-K.

## Results of Operations

### *Revenues.*

We had no revenues for the three and six-month periods ended June 30, 2017 and 2016.

### *Research and Development Expenses.*

Research and development expenses for the three and six-month periods ended June 30, 2017 were \$2,451,751 and \$5,265,680, respectively, including stock-based compensation expense in each of the three and six-month periods of \$223,552 and \$429,904, respectively. Research and development expenses for the three and six-month periods ended June 30, 2016 were \$2,508,897 and \$6,055,288 respectively, including stock-based compensation expense in each of the three and six-month periods of \$164,392 and \$258,175, respectively. Research and development expenses, in the aggregate, represented approximately 59% and 59% of total operating costs and expenses for the three and six-month periods ended June 30, 2017 and 52% and 55% for the three and six-month periods ended June 30, 2016, 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 six months ended June 30, 2017, excluding stock-based compensation, decreased compared to amounts expended in the same period in 2016. Research and development expenses in the first half of 2016 primarily included, among other items, (i) regulatory affairs and legal costs associated with the receipt of the refusal-to-file letter in February 2016, (ii) costs relating to the close-out of our first Phase 3 trial evaluating Firdapse® for the treatment of LEMS, and (iii) costs incurred to build up inventory to launch Firdapse® in the summer of 2016 (which did not occur as anticipated). Research and development expenses in the first half of 2017 primarily included, among other items, costs associated with our ongoing second Phase 3 trial evaluating Firdapse® for the treatment of LEMS and our ongoing clinical trial evaluating Firdapse® for the treatment of CMS. We expect that research and development costs will continue to be substantial during the balance of 2017 and into 2018 as we complete our second Phase 3 trial evaluating Firdapse® for the treatment of LEMS, continue our clinical trial evaluating Firdapse® for the treatment of CMS, and prepare for the submission of an NDA for Firdapse®.

### *Selling and Marketing Expenses.*

We had no selling expenses for the six month periods ended June 30, 2017 and 2016. In 2016, 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 RTF letter, we put most of these activities on hold in order to conserve cash. We currently expect to recommence developing our commercialization plans for Firdapse® during the second half of 2017. Pre-commercialization costs are included in general and administrative expenses.

### *General and Administrative Expenses.*

General and administrative expenses for the three and six months ended June 30, 2017 were \$1,729,520 and \$3,595,462, respectively, including stock-based compensation expense in each of the three and six-month periods ending June 30, 2017 of \$415,017 and \$962,809, respectively. General and administrative expenses for the three and six months ended June 30, 2016 were \$2,305,555 and \$4,996,700, respectively, including stock-based compensation expense in each of the three and six-month periods ending June 30, 2016 of \$192,877 and \$557,063, respectively. General and administrative expenses represented 41% and 41% of total operating costs and expenses for the three and six months ended June 30, 2017 and 48% and 45% for the three and six months ended June 30, 2016, respectively. The decrease in general and administrative expenses for the six months ended June 30, 2017 when compared to the same period in 2016 is primarily due to decreased employee costs due to a reduction in headcount during May of 2016, and decreases in recruiting expenses and consulting costs for pre-commercialization expenses. We expect that general and administrative costs, excluding pre-commercialization costs, will remain consistent with the amount incurred in the first half of 2017 for the balance of 2017.

### *Stock-Based Compensation.*

Total stock-based compensation for the three and six-month periods ended June 30, 2017 were \$638,569 and \$1,392,713 and for the three and six-month periods ended June 30, 2016 were \$357,269 and \$815,238, respectively. The increase in stock-based compensation for the six-month periods ended June 30, 2017 when compared to the same period in 2016, is primarily due to the expense of options granted to employees and directors during the first half of 2017.

## [Table of Contents](#)

### *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 \$0 and \$122,226 at June 30, 2017 and December 31, 2016, 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 six months ended June 30, 2017, we recognized a gain of \$210,331 and a loss of \$186,904, respectively, due to the change in the fair value of the warrants liability. For the three and six months ended June 30, 2016, we recognized gains of \$152,783 and \$886,139, respectively. The gain during the three months ended June 30, 2017 was principally a result of the decrease of our stock price between March 31, 2017 and the warrants liability expiration date on May 2, 2017. The loss during the six months ended June 30, 2017 was principally a result of the increase of our stock price between December 31, 2016 and the warrants liability expiration date on May 2, 2017. The gains during the three and six months ended June 30, 2016 were principally a result of the decrease of our stock price between March 31, 2016 and June 30, 2016 and between December 31, 2015 and June 30, 2016, respectively.

### *Other Income, Net.*

We reported other income, net in all periods relating to our investment of funds received from offerings of our securities. The slight decrease in other income, net for the six months ended June 30, 2017 when compared to the same period in 2016 is due to lower average investment balances from funding working capital. 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 six-month periods ended June 30, 2017 and 2016, 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 \$3,879,901 and \$8,847,030, respectively, for the three and six months ended June 30, 2017 (\$0.05 and \$0.11, respectively, per basic and diluted share) as compared to a net loss of \$4,568,914 and \$9,955,151, respectively, for the three and six months ended June 30, 2016 (\$0.06 and \$0.12, respectively, per basic and diluted share).

### *Non-GAAP Net Loss.*

Our non-GAAP net loss, which excludes for the three and six months ended June 30, 2017 a gain of \$210,331 and a loss of \$186,904, respectively, associated with the change in the fair value of liability classified warrants, was \$4,090,232 and \$8,660,126 for the three and six months ended June 30, 2017 (\$0.05 and \$0.10 respectively, per basic and diluted share). Our non-GAAP net loss, which excludes for the three and six months ended June 30, 2016 a gain of \$152,783 and a gain of \$886,139, respectively, associated with the change in the fair value of liability classified warrants, was \$4,721,697 and \$10,841,290 for the three and six months ended June 30, 2016 (\$0.06 and \$0.13, 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 June 30, 2017, we had cash and cash equivalents and short-term investments aggregating \$35.1 million and working capital of \$33.9 million. At December 31, 2016, we had cash and cash equivalents and short-term investments aggregating \$40.4 million and working capital of \$39.4 million. At June 30, 2017, substantially all of our cash and cash equivalents were deposited with one financial institution, and such balances were in excess of federally insured limits.

## [Table of Contents](#)

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

Based on forecasts of available cash, we currently believe that we have sufficient resources to fund our operations for at least the next 12 months. These expectations are based on current information available to us. We will also require additional working capital to support our operations beyond that time.

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

- the scope, rate of progress and cost of our clinical trials and other product development activities;
- future clinical trial results;
- the terms and timing of any collaborative, licensing and other arrangements that we may establish;
- the cost and timing of regulatory approvals;
- the cost and delays in product development as a result of any changes in regulatory oversight applicable to our products;
- the cost and timing of establishing sales, marketing and distribution capabilities;
- the effect of competition and market developments;
- the cost of filing and potentially prosecuting, defending and enforcing any patent claims and other intellectual property rights; and
- the extent to which we acquire or invest in other products.

We plan to raise additional funds to support our product development activities and working capital requirements, through public or private equity offerings, debt financings, 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 July 12, 2017, we filed a universal shelf Registration Statement on Form S-3 (the 2017 Shelf Registration Statement) with the SEC to sell up to \$150 million of common stock, preferred stock, warrants to purchase common stock, or debt securities (including debt securities that may be convertible or exchangeable for common stock or other securities), which securities may be offered separately or together in units or multiple series. The 2017 Shelf Registration Statement (file No. 333-219259) was declared effective by the SEC on July 26, 2017. No sales have been conducted to date under the 2017 Shelf Registration Statement.

On December 23, 2016, we filed a Shelf Registration Statement on Form S-3 (the 2016 Shelf Registration Statement) with the SEC to sell up to approximately \$33.8 million of common stock. The 2016 Shelf Registration Statement (file No. 333-215315) was declared effective by the SEC on January 9, 2017. No sales have been conducted to date under the 2016 Shelf Registration Statement.

## [Table of Contents](#)

As of the date of this Form 10-Q, the full amount of our 2016 Shelf Registration Statement and the full amount of our 2017 Shelf Registration Statement remain available for future sales. However, if our public float (the market value of our common stock held by non-affiliate stockholders) were to fall below \$75 million, we would be subject to a further limitation under which we could sell no more than one-third (1/3) of our public float during any 12-month period. Further, the number of shares that we can sell at any one time may be limited under certain circumstances to 20% of the outstanding common stock under applicable NASDAQ marketplace rules.

On March 19, 2017, the shelf registration statement that we filed with the SEC in 2014 (file No. 333-193699) expired.

### *Cash Flows*

Net cash used in operating activities was \$7,079,864 and \$10,273,738, respectively, for the six-month periods ended June 30, 2017 and 2016. During the six months ended June 30, 2017, net cash used in operating activities was primarily attributable to our net loss of \$8,847,030, decreases of \$240,831 in accounts payable and \$23,921 in accrued expenses and other liabilities. This was partially offset by a \$426,386 decrease in prepaid expenses and other current assets and deposits, \$186,904 of non-cash change in fair value of warrants liability and \$1,418,628 of other non-cash expenses. During the six months ended June 30, 2016, net cash used in operating activities was primarily attributable to our net loss of \$9,955,151, decreases of \$1,086,835 in accounts payable and \$36,572 in accrued expenses and other liabilities and \$886,139 of non-cash change in fair value of warrants liability. This was partially offset by \$853,761 decrease in prepaid expenses and other current assets and deposits and \$837,198 of other non-cash expenses. Other non-cash expenses include depreciation and stock-based compensation expense.

Net cash used in investing activities during the six-month period ended June 30, 2017 was \$34,910, consisting of the purchase of short-term investments. Net cash provided by investing activities during the six-month period ended June 30, 2016 was \$767,091, consisting primarily of \$949,283 of proceeds from certificates of deposit, offset by \$88,929 of capital expenditures and \$93,263 of purchase of short-term investments.

Net cash provided by financing activities during the six-month period ended June 30, 2017 was \$1,805,437, consisting of the net proceeds from exercise of warrants. Net cash used in financing activities during the six-month period ended June 30, 2016 was \$11,265, for payment of employee withholding tax related to stock based compensation.

### *Contractual Obligations*

We have entered into the following contractual arrangements:

- *Payments to BioMarin and others under our license agreement with BioMarin.* We have agreed to pay certain payments under to our license agreement with BioMarin.
  - *Royalties:* We have agreed to pay (i) royalties to BioMarin 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; and (ii) royalties to the third-party licensor of the rights sublicensed to us 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.
  - *Milestone Payments.* We have agreed to pay certain milestone payments that BioMarin is obligated to pay to both the third-party licensor and to the former stockholders of Huxley Pharmaceuticals (“Huxley”) under an earlier stock purchase agreement between BioMarin and the former Huxley stockholders. These milestones aggregate (i) up to approximately \$2.6 million due upon acceptance by the FDA of a filing of an NDA for Firdapse® for the treatment of LEMS or CMS, and (ii) up to approximately \$7.2 million due on the unconditional approval by the FDA of an NDA for Firdapse® for the treatment of LEMS; *provided, however* that the total milestone payments that we are obligated to pay if we meet milestones (i) and/or (ii) above will be reduced to an aggregate of \$150,000 and \$3.0 million, respectively, if either of these milestones are satisfied after April 20, 2018 (the date on which BioMarin’s obligations to pay milestone payments to the former stockholders of Huxley expire).

## Table of Contents

- *Cost Sharing Payments.* We have agreed to share in the cost of certain post-marketing studies being conducted by BioMarin, and, as of June 30, 2017, the Company had paid BioMarin \$3.8 million related to expenses in connection with Firdapse® studies and trials.
- *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 \$205,000, at June 30, 2017 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 \$485,000 in 2017. 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 intended to identify forward-looking statements. Such statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by such forward looking statements. The forward-looking statements made in this report are based on current expectations that involve numerous risks and uncertainties.

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 phosphate (3,4-DAP) for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS), Congenital Myasthenic Syndromes (CMS), or any other indication, before we do;
- whether the clinical studies or trials that are required to be completed before the FDA will accept an NDA submission for Firdapse® for the treatment of either LEMS or CMS will be successful;
- what additional supporting information, including any additional clinical studies or trials, will be required before the FDA will accept our NDA submission for Firdapse® for the treatment of either LEMS or CMS (or any other condition or disease);

## Table of Contents

- whether any NDA that we may submit for Firdapse® will be accepted for filing by the FDA, and, if accepted, whether it will be granted a priority review;
- 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 for any of the submitted indications;
- whether the receipt of breakthrough therapy designation for Firdapse® for LEMS will result in an expedited 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 or contract with a sales and marketing organization that can successfully market Firdapse® while maintaining full compliance with applicable federal and state laws, rules and regulations;
- whether any future trial that we undertake evaluating Firdapse® for the treatment of MuSK-MG will be successful and whether we can obtain the funding required for such trial;
- 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, 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 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, and whether our trials and studies will be successful;
- our ability to complete our trials and studies on a timely basis and within the budgets we establish for such trials and studies;
- the scope, rate of progress and expense of our clinical trials and studies, pre-clinical studies, proof-of-concept studies, and our other development activities;
- the ability of our third-party suppliers and contract manufacturers to maintain compliance with cGMP;
- whether our estimates of the size of the market for our drug candidates will turn out to be accurate;
- the pricing of our products that we may be able to achieve if we are granted the ability to commercialize our drug candidates; and
- changes in the healthcare industry occasioned by any future repeal and replacement of the Affordable Care Act, in laws relating to the pricing of drug products, or changes in the healthcare industry generally;

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

## [Table of Contents](#)

inaccurate. In light of the significant uncertainties inherent in the forward-looking statements we have made herein, which reflect our views only as of the date of this report, you should not place undue reliance upon such statements. We undertake no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

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

Our market risks during the three and six months ended June 30, 2017 have not materially changed from those discussed in Item 7A of our Annual Report on Form 10-K for the year ended December 31, 2016.

### **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 June 30, 2017, 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 June 30, 2017, 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.

## PART II. OTHER INFORMATION

### ITEM 1. LEGAL PROCEEDINGS

The Company is not a party to any material 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 2016 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

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

**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: August 9, 2017

**Exhibit Index**

<u>Exhibit Number</u>	<u>Description</u>
31.1	Certification of Principal Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002
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101.LAB	XBRL Taxonomy Extension Label Linkbase
101.PRE	XBRL Taxonomy Extension Presentation Linkbase

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**Certification of Principal Executive Officer**

I, Patrick J. McEnany, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Catalyst Pharmaceuticals, Inc.;
2. Based on my knowledge, this quarterly report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15(d)-15(f)) for the registrant and have:
  - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of registrant's board of directors (or persons performing the equivalent functions):
  - a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 9, 2017

/s/ Patrick J. McEnany

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Patrick J. McEnany  
Chief Executive Officer  
(Principal Executive Officer)

**Certification of Principal Financial Officer**

I, Alicia Grande, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Catalyst Pharmaceuticals, Inc.;
2. Based on my knowledge, this quarterly report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15(d)-15(f)) for the registrant and have:
  - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of registrant's board of directors (or persons performing the equivalent functions):
  - a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 9, 2017

/s/ Alicia Grande

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Alicia Grande  
Chief Financial Officer  
(Principal Financial Officer)

**Certification Required by 18 U.S.C. Section 1350  
(as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002)**

I, Patrick J. McEnany as Principal Executive Officer of Catalyst Pharmaceuticals, Inc. (the "Company"), certify, pursuant to 18 U.S.C. Section 1350 (as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002), that to my knowledge:

1. the accompanying Quarterly Report on Form 10-Q of the Company for the quarterly period ended June 30, 2017 (the "Report"), filed with the U.S. Securities and Exchange Commission, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and
2. the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: August 9, 2017

/s/ Patrick J. McEnany

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Patrick J. McEnany  
Chief Executive Officer  
(Principal Executive Officer)

**Certification Required by 18 U.S.C. Section 1350  
(as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002)**

I, Alicia Grande as Principal Financial Officer of Catalyst Pharmaceuticals, Inc. (the "Company"), certify, pursuant to 18 U.S.C. Section 1350 (as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002), that to my knowledge:

1. the accompanying Quarterly Report on Form 10-Q of the Company for the quarterly period ended June 30, 2017 (the "Report"), filed with the U.S. Securities and Exchange Commission, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and
2. the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: August 9, 2017

/s/ Alicia Grande

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Alicia Grande  
Chief Financial Officer  
(Principal Financial Officer)