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

FORM 10-Q

[Mark One]

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

For the Quarterly Period Ended September 30, 2019

OR

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

Commission File No. 001-33057

CATALYST PHARMACEUTICALS, INC.
(Exact name of registrant as specified in its charter)

Delaware	**76-0837053**
(State or other jurisdiction of incorporation or organization)	**(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

Securities registered pursuant to Section 12(b) of the Act:

Title of Each Class	Ticker Symbol(s)	Name of Exchange on Which Registered
Common Stock, par value $0.001 per share	**CPRX**	**NASDAQ Capital Market**

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 every Interactive Data File required to be submitted 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 such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, anon-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:

Large accelerated filer	☐	Accelerated Filer	☒
Non-accelerated filer	☐	Smaller reporting company	☒
Emerging growth company	☐		

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 103,047,033 shares of common stock, $0.001 par value per share, were outstanding as of November 8, 2019.

CATALYST PHARMACEUTICALS, INC.

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CATALYST PHARMACEUTICALS, INC.
CONSOLIDATED BALANCE SHEETS

	September 30, 2019 (unaudited)	December 31, 2018
ASSETS		
Current Assets:		
Cash and cash equivalents	$ 44,983,218	$ 16,559,400
Short-term investments	31,561,673	36,922,213
Accounts receivable, net	10,095,352	—
Inventory	599,801	56,012
Prepaid expenses and other current assets	3,339,399	1,649,781
Total current assets	90,579,443	55,187,406
Investments	5,008,800	5,008,243
Operating lease right-of-use asset	952,340	—
Property and equipment, net	141,088	245,425
Deposits	8,888	8,888
Total assets	$ 96,690,559	$ 60,449,962
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current Liabilities:		
Accounts payable	$ 4,147,029	$ 2,337,367
Accrued expenses and other liabilities	13,835,292	7,173,987
Total current liabilities	17,982,321	9,511,354
Accrued expenses and other liabilities, non-current	—	154,799
Operating lease liability, net of current portion	725,700	—
Total liabilities	18,708,021	9,666,153
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, $0.001 par value, 5,000,000 shares authorized: none issued and outstanding at September 30, 2019 and December 31, 2018	—	—
Common stock, $0.001 par value, 150,000,000 shares authorized; 103,041,033 shares and 102,739,257 shares issued and outstanding at September 30, 2019 and December 31, 2018, respectively	103,041	102,739
Additional paid-in capital	214,478,406	211,265,279
Accumulated deficit	(136,618,337)	(160,563,961)
Accumulated other comprehensive income (loss)	19,428	(20,248)
Total stockholders' equity	77,982,538	50,783,809
Total liabilities and stockholders' equity	$ 96,690,559	$ 60,449,962

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

CATALYST PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE INCOME (LOSS)
(unaudited)

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2019	2018	2019	2018
Product revenue, net	$ 30,897,444	$ —	$ 72,183,782	$ —
Operating costs and expenses:				
Cost of sales	4,387,461	—	10,360,874	—
Research and development	4,597,039	4,538,369	12,534,362	11,502,235
Selling, general and administrative	8,067,792	3,644,234	25,471,974	8,949,663
Total operating costs and expenses	17,052,292	8,182,603	48,367,210	20,451,898
Operating income (loss)	13,845,152	(8,182,603)	23,816,572	(20,451,898)
Other income, net	393,415	343,730	1,187,091	947,993
Net income (loss) before income taxes	14,238,567	(7,838,873)	25,003,663	(19,503,905)
Provision for income taxes	608,388	—	1,058,039	—
Net income (loss)	$ 13,630,179	$ (7,838,873)	$ 23,945,624	$ (19,503,905)
Net income (loss) per share:				
Basic	$ 0.13	$ (0.08)	$ 0.23	$ (0.19)
Diluted	$ 0.13	$ (0.08)	$ 0.23	$ (0.19)
Weighted average shares outstanding:				
Basic	102,974,105	102,641,504	102,864,571	102,598,740
Diluted	107,045,234	102,641,504	105,821,609	102,598,740
Net income (loss)	$ 13,630,179	$ (7,838,873)	$ 23,945,624	$ (19,503,905)
Other comprehensive income (loss):				
Unrealized gain (loss) on available-for-sale securities	(2,330)	(9,450)	39,676	(45,948)
Comprehensive income (loss)	$ 13,627,849	$ (7,848,323)	$ 23,985,300	$ (19,549,853)

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

CATALYST PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY (unaudited)
For the three and nine months ended September 30, 2019 and 2018

	Preferred Stock	Common Stock	Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Gain (Loss)	Total
Balance at December 31, 2018	$ —	$102,739	$ 211,265,279	$(160,563,961)	$ (20,248)	$ 50,783,809
Issuance of stock options for services	—	—	933,411	—	—	933,411
Exercise of stock options for common stock	—	65	89,285	—	—	89,350
Other comprehensive gain (loss)	—	—	—	—	13,560	13,560
Net income (loss)	—	—	—	(644,503)	—	(644,503)
Balance at March 31, 2019	—	102,804	212,287,975	(161,208,464)	(6,688)	51,175,627
Issuance of stock options for services	—	—	924,996	—	—	924,996
Exercise of stock options for common stock	—	125	192,425	—	—	192,550
Other comprehensive gain (loss)	—	—	—	—	28,446	28,446
Net income (loss)	—	—	—	10,959,948	—	10,959,948
Balance at June 30, 2019	—	102,929	213,405,396	(150,248,516)	21,758	63,281,567
Issuance of stock options for services	—	—	817,060	—	—	817,060
Exercise of stock options for common stock	—	112	255,950	—	—	256,062
Other comprehensive gain (loss)	—	—	—	—	(2,330)	(2,330)
Net income (loss)	—	—	—	13,630,179	—	13,630,179
Balance at September 30, 2019	$ —	$103,041	$ 214,478,406	$(136,618,337)	$ 19,428	$ 77,982,538

	Preferred Stock	Common Stock	Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Gain (Loss)	Total
Balance at December 31, 2017	$ —	$102,549	$ 207,421,710	$(126,560,447)	$ —	$ 80,963,812
Issuance of stock options for services	—	—	971,340	—	—	971,340
Exercise of stock options for common stock	—	37	32,995	—	—	33,032
Other comprehensive gain (loss)	—	—	—	—	(21,826)	(21,826)
Net income (loss)	—	—	—	(5,699,892)	—	(5,699,892)
Balance at March 31, 2018	—	102,586	208,426,045	(132,260,339)	(21,826)	76,246,466
Issuance of common stock, net	—	3	10,546	—	—	10,549
Issuance of stock options for services	—	—	776,510	—	—	776,510
Exercise of stock options for common stock	—	10	8,490	—	—	8,500
Other comprehensive gain (loss)	—	—	—	—	(14,672)	(14,672)
Net income (loss)	—	—	—	(5,965,140)	—	(5,965,140)
Balance at June 30, 2018	—	102,599	209,221,591	(138,225,479)	(36,498)	71,062,213
Issuance of stock options for services	—	—	758,176	—	—	758,176
Exercise of stock options for common stock	—	90	104,442	—	—	104,532
Other comprehensive gain (loss)	—	—	—	—	(9,450)	(9,450)
Net income (loss)	—	—	—	(7,838,873)	—	(7,838,873)
Balance at September 30, 2018	$ —	$102,689	$ 210,084,209	$(146,064,352)	$ (45,948)	$ 64,076,598

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

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

	For the Nine Months Ended September 30,	
	2019	**2018**
Operating Activities:		
Net income (loss)	$ 23,945,624	$(19,503,905)
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities:		
Depreciation	26,718	25,485
Non-cash change in right-of-use asset	181,301	—
Stock-based compensation	2,675,467	2,521,023
Change in accrued interest and accretion of discount on investments	(185,536)	(405,082)
(Increase) decrease in:		
Accounts receivable, net	(10,095,352)	—
Inventory	(543,789)	—
Prepaid expenses and other current assets and deposits	(1,689,618)	356,924
Increase (decrease) in:		
Accounts payable	1,809,662	(604,591)
Accrued expenses and other liabilities	6,400,279	(185,812)
Operating lease liability	(204,724)	—
Net cash provided by (used in) operating activities	22,320,032	(17,795,958)
Investing Activities:		
Purchases of property and equipment	(19,370)	(35,193)
Purchases of investments	(34,725,401)	(36,790,854)
Proceeds from sales/maturities of investments	40,310,595	7,600,000
Net cash provided by (used in) investing activities	5,565,824	(29,226,047)
Financing Activities:		
Payment of employee withholding tax related to stock-based compensation	—	(4,448)
Proceeds from exercise of stock options	537,962	146,064
Net cash provided by (used in) financing activities	537,962	141,616
Net increase (decrease) in cash and cash equivalents	28,423,818	(46,880,389)
Cash and cash equivalents - beginning of period	16,559,400	57,496,702
Cash and cash equivalents - end of period	$ 44,983,218	$ 10,616,313
Non-cash investing and financing activities:		
Unrealized gain (loss) on available-for-sale securities	$ 39,676	$ (45,948)

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

CATALYST PHARMACEUTICALS, INC.
NOTES TO UNAUDITED CONSOLIDATED FINANCIAL STATEMENTS

1. Organization and Description of Business.

Catalyst Pharmaceuticals, Inc. and subsidiary (collectively, the Company) is a biopharmaceutical company focused on developing and commercializing innovating therapies for people with rare debilitating, chronic neuromuscular and neurological diseases, including Lambert-Eaton Myasthenic Syndrome (LEMS), Anti-MuSK antibody positive myasthenia gravis (MuSK-MG), Congenital Myasthenic Syndromes (CMS), and Spinal Muscular Atrophy (SMA) Type 3.

On November 28, 2018, the U.S. Food and Drug Administration, or FDA, granted approval of Firdapse® for the treatment of adults with LEMS. On January 15, 2019, the Company launched its first product, Firdapse®, in the United States for the treatment of adults with LEMS (age 17 and above).

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, raising capital, and selling its product. The Company incurred operating losses in each period from inception through March 31, 2019, and started reporting operating income in the three and six month periods ended June 30, 2019. The Company has been able to fund its cash needs to date through several public and private offerings of its securities and from revenues from its product sales. See Note 12.

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 from the issuance date of this Form 10-Q.

The Company may raise required funds in the future through public or private equity offerings, debt financings, corporate collaborations, governmental research grants or other means. The Company may also seek to raise new capital to fund additional product development efforts, even if it has sufficient funds for its planned operations. Any sale by the Company of additional equity or convertible debt securities could result in dilution to the Company's current stockholders. There can be no assurance that any 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 consolidated 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 consolidated balance sheet as of December 31, 2018 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 consolidated 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 consolidated statements should be read in conjunction with the financial statements and notes thereto for the year ended December 31, 2018 included in the 2018 Annual Report on Form 10-K filed by the Company with the SEC. The results of operations for the nine months ended September 30, 2019 are not necessarily indicative of the results to be expected for any future period or for the full 2019 fiscal year.

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

b. **PRINCIPLES OF CONSOLIDATION**. The consolidated financial statements include the Company's accounts and those of its wholly-owned subsidiary Catalyst Pharmaceuticals Ireland, Ltd. ("Catalyst Ireland"). All intercompany accounts and transactions have been eliminated in consolidation. Catalyst Ireland was organized in 2017.

c. **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.

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

e. **INVESTMENTS**. The Company invests in high credit-quality funds in order to obtain higher yields on its cash available for investments. At September 30, 2019 and December 31, 2018, investments consisted of a short-term bond fund and U.S. Treasuries. Such investments are not insured by the Federal Deposit Insurance Corporation.

Short-Term Bond Fund

The short-term bond fund is classified in 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. At September 30, 2019 and December 31, 2018, the only investment classified as trading securities was the short-term bond fund. Realized losses on trading securities were $0 and $4,980, respectively, for the three and nine months ended September 30, 2019. There were no sales of trading securities for the three and nine months ended September 30, 2018. Unrealized gain (loss) on trading securities was $0 and $89,405, respectively, for the three and nine months ended September 30, 2019, and $0 and ($29,430), respectively, for the three and nine months ended September 30, 2018 and is included in other income, net in the accompanying consolidated statements of operations.

U.S. Treasuries

U.S. Treasuries are classified as available-for-sale securities. The Company classifies available-for-sale securities with stated maturities of greater than three months and less than one year from the date of purchase as short-term investments. Available-for-sale securities with stated maturities greater than one year are classified as non-current investments in the accompanying consolidated balance sheets. The Company records available-for-sale securities at fair value with unrealized gains and losses reported in accumulated other comprehensive income (loss) in stockholders' equity. Realized gains and losses are included in other income, net and are derived using the specific identification method for determining the cost of securities sold. Interest income is recognized when earned and is included in other income, net in the consolidated statements of operations. The Company recognizes a charge when the declines in the fair value below the amortized cost basis of its available-for-sale securities are judged to be other-than-temporary. The Company considers various factors in determining whether to recognize an other-than-temporary charge, including whether the Company intends to sell the security or whether it is more likely than not that the Company would be required to sell the security before recovery of the amortized cost basis. The Company has not recorded any other-than-temporary impairment charges on its available-for-sale securities. See Note 3.

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

f. ACCOUNTS RECEIVABLE, NET. Accounts receivable are recorded net of customer allowance for distribution fees, trade discounts, prompt payment discounts, chargebacks and doubtful accounts. Allowances for distribution fees, prompt payment discounts and chargebacks are based on contractual terms. The Company estimates the allowance for doubtful accounts based on existing contractual payment terms, actual payment patterns of its customer and individual customer circumstances. At September 30, 2019, the Company determined that an allowance for doubtful accounts was not required. No accounts were written off during the periods presented.

g. INVENTORY. Inventories are stated at the lower of cost or net realizable value with cost determined under the first-in-first-out (FIFO) cost method. Inventories consist of raw materials and supplies, work in process and finished goods. Costs to be capitalized as inventories include third party manufacturing costs, associated compensation related costs of personnel indirectly involved in the manufacturing process and other overhead costs. The Company began capitalizing inventories post FDA approval of Firdapse® on November 28, 2018 as the related costs were expected to be recoverable through the commercialization of the product. Costs incurred prior to the FDA approval of Firdapse® were recorded as research and development expenses in the consolidated statements of operations. If information becomes available that suggests that inventories may not be realizable, the Company may be required to expense a portion or all of the previously capitalized inventories. As of September 30, 2019 inventory consisted mainly of finished goods. As of December 31, 2018, inventory consisted mainly of packaging and labeling costs.

Products that have been approved by the FDA or other regulatory authorities, such as Firdapse®, are also used in clinical programs to assess the safety and efficacy of the products for usage in treating diseases that have not been approved by the FDA or other regulatory authorities. The form of Firdapse® utilized for both commercial and clinical programs is identical and, as a result, the inventory has an "alternative future use" as defined in authoritative guidance. Raw materials and purchased drug product associated with clinical development programs are included in inventory and charged to research and development expense when the product enters the research and development process and no longer can be used for commercial purposes and, therefore, does not have an "alternative future use".

The Company evaluates for potential excess inventory by analyzing current and future product demand relative to the remaining product shelf life. The Company builds demand forecasts by considering factors such as, but not limited to, overall market potential, market share, market acceptance, and patient usage.

h. PREPAID EXPENSES AND OTHER CURRENT ASSETS. Prepaid expenses and other current assets consist primarily of prepaid research fees, prepaid commercialization expenses, prepaid insurance, prepaid subscription fees and prepaid manufacturing. Prepaid research fees consist of advances for the Company's product development activities, including contracts for pre-clinical studies, clinical trials and studies, regulatory affairs and consulting. Prepaid manufacturing consists of advances for the Company's drug manufacturing activities. Such advances are recorded as expense as the related goods are received or the related services are performed.

i. FAIR VALUE OF FINANCIAL INSTRUMENTS. The Company's financial instruments consist of cash and cash equivalents, investments, accounts receivable, accounts payables and accrued expenses and other liabilities. At September 30, 2019 and December 31, 2018, the fair value of these instruments approximated their carrying value.

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

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

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

		Fair Value Measurements at Reporting Date Using		
	Balances as of September 30, 2019	Quoted Prices in Active Markets for Identical Assets/Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Cash and cash equivalents:				
Money market funds	$43,011,647	$ 43,011,647	$ —	$ —
Short-term investments:				
Short-term bond fund	$16,604,773	$ 16,604,773	$ —	$ —
U.S. Treasuries	$14,956,900	$ —	$14,956,900	$ —
Investments:				
U.S. Treasuries	$ 5,008,800	$ —	$ 5,008,800	$ —

	Balances as of December 31, 2018	Quoted Prices in Active Markets for Identical Assets/Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Cash and cash equivalents:				
Money market funds	$14,462,087	$ 14,462,087	$ —	$ —
Short-term investments:				
Short-term bond fund	$26,541,349	$ 26,541,349	$ —	$ —
U.S. Treasuries	$10,380,864	$ —	$10,380,864	$ —
Investments:				
U.S. Treasuries	$ 5,008,243	$ —	$ 5,008,243	$ —

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

k. OPERATING LEASES. Effective January 1, 2019, the Company determined if an arrangement is a lease at inception. Operating leases are included in operating lease right-of-use ("ROU") assets, other current liabilities, and operating lease liabilities on its consolidated balance sheets.

Operating lease ROU assets and operating lease liabilities are recognized based on the present value of the future minimum lease payments over the lease term at commencement date. As the Company's leases do not provide an implicit rate, the Company uses its incremental borrowing rate based on the information available at commencement date in determining the present value of future payments. The operating lease ROU asset also includes any lease payments made and excludes lease incentives and initial direct costs incurred. The Company's lease terms do not include options to extend or terminate the lease as it is not reasonably certain that it will exercise these options. Lease expense for minimum lease payments is recognized on a straight-line basis over the lease term. The Company has lease agreements with lease and non-lease components, which are generally accounted for separately. Refer to Note 2.r. for discussion on adoption method.

l. REVENUE RECOGNITION. Prior to the January 2019 launch of Firdapse®, the Company did not generate any product revenue. Therefore, on January 1, 2019, the Company adopted Accounting Standards Codification ("ASC") Topic 606 – Revenue from Contracts with Customers ("Topic 606"). This standard applies to all contracts with customers, except for contracts that are within the scope of other standards, such as leases, collaborative arrangements and financial instruments. Under Topic 606, an entity recognizes revenue when its customer obtains control of the promised goods or services, in an amount that reflects the consideration which the entity expects to be entitled in exchange for these goods or services. The Company had no contracts with customers until the FDA approved Firdapse® in November 2018. Subsequent to receiving FDA approval, the Company entered into an arrangement with one distributor (the "Customer"), who is the exclusive distributor of Firdapse® in the United States. The Customer subsequently resells Firdapse® to a small group of exclusive specialty pharmacies ("SPs") whose dispensing activities for patients with specific payors may result in government-mandated or privately negotiated rebate obligations for the Company with respect to the purchase of Firdapse®.

To determine revenue recognition for arrangements that are within the scope of Topic 606, the Company performs the following five steps: (i) identify the contract(s) with a customer, (ii) identify the performance obligations in the contract, (iii) determine the transaction price, (iv) allocate the transaction price to the performance obligations in the contract, and (v) recognize revenue when (or as) the entity satisfies a performance obligation. The Company only applies the five-step model to arrangements that meet the definition of a contract under Topic 606, including when it is probable that the Company will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, once the contract is determined to be within the scope of Topic 606, the Company assesses the goods or services promised within each contract and determines those that are performance obligations and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied. For a complete discussion of accounting for product revenue, see *Product Revenue, Net* below.

The Company also generates revenues from payments received under a collaborative agreement. Collaborative agreement payments may include nonrefundable fees at the inception of the agreements, milestone and event-based payments for specific achievements designated in the collaborative agreements, and/or royalties on sales of products resulting from a collaborative arrangement. For a complete discussion of accounting for collaborative arrangements, see *Revenues from Collaborative Arrangement* below.

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

Product Revenue, Net: The Company sells Firdapse® to a Customer (its exclusive distributor) who subsequently resells Firdapse® to both a small group of SPs who have exclusive contracts with the Company to distribute the Company's products to patients and potentially to medical centers or hospitals on an emergency basis. In addition to the distribution agreement with its Customer, the Company enters into arrangements with health care providers and payors that provide for government-mandated and/or privately negotiated rebates, chargebacks, and discounts with respect to the purchase of the Company's products.

The Company recognizes revenue on product sales when the Customer obtains control of the Company's product, which occurs at a point in time (upon delivery). Product revenue is recorded net of applicable reserves for variable consideration, including discounts and allowances. The Company's payment terms range between 15 and 60 days.

Shipping and handling costs for product shipments occur prior to the customer obtaining control of the goods, and are recorded in cost of sales.

If taxes should be collected from the Customer relating to product sales and remitted to governmental authorities, they will be excluded from revenue. The Company expenses incremental costs of obtaining a contract when incurred, if the expected amortization period of the asset that the Company would have recognized is one year or less. However, no such costs were incurred during the three and nine months ended September 30, 2019.

As of September 30, 2019, all of the Company's sales are to its Customer.

Reserves for Variable Consideration: Revenue from product sales are recorded at the net sales price (transaction price), which includes estimates of variable consideration for which reserves are established. Components of variable consideration include trade discounts and allowances, product returns, provider chargebacks and discounts, government rebates, and other incentives, such as voluntary patient assistance, and other allowances that are offered within contracts between the Company and its Customer, payors, and other indirect customers relating to the Company's sale of its products. These reserves, as detailed below, are based on the amounts earned, or to be claimed on the related sales, and are classified as reductions of accounts receivable (if the amount is payable to the Customer) or a current liability (if the amount is payable to a party other than a customer). These estimates take into consideration a range of possible outcomes which are probability-weighted in accordance with the expected value method in Topic 606 for relevant factors such as current contractual and statutory requirements, specific known market events and trends, industry data, and forecasted customer buying and payment patterns. Overall, these reserves reflect the Company's best estimates of the amount of consideration to which it is entitled based on the terms of the respective underlying contracts.

The amount of variable consideration which is included in the transaction price may be constrained, and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized under the contract will not occur in a future period. The Company's analyses also contemplated application of the constraint in accordance with the guidance, under which it determined a material reversal of revenue would not occur in a future period for the estimates detailed below as of September 30, 2019 and, therefore, the transaction price was not reduced further during the three and nine months ended September 30, 2019. Actual amounts of consideration ultimately received may differ from the Company's estimates. If actual results in the future vary from the Company's estimates, the Company will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known.

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

Trade Discounts and Allowances: The Company provides its Customer with a discount that is explicitly stated in its contract and is recorded as a reduction of revenue in the period the related product revenue is recognized. In addition, the Company receives sales order management, data and distribution services from the Customer. To the extent the services received are distinct from the sale of Firdapse® to the Customer, these payments are classified in selling, general and administrative expenses in the Company's consolidated statement of operations and comprehensive income (loss). However, if the Company has determined such services received to date are not distinct from the Company's sale of products to the Customer, these payments have been recorded as a reduction of revenue within the statement of operations and comprehensive income (loss) through September 30, 2019, as well as a reduction to accounts receivables, net on the consolidated balance sheets.

Funded Co-pay Assistance Program: The Company contracts with a third-party to manage the co-pay assistance program intended to provide financial assistance to qualified insured patients. The calculation of the accrual for co-pay assistance is based on an estimate of claims and the cost per claim that the Company expects to receive associated with Firdapse® that has been recognized as revenue, but remains in the distribution channel at the end of each reporting period. These payments are considered payable to the customer and the related reserve is recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included in accrued expenses and other current liabilities in the consolidated balance sheets.

Product Returns: Consistent with industry practice, the Company offers the SPs and its distributor limited product return rights for damaged and expiring product, provided it is within a specified period around the product expiration date as set forth in the applicable individual distribution agreement. The Company estimates the amount of its product sales that may be returned by its Customer and records this estimate as a reduction of revenue in the period the related product revenue is recognized, as well as reductions to accounts receivable, net on the consolidated balance sheets. The Company currently estimates product return liabilities using available industry data and its own sales information, including its visibility into the inventory remaining in the distribution channel. The Company has an insignificant amount of returns to date and believes that returns of its products will continue to be minimal.

Provider Chargebacks and Discounts: Chargebacks for fees and discounts to providers represent the estimated obligations resulting from contractual commitments to sell products to qualified healthcare providers at prices lower than the list prices charged to the Customer who directly purchases the product from the Company. The Customer charges the Company for the difference between what they pay for the product and the ultimate selling price to the qualified healthcare providers. These reserves are established in the same period that the related revenue is recognized, resulting in a reduction of product revenue and accounts receivables, net. Chargeback amounts are generally determined at the time of resale to the qualified healthcare provider by the Customer, and the Company generally issues credits for such amounts within a few weeks of the Customer's notification to the Company of the resale. Reserves for chargebacks consist principally of chargebacks that the Customer has claimed, but for which the Company has not yet issued a credit.

Government Rebates: The Company is subject to discount obligations under state Medicaid programs and Medicare. These reserves are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included in accrued expenses and other current liabilities on the consolidated balance sheets. For Medicare, the Company also estimates the number of patients in the prescription drug coverage gap for whom the Company will owe an additional liability under the Medicare Part D program. The Company's liability for these rebates consists of invoices received for claims from prior quarters that have not been paid or for which an invoice has not yet been received, estimates of claims for the current quarter, and estimated future claims that will be made for product that has been recognized as revenue, but which remains in the distribution channel inventories at the end of each reporting period.

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

Bridge and Patient Assistance Programs: The Company provides free Firdapse® to uninsured patients who satisfypre-established criteria for either the Bridge Program or the Patient Assistance Program. Patients who meet the Bridge Program eligibility criteria and are transitioning from investigational product while they are waiting for a coverage determination, or later, for patients whose access is threatened by the complications arising from a change of insurer may receive a temporary supply of free Firdapse® while the Company is determining the patient's third-party insurance, prescription drug benefit or other third-party coverage for Firdapse®. The Patient Assistance Program provides free Firdapse® for longer periods of time for those who are uninsured or functionally uninsured with respect to Firdapse® because they are unable to obtain coverage from their payer despite having health insurance. The Company does not recognize any revenue related to these free products and the associated costs are classified in selling, general and administrative expenses in the Company's consolidated statements of operations.

Revenues from Collaborative Arrangement: The Company has entered into a collaboration agreement for the further development and commercialization of generic Sabril® (vigabatrin) tablets. Pursuant to the terms of this agreement, collaborators could be required to make various payments to the Company, including upfront license fees, milestone payments based on achievement of regulatory approvals, and royalties on sales of products resulting from collaborative agreement.

Nonrefundable upfront license fees are recognized upon receipt as persuasive evidence of an arrangement exists, the price to the collaborator is fixed or determinable and collectability is reasonably assured. For the three and nine months ended September 30, 2019 and 2018, no revenue was recognized.

Refer to Note 8, Collaborative Arrangement, for further discussion on the Company's collaborative arrangement.

m. CONCENTRATION OF RISK. The Company sells its product in the United States through an exclusive distributor to specialty pharmacies. Therefore, its distributor and specialty pharmacies account for all of its trade receivables and net product revenues. The creditworthiness of its Customer is continuously monitored, and the Company has internal policies regarding customer credit limits. The Company estimates an allowance for doubtful accounts primarily based on the credit worthiness of its Customer, historical payment patterns, aging of receivable balances and general economic conditions.

The Company currently has a single product with limited commercial sales experience, which makes it difficult to evaluate its current business, predict its future prospects and forecast financial performance and growth. The Company has invested a significant portion of its efforts and financial resources in the development and commercialization of the lead product, Firdapse®, and expects Firdapse® to constitute virtually all of product revenue for the foreseeable future. The Company's success depends on its ability to effectively commercialize Firdapse®.

The Company relies exclusively on third parties to formulate and manufacture Firdapse® and its drug candidates. The commercialization of Firdapse® and any other drug candidates, if approved, could be stopped, delayed or made less profitable if those third parties fail to provide sufficient quantities of product or fail to do so at acceptable quality levels or prices. The Company does not intend to establish its own manufacturing facilities. The Company is using the same third-party contractors to manufacture, supply, store and distribute drug supplies for clinical trials and the commercialization of Firdapse®. If the Company is unable to continue its relationships with one or more of these third-party contractors, it could experience delays in the development or commercialization efforts as it locates and qualifies new manufacturers. The Company intends to rely on one or more third-party contractors to manufacture the commercial supply of drugs.

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

n. ROYALTIES. Royalties incurred in connection with the Company's license agreement with BioMarin, as disclosed in Note 10 – Agreements, are expensed to cost of sales as revenue from product sales is recognized.

o. STOCK-BASED COMPENSATION. The Company recognizes expense in the consolidated statements of operations for the fair value of all stock-based payments to employees, directors 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 five years. Forfeitures are recognized as a reduction of stock-based compensation expense as they occur.

p. COMPREHENSIVE INCOME (LOSS). U.S. GAAP requires 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. The Company's comprehensive income (loss) is shown on the consolidated statements of operations and comprehensive income (loss) for the three and nine months ended September 30, 2019 and 2018, and is comprised of net unrealized gains (losses) on the Company's available-for-sale securities.

q. NET INCOME (LOSS) PER COMMON SHARE. Basic net income (loss) per share is computed by dividing net income (loss) for the period by the weighted average number of common shares outstanding during the period. With regard to common stock subject to vesting requirements, the calculation includes only the vested portion of such stock and units.

Diluted net income per common share is computed by dividing net income (loss) by the weighted average number of common shares outstanding, increased by the assumed conversion of other potentially dilutive securities during the period.

The following table reconciles basic and diluted weighted average common shares:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2019	2018	2019	2018
Basic weighted average common shares outstanding	102,974,105	102,641,504	102,864,571	102,598,740
Effect of dilutive securities:				
Common stock issuable upon the exercise of stock options	4,071,129	—	2,957,038	—
Diluted weighted average common shares outstanding	107,045,234	102,641,504	105,821,609	102,598,740

Outstanding common stock equivalents totaling approximately 0.4 million and 2.9 million, respectively, were excluded from the calculation of diluted net income (loss) per common share for the three and nine months ended September 30, 2019 as their effect would be anti-dilutive. For the three and nine months ended September 30, 2018, approximately 8.1 million shares of outstanding stock options were excluded from the calculation of diluted net loss per common share because a net loss was reported in each of these periods and therefore their effect was anti-dilutive. Potentially dilutive options to purchase common stock for both the three and nine months ended September 30, 2018, had exercise prices ranging from $0.79 to $4.64.

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

r. RECENTLY ISSUED ACCOUNTING STANDARDS. In February 2016, the FASB issued ASUNo. 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 also requires 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. The Company adopted the standard as of January 1, 2019, using the modified retrospective approach in which prior comparative periods are not adjusted. The Company elected the package of practical expedients permitted under the transition guidance within the new standard, which among other things, allows the Company to carry forward historical lease classification. The Company has operating leases for its office facilities, which expire on November 30, 2022. As of January 1, 2019, the Company recognized an additional right-of-use asset and corresponding operating lease liability related to its facility lease on the consolidated balance sheet. No cumulative effect adjustment was recognized as the amount was not material. The standard did not materially impact the Company's consolidated statement of operations or cash flows. See Note 5 for the financial position impact and additional disclosures.

In June 2018, the FASB issued ASUNo. 2018-07, *Compensation—Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting* that largely aligns the accounting for share-based payment awards issued to employees and nonemployees. Under this ASU, most of the guidance on such payments to nonemployees would be aligned with the requirements for share-based payments granted to employees. ASU 2018-07 is effective for all entities for annual reporting periods beginning after December 15, 2018, including interim reporting periods within each annual reporting period, with early adoption permitted. The Company has adopted this standard as of January 1, 2019. The adoption of this standard did not have a material impact on the Company's consolidated financial statements.

In June 2016, the FASB issued ASUNo. 2016-13, *Financial Instruments — Credit Losses (Topic 326), Measurement of Credit Losses on Financial Instruments.* The standard amends the impairment model by requiring entities to use a forward-looking approach based on expected losses to estimate credit losses for most financial assets and certain other instruments that aren't measured at fair value through net income. For available-for-sale debt securities, entities will be required to recognize an allowance for credit losses rather than a reduction in carrying value of the asset. Entities will no longer be permitted to consider the length of time that fair value has been less than amortized cost when evaluating when credit losses should be recognized. The Company plans to adopt the new standard on January 1, 2020. The Company does not anticipate the adoption will have a material impact on its consolidated financial position or results of operations.

s. RECLASSIFICATIONS. Certain prior year amounts in the consolidated financial statements have been reclassified to conform to the current year presentation.

3. Investments.

Available-for-sale investments by security type were as follows:

	Estimated Fair Value	Gross Unrealized Gains	Gross Unrealized Losses	Amortized Cost
At September 30, 2019:				
U.S. Treasuries - ST	$14,956,900	$ 9,794	$ —	$14,947,106
U.S. Treasuries - LT	5,008,800	9,634	—	4,999,166
Total	$19,965,700	$ 19,428	$ —	$19,946,272
At December 31, 2018:				
U.S. Treasuries - ST	$10,380,864	$ —	$ (1,835)	$10,382,699
U.S. Treasuries - LT	5,008,243	—	(18,413)	5,026,656
Total	$15,389,107	$ —	$ (20,248)	$15,409,355

There were no realized gains or losses fromavailable-for-sale securities for the three or nine months ended September 30, 2019 or 2018.

The Company did not hold any securities in an unrealized loss position for more than 12 months as of September 30, 2019.

The estimated fair values ofavailable-for-sale securities at September 30, 2019, by contractual maturity, are summarized as follows:

	September 30, 2019
Due in one year or less	$ 19,965,700
Due after one year	—
	$ 19,965,700

4. Prepaid Expenses and Other Current Assets.

Prepaid expenses and other current assets consist of the following:

	September 30, 2019	December 31, 2018
Prepaid research fees	$ 372,784	$ 358,209
Prepaid insurance	151,474	800,261
Prepaid commercialization fees	112,356	17,030
Prepaid subscription fees	537,587	170,552
Prepaid manufacturing	1,734,360	—
Other	430,838	303,729
Total prepaid expenses and other current assets	$ 3,339,399	$ 1,649,781

5. Operating Leases.

The Company has operating lease agreements for its corporate office. The leases include options to extend the leases for up to 1 year and options to terminate the lease within 1 year. There are no obligations under finance leases.

The components of lease expense were as follows:

	For the Three Months Ended September 30, 2019	For the Nine Months Ended September 30, 2019
Operating lease cost	$ 74,079	$ 222,237

Supplemental cash flow information related to leases was as follows:

	September 30, 2019
Cash paid for amounts included in the measurement of lease liabilities:	
Operating cash flows	$ 245,663
Right-of-use assets obtained in exchange for lease obligations:	
Operating leases	$ 15,915

Supplemental balance sheet information related to leases was as follows:

	September 30, 2019
Operating lease right-of-use assets	$ 952,340
Other current liabilities	$ 294,433
Operating lease liabilities, net of current portion	725,700
Total operating lease liabilities	$ 1,020,133
Weighted Average Remaining Lease Term	3.2 years
Weighted Average Discount Rate	4.81%

Payments of lease liabilities as of September 30, 2019 were as follows:

2019 (remaining three months)	$ 84,061
2020	339,605
2021	349,788
2022	329,662
Total lease payments	1,103,116
Less imputed interest	(82,983)
Total	$1,020,133

6. Property and Equipment, Net.

Property and equipment, net consists of the following:

	September 30, 2019	December 31, 2018
Computer equipment	$ 50,904	$ 52,704
Furniture and equipment	230,741	212,451
Leasehold improvements	—	177,417
	281,645	442,572
Less: Accumulated depreciation	(140,557)	(197,147)
Total property and equipment, net	$ 141,088	$ 245,425

Depreciation expense was $6,532 and $26,718, respectively, for the three and nine-month periods ended September 30, 2019 and $9,294 and $25,485, respectively for the three and nine-month periods ended September 30, 2018.

7. Accrued Expenses and Other Liabilities.

Accrued expenses and other liabilities consist of the following:

	September 30, 2019	December 31, 2018
Accrued preclinical and clinical trial expenses	$ 768,114	$ 821,633
Accrued professional fees	1,232,567	1,311,061
Accrued compensation and benefits	1,739,719	1,941,449
Accrued license fees	6,880,265	3,000,000
Operating lease liability	294,433	—
Accrued variable consideration	1,330,981	—
Deferred rent and lease incentive	—	33,408
Accrued income tax	1,058,039	—
Other	531,174	66,436
Current accrued expenses and other liabilities	13,835,292	7,173,987
Lease liability - non-current	725,700	—
Deferred rent and lease incentive – non-current	—	154,799
Non-current accrued expenses and other liabilities	725,700	154,799
Total accrued expenses and other liabilities	$ 14,560,992	$ 7,328,786

8. Collaborative Arrangement.

In December 2018, the Company entered into a collaboration and license agreement (collaboration) with Endo International plc's subsidiary, Endo Ventures Limited (Endo), for the further development and commercialization of generic Sabril® (vigabatrin) tablets through Endo's U.S. Generic Pharmaceuticals segment, doing business as Par Pharmaceutical.

Endo has assumed all development, manufacturing, clinical, regulatory, sales and marketing costs under the collaboration, while the Company is responsible for exercising commercially reasonable efforts to develop, or cause the development of, a final finished, stable dosage form of generic Sabril® tablets.

Under the terms of the Collaboration, the Company has received anup-front payment, and will receive milestone payments based on achievement of regulatory approvals, a sharing of defined net profits upon commercialization from Endo consisting of a mid-double digit percent of net sales of generic Sabril® and a sharing of certain development expenses. Unless terminated earlier in accordance with its terms, the collaboration continues in effect until the date that is ten years following the commercial launch. For the year ended December 31, 2018, a $500,000 upfront license fee was recognized.

For the three and nine-month periods ending September 30, 2019, no collaborative arrangement revenue has been received or recognized. Expenses incurred, net, in connection with the collaborative arrangement for the three and nine months ended September 30, 2019 was approximately $31,924 and $70,102, respectively, and have been included in research and development expenses in the accompanying consolidated statements of operations.

9. Commitments and Contingencies.

In 2018, the Company became aware that certain patents granted to Northwestern University (which patents have been licensed by Northwestern to a third party) for a new GABA aminotransferase inhibitor were developed from CPP-115, which had previously been licensed to the Company by Northwestern. As a result, on October 26, 2018, the Company terminated the license agreement for CPP-115 and commenced an arbitration proceeding against Northwestern seeking damages for alleged breaches of the license agreement. Shortly thereafter, Northwestern filed counterclaims against the Company in the arbitration action seeking damages for alleged breaches by the Company of the license agreement. On May 21, 2019, the Company entered into a settlement agreement with Northwestern that resolved all pending disputes between the parties with no admission of liability by either party, released all claims of liability or wrongdoing between the Company and Northwestern, and dismissed the pending arbitration. Under the settlement agreement, the Company received a $100,000 payment on May 21, 2019, which is reported as income in other income, net in the consolidated statement of operations. The Company is also entitled to receive certain contingent compensation that will be reported when and if received.

In May 2019, the FDA approved an NDA for Jacobus Pharmaceuticals for Ruzurgi®, their version of amifampridine (3,4-DAP), for the treatment of pediatric LEMS patients (ages 6 to under 17). The Company believes that Jacobus is offering Ruzurgi® at a lower price than the Company is offering Firdapse®. In addition, while the NDA for Ruzurgi® only covers pediatric patients, the Company believes Ruzurgi® is being prescribed off label to adult LEMS patients. If Jacobus is able to successfully sell Ruzurgi® off-label to adult LEMS patients, it could have a material adverse effect on the Company's business, financial condition and results of operations.

The Company believes that the FDA's approval of Ruzurgi® violated its statutory rights and was in multiple other respects arbitrary, capricious and contrary to law. As a result, in June 2019 the Company filed suit against the FDA and several related parties challenging this approval and related drug labeling. The Company's complaint, which was filed in the federal district court for the Southern District of Florida, alleges that the FDA's approval of Ruzurgi® violated multiple provisions of FDA regulations regarding labeling, resulting in misbranding in violation of the Federal Food, Drug, and Cosmetic Act (FDCA); violated its statutory rights to Orphan Drug Exclusivity and New Chemical Entity Exclusivity under the FDCA; and was in multiple other respects arbitrary, capricious, and contrary to law, in violation of the Administrative Procedure Act. Among other remedies, the suit seeks an order vacating the FDA's approval of Ruzurgi®. The FDA has answered the Company's complaint and the matter is currently in the discovery phase. There can be no assurance as to the outcome of this lawsuit.

Additionally, from time to time the Company may become involved in legal proceedings arising in the ordinary course of business. Except as set forth above, the Company believes that there is no other litigation pending at this time that could have, individually or in the aggregate, a material adverse effect on its results of operations, financial condition or cash flows.

10. Agreements.

a. **LICENSE AGREEMENT WITH BIOMARIN (FIRDAPSE®)**. On October 26, 2012, the Company entered into a license agreement with BioMarin Pharmaceutical, Inc. (BioMarin) for the North American rights to Firdapse®. Under the BioMarin license agreement, the Company pays: (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.

On May 29, 2019, the Company entered into an amendment to its License Agreement with BioMarin for Firdapse®. Under the amendment, the Company has expanded its commercial territory for Firdapse®, which originally was comprised of North America, to include Japan. Additionally, the Company has an option to further expand its territory under the license agreement to include most of Asia, as well as Central and South America, upon the achievement of certain milestones in Japan. Under the amendment, the Company will pay royalties on net sales in Japan of a similar percentage to the royalties that the Company is currently paying under its original license agreement for North America.

10. Agreements (continued).

b. AGREEMENTS FOR DRUG MANUFACTURING, DEVELOPMENT, PRECLINICAL AND CLINICAL STUDIES. The Company has entered into agreements with contract manufacturers for the manufacture of commercial drug and 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, most of these agreements are cancellable at anytime, but obligate the Company to reimburse the providers for any time or costs incurred through the date of termination.

11. Income Taxes.

The Company's effective income tax rate is the ratio of income tax expense (benefit) over its income (loss) before income taxes. The effective income tax rate was 4.18% and 0.00% for the nine months ended September 30, 2019 and 2018, respectively. Differences in the effective tax and the statutory Federal income tax rate of 21% is driven by state income taxes and anticipated annual permanent differences, including orphan drug credit expense limitations and other items.

The Company had no uncertain tax positions as of September 30, 2019 and December 31, 2018. The Company has a full valuation allowance at September 30, 2019 and December 31, 2018.

12. Stockholders' Equity.

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

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.

On November 28, 2017, the Company filed a prospectus supplement and offered for sale 16,428,572 shares of its common stock at a price of $3.50 per share in an underwritten public offering under the 2017 Shelf Registration. The Company received gross proceeds in the public offering of approximately $57.5 million before underwriting commission and incurred expenses of approximately $3.7 million.

At September 30, 2019, there is approximately $92.5 million available for future sale under the 2017 Shelf Registration Statement.

13. Stock Compensation.

For the three and nine-month periods ended September 30, 2019 and 2018, the Company recorded stock-based compensation expense as follows:

	Three months ended September 30,		Nine months ended September 30,	
	2019	2018	2019	2018
Research and development	$242,867	$240,122	$ 803,800	$ 820,062
Selling, general and administrative	574,193	518,054	1,871,667	1,700,961
Total stock-based compensation	$817,060	$758,176	$2,675,467	$2,521,023

13. Stock Compensation (continued).

Stock Options

As of September 30, 2019, there were outstanding stock options to purchase 10,135,334 shares of common stock, of which stock options to purchase 5,345,825 shares of common stock were exercisable as of September 30, 2019.

During the three and nine-month periods ended September 30, 2019, the Company granted seven-year term options to purchase an aggregate of 172,500 and 484,500 shares, respectively, of the Company's common stock to employees. The Company recorded stock-based compensation related to stock options totaling $817,060 and $2,675,467, respectively, during the three and nine-month periods ended September 30, 2019. During the three and nine-month periods ended September 30, 2019, respectively, 74,998 and 1,365,827 options vested.

During the three and nine-month periods ended September 30, 2018, the Company granted seven-year term options to purchase an aggregate of 550,000 and 3,267,500 shares, respectively, of the Company's common stock to employees and directors. The Company recorded stock-based compensation related to stock options totaling $758,176 and $2,506,026, respectively, during the three and nine-month periods ended September 30, 2018. During the three and nine-month periods ended September 30, 2018, respectively, 5,000 and 1,314,998 options vested.

During the three and nine-month periods ended September 30, 2019, options to purchase 108,332 shares and 298,332 shares, respectively, of the Company's common stock were exercised, with proceeds of $256,062 and $537,962, respectively, to the Company. During both the three and nine-month periods ended September 30, 2019, options to purchase 6,666 shares of the Company's common stock were exercised on a "cashless" basis, resulting in the issuance of an aggregate of 3,444 shares of the Company's common stock.

During the three and nine-month periods ended September 30, 2018, options to purchase 89,999 shares and 136,665 shares, respectively, of the Company's common stock were exercised, with proceeds of $104,532 and $146,064, respectively, to the Company.

As of September 30, 2019, there was approximately $6,400,076 of unrecognized compensation expense related tonon-vested stock option awards granted under the 2014 and 2018 Stock Incentive Plans. The cost is expected to be recognized over a weighted average period of approximately 2.0 years.

Common Stock

There were no grants of common stock to employees during the three and nine-month periods ended September 30, 2019. During both the three and nine-month periods ended September 30, 2018, the Company granted 3,094 net shares of common stock to employees as compensation. The Company recorded stock-based compensation related to common stock issued to employees totaling $0 and approximately $15,000, respectively, during the three and nine-month periods ended September 30, 2018.

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 the current status of 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 consolidated financial statements for the third quarter and first nine months of fiscal 2019.
- *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 consolidated financial statements that are included in this report.
- *Results of Operations.* This section provides an analysis of our results of operations for the three and nine-month periods ended September 30, 2019 as compared to the same periods ended September 30, 2018.
- *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, chronic neuromuscular and neurological diseases. We are dedicated to making a meaningful impact on the lives of those suffering from rare diseases, and we believe in putting patients first in everything we do.

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). When we acquired the rights to the product, it had already been granted orphan drug designation by the United States Food and Drug Administration (FDA) for the treatment of patients with Lambert-Eaton Myasthenic Syndrome (LEMS), a rare and sometimes fatal autoimmune disease characterized by muscle weakness. Additionally, in August 2013, we were granted "breakthrough therapy designation" by the FDA for Firdapse® for the treatment of LEMS. Further, the FDA has granted Orphan Drug Designation for Firdapse® for the treatment of Congenital Myasthenic Syndromes (CMS) and Myasthenia Gravis (MG).

On November 28, 2018, we received approval from the FDA for Firdapse® 10 mg tablets for the treatment of adults with LEMS (age 17 and above). In January 2019, we launched Firdapse® in the United States, selling through a field force experienced in neurologic, central nervous system or rare disease products consisting of approximately 20 field personnel, including sales (Regional Account Managers), patient assistance and insurance navigation support (Patient Access Liaisons), and payer reimbursement (National Account Managers) personnel. We also have a field-based force of six medical science liaisons who are helping educate the medical communities and patients about LEMS and about our ongoing clinical trial activities evaluating Firdapse® for other ultra-orphan, neuromuscular diseases. Finally, we are working with several rare disease advocacy organizations (including Global Genes, the National Organization for Rare Disorders (NORD), and the Myasthenia Gravis Foundation of America) to help increase awareness and level of support for patients living with LEMS, Anti-MuSK antibody positive myasthenia gravis, or MuSK-MG, CMS, and Spinal Muscular Atrophy (SMA) Type 3, and to provide education for the physicians who treat these rare diseases and the patients they treat.

We are supporting the distribution of Firdapse® through "Catalyst Pathways™," our personalized treatment support program. "Catalyst Pathways" is a single source for personalized treatment support, education and guidance through the challenging dosing and titration regimen to an effective therapeutic dose. It also includes distributing the drug through a very small group of exclusive specialty pharmacies (primarily AnovoRx), which is consistent with the way that most pharmaceutical products for ultra-orphan diseases are distributed and dispensed to patients. We believe that by using specialty pharmacies in this way, the difficult task of navigating the health care system is far better for the patient needing treatment for their rare disease and the health care community in general.

In order to help adult LEMS patients afford their medication, we, like other pharmaceutical companies which are marketing drugs for ultra-orphan conditions, have developed an array of financial assistance programs that are available to reduce patient co-pays and deductibles to a nominal affordable amount. For eligible patients with commercial coverage, a co-pay assistance program designed to keep out-of-pocket costs to $0.00 per month is available for all LEMS patients prescribed Firdapse®. We are also donating, and committing to continue to donate, money to qualified, independent charitable foundations dedicated to providing assistance to any U.S. LEMS patients in financial need. Subject to compliance with regulatory requirements, our goal is that no LEMS patient is ever denied access to Firdapse® for financial reasons.

In May 2019, the FDA approved an NDA for Jacobus Pharmaceuticals for Ruzurgi®, their version of amifampridine (3,4-DAP), for the treatment of pediatric LEMS patients (ages 6 to under 17). We believe that Jacobus is offering Ruzurgi® at a lower price than we are offering Firdapse®. In addition, while the NDA for Ruzurgi® only covers pediatric patients, we believe that Ruzurgi® is being prescribed off label to adult LEMS patients. If Jacobus is able to successfully sell Ruzurgi® off-label to adult LEMS patients, it could have a material adverse effect on our business, financial condition and results of operations.

We believe that the FDA's approval of Ruzurgi® violated our statutory rights and was in multiple other respects arbitrary, capricious and contrary to law. As a result, in June 2019 we filed suit against the FDA and several related parties challenging this approval and related drug labeling. Our complaint, which was filed in the federal district court for the Southern District of Florida, alleges that the FDA's approval of Ruzurgi® violated multiple provisions of FDA regulations regarding labeling, resulting in misbranding in violation of the Federal Food, Drug, and Cosmetic Act (FDCA); violated our statutory rights to Orphan Drug Exclusivity and New Chemical Entity Exclusivity under the FDCA; and was in multiple other respects arbitrary, capricious, and contrary to law, in violation of the Administrative Procedure Act. Among other remedies, the suit seeks an order vacating the FDA's approval of Ruzurgi®. The FDA has answered our complaint and the matter is currently in the discovery phase. There can be no assurance as to the outcome of this lawsuit.

We are currently conducting a Phase 3 clinical trial evaluating Firdapse® for the treatment of adults with MuSK-MG under a Special Protocol Assessment (SPA) with the FDA. The trial is a multi-site, international (United States and Italy), double-blind, placebo-controlled, clinical trial that is targeted to enroll approximately 60 subjects diagnosed with MuSK-MG. The trial will also enroll up to 10 generalized myasthenia gravis patients who will be assessed with the same clinical endpoints but achieving statistical significance in this subgroup of patients is not required and only summary statistics will be provided. While there can be no assurance, based on currently available information we expect to complete enrollment in this trial by the end of 2019 and to report top-line results from this trial in the first half of 2020. Details of this trial are available on www.clinicaltrials.gov (NCT03304054).

We previously conducted a Phase 3 clinical trial evaluating Firdapse® for the treatment of genetically confirmed CMS patients. Our trial was the first ever double-blind, placebo-controlled, clinical trial conducted in genetically confirmed CMS patients. In the trial, 20 subjects were enrolled and 16 randomized, in a 2 period, 2 treatment crossover study designed to evaluate the efficacy and safety of amifampridine phosphate in patients (aged 2 years and above) diagnosed with certain genetic subtypes of CMS. While individual patient improvements were observed, the trial did not meet its primary endpoint of subject global impression (SGI) or the secondary endpoint of muscle function measure (MFM) across all tested subtypes. Due to the rarity of CMS, this trial took almost 4 years to recruit.

We are currently scheduled to meet with the FDA before the end of the year to discuss the outcome of the CMS trial and potential paths forward to seek approval of amifampridine phosphate for the symptomatic treatment of some subset of genetic subtypes of CMS. After receiving such additional guidance, we will provide future updates on our plans for this indication.

There can be no assurance as to whether we will be permitted to submit an sNDA for this indication, or, even if we are permitted to submit such application, whether it will ever be approved (and, even if it is approved, for what genetic subtypes of CMS it will be approved).

Because the FDA has granted Orphan Drug Designation for Firdapse® for the treatment of patients with CMS and Myasthenia Gravis (MG), if we are the first to receive approvals in the future for Firdapse® for the treatment of CMS or MuSK-MG, of which there can be no assurance, we would be eligible to receive seven years of marketing exclusivity for those indications added to our Firdapse® labeling.

We are conducting a proof-of-concept clinical study evaluating Firdapse® as a symptomatic treatment for patients with Spinal Muscular Atrophy (SMA) Type 3, ambulatory. The study is designed as a randomized (1:1), double-blind, 2-period, 2-treatment, crossover, outpatient proof-of-concept study to evaluate the safety, tolerability and potential efficacy of amifampridine in ambulatory patients diagnosed with SMA Type 3. The study is planned to include approximately 12 patients, and we currently expect to report top-line results from this study in the first half of 2020. Details of this trial are available on www.clinicaltrials.gov (NCT03781479).

There can be no assurance that our clinical programs evaluating Firdapse® for the treatment of MuSK-MG, CMS, SMA Type 3, or any trials we may undertake in the future to evaluate Firdapse® for the treatment of other rare neuromuscular diseases, will be successful. Further, there can be no assurance that we will ever be granted the right to commercialize Firdapse® for any of these additional indications.

In October 2019, we submitted an NDS in Canada seeking approval of Firdapse® for the treatment of LEMS. There can be no assurance that our application will be accepted for review, and even if accepted for review, approved. We have also begun our efforts to develop a sustained release formulation for Firdapse®, which is part of a longer term strategy for the product. There can be no assurance that we will be successful in our efforts to develop a sustained release formulation of Firdapse.

On May 29, 2019, we entered into an amendment to our license agreement with BioMarin for Firdapse®. Under the amendment, we have expanded our commercial territory for Firdapse®, which originally was comprised of North America, to include Japan. Additionally, we have an option to further expand our territory under the license agreement to include most of Asia, as well as Central and South America, upon the achievement of certain milestones in Japan. Under the amendment, we will pay royalties on net sales in Japan of a similar percentage to the royalties that we are currently paying under our original license agreement for North America.

All of our patent rights for Firdapse® are derived from our license agreement with BioMarin. Under the BioMarin License Agreement, we licensed two pending patents and certain trademarks for Firdapse®. One of the licensed applications, U.S. App. No. 10/467,082 is abandoned as are its children (U.S. App. No. 14/085,017 and 14/818,848) such that we are no longer pursuing patent protection out of this family of applications. The second licensed patent application claims methods of administering Firdapse®. We recently received an office action from the United States Patent and Trademark Office responding to our second application, and we are in the process of responding to that office action. There can be no assurance that our pending patent will be granted or as to the protection from competition that it will provide us if it is granted.

Further, there can be no assurance that we do not or will not infringe on patents held by third parties or that third parties in the future will not claim that we have infringed on their patents. In the event that our products or technologies infringe or violate the patent or other proprietary rights of third parties, we may be prevented from pursuing product development, manufacturing or commercialization of our products that utilize such technologies. For example, there may be patents or patent applications held by others that contain claims that our products or operations might be determined to infringe or that may be broader than we believe them to be. Given the complexities and uncertainties of patent laws, there can be no assurance as to the impact that future patent claims against us may have on our business, financial condition, results of operations, or prospects.

Generic Sabril®

In September 2015, we announced the initiation of a project to develop generic versions of Sabril® (vigabatrin). Sabril® is marketed by Lundbeck Inc. in the United States in two dosage forms (powder sachets and tablets) for the treatment of infantile spasms and refractory complex partial seizures. Par Pharmaceutical brought the first generic version of the powder sachet to market, and, to date, several generic versions of the powder sachets have been approved. However, at this time, there is only one approved generic version of the tablets.

On December 18, 2018, we entered into a definitive agreement with Endo International plc's subsidiary, Endo Ventures Limited ("Endo"), for the further development and commercialization of generic Sabril® tablets through Endo's United States Generic Pharmaceuticals segment, Par Pharmaceutical. Pursuant to the agreement, we have received an up-front payment of $500,000, and we will receive milestone payments based on achievement of regulatory approvals, and a sharing of defined net profits upon commercialization and certain expenses for development.

There can be no assurance that our collaboration with Endo for the development of generic Sabril® (vigabatrin) tablets will be successful and that if an abbreviated new drug application (ANDA) is approved for vigabatrin tablets in the future, that it will be profitable to us.

Available Capital Resources

At September 30, 2019, we had cash and investments of approximately $81.6 million. Based on our current financial condition and forecasts of available cash, we believe that we have sufficient funds to support our operations for at least the next 12 months from the date of this report. There can be no assurance that we will remain profitable, or as to whether we will require additional funding in the future (and whether any such required funding will be available). See "Liquidity and Capital Resources" below for further information on our liquidity and cash flow.

Basis of Presentation

Revenues.

Prior to the launch of Firdapse® in January 2019 we did not generate revenues for product sales. In the periods ended September 30, 2019 we have generated revenues from product sales of Firdapse®. We expect these revenues to fluctuate in future periods based on our sales of Firdapse®. At September 30, 2018 we were a development stage company, as we had no revenues from product sales.

Cost of Sales.

Cost of sales consists of third-party manufacturing costs, freight, royalties, and indirect overhead costs associated with sales of Firdapse®. Cost of sales may also include period costs related to certain inventory manufacturing services, inventory adjustments charges, unabsorbed manufacturing and overhead costs, and manufacturing variances. Prior to FDA approval in November 2018, the cost of manufacturing Firdapse® was expensed, including our build-up of anticipated launch product. This will cause the cost of sales to appear artificially low for product manufactured prior to approval, until we deplete such product and additional product is manufactured.

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, 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 Firdapse®, CPP-109 (our version of vigabatrin), and formerly CPP-115, and we currently expect that our future development costs will be attributable principally to the continued development of Firdapse®.

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 consolidated 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 they begin, and incurring additional expenditures as the study or trial progresses and reaches certain milestones.

Selling, General and Administrative Expenses.

During the first nine months of 2019, we actively committed funds to developing our commercialization program for Firdapse® and have continued to incur commercialization expenses, inclusive of sales, marketing and other commercialization related expenses as we have begun our sales program for Firdapse®. We had no product sales or selling expenses in the first nine months of 2018.

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, cost for preparation for commercialization, 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, 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.

Income Taxes.

Our effective income tax rate is the ratio of income tax expense (benefit) over our income (loss) before income taxes.

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 consolidated financial statements included in this report.

Critical Accounting Policies and Estimates

Our discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with U.S. GAAP. The preparation of these consolidated 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 2018 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, 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 2018 Annual Report on Form 10-K other than accounting policies for revenue recognition and leases as discussed in Note 2, "Basis of Presentation and Significant Accounting Policies," in the interim consolidated financial statements included in this report.

Results of Operations

Revenues.

For the three and nine-month periods ended September 30, 2019, we recognized $30.9 million and $72.2 million, respectively, in net revenue from product sales of Firdapse®. We had no revenues from product sales for the three and nine-month periods ended September 30, 2018.

Cost of Sales.

Cost of sales was $4.4 million and $10.4 million for the three and nine-months ended September 30, 2019, respectively, compared to $0 for both the three and nine-months ended September 30, 2018. The increase in cost of sales was entirely attributable to the commercial launch of Firdapse® in January 2019. Cost of sales includes royalty payments which are based on net revenue as defined in the applicable license agreement. Further, cost of sales may be artificially low until we fully utilize product manufactured prior to approval.

Research and Development Expenses.

Research and development expenses for the three-month periods ended September 30, 2019 and 2018 were approximately $4.6 million and $4.5 million, respectively, and represented approximately 27% and 55% of total operating costs and expenses for the three-month periods ended September 30, 2019, and 2018 respectively. Research and development expenses for the three-months ended September 30, 2019 and 2018 were as follows:

	Three months ended September 30,		Change	
	2019	2018	$	%
Research and development expenses	$ 4,354,172	$ 4,298,247	55,925	1.3%
Employee stock-based compensation	242,867	240,122	2,745	1.1%
Total research and development expenses	$ 4,597,039	$ 4,538,369	58,670	1.3%

For the three months ended September 30, 2019, research and development were in-line with those in the same period in 2018.

Research and development expenses for the nine-month periods ended September 30, 2019 and 2018 were approximately $12.5 million and $11.5 million, respectively, and represented approximately 26% and 56% of total operating costs and expenses for the nine-month periods ended September 30, 2019, and 2018 respectively. Research and development expenses for the nine-months ended September 30, 2019 and 2018 were as follows:

	Nine months ended September 30,		Change	
	2019	2018	$	%
Research and development expenses	$11,730,562	$10,682,173	1,048,389	9.8%
Employee stock-based compensation	803,800	820,062	(16,262)	(2.0%)
Total research and development expenses	$12,534,362	$11,502,235	1,032,127	9.0%

For the nine months ended September 30, 2019, research and development expenses increased $1,032,127, respectively, compared to the same period in 2018, primarily attributable to increases in medical and regulatory affairs and quality assurance expenses to support our commercial launch of Firdapse® and ongoing clinical trials evaluating Firdapse® for the treatment of other ultra-orphan, neuromuscular diseases; partially offset by a decrease in milestone payments made as part of the settlement agreement with Huxley stockholders during the third quarter of 2018 and a decrease in consulting expenses related to the NDA submission in 2018.

We expect that research and development expenses will continue to be substantial during the balance of 2019 and into 2020 as we continue our clinical program evaluating Firdapse® for the treatment of MuSK-MG, continue our CMS clinical trial closeout, continue our proof-of-concept trial for SMA Type 3, continue our Expanded Access Program, take steps to develop a sustained release formulation of Firdapse®, and potentially prepare an sNDA for Firdapse® for the treatment of MuSK-MG and/or CMS.

Selling, General and Administrative Expenses.

Selling, general and administrative expenses for the three months ended September 30, 2019 and 2018 were approximately $8.1 million and $3.6 million, respectively, and represented 47% and 45% of total operating costs and expenses for the three months ended September 30, 2019 and 2018, respectively. Selling, general and administrative expenses for the three months ended September 30, 2019 and 2018 were as follows:

	Three months ended September 30,		Change	
	2019	2018	$	%
Selling	$4,670,568	$ —	4,670,568	0%
General and administrative	2,823,031	3,126,180	(303,149)	(9.7%)
Employee stock-based compensation	574,193	518,054	56,139	10.8%
Total selling, general and administrative expenses	$8,067,792	$3,644,234	4,423,558	121.4%

Selling, general and administrative expenses for the nine months ended September 30, 2019 and 2018 were approximately $25.5 million and $8.9 million, respectively, and represented 53% and 44% of total operating costs and expenses for the nine months ended September 30, 2019 and 2018, respectively. Selling, general and administrative expenses for the nine months ended September 30, 2019 and 2018 were as follows:

	Nine months ended September 30,		Change	
	2019	2018	$	%
Selling	$14,655,613	$ —	14,655,613	0%
General and administrative	8,944,694	7,248,702	1,695,992	23.4%
Employee stock-based compensation	1,871,667	1,700,961	170,706	10.0%
Total selling, general and administrative expenses	$25,471,974	$8,949,663	16,522,311	184.6%

For the three and nine months ended September 30, 2019, selling, general and administrative expenses increased approximately $4.4 million and $16.5 million, respectively, compared to the same period in 2018, primarily attributable to the following:

- increases in selling (commercialization) expenses, which consist primarily of commercial systems implementation costs, hiring of the sales force and supporting personnel, product launch costs, and costs of our market access and market research efforts (pre-commercial expenses incurred in 2018 before we began preparing our commercial program for Firdapse® were included in general and administrative expenses);
- increases in general and administrative expenses for the nine month period are primarily due to our efforts to expand our operations and headcount in connection with the commercialization of Firdapse®, donations to qualified, independent charitable foundations dedicated to providing assistance to any U.S. LEMS patients in financial need, and professional fees associated with our suit against the FDA, partially offset by a decrease in pre-commercialization expenses, recorded in general and administrative expenses in 2018; and
- increases in employee stock-based compensation which is non cash and relates to the expense of stock options awarded to certain employees, officers and directors.

We expect that selling, general and administrative costs, including commercialization costs, will continue to increase during the balance of 2019 and into 2020 as we continue to expand our sales activities for Firdapse®, continue to build-up our infrastructure to support our operations, and pursue our lawsuit against the FDA.

Stock-Based Compensation.

Total stock-based compensation for the three and nine-month periods ended September 30, 2019 were $817,060 and $2,675,467, respectively, and for the three and nine-month periods ended September 30, 2018 were $758,176 and $2,521,023, respectively. The increase in stock-based compensation for the three and nine-month periods ended September 30, 2019, when compared to the same period in 2018, is primarily due to the expense of grants to new employees hired in connection with the launch of Firdapse®.

Other Income, Net.

We reported other income, net in all periods primarily relating to our investment of funds received from offerings of our securities and product sales. The increase in other income, net for the three and nine months ended September 30, 2019 when compared to the same period in 2018 is primarily due to higher invested balances and higher yields on investments. Other income, net, consists of interest income, dividend income, and unrealized and realized gain (loss) on trading securities. For the nine month period ended September 30, 2019, other income, net also includes $100,000 received as part of a settlement agreement between us and Northwestern. These proceeds are used to fund our drug development activities and our operations.

Income Taxes.

Our effective income tax rate was 4.18% and 0.00% for the nine months ended September 30, 2019 and 2018, respectively. Differences in the effective tax and the statutory federal income tax rate of 21% is driven by state income taxes and anticipated annual permanent differences, including orphan drug credit expense limitations and other items.

We had no uncertain tax positions as of September 30, 2019 and December 31, 2018. We have a full valuation allowance at September 30, 2019 and December 31, 2018.

Net Income (Loss).

Our net income was $13,630,179 and $23,945,624, respectively, for the three and nine-months ended September 30, 2019 ($0.13 and $0.23, respectively, per basic and diluted share), as compared to a net loss of ($7,838,873) and ($19,503,905), respectively, for the three and nine months ended September 30, 2018 (($0.08) and ($0.19), respectively, per basic and diluted share).

Liquidity and Capital Resources

Since our inception, we have financed our operations primarily through multiple public and private offerings of our securities. However, during January 2019, we launched our initial product, Firdapse®, and began to receive funds from product sales. At September 30, 2019, we had cash and cash equivalents and investments aggregating $81.6 million and working capital of $72.6 million. At December 31, 2018, we had cash and cash equivalents and short-term investments aggregating $58.5 million and working capital of $45.7 million. At September 30, 2019, substantially all of our cash and cash equivalents were deposited with one financial institution, and such balances were in excess of federally insured limits. Further, as of such date, substantially all such funds were invested in short-term interest-bearing obligations, a short-term bond fund, and U.S. Treasuries.

We incurred operating losses through the quarter ended March 31, 2019 and reported operating income for the first time during the three and six month periods ended June 30, 2019. We expect to continue to spend substantial dollars on our current and future drug development programs.

Based on forecasts of available cash, we believe that we have sufficient resources to support our currently anticipated operations for at least the next 12 months from the date of this report. There can be no assurance that we will remain profitable or that we will be able to obtain any additional funding that we may require in the future.

We may also require additional working capital to support our operations depending on our future success with Firdaps® sales and whether our results continue to be cash flow positive. There can be no assurance as to the amount of any such funding that will be required for these purposes or whether any such funding will be available to us when it is required.

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

- the scope, rate of progress and cost of our clinical trials and other product development activities;
- future clinical trial results;
- the terms and timing of any collaborative, licensing and other arrangements that we may establish;
- the cost and timing of regulatory approvals;
- the cost and delays in product development as a result of any changes in regulatory oversight applicable to our products;
- the cost and timing of establishing sales, marketing and distribution capabilities;
- the level of revenues that we report from sales of Firdapse®;
- 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 that we may require in the future 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 further 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 shelf registration statement with the SEC to sell up to $150 million of common stock, preferred stock, warrants to purchase common stock, debt securities and units consisting of one or more of such securities (the “2017 Shelf Registration Statement”). The 2017 Shelf Registration Statement (file no. 333-219259) was declared effective by the SEC on July 26, 2017. We have completed one offering under the 2017 Shelf Registration Statement, raising net proceeds of approximately $53.8 million from the sale of 16,428,572 shares of our common stock on November 28, 2017.

On December 23, 2016, we filed a shelf registration statement with the SEC to sell up to $33.8 million of common stock (the "2016 Shelf Registration Statement"). This shelf registration statement was declared effective by the SEC on January 9, 2017. We have made no sales under the 2016 Shelf Registration Statement.

As of the date of this Form 10-Q, the full amount of our 2016 Shelf Registration Statement and $92.5 million of our 2017 Shelf Registration Statement remains available for future sales.

Cash Flows.

Net cash provided by (used in) operating activities was $22,320,032 and ($17,795,958), respectively, for the nine-month periods ended September 30, 2019 and 2018. During the nine months ended September 30, 2019, net cash provided by operating activities was primarily attributable to our net income of $23,945,624, increases of $1,809,662 in accounts payable, $6,400,279 in accrued expenses and other liabilities, and of $2,697,950 of non-cash expenses. This was partially offset by increases of $10,095,352 in accounts receivable, net, $543,789 in inventory, and $1,689,618 in prepaid expenses and other current and non-current assets and a decrease of $204,724 in operating lease liability. During the nine months ended September 30, 2018, net cash used in operating activities was primarily attributable to our net loss of $19,503,905, and decreases of $604,591 in accounts payable, and $185,812 in accrued expenses and other liabilities. This was partially offset by a $356,924 decrease in prepaid expenses and other current assets, and $2,141,426 of non-cash expenses. Such additional non-cash expenses consist of depreciation, change in right-of-use asset, stock-based compensation expense, and change in accrued interest and accretion of discount on investments.

Net cash provided by investing activities was $5,565,824 for the nine-month period ended September 30, 2019, consisting primarily of sales/maturities of investments of $40,310,595 and partially offset by purchases of investments of $34,725,401. Net cash used in investing activities was $29,226,047, for the nine-month period ended September 30, 2018, consisting primarily of purchases of investments of $36,790,854 and partially offset by sales/maturities of investments of $7,600,000.

Net cash provided by financing activities during the nine-month periods ended September 30, 2019 and 2018 was $537,962 and $141,616, respectively, consisting primarily of proceeds from the exercise of options to purchase common stock.

Contractual Obligations.

We have entered into the following contractual arrangements:

- *Payments to BioMarin and others under our license agreement with BioMarin.* Under our license agreement with BioMarin 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. For the three and nine-months ended September 30, 2019, we recognized approximately $4.1 million and $9.6 million, respectively, of royalties, which is included in cost of sales in the accompanying consolidated statement of operations.
- *Purchase commitments.* We have entered into purchase commitments with our contract manufacturing organizations aggregating approximately $950,000 per year. The agreements expire on various dates through 2024.
- *Employment agreements.* We have entered into an employment agreement with our Chief Executive Officer that requires us to make base salary payments of approximately $546,000 in 2019. The agreement expires in November 2020.
- *Lease for office space.* We operate our business in leased office space in Coral Gables, Florida. We currently lease approximately 7,800 square feet of office space for which we pay annual rent of approximately $330,000.

Off-Balance Sheet Arrangements.

We currently have no debt or finance 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, including the uncertainty of:

- The effect on our business and future results of operations arising from the recent approval by the FDA of an NDA for Jacobus Pharmaceuticals for their version of 3,4-DAP for the treatment of pediatric LEMS patients (ages 6 to under 17);
- whether our suit against the FDA seeking to vacate the FDA’s approval of Ruzurgi® will be successful;
- if the approval of Ruzurgi® is not overturned, whether we can successfully compete for adult LEMS patients;
- our estimates regarding anticipated capital requirements and our needs for additional financing in the future;
- the impact on Firdapse® of adverse changes in potential reimbursement and coverage policies from government and private payors such as Medicare, Medicaid, insurance companies, health maintenance organizations and other plan administrators, or the impact of pricing pressures enacted by industry organizations, the federal government or the government of any state, including as a result of increased scrutiny over pharmaceutical pricing or otherwise;
- the impact on our business and results of operations of public statements by Senator Bernie Sanders and a vocal group of LEMS patients and doctors who object to our pricing of Firdapse®;
- whether we will be able to successfully market Firdapse® while maintaining full compliance with applicable federal and state laws, rules and regulations;
- whether our estimates of the size of the market for our drug candidates will turn out to be accurate;
- whether we will be able to locate LEMS patients who are undiagnosed or are misdiagnosed with other diseases;
- whether our efforts to commercialize Firdapse® will be successful and, even if they are successful, whether we will remain profitable;
- whether payors will reimburse for our product;
- whether payors will, because of the lower prices of Ruzurgi®, require that patients try off-label Ruzurgi® first before they will approve Firdapse® as a treatment for adult LEMS patients;
- changes in the healthcare industry and the effect of political pressure from President Trump, Congress and/or medical professionals seeking to reduce prescription drug costs;
- changes to 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;
- 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;
- whether the trials that we are currently undertaking to evaluate Firdapse® for the treatment of Anti-MuSK antibody positive myasthenia gravis (MuSK-MG), and Spinal Muscular Atrophy (SMA) Type 3, or any other trials that we undertake in the future, will be successful;
- whether the FDA will permit us to submit a sNDA for CMS orMuSK-MG and whether any such application will be accepted for filing (and even if accepted, whether such application will be approved);
- whether we will be the first company to receive approval of3,4-DAP for the treatment of CMS;
- whether our NDS filing in Canada for Firdapse® will be accepted for filing, and even if it is accepted for filing, whether it will be approved;
- whether Firdapse® will ever be approved for the treatment ofMuSK-MG, CMS, SMA Type 3, or any other neuromuscular disease;
- whether our version of generic vigabatrin tablets will ever be approved by the United States Food and Drug Administration (FDA);
- even if vigabatrin tablets are approved for commercialization, whether Endo Ventures/Par Pharmaceutical will be successful in marketing the product;
- whether Catalyst will earn milestone payments on approval of an Abbreviated New Drug Application (ANDA) for generic vigabatrin tablets and royalties on sales of generic vigabatrin tablets;

- the ability of our third-party suppliers and contract manufacturers to maintain compliance with current Good Manufacturing Practices (cGMP);
- the ability of our distributor and the specialty pharmacies that distribute our product to maintain compliance with applicable law; and
- our ability to maintain compliance with applicable rules relating to our patient assistance programs and our contributions to 501(c)(3) organizations that support LEMS patients.

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

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURE ABOUT MARKET RISK

Not applicable.

ITEM 4. CONTROLS AND PROCEDURES

a. We have carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Based on such evaluation, our principal executive officer and principal financial officer have concluded that as of September 30, 2019, our disclosure controls and procedures were effective to ensure that the information required to be disclosed by us in the reports filed or submitted by us under the Exchange Act, was recorded, processed, summarized or reported within the time periods specified in the rules and regulations of the SEC, and include controls and procedures designed to ensure that information required to be disclosed by us in such reports was accumulated and communicated to management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosures.

b. During the three months ended September 30, 2019, 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 other than controls that were added as a result of the product launch of Firdapse®.

PART II. OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

In 2018, we became aware that certain patents granted to Northwestern University (which patents have been licensed by Northwestern to a third party) for a new GABA aminotransferase inhibitor were developed from CPP-115, which had previously been licensed to us by Northwestern. As a result, on October 26, 2018, we terminated the license agreement for CPP-115 and commenced an arbitration proceeding against Northwestern seeking damages for alleged breaches of the license agreement. Shortly thereafter, Northwestern filed counterclaims against us in the arbitration action seeking damages for alleged breaches by us of the license agreement. On May 21, 2019, we entered into a settlement agreement with Northwestern that resolved all pending disputes between the parties with no admission of liability by either party, released all claims of liability or wrongdoing between us and Northwestern, and dismissed the pending arbitration. Under the settlement agreement, we received $100,000 at the time the settlement agreement was executed and we will also be entitled to receive certain contingent compensation that will be reported when and if it is received.

In May 2019, the FDA approved an NDA for Jacobus Pharmaceuticals for Ruzurgi®, their version of amifampridine (3,4-DAP), for the treatment of pediatric LEMS patients (ages 6 to under 17). We believe that Jacobus is offering Ruzurgi® at a lower price than we are offering Firdapse®. In addition, while the NDA for Ruzurgi® only covers pediatric patients, we believe that Ruzurgi® is being prescribed off-label to adult LEMS patients. If Jacobus is able to successfully sell Ruzurgi® off-label to adult LEMS patients, it could have a material adverse effect on our business, financial condition and results of operations.

We believe that the FDA's approval of Ruzurgi® violated our statutory rights and was in multiple other respects arbitrary, capricious and contrary to law. As a result, in June 2019 we filed suit against the FDA and several related parties challenging this approval and related drug labeling. Our complaint, which was filed in the federal district court for the Southern District of Florida, alleges that the FDA's approval of Ruzurgi® violated multiple provisions of FDA regulations regarding labeling, resulting in misbranding in violation of the Federal Food, Drug, and Cosmetic Act (FDCA); violated our statutory rights to Orphan Drug Exclusivity and New Chemical Entity Exclusivity under the FDCA; and was in multiple other respects arbitrary, capricious, and contrary to law, in violation of the Administrative Procedure Act. Among other remedies, the suit seeks an order vacating the FDA's approval of Ruzurgi®. The FDA has answered our complaint and the matter is currently in the discovery phase. There can be no assurance as to the outcome of this lawsuit.

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

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 2018 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.

The following is an additional risk factor regarding factors that you should consider that has recently arisen:

The FDA has approved Ruzurgi®, another formulation of amifampridine (3,4-DAP) for LEMS for the treatment of pediatric patients.

In May 2019, we became aware that Jacobus Pharmaceutical had been granted approval of an NDA for Ruzurgi®, their version of amifampridine (3,4-DAP) for pediatric LEMS patients (ages 6 to under 17). We believe that Jacobus is offering Ruzurgi® at a lower price than we are offering Firdapse®. In addition, while the NDA for Ruzurgi® only covers pediatric patients, we believe Ruzurgi® is being prescribed off-label in adult patients. While we believe that this approval was violative of our statutory rights and was in multiple other respects arbitrary, capricious, and contrary to law, and we have filed suit to that effect, there can be no assurance that our claims will be successful. If Jacobus is able to successfully sell Ruzurgi® off-label for adult LEMS patients, it could have a material adverse effect on our business, financial condition and results of operations.

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: November 12, 2019

Exhibit 31.1

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 Rules13a-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, including its consolidated subsidiary, 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: November 12, 2019

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

Exhibit 31.2

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 Rules13a-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, including its consolidated subsidiary, 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: November 12, 2019

/s/ Alicia Grande
Alicia Grande
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

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 September 30, 2019 (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: November 12, 2019

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

Exhibit 32.2

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 September 30, 2019 (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: November 12, 2019

/s/ Alicia Grande

Alicia Grande
Chief Financial Officer
(Principal Financial Officer)