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 June 30, 2020

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	**33134**
(Address of principal executive offices)	**(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	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, a non-accelerated filer, a smaller reporting company or an emerging growth company. See definitions of "accelerated filer", "large accelerated filer", "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act:

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 103,463,460 shares of common stock, $0.001 par value per share, were outstanding as of August 6, 2020.

CATALYST PHARMACEUTICALS, INC.

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PART I. FINANCIAL INFORMATION

PART II. OTHER INFORMATION

CATALYST PHARMACEUTICALS, INC.
CONSOLIDATED BALANCE SHEETS

	June 30, 2020 (unaudited)	December 31, 2019
ASSETS		
Current Assets:		
Cash and cash equivalents	$ 115,052,248	$ 89,511,710
Short-term investments	—	5,007,050
Accounts receivable, net	6,762,262	10,536,997
Inventory	1,827,924	1,956,792
Prepaid expenses and other current assets	7,521,253	4,351,074
Total current assets	131,163,687	111,363,623
Operating lease right-of-use asset	71,711	793,252
Property and equipment, net	167,514	210,467
Deposits	8,888	8,888
Total assets	$ 131,411,800	$ 112,376,230
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current Liabilities:		
Accounts payable	$ 5,804,778	$ 4,117,447
Accrued expenses and other liabilities	14,405,597	19,981,295
Total current liabilities	20,210,375	24,098,742
Operating lease liability, net of current portion	—	647,532
Total liabilities	20,210,375	24,746,274
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, $0.001 par value, 5,000,000 shares authorized: none issued and outstanding at June 30, 2020 and December 31, 2019	—	—
Common stock, $0.001 par value, 150,000,000 shares authorized; 103,422,032 shares and 103,397,033 shares issued and outstanding at June 30, 2020 and December 31, 2019, respectively	103,422	103,397
Additional paid-in capital	219,581,816	216,205,678
Accumulated deficit	(108,482,622)	(128,688,624)
Accumulated other comprehensive income (loss)	(1,191)	9,505
Total stockholders' equity	111,201,425	87,629,956
Total liabilities and stockholders' equity	$ 131,411,800	$ 112,376,230

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 June 30,		For the Six Months Ended June 30,	
	2020	2019	2020	2019
Product revenue, net	$ 29,604,764	$ 28,837,900	$ 58,741,236	$ 41,286,338
Operating costs and expenses:				
Cost of sales	4,139,873	4,261,625	8,290,739	5,973,413
Research and development	4,349,643	4,629,364	8,572,454	7,937,323
Selling, general and administrative	10,833,358	8,987,722	20,896,406	17,404,182
Total operating costs and expenses	19,322,874	17,878,711	37,759,599	31,314,918
Operating income (loss)	10,281,890	10,959,189	20,981,637	9,971,420
Other income, net	111,269	450,410	447,502	793,676
Net income (loss) before income taxes	10,393,159	11,409,599	21,429,139	10,765,096
Provision for income taxes	613,172	449,651	1,223,137	449,651
Net income (loss)	$ 9,779,987	$ 10,959,948	$ 20,206,002	$ 10,315,445
Net income (loss) per share:				
Basic	$ 0.09	$ 0.11	$ 0.20	$ 0.10
Diluted	$ 0.09	$ 0.10	$ 0.19	$ 0.10
Weighted average shares outstanding:				
Basic	103,414,523	102,869,202	103,410,881	102,808,897
Diluted	106,730,423	105,928,970	106,433,862	105,098,930
Net income (loss)	$ 9,779,987	$ 10,959,948	$ 20,206,002	$ 10,315,445
Other comprehensive income (loss):				
Unrealized gain (loss) on available-for-sale securities	(84,942)	28,446	(10,696)	42,006
Comprehensive income (loss)	$ 9,695,045	$ 10,988,394	$ 20,195,306	$ 10,357,451

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 six months ended June 30, 2020 and 2019

	Preferred Stock	Common Stock		Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Gain (Loss)	Total
		Shares	Amount				
Balance at December 31, 2019	$ —	103,397,033	$103,397	$216,205,678	$(128,688,624)	$ 9,505	$ 87,629,956
Issuance of stock options for services	—	—	—	1,383,672	—	—	1,383,672
Exercise of stock options for common stock	—	11,666	12	26,137	—	—	26,149
Amortization of restricted stock for services	—	—	—	135,679	—	—	135,679
Other comprehensive gain (loss)	—	—	—	—	—	74,246	74,246
Net income (loss)	—	—	—	—	10,426,015	—	10,426,015
Balance at March 31, 2020	—	103,408,699	103,409	217,751,166	(118,262,609)	83,751	99,675,717
Issuance of stock options for services	—	—	—	1,627,105	—	—	1,627,105
Exercise of stock options for common stock	—	13,333	13	36,188	—	—	36,201
Amortization of restricted stock for services	—	—	—	167,357	—	—	167,357
Other comprehensive gain (loss)	—	—	—	—	—	(84,942)	(84,942)
Net income (loss)	—	—	—	—	9,779,987	—	9,779,987
Balance at June 30, 2020	$ —	103,422,032	$103,422	$219,581,816	$(108,482,622)	$ (1,191)	$111,201,425

	Preferred Stock	Common Stock		Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Gain (Loss)	Total
		Shares	Amount				
Balance at December 31, 2018	$ —	102,739,257	$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	—	65,000	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	—	102,804,257	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	—	125,000	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	$ —	102,929,257	$102,929	$213,405,396	$(150,248,516)	$ 21,758	$63,281,567

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

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

	For the Six Months Ended June 30,	
	2020	**2019**
Operating Activities:		
Net income (loss)	$ 20,206,002	$ 10,315,445
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities:		
Depreciation	42,953	20,186
Amortization of right-of-use asset	721,541	120,051
Stock-based compensation	3,313,813	1,858,407
Change in accrued interest and accretion of discount on investments	(3,646)	(77,547)
(Increase) decrease in:		
Accounts receivable, net	3,774,735	(10,376,427)
Inventory	128,868	(213,867)
Prepaid expenses and other current assets and deposits	(3,170,179)	161,178
Increase (decrease) in:		
Accounts payable	1,687,331	991,359
Accrued expenses and other liabilities	(5,473,075)	3,393,753
Operating lease liability	(750,155)	(135,123)
Net cash provided by (used in) operating activities	20,478,188	6,057,415
Investing Activities:		
Purchases of property and equipment	—	(19,370)
Purchases of investments	—	(29,772,428)
Proceeds from maturities and sales of investments	5,000,000	30,310,595
Net cash provided by (used in) investing activities	5,000,000	518,797
Financing Activities:		
Proceeds from exercise of stock options	62,350	281,900
Net cash provided by (used in) financing activities	62,350	281,900
Net increase (decrease) in cash and cash equivalents	25,540,538	6,858,112
Cash and cash equivalents—beginning of period	89,511,710	16,559,400
Cash and cash equivalents—end of period	$115,052,248	$ 23,417,512
Supplemental disclosures of cash flow information:		
Cash paid for income taxes	$ 49,500	$ —
Non-cash investing and financing activities:		
Unrealized gain (loss) on available-for-sale securities	$ (10,696)	$ 42,006

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

1. Organization and Description of Business.

Catalyst Pharmaceuticals, Inc. and subsidiary (collectively, the "Company") is a commercial-stage biopharmaceutical company focused on developing and commercializing innovative 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), 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 (ages 17 and above). On January 15, 2019, the Company launched its first product, Firdapse®, in the United States for the treatment of adults with LEMS.

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, and started reporting operating income during the year ended December 31, 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 product sales. See Note 11 (Stockholders' Equity).

Capital Resources

While there can be no assurance, based on currently available information, the Company estimates that it currently 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.

Risks and Uncertainties

There are many uncertainties regarding the novel coronavirus (COVID-19) pandemic, and the Company is closely monitoring the impact of the pandemic on all aspects of its business, including how the pandemic is impacting its patients, employees, suppliers, vendors, business partners, clinical trials, and distribution channels. The Company is unable to predict the impact that COVID-19 will have on its financial position and operating results in future periods due to numerous uncertainties. The Company will continue to assess the evolving impact of the COVID-19 pandemic and make adjustments to its operations as necessary.

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, 2019 included in this Form 10-Q was derived from the audited financial statements and does not include all disclosures required by U.S. GAAP.

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

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, 2019 included in the 2019 Annual Report on Form 10-K filed by the Company with the SEC. The results of operations for the six months ended June 30, 2020 are not necessarily indicative of the results to be expected for any future period or for the full 2020 fiscal year.

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 and U.S Treasuries. The Company has substantially all of its cash and cash equivalents deposited with one financial institution. These amounts at times may exceed federally insured limits.

e. **INVESTMENTS**. The Company invests in high credit-quality instruments in order to obtain higher yields on its cash available for investments. At June 30, 2020 and December 31, 2019, investments consisted of U.S. Treasuries. Such investments are not insured by the Federal Deposit Insurance Corporation.

Short-Term Bond Fund

The Company previously owned a short-term bond fund that was classified as 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 June 30, 2020 and December 31, 2019, there were no investments classified as trading securities, as the Company sold its interest in the short-term bond fund in 2019. There was no realized or unrealized gain (loss) on trading securities for the three and six months ended June 30, 2020. Realized losses on trading securities during the three and six months ended June 30, 2019 were $0 and $4,980, respectively. Unrealized gain (loss) on trading securities was $36,664 and $89,405 for the three and six months ended June 30, 2019 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 as short-term investments. Available-for-sale securities with stated maturities greater than one year are classified as non-current investments in its 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 and comprehensive income (loss). 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 (Investments).

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

f. **ACCOUNTS RECEIVABLE, NET.** Accounts receivable is recorded net of customer allowance for distribution fees, trade discounts, prompt payment discounts, chargebacks and doubtful accounts. Allowances for distribution fees, trade discounts, prompt payment discounts and chargebacks are based on contractual terms. The Company estimates the allowance for expected credit loss based on existing contractual payment terms, actual payment patterns of its Customer and individual Customer circumstances. At June 30, 2020 and December 31, 2019, the Company determined that an allowance for expected credit loss 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 primarily include third party manufacturing costs 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 prior years' consolidated statements of operations and comprehensive income (loss). 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 June 30, 2020 and December 31, 2019, inventory consisted mainly of work-in-process and finished goods.

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 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 insurance, prepaid commercialization expenses, 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 June 30, 2020 and December 31, 2019, the fair value of these instruments approximated their carrying value.

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).

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

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

		Fair Value Measurements at Reporting Date Using		
	Balances as of June 30, 2020	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	$19,628,370	$ 19,628,370	$ —	$ —
U.S. Treasuries	$84,990,900	$ —	$ 84,990,900	$ —

	Balances as of December 31, 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	$23,963,617	$ 23,963,617	$ —	$ —
U.S. Treasuries	$59,932,200	$ —	$ 59,932,200	$ —
Short-term investments:				
U.S. Treasuries	$ 5,007,050	$ —	$ 5,007,050	$ —

k. OPERATING LEASES. The Company determines 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.

l. REVENUE RECOGNITION. The Company recognizes revenue when its customer obtains control of the promised goods or services, in an amount that reflects the consideration to 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®.

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

To determine revenue recognition for arrangements that are within the scope of Accounting Standards Codification ("ASC") Topic 606 – Revenue from Contracts with Customers ("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 may generate 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.

Product Revenue, Net: The Company sells Firdapse® to the 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 30 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 six month periods ended June 30, 2020 and 2019.

During the three and six months ended June 30, 2020 and 2019, all of the Company's sales were 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.

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

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 June 30, 2020 and, therefore, the transaction price was not reduced further during the three and six months ended June 30, 2020 and 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.

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 consolidated statement of operations and comprehensive income (loss) through June 30, 2020 and 2019, as well as a reduction to accounts receivable, 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 commercially-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. 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, net and accounts receivable, 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 satisfy pre-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 payor despite having health insurance, to the extent allowed by applicable law. 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 and comprehensive income (loss).

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, collaborator 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 the 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.

The collaborative agreement provides for a milestone payment upon achievement of development and regulatory events. The Company accounts for milestone payments in accordance with the provisions of Accounting Standards Update (ASU) No. 2010-17, Revenue Recognition – Milestone Method ("Milestone Method of Accounting"). The Company recognizes consideration that is contingent upon the achievement of a milestone in its entirety as revenue in the period in which the milestone is achieved only if the milestone is substantive in its entirety. A milestone is considered substantive when it meets all of the following criteria:

1. The consideration is commensurate with either the entity's performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the entity's performance to achieve the milestone;

2. The consideration relates solely to past performance; and

3. The consideration is reasonable relative to all of the deliverables and payment terms within the arrangement.

A milestone is defined as an event (i) that can only be achieved based in whole or in part on either the entity's performance or on the occurrence of a specific outcome resulting from the entity's performance, (ii) for which there is substantive uncertainty at the date the arrangement is entered into that the event will be achieved, and (iii) that would result in additional payments being due to the vendor.

The Company believes that achievement of the milestone will be substantive and there will be no substantive uncertainty once the milestone is achieved. The milestone was not achieved in the three and six months ended June 30, 2020 and 2019.

Since the Company will receive royalty reports 60 days after quarter end, royalty revenue from sales of collaboration products by our collaborator will be recognized in the quarter following the quarter in which the corresponding sales occurred. For the three and six months ended June 30, 2020 and 2019, there was no royalty revenue from sales of the collaborative product.

Refer to Note 7 (Collaborative Arrangement), for further discussion on the Company's collaborative arrangement.

m. **RESEARCH AND DEVELOPMENT.** Costs incurred in connection with research and development activities are expensed as incurred. These costs consist of direct and indirect costs associated with specific projects, as well as fees paid to various entities that perform research related services for the Company.

n. **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.

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

o. CONCENTRATION OF RISK. The financial instruments that potentially subject the Company to concentration of credit risk are cash equivalents (i.e., money market funds), investments and accounts receivable, net. The Company places its cash and cash equivalents with high-credit quality financial institutions. These amounts at times may exceed federally insured limits. The Company has not experienced any credit losses in these accounts.

The Company sells its product in the United States through an exclusive distributor (its Customer) 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 expected credit loss 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 for 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 its drugs.

p. ROYALTIES. Royalties incurred in connection with the Company's license agreement, as disclosed in Note 9 (Agreements), are expensed to cost of sales as revenue from product sales is recognized.

q. INCOME TAXES. The Company utilizes the asset and liability method of accounting for income taxes. Under this method, deferred tax assets and liabilities are determined based on differences between the financial reporting and tax basis of assets and liabilities and are measured using enacted tax rates and laws that will be in effect when the differences are expected to reverse. A valuation allowance is provided when it is more likely than not that some portion or all of a deferred tax asset will not be realized.

The Company recognizes the financial statement benefit of a tax position only after determining that the relevant tax authority would more likely than not sustain the position following an audit. For tax positions meeting the more-likely-than-not threshold, the amount recognized in the financial statements is the largest benefit that has a greater than 50 percent likelihood of being realized upon ultimate settlement with the relevant tax authority. The Company is subject to income taxes in the U.S. federal jurisdiction and various state jurisdictions. Tax regulations within each jurisdiction are subject to the interpretation of the related tax laws and regulations and require significant judgment to apply. The Company is not subject to U.S. federal, state and local tax examinations by tax authorities for years before 2016. If the Company were to subsequently record an unrecognized tax benefit, associated penalties and tax related interest expense would be reported as a component of income tax expense.

On March 27, 2020, the Coronavirus Aid, Relief, and Economic Security (CARES) Act was signed into law making several changes to the Internal Revenue Code. The changes include, but are not limited to: increasing the limitation on the amount of deductible interest expense, allowing companies to carryback certain net operating losses, and increasing the amount of net operating loss carryforwards that corporations can use to offset taxable income.

The tax law changes in the CARES Act did not have a material impact on the Company's income tax provision.

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

r. 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 six months ended June 30, 2020 and 2019, and is comprised of net unrealized gains (losses) on the Company's available-for-sale securities.

s. 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 (loss) 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 June 30,		For the Six Months Ended June 30,	
	2020	2019	2020	2019
Basic weighted average common shares outstanding	103,414,523	102,869,202	103,410,881	102,808,897
Effect of dilutive securities	3,315,900	3,059,768	3,022,981	2,290,033
Dilutive weighted average common shares outstanding	106,730,423	105,928,970	106,433,862	105,098,930

Outstanding common stock equivalents totaling approximately 3.5 million and 4.8 million, were excluded from the calculation of diluted net income (loss) per common share for the three and six months ended June 30, 2020 as their effect would be anti-dilutive. For the three and six months ended June 30, 2019, approximately 3.0 million and 3.6 million shares of outstanding stock options were excluded from the calculation of diluted net income (loss) per common share as their effect would be anti-dilutive.

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

u. RECENTLY ISSUED ACCOUNTING STANDARDS. In November 2018, the FASB issued ASU 2018-18, Collaborative Arrangements (Topic 808), which amends ASC 808 to clarify when transactions between participants in a collaborative arrangement under ASC 808 are within the scope of the FASB's new revenue standard, ASU 2014-09 (codified in ASC 606). The amendments require the application of ASC 606 existing guidance to determine the units of account that are distinct in a collaborative arrangement for purposes of identifying transactions with customers. If a unit of account within the collaborative arrangement is distinct and is with a customer, an entity shall apply the guidance in Topic 606 to that unit of account. In a transaction between collaborative participants, an entity is precluded by ASU 2018-18 from presenting a transaction together with "revenue from contracts with customers" unless the unit of account is within the scope of ASC 606 and the entity applies the guidance in ASC 606 to such unit of account. The Company adopted the new standard on January 1, 2020. The Company has a collaboration agreement with Endo Ventures Limited (Endo). See Note 7 (Collaborative Arrangement). However, these amendments did not have an impact on the Company's consolidated financial statements, as Endo does not meet the definition of a customer.

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

In June 2016, the FASB issued ASU No. 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 adopted the new standard on January 1, 2020. The adoption of this standard did not have a material impact on the Company's consolidated financial statements.

In August 2018, the FASB issued ASU 2018-15, *Intangibles – Goodwill and Other – Internal-Use Software (Subtopic 350-40), Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract.* The amendments in this update align the requirements for capitalizing implementation costs incurred in a hosting arrangement that is a services contract with the requirements for capitalizing implementation costs incurred to develop or obtain internal-use software (and hosting arrangements that include an internal-use software license). Accordingly, the amendments in this update require an entity in a hosting arrangement that is a service contract to follow the guidance in Subtopic 350-40 to determine which implementation costs to capitalize as an asset related to the service contract and which costs to expense. The Company adopted the new standard on January 1, 2020 and applied prospectively to all implementation costs incurred after the date of adoption. The adoption of this standard did not have a material impact on the Company's consolidated financial statements.

3. Investments.

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

	Estimated Fair Value	Gross Unrealized Gains	Gross Unrealized Losses	Amortized Cost
At June 30, 2020:				
U.S. Treasuries – Cash equivalents	$84,990,900	$ —	$ (1,191)	$84,992,091
At December 31, 2019:				
U.S. Treasuries – Cash equivalents	$59,932,200	$ 2,042	$ —	$59,930,158
U.S. Treasuries – ST	5,007,050	7,463	—	4,999,587
Total	$64,939,250	$ 9,505	$ —	$64,929,745

There were no realized gains or losses from available-for-sale securities for the three or six months ended June 30, 2020 or 2019. The Company did not hold any securities in an unrealized position for more than 12 months as of June 30, 2020.

The estimated fair values of available-for-sale securities at June 30, 2020, by contractual maturity, are summarized as follows:

	June 30, 2020
Due in one year or less	$84,990,900

4. Prepaid Expenses and Other Current Assets.

Prepaid expenses and other current assets consist of the following:

	June 30, 2020	December 31, 2019
Prepaid manufacturing costs	$ 5,499,746	$ 1,526,013
Prepaid insurance	622,350	1,263,129
Prepaid subscription fees	429,155	501,251
Prepaid research fees	458,937	481,057
Prepaid commercialization expenses	218,651	62,959
Other	292,414	516,665
Total prepaid expenses and other current assets	$ 7,521,253	$ 4,351,074

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 Company entered into an agreement in May 2020 that amended its lease for its office facilities. Under the amended lease, the Company's leased space will increase from approximately 7,800 square feet of space to approximately 10,700 square feet of space. The lessor is currently building this space. The lease is expected to commence in early 2021 when construction of the asset is completed and available for use. The lease disclosures for the three and six months periods ended June 30, 2020 in these financial statements have been adjusted for the modification of the current lease.

The components of lease expense were as follows:

	For the Three Months Ended June 30, 2020	For the Six Months Ended June 30, 2020
Operating lease cost	$ 65,434	$ 139,513

Supplemental cash flow information related to leases was as follows:

	June 30, 2020
Cash paid for amounts included in the measurement of lease liabilities:	
Operating cash flows	$ 168,122
Right-of-use assets obtained in exchange for lease obligations:	
Operating leases	$ 19,255

Supplemental balance sheet information related to leases was as follows:

	June 30, 2020
Operating lease right-of-use assets	$ 71,711
Other current liabilities	$ 197,896
Operating lease liabilities, net of current portion	—
Total operating lease liabilities	$ 197,896
Weighted average remaining lease term	0.6 years
Weighted average discount rate	3.68%

Remaining payments of lease liabilities as of June 30, 2020 were as follows:

2020 (remaining six months)	$ 171,483
2021	28,860
2022	—
Total lease payments	200,343
Less imputed interest	(2,447)
Total	$ 197,896

6. Accrued Expenses and Other Liabilities.

Accrued expenses and other liabilities consist of the following:

	June 30, 2020	December 31, 2019
Accrued preclinical and clinical trial expenses	$ 651,692	$ 1,183,513
Accrued professional fees	2,464,197	1,241,526
Accrued compensation and benefits	2,212,733	3,064,645
Accrued license fees	5,866,161	8,751,991
Accrued purchases	216,967	1,313,310
Accrued contributions	660,000	1,535,000
Operating lease liability	197,896	300,518
Accrued variable consideration	1,023,537	884,764
Accrued income tax	1,081,912	1,533,696
Other	30,502	172,332
Current accrued expenses and other liabilities	14,405,597	19,981,295
Lease liability—non-current	—	647,532
Non-current accrued expenses and other liabilities	—	647,532
Total accrued expenses and other liabilities	$14,405,597	$ 20,628,827

7. Collaborative Arrangement.

In December 2018, the Company entered into a collaboration and license agreement (Collaboration) with 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.

Under the Collaboration, Endo assumes 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 an up-front payment, and will receive a milestone payment, and a sharing of defined net profits upon commercialization from Endo consisting of a mid-double-digit percent of net sales of generic Sabril®. The Company has also agreed to 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 of the product.

The collaborative agreement provides for a $2.0 million milestone payment on the commercial launch of the product by Par. As of June 30, 2020 and 2019, no milestone payments have been earned.

There were no revenues from collaborative arrangement for the three or six months ended June 30, 2020 and 2019. Total expenses incurred, net, in connection with the collaborative agreement for three and six months ended June 30, 2020 were approximately $8,554 and $4,206, respectively. Total expenses incurred, net, in connection with the collaborative agreement for three and six months ended June 30, 2019 were approximately $26,178 and $38,178, respectively. These expenses have been included in research and development expenses in the accompanying consolidated statements of operations.

8. 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.

8. Commitments and Contingencies (continued).

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®. Jacobus has intervened in the case. Each party has filed a cross motion for summary judgement. There can be no assurance as to the outcome of this lawsuit. See Note 13 (Subsequent Events).

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.

9. 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 license agreement, the Company pays: (i) royalties to the licensor 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 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.

During January 2020, the Company was advised that BioMarin has transferred certain rights under the license agreement to SERB S.A.

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, these agreements are cancellable at any time, but obligate the Company to reimburse the providers for any time or costs incurred through the date of termination.

10. Income Taxes.

The Company is subject to income taxes in the U.S. federal jurisdiction and various states jurisdictions. Tax regulations within each jurisdiction are subject to the interpretation of the related tax laws and regulations and require significant judgment to apply. The Company is not subject to U.S. federal, state and local tax examinations by tax authorities for any years before 2016. If the Company were to subsequently record an unrecognized tax benefit, associated penalties and tax related interest expense would be reported as a component of income tax expense.

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. As of June 30, 2020 and December 31, 2019, based on the Company's history of operating losses, the Company has concluded that it is more likely than not that the benefit of its deferred tax assets will not be realized. Accordingly, the Company has provided a full valuation allowance for deferred tax assets including NOL and tax credit carryovers as of June 30, 2020 and December 31, 2019.

11. Stockholders' Equity.

Preferred Stock

The Company has 5,000,000 shares of authorized preferred stock, $0.001 par value per share, at June 30, 2020 and December 31, 2019. No shares of preferred stock were outstanding at June 30, 2020 and December 31, 2019.

Common Stock

The Company has 150,000,000 shares of authorized common stock, par value $0.001 per share. At June 30, 2020 and December 31, 2019, 103,422,032 and 103,397,033 shares, respectively, of common stock were issued and outstanding. Each holder of common stock is entitled to one vote of each share of common stock held of record on all matters on which stockholders generally are entitled to vote.

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. Subsequent to June 30, 2020, on July 26, 2020, the 2017 Shelf Registration Statement expired.

2020 Shelf Registration Statement

Subsequent to quarter end, on July 23, 2020, the Company filed a shelf registration statement with the SEC to sell up to $200 million of common stock, preferred stock, warrants to purchase common stock, debt securities and units consisting of one or more of such securities (the "2020 Shelf Registration Statement"). The 2020 Shelf Registration Statement (file no. 333-240052) was declared effective by the SEC on July 31, 2020. As of the date of this report, no offerings have been completed under the Company's 2020 Shelf Registration Statement. See Note 13 (Subsequent Events).

12. Stock Compensation.

For the three and six-month periods ended June 30, 2020 and 2019, the Company recorded stock-based compensation expense as follows:

	Three months ended June 30,		Six months ended June 30,	
	2020	2019	2020	2019
Research and development	$ 421,220	$ 273,212	$ 839,273	$ 560,933
Selling, general and administrative	1,373,242	651,784	2,474,540	1,297,474
Total stock-based compensation	$1,794,462	$ 924,996	$3,313,813	$1,858,407

Stock Options

As of June 30, 2020, there were outstanding stock options to purchase 12,322,001 shares of common stock, of which stock options to purchase 7,014,645 shares of common stock were exercisable as of June 30, 2020.

During the three and six-month periods ended June 30, 2020, the Company granted seven-year term options to purchase an aggregate of 260,000 and 995,000 shares, respectively, of the Company's common stock to employees and directors. The Company recorded stock-based compensation related to stock options totaling $1,627,105 and $3,010,777, respectively, during the three and six-month periods ended June 30, 2020. During the three and six-month periods ended June 30, 2020, respectively, 339,998 and 1,194,829 options vested.

During the three and six-month periods ended June 30, 2019, the Company granted seven-year term options to purchase an aggregate of 105,000 and 312,000 shares, respectively, of the Company's common stock to employees. The Company recorded stock-based compensation related to stock options totaling $924,996 and $1,858,407, respectively, during the three and six-month periods ended June 30, 2019. During the three and six-month periods ended June 30, 2019, respectively, 306,665 and 1,290,829 options vested.

During the three and six-month periods ended June 30, 2020, options to purchase 13,333 shares and 24,999 shares, respectively, of the Company's common stock were exercised, with proceeds of $36,201 and $62,350 respectively, to the Company.

During the three and six-month periods ended June 30, 2019, options to purchase 125,000 shares and 190,000 shares, respectively, of the Company's common stock were exercised, with proceeds of $192,550 and $281,900 respectively, to the Company.

As of June 30, 2020, there was approximately $9.2 million of unrecognized compensation expense related to non-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 2.3 years.

Restricted Stock Units

The Company granted 30,000 restricted stock units during the three and six-month periods ended June 30, 2020. There were no restricted stock units granted during the three and six-month periods ended June 30, 2019. During the three and six-month periods ended June 30, 2020, the Company recorded non-cash stock-based compensation expense related to restricted stock units totaling $167,357 and $303,036, respectively. No stock-based compensation related to restricted stock units was recorded during the three and six-month periods ended June 30, 2019.

As of June 30, 2020, there was approximately $1.3 million of unrecognized compensation expense related to non-vested restricted stock units granted under the 2018 Stock Incentive Plan. The cost is expected to be recognized over a weighted average period of approximately 2.5 years.

13. Subsequent Events.

Subsequent to quarter end, on July 23, 2020, the Company filed a shelf registration statement with the SEC to sell up to $200 million of common stock, preferred stock, warrants to purchase common stock, debt securities and units consisting of one or more of such securities (the "2020 Shelf Registration Statement"). On July 31, 2020, the 2020 Shelf Registration Statement was declared effective. To the date of this report, no offerings have been completed under the 2020 Shelf Registration Statement. See Note 11 (Stockholders' Equity).

On July 30, 2020, the Magistrate Judge considering the Company's lawsuit against the FDA filed a Report and Recommendation in which she recommended to the District Judge handling the case that she grant the FDA's and Jacobus' motions for summary judgement and deny the Company's motion for summary judgement. The decision on whether to grant or deny the Company's motion for summary judgement remains with the District Judge handling the case. See Note 8 (Commitments and Contingencies).

Subsequent to June 30, 2020, the Company received approval for its NDS in Canada for Firdapse® for the symptomatic treatment of LEMS.

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

Introduction

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

- *Overview.* This section provides a general description of our business and information about our business that we believe is important in understanding our financial condition and results of operations.
- *Basis of Presentation.* This section provides information about key accounting estimates and policies that we followed in preparing our consolidated financial statements for the second quarter and first half of fiscal 2020.
- *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 the 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 six-month periods ended June 30, 2020 as compared to the same periods ended June 30, 2019.
- *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.

Impact of the COVID-19 pandemic on our business

The COVID-19 pandemic has resulted, and is expected to continue to result, in significant economic disruption, and has adversely affected and will likely continue to adversely affect our business. As of the date of this report, significant uncertainty continues to exist concerning the magnitude of the impact and duration of the COVID-19 pandemic. We are actively monitoring the situation and are taking those actions that may be required by federal, state or local authorities or that we determine are in the best interests of our patients, investigators, employees and stockholders. While we are unable to determine or predict the nature, duration or scope of the overall impact that the COVID-19 pandemic will have on our business and business prospects and our financial condition, we believe that it is important to share where our company stands today, how our response to COVID-19 is progressing, and how our operations and financial condition may change as the fight against COVID-19 progresses.

In March 2020, in light of worsening conditions as a result of the pandemic, we implemented a number of safety related initiatives among our employees, including a travel ban and a work from home policy for all employees. This included our customer-facing employees, who began working remotely and utilizing telephone and web-based technologies to provide support to patients and their healthcare providers. Since many healthcare providers were delaying seeing patients other than those affected by COVID-19, we believe that this has delayed the diagnosis of new LEMS patients and their initiating therapy, which has slowed our efforts to locate new patients who could benefit from our therapy. However, as of the date of this report, we believe that some healthcare providers are beginning to again see patients and sales representatives face-to-face, and, although we cannot determine with certainty, we are hopeful that over time the impact of this aspect of the COVID-19 pandemic will lessen.

Our Firdapse® supply chain remains robust and thus far we have observed no disruptions in the production of Firdapse®. We reiterate that we are committed to providing patients with the ability to obtain an uninterrupted supply of Firdapse®, and we believe that we have an adequate supply of Firdapse® to address patients' needs through at least June 2021. Further, we are advised by our U.S. manufacturing partners that they have implemented contingency plans to remain in operation. We are committed to meeting our "patients" needs for Firdapse® and believe that our supply chain will remain solid and uninterrupted through the COVID-19 outbreak and beyond.

Further, the COVID-19 pandemic delayed our closing out of trial sites and data-collection from our MuSK-MG study, which delayed our ability to report on the top-line results from this trial until August 2020. It has also delayed completion of our SMA Type 3 proof-of-concept study.

While we are doing our best to monitor and react to the impact of the COVID-19 health crisis on our business, there can be no assurance as to the ultimate manner in which the COVID-19 crisis will impact our business and our results of operations.

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. When we acquired the rights to the product, it had already been granted orphan drug designation by the Food and Drug Administration (FDA) for the treatment of patients with 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 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 at the time of approximately 20 field personnel, including sales (Regional Account Managers), patient assistance and insurance navigation support (Patient Access Liaisons), and payor 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, and Spinal Muscular Atrophy (SMA) Type 3, and to provide education for the physicians who treat these rare diseases and the patients they treat.

In early 2020, we expanded our field sales group by almost one hundred percent and contracted with a rare-disease experienced inside sales agency. Through this recent expansion of our sales team, we hope to expand our sales efforts beyond the neuromuscular specialists who regularly treat LEMS patients to reach roughly 9,000 neurology and neuromuscular healthcare providers that may be treating an adult LEMS patient who can benefit from Firdapse®. We are also making available our no-cost LEMS voltage gated calcium channel (VGCC) antibody testing program (using a commercially available test approved by the FDA) for use by physicians who suspect that one of their patient may have LEMS and wish to reach a definitive diagnosis.

Because of the COVID-19 pandemic, in March 2020 we implemented a number of safety related initiatives among our employees, including a travel ban and a work from home policy for all employees. This included our customer-facing employees, who are working remotely and utilizing telephone and web-based technologies to provide support to patients and their healthcare providers. We are also continuing to expand our digital and social media activities in order to introduce our product to potential patients and their healthcare providers. While we are starting to see that some healthcare providers are beginning to again see patients and sales representatives face to face (and we hope that trend will continue), since many healthcare providers have delayed seeing patients other than those affected by COVID-19, this has limited our ability to locate new patients who might benefit from our drug and slowed our efforts to increase our sales from prior periods.

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 not more than $10.00 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 a New Drug Application (NDA) for Ruzurgi®, another version of amifampridine (3,4-DAP), for the treatment of pediatric LEMS patients (ages 6 to under 17). Based on publicly available information, we believe that Jacobus Pharmaceuticals is offering Ruzurgi® at a cost for a patient taking a daily dose of 60 mg per day of approximately $175,200 annually and a cost for a patient taking a daily dose of 100 mg of approximately $292,000 annually. Both prices are lower than the list price for an equivalent amount of Firdapse®. In addition, while the NDA for Ruzurgi® only covers pediatric patients, we believe that Ruzurgi® is regularly being prescribed off label to adult LEMS patients.

We believe that under applicable law, Jacobus is not permitted to market its amifampridine product to adult LEMS patients in the United States, and we are continuing to aggressively take all steps available to us to protect Firdapse®'s exclusivity under the Orphan Drug Act. There can be no assurance, however, that we will be able to stop the off-label prescribing of Ruzurgi® to adult LEMS patients, and if Jacobus is able to successfully sell Ruzurgi® off-label to additional adult LEMS patients, it could have a material adverse effect on our business, financial condition and results of operations.

We also 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 setting aside the FDA's approval of Ruzurgi®. We have filed a motion for summary judgement in our case, and the FDA has filed a cross motion for summary judgement. Further, Jacobus has intervened in the case and has filed their own cross motion for summary judgement.

On July 30, 2020, the Magistrate Judge considering Catalyst's lawsuit against the FDA filed a Report and Recommendation in which she recommended to the District Judge handling the case that she grant the FDA's and Jacobus' motions for summary judgement and deny Catalyst's motion for summary judgement. We are currently reviewing the Magistrate Judge's decision, which we believe to be incorrect as a matter of law and contrary to the plain language of the Orphan Drug Act, and we intend to pursue the case further with the District Judge. The decision on whether to grant or deny our motion for summary judgement remains with the District Judge handling the case. We believe that if the Magistrate Judge's recommendation is correct on the law, it means that the FDA has the authority to effectively eliminate the benefits of exclusivity under the Orphan Drug Act, which we believe will chill the incentive for drug companies like ourselves to spend the millions of dollars necessary to develop an orphan drug.

On August 10, 2020, we announced the top-line results from our Phase 3 clinical trial (MSK-002) evaluating Firdapse® for the treatment of adults with MuSK-MG. Our trial was a multi-site, international (United States, Italy and Serbia), double-blind, placebo-controlled, clinical trial being conducted under a Special Protocol Assessment (SPA) with the FDA. The trial enrolled more than 60 MuSK antibody positive patients. It also enrolled more than 10 generalized myasthenia gravis patients who were assessed with the same clinical endpoints. However, achieving statistical significance in this subgroup of patients was not required. Details of this trial are available on www.clinicaltrials.gov (NCT03304054).

The MSK-002 trial did not achieve statistical significance on the primary endpoint, which was the eight-item Myasthenia Gravis Activities of Daily Living (MG-ADL) total score change from baseline to day 10, in this randomized withdrawal trial with a score of ($p=0.2196$). The secondary endpoint, Quantitative Myasthenia Gravis (QMG) scale also did not achieve statistical significance ($p=0.3736$). QMG is a thirteen-item evaluation of ocular, facial, bulbar, gross motor, axial, and respiratory weaknesses. Firdapse® was safe and well tolerated during the MSK-002 trial, and demonstrated a safety profile similar to that seen for Firdapse® used for the treatment LEMS.

After all of the data from the MSK-002 trial has been thoroughly evaluated, we intend to meet with our neuromuscular advisors and determine our path forward for this indication. We plan to make the results of this study available in a future scientific forum.

We are currently conducting a proof-of-concept clinical study evaluating Firdapse® as a symptomatic treatment for ambulatory patients with Spinal Muscular Atrophy (SMA) Type 3. The study, which is being conducted at trial sites in Italy and Serbia, has enrolled the anticipated 12 subjects in a randomized (1:1), double-blind, 2-period, 2-treatment, crossover, outpatient proof-of-concept study evaluating the safety, tolerability and potential efficacy of amifampridine in ambulatory patients diagnosed with SMA Type 3. Details of this trial are available on www.clinicaltrials.gov (NCT03781479). We believe that our SMA Type 3 study will be completed this year and that we will be in a position to report top-line results from this study before the end of 2020.

We are also supporting investigator-sponsored studies evaluating Firdapse® as a treatment for Kennedy's Disease and Hereditary Neuropathy with liability to Pressure Palsies (HNPP).

There can be no assurance that our study evaluating Firdapse® for the treatment of SMA Type 3, or any trials we may undertake or support 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 MuSK-MG, SMA Type 3, or any other additional indications.

We are currently in the early stages of developing a long-acting formulation of amifampridine. We have retained a contractor which is currently assisting us in developing the formulation of the product. We currently anticipate that initial formulation candidates and their drug release and absorption properties shall be determined during 2020. There can be no assurance that we will be able to successfully develop a sustained release formulation of Firdapse®, that any such formulation will be approved for marketing, or that any such formulation will be commercially viable.

The Canadian NDS for Firdapse® for the symptomatic treatment of LEMS that we submitted in October 2019, was approved by Health Canada on August 4, 2020. Since there is no orphan exclusivity in Canada, there can be no assurance that any application for amifampridine filed by other parties will not be approved as well. As a result, we may face competition in Canada for LEMS patients in the future.

We are currently in discussions with a potential marketing and distribution partner in Canada. There can be no assurance that we will reach an agreement for a marketing and collaboration agreement in Canada with this or any other potential partners.

In May 2019, we entered into an amendment to our license agreement 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.

We recently met with Japanese regulatory authorities and believe that we have reached a tentative agreement with them as to the scope of the clinical trial that we will be required to undertake in Japan before we will be permitted to submit an application to the Japanese regulatory authorities to seek to commercialize Firdapse® for the treatment of LEMS in Japan. We also intend to apply for orphan drug designation in Japan for the symptomatic treatment of LEMS. There can be no assurance that we will successfully obtain the right to commercialize Firdapse® in Japan or obtain orphan drug designation.

All of our patent rights for Firdapse® are derived from our license agreement. Under the 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 have responded 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, there is a possibility we may be prevented from pursuing product development, manufacturing or commercialization of our products that utilize such technologies until the underlying patent dispute is resolved. 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 December 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, in December 2018, we received an up-front payment of $500,000. We will be entitled to receive a milestone payment of $2.0 million on the commercial launch of the product. Further, we will receive a sharing of defined net profits upon commercialization and we are obligated to share the costs of certain development expenses.

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.

Capital Resources

At June 30, 2020, we had cash and investments of approximately $115.1 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 Form 10-Q. There can be no assurance that we will continue to be successful in commercializing Firdapse® or will continue to be profitable and cash flow positive. Further, there can be no assurance that if we need additional funding in the future, whether such funding will be available to us. See "Liquidity and Capital Resources" below for further information on our liquidity and cash flow.

Basis of Presentation

Revenues.

At June 30, 2020 we continued to generate revenues from product sales of Firdapse®. We expect these revenues to fluctuate in future periods based on our sales of Firdapse®. At June 30, 2020 and June 30, 2019, we did not generate revenues under our collaborative agreement with Endo. We expect our revenues from the collaborative agreement to fluctuate in future periods based on our collaborator's ability to meet various regulatory milestones set forth in such agreement.

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 has caused cost of sales to appear artificially low as we consumed product manufactured prior to approval, and will continue to do so until we deplete such product and additional product is manufactured and distributed.

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 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 2019, we actively committed funds to developing our commercialization program for Firdapse® and we have continued to incur commercialization expenses, including sales, marketing, patient services, patient advocacy and other commercialization related expenses, as we have continued our sales program for Firdapse®.

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, 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 accounting principles generally accepted in the U.S. (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.

We are currently conducting a study of the availability for use of our net operating loss carryforwards and other credits under Section 382 of the Internal Revenue Code, and the results of this study could impact the amounts of net operating losses and other credits that we have available for use in future periods, and the timing of their use.

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.

Non-GAAP Financial Measures.

We prepare our consolidated financial statements and notes thereto which accompany this report in accordance with U.S. GAAP. To supplement our financial results presented on a U.S. GAAP basis, we may use non-GAAP financial measures in our reports filed with the Commission and/or our communications with investors. Non-GAAP measures are provided as additional information and not as an alternative to our consolidated financial statements presented in accordance with GAAP. Our non-GAAP financial measures are intended to enhance an overall understanding of our current financial performance. We believe that the non-GAAP financial measures we present provide investors and prospective investors with an alternative method for assessing our operating results in a manner that we believe is focused on the performance of ongoing operations and provide a more consistent basis for comparison between periods.

The non-GAAP financial measure that we present excludes from the calculation of net income the non-cash expense associated with stock-based compensation. Further, we often report non-GAAP net income (loss) per share, which is calculated by dividing non-GAAP net income (loss) by the weighted average common shares outstanding.

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

Critical Accounting Policies and Estimates

Our discussion and analysis of our financial condition and results of operations is based on our 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 and liabilities and the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, as well as the reported revenues and expenses during the reporting periods. For a full discussion of our accounting policies, please refer to Note 2 on the Financial Statements included in our 2019 Annual Report on Form 10-K filed with the SEC. Our most critical accounting policies and estimates include: revenue recognition, leases, accounting for research and development expenses, 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* of our 2019 Annual Report on Form 10-K.

Results of Operations

Revenues.

For the three and six-month periods ended June 30, 2020, we recognized approximately $29.6 million and $58.7 million, respectively, in net revenue from product sales from Firdapse® compared to approximately $28.8 million and $41.3 million for the three and six-month periods ended June 30, 2019. We had no revenues from our collaborative arrangement for the three and six-month periods ended June 30, 2020 and 2019.

Cost of Sales.

Cost of sales was approximately $4.1 million and $8.3 million for the three and six-months ended June 30, 2020 compared to approximately $4.3 million and $6.0 million for the three and six-months ended June 30, 2019. Cost of sales consists principally of 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 that was recorded as expense prior to FDA approval of Firdapse®.

Research and Development Expenses.

Research and development expenses for the three-month periods ended June 30, 2020 and 2019 were approximately $4.3 million and $4.6 million, respectively, and represented approximately 22.5% and 25.9% of total operating costs and expenses, respectively. Research and development expenses for the three months ended June 30, 2020 and 2019 were as follows:

	Three months ended June 30,		Change	
	2020	2019	$	%
Research and development expenses	$3,928,423	$4,356,152	(427,729)	(9.8)
Employee stock-based compensation	421,220	273,212	148,008	54.2
Total research and development expenses	$4,349,643	$4,629,364	(279,721)	(6.0)

Research and development expenses for the six-month periods ended June 30, 2020 and 2019 were approximately $8.6 million and $7.9 million, respectively, and represented approximately 22.7% and 25.3% of total operating costs and expenses for the six-month periods ended June 30, 2020 and 2019, respectively. Research and development expenses for the six months ended June 30, 2020 and 2019 were as follows:

	Six months ended June 30,		Change	
	2020	2019	$	%
Research and development expenses	$7,733,181	$7,376,390	356,791	4.8
Employee stock-based compensation	839,273	560,933	278,340	49.6
Total research and development expenses	$8,572,454	$7,937,323	635,131	8.0

For the three and six months ended June 30, 2020, research and development expenses decreased approximately $0.3 million and increased approximately $0.6 million, respectively, compared to the same period in 2019, primarily attributable to the following:

- increases in headcount, medical and regulatory affairs and quality assurance expenses and expenses from our ongoing clinical trials evaluating Firdapse® for the treatment of MuSK-MG, and our proof-of-concept trial evaluating Firdapse® for the treatment of SMA Type 3; and
- increases in employee stock-based compensation which is non-cash and relates to the expense of stock options awards to certain employees, due to increase in headcount.

We expect that research and development expenses will continue to be substantial in 2020 as we continue our clinical program evaluating Firdapse® for the treatment of MuSK-MG, 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®, continue our regulatory path to seek approval of Firdapse® in Japan, and begin to evaluate Firdapse® as a treatment for other neuromuscular diseases.

Selling, General and Administrative Expenses.

Selling, general and administrative expenses for the three months ended June 30, 2020 and 2019 were approximately $10.8 million and $9.0 million, respectively, and represented 56.1% and 50.3% of total operating costs and expenses for the three months ended June 30, 2020 and 2019, respectively. Selling, general and administrative expenses for the three months ended June 30, 2020 and 2019 were as follows:

	Three months ended June 30,		Change	
	2020	2019	$	%
Selling	$ 5,621,780	$4,881,137	740,643	15.2
General and administrative	3,838,336	3,454,801	383,535	11.1
Employee stock-based compensation	1,373,242	651,784	721,458	110.7
Total selling, general and administrative expenses	$10,833,358	$8,987,722	1,845,636	20.5

Selling, general and administrative expenses for the six months ended June 30, 2020 and 2019 were approximately $20.9 million and $17.4 million, respectively, and represented 55.3% and 55.6% of total operating costs and expenses for the six months ended June 30, 2020 and 2019, respectively. Selling, general and administrative expenses for the six months ended June 30, 2020 and 2019 were as follows:

	Six months ended June 30,		Change	
	2020	2019	$	%
Selling	$11,425,925	$ 9,985,045	1,440,880	14.4
General and administrative	6,995,941	6,121,663	874,278	14.3
Employee stock-based compensation	2,474,540	1,297,474	1,177,066	90.7
Total selling, general and administrative expenses	$20,896,406	$17,404,182	3,492,224	20.1

For the three and six months ended June 30, 2020, selling, general and administrative expenses increased approximately $1.8 million and $3.5 million, respectively, compared to the same periods in 2019, primarily attributable to the following:

- increases in selling (commercialization) expenses, which consist primarily of the costs of our expansion of the sales force and the cost of contracting with a rare-disease experience inside sales agency;
- increases in general and administrative expenses, which are primarily due to the expansion of our operations and headcount to support our ongoing efforts to expand our net revenues from sales of Firdapse®; and
- increases in employee stock-based compensation which is non-cash and relates to the expense of stock options awards to certain employees and directors due to increase in headcount.

We expect that selling, general and administrative expenses will be substantial in future periods as we continue our efforts to sell Firdapse® and take steps that we hope will help us expand our business.

Stock-Based Compensation.

Total stock-based compensation for the three and six-month periods ended June 30, 2020 were $1.8 million and $3.3 million, respectively, and for the three and six-month periods ended June 30, 2019 were $925,000 and $1.9 million, respectively. In the first half of 2020, grants were principally of stock options relating to 2019 year-end bonus awards. In the first half of 2019, most of the option grants were to new employees hired in connection with the launch of Firdapse®.

Other Income, Net.

We reported other income, net in all periods relating to our investment of funds received from offerings of our securities and product sales. For the three and six-months ended June 30, 2019, other income, net also included $100,000 received as part of the settlement agreement between us and Northwestern. Excluding the settlement income, the decrease in other income, net of approximately $246,000 for the six months ended June 30, 2020 when compared to the same period in 2019 is primarily due to lower yields on investments, despite higher invested balances. Other income, net, generally consists of interest income, dividend income and unrealized and realized gain (loss) on trading securities.

Income Taxes.

We incurred net operating losses since inception through the three-month period ended March 31, 2019. Our effective income tax rate was 5.70% and 4.21% for the six months ended June 30, 2020 and 2019, respectively. Differences in the effective tax and the statutory federal income tax rate of 21% are 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 June 30, 2020 and December 31, 2019. We have a full valuation allowance for our deferred tax assets at June 30, 2020 and December 31, 2019.

Net Income (Loss).

Our net income was approximately $9.8 million and $20.2 million, respectively, for the three and six months ended June 30, 2020 ($0.09 and $0.20, respectively, per basic share and $0.09 and $0.19, respectively, per diluted share) as compared to net income of approximately $11.0 million and $10.3 million, respectively, for the three and six months ended June 30, 2019 ($0.11 and $0.10, respectively, per basic share and $0.10 and $0.10, respectively, per diluted share).

Non-GAAP Net Income.

Our non-GAAP net income, which excludes for the three and six months ended June 30, 2020 an approximately $1.8 million and $3.3 million, respectively, expense associated with non-cash stock-based compensation was approximately $11.6 million and $23.5 million ($0.11 and $0.23, respectively, per basic share and $0.11 and $0.22, respectively, per diluted share). Our non-GAAP net income for the three and six months ended June 30, 2019 was approximately $11.9 million and $12.2 million, respectively ($0.12 per basic and $0.11 per diluted share and $0.12 per basic and diluted share, respectively), which excludes non-cash stock-based compensation of approximately $925,000 and $1.9 million, respectively.

Liquidity and Capital Resources

Since our inception, we have financed our operations primarily through multiple public and private offering of our securities and, since January 2019, from revenues from product sales of Firdapse®. At June 30, 2020, we had cash and cash equivalents and investments aggregating approximately $115.1 million and working capital of approximately $111.0 million. At December 31, 2019, we had cash and cash equivalents and investments aggregating approximately $94.5 million and working capital of approximately $87.3 million. At June 30, 2020, 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 money market accounts 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 and cash-flow positive or that we will be able to obtain any additional funding that we may require in the future.

In the future, we may require additional working capital to support our operations depending on our future success with Firdapse® sales and whether our results continue to be profitable and 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 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. The 2017 Shelf Registration Statement expired on July 26, 2020.

On July 23, 2020, we filed a shelf registration statement with the SEC to sell up to $200 million of common stock, preferred stock, warrants to purchase common stock, debt securities and units consisting of one or more of such securities (the "2020 Shelf Registration Statement"). The 2020 Shelf Registration Statement (file no. 333-240052) was declared effective by the SEC on July 31, 2020. As of the date of this report, no offerings have been completed under the 2020 Shelf Registration Statement.

Cash Flows.

Net cash provided by operating activities was $20,478,188 and $6,057,415, respectively, for the six-month periods ended June 30, 2020 and 2019. During the six months ended June 30, 2020 net cash provided by operating activities was primarily attributable to our net income of $20,206,002, increases of $3,774,735 in accounts receivable, net and $1,687,331 in accounts payable and decreases of $128,868 in inventory and $4,074,661 of non-cash expenses. This was partially offset by an increase of $3,170,179 in prepaid expenses and other current and non-current assets and decreases of $5,473,075 in accrued expenses and other liabilities and $750,155 in operating lease liability. During the six months ended June 30, 2019, net cash provided by operating activities was primarily attributable to our net income of $10,315,445, decreases of $161,178 in prepaid expenses and other current assets and increases of $991,359 in accounts payable and $3,393,753 in accrued expenses and other liabilities and $1,921,097 of non-cash expenses. This was partially offset by increases of $10,376,427 in accounts receivable, net and $213,867 in inventory and a decrease of $135,123 in operating lease liability.

Net cash provided by investing activities was $5,000,000, for the six-month period ended June 30, 2020, consisting of proceeds from maturities of investments. Net cash provided by investing activities was $518,797 for the six-month period ended June 30, 2019, consisting primarily of proceeds from sales and maturities of investments of $30,310,595, partially offset by purchases of investments of $29,772,428.

Net cash provided by financing activities during the six-month periods ended June, 2020 and 2019 was $62,350 and $281,900, respectively, consisting of proceeds from the exercise of options to purchase common stock.

Contractual Obligations and Arrangements.

We have entered into the following contractual arrangements:

- *Payments under our license agreement*. Under our license agreement, we have agreed to pay (i) royalties to our licensor 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 six-months ended June 30, 2020, we recognized approximately $3.9 million and $7.8 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 to 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 $600,000 in 2020. 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. We entered into an agreement in May 2020 that amended its lease for its office facilities. Under the amended lease, our leased space will increase from approximately 7,800 square feet of space to approximately 10,700 square feet of space. The lessor is currently building out this space. The lease is expected to commence in early 2021 when construction of the asset is expected to be completed and available for use.

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 other achievements to be materially different from any future results, performances or achievements expressed or implied by such forward-looking statements. Factors that might cause such differences include, but are not limited to, those discussed in the section entitled “Item 1A – Risk Factors” in our 2019 Annual Report on Form 10-K.

The continued successful commercialization of Firdapse® and the development of additional indications for Firdapse® is highly uncertain. Factors that will affect our success include the uncertainty of:

- The impact of the recent outbreak of a novel strain of coronavirus on our business or on the economy generally;
- Whether we will be able to continue 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 Firdapse® for the treatment of Lambert-Eaton Myasthenic Syndrome (“LEMS”) 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 patients will discontinue from the use of our drug at rates that are higher than historically experienced or are higher than we project;
- If the average daily dose taken by patients changes over time, it could affect our results of operations;
- Whether Firdapse® patients can be successfully titrated to stable therapy;
- Whether we can continue to market Firdapse® on a profitable and cash flow positive basis;
- Whether any revenue guidance that we provide to the public market will turn out to be accurate;
- Whether payors will continue to reimburse for our product at the price that we charge for the product;
- 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;
- 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;
- The scope of our intellectual property and the outcome of any future challenges or opposition to our intellectual property, and, conversely, whether any third-party intellectual property presents unanticipated obstacles for Firdapse®;
- The effect on our business and future results of operations arising from the approval by the FDA of Ruzurgi® for the treatment of pediatric LEMS patients (ages 6 to under 17);
- Whether our suit against the United States FDA seeking to vacate the FDA’s approval of Ruzurgi® will be successful;
- Whether we can continue to compete successfully if the approval of Ruzurgi® is not overturned and Ruzurgi® continues to be prescribed for off-label use by adult LEMS patients;
- Whether, because of the lower price of Ruzurgi®, payors will require that patients try off-label Ruzurgi® first before they approve Firdapse® as a treatment for adult LEMS patients;
- 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 organization, 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 politicians and a vocal group of LEMS patients and doctors who object to our pricing of Firdapse®;

- Changes in the healthcare industry and the effect of political pressure from and actions by President Trump, Congress and/or medical professionals seeking to reduce prescription drug costs;
- The state of the economy generally and its impact on our business;
- 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 recent coronavirus outbreak will further affect the timing of our currently ongoing clinical trials;
- Whether the trial that we are currently undertaking to evaluate Firdapse® for the treatment of Spinal Muscular Atrophy (SMA) Type 3, or any other trials that we may undertake in the future, will be successful;
- Whether Firdapse® will ever be approved for the treatment of MuSK-MG, SMA Type 3, or any other neuromuscular disease;
- Whether we can successfully commercialize Firdapse® in Canada on a profitable basis;
- The impact on sales of Firdapse® in the United States if an amifampridine product is purchased in Canada for use in the United States;
- Whether we will be able to successfully complete the clinical trial in Japan that will be required to seek approval to commercialize Firdapse® in Japan;
- Whether we will be able to obtain approval to commercialize Firdapse® in Japan;
- Whether we can successfully develop, obtain approval of and successfully market a sustained release version of Firdapse®;
- Whether our efforts to grow our business beyond Firdapse® through acquisitions of companies or in-licensing of product opportunities in the neuromuscular or neurology therapeutic areas will be successful;
- Whether we will have sufficient capital to finance any such acquisitions;
- Whether our version of generic vigabatrin tablets will ever be approved by the FDA;
- Even if our version of vigabatrin tablets is approved for commercialization, whether Endo Ventures/Par Pharmaceutical (our collaborator in this venture) will be successful in marketing the product; and
- Whether we will earn milestone payments on the first commercial sale of vigabatrin tablets and royalties on sales of generic vigabatrin tablets.

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

Market risk represents the risk of changes in the value of market risk-sensitive instruments caused by fluctuations in interest rates, foreign exchange rates and commodity prices. Changes in these factors could cause fluctuations in our results of operations and cash flows.

Our exposure to interest rate risk is currently confined to our cash and short-term investments that are from time to time invested in highly liquid money market funds and U.S. Treasuries. The primary objective of our investment activities is to preserve our capital to fund operations. We also seek to maximize income from our investments without assuming significant risk. We do not use derivative financial instruments in our investment portfolio. Our cash and investments policy emphasizes liquidity and preservation of principal over other portfolio considerations.

ITEM 4. CONTROLS AND PROCEDURES

a. We have carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Based on such evaluation, our principal executive officer and principal financial officer have concluded that as of June 30, 2020, our disclosure controls and procedures were effective to ensure that the information required to be disclosed by us in the reports filed or submitted by us under the Exchange Act, was recorded, processed, summarized or reported within the time periods specified in the rules and regulations of the SEC, and include controls and procedures designed to ensure that information required to be disclosed by us in such reports was accumulated and communicated to management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosures.

b. During the three months ended June 30, 2020, there were no changes in our internal controls or in other factors that could have a material effect, or are reasonably likely to have a material effect, on our internal control over financial reporting.

PART II. OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

Ruzurgi®

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®.

We recently filed a motion for summary judgement in our case, and the FDA has filed a cross motion for summary judgement. Further, Jacobus has intervened in our case and filed their own cross-motion for summary judgement. Based on currently available information, we expect that there will be a decision in the case sometime later this year. There can be no assurance as to the outcome of this lawsuit, the timing of any decision, or the likelihood of an appeal if our suit is successful.

On July 30, 2020, the Magistrate Judge considering our lawsuit against the FDA filed a Report and Recommendation in which she recommended to the District Judge handling the case that she grant the FDA's and Jacobus' motions for summary judgement and deny our motion for summary judgement. We are currently reviewing the Magistrate Judge's decision, which we believe to be incorrect as a matter of law and contrary to the plain language of the Orphan Drug Act, and we intend to pursue the case further with the District Judge. The decision on whether to grant or deny our motion for summary judgement remains with the District Judge handling the case.

Other Litigation

From time to time we may become involved in legal proceedings arising in the ordinary course of business. Other than as set forth above, we believe that there is no 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 2019 Annual Report on Form 10-K filed with the SEC, which contain a description of significant factors that might cause our actual results of operations in future periods to differ materially from those currently expected or desired.

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

None

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None

ITEM 4. MINE SAFETY DISCLOSURE

Not applicable

ITEM 5. OTHER INFORMATION

None

ITEM 6. EXHIBITS

31.1	Certification of Principal Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Certification of Principal Financial Officer under Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Certification of Principal Executive Officer under Section 906 of the Sarbanes-Oxley Act of 2002
32.2	Certification of Principal Financial Officer under Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.
101.SCH	Inline XBRL Taxonomy Extension Schema Document
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104	The cover page for the Company’s Quarterly Report on Form 10-Q for the quarter ended June 30, 2020, has been formatted in Inline XBRL.

SIGNATURES

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

Catalyst Pharmaceuticals, Inc.

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

Date: August 10, 2020

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 Rules 13a-15(f) and 15(d)-15(f)) for the registrant and have:

   a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, 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: August 10, 2020

/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 Rules 13a-15(f) and 15(d)-15(f)) for the registrant and have:

   a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, 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: August 10, 2020

/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 June 30, 2020 (the "Report"), filed with the U.S. Securities and Exchange Commission, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

2. the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: August 10, 2020

/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 June 30, 2020 (the "Report"), filed with the U.S. Securities and Exchange Commission, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

2. the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: August 10, 2020

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