UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

FORM 10-Q

(Mark one)

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

For the quarterly period ended September 30, 2020
or

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

For the transition period from _____ to _____

Commission file number **001-15771**

ABEONA THERAPEUTICS INC.

(Exact name of registrant as specified in its charter)

Delaware	**83-0221517**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer I.D. No.)

1330 Avenue of the Americas, 33rd Floor, New York, NY 10019
(Address of principal executive offices, zip code)

(646) 813-4701
(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Securities Exchange Act of 1934:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.01 par value	ABEO	Nasdaq Capital Market

Indicate by check mark 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 reports), 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 (§232.405 of this chapter) 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 the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer ☐
Non-accelerated filer ☐
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 standards provided 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 Act). Yes ☐No ☒

The number of shares outstanding of the registrant's common stock as of November 2, 2020 was98,527,723 shares.

ABEONA THERAPEUTICS INC.

INDEX

FORWARD-LOOKING STATEMENTS

This Form 10-Q contains statements that express management's opinions, expectations, beliefs, plans, objectives, assumptions or projections regarding future events or future results and therefore are, or may be deemed to be, "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Words such as "expects," "predicts," "intends," "plans," "believes," "could," "would," "estimates," and variations of such words and similar expressions, and the negatives thereof, are intended to identify such forward-looking statements. Such "forward-looking statements" speak only as of the date made and are not guarantees of future performance and involve certain risks, uncertainties, estimates, and assumptions by management that are difficult to predict. Various factors, some of which are beyond the Company's control, could cause actual results to differ materially from those expressed in, or implied by, such forward-looking statements. In addition, we disclaim any obligation to update any forward-looking statements to reflect events or circumstances after the date of this report, except as may otherwise be required by the federal securities laws.

Forward-looking statements necessarily involve risks and uncertainties, and our actual results could differ materially from those anticipated in forward-looking statements due to a number of factors. These statements include statements about: the Company's plans to review strategic options focused on advancing the Company's mission and maximizing stakeholder value, including the sale of some or all of its assets or sale of the Company; the potential impacts of the COVID-19 pandemic on our business, operations, and financial condition; the achievement of or expected timing, progress and results of clinical development, clinical trials and potential regulatory approvals; our Phase III clinical trial (VIITAL™) for patients with recessive dystrophic epidermolysis bullosa ("RDEB") and our beliefs relating thereto; our ability to identify and enroll patients in the Phase III clinical trial; our pipeline of product candidates; our intended use of the proceeds from the Paycheck Protection Program loan and our eligibility for loan forgiveness under the Coronavirus Aid, Relief and Economic Security Act, as amended; our belief that we have sufficient resources to fund operations for the next 12 months; the arbitration proceeding with REGENXBIO; the dilutive effect that raising additional funds by selling additional equity securities would have on the relative equity ownership of our existing investors; our belief that EB-101 could potentially benefit patients with RDEB; our belief that adeno-associated virus ("AAV") gene therapy could potentially benefit patients with Sanfilippo syndrome type A ("MPS IIIA") and Sanfilippo syndrome type B ("MPS IIIB"); our ability to develop our novel AAV-based gene therapy platform technology; our belief in the adequacy of the data from clinical trials, including VIITAL™ and our Phase I/II clinical trials in ABO-102 (AAV-SGSH) for MPS IIIA and ABO-101 (AAV-NAGLU) for MPS IIIB, together with the data generated in the program to date, to support regulatory approvals; the existence of intellectual property, a license to which might be required to market MPS IIIA and MPS IIIB; our dependence upon our third-party and related-party customers and vendors and their compliance with regulatory bodies; our intellectual property position and our ability to obtain, maintain and enforce intellectual property protection and exclusivity for our proprietary assets; our estimates regarding the size of the potential markets for our product candidates, the strength of our commercialization strategies and our ability to serve and supply those markets; and future economic conditions or performance.

Important factors that could affect performance and cause results to differ materially from management's expectations are described in the sections entitled "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" in the Company's Form 10-K for the fiscal year ended December 31, 2019, as updated from time to time in the Company's Securities and Exchange Commission filings, including this Form 10-Q. These factors include: the impact of the COVID-19 pandemic on our business, operations (including our clinical trials), and financial condition, and on our ability to access the capital markets; our estimates regarding expenses, future revenues, capital requirements, and needs for additional financing; our ability to raise capital; our ability to fund our operating expenses and capital expenditure requirements for at least the next 12 months with our existing cash and cash equivalents; our ability to obtain additional equity funding from current or new stockholders, out-licensing technology and/or other assets, deferring and/or eliminating planned expenditures, restructuring operations and/or reducing headcount, and sales of assets; the dilutive effect that raising additional funds by selling additional equity securities would have on the relative equity ownership of our existing investors; our ability to continue to develop our novel AAV-based gene therapy platform technology; the outcome of any interactions with the U.S. Food and Drug Administration ("FDA") or other regulatory agencies relating to any of our products or product candidates; our ability to execute a Phase III clinical trial for patients with RDEB; our ability to complete enrollment of patients into clinical trials to secure sufficient data to assess efficacy and safety; our ability to identify additional patients for our Phase I/II clinical trial for patients with MPS IIIA and MPS IIIB; our ability to continue to secure and maintain regulatory designations for our product candidates; our ability to develop manufacturing capability compliant with current good manufacturing practices for our product candidates; our ability to manufacture gene and cell therapy products and produce an adequate product supply to support clinical trials and potentially future commercialization; the rate and degree of market acceptance of our product candidates for any indication once approved; and our ability to meet our obligations contained in license agreements to which we are party.

PART I – FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

Abeona Therapeutics Inc. and Subsidiaries

Condensed Consolidated Balance Sheets

	September 30, 2020 (Unaudited)	December 31, 2019
ASSETS		
Current assets:		
Cash and cash equivalents	$ 8,424,000	$ 129,258,000
Receivables	7,000,000	-
Short-term investments	88,447,000	-
Prepaid expenses and other current assets	689,000	3,132,000
Total current assets	104,560,000	132,390,000
Property and equipment, net	12,095,000	13,157,000
Right-of-use lease assets	7,295,000	8,047,000
Licensed technology, net	1,881,000	36,178,000
Goodwill	32,466,000	32,466,000
Other assets and restricted cash	1,068,000	1,144,000
Total assets	$ 159,365,000	$ 223,382,000
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 1,262,000	$ 3,763,000
Accrued expenses	3,585,000	5,543,000
Loan payable	1,758,000	-
Current portion of lease liability	1,709,000	1,699,000
Payable to licensor	30,127,000	27,400,000
Deferred revenue	296,000	296,000
Total current liabilities	38,737,000	38,701,000
Long-term lease liabilities	5,517,000	6,251,000
Total liabilities	44,254,000	44,952,000
Commitments and contingencies		
Stockholders' equity:		
Common stock - $0.01 par value; authorized 200,000,000 shares; issued and outstanding 84,516,161 at September 30, 2020 and 83,622,135 at December 31, 2019	845,000	836,000
Additional paid-in capital	669,125,000	664,064,000
Accumulated deficit	(554,876,000)	(486,470,000)
Accumulated other comprehensive income	17,000	-
Total stockholders' equity	115,111,000	178,430,000
Total liabilities and stockholders' equity	$ 159,365,000	$ 223,382,000

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

Abeona Therapeutics Inc. and Subsidiaries

Condensed Consolidated Statements of Operations and Comprehensive Loss
(Unaudited)

	For the three months ended September 30,		For the nine months ended September 30,	
	2020	2019	2020	2019
Revenues				
License and other revenues	$ 7,000,000	$ -	$ 7,000,000	$ -
Expenses:				
Research and development	7,969,000	10,917,000	20,896,000	38,961,000
General and administrative	4,432,000	4,700,000	16,382,000	15,971,000
Depreciation and amortization	847,000	2,032,000	3,746,000	5,747,000
Licensed technology impairment charge	-	-	32,916,000	-
Total expenses	13,248,000	17,649,000	73,940,000	60,679,000
Loss from operations	(6,248,000)	(17,649,000)	(66,940,000)	(60,679,000)
Interest and miscellaneous income	338,000	277,000	1,261,000	828,000
Interest and other expense	(1,327,000)	-	(2,727,000)	-
Net loss	$ (7,237,000)	$ (17,372,000)	$ (68,406,000)	$ (59,851,000)
Basic and diluted loss per common share	$ (0.08)	$ (0.35)	$ (0.73)	$ (1.22)
Weighted average number of common shares outstanding – basic and diluted	93,772,712	49,721,753	93,199,679	48,883,883
Other comprehensive income/(loss):				
Change in unrealized (losses) gains related to available-for-sale debt securities	(116,000)	-	17,000	-
Comprehensive loss	$ (7,353,000)	$ (17,372,000)	$ (68,389,000)	$ (59,851,000)

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

Abeona Therapeutics Inc. and Subsidiaries

Condensed Consolidated Statements of Stockholders' Equity
(Unaudited)

	Common Stock		Additional Paid-in	Accumulated	Accumulated Other Comprehensive	Total Stockholders'
	Shares	Amount	Capital	Deficit	Income	Equity
For the three months ended September 30, 2020						
Balance, June 30, 2020	84,781,241	$ 848,000	$ 667,712,000	$ (547,639,000)	$ 133,000	$ 121,054,000
Stock option-based compensation expense	-	-	1,249,000	-	-	1,249,000
Restricted stock-based compensation expense	-	-	161,000	-	-	161,000
Cancellation of restricted share awards	(265,080)	(3,000)	3,000	-	-	-
Net loss	-	-	-	(7,237,000)	-	(7,237,000)
Other comprehensive loss	-	-	-	-	(116,000)	(116,000)
Balance, September 30, 2020	84,516,161	$ 845,000	$ 669,125,000	$ (554,876,000)	$ 17,000	$ 115,111,000
For the three months ended September 30, 2019						
Balance, June 30, 2019	49,249,497	$ 492,000	$ 559,335,000	$ (452,667,000)	$ -	$ 107,160,000
Stock option-based compensation expense	-	-	1,826,000	-	-	1,826,000
Restricted stock-based compensation expense	-	-	98,000	-	-	98,000
Issuance of common stock in connection with restricted share awards	376,625	4,000	(4,000)	-	-	-
Common stock issued for cash under open market sale agreement	1,428,273	15,000	4,325,000	-	-	4,340,000
Net loss	-	-	-	(17,372,000)	-	(17,372,000)
Balance, September 30, 2019	51,054,395	$ 511,000	$ 565,580,000	$ (470,039,000)	$ -	$ 96,052,000
For the nine months ended September 30, 2020						
Balance, December 31, 2019	83,622,135	$ 836,000	$ 664,064,000	$ (486,470,000)	$ -	$ 178,430,000
Stock option-based compensation expense	-	-	4,083,000	-	-	4,083,000
Restricted stock-based compensation expense	-	-	812,000	-	-	812,000
Common stock issued for cash exercise of options	75,793	1,000	174,000	-	-	175,000
Issuance of common stock in connection with restricted share awards, net of cancellations	818,233	8,000	(8,000)	-	-	-
Net loss	-	-	-	(68,406,000)	-	(68,406,000)
Other comprehensive income	-	-	-	-	17,000	17,000
Balance, September 30, 2020	84,516,161	$ 845,000	$ 669,125,000	$ (554,876,000)	$ 17,000	$ 115,111,000
For the nine months ended September 30, 2019						
Balance, December 31, 2018	47,944,486	$ 479,000	$ 543,754,000	$ (410,188,000)	$ -	$ 134,045,000
Stock option-based compensation expense	-	-	5,607,000	-	-	5,607,000
Restricted stock-based compensation expense	-	-	348,000	-	-	348,000
Common stock issued for cash exercise of options	96,334	1,000	412,000	-	-	413,000
Common stock issued for cash under open market sale agreement	3,086,950	32,000	16,930,000	-	-	16,962,000
Issuance of common stock in connection with restricted share awards	376,625	4,000	(4,000)	-	-	-
Shares returned in connection with arbitration ruling on licensing agreement	(450,000)	(5,000)	(1,467,000)	-	-	(1,472,000)
Net loss	-	-	-	(59,851,000)	-	(59,851,000)
Balance, September 30, 2019	51,054,395	$ 511,000	$ 565,580,000	$ (470,039,000)	$ -	$ 96,052,000

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

Abeona Therapeutics Inc. and Subsidiaries

Condensed Consolidated Statements of Cash Flows
(Unaudited)

	2020	2019
	For the nine months ended September 30,	
	2020	2019
Cash flows from operating activities:		
Net loss	$ (68,406,000)	$ (59,851,000)
Adjustments to reconcile net loss to cash used in operating activities:		
Non-cash licensed technology impairment charge	32,916,000	-
Depreciation and amortization	3,746,000	5,747,000
Stock option-based compensation expense	4,083,000	5,607,000
Restricted stock-based compensation expense	812,000	348,000
Non-cash interest expense	600,000	-
Accretion and interest on short-term investments	(237,000)	(1,090,000)
Accretion of right-of-use lease assets	752,000	619,000
Non-cash loss on arbitration ruling on licensing agreement	-	367,000
Change in operating assets and liabilities:		
Receivables	(7,000,000)	66,000
Prepaid expenses and other current assets	2,443,000	2,148,000
Other assets	(62,000)	4,000
Accounts payable, accrued expenses and lease liabilities	(5,183,000)	(2,539,000)
Change in payable to licensor	2,127,000	-
Net cash used in operating activities	(33,409,000)	(48,574,000)
Cash flows from investing activities:		
Capital expenditures	(1,303,000)	(6,187,000)
Acquisition of licensed technology	-	(199,000)
Purchases of short-term investments	(139,230,000)	-
Proceeds from maturities of short-term investments	51,037,000	67,308,000
Net cash (used in)/provided by investing activities	(89,496,000)	60,922,000
Cash flows from financing activities:		
Proceeds from loan payable	1,758,000	-
Proceeds from open market sales of common stock	-	16,962,000
Proceeds from exercise of stock options	175,000	413,000
Net cash provided by financing activities	1,933,000	17,375,000
Net (decrease)/increase in cash, cash equivalents and restricted cash	(120,972,000)	29,723,000
Cash, cash equivalents and restricted cash at beginning of period	130,368,000	19,310,000
Cash, cash equivalents and restricted cash at end of period	$ 9,396,000	$ 49,033,000
Supplemental cash flow information:		
Cash and cash equivalents	$ 8,424,000	$ 47,923,000
Restricted cash	972,000	1,110,000
Total cash, cash equivalents and restricted cash	$ 9,396,000	$ 49,033,000
Shares returned in connection with arbitration ruling on licensing agreement	$ -	$ 1,472,000
Cash paid for interest	$ -	$ -
Cash paid for taxes	$ -	$ -

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

NOTE 1 – NATURE OF OPERATIONS AND SIGNIFICANT ACCOUNTING POLICIES

Background

Abeona Therapeutics Inc., a Delaware corporation (together with our subsidiaries, "we," "our," "Abeona" or the "Company"), is a clinical-stage biopharmaceutical company developing gene and cell therapies for life-threatening rare genetic diseases. Our lead clinical programs consist of: (i) EB-101, an autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa ("RDEB"), (ii) ABO-102, an adeno-associated virus ("AAV")-based gene therapy for Sanfilippo syndrome type A ("MPS IIIA"), and (iii) ABO-101, an AAV-based gene therapy for Sanfilippo syndrome type B ("MPS IIIB"). We have additional AAV-based gene therapies in various developmental stages designed to treat the CLN3 form of Batten Disease, cystic fibrosis and retinal diseases. In addition, we are developing next-generation AAV-based gene therapies using the novel AIM™ capsid platform that we have exclusively licensed from the University of North Carolina at Chapel Hill, and internal AAV vector research programs. Our efforts have been principally devoted to research and development, resulting in significant losses.

Basis of Presentation

The condensed consolidated balance sheet as of September 30, 2020 and the condensed consolidated statements of operations and comprehensive loss, stockholders' equity and cash flows for the three and nine months ended September 30, 2020 and 2019 were prepared by management without audit. In the opinion of management, all adjustments, consisting only of normal recurring adjustments, except as otherwise disclosed, necessary for the fair presentation of the financial position, results of operations, and changes in financial position for such periods, have been made.

Certain information and footnote disclosures normally included in financial statements prepared in accordance with accounting principles generally accepted in the United States of America ("U.S. GAAP") have been condensed or omitted. These interim financial statements should be read in conjunction with the consolidated financial statements and notes thereto included in our Form 10-K for the year ended December 31, 2019. The results of operations for the period ended September 30, 2020 are not necessarily indicative of the operating results that may be expected for a full year. The condensed consolidated balance sheet as of December 31, 2019 contains financial information taken from the audited Abeona consolidated financial statements as of that date.

Uses and Sources of Liquidity

The financial statements have been prepared on the going concern basis, which assumes the Company will have sufficient cash to pay its operating expenses, as and when they become payable, for a period of at least 12 months from the date the financial report was issued.

As of September 30, 2020, we had cash, cash equivalents, receivables and short-term investments of $103.9 million and net assets of $115.1 million. For the nine months ended September 30, 2020, we had cash outflows from operations of $33.4 million. We have not generated any significant product revenues and have not achieved profitable operations. There is no assurance that profitable operations will ever be achieved, and, if achieved, could be sustained on a continuing basis. In addition, development activities, clinical and nonclinical testing, and commercialization of our products will require significant additional financing.

We are subject to a number of risks similar to other life science companies, including, but not limited to, risks related to the successful discovery and development of product candidates, obtaining the necessary regulatory approval to market our product candidates, raising additional capital to continue to fund our operations, development of competing drugs and therapies, protection of proprietary technology and market acceptance of our products. As a result of these and other risks and the related uncertainties, there can be no assurance of our future success.

Based upon our current operating plans, we believe that we have sufficient resources to fund operations through the next 12 months with our existing cash, cash equivalents and short-term investments. We will need to secure additional funding in the future, to carry out all our planned research and development activities. If we are unable to obtain additional financing or generate license or product revenue, the lack of liquidity and sufficient capital resources could have a material adverse effect on our future prospects. In October 2020, the Company announced that it had retained Jefferies LLC as its financial advisor to assist with the review of strategic options focused on advancing the Company's mission and maximizing stockholder value. In an effort to unlock potential additional value, the Company initiated this formal process to explore a broad range of strategic alternatives, including but not limited to the partnering of its various clinical and pre-clinical programs, or a sale or merger of the Company.

Use of Estimates

The preparation of consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amount of assets and disclosure of contingent assets and liabilities at the date of the consolidated financial statements and the reported amounts of revenue and expenses during the reported period. Actual results could differ from these estimates and assumptions.

Cash and Cash Equivalents

We consider all highly liquid investments with a maturity of three months or less when purchased to be cash equivalents. We maintain deposits primarily in financial institutions, which may at times exceed amounts covered by insurance provided by the U.S. Federal Deposit Insurance Corporation ("FDIC"). We have not experienced any losses related to amounts in excess of FDIC limits.

Receivables

Receivables are reported at net realizable value. We continually evaluate the creditworthiness of our customers and their financial condition and generally do not require collateral. The need for an allowance for doubtful accounts is based upon reviews of specific customer balances, historic losses, and general economic conditions. As of September 30, 2020, no allowance was recorded as the receivables are considered collectible. There were no receivables outstanding as of December 31, 2019.

Short-term Investments

Short-term investments consist of investments in U.S. government, U.S. agency and U.S. treasury securities. We determine the appropriate classification of the securities at the time they are acquired and evaluate the appropriateness of such classifications at each balance sheet date. We classify our short-term investments as available-for-sale pursuant to Accounting Standards Codification ("ASC") 320, *Investments – Debt and Equity Securities.* Investments classified as current have maturities of less than one year. We review our short-term investments for other-than-temporary impairment whenever the fair value of a marketable security is less than the amortized cost and evidence indicates that a short-term investment's carrying amount is not recoverable within a reasonable period of time.

Leases

We account for leases in accordance with ASC 842, *Leases*. Right-of-use lease assets represent our right to use an underlying asset for the lease term and lease liabilities represent our obligation to make lease payments arising from the lease. The measurement of lease liabilities is based on the present value of future lease payments over the lease term. As our leases do not provide an implicit rate, we use our incremental borrowing rate based on the information available at the lease commencement date in determining the present value of future lease payments. The right-of-use asset is based on the measurement of the lease liability and includes any lease payments made prior to or on lease commencement and excludes lease incentives and initial direct costs incurred, as applicable. Rent expense for our operating leases is recognized on a straight-line basis over the lease term. We do not have any leases classified as finance leases.

Our leases do not have significant rent escalation, holidays, concessions, material residual value guarantees, material restrictive covenants or contingent rent provisions. Our leases include both lease (e.g., fixed payments including rent, taxes, and insurance costs) and non-lease components (e.g., common-area or other maintenance costs), which are accounted for as a single lease component as we have elected the practical expedient to group lease and non-lease components for all leases.

Most leases include one or more options to renew. The exercise of lease renewal options is typically at our sole discretion; therefore, the majority of renewals to extend the lease terms are not included in our right-of-use assets and lease liabilities as they are not reasonably certain of exercise. We regularly evaluate the renewal options and when they are reasonably certain of exercise, we include the renewal period in our lease term.

Additional information and disclosures required under ASC 842 is included in Note 8.

Restricted Cash

In November 2016, the Financial Accounting Standards Board issued Accounting Standards Update ("ASU") 2016-18, *Statement of Cash Flows (Topic 230): Restricted Cash*, requiring restricted cash and restricted cash equivalents to be included with cash and cash equivalents on the statement of cash flows when reconciling the beginning-of-period and end-of-period total amounts shown on the statement of cash flows. We adopted this standard during the first quarter of 2018. Restricted cash is now included as a component of cash, cash equivalents and restricted cash on our consolidated statements of cash flows. Restricted cash is recorded within other assets and restricted cash in the accompanying consolidated balance sheets.

Revenue Recognition

We account for contracts with customers in accordance with ASC 606, *Revenue from Contracts with Customers.* ASC 606 applies to all contracts with customers, except for contracts that are within the scope of other standards. Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that an entity determines are within the scope of ASC 606, the entity 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. Additional information and disclosures required under ASC 606 are included in Note 6.

Loss Per Common Share

We have presented basic and diluted loss per common share on the statement of operations. Basic and diluted net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock and shares underlying "pre-funded" warrants outstanding during the period. At September 30, 2020, 84,516,161 shares of common stock were outstanding. The "pre-funded" warrants are included in the computation of basic net loss per share as the exercise price is negligible and they are fully vested and exercisable.

There were 9,017,055 "pre-funded" warrants included in the computation of basic net loss per share for the three and nine months ended September 30, 2020. There were no "pre-funded" warrants included in the computation of basic net loss per share for the three and nine months ended September 30, 2019. In October 2020, all of the pre-funded warrants were exercised and converted into shares of common stock.

We do not include the potential impact of dilutive securities in diluted net loss per share, as the impact of these items is anti-dilutive. Potential dilutive securities result from outstanding stock options and "non-pre-funded" warrants. We did not include the following potentially dilutive securities in the computation of diluted net loss per common share during the periods presented:

	For the three months ended September 30,		For the nine months ended September 30,	
	2020	2019	2020	2019
"Non-pre-funded" warrants	-	1,820,686	-	1,820,686
Stock options	6,431,183	6,697,980	6,431,183	6,697,980
Total	6,431,183	8,518,666	6,431,183	8,518,666

NOTE 2 – SHORT-TERM INVESTMENTS

The following table summarizes the carrying value of the available-for-sale debt securities held:

Description	September 30, 2020	December 31, 2019
U.S. government and agency securities and treasuries	$ 88,447,000	$ -

The amortized cost of the available-for-sale debt securities, which is adjusted for amortization of premiums and accretion of discounts to maturity, was $88,430,000 as of September 30, 2020. There were no significant realized gains or losses recognized on the sale or maturity of available-for-sale debt securities during the nine months ended September 30, 2020 or 2019.

NOTE 3 – LICENSED TECHNOLOGY

On November 4, 2018, we entered into a license agreement with REGENXBIO Inc. ("REGENXBIO") to obtain rights to an exclusive worldwide license (subject to certain non-exclusive rights previously granted for MPS IIIA), with rights to sublicense, to REGENXBIO's NAV AAV9 vector for gene therapies for treating MPS IIIA, MPS IIIB, CLN1 Disease and CLN3 Disease. Consideration for the rights granted under the original agreement included fees totaling $180 million and a running royalty on net sales, including: (i) an initial fee of $20 million, $10 million of which was due to REGENXBIO shortly after the effective date of the agreement, and $10 million of which was to be due on the first anniversary of the effective date of the agreement in November 2019, (ii) annual fees totaling up to $100 million, payable in $20 million annual installments beginning on the second anniversary of the effective date (the first of which was to remain payable if the agreement were terminated before the second anniversary in November 2020), (iii) sales milestone payments totaling $60 million, and (iv) royalties payable in the low double digits to low teens on net sales of products covered under the agreement. The license was being amortized over the life of the patent of eight years. On November 1, 2019, we entered into an amendment of the original license agreement. The amended agreement replaced the $10 million payment due on November 4, 2019 with a $3 million payment due on November 4, 2019 and an additional $8 million payment (which included $1 million of interest) that would have been due no later than April 1, 2020. That $8 million payment had been scheduled to be paid by April 1, 2020 and the $20 million that had been due to be paid on November 4, 2020, and both were recorded as payable to licensor on the consolidated balance sheet. As discussed below, the Company has disputed that it is responsible for the $8 million and $20 million payments, and those payments are the subject of a current arbitration between the Company and REGENXBIO, as further discussed below.

Prior to the April 1, 2020 deadline, we engaged REGENXBIO in discussions in an attempt to renegotiate the financial terms of the agreement, but we were unable to reach a mutual understanding that we believed would have been favorable for the Company or our programs, and we did not make the $8 million payment due by April 1, 2020. On April 17, 2020, REGENXBIO sent us a written demand for the $8 million fee, payable within a 15-day cure period after receipt of the demand letter. The license terminated on May 2, 2020, when the 15-day period expired. There were no penalties for early termination of the license. On May 25, 2020, we filed an arbitration claim with the American Arbitration Association alleging that REGENXBIO materially breached the license agreement prior to termination and seeking, among other things, a declaration that as a result of REGENXBIO's material breach, we are not responsible for payments totaling $28 million (which would otherwise have been due in 2020) plus accrued interest ($2.1 million as of September 30, 2020). REGENXBIO disputes our arbitration claim and has filed a counterclaim seeking payment of the $28 million plus interest, which REGENXBIO argues remains due. Additional information is included in Note 8.

As of March 31, 2020, we considered the status of our discussions with REGENXBIO as a potential indicator of impairment in accordance with ASC 360-10-35-21. Since our impairment testing indicated that the carrying value of the license agreement exceeded its fair value, we recorded a $32.9 million non-cash impairment charge in the three months ended March 31, 2020.

On May 15, 2015, we acquired Abeona Therapeutics LLC, which had an exclusive license through Nationwide Children's Hospital to the AB-101 and AB-102 patent portfolios for developing treatments for patients with Sanfilippo Syndrome Type A and Type B. The license is amortized over the life of the license of 20 years.

Licensed technology consists of the following:

	September 30, 2020	December 31, 2019
Licensed technology	$ 2,606,000	$ 42,606,000
Less accumulated amortization	725,000	6,428,000
Licensed technology, net	$ 1,881,000	$ 36,178,000

The aggregate estimated amortization expense for intangible assets remaining as of September 30, 2020 is as follows:

2020, remainder	$ 43,000
2021	174,000
2022	174,000
2023	174,000
2024	174,000
Thereafter	1,142,000
Total	$ 1,881,000

Amortization of licensed technology was $43,000 and $1,381,000 for the three and nine months ended September 30, 2020, respectively, and $1,293,000 and $3,931,000 for the three and nine months ended September 30, 2019, respectively.

NOTE 4 – FAIR VALUE MEASUREMENTS

We calculate the fair value of our assets and liabilities that qualify as financial instruments and include additional information in the notes to the consolidated financial statements when the fair value is different than the carrying value of these financial instruments. The estimated fair value of receivables, prepaid expenses, other assets, accounts payable, accrued expenses, payable to licensor and deferred revenue approximate their carrying amounts due to the relatively short maturity of these instruments.

U.S. GAAP defines fair value as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants at the measurement date. This guidance establishes a three-level fair value hierarchy that prioritizes the inputs used to measure fair value. The hierarchy requires entities to maximize the use of observable inputs and minimize the use of unobservable inputs. The three levels of inputs used to measure fair value are as follows:

- Level 1 – Quoted prices in active markets for identical assets or liabilities.
- Level 2 – Observable inputs other than quoted prices included in Level 1, such as quoted prices for similar assets and liabilities in active markets; quoted prices for identical or similar assets and liabilities in markets that are not active; or other inputs that are observable or can be corroborated by observable market data.
- Level 3 – Unobservable inputs that are supported by little or no market activity and that are significant to the fair value of the assets and liabilities. This includes certain pricing models, discounted cash flow methodologies and similar valuation techniques that use significant unobservable inputs.

The guidance requires an entity to maximize the use of observable inputs and minimize the use of unobservable inputs when measuring fair value.

We have segregated all financial assets and liabilities that are measured at fair value on a recurring basis (at least annually) into the most appropriate level within the fair value hierarchy based on the inputs used to determine the fair value at the measurement date in the table below.

Financial assets and liabilities measured at fair value on a recurring and non-recurring basis as of September 30, 2020 and December 31, 2019 are summarized below:

Description	September 30, 2020	Level 1	Level 2	Level 3
Recurring				
Assets:				
Short-term investments	$ 88,447,000	$ -	$ 88,447,000	$ -
Non-recurring				
Assets:				
Licensed technology, net	$ 1,881,000	$ -	$ -	$ 1,881,000
Goodwill	32,466,000	-	-	32,466,000

Description	December 31, 2019	Level 1	Level 2	Level 3
Non-recurring				
Assets:				
Licensed technology, net	$ 36,178,000	$ -	$ -	$ 36,178,000
Goodwill	32,466,000	-	-	32,466,000

NOTE 5 – LOAN PAYABLE

On May 2, 2020, we received loan proceeds in the amount of approximately $1.8 million (the "PPP Loan") under the Paycheck Protection Program ("PPP"). The PPP was established under the Coronavirus Aid, Relief and Economic Security Act, as amended ("CARES Act") and is administered by the U.S. Small Business Administration ("SBA"). Under the terms of the CARES Act, PPP loan recipients can apply for loan forgiveness. The potential loan forgiveness for all or a portion of PPP loans is determined, subject to limitations, based on the use of loan proceeds over the 24 weeks after the loan proceeds are disbursed for payment of payroll costs and any payments of mortgage interest, rent, and utilities. The amount of loan forgiveness will be reduced if PPP loan recipients terminate employees or reduce salaries during the covered period. The unforgiven portion of our PPP Loan, if any, is payable over two years at an interest rate of 1%, with a deferral of payments for the first six months, beginning on May 2, 2020. We believe that we have used the proceeds from our PPP Loan for purposes consistent with the PPP. While we currently believe that our use of the loan proceeds will meet the conditions for forgiveness of our PPP Loan, there can be no assurance that forgiveness for any portion of the PPP Loan will be obtained.

NOTE 6 – REVENUE FROM CONTRACTS WITH CUSTOMERS

On August 14, 2020, we entered into sublicense and inventory purchase agreements with Taysha Gene Therapies ("Taysha") relating to a potential gene therapy for CLN1 disease. Under the sublicense agreement, Taysha received worldwide exclusive rights to intellectual property and know-how relating to the research, development and manufacture of the potential gene therapy, which we had referred to as ABO-202. Under the inventory purchase agreement, we sold to Taysha certain inventory and other items related to ABO-202.

We assessed these contracts at contract inception and determined that, under ASC 606, the two contracts would be combined and accounted for a single contract, with a single performance obligation. We assessed the nature of the promised license to determine whether the license has significant stand-alone functionality and evaluated whether such functionality can be retained without ongoing activities of the entity and determined that the license has significant stand-alone functionality. Furthermore, we have no ongoing activities associated with the license to support or maintain the license's utility. Based on this, we determined that the pattern of transfer of control of the license to the customer was at a point in time.

The transaction price of the contract includes (i) $7.0 million of fixed consideration, (ii) up to $26.0 million of variable consideration in the form of event-based milestone payments, (iii) up to $30.0 million of variable consideration in the form of sales-based milestone payments, and (iv) other royalty-based payments based on net sales. The event-based milestone payments are based on certain development and regulatory events occurring. We evaluated whether the milestone conditions have been achieved and if it is probable that a significant revenue reversal would not occur before recognizing the associated revenue. We determined that these milestone payments are not within our control or the licensee's control, such as regulatory approvals, and are not considered probable of being achieved until those approvals are received. Accordingly, we have fully constrained the $26.0 million of event-based milestone payments until such time that it is probable that significant revenue reversal would not occur. The sales-based milestone payments and other royalty-based payments are based on a level of sales for which the license is deemed to be the predominant item to which the royalties relate. We will recognize revenue for these payments at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied or partially satisfied. To date, we have not recognized any sales-based or royalty revenue resulting from this licensing arrangement.

Under this arrangement, we recognized $7.0 million of revenue during the three months ended September 30, 2020, which amount related solely to fixed consideration. In addition, we have $7.0 million of related accounts receivable at September 30, 2020; this receivable was paid by Taysha in October 2020. We do not have any contract assets or contract liabilities as a result of this transaction.

NOTE 7 – STOCK-BASED COMPENSATION

Stock Options:

The following table summarizes stock option-based compensation for the three and nine months ended September 30, 2020 and 2019:

	For the three months ended September 30,		For the nine months ended September 30,	
	2020	2019	2020	2019
Research and development	$ 765,000	$ 972,000	$ 2,380,000	$ 3,013,000
General and administrative	484,000	854,000	1,703,000	2,594,000
Stock option-based compensation expense included in operating expense	1,249,000	1,826,000	4,083,000	5,607,000
Total stock option-based compensation expense	1,249,000	1,826,000	4,083,000	5,607,000
Tax benefit	-	-	-	-
Stock option-based compensation expense, net of tax	$ 1,249,000	$ 1,826,000	$ 4,083,000	$ 5,607,000

We estimate the fair value of each option award on the date of grant using the Black-Scholes option valuation model. We then recognize the grant date fair value of each option as compensation expense ratably using the straight-line attribution method over the service period (generally the vesting period). The Black-Scholes model incorporates the following assumptions:

- Expected volatility – we estimate the volatility of our share price at the date of grant using a "look-back" period which coincides with the expected term, defined below. We believe using a "look-back" period which coincides with the expected term is the most appropriate measure for determining expected volatility.
- Expected term – we estimate the expected term using the "simplified" method, as outlined in Staff Accounting Bulletin No. 107, "Share-Based Payment."
- Risk-free interest rate – we estimate the risk-free interest rate using the U.S. Treasury yield curve for periods equal to the expected term of the options in effect at the time of grant.
- Dividends – we use an expected dividend yield of zero because we have not declared or paid a cash dividend, nor do we have any plans to declare a dividend.

We used the following weighted-average assumptions to estimate the fair value of the options granted for the periods indicated:

	For the three months ended September 30,		For the nine months ended September 30,	
	2020	2019	2020	2019
Expected volatility	110%	103%	111%	108%
Expected term	6.25 years	6.25 years	6.25 years	5.09 years
Risk-free interest rate	0.16%	1.83%	0.29%	2.21%
Expected dividend yield	0%	0%	0%	0%

The following table summarizes the options granted for the periods indicated:

	For the three months ended September 30,		For the nine months ended September 30,	
	2020	2019	2020	2019
Options granted	342,100	105,600	3,415,146	1,490,490
Weighted-average:				
Exercise price	$ 3.08	$ 2.58	$ 2.38	$ 6.53
Grant date fair value	$ 2.56	$ 2.09	$ 1.99	$ 5.14

Restricted Common Stock:

The following table summarizes restricted common stock compensation expense for the three and nine months ended September 30, 2020 and 2019:

	For the three months ended September 30,		For the nine months ended September 30,	
	2020	2019	2020	2019
Research and development	$ 89,000	$ 66,000	$ 561,000	$ 66,000
General and administrative	72,000	32,000	251,000	282,000
Restricted stock-based compensation expense included in operating expense	161,000	98,000	812,000	348,000
Total restricted stock-based compensation expense	161,000	98,000	812,000	348,000
Tax benefit	-	-	-	-
Restricted stock-based compensation expense, net of tax	$ 161,000	$ 98,000	$ 812,000	$ 348,000

We granted 818,233 shares of restricted common stock, net of cancellations, during the nine months ended September 30, 2020. There were 265,080 shares of restricted common stock canceled during the three months ended September 30, 2020. We granted 376,625 shares of restricted common stock during the three and nine months ended September 30, 2019.

NOTE 8 – COMMITMENTS AND CONTINGENCIES

Arbitration Proceeding

We are currently engaged in an arbitration proceeding with REGENXBIO regarding the former license agreement between the parties relating to use of the AAV9 capsid in our MPS IIIA, MPS IIIB, CLN1 (which has now been sold to Taysha Gene Therapies, as discussed in Note 6 above), and CLN3 programs. The license terminated on May 2, 2020, and on May 25, 2020, we filed an arbitration claim with the American Arbitration Association alleging that REGENXBIO materially breached the license agreement prior to termination and seeking, among other things, a declaration that as a result of REGENXBIO's material breach, we are not responsible for payments totaling $28 million (which would otherwise have been due in 2020) plus accrued interest ($2.1 million as of September 30, 2020). REGENXBIO disputes our arbitration claim and has filed a counterclaim seeking payment of these amounts. Under the current schedule, the arbitration is expected to be completed in the first half of 2021.

Commitment with Contract Manufacturer

We engaged a contract manufacturer to assist us with developing and defining the processes necessary to manufacture our RDEB product candidate and had a remaining commitment of $6.3 million at March 31, 2020. During the second quarter of 2020, we cancelled the remaining stages of work with the contract manufacturer. We have no remaining commitment at September 30, 2020.

Operating Leases

We lease space under operating leases for manufacturing and laboratory facilities and administrative offices in Cleveland, Ohio, as well as administrative offices in New York, New York. We also lease office space in Madrid, Spain as well as certain office equipment under operating leases, which have a non-cancelable lease term of less than one year and, therefore, we have elected the practical expedient to exclude these short-term leases from our right-of-use assets and lease liabilities.

Components of lease cost are as follows:

	For the three months ended September 30,		For the nine months ended September 30,	
	2020	2019	2020	2019
Operating lease cost	$ 434,000	$ 434,000	$ 1,302,000	$ 1,157,000
Variable lease cost	$ 81,000	$ 82,000	$ 256,000	$ 241,000
Short-term lease cost	$ 19,000	$ 32,000	$ 43,000	$ 113,000

The following table presents information about the amount and timing of cash flows arising from operating leases as of September 30, 2020:

Maturity of lease liabilities:		
2020, remainder	$	425,000
2021		1,713,000
2022		1,727,000
2023		1,741,000
2024		1,781,000
Thereafter		1,885,000
Total undiscounted operating lease payments		9,272,000
Less: imputed interest		2,046,000
Present value of operating lease liabilities	$	7,226,000
Balance sheet classification:		
Current portion of lease liability	$	1,709,000
Long-term lease liability		5,517,000
Total operating lease liabilities	$	7,226,000
Other information:		
Weighted-average remaining lease term for operating leases		64 months
Weighted-average discount rate for operating leases		9.6%

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

OVERVIEW

Abeona Therapeutics Inc., a Delaware corporation (together with our subsidiaries, "we," "our," "Abeona" or the "Company"), is a clinical-stage biopharmaceutical company developing gene and cell therapies for life-threatening rare genetic diseases. Our lead clinical programs consist of: (i) EB-101, an autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa ("RDEB"), (ii) ABO-102, an adeno-associated virus ("AAV")-based gene therapy for Sanfilippo syndrome type A ("MPS IIIA"), and (iii) ABO-101, an AAV-based gene therapy for Sanfilippo syndrome type B ("MPS IIIB"). We have additional AAV-based gene therapies in various developmental stages designed to treat the CLN3 form of Batten Disease, cystic fibrosis and retinal diseases. Moreover, we are developing next-generation AAV-based gene therapies using the novel AIM™ capsid platform that we have exclusively licensed from the University of North Carolina at Chapel Hill, and internal AAV vector research programs. We believe our product candidates are eligible for orphan drug designation, breakthrough therapy designation, or other expedited review processes in the U.S., Europe or Japan. Our pipeline includes four product candidates for which we hold several U.S. and EU regulatory designations:

Our robust and diverse pipeline features early-stage and late-stage candidates with the potential to transform the treatment of devastating genetic diseases, and we are conducting clinical trials in the U.S. and abroad.

Our Mission and Strategy

Abeona is at the forefront of gene and cell therapy research and development. We are a fully integrated company featuring innovative research, therapies in clinical development, in-house manufacturing facilities, a robust pipeline, and scientific, clinical, and commercial leadership. We see our mission as working together to create, develop, manufacture and deliver gene and cell therapies to patients impacted by serious diseases. We partner with leading academic researchers, patient advocacy organizations and caregivers to bring therapies that address the underlying cause of a broad spectrum of rare genetic diseases where no effective treatment options exist today.

Since our last fiscal year, we have made significant progress toward fulfilling our goal of harnessing the promise of genetic medicine to transform the lives of people impacted by serious diseases and redefine the standard of care through gene and cell therapies. Our strategy to achieve this goal consists of:

Advancing our Clinical Gene and Cell Therapy Programs and Research and Development with a Focus on Rare and Orphan Diseases.

We have three programs in clinical development—EB-101, ABO-101 and ABO-102—and a pipeline of additional earlier stage programs. Through our gene and cell therapy expertise in research and development, we believe we are positioned to rapidly introduce novel therapeutics to transform the standard of care in devastating diseases and establish our leadership position in the field.

Applying Novel Next Generation AIM™ Capsid Technology to Develop New In-Vivo Gene Therapies.

We are researching and developing next-generation AAV-based gene therapy using novel capsids developed from the AIM™ Capsid Technology Platform and additional Company-invented AAV capsids. We aim to continue to develop chimeric AAV capsids capable of improved tissue targeting for various indications and potentially evading immunity to wildtype AAV vectors.

Establishing Leadership Position in Commercial-Scale Gene and Cell-Therapy Manufacturing.

We established current Good Manufacturing Practice ("cGMP"), clinical-scale manufacturing capabilities for gene-corrected cell therapy and AAV-based gene therapies in our state-of-the-art Cleveland, OH facility. We believe that our platform provides us with distinct advantages, including flexibility, scalability, reliability, and the potential for reduced development risk, cost, and faster times to market. We have focused on establishing internal Chemistry, Manufacturing and Controls ("CMC") capabilities that drive value for our organization through process development, assay development and manufacturing. We have also deployed robust quality systems governing all aspects of product lifecycle from preclinical through commercial stage.

Establishing Additional Gene and Cell Therapy Franchises and Adjacencies through In-Licensing and Strategic Partnerships.

We seek to be the partner of choice in rare disease and have closely collaborated with leading academic institutions, key opinion leaders, patient foundations and industry partners to generate novel intellectual property, accelerate research and development, and understand the needs of patients and their families.

Maintaining and Growing IP Portfolio.

We strive to have a leading intellectual property portfolio. To that end, we seek patent rights for various aspects of our programs, including vector engineering and construct design, product packaging, production processes, and all features of our clinical products including composition of matter and method of administration and delivery. We expect to continue to expand our intellectual property portfolio by aggressively seeking patent rights for promising aspects of our product engine and product candidates.

IMPACT OF COVID-19 PANDEMIC ON OUR BUSINESS

We continue to assess the impact of the COVID-19 pandemic on our business and take appropriate actions to manage our spending activities and preserve our cash resources. We continue to actively monitor the situation and may take further actions to adjust our business operations that we determine are in the best interests of our patients, employees, suppliers and stockholders. While we are unable to determine or predict the extent, duration or scope of the overall impact the COVID-19 pandemic will have on our business, operations, financial condition or liquidity, we believe it is important to keep our stakeholders informed about how our response to COVID-19 is progressing and how our operations and financial condition may change.

Clinical Program Activities

We remain committed to advancing our clinical programs and have implemented measures to minimize disruption. We also are regularly reassessing plans along with associated processes and policies to ensure our patients and employees are safe, and that continuity in our operations remains.

All current clinical trial sites are now active. We are also providing virtual and remote follow-up to ensure compliance with safety oversight. In June 2020, we resumed patient enrollment in our Phase III VIITAL™ study of EB-101 after the study was paused in March 2020 to ensure the safety of study participants and site staff during the pandemic. The ongoing Phase I/II clinical trials of our investigational AAV-based gene therapies for MPS IIIA and IIIB (ABO-102 and ABO-101, respectively) have continued to treat patients, with additional enrollment expected in those programs.

Manufacturing Activities

Operations at our Cleveland manufacturing facility were significantly scaled back from March 2020 until early June 2020 to ensure the safety of employees and those around them, and to accommodate reduced manufacturing and clinical development activities. We had paused our manufacturing activities for EB-101 clinical material, pending patient enrollment, as well as our AAV manufacturing and process development activities. During this pause period, we took the opportunity to complete maintenance and monitoring projects.

In June 2020, we resumed our EB-101 manufacturing activities, including process development for the internal production of retrovirus as well as our AAV process development and manufacturing activities.

Business Operations

Many of the additional protective measures we instituted during the first quarter in response to the COVID-19 pandemic remain in place, and we continue to regularly assess and improve our safety practices and policies.

The extent of the impact of the COVID-19 pandemic on our business, operations, and clinical trials continues to evolve and will depend on certain developments, including: (i) the duration of the declared health emergencies; (ii) future actions taken by governmental authorities and regulators with respect to the pandemic, including reinstituting state and local lockdowns; (iii) the impact on our partners, collaborators, and suppliers; and (iv) actions being taken by us in response to this crisis. We remain dedicated to communicating regularly and openly with our stakeholders as more information becomes available, including updates on material changes to prior guidance as we continue to follow applicable government, regulatory and institutional guidelines.

RESULTS OF OPERATIONS FOR THREE MONTHS ENDED SEPTEMBER 30, 2020 COMPARED TO THREE MONTHS ENDED SEPTEMBER 30, 2019

License and other revenues for the third quarter of 2020 were $7.0 million, as compared to nil for the same period of 2019. The increase in revenue was due to the sublicense and inventory purchase agreements we entered into with Taysha Gene Therapies ("Taysha") in August 2020 for ABO-202, an AAV gene therapy for CLN1 disease (also known as infantile Batten disease). The agreements grant to Taysha worldwide exclusive rights to intellectual property developed by scientists at the University of North Carolina at Chapel Hill and us, and our know-how relating to the research, development and manufacture of the gene therapy.

Total research and development spending for the third quarter of 2020 was $8.0 million, as compared to $10.9 million for the same period of 2019, a decrease of $2.9 million. The decrease in expenses was primarily due to:

- decreased clinical and development work for our gene and cell therapy product candidates ($2.6 million), due to scaled back manufacturing, clinical and non-clinical development activities resulting from the effects of the COVID-19 pandemic, as well as cost savings from the decision to internally manufacture retrovirus for the EB-101 program; and
- decreased salary and related costs ($0.3 million).

Total general and administrative expenses were $4.4 million for the third quarter of 2020, as compared to $4.7 million for the same period of 2019, a decrease of $0.3 million. The decrease in expenses was primarily due to:

- decreased salary and related costs ($0.3 million); and
- decreases in net other general and administrative expenses ($0.1 million); partially offset by
- increased professional fees ($0.1 million).

Depreciation and amortization was $0.8 million for the third quarter of 2020, as compared to $2.0 million for the same period in 2019, a decrease of $1.3 million. The decrease was driven primarily by decreased amortization expense on licensed technology due to the write-off of the REGENXBIO licensed technology in the first quarter of 2020.

Interest and miscellaneous income was $0.3 million for the third quarter of 2020, as compared to $0.3 million of the same period in 2019.

Interest and other expense was $1.3 million for the third quarter of 2020, as compared to nil for the same period of 2019. The increase results primarily from accrued interest on the amounts that we may owe to REGENXBIO under the prior license agreement, which amount is subject to the arbitration discussed in Note 3 of the Notes to Condensed Consolidated Financial Statements in Part I, Item 1. As described in more detail in Note 3, we have filed an arbitration claim alleging that REGENXBIO materially breached the license agreement and seeking, among other things, a declaration that we are not responsible for such payments.

Net loss for the third quarter of 2020 was $7.2 million, or a $0.08 basic and diluted loss per common share as compared to a net loss of $17.4 million, or a $0.35 basic and diluted loss per common share, for the same period in 2019. The decrease in the net loss resulted primarily from increased license and other revenues along with decreased clinical and development expenses and scaled back activities in manufacturing, clinical and non-clinical development arising from the effects of the COVID-19 pandemic.

RESULTS OF OPERATIONS FOR NINE MONTHS ENDED SEPTEMBER 30, 2020 COMPARED TO NINE MONTHS ENDED SEPTEMBER 30, 2019

License and other revenues for the first nine months of 2020 were $7.0 million, as compared to nil for the same period of 2019. The increase in revenue was due to the aforementioned sublicense and inventory purchase agreements we entered into with Taysha in August 2020.

Total research and development spending for the first nine months of 2020 was $20.9 million, as compared to $39.0 million for the same period of 2019, a decrease of $18.1 million. The decrease in expenses was primarily due to:

- decreased clinical and development work for our gene and cell therapy product candidates ($16.5 million), partially due to scaled back manufacturing, clinical and non-clinical development activities resulting from the effects of the COVID-19 pandemic, as well as cost savings from the decision to internally manufacture retrovirus for the EB-101 program;
- decreased salary and related costs ($1.1 million); and
- decreased other research and development costs ($0.5 million).

Total general and administrative expenses were $16.4 million for the first nine months of 2020, as compared to $16.0 million for the same period of 2019, an increase of $0.4 million. The increase in expenses was primarily due to:

- increased salary and related costs ($1.1 million), partially resulting from severance costs associated with management changes; and
- increases in net other general and administrative expenses ($0.1 million); partially offset by
- decreased professional fees ($0.8 million).

Depreciation and amortization was $3.7 million for the first nine months of 2020, as compared to $5.7 million for the same period in 2019, a decrease of $2.0 million. The decrease was driven primarily by decreased amortization expense of $2.6 million on licensed technology due to the write-off of the REGENXBIO licensed technology in the first quarter of 2020, partially offset by increased depreciation expense of $0.6 million.

Our license agreement with REGENXBIO terminated on May 2, 2020. Since our impairment testing indicated that the carrying value of the license agreement with REGENXBIO exceeded its fair value, we recorded a $32.9 million non-cash impairment charge in the first nine months of 2020.

Interest and miscellaneous income was $1.3 million for the first nine months of 2020, as compared to $0.8 million of the same period in 2019. The increase results from higher earnings on short-term investments driven by a higher average balance of short-term investments.

Interest and other expense was $2.7 million for the first nine months of 2020, as compared to nil for the same period of 2019. The increase results primarily from accrued interest on the amounts that we may owe to REGENXBIO under the prior license agreement, which amount is subject to the arbitration discussed in Note 3 of the Notes to Condensed Consolidated Financial Statements in Part I, Item 1. As described in more detail in Note 3, we have filed an arbitration claim alleging that REGENXBIO materially breached the license agreement and seeking, among other things, a declaration that we are not responsible for such payments.

Net loss for the first nine months of 2020 was $68.4 million, or a $0.73 basic and diluted loss per common share as compared to a net loss of $59.9 million, or a $1.22 basic and diluted loss per common share, for the same period in 2019. The increase in the net loss results primarily from a licensed technology impairment charge of $32.9 million, partially offset by increased license and other revenues along with decreased clinical and development expenses.

LIQUIDITY AND CAPITAL RESOURCES

We have historically funded our operations primarily through sale of common stock. The COVID-19 pandemic has negatively affected the global economy and created significant volatility and disruption of financial markets. An extended period of economic disruption could negatively affect our business, financial condition, and access to sources of liquidity.

Our principal source of liquidity is cash, cash equivalents and short-term investments. As of September 30, 2020 and December 31, 2019, our cash, cash equivalents, receivables and short-term investments were $103.9 million and $129.3 million, respectively. Based upon our current operating plans, we believe that we have sufficient resources to fund operations through the next 12 months with our existing cash, cash equivalents, receivables and short-term investments. We will need to secure additional funding in the future, to carry out all our planned research and development activities. If we are unable to obtain additional financing or generate license or product revenue, the lack of liquidity and sufficient capital resources could have a material adverse effect on our future prospects.

In October 2020, we announced that we have retained Jefferies LLC as our financial advisor to assist with the review of strategic options focused on advancing our mission and maximizing stakeholder value. We initiated this formal process to explore a broad range of strategic alternatives, including, but not limited to, the partnering of our various clinical and pre-clinical programs, or a sale or merger of the Company, in an effort to unlock the potential of those assets. There can be no assurance this strategic review will result in the completion of any particular course of action, and there is no defined timeline for completion of the review process.

As of September 30, 2020 and December 31, 2019, our working capital was $65.8 million and $93.7 million, respectively. The decrease in working capital at September 30, 2020 resulted primarily from $33.4 million of cash used for operating activities.

On May 2, 2020, we received loan proceeds in the amount of approximately $1.8 million under the PPP, which was established under the CARES Act and is administered by the U.S. Small Business Administration (“SBA”). Under the terms of the CARES Act, PPP loan recipients can apply for loan forgiveness. The potential loan forgiveness for all or a portion of PPP loans is determined, subject to limitations, based on the use of loan proceeds over the 24 weeks after the loan is funded for payment of payroll costs and any payments of mortgage interest, rent, and utilities. The amount of loan forgiveness will be reduced if PPP loan recipients terminate employees or reduce salaries during the covered period. The unforgiven portion of our PPP Loan, if any, is payable over two years at an interest rate of 1%, with a deferral of payments for the first six months, beginning on May 2, 2020. We believe that we have used the proceeds from our PPP Loan for purposes consistent with the PPP. While we currently believe that our use of the loan proceeds will meet the conditions for forgiveness of the PPP Loan, there can be no assurance that forgiveness for any portion of the PPP Loan will be obtained.

On December 24, 2019, we closed an underwritten public offering of 32,382,945 shares of common stock at a public offering price of $2.50 per share. In addition, as part of the offering, we sold to an existing investor “pre-funded” warrants to purchase up to an aggregate of 9,017,055 shares of common stock at a purchase price of $2.4999 per pre-funded warrant, which equals the public offering price per share of the common stock less the $0.0001 per share exercise price of each pre-funded warrant. The gross proceeds to the Company were approximately $103.5 million, before deducting the underwriting discounts and commissions and estimated offering expenses payable by the Company. In October 2020, all of the pre-funded warrants were exercised and converted into shares of common stock.

On August 17, 2018, we entered into an open market sale agreement with Jefferies LLC. Pursuant to the terms of this agreement, we may sell from time to time, through Jefferies LLC, shares of our common stock for an aggregate sales price of up to $150.0 million. Any sales of shares pursuant to this agreement are made under our effective “shelf” registration statement on Form S-3 that is on file with and has been declared effective by the SEC. We did not sell any shares of our common stock under this agreement during the nine months ended September 30, 2020. Cumulatively, as of September 30, 2020, we have sold an aggregate of 3,086,950 shares of our common stock under this agreement and received $17.0 million of proceeds.

License Agreement

On November 4, 2018, we entered into a license agreement with REGENXBIO Inc. ("REGENXBIO") to obtain rights to an exclusive worldwide license (subject to certain non-exclusive rights previously granted for MPS IIIA), with rights to sublicense, to REGENXBIO's NAV AAV9 vector for gene therapies for treating MPS IIIA, MPS IIIB, CLN1 Disease and CLN3 Disease. Consideration for the rights granted under the original agreement included fees totaling $180 million and a running royalty on net sales, including: (i) an initial fee of $20 million, $10 million of which was due to REGENXBIO shortly after the effective date of the agreement, and $10 million of which was to be due on the first anniversary of the effective date of the agreement in November 2019, (ii) annual fees totaling up to $100 million, payable in $20 million annual installments beginning on the second anniversary of the effective date (the first of which was to remain payable if the agreement were terminated before the second anniversary in November 2020), (iii) sales milestone payments totaling $60 million, and (iv) royalties payable in the low double digits to low teens on net sales of products covered under the agreement. The license was being amortized over the life of the patent of eight years. On November 1, 2019, we entered into an amendment of the original license agreement. The amended agreement replaced the $10 million payment due on November 4, 2019 with a $3 million payment due on November 4, 2019 and an additional $8 million payment (which included $1 million of interest) that would have been due no later than April 1, 2020. That $8 million payment had been scheduled to be paid by April 1, 2020 and the $20 million that had been due to be paid on November 4, 2020, and both were recorded as payable to licensor on the consolidated balance sheet. As discussed below, the Company has disputed that it is responsible for the $8 million and $20 million payments, and those payments are the subject of a current arbitration between the Company and REGENXBIO, as further discussed below.

Prior to the April 1, 2020 deadline, we engaged REGENXBIO in discussions in an attempt to renegotiate the financial terms of the agreement, but we were unable to reach a mutual understanding that we believed would have been favorable for the Company or our programs, and we did not make the $8 million payment due by April 1, 2020. On April 17, 2020, REGENXBIO sent us a written demand for the $8 million fee, payable within a 15-day cure period after receipt of the demand letter. The license terminated on May 2, 2020, when the 15-day period expired. There were no penalties for early termination of the license. On May 25, 2020, we filed an arbitration claim with the American Arbitration Association alleging that REGENXBIO materially breached the license agreement prior to termination and seeking, among other things, a declaration that as a result of REGENXBIO's material breach, we are not responsible for payments totaling $28 million (which would otherwise have been due in 2020) plus accrued interest (of $2.1 million as of September 30, 2020). REGENXBIO disputes our arbitration claim and has filed a counterclaim seeking payment of the $28 million plus interest, which REGENXBIO argues remains due. For additional information, refer to Part II, Item 1. Legal Proceedings of this Form 10-Q.

Since our inception, we have incurred negative cash flows from operations and have expended, and expect to continue to expend, substantial funds to complete our planned product development efforts. Since inception, our expenses have significantly exceeded revenues, resulting in an accumulated deficit of $554.9 million as of September 30, 2020. We have not been profitable since inception and to date have received limited revenues from the sale of products. We expect to incur losses for the next several years as we continue to invest in product research and development, preclinical studies, clinical trials and regulatory compliance and cannot provide assurance that we will ever be able to generate sufficient product sales or royalty revenue to achieve profitability on a sustained basis, or at all.

If we raise additional funds by selling additional equity securities, the relative equity ownership of our existing investors will be diluted, and the new investors could obtain terms more favorable than previous investors. If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs, or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financing when needed, we may be required to delay, limit or terminate our product development programs or any future commercialization efforts or grant rights to develop and market product candidates to third parties that we would otherwise prefer to develop and market ourselves.

We are carefully and continually re-assessing key business activities and all associated spending decisions as the COVID-19 pandemic continues to evolve. Nonetheless, we are spending necessary funds on manufacturing activities and preclinical studies and clinical trials of potential products, including research and development with respect to our acquired and developed technology. Our future capital requirements and adequacy of available funds depend on many factors, including:

- the evolving impact to our business, operations, and clinical programs from the COVID-19 pandemic and related effects on the U.S. and global economy;
- the successful development and commercialization of our gene and cell therapy and other product candidates;
- the ability to establish and maintain collaborative arrangements with corporate partners for the research; development and commercialization of products;

- continued scientific progress in our research and development programs;
- the magnitude, scope and results of preclinical testing and clinical trials;
- the costs involved in filing, prosecuting and enforcing patent claims;
- the costs involved in conducting clinical trials;
- competing technological developments;
- the cost of manufacturing and scale-up;
- the ability to establish and maintain effective commercialization arrangements and activities; and
- the successful outcome of our regulatory filings.

Due to uncertainties and certain of the risks described above, including those relating to the COVID-19 pandemic and our ability to successfully commercialize our product candidates, our ability to obtain applicable regulatory approval to market our product candidates, our ability to obtain necessary additional capital to fund operations in the future, our ability to successfully manufacture our products and our product candidates in clinical quantities or for commercial purposes, government regulation to which we are subject, the uncertainty associated with preclinical and clinical testing, intense competition that we face, market acceptance of our products, the potential necessity of licensing technology from third parties and protection of our intellectual property, it is not possible to reliably predict future spending or time to completion by project or product category or the period in which material net cash inflows from significant projects are expected to commence. If we are unable to timely complete a particular project, our research and development efforts could be delayed or reduced, our business could suffer depending on the significance of the project and we might need to raise additional capital to fund operations, as discussed in the risks above, including those relating to the uncertainty of the success of our research and development activities and our ability to obtain necessary additional capital to fund operations in the future.

We plan to continue our policy of investing any available funds in suitable certificates of deposit, money market funds, government securities and investment-grade, interest-bearing securities. We do not invest in derivative financial instruments.

OFF-BALANCE SHEET ARRANGEMENTS

We did not have, during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under applicable SEC rules.

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

Our business and financial results are not materially affected by fluctuations in currency exchange rates or interest rates. We do not use derivative financial instruments for trading or speculative purposes.

Interest Rate Risk

Our exposure to market risk for changes in interest rates is limited to our investments portfolio. Our investment strategy has been focused on preserving capital and supporting our liquidity requirements, while earning a reasonable market return. We invest only in U.S. government, U.S. agency and U.S. treasury securities. The market value of our investments would not materially decline if current market interest rates rise given the short duration of our investments.

Concentrations of Risk

We invest excess cash in short-term, fixed-rate debt securities, and diversify the investments between financial institutions.

Foreign Currency Fluctuation Risk

We are not currently exposed to significant market risk related to changes in foreign currency exchange rates; however, we have contracted with and may continue to contract with foreign vendors that are located in Europe and Australia.

Inflation Fluctuation Risk

Inflation can affect us by increasing our cost of labor and clinical trial costs. We do not believe that inflation had a material effect on our business, financial condition or results of operations during the three or nine months ended September 30, 2020 or 2019.

ITEM **4.** CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

Under the supervision and with the participation of our management and consultants, including the Chief Operating Officer (our principal executive officer) and Chief Accounting Officer (our principal financial officer), we have conducted an evaluation of the effectiveness of the design and operation of our disclosure controls and procedures ("Disclosure Controls and Procedures"), as of September 30, 2020, as such term is defined in Exchange Act Rules 13a-15(e) and 15d-15(e) of the Securities Exchange Act of 1934, as amended (the "Exchange Act").

Conclusion of Evaluation — Based on this Disclosure Controls and Procedures evaluation, the Chief Operating Officer and Chief Accounting Officer concluded that our Disclosure Controls and Procedures as of September 30, 2020 were effective.

Changes in Internal Control Over Financial Reporting— There were no changes in our internal control over financial reporting that occurred during the quarter ended September 30, 2020 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II — OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

We are currently engaged in an arbitration proceeding with REGENXBIO regarding the former license agreement between the parties relating to use of the AAV9 capsid in our MPS IIIA, MPS IIIB, CLN1, and CLN3 programs. The license terminated on May 2, 2020, and on May 25, 2020, we filed an arbitration claim with the American Arbitration Association alleging that REGENXBIO materially breached the license agreement prior to termination and seeking, among other things, a declaration that as a result of REGENXBIO's material breach, we are not responsible for payments totaling $28 million (which would otherwise have been due in 2020) plus accrued interest ($2.1 million as of September 30, 2020). REGENXBIO disputes our arbitration claim and has filed a counterclaim seeking payment of these amounts. It is estimated that an arbitration hearing will take place in March 2021.

ITEM **1A.** RISK FACTORS

Our business and financial results are subject to numerous risks and uncertainties. As a result, the risks and uncertainties discussed in Part I, Item 1A. Risk Factors in our Form 10-K for the year ended December 31, 2019 should be carefully considered. There have been no material changes in the assessment of other risk factors set forth in our 2019 Form 10-K, except for the additional risk factors noted below, which update the risk factors included in Part II, Item 1A of our Quarterly Reports on Form 10-Q for the quarters ended March 31, 2020 and June 30, 2020:

The COVID-19 pandemic and efforts to reduce its spread has affected our operations and significantly impacted worldwide economic conditions, and could continue to have a material effect on our operations, business and financial condition.

To date, the COVID-19 pandemic resulted in intermittent shutdowns of non-essential businesses throughout Europe and the U.S., including in Spain and Australia, where we also conduct operations. The impact of the COVID-19 pandemic has also resulted in social, economic, and labor instability in the countries in which we, or the third parties with whom we engage, operate. Public health officials have recommended precautions to mitigate the spread of the coronavirus, including prohibitions on congregating in heavily populated areas and shelter-in-place orders. As a result, our operations at our Cleveland manufacturing facility were significantly scaled back in the first nine months of 2020 to ensure that our employees and those around them have the best chance to remain safe and to accommodate reduced manufacturing and clinical development activities during this uncertain time.

The COVID-19 pandemic has substantially burdened healthcare systems worldwide, delaying enrollment in and progression of our clinical trials. Required inspections and reviews by regulatory agencies have also been delayed due to the focus of resources on COVID-19, as well as travel and other restrictions. For example, our Phase III VIITAL™ clinical trial was temporarily paused in March 2020 due to the COVID-19 pandemic but resumed in June 2020. Significant delays in the timing of our clinical trials and in regulatory reviews could adversely affect our ability to commercialize our product candidates.

Although we remain committed to advancing our clinical programs, we recognize some delays are inevitable in light of the closure of non-essential businesses, stay at home orders, and economic impacts related to the COVID-19 pandemic, especially as healthcare resources are justly redirected to those who need them most. Many of the third parties with whom we engage, including suppliers, clinical trial sites, regulators and other third parties with whom we conduct business, are also experiencing shutdowns or other business disruptions. Despite our current clinical trial sites gradually resuming activities on site and us having resumed our EB-101 manufacturing activities, we may continue to experience disruptions that could severely impact our business, supply chain, manufacturing operations, clinical trials and pre-clinical studies, including:

- continued interruption of key clinical trial activities, including continued limitations on travel imposed or recommended by federal or state governments, employers and others;
- continued delays or inability to obtain raw material or ingredients;
- continued delays or difficulties in enrolling patients in our clinical trials;
- continued delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;
- delays or difficulties in manufacturing clinical drug material;
- continued diversion of healthcare resources away from the conduct of clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials; and
- continued limitations in employee resources that would otherwise be focused on the conduct of our manufacturing operations, clinical trials and preclinical studies, including because of sickness of employees or their families or the desire of employees to avoid contact with large groups of people.

The ultimate impact of the COVID-19 pandemic remains uncertain and subject to change. We do not yet know the full extent of potential delays or impacts on our business, operations, or financial condition, or on healthcare systems or the global economy as a whole. However, these effects could have a material impact on our ability to access the capital markets as needed and on our operations and business, and those of the third parties on which we rely.

Our loan under the Paycheck Protection Program may not be forgiven or may subject us to challenges and investigations regarding qualification for the loan.

We have received loan proceeds in the amount of approximately $1.8 million under the PPP, which was established under the CARES Act and is administered by the SBA. Under the terms of the CARES Act, PPP loan recipients can apply for loan forgiveness. The potential loan forgiveness for all or a portion of PPP loans is determined, subject to limitations, based on the use of loan proceeds over the 24 weeks after the loan proceeds are disbursed for payment of payroll costs and any payments of mortgage interest, rent, and utilities. The amount of loan forgiveness will be reduced if PPP loan recipients terminate employees or reduce salaries during the covered period. The unforgiven portion of our PPP Loan, if any, is payable over two years at an interest rate of 1%, with a deferral of payments for the first six months, beginning on May 2, 2020. We believe that we have used the proceeds from the PPP Loan for purposes consistent with the PPP. While we currently believe that our use of the loan proceeds will meet the conditions for forgiveness of the PPP Loan, there can be no assurance that forgiveness for any portion of the PPP Loan will be obtained.

Additionally, the PPP loan application required us to certify that the current economic uncertainty made the PPP loan request necessary to support our ongoing operations. While we made this certification in good faith after analyzing, among other things, our financial situation and access to alternative forms of capital, and believe that we satisfied all eligibility criteria for the PPP loans and that our receipt of the PPP loans is consistent with the broad objectives of the Paycheck Protection Program of the CARES Act, the certification described above contains subjective criteria and is subject to interpretation. In addition, the SBA has stated that it is unlikely that a public company with substantial market value and access to capital markets will be able to make the required certification in good faith. The lack of clarity regarding loan eligibility under the program has resulted in significant media coverage and controversy with respect to public companies applying for and receiving loans. If, despite our good faith belief that we satisfied all eligibility requirements for the PPP loan, the SBA concludes we have been ineligible to receive the PPP loan or in violation of any of the laws or regulations that apply to us in connection with the PPP loan, including the False Claims Act, we may be subject to penalties, including significant civil, criminal and administrative penalties and could be required to repay the PPP loan. In the event that we seek forgiveness of all or a portion of the PPP Loan, we will also be required to make certain certifications that will be subject to audit and review by government entities and could subject us to significant penalties and liabilities if found to be inaccurate. In addition, a review or audit by the SBA or other government entity or claims under the False Claims Act could consume significant financial and management resources. Any of these events could harm our business, results of operations or financial condition.

If we fail to comply with our obligations under existing license agreements, the licensor may have the right to terminate such license, in which event we would not be able to develop, manufacture, or market products covered by the license or may face other penalties under the agreements, or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology.

If we fail to comply with our obligations under these license agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we may face challenges for patent infringement if we continue to develop, manufacture, or market products covered by the license, or may face other penalties under the agreements. Termination of these agreements or reduction or elimination of our rights under these agreements may result in our having to negotiate new or reinstated agreements with less favorable terms or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology.

It is possible that such termination may occur even if we believe that we have complied with our obligations under a license agreement, if a dispute arises between us and a licensor. Our license agreement with REGENXBIO had granted us an exclusive worldwide license (subject to certain non-exclusive rights previously granted for MPS IIIA), with rights to sublicense, to use REGENXBIO's NAV AAV9 capsid in gene therapies for treating MPS IIIA, MPS IIIB, CLN1 Disease, and CLN3 Disease. On May 2, 2020, REGENXBIO terminated the license agreement. We filed an arbitration claim against REGENXBIO relating to $28 million plus interest that REGENXBIO argues remains due following the agreement's termination, and that arbitration proceeding is ongoing. We may not prevail in the arbitration proceeding. Even if we do prevail, it is possible that REGENXBIO may in the future assert that our proposed products infringe one or more of REGENXBIO's AAV9 patent claims, and we still may ultimately need a license to use the AAV9 capsid in our proposed MPS IIIA, MPS IIIB, CLN1, and CLN3 products. Absent such a license, if we are found to infringe an AAV9 patent claim, it is possible that a court may enjoin the sale of one or more of our proposed AAV9-based products, order us to pay a less favorable royalty rate to REGENXBIO than the royalty rate in the original license agreement, or order us to pay other damages.

ITEM 6. EXHIBITS

See Exhibit Index below, which is incorporated by reference herein.

Exhibit Index

Exhibits:

31.1	Principal Executive Officer Certification Pursuant to Rule 13a-14(a) of the Securities Exchange Act of 1934.
31.2	Principal Financial Officer Certification Pursuant to Rule 13a-14(a) of the Securities Exchange Act of 1934.
32*	Certification Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101	The following materials from Abeona's Quarterly Report on Form 10-Q for the quarter ended September 30, 2020, formatted in Inline XBRL (Extensible Business Reporting Language): (i) Condensed Consolidated Balance Sheets at September 30, 2020 and December 31, 2019, (ii) Condensed Consolidated Statements of Operations and Comprehensive Loss for the three and nine months ended September 30, 2020 and 2019, (iii) Condensed Consolidated Statements of Stockholders' Equity for the three and nine months ended September 30, 2020 and 2019, (iv) Condensed Consolidated Statements of Cash Flows for the nine months ended September 30, 2020 and 2019, and (v) Notes to Condensed Consolidated Financial Statements.
104	Cover Page Interactive Data File (embedded within the Inline XBRL document and included in Exhibit 101).

* Pursuant to Item 601(b)(32)(ii) of Regulation S-K, this exhibit shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934 or otherwise subject to the liabilities of that Section, nor shall it be deemed incorporated by reference in any filings under the Securities Act of 1933 or the Securities Exchange Act of 1934, whether made before or after the date hereof and irrespective of any general incorporation language in any filing.

SIGNATURES

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

ABEONA THERAPEUTICS INC.

Date: November 9, 2020

By: */s/ Michael Amoroso*
Michael Amoroso
Chief Operating Officer
(Principal Executive Officer)

Date: November 9, 2020

By: */s/ Edward Carr*
Edward Carr
Chief Accounting Officer
(Principal Financial Officer)

EXHIBIT 31.1

PRINCIPAL EXECUTIVE OFFICER CERTIFICATION PURSUANT TO 18 U.S.C.
SECTION 1350, AS ADOPTED PURSUANT TO SECTION 302
OF THE SARBANES-OXLEY ACT OF 2002

I, Michael Amoroso, certify that:

1. I have reviewed this report on Form 10-Q of Abeona Therapeutics Inc.;

2. Based on my knowledge, this 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 15d-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 subsidiaries, 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 (the registrant's fourth fiscal quarter in the case of an annual report) 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 the registrant's board of directors (or persons performing the equivalent functions):

   a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 9, 2020

By: */s/ Michael Amoroso*
Michael Amoroso
Chief Operating Officer
(Principal Executive Officer)

EXHIBIT 31.2

PRINCIPAL FINANCIAL OFFICER CERTIFICATION PURSUANT TO 18 U.S.C.
SECTION 1350, AS ADOPTED PURSUANT TO SECTION 302
OF THE SARBANES-OXLEY ACT OF 2002

I, Edward Carr, certify that:

1. I have reviewed this report on Form 10-Q of Abeona Therapeutics Inc.;

2. Based on my knowledge, this 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 15d-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 subsidiaries, 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 (the registrant's fourth fiscal quarter in the case of an annual report) 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 the registrant's board of directors (or persons performing the equivalent functions):

   a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 9, 2020

By: */s/ Edward Carr*
Edward Carr
Chief Accounting Officer
(Principal Financial Officer)

EXHIBIT 32

CERTIFICATION PURSUANT TO 18 U.S.C.
SECTION 1350 AS ADOPTED PURSUANT TO SECTION 906
OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report of Abeona Therapeutics Inc. (the "Company") on Form 10-Q for the three months ended September 30, 2020 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), we, Michael Amoroso, Chief Operating Officer of the Company, and Edward Carr, Chief Accounting Officer of the Company, certify, pursuant to 18 U.S.C. 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

(1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

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

Date: November 9, 2020

By: */s/ Michael Amoroso*
Michael Amoroso
Chief Operating Officer
(Principal Executive Officer)

Date: November 9, 2020

By: */s/ Edward Carr*
Edward Carr
Chief Accounting Officer
(Principal Financial Officer)