

UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

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

For the Quarterly Period Ended June 30, 2020

Or

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

For the Transition Period from \_\_\_\_ to \_\_\_\_.

Commission File Number 333-88480

**NEUBASE THERAPEUTICS, INC.**

(Exact name of registrant as specified in its charter)

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

**46-5622433**  
(I.R.S. Employer  
Identification No.)

**700 Technology Drive, Third Floor, Pittsburgh, PA 15219**  
(Address of principal executive offices and zip code)

**(646) 450-1790**  
(Registrant's telephone number, including area code)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, \$0.0001 par value per share	NBSE	The Nasdaq Stock Market LLC

Securities registered pursuant to Section 12(g) of the Act: None.

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	<input type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input checked="" type="checkbox"/>	Smaller reporting company	<input checked="" type="checkbox"/>
		Emerging growth company	<input type="checkbox"/>

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards 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

As of August 12, 2020, 23,122,353 shares of the common stock, par value \$0.0001, of the registrant were outstanding.

## Table of Contents

<u>PART I.</u>		
<u>ITEM 1.</u>	<u>FINANCIAL STATEMENTS</u>	<u>1</u>
<u>ITEM 2.</u>	<u>MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS</u>	<u>14</u>
<u>ITEM 3.</u>	<u>QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK</u>	<u>22</u>
<u>ITEM 4.</u>	<u>CONTROLS AND PROCEDURES</u>	<u>22</u>
<u>PART II.</u>		
<u>ITEM 1.</u>	<u>LEGAL PROCEEDINGS</u>	<u>23</u>
<u>ITEM 1A.</u>	<u>RISK FACTORS</u>	<u>24</u>
<u>ITEM 2.</u>	<u>UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS</u>	<u>65</u>
<u>ITEM 3.</u>	<u>DEFAULTS UPON SENIOR SECURITIES</u>	<u>65</u>
<u>ITEM 4.</u>	<u>MINE SAFETY DISCLOSURES</u>	<u>65</u>
<u>ITEM 5.</u>	<u>OTHER INFORMATION</u>	<u>65</u>
<u>ITEM 6.</u>	<u>EXHIBITS</u>	<u>66</u>
	<u>SIGNATURES</u>	<u>67</u>

---

As previously disclosed on July 12, 2019, Ohr Pharmaceutical, Inc., a Delaware corporation (“Ohr”), completed a Merger with NeuBase Therapeutics, Inc., a Delaware corporation (“Legacy NeuBase”), in accordance with the terms of the Agreement and Plan of Merger and Reorganization (the “Merger Agreement”) entered into on January 2, 2019. Pursuant to the Merger Agreement, (i) a subsidiary of Ohr merged with and into Legacy NeuBase, with Legacy NeuBase (renamed as “NeuBase Corporation”) continuing as a wholly-owned subsidiary of Ohr and the surviving corporation of the merger and (ii) Ohr was renamed as “NeuBase Therapeutics, Inc.” (the “Merger”).

For accounting purposes, the Merger was treated as a “reverse asset acquisition” under generally accepted accounting principles in the United States (“U.S. GAAP”) and Legacy NeuBase was considered the acquirer. Accordingly, Legacy NeuBase’s historical results of operations replaced the Company’s (as defined below) historical results of operations for all periods prior to the Merger and, for all periods following the Merger, the results of operations of the post-merger combined company will be included in the Company’s financial statements.

This quarterly report on Form 10-Q relates to the Company’s quarter ended June 30, 2020 and is therefore the Company’s fourth periodic report that includes results of operations for the combined company, including Legacy NeuBase.

Unless the context otherwise requires, references to the “Company,” the “combined company,” “we,” “our” or “us” in this report refer to NeuBase Therapeutics, Inc. and its subsidiaries, references to “NeuBase” refer to the Company following the completion of the Merger and references to “Ohr” refer to the Company prior to the completion of the Merger.

Except as otherwise noted, references to “common stock” in this report refer to the common stock, par value \$0.0001 per share, of the Company.

---

**PART I.**

**ITEM 1. FINANCIAL STATEMENTS**

**NeuBase Therapeutics, Inc. and Subsidiaries  
Condensed Consolidated Balance Sheets  
(Unaudited)**

	<u>June 30, 2020</u>	<u>September 30, 2019</u>
<b>ASSETS</b>		
<b>CURRENT ASSETS</b>		
Cash and cash equivalents	\$ 35,924,756	\$ 10,313,966
Prepaid insurance	676,515	449,583
Other prepaid expenses and current assets	287,116	265,686
Total Current Assets	<u>36,888,387</u>	<u>11,029,235</u>
EQUIPMENT, net	<u>705,798</u>	<u>430,995</u>
<b>OTHER ASSETS</b>		
Intangible assets, net	-	145,833
Investment	367,829	586,418
Long-term prepaid insurance	193,666	338,916
Total Other Assets	<u>561,495</u>	<u>1,071,167</u>
<b>TOTAL ASSETS</b>	<u>\$ 38,155,680</u>	<u>\$ 12,531,397</u>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
<b>CURRENT LIABILITIES</b>		
Accounts payable	\$ 1,310,468	\$ 1,477,152
Accrued expenses and other current liabilities	434,974	405,599
Warrant liabilities	1,405,817	496,343
Insurance note payable	320,424	122,919
Total Liabilities	<u>3,471,683</u>	<u>2,502,013</u>
<b>COMMITMENTS AND CONTINGENCIES</b>		
<b>STOCKHOLDERS' EQUITY</b>		
Preferred stock, \$0.0001 par value; 10,000,000 shares authorized; no shares issued and outstanding as of June 30, 2020 and September 30, 2019	-	-
Common stock, \$0.0001 par value; 250,000,000 shares authorized; 23,120,196 and 17,077,873 shares issued and outstanding as of June 30, 2020 and September 30, 2019, respectively	2,312	1,708
Additional paid-in capital	74,365,592	37,027,875
Accumulated deficit	(39,683,907)	(27,000,199)
Total stockholders' equity	<u>34,683,997</u>	<u>10,029,384</u>
<b>TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY</b>	<u>\$ 38,155,680</u>	<u>\$ 12,531,397</u>

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

**NeuBase Therapeutics, Inc. and Subsidiaries**  
**Condensed Consolidated Statements of Operations**  
(Unaudited)

	<u>Three Months Ended June 30,</u>		<u>Nine Months Ended June 30,</u>	
	<u>2020</u>	<u>2019</u>	<u>2020</u>	<u>2019</u>
<b>OPERATING EXPENSES</b>				
General and administrative	\$ 2,331,283	\$ 1,655,189	\$ 7,624,984	\$ 3,991,567
Research and development	1,492,881	305,204	4,336,576	343,206
Research and development expense- license acquired	-	-	-	1,046,965
<b>TOTAL OPERATING EXPENSES</b>	<u>3,824,164</u>	<u>1,960,393</u>	<u>11,961,560</u>	<u>5,381,738</u>
<b>LOSS FROM OPERATIONS</b>	(3,824,164)	(1,960,393)	(11,961,560)	(5,381,738)
<b>OTHER INCOME (EXPENSE)</b>				
Interest expense	(1,573)	(60,944)	(3,226)	(119,195)
Change in fair value of warrant liabilities	(285,284)	(13,240)	(909,474)	(51,942)
Loss on disposal of fixed asset	-	-	(3,230)	-
Equity in losses on equity method investment	(101,238)	-	(218,589)	-
Other income	412,371	-	412,371	-
<b>Total other income (expenses), net</b>	<u>24,276</u>	<u>(74,184)</u>	<u>(722,148)</u>	<u>(171,137)</u>
<b>NET LOSS</b>	<u>\$ (3,799,888)</u>	<u>\$ (2,034,577)</u>	<u>\$ (12,683,708)</u>	<u>\$ (5,552,875)</u>
<b>BASIC AND DILUTED LOSS PER SHARE</b>	<u>\$ (0.18)</u>	<u>\$ (0.38)</u>	<u>\$ (0.69)</u>	<u>\$ (0.96)</u>
<b>WEIGHTED AVERAGE SHARES OUTSTANDING:</b>				
BASIC AND DILUTED	<u>21,193,387</u>	<u>5,328,767</u>	<u>18,441,961</u>	<u>5,754,791</u>

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

**NeuBase Therapeutics, Inc. and Subsidiaries**  
**Condensed Consolidated Statements of Stockholders' Equity (Deficit)**  
**For the Three and Nine Months Ended June 30, 2020 and 2019**  
**(Unaudited)**

	Treasury Stock		Common Stock		Additional Paid-In Capital	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount			
<b>Balance as of March 31, 2020</b>	-	\$ -	17,080,625	\$ 1,708	\$ 39,871,511	\$ (35,884,019)	\$ 3,989,200
Stock-based compensation expense	-	-	-	-	1,210,715	-	1,210,715
Issuance of common stock, net of issuance costs	-	-	6,037,500	604	33,283,366	-	33,283,970
Issuance of restricted stock for services	-	-	2,071	-	-	-	-
Net loss	-	-	-	-	-	(3,799,888)	(3,799,888)
<b>Balance as of June 30, 2020</b>	-	\$ -	23,120,196	\$ 2,312	\$ 74,365,592	\$ (39,683,907)	\$ 34,683,997

	Treasury Stock		Common Stock		Additional Paid-In Capital	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount			
<b>Balance as of September 30, 2019</b>	-	\$ -	17,077,873	\$ 1,708	\$ 37,027,875	\$ (27,000,199)	\$ 10,029,384
Stock-based compensation expense	-	-	-	-	4,054,351	-	4,054,351
Issuance of common stock, net of issuance costs	-	-	6,037,500	604	33,283,366	-	33,283,970
Issuance of restricted stock for services	-	-	4,823	-	-	-	-
Net loss	-	-	-	-	-	(12,683,708)	(12,683,708)
<b>Balance as of June 30, 2020</b>	-	\$ -	23,120,196	\$ 2,312	\$ 74,365,592	\$ (39,683,907)	\$ 34,683,997

	Treasury Stock		Common Stock		Additional Paid-In Capital	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount			
<b>Balance as of March 31, 2019</b>	-	\$ -	6,679,305	\$ 668	\$ 2,392,932	\$ (3,560,250)	\$ (1,166,650)
Stock-based compensation expense	-	-	-	-	819,568	-	819,568
Net loss	-	-	-	-	-	(2,034,577)	(2,034,577)
<b>Balance as of June 30, 2019</b>	-	\$ -	6,679,305	\$ 668	\$ 3,212,500	\$ (5,594,827)	\$ (2,381,659)

	Treasury Stock		Common Stock		Additional Paid-In Capital	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount			
<b>Balance as of September 30, 2018</b>	-	\$ -	5,727,090	\$ 573	\$ (517)	\$ (41,952)	\$ (41,896)
Stock-based compensation expense	-	-	-	-	2,367,042	-	2,367,042
Repurchase of common stock	(1,401,202)	(140)	-	-	126	-	(14)
Retirement of common stock	1,401,202	140	(1,401,202)	(140)	-	-	-
Common stock for research and development expense- license acquired	-	-	835,625	84	844,516	-	844,600
Issuance of restricted stock for services	-	-	1,517,792	151	1,323	-	1,474
Cash received for prefunded warrants	-	-	-	-	10	-	10
Net loss	-	-	-	-	-	(5,552,875)	(5,552,875)
<b>Balance as of June 30, 2019</b>	-	\$ -	6,679,305	\$ 668	\$ 3,212,500	\$ (5,594,827)	\$ (2,381,659)

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

**NeuBase Therapeutics, Inc. and Subsidiaries**  
**Condensed Consolidated Statements of Cash Flows**  
(Unaudited)

	<b>Nine Months Ended June 30,</b>	
	<b>2020</b>	<b>2019</b>
<b>Cash flows from operating activities:</b>		
Net loss	\$ (12,683,708)	\$ (5,552,875)
<b>Adjustments to reconcile net loss to net cash used in operating activities</b>		
Stock-based compensation	4,054,351	2,367,042
Research and development expense - license acquired	-	1,046,965
Change in fair value of warrant liabilities	909,474	51,942
Depreciation and amortization	230,715	8,820
Loss on disposal of fixed asset	3,230	-
Equity in losses on equity method investment	218,589	-
Non-cash amortization on convertible notes	-	88,587
Non-cash interest expense on convertible notes	-	30,155
<b>Changes in operating assets and liabilities</b>		
Prepaid insurance, other prepaid expenses and current assets	117,068	(254,046)
Long-term prepaid insurance	145,250	-
Accounts payable	(166,684)	1,611,444
Accrued expenses and other current liabilities	29,375	207,187
<b>Net cash used in operating activities</b>	<u>(7,142,340)</u>	<u>(394,779)</u>
<b>Cash flows from investing activities</b>		
Purchase of laboratory and office equipment	(362,915)	(281,917)
Payment of transaction costs for licenses acquired	-	(43,463)
Cash paid for license acquired	-	(54,000)
<b>Net cash used in investing activities</b>	<u>(362,915)</u>	<u>(379,380)</u>
<b>Cash flows from financing activities</b>		
Principal payment of financed insurance	(167,925)	(8,814)
Proceeds from issuance of stock, net of issuance costs	33,283,970	-
Proceeds from issuance of convertible notes	-	600,000
Proceeds from issuance of common shares for services	-	1,474
Proceeds from prefunded warrant	-	10
Repurchase of common stock	-	(14)
<b>Net cash provided by financing activities</b>	<u>33,116,045</u>	<u>592,656</u>
<b>Net increase (decrease) in cash and cash equivalents</b>	<u>25,610,790</u>	<u>(181,503)</u>
<b>Cash and cash equivalents, beginning of period</b>	<u>10,313,966</u>	<u>249,600</u>
<b>Cash and cash equivalents, end of period</b>	<u><u>\$ 35,924,756</u></u>	<u><u>\$ 68,097</u></u>
<b>Supplemental disclosure of cash flow information:</b>		
Cash paid for interest	\$ 3,244	\$ -
Cash paid for income taxes	\$ -	\$ -
<b>Non-cash investing and financing activities:</b>		
Issuance of common stock for research and development expense- licenses acquired	\$ -	\$ 844,600
Fair value of warrant liability issued for research and development expense- licenses acquired	\$ -	\$ 104,902
Insurance financed through note payable	\$ 365,430	\$ 90,000

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

**NeuBase Therapeutics, Inc. and Subsidiaries**  
**Notes to Condensed Consolidated Financial Statements**  
**(Unaudited)**

**1. Organization and Description of Business**

NeuBase Therapeutics, Inc. (together with its subsidiaries, the “Company” or “NeuBase”) is developing a modular peptide-nucleic acid antisense oligonucleotide (“PATrOL™”) platform to address genetic diseases caused by mutant proteins, with a single, cohesive approach. The systemically-deliverable PATrOL™ therapies are designed to improve upon current gene silencing treatments by combining the advantages of synthetic approaches with the precision of antisense technologies. NeuBase plans to use its platform to address genetic diseases, with an initial focus on Huntington’s Disease (“HD”) and Myotonic Dystrophy Type 1 (“DM1”), as well as other genetic disorders and cancer.

NeuBase is a pre-clinical-stage biopharmaceutical company and continues to develop its clinical and regulatory strategy with its internal research and development team with a view toward prioritizing market introduction as quickly as possible. NeuBase’s lead programs are NT0100 and NT0200.

The NT0100 program is a PATrOL™-enabled therapeutic program being developed to target the mutant expansion in the HD messenger ribonucleic acid (“RNA”). NT0100 falls into the category of peptide nucleic acids (“PNAs”), which have the potential to be highly selective for the mutant transcript vs. the wild-type transcribed allele and the expectation to be applicable for all HD patients as it directly targets the expansion itself, and can be delivered systemically. PATrOL™-enabled drugs also have the unique ability to open RNA secondary structures and bind to either the primary nucleotide sequences or the secondary and/or tertiary structures. NeuBase believes the NT0100 program addresses an unmet need for a disease which currently has no effective therapeutics that target the core etiology of the condition. NeuBase believes there is a large opportunity in the U.S. and European markets for drugs in this space.

The NT0200 program is a PATrOL™-enabled therapeutic program being developed to target the mutant expansion in the DM1 disease mRNA. NT0200 falls into the category of PNAs, which have the potential to be highly selective for the mutant transcript versus the wild-type transcribed allele and the expectation to be applicable for all DM1 patients as it directly targets the expansion itself. NeuBase believes the NT0200 program addresses an unmet need for a disease which currently has no effective therapeutics that target the core etiology of the condition. NeuBase believes there is a large opportunity in the U.S. and European markets for drugs in this space.

***Acquisition of Ohr Pharmaceutical, Inc.***

On July 12, 2019, the Company (formerly known as Ohr Pharmaceutical, Inc. (“Ohr”)) completed a reverse acquisition transaction in accordance with the terms of the Agreement and Plan of Merger and Reorganization, dated as of January 2, 2019, by and among the Company, Ohr Acquisition Corp. (“Merger Sub”), and NeuBase Therapeutics, Inc. (“Legacy NeuBase”), as amended by the First Amendment thereto made and entered into as of June 27, 2019 (as amended, the “Acquisition Agreement”), pursuant to which Merger Sub merged with and into Legacy NeuBase, with Legacy NeuBase (renamed as “NeuBase Corporation”) surviving as a wholly owned subsidiary of the Company (the “Ohr Acquisition”). On July 12, 2019, immediately after completion of the Ohr Acquisition, the Company changed its name to “NeuBase Therapeutics, Inc.”

The Ohr Acquisition was accounted for as a “reverse asset acquisition,” whereby Legacy NeuBase was determined to be the accounting acquirer. The historical financial statements, outstanding shares and all other historical share information have been adjusted by multiplying the respective share amount by the exchange ratio provided for in the Acquisition Agreement as if the exchange ratio had been in effect for all periods presented.

**2. Significant Accounting Policies**

***Basis of Presentation***

The accompanying unaudited interim condensed consolidated financial statements should be read in conjunction with the audited financial statements and notes thereto as of and for the year ended September 30, 2019 included in the Company’s Annual Report on Form 10-K (the “Annual Report”) filed with the SEC on January 10, 2020. The accompanying unaudited condensed consolidated financial statements have been prepared by the Company in accordance with U.S. GAAP for interim financial information and in accordance with the instructions to Form 10-Q and Article 8 of Regulation S-X. Accordingly, certain information and footnote disclosures normally included in financial statements prepared in accordance with U.S. GAAP have been condensed or omitted. In the opinion of management, the accompanying unaudited condensed consolidated financial statements for the periods presented reflect all adjustments, consisting of only normal, recurring adjustments, necessary to fairly state the Company’s financial position, results of operations and cash flows. The unaudited condensed consolidated financial statements for the interim periods are not necessarily indicative of results for the full year. The preparation of these unaudited condensed consolidated financial statements requires the Company to make estimates and judgments that affect the amounts reported in the financial statements and the accompanying notes. The Company’s actual results may differ from these estimates under different assumptions or conditions.



**NeuBase Therapeutics, Inc. and Subsidiaries**  
**Notes to Condensed Consolidated Financial Statements**  
**(Unaudited)**

***Use of Estimates***

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. The most significant estimates in the Company's condensed consolidated financial statements relate to the valuation of share-based compensation, the valuation of licenses, the fair value of warrant liabilities and the valuation allowance of deferred tax assets resulting from net operating losses. These estimates and assumptions are based on current facts, historical experience and various other factors believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. The Company assesses and updates estimates each period to reflect current information, such as the economic considerations related to the impact that the novel coronavirus disease (COVID-19) could have on our significant accounting estimates (see Part II, Item 1A – Risk Factors—“*Our operations may be adversely affected by the coronavirus outbreak, and we face risks that could impact our business*” for further discussion of the effect of the COVID-19 pandemic on our operations). Actual results may differ materially and adversely from these estimates. To the extent there are material differences between the estimates and actual results, the Company's future results of operations will be affected.

***Fair Value Measurements***

Fair value measurements are based on the premise that fair value is an exit price representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As such, fair value is a market-based measurement that should be determined based on assumptions that market participants would use in pricing an asset or liability. As a basis for considering such assumptions, the following three-tier fair value hierarchy has been used in determining the inputs used in measuring fair value:

Level 1 – Quoted prices in active markets for identical assets or liabilities on the reporting date.

Level 2 – Pricing inputs are based on quoted prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets that are not active and model-based valuation techniques for which all significant assumptions are observable in the market or can be corroborated by observable market data for substantially the full term of the assets or liabilities.

Level 3 – Pricing inputs are generally unobservable and include situations where there is little, if any, market activity for the investment. The inputs into the determination of fair value require management's judgment or estimation of assumptions that market participants would use in pricing the assets or liabilities. The fair values are therefore determined using factors that involve considerable judgment and interpretations, including but not limited to private and public comparables, third-party appraisals, discounted cash flow models, and fund manager estimates.

Financial instruments measured at fair value are classified in their entirety based on the lowest level of input that is significant to the fair value measurement. Management's assessment of the significance of a particular input to the fair value measurement in its entirety requires judgment and considers factors specific to the asset or liability. The use of different assumptions and/or estimation methodologies may have a material effect on estimated fair values. Accordingly, the fair value estimates disclosed or initial amounts recorded may not be indicative of the amount that the Company or holders of the instruments could realize in a current market exchange.

**NeuBase Therapeutics, Inc. and Subsidiaries**  
**Notes to Condensed Consolidated Financial Statements**  
(Unaudited)

The following tables present the Company's fair value hierarchy for its warrant liabilities measured at fair value on a recurring basis at June 30, 2020 and September 30, 2019:

	Fair Value Measurements as of June 30, 2020			Total
	(Level 1)	(Level 2)	(Level 3)	
Liabilities				
Warrant liabilities	\$ -	-	1,405,817	\$ 1,405,817

	Fair Value Measurements as of September 30, 2019			Total
	(Level 1)	(Level 2)	(Level 3)	
Liabilities				
Warrant liabilities	\$ -	-	496,343	\$ 496,343

The fair value of the warrant liabilities were determined using the Black-Scholes option pricing model. The following assumptions were used in determining the fair value of the warrant liabilities:

	As of June 30, 2020
Remaining contractual term (years)	1.5- 1.8
Common stock price volatility	87.9% - 89.8%
Risk-free interest rate	0.16%
Expected dividend yield	-

The change in fair value of the warrant liabilities for the three and nine months ended June 30, 2020 and 2019 is as follows:

Fair value as of September 30, 2019	\$ 496,343
Change in fair value	694,134
Fair value as of December 31, 2019	1,190,477
Change in fair value	(69,944)
Fair value as of March 31, 2020	\$ 1,120,533
Change in fair value	285,284
Fair value as of June 30, 2020	\$ 1,405,817
Fair value as of September 30, 2018	\$ -
Warrants issued in connection with license acquired	104,902
Fair value as of December 31, 2018	104,902
Change in fair value	38,702
Fair value as of March 31, 2019	\$ 143,604
Change in fair value	13,240
Fair value as of June 30, 2019	\$ 156,844

As of June 30, 2020 and September 30, 2019, the recorded values of cash and cash equivalents, accounts payable and the insurance note payable approximate fair value due to the short-term nature of these instruments.

**Stock-based Compensation**

The Company expenses stock-based compensation to employees, non-employees and board members over the requisite service period based on the estimated grant-date fair value of the awards and actual forfeitures. The Company accounts for forfeitures as they occur. Stock-based awards with graded-vesting schedules are recognized on a straight-line basis over the requisite service period for each separately vesting portion of the award. The Company estimates the fair value of stock option grants using the Black-Scholes option pricing model, and the assumptions used in calculating the fair value of stock-based awards represent management's best estimates and involve inherent uncertainties and the application of management's judgment. All stock-based compensation costs are recorded in general and administrative or research and development costs in the consolidated statements of operations based upon the underlying individual's role at the Company.

**NeuBase Therapeutics, Inc. and Subsidiaries**  
**Notes to Condensed Consolidated Financial Statements**  
**(Unaudited)**

**Operating Leases**

Effective October 1, 2019, the Company determines if an arrangement is a lease at inception. Operating leases are included in operating right-of-use (“ROU”) assets and operating lease liabilities on the consolidated balance sheets. Prior to October 1, 2019, the Company recorded rent expense associated with its operating lease on a straight-line basis over the term of the lease.

Lease ROU assets and operating lease liabilities are initially recognized based on the present value of the future minimum lease payments over the lease term at commencement date calculated using the Company’s incremental borrowing rate applicable to the lease asset, unless the implicit rate is readily determinable. ROU assets also include any lease payments made at or before lease commencement and exclude any lease incentives received. The Company’s lease terms may include options to extend or terminate the lease when it is reasonably certain that the Company will exercise that option. Leases with a term of 12 months or less are not recognized on the Company’s condensed consolidated balance sheet. Lease expense for minimum lease payments is recognized on a straight-line basis over the lease term. The Company accounts for lease and non-lease components as a single lease component for all its leases.

As of June 30, 2020, the Company’s leases had original terms of less than 12 months. The Company does not recognize ROU assets and lease liabilities that arise from leases with an original term of 12 months or less. Rather, the Company recognizes the lease expense on a straight-line basis over the term of the lease.

**Other Income**

During the three and nine months ended June 30, 2020, the Company recognized \$0.4 million of other income associated with a license of certain clinical trial data to a third party.

**Net Loss Per Share**

Basic net loss per share is computed by dividing net loss applicable to common stockholders by the weighted average number of shares of common stock outstanding during each period. Diluted net loss per share includes the dilutive effect, if any, from the potential exercise or conversion of securities, such as convertible debt, warrants and stock options that would result in the issuance of incremental shares of common stock. In computing the basic and diluted net loss per share applicable to common stockholders, the weighted average number of shares remains the same for both calculations due to the fact that when a net loss exists, dilutive shares are not included in the calculation as the impact is anti-dilutive.

The following potentially dilutive securities outstanding as of June 30, 2020 and 2019 have been excluded from the computation of diluted weighted average shares outstanding, as they would be anti-dilutive:

	<b>As of June 30,</b>	
	<b>2020</b>	<b>2019</b>
Common stock purchase options	6,514,466	3,337,406
Unvested restricted stock	1,250	1,307,001
Common stock purchase warrants	715,939	101,847
Convertible notes	-	613,276
	<b>7,231,655</b>	<b>5,359,530</b>

**Recently Adopted Accounting Pronouncements**

In February 2016, the FASB issued ASU 2016-2, *Leases*. The new standard establishes a right-of-use (“ROU”) model that requires a lessee to record a ROU asset and a lease liability on the balance sheet for all leases with terms longer than 12 months. Leases will be classified as either finance or operating, with classification affecting the pattern of expense recognition in the income statement. The Company adopted this new lease standard on October 1, 2019 using a modified retrospective transition approach for leases existing at, or entered into after, the beginning of the earliest comparative period presented in the financial statements, with certain practical expedients available. The adoption of the new lease standard did not have an impact on the Company’s condensed consolidated financial statements as the Company did not have any leases with original terms longer than 12 months.

**NeuBase Therapeutics, Inc. and Subsidiaries**  
**Notes to Condensed Consolidated Financial Statements**  
**(Unaudited)**

**Recent Accounting Pronouncements**

In December 2019, the FASB issued ASU No. 2019-12, “Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes” (“ASU 2019-12”), which is intended to simplify various aspects related to accounting for income taxes. ASU 2019-12 removes certain exceptions to the general principles in Topic 740 and also clarifies and amends existing guidance to improve consistent application. This guidance is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2020, with early adoption permitted. The Company plans to evaluate the impact of this standard on its consolidated financial statements and related disclosures.

**3. Liquidity**

The Company has had no revenues from product sales and has incurred operating losses since inception. As of June 30, 2020, the Company had \$35.9 million in cash and cash equivalents and during the nine months ended June 30, 2020, incurred a loss from operations of \$12.0 million and used \$7.1 million of cash in operating activities.

The Company has historically funded its operations through the sale of common stock and the issuance of convertible notes and warrants. The Company expects to continue to incur significant operating losses for the foreseeable future and may never become profitable. As a result, the Company will likely need to raise additional capital through one or more of the following: issuance of additional debt or equity, or complete a licensing transaction for one or more of the Company’s pipeline assets. Management believes that it has sufficient working capital on hand to fund operations through at least the next twelve months from the date these unaudited condensed consolidated financial statements were available to be issued. There can be no assurance that the Company will be successful in acquiring additional funding, that the Company’s projections of its future working capital needs will prove accurate, or that any additional funding would be sufficient to continue operations in future years.

**4. Stockholders’ Equity**

**Common Stock Offering**

On April 30, 2020, the Company closed an underwritten public offering of 6,037,500 shares of its common stock (inclusive of 787,500 shares that were sold pursuant to the underwriters’ full exercise of their option to purchase additional shares of the Company’s common stock), at a price to the public of \$6.00 per share. The Company received net proceeds from the offering of approximately \$33.3 million, after deducting the underwriting discounts and commissions and other estimated offering expenses payable by the Company. The Company intends to use the net proceeds from this offering for working capital and general corporate purposes and to advance the development of its product candidates and expand its pipeline.

**Warrants**

Below is a summary of the Company’s issued and outstanding warrants as of June 30, 2020:

	<b>Warrants</b>	<b>Weighted Average Exercise Price</b>	<b>Weighted Average Remaining Contractual Life (in years)</b>
Outstanding as of September 30, 2019	728,439	\$ 20.99	
Expired	(12,500)	20.00	
Outstanding as of June 30, 2020	<u>715,939</u>	<u>\$ 21.01</u>	1.77

**5. Stock-Based Compensation**

As of June 30, 2020, an aggregate of 3,783,114 shares of common stock were authorized under the Company’s 2019 Stock Incentive Plan (the “2019 Plan”), subject to an “evergreen” provision that will automatically increase the maximum number of shares of common stock that may be issued under the term of the 2019 Plan. As of June 30, 2020, 692,039 common shares were available for future grants under the 2019 Plan. As of June 30, 2020, 291,667 shares of common stock were authorized under the Company’s 2016 Consolidated Stock Incentive Plan (the “2016 Plan”) and 147,041 common shares were available for future grants under the 2016 Plan.

**NeuBase Therapeutics, Inc. and Subsidiaries**  
**Notes to Condensed Consolidated Financial Statements**  
**(Unaudited)**

The Company recorded stock-based compensation expense in the following expense categories of its condensed consolidated statements of operations for the three and nine months ended June 30, 2020 and 2019:

	<u>Three Months Ended June 30,</u>		<u>Nine Months Ended June 30,</u>	
	<u>2020</u>	<u>2019</u>	<u>2020</u>	<u>2019</u>
General and administrative	\$ 847,921	\$ 814,657	\$ 2,915,273	\$ 2,348,957
Research and development	362,794	4,911	1,139,078	18,085
<b>Total</b>	<b>\$ 1,210,715</b>	<b>\$ 819,568</b>	<b>\$ 4,054,351</b>	<b>\$ 2,367,042</b>

*Stock Options*

Below is a table summarizing the options issued and outstanding as of and for the nine months ended June 30, 2020:

	<u>Stock Options</u>	<u>Weighted Average Exercise Price</u>	<u>Weighted Average Remaining Contractual Life (in years)</u>	<u>Total Aggregate Intrinsic Value</u>
Outstanding as of September 30, 2019	6,375,966	\$ 2.70		
Granted	148,500	6.41		
Forfeited	(10,000)	7.23		
Outstanding at June 30, 2020	<u>6,514,466</u>	\$ 2.78	8.7	\$ 39,565,558
Exercisable as of June 30, 2020	<u>4,331,730</u>	1.43	8.5	32,306,881

As of June 30, 2020, unrecognized compensation costs associated with the stock options of \$3.7 million will be recognized over an estimated weighted-average amortization period of 1.1 years.

The weighted average grant date fair value of options granted during the nine months June 30, 2020 was \$4.67.

Key assumptions used to estimate the fair value of the stock options granted during the nine months ended June 30, 2020 included:

	<u>Nine Months ended June 30, 2020</u>
Expected term of options (years)	6.1 - 7.0
Expected common stock price volatility	78% - 84.3%
Risk-free interest rate	0.3% - 1.8%
Expected dividend yield	-

*Restricted Stock*

A summary of the changes in the unvested restricted stock during the nine months ended June 30, 2020 is as follows:

	<u>Unvested Restricted Stock</u>	<u>Weighted Average Grant Date Fair Value Price</u>
Unvested as of September 30, 2019	6,875	\$ 6.24
Granted	4,823	6.36
Vested	(10,448)	6.28
Unvested as of June 30, 2020	<u>1,250</u>	\$ 6.24
Total unrecognized expense remaining	<u>\$ 1,035</u>	
Weighted-average years expected to be recognized over	0.1	

**NeuBase Therapeutics, Inc. and Subsidiaries**  
**Notes to Condensed Consolidated Financial Statements**  
(Unaudited)

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

The Company's prepaid expenses and other current assets consisted of the following:

	June 30, 2020	September 30, 2019
Prepaid research and development expense	\$ -	\$ 223,510
Other income receivable	174,620	-
Other prepaid expenses and other current assets	112,496	42,176
Total	<u>\$ 287,116</u>	<u>\$ 265,686</u>

At June 30, 2020, the Company recognized a receivable associated with the income from a license of certain clinical trial data to a third party.

**7. Equipment**

The Company's equipment consisted of the following:

	June 30, 2020	September 30, 2019	Estimated useful life (in years)
Laboratory equipment	\$ 809,665	\$ 452,817	5
Office equipment	5,052	2,383	3
Total	814,717	455,200	
Accumulated depreciation	(108,919)	(24,205)	
Property, plant and equipment, net	<u>\$ 705,798</u>	<u>\$ 430,995</u>	

Depreciation expense for the three months ended June 30, 2020 and 2019 was approximately \$0.03 million and \$0.01 million, respectively. Depreciation expense for the nine months ended June 30, 2020 and 2019 was approximately \$0.08 million and \$0.01 million, respectively.

**8. Intangible Assets**

The Company's intangible assets consisted of the following:

	June 30, 2020	September 30, 2019	Estimated useful life (in months)
Clinical trial data	\$ 250,000	\$ 250,000	6
Accumulated amortization	(250,000)	(104,167)	
Intangible assets, net	<u>\$ -</u>	<u>\$ 145,833</u>	

Amortization expense for the three months ended June 30, 2020 and 2019 was \$0. Amortization expense for the nine months ended June 30, 2020 and 2019 was approximately \$0.15 million and \$0, respectively.

**9. Investment**

The Company owns common and preferred shares of DepYmed, Inc. ("DepYmed"), which in aggregate represents approximately 14.84% ownership of DepYmed. In addition, the Company is entitled to hold two of the six seats on DepYmed's board of directors.

The Company accounts for its investment in DepYmed common shares using the equity method of accounting and records its proportionate share of DepYmed's net income and losses in the accompanying condensed consolidated statements of operations. Equity in losses for the three and nine months ended June 30, 2020 was approximately \$0.1 million and \$0.2 million, respectively.

The Company accounts for its investment in preferred shares of DepYmed at cost, less any impairment, as the Company determined the preferred stock did not have a readily determinable fair value.

As of June 30, 2020 and September 30, 2019, the carrying amount of the Company's aggregate investment in DepYmed was \$0.4 million and \$0.6 million, respectively.

**NeuBase Therapeutics, Inc. and Subsidiaries**  
**Notes to Condensed Consolidated Financial Statements**  
**(Unaudited)**

**10. Accrued Expenses and Other Current Liabilities**

The Company's accrued expenses and other current liabilities consisted of the following:

	<b>June 30, 2020</b>	<b>September 30, 2019</b>
Accrued compensation and benefits	\$ 20,035	\$ 34,625
Accrued interest	3,707	10,830
Accrued professional fees	158,945	156,919
Accrued research and development	177,877	88,553
Other accrued expenses	74,410	114,672
Total	<u>\$ 434,974</u>	<u>\$ 405,599</u>

**11. Commitments and Contingencies**

***Operating Leases***

The Company leases its office and operating space under operating leases with original terms of less than 12 months and which expire at various dates through September 2020; therefore, the Company's operating leases are not recognized as right-of-use assets on the condensed consolidated balance sheet as of June 30, 2020.

Rent expense under the Company's operating leases totaled approximately \$0.03 million and \$0.01 million for the three months ended June 30, 2020 and 2019, respectively. Rent expense under the Company's operating leases totaled approximately \$0.07 million and \$0.01 million for the nine months ended June 30, 2020 and 2019, respectively.

On February 26, 2020, the Company exercised its option to extend the term of its operating lease in Pittsburgh until September 30, 2020. All terms and conditions remain the same from the current lease. Future minimum rental payments under operating leases with non-cancelable terms as of June 30, 2020 due during the year ended September 2020 are approximately \$0.02 million.

***Litigation***

The Company has become involved in certain legal proceedings and claims which arise in the normal course of business. If an unfavorable ruling were to occur, there exists the possibility of a material adverse impact on the Company's results of operations, cash flows and financial position.

On February 14, 2018, plaintiff Jeevesh Khanna, commenced an action in the Southern District of New York, against Ohr and several current and former officers and directors of Ohr, alleging that they violated federal securities laws between June 24, 2014 and January 4, 2018. On August 7, 2018, the lead plaintiffs, now George Lehman and Insured Benefit Plans, Inc., filed an amended complaint, stating the class period to be April 8, 2014 through January 4, 2018. The plaintiffs did not quantify any alleged damages in their complaint but, in addition to attorneys' fees and costs, they seek to maintain the action as a class action and to recover damages on behalf of themselves and other persons who purchased or otherwise acquired Ohr common stock during the putative class period and purportedly suffered financial harm as a result. The Company and the individuals dispute these claims and intend to defend the matter vigorously. On September 17, 2018, Ohr filed a motion to dismiss the complaint. On September 20, 2019, the Court entered an order granting the defendants' motion to dismiss. On October 23, 2019, the plaintiffs filed a notice of appeal of that order dismissing the action and other related orders by the Court, and the plaintiffs filed their appellate brief with respect to such matters with the Court on February 5, 2020. Scheduled briefing on plaintiffs' appeal has concluded, and oral argument is calendared for September 21, 2020. This litigation could result in substantial costs and a diversion of management's resources and attention, which could harm the Company's business and the value of its common stock.

On May 3, 2018, plaintiff Adele J. Barke, derivatively on behalf of Ohr, commenced an action against certain former directors of Ohr, including Michael Ferguson, Orin Hirschman, Thomas M. Riedhammer, June Almenoff and Jason S. Slakter in the Supreme Court, State of New York, alleging that the action was brought in the right and for the benefit of Ohr seeking to remedy their "breach of fiduciary duties, corporate waste and unjust enrichment that occurred between June 24, 2014 and the present." It does not quantify any alleged damages. The Company and the individuals dispute these claims and intend to defend the matter vigorously. Such litigation has been stayed pursuant to a stipulation by the parties, which has been so ordered by the court, pending a decision in the Southern District case on the motion to dismiss, but that status could change. This litigation could result in substantial costs and a diversion of management's resources and attention, which could harm the Company's business and the value of its common stock.

**NeuBase Therapeutics, Inc. and Subsidiaries**  
**Notes to Condensed Consolidated Financial Statements**  
**(Unaudited)**

On March 20, 2019, a putative class action lawsuit was filed in the United States District Court for District of Delaware naming as defendants Ohr and its board of directors, Legacy NeuBase, and Merger Sub, captioned *Wheby v. Ohr Pharmaceutical, Inc., et al.*, Case No. 1:19-cv-00541-UNA (the “Wheby Action”). The plaintiffs in the Wheby Action allege that the preliminary joint proxy/prospectus statement filed by Ohr with the Securities and Exchange Commission (“SEC”) on March 8, 2019 contained false and misleading statements and omitted material information in violation of Section 14(a) of the Securities Exchange Act of 1934, as amended (the “Exchange Act”) and SEC Rule 14a-9 promulgated thereunder, and further that the individual defendants are liable for those alleged misstatements and omissions under Section 20(a) of the Exchange Act. The complaint in the Wheby Action has not been served on, nor was service waived by, any of the named defendants in that action. The action seeks, among other things, to rescind the Ohr Acquisition or an award of damages, and an award of attorneys’ and experts’ fees and expenses. The defendants dispute the claims raised in the Wheby Action. Management believes that the likelihood of an adverse decision from the sole remaining action is unlikely; however, the litigation could result in substantial costs and a diversion of management’s resources and attention, which could harm the Company’s business and the value of the Company’s common stock.



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

### *Disclosures Regarding Forward-Looking Statements*

*The following should be read in conjunction with the unaudited condensed consolidated financial statements and the related notes that appear elsewhere in this report as well as in conjunction with the Risk Factors section in our Annual Report on Form 10-K for the year ended September 30, 2019 as filed with the United States Securities and Exchange Commission (“SEC”) on January 10, 2020. This report and our Form 10-K include forward-looking statements made based on current management expectations pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, as amended.*

*This report includes “forward-looking statements” within the meaning of Section 21E of the Exchange Act. Those statements include statements regarding the intent, belief or current expectations of the Company and its subsidiaries and our management team. Any such forward-looking statements are not guarantees of future performance and involve risks and uncertainties, and actual results may differ materially from those projected in the forward-looking statements. These risks and uncertainties include but are not limited to those risks and uncertainties set forth in Part II, Item 1A – Risk Factors of this Quarterly Report and in Part I, Item 1A – Risk Factors of our Annual Report. In light of the significant risks and uncertainties inherent in the forward-looking statements included in this Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K, the inclusion of such statements should not be regarded as a representation by us or any other person that our objectives and plans will be achieved. Further, these forward-looking statements reflect our view only as of the date of this report. Except as required by law, we undertake no obligations to update any forward-looking statements and we disclaim any intent to update forward-looking statements after the date of this report to reflect subsequent developments. Accordingly, you should also carefully consider the factors set forth in other reports or documents that we file from time to time with the SEC.*

### **Overview**

#### **Recent Developments**

##### *Announcement of Positive Preclinical Data and Target Milestones*

On March 31, 2020, we announced positive preclinical data from our pharmacokinetics studies in non-human primates (“NHPs”) and *in vitro* pharmacodynamics data in patient-derived cell lines. Our pharmacokinetics studies in NHPs demonstrated, among other things:

- rapid uptake of our PATrOL™-enabled compound out of the body’s circulation after single-dose systemic intravenous administration, with a half-life in circulation of approximately 1.5 hours;
- penetration by our PATrOL™-enabled compound in every organ system studied, including the central nervous system and skeletal muscle; and
- retention of therapeutically relevant doses for greater than one week after single-dose injection.

Our pharmacodynamics studies in patient-derived cell lines demonstrated, among other things:

- activity in engaging target disease-causing transcripts and knocking-down resultant malfunctioning mutant huntingtin (“mHTT”) protein levels preferentially over normal huntingtin (“HTT”) protein knock-down; and
- dose-limiting toxicities were not observed relative to a control either at or above the doses demonstrating activity in human cells *in vitro*.

In addition, PATrOL™ enabled compounds were generally well-tolerated *in vivo* after systemic administration, both after single-dose administration in NHPs and multi-dose administration in mice for over a month.

We believe that the data from these studies support the advancement of the Company’s HD and DM1 programs into lead optimization and subsequent IND-enabling studies. We announced the following target milestones for our NT0100 and NT0200 programs:

#### NT0100 Program for HD

- Lead candidate selection by the end of the calendar year 2020
- Initiation of IND-enabling studies during the first half of the calendar year 2021
- IND filing by the end of the calendar year 2021
- Clinical data by the end of the calendar year 2022

#### NT0200 Program for DM1

- Lead candidate selection during the first half of the calendar year 2021
- Initiation of IND-enabling studies during the second half of the calendar year 2021
- IND filing during the second half of the calendar year 2022

The intersection of the NHP pharmacokinetic data and the *in vitro* patient-derived pharmacodynamic data provides a roadmap to create a pipeline of therapeutic candidates which can reach target tissues of interest after systemic administration and achieve the desired activity at that dose.

#### **Description of the Company**

We are a biotechnology company focused on developing next-generation therapies to treat rare genetic diseases and cancer caused by mutant genes. Our modular peptide-nucleic acid antisense oligonucleotide (“PATrOL™”) platform is designed to improve upon current gene silencing treatments by combining the specificity of antisense oligonucleotides (“ASOs”) with the broad tissue distribution capabilities of small molecules. Given that every human disease may have a genetic component, we believe that our differentiated platform technology has the potential for broad impact. We plan to use our platform to address genetic diseases and we are initially focused on Huntington’s disease (“HD”) and myotonic dystrophy type 1 (“DM1”).

Mutated proteins resulting from errors in deoxyribonucleic acid (“DNA”) sequences cause many rare genetic diseases and cancer. DNA in each cell of the body is transcribed into pre-messenger ribonucleic acid (“mRNA”), which is then processed (spliced) into mRNA, which is exported into the cytoplasm of the cell and translated into protein. This is termed the “central dogma” of biology. Therefore, when errors in a DNA sequence occur, they are propagated to RNAs and can become a damaging protein.

We are developing ASO therapies. ASOs are short single strands of nucleic acids (traditionally thought of as single-stranded RNA molecules) which will bind to defective RNA targets in cells and inhibit their ability to be translated into defective proteins. We believe we are a leader in the discovery and development of the class of RNA-targeted ASO drugs called peptide-nucleic acids (“PNAs”). Our proprietary PATrOL™ platform allows for a more efficient discovery of drug product candidates, potentially transforming the treatment paradigm for people affected by rare genetic diseases and cancer.

The PATrOL™ platform allows for a potentially more efficient discovery of drug product candidates because of manufacturing consistency and because we are not constrained by folded regions of the target RNA molecule (secondary structures). The peptide backbone of our ASOs is rigid, and once linked together to form a series of backbone subunits, forms a single pre-organized structure.

At a more detailed level, each subunit of the peptide backbone has only a single chiral center – a point in the chemical structure where the conformation of the backbone could fluctuate – and this chiral center is locked into one conformation, and thus, is pre-organized to form only a single conformation or stereoisomer. A stereoisomer is a term used in the ASO therapeutics field to mean a string of backbone subunits with attached nucleobases that are linked together into a specific sequence that matches (complements) the target RNA sequence; however, because of the nature of the backbone subunits used, the drug assumes various conformations often with varying affinity for the target sequence. These stereoisomers often require a manufacturing step to purify the heterogeneous mixture of conformations into a more homogenous mixture or even a single conformation of the drug in order to obtain the intended therapeutic effect. Our PNAs assume predominantly a single conformation with any constellation of nucleobases added to the backbone or any oligomer length. This backbone also has a neutral charge, as opposed to the negatively charged backbones of DNA and RNA. This neutral charge allows our ASO to open up RNAs which are folded upon themselves and bind to their target sequence. This potentially accelerates identification of drug candidates which have the desired activity.

In addition to the backbone conformational purity that allows for more efficient discovery of drug product candidates, we also have a kit of proprietary bi-facial (also known as bi-specific) nucleotides (traditional nucleotides only have a single binding face and thus are restricted to only binding single-stranded RNA targets) which can be used in any combination to access RNA secondary structures (double stranded RNA targets which are folded upon themselves) such as hairpins. This allows us to potentially access regions of the target transcript, which may be unique in secondary structure to allow enhanced selectivity for the target (mutant) RNA as compared to the normal RNA. Enhanced selectivity for mutant RNAs as compared to normal RNAs is critical as normal RNAs are likely required for effective functioning of the cell. These bi-specific nucleotides can also target genomic loci and microRNAs in their double-stranded form.

In addition to the backbone and modified nuclear bases, the platform toolkit also includes linker technology which, when added to both ends of the PNAs, allow cooperative binding between individual drug molecules once they are engaged with the target RNA to form longer and more tightly bound drugs.

The final component of the platform is a chemical moiety, which is used to decorate the peptide backbone in a proprietary manner and allows the PNAs to penetrate cell membranes and distribute throughout the body when administered systemically, including across the blood brain barrier and into the central nervous system.

This relatively simple toolkit of components forms the PATrOL™ platform and allows us to manufacture genome and transcript-specific PNAs quickly for screening.

We are currently focused on therapeutic areas in which we believe our drugs will provide the greatest benefit with a significant market opportunity. We intend to utilize our technology to build out a pipeline of custom designed therapeutics for additional high-value disease targets. We are developing several preclinical programs using our PATrOL™ platform, including the NT0100 program, targeted at HD, a repeat expansion disorder, and the NT0200 program, targeted at DM1. Preclinical studies are being conducted to evaluate the PATrOL™ platform technology and program candidates in the areas of pharmacokinetics, pharmacodynamics, and safety, and we reported results from certain of those studies in the first calendar quarter of 2020. We expect to report the results of certain additional studies in the fourth quarter of calendar year 2020 via press release or in a scientific publication or presentation at a scientific conference. In addition, the emerging pipeline of other assets that target primary and secondary RNA structure and genomic DNA allows a unique market advantage across a variety of rare diseases and oncology targets.

Using our PATrOL™ platform, we believe we can create ASOs that have distinct advantages over other chemical entities currently in the market or in development for gene silencing applications. These advantages include, among others: a backbone that has only one chiral center and thus forms predominantly only one stereoisomer; the ability of the PNA backbone to invade, open up secondary and tertiary structures (RNA molecules that interact with other RNA molecules in the cell) and bind within these double-stranded RNA in a highly selective manner; a proprietary set of engineered nucleobases that increase selectivity to specific target sequences including secondary and tertiary structures that has been licensed exclusively from Carnegie Mellon University (“CMU”); technology to allow self-assembly of our small PNAs at the RNA target to increase selectivity which has been licensed exclusively from CMU; the ability to modulate cell permeability and be broadly distributed throughout the body after systemic administration including into the brain; the lack of innate or acquired immune responses of similar PNAs in preclinical models; and potential minimal toxicity based on previous *in-vivo* studies in rodent models. With these advantages, our PATrOL™ platform-enabled therapies can potentially address a multitude of rare genetic diseases and cancer, among other indications.

## Product Pipeline

### *Huntington’s Disease*

HD is a devastating rare neurodegenerative disorder. After onset, symptoms such as uncontrolled movements, cognitive impairments and emotional disturbances worsen over time. HD is caused by toxic aggregation of mutant huntingtin protein, leading to progressive neuron loss in the striatum and cortex of the brain. The wildtype huntingtin gene (“*HTT*”) has a region in which a three-base DNA sequence, CAG, is repeated many times. When the DNA sequence CAG is repeated 26 or fewer times in this region, the resulting protein behaves normally. While the wildtype function of *HTT* is largely uncharacterized, the protein is known to be essential for normal brain development. When the DNA sequence CAG is repeated 40 times or more in this region, the resulting protein becomes toxic and causes HD. Every person has two copies, or alleles, of the *HTT*. Only one of the alleles (the “mutant” allele) needs to bear at least 40 CAG repeats for HD to occur. HD is one of many known repeat expansion disorders, which are a set of genetic disorders caused by a mutation that leads to a repeat of nucleotides exceeding the normal threshold. Current therapies for patients with HD can only manage individual symptoms. There is no approved therapy that has been shown to delay or halt disease progression. There are approximately 30,000 symptomatic patients in the U.S. and more than 200,000 at-risk of inheriting the disease globally.

#### ***NT0100 Program – PATrOL™ Enabled PNA for Huntington’s Disease***

We are initially focused on HD, a fatal rare genetic repeat expansion disorder with no viable treatment options.

One especially important advantage of the PATrOL™ platform that makes it promising for the treatment of repeat expansion disorders like HD is the ability of our small ASOs to potentially self-assemble within an RNA hairpin. As the number of repeats increases, the PATrOL™ oligonucleotides bind more tightly to each other and the mutant RNA. This allows our therapies to potentially inactivate mutant *HTT* mRNA before it can be translated into harmful protein via selective binding to the expanded CAG repeats while leaving the normal *HTT* mRNA largely unbound to drug and producing functional protein. Achieving mutant allele selectivity would be a key advantage for any RNA-based approach aiming to treat HD. The PATrOL™-enabled NT0100 program is currently in preclinical development for the treatment of HD.

#### ***NT0200 Program – PATrOL™ Enabled PNA for Myotonic Dystrophy Type 1***

Our pipeline also contains a second potentially transformative medicine, which we believe has significant potential for DM1, a severe and rare trinucleotide repeat disease. DM1 is a multisystem disorder that primarily affects skeletal and smooth muscle. DM1 is caused by expansion of a CTG trinucleotide repeat in the noncoding region of the DM1 Protein Kinase gene (*DMPK*), which captures and sequesters splice proteins. Sequestered splice proteins cannot then fulfill their normal functions. The diagnosis of DM1 is suspected in individuals with characteristic muscle weakness and is confirmed by molecular genetic testing of *DMPK*. CTG repeat length exceeding 34 repeats is abnormal. Molecular genetic testing detects pathogenic variants in nearly 100% of affected individuals. It is estimated that the global prevalence of DM1 is 1 in 20,000 individuals. Our development strategy targets the DM1 expanded allele with PATrOL™-enabled drug candidates to disrupt and/or open the mutant hairpin and allow release of sequestered splice proteins.

#### ***Additional Indications***

In addition, we are in the process of building an early stage pipeline of other therapies that focus on the unique advantages of our technology across a variety of diseases.

#### **Critical Accounting Estimates and Policies**

The preparation of financial statements in accordance with United States generally accepted accounting principles (“U.S. GAAP”) requires management to make estimates and assumptions that affect the amounts reported in our unaudited condensed consolidated financial statements and accompanying notes. Management bases its estimates on historical experience, market and other conditions, and various other assumptions it believes to be reasonable. Although these estimates are based on management’s best knowledge of current events and actions that may impact us in the future, the estimation process is, by its nature, uncertain given that estimates depend on events over which we may not have control. If market and other conditions change from those that we anticipate, our unaudited condensed consolidated financial statements may be materially affected. In addition, if our assumptions change, we may need to revise our estimates, or take other corrective actions, either of which may also have a material effect in our unaudited condensed consolidated financial statements. We review our estimates, judgments, and assumptions used in our accounting practices periodically and reflect the effects of revisions in the period in which they are deemed to be necessary. We believe that these estimates are reasonable; however, our actual results may differ from these estimates.

Our critical accounting policies and estimates are discussed in our Annual Report on Form 10-K for the fiscal year ended September 30, 2019 and there have been no material changes to such policies or estimates during the nine months ended June 30, 2020.

#### **Recent Accounting Pronouncements**

Please refer to Note 2, *Significant Accounting Policies—Recent Accounting Pronouncements*, in Item 1, *Financial Statements* for a discussion of recent accounting pronouncements.

## Results of Operations

Results of operations for the quarter ended June 30, 2020 reflect the following changes from the quarter ended June 30, 2019:

	Three Months Ended June 30,		Change
	2020	2019	
<b>OPERATING EXPENSES</b>			
General and administrative expenses	\$ 2,331,283	\$ 1,655,189	\$ 676,094
Research and development expenses	1,492,881	305,204	1,187,677
<b>TOTAL OPERATING EXPENSES</b>	<b>3,824,164</b>	<b>1,960,393</b>	<b>1,863,771</b>
<b>LOSS FROM OPERATIONS</b>	<b>(3,824,164)</b>	<b>(1,960,393)</b>	<b>(1,863,771)</b>
<b>OTHER INCOME (EXPENSE)</b>			
Interest expense	(1,573)	(60,944)	59,371
Change in fair value of warrant liabilities	(285,284)	(13,240)	(272,044)
Equity in losses on equity method investment	(101,238)	-	(101,238)
Other income	412,371	-	412,371
Total income (expenses), net	24,276	(74,184)	98,460
<b>NET LOSS</b>	<b>\$ (3,799,888)</b>	<b>\$ (2,034,577)</b>	<b>\$ (1,765,311)</b>

During the quarter ended June 30, 2020, our operating loss increased by \$1.9 million compared to the quarter ended June 30, 2019. Our net loss increased by \$1.8 million for the quarter ended June 30, 2020, as compared to the quarter ended June 30, 2019. Until we are able to generate revenue from product sales, our management expects to continue to incur net losses.

### *General and Administrative Expenses*

General and administrative expenses consist primarily of legal and professional fees, wages and stock-based compensation. General and administrative expenses increased by \$0.7 million for the quarter ended June 30, 2020, as compared to the quarter ended June 30, 2019, primarily due to an increase in stock-based compensation expense, employee head count and need for legal and professional services.

### *Research and Development Expenses*

Research and development expenses consist primarily of professional fees, research, development, and manufacturing expenses, and wages and stock-based compensation. Research and development expenses increased by \$1.2 million for the quarter ended June 30, 2020, as compared to the quarter ended June 30, 2019, primarily due to an increase in stock-based compensation, employee head count and the ramp up of research and development activities.

### *Interest Expense*

Interest expense consists primarily of interest on convertible notes and notes payable. Interest expense decreased by \$0.06 million for the quarter ended June 30, 2020, as compared to the quarter ended June 30, 2019 primarily due to a decrease in outstanding convertible notes. No convertible notes were outstanding during the quarter ended June 30, 2020.

### *Change in fair value of warrant liabilities*

Change in fair value of warrant liabilities reflects the changes in the fair value of outstanding warrants which is primarily driven by changes in our stock price. Change in fair value of warrant liabilities was a loss of \$0.3 million for the quarter ended June 30, 2020, as compared to a loss of \$0.01 million for the quarter ended June 30, 2019, due to the change in valuation of warrants acquired in the Merger with Ohr, which did not exist in the comparative prior year period.

### *Equity in losses on equity method investment*

The Company accounts for its investment in DepYmed common shares using the equity method of accounting and records its proportionate share of DepYmed's net income and losses. Equity in losses for the three months ended June 30, 2020 was approximately \$0.1 million. No such investment existed in the comparative prior year period.

### **Other income**

Other income reflects the Company's license, and completed transfer, of certain clinical trial data during the three months ended June 30, 2020. No such license and data transfer occurred during the comparative prior year period.

**Results of operations for the nine months ended June 30, 2020 reflect the following changes from the nine months ended June 30, 2019:**

	<u>Nine Months Ended June 30,</u>		<u>Change</u>
	<u>2020</u>	<u>2019</u>	
<b>OPERATING EXPENSES</b>			
General and administrative expenses	\$ 7,624,984	\$ 3,991,567	\$ 3,633,417
Research and development expenses	4,336,576	343,206	3,993,370
Research and development expense- license acquired	-	1,046,965	(1,046,965)
<b>TOTAL OPERATING EXPENSES</b>	<u>11,961,560</u>	<u>5,381,738</u>	<u>6,579,822</u>
<b>LOSS FROM OPERATIONS</b>	<u>(11,961,560)</u>	<u>(5,381,738)</u>	<u>(6,579,822)</u>
<b>OTHER INCOME (EXPENSE)</b>			
Interest expense	(3,226)	(119,195)	115,969
Change in fair value of warrant liabilities	(909,474)	(51,942)	(857,532)
Loss on disposal of fixed asset	(3,230)	-	(3,230)
Equity in losses on equity method investment	(218,589)	-	(218,589)
Other income	412,371	-	412,371
<b>Total other expenses, net</b>	<u>(722,148)</u>	<u>(171,137)</u>	<u>(551,011)</u>
<b>NET LOSS</b>	<u>\$ (12,683,708)</u>	<u>\$ (5,552,875)</u>	<u>\$ (7,130,833)</u>

During the nine months ended June 30, 2020, our operating loss increased by \$6.6 million compared to the nine months ended June 30, 2019. Our net loss increased by \$7.1 million for the nine months ended June 30, 2020, as compared to the nine months ended June 30, 2019. Until we are able to generate revenue from product sales, our management expects to continue to incur net losses.

### **General and Administrative Expenses**

General and administrative expenses consist primarily of legal and professional fees, wages and stock-based compensation. General and administrative expenses increased by \$3.6 million for the nine months ended June 30, 2020, as compared to the nine months ended June 30, 2019, primarily due to an increase in stock-based compensation expense, employee head count and need for legal and professional services.

### **Research and Development Expenses**

Research and development expenses consist primarily of professional fees, research, development, and manufacturing expenses, and wages and stock-based compensation. Research and development expenses increased by \$4.0 million for the nine months ended June 30, 2020, as compared to the nine months ended June 30, 2019, primarily due to an increase in stock-based compensation, employee head count and the ramp up of research and development activities.

### **Research and Development Expense- licenses acquired**

Research and development expense- licenses acquired during the nine months ended June 30, 2019 consists of the license acquired from CMU. Research and development expense- licenses acquired decreased by \$1.0 million, for the nine months ended June 30, 2020, as compared to the nine months ended June 30, 2019, due to our acquisition of license rights in the 2019 period.

### **Interest Expense**

Interest expense consists primarily of interest on convertible notes and notes payable. Interest expense decreased by \$0.1 million for the nine months ended June 30, 2020, as compared to the nine months ended June 30, 2019, primarily due to a decrease in convertible notes. No convertible notes were outstanding during the nine months ended June 30, 2020.

### ***Change in fair value of warrant liabilities***

Change in fair value of warrant liabilities reflects the changes in the fair value of outstanding warrants which is primarily driven by changes in our stock price. Change in fair value of warrant liabilities was \$0.9 million for the nine months ended June 30, 2020, as compared to \$0.05 million for the nine months ended June 30, 2019, due to the change in valuation of warrants acquired in the Merger with Ohr, which did not exist in the comparative prior year period.

### ***Equity in losses on equity method investment***

The Company accounts for its investment in DepYmed common shares using the equity method of accounting and records its proportionate share of DepYmed's net income and losses. Equity in losses for the nine months ended June 30, 2020 was approximately \$0.2 million. No such investment existed in the comparative prior year period.

### ***Other income***

Other income reflects the Company's license, and completed transfer, of certain clinical trial data during the nine months ended June 30, 2020. No such license and data transfer occurred during the comparative prior year period.

### **Liquidity, Capital Resources and Financial Condition**

We have incurred substantial operating losses since our inception and expect to continue to incur significant operating losses for the foreseeable future and may never become profitable. As of June 30, 2020, we had an accumulated deficit of \$39.7 million. We are reliant, at present, upon our capital reserves for ongoing operations and have no product revenue.

Net working capital increased from September 30, 2019 to the period ended June 30, 2020 by \$24.9 million (to \$33.4 million from \$8.5 million) primarily due to our April 2020 underwritten public offering of 6,037,500 shares of common stock, at a price to the public of \$6.00 per share, offset, in part, by costs for the development of our PATROL™ platform technology and lead programs. We received net proceeds from the offering of approximately \$33.3 million, after deducting the underwriting discounts and commissions and other estimated offering expenses payable by the Company. Our cash burn for the period increased significantly compared to prior periods due to increased research and development activities. We anticipate that our cash needs in the future will increase relative to prior periods as we proceed with our research and development objectives.

We believe that our current cash balance will provide sufficient capital to continue operations into the second quarter of calendar 2022. In particular, we expect that these funds will allow us to achieve certain milestones for our NT0100 program for HD and our NT0200 program for DM1, but we expect that we will need to obtain additional funding to obtain clinical data for our NT0100 program and to submit an IND for our NT0200 program. Despite these expectations, our forecast of the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement that involves risks and uncertainties, and actual results could vary materially. We have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we expect. We will continue to assess our working capital requirements, and, if circumstances warrant, we will make appropriate adjustments to our operating plan. Furthermore, we are closely monitoring ongoing developments in connection with the COVID-19 pandemic, which may negatively impact our commercial prospects in fiscal 2020 and beyond. Please see Part II, Item 1A – Risk Factors —“*Our operations may be adversely affected by the coronavirus outbreak, and we face risks that could impact our business*” for further discussion of the effect of the COVID-19 pandemic on our operations.

At present, we have no bank line of credit or other fixed source of capital reserves. Should we need additional capital in the future, we will be primarily reliant upon a private or public placement of our equity or debt securities, or a strategic transaction, for which there can be no warranty or assurance that we may be successful in such efforts. If the Company is unable to maintain sufficient financial resources, its business, financial condition and results of operations will be materially and adversely affected. This could affect future development and business activities and potential future clinical studies and/or other future ventures. Failure to obtain additional equity or debt financing will have a material, adverse impact on the Company's business operations. There can be no assurance that the Company will be able to obtain the needed financing to achieve its goals on acceptable terms or at all.

## Cash Flow Summary

The following table summarizes selected items in our unaudited condensed consolidated statements of cash flows:

	Nine Months Ended June 30,	
	2020	2019
Net cash used in operating activities	\$ (7,142,340)	\$ (394,779)
Net cash used in investing activities	(362,915)	(379,380)
Net cash provided by financing activities	33,116,045	592,656
Net increase (decrease) in cash and cash equivalents	\$ 25,610,790	\$ (181,503)

### *Operating Activities*

Net cash used in operating activities was approximately \$7.1 million for the nine months ended June 30, 2020, as compared to approximately \$0.4 million for the nine months ended June 30, 2019. Net cash used in operating activities in the nine months ended June 30, 2020 was primarily the result of our net loss, offset by our stock-based compensation expense, the change in fair value of warrant liabilities, depreciation and amortization expense and the loss on equity method investment. Net cash used in operating activities in the nine months ended June 30, 2019 was primarily the result of our net loss, offset by research and development expense-license acquired, stock based compensation and changes in operating assets and liabilities.

### *Investing Activities*

Net cash used in investing activities was approximately \$0.4 million for the nine months ended June 30, 2020, as compared to \$0.4 million for the nine months ended June 30, 2019. Net cash used in investing activities in the nine months ended June 30, 2020 was primarily the result of purchases of laboratory equipment. Net cash used in investing activities in the nine months ended June 30, 2019 was primarily the result of purchases of laboratory equipment and cash consideration and expenses paid in connection with the acquisition of the CMU License.

### *Financing Activities*

Net cash provided by financing activities was approximately \$33.1 million for the nine months ended June 30, 2020, as compared to \$0.6 million for the nine months ended June 30, 2019. Net cash provided by financing activities for the nine months ended June 30, 2020 reflects the proceeds from the issuance of common stock of \$33.3 million, net of issuance costs, and the principal payments of financed insurance. Net cash provided by financing activities for the nine months ended June 30, 2019 primarily reflects the net proceeds received from the issuance of convertible notes.

## Off-Balance Sheet Arrangements

As of June 30, 2020, we did not have any off-balance sheet arrangements as defined in Item 303(a)(4)(ii) of Regulation S-K.



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

We are a smaller reporting company, as defined by Rule 12b-2 of the Securities Exchange Act of 1934, as amended, and are not required to provide the information required under this item.

### ITEM 4. CONTROLS AND PROCEDURES

#### *Evaluation of Disclosure Controls and Procedures*

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, has evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) as of June 30, 2020. Based on that evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that as of June 30, 2020, due to the existence of the material weakness in our internal control over financial reporting described below, our disclosure controls and procedures were not effective to provide reasonable assurance that the information required to be disclosed in the reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms, and that such information is accumulated and communicated to management as appropriate to allow timely decisions regarding required disclosure.

#### *Material Weakness in Internal Control over Financial Reporting*

As previously disclosed in "Item 9A – Controls and Procedures" of our Annual Report on Form 10-K for the fiscal year ended September 30, 2019, in connection with the preparation of the Company's consolidated financial statements for the fiscal year ended September 30, 2019, but prior to the issuance of those financial statements, our management and the audit committee of our board of directors determined that our accounting treatment and valuations pertaining to the PATrOL™ technology license acquired during the first quarter of fiscal 2019 should be modified. This change in accounting treatment resulted in an increase in total operating expenses of approximately \$0.9 million on our consolidated statements of operations for the fiscal year ended September 30, 2019 and a decrease in intangible assets of approximately \$1.5 million on our consolidated balance sheet as of and for the fiscal year ended September 30, 2019. In connection with the valuation adjustments to the PATrOL™ technology license, we also determined that valuations pertaining to certain share-based awards, due to their proximity to the valuation of the consideration issued in connection with the PATrOL™ license, should also be adjusted. This change in valuation to share-based awards resulted in a decrease in total operating expenses of approximately \$0.3 million on our consolidated statements of operations for the fiscal year ended September 30, 2019.

In connection with such revisions, our management identified a material weakness in our internal control over financial reporting due to a lack of expertise in complex accounting transactions, the controls over which were not operating effectively to provide reasonable assurance that complex transactions were accounted for correctly. A material weakness is defined as a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected on a timely basis. This material weakness resulted in misstatements that were corrected in the restatements included in our Quarterly Reports on Form 10-Q for the quarters ended December 31, 2019 and March 31, 2020.

During the fiscal year ended September 30, 2019, we undertook remediation measures by hiring a financial accounting consultant to provide accounting advisory services on complex transactions and accounting matters. We continue to implement these remedial actions, but these efforts are not complete and are ongoing and subject to ongoing management review and the oversight of our board of directors.

#### *Changes in Internal Control over Financial Reporting*

There have been no changes in our internal control over financial reporting that materially affected, or are reasonably likely to materially affect, our internal control over financial reporting during the quarterly period ended June 30, 2020.

## PART II.

### ITEM 1. LEGAL PROCEEDINGS

We have become involved in certain legal proceedings and claims which arise in the normal course of business. If an unfavorable ruling were to occur, there exists the possibility of a material adverse impact on our results of operations, prospects, cash flows, financial position and brand.

On February 14, 2018, plaintiff Jeevesh Khanna, commenced an action in the Southern District of New York, against Ohr and several current and former officers and directors, alleging that they violated federal securities laws between June 24, 2014 and January 4, 2018. On August 7, 2018, the lead plaintiffs, now George Lehman and Insured Benefit Plans, Inc., filed an amended complaint, stating the class period to be April 8, 2014 through January 4, 2018. The plaintiffs did not quantify any alleged damages in their complaint but, in addition to attorneys' fees and costs, they seek to maintain the action as a class action and to recover damages on behalf of themselves and other persons who purchased or otherwise acquired Ohr common stock during the putative class period and purportedly suffered financial harm as a result. We and the individuals dispute these claims and intend to defend the matter vigorously. On September 17, 2018, Ohr filed a motion to dismiss the complaint. On September 20, 2019, the Court entered an order granting the defendants' motion to dismiss. On October 23, 2019, the plaintiffs filed a notice of appeal of that order dismissing the action and other related orders by the Court, and the plaintiffs filed their appellate brief with respect to such matters with the Court on February 5, 2020. Scheduled briefing on plaintiffs' appeal has concluded, and oral argument is calendared for September 21, 2020. This litigation could result in substantial costs and a diversion of management's resources and attention, which could harm our business and the value of our common stock.

On May 3, 2018, plaintiff Adele J. Barke, derivatively on behalf of Ohr, commenced an action against certain former directors of Ohr, including Michael Ferguson, Orin Hirschman, Thomas M. Riedhammer, June Almenoff and Jason S. Slakter in the Supreme Court, State of New York, alleging that the action was brought in the right and for the benefit of Ohr seeking to remedy their "breach of fiduciary duties, corporate waste and unjust enrichment that occurred between June 24, 2014 and the present." It does not quantify any alleged damages. We and the individuals dispute these claims and intend to defend the matter vigorously. Such litigation has been stayed pursuant to a stipulation by the parties, which has been so ordered by the court, pending a decision in the Southern District case on the motion to dismiss, but that status could change. This litigation could result in substantial costs and a diversion of management's resources and attention, which could harm our business and the value of our common stock.

On March 20, 2019, a putative class action lawsuit was filed in the United States District Court for District of Delaware naming as defendants Ohr and its board of directors, Legacy NeuBase, and Ohr Acquisition Corp., captioned *Wheby v. Ohr Pharmaceutical, Inc., et al.*, Case No. 1:19-cv-00541-UNA (the "Wheby Action"). The plaintiffs in the Wheby Action allege that the preliminary joint proxy/prospectus statement filed by Ohr with the SEC on March 8, 2019 contained false and misleading statements and omitted material information in violation of Section 14(a) of the Exchange Act and SEC Rule 14a-9 promulgated thereunder, and further that the individual defendants are liable for those alleged misstatements and omissions under Section 20(a) of the Exchange Act. The complaint in the Wheby Action has not been served on, nor was service waived by, any of the named defendants in that action. The action seeks, among other things, to rescind the Merger or an award of damages, and an award of attorneys' and experts' fees and expenses. The defendants dispute the claims raised in the Wheby Action. Management believes that the likelihood of an adverse decision from the sole remaining action is unlikely; however, the litigation could result in substantial costs and a diversion of management's resources and attention, which could harm our business and the value of our common stock.

## ITEM 1A. RISK FACTORS

*We operate in a dynamic and rapidly changing environment that involves numerous risks and uncertainties. Certain factors may have a material adverse effect on our business, prospects, financial condition and results of operations, and you should carefully consider them. Accordingly, in evaluating our business, we encourage you to consider the following discussion of risk factors, in its entirety, in addition to other information contained in this Quarterly Report on Form 10-Q and our other public filings with the Securities and Exchange Commission (the "SEC"). Other events that we do not currently anticipate or that we currently deem immaterial may also affect our business, prospects, financial condition and results of operations. Any material changes from risk factors as previously disclosed in our Annual Report on Form 10-K are marked herein with three asterisks (\*\*\*)*

### **Risks Relating to the Company**

***We are a preclinical-stage company, have a very limited operating history, are not currently profitable, do not expect to become profitable in the near future and may never become profitable.***

We are a preclinical-stage biotechnology company specializing in the discovery and development of a class of deoxy-ribonucleic acid and ribonucleic acid-targeted drugs called peptide nucleic acids, which did not change as a result of the merger between Ohr Pharmaceutical, Inc., a Delaware corporation ("Ohr"), and NeuBase Therapeutics, Inc., a Delaware corporation ("Legacy NeuBase"), in accordance with the terms of the Agreement and Plan of Merger Reorganization entered into on January 2, 2019 (the "Merger Agreement"). Since our incorporation, we have focused primarily on the development of our proprietary Peptide-nucleic acid Antisense Oligonucleotide ("PATrOL™") platform and preclinical-stage therapeutic candidates. Our platform technology and all of our therapeutic candidates are in the preclinical development stage, and we have not initiated clinical trials for any of our product candidates, nor have any products been approved for commercial sale and we have not generated any revenue. To date, we have not completed a clinical trial (including a pivotal clinical trial), obtained marketing approval for any product candidates, manufactured a commercial scale product or arranged for a third party to do so on our behalf, or conducted sales and marketing activities necessary for successful product commercialization. Drug development is also a highly uncertain undertaking and involves a substantial degree of risk.

As a result, we have no meaningful historical operations upon which to evaluate our business and prospects and have not yet demonstrated an ability to obtain marketing approval for any of our product candidates or successfully overcome the risks and uncertainties frequently encountered by companies in the pharmaceutical industry. We also have not generated any revenues from collaboration and licensing agreements or product sales to date and continue to incur research and development and other expenses. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' deficit and working capital, and our future success is subject to significant uncertainty.

For the foreseeable future, we expect to continue to incur losses, which will increase significantly from recent historical levels as we expand our drug development activities, seek regulatory approvals for our product candidates and begin to commercialize them if they are approved by the U.S. Food and Drug Administration (the "FDA"), the European Medicines Agency (the "EMA") or comparable foreign authorities. Even if we succeed in developing and commercializing one or more product candidates, we may never become profitable.

***The approach we are taking to discover and develop nucleic acid therapeutics is novel and may never lead to marketable products.***

We have concentrated our efforts and research and development activities on nucleic acid therapeutics and our synthetic chemistry drug discovery and development platform comprised of peptide nucleic acids with natural and engineered nucleotides. Our future success depends on the successful development and manufacturing of such therapeutics and the effectiveness of our platform. The scientific discoveries that form the basis for our efforts to discover and develop new drugs, including our discoveries about the relationships between oligonucleotide stereochemistry and pharmacology, are relatively new. The scientific evidence to support the feasibility of developing drugs based on these discoveries or peptide nucleic acids ("PNAs") in general is limited. Skepticism as to the feasibility of developing nucleic acid therapeutics and PNAs generally has been, and may continue to be, expressed in scientific literature. In addition, decisions by, and negative results of, other companies with respect to their oligonucleotide development efforts may increase skepticism in the marketplace regarding the potential for oligonucleotides and PNAs.

Relatively few nucleic acid therapeutic product candidates have been tested in humans, and a number of clinical trials for such therapeutics conducted by other companies have not been successful. Few nucleic acid therapeutics have received regulatory approval. The pharmacological properties ascribed to the investigational compounds we are testing in laboratory studies may not be positively demonstrated in clinical trials in patients, and they may interact with human biological systems in unforeseen, ineffective or harmful ways. If our nucleic acid product candidates prove to be ineffective, unsafe or commercially unviable, our entire platform and pipeline would have little, if any, value, which would substantially harm our business, financial condition, results of operations and prospects.

In addition, our approach, which focuses on using nucleic acid therapeutics for drug development, as opposed to multiple or other, more advanced proven technologies, may expose us to additional financial risks and make it more difficult to raise additional capital if we are not successful in developing a nucleic acid therapeutic that is timely and cost effective to manufacture and achieves proof of concept in animal models, desired tissue distribution, selectivity for the target, and/or regulatory approval. Because our programs are all in the preclinical stage, we have not yet been able to assess safety in humans, and there may be long-term effects from treatment with any product candidates that we develop using our platform that we cannot predict at this time. Any product candidates the Company may develop will act at the level of deoxyribonucleic acid (“DNA”) or ribonucleic acid (“RNA”), and because animal DNA and RNA often differs from human DNA or RNA at the sequence level, in its regulation and degradation, secondary and tertiary structural conformations and ultimately in being translated into proteins with varying amino acid sequences conformations and functions, testing of our product candidates in animal models may not be predictive of the results we observe in human clinical trials of our product candidates for either safety or efficacy. Also, animal models may not exist for some of the diseases we choose to pursue in our programs. As a result of these factors, it is more difficult for us to predict the time and cost of product candidate development, and we cannot predict whether the application of our gene silencing technology, or any similar or competitive gene silencing technologies, will result in the identification, development and regulatory approval of any products. There can be no assurance that any development problems we experience in the future related to our gene silencing technology or any of our research programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. Should we encounter development problems, including unfavorable preclinical or clinical trial results, the FDA and foreign regulatory authorities may refuse to approve our product candidates, or may require additional information, tests or trials, which could significantly delay product development and significantly increase our development costs. Moreover, even if we are able to provide the requested information or trials to the FDA, there would be no guarantee that the FDA would accept them or approve our product candidates. We may also experience delays in developing a sustainable, reproducible and scalable manufacturing process or developing or qualifying and validating product release assays, other testing and manufacturing methods, and our equipment and facilities in a timely manner, which may prevent us from completing our clinical trials or commercializing our product candidates on a timely or profitable basis, if at all. Any of these factors may prevent us from completing our preclinical studies or any clinical trials that we may initiate or from commercializing any product candidates we may develop on a timely or profitable basis, if at all.

***We are highly dependent on the success of our initial product candidates targeting rare genetic diseases and our platform technology in general, and we cannot be certain that any of them will receive regulatory approval or be commercialized.***

We have spent time, money and effort on the licensing and development of our core asset: our PATrOL™ platform. To date, we have not submitted an Investigational New Drug application (“IND”) to the FDA, and no clinical trials have commenced for any of our product candidates. All of our product candidates will require additional development, including further preclinical studies and bioanalytic method development as well as clinical trials to evaluate their toxicology, carcinogenicity and pharmacokinetics, efficacy, and optimize their formulation, and regulatory clearances before they can be commercialized. Positive results obtained during early development do not necessarily mean later development will succeed or that regulatory clearances will be obtained. Our drug development efforts may not lead to commercial drugs, either because our product candidates or our PATrOL™ platform are not deemed safe and effective, because of competitive or market forces, intellectual property issues or because we have inadequate financial or other resources to advance our product candidates through the clinical development and approval processes. If any of our product candidates, or our PATrOL™ platform, fail to demonstrate safety or efficacy at any time or during any phase of development, we would experience potentially significant delays in, or be required to abandon, development of the product candidate.

We do not anticipate that any of our current product candidates will be eligible to receive regulatory approval from the FDA, the EMA or comparable foreign authorities and begin commercialization for a number of years, if ever. Even if we ultimately receive regulatory approval for any of these product candidates, we or our potential future partners, if any, may be unable to commercialize them successfully for a variety of reasons. These include, for example, the availability of alternative treatments, lack of cost-effectiveness, the cost of manufacturing the product on a commercial scale and competition with other drugs. The success of our product candidates and our PATrOL™ platform may also be limited by the prevalence and severity of any adverse side effects. If we fail to commercialize one or more of our current product candidates, we may be unable to generate sufficient revenues to attain or maintain profitability, and our financial condition may decline.

***If development of our candidates does not produce favorable results, we and our collaborators, if any, may be unable to commercialize these products.***

To receive regulatory approval for the commercialization of our use of the PATrOL™ platform, or any product candidates that we may develop, adequate and well-controlled clinical trials must be conducted to demonstrate safety and efficacy in humans to the satisfaction of the FDA, the EMA and comparable foreign authorities. In order to support marketing approval, these agencies typically require successful results in one or more Phase III clinical trials, which our current product candidates have not yet reached and may never reach. The development process is expensive, can take many years and has an uncertain outcome. Failure can occur at any stage of the process. We may experience numerous unforeseen events during, or as a result of, the development process that could delay or prevent commercialization of our current or future product candidates, including the following:

- preclinical studies conducted with product candidates for potential clinical development to evaluate their toxicology, carcinogenicity and pharmacokinetics and optimize their formulation, among other things, may produce unfavorable results;
- patient recruitment and enrollment in clinical trials may be slower than we anticipate;
- clinical trials may produce negative or inconclusive results;
- costs of development may be greater than we anticipate;
- the potential advantages of the PATrOL™-enabled drug candidates may not materialize and thus would confer no benefits to patients over other parties' products that may emerge;
- our product candidates or our PATrOL™ platform may cause undesirable side effects that delay or preclude regulatory approval or limit their commercial use or market acceptance, if approved;
- collaborators who may be responsible for the development of our product candidates may not devote sufficient resources to these clinical trials or other preclinical studies of these candidates or conduct them in a timely manner; or
- we may face delays in obtaining regulatory approvals to commence one or more clinical trials.

Additionally, because our technology potentially involves gene silencing via genome binding and/or editing across multiple cell and tissue types, we are subject to many of the challenges and risks that advanced therapies, such as gene therapies, face, including:

- regulatory requirements governing gene and cell therapy products have changed frequently and may continue to change in the future;
- improper modification of a gene sequence in a patient's genome could lead to lymphoma, leukemia or other cancers, or other aberrantly functioning cells; and
- the FDA recommends a follow-up observation period of 15 years or longer for all patients who receive treatment using gene therapies, and we may need to adopt and support such an observation period for our product candidates.

Success in early development does not mean that later development will be successful because, for example, product candidates in later-stage clinical trials may fail to demonstrate sufficient safety and efficacy despite having progressed through initial clinical trials.

Furthermore, we have licensed or acquired virtually all of the intellectual property related to our product candidates from Carnegie Mellon University (“CMU”). Much of our preclinical studies and other analyses performed to date with respect to our product candidates have been conducted by their original owners or collaborators. Therefore, as a company, we have limited experience in conducting research on our platform technology and preclinical trials for our product candidates. Since our experience with our platform technology and product candidates is limited, we will need to train our existing personnel or hire additional personnel in order to successfully administer and manage our preclinical studies and clinical trials as anticipated, which may result in delays in completing such anticipated preclinical trials and clinical studies.

We currently do not have strategic collaborations in place for clinical development of our platform technology and any of our current product candidates. Therefore, in the future, we or any potential future collaborative partner will be responsible for establishing the targeted endpoints and goals for development of our product candidates. These targeted endpoints and goals may be inadequate to demonstrate the safety and efficacy levels required for regulatory approvals. Even if we believe data collected during the development of our product candidates are promising, such data may not be sufficient to support marketing approval by the FDA, the EMA or comparable foreign authorities. Further, data generated during development can be interpreted in different ways, and the FDA, the EMA or comparable foreign authorities may interpret such data in different ways than we or our collaborators. Our failure to adequately demonstrate the safety and efficacy of our platform technology and any of our product candidates would prevent our receipt of regulatory approval, and such failure would ultimately prevent the potential commercialization of these product candidates.

Since we do not currently possess the resources necessary to independently develop and commercialize our product candidates or any other product candidates that we may develop, we may seek to enter into collaborative agreements to assist in the development and potential future commercialization of some or all of these assets as a component of our strategic plan. Our discussions with potential collaborators, however, may not lead to the establishment of collaborations on acceptable terms, if at all, or it may take longer than expected to establish new collaborations, leading to development and potential commercialization delays, which would adversely affect our business, financial condition and results of operations.

***We expect to continue to incur significant research and development expenses, which may make it difficult for us to attain profitability.***

We expect to expend substantial funds in research and development, including preclinical studies and clinical trials for our platform technology and product candidates, and to manufacture and market any product candidates in the event they are approved for commercial sale. We will likely need additional funding to develop or acquire complementary companies, technologies and assets, as well as for working capital requirements and other operating and general corporate purposes. Moreover, an increase in our headcount would dramatically increase our costs in the near and long-term.

Such spending may not yield any commercially viable products. Due to our limited financial and managerial resources, we must focus on a limited number of research programs and product candidates and on specific indications. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities.

Because the successful development of our product candidates is uncertain, we are unable to precisely estimate the actual funds we will require to develop and potentially commercialize them. In addition, we may not be able to generate sufficient revenue, even if we are able to commercialize any of our product candidates, to become profitable.

***We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.***

Because we have limited financial and managerial resources, we will initially develop our lead product candidate for particular rare genetic diseases. As a result, we may forego or delay pursuit of opportunities in other types of diseases that may prove to have greater treatment potential. Likewise, we may forego or delay the pursuit of opportunities with other potential product candidates that may prove to have greater commercial potential.

Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable product candidates. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other similar arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to the product candidate.

**\*\*\* Given our lack of current cash flow, we will need to raise additional capital to achieve our goals; however, it may be unavailable to us or, even if capital is obtained, may cause dilution or place significant restrictions on our ability to operate our business.**

Since we will be unable to generate sufficient, if any, cash flow to fund our operations for the foreseeable future, we will need to seek additional equity or debt financing to provide the capital required to maintain or expand our operations.

We believe that our existing balance of cash and cash equivalents will enable us to fund our operations for at least the next 12 months from the date this Quarterly Report on Form 10-Q is filed with the SEC, and ideally into the second quarter of calendar year 2022. In particular, we expect that these funds will allow us to achieve certain milestones for our NT0100 program for HD and our NT0200 program for DM1, but we expect that we will need to obtain additional funding to obtain clinical data for our NT0100 program and submit an IND for our NT0200 program. We have based these estimates on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect. Our operating plans and other demands on our cash resources may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings or other capital sources, including potentially collaborations, licenses and other similar arrangements. In addition, we may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans.

There can be no assurance that we will be able to raise sufficient additional capital on acceptable terms or at all. If such additional financing is not available on satisfactory terms, or is not available in sufficient amounts, we may be required to delay, limit or eliminate the development of business opportunities, and our ability to achieve our business objectives, our competitiveness, and our business, financial condition and results of operations may be materially adversely affected. Furthermore, we will not be able to access the capital markets as quickly due to our non-timely filing of our Quarterly Report on Form 10-Q for the quarterly period ended December 31, 2019 and the loss of our eligibility to file a new registration statement on Form S-3 that would allow us to continuously incorporate by reference our Exchange Act reports filed with the SEC into such registration statement, or to use new “shelf” registration statements to conduct offerings, until approximately one year from the date we regain and maintain status as a timely filer. In addition, we may be required to grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. Our inability to fund our business could lead to the loss of your investment.

Our future capital requirements will depend on many factors, including, but not limited to:

- the scope, rate of progress, results and cost of our preclinical studies, clinical trials and other related activities;
- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of such arrangements;
- the timing of, and the costs involved in, obtaining regulatory approvals for any of our current or future product candidates;
- the number and characteristics of the product candidates it seeks to develop or commercialize;
- the cost of manufacturing clinical supplies, and establishing commercial supplies, of our product candidates;
- the cost of commercialization activities if any of our current or future product candidates are approved for sale, including marketing, sales and distribution costs;
- the expenses needed to attract and retain skilled personnel;
- the costs associated with being a public company;
- the amount of revenue, if any, received from commercial sales of our product candidates, should any of our product candidates receive marketing approval; and

the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing possible patent claims, including litigation costs and the outcome of any such litigation.

Any additional capital efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. Moreover, if we raise additional capital by issuing equity securities, the percentage ownership of our existing stockholders may be reduced, and accordingly these stockholders may experience substantial dilution. We may also issue equity securities that provide for rights, preferences and privileges senior to those of our common stock.

Given our need for cash and that equity issuances are the most common type of fundraising for similarly situated companies, the risk of dilution is particularly significant for our stockholders. Furthermore, the incurrence of indebtedness would result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt and other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to seek funds through arrangements with collaborators or otherwise at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our product candidates or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects.

**\*\*\* The report of our former independent registered public accounting firm expressed substantial doubt about our ability to continue as a going concern. Such “going concern” opinion could impair our ability to obtain financing.**

Our former independent registered public accounting firm, CohnReznick LLP, indicated in their report on our financial statements for the fiscal year ended September 30, 2019 that conditions exist that raise substantial doubt about our ability to continue as a going concern due to our recurring losses from operations. A “going concern” opinion could impair our ability to finance our operations through the sale of equity, incurring debt, or other financing alternatives. Our ability to continue as a going concern will depend upon the availability and terms of future funding. If we are unable to achieve this goal, our business would be jeopardized and we may not be able to continue. If we cease operations, it is likely that all of our investors would lose their investment.

**Our efforts to discover product candidates beyond our current product candidates may not succeed, and any product candidates we recommend for clinical development may not actually begin clinical trials.**

We intend to use our technology, including our licensed technology, knowledge and expertise, to develop novel drug candidates to address some of the world’s most devastating and costly central nervous system, muscular, and other disorders, including orphan genetic and oncology indications. We intend to expand our existing pipeline of core assets by advancing drug candidate compounds from discovery programs into clinical development. However, the process of researching and discovering drug candidate compounds is expensive, time-consuming and unpredictable. Data from our current preclinical programs may not support the clinical development of our lead compounds or other compounds from these programs, and we may not identify any additional drug compounds suitable for recommendation for clinical development. Moreover, any drug compounds we recommend for clinical development may not demonstrate, through preclinical studies, indications of safety and potential efficacy that would support advancement into clinical trials. Such findings would potentially impede our ability to maintain or expand our clinical development pipeline. Our ability to identify new drug compounds and advance them into clinical development also depends upon our ability to fund our research and development operations, and we cannot be certain that additional funding will be available on acceptable terms, or at all.

**We are significantly dependent on the success of our PATROL™ platform and our product candidates based on this platform. A failure of any product candidate based on this platform in clinical development would adversely affect our business and may require us to discontinue development of other product candidates based on the same therapeutic approach.**

We have invested, and we expect to continue to invest, significant efforts and financial resources in the development of product candidates based on our PATROL™ platform. Our ability to generate meaningful revenue, which may not occur for the foreseeable future, if ever, will depend heavily on the successful development, regulatory approval and commercialization of one or more of these product candidates using our PATROL™ platform. We will not be able to develop new product candidates if it is found that the PATROL™ platform does not work or creates product candidates that are not safe for use in humans. Since all of our product candidates in our current pipeline are based on our PATROL™ platform, if any product candidate fails in development as a result of an underlying problem with our PATROL™ platform, then we may be required to discontinue development of all product candidates that are based on our therapeutic approach. If we were required to discontinue the development of such product candidates based on the PATROL™ platform, or if any of them were to fail to receive regulatory approval or achieve sufficient market acceptance, we could be prevented from or significantly delayed in achieving profitability. We can provide no assurance that we would be successful at developing other product candidates based on an alternative therapeutic approach from our PATROL™ platform.



*The pharmaceutical market and biotechnology industry are intensely competitive and involve a high degree of risk. If we are unable to compete effectively with existing drugs, new treatment methods and new technologies, we may be unable to commercialize successfully any drug candidates that we develop.*

The pharmaceutical market and biotechnology industry are intensely competitive and rapidly changing. Many large pharmaceutical and biotechnology companies, academic institutions, governmental agencies and other public and private research organizations, both in the U.S. and worldwide, are pursuing the development of novel drugs for the same diseases that we are targeting or expect to target. Many of our competitors have, either alone or with strategic partners:

- much greater financial, research, technical and human resources than we have at every stage of the discovery, development, manufacture and commercialization of products and product candidates;
- more extensive experience in designing and conducting preclinical studies and clinical trials, obtaining regulatory approvals, and in manufacturing, marketing and selling pharmaceutical products and product candidates;
- product candidates that are based on previously tested or accepted technologies;
- products and product candidates that have been approved or are in late stages of development; and
- collaborative arrangements in our target markets with leading companies and research institutions.

We will face intense competition from drugs that have already been approved and accepted by the medical community for the treatment of the conditions for which we may develop drug candidates. We also expect to face competition from new drugs that enter the market. We believe there are a significant number of drugs currently under development that may become commercially available in the future, for the treatment of conditions for which we may try to develop drugs. These drugs may be more effective, safer, less expensive, introduced to market earlier, or marketed and sold more effectively or on a more cost-effective basis, than any product candidates we develop. It is possible that the potential advantages of PATrOL™-derived therapeutic candidates (including, among other potential advantages, the ability to systemically deliver drugs and get broad tissue distribution and penetration across the blood-brain barrier, minimal to no innate or adaptive immune responses after single dose or multiple-dose administration, preferential selectivity to mutant targets, and dose schedules to address the disease appropriately or that is viable in the marketplace) do not materialize.

Our competitors may develop or commercialize products with significant advantages over any product candidates we are able to develop and commercialize based on many different factors, including:

- the safety and effectiveness of our product candidates relative to alternative therapies, if any;
- the ease with which our product candidates can be administered and the extent to which patients accept relatively new routes of administration;
- the timing and scope of regulatory approvals for these product candidates;
- the availability and cost of manufacturing, marketing and sales capabilities;
- price;
- reimbursement coverage from governments and other third-party payors; and
- patent position and intellectual property protection.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are viewed as safer, more effective, more convenient or less expensive than any products that we may develop. Our competitors may also obtain FDA or other regulatory approval for their competing products more rapidly than we may obtain approval for any of our product candidates, which could result in our competitors establishing a strong market position before we are able to enter the market. Further, we expect that we will also compete with others when recruiting clinical trial sites and subjects for our clinical trials and when recruiting and retaining qualified scientific and management personnel.

While there are currently no approved treatments available to slow the progression of Huntington's Disease ("HD"), publicly available information shows that a number of companies are pursuing product candidates related to HD. These include an investigational drug in Phase III clinical development, several ongoing clinical and preclinical programs targeting the underlying disease in HD and the development of drugs focused on treating the symptoms associated with HD. Similarly, both companies and non-commercial sponsors are investigating agents and conducting research, clinical trials and controlled studies regarding different treatments for Myotonic Dystrophy. The success of any of these competitors could reduce or eliminate our commercial opportunity.

***Any collaboration arrangement that we may enter into in the future may not be successful, which could adversely affect our ability to develop and commercialize our current and potential future product candidates.***

We may seek collaboration arrangements with pharmaceutical companies for the development or commercialization of our current and potential future product candidates. To the extent that we decide to enter into collaboration agreements, we will face significant competition in seeking appropriate collaborators. Moreover, collaboration arrangements are complex and time consuming to negotiate, execute and implement. We may not be successful in our efforts to establish and implement collaborations or other alternative arrangements should we choose to enter into such arrangements, and the terms of the arrangements may not be favorable to us. If and when we collaborate with a third party for development and commercialization of a product candidate, we can expect to relinquish some or all of the control over the future success of that product candidate to the third party. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations. As such, our inability to control our collaborators, and the potentially adverse results of our collaborators, may materially and adversely affect our product candidates and, more generally, our PATROL™ platform, and we may not be able to conduct our program in the manner or on the time schedule it currently contemplates, which could negatively impact our business.

If our potential future collaborations do not result in the successful discovery, development and commercialization of products or if one of our collaborators terminates our agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of our platform technology and product candidates could be delayed and we may need additional resources to develop product candidates and our technology.

Finally, disagreements between parties to a collaboration arrangement can lead to delays in developing or commercializing the applicable product candidate and can be difficult to resolve in a mutually beneficial manner. In some cases, collaborations with biopharmaceutical companies and other third parties are terminated or allowed to expire by the other party. Any such termination or expiration could adversely affect our business, financial condition and results of operations.

***We, or any future collaborators, may not be able to obtain orphan drug designation or orphan drug exclusivity for our product candidates.***

Regulatory authorities in some jurisdictions, including the U.S. and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the U.S. In the U.S. and Europe, obtaining orphan drug approval may allow us to obtain financial incentives, such as an extended period of exclusivity during which only we are allowed to market the orphan drug for the orphan indications that we are developing. While we may seek orphan drug designation from the FDA for any of our product candidates, we, or any future collaborators, may not be granted orphan drug designations for our product candidates in the U.S. or in other jurisdictions.

Even if we or any future collaborators obtain orphan drug designation for a product candidate, we or such collaborators may not be able to obtain orphan drug exclusivity for that product candidate. Generally, a product with orphan drug designation only becomes entitled to orphan drug exclusivity if it receives the first marketing approval for the indication for which it has such designation, in which case the FDA or the EMA will be precluded from approving another marketing application for the same drug for that indication for the applicable exclusivity period. The applicable exclusivity period is seven years in the U.S. and ten years in Europe. The European exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or the EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

Even if we or any future collaborators obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because FDA has taken the position that, under certain circumstances, another drug with the same active chemical and pharmacological characteristics, or moiety, can be approved for the same condition. Specifically, the FDA's regulations provide that it can approve another drug with the same active moiety for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care.

**\*\*\* Our operations may be adversely affected by the coronavirus outbreak, and we face risks that could impact our business.**

A novel strain of coronavirus, COVID-19, originated in Wuhan, China, in December 2019. The virus has spread globally and includes a significant number of cases in the U.S., Europe, and Asia. We have relationships with contract research organizations to conduct certain pre-clinical programs and testing and other services in Europe and those business operations are subject to potential business interruptions arising from protective measures that may be taken by the governmental or other agencies or governing bodies. We also conduct limited operations within Asia through third-party contract manufacturing organizations whose operations may be negatively affected by the coronavirus outbreak. In addition, certain of our collaborative relationships with academic research institutions in the United States may be materially and adversely impacted by protective measures taken by those institutions or federal and state agencies and governing bodies to restrict access to, or suspend operations at, such facilities. Such protective measures, including quarantines, travel restrictions and business shutdowns, may also negatively affect our core operations. We have taken temporary precautionary measures intended to help minimize the risk of the virus to our employees, including temporarily requiring some employees to work remotely. We have suspended non-essential travel worldwide for our employees and are discouraging employee attendance at other gatherings. Business disruptions elsewhere in the world could also negatively affect the sources and availability of components and materials that are essential to the operation of our business in Europe, the United States and Asia.

Extended periods of interruption to our U.S. operations or those of our contract research and manufacturing organizations due to the coronavirus outbreak could adversely impact the growth of our business and could cause us to cease or delay operations. In such instances, we may need to locate an appropriate replacement third-party facility and establish a contractual relationship in connection with such facilities, which may not be readily available or on acceptable terms that would cause additional delay and increased expense, including as a result of additional required FDA approvals, and may have a material adverse effect on our business.

The extent to which the coronavirus impacts our business and results of operations will depend on future developments, which are highly uncertain and cannot be predicted. This includes new information that may emerge concerning the severity of the coronavirus, the spread and proliferation of the coronavirus around the world, and the actions taken to contain the coronavirus or treat its impact, among others.

**\*\*\* We are subject to a multitude of manufacturing risks, any of which could substantially increase our costs and limit supply of our product candidates.**

The process of manufacturing our product candidates is complex, highly regulated, and subject to several risks. For example, the process of manufacturing our product candidates is extremely susceptible to product loss due to contamination, equipment failure or improper installation or operation of equipment, or vendor or operator error. Even minor deviations from normal manufacturing processes for any of our product candidates could result in reduced production yields, product defects, and other supply disruptions. If microbial, viral, or other contaminations are discovered in our product candidates or in the manufacturing facilities in which our product candidates are made, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. In addition, the manufacturing facilities in which our product candidates are made could be adversely affected by equipment failures, labor shortages, natural disasters, public health crises, pandemics and epidemics, such as a novel strain of coronavirus (COVID-19), power failures and numerous other factors. For instance, our therapeutic molecules are complex and comprised of both peptides and nucleic acids, and it may be difficult or impossible to find Good Laboratory Practice- ("GLP") and Current Good Manufacturing Practice- ("cGMP") grade manufacturers, manufacturing may be cost prohibitive, we or our third-party manufacturers may not be able to manufacture product candidates in a timely manner, or manufacturing may not be available to fulfill regulatory requirements. In addition, we or our third-party manufacturers may not be able to manufacture our product candidates in a timely manner or at the necessary scale to meet our development and commercialization requirements.

In addition, any adverse developments affecting manufacturing operations for our product candidates may result in shipment delays, inventory shortages, lot failures, withdrawals or recalls, or other interruptions in the supply of our product candidates. We also may need to take inventory write-offs and incur other charges and expenses for product candidates that fail to meet specifications, undertake costly remediation efforts or seek costlier manufacturing alternatives.

***We rely, and will continue to rely, predominantly, on third parties to manufacture our preclinical and clinical drug supplies and our business, financial condition and results of operations could be harmed if those third parties fail to provide us with sufficient quantities of drug product, or fail to do so at acceptable quality levels, prices, or timelines.***

We have the capability internally to manufacture small quantities of our drugs for preclinical studies. However, we do not currently have, nor do we plan to acquire, the infrastructure or capability internally to manufacture our clinical drug supplies for use in our clinical trials, and we lack the resources and the capability to manufacture any of our product candidates on a clinical or commercial scale. We rely on our manufacturers to purchase from third-party suppliers the materials necessary to produce our product candidates for our clinical trials. There are a limited number of suppliers for raw materials that we use to manufacture our product candidates, and there may be a need to identify alternate suppliers to prevent a possible disruption of the manufacture of the materials necessary to produce our product candidates for our clinical trials, and, if approved, ultimately for commercial sale. We do not have any control over the process or timing of the acquisition of these raw materials by our manufacturers. Any significant delay or discontinuity in the supply of a product candidate, or the raw material components thereof, for an ongoing clinical trial due to the need to replace a third-party manufacturer could considerably delay completion of our clinical trials, product testing and potential regulatory approval of our product candidates, which could harm our business, financial condition and results of operations.

***If we are unable to develop our own commercial organization or enter into agreements with third parties to sell and market our product candidates, we may be unable to generate significant revenues.***

We do not have a sales and marketing organization, and we have no experience as a company in the sales, marketing and distribution of pharmaceutical products. If any of our product candidates are approved for commercialization, we may be required to develop our own sales, marketing and distribution capabilities, or make arrangements with a third party to perform sales and marketing services. Developing a sales force for any resulting product or any product resulting from any of our other product candidates is expensive and time consuming and could delay any product launch. We may be unable to establish and manage an effective sales force in a timely or cost-effective manner, if at all, and any sales force we do establish may not be capable of generating sufficient demand for our product candidates. To the extent that we enter into arrangements with collaborators or other third parties to perform sales and marketing services, our product revenues are likely to be lower than if we marketed and sold our product candidates independently. If we are unable to establish adequate sales and marketing capabilities, independently or with others, we may not be able to generate significant revenues and may not become profitable.

***The commercial success of our product candidates depends upon their market acceptance among physicians, patients, healthcare payors and the medical community.***

Even if our product candidates obtain regulatory approval, our products, if any, may not gain market acceptance among physicians, patients, healthcare payors and the medical community. The degree of market acceptance of any of our approved product candidates will depend on a number of factors, including:

- the effectiveness of our approved product candidates as compared to currently available products;
- patient willingness to adopt our approved product candidates in place of current therapies;
- our ability to provide acceptable evidence of safety and efficacy;
- relative convenience and ease of administration;

- the prevalence and severity of any adverse side effects;
- restrictions on use in combination with other products;
- availability of alternative treatments;
- pricing and cost-effectiveness assuming either competitive or potential premium pricing requirements, based on the profile of our product candidates and target markets;
- effectiveness of our or our partners' sales and marketing strategy;
- our ability to obtain sufficient third-party coverage or reimbursement; and
- potential product liability claims.

In addition, the potential market opportunity for our product candidates is difficult to precisely estimate. Our estimates of the potential market opportunity for our product candidates include several key assumptions based on our industry knowledge, industry publications, third-party research reports and other surveys. Independent sources have not verified all of our assumptions. If any of these assumptions proves to be inaccurate, then the actual market for our product candidates could be smaller than our estimates of our potential market opportunity. If the actual market for our product candidates is smaller than we expect, our product revenue may be limited, it may be harder than expected to raise funds and it may be more difficult for us to achieve or maintain profitability. If we fail to achieve market acceptance of our product candidates in the U.S. and abroad, our revenue will be limited, and it will be more difficult to achieve profitability.

***If we fail to obtain and sustain an adequate level of reimbursement for our potential products by third-party payors, potential future sales would be materially adversely affected.***

There will be no viable commercial market for our product candidates, if approved, without reimbursement from third-party payors. Reimbursement policies may be affected by future healthcare reform measures. We cannot be certain that reimbursement will be available for our current product candidates or any other product candidate we may develop. Additionally, even if there is a viable commercial market, if the level of reimbursement is below our expectations, our anticipated revenue and gross margins will be adversely affected.

Third-party payors, such as government or private healthcare insurers, carefully review and increasingly question and challenge the coverage of and the prices charged for drugs. Reimbursement rates from private health insurance companies vary depending on the company, the insurance plan and other factors. Reimbursement rates may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. There is a current trend in the U.S. healthcare industry toward cost containment.

Large public and private payors, managed care organizations, group purchasing organizations and similar organizations are exerting increasing influence on decisions regarding the use of, and reimbursement levels for, particular treatments. Such third-party payors, including Medicare, may question the coverage of, and challenge the prices charged for, medical products and services, and many third-party payors limit coverage of or reimbursement for newly approved healthcare products. In particular, third-party payors may limit the covered indications. Cost-control initiatives could decrease the price we might establish for products, which could result in product revenues being lower than anticipated. We believe our drugs will be priced significantly higher than existing generic drugs and consistent with current branded drugs. If we are unable to show a significant benefit relative to existing generic drugs, Medicare, Medicaid and private payors may not be willing to provide reimbursement for our drugs, which would significantly reduce the likelihood of our products gaining market acceptance.

We expect that private insurers will consider the efficacy, cost-effectiveness, safety and tolerability of our product candidates in determining whether to approve reimbursement for such product candidates and at what level. Obtaining these approvals can be a time consuming and expensive process. Our business, financial condition and results of operations would be materially adversely affected if we do not receive approval for reimbursement of our product candidates from private insurers on a timely or satisfactory basis. Limitations on coverage could also be imposed at the local Medicare carrier level or by fiscal intermediaries. Medicare Part B, which covers medical insurance to Medicare patients as discussed below, does not require participating insurance plans to cover all drugs that have been approved by the FDA. Our business, financial condition and results of operations could be materially adversely affected if Part B medical insurance were to limit access to, or deny or limit reimbursement of, our product candidates.

Reimbursement systems in international markets vary significantly by country and by region, and reimbursement approvals must be obtained on a country-by-country basis. In many countries, the product candidate cannot be commercially launched until reimbursement is approved. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. The negotiation process in some countries can exceed 12 months. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidates to other available therapies.

If the prices for our product candidates are reduced or if governmental and other third-party payors do not provide adequate coverage and reimbursement of our drugs, our future revenue, cash flows and prospects for profitability will suffer.

***We are exposed to product liability, non-clinical and clinical liability risks which could place a substantial financial burden upon us, should lawsuits be filed against us.***

Our business exposes us to potential product liability and other liability risks that are inherent in the testing, manufacturing and marketing of pharmaceutical formulations and products. In addition, the use in our anticipated clinical trials of pharmaceutical products and the subsequent sale of product candidates by us, if approved, or our potential collaborators may cause us to bear a portion of or all product liability risks. A successful liability claim or series of claims brought against us could have a material adverse effect on our business, financial condition and results of operations.

Because we do not currently have any clinical trials ongoing, we do not currently carry product liability insurance. We anticipate obtaining such insurance upon initiation of our clinical development activities; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. On occasion, large judgments have been awarded in class action lawsuits based on drugs that had unanticipated adverse effects. A successful product liability claim or series of claims brought against us could adversely affect our results of operations and business if judgments therewith exceed our insurance coverage.

***\*\*\* If we fail to retain current members of our management, or to attract and keep additional key personnel, we may be unable to successfully develop or commercialize our product candidates.***

Our success depends on our continued ability to attract, retain and motivate highly qualified management and scientific personnel. As of August 12, 2020, we had fifteen full-time employees. We will rely primarily on outsourcing research, development and clinical trial activities, and manufacturing operations, as well as other functions critical to our business. We believe this approach enhances our ability to focus on our core product opportunities, allocate resources efficiently to different projects and allocate internal resources more effectively. We have filled several key open positions and are currently recruiting for a few remaining positions. However, competition for qualified personnel is intense. We may not be successful in attracting qualified personnel to fulfill our current or future needs. In the event we are unable to fill critical open employment positions, we may need to delay our operational activities and goals, including the development of our product candidates, and may have difficulty in meeting our obligations as a public company. We do not maintain “key person” insurance on any of our employees.

In addition, competitors and others are likely in the future to attempt to recruit our employees. The loss of the services of any of our key personnel, the inability to attract or retain highly qualified personnel in the future or delays in hiring such personnel, particularly senior management and other technical personnel, could materially and adversely affect our business, financial condition and results of operations. In addition, the replacement of key personnel likely would involve significant time and costs, and may significantly delay or prevent the achievement of our business objectives.

From time to time, our management seeks the advice and guidance of certain scientific advisors and consultants regarding clinical and regulatory development programs and other customary matters. These scientific advisors and consultants are not our employees and may have commitments to, or consulting or advisory contracts with, other entities that may limit their availability to us. In addition, our scientific advisors may have arrangements with other companies to assist those companies in developing products or technologies that may compete with us.

***We will need to increase the size of our organization and may not successfully manage our growth.***

We are a preclinical-stage pharmaceutical company with a small number of employees, and our management systems currently in place are not likely to be adequate to support our future growth plans. Our ability to grow and to manage our growth effectively will require us to hire, train, retain, manage and motivate additional employees and to implement and improve our operational, financial and management systems. These demands also may require the hiring of additional senior management personnel or the development of additional expertise by our senior management personnel. Hiring a significant number of additional employees, particularly those at the management level, would increase our expenses significantly. Moreover, if we fail to expand and enhance our operational, financial and management systems in conjunction with our potential future growth, such failure could have a material adverse effect on our business, financial condition and results of operations.

***Because our Chief Executive Officer is involved with several unaffiliated privately-held companies, he may experience conflicts of interest and competing demands for his time and attention.***

Dietrich Stephan, Ph.D., our Chief Executive Officer, is a member of the governing bodies of several unaffiliated privately-held companies, as well as a general partner of Cyto Ventures. Although Dr. Stephan expects to devote substantially all of his time to us, he expects to continue in each of these positions for the foreseeable future. Conflicts of interest could arise with respect to business opportunities that could be advantageous to third party organizations affiliated with Dr. Stephan, on the one hand, and us, on the other hand.

***The majority of our current management lacks public company experience, which could put us at greater risk of incurring fines or regulatory actions for failure to comply with federal securities laws and could put us at a competitive disadvantage and require our management to devote additional time and resources to ensure compliance with applicable corporate governance requirements.***

The majority of our current executive management do not have experience in managing and operating a public company, which could have an adverse effect on our ability to quickly respond to problems or adequately address issues and matters applicable to public companies. Any failure to comply with federal securities laws, rules or regulations could subject us to fines or regulatory actions, which may materially adversely affect our business, financial condition and results of operations. Further, since certain of our current executive officers do not have experience managing and operating a public company, we may need to dedicate additional time and resources to comply with legally mandated corporate governance policies relative to our competitors whose management teams have more public company experience.

***\*\*\* We rely significantly on information technology and any failure, inadequacy, interruption or security lapse of that technology, including any cybersecurity incidents, could harm our ability to operate our business effectively.***

Despite the implementation of security measures, our internal computer systems and those of third parties with which we contract are vulnerable to damage from cyber-attacks, computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. System failures, accidents or security breaches could cause interruptions in our operations, and could result in a material disruption of our drug development and preclinical and clinical activities and business operations, in addition to possibly requiring substantial expenditures of resources to remedy. To the extent that any disruption or security breach was to result in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur material legal claims and liability, damage to our reputation, suffer loss or harm to our intellectual property rights and the further research, development and commercial efforts of our products and product candidates could be delayed. The loss of drug development or clinical trial data could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and our development programs and the development of our product candidates could be delayed. In addition, if we are held liable for a claim against which we are not insured or for damages exceeding the limits of our insurance coverage, whether arising out of cybersecurity matters, or from some other matter, that claim could have a material adverse effect on our results of operations.

***Our employees, consultants, third-party vendors and collaborators may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.***

We are exposed to the risk of employee, consultant, third-party vendor or collaborator fraud or other misconduct. Misconduct by our employees, consultants, third-party vendors or collaborators could include, among other things, intentional failures to comply with FDA regulations, provide accurate information to the FDA, comply with manufacturing standards, comply with federal and state healthcare fraud and abuse laws and regulations, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commissions, customer incentive programs and other business arrangements. Employee, consultant, vendor or collaborator misconduct also could involve the improper use of information obtained in the course of preclinical or clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter such misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a material adverse effect on our business, financial condition and results of operations, and result in the imposition of significant fines or other sanctions against us.

***Business disruptions such as natural disasters could seriously harm our future revenues and financial condition and increase our costs and expenses.***

We and our suppliers may experience a disruption in our and their business as a result of natural disasters. A significant natural disaster, such as an earthquake, hurricane, flood or fire, could severely damage or destroy our headquarters or facilities or the facilities of our manufacturers or suppliers, which could have a material and adverse effect on our business, financial condition and results of operations. In addition, terrorist acts or acts of war targeted at the U.S., and specifically the Pittsburgh, Pennsylvania and greater New York, New York regions, could cause damage or disruption to us, our employees, facilities, partners and suppliers, which could have a material adverse effect on our business, financial condition and results of operations.

***We may engage in strategic transactions that could impact our liquidity, increase our expenses and present significant distractions to our management.***

From time to time, we may consider strategic transactions, such as spin-offs, strategic partnerships, joint ventures, restructurings, divestitures, business combinations and investments. Additional potential transactions that we may consider include a variety of different business arrangements, including acquisitions of companies, asset purchases and out-licensing or in-licensing of products, product candidates or technologies. Any such transaction may require us to incur non-recurring or other charges, may increase our near- and long-term expenditures and may pose significant integration challenges or disrupt our management or business, which could adversely affect our business, financial condition and results of operations. For example, these transactions may entail numerous operational and financial risks, including:

- exposure to unknown liabilities;
- disruption of our business and diversion of our management's time and attention in order to develop acquired products, product candidates or technologies;
- incurrence of substantial debt or dilutive issuances of equity securities to pay for any of these transactions;
- higher-than-expected transaction and integration costs;
- write-downs of assets or goodwill or impairment charges;
- increased amortization expenses;
- difficulty and cost in combining the operations and personnel of any acquired businesses or product lines with our operations and personnel;
- impairment of relationships with key suppliers or customers of any acquired businesses or product lines due to changes in management and ownership; and
- inability to retain key employees of any acquired businesses.



Accordingly, although there can be no assurance that we will undertake or successfully complete any transactions of the nature described above, any transactions that we do complete may be subject to the foregoing or other risks, and could have a material adverse effect on our business, financial condition and results of operations.

***The estimates and judgments we make, or the assumptions on which we rely, in preparing our financial statements could prove inaccurate.***

Our financial statements have been prepared in accordance with U.S. GAAP. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities, revenues and expenses, the amounts of charges accrued by us and related disclosure of contingent assets and liabilities. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances. We cannot assure, however, that our estimates, or the assumptions underlying them, will not change over time or otherwise prove inaccurate. For example, our estimates as they relate to anticipated timelines and milestones for our preclinical development or clinical trials may prove to be inaccurate. If this is the case, we may be required to restate our financial statements, which could, in turn, subject us to securities class action litigation or regulatory investigation or action. Defending against such potential litigation or regulatory action relating to a restatement of our financial statements would be expensive and would require significant attention and resources of our management. Moreover, our insurance to cover our obligations with respect to the ultimate resolution of any such litigation or regulatory action may be inadequate. As a result of these factors, any such potential litigation or regulatory action could have a material adverse effect on our financial results or harm our business.

***We may be unable to sell or otherwise monetize, the assets and technologies of the Company as conducted prior to the completion of the Merger, in which case we may be required to take write-downs, write-offs and impairment or other charges associated with the carrying values of such assets. Any such charges could negatively affect our business, assets, liabilities, prospects, outlook, financial condition and results of operations.***

Previously, Ohr acquired the SKS Ocular 1 LLC sustained release technology, which was designed to develop best in class drug formulations for ocular disease (the “SKS Assets”) and the exclusive rights to an animal model for dry macular degeneration and rights to produce and use carboxyethylpyrrole (“CEP”) for research, clinical and commercial applications (the “CEP Assets”). In September 2019, we terminated the licenses for the SKS Assets and the CEP Assets, as there would be substantial costs associated with continuing to maintain such assets and attempts to monetize such assets would be a distraction to our management and other employees and an inefficient use of their time. Our management and board of directors continue to evaluate whether to further pursue monetizing the remaining assets associated with our pre-Merger activities, including selling, discontinuing or adjusting such assets. There can be no assurance, however, that we will be successful at such efforts or sell or otherwise monetize such assets on acceptable terms, if at all. We may be required to take write-offs or write-downs, and impairment or other charges associated with classifying such remaining assets as held-for-sale and recording the carrying values of such assets at fair market value. As a result, we may be forced to write-down or write-off such assets, in some cases completely, or incur impairment or other charges that could negatively affect our business, assets, liabilities, prospects, outlook, financial condition and results of operations.

#### **Risks Related to Our Intellectual Property**

***We may not be successful in obtaining or maintaining necessary rights to our product candidates through acquisitions and in-licenses.***

Because several of our programs currently require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to maintain and exploit these proprietary rights. In addition, we may need to acquire or in-license additional intellectual property in the future. We may be unable to acquire or in-license any compositions, methods of use, processes or other intellectual property rights from third parties that we identify as necessary for our product candidates. We face competition with regard to acquiring and in-licensing third-party intellectual property rights, including from a number of more established companies. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license intellectual property rights to us. We also may be unable to acquire or in-license third-party intellectual property rights on terms that would allow it to make an appropriate return on our investment, and we may not be able to market products or perform research and development or other activities covered by these patents.

We may enter into collaboration agreements with U.S. and foreign academic institutions to accelerate development of our current or future preclinical product candidates. Typically, these agreements include an option for the company to negotiate a license to the institution's intellectual property rights resulting from the collaboration. Even with such an option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to license rights from a collaborating institution, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our desired program.

If we are unable to successfully obtain required third-party intellectual property rights or maintain our existing intellectual property rights, we may need to abandon development of the related program and our business, financial condition and results of operations could be materially and adversely affected.

*If we fail to comply with our obligations in the agreements under which we in-license intellectual property and other rights from third parties or otherwise experiences disruptions to our business relationships with our licensors, we could lose intellectual property rights that are important to our business.*

Our license agreement with CMU (the "CMU License Agreement"), as the licensor (the "Licensor"), is important to our business, and we expect to enter into additional license agreements in the future. The CMU License Agreement imposes, and we expect that future license agreements will impose, various royalties, sublicensing fees and other obligations on us. If we fail to comply with our obligations under these agreements, or if we file for bankruptcy, we may be required to make certain payments to the Licensor, we may lose the exclusivity of our license, or the Licensor may have the right to terminate the license, in which event we would not be able to develop or market products covered by the license. Additionally, the royalties and other payments associated with these licenses could materially and adversely affect our business, financial condition and results of operations.

Pursuant to the terms of the CMU License Agreement, the Licensor has the right to terminate the CMU License Agreement with respect to the program licensed under certain circumstances, including, but not limited to: (i) if we do not pay amounts when due and within the applicable cure periods or (ii) if we file or have filed against us a petition in bankruptcy or makes an assignment for the benefit of creditors. In the event the CMU License Agreement is terminated by the Licensor, all licenses (or, in the determination of the Licensor, the exclusivity of such licenses) granted to us by the Licensor will terminate immediately.

In some cases, patent prosecution of our licensed technology may be controlled solely by the licensor. If our licensor fails to obtain and maintain patent or other protection for the proprietary intellectual property we in-license, then we could lose our rights to the intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products using the intellectual property. In certain cases, we may control the prosecution of patents resulting from licensed technology. In the event we breach any of our obligations related to such prosecution, we may incur significant liability to our licensing partners. Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise regarding intellectual property subject to a licensing agreement, including, but not limited to:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our collaborators; and
- the priority of invention of patented technology.

If disputes over intellectual property and other rights that we have in-licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates. If we fail to comply with any such obligations to our licensor, such licensor may terminate their licenses to us, in which case we would not be able to market products covered by these licenses. The loss of our licenses would have a material adverse effect on our business, financial condition and results of operations.

***We may be required to pay royalties and sublicensing fees pursuant to the CMU License Agreement, which could adversely affect the overall profitability for us of any product candidates that we may seek to commercialize.***

Under the terms of the CMU License Agreement, we will be required to pay royalties on future worldwide net product sales and a percentage of sublicensing fees that we may earn. These royalty payments and sublicensing fees could adversely affect the overall profitability for us of any product candidates that we may seek to commercialize.

***We may not be able to protect our proprietary or licensed technology in the marketplace.***

We depend on our ability to protect our proprietary or licensed technology. We rely on trade secret, patent, copyright and trademark laws, and confidentiality, licensing and other agreements with employees and third parties, all of which offer only limited protection. Our success depends in large part on our ability and any licensor's or licensee's ability to obtain and maintain patent protection in the U.S. and other countries with respect to our proprietary or licensed technology and product candidates. We currently in-license some of our intellectual property rights to develop our product candidates and may in-license additional intellectual property rights in the future. We cannot be certain that patent enforcement activities by our current or future licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents or other intellectual property rights. We also cannot be certain that our current or future licensors will allocate sufficient resources or prioritize their or our enforcement of such patents. Even if we are not a party to these legal actions, an adverse outcome could prevent us from continuing to license intellectual property that we may need to operate our business, which would have a material adverse effect on our business, financial condition and results of operations.

We believe we will be able to obtain, through prosecution of patent applications covering our owned technology and technology licensed from others, adequate patent protection for our proprietary drug technology, including those related to our in-licensed intellectual property. If we are compelled to spend significant time and money protecting or enforcing our licensed patents and future patents we may own, designing around patents held by others or licensing or acquiring, potentially for large fees, patents or other proprietary rights held by others, our business, financial condition and results of operations may be materially and adversely affected. If we are unable to effectively protect the intellectual property that we own or in-license, other companies may be able to offer the same or similar products for sale, which could materially adversely affect our business, financial condition and results of operations. The patents of others from whom we may license technology, and any future patents we may own, may be challenged, narrowed, invalidated or circumvented, which could limit our ability to stop competitors from marketing the same or similar products or limit the length of term of patent protection that we may have for our products.

***Obtaining and maintaining patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection for licensed patents, licensed pending patent applications and potential future patent applications and patents could be reduced or eliminated for non-compliance with these requirements.***

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or patent applications will be due to be paid to the U.S. Patent and Trademark Office ("USPTO") and various governmental patent agencies outside of the U.S. in several stages over the lifetime of the applicable patent and/or patent application. The USPTO and various non-U.S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. If this occurs with respect to our in-licensed patents or patent applications we may file in the future, our competitors might be able to use our technologies, which would have a material adverse effect on our business, financial condition and results of operations.

The patent positions of pharmaceutical products are often complex and uncertain. The breadth of claims allowed in pharmaceutical patents in the U.S. and many jurisdictions outside of the U.S. is not consistent. For example, in many jurisdictions, the support standards for pharmaceutical patents are becoming increasingly strict. Some countries prohibit method of treatment claims in patents. Changes in either the patent laws or interpretations of patent laws in the U.S. and other countries may diminish the value of our licensed or owned intellectual property or create uncertainty. In addition, publication of information related to our current product candidates and potential products may prevent us from obtaining or enforcing patents relating to these product candidates and potential products, including without limitation composition-of-matter patents, which are generally believed to offer the strongest patent protection.

Patents that we currently license and patents that we may own or license in the future do not necessarily ensure the protection of our licensed or owned intellectual property for a number of reasons, including, without limitation, the following:

- the patents may not be broad or strong enough to prevent competition from other products that are identical or similar to our product candidates;
- there can be no assurance that the term of a patent can be extended under the provisions of patent term extensions afforded by U.S. law or similar provisions in foreign countries, where available;
- the issued patents and patents that we may obtain or license in the future may not prevent generic entry into the market for our product candidates;
- we, or third parties from whom we in-license or may license patents, may be required to disclaim part of the term of one or more patents;
- there may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim;
- there may be prior art of which we are aware, which we do not believe affects the validity or enforceability of a patent claim, but which, nonetheless, ultimately may be found to affect the validity or enforceability of a patent claim;
- there may be other patents issued to others that will affect our freedom to operate;
- if the patents are challenged, a court could determine that they are invalid or unenforceable;
- there might be a significant change in the law that governs patentability, validity and infringement of our licensed patents or any future patents we may own that adversely affects the scope of our patent rights;
- a court could determine that a competitor's technology or product does not infringe our licensed patents or any future patents we may own; and
- the patents could irretrievably lapse due to failure to pay fees or otherwise comply with regulations or could be subject to compulsory licensing.

If we encounter delays in our development or clinical trials, the period of time during which we could market our potential products under patent protection would be reduced.

Our competitors may be able to circumvent our licensed patents or future patents we may own by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may seek to market generic versions of any approved products by submitting abbreviated new drug applications to the FDA in which our competitors claim that our licensed patents or any future patents we may own are invalid, unenforceable or not infringed. Alternatively, our competitors may seek approval to market their own products similar to or otherwise competitive with our product candidates. In these circumstances, we may need to defend or assert our licensed patents or any future patents we may own, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find our licensed patents or any future patents we may own invalid or unenforceable. We may also fail to identify patentable aspects of our research and development before it is too late to obtain patent protection. Even if we own or in-license valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives.

The issuance of a patent is not conclusive as to its inventorship, scope, ownership, priority, validity or enforceability. In this regard, third parties may challenge our licensed patents or any future patents we may own in the courts or patent offices in the U.S. and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and potential products. In addition, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized.

*We may infringe the intellectual property rights of others, which may prevent or delay our drug development efforts and prevent us from commercializing or increase the costs of commercializing our product candidates.*

Our commercial success depends significantly on our ability to operate without infringing the patents and other intellectual property rights of third parties. For example, there could be issued patents of which we are not aware that our current or potential future product candidates infringe. There also could be patents that we believe we do not infringe, but that we may ultimately be found to infringe. We have licensed intellectual property from CMU under the CMU License Agreement, and prior generation intellectual property was licensed to other entities. Such intellectual property, in conjunction with further developed technologies for gene editing therapies using such intellectual property, may overlap with our own intellectual property.

Furthermore, because the nucleic acid therapeutics intellectual property landscape is still evolving and our product candidates have not been through clinical trials or commercialized, it is difficult to conclusively assess our freedom to operate without infringing third party rights. There are numerous companies that have pending patent applications and issued patents directed to certain aspects of nucleic acid therapeutics. We are aware of third-party competitors in the oligonucleotide therapeutics space, whose patent filings and/or issued patents may include claims directed to targets and/or products related to some of our programs. It is possible that at the time that we commercialize our products these third-party patent portfolios may include issued patent claims that cover our product candidates or critical features of their production or use. Our competitive position may suffer if patents issued to third parties or other third-party intellectual property rights cover, or may be alleged to cover, our product candidates or elements thereof, or methods of manufacture or use relevant to our development plans. In such cases, we may not be in a position to develop or commercialize product candidates unless we successfully pursue litigation to nullify or invalidate the third-party intellectual property right concerned or enter into a license agreement with the intellectual property right holder, if available on commercially reasonable terms.

Moreover, patent applications are in some cases maintained in secrecy until patents are issued. The publication of discoveries in the scientific or patent literature frequently occurs substantially later than the date on which the underlying discoveries were made and patent applications were filed. Because patents can take many years to issue, there may be currently pending applications of which we are unaware that may later result in issued patents that our product candidates or potential products infringe. For example, pending applications may exist that claim or can be amended to claim subject matter that our product candidates or potential products infringe. Competitors may file continuing patent applications claiming priority to already issued patents in the form of continuation, divisional, or continuation-in-part applications, in order to maintain the pendency of a patent family and attempt to cover our product candidates.

Third parties may assert that we are employing their proprietary technology without authorization and may sue us for patent or other intellectual property infringement. These lawsuits are costly and could adversely affect our business, financial condition and results of operations and divert the attention of managerial and scientific personnel. If we are sued for patent infringement, we would need to demonstrate that our product candidates, potential products or methods either do not infringe the claims of the relevant patent or that the patent claims are invalid, and we may not be able to do this. Proving invalidity is difficult. For example, in the U.S., proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could have a material adverse effect on us. In addition, we may not have sufficient resources to bring these actions to a successful conclusion. If a court holds that any third-party patents are valid, enforceable and cover our products or their use, the holders of any of these patents may be able to block our ability to commercialize our products unless it acquires or obtains a license under the applicable patents or until the patents expire.

We may not be able to enter into licensing arrangements or make other arrangements at a reasonable cost or on reasonable terms. Any inability to secure licenses or alternative technology could result in delays in the introduction of our product candidates or lead to prohibition of the manufacture or sale of products by us. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. We could be forced, including by court order, to cease commercializing the infringing technology or product candidate. In addition, in any such proceeding or litigation, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially and adversely affect our business, financial condition and results of operations. Any claims by third parties that we have misappropriated their confidential information or trade secrets could have a similar material and adverse effect on our business, financial condition and results of operations. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations.

Our product candidates may also require specific formulations to work effectively and efficiently. These formulations may be covered by intellectual property rights held by others. We may develop products containing our compounds and pre-existing pharmaceutical compounds. These pharmaceutical compounds may be covered by intellectual property rights held by others. We may be required by the FDA or comparable foreign regulatory authorities to provide a companion diagnostic test or tests with our product candidates. These diagnostic test or tests may be covered by intellectual property rights held by others. We may be unable to acquire or in-license any relevant third-party intellectual property rights that we identify as necessary or important to our business operations.

We, or our licensors, may not be able to detect infringement against our owned or in-licensed patents, as the case may be, which may be especially difficult for manufacturing processes or formulation patents. Even if we or our licensors detect infringement by a third party of our owned or in-licensed patents, we or our licensors, as the case may be, may choose not to pursue litigation against or settlement with the third party. If we, or our licensors, later sue such third party for patent infringement, the third party may have certain legal defenses available to it, which otherwise would not be available except for the delay between when the infringement was first detected and when the suit was brought. Such legal defenses may make it impossible for us or our licensors to enforce our owned or in-licensed patents, as the case may be, against such third party.

***Any claims or lawsuits relating to infringement of intellectual property rights brought by or against us will be costly and time consuming and may adversely affect our business, financial condition and results of operations.***

We may be required to initiate litigation to enforce or defend our licensed and owned intellectual property. Lawsuits to protect our intellectual property rights can be very time consuming and costly. There is a substantial amount of litigation involving patent and other intellectual property rights in the pharmaceutical industry generally. Such litigation or proceedings could substantially increase our operating expenses and reduce the resources available for development activities or any future sales, marketing or distribution activities.

In any infringement litigation, any award of monetary damages we receive may not be commercially valuable. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during litigation. Moreover, there can be no assurance that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are resolved. Further, any claims we assert against a perceived infringer could provoke these parties to assert counterclaims against us alleging that we have infringed their patents. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

In addition, our licensed patents and patent applications, and patents and patent applications that we may apply for, own or license in the future, could face other challenges, such as interference proceedings, opposition proceedings, re-examination proceedings and other forms of post-grant review. Any of these challenges, if successful, could result in the invalidation of, or in a narrowing of the scope of, any of our licensed patents and patent applications and patents and patent applications that we may apply for, own or license in the future subject to challenge. Any of these challenges, regardless of their success, would likely be time-consuming and expensive to defend and resolve and would divert our management and scientific personnel's time and attention.

***Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our product candidates or potential products.***

As is the case with other pharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the pharmaceutical industry involves both technological and legal complexity and is costly, time-consuming and inherently uncertain. For example, the U.S. previously enacted and is currently implementing wide-ranging patent reform legislation. Specifically, on September 16, 2011, the Leahy-Smith America Invents Act (the “Leahy-Smith Act”) was signed into law and included a number of significant changes to U.S. patent law, and many of the provisions became effective in March 2013. However, it may take the courts years to interpret the provisions of the Leahy-Smith Act, and the implementation of the statute could increase the uncertainties and costs surrounding the prosecution of our licensed and future patent applications and the enforcement or defense of our licensed and future patents, all of which could have a material adverse effect on our business, financial condition and results of operations.

In addition, the U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. The recent decision by the U.S. Supreme Court in *Association for Molecular Pathology v. Myriad Genetics, Inc.* precludes a claim to a nucleic acid having a stated nucleotide sequence which is identical to a sequence found in nature and unmodified. We are currently not aware of an immediate impact of this decision on our patents or patent applications which contain modifications that we believe are not found in nature. However, this decision has yet to be clearly interpreted by courts and by the USPTO. We cannot make assurances that the interpretations of this decision or subsequent rulings will not adversely impact our patents or patent applications. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce patents that we might obtain in the future.

***We may not be able to protect our intellectual property rights throughout the world.***

Filing, prosecuting and defending patents on product candidates throughout the world would be prohibitively expensive. Competitors may use our licensed and owned technologies in jurisdictions where we have not licensed or obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we may obtain or license patent protection, but where patent enforcement is not as strong as that in the U.S. These products may compete with our product candidates in jurisdictions where we do not have any issued or licensed patents, and any future patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to pharmaceuticals, which could make it difficult for us to stop the infringement of our licensed patents and future patents we may own, or marketing of competing products in violation of our proprietary rights generally. Further, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the U.S. As a result, we may encounter significant problems in protecting and defending our licensed and owned intellectual property both in the U.S. and abroad. For example, China currently affords less protection to a company’s intellectual property than some other jurisdictions. As such, the lack of strong patent and other intellectual property protection in China may significantly increase our vulnerability regarding unauthorized disclosure or use of our intellectual property and undermine our competitive position. Proceedings to enforce our future patent rights, if any, in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business.

***We may be unable to adequately prevent disclosure of trade secrets and other proprietary information.***

In order to protect our proprietary and licensed technology and processes, we rely in part on confidentiality agreements with our corporate partners, employees, consultants, manufacturers, outside scientific collaborators and sponsored researchers and other advisors. These agreements may not effectively prevent disclosure of our confidential information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. In addition, others may independently discover our trade secrets and proprietary information. Failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

***We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties.***

We expect to employ individuals who were previously employed at other pharmaceutical companies. Although we have no knowledge of any such claims against us, we may be subject to claims that us or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed confidential information of our employees’ former employers or other third parties. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and even if we are successful, litigation could result in substantial cost and be a distraction to our management and other employees.

***We may be subject to claims challenging the inventorship of our licensed patents, any future patents we may own and other intellectual property.***

Although we are not currently experiencing any claims challenging the inventorship of our licensed patents or our licensed or owned intellectual property, we may in the future be subject to claims that former employees, collaborators or other third parties have an interest in our licensed patents or other licensed or owned intellectual property as an inventor or co-inventor. For example, we may have inventorship disputes arise from conflicting obligations of consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business, financial condition and results of operations. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

***If we do not obtain additional protection under the Hatch-Waxman Amendments and similar foreign legislation extending the terms of our licensed patents and any future patents we may own, our business, financial condition and results of operations may be materially and adversely affected.***

Depending upon the timing, duration and specifics of FDA regulatory approval for our product candidates, one or more of our licensed U.S. patents or future U.S. patents that we may license or own may be eligible for limited patent term restoration under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during drug development and the FDA regulatory review process. This period is generally one-half the time between the effective date of an IND (falling after issuance of the patent) and the submission date of a New Drug Application (“NDA”), plus the time between the submission date of an NDA and the approval of that application. Patent term restorations, however, cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval by the FDA.

The application for patent term extension is subject to approval by the USPTO, in conjunction with the FDA. It takes at least six months to obtain approval of the application for patent term extension. We may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration or the term of any such extension is less than we request, the period during which we will have the right to exclusively market our product will be shortened and our competitors may obtain earlier approval of competing products, and our ability to generate revenues could be materially adversely affected.

#### **Risks Related to Government Regulation**

***We are very early in our development efforts. All of our product candidates are still in preclinical development. If we are unable to advance our product candidates to clinical development, obtain regulatory approval and ultimately commercialize our product candidates or experience significant delays in doing so, our business will be materially harmed.***

We are very early in our development efforts, and all of our product candidates are still in preclinical development. We have invested substantially all of our efforts and financial resources in the identification and preclinical development of antisense oligonucleotide therapies (“ASOs”), including the development program for the treatment of Huntington’s Disease. Our ability to generate product revenues, which it does not expect will occur for many years, if ever, will depend on the successful development and eventual commercialization of our product candidates, which may never occur. We currently generate no revenue from sales of any products, and we may never be able to develop or commercialize a marketable product. In addition, certain of our product candidate development programs contemplate the development of companion diagnostics, which are assays or tests to identify an appropriate patient population. Companion diagnostics are subject to regulation as medical devices and must themselves be approved for marketing by the FDA or certain other foreign regulatory agencies before we may commercialize our product candidates. The success of our product candidates will depend on several factors, including the following:



- successful completion of preclinical studies;
- approval of INDs for our planned clinical trials or future clinical trials;
- successful enrollment in, and completion of, clinical trials;
- successful development of companion diagnostics for use with certain of our product candidates;
- receipt of regulatory approvals from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers for clinical supply and commercial manufacturing;
- obtaining and maintaining patent and trade secret protection or regulatory exclusivity for our product candidates;
- launching commercial sales of our product candidates, if and when approved, whether alone or in collaboration with others;
- acceptance of the product candidates, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies;
- obtaining and maintaining third-party insurance coverage and adequate reimbursement;
- enforcing and defending intellectual property rights and claims; and
- maintaining a continued acceptable safety profile of the product candidates following approval, if approved.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations.

Furthermore, the FDA has relatively limited experience with nucleic acid therapeutics, particularly PNAs, which may increase the complexity, uncertainty and length of the regulatory review process for our product candidates. To date, the FDA has approved few nucleic acid therapeutics for marketing and commercialization, and the FDA and our foreign counterparts have not yet established any definitive policies, practices or guidelines specifically in relation to these drugs. The lack of policies, practices or guidelines specific to nucleic acid therapeutics may hinder or slow review by the FDA of any regulatory filings that we may submit. Moreover, the FDA may respond to these submissions by defining requirements we may not have anticipated. Such responses could lead to significant delays in the development of our product candidates. In addition, because there may be approved treatments for some of the diseases for which we may seek approval, in order to receive regulatory approval, we may need to demonstrate through clinical trials that the product candidates we develop to treat these diseases, if any, are not only safe and effective, but safer or more effective than existing products. Furthermore, in recent years, there has been increased public and political pressure on the FDA with respect to the approval process for new drugs, and the FDA's standards, especially regarding drug safety, appear to have become more stringent. As a result of the foregoing factors, we may never receive regulatory approval to market and commercialize any product candidate.

***Preclinical and clinical trials are expensive, time-consuming and difficult to design and implement, and involve uncertain outcomes. Furthermore, results of earlier preclinical studies and clinical trials may not be predictive of results of future preclinical studies or clinical trials.***

All of our product candidates are still in the preclinical stage, and their risk of failure is high. Before we can commence clinical trials for a product candidate, we must complete extensive preclinical testing and studies that support our planned INDs in the U.S., or similar applications in other jurisdictions. We cannot be certain of the timely completion or outcome of our preclinical testing and studies and cannot predict if the FDA or other regulatory authorities will accept our proposed clinical programs or if the outcome of our preclinical testing and studies will ultimately support the further development of our programs. It is also impossible to predict when or if any of our product candidates will complete clinical trials evaluating their safety and effectiveness in humans or will receive regulatory approval. To obtain the requisite regulatory approvals to market and sell any of our product candidates, we must demonstrate through extensive preclinical studies and clinical trials that our PATROL™ platform and product candidates are safe and effective in humans for use in each target indication. To date, we have never advanced a product candidate into a clinical trial. Preclinical and clinical testing is expensive and can take many years to complete, and the outcome is inherently uncertain. Failure can occur at any time during the preclinical or clinical trial process. Our preclinical programs may experience delays or may never advance to clinical trials, which would adversely affect our ability to obtain regulatory approvals or commercialize these programs on a timely basis or at all, which would have an adverse effect on our business, financial condition and results of operations.

Additionally, the results of preclinical studies and future clinical trials of product candidates may not be predictive of the results of later-stage clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy results despite having progressed through preclinical studies and initial clinical trials. Many companies in the pharmaceutical industry have suffered significant setbacks in advanced clinical trials due to adverse safety profiles or lack of efficacy, notwithstanding promising results in earlier studies. Similarly, our future clinical trial results may not be successful for these or other reasons.

This product candidate development risk is heightened by any changes in the anticipated clinical trials compared to the completed clinical trials. As product candidates are developed from preclinical through early to late stage clinical trials towards approval and commercialization, it is customary that various aspects of the development program, such as manufacturing and methods of administration, are altered along the way in an effort to optimize processes and results. While these types of changes are common and are intended to optimize the product candidates for late stage clinical trials, approval and commercialization, such changes carry the risk that they will not achieve these intended objectives.

Any of these changes could make the results of our anticipated clinical trials or other future clinical trials we may initiate less predictable and could cause our product candidates to perform differently, including causing toxicities, which could delay completion of our clinical trials, delay approval of our product candidates, and/or jeopardize our ability to commence product sales and generate revenues.

***We may rely on third parties to conduct investigator-sponsored clinical trials of our product candidates. Any failure by a third party to meet our obligations with respect to the clinical development of our product candidates may delay or impair our ability to obtain regulatory approval for other product candidates.***

We may rely on academic and private non-academic institutions to conduct and sponsor preclinical and clinical trials relating to our product candidates. We will not control the design or conduct of the investigator-sponsored trials, and it is possible that the FDA or non-U.S. regulatory authorities will not view these investigator-sponsored trials as providing adequate support for future preclinical and clinical trials, whether controlled by us or third parties, for any one or more reasons, including elements of the design or execution of the trials or safety concerns or other trial results. For example, we collaborate with, and rely on, academic centers to conduct preclinical and non-investigator-sponsored research and it is possible that the interests of such academic centers may not be aligned with our interests.

Such arrangements will likely provide us certain information rights with respect to the investigator-sponsored trials, including access to and the ability to use and reference the data, including for our own regulatory filings, resulting from the investigator-sponsored trials. However, we would not have control over the timing and reporting of the data from investigator-sponsored trials, nor would we own the data from the investigator-sponsored trials. If we are unable to confirm or replicate the results from the investigator-sponsored trials or if negative results are obtained, we would likely be further delayed or prevented from advancing further clinical development of our product candidates. Further, if investigators or institutions breach their obligations with respect to the clinical development of our product candidates, or if the data proves to be inadequate compared to the first-hand knowledge we might have gained had the investigator-sponsored trials been sponsored and conducted by us, then our ability to design and conduct any future preclinical or clinical trials ourselves may be adversely affected.

Additionally, the FDA or non-U.S. regulatory authorities may disagree with the sufficiency of our right of reference to the preclinical, manufacturing or clinical data generated by these investigator-sponsored trials, or our interpretation of preclinical, manufacturing or clinical data from these investigator-sponsored trials. If so, the FDA or other non-U.S. regulatory authorities may require us to obtain and submit additional preclinical, manufacturing, or clinical data before we may initiate our anticipated trials and/or may not accept such additional data as adequate to initiate our anticipated trials.

*Our product candidates may cause undesirable side effects that could delay or prevent their regulatory approval or commercialization or have other significant adverse implications on our business, financial condition and results of operations.*

Undesirable side effects observed in preclinical studies or in clinical trials with our product candidates could interrupt, delay or halt their development and could result in the denial of regulatory approval by the FDA, the EMA or comparable foreign authorities for any or all targeted indications or adversely affect the marketability of any product candidates developed using our PATrOL™ platform that receive regulatory approval. In turn, this could eliminate or limit our ability to commercialize our product candidates.

Our product candidates may exhibit adverse effects in preclinical toxicology studies and adverse interactions with other drugs. There are also risks associated with additional requirements the FDA, the EMA or comparable foreign authorities may impose for marketing approval with regard to a particular disease.

Our product candidates may require a risk management program that could include patient and healthcare provider education, usage guidelines, appropriate promotional activities, a post-marketing observational study and ongoing safety and reporting mechanisms, among other requirements. Prescribing could be limited to physician specialists or physicians trained in the use of the drug, or could be limited to a more restricted patient population. Any risk management program required for approval of our product candidates could potentially have an adverse effect on our business, financial condition and results of operations.

Undesirable side effects involving our product candidates may have other significant adverse implications on our business, financial condition and results of operations. For example:

- we may be unable to obtain additional financing on acceptable terms, if at all;
- our collaborators may terminate any development agreements covering these product candidates;
- if any development agreements are terminated, we may determine not to further develop the affected product candidates due to resource constraints and may not be able to establish additional collaborations for their further development on acceptable terms, if at all;
- if we were to later continue the development of these product candidates and receive regulatory approval, earlier findings may significantly limit their marketability and thus significantly lower our potential future revenues from their commercialization;
- we may be subject to product liability or stockholder litigation; and
- we may be unable to attract and retain key employees.

In addition, if any of our product candidates receive marketing approval and we or others later identify undesirable side effects caused by the product:

- regulatory authorities may withdraw their approval of the PATrOL™ platform and the product, or we or our partners may decide to cease marketing and sale of the product voluntarily;
- we may be required to change the way the product is administered, conduct additional preclinical studies or additional clinical trials after initial clinical trials regarding the product, change the labeling of the product, or change the product's manufacturing facilities; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product and could substantially increase the costs and expenses of commercializing the product, which in turn could delay or prevent us from generating significant revenues from the sale of the product.

***Delays in the commencement or completion of clinical trials could result in increased costs to us and delay our ability to establish strategic collaborations.***

Delays in the commencement or completion of clinical trials could significantly impact our drug development costs. We do not know whether anticipated clinical trials will begin on time or be completed on schedule, if at all. The commencement of clinical trials can be delayed for a variety of reasons, including, but not limited to, delays related to:

- obtaining regulatory approval to commence one or more clinical trials;
- reaching agreement on acceptable terms with prospective third-party contract research organizations (“CROs”) and clinical trial sites;
- manufacturing sufficient quantities of a product candidate or other materials necessary to conduct clinical trials;
- obtaining institutional review board approval to conduct one or more clinical trials at a prospective site;
- recruiting and enrolling patients to participate in one or more clinical trials; and
- the failure of our collaborators to adequately resource our product candidates due to their focus on other programs or as a result of general market conditions.

In addition, once a clinical trial has begun, it may be suspended or terminated by us, our collaborators, the institutional review boards or data safety monitoring boards charged with overseeing our clinical trials, the FDA, the EMA or comparable foreign authorities due to a number of factors, including:

- failure to conduct the clinical trial in accordance with regulatory requirements or clinical protocols;
- inspection of the clinical trial operations or clinical trial site by the FDA, the EMA or comparable foreign authorities resulting in the imposition of a clinical hold;
- unforeseen safety issues;
- lack of adequate funding to continue the clinical trials; and
- lack of patient enrollment in clinical studies.

If we experience delays in the completion or termination of any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, and our ability to commence product sales and generate product revenues from any of our product candidates will be delayed. In addition, any delays in completing our clinical trials will increase our costs and slow down our product candidate development and approval process. Delays in completing our clinical trials could also allow our competitors to obtain marketing approval before we do or shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. Any of these occurrences may harm our business, financial condition and prospects significantly. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

***If we experience delays in the enrollment of patients in our clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.***

We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or other regulatory authorities. Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors, including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, competing clinical trials and clinicians’ and patients’ perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating.

If we fail to enroll and maintain the number of patients for which the clinical trial was designed, the statistical power of that clinical trial may be reduced, which would make it harder to demonstrate that the product candidate being tested in such clinical trial is safe and effective. Additionally, enrollment delays in our clinical trials may result in increased development costs for our product candidates, which would cause our value to decline and limit our ability to obtain additional financing. Our inability to enroll a sufficient number of patients for any of our future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

***We intend to rely on third parties to conduct our preclinical studies and clinical trials and perform other tasks. If these third parties do not successfully carry out their contractual duties, meet expected deadlines, or comply with regulatory requirements, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business, financial condition and results of operations could be substantially harmed.***

We intend to rely upon third-party CROs, medical institutions, clinical investigators and contract laboratories to monitor and manage data for our ongoing preclinical and anticipated clinical programs. Nevertheless, we maintain responsibility for ensuring that each of our preclinical studies are, and anticipated clinical studies will be, conducted in accordance with the applicable protocol, legal, regulatory, and scientific standards and our reliance on these third parties does not relieve the Company of our regulatory responsibilities. The Company and our CROs and other vendors are required to comply with current requirements on cGMP, good clinical practices (“GCP”) and GLP, which are a collection of laws and regulations enforced by the FDA, the EMA and comparable foreign authorities for all of our product candidates in clinical development. Regulatory authorities enforce these regulations through periodic inspections of preclinical study and clinical trial sponsors, principal investigators, preclinical study and clinical trial sites, and other contractors. If we or any of our CROs or vendors fails to comply with applicable regulations, the data generated in our preclinical studies and clinical trials may be deemed unreliable and the FDA, the EMA or comparable foreign authorities may require us to perform additional preclinical studies and clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with GCP regulations. In addition, our clinical trials must be conducted with products produced consistent with cGMP regulations. Our failure to comply with these regulations may require it to repeat clinical trials, which would delay the development and regulatory approval processes.

We may also not be able to enter into arrangements with CROs on commercially reasonable terms, or at all. In addition, our CROs will not be our employees, and except for remedies available to us under our agreements with such CROs, we will not be able to control whether or not they devote sufficient time and resources to our ongoing preclinical and clinical programs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our protocols, regulatory requirements, or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. CROs may also generate higher costs than anticipated. As a result, our business, financial condition and results of operations and the commercial prospects for our product candidates could be materially and adversely affected, our costs could increase, and our ability to generate revenue could be delayed.

Switching or adding additional CROs, medical institutions, clinical investigators or contract laboratories involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work replacing a previous CRO. As a result, delays occur, which can materially impact our ability to meet our desired development timelines. There can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse effect on our business, financial condition or results of operations.

***Our product candidates are subject to extensive regulation under the FDA, the EMA or comparable foreign authorities, which can be costly and time consuming, cause unanticipated delays or prevent the receipt of the required approvals to commercialize our product candidates.***

The clinical development, manufacturing, labeling, storage, record-keeping, advertising, promotion, export, marketing and distribution of our product candidates are subject to extensive regulation by the FDA and other U.S. regulatory agencies, the EMA or comparable authorities in foreign markets. In the U.S., neither us nor our collaborators are permitted to market our product candidates until we or our collaborators receive approval of an NDA from the FDA or receive similar approvals abroad. The process of obtaining these approvals is expensive, often takes many years, and can vary substantially based upon the type, complexity and novelty of the product candidates involved. Approval policies or regulations may change and may be influenced by the results of other similar or competitive products, making it more difficult for us to achieve such approval in a timely manner or at all. Any guidance that may result from recent FDA advisory panel discussions may make it more expensive to develop and commercialize such product candidates. In addition, as a company, we have not previously filed NDAs with the FDA or filed similar applications with other foreign regulatory agencies. This lack of experience may impede our ability to obtain FDA or other foreign regulatory agency approval in a timely manner, if at all, for our product candidates for which development and commercialization is our responsibility.

Despite the time and expense invested, regulatory approval is never guaranteed. The FDA, the EMA or comparable foreign authorities can delay, limit or deny approval of a product candidate for many reasons, including:

- a product candidate may not be deemed safe or effective;
- agency officials of the FDA, the EMA or comparable foreign authorities may not find the data from non-clinical or preclinical studies and clinical trials generated during development to be sufficient;
- the FDA, the EMA or comparable foreign authorities may not approve our third-party manufacturers' processes or facilities; or
- the FDA, the EMA or a comparable foreign authority may change our approval policies or adopt new regulations.

Our inability to obtain these approvals would prevent us from commercializing our product candidates.

***The FDA, the NIH and the EMA have demonstrated caution in their regulation of gene therapy treatments, and ethical and legal concerns about gene therapy and genetic testing may result in additional regulations or restrictions on the development and commercialization of our product candidates, which may be difficult to predict.***

The FDA, National Institutes of Health ("NIH") and the EMA have each expressed interest in further regulating biotechnology, including gene therapy and genetic testing. For example, the EMA advocates a risk-based approach to the development of a gene therapy product. Agencies at both the federal and state level in the United States, as well as U.S. congressional committees and other governments or governing agencies, have also expressed interest in further regulating the biotechnology industry. Such action may delay or prevent commercialization of some or all of our product candidates.

Regulatory requirements in the U.S. and in other jurisdictions governing gene therapy products have changed frequently and may continue to change in the future. The FDA established the Office of Cellular, Tissue and Gene Therapies within its Center for Biologics Evaluation and Research to consolidate the review of gene therapy and related products, and established the Cellular, Tissue and Gene Therapies Advisory Committee to advise this review. Prior to submitting an IND, our human clinical trials will be subject to review by the NIH Office of Biotechnology Activities ("OBA") Recombinant DNA Advisory Committee (the "RAC"). Following an initial review, RAC members make a recommendation as to whether the protocol raises important scientific, safety, medical, ethical or social issues that warrant in-depth discussion at the RAC's quarterly meetings. Even though the FDA decides whether individual gene therapy protocols may proceed under an IND, the RAC's recommendations are shared with the FDA and the RAC public review process, if undertaken, can delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and details and has not objected to its initiation or has notified the sponsor that the study may begin. Conversely, the FDA can put an IND on a clinical hold even if the RAC has provided a favorable review or has recommended against an in-depth, public review. Moreover, under guidelines published by the NIH, patient enrollment in our future gene silencing clinical trials cannot begin until the investigator for such clinical trial has received a letter from the OBA indicating that the RAC review process has been completed; and Institutional Biosafety Committee ("IBC") approval as well as all other applicable regulatory authorizations have been obtained. In addition to the government regulators, the IBC and IRB of each institution at which we will conduct clinical trials of our product candidates, or a central IRB if appropriate, would need to review the proposed clinical trial to assess the safety of the trial. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of any of our product candidates. Similarly, the EMA governs the development of gene therapies in the European Union and may issue new guidelines concerning the development and marketing authorization for gene therapy products and require that we comply with these new guidelines. These regulatory review agencies and committees and the new requirements or guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies or trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory agencies and committees and comply with applicable requirements and guidelines. If we fail to do so, we may be required to delay or discontinue development of such product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of an increased or lengthier regulatory approval process or further restrictions on the development of our product candidates can be costly and could negatively impact our or our collaborators' ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all.

***Even if our product candidates receive regulatory approval in the U.S., it may never receive approval or commercialize our products outside of the U.S.***

In order to market any products outside of the U.S., we must establish and comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy. Approval procedures vary among countries and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ from that required to obtain FDA approval. The regulatory approval process in other countries may include all of the risks detailed above regarding FDA approval in the U.S. as well as other risks. Regulatory approval in one country does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory process in others. Failure to obtain regulatory approval in other countries or any delay seeking or obtaining such approval would impair our ability to develop foreign markets for our product candidates.

***Even if any of our product candidates receive regulatory approval, our product candidates may still face future development and regulatory difficulties.***

If any of our product candidates receive regulatory approval, the FDA, the EMA or comparable foreign authorities may still impose significant restrictions on the indicated uses or marketing of the product candidates or impose ongoing requirements for potentially costly post-approval studies and trials. In addition, regulatory agencies subject a product, our manufacturer and the manufacturer's facilities to continual review and periodic inspections. If a regulatory agency discovers previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions on that product, our collaborators or us, including requiring withdrawal of the product from the market. Our product candidates will also be subject to ongoing FDA, EMA or comparable foreign authorities' requirements for the labeling, packaging, storage, advertising, promotion, record-keeping and submission of safety and other post-market information on the drug. If our product candidates fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning letters or other notices of possible violations;
- impose civil or criminal penalties or fines or seek disgorgement of revenue or profits;
- suspend any ongoing clinical trials;
- refuse to approve pending applications or supplements to approved applications filed by us or our collaborators;
- withdraw any regulatory approvals;
- impose restrictions on operations, including costly new manufacturing requirements, or shut down our manufacturing operations; or
- seize or detain products or require a product recall.

***The FDA, the EMA and comparable foreign authorities actively enforce the laws and regulations prohibiting the promotion of off-label uses.***

The FDA, the EMA and comparable foreign authorities strictly regulate the promotional claims that may be made about prescription products, such as our product candidates, if approved. In particular, a product may not be promoted for uses that are not approved by the FDA, the EMA or comparable foreign authorities as reflected in the product's approved labeling. If we receive marketing approval for our product candidates for our proposed indications, physicians may nevertheless use our products for their patients in a manner that is inconsistent with the approved label, if the physicians personally believe in their professional medical judgment that our products could be used in such manner. However, if we are found to have promoted our product candidates, if approved, for any off-label uses, the federal government could levy civil, criminal or administrative penalties, and seek fines against us. Such enforcement has become more common in the industry. The FDA, the EMA or comparable foreign authorities could also request that we enter into a consent decree or a corporate integrity agreement, or seek a permanent injunction against us under which specified promotional conduct is monitored, changed or curtailed. If we cannot successfully manage the promotion of our product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business, financial condition and results of operations.

***We and our potential contract manufacturers are subject to significant regulation with respect to manufacturing our product candidates. The manufacturing facilities on which we will rely may not continue to meet regulatory requirements.***

All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including our potential contract manufacturers for our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of contaminants or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our potential contract manufacturers must supply all necessary documentation in support of an NDA or marketing authorization application ("MAA") on a timely basis and must adhere to GLP and cGMP regulations enforced by the FDA, the EMA or comparable foreign authorities through their facilities inspection program. Some of our potential contract manufacturers may not have produced a commercially approved pharmaceutical product and therefore may not have obtained the requisite regulatory authority approvals to do so. The facilities and quality systems of some or all of our potential third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates or any of our other potential product candidates. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or the associated quality systems for compliance with the regulations applicable to the activities being conducted. Although we plan to oversee the contract manufacturers, we cannot control the manufacturing process of, and will be completely dependent on, our contract manufacturing partners for compliance with applicable regulatory requirements. If these facilities do not pass a pre-approval plant inspection, regulatory approval of the product candidates may not be granted or may be substantially delayed until any violations are corrected to the satisfaction of the regulatory authority, if ever.

The regulatory authorities also may, at any time following approval of a product for sale, audit the manufacturing facilities of our potential third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly or time consuming for us or a third party to implement, and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we may contract could materially harm our business, financial condition and results of operations.

If we or any of our potential third-party manufacturers fail to maintain regulatory compliance, the FDA, the EMA or comparable foreign authorities can impose regulatory sanctions including, among other things, refusal to approve a pending application for a product candidate, withdrawal of an approval, or suspension of production. As a result, our business, financial condition and results of operations may be materially and adversely affected.



Additionally, if supply from one manufacturer is interrupted, an alternative manufacturer would need to be qualified through an NDA supplement or MAA variation, or equivalent foreign regulatory filing, which could result in further delay. The regulatory agencies may also require additional studies or trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause us to incur higher costs and could cause the delay or termination of clinical trials, regulatory submissions, required approvals, or commercialization of our product candidates. Furthermore, if our suppliers fail to meet contractual requirements and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed or we could lose potential revenue.

***\*\*\* Current and future legislation may increase the difficulty and cost of commercializing our product candidates and may affect the prices we may obtain if our product candidates are approved for commercialization.***

In the U.S. and some foreign jurisdictions, there have been a number of adopted and proposed legislative and regulatory changes regarding the healthcare system that could prevent or delay regulatory approval of our product candidates, restrict or regulate post-marketing activities and affect our ability to profitably sell any of our product candidates for which we obtain regulatory approval.

In the U.S., the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (“MMA”) changed the way Medicare covers and pays for pharmaceutical products. Cost reduction initiatives and other provisions of this legislation could limit the coverage and reimbursement rate that we receive for any of our approved product candidates. While the MMA only applies to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the MMA may result in a similar reduction in payments from private payors.

In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively the “ACA”), was enacted. The ACA was intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against healthcare fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The ACA increased manufacturers’ rebate liability under the Medicaid Drug Rebate Program by increasing the minimum rebate amount for both branded and generic drugs and revised the definition of “average manufacturer price” (“AMP”), which may also increase the amount of Medicaid drug rebates manufacturers are required to pay to states. The legislation also expanded Medicaid drug rebates and created an alternative rebate formula for certain new formulations of certain existing products that is intended to increase the rebates due on those drugs. The Centers for Medicare & Medicaid Services, which administers the Medicaid Drug Rebate Program, also has proposed to expand Medicaid rebates to the utilization that occurs in the territories of the U.S., such as Puerto Rico and the Virgin Islands. Further, beginning in 2011, the ACA imposed a significant annual fee on companies that manufacture or import branded prescription drug products. Legislative and regulatory proposals have been introduced at both the state and federal level to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products.

There have been recent public announcements by members of the U.S. Congress, President Trump and his administration regarding their plans to repeal and replace the ACA and Medicare. For example, on December 22, 2017, President Trump signed into law the Tax Cuts and Jobs Act of 2017, which, among other things, eliminated the individual mandate requiring most Americans (other than those who qualify for a hardship exemption) to carry a minimum level of health coverage, effective January 1, 2019. We are not sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA’s approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing approval testing and other requirements.

Additionally, there has been heightened governmental scrutiny in the U.S. of pharmaceutical pricing practices in light of the rising cost of prescription drugs and biologics. Such scrutiny has resulted in several recent congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. At the federal level, the Trump administration's budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. Further, the Trump administration released a "Blueprint," or plan, to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase drug manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products, and reduce the out of pocket costs of drug products paid by consumers. The U.S. Department of Health and Human Services has already started the process of soliciting feedback on some of these measures and, at the same, is immediately implementing others under its existing authority. While some proposed measures will require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

We expect that these and other healthcare reform measures that may be adopted in the future may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved drug. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our drugs.

In June 2016, the United Kingdom ("UK") held a referendum pursuant to which voters elected to leave the European Union ("EU"), commonly referred to as Brexit. The UK formally left the EU on January 31, 2020 and began a transition period that is scheduled to end on December 31, 2020. During the transition period, the UK remains subject to EU law while they negotiate their future relationship with the EU, but is no longer part of the EU's political institutions and agencies. Although the long-term effects of Brexit will depend on any agreements the UK makes to retain access to the EU markets, Brexit has created additional uncertainties that may ultimately result in new regulatory costs and challenges for biotechnology companies. We cannot predict what consequences the withdrawal of the United Kingdom from the European Union, if it occurs, might have on the regulatory frameworks of the United Kingdom or the European Union, or on our future operations, if any, in these jurisdictions.

***Changes in government funding for the FDA and other government agencies could hinder their ability to hire and retain key leadership and other personnel, properly administer drug innovation, or prevent our product candidates from being developed or commercialized, which could negatively impact our business, financial condition and results of operations.***

The ability of the FDA to review and approve new products can be affected by a variety of factors, including budget and funding levels, ability to hire and retain key personnel, and statutory, regulatory and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

In December 2016, the 21st Century Cures Act was signed into law. This new legislation is designed to advance medical innovation and empower the FDA with the authority to directly hire positions related to drug and device development and review. However, government proposals to reduce or eliminate budgetary deficits may include reduced allocations to the FDA and other related government agencies. These budgetary pressures may result in a reduced ability by the FDA to perform their respective roles; including the related impact to academic institutions and research laboratories whose funding is fully or partially dependent on both the level and timing of funding from government sources.

Disruptions at the FDA and other agencies may also slow the time necessary for our product candidates to be reviewed or approved by necessary government agencies, which could adversely affect our business, financial condition and results of operations.

***We are subject to "fraud and abuse" and similar laws and regulations, and a failure to comply with such regulations or prevail in any litigation related to noncompliance could harm our business, financial condition and results of operations.***

In the U.S., we are subject to various federal and state healthcare “fraud and abuse” laws, including anti-kickback laws, false claims laws and other laws intended, among other things, to reduce fraud and abuse in federal and state healthcare programs. The federal Anti-Kickback Statute makes it illegal for any person, including a prescription drug manufacturer, or a party acting on its behalf, to knowingly and willfully solicit, receive, offer or pay any remuneration that is intended to induce the referral of business, including the purchase, order or prescription of a particular drug, or other good or service for which payment in whole or in part may be made under a federal healthcare program, such as Medicare or Medicaid. Although we seek to structure our business arrangements in compliance with all applicable requirements, these laws are broadly written, and it is often difficult to determine precisely how the law will be applied in specific circumstances. Accordingly, it is possible that our practices may be challenged under the federal Anti-Kickback Statute.

The federal False Claims Act prohibits anyone from, among other things, knowingly presenting or causing to be presented for payment to the government, including the federal healthcare programs, claims for reimbursed drugs or services that are false or fraudulent, claims for items or services that were not provided as claimed, or claims for medically unnecessary items or services. Under the Health Insurance Portability and Accountability Act of 1996, we are prohibited from knowingly and willfully executing a scheme to defraud any healthcare benefit program, including private payors, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services to obtain money or property of any healthcare benefit program. Violations of fraud and abuse laws may be punishable by criminal or civil sanctions, including penalties, fines or exclusion or suspension from federal and state healthcare programs such as Medicare and Medicaid and debarment from contracting with the U.S. government. In addition, private individuals have the ability to bring actions on behalf of the government under the federal False Claims Act as well as under the false claims laws of several states.

Many states have adopted laws similar to the federal Anti-Kickback Statute, some of which apply to the referral of patients for healthcare services reimbursed by any source, not just governmental payors. In addition, some states have passed laws that require pharmaceutical companies to comply with the April 2003 Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers or the Pharmaceutical Research and Manufacturers of America’s Code on Interactions with Healthcare Professionals. Several states also impose other marketing restrictions or require pharmaceutical companies to make marketing or price disclosures to the state. There are ambiguities as to what is required to comply with these state requirements and if we fail to comply with an applicable state law requirement, we could be subject to penalties.

Neither the government nor the courts have provided definitive guidance on the application of fraud and abuse laws to our business. Law enforcement authorities are increasingly focused on enforcing these laws, and it is possible that some of our practices may be challenged under these laws. Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. If we are found in violation of one of these laws, we could be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from governmental funded federal or state healthcare programs and the curtailment or restructuring of our operations. If this occurs, our business, financial condition and results of operations may be materially adversely affected.

***If we face allegations of noncompliance with the law and encounter sanctions, our reputation, revenues and liquidity may suffer, and any of our product candidates that are ultimately approved for commercialization could be subject to restrictions or withdrawal from the market.***

Any government investigation of alleged violations of law could require us to expend significant time and resources in response, and could generate negative publicity. Any failure to comply with ongoing regulatory requirements may significantly and adversely affect our ability to generate revenues from any of our product candidates that are ultimately approved for commercialization. If regulatory sanctions are applied or if regulatory approval is withdrawn, our business, financial condition and results of operations will be adversely affected. Additionally, if we are unable to generate revenues from product sales, our potential for achieving profitability will be diminished and our need to raise capital to fund our operations will increase.

#### **Risks Related to Our Common Stock**

***\*\*\*The market price of our common stock is expected to be volatile***

The trading price of our stock is likely to be volatile. Our stock price could be subject to wide fluctuations in response to a variety of factors, including the following:

- our ability to conduct and achieve continued positive outcomes from our preclinical activities on the PATrOL™ platform and disease specific programs;
- public health crises, pandemics and epidemics, such as a novel strain of coronavirus (COVID-19) and their effects on our preclinical activities;
- results from, and any delays in, anticipated in-vitro or in-vivo preclinical studies;
- contracting with third parties such as academic institutions, and various CROs who will perform such studies, or the potential lack of performance of such organizations;
- acceptance of INDs by the FDA or similar regulatory filing by comparable foreign regulatory authorities for the conduct of clinical trials of our product candidates and our proposed design of future clinical trials;
- clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain, so failure can occur at any time during the clinical trial process;
- delays in publications of research findings;
- significant lawsuits, including patent or stockholder litigation;
- inability to obtain additional funding or funding on favorable terms;
- failure to successfully develop and commercialize our product candidates;
- changes in laws or regulations applicable to our product candidates;
- inability to obtain adequate product supply for our product candidates, or the inability to do so at acceptable prices or in an acceptable timeframe;
- unanticipated serious safety concerns related to our PATrOL™ platform or any of our product candidates;
- adverse regulatory decisions;
- introduction of new products or technologies by our competitors;
- adverse events or results for our competitors or our product candidate target areas that could generally adversely affect us our or our industry;
- failure to meet or exceed drug development or financial projections we provide to the public;
- failure to meet or exceed the estimates, expectations and projections of the investment community and our stockholders;
- the perception of the pharmaceutical industry by the public, legislatures, regulators and the investment community;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;

- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our licensed and owned technologies;
- additions or departures of key scientific or management personnel;
- changes in the market valuations of similar companies;
- general economic and market conditions and overall fluctuations in the U.S. equity market;
- sales of our common stock by us or our stockholders in the future;
- trading volume of our common stock;
- period-to-period fluctuations in our financial results;
- any identified material weakness in our internal control over financial reporting;
- changes in the structure of health care payments;
- changes in the Nasdaq listing of our stock; and
- recommendations of equity analysts covering our stock.

In addition, the stock market, and equity values of small pharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. Further, a decline in the financial markets and related factors beyond our control may cause our stock price to decline rapidly and unexpectedly.

In the past, following periods of volatility in the market price of a company's securities, stockholders have often instituted class action securities litigation against those companies. Such litigation, if instituted, could result in substantial costs and diversion of management attention and resources, which could significantly harm our profitability and reputation.

As previously disclosed in our Annual Report on Form 10-K for the fiscal year ended September 30, 2019 (the "Annual Report"), in connection with the preparation of the Company's consolidated financial statements for the fiscal year ended September 30, 2019, but prior to the issuance of such financial statements, the Company determined the accounting treatment and valuations pertaining to the PATrOL™ technology license acquired during the first quarter of fiscal 2019 should be modified. The Annual Report disclosed that the change in accounting treatment and valuations resulted in an increase in total operating expenses of approximately \$0.9 million on the Company's consolidated statements of operations for the fiscal year ended September 30, 2019 and a decrease in intangible assets of approximately \$1.5 million on the Company's consolidated balance sheet as of and for the fiscal year ended September 30, 2019, as well as a decrease in total operating expenses of approximately \$0.3 million on the Company's consolidated statements of operations in connection with the adjustment of the valuation of certain share-based awards for the fiscal year ended September 30, 2019. If we are required to restate any of our financial statements in the future due to our inability to adequately remedy the issues that gave rise to these modifications or for any other reason, we may be subject to regulatory penalties and investors could lose confidence in the accuracy and completeness of our financial statements, which could cause our share price to decline.

***Our management owns a significant percentage of our stock and is able to exert significant control over matters subject to stockholder approval.***

Dr. Stephan, our President, Chief Executive Officer and a director of us, holds a significant number of shares of our outstanding common stock and an option to purchase additional shares of common stock. Accordingly, Dr. Stephan has the ability to influence us through his ownership position.

This significant concentration of stock ownership may adversely affect the trading price for our common stock because investors often perceive disadvantages in owning stock in companies with controlling stockholders. As a result, Dr. Stephan could significantly influence all matters requiring approval by our stockholders, including the election of directors and the approval of mergers or other business combination transactions. Dr. Stephan may be able to determine all matters requiring stockholder approval. The interests of these stockholders may not always coincide with our interests or the interests of other stockholders. This may also prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interests as one of our stockholders, and he may act in a manner that advances his best interests and not necessarily those of other stockholders, including seeking a premium value for his common stock, and might affect the prevailing market price for our common stock.

***\*\*\* We have identified a material weakness in our disclosure controls and internal control over financial reporting. If we experience additional material weaknesses or other deficiencies in the future or otherwise fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately and timely report our financial results, in which case our business may be harmed, investors may lose confidence in the accuracy and completeness of our financial reports, and the price of our common stock may decline.***

Our management is required to report on the effectiveness of our internal control over financial reporting. The rules governing the standards that must be met for our management to assess our internal control over financial reporting are complex and require significant documentation, testing and possible remediation. We expect that compliance with these rules and regulations will continue to substantially increase our legal and financial compliance costs and will make some activities more time-consuming and costly, and our management and other personnel will devote a substantial amount of time to these compliance requirements.

In connection with the implementation of the necessary procedures and practices related to internal control over financial reporting, we may identify material weaknesses that we may not be able to remediate in time to meet the deadline imposed by the Sarbanes-Oxley Act for compliance with the requirements of Section 404. In addition, we may encounter problems or delays in completing the implementation of any improvements and receiving a favorable attestation in connection with the attestation provided by our independent registered public accounting firm, if and when required. Failure to achieve and maintain an effective internal control environment could have a material adverse effect on our business, financial condition and results of operations and could limit our ability to report our financial results accurately and in a timely manner.

In connection with an evaluation of the effectiveness of our disclosure controls and procedures and any changes in our internal control over financial reporting, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were not effective as of June 30, 2020 due to a material weakness in our internal control over financial reporting regarding complex accounting transactions. As a result, certain remediation actions have been recommended and we are in the process of implementing them, but our remediation efforts are not complete and are ongoing. Further remediation efforts may place a significant burden on management and add increased pressure to our financial resources and processes. As a result, we may not be successful in making the improvements necessary to identify or remediate additional control deficiencies identified by management, including material weaknesses, or be able to do so in a timely manner, in the future.

If we are unable to successfully remediate future material weaknesses or other deficiencies in our disclosure controls or internal control over financial reporting, the accuracy and timing of our financial reporting may be adversely affected; our liquidity, our access to capital markets and our ability to complete acquisitions may be adversely affected; we may be unable to maintain or regain compliance with applicable securities laws, The Nasdaq Stock Market LLC (“Nasdaq”) listing requirements, and the covenants under certain agreements regarding the timely filing of periodic reports; we may be subject to regulatory investigations and penalties; investors may lose confidence in our financial reporting; and our stock price may decline.

***\*\*\* We restated our previously issued unaudited financial statements for the three months ended December 31, 2019 and the three and six months ended March 31, 2019. As a result, and if we identify errors in our financial reporting in the future that require us to restate other previously issued financial statements, such restatements may subject us to unanticipated costs or regulatory penalties and could cause investors to lose confidence in the accuracy and completeness of our financial statements, which could cause the price of our common stock to decline.***

As discussed further in our Quarterly Reports on Form 10-Q for the quarters ended December 31, 2019 and March 31, 2020, we restated our unaudited condensed consolidated financial statements and related disclosures for the three months ended December 31, 2018 and the three and six months ended March 31, 2019, following the identification of misstatements therein in connection with the preparation of our financial statements for the quarterly period ended December 31, 2019. The misstatements were quantitatively material to the period presented in such prior financial statements, and we determined that it would be appropriate to correct the misstatements in such previously issued interim financial statements by restating such financial statements. We may be subject to unanticipated costs and regulatory penalties and investors could lose confidence in the accuracy and completeness of our financial statements, which could cause our share price to decline, due to such restatement and if we are required to restate any of our other financial statements in the future.

***\*\*\* As a result of our failure to timely file our Quarterly Report on Form 10-Q for the quarter ended December 31, 2019, we are currently ineligible to file new short form registration statements on Form S-3, which may impair our ability to raise capital on terms favorable to us, in a timely manner or at all.***

Form S-3 permits eligible issuers to conduct registered offerings using a short form registration statement that allows the issuer to incorporate by reference its past and future filings and reports made under the Securities Exchange Act of 1934, as amended (the "Exchange Act"). In addition, Form S-3 enables eligible issuers to conduct primary offerings "off the shelf" under Rule 415 of the Securities Act of 1933, as amended (the "Securities Act"). The shelf registration process, combined with the ability to forward incorporate information, allows issuers to avoid delays and interruptions in the offering process and to access the capital markets in a more expeditious and efficient manner than raising capital in a standard registered offering pursuant to a registration statement on Form S-1. The ability to newly register securities for resale may also be limited as a result of the loss of Form S-3 eligibility with respect to such registrations.

As previously disclosed in our Annual Report on Form 10-K for the fiscal year ended September 30, 2019 (the "Annual Report"), in connection with the preparation of the Company's consolidated financial statements for the fiscal year ended September 30, 2019, but prior to the issuance of such financial statements, the Company determined the accounting treatment and valuations pertaining to the PATROL™ technology license acquired during the first quarter of fiscal 2019 should be modified. The Annual Report disclosed that the change in accounting treatment and valuations resulted in an increase in total operating expenses of approximately \$0.9 million on the Company's consolidated statements of operations for the fiscal year ended September 30, 2019 and a decrease in intangible assets of approximately \$1.5 million on the Company's consolidated balance sheet as of and for the fiscal year ended September 30, 2019, as well as a decrease in total operating expenses of approximately \$0.3 million on the Company's consolidated statements of operations in connection with the adjustment of the valuation of certain share-based awards for the fiscal year ended September 30, 2019. In addition, on February 12, 2020, we dismissed our former independent registered public accounting firm. Accordingly, we were unable to complete the compilation, analysis and review of information required to be included in our Quarterly Report on Form 10-Q for the quarter ended December 31, 2019 until after the deadline for such filing.

As a result of our failure to timely file our Quarterly Report on Form 10-Q for the quarter ended December 31, 2019, we are currently ineligible to file new short form registration statements on Form S-3, and, absent a waiver of the Form S-3 eligibility requirements, we will no longer be permitted to use our existing registration statements on Form S-3 upon the earlier to occur of the expiration of such registration statements on Form S-3, the filing of our Annual Report on Form 10-K for the fiscal year ending September 30, 2020 or the occurrence of a fundamental change which would require a post-effective amendment to any such registration statements pursuant to Item 512 of Regulation S-K and Section 10(a)(3) of the Securities Act. As a consequence, we might not be permitted to sell all of the amount of common stock we could otherwise sell prior to such time, subject to the limits of General Instruction I.B.6 of Form S-3, which could adversely affect our ability to run our operations and progress our clinical and product development programs. We will not be permitted to conduct an "at the market offering" absent an effective primary registration statement on Form S-3.

Our inability to file new registration statements on Form S-3 may significantly impair our ability to raise necessary capital to run our operations and progress our clinical and product development programs. If we seek to access the capital markets through a registered offering during the period of time that we are unable to file a new registration statement on Form S-3, we may be required to publicly disclose the proposed offering and the material terms thereof before the offering commences, we may experience delays in the offering process due to SEC review of a Form S-1 registration statement and we may incur increased offering and transaction costs and other considerations. Disclosing a public offering prior to the formal commencement of an offering may result in downward pressure on our stock price. If we are unable to raise capital through a registered offering, we would be required to conduct our equity financing transactions on a private placement basis, which may be subject to pricing, size and other limitations imposed under Nasdaq rules, or seek other sources of capital.

Absent a waiver of the Form S-3 eligibility requirements and assuming we continue to timely file our required Exchange Act reports, the earliest we would regain the ability to file a new registration statement on Form S-3 is April 1, 2021.

***We may take advantage of specified reduced disclosure requirements applicable to a “smaller reporting company” under Regulation S-K, and the information that we provide to stockholders may be different than they might receive from other public companies.***

We are a “smaller reporting company,” as defined under Regulation S-K. As a smaller reporting company, we may take advantage of specified reduced disclosure and other requirements that are otherwise applicable generally to public companies. These provisions include, among other things, scaled disclosure requirements, including about our executive compensation arrangements.

We intend to continue to take advantage of certain of the scaled disclosure requirements of smaller reporting companies. We may continue to take advantage of these allowances until we are no longer a smaller reporting company. We will cease to be a smaller reporting company if we have (i) more than \$250 million in market value of our shares held by non-affiliates as of the last business day of our second fiscal quarter or (ii) more than \$100 million of annual revenues in our most recent fiscal year completed before the last business day of our second fiscal quarter and a market value of our shares held by non-affiliates more than \$700 million as of the last business day of our second fiscal quarter. We may choose to take advantage of some but not all of these scaled disclosure requirements. Therefore, the information that we provide stockholders may be different than one might get from other public companies. Further, if some investors find our shares of common stock less attractive as a result, there may be a less active trading market for our shares of common stock and the market price of such shares of common stock may be more volatile.

***If securities or industry analysts do not publish research, or publish inaccurate or unfavorable research, about our business, our stock price and trading volume could decline.***

The trading market for our common stock depends, in part, on the research and reports that securities or industry analysts publish about us or our business. If one or more of the analysts who cover us downgrade our stock or publish inaccurate or unfavorable research about our business, our stock price would likely decline. In addition, if our operating results fail to meet the forecast of analysts, our stock price would likely decline. If one or more of these analysts cease coverage of us or fail to publish reports on us regularly, demand for our common stock could decrease, which might cause our stock price and trading volume to decline.

***Sales of a substantial number of shares of our common stock in the public market by our stockholders, future issuances of our common stock or rights to purchase our common stock, could cause our stock price to fall.***

Sales of a substantial number of shares of our common stock by our existing stockholders in the public market, or the perception that these sales might occur, could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity securities. We are unable to predict the effect that such sales may have on the prevailing market price of our common stock.

***Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware and the federal district courts of the United States are the exclusive forums for substantially all disputes between us and our stockholders other than actions arising under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, which could limit our stockholders’ ability to obtain a favorable judicial forum for disputes with us or our directors, officers, or employees.***

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for:

- any derivative action or proceeding brought on our behalf;
- any action asserting a breach of fiduciary duty;



- any action asserting a claim against us arising under the General Corporation Law of the State of Delaware (the “DGCL”), our amended and restated certificate of incorporation or our amended and restated bylaws; and
- any action asserting a claim against us that is governed by the internal-affairs doctrine.

These exclusive-forum provisions do not apply to claims under the Securities Act, the Exchange Act or any other claims for which the federal courts have exclusive jurisdiction.

These exclusive forum provisions may limit a stockholder’s ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers, and other employees. If a court were to find either exclusive forum provision in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, it may incur additional costs associated with resolving the dispute in other jurisdictions, which could harm our business.

**\*\*\* We are subject to securities class action litigation and derivative shareholder litigation. If an unfavorable ruling were to occur, there exists the possibility of a material adverse impact on us.**

On February 14, 2018, plaintiff Jeevesh Khanna, commenced an action in the Southern District of New York, against Ohr and several current and former officers and directors, alleging that they violated federal securities laws between June 24, 2014 and January 4, 2018. On August 7, 2018, the lead plaintiffs, now George Lehman and Insured Benefit Plans, Inc., filed an amended complaint, stating the class period to be April 8, 2014 through January 4, 2018. The plaintiffs did not quantify any alleged damages in their complaint but, in addition to attorneys’ fees and costs, they seek to maintain the action as a class action and to recover damages on behalf of themselves and other persons who purchased or otherwise acquired Ohr common stock during the putative class period and purportedly suffered financial harm as a result. We and the individuals dispute these claims and intend to defend the matter vigorously. On September 17, 2018, Ohr filed a motion to dismiss the complaint. On September 20, 2019, the Court entered an order granting the defendants’ motion to dismiss. On October 23, 2019, the plaintiffs filed a notice of appeal of that order dismissing the action and other related orders by the Court, and the plaintiffs filed their appellate brief with respect to such matters with the Court on February 5, 2020. Scheduled briefing on plaintiffs’ appeal has concluded, and oral argument is calendared for September 21, 2020. This litigation could result in substantial costs and a diversion of management’s resources and attention, which could harm our business and the value of our common stock.

On May 3, 2018, plaintiff Adele J. Barke, derivatively on behalf of Ohr, commenced an action against certain former directors of Ohr, including Michael Ferguson, Orin Hirschman, Thomas M. Riedhammer, June Almenoff and Jason S. Slakter in the Supreme Court, State of New York, alleging that the action was brought in the right and for the benefit of Ohr seeking to remedy their “breach of fiduciary duties, corporate waste and unjust enrichment that occurred between June 24, 2014 and the present.” It does not quantify any alleged damages. We and the individuals dispute these claims and intend to defend the matter vigorously. Such litigation has been stayed pursuant to a stipulation by the parties, which has been so ordered by the court, pending a decision in the Southern District case on the motion to dismiss, but that status could change. This litigation could result in substantial costs and a diversion of management’s resources and attention, which could harm our business and the value of our common stock.

On March 20, 2019, a putative class action lawsuit was filed in the United States District Court for District of Delaware naming as defendants Ohr and its board of directors, Legacy NeuBase and Ohr Acquisition Corp., captioned *Wheby v. Ohr Pharmaceutical, Inc., et al.*, Case No. 1:19-cv-00541-UNA (the “Wheby Action”). The plaintiffs in the Wheby Action allege that the preliminary joint proxy/prospectus statement filed by Ohr with the SEC on March 8, 2019 contained false and misleading statements and omitted material information in violation of Section 14(a) of the Exchange Act and SEC Rule 14a-9 promulgated thereunder, and further that the individual defendants are liable for those alleged misstatements and omissions under Section 20(a) of the Exchange Act. The complaint in the Wheby Action has not been served on, nor was service waived by, any of the named defendants in that action. The action seeks, among other things, to rescind the Merger or an award of damages, and an award of attorneys’ and experts’ fees and expenses. The defendants dispute the claims raised in the Wheby Action. Management believes that the likelihood of an adverse decision from the sole remaining action is unlikely; however, the litigation could result in substantial costs and a diversion of management’s resources and attention, which could harm our business and the value of our common stock.

***Anti-takeover provisions in our charter documents and under Delaware law could make an acquisition of the Company more difficult and may prevent attempts by our stockholders to replace or remove our management.***

Provisions in our amended and restated certificate of incorporation and amended and restated bylaws may significantly reduce the value of shares of our common stock to a potential acquirer or delay or prevent an acquisition or a change in management without the consent of our board of directors. The provisions in our amended and restated certificate of incorporation and amended and restated bylaws include the following:

- a classified board of directors with three-year staggered terms, which may delay the ability of stockholders to change the membership of a majority of our board of directors;
- no cumulative voting in the election of directors, which limits the ability of minority stockholders to elect director candidates;
- the exclusive rights of our board of directors to establish the authorized number of directors and to elect a director to fill a vacancy created by the expansion of our board of directors or the death, resignation, disqualification, retirement or removal of a director, which prevents stockholders from being able to fill vacancies on our board of directors;
- a provision that directors may be removed by our stockholders only for cause;
- the ability of our board of directors to authorize the issuance of shares of preferred stock and to determine the price and other terms of those shares, including preferences and voting rights, without stockholder approval, which could be used to significantly dilute the ownership of a hostile acquirer;
- the ability of our board of directors to make, alter or appeal our amended and restated bylaws without obtaining stockholder approval;
- the affirmative vote of the holders of at least 66 2/3% of the voting power of all of the then-outstanding shares of our capital stock entitled to vote generally in the election of directors is required to amend, alter, repeal or adopt any provision inconsistent with, several of the provisions of our amended and restated certificate of incorporation and amended and restated bylaws;
- a prohibition on stockholder action by written consent, which forces stockholder action to be taken at an annual or special meeting of our stockholders;
- the requirement that a special meeting of stockholders may be called only by our board of directors, chief executive officer or president, which may delay the ability of our stockholders to force consideration of a proposal or to take action, including the removal of directors;
- a restriction on the forum for certain litigation against us to Delaware; and
- advance notice procedures that stockholders must comply with in order to nominate candidates to our board of directors or to propose matters to be acted upon at a stockholders' meeting, which may discourage or deter a potential acquirer from conducting a solicitation of proxies to elect the acquirer's own slate of directors or otherwise attempting to obtain control of us.

Although we believe these provisions collectively will provide for an opportunity to receive higher bids by requiring potential acquirers to negotiate with our board of directors, they would apply even if the offer may be considered beneficial by some stockholders. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove then current management by making it more difficult for stockholders to replace members of the board of directors, which is responsible for appointing the members of management.

Certain provisions of the DGCL deter hostile takeovers. Specifically, Section 203 of the DGCL prohibits a Delaware corporation from engaging in a business combination with an “interested stockholder” for a period of three years following the date the person first became an interested stockholder, unless (with certain exceptions) the business combination or the transaction by which the person became an interested stockholder is approved in a prescribed manner. Generally, a “business combination” includes a merger, asset or stock sale, or certain other transactions resulting in a financial benefit to the interested stockholder. Generally, an “interested stockholder” is a person who, together with affiliates and associates, beneficially owns or within three years prior to becoming an “interested stockholder” did own, 15% or more of a corporation’s outstanding voting stock. While this statute permits a corporation to opt out of these protective provisions in its certificate of incorporation, our certificate of incorporation does not include any such opt-out provision.

***Claims for indemnification by our directors and officers may reduce our available funds to satisfy successful third-party claims against us and may reduce the amount of money available to us.***

Our amended and restated certificate of incorporation provides that we will indemnify our directors and officers, in each case to the fullest extent permitted by Delaware law.

In addition, as permitted by Section 145 of the General Corporation Law of the State of Delaware, or the DGCL, our amended and restated certificate of incorporation and our indemnification agreements that we have entered into with our directors and officers provide that:

- We will indemnify our directors and officers for serving us in those capacities or for serving other business enterprises at our request, to the fullest extent permitted by Delaware law. Delaware law provides that a corporation may indemnify such person if such person acted in good faith and in a manner such person reasonably believed to be in or not opposed to the best interests of the registrant and, with respect to any criminal proceeding, had no reasonable cause to believe such person’s conduct was unlawful.
- We may, in our discretion, indemnify employees and agents in those circumstances where indemnification is permitted by applicable law.
- We are required to advance expenses actually and reasonably incurred by our directors and officers in connection with any proceeding, except that such directors or officers shall undertake to repay such advances if it is ultimately determined by a court of competent jurisdiction that such person is not entitled to indemnification.
- We will not be obligated pursuant to our amended and restated certificate of incorporation to indemnify a person with respect to proceedings initiated by that person against us or our other indemnitees, except with respect to proceedings authorized by our board of directors or brought to enforce a right to indemnification.
- The rights to indemnification conferred in our amended and restated certificate of incorporation are not exclusive, and we are authorized to enter into indemnification agreements with our directors, officers, employees and agents and to obtain insurance to indemnify such persons.
- We may not retroactively amend our amended and restated certificate of incorporation provisions to reduce our indemnification obligations to current or former directors or officers.

This indemnification policy could result in substantial expenditures by us, which we will be unable to recover.

*Our pre-Merger net operating loss carryforwards and certain other tax attributes will likely be subject to limitations. The pre-Merger net operating loss carryforwards and certain other tax attributes of us may also be subject to limitations as a result of ownership changes resulting from the Merger.*

In general, a corporation that undergoes an “ownership change,” as defined in Section 382 of the Internal Revenue Code of 1986, as amended, is subject to limitations on its ability to utilize its pre-change net operating losses (“NOLs”) to offset future taxable income (the “Section 382 Limitation”). Such an ownership change occurs if the aggregate stock ownership of certain stockholders, generally stockholders beneficially owning five percent or more of a corporation’s common stock, applying certain look-through and aggregation rules, increases by more than 50 percentage points over such stockholders’ lowest percentage ownership during the testing period, generally three years. Due to the ownership change of the Company upon completion of the Merger, our NOLs and certain other tax attributes will be subject to the Section 382 Limitation. Consequently, even if we achieve profitability, we may not be able to utilize a material portion of our NOLs and certain other tax attributes because of the Section 382 Limitation, which could have a material adverse effect on cash flow and results of operations. As of September 30, 2019, we estimated that we had approximately \$6.4 million in NOL carryforwards. The company has not completed an analysis regarding the limitation of net operating loss carryforwards, however, it is likely that the Section 382 Limitation will cause a significant portion of our NOL carryforwards to never be utilized. In addition, if we are determined to have discontinued our historic business following the completion of the Merger, subject to certain exceptions, the Section 382 Limitation could eliminate all possibility of utilizing our NOL carryforwards.

*We may never pay dividends on our common stock so any returns would be limited to the appreciation of our stock.*

We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate we will declare or pay any cash dividends for the foreseeable future. In addition, the terms of any future debt agreements may preclude us from paying dividends. Any return to stockholders will therefore be limited to the appreciation of their stock.

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

Not applicable.

## **ITEM 3. DEFAULTS UPON SENIOR SECURITIES**

Not applicable

## **ITEM 4. MINE SAFETY DISCLOSURES**

Not applicable

## **ITEM 5. OTHER INFORMATION**

Not applicable

ITEM 6. EXHIBITS

Exhibit Number	Description	Incorporated by Reference			
		Form	File Number	Filing Date	Exhibit
<a href="#">2.1+</a>	<a href="#">Agreement and Plan of Merger and Reorganization, dated as of January 2, 2019, by and among Ohr Pharmaceutical, Inc., Ohr Acquisition Corp. and NeuBase Therapeutics, Inc.</a>	<a href="#">8-K</a>	<a href="#">001-35963</a>	<a href="#">1/3/2019</a>	<a href="#">2.1</a>
<a href="#">2.2</a>	<a href="#">First Amendment to the Agreement and Plan of Merger and Reorganization, dated as of June 27, 2019, by and among Ohr Pharmaceutical, Inc., Ohr Acquisition Corp. and NeuBase Therapeutics, Inc.</a>	<a href="#">8-K</a>	<a href="#">001-35963</a>	<a href="#">7/3/2019</a>	<a href="#">2.1</a>
<a href="#">3.1</a>	<a href="#">Amended and Restated Certificate of Incorporation of the Company.</a>	<a href="#">8-K</a>	<a href="#">001-35963</a>	<a href="#">7/12/2019</a>	<a href="#">3.1</a>
<a href="#">3.2</a>	<a href="#">Amended and Restated Bylaws of the Company.</a>	<a href="#">8-K</a>	<a href="#">001-35963</a>	<a href="#">9/23/2019</a>	<a href="#">3.1</a>
<a href="#">4.1</a>	<a href="#">Form of Consulting Warrants.</a>	<a href="#">10-Q</a>	<a href="#">001-35963</a>	<a href="#">8/15/2011</a>	<a href="#">10.21</a>
<a href="#">4.2</a>	<a href="#">Form of Series A Warrant issued to investors pursuant to the Securities Purchase Agreement, dated December 7, 2016, by and among Ohr Pharmaceutical, Inc. and the purchasers listed therein.</a>	<a href="#">8-K</a>	<a href="#">001-35963</a>	<a href="#">12/8/2016</a>	<a href="#">4.1</a>
<a href="#">4.3</a>	<a href="#">Form of Warrant issued to investors pursuant to the Securities Purchase Agreement, dated as of April 5, 2017, by and among Ohr Pharmaceutical, Inc. and the purchasers listed therein.</a>	<a href="#">8-K</a>	<a href="#">001-35963</a>	<a href="#">4/6/2017</a>	<a href="#">4.1</a>
<a href="#">4.4</a>	<a href="#">Form of Common Stock Certificate.</a>	<a href="#">S-8</a>	<a href="#">333-233346</a>	<a href="#">8/16/2019</a>	<a href="#">4.17</a>
<a href="#">31.1*</a>	<a href="#">Certification of Chief Executive Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</a>				
<a href="#">31.2*</a>	<a href="#">Certification of Chief Financial Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</a>				
<a href="#">32.1*</a>	<a href="#">Certification of Chief Executive Officer and Chief Financial Officer Pursuant to 18 U.S.C Section 1350, As Adopted Pursuant to Section 906 of the Sarbanes Oxley Act of 2002.</a>				
101.INS*	XBRL Instance Document.				
101.SCH*	XBRL Taxonomy Extension Schema Document.				
101.CAL*	XBRL Taxonomy Extension Calculation Linkbase Document.				
101.DEF*	XBRL Taxonomy Extension Definition Linkbase Document.				
101.LAB*	XBRL Taxonomy Extension Label Linkbase Document.				
101.PRE*	XBRL Taxonomy Extension Presentation Linkbase Document.				

\* Filed herewith.

+ All schedules and exhibits to the agreement have been omitted pursuant to Item 601(b)(2) of Regulation S-K. A copy of any omitted schedule and/or exhibit will be furnished to the SEC upon request.

**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.

NeuBase Therapeutics, Inc.

Date: August 13, 2020

/s/ Sam Backenroth  
Sam Backenroth  
Chief Financial Officer  
*(Principal Financial and Accounting Officer)*

**CERTIFICATION PURSUANT TO  
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,  
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Dietrich Stephan, Ph.D., certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of NeuBase 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(s) 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(s) 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: August 13, 2020

By: \_\_\_\_\_  
/s/ Dietrich Stephan, Ph.D.  
Dietrich Stephan, Ph.D.  
*President and Chief Executive Officer*  
(Principal Executive Officer)

**CERTIFICATION PURSUANT TO  
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,  
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Sam Backenroth, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of NeuBase 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(s) 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(s) 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: August 13, 2020

By:

\_\_\_\_\_  
/s/ Sam Backenroth  
Sam Backenroth  
Chief Financial Officer  
(Principal Financial Officer)



**CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER AND PRINCIPAL FINANCIAL OFFICER  
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 on Form 10-Q of NeuBase Therapeutics, Inc. (the “Company”) for the period ended June 30, 2020 as filed with the Securities and Exchange Commission on the date hereof (the “Report”), the undersigned hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, to their knowledge that:

- (1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and
- (2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

By: \_\_\_\_\_  
                  /s/ Dietrich Stephan, Ph.D.  
                  Dietrich Stephan, Ph.D.  
                  *President and Chief Executive Officer*  
                  (Principal Executive Officer)  
                  August 13, 2020

By: \_\_\_\_\_  
                  /s/ Sam Backenroth  
                  Sam Backenroth  
                  *Chief Financial Officer*  
                  (Principal Financial and Accounting Officer)  
                  August 13, 2020

A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.

This certification accompanies the Report, is not deemed filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act (whether made before or after the date of the Report), irrespective of any general incorporation language contained in such filing.

---