

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, 2019

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)

(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, par value \$0.0001 per share	NBSE	NASDAQ Capital Market

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes No

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this Chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes No

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer", "accelerated filer", "smaller reporting company", and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer Accelerated filer
Non-accelerated filer Smaller reporting company
Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards 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 Exchange Act). Yes No

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date: 17,070,373 shares of Common Stock outstanding as of August 14, 2019.

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Item 1. Financial Statements.

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NEUBASE THERAPEUTICS, INC.
(Formerly Ohr Pharmaceutical, Inc.)
Consolidated Balance Sheets
(Unaudited)

	<u>June 30,</u> <u>2019</u>	<u>September 30,</u> <u>2018</u>
<u>ASSETS</u>		
CURRENT ASSETS		
Cash	\$ 1,424,596	\$ 3,750,436
Prepaid expenses and other current assets	133,501	247,998
Total Current Assets	1,558,097	3,998,434
EQUIPMENT, net	12,595	15,763
OTHER ASSETS		
Intangible assets, net	7,122,218	7,611,918
TOTAL ASSETS	\$ 8,692,910	\$ 11,626,115
<u>LIABILITIES AND STOCKHOLDERS' EQUITY</u>		
CURRENT LIABILITIES		
Accounts payable and accrued expenses	\$ 751,701	\$ 651,781
Notes payable	—	73,217
Total Current Liabilities	751,701	724,998
TOTAL LIABILITIES	751,701	724,998
STOCKHOLDERS' EQUITY		
Preferred stock, Series B, \$0.0001 par value; 6,000,000 shares authorized, 0 shares issued and outstanding as of June 30, 2019 and September 30, 2018	—	—
Common stock, \$0.0001 par value; 180,000,000 shares authorized, 2,829,248 shares issued and outstanding as of June 30, 2019 and September 30, 2018	283	283
Additional paid-in capital	132,440,080	132,226,341
Accumulated deficit	(124,499,154)	(121,325,507)
Total Stockholders' Equity	7,941,209	10,901,117
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	\$ 8,692,910	\$ 11,626,115

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

NEUBASE THERAPEUTICS, INC.
(Formerly Ohr Pharmaceutical, Inc.)
Consolidated Statements of Operations
(Unaudited)

	For the Three Months Ended June 30,		For the Nine Months Ended June 30,	
	2019	2018	2019	2018
OPERATING EXPENSES				
General and administrative	\$ 888,538	\$ 834,703	\$ 2,463,964	\$ 2,935,919
Research and development	82,751	52,630	235,289	4,242,307
Depreciation and amortization	165,647	279,008	496,936	842,519
Loss on impairment of goodwill	—	—	—	740,912
Gain on settlement of liabilities	—	—	—	(1,228,805)
OPERATING LOSS	1,136,936	1,166,341	3,196,189	7,532,852
OTHER INCOME				
Other income	—	610,383	—	592,584
Interest income	3,961	10,930	22,542	59,115
Total Other Income	3,961	621,313	22,542	651,699
NET LOSS	\$ (1,132,975)	\$ (545,028)	\$ (3,173,647)	\$ (6,881,153)
BASIC AND DILUTED LOSS PER SHARE (in dollars per share)	\$ (0.40)	\$ (0.19)	\$ (1.12)	\$ (3.36)
WEIGHTED AVERAGE NUMBER OF SHARES OUTSTANDING:				
BASIC AND DILUTED	2,829,248	2,823,321	2,829,248	2,045,829

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

NEUBASE THERAPEUTICS, INC.
(Formerly Ohr Pharmaceutical, Inc.)
Consolidated Statements of Cash Flows
(Unaudited)

	For the Nine Months Ended June 30,	
	2019	2018
OPERATING ACTIVITIES		
Net loss	\$ (3,173,647)	\$ (6,881,153)
Adjustments to reconcile net loss to net cash used by operating activities:		
Common stock issued for services	—	145,301
Stock option and warrant expense	213,739	804,374
Depreciation	7,236	7,877
Amortization of intangible assets	489,701	834,642
Gain on settlement of liabilities	—	1,228,805
Loss on sale of property and equipment	—	17,814
Gain on sale of intangible assets	—	(460,383)
Loss on impairment of goodwill	—	740,912
Accounts receivable	(2,800)	—
Changes in operating assets and liabilities		
Prepaid expenses and other current assets	117,297	165,533
Accounts payable and accrued expenses	99,920	(5,499,525)
Net Cash Used in Operating Activities	(2,248,554)	(8,895,803)
INVESTING ACTIVITIES		
Purchase of property and equipment	(4,069)	—
Proceeds from sale of property, equipment and intangible assets	—	508,078
Net Cash (Used in) Provided by Investing Activities	(4,069)	508,078
FINANCING ACTIVITIES		
Proceeds from warrants exercised for cash	—	270,000
Repayments of short-term notes payable	(73,217)	(247,933)
Net Cash Provided by/ (Used in) Financing Activities	(73,217)	22,067
NET CHANGE IN CASH	(2,325,840)	(8,365,658)
CASH AT BEGINNING OF PERIOD	3,750,436	12,801,085
CASH AT END OF PERIOD	\$ 1,424,596	\$ 4,435,427
SUPPLEMENTAL DISCLOSURES OF CASH FLOW INFORMATION CASH PAID FOR:		
Interest	\$ 779	\$ 7,540
Income Taxes	—	—
NON CASH FINANCING ACTIVITIES:		
Financing of insurance premiums through issuance of short term notes	—	323,904

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

NEUBASE THERAPEUTICS, INC.
(Formerly Ohr Pharmaceutical, Inc.)
Consolidated Statements of Stockholders' Equity
(Unaudited)

	Common Stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount			
Balance, September 30, 2017	2,815,748	\$ 282	\$ 130,933,290	\$ (108,088,607)	\$ 22,844,965
Exercise of warrants for cash	11,250	1	224,999	—	225,000
Common stock issued for services	—	—	135,701	—	135,701
Fair value of employee stock options	—	—	629,286	—	629,286
Net loss for the three months ended December 31, 2017	—	—	—	(4,151,358)	(4,151,358)
Balance, December 31, 2017	2,826,998	\$ 283	\$ 131,923,276	\$ (112,239,965)	\$ 19,683,594
Exercise of warrants for cash	2,250	—	45,000	—	45,000
Common stock issued for services	—	—	9,600	—	9,600
Fair value of employee stock options	—	—	101,711	—	101,711
Net loss for the three months ended March 31, 2018	—	—	—	(2,184,767)	(2,184,767)
Balance, March 31, 2018	2,829,248	\$ 283	\$ 132,079,587	\$ (114,424,732)	\$ 17,655,138
Fair value of employee stock options	—	—	73,377	—	73,377
Net loss for the three months ended June 30, 2018	—	—	—	(545,028)	(545,028)
Balance, June 30, 2018	2,829,248	\$ 283	\$ 132,152,964	\$ (114,969,760)	\$ 17,183,487

	Common Stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount			
Balance, September 30, 2018	2,829,248	283	\$ 132,226,341	\$ (121,325,507)	\$ 10,901,117
Fair value of employee stock options	—	—	73,377	—	73,377
Net loss for the three months ended December 31, 2018	—	—	—	(897,624)	(897,624)
Balance, December 31, 2018	2,829,248	\$ 283	\$ 132,299,718	\$ (122,223,131)	\$ 10,076,870
Fair value of employee stock options	—	—	73,377	—	73,377
Net loss for the three months ended March 31, 2019	—	—	—	(1,143,048)	(1,143,048)
Balance, March 31, 2019	2,829,248	\$ 283	\$ 132,373,095	\$ (123,366,179)	\$ 9,007,199
Fair value of employee stock options	—	—	66,985	—	66,985
Net loss for the three months ended June 30, 2019	—	—	—	(1,132,975)	(1,132,975)
Balance, June 30, 2019	2,829,248	\$ 283	\$ 132,440,080	\$ (124,499,154)	\$ 7,941,209

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

NEUBASE THERAPEUTICS, INC.
(Formerly Ohr Pharmaceutical, Inc.)
Notes to Unaudited Consolidated Financial Statements
June 30, 2019

NOTE 1 – BASIS OF PRESENTATION AND DESCRIPTION OF BUSINESS

The accompanying unaudited consolidated financial statements include the accounts of NeuBase Therapeutics, Inc. and its subsidiaries (the “Company”). The accompanying unaudited consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States (“GAAP”) for interim financial information and with the instructions to Form 10-Q and Rule 10-01 of Regulation S-X related to interim period financial statements. Accordingly, these consolidated financial statements do not include certain information and footnotes required by GAAP for complete financial statements. However, in the opinion of management, all adjustments (which include only normal recurring adjustments) necessary to present fairly the financial position, results of operations, and cash flows at June 30, 2019, and for all periods presented herein, have been made.

It is suggested that these unaudited consolidated financial statements be read in conjunction with the consolidated financial statements and notes thereto included in the Company’s Annual Report on Form 10-K for the fiscal year ended September 30, 2018. The results of operations for the three and nine month periods ended June 30, 2019 and 2018 are not necessarily indicative of the operating results for the full years.

Merger

On July 12, 2019, the Company completed a reverse merger transaction (the “Merger”) with NeuBase Corporation, a Delaware corporation (formerly known as NeuBase Therapeutics, Inc.) (“Legacy NeuBase”). At the closing of the Merger, each outstanding share of Legacy NeuBase’s capital stock was converted into the right to receive 1.019055643 shares of the Company’s common stock. Upon completion of the Merger, the Company changed its name to NeuBase Therapeutics, Inc., and will focus on developing next generation gene silencing therapies to treat rare genetic diseases caused by mutant proteins. Shares of the Company’s common stock commenced trading on the Nasdaq Capital Market under the ticker symbol “NBSE” as of market open on July 15, 2019. The Company’s previous ticker symbol was “OHRP”.

The financial information included in the Notes to the Unaudited Consolidated Financial Statements is that of Ohr Pharmaceutical, Inc. prior to the Merger because the Merger was consummated after the period covered by the financial statements included in this Quarterly Report. Accordingly, the historical financial information included in this Quarterly Report, unless otherwise indicated or as the context otherwise requires, is that of Ohr Pharmaceutical, Inc. prior to the Merger.

Reverse Stock Split

On January 18, 2019, following a special meeting of the Company’s stockholders, the board of directors of the Company approved a one-for-twenty reverse stock split of the Company’s issued and outstanding shares of common stock (the “Reverse Stock Split”). On January 23, 2019, the Company filed with the Secretary of State of the State of Delaware a Certificate of Amendment to its Certificate of Incorporation to effect the Reverse Stock Split. The Company’s common stock began trading on a split-adjusted basis when the market opened on February 4, 2019. As a result of the Reverse Stock Split, the outstanding common stock has decreased from 56,466,428 shares of common stock, par value \$0.0001 per share, to 2,829,248 shares of common stock, par value \$0.0001 per share. Unless otherwise noted, impacted amounts and share information included in the financial statements and notes thereto, and elsewhere in this Form 10-Q, have been retroactively adjusted for the Reverse Stock Split as if such Reverse Stock Split occurred on the first day of the first period presented. Certain amounts in the financial statements, the notes thereto, and elsewhere in this Form 10-Q, may be slightly different than previously reported due to rounding of fractional shares as a result of the Reverse Stock Split.

Business

The Company is a biotechnology company focused on developing next generation gene silencing therapies to treat rare genetic diseases caused by mutant proteins. The type of therapies that the Company is developing are termed antisense oligonucleotide therapies (“ASOs”), which 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 that cause disease. The Company is a leader in the discovery and development of the class of ribonucleic acid (“RNA”)-targeted ASO drugs called peptide nucleic acids (“PNAs”). Its proprietary gamma Peptide-nucleic acid AnTisense OLigonucleotide (“PATrOL™”) platform allows for a more efficient discovery of drug product candidates, potentially transforming the treatment paradigm for people affected by rare genetic diseases, with an initial focus on neurological disorders.

The Company is developing several preclinical programs using its PATROL™ platform, including: NT0100, targeted at Huntington's Disease, a repeat expansion disorder, and NT0200, targeted at myotonic dystrophy type 1 (DM1). In addition, the emerging pipeline of other assets that target secondary RNA structure allows a unique market advantage across a variety of rare diseases.

The Company is subject to risks common to companies in the biotechnology industry including, but not limited to, new technological innovations, protection of proprietary technology, dependence on key personnel, compliance with government regulations and the need to obtain additional financing. Pre-clinical programs currently under development will require significant additional research and development efforts, including extensive pre-clinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure and extensive compliance-reporting capabilities. There can be no assurance that the Company's research and development will be successful, that adequate protection for the Company's intellectual property will be obtained, that any products developed will obtain necessary government regulatory approval or that any approved products will be commercially viable. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will generate significant revenue from product sales. The Company operates in an environment of rapid change in technology and substantial competition from pharmaceutical and biotechnology companies. In addition, the Company is dependent upon the services of its employees and consultants.

Liquidity

The Company's independent registered public accounting firm expressed in its report on the Company's financial statements for the years ended September 30, 2018 and 2017, that there was substantial doubt about the Company's ability to continue as a going concern. The Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that the consolidated financial statements are issued. To date, the Company has no revenue from product sales and management expects continuing operating losses and negative cash outflows in the future. The accompanying consolidated financial statements have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the ordinary course of business. Alongside the closing of the Merger, NeuBase completed two financings raising gross proceeds of approximately \$14 million, and we believe that our current cash balance will be sufficient to fund our operating expenses and capital expenditure requirements for at least the next 12 months from issuance of the financial statements as of, and for the period ended, June 30, 2019. Accordingly, based on management's plans and the significant capital raised in connection with the Merger in July 2019, that substantial doubt has been resolved.

NOTE 2 – SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Use of Estimates

The preparation of financial statements in conformity with accounting principles generally accepted in the United States of America requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities at the date of the financial statements and the reported amounts of revenue and expenses during the reporting period. Actual results could differ from those estimates. Estimates subject to change in the near term include impairment (if any) of long-lived assets and fair value of liabilities.

Impairment of Long-Lived Assets

Long-lived tangible assets and definite-lived intangible assets are reviewed for possible impairment whenever events or changes in circumstances indicate that the carrying amount of such assets may not be recoverable. The Company uses an estimate of undiscounted future net cash flows of the assets over the remaining useful lives in determining whether the carrying value of the assets is recoverable. If the carrying values of the assets exceed the expected future cash flows of the assets, the Company recognizes an impairment loss equal to the difference between the carrying values of the assets and their estimated fair values. Impairment of long-lived assets is assessed at the lowest levels for which there are identifiable cash flows that are independent from other groups of assets. The evaluation of long-lived assets requires the Company to use estimates of future cash flows. However, actual cash flows may differ from the estimated future cash flows used in these impairment tests.

Fair Value of Financial Instruments

In accordance with ASC 820, the carrying value of cash and cash equivalents and accounts payable approximates fair value due to the short-term maturity of these instruments. ASC 820 clarifies the definition of fair value, prescribes methods for measuring fair value, and establishes a fair value hierarchy to classify the inputs used in measuring fair value as follows:

Level 1 - Inputs are unadjusted quoted prices in active markets for identical assets or liabilities available at the measurement date.

Level 2 - Inputs are unadjusted quoted prices for similar assets and liabilities in active markets, quoted prices for identical or similar assets and liabilities in markets that are not active, inputs other than quoted prices that are observable, and inputs derived from or corroborated by observable market data.

Level 3 - Unobservable inputs, where there is little or no market activity for the asset or liability. These inputs reflect the reporting entity's own beliefs about the assumptions that market participants would use in pricing the asset or liability, based on the best information available in the circumstances.

A financial instrument's categorization within the valuation hierarchy is based upon the lowest level of input that is significant to the fair value measurement.

There were no financial instruments required to be measured at fair value on a recurring basis as of September 30, 2018 and June 30, 2019.

Goodwill and Intangibles

The Company evaluates goodwill and other finite-lived intangible assets in accordance with FASB ASC Topic 350, "Intangibles — Goodwill and Other." Goodwill is recorded at the time of an acquisition and is calculated as the difference between the total consideration paid for an acquisition and the fair value of the net tangible and intangible assets acquired. Accounting for acquisitions requires extensive use of accounting estimates and judgments to allocate the purchase price to the fair value of the net tangible and intangible assets acquired, including in-process research and development ("IPR&D"). Goodwill is deemed to have an indefinite life and is not amortized, but is subject to annual impairment tests. If the assumptions and estimates used to allocate the purchase price are not correct, or if business conditions change, purchase price adjustments or future asset impairment charges could be required. The value of our goodwill could be impacted by future adverse changes such as: (i) any future declines in our operating results, (ii) a decline in the valuation of technology, including the valuation of our common stock, (iii) a significant slowdown in the worldwide economy or (iv) any failure to meet the performance projections included in our forecasts of future operating results. In accordance with FASB ASC Topic 350, the Company tests goodwill for impairment on an annual basis or more frequently if the Company believes indicators of impairment exist. Impairment evaluations involve management estimates of asset useful lives and future cash flows. Significant management judgment is required in the forecasts of future operating results that are used in the evaluations. It is possible, however, that the plans and estimates used may be incorrect. If our actual results, or the plans and estimates used in future impairment analysis, are lower than the original estimates used to assess the recoverability of these assets, we could incur additional impairment charges in a future period.

The Company performs its annual impairment review of goodwill in September of each fiscal year, and when a triggering event occurs between annual impairment tests for both goodwill and other finite-lived intangible assets. During the year ended September 30, 2018, the Company determined that due to the reduced price of the Company's common stock and the market capitalization of the Company relative to the value of the intangible assets and goodwill, an impairment analysis was required for the intangible assets and goodwill. The Company performed the tests and concluded that the intangible assets were impaired and recorded a loss of \$5,313,640, and wrote off the \$740,912 goodwill balance.

The Company's finite-lived intangible assets consist of license rights and patents. The Company amortizes its patents over the life of each patent and license rights over the remaining life of the patents that it has rights for. During the three months and nine months ended June 30, 2019, the Company recognized \$163,234 and \$489,701 respectively in amortization expense on the patents and license rights.

Research and Development

Research and development expenses are expensed in the consolidated statements of operations as incurred in accordance with FASB ASC 730, *Research and Development*. Research and development expenses include salaries, related employee expenses, clinical trial expenses, research expenses, manufacturing expenses, consulting fees, and laboratory costs. The Company incurred net research and development expenses of \$235,289, and \$4,242,307, during the nine months ended June 30, 2019, and 2018, respectively.

Share-Based Compensation

The Company follows the provisions of ASC 718, "Share-Based Payments" which requires all share-based payments to employees, including grants of employee stock options, be recognized in the income statement based on their fair values. The Company uses the Black Scholes pricing model for determining the fair value of stock options and the stock price on the date of issuance to determine the fair value of restricted stock awards.

In accordance with ASC 505, equity instruments issued to non-employees for goods or services are accounted for at fair value and are marked to market until service is complete or a performance commitment date is reached, whichever is earlier.

Stock-based compensation expense is recognized in the Company's financial statements on a straight-line basis over the awards' vesting periods. The stock-based compensation awards generally vest over a period of up to ten years.

Loss Per Share

Basic loss per common share is computed by dividing losses attributable to common shareholders by the weighted-average number of shares of common stock outstanding during the period.

Diluted loss per common share is computed by dividing losses attributable to common shareholders by the weighted-average number of shares of common stock outstanding during the period increased to include the number of additional shares of common stock that would have been outstanding if the potentially dilutive securities had been issued. Potentially dilutive securities include outstanding stock options and warrants.

For the nine months ended June 30, 2019, there were no potentially dilutive securities (warrants or options).

Recent Accounting Pronouncements

The Company has implemented all new relevant accounting pronouncements that are in effect through the date of these financial statements. The pronouncements did not have any material impact on the financial statements unless otherwise disclosed, and the Company does not believe that there are any other new accounting pronouncements that have been issued that might have a material impact on its consolidated financial position or results of operations.

NOTE 3 – INTANGIBLE ASSETS

Intangible assets at June 30, 2019 and September 30, 2018:

	June 30, 2019	September 30, 2018
License Rights	\$ 17,712,991	\$ 17,712,991
Patent Costs	100,000	100,000
	17,812,991	17,812,991
Accumulated Amortization and impairment	(10,690,773)	(10,201,073)
Total Intangible Assets	\$ 7,122,218	\$ 7,611,918

During the three and nine month periods ended June 30, 2018 the Company recognized \$276,667 and \$843,642, respectively, in amortization expense on the patents and license rights. During the three and nine month periods ended June 30, 2019 the Company recognized \$163,234 and \$489,701, respectively, in amortization expense on the patents and license rights.

NOTE 4 – NOTES PAYABLE

On February 28, 2018, the Company entered into a premium financing arrangement for its directors' and officers' insurance policy in the amount of \$323,094. The financing arrangement was a short term note, bore interest at a rate of 7.29% per annum, matured on November 28, 2018, and was secured by the underlying insurance policies and rights thereunder. During the nine months ended June 30, 2019, the Company had repaid the remaining \$73,217 and recorded interest of \$779.

NOTE 5 – EQUITY

Common Stock Warrants

Below is a table summarizing the warrants issued and outstanding as of June 30, 2019 ("Price" reflects the weighted average exercise price per share):

	<u>Warrants</u>	<u>Price</u>
Outstanding at September 30, 2018	805,968	\$ 24.39
Granted		
Investor warrants	—	—
Stock-based compensation warrants	—	—
Exercised		
Investor warrants	—	—
Stock-based compensation warrants	—	—
Forfeited or expired		
Investor warrants	—	—
Stock-based compensation warrants	(1,028)	157.60
Outstanding at June 30, 2019	<u>804,940</u>	<u>\$ 24.22</u>
Exercisable at June 30, 2019	<u>804,940</u>	<u>\$ 24.22</u>

As of June 30, 2019, the warrants have a weighted average remaining term of 2.70 years and have no intrinsic value.

Stock Based Compensation

The Company's Consolidated 2016 Stock Plan ("the Plan") provides for granting stock options and restricted stock awards to employees, directors and consultants of the Company. The Company uses the Black-Scholes pricing model for determining the fair value of stock options and warrants granted as share based compensation.

Warrants. During the nine month period ended June 30, 2019, the Company did not recognize any expense related to warrants granted as stock based compensation. There is no unamortized expense as of June 30, 2019 for outstanding warrants issued as stock based compensation. Refer to the Common Stock Warrants table within this note for information regarding all outstanding warrants.

Options. During the nine month period ended June 30, 2019, the Company recognized \$213,739 of expense related to options granted in prior years. Unamortized option expense as of June 30, 2019 for all options outstanding amounted to \$72,268. The Company expects to recognize this compensation cost over a weighted-average period of .22 years.

Below is a table summarizing the Company's activity for the nine month period ended June 30, 2019 ("Price" reflects the weighted average exercise price per share):

	Options	Price
Outstanding at September 30, 2018	156,625	\$ 57.86
Granted	—	\$ —
Exercised	—	\$ —
Forfeited or expired	—	\$ —
Outstanding at June 30, 2019	156,625	\$ 57.86
Exercisable at June 30, 2019	124,370	\$ 69.40

As of June 30, 2019, the outstanding options have a weighted average remaining term of 3.28 years and no intrinsic value.

Restricted Stock. During the nine month period ended June 30, 2019, the Company did not recognize any expense related to restricted stock awards. As of June 30, 2019, all restricted stock shares are fully vested, and there is no remaining unamortized expense.

NOTE 6 – COMMITMENTS AND CONTINGENCIES

Legal Proceedings

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, 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 Pharmaceutical, Inc. ("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. Ohr 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 November 13, 2018, plaintiffs filed a motion to strike exhibits appended to the motion to dismiss, which was fully briefed by the parties prior to proceeding on the Defendants' motion to dismiss. On May 10, 2019, the Court entered an order concluding that it is unable to decide the Plaintiffs' motion to strike independently of the Defendants' motion to dismiss and will consider the motions together. Briefing on Defendants' motion to dismiss has now concluded, and the parties are awaiting further ruling of the Court. 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 the Company's common stock.

On May 3, 2018, plaintiff Adele J. Barke, derivatively on behalf of Ohr, commenced an action against certain former directors of the Company, including Michael Ferguson, Orin Hirschman, Thomas M. Riedhammer, June Almenoff and Jason 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. Ohr 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 the Company's common stock.

Following the filing on March 8, 2019 with the SEC of Ohr's preliminary joint proxy statement/prospectus, on March 18, 2019, the Gomez Action was filed by an individual shareholder in the United States District Court for the Southern District of New York against Ohr and former members of its board of directors. The plaintiff in the Gomez Action alleges that the preliminary joint proxy/prospectus statement 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. On March 19, 2019, the Barke Action was filed in the United States District Court for the Southern District of New York asserting similar Section 14(a) and Section 20(a) claims against former members of Ohr's board of directors and additionally naming Legacy NeuBase and Ohr Acquisition Corp., but not Ohr, as defendants. On March 20, 2019, the Wheby Action was filed in the United States District Court for District of Delaware asserting similar claims under Section 14(a) and Section 20(a) and naming as defendants Ohr and former members of its board of directors, Legacy NeuBase, and Ohr Acquisition Corp. The complaint in the Wheby action has not been served on, nor was service waived by, any of the named Defendants in that action. On March 20, 2019, the Lowinger Action was filed in the Court of Chancery of the State of Delaware asserting a breach of fiduciary duty claim against former members of Ohr's board of directors arising out of the same facts and circumstances regarding certain alleged omissions in the preliminary joint proxy/prospectus statement. On April 4, 2019, the Garaygordobil Action was filed in the United States District Court for the Southern District of New York asserting similar Section 14(a) and Section 20(a) claims against Ohr and its board of directors.

Each of the Gomez, Barke, Garaygordobil, and Lowinger Actions have been dismissed, and on July 12, 2019, Ohr and Legacy NeuBase consummated the Merger. The sole remaining action seeks, among other things, to rescind the Merger or an award of damages, and an award of attorneys' and experts' fees and expenses. 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.

NOTE 7 – RELATED PARTY TRANSACTION

The Contract Research Organization ("CRO") that ran the Company's clinical trial contracted with Jason S. Slakter, M.D., P.C., d/b/a Digital Angiography Reading Center ("DARC"), a well-known digital reading center, which is owned by Dr. Jason Slakter, the Company's former CEO, pursuant to the Company's related party transactions policy, with the review and approval of the Audit Committee, to provide digital reading and imaging services in connection with the clinical study. During the nine months ended June 30, 2019, and 2018, the Company's CRO was paid \$0 and \$899,001, respectively, for pass through DARC expenses.

NOTE 8 – SUBSEQUENT EVENTS

Merger

On July 12, 2019, the Company completed the Merger with Legacy NeuBase, pursuant to which Ohr Acquisition Corp. merged with and into Legacy NeuBase, with Legacy NeuBase surviving as the Company's wholly-owned subsidiary. In connection with the Merger, Legacy NeuBase shareholders, warrant holders, and investors in the Pre-Merger Financing exchanged their Legacy NeuBase shares into an aggregate of 12,694,971 newly issued shares of the Company's common stock. See Note 1 to the Unaudited Consolidated Financial Statements above for additional information on the Merger.

Pre-Merger Financing

On July 11, 2019, prior to the completion of the Merger, Legacy NeuBase completed transactions contemplated by certain financing agreements (the "Pre-Merger Financing") resulting in gross proceeds to Legacy NeuBase of approximately \$9.0 million, consisting of (i) a private placement with certain accredited investors, whereby, among other things, Legacy NeuBase issued to such investors shares of Legacy NeuBase common stock for an aggregate purchase price of approximately \$8.4 million (the "Legacy NeuBase Equity Financing") and (ii) the conversion of outstanding convertible notes of Legacy NeuBase with an aggregate principal amount of \$600,000 (the "Legacy NeuBase Debt Financing"), which were automatically converted into Legacy NeuBase common stock immediately preceding the closing of the Legacy NeuBase Equity Financing at a conversion price equal to 90% of the purchase price per share of the Legacy NeuBase common stock issued in the Legacy NeuBase Equity Financing.

Post-Merger Financing

On July 16, 2019, the Company completed a private placement with certain accredited investors for the sale by the Company of an aggregate 1,538,462 shares of the Company's common stock for aggregate gross proceeds of \$5.0 million.

Issuances

On July 12, 2019, the Company issued options to purchase an aggregate of 2,909,827 shares of common stock to directors, employees, and consultants of the Company. The options have a ten year term, vest over a period of up to four years, and have an exercise price of \$5.39.

On August 9, 2019, the Company issued 7,692 shares of restricted common stock to a contractor for services to be provided to the Company.

Item 2. Management’s Discussion and Analysis of Financial Condition and Results of Operations.

Our discussion and analysis of the business and subsequent discussion of financial conditions may contain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Statements that are not historical in nature, including statements about beliefs and expectations, are forward-looking statements. Words such as “may,” “will,” “should,” “estimates,” “predicts,” “believes,” “anticipates,” “plans,” “expects,” “intends” and similar expressions are intended to identify these forward-looking statements, but are not the exclusive means of identifying such statements. Such statements are based on currently available operating, financial and competitive information and are subject to various risks and uncertainties as described in greater detail in Item 1A, Part II, our “Risk Factors” beginning on page 21 of this Report. You are cautioned that these forward-looking statements reflect management’s estimates only as of the date hereof, and we assume no obligation to update these statements, even if new information becomes available or other events occur in the future, except as required by law. Actual future results, events and trends may differ materially from those expressed in or implied by such statements depending on a variety of factors, including, but not limited to those set forth in our filings with the Securities and Exchange Commission (“SEC”). Specifically, and not in limitation of these factors, we may alter our plans, strategies, objectives or business.

The financial information included in this Management’s Discussion and Analysis of Financial Condition and Results of Operations is that of Ohr Pharmaceutical, Inc. prior to the Merger because the Merger was consummated after the period covered by the financial statements included in this Quarterly Report. Accordingly, the historical financial information included in this Quarterly Report, unless otherwise indicated or as the context otherwise requires, is that of Ohr Pharmaceutical, Inc. prior to the Merger.

Recent Developments

Merger

On July 12, 2019, NeuBase Therapeutics, Inc. (“we,” “us,” “our,” “NeuBase,” or the “Company”) completed a reverse merger transaction (the “Merger”) with NeuBase Corporation, a Delaware corporation (formerly known as NeuBase Therapeutics, Inc.) (“Legacy NeuBase”). At the closing of the Merger, each outstanding share of Legacy NeuBase’s capital stock was converted into the right to receive 1.019055643 shares of the Company’s common stock. Upon completion of the Merger, the Company changed its name to NeuBase Therapeutics, Inc., and will focus on developing next generation gene silencing therapies to treat rare genetic diseases caused by mutant proteins. Shares of the Company’s common stock commenced trading on the Nasdaq Capital Market under the ticker symbol “NBSE” as of market open on July 15, 2019. The Company’s previous ticker symbol was “OHRP”.

Pre-Merger Financing

On July 11, 2019, prior to the completion of the Merger, Legacy NeuBase completed transactions contemplated by certain financing agreements (the “Pre-Merger Financing”) resulting in gross proceeds to Legacy NeuBase of approximately \$9.0 million, consisting of (i) a private placement with certain accredited investors, whereby, among other things, Legacy NeuBase issued to such investors shares of Legacy NeuBase common stock for an aggregate purchase price of approximately \$8.40 million (the “Legacy NeuBase Equity Financing”) and (ii) the conversion of outstanding convertible notes of Legacy NeuBase with an aggregate principal amount of \$600,000 (the “Legacy NeuBase Debt Financing”), which were automatically converted into Legacy NeuBase common stock immediately preceding the closing of the Legacy NeuBase Equity Financing at a conversion price equal to 90% of the purchase price per share of the Legacy NeuBase common stock issued in the Legacy NeuBase Equity Financing.

Post-Merger Financing

On July 16, 2019, the Company completed a private placement with certain accredited investors for the sale by the Company of an aggregate 1,538,462 shares of the Company’s common stock for aggregate gross proceeds of \$5.0 million.

Company Overview

We are a biotechnology company focused on developing the next generation of gene silencing therapies to treat rare genetic diseases caused by mutant proteins. The type of therapies that the Company is developing are termed antisense oligonucleotide therapies (“ASOs”), which 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 that cause disease. The Company is a leader in the discovery and development of the class of ribonucleic acid (“RNA”)-targeted ASO drugs called peptide nucleic acids (“PNAs”). Its proprietary gamma Peptide-nucleic acid AnTisense OLigonucleotide (“PATrOL™”) platform allows for a more efficient discovery of drug product candidates, potentially transforming the treatment paradigm for people affected by rare genetic diseases, with an initial focus on neurological disorders.

The PATrOL™ platform allows for a more efficient discovery of drug product candidates because the peptide backbone is rigid, and once strung together to form a series of backbone subunits, forms a single pre-organized structure. At a more detailed level, each molecule or 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 pre-organized to form only a single stereoisomer. A stereoisomer is a term used in the ASO therapeutics field to mean a string of backbone subunits (usually sugars or modified sugars) with nuclear bases attached that are put together into a specific sequence that matches the target sequence, but 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 hoped-for therapeutic effect. Our PNAs assume only a single conformation with any constellation of nuclear bases added to the backbone or any oligomer length.

In addition to the backbone conformational purity which allows for a more efficient discovery of drug product candidates, NeuBase also has a kit of proprietary bi-facial or 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 (RNA targets which are folded upon themselves) such as hairpins. This allows the company to access regions of the target transcript which may be unique in secondary structure to allow enhanced selectivity for the target (mutant) RNA vs. the normal RNA. Enhanced selectivity for mutant RNAs vs. 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.

In addition to the backbone and modified nuclear bases, the platform toolkit also includes linkers which, when added to both ends of the PNAs, allow cooperative binding at the target RNA to form longer and more tightly bound drugs.

The final component of the platform is a proprietary chemical moiety, which is used to decorate the peptide backbone and allows the PNAs to penetrate both cell membranes and move across the blood-brain barrier when administered systemically.

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

The Company is currently focused on therapeutic areas in which it believes its drugs will provide the greatest benefit with a significant market opportunity and intends to utilize its technology to build out a pipeline of custom designed therapeutics for additional high-value disease targets. The Company is developing several preclinical programs using its PATrOL™ platform, including: NT0100, targeted at Huntington’s Disease, a repeat expansion disorder, and NT0200, targeted at myotonic dystrophy (DM1). Preclinical studies are being conducted to evaluate the PATrOL platform technology and lead program candidates in the areas of pharmacokinetics and pharmacodynamics, with results from those studies expected by year end 2019 and into early 2020. In addition, the emerging pipeline of other assets that target secondary RNA structure and genomic DNA allows a unique market advantage across a variety of rare diseases and oncology targets.

Using its PATrOL™ platform, NeuBase can create antisense oligonucleotides (“ASO”) that have distinct potential 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 only one stereoisomer; the ability to intercalate, open up secondary and tertiary structures and bind within RNA hairpins in a highly selective manner; a proprietary set of engineered nuclear bases which increase selectivity to specific target sequences including secondary and tertiary structures that has been licensed exclusively from Carnegie Mellon University; technology to allow self-assembly of small gamma peptide-nucleic acid (“gamma-PNA”) at the RNA target to increase selectivity which has been licensed exclusively from Carnegie Mellon University; the ability to modulate cell permeability and the ability to pass the blood-brain barrier when administered systemically; the lack of innate or acquired immune responses of similar gamma-PNA’s in pre-clinical models; and potential minimal toxicity based on previous in-vivo studies in rodent models. With these advantages, NeuBase’s PATrOL™ platform-enabled therapies can potentially address a multitude of rare genetic diseases, among other indications.

LEGACY OHR ASSETS AND TECHNOLOGIES

As a result of the Merger, we expect that our going-forward operations will be primarily those of Legacy NeuBase. The assets and technologies described below were the Company's assets and technologies before the consummation of the Merger. Despite the Company's expectation that its primary operations will be those of Legacy NeuBase on a going-forward basis, the Company may choose to pursue further development of the assets and technologies below in the future.

(a) SKS SUSTAINED RELEASE OCULAR DRUG DELIVERY PLATFORM TECHNOLOGY

The SKS sustained release technology was designed to develop best-in-class drug formulations for ocular disease. The technology employs micro fabrication techniques to create nano, micro and macroparticle drug formulations that can provide sustained and predictable release of a therapeutic drug over a three to six month period. The versatility of this delivery technology makes it well suited to potentially deliver hydrophilic or hydrophobic small molecules, as well as proteins with complex structures.

In February 2017, the Company suspended activities at its lab facility in San Diego, CA where research regarding the SKS sustained release technology had been conducted. However, the Company continues to explore strategies and pathways for applications of its sustained release technology and potential avenues to monetize it.

(b) CEP ASSETS

As part of the SKS acquisition, the Company acquired the exclusive rights to an animal model for dry-AMD whereby mice are immunized with a carboxyethylpyrrole ("CEP") which is bound to mouse serum albumin ("MSA") as well as the rights to produce and use CEP for research, clinical, and commercial applications. CEP is produced following the oxidation of docosahexaenoic acid, which is abundant in the photoreceptor outer segments that are phagocytosed by the retinal pigment epithelium ("RPE"). A number of CEP-adducted proteins have been identified in proteomic studies examining the composition of drusen and other subretinal deposits found in the eyes of patients with dry-AMD. Studies have shown that immunization of CEP-MSA can lead to an ophthalmic phenotype very similar to dry-AMD, including deposition of complement in the RPE, thickening of the Bruch's membrane, upregulation of inflammatory cytokines, and immune cell influx into the eye. Upon immunization with CEP, a marked decrease in contrast sensitivity which precedes a loss of visual acuity, was observed, similar to what occurs in many patients with dry AMD. The Company has not yet monetized this technology.

(c) DEPYMED JOINT VENTURE

Ohr also owns various other compounds in earlier stages of development, including the PTP1b inhibitor trodusquemine and related analogs. On February 26, 2014, the Company entered into a Joint Venture Agreement and related agreements with Cold Spring Harbor Laboratory ("CSHL") pursuant to which a joint venture, DepYmed Inc. ("DepYmed"), was formed to further preclinical and clinical development of Ohr's trodusquemine and analogues as PTP1B inhibitors for oncology and rare disease indications. DepYmed licenses research from CSHL and intellectual property from us. Ohr is a passive joint venturer in DepYmed and has no ongoing obligations (monetary or otherwise) to DepYmed.

Corporate and Historical Information

We are a Delaware corporation that was organized on August 4, 2009, as successor to BBM Holdings, Inc. (formerly Prime Resource, Inc., which was organized March 29, 2002 as a Utah corporation) pursuant to a reincorporation merger. On August 4, 2009, we reincorporated in Delaware as Ohr Pharmaceutical, Inc. We completed the acquisition of Legacy NeuBase in July 2019 and, as part of the Merger, the Company was renamed as "NeuBase Therapeutics, Inc."

Reverse Stock Split

On January 18, 2019, following a special meeting of the Company's stockholders, the board of directors of the Company approved a one- for-twenty reverse stock split of the Company's issued and outstanding shares of common stock (the "Reverse Stock Split"). On January 23, 2019, the Company filed with the Secretary of State of the State of Delaware a Certificate of Amendment to its Certificate of Incorporation to effect the Reverse Stock Split. The Company's common stock began trading on a split-adjusted basis when the market opened on February 4, 2019. As a result of the Reverse Stock Split, the outstanding common stock has decreased from 56,466,428 shares of common stock, par value \$0.0001 per share, to 2,829,248 shares of common stock, par value \$0.0001 per share. Unless otherwise noted, impacted amounts and share information included in the financial statements and notes thereto, and elsewhere in this Form 10-Q have been retroactively adjusted for the Reverse Stock Split as if such Reverse Stock Split occurred on the first day of the first period presented. Certain amounts in the financial statements, the notes thereto, and elsewhere in this form 10-Q, may be slightly different than previously reported due to rounding of fractional shares as a result of the Reverse Stock Split.

Liquidity and Sources of Capital

The Company has limited working capital reserves with which to fund its continuing operations. The Company is reliant, at present, upon its capital reserves for ongoing operations and has no revenues.

Net working capital reserves decreased from end of fiscal 2018 to the end of the third quarter in fiscal 2019 by \$2,467,040 (to \$806,396 from \$3,273,436) primarily due to costs incurred from operations. Our quarterly cash burn has decreased significantly compared to prior periods in calendar 2017 and 2018 due to the discontinuation of the squalamine program. We expect our cash burn to increase in future periods in calendar 2019, due to the costs associated with the Merger and ramped up research and development and operations after the closing of the Merger. Alongside the closing of the Merger, NeuBase completed two financings raising gross proceeds of approximately \$14 million, and we believe that our current cash balance will provide sufficient capital to continue operations to the end of fiscal 2020. At present, the Company has no bank line of credit or other fixed source of capital reserves. Should the Company need additional capital in the future, it will be primarily reliant upon private or public placement of its equity or debt securities, or a strategic transaction, for which there can be no warranty or assurance that the Company may be successful in such efforts.

Company Overview

As a result of the Merger, we expect that our going-forward operations will be primarily those of Legacy NeuBase. Accordingly, the results of operations reported for the three and nine months ended June 30, 2019 and 2018, in this Management's Discussion and Analysis are not indicative of the results of operations expected for the remainder of 2019 and future years due to the transition of our historic business operations to primarily those of Legacy NeuBase.

Results of Operations

Three Months Ended June 30, 2019 Compared to the Three Months Ended June 30, 2018

Results of operations for the three months ended June 30, 2019 reflect the following changes from the three months ended June 30, 2018.

	For the Three Months Ended June 30, 2019	For the Three Months Ended June 30, 2018	Change
General and administrative	\$ 888,538	\$ 834,703	\$ 53,835
Research and development	82,751	52,630	30,121
Depreciation and amortization	165,647	279,008	(113,361)
Total Operating Expenses	1,136,936	1,166,341	(29,405)
Operating Loss	(1,136,936)	(1,166,341)	29,405
Other income (expense)	—	610,383	(610,383)
Interest income (expense)	3,961	10,930	(6,969)
Net Loss	\$ (1,132,975)	\$ (545,028)	\$ (587,947)

For the three months ended June 30, 2019, the Company had no revenues and had operating expenses of \$1,136,936. The loss from operations was comprised of \$888,538 in general and administrative expenses, \$82,751 in research and development costs, and \$165,647 in depreciation and amortization.

During the three months ended June 30, 2018, the Company reported no revenues, and had operating expenses of \$1,166,341, which was comprised of \$834,703 in general and administrative expenses, \$52,630 in research and development costs, and \$279,008 in depreciation and amortization.

The operating expenses of the Company decreased quarter-to-quarter by \$29,405. General and administrative expenses increased quarter-to-quarter by \$53,835. The increase is primarily a result of the Merger. Research and development expenses increased quarter-to-quarter by \$30,121. The increase is primarily related to expenses related to maintaining the Company's intellectual property. Depreciation and amortization decreased by \$113,361 quarter-to-quarter. The decrease was related to reduced amortization of long lived intangible assets due to a significant write down of such assets at September 30, 2018.

The net loss for the three months ended June 30, 2019 was \$1,132,975 as compared to \$545,028 for the same period in 2018. Until the Company is able to generate revenues, management expects to continue to incur net losses.

Nine Months Ended June 30, 2019 Compared to the Nine Months Ended June 30, 2018

	For the Nine Months Ended June 30, 2019	For the Nine Months Ended June 30, 2018	Change
General and administrative	\$ 2,463,964	\$ 2,935,919	\$ (471,955)
Research and development	235,289	4,242,307	(4,007,018)
Depreciation and amortization	496,936	842,519	(345,583)
Loss on impairment of goodwill	—	740,912	(740,912)
Gain on settlement of liabilities	—	(1,228,805)	1,228,805
Total Operating Expenses	<u>3,196,189</u>	<u>7,532,852</u>	<u>(4,336,663)</u>
Operating Loss	(3,196,189)	(7,532,852)	4,336,663
Other income (expense)	—	592,584	(592,584)
Interest income (expense)	22,542	59,115	(36,573)
Net Loss	<u>\$ (3,173,647)</u>	<u>\$ (6,881,153)</u>	<u>\$ 3,707,506</u>

For the nine months ended June 30, 2019, the Company had no revenues, and had operating expenses of \$3,196,189. The loss from operations was comprised of \$2,463,964 in general and administrative expenses, \$235,289 in research and development costs, and \$496,936 in depreciation and amortization.

During the nine months ended June 30, 2018, the Company reported no revenues and had operating expenses of \$7,532,852, which was comprised of \$2,935,919 in general and administrative expenses, \$4,242,307 in research and development costs, \$842,519 in depreciation and amortization, \$740,912 in loss on impairment of goodwill, and \$1,228,805 in gain on settlement of liabilities.

The operating expenses of the Company decreased period-to-period by \$4,336,663. The decrease is primarily the result of reduced operations due to the closure of the squalamine program. General and administrative expenses decreased period-to-period by \$471,955. The decrease is primarily a result of a reduction in employee headcount and stock-based compensation. Research and development expenses decreased period-to-period by \$4,007,018. The decrease is primarily related to completion of the MAKO study in wet-AMD in the second fiscal quarter of 2018. Depreciation and amortization decreased by \$345,583 period-to-period. The decrease was related to reduced amortization of long lived intangible assets due to a significant write down of such assets at September 30, 2018.

The net loss for the nine months ended June 30, 2019 was \$3,173,647 as compared to \$6,881,153 for the same period in 2018. Until the Company is able to generate revenues, management expects to continue to incur net losses.

Off-Balance Sheet Arrangements

As of June 30, 2019, the Company 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 Risk.

As a smaller reporting company, the Company is not required to provide the information required by this Item.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

The Company, under the supervision and with the participation of its management, including the Chief Executive Officer and the Chief Financial Officer, evaluated the effectiveness of the design and operation of the Company's "disclosure controls and procedures" (as defined in Rule 13a-15(e) under the Securities Exchange Act of 1934, as amended) as of the end of the period covered by this Report. Based on that evaluation, the Chief Executive Officer and the Chief Financial Officer concluded the Company's disclosure controls were effective at a reasonable assurance level. In designing and evaluating the disclosure controls and procedures, our management, including the Chief Executive Officer and the Chief Financial Officer, recognized that any controls and procedures, no matter how well designed and operated, can only provide reasonable assurance of achieving the desired control objectives, and management was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our disclosure controls and procedures are designed to provide a reasonable level of assurance of reaching our desired disclosure controls objectives.

Changes in Internal Control over Financial Reporting

During the period covered by this Report there has been no change in our internal control over financial reporting that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II OTHER INFORMATION

Item 1. Legal Proceedings.

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, 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 Pharmaceutical, Inc. ("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. Ohr 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 November 13, 2018, plaintiffs filed a motion to strike exhibits appended to the motion to dismiss, which was fully briefed by the parties prior to proceeding on the Defendants' motion to dismiss. On May 10, 2019, the Court entered an order concluding that it is unable to decide the Plaintiffs' motion to strike independently of the Defendants' motion to dismiss and will consider the motions together. Briefing on Defendants' motion to dismiss has now concluded, and the parties are awaiting further ruling of the Court. 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 the Company's common stock.

On May 3, 2018, plaintiff Adele J. Barke, derivatively on behalf of Ohr, commenced an action against certain former directors of the Company, including Michael Ferguson, Orin Hirschman, Thomas M. Riedhammer, June Almenoff and Jason 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. Ohr 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 the Company’s common stock.

Following the filing on March 8, 2019 with the SEC of Ohr’s preliminary joint proxy statement/prospectus, on March 18, 2019, the Gomez Action was filed by an individual shareholder in the United States District Court for the Southern District of New York against Ohr and former members of its board of directors. The plaintiff in the Gomez Action alleges that the preliminary joint proxy/prospectus statement 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. On March 19, 2019, the Barke Action was filed in the United States District Court for the Southern District of New York asserting similar Section 14(a) and Section 20(a) claims against former members of Ohr’s board of directors and additionally naming Legacy NeuBase and Ohr Acquisition Corp., but not Ohr, as defendants. On March 20, 2019, the Wheby Action was filed in the United States District Court for District of Delaware asserting similar claims under Section 14(a) and Section 20(a) and naming as defendants Ohr and former members of its board of directors, Legacy NeuBase, and Ohr Acquisition Corp. The complaint in the Wheby action has not been served on, nor was service waived by, any of the named Defendants in that action. On March 20, 2019, the Lowinger Action was filed in the Court of Chancery of the State of Delaware asserting a breach of fiduciary duty claim against former members of Ohr’s board of directors arising out of the same facts and circumstances regarding certain alleged omissions in the preliminary joint proxy/prospectus statement. On April 4, 2019, the Garaygordobil Action was filed in the United States District Court for the Southern District of New York asserting similar Section 14(a) and Section 20(a) claims against Ohr and its board of directors.

Each of the Gomez, Barke, Garaygordobil, and Lowinger Actions have been dismissed, and on July 12, 2019, Ohr and Legacy NeuBase consummated the Merger. The sole remaining action seeks, among other things, to rescind the Merger or an award of damages, and an award of attorneys’ and experts’ fees and expenses. 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 1A. Risk Factors.

You should carefully consider the following factors which may affect future results of operations. If any of the adverse events described below actually occur, our business, financial condition and operating results could be materially adversely affected and you may lose part or all of the value of your investment. If you choose to invest in our securities, you should be able to bear a complete loss of your investment.

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 the class of deoxy-ribonucleic acid and ribonucleic acid-targeted drugs called peptide nucleic acids, which will not change as a result of the Merger. Since our incorporation, we have focused primarily on the development of preclinical-stage therapeutic candidates. 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. The Company's prior losses, combined with expected future losses, have had and will continue to have an adverse effect on its stockholders' deficit and working capital, and the future success of the Company is subject to significant uncertainty.

For the foreseeable future, the Company expects to continue to incur losses, which will increase significantly from recent historical levels as the Company expands its drug development activities, seeks regulatory approvals for its product candidates and begins 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 the Company succeeds in developing and commercializing one or more product candidates, the Company may never become profitable.

The approach the Company is taking to discover and develop nucleic acid therapeutics is novel and may never lead to marketable products.

The Company has concentrated its efforts and research and development activities on nucleic acid therapeutics and its synthetic chemistry drug discovery and development platform comprised of peptide nucleic acids with natural and engineered nucleotides. The Company's future success depends on the successful development and manufacturing of such therapeutics and the effectiveness of its platform. The scientific discoveries that form the basis for the Company's efforts to discover and develop new drugs, including the Company's 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 is limited. Skepticism as to the feasibility of developing nucleic acid therapeutics 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.

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 the Company is 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 the Company's nucleic acid product candidates prove to be ineffective, unsafe or commercially unviable, the Company's entire platform and pipeline would have little, if any, value, which would substantially harm the Company's business, financial condition, results of operations and prospects.

In addition, the Company's approach, which focuses on using nucleic acid therapeutics for drug development, as opposed to multiple or other, more advanced proven technologies, may expose the Company to additional financial risks and make it more difficult to raise additional capital if the Company is not successful in developing a nucleic acid therapeutic that achieves proof of concept in animal models, desired tissue distribution, selectivity for the target, and/or regulatory approval. Because the Company's programs are all in the research or preclinical stage, the Company has not yet been able to assess safety in humans, and there may be long-term effects from treatment with any product candidates that the Company develops that the Company cannot predict at this time. Any product candidates the Company may develop will act at the level of DNA or 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 the Company's product candidates in animal models may not be predictive of the results it observes in human clinical trials of its product candidates for either safety or efficacy. Also, animal models may not exist for some of the diseases the Company chooses to pursue in its programs. As a result of these factors, it is more difficult for the Company to predict the time and cost of product candidate development, and the Company cannot predict whether the application of its 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 the Company experiences in the future related to its gene silencing technology or any of its research programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. Any of these factors may prevent the Company from completing its preclinical studies or any clinical trials that it may initiate or from commercializing any product candidates the Company may develop on a timely or profitable basis, if at all.

The Company is highly dependent on the success of its initial product candidates targeting rare genetic diseases, and the Company cannot be certain that any of them will receive regulatory approval or be commercialized.

The Company has spent time, money and effort on the licensing and development of its core asset: the PATrOL™ platform. To date, the Company has not submitted an IND to the FDA, and no clinical trials have commenced with any of the Company's product candidates. All of the Company's 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. The Company's drug development efforts may not lead to commercial drugs, either because the Company's product candidates are not deemed safe and effective, because of competitive or market forces, intellectual property issues or because the Company has inadequate financial or other resources to advance the Company's product candidates through the clinical development and approval processes. If any of the Company's product candidates fail to demonstrate safety or efficacy at any time or during any phase of development, the Company would experience potentially significant delays in, or be required to abandon, development of the product candidate.

The Company does not anticipate that any of its 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 the Company ultimately receives regulatory approval for any of these product candidates, the Company or its 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 the Company's product candidates may also be limited by the prevalence and severity of any adverse side effects. If the Company fails to commercialize one or more of its current product candidates, the Company may be unable to generate sufficient revenues to attain or maintain profitability, and the Company's financial condition may decline.

If development of the Company's product candidates does not produce favorable results, the Company and its collaborators, if any, may be unable to commercialize these products.

To receive regulatory approval for the commercialization of the Company's use of the PATrOL™ platform, or any other product candidates that the Company 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 3 clinical trials, which the Company's 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. The Company may experience numerous unforeseen events during, or as a result of, the development process that could delay or prevent commercialization of the Company's 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 the Company anticipates;
- clinical trials may produce negative or inconclusive results;
- costs of development may be greater than the Company anticipates;
- the potential market advantages of the PATrOL™-enabled drugs may not materialize and thus would confer no benefits to patients over other products that may emerge;
- the Company's product candidates 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 the Company's 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
- the Company may face delays in obtaining regulatory approvals to commence one or more clinical trials.

Additionally, because the Company's technology potentially involves gene silencing via genome binding and/or editing across multiple cell and tissue types, the Company is 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 the Company may need to adopt and support such an observation period for its 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, the Company has licensed or acquired virtually all of the intellectual property related to its product candidates from Carnegie Mellon University. All preclinical studies and other analyses performed to date with respect to the Company's product candidates have been conducted by their original owners or collaborators. Therefore, as a company, the Company has limited experience in conducting preclinical trials for its product candidates. Since the Company's experience with its product candidates is limited, the Company will need to train its existing personnel or hire additional personnel in order to successfully administer and manage its preclinical studies and clinical trials as anticipated, which may result in delays in completing such anticipated preclinical trials and clinical studies.

The Company currently does not have strategic collaborations in place for clinical development of any of its current product candidates. Therefore, in the future, the Company or any potential future collaborative partner will be responsible for establishing the targeted endpoints and goals for development of its product candidates. These targeted endpoints and goals may be inadequate to demonstrate the safety and efficacy levels required for regulatory approvals. Even if the Company believes data collected during the development of its 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 the Company or its collaborators. The Company's failure to adequately demonstrate the safety and efficacy of the Company's product candidates would prevent the Company's receipt of regulatory approval, and such failure would ultimately prevent the potential commercialization of these product candidates.

Since the Company does not currently possess the resources necessary to independently develop and commercialize its product candidates or any other product candidates that the Company may develop, the Company 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 the Company's strategic plan. The Company's 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 the Company's business, financial condition and results of operations.

The Company expects to continue to incur significant research and development expenses, which may make it difficult for the Company to attain profitability.

The Company expects to expend substantial funds in research and development, including preclinical studies and clinical trials of its product candidates, and to manufacture and market any product candidates in the event they are approved for commercial sale. The Company 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 the Company's headcount would dramatically increase the Company's costs in the near and long-term.

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

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

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

The Company's resource allocation decisions may cause it to fail to capitalize on viable commercial products or profitable market opportunities. The Company's spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable product candidates. If the Company does not accurately evaluate the commercial potential or target market for a particular product candidate, the Company 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 the Company to retain sole development and commercialization rights to the product candidate.

Given the Company's lack of current cash flow, the Company will need to raise additional capital; however, it may be unavailable to the Company or, even if capital is obtained, may cause dilution or place significant restrictions on the Company's ability to operate its business.

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

There can be no assurance that the Company 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, the Company may be required to delay, limit or eliminate the development of business opportunities, and its ability to achieve its business objectives, its competitiveness, and its business, financial condition and results of operations may be materially adversely affected. In addition, the Company may be required to grant rights to develop and market product candidates that it would otherwise prefer to develop and market itself. The Company's inability to fund its business could lead to the loss of your investment.

The Company's future capital requirements will depend on many factors, including, but not limited to:

- the scope, rate of progress, results and cost of its preclinical studies, clinical trials and other related activities;
- its 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 its 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 its product candidates;
- the cost of commercialization activities if any of its 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 its product candidates, should any of its 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 the Company's management from their day-to-day activities, which may adversely affect its ability to develop and commercialize its product candidates. Moreover, if the Company raises additional capital by issuing equity securities, the percentage ownership of its existing stockholders may be reduced, and accordingly these stockholders may experience substantial dilution. The Company may also issue equity securities that provide for rights, preferences and privileges senior to those of its common stock. Given the Company's 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 the Company's stockholders. Furthermore, the incurrence of indebtedness would result in increased fixed payment obligations and the Company may be required to agree to certain restrictive covenants, such as limitations on its ability to incur additional debt and other operating restrictions that could adversely impact the Company's ability to conduct its business. The Company could also be required to seek funds through arrangements with collaborators or otherwise at an earlier stage than otherwise would be desirable and the Company may be required to relinquish rights to some of its product candidates or otherwise agree to terms unfavorable to the Company, any of which may have a material adverse effect on its business, operating results and prospects.

The Company's efforts to discover product candidates beyond the Company's current product candidates may not succeed, and any product candidates the Company recommends for clinical development may not actually begin clinical trials.

The Company intends to use its technology, including its licensed technology, knowledge and expertise to develop novel drugs to address some of the world's most devastating and costly central nervous system and other disorders, including orphan genetic and oncology indications. The Company intends to expand its existing pipeline of core assets by advancing drug compounds from current ongoing discovery programs into clinical development. However, the process of researching and discovering drug compounds is expensive, time-consuming and unpredictable. Data from the Company's current preclinical programs may not support the clinical development of its lead compounds or other compounds from these programs, and the Company may not identify any additional drug compounds suitable for recommendation for clinical development. Moreover, any drug compounds the Company recommends 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 the Company's ability to maintain or expand the Company's clinical development pipeline. The Company's ability to identify new drug compounds and advance them into clinical development also depends upon the Company's ability to fund its research and development operations, and the Company cannot be certain that additional funding will be available on acceptable terms, or at all.

The pharmaceutical market is intensely competitive. If the Company is unable to compete effectively with existing drugs, new treatment methods and new technologies, the Company may be unable to commercialize successfully any drugs that the Company develops.

The pharmaceutical market is intensely competitive and rapidly changing. Many large pharmaceutical and biotechnology companies, academic institutions, governmental agencies and other public and private research organizations are pursuing the development of novel drugs for the same diseases that the Company is targeting or expect to target. Many of the Company's competitors have:

- much greater financial, technical and human resources than the Company has 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 the Company's target markets with leading companies and research institutions.

The Company 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 the Company may develop drugs. The Company also expects to face competition from new drugs that enter the market. The Company believes 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 the Company may try to develop drugs. These drugs may be more effective, safer, less expensive, or marketed and sold more effectively, than any products the Company develops. It is possible that the potential advantages of PATrOL™-derived therapies (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.

The Company's competitors may develop or commercialize products with significant advantages over any products the Company is able to develop and commercialize based on many different factors, including:

- the safety and effectiveness of the Company's products relative to alternative therapies, if any;
- the ease with which the Company's products can be administered and the extent to which patients accept relatively new routes of administration;
- the timing and scope of regulatory approvals for these products;
- the availability and cost of manufacturing, marketing and sales capabilities;
- price;
- reimbursement coverage; and
- patent position.

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

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

If the Company's potential future collaborations do not result in the successful discovery, development and commercialization of products or if one of the Company's collaborators terminates its agreement with the Company, the Company may not receive any future research funding or milestone or royalty payments under the collaboration. If the Company does not receive the funding it expects under these agreements, the Company's development of its technology and product candidates could be delayed and the Company may need additional resources to develop product candidates and its 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 would adversely affect the Company's business, financial condition and results of operations.

The Company, or any future collaborators, may not be able to obtain orphan drug designation or orphan drug exclusivity for the Company's 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 the Company to obtain financial incentives, such as an extended period of exclusivity during which only the Company is allowed to market the orphan drug for the orphan indications that the Company is developing. While the Company may seek orphan drug designation from the FDA for any of its product candidates, the Company, or any future collaborators, may not be granted orphan drug designations for its product candidates in the U.S. or in other jurisdictions.

Even if the Company or any future collaborators obtain orphan drug designation for a product candidate, the Company 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 the Company 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.

The Company is subject to a multitude of manufacturing risks, any of which could substantially increase the Company's costs and limit supply of its product candidates.

The process of manufacturing the Company's product candidates is complex, highly regulated, and subject to several risks. For example, the process of manufacturing the Company's 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 the Company's product candidates could result in reduced production yields, product defects, and other supply disruptions. If microbial, viral, or other contaminations are discovered in the Company's product candidates or in the manufacturing facilities in which its 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 its product candidates are made could be adversely affected by equipment failures, labor shortages, natural disasters, power failures and numerous other factors. For instance, the Company's therapeutic molecules are complex and comprised of both peptides and nucleic acids, and it may be difficult or impossible to find GLP and GMP-grade manufacturers, manufacturing may be cost prohibitive and manufacturing may not be available to fulfill regulatory requirements.

In addition, any adverse developments affecting manufacturing operations for the Company's product candidates may result in shipment delays, inventory shortages, lot failures, withdrawals or recalls, or other interruptions in the supply of the Company's product candidates. The Company 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.

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

The Company has the capability internally to manufacture small quantities of its drugs for preclinical studies. However, the Company does not currently have, nor does the Company plan to acquire, the infrastructure or capability internally to manufacture the Company's clinical drug supplies for use in its clinical trials, and the Company lacks the resources and the capability to manufacture any of the Company's product candidates on a clinical or commercial scale. The Company relies on its manufacturers to purchase from third-party suppliers the materials necessary to produce the Company's product candidates for the Company's clinical trials. There are a limited number of suppliers for raw materials that the Company uses to manufacture its 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 the Company's product candidates for its clinical trials, and, if approved, ultimately for commercial sale. The Company does not have any control over the process or timing of the acquisition of these raw materials by the Company's 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 the Company's clinical trials, product testing and potential regulatory approval of the Company's product candidates, which could harm the Company's business, financial condition and results of operations.

If the Company is unable to develop its own commercial organization or enter into agreements with third parties to sell and market the Company's product candidates, the Company may be unable to generate significant revenues.

The Company does not have a sales and marketing organization, and the Company has no experience as a company in the sales, marketing and distribution of pharmaceutical products. If any of the Company's product candidates are approved for commercialization, the Company may be required to develop its 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 the Company's other product candidates is expensive and time consuming and could delay any product launch. The Company 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 the Company does establish may not be capable of generating sufficient demand for the Company's product candidates. To the extent that the Company enters into arrangements with collaborators or other third parties to perform sales and marketing services, the Company's product revenues are likely to be lower than if the Company marketed and sold its product candidates independently. If the Company is unable to establish adequate sales and marketing capabilities, independently or with others, the Company may not be able to generate significant revenues and may not become profitable.

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

Even if the Company's product candidates obtain regulatory approval, the Company's 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 the Company's approved product candidates will depend on a number of factors, including:

- the effectiveness of the Company's approved product candidates as compared to currently available products;
- patient willingness to adopt the Company's approved product candidates in place of current therapies;
- the Company's 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 the Company's product candidates and target markets;
- effectiveness of the Company's or its partners' sales and marketing strategy;
- the Company's ability to obtain sufficient third-party coverage or reimbursement; and
- potential product liability claims.

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

If the Company fails to obtain and sustain an adequate level of reimbursement for its potential products by third-party payors, potential future sales would be materially adversely affected.

There will be no viable commercial market for the Company's product candidates, if approved, without reimbursement from third-party payors. Reimbursement policies may be affected by future healthcare reform measures. The Company cannot be certain that reimbursement will be available for its current product candidates or any other product candidate the Company may develop. Additionally, even if there is a viable commercial market, if the level of reimbursement is below the Company's expectations, the Company's 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 the Company might establish for products, which could result in product revenues being lower than anticipated. The Company believes its drugs will be priced significantly higher than existing generic drugs and consistent with current branded drugs. If the Company is unable to show a significant benefit relative to existing generic drugs, Medicare, Medicaid and private payors may not be willing to provide reimbursement for the Company's drugs, which would significantly reduce the likelihood of the Company's products gaining market acceptance.

The Company expects that private insurers will consider the efficacy, cost-effectiveness, safety and tolerability of the Company's potential products in determining whether to approve reimbursement for such products and at what level. Obtaining these approvals can be a time consuming and expensive process. The Company's business, financial condition and results of operations would be materially adversely affected if the Company does not receive approval for reimbursement of its potential products 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. The Company's 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, the Company's product candidates or other potential products.

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 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, the Company may be required to conduct a clinical trial that compares the cost-effectiveness of its products to other available therapies.

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

The Company is exposed to product liability, non-clinical and clinical liability risks which could place a substantial financial burden upon the Company, should lawsuits be filed against the Company.

The Company's business exposes it 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 the Company's anticipated clinical trials of pharmaceutical products and the subsequent sale of these products by the Company or its potential collaborators may cause the Company to bear a portion of or all product liability risks. A successful liability claim or series of claims brought against the Company could have a material adverse effect on the Company's business, financial condition and results of operations.

Because the Company does not currently have any clinical trials ongoing, it does not currently carry product liability insurance. The Company anticipates obtaining such insurance upon initiation of its clinical development activities; however, the Company 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 the Company could adversely affect the Company's results of operations and business if judgments therewith exceed the Company's insurance coverage.

If the Company fails to retain current members of its management, or to attract and keep additional key personnel, the Company may be unable to successfully develop or commercialize the Company's product candidates.

The Company's success depends on the Company's continued ability to attract, retain and motivate highly qualified management and scientific personnel. As of August 9, 2019, the Company had six employees. The Company will rely primarily on outsourcing research, development and clinical trial activities, and manufacturing operations, as well as other functions critical to its business. The Company believes this approach enhances its ability to focus on its core product opportunities, allocate resources efficiently to different projects and allocate internal resources more effectively. The Company has filled several key open positions and is currently recruiting for a few remaining positions. However, competition for qualified personnel is intense. The Company may not be successful in attracting qualified personnel to fulfill the Company's current or future needs. In the event the Company is unable to fill critical open employment positions, the Company may need to delay its operational activities and goals, including the development of its product candidates, and may have difficulty in meeting its obligations as a public company. The Company does not maintain "key person" insurance on any of its employees.

In addition, competitors and others are likely in the future to attempt to recruit the Company's employees. The loss of the services of any of the Company's 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 the Company's 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 the Company's business objectives.

From time to time, the Company's 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 the Company's employees and may have commitments to, or consulting or advisory contracts with, other entities that may limit their availability to the Company. In addition, the Company's scientific advisors may have arrangements with other companies to assist those companies in developing products or technologies that may compete with the Company's.

The Company will need to increase the size of the Company's organization and may not successfully manage the Company's growth.

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

Because the Company's 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.

Dr. Dietrich Stephan, the Company's 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 the Company, 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 the Company, on the other hand.

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

The majority of the Company's current executive officers do not have experience in managing and operating a public company, which could have an adverse effect on their 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 the Company to fines or regulatory actions, which may materially adversely affect the Company's business, financial condition and results of operations. Further, since the Company's current executive officers do not have experience managing and operating a public company, the Company may need to dedicate additional time and resources to comply with legally mandated corporate governance policies relative to the Company's competitors whose management teams have more public company experience.

The Company relies significantly on information technology and any failure, inadequacy, interruption or security lapse of that technology, including any cybersecurity incidents, could harm the Company's ability to operate the Company's business effectively.

Despite the implementation of security measures, the Company's internal computer systems and those of third parties with which the Company contracts 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 the Company's operations, and could result in a material disruption of the Company's drug development and preclinical and clinical activities and business operations, in addition to possibly requiring substantial expenditures of resources to remedy. The loss of drug development or clinical trial data could result in delays in the Company's regulatory approval efforts and significantly increase the Company's 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, the Company's data or applications, or inappropriate disclosure of confidential or proprietary information, the Company could incur liability and its development programs and the development of its product candidates could be delayed.

The Company's employees and consultants may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

The Company is exposed to the risk of employee or consultant fraud or other misconduct. Misconduct by the Company's employees or consultants 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 the Company. 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 and consultant misconduct also could involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to the Company's reputation. It is not always possible to identify and deter such misconduct, and the precautions the Company takes to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting the Company 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 the Company, and the Company is not successful in defending itself or asserting the Company's rights, those actions could have a material adverse effect on the Company's business, financial condition and results of operations, and result in the imposition of significant fines or other sanctions against the Company.

Business disruptions such as natural disasters could seriously harm the Company's future revenues and financial condition and increase its costs and expenses.

The Company and its suppliers may experience a disruption in 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 the Company's headquarters or facilities or the facilities of the Company's manufacturers or suppliers, which could have a material and adverse effect on the Company's 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 the Company, its employees, facilities, partners and suppliers, which could have a material adverse effect on the Company's business, financial condition and results of operations.

The Company may engage in strategic transactions that could impact its liquidity, increase its expenses and present significant distractions to its management.

From time to time, the Company may consider strategic transactions, such as acquisitions of companies, asset purchases and out-licensing or in-licensing of products, product candidates or technologies. Additional potential transactions that the Company may consider include a variety of different business arrangements, including spin-offs, strategic partnerships, joint ventures, restructurings, divestitures, business combinations and investments. Any such transaction may require the Company to incur non-recurring or other charges, may increase the Company's near- and long-term expenditures and may pose significant integration challenges or disrupt the Company's management or business, which could adversely affect the Company's 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 the Company's business and diversion of the Company's 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 the Company's 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 the Company will undertake or successfully complete any transactions of the nature described above, any transactions that the Company does complete may be subject to the foregoing or other risks, and could have a material adverse effect on the Company's business, financial condition and results of operations.

The estimates and judgments the Company makes, or the assumptions on which the Company relies, in preparing its financial statements could prove inaccurate.

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

We may be unable to continue the development of, or sell or otherwise monetize, the assets, technologies and operations 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.

Although at present the Company intends to primarily conduct the operations of Legacy NeuBase on a going-forward basis, the Company's management has not fully evaluated whether to further pursue the operations of the Company as conducted prior to completion of the Merger. The Company may further pursue such opportunities or may explore strategic alternatives for the assets associated with our pre-Merger activities, including a sale of such assets. There can be no assurance, however, that we will be successful at such efforts or sell or other monetize such assets on acceptable terms, if at all. As a result of the proposed operations of the Company, we may be required to take write-offs or write-downs, and impairment or other charges associated with classifying such 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 the Company's Intellectual Property

The Company may not be successful in obtaining or maintaining necessary rights to its product candidates through acquisitions and in-licenses.

Because several of the Company's programs currently require the use of proprietary rights held by third parties, the growth of the Company's business will likely depend in part on the Company's ability to maintain and exploit these proprietary rights. In addition, the Company may need to acquire or in-license additional intellectual property in the future. The Company may be unable to acquire or in-license any compositions, methods of use, processes or other intellectual property rights from third parties that the Company identifies as necessary for its product candidates. The Company faces 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 the Company due to their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive the Company to be a competitor may be unwilling to assign or license intellectual property rights to the Company. The Company 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 the Company's investment, and the Company may not be able to market products or perform research and development or other activities covered by these patents.

The Company may enter into collaboration agreements with U.S. and foreign academic institutions to accelerate development of the Company's 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, the Company may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to the Company. If the Company is unable to license rights from a collaborating institution, the institution may offer the intellectual property rights to other parties, potentially blocking the Company's ability to pursue its desired program.

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

If the Company fails to comply with its obligations in the agreements under which the Company in-licenses intellectual property and other rights from third parties or otherwise experiences disruptions to the Company's business relationships with the Company's licensors, the Company could lose intellectual property rights that are important to its business.

The Company's license agreement with Carnegie Mellon University (the "CMU License Agreement"), as the licensor (the "Licensor"), is important to the Company's business, and the Company expects to enter into additional license agreements in the future. The CMU License Agreement imposes, and the Company expects that future license agreements will impose, various royalties, sublicensing fees and other obligations on the Company. If the Company fails to comply with the Company's obligations under these agreements, or if the Company files for bankruptcy, the Company may be required to make certain payments to the Licensor, the Company may lose the exclusivity of its license, or the Licensor may have the right to terminate the license, in which event the Company 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 the Company's 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 the Company does not pay amounts when due and within the applicable cure periods or (ii) if the Company files or has filed against the Company 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 the Company by the Licensor will terminate immediately.

In some cases, patent prosecution of the Company's licensed technology may be controlled solely by the licensor. If the Company's licensor fails to obtain and maintain patent or other protection for the proprietary intellectual property the Company in-licenses, then the Company could lose its rights to the intellectual property or its exclusivity with respect to those rights, and its competitors could market competing products using the intellectual property. In certain cases, the Company may control the prosecution of patents resulting from licensed technology. In the event the Company breaches any of the Company's obligations related to such prosecution, the Company may incur significant liability to the Company's licensing partners. Licensing of intellectual property is of critical importance to the Company's 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 the Company's 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;
- the Company's 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 the Company's licensors and the Company and the Company's collaborators; and
- the priority of invention of patented technology.

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

The Company may be required to pay royalties and sublicensing fees pursuant to the CMU License Agreement, which could adversely affect the overall profitability for the Company of any products that the Company may seek to commercialize.

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

The Company may not be able to protect its proprietary or licensed technology in the marketplace.

The Company depends on the Company's ability to protect its proprietary or licensed technology. The Company relies 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. The Company's success depends in large part on the Company's 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 the Company's proprietary or licensed technology and products. The Company currently in-licenses some of the Company's intellectual property rights to develop the Company's product candidates and may in-license additional intellectual property rights in the future. The Company cannot be certain that patent enforcement activities by its 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. The Company also cannot be certain that its current or future licensors will allocate sufficient resources or prioritize their or the Company's enforcement of such patents. Even if the Company is not a party to these legal actions, an adverse outcome could prevent the Company from continuing to license intellectual property that the Company may need to operate its business, which would have a material adverse effect on its business, financial condition and results of operations.

The Company believes it will be able to obtain, through prosecution of patent applications covering the Company's owned technology and technology licensed from others, adequate patent protection for the Company's proprietary drug technology, including those related to the Company's in-licensed intellectual property. If the Company is compelled to spend significant time and money protecting or enforcing its licensed patents and future patents the Company may own, designing around patents held by others or licensing or acquiring, potentially for large fees, patents or other proprietary rights held by others, the Company's business, financial condition and results of operations may be materially and adversely affected. If the Company is unable to effectively protect the intellectual property that the Company owns or in-licenses, other companies may be able to offer the same or similar products for sale, which could materially adversely affect the Company's business, financial condition and results of operations. The patents of others from whom the Company may license technology, and any future patents the Company may own, may be challenged, narrowed, invalidated or circumvented, which could limit the Company's ability to stop competitors from marketing the same or similar products or limit the length of term of patent protection that the Company may have for its 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 the Company's 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 the Company's in-licensed patents or patent applications the Company may file in the future, the Company's competitors might be able to use its technologies, which would have a material adverse effect on the Company's 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 the Company's licensed or owned intellectual property or create uncertainty. In addition, publication of information related to the Company's current product candidates and potential products may prevent the Company 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 the Company currently licenses and patents that the Company may own or license in the future do not necessarily ensure the protection of the Company's 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 the Company's 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 the Company may obtain or license in the future may not prevent generic entry into the market for the Company's product candidates;
- the Company, or third parties from whom the Company in-licenses 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 the Company is not aware that may affect the validity or enforceability of a patent claim;
- there may be prior art of which the Company is aware, which the Company does 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 the Company's 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 the Company's licensed patents or any future patents the Company may own that adversely affects the scope of the Company's patent rights;
- a court could determine that a competitor's technology or product does not infringe the Company's licensed patents or any future patents the Company 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 the Company encounters delays in the Company's development or clinical trials, the period of time during which the Company could market its potential products under patent protection would be reduced.

The Company's competitors may be able to circumvent its licensed patents or future patents the Company may own by developing similar or alternative technologies or products in a non-infringing manner. The Company's competitors may seek to market generic versions of any approved products by submitting abbreviated new drug applications to the FDA in which the Company's competitors claim that the Company's licensed patents or any future patents the Company may own are invalid, unenforceable or not infringing. Alternatively, the Company's competitors may seek approval to market their own products similar to or otherwise competitive with the Company's products. In these circumstances, the Company may need to defend or assert the Company's licensed patents or any future patents the Company 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 the Company's licensed patents or any future patents the Company may own invalid or unenforceable. The Company may also fail to identify patentable aspects of its research and development before it is too late to obtain patent protection. Even if the Company owns or in-licenses valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve the Company's 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 the Company's licensed patents or any future patents the Company 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 the Company's ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of the Company's 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.

The Company may infringe the intellectual property rights of others, which may prevent or delay its drug development efforts and prevent the Company from commercializing or increase the costs of commercializing the Company's products.

The Company's commercial success depends significantly on the Company's ability to operate without infringing the patents and other intellectual property rights of third parties. For example, there could be issued patents of which the Company is not aware that the Company's current or potential future product candidates infringe. There also could be patents that the Company believes the Company does not infringe, but that the Company may ultimately be found to infringe. The Company has licensed intellectual property from Carnegie Mellon University 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 the Company's own intellectual property.

Furthermore, because the nucleic acid therapeutics intellectual property landscape is still evolving and the Company's product candidates have not been through clinical trials or commercialized, it is difficult to conclusively assess the Company's 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. The Company is 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 the Company's programs. It is possible that at the time that the Company commercializes its products these third-party patent portfolios may include issued patent claims that cover the Company's products or critical features of their production or use. The Company's competitive position may suffer if patents issued to third parties or other third party intellectual property rights cover, or may be alleged to cover, the Company's products or elements thereof, or methods of manufacture or use relevant to the Company's development plans. In such cases, the Company may not be in a position to develop or commercialize product candidates unless the Company successfully pursues 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 the Company is unaware that may later result in issued patents that the Company's product candidates or potential products infringe. For example, pending applications may exist that claim or can be amended to claim subject matter that the Company's 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 the Company's product candidates.

Third parties may assert that the Company is employing their proprietary technology without authorization and may sue the Company for patent or other intellectual property infringement. These lawsuits are costly and could adversely affect the Company's business, financial condition and results of operations and divert the attention of managerial and scientific personnel. If the Company is sued for patent infringement, the Company would need to demonstrate that its product candidates, potential products or methods either do not infringe the claims of the relevant patent or that the patent claims are invalid, and the Company 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 the Company is successful in these proceedings, the Company may incur substantial costs and the time and attention of the Company's management and scientific personnel could be diverted in pursuing these proceedings, which could have a material adverse effect on the Company. In addition, the Company 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 the Company's products or their use, the holders of any of these patents may be able to block the Company's ability to commercialize its products unless it acquires or obtains a license under the applicable patents or until the patents expire.

The Company 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 the Company's products or lead to prohibition of the manufacture or sale of products by the Company. Even if the Company is able to obtain a license, it may be non-exclusive, thereby giving the Company's competitors access to the same technologies licensed to the Company. The Company could be forced, including by court order, to cease commercializing the infringing technology or product. In addition, in any such proceeding or litigation, the Company could be found liable for monetary damages, including treble damages and attorneys' fees, if the Company is found to have willfully infringed a patent. A finding of infringement could prevent the Company from commercializing its product candidates or force the Company to cease some of its business operations, which could materially and adversely affect the Company's business, financial condition and results of operations. Any claims by third parties that the Company has misappropriated their confidential information or trade secrets could have a similar material and adverse effect on the Company's 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 the Company's ability to raise the funds necessary to continue the Company's operations.

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

The Company may be required to initiate litigation to enforce or defend its licensed and owned intellectual property. Lawsuits to protect the Company's 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 the Company's 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 the Company receives 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 the Company's confidential information could be compromised by disclosure during litigation. Moreover, there can be no assurance that the Company 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 the Company asserts against a perceived infringer could provoke these parties to assert counterclaims against the Company alleging that the Company has infringed their patents. Some of the Company's competitors may be able to sustain the costs of such litigation or proceedings more effectively than the Company 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 the Company's ability to compete in the marketplace.

In addition, the Company's licensed patents and patent applications, and patents and patent applications that the Company 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 the Company's licensed patents and patent applications and patents and patent applications that the Company 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 the Company's management and scientific personnel's time and attention.

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

As is the case with other pharmaceutical companies, the Company's 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 the Company's licensed and future patent applications and the enforcement or defense of the Company's licensed and future patents, all of which could have a material adverse effect on the Company's 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. The Company currently is not aware of an immediate impact of this decision on the Company's patents or patent applications which contain modifications that the Company believes are not found in nature. However, this decision has yet to be clearly interpreted by courts and by the USPTO. The Company cannot make assurances that the interpretations of this decision or subsequent rulings will not adversely impact the Company's patents or patent applications. In addition to increasing uncertainty with regard to the Company's 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 the Company's ability to obtain new patents or to enforce patents that the Company might obtain in the future.

The Company may not be able to protect its intellectual property rights throughout the world.

Filing, prosecuting and defending patents on product candidates throughout the world would be prohibitively expensive. Competitors may use the Company's licensed and owned technologies in jurisdictions where the Company has not licensed or obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where the Company 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 the Company's products in jurisdictions where the Company does 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 the Company to stop the infringement of the Company's licensed patents and future patents the Company may own, or marketing of competing products in violation of the Company's 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, the Company may encounter significant problems in protecting and defending its 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 the Company's vulnerability regarding unauthorized disclosure or use of its intellectual property and undermine its competitive position. Proceedings to enforce the Company's future patent rights, if any, in foreign jurisdictions could result in substantial cost and divert its efforts and attention from other aspects of the Company's business.

The Company may be unable to adequately prevent disclosure of trade secrets and other proprietary information.

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

The Company may be subject to claims that the Company's employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties.

The Company expects to employ individuals who were previously employed at other pharmaceutical companies. Although the Company has no knowledge of any such claims against the Company, the Company may be subject to claims that it or its employees, consultants or independent contractors have inadvertently or otherwise used or disclosed confidential information of the Company's 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 the Company is successful, litigation could result in substantial cost and be a distraction to the Company's management and other employees.

The Company may be subject to claims challenging the inventorship of its licensed patents, any future patents the Company may own and other intellectual property.

Although the Company is not currently experiencing any claims challenging the inventorship of its licensed patents or the Company's licensed or owned intellectual property, the Company may in the future be subject to claims that former employees, collaborators or other third parties have an interest in the Company's licensed patents or other licensed or owned intellectual property as an inventor or co-inventor. For example, the Company may have inventorship disputes arise from conflicting obligations of consultants or others who are involved in developing the Company's product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship. If the Company fails in defending any such claims, in addition to paying monetary damages, the Company 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 the Company's business, financial condition and results of operations. Even if the Company is successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

If the Company does not obtain additional protection under the Hatch-Waxman Amendments and similar foreign legislation extending the terms of the Company's licensed patents and any future patents the Company may own, the Company's 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 the Company's product candidates, one or more of its licensed U.S. patents or future U.S. patents that the Company 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 an 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. The Company 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 the Company requests. If the Company is unable to obtain patent term extension or restoration or the term of any such extension is less than the Company requests, the period during which the Company will have the right to exclusively market its product will be shortened and the Company's competitors may obtain earlier approval of competing products, and the Company's ability to generate revenues could be materially adversely affected.

Risks Related to Government Regulation of the Company

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

The Company is very early in its development efforts, and all of its product candidates are still in preclinical development. The Company has invested substantially all of its efforts and financial resources in the identification and preclinical development of ASOs, including the development program for the treatment of Huntington's Disease. The Company's 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 its product candidates, which may never occur. The Company currently generates no revenue from sales of any products, and it may never be able to develop or commercialize a marketable product. In addition, certain of the Company's 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 the Company may commercialize its products. The success of its product candidates will depend on several factors, including the following:

- successful completion of preclinical studies;
- approval of INDs for the Company's 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 the Company's 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 the Company's product candidates;
- launching commercial sales of the Company's 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 the Company does not achieve one or more of these factors in a timely manner or at all, it could experience significant delays or an inability to successfully commercialize its product candidates, which would materially harm its business. If the Company does not receive regulatory approvals for its product candidates, the Company may not be able to continue its operations.

Furthermore, the FDA has relatively limited experience with nucleic acid therapeutics, which may increase the complexity, uncertainty and length of the regulatory review process for the Company's product candidates. To date, the FDA has approved few nucleic acid therapeutics for marketing and commercialization, and the FDA and its 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 the Company may submit. Moreover, the FDA may respond to these submissions by defining requirements the Company may not have anticipated. Such responses could lead to significant delays in the development of the Company's product candidates. In addition, because there may be approved treatments for some of the diseases for which the Company may seek approval, in order to receive regulatory approval, the Company may need to demonstrate through clinical trials that the product candidates the Company develops 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, the Company 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 the Company's product candidates are still in the preclinical stage, and their risk of failure is high. Before the Company can commence clinical trials for a product candidate, it must complete extensive preclinical testing and studies that support the Company's planned INDs in the U.S., or similar applications in other jurisdictions. The Company cannot be certain of the timely completion or outcome of its preclinical testing and studies and cannot predict if the FDA or other regulatory authorities will accept the Company's proposed clinical programs or if the outcome of the Company's preclinical testing and studies will ultimately support the further development of its programs. It is also impossible to predict when or if any of the Company's 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 the Company's product candidates, the Company must demonstrate through extensive preclinical studies and clinical trials that its product candidates are safe and effective in humans for use in each target indication. To date, the Company has 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. The Company's preclinical programs may experience delays or may never advance to clinical trials, which would adversely affect its ability to obtain regulatory approvals or commercialize these programs on a timely basis or at all, which would have an adverse effect on its business.

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, the Company's 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 the Company's anticipated clinical trials or other future clinical trials the Company may initiate less predictable and could cause the Company's product candidates to perform differently, including causing toxicities, which could delay completion of the Company's clinical trials, delay approval of its product candidates, and/or jeopardize the Company's ability to commence product sales and generate revenues.

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

The Company may rely on academic and private non-academic institutions to conduct and sponsor clinical trials relating to its product candidates. The Company 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 clinical trials, whether controlled by the Company 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.

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

Additionally, the FDA or non-U.S. regulatory authorities may disagree with the sufficiency of the Company's right of reference to the preclinical, manufacturing or clinical data generated by these investigator-sponsored trials, or its 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 the Company to obtain and submit additional preclinical, manufacturing, or clinical data before the Company may initiate its anticipated trials and/or may not accept such additional data as adequate to initiate its anticipated trials.

The Company's product candidates may cause undesirable side effects that could delay or prevent their regulatory approval or commercialization or have other significant adverse implications on the Company's business, financial condition and results of operations.

Undesirable side effects observed in preclinical studies or in clinical trials with the Company's 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 such product candidates that receive regulatory approval. In turn, this could eliminate or limit the Company's ability to commercialize its product candidates.

The Company's 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.

The Company's 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 the Company's product candidates could potentially have an adverse effect on the Company's business, financial condition and results of operations.

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

- the Company may be unable to obtain additional financing on acceptable terms, if at all;
- the Company's collaborators may terminate any development agreements covering these product candidates;
- if any development agreements are terminated, the Company 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 the Company 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 the Company's potential future revenues from their commercialization;
- the Company may be subject to product liability or stockholder litigation; and
- the Company may be unable to attract and retain key employees.

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

- regulatory authorities may withdraw their approval of the product, or the Company or the Company's partners may decide to cease marketing and sale of the product voluntarily;
- the Company 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
- the Company's reputation may suffer.

Any of these events could prevent the Company 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 the Company 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 the Company and delay the Company's ability to establish strategic collaborations.

Delays in the commencement or completion of clinical trials could significantly impact the Company's drug development costs. The Company does 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 the Company's collaborators to adequately resource the Company's 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 the Company, the Company's collaborators, the institutional review boards or data safety monitoring boards charged with overseeing the Company's 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 the Company experiences delays in the completion or termination of any clinical trial of its product candidates, the commercial prospects of the Company's product candidates will be harmed, and the Company's ability to commence product sales and generate product revenues from any of the Company's product candidates will be delayed. In addition, any delays in completing the Company's clinical trials will increase the Company's costs and slow down its product candidate development and approval process. Delays in completing the Company's clinical trials could also allow the Company's competitors to obtain marketing approval before the Company does or shorten the patent protection period during which the Company may have the exclusive right to commercialize its product candidates. Any of these occurrences may harm the Company's 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 the Company's product candidates.

If the Company experiences delays in the enrollment of patients in its clinical trials, the Company's receipt of necessary regulatory approvals could be delayed or prevented.

The Company may not be able to initiate or continue clinical trials for the Company's product candidates if the Company is 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 the Company is investigating.

If the Company fails 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 the Company's clinical trials may result in increased development costs for the Company's product candidates, which would cause the value of the Company to decline and limit its ability to obtain additional financing. The Company's inability to enroll a sufficient number of patients for any of its future clinical trials would result in significant delays or may require the Company to abandon one or more clinical trials altogether.

The Company intends to rely on third parties to conduct its 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, the Company may not be able to obtain regulatory approval for or commercialize its product candidates and its business, financial condition and results of operations could be substantially harmed.

The Company intends to rely upon third-party CROs, medical institutions, clinical investigators and contract laboratories to monitor and manage data for the Company's ongoing preclinical and anticipated clinical programs. Nevertheless, the Company maintains responsibility for ensuring that each of the Company's preclinical studies are, and anticipated clinical studies will be, conducted in accordance with the applicable protocol, legal, regulatory, and scientific standards and the Company's reliance on these third parties does not relieve the Company of its regulatory responsibilities. The Company and its CROs and other vendors are required to comply with current requirements on good manufacturing practices ("cGMP"), good clinical practices ("GCP") and good laboratory practices ("GLP"), which are a collection of laws and regulations enforced by the FDA, the EMA and comparable foreign authorities for all of the Company's 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 the Company or any of its CROs or vendors fails to comply with applicable regulations, the data generated in the Company's preclinical studies and clinical trials may be deemed unreliable and the FDA, the EMA or comparable foreign authorities may require the Company to perform additional preclinical studies and clinical trials before approving the Company's marketing applications. The Company cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of the Company's clinical trials comply with GCP regulations. In addition, the Company's clinical trials must be conducted with products produced consistent with cGMP regulations. The Company's failure to comply with these regulations may require it to repeat clinical trials, which would delay the development and regulatory approval processes.

The Company may also not be able to enter into arrangements with CROs on commercially reasonable terms, or at all. In addition, the Company's CROs will not be the Company's employees, and except for remedies available to the Company under its agreements with such CROs, the Company will not be able to control whether or not they devote sufficient time and resources to the Company's 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 the Company's protocols, regulatory requirements, or for other reasons, the Company's clinical trials may be extended, delayed or terminated and the Company may not be able to obtain regulatory approval for or successfully commercialize the Company's product candidates. CROs may also generate higher costs than anticipated. As a result, the Company's business, financial condition and results of operations and the commercial prospects for the Company's product candidates could be materially and adversely affected, its costs could increase, and its 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 the Company's ability to meet its desired development timelines. There can be no assurance that the Company will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse effect on the Company's business, financial condition or results of operations.

The Company's 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 the Company's product candidates.

The clinical development, manufacturing, labeling, storage, record-keeping, advertising, promotion, export, marketing and distribution of the Company's 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 the Company nor the Company's collaborators are permitted to market the Company's product candidates until the Company or the Company's collaborators receive approval of a new drug application ("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 the Company 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, the Company has not previously filed NDAs with the FDA or filed similar applications with other foreign regulatory agencies. This lack of experience may impede the Company's ability to obtain FDA or other foreign regulatory agency approval in a timely manner, if at all, for the Company's product candidates for which development and commercialization is the Company's 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 the Company's third-party manufacturers' processes or facilities; or
- the FDA, the EMA or a comparable foreign authority may change its approval policies or adopt new regulations.

The Company's inability to obtain these approvals would prevent the Company from commercializing its 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 the Company's product candidates, which may be difficult to predict.

The FDA, 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 the Company's 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, the Company's 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 the Company's 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, or IBC, approval as well as all other applicable regulatory authorizations have been obtained. In addition to the government regulators, the IBC and institutional review board ("IRB") of each institution at which the Company will conduct clinical trials of its 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 the Company's 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 the Company complies 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 the Company to perform additional studies or trials, increase the Company's development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of the Company's product candidates or lead to significant post-approval limitations or restrictions. As the Company advances its product candidates, the Company will be required to consult with these regulatory agencies and committees and comply with applicable requirements and guidelines. If the Company fails to do so, it 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 the Company otherwise would have expected. Delays as a result of an increased or lengthier regulatory approval process or further restrictions on the development of the Company's product candidates can be costly and could negatively impact the Company's or its collaborators' ability to complete clinical trials and commercialize the Company's current and future product candidates in a timely manner, if at all.

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

In order to market any products outside of the U.S., the Company 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 the Company's ability to develop foreign markets for its product candidates.

Even if any of the Company's product candidates receive regulatory approval, its product candidates may still face future development and regulatory difficulties.

If any of the Company's 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, its 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, the Company's collaborators or the Company, including requiring withdrawal of the product from the market. The Company's 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 the Company's 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 the Company or the Company's collaborators;
- withdraw any regulatory approvals;
- impose restrictions on operations, including costly new manufacturing requirements, or shut down the Company's 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 the Company's 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 the Company receives marketing approval for its product candidates for the Company's proposed indications, physicians may nevertheless use the Company's 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 the Company's products could be used in such manner. However, if the Company is found to have promoted its products for any off-label uses, the federal government could levy civil, criminal or administrative penalties, and seek fines against the Company. Such enforcement has become more common in the industry. The FDA, the EMA or comparable foreign authorities could also request that the Company enter into a consent decree or a corporate integrity agreement, or seek a permanent injunction against the Company under which specified promotional conduct is monitored, changed or curtailed. If the Company cannot successfully manage the promotion of its product candidates, if approved, the Company could become subject to significant liability, which would materially adversely affect the Company's business, financial condition and results of operations.

The Company and its potential contract manufacturers are subject to significant regulation with respect to manufacturing the Company's product candidates. The manufacturing facilities on which the Company will rely may not continue to meet regulatory requirements.

All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including the Company's potential contract manufacturers for its 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 the Company's product candidates that may not be detectable in final product testing. The Company or its 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 the Company's 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 the Company's potential third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of the Company's product candidates or any of its other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of the Company's product candidates or any of the Company's other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. Although the Company plans to oversee the contract manufacturers, the Company cannot control the manufacturing process of, and will be completely dependent on, the Company's contract manufacturing partners for compliance with the regulatory requirements. If these facilities do not pass a pre-approval plant inspection, regulatory approval of the products 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 the Company's potential third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of the Company's product specifications or applicable regulations occurs independent of such an inspection or audit, the Company or the relevant regulatory authority may require remedial measures that may be costly or time consuming for the Company 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 the Company or third parties with whom the Company may contract could materially harm the Company's business, financial condition and results of operations.

If the Company or any of its 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, the Company's 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 the Company's desired clinical and commercial timelines.

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

Current and future legislation may increase the difficulty and cost of commercializing the Company's product candidates and may affect the prices the Company may obtain if the Company's 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 the Company's product candidates, restrict or regulate post-marketing activities and affect the Company's ability to profitably sell any of the Company's product candidates for which the Company obtains 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 the Company receives for any of its approved products. 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. The Company is 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 the Company's 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 the Company 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.

The Company expects 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 the Company receives 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 the Company from being able to generate revenue, attain profitability, or commercialize its drugs.

In Europe, the United Kingdom has indicated its intent to withdraw from the European Union in the future. A significant portion of the regulatory framework in the United Kingdom is derived from the regulations of the European Union. The Company 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 the Company's 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 the Company's product candidates from being developed or commercialized, which could negatively impact the Company's 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 the Company's product candidates to be reviewed or approved by necessary government agencies, which could adversely affect its business, financial condition and results of operations.

The Company is 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 the Company’s business, financial condition and results of operations.

In the U.S., the Company is 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 the Company seeks to structure its 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 the Company’s 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, the Company is 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 the Company fails to comply with an applicable state law requirement, it could be subject to penalties.

Neither the government nor the courts have provided definitive guidance on the application of fraud and abuse laws to the Company’s business. Law enforcement authorities are increasingly focused on enforcing these laws, and it is possible that some of the Company’s practices may be challenged under these laws. Efforts to ensure that the Company’s business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. If the Company is found in violation of one of these laws, the Company 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 the Company’s operations. If this occurs, the Company’s business, financial condition and results of operations may be materially adversely affected.

If the Company faces allegations of noncompliance with the law and encounter sanctions, its reputation, revenues and liquidity may suffer, and any of the Company’s 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 the Company 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 the Company’s ability to generate revenues from any of its product candidates that are ultimately approved for commercialization. If regulatory sanctions are applied or if regulatory approval is withdrawn, the Company’s business, financial condition and results of operations will be adversely affected. Additionally, if the Company is unable to generate revenues from product sales, the Company’s potential for achieving profitability will be diminished and the Company’s need to raise capital to fund its operations will increase.

Risks Related to our Common Stock

The market price of our common stock is expected to be volatile; we may also incur significant costs from class action litigation due to the Merger.

The trading price of the Company's 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 positive outcomes from our preclinical activities on the PATrOL™ platform and disease specific programs;
- 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 the Company's product candidates and the Company's 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;
- failure to successfully develop and commercialize the Company's product candidates;
- changes in laws or regulations applicable to the Company's product candidates;
- inability to obtain adequate product supply for the Company's product candidates, or the inability to do so at acceptable prices or in an acceptable timeframe;
- unanticipated serious safety concerns related to any of the Company's product candidates;
- adverse regulatory decisions;
- introduction of new products or technologies by the Company's competitors;
- failure to meet or exceed drug development or financial projections the Company provides to the public;
- failure to meet or exceed the estimates and projections of the investment community;
- 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 the Company or the Company's competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and the Company's ability to obtain patent protection for the Company's 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 the Company's common stock by the Company or its stockholders in the future;
- trading volume of the Company's common stock;
- period-to-period fluctuations in the Company's financial results;
- changes in the structure of health care payments;
- changes in the Nasdaq listing of the Company's stock; and
- recommendations of equity analysts covering the Company's 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 the Company's 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 the Company's profitability and reputation.

The Company's management owns a significant percentage of the Company's 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 the Company, holds a significant number of shares of the Company's outstanding Common Stock and an option to purchase additional shares of Common Stock. Accordingly, Dr. Stephan has the ability to influence the Company through this ownership position.

This significant concentration of stock ownership may adversely affect the trading price for the Company's 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 the Company's 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 the Company's interests or the interests of other stockholders. This may also prevent or discourage unsolicited acquisition proposals or offers for the Company's common stock that you may feel are in your best interests as one of the Company's 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 the Company's common stock.

The Company's internal control over financial reporting may not meet the standards required by Section 404 of the Sarbanes-Oxley Act, and failure to achieve and maintain effective internal control over financial reporting in accordance with Section 404 of the Sarbanes-Oxley Act, could have a material adverse effect on the Company's business and share price.

If and when the Company ceases to be a "non-accelerated filer," our management will be required to report on the effectiveness of the Company's internal control over financial reporting. The rules governing the standards that must be met for the Company's management to assess the Company's internal control over financial reporting are complex and require significant documentation, testing and possible remediation. The Company expects that compliance with these rules and regulations will continue to substantially increase the Company's legal and financial compliance costs and will make some activities more time-consuming and costly, and the Company's 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 deficiencies or 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 requested improvements and receiving a favorable attestation in connection with the attestation provided by our independent registered public accounting firm. 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 the Company's financial results accurately and in a timely manner.

The Company may take advantage of specified reduced disclosure requirements applicable to a "smaller reporting company" under Regulation S-K, and the information that the Company provides to stockholders may be different than they might receive from other public companies.

The Company is a "smaller reporting company," as defined under Regulation S-K. As a smaller reporting company, the Company 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. The Company may continue to take advantage of these allowances until it is no longer a smaller reporting company. The Company will cease to be a smaller reporting company if the Company has (i) more than \$250 million in market value of its shares held by non-affiliates as of the last business day of its second fiscal quarter or (ii) more than \$100 million of annual revenues in its most recent fiscal year completed before the last business day of its second fiscal quarter and a market value of its shares held by non-affiliates more than \$700 million as of the last business day of its second fiscal quarter. The Company may choose to take advantage of some but not all of these scaled disclosure requirements. Therefore, the information that Company provides stockholders may be different than one might get from other public companies. Further, if some investors find the Company's ordinary shares less attractive as a result, there may be a less active trading market for the Company's ordinary shares and the market price of such ordinary shares may be more volatile.

If securities or industry analysts do not publish research, or publish inaccurate or unfavorable research, about the Company's business, the Company's stock price and trading volume could decline.

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

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

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

The Company's 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 the Company and its stockholders other than actions arising under the Securities Act or the Exchange Act, which could limit the Company's stockholders' ability to obtain a favorable judicial forum for disputes with the Company or its directors, officers, or employees.

The Company's 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 its behalf;
- any action asserting a breach of fiduciary duty;
- any action asserting a claim against the Company arising under the Delaware General Corporation Law, the Company's amended and restated certificate of incorporation, or the Company's bylaws; and
- any action asserting a claim against the Company that is governed by the internal-affairs doctrine.

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 the Company or its directors, officers, or other employees, which may discourage lawsuits against the Company and its directors, officers, and other employees. If a court were to find either exclusive forum provision in the Company's 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 the Company's business.

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

Provisions in the Company's certificate of incorporation and bylaws may delay or prevent an acquisition or a change in management. These provisions include a classified board of directors and the ability of the board of directors to issue preferred stock without stockholder approval. Although the Company believe these provisions collectively will provide for an opportunity to receive higher bids by requiring potential acquirers to negotiate with the Company's 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 the Company's 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 Delaware corporate law deter hostile takeovers. Specifically, Section 203 of the Delaware General Corporation Law 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, the Company’s certificate of incorporation does not include any such opt-out provision.

Our pre-Merger net operating loss carryforwards and certain other tax attributes may 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 Code, is subject to limitations on its ability to utilize its pre-change 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, the Company’s NOLs and certain other tax attributes will be subject to the Section 382 Limitation. Consequently, even if the Company achieves profitability, it may not be able to utilize a material portion of its 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 December 31, 2018, the Company estimated that it had approximately \$69 million in NOL carryforwards. It is likely that the Section 382 Limitation will cause a significant portion of the Company’s net operating loss carryforwards to never be utilized. In addition, if the Company is determined to have discontinued its historic business following the completion of the Merger, subject to certain exceptions, the Section 382 Limitation could eliminate all possibility of utilizing its NOL carryforwards.

The Company may never pay dividends on the Company’s common stock so any returns would be limited to the appreciation of the Company’s stock.

The Company currently anticipates that it will retain future earnings for the development, operation and expansion of the Company’s business and do not anticipate it will declare or pay any cash dividends for the foreseeable future. In addition, the terms of any future debt agreements may preclude the Company 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.

Except as previously disclosed on a Current Report on Form 8-K, there were no unregistered sales of common stock or other equity securities during the three months ended June 30, 2019.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

None.

Item 6. Exhibits.

<u>Exhibit Number</u>	<u>Exhibit</u>
2.1	Agreement and Plan of Merger and Reorganization, dated as of January 2, 2019, by and among the Company, Ohr Acquisition Corp. and NeuBase Therapeutics, Inc. (incorporated herein by reference to Exhibit 2.1 to the Company's Current Report on Form 8-K, filed on January 3, 2019)
2.2	First Amendment to the Agreement and Plan of Merger and Reorganization, dated as of June 27, 2019, by and among the Company, Ohr Acquisition Corp. and NeuBase Therapeutics, Inc. (incorporated herein by reference to Exhibit 2.1 to the Company's Current Report on Form 8-K, filed on July 3, 2019)
2.3	Form of Support Agreements by and among the Company, NeuBase Therapeutics Inc. and the officers and directors of the Company (incorporated herein by reference to Exhibit 2.2 to the Company's Current Report on Form 8-K, filed on January 3, 2019)
2.4	Form of Support Agreement by and among NeuBase Therapeutics, Inc., the Company and its officers, directors and certain stockholders of NeuBase Therapeutics, Inc. (incorporated herein by reference to Exhibit 2.3 to the Company's Current Report on Form 8-K, filed on January 3, 2019)
2.5	Form of the Company and NeuBase Therapeutics, Inc. Lock-Up Agreements (incorporated herein by reference to Exhibit 2.4 to the Company's Current Report on Form 8-K, filed on January 3, 2019)
2.6	Contribution Agreement, dated May 14, 2014, among the Company, certain affiliates of the Company, SKS Ocular, LLC, SKS Ocular 1, LLC, and the controlling members of SKS (incorporated herein by reference to Exhibit 2.1 to the Company's Current Report on Form 8-K, filed on May 16, 2014)
2.7	Agreement and Plan of Merger, dated May 30, 2014, by and among the Company, Ohr Holdco, Inc., and Ohr Merger Sub, Inc. (incorporated herein by reference to Exhibit 2.2 to the Company's Current Report on Form 8-K, filed on June 2, 2014)
2.8	Asset Purchase Agreement, dated August 21, 2009, between the Company and Genaera Liquidating Trust (incorporated herein by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed on August 26, 2009)
3.1	Certificate of Incorporation of the Company (incorporated herein by reference to Exhibit 3.1(a) to the Company's Current Report on Form 8-K, filed on June 2, 2014)
3.2	Certificate of Amendment to Certificate of Incorporation of the Company (incorporated herein by reference to Exhibit 3.1(b) to the Company's Current Report on Form 8-K, filed on June 2, 2014)
3.3	Certificate of Amendment to Certificate of Incorporation of the Company (incorporated herein by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K, filed on January 23, 2019)
3.4	Amended and Restated Certificate of Incorporation of the Company (incorporated herein by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K, filed on July 12, 2019)
3.5	By-Laws of the Company (incorporated herein by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K, filed on June 2, 2014)
31.1	Certification of Chief Executive Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Certification of Chief Financial Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Certification of Chief Executive Officer Pursuant to 18 U.S.C Section 1350, As Adopted Pursuant to Section 906 of the Sarbanes Oxley Act of 2002
32.2	Certification of Chief Financial Officer Pursuant to 18 U.S.C Section 1350, As Adopted Pursuant to Section 906 of the Sarbanes Oxley Act of 2002
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

SIGNATURES

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

Date: August 14, 2019

NEUBASE THERAPEUTICS, INC.
(Registrant)

By: /s/ Dr. Dietrich Stephan
Dr. Dietrich Stephan
Chief Executive Officer
(Principal Executive Officer)

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

**Certification of Principal Executive Officer
Pursuant to Section 302 of the
Sarbanes-Oxley Act of 2002**

I, Dr. Dietrich Stephan, certify that:

1. I have reviewed this 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 internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15(d)-15(f)) for the registrant and have:
 - a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated 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.

Dated: August 14, 2019

By: /s/ Dr. Dietrich Stephan
Dr. Dietrich Stephan
Chief Executive Officer
(Principal Executive Officer)

**Certification of Principal Financial Officer
Pursuant to Section 302 of the
Sarbanes-Oxley Act of 2002**

I, Sam Backenroth, certify that:

1. I have reviewed this 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 internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15(d)-15(f)) for the registrant and have:
 - a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated 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.

Dated: August 14, 2019

By: /s/ Sam Backenroth
Title: Chief Financial Officer
(Principal Financial and Accounting Officer)

**Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, As Adopted Pursuant to
Section 906 of the Sarbanes-Oxley Act of 2002**

Not Filed Pursuant to the Securities Exchange Act of 1934

In connection with the Quarterly Report of NeuBase Therapeutics, Inc. (the "*Company*") on Form 10-Q for the quarterly period ending June 30, 2019, as filed with the Securities and Exchange Commission on the date hereof (the "*Report*"), I, Dr. Dietrich Stephan, Chief Executive Officer (Principal Executive Officer), of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

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

Dated: August 14, 2019

/s/ Dr. Dietrich Stephan

Name: Dr. Dietrich Stephan
Title: Chief Executive Officer
(Principal Executive Officer)

**Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, As Adopted Pursuant to
Section 906 of the Sarbanes-Oxley Act of 2002**

Not Filed Pursuant to the Securities Exchange Act of 1934

In connection with the Quarterly Report of NeuBase Therapeutics, Inc. (the "*Company*") on Form 10-Q for the quarterly period ending June 30, 2019, as filed with the Securities and Exchange Commission on the date hereof (the "*Report*"), I, Sam Backenroth, Chief Financial Officer (Principal Financial Officer), of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

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

Dated: August 14, 2019

/s/ Sam Backenroth

Name: Sam Backenroth

Title: Chief Financial Officer

(Principal Financial and Accounting Officer)