December 23, 2020

neubase

# NeuBase Therapeutics Reports Business Update and Financial Results for Fiscal Year 2020

*Data presented throughout 2020 have validated the potential of the PATrOL™ platform to develop highly targeted therapies that increase, decrease or change causal protein function*

*Plan to provide updates on development pipeline, including the myotonic dystrophy type 1 (DM1) and Huntington's disease (HD) programs, at an R&D day in the first half of CY2021*

PITTSBURGH, Pa., Dec. 23, 2020 (GLOBE NEWSWIRE) -- NeuBase Therapeutics, Inc. (Nasdaq: NBSE) ("NeuBase" or the "Company"), a biotechnology company accelerating the genetic revolution using a new class of synthetic medicines, today reported its financial results for the fiscal year ended September 30, 2020.

"Throughout 2020, we successfully executed against our development strategy, most notably with the generation of two very exciting datasets supporting our initial therapeutic pipeline, including our DM1 and Huntington's disease programs. These initial data are a clear validation of our transformative platform and provide a strong foundation on which to build our IND-enabling studies for both programs," said Dietrich A. Stephan, Ph.D., chief executive officer of NeuBase. "In addition, these data support the broad potential of our PATrOL™ platform as a viable synthetic approach to genetic medicine. We believe the platform's unique capabilities, which include increasing, decreasing and changing protein function, have the potential to redefine treatment for a multitude of patients suffering from both common and rare genetic conditions with insufficient or no therapeutic options."

"As we plan for the future, we've expanded our team with several key hires in the second half of the year, including the appointments of Drs. Curt Bradshaw and William Mann as chief scientific officer and chief operating officer, respectively. The expanded capabilities across our entire clinical team are expected to be a positive driver of our development activities as we enter the new year, and advance and expand our pipeline. In order to support this larger team, we recently signed a lease for a new headquarters which will offer more space and state-of-the-art lab facilities to support the rapid development of our pipeline. We look forward to providing greater insight into these activities during an investor R&D day expected to take place in the first half of CY2021," continued Dr. Stephan.

**Fourth Fiscal Quarter of 2020 and Recent Operating Highlights**

- Announced positive preclinical *in vitro* and *in vivo* data for PATrOL-enabled anti-gene for the treatment of myotonic dystrophy type 1 (DM1), which further validate the potential of the Company's proprietary platform to develop highly targeted genetic therapies
- Appointed Curt Bradshaw, Ph.D., seasoned industry veteran and former chief scientific

officer at Arrowhead Pharmaceuticals, as the new chief scientific officer of NeuBase
- Further strengthened the management team with the appointment of William Mann, Ph.D., MBA, an experienced executive with a track record that spans the biopharma life cycle, as chief operating officer
- Expanded the Company's Scientific Advisory Board (SAB) with the appointments of Peter Nielsen, Ph.D., inventor of peptide nucleic acid technology, Eriks Rozners, Ph.D., an expert in alternative binding modes of peptide nucleic acids, and Randy Davis, MBA, a leader in biotech development, which complement the SAB's existing team of renowned experts
- Signed a lease for a new headquarters with office and lab space in Pittsburgh that will offer more space to support the Company's expanding development activities around its rapidly advancing pipeline of PATrOL-enabled therapies

**Financial Results for the Fiscal Year Ended September 30, 2020:**

- At September 30, 2020, the Company had cash and cash equivalents of approximately $32.0 million, compared with cash and cash equivalents of approximately $10.3 million at September 30, 2019. NeuBase estimates its cash and cash equivalents are sufficient to fund the currently planned operating and capital expenditures into the first quarter of CY2022;
- For the fiscal year ended September 30, 2020, the Company reported a net loss of approximately $17.4 million, or a net loss of $0.89 per share, compared with a net loss of approximately $26.1 million, or a net loss of $3.16 per share, for the fiscal year ended September 30, 2019; and
- For the fiscal year ended September 30, 2020, total operating expenses were approximately $17.1 million, consisting of approximately $10.1 million in general and administrative expenses and $6.9 million of research and development expenses. This compares with total operating expenses of $25.5 million for the fiscal year ended September 30, 2019, which was comprised of approximately $9.1 million in general and administrative expenses, $3.4 million in research and development expenses, and $13.0 million in research and development-license acquired expenses.

**About NeuBase Therapeutics**
NeuBase is accelerating the genetic revolution using a new class of synthetic medicines. NeuBase's designer PATrOL™ therapies are centered around its proprietary drug scaffold to address genetic diseases at the source by combining the highly targeted approach of traditional genetic therapies with the broad organ distribution capabilities of small molecules. With an initial focus on silencing disease-causing mutations in debilitating neurological, neuromuscular and oncologic disorders, NeuBase is committed to redefining medicine for the millions of patients with both common and rare conditions. To learn more, visit www.neubasetherapeutics.com.

**Use of Forward-Looking Statements**
This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act. These forward-looking statements are distinguished by use of words such as "will," "would," "anticipate," "expect," "believe," "designed," "plan," or "intend," the negative of these terms, and similar references to future periods. These forward-looking statements include, among others, those related to the potential significance and implications of the Company's positive in vitro and in vivo preclinical data for its

PATrOL™-enabled anti-gene therapies for the treatment of myotonic dystrophy and our plan to provide updates on development pipeline, including the myotonic dystrophy type 1 (DM1) and Huntington's disease (HD) programs, at an R&D day in the first half of CY2021. These views involve risks and uncertainties that are difficult to predict and, accordingly, our actual results may differ materially from the results discussed in our forward-looking statements. Our forward-looking statements contained herein speak only as of the date of this press release. Factors or events that we cannot predict, including those risk factors contained in our filings with the U.S. Securities and Exchange Commission, may cause our actual results to differ from those expressed in forward-looking statements. The Company may not actually achieve the plans, carry out the intentions or meet the expectations or projections disclosed in the forward-looking statements, and you should not place undue reliance on these forward-looking statements. Because such statements deal with future events and are based on the Company's current expectations, they are subject to various risks and uncertainties, and actual results, performance or achievements of the Company could differ materially from those described in or implied by the statements in this press release, including: the Company's plans to develop and commercialize its product candidates; the timing of initiation of the Company's planned clinical trials; the risks that prior data will not be replicated in future studies; the timing of any planned investigational new drug application or new drug application; the Company's plans to research, develop and commercialize its current and future product candidates; the clinical utility, potential benefits and market acceptance of the Company's product candidates; the Company's commercialization, marketing and manufacturing capabilities and strategy; global health conditions, including the impact of COVID-19; the Company's ability to protect its intellectual property position; and the requirement for additional capital to continue to advance these product candidates, which may not be available on favorable terms or at all, as well as those risk factors contained in our filings with the U.S. Securities and Exchange Commission. Except as otherwise required by law, the Company disclaims any intention or obligation to update or revise any forward-looking statements, which speak only as of the date hereof, whether as a result of new information, future events or circumstances or otherwise.

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**NeuBase Therapeutics, Inc. and Subsidiaries**
**Consolidated Balance Sheets**

| | September 30, | |
|---|---|---|
| | **2020** | **2019** |

| | | |
|---|---|---|
| ASSETS | | |
| CURRENT ASSETS | | |
| Cash and cash equivalents | $ 31,992,283 | $ 10,313,966 |
| Prepaid insurance | 521,617 | 449,583 |
| Other prepaid expenses and current assets | 294,640 | 265,686 |
| Total Current Assets | 32,808,540 | 11,029,235 |
| EQUIPMENT, net | 1,166,934 | 430,995 |
| OTHER ASSETS | | |
| Intangible assets, net | - | 145,833 |
| Investment | 323,557 | 586,418 |
| Long-term prepaid insurance | 145,250 | 338,916 |
| Total Other Assets | 468,807 | 1,071,167 |
| TOTAL ASSETS | $ 34,444,281 | $ 12,531,397 |
| LIABILITIES AND STOCKHOLDERS' EQUITY | | |
| CURRENT LIABILITIES | | |
| Accounts payable | $ 1,505,042 | $ 1,477,152 |
| Accrued expenses and other current liabilities | 555,883 | 405,599 |
| Warrant liabilities | 950,151 | 496,343 |
| Insurance note payable | 138,557 | 122,919 |
| Total Liabilities | 3,149,633 | 2,502,013 |
| COMMITMENTS AND CONTINGENCIES (Note 16) | | |
| STOCKHOLDERS' EQUITY | | |
| Preferred stock, $0.0001 par value; 10,000,000 shares authorized; no shares issued and outstanding as of September 30, 2020 and 2019 | - | - |
| Common stock, $0.0001 par value; 250,000,000 shares authorized; 23,154,084 and 17,077,873 shares issued and outstanding as of September 30, 2020 and 2019, respectively | 2,315 | 1,708 |
| Additional paid-in capital | 74,850,935 | 36,201,758 |
| Accumulated deficit | (43,558,602) | (26,174,082) |
| Total stockholders' equity | 31,294,648 | 10,029,384 |
| TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY | $ 34,444,281 | $ 12,531,397 |

**NeuBase Therapeutics, Inc. and Subsidiaries**
**Consolidated Statements of Operations**

| | Year Ended September 30, | |
|---|---|---|
| | **2020** | **2019** |
| OPERATING EXPENSES | | |
| General and administrative | $ 10,123,298 | $ 9,095,674 |
| Research and development | 6,946,008 | 3,447,201 |
| Research and development expense- license acquired | - | 12,967,415 |
| TOTAL OPERATING EXPENSES | 17,069,306 | 25,510,290 |
| LOSS FROM OPERATIONS | (17,069,306) | (25,510,290) |
| OTHER INCOME (EXPENSE) | | |
| Interest expense | (7,686) | (128,951) |
| Change in fair value of warrant liabilities | (453,808) | (492,889) |
| Loss on disposal of fixed asset | (3,230) | - |
| Equity in losses on equity method investment | (262,861) | - |
| Other income | 412,371 | - |
| Total other income (expenses), net | (315,214) | (621,840) |
| NET LOSS | $(17,384,520) | $(26,132,130) |
| BASIC AND DILUTED LOSS PER SHARE | $ (0.89) | $ (3.16) |
| WEIGHTED AVERAGE SHARES OUTSTANDING: | | |
| BASIC AND DILUTED | 19,620,291 | 8,271,707 |

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Source: NeuBase Therapeutics, Inc.