May 13, 2021

NeuBase Therapeutics Reports Financial Results for the Second Quarter of Fiscal Year 2021

- *Extended cash runway into CY2023 following completion of an equity financing from leading healthcare investors generating $42.6 million in net proceeds; supports advancing the Company's lead program into the clinic and scaling of its precision genetic medicine pipeline*
- *Completed acquisition of peptide-nucleic acid ("PNA") scaffold technologies, to expand the PATrOL™ platform capabilities and consolidate PNA intellectual property*
- *Scheduled to host an R&D day on June 8th to present new preclinical data, updates on myotonic dystrophy type 1 (DM1) and Huntington's disease (HD) programs, and unveil a new program in oncology*

PITTSBURGH, May 13, 2021 (GLOBE NEWSWIRE) -- NeuBase Therapeutics, Inc. (Nasdaq: NBSE) ("NeuBase" or the "Company"), a biotechnology company accelerating the genetic revolution using a new class of precision genetic medicines, today reported its financial results for the three- and six-month periods ended March 31, 2021.

"We continue to expand and scale our unique precision genetic medicine platform that we believe can turn genes on, off, or edit them *in vivo*, and thus address most mechanisms that cause diseases in a single industry-unifying solution," said Dietrich A. Stephan, Ph.D., Founder, CEO and Chairman of NeuBase. "Our recent financing led by top-tier healthcare investors enables us to advance our lead program into the clinic next year and expand our pipeline to address historically undruggable oncogenic driver mutations. We look forward to hosting our R&D day on June 8th, during which we will present an update on our current pipeline programs, as well as introduce an oncology program targeting a genetic driver mutation in a high value indication."

Second Quarter of Fiscal Year 2021 and Recent Operating Highlights

- Strengthened the balance sheet with a public equity offering generating $42.6 million in net proceeds, which will enable the Company to advance its lead program into the clinic and expand the pipeline of therapies for rare and common diseases
- Expanded platform capabilities and consolidated PNA intellectual property through the acquisition of new scaffold technologies from Vera Therapeutics, formerly known as TruCode Gene Repair, Inc., in late April 2021
- Presented positive *in vitro* and *in vivo* preclinical data for the Company's lead drug candidate for the treatment of DM1 at the 2021 Muscular Dystrophy Association (MDA) Virtual Clinical & Scientific Conference
- Moved into a newly built state-of-the-art laboratory and office space in Pittsburgh, providing the infrastructure to support accelerating R&D activities
- Appointed Gerald McDougall, industry veteran, to the Board of Directors, bringing

large-scale partnership and strategy expertise to the Company

Financial Results for the Second Fiscal Quarter Ended March 31, 2021

- As of March 31, 2021, the Company had cash and cash equivalents of approximately $24.2 million, compared with cash and cash equivalents of approximately $32.0 million as of September 30, 2020
    - Subsequent to the end of the fiscal quarter ended March 31, 2021, the Company completed a public equity offering generating net proceeds of approximately $42.6 million
    - NeuBase estimates its current cash and cash equivalents are sufficient to fund currently planned operating and capital expenditures into the first quarter of CY2023
- For the three-month period ended March 31, 2021, the Company reported a net loss of approximately $5.5 million, or a net loss of $0.24 per share, compared with a net loss of approximately $4.4 million, or a net loss of $0.26 per share, for the three-month period ended March 31, 2020
- For the three-month period ended March 31, 2021, total operating expenses were approximately $5.9 million, consisting of approximately $2.7 million in general and administrative expenses and $3.2 million of research and development expenses; compared with total operating expenses of $4.4 million for the three-month period ended March 31, 2020, which was comprised of approximately $2.7 million in general and administrative expenses and $1.6 million in research and development expenses

Financial Results for the Six-Month Period Ended March 31, 2021

- For the six-month period ended March 31, 2021, the Company reported a net loss of approximately $9.6 million, or a net loss of $0.41 per share, compared with a net loss of approximately $8.9 million, or a net loss of $0.52 per share, for the same period last year
- For the six-month period ended March 31, 2021, total operating expenses were approximately $10.6 million, consisting of approximately $5.4 million in general and administrative expenses and $5.2 million of research and development expenses. This compares with total operating expenses of $8.1 million for the same period last year, which was comprised of approximately $5.3 million in general and administrative expenses and $2.8 million in research and development expenses

About NeuBase Therapeutics
NeuBase is accelerating the genetic revolution by developing a new class of precision genetic medicines which can be designed to increase, decrease, or change gene function, as appropriate, to resolve genetic defects that drive disease. NeuBase's targeted PATrOL™ therapies are centered around its proprietary drug scaffold to address genetic diseases at the DNA or RNA level 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 type 1 (DM1), the plan to provide updates on the Company's development pipeline, including the myotonic dystrophy type 1 (DM1) and Huntington's disease (HD) programs and an oncology program targeting a genetic driver mutation in a high value indication, at an R&D day in June 2021, the anticipated use of proceeds from the Company's April 2021 public equity offering and the Company's anticipated capital requirements over approximately the next twelve months. 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 (the "SEC"), 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 SEC. 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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