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

Recently demonstrated single-dose intravenous administration of a PATrOL™-enabled compound resolves the causal genetic defect in myotonic dystrophy type 1 (DM1) in transgenic animals; Company on course to move one program into clinical development in CY2022

Plans to host an R&D day in the first half of CY2021 to provide updates on platform innovations, the DM1 and Huntington's disease (HD) programs and pipeline expansion in high value indications

Expects to complete consolidation of intellectual property in the space through acquisition of gene modulating technology from Vera Therapeutics in Q1 CY2021

PITTSBURGH, Feb. 11, 2021 (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 three-month period ended December 31, 2020.

"In 2020 we validated that our PATrOL™ platform technology can deliver compounds that are broadly biodistributed, mutant allele-specific and well tolerated, including in non-human primates (NHPs). Thereafter we finalized screening compound libraries and moved into *in vivo* efficacy and tolerability studies that demonstrated that administration of a PATrOL™-enabled compound resolves the causal genetic insult in an established transgenic animal model of myotonic dystrophy type 1 (DM1), a severe genetic disease with no effective therapies. This momentum is being carried forward into 2021 as we set the stage to enter the clinic in CY2022," said Dietrich A. Stephan, Ph.D., chief executive officer of NeuBase. "Predicated on our progress, we recently announced an agreement to acquire additional gene modulating technology to consolidate the intellectual property to protect and enhance value creation with this unique therapeutic modality."

"We look forward to providing more data and insights during an investor R&D day in the first half of CY2021, including updates on platform innovations, continued progress in Huntington's disease (HD) and DM1 and new pipeline programs. This event will provide an opportunity for us to introduce the expanded team, including Dr. Curt Bradshaw, Ph.D. chief scientific officer, who has overseen several development programs into the clinic and complements a world-class team of technical experts and drug developers."

First Quarter of Fiscal Year 2021 and Recent Operating Highlights

- Announced positive preclinical *in vitro* and *in vivo* data for PATrOL™-enabled anti-gene for the treatment of DM1, which further validate the Company's core

technological foundation as well as the potential of its proprietary approach to comprehensively treat the underlying cause of DM1 and many other genetically driven diseases

- Further strengthened the management team with a key appointment to the role of chief scientific officer and the addition of the original inventor of peptide nucleic acid chemistry to the Scientific Advisory Board
- Entered a binding agreement to acquire gene modulating technology to consolidate and expand the capabilities within our technology platform which we expect to close in Q1 CY2021
- Initiated the buildout of a new headquarters with office and lab space that will support and enable the Company's expanding development activities around its rapidly advancing pipeline of PATrOL™-enabled therapies

Financial Results for the Fiscal Quarter Ended December 31, 2020

- At December 31, 2020, the Company had cash and cash equivalents of approximately \$28.0 million, compared with cash and cash equivalents of approximately \$32.0 million at September 30, 2020. 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 three-month period ended December 31, 2020, the Company reported a net loss of approximately \$4.1 million, or a net loss of \$0.18 per share, compared with a net loss of approximately \$4.5 million, or a net loss of \$0.26 per share, for the three-month period ended December 31, 2019
- For the three-month period ended December 31, 2020, total operating expenses were approximately \$4.7 million, consisting of approximately \$2.6 million in general and administrative expenses and \$2.0 million of research and development expenses. This compares with total operating expenses of \$3.8 million for the three-month period ended December 31, 2019, which was comprised of approximately \$2.6 million in general and administrative expenses and \$1.2 million in research and development expenses

About NeuBase Therapeutics

NeuBase is accelerating the genetic revolution using a new class of synthetic medicines which have been shown to be able to increase, decrease and change gene function, as appropriate, to resolve causal genetic defects in living systems. 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 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, at an R&D day in the first half of CY2021 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, 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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