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NeuBase Therapeutics Reports Financial Results for the Second Fiscal Quarter of 2020

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Announced positive preclinical data that validates the key advantages of the proprietary NeuBase peptide-nucleic acid antisense oligonucleotide (PATrOL™) platform

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Completed a public offering with net proceeds of approximately $33.3 million in April 2020, strengthening the Company's balance sheet to support the ongoing development of its product candidates and expand its pipeline

PITTSBURGH, May 14, 2020 (GLOBE NEWSWIRE) -- NeuBase Therapeutics, Inc. (Nasdaq: NBSE) ("NeuBase" or the "Company"), a biotechnology company developing next-generation antisense oligonucleotide (ASO) therapies using its scalable PATrOL™ platform to address genetic diseases, today reported its financial results for the three and six month periods ended March 31, 2020.

"We have generated a solid foundation of data that we expect will enable us to produce mutation-specific genetic medicines for a multitude of diseases. The positive non-human primate pharmacokinetic (biodistribution) and patient-derived cell line pharmacodynamic (activity) data released on March 31st illustrate our ability to create a new class of mutation silencing therapies. In addition, our proprietary targeting technology enables our PATrOL compounds to achieve therapeutically relevant drug concentrations throughout the body, including in the brain," said Dietrich A. Stephan, Ph.D., chief executive officer of NeuBase.

"Our therapeutic development programs continue to drive forward, despite the difficult macroenvironment, as evidenced by the recent positive platform validation data and well-received public offering. As we look ahead, our initial focus is to continue advancing our Huntington's disease ("HD") and myotonic dystrophy type 1 ("DM1") programs through lead optimization and IND-enabling studies. We expect to announce the lead candidate for the NT0100 Program for HD by the end of calendar year 2020, followed by the initiation of IND-enabling studies during the first half of calendar year 2021. Finally, our unique biodistribution profile allows us the opportunity to develop therapies against targets and organ systems that we believe other antisense companies cannot currently reach in the body, including into the brain after systemic delivery, which is one of the grand challenges in drug delivery of macromolecules. We believe this unique ability validates our large opportunity in the antisense space," continued Dr. Stephan.

Second Fiscal Quarter of 2020 and Recent Operating Highlights

- Announced positive, preclinical pharmacokinetic ("PK") and pharmacodynamic ("PD") data validating the first-in-class genetic therapy PATrOL™ platform:

- **PK studies** of the PATrOL™-enabled compound in non-human primates (NHPs) demonstrated, among other things, rapid uptake of the compound out of the body's circulation after systemic intravenous administration, penetration by the compound into every organ and tissue system studied, and retention of therapeutically relevant doses for greater than one week after single-dose injection;
- **PD studies** in patient-derived cell lines demonstrated, among other things, activity in engaging target disease-causing transcripts and knocking-down resultant malfunctioning mutant protein levels preferentially over normal protein knock-down; and dose-limiting toxicities were not observed relative to a control either at or above the doses demonstrating activity in human cells *in vitro*; and
- PATrOL™ enabled compounds were generally well-tolerated *in vivo* after systemic administration, both after single dose administration in NHPs and multi dose administration in mice for over a month.

Financial Results for the Fiscal Quarter Ended March 31, 2020:

- At March 31, 2020, the Company had cash and cash equivalents of approximately $5.8 million, compared with cash and cash equivalents of approximately $10.3 million at September 30, 2019. Subsequent to the end of the fiscal second quarter of 2020, the Company completed a public equity offering that generated net proceeds of approximately $33.3 million. The Company believes that its current cash balance will provide sufficient capital to fund operations into the second calendar quarter of 2022;
- For the three month period ended March 31, 2020, the Company reported a net loss of approximately $4.4 million, or a net loss of $0.26 per share, compared with a net loss of approximately $2.0 million, or a net loss of $0.33 per share, for the same period last year; and
- For the three month period ended March 31, 2020, total operating expenses were approximately $4.4 million, consisting of approximately $2.8 million in general and administrative expenses and $1.6 million of research and development expenses. This compares with total operating expenses of $1.9 million for the same period last year, which was comprised of approximately $1.9 million in general and administrative expenses and $0.03 million in research and development expenses.

Financial Results for the Six Month Period Ended March 31, 2020:

- For the six month period ended March 31, 2020, the Company reported a net loss of approximately $8.9 million, or a net loss of $0.52 per share, compared with a net loss of approximately $3.5 million, or a net loss of $0.59 per share, for the same period last year; and
- For the six month period ended March 31, 2020, total operating expenses were approximately $8.1 million, consisting of approximately $5.3 million in general and administrative expenses and $2.8 million of research and development expenses. This compares with total operating expenses of $3.4 million for the same period last year, which was comprised of approximately $2.3 million in general and administrative expenses and $1.1 million in research and development and research and development- license acquired expenses.

About NeuBase Therapeutics

NeuBase Therapeutics, Inc. is developing the next generation of gene silencing therapies with its flexible, highly specific synthetic antisense oligonucleotides. The proprietary

NeuBase peptide-nucleic acid (PNA) antisense oligonucleotide (PATrOL™) platform allows for the rapid development of targeted drugs, increasing the treatment opportunities for the hundreds of millions of people affected by rare genetic diseases, including those that can only be treated through accessing of secondary RNA structures. Using PATrOL™ technology, NeuBase aims to first tackle rare, genetic diseases.

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 include, among other things, statements regarding the Company's goals and plans and expectations regarding the timing of our Huntington's disease (NT0100) and myotonic dystrophy type 1 (NT0200) programs, as well as expanding our pipeline and the potential for the Company's technologies generally. 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 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 timing of the availability of data from the Company's clinical trials; 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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