March 26, 2020

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

NeuBase expects to announce pharmacokinetic data in non-human primates and in-vitro pharmacodynamic data for the PATrOL™ platform during the week of March 30th as planned

PITTSBURGH, March 26, 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 month period ended December 31, 2019.

"We continue to advance the development of our neurological and neuromuscular programs, which have not been significantly impacted by the COVID-19 pandemic. Next week, we plan to announce the results from a pharmacokinetic study in non-human primates, as well as pharmacodynamic data in patient-derived cell lines. We also expect to receive additional data from *in vivo* mouse pharmacokinetic studies later in the second calendar quarter of 2020 and present those results in a peer-reviewed publication or at a scientific conference in the second half of the calendar year," said Dietrich A. Stephan, Ph.D., chief executive officer of NeuBase.

"Recent FDA approvals in the RNA therapeutics industry continue to confirm the broad utility of the gene silencing approach, with neutral backbone ASOs now representing a significant portion of approved RNA-based drugs. We believe that the differentiated features of our PATrOL™ platform bodes well for our participation in the industry as we help fulfill the promise of scalable drug development using genetic sequence-based drugs. As previously announced, we plan to initially focus on Huntington's disease and myotonic dystrophy to address the critical unmet needs of the patients impacted by these diseases, and then expand our development pipeline into other high value disease targets and cancer," concluded Dr. Stephan.

First Fiscal Quarter of 2020 and Recent Operating Highlights

- U.S. Patent and Trademark Office issued NeuBase a foundational patent covering proprietary DNA and RNA binding technology, which enables PATrOL™-based therapies to target the secondary structures of DNA and RNA
- Cancer biologist and RNA therapeutics pioneer, Steven Dowdy, Ph.D., appointed to the NeuBase Scientific Advisory Board

Financial Results for the Fiscal Quarter Ended December 31, 2019:

- For the three month period ended December 31, 2019, the Company reported a net loss of approximately $4.5 million, or a net loss of $0.26 per share, compared with a

net loss of approximately $1.5 million, or a net loss of $0.25 per share, for the same period last year.

- For the three month period ended December 31, 2019, total operating expenses were approximately $3.8 million, consisting of approximately $2.6 million in general and administrative expenses and $1.2 million of research and development expenses. This compares with total operating expenses of $1.5 million for the same period last year, which was comprised of approximately $0.4 million in general and administrative expenses and $1.1 million in research and development and research and development-licenses acquired expenses.
- At December 31, 2019, the Company had cash and cash equivalents of approximately $7.7 million, compared with cash and cash equivalents of approximately $10.3 million at September 30, 2019. The Company believes that its current cash balance will provide sufficient capital to fund operations through the end of fiscal year 2020.

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 for completing and reporting data from preclinical studies evaluating the pharmacokinetic (“PK”) and pharmacodynamic (“PD”) properties of our PATrOL™ platform and PATrOL™-enabled candidates, 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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