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

Strong Pharmacokinetic and Pharmacodynamic Data Presented in March Validate Platform and Position Company for Scalable Output of Synthetic Precision Genetic Medicines

Company Continues to Progress Candidates in Huntington's Disease (HD) and Myotonic Dystrophy (DM1)

PITTSBURGH, Aug. 13, 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 nine month periods ended June 30, 2020.

"We are pleased with the continued execution of our development programs during 2020. This includes the announcement in late-March of compelling data that firmly validate our platform as a viable fully synthetic approach to genetic medicine," said Dietrich A. Stephan, Ph.D., chief executive officer of NeuBase. "Notably, these data confirm that our therapies penetrate into the brain when administered systemically – overcoming one of the grand challenges of drug delivery. PATrOL-enabled compounds can also access tissues throughout the entire body, opening our platform up to unexplored indications that have not previously been accessible by genetic medicine technologies. These positive pharmacokinetic and pharmacodynamic data position our unique technology to output a vast pipeline of therapeutics to resolve innumerable human diseases. We anticipate presenting additional new data with respect to our ongoing progress in the fourth calendar quarter of this year."

"A key objective for our company shortly after the March data announcement was to strengthen our balance sheet in order to fully advance our strategies in HD and DM1, and build out our pipeline. This was accomplished in April with the closing of our oversubscribed capital raise of approximately \$33.3 million in net proceeds that was led by fundamental healthcare investors and significantly increased our institutional shareholder base. We expect this to support our R&D and general corporate expenses into the second calendar quarter of 2022," continued Dr. Stephan.

Third Fiscal Quarter of 2020 and Recent Operating Highlights

- Expanded the senior management team with the appointment of industry veteran Dr. William Mann as Chief Operating Officer
- Strengthened the balance sheet through an oversubscribed public offering in the third quarter of fiscal year 2020 for net proceeds of approximately \$33.3 million, which will

support the continued development of the Company's therapeutic programs and pipeline expansion

Financial Results for the Fiscal Quarter Ended June 30, 2020:

- At June 30, 2020, the Company had cash and cash equivalents of approximately \$35.9 million, compared with cash and cash equivalents of approximately \$10.3 million at September 30, 2019;
- For the three month period ended June 30, 2020, the Company reported a net loss of approximately \$3.8 million, or a net loss of \$0.18 per share, compared with a net loss of approximately \$2.0 million, or a net loss of \$0.38 per share, for the same period last year; and
- For the three month period ended June 30, 2020, total operating expenses were approximately \$3.8 million, consisting of approximately \$2.3 million in general and administrative expenses and \$1.5 million of research and development expenses. This compares with total operating expenses of \$2.0 million for the same period last year, which was comprised of approximately \$1.7 million in general and administrative expenses and \$0.3 million in research and development expenses.

Financial Results for the Nine Month Period Ended June 30, 2020:

- For the nine month period ended June 30, 2020, the Company reported a net loss of approximately \$12.7 million, or a net loss of \$0.69 per share, compared with a net loss of approximately \$5.6 million, or a net loss of \$0.96 per share, for the same period last year; and
- For the nine month period ended June 30, 2020, total operating expenses were approximately \$12.0 million, consisting of approximately \$7.6 million in general and administrative expenses and \$4.3 million of research and development expenses. This compares with total operating expenses of \$5.4 million for the same period last year, which was comprised of approximately \$4.0 million in general and administrative expenses and \$1.4 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, our capital and liquidity outlook, 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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