August 12, 2021

NeuBase Therapeutics Reports Financial Results for the Third Quarter of Fiscal Year 2021 and Recent Operating Highlights

- *Further demonstrated broad potential of novel genetic medicine platform with in vivo data in three diseases driven by different genetic mechanisms of disease*
- *Presented preclinical data showing compounds enabled with NeuBase's proprietary delivery technology are well tolerated at pharmacologically active doses and are delivered beyond the liver after systemic administration*
- *Recent data support advancement of development of the myotonic dystrophy type 1 (DM1) program; IND filing expected in the fourth quarter of CY 2022*
- *Cash runway expected to fund currently planned operating and capital expenditures into the first quarter of CY 2023*

PITTSBURGH, Aug. 12, 2021 (GLOBE NEWSWIRE) -- NeuBase Therapeutics, Inc. (Nasdaq: NBSE) ("NeuBase" or the "Company"), a biotechnology platform company Drugging the Genome™ to address disease at the base level using a new class of precision genetic medicines, today reported its financial results for the three- and nine-month periods ended June 30, 2021.

"In June, we presented preclinical *in vivo* data of novel compounds demonstrating selective silencing of disease-causing mutations at the DNA or RNA level in three diseases, each of which is caused by a different underlying genetic mechanism. These new data further illustrate the broad applicability of our genetic medicine platform," said Dietrich A. Stephan, Ph.D., Founder, CEO, and Chairman of NeuBase. "Following intravenous or subcutaneous dosing, these compounds were well tolerated at pharmacologically active doses. In addition, the compounds achieved targeted delivery into brain and muscle, which further support our claim of offering the unique ability to deliver genetic medicines throughout the body."

"For our lead program in DM1, recent data support a differentiated therapeutic approach to maintain DMPK function while selectively silencing the disease-driving mutation. With these positive data in hand, we believe we have a clear path towards entering the clinic and are planning for an IND filing in the fourth quarter of calendar year 2022," continued Dr. Stephan. "We are continuing to advance our therapeutic program for Huntington's disease and we believe our proprietary delivery technology will allow our compounds to advance beyond intrathecal delivery, overcoming challenges seen with other programs."

Dr. Stephan concluded, "Finally, we have shown that we can silence activating *KRAS* point mutations *in vivo* to inhibit protein production, which has the potential to target G12D and G12V, the two most common and historically 'undruggable' *KRAS* driver mutations that represent the majority of KRAS-driven tumors. This sets the stage for generating new precision genetic medicines capable of selectively targeting mutations at the single-base level to treat both rare and common diseases."

Third Quarter of Fiscal Year 2021 and Recent Operating Highlights

- Completed an oversubscribed public offering led by noted fundamental healthcare investors for net proceeds of $42.6 million, providing a cash runway into CY 2023.
- Presented preclinical data demonstrating:
    - Proprietary genetic medicines platform generates novel compounds that selectively silence disease-driving genetic mutations *in vivo* without permanently modifying the genome
    - Functional rescue of myotonic dystrophy type 1 (DM1) phenotype*in vivo* after subcutaneous dosing, as well as tolerability at pharmacologically active doses
    - *In vivo* proof of concept in Huntington's disease with allele-selective mutant protein knock-down after delivering compounds across the blood-brain barrier following subcutaneous dosing
    - Pharmacologic activity against historically 'undruggable' KRAS driver mutations in a variety of patient-derived tumor lines in xenograft models
    - Penetration of the blood-brain barrier with pharmacologic activity in the CNS and additional broad tissue distribution and activity of compounds after subcutaneous administration
    - Compounds are well tolerated, and consistent with historical data
- Expanded the executive management team with the appointment of Sandra Rojas-Caro, M.D., as Chief Medical Officer to oversee the preclinical and clinical development, medical, and regulatory strategy of the Company's pipeline; and Kia Motesharei, Ph.D., as Chief Business and Strategy Officer to oversee business development and alliance management and work with the CEO on corporate strategy

Financial Results for the Third Fiscal Quarter Ended June 30, 2021

- As of June 30, 2021, the Company had cash and cash equivalents of approximately $58.8 million, compared with approximately $32.0 million as of September 30, 2020
- 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 June 30, 2021, the Company reported a net loss of approximately $8.7 million, or a net loss of $0.29 per share, compared with a net loss of approximately $3.8 million, or a net loss of $0.18 per share, for the three-month period ended June 30, 2020
- For the three-month period ended June 30, 2021, total operating expenses were approximately $8.8 million, consisting of approximately $3.5 million in general and administrative expenses, $2.5 million of research and development expenses and $2.9 million in research and development- Vera acquisition expenses. This compares with total operating expenses of approximately $3.8 million for the three-month period ended June 30, 2020, consisting of approximately $2.3 million in general and administrative expenses, and $1.5 million in research and development expenses

Financial Results for the Nine-Month Period Ended June 30, 2021

- For the nine-month period ended June 30, 2021, the Company reported a net loss of approximately $18.3 million, or a net loss of $0.72 per share, compared with a net loss of approximately $12.7 million, or a net loss of $0.69 per share, for the same period last year
- For the nine-month period ended June 30, 2021, total operating expenses were

approximately $19.4 million, consisting of approximately $8.8 million in general and administrative expenses, $7.7 million of research and development expenses, $2.9 million in research and development- Vera acquisition expenses. This compares with total operating expenses of approximately $12.0 million for the same period last year, consisting of approximately $7.6 million in general and administrative expenses and $4.3 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 neuromuscular, neurological 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 prospects of DM1 and the Company's expectation to make an IND filing for DM1 in the fourth quarter of CY 2022, the Company's therapeutic program for Huntington's disease, the Company's ability to target G12D and G12V and the Company's expectation that its cash will fund currently planned operating and capital expenditures into the first quarter of CY 2023. 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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