February 10, 2022

NeuBase Therapeutics Reports Business Update and Financial Results for the First Quarter of Fiscal Year 2022

- *Investigational New Drug (IND)-enabling studies for the myotonic dystrophy type 1 (DM1) program development candidate continue as planned; invited to present new rodent pharmacokinetic (PK) and bioavailability data in an oral presentation at an upcoming scientific meeting; expects to submit an IND application to the U.S. Food and Drug Administration (FDA) in the fourth quarter (Q4) of calendar year (CY) 2022*
- *Good Manufacturing Practice (GMP) manufacturing to support Phase 1/2 clinical trials for DM1 program successfully implemented*
- *Expects to nominate a development candidate and initiate scale-up and toxicology activities for a systemically administered, allele-selective candidate for the Huntington's Disease (HD) program in CY2022 with a goal of filing an IND application with the FDA in CY2023*
- *Progressed KRAS G12D and G12V oncology programs supported with mechanistic work and in vivo pharmacology*
- *Strengthened senior management team with appointment of Todd P. Branning as Chief Financial Officer (CFO)*

PITTSBURGH and CAMBRIDGE, Mass., Feb. 10, 2022 (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-month period ended December 31, 2021, and other recent developments.

"We are making significant progress in advancing the IND-enabling studies for the development candidate for our myotonic dystrophy type 1 (DM1) program, and we expect to file an IND with the FDA in Q4 CY2022," said Dietrich A. Stephan, Ph.D., Founder, Chief Executive Officer, and Chairman of NeuBase. "These studies are on track for data readouts to occur throughout CY2022, with the first presentation of rodent pharmacokinetic and bioavailability data to occur at an upcoming scientific meeting. We expect these data to illustrate the differentiated potential for our candidate to be a whole-body solution to treat DM1 and the unique ability of our delivery shuttle for distribution into the brain. The ability to cross the blood brain barrier and reach the deep brain is also especially relevant for our Huntington's disease program, where we are planning to initiate scale-up and toxicology activities this year."

Dr. Stephan added, "In addition, I'm especially excited to have welcomed Todd to the executive team as Chief Financial Officer. The team and science are strong, and I believe we are at a pivotal moment for NeuBase as we are building a robust data package that is expected to support bringing our first candidate into the clinic for DM1, validate our genetic medicine technology platform to efficiently deliver genetic medicines with broad tissue

distribution, including into the deep brain, and to precisely engage genetic mutations in a manner that is well-tolerated with the potential for sustained efficacy."

First Quarter of Fiscal Year 2022 and Recent Operating Highlights

- **Myotonic Dystrophy Type 1 (DM1) Program:** NeuBase is making steady progress advancing IND-enabling studies for its development candidate in the DM1 program, which includes PK, absorption, distribution, metabolism, and excretion (ADME), and bioavailability via intravenous (IV) and subcutaneous (SQ) routes of administration, exploratory and IND-enabling Good Laboratory Practice (GLP) toxicology, and mechanism of action studies. In addition, GMP manufacturing of NeuBase's development candidate to support Phase 1/2 clinical trials has been successfully implemented via contract manufacturing organizations. The Company plans to announce a robust data package through posters and presentations at scientific meetings and peer-reviewed publications throughout CY2022. NeuBase expects these data to support the submission of an IND filing to the FDA in the Q4 CY2022.
- **Huntington's Disease (HD) Program:** The HD program is currently in preclinical development. In CY2022, NeuBase expects to nominate a development candidate and initiate scale-up and toxicology activities to support an IND filing to the FDA in CY2023.
- ***KRAS* Oncology Program:** The Company is conducting *in vitro* mechanistic studies and *in vivo* pharmacology studies for the *KRAS* program (*KRAS* G12V and G12D mutations).
- **Appointed New Chief Financial Officer:** The Company appointed Todd P. Branning as CFO. Mr. Branning brings more than 25 years of experience leading corporate finance and accounting, tax, financial planning and analysis, and investor relations for several publicly traded pharmaceutical companies.

Financial Results for the Fiscal Quarter Ended December 31, 2021

- As of December 31, 2021, the Company had cash and cash equivalents of approximately $47.3 million, compared with approximately $52.9 million as of September 30, 2021.
- 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 fiscal quarter ended December 31, 2021, the Company reported a net loss of approximately $7.7 million, or a net loss of $0.24 per share, compared with a net loss of approximately $4.1 million, or a net loss of $0.18 per share, for the same period last year.
- For the fiscal quarter ended December 31, 2021, total operating expenses were approximately $7.3 million, consisting of approximately $2.9 million in general and administrative expenses and $4.4 million of research and development expenses. This compares with total operating expenses of approximately $4.7 million for the same period last year, consisting of approximately $2.7 million in general and administrative expenses and $2.0 million in research and development expenses.

About NeuBase Therapeutics

NeuBase is accelerating the genetic revolution by developing a new class of precision genetic medicines that Drug the Genome™. The Company's therapies are built on a proprietary platform called PATrOL™ that encompasses a novel peptide-nucleic acid antisense oligonucleobase technology combined with a novel delivery shuttle that overcome

many of the hurdles to selective mutation engagement, repeat dosing, and systemic delivery of genetic medicines. 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, who currently have limited to no treatment options. 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 our expectations to submit an IND filing to the FDA for our DM1 program in 4Q CY2022 and our other expectations for our DM1 program, our expectation to initiate scale-up and toxicology activities for development of a systemically administered allele-selective NT-0100 program to treat HD in CY2022 and targeting an IND filing for this program to the FDA for CY2023, the potential of our therapeutic program for HD and the potential for our PATrOL™-enabled compounds to silence activating *KRAS* point mutations *in vivo* to inhibit protein production. 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.

NeuBase Investor Contact:
Dan Ferry
Managing Director

LifeSci Advisors, LLC
daniel@lifesciadvisors.com
OP: (617) 430-7576

NeuBase Media Contact:
Jessica Yingling, Ph.D.
Little Dog Communications Inc.
(858) 344-8091
jessica@litldog.com

neubase

Source: NeuBase Therapeutics, Inc.