May 12, 2022

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

- *Investigational New Drug (IND)-enabling studies continue to generate promising data that support further development of the company's lead myotonic dystrophy type 1 (DM1) candidate; new preclinical data to be presented at the American Society of Gene and Cell Therapy (ASGCT) 25th Annual Meeting; on track to submit a planned IND application to the U.S. Food and Drug Administration (FDA) in the fourth quarter of calendar year (CY) 2022*
- *On track 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*
- *Progressed KRAS G12D and G12V oncology programs supported with mechanistic work and in vivo pharmacology*

PITTSBURGH and CAMBRIDGE, Mass., May 12, 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 March 31, 2022, and other recent developments.

"We are pleased with the progress being made across our development pipeline of therapeutic programs to treat DM1, HD, and *KRAS*-driven cancers. The new data we've announced to date this year have further validated the use of our PATrOL™ platform to design novel genetic medicines that target and rescue gene dysfunctions, with the potential for clinically impactful outcomes in both rare and common diseases," said Dietrich A. Stephan, Ph.D., Founder, Chief Executive Officer, and Chairman of NeuBase. "We continue to execute the development strategy for our DM1 program, which includes a series of IND-enabling studies scheduled to report data throughout CY2022. Last quarter, we presented pharmacodynamic data that illustrated a single intravenous (IV) dose or multiple subcutaneous (SC) doses of our DM1 development candidate resolves the genetic defect and myotonia in skeletal muscle of the gold-standard mouse model of the disease. Building off these results, we plan on announcing at ASGCT additional pharmacokinetic (PK) data, which will illustrate the exposure levels of our development candidate when administered via systemic administration in skeletal muscles, heart, and brain, tissues that are affected in DM1. We expect these data to support further advancement of our lead candidate for DM1 and validate a differentiated whole-body solution for this disease. Considering this progress, we believe the submission of an IND application to the FDA is on track for the fourth quarter of CY2022."

Second 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 IV and SC routes of administration, exploratory and IND-enabling Good Laboratory Practice (GLP) toxicology, and mechanism of action studies. In addition, Good Manufacturing Practice (GMP) of NeuBase's development candidate to support Phase 1/2 clinical trials has been successfully implemented via contract manufacturing organizations.
    - In March 2022, the Company presented a robust data package through posters and presentations at the 2022 MDA Clinical & Scientific Conference demonstrating that systemic administration of the Company's lead DM1 candidate, NT-0231.F, in the HSA^{LR} model achieves clinically relevant molecular and functional rescue, including genetic target engagement, displacement of sequestered MBNL1, resolution of nuclear aggregates, rescue of the spliceopathy, and reversal of myotonia (delayed muscle relaxation after contraction). In PK studies of NT-0231.F in wild-type BALB/C mice, a single IV or SC dose showed high volume of distribution, suggesting wide tissue distribution.
    - The Company announced that two abstracts have been accepted for presentation at the ASGCT 25th Annual Meeting. The presentations will include new preclinical data on the biodistribution in key tissues of NT-0231.F.
    - With continued positive results from *in vitro* and *in vivo* preclinical studies, the Company expects to file an IND application for NT-0231.F in the fourth quarter of CY2022.
- **Huntington's Disease (HD) Program:** The HD program is currently in preclinical development. In CY2022, NeuBase expects to present new preclinical data describing the pharmacology of a candidate compound in the brain after systemic administration, nominate a development candidate and initiate scale-up and toxicology activities.
- ***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). Existing *in vivo* data show activity illustrating allele-selective engagement of mutant *KRAS* at the DNA and RNA levels, with abrogation of downstream hyperactive signaling through multiple RAS pathway members, resulting in anti-tumor activity.

Financial Results for the Second Fiscal Quarter Ended March 31, 2022

- As of March 31, 2022, the Company had cash and cash equivalents of approximately $39.0 million, compared with approximately $52.9 million as of September 30, 2021.
- For the fiscal quarter ended March 31, 2022, the Company reported a net loss of approximately $9.9 million, or a net loss of $0.30 per share, compared with a net loss of approximately $5.5 million, or a net loss of $0.24 per share, for the same period last year.
- For the fiscal quarter ended March 31, 2022, total operating expenses were approximately $9.9 million, consisting of approximately $3.1 million in general and administrative expenses and $6.8 million of research and development expenses. This compares with total operating expenses of approximately $5.9 million for the same period last year, consisting of approximately $2.7 million in general and administrative expenses and $3.2 million in research and development expenses.

Financial Results for the Six-Month Period Ended March 31, 2022

- For the six-month period ended March 31, 2022, the Company reported a net loss of approximately $17.7 million, or a net loss of $0.54 per share, compared with a net loss of approximately $9.6 million, or a net loss of $0.41 per share, for the same period last year.
- For the six-month period ended March 31, 2022, total operating expenses were approximately $17.2 million, consisting of approximately $6.0 million in general and administrative expenses and $11.2 million of research and development expenses. This compares with total operating expenses of approximately $10.6 million for the same period last year, consisting of approximately $5.4 million in general and administrative expenses and $5.2 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 the plan to provide updates on the Company's development pipeline, in particular the DM1 program, at the ASGCT 25th Annual Meeting, the potential and prospects of the Company's proprietary PATrOL™ platform and DM1 program, the Company's expectation that it will submit an IND application for the DM1 program to the U.S. Food and Drug Administration in the fourth quarter of CY2022, our expectations to initiate scale-up and toxicology activities for development of a systemically administered allele-selective NT-0100 program to treat HD in CY2022, 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.

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Source: NeuBase Therapeutics, Inc.