August 11, 2022

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

- *Lead myotonic dystrophy type 1 (DM1) candidate continues to show promising preclinical data, including the pharmacokinetics (PK) and biodistribution data presented at the American Society of Gene and Cell Therapy (ASGCT) 25th Annual Meeting supporting a differentiated whole-body treatment solution for DM1*
- *Management decision to conduct additional preclinical studies expected to further strengthen the planned Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA), expected to be submitted in mid-calendar year (CY) 2023*
- *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*
- *Company expects to present preclinical data for the HD program at upcoming scientific conferences*

PITTSBURGH and CAMBRIDGE, Mass., Aug. 11, 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 June 30, 2022, and other recent developments.

"Thus far in 2022, we have generated new data that strengthens application of the PATrOL™ platform and its potential to output novel genetic medicines that are broadly biodistributed after systemic routes of administration and engage double-stranded nucleic acid targets in the nucleus to resolve gene dysfunction. This includes new PK and biodistribution data for our DM1 development candidate, which were presented at the ASGCT 25th Annual Meeting, that support a differentiated whole-body solution for DM1 patients," said Dietrich A. Stephan, Ph.D., Founder, Chief Executive Officer, and Chairman of NeuBase. "The data in each tissue we evaluated also displayed an extended elimination phase with tissue concentrations measurable for at least four weeks following a single intravenous (IV) dose administration and consistent with the durable pharmacology in the transgenic animal models. With these data in hand, we decided to perform additional IND-enabling studies to further characterize our development candidate and confirm our expectations of safety and efficacy. While conducting these additional studies will require us to push our guidance on the timing for our DM1 IND filing to mid-CY2023, patient safety and benefit are of utmost importance to us. We also believe the information that will be generated by these studies will benefit not only our DM1 program but all of our programs and better prepare us to enter the clinic and fulfill our mission of delivering new medicines to patients who currently have no therapeutic options."

Dr. Stephan continued, “We continue to generate positive data from other programs in our pipeline, including in HD, that support the ability of the PATrOL™ platform to develop differentiated genetic medicines. We are confident in the ability of our platform and team to advance these drug candidates to clinical trials, and we look forward to providing further updates on our progress.”

Third 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 subcutaneous 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 May 2022, the Company presented preclinical PK and biodistribution data at the ASGCT 25th Annual Meeting for its lead development candidate, NT-0231.F, supporting a differentiated whole body treatment solution for DM1. Following a single IV injection of 30 mg/kg in wild-type BALB/c mice, NT-0231.F was cleared rapidly from the systemic compartment and demonstrated rapid and wide distribution into tibialis anterior muscle, heart muscle, and brain tissues. NT-0231.F rapidly cleared the plasma, and each tissue evaluated displayed an extended elimination phase with tissue concentrations measurable for at least four weeks following a single IV dose administration.
    - Completed exploratory toxicology work in non-human primates and rats.
    - Initiated a collaboration with Inserm to further study NT-0231.F using the DMSXL mouse model to measure central nervous system and heart pharmacology and to refine the human equivalent dose.
    - Committed to support the Myotonic Dystrophy Clinical Research Network, the world’s largest patient organization focused solely on myotonic dystrophy.
    - The Company expects to submit an IND application to the FDA for NT-0231.F in mid-CY2023.
- **Huntington’s Disease (HD) Program:** Preclinical development activities in the Company’s HD program progressed during the second quarter of CY2022. During the second half of CY2022, NeuBase expects to present new preclinical data at upcoming scientific conferences 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 continued *in vitro* mechanistic studies and *in vivo* pharmacology studies for the *KRAS* program (*KRAS* G12V and G12D mutations) to support the development of an allele-selective approach to engaging mutant *KRAS* at the DNA and RNA levels.
- **Management Update:** William (Bill) Mann, Ph.D. was promoted to President of the Company. Dr. Mann continues to serve as NeuBase’s Chief Operating Officer.

Financial Results for the Third Fiscal Quarter Ended June 30, 2022

- As of June 30, 2022, the Company had cash and cash equivalents of approximately

$29.8 million, compared with approximately $52.9 million as of September 30, 2021.

- For the fiscal quarter ended June 30, 2022, the Company reported a net loss of approximately $8.5 million, or a net loss of $0.26 per share, compared with a net loss of approximately $8.7 million, or a net loss of $0.29 per share, for the same period last year.
- For the fiscal quarter ended June 30, 2022, total operating expenses were approximately $8.4 million, consisting of approximately $3.6 million in general and administrative expenses and $4.8 million of research and development expenses. This compares with total operating expenses of approximately $8.8 million for the same period last year, consisting of approximately $3.5 million in general and administrative expenses and $5.3 million in research and development expenses.

Financial Results for the Nine-Month Period Ended June 30, 2022

- For the nine-month period ended June 30, 2022, the Company reported a net loss of approximately $26.1 million, or a net loss of $0.80 per share, compared with a net loss of approximately $18.3 million, or a net loss of $0.72 per share, for the same period last year.
- For the nine-month period ended June 30, 2022, total operating expenses were approximately $25.6 million, consisting of approximately $9.6 million in general and administrative expenses and $16.0 million of research and development expenses. This compares with total operating expenses of approximately $19.4 million for the same period last year, consisting of approximately $8.8 million in general and administrative expenses and $10.6 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 potential and prospects of the Company's proprietary PATrOL™ platform, DM1, HD and KRAS programs, including the plan to provide updates to the HD program at scientific conferences in CY2022 and 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 middle of CY2023. 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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