December 23, 2021

NeuBase Therapeutics Reports Business Update and Financial Results for Fiscal Year 2021

- *Validated ability to Drug the Genome™ to address both rare and common diseases without the limitations of early precision genetic medicine technologies; the Company's delivery shuttle enables pharmacology across programs in multiple tissues, including in the brain and muscle, after subcutaneous administration*
- *Nominated the development candidate for the myotonic dystrophy type 1 (DM1) program with potential for best-in-class features; initiated IND-enabling studies; and expect to submit an IND filing to the FDA in 4Q CY2022*
- *Established clinical development and CMC teams at new Cambridge, Mass. site; finalized formulation for systemic routes; and scaled-up manufacturing to support GLP toxicology and Phase 1/2 clinical trials for the DM1 program*
- *Expect to initiate scale-up and toxicology activities for the development of a systemically administered allele-selective NT-0100 program to treat Huntington's disease (HD) in CY2022; and targeting an IND filing to the FDA for CY2023*
- *Initiated KRAS G12D and G12V programs supported with* in vivo *pharmacology; and finalized a proprietary mutational database to prioritize pipeline expansion and partnering opportunities*

PITTSBURGH and CAMBRIDGE, Mass., Dec. 23, 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 fiscal year ended September 30, 2021, and other recent developments.

"NeuBase is focused on significantly reducing the burden of untreatable morbidity and mortality caused by rare and common diseases across the globe. To achieve this goal, we designed, built, and validated a new precision genetic medicines platform technology that can uniquely drug the double-stranded human genome and address disease at the root of causality without many of the limitations of early precision genetic medicine technologies. We are poised to file our first Investigational New Drug ('IND') applications with the U.S. Food and Drug Administration ('FDA') beginning in calendar year 2022 and intend to scale into additional indications with increasing speed and efficiency thereafter," said Dietrich A. Stephan, Ph.D., Founder, Chief Executive Officer, and Chairman of NeuBase.

"This past year, we validated the ability of our technology in proof-of-concept studies to directly drug the double-helix of the human genome, including difficult double-stranded structures of RNA targets, and engage with mutant genes to resolve most causal mechanisms of disease. The validation of our platform's capabilities included data describing that we have overcome many limitations of early precision genetic medicine technologies, such as biodistribution, tolerability, selectivity, manufacturability, durability, and

scalability. We also presented data that our delivery shuttle enables compounds to elicit pharmacologic effects in multiple tissues, including in the brain and muscle, after subcutaneous administration in preclinical animal models," said William Mann, Ph.D., M.B.A., Chief Operating Officer of NeuBase.

"We recently nominated the development candidate for our myotonic dystrophy type 1 (DM1) program, which we believe has the potential to be a best-in-class therapy that offers a patient-friendly route of administration, a whole-body solution for the muscle, heart, and brain manifestations of the disease. Furthermore, the mechanism of action of our development candidate is designed to engage with the toxic RNA hairpin structure to release the splicing proteins, restoring normal RNA splicing and downstream protein production, including DMPK. We have initiated IND-enabling studies for this candidate, with data read-outs expected across CY2022. We expect these data will support the submission of an IND filing to the FDA in the fourth quarter of CY2022," stated Sandra Rojas-Caro, M.D., Chief Medical Officer of NeuBase.

"As a result of the nomination of our DM1 program candidate, we established CMC expertise at our new facility in Cambridge, Massachusetts that is co-located with our clinical development team, finalized the formulation of our development candidate to enable systemic routes, and completed process development. We also scaled-up manufacturing in-house and with contract manufacturing partners to support non-clinical toxicology, product stability, and Phase 1/2 clinical trials," said Tony Rossomando, Ph.D., Chief Technology Officer of NeuBase.

Dr. Stephan concluded, "In parallel, we are making significant progress in our therapeutic program for Huntington's disease. For example, we have illustrated with preclinical *in vivo* data that our proprietary delivery technology allows our genome-targeting compounds to advance beyond intrathecal delivery and enabling a systemically administered allele-selective therapy, overcoming challenges seen with other programs. Furthermore, preclinical data show that our PATrOL™-enabled compounds 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' cancer-driving point mutations that represent the majority of *KRAS* tumors. We believe these data set the stage for a potentially first-in-class precision genetic medicine approach for oncology capable of selectively targeting mutations at the single-base level."

Fourth Quarter of Fiscal Year 2021 and Recent Operating Highlights

- **Myotonic Dystrophy Type 1 (DM1) Program:** NeuBase recently nominated its development candidate for the DM1 program and initiated chemistry manufacturing controls ("CMC") scale-up for IND-enabling toxicology and Phase 1/2 clinical trials. In CY2022, NeuBase plans to conduct pharmacokinetic and absorption, distribution, metabolism, excretion (PK/ADME) and bioavailability (IV/SQ), exploratory toxicology, IND-enabling GLP toxicology, and mechanism of action studies, which are expected to support an IND filing to the FDA in the fourth quarter of CY2022.
- **Huntington's Disease (HD) Program:** The NT-0100 program is currently in preclinical development as a potential treatment for HD. In CY2022, NeuBase expects to initiate scale-up and toxicology activities to support an IND filing to the FDA in CY2023.
- **KRAS Oncology Program:** NeuBase expanded its pipeline into oncology with the advancement of the *KRAS* program (*KRAS* G12V and G12D mutations) from concept

into *in vivo* proof-of-principle.
- **Genetic Target Prioritization:** The Company finalized a rank-ordered mutational database. All available monogenic and cancer-causing mutations have been ranked for internal pipeline expansion and prioritize partnering opportunities.

Financial Results for the Fiscal Year Ended September 30, 2021

- As of September 30, 2021, the Company had cash and cash equivalents of approximately $52.9 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 fiscal year ended September 30, 2021, the Company reported a net loss of approximately $25.4 million, or a net loss of $0.93 per share, compared with a net loss of approximately $17.4 million, or a net loss of $0.89 per share, for the same period last year
- For the fiscal year ended September 30, 2021, total operating expenses were approximately $26.6 million, consisting of approximately $12.2 million in general and administrative expenses, $11.5 million of research and development expenses, and $2.9 million in research and development expenses related to the acquisition of assets of Vera Therapeutics, Inc. This compares with total operating expenses of approximately $17.1 million for the same period last year, consisting of approximately $10.1 million in general and administrative expenses and $6.9 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 oligonucleotide technology combined with novel delivery shuttles 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, among other statements, our expectations to submit an IND filing to the FDA for our myotonic dystrophy type 1 (DM1) program in 4Q CY2022 and our other expectations for our myotonic dystrophy type 1 (DM1) program, our expectation to initiate scale-up and toxicology activities for development of a systemically administered allele-selective NT-0100 program to treat Huntington's Disease (HD) in CY2022 and targeting an IND filing for this program to the FDA for CY2023, the potential of our therapeutic program for Huntington's disease 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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