May 17, 2022

NeuBase Presents New Preclinical Data at ASGCT 2022 for Its DM1 Program Demonstrating Wide Tissue Distribution and Supporting a Differentiated Whole-Body Treatment Solution

PITTSBURGH and CAMBRIDGE, Mass., May 17, 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 announced the presentation of preclinical pharmacokinetics (PK) and biodistribution data for its lead development candidate, NT-0231.F, supporting a differentiated whole body treatment solution for myotonic dystrophy type 1 (DM1). These new data are being presented today in a poster session at the American Society of Gene and Cell Therapy (“ASGCT”) 25th Annual Meeting, taking place virtually and in person in Washington, D.C., May 16-19, 2022.

Following a single intravenous (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.

Sandra Rojas-Caro, M.D., Chief Medical Officer of NeuBase, said, “We continue to build a robust and compelling data set potentially supporting a whole body treatment solution for DM1. These PK and biodistribution data presented today show IV administration of our lead candidate results in exposure in the major tissues that are affected by DM1. We believe the ability of our drug candidate to reach skeletal and heart muscles as well as brain tissue may potentially deliver a differentiated therapeutic approach to not only treat the myotonia, muscle weakness, and cardiac effects but also the cognitive impairments seen in DM1. We have now demonstrated in preclinical models that our lead candidate NT-0231.F achieves clinically relevant molecular and functional rescue as well as whole-body distribution.”

The data will be presented today from 5:30 pm to 6:30 pm ET in a poster entitled, “Pharmacokinetics, Biodistribution, and CNS Penetration of a PATrOL™-Enabled Investigational Genetic Therapy for Myotonic Dystrophy Type 1 Following Systemic Administration in Mice.”

Further details of the presentation and the poster can be foundhere.

About NeuBase’s DM1 Program

Patients with DM1 suffer from cognitive deficits and muscle pathology caused by a trinucleotide expansion in the DMPK gene which, when transcribed, results in an RNA hairpin structure that sequesters RNA splice proteins. NeuBase's DM1 investigational genetic therapy, NT-0231.F, targets mutant DMPK pre-mRNA with a novel peptide-nucleic acid (PNA) pharmacophore and is designed to selectively engage with the toxic RNA hairpin structure, release the splicing proteins, and restore RNA splicing and downstream protein production. The PNA pharmacophore is conjugated to NeuBase's novel delivery technology that is designed for broad distribution, including into the deep brain, with the potential for a whole body, disease-modifying solution for DM1. For more information, visit https://www.neubasetherapeutics.com/pipeline/.

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, the presentation of data for the myotonic dystrophy type 1 (DM1) program at the American Society of Gene and Cell Therapy ("ASGCT") 25th Annual Meeting and the potential and prospects of the Company's proprietary PATrOL™ platform and the Company's DM1 program. 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.
President
Little Dog Communications Inc.
jessica@litldog.com
OP: (858) 344-8091

neubase

Source: NeuBase Therapeutics, Inc.