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NeuBase Therapeutics Announces Presentations at the American Society of Gene & Cell Therapy (ASGCT) 2022 Annual Meeting

PITTSBURGH and CAMBRIDGE, Mass., May 02, 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 two abstracts have been accepted for presentation 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.

NeuBase will present new preclinical data on the biodistribution in key tissues of the company's lead candidate, NT-0231.F, a precision genetic medicine in development to treat myotonic dystrophy, type 1 ("DM1"). NeuBase recently presented data at the 2022 MDA Clinical & Scientific Conference demonstrating that systemic administration of NT-0231.F in the HSA^{LR} model achieves clinically relevant molecular and functional rescue, including rescue of the spliceopathy, resolution of nuclear aggregates, and reversal of myotonia (delayed muscle relaxation after contraction). Pharmacokinetic studies of NT-0231.F in wild-type BALB/C mice showed a single IV or SC dose achieved high volume of distribution.

The presentations are listed below, and the full abstracts are available on theASGCT meeting website. All times are listed in Eastern Time (ET).

Title: Pharmacokinetics, Biodistribution, and CNS Penetration of a PATrOL™-Enabled Investigational Genetic Therapy for Myotonic Dystrophy-Type 1 Following Systemic Administration Systemic Administration in Mice
Presenter: Noel Monks, Ph.D.
Session Date/Time: Tuesday, May 17, 5:30 PM - 6:30 PM
Poster Board Number: Tu-90
Session Title: Oligonucleotide Therapeutics II
Room: Hall D
Abstract Number: 585

Title: Pharmacology, Biodistribution and Tolerability of a PATrOL™-Enabled Investigational Genetic Therapy for Myotonic Dystrophy, Type 1
Presenter: Sandra Rojas-Caro, M.D.
Session Date/Time: Wednesday, May 18, 5:30 PM - 6:30 PM
Poster Board Number: W-175
Session Title: Musculo-skeletal Diseases
Room: Hall D
Abstract Number: 1049

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 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 oligonucleotide 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 and the Company's expectation that it will submit an Investigational New Drug application for the DM1 program to the U.S. Food and Drug Administration in the fourth quarter of calendar year 2022. 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 ("SEC"), 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 SEC. 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

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