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NeuBase Therapeutics Presents New Preclinical Data at MDA 2022 for Its Myotonic Dystrophy Type 1 Program Demonstrating Splice Rescue, Nuclear Aggregate Resolution, and Myotonia Reversal

- *A single intravenous dose of NeuBase's Myotonic Dystrophy Type 1 (DM1) development candidate, NT-0231.F, potently rescues splicing, restores chloride channel (Clcn1) function, and durably reverses myotonia in the HSA^{LR} model*
- *Multiple subcutaneous doses also lead to molecular and functional rescue in the HSA^{LR} model demonstrating efficacy in a dose-dependent manner and potential feasibility of this patient-friendly administration route*
- *Company remains on track to submit an Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA) in 4Q CY2022*
- *NeuBase management to host a conference call and webcasttomorrow, March 14, at 8:00 a.m. EDT*

PITTSBURGH and CAMBRIDGE, Mass., March 13, 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 new preclinical data for its lead development candidate, NT-0231.F, to treat myotonic dystrophy type 1 (DM1). These data are being presented in poster and oral presentations at the 2022 MDA Clinical & Scientific Conference, which is taking place virtually and in-person in Nashville, Tennessee from March 13-16, 2022. These data will also be presented at the RNA Leaders World Congress in Basel on March 17th.

Sandra Rojas-Caro, M.D., Chief Medical Officer of NeuBase, said, "DM1 is a significant unmet medical need characterized by myotonia, muscle weakness and wasting, and cognitive impairments. We are excited to present new preclinical data for our lead candidate, NT-0231.F, to support our differentiated approach for DM1. Systemic administration of NT-0231.F achieves clinically relevant molecular and functional rescue in the muscle in the HSA^{LR} model, which reproduces many components of the disease. Initial pharmacokinetic data supports a whole-body solution to the disease, and additional preclinical work is in progress to assess biodistribution in key tissues. These data are an important milestone in our DM1 development program and support our pursuit of a best-in-class therapeutic profile for the disease."

A single intramuscular dose confirmed that NT-0231.F is pharmacologically active in the

muscle and drives molecular and functional rescue in the HSA^{LR} model, including splice rescue, nuclear aggregate resolution, and myotonia (delayed muscle relaxation after contraction) reversal. A single intravenous (IV) dose of NT-0231.F or multiple subcutaneous (SC) doses over a 28-day period broadly rescued splicing, including the chloride channel *(Clcn1)* transcript, and reversed myotonia in the model. A single IV dose of NT-0231.F provides initial splice rescue at around two weeks, with significant splice rescue around three weeks. Myotonia reversal was achieved at around four weeks, with effects enduring to at least six weeks, the longest time point tested so far. A time course of multiple SC doses across increasing concentrations of NT-0231.F was also investigated and showed splice rescue and myotonia reversal in a dose-responsive manner, illustrating feasibility of the differentiated and patient-friendly SC route. In pharmacokinetic 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.

Dietrich A. Stephan, Ph.D., Founder, Chief Executive Officer, and Chairman of NeuBase, said, “We now have preclinical data in the gold-standard animal model for our development candidate demonstrating robust reversal of myotonia, as measured by muscle relaxation in these studies. We believe we are the only company that has shown improved muscle relaxation after systemic routes of administration. The HSA^{LR} model is also a high bar for human disease in that it contains at least 10x more mutant gene CUG- repeat targets than patients, giving us further conviction in the robustness of our approach. Not only does this data support the further advancement of our lead program in DM1 and keep us on track for submitting an Investigational New Drug application to the U.S. Food and Drug Administration in 4Q CY2022, it also validates that we can utilize our PATrOL™ platform to design novel genetic medicines that target and rescue many other gene dysfunctions, with the potential for clinically impactful outcomes in both rare and common diseases.”

Conference Call and Webcast Details
NeuBase Therapeutics, Inc. will discuss these data during a webcasted conference call accompanied by slides tomorrow, March 14, 2022, at 8:00 a.m. EDT. To access the webcast, please click here. An archived recording of this presentation will be available following the call through the Investor Relations Calendar page on the Investors section of the Company’s website, www.neubasetherapeutics.com.

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 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, including the myotonic dystrophy type 1 (DM1) at the 2022 MDA Clinical & Scientific Conference and the 2022 RNA Leaders World Congress, 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 ("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.

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