Nido Biosciences Presents Clinical Data Supporting the Continued Development of NIDO-361 in SBMA Patients at the 2023 International Kennedy's Disease Patient Conference

NIDO-361 was generally well-tolerated when administered in Ph1 study as a single- or as multiple-oral doses in healthy subjects

Observational study confirms MRI may serve as reliable endpoint for future clinical development program

Watertown, MA – November 5, 2023 – Nido Biosciences (Nido Bio), a clinical stage company developing precision medicines for debilitating neurological diseases, today announced positive results from its Phase 1 study of NIDO-361 in healthy male subjects and interim results from its observational study at the 2023 International Kennedy's Disease Patient Conference in London, November 4 – 5. The company believes data from both studies support the advancement of the clinical evaluation of NIDO-361 in patients.

NIDO-361 is in development for the treatment of patients suffering from Spinal and Bulbar Muscular Atrophy (SBMA), also known as Kennedy's disease. SBMA is caused by an X-linked genetic mutation on the androgen receptor that results in the progressive loss of skeletal muscle and motor neuron function. The novel small molecule binds to a distinct site on the androgen receptor and corrects transcriptional dysregulation to restore healthy cell function.

The Phase 1 study of NIDO-361 was a randomized double-blind placebo-controlled trial in healthy male subjects. The study was designed to evaluate the safety, tolerability, and pharmacokinetics (PK) of NIDO-361 delivered orally as a single dose in dose-escalated cohorts followed by multiple doses in dose-escalated cohorts. Sixty-four subjects were enrolled and received study drug, with eight enrolled in each cohort at a ratio of 3:1, active: placebo. The positive results presented at the conference demonstrate that NIDO-361 was generally safe and well-tolerated when administered as a single or as multiple doses, with no serious Treatment Emergent Adverse Events (TEAE).

Additionally, Nido Bio has announced the completion of interim analyses in patients with SBMA enrolled in an ongoing observational study. The two-year observational study being conducted at the National Institute of Neurological Disorders and Stroke (NINDS)/National Institute of Health (NIH), the University College London (UCL), and the Department of Neurology, Nagoya University Graduate School of Medicine (Nagoya), was designed to assess the relationship between muscle MRI and clinical outcomes. Interim analyses conducted to date with available data at 6 months and 12 months post-baseline generated results in support of the hypothesis that muscle MRI measures are associated with disease progression measures.

"The Phase 1 study results and interim analyses of our ongoing observational study support the further advancement of our clinical program by enabling the selection of active and safe doses and confirming

muscle MRI as a reliable primary endpoint for our upcoming Phase 2 trial which we anticipate will begin enrollment in Q1 2024," said Vissia Viglietta, M.D., Ph.D., CMO of Nido Bio. "We are excited by the potential of NIDO-361 to be a medicine that matters for SBMA patients as we continue our efforts to bring transformative therapies to patients suffering from debilitating neurological diseases."

Presentation Details
Title: Nido-361 for the Treatment of SBMA: Updates on clinical development program
Presented by: Vissia Viglietta, M.D., Ph.D., Chief Medical Officer, Nido Biosciences
Date: November 5, 2023
Time: 2:00 p.m. GMT

About Spinal and Bulbar Muscular Atrophy (SBMA)

SBMA, also known as Kennedy's disease, is a rare inherited X-linked neuromuscular disorder caused by a genetic mutation of the androgen receptor (AR) that results in the loss of skeletal muscle and motor neuron function. Manifesting in men, SBMA causes progressive weakness and wasting of limb, facial and swallowing muscles, which results in impaired mobility, speech, and swallowing.

About Nido Biosciences

Nido Bio is translating today's neuroscience breakthroughs into tomorrow's treatments for severe neurological diseases. Leveraging human genetics, we develop precision medicines that address the fundamental biology of disease and restore healthy cell function. NIDO-361, our clinical-stage candidate, is a treatment for Spinal and Bulbar Muscular Atrophy which is a rare and debilitating neuromuscular disease. Additional pipeline programs center around a novel target with the potential to address multiple disease mechanisms and that has broad clinical application across neurodegenerative and peripheral inflammatory diseases. We are creating a sustainable pipeline for the company by utilizing a functional genomics discovery platform based on human cell lines to identify novel targets. Through our approach we seek to transform patient lives in meaningful ways. www.nidobio.com.

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