



Nido Biosciences Announces Publication of Collaborative Research Revealing Clinically Meaningful Endpoints for Spinal and Bulbar Muscular Atrophy

Study powered by largest global SBMA dataset known to date

BOSTON, MA – December 4, 2024 – [Nido Biosciences](#) (Nido Bio), a clinical stage company developing precision medicines for debilitating neurological diseases, today announced a new publication in *Neurology*[®], the medical journal of the American Academy of Neurology, titled “Functional Outcome Measures to Optimize Drug Development in Spinal and Bulbar Muscular Atrophy.” Led by Nido’s team, researchers from institutions around the world contributed their individual natural history studies that were integrated to generate the largest global SBMA dataset known to date.

Through robust analyses of this dataset, highly sensitive functional endpoints were identified that will inform future interventional clinical trials in SBMA. The SBMA functional rating scale (SBMAFRS) and the 6-minute walk test were shown to be the most sensitive outcome measures to monitor progression across a wide range of disease states. Authors also created a modified SBMAFRS with improved performance by including lower limb and trunk measures, while excluding items that contributed less to the overall score.

The publication reports results of integrated data from six longitudinal SBMA natural history studies conducted across the United States, Italy, South Korea, United Kingdom, Denmark, and Japan. With data from 278 patients and up to three years of follow-up for the majority of patients, researchers were able to leverage the most comprehensive SBMA dataset worldwide.

“The slowly progressive nature of SBMA has been a major obstacle for the development of pharmaceutical treatments, making it clear that we required more sensitive clinical measures and reliable biomarkers for use in clinical trials,” said Vissia Viglietta, M.D., Ph.D., CMO of Nido Bio. “With our lead candidate NIDO-361 currently in Phase 2 clinical trials, research that drives a better understanding of disease evolution metrics, like this publication, allows us to design more efficient clinical studies to assess the impact of therapies for patients suffering from this debilitating disease.”

About Spinal and Bulbar Muscular Atrophy (SBMA) and NIDO-361

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. NIDO-361 is a novel small molecule that binds to a distinct site on the androgen receptor and corrects transcriptional dysregulation to restore healthy cell function.

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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