Neuron23 Announces First-in-Human Dose in Phase 1 Clinical Trial of its Best-in-Class Brain-Penetrant LRRK2 Inhibitor for Parkinson's Disease

NEU-723 is a highly potent and selective leucine-rich repeat kinase 2 (LRRK2) inhibitor

First-of-its-kind companion diagnostic paired with NEU-723 will enable selection of patients likely to respond to therapy

SOUTH SAN FRANCISCO, Calif. – February 7, 2023 – Neuron23™ Inc., a clinical-stage biotechnology company focused on developing precision medicines for genetically defined neurological and immunological diseases, today announced that the Company recently initiated dosing in a Phase 1 clinical trial of NEU-723 for the treatment of Parkinson's disease. NEU-723 is a highly potent and selective brain-penetrant leucine-rich repeat kinase 2 (LRRK2) inhibitor. Variants in the LRRK2 gene are associated with Parkinson's disease and systemic inflammatory diseases.

"We are excited to begin this clinical trial of NEU-723, marking our first program to enter clinical development and only the second LRRK2 small molecule currently in the clinic for the modification of Parkinson's disease," said Nancy Stagliano, Ph.D., chief executive officer of Neuron23. "A best-in-class LRRK2 inhibitor for Parkinson's disease, NEU-723 has been shown in preclinical studies to have strong disease-modifying potential with a higher therapeutic index than other candidates in development. "

"Parkinson's disease is one of the most devastating neurodegenerative diseases, with no cure and no existing therapies that modify the progression of the disease. We look forward to advancing this clinical trial along with our entire LRRK2 program and pipeline with the goal of treating the right patients with the right medicines," said Sam Jackson, M.D., chief medical officer of Neuron23.

Neuron23 recently announced a collaboration with QIAGEN to develop a companion diagnostic identifying a sub-population of Parkinson's disease patients who are likely to respond to a LRRK2 inhibitor even though they do not carry one of the rare, disease-causing mutations in the LRRK2 enzyme.

"The combination of best-in-class clinical candidates with a first-of-its-kind companion diagnostic places Neuron23 in a leadership position in Parkinson's disease," added Dr. Stagliano.

This first-in-human Phase 1 study (NCT05633745) is a randomized, double-blind, placebo-controlled, single-ascending dose, multiple-ascending dose, single-site clinical trial designed to evaluate the safety, tolerability, and pharmacokinetics of NEU-723 in adult healthy volunteers. More information about this clinical trial can be found at www.clinicaltrials.gov.

Neuron23 has numerous LRRK2 candidates in its pipeline, including a follow-on brain penetrant inhibitor, NEU-411 for Parkinson's disease, which will enter the clinic in 2023. The Company is also advancing brain-penetrant and peripherally restricted programs against tyrosine kinase 2 (TYK2), a JAK family protein that plays a role in pathological immune signaling.

About Parkinson's disease
Parkinson's disease is a brain disorder that causes unintended or uncontrollable movements, such as shaking, stiffness, and difficulty with balance and coordination. Symptoms usually begin gradually and worsen over time. As the disease progresses, people may have difficulty walking and talking. They may also have mental and behavioral changes, sleep problems, depression, memory difficulties, and fatigue.

Some cases of Parkinson's disease appear to be hereditary, and a few cases can be traced to specific genetic mutations. While genetics is thought to play a role in Parkinson's, in most cases the disease does not seem to run in families. Many researchers now believe that Parkinson's results from a combination of genetic and environmental factors, such as exposure to toxins.

No laboratory tests are currently available for the diagnosis of non-genetic cases for Parkinson's disease. Usually, the disease is diagnosed based on medical history and neurological examination. Although no cure currently exists for Parkinson's disease, therapies are used to alleviate some symptoms.

Source: National Institute on Aging, National Institutes of Health

About LRRK2
LRRK2 is a complex, multidomain protein found in neurons and many other cell types and tissues throughout the body. Mutations in the LRRK2 gene are one of the most common causes of familial Parkinson's disease. Individuals who inherit gain of function mutations in LRRK2 are at higher risk of developing the disease in later life. Additionally, there is emerging evidence that LRRK2 activity may play a role in a subset of the larger population of patients with non-familial Parkinson's disease, suggesting that therapies targeting LRRK2 could be beneficial to a broader patient population. Recent investigations have shown that small-molecule LRRK2 inhibitors can be neuroprotective and therefore have the potential to be disease modifying.

About Neuron23™
Neuron23™ Inc. is a clinical-stage biotechnology company focused on developing precision medicines for genetically defined neurological and immunological diseases. Neuron23 combines recent advances in human genetics with a state-of-the-art drug discovery and biomarker platform using advanced techniques in machine learning and artificial intelligence to advance therapeutics for devastating diseases. The Company's focus areas are neurodegenerative diseases, neuroinflammatory diseases, and systemic autoimmune and inflammatory diseases. Founded in 2018, Neuron23 has assembled a world-class team of experts and entrepreneurs located in South San Francisco, CA. For more information, please visit www.neuron23.com.

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