Yumaniy Therapeutics Initiates Phase 1 Clinical Trial of Lead Candidate YTX-7739 for the Treatment of Parkinson's Disease

YTX-7739 represents a novel, first-in-class, potentially disease-modifying therapy

Data from Phase 1 study expected in the first quarter of 2020

CAMBRIDGE, Mass. – October 7, 2019 – Yumaniy Therapeutics, a company focused on protecting the vitality of the mind by discovering and developing transformative brain-penetrating small molecule drugs to treat neurodegenerative diseases, today announced that the first subject cohort has been dosed in a Phase 1 clinical trial evaluating the safety and tolerability of YTX-7739 in healthy volunteers. YTX-7739, the company’s lead investigational therapy, is designed to inhibit Stearoyl-CoA-Desaturase (SCD), a validated biologic target that has recently shown potential in neurodegenerative diseases by protecting cells from a-synuclein toxicity, a major driver of Parkinson’s disease.

“Developing effective therapies for patients with devastating neurodegenerative diseases has been challenging because too few hypotheses and novel targets have been explored,” said Kenneth Rhodes, Ph.D., chief scientific officer at Yumaniy Therapeutics. “We advanced YTX-7739, an orally-active SCD inhibitor, into clinical development because of recent evidence established at Yumaniy Therapeutics demonstrating its promise to protect cells from a-synuclein toxicity. We look forward to fully characterizing the potential clinical use of YTX-7739, which is clearly differentiated from currently available Parkinson’s disease therapies that only address the symptoms, not the underlying causes.”

The double-blind, placebo-controlled, dose-escalation, crossover study is intended to evaluate the safety, tolerability and pharmacokinetics of single ascending doses of YTX-7739 in adult healthy volunteers. A second study, exploring multiple ascending doses in adult healthy volunteers and patients with Parkinson’s, will follow. Approximately 40 participants will be enrolled in this Phase 1 single ascending dose study. Following completion of the Phase 1 studies, Yumaniy Therapeutics expects to advance YTX-7739 into a Phase 1b proof-of-concept clinical trial in the second half of 2020.

“Since Yumaniy Therapeutics’ inception, our goal has been to uncover novel pathways and targets to tackle significant medical challenges,” said Richard Peters, M.D., Ph.D., chief executive officer of Yumaniy Therapeutics. “Moving from target identification of SCD to initial clinical development of YTX-7739 in just three years is a testament to the enormous potential of our discovery platform to reproducibly identify previously unexplored biology and new, druggable targets that have the potential to protect cells from neurodegeneration. This Phase 1 trial will provide important validation for the broad application of our technology to help address arguably the most important therapeutic challenges of our time.”

About YTX-7739

YTX-7739 is Yumaniy Therapeutics’ proprietary lead investigational therapy designed to penetrate the blood-brain barrier and inhibit the activity of a novel target that plays an important and previously unrecognized role in the neurotoxicity caused by the α-synuclein protein, a major driver of Parkinson’s disease and related neurodegenerative disorders. Misfolding and aggregation of the α-synuclein protein triggers a cascade of events, ultimately resulting in neurotoxicity and the subsequent disorders in movement and cognition that affect patients living with these diseases. YTX-7739 has been shown to inhibit many of the key aspects of α-synuclein toxicity and the company is assessing its potential utility in Parkinson’s disease.

About Parkinson’s Disease

Parkinson’s disease is a progressive neurological disorder that affects the central nervous system and impacts both motor and non-motor functions. It is one of the most common age-related neurodegenerative diseases, affecting an estimated 0.5 to 1 percent of people 65 to 69 years of age, rising to 1 to 3 percent of
the population over the age of 80.\textsuperscript{1} Symptom severity and disease progression differ between individuals, but typically include slowness of movement (bradykinesia), trembling in the extremities (tremors), stiffness (rigidity), cognitive or behavioral abnormalities, sleep disturbances and sensory dysfunction.\textsuperscript{2} There is no laboratory or blood test for Parkinson’s disease, so diagnosis is made based on clinical observation.\textsuperscript{3} Currently, there is no cure and available treatments only address the symptoms of Parkinson’s disease, not the underlying causes.

**About Yumanity Therapeutics**
Yumanity Therapeutics is transforming drug discovery for neurodegenerative diseases caused by protein misfolding. Formed in 2014 by renowned biotech industry leader, Tony Coles, M.D., and protein folding science pioneer, Susan Lindquist, Ph.D., the company is focused on discovering disease-modifying therapies for patients with Parkinson’s disease and related disorders, amyotrophic lateral sclerosis (ALS), and Alzheimer’s disease. Leveraging its proprietary discovery engine, Yumanity Therapeutics’ innovative new approach to drug discovery and development concentrates on reversing the cellular phenotypes and disease pathologies caused by protein misfolding. For more information, please visit [yumanity.com](http://yumanity.com).

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