Neurona Therapeutics Receives IND Clearance to Initiate Phase 1/2 Clinical Trial of Neural Cell Therapy NRTX-1001 in Chronic Focal Epilepsy Patients

San Francisco, CA, November 4, 2021 – Neurona Therapeutics, a biotherapeutics company advancing restorative neural cell therapies for the treatment of chronic neurological disorders, today announced that it has received clearance of its Investigational New Drug (IND) application from the U.S. Food and Drug Administration (FDA) to initiate a first-in-human Phase 1/2 clinical trial to evaluate the safety and efficacy of NRTX-1001 in people with drug-resistant mesial temporal lobe epilepsy (MTLE). NRTX-1001 is a regenerative neural cell therapy with the potential to repair hyperexcitable neural networks that underlie epilepsy as well as other disorders of the nervous system.

"The clearance of our first IND is a key milestone for Neurona and a testament to the talent, experience, and hard work of the entire Neurona team," said Cory Nicholas, Ph.D., Neurona's president and chief executive officer. "This milestone is especially rewarding and timely given that November is Epilepsy Awareness Month. Epilepsy is one of the most common neurological disorders, affecting over three million people in the U.S. of whom approximately one-third have drug-resistant disease. NRTX-1001 is a new type of inhibitory cell therapy that is targeted to the focal seizure onset region in the brain and, in a single treatment, has the potential to significantly improve the lives of people living with focal epilepsy."

"To our knowledge, NRTX-1001 is the first human cell therapy candidate to enter clinical trials for epilepsy," said David Blum, M.D., head of clinical development at Neurona Therapeutics. "Unlike many other interventions for drug-resistant focal epilepsy, including surgical removal or ablation of brain tissue, NRTX-1001 has the potential to achieve seizure freedom in a non-tissue destructive manner."

Neurona's proprietary regenerative cell technology is based on research by the company's founders at the University of California, San Francisco. Neurona has generated compelling preclinical data in a model of drug-resistant focal epilepsy that demonstrated seizure-freedom in over two-thirds of the NRTX-1001 treatment group versus 5% of the control group. Furthermore, NRTX-1001 implantation resulted in reduced mesiotemporal sclerosis, or tissue damage in the affected seizure-onset area of the brain. No adverse anatomical or behavioral effects were detected in extensive preclinical studies.

Neurona's multi-center, Phase 1/2 clinical trial is designed to evaluate the safety, tolerability, and efficacy of a single administration of NRTX-1001. The first stage of the trial is an open label dose-escalation study in up to 10 people with MTLE. The second stage will consist of a randomized, blinded investigation of NRTX-1001 compared to a control group to determine safety and efficacy in up to 30 people with MTLE. Patient recruitment will soon begin at approximately 10 clinical epilepsy centers across the United States.

About NRTX-1001
NRTX-1001 is an inhibitory nerve cell therapy derived from human pluripotent stem cells. The nerve cells, called interneurons, secrete the inhibitory neurotransmitter gamma-aminobutyric acid (GABA). Delivered as a one-time dose, the human interneurons integrate and innervate on-target, providing long-term GABAergic inhibition to repair hyperexcitable neural networks that underlie epilepsy as well as other disorders of the nervous system. Neurona is initially focused on developing NRTX-1001 as a restorative treatment for mesial temporal lobe epilepsy (MTLE).

About MTLE
MTLE primarily affects the internal structures of the temporal lobe, where seizures often begin in a structure called the hippocampus. MTLE is the most common type of focal epilepsy. For people with seizures resistant to drugs, epilepsy surgery can be an option for some, where the damaged temporal lobe is surgically removed or laser-ablated. However, current surgical options are not available or effective for all, are tissue-destructive, and can have significant adverse effects.

About Neurona
Neurona's cell therapies have single-dose curative potential. Based on a novel neural cell lineage developed by the company's scientific founders, Neurona has built a robust regenerative platform and is developing off-the-shelf, allogeneic neuronal, glial, and gene-edited cell therapy candidates that provide long-term integration and repair of dysfunctional neural networks for multiple neurological disorders. For more information about Neurona, visit www.neuronatherapeutics.com

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