



**Neurona Therapeutics Presents Preclinical Data from Lead Cell Therapy Candidate  
NRTX-1001 at Neuroscience 2021**

*IND cleared by the FDA to enable initiation of Phase 1/2 clinical trial of NRTX-1001 in people with drug-resistant focal epilepsy*

*Supporting preclinical data demonstrate seizure-freedom in up to 68% of the NRTX-1001 treated group*

San Francisco, CA, November 8, 2021 – [Neurona Therapeutics](#), a biotherapeutics company advancing restorative neural cell therapies to treat chronic neurological disorders, today announced three presentations of supporting preclinical data for its lead clinical candidate, NRTX-1001, an inhibitory neuronal cell therapy. An Investigational New Drug (IND) application has been cleared by the U.S. Food and Drug Administration (FDA) to enable Neurona to initiate a first-in-human Phase 1/2 clinical trial to evaluate NRTX-1001 in people with drug-resistant mesial temporal lobe epilepsy (MTLE).

The data were previewed November 3-7 at Neuroscience 2021, the 50th Annual Meeting of the Society for Neuroscience, and were presented during the official meeting November 8-11.

“We are excited to advance NRTX-1001 into a first-in-human clinical study in people with chronic MTLE and to share the data that support this pioneering program,” said Cory Nicholas, Ph.D., Neurona’s president and chief executive officer. “Drug resistant focal epilepsy is a significant unmet medical need, and we believe that Neurona’s novel neuronal cell therapy has the potential to provide a new therapeutic solution for people who currently have limited options.”

In three presentations, Neurona’s scientists reported data that support the clinical development of NRTX-1001 for the treatment of focal seizures. The presentations were part of the session: Antiepileptic Therapies IV, at Neuroscience 2021, and are summarized below:

**P152.01:** *Longitudinal characterization of hippocampal pathology in the intrahippocampal kainate mouse model of temporal lobe epilepsy.* (D. Traver et al.)

Neurona scientists undertook a long-term characterization of the intrahippocampal kainate epilepsy model, a model of pharmacoresistant chronic mesiotemporal seizures. The study was over a year in duration and measured the progression of tissue damage in the mesial temporal lobe, including hippocampus; damage which resembles hallmark mesial temporal sclerosis often observed in people with MTLE. The study establishes a baseline progression of hippocampal damage that has been important in the preclinical characterization of NRTX-1001 and could facilitate the future investigation of other anti-seizure therapeutic candidates.

**P152.02:** *Co-administration of anti-seizure drugs with NRTX-1001 GABAergic interneuron therapy is well-tolerated and effective in mice.* (E. Sevilla et al.)



Preclinical data were also presented that demonstrated in this same model of focal epilepsy that NTRX-1001 treatment was well-tolerated and maintained efficient interneuron cell engraftment and disease modifying activity when combined with the commonly used anti-seizure medications levetiracetam and diazepam.

**P152.03** *Preclinical development of the human inhibitory interneuron cellular therapeutic NRTX-1001 for the treatment of chronic focal epilepsy.* (P. Hampel et al.)

The data demonstrated that NRTX-1001 transplantation into the hippocampus stably suppressed focal seizures (up to 68% of the cell therapy group became seizure-free, versus zero seizure-freedom observed in the control group) and significantly reduced hippocampal pathology in the mouse model of MTLE over the 7-month study. No unwanted behavioral effects were observed as a result of treatment. Transplanted NRTX-1001 interneuron cells persisted and distributed in the hippocampus throughout the course of the study, and were not proliferative. This work was supported in part by the California Institute for Regenerative Medicine (CIRM TRAN1-11611).

Neurona is now launching a first-in-human multicenter clinical study. Stage 1 will be an open-label, dose-escalation design to investigate the safety of NRTX-1001 in up to 10 adults with chronic MTLE. Subjects will receive a single administration of the NRTX-1001 cellular therapeutic. The primary endpoints of the proposed clinical study are safety and tolerability however, other secondary endpoints will be evaluated, including changes in seizure frequency and severity, quality of life and neuropsychological outcomes. Stage 2, which will start after the completion of Stage 1, will be a double-blind sham-controlled study in approximately 30 subjects with chronic MTLE.

**About MTLE**

Mesial temporal lobe epilepsy (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 - where the damaged temporal lobe is surgically removed or ablated by laser - can be an option for some. However, these current surgical options are not available or effective for all, are tissue-destructive, and can have significant adverse effects.

**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 are intended to 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.

**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-



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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](http://www.neuronatherapeutics.com)

**Neurona Therapeutics**

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