Neurona Doses First Patient in Phase 1/2 Trial of NRTX-1001 Cell Therapy in Adults with Drug-Resistant, Bilateral Mesial Temporal Lobe Epilepsy (MTLE)

- First patient received bilateral administration of NRTX-1001; no significant surgical complications or adverse events reported to date -

- Trial funded by a $14 million grant from California Institute for Regenerative Medicine -

- Data from this bilateral trial, together with results from the previously announced Phase 3 EPIC study in unilateral MTLE, are intended to support a future BLA submission for NRTX-1001 in drug-resistant MTLE –

- Full enrollment of an eight-subject expansion cohort in unilateral MTLE trial also announced -

San Francisco, CA, June 18, 2025 – Neurona Therapeutics, a clinical-stage biotherapeutics company advancing regenerative cell therapies for disorders of the nervous system, today announced dosing of the first patient in its Phase 1/2 clinical trial of NRTX-1001 cell therapy in adults with drug-resistant bilateral mesial temporal lobe epilepsy (MTLE). MTLE is the most common type of epilepsy in adults and is characterized by seizures originating primarily in the hippocampus from either one hemisphere of the brain (unilateral) or both hemispheres (bilateral).

Building upon preliminary efficacy and safety data from its ongoing Phase 1/2 trial in unilateral MTLE, most recently presented at the 2025 American Academy of Neurology (AAN) Annual Meeting, Neurona has initiated a second Phase 1/2 trial of NRTX-1001 inhibitory interneuron cell therapy in bilateral MTLE and has additionally announced completed enrollment of an expansion cohort of eight patients for its unilateral MTLE study. Neurona previously announced the design of its upcoming Phase 3 EPIC (**EPI**lepsy **C**ell Therapy) trial, based on its discussion with FDA under the Regenerative Medicine Advanced Therapy (RMAT) designation. The results from the Phase 1/2 and Phase 3 trials in both unilateral and bilateral MTLE populations are intended to support a prospective marketing application for the treatment of drug-resistant MTLE.

The bilateral MTLE study (NCT06422923) is a multicenter, open-label trial that will enroll ten adult subjects who will receive a one-time image-guided intracerebral administration of NRTX-1001 into both temporal lobe regions of the brain. The study is designed to evaluate safety, tolerability, and preliminary efficacy, with follow up assessments at approximately quarterly intervals for two years after administration.

Effective treatment options are currently lacking for drug-refractory bilateral MTLE patients. Surgical lobectomy to remove or laser ablate the seizure-prone region of the brain is not typically

indicated for bilateral MTLE patients due to the risk of irreversible memory loss from the surgical destruction of both temporal lobes. While neurostimulator device implantation into the brain can be an option for some patients, randomized controlled trials have shown limited efficacy.

“NRTX-1001 cell therapy is highly differentiated from current treatments for drug-refractory epilepsy, in that it is designed to target the underlying biology of this type of epilepsy,” said Peter Warnke, MD, Director of Stereotactic and Functional Neurosurgery at the University of Chicago Medicine, the trial's local principal investigator. “The current surgical treatments, whether it be surgical resection or laser ablation to remove or ablate problematic tissue, are tissue-destructive and not suitable for bilateral MTLE patients due to the high risk of permanent cognitive impairment. As such, NRTX-1001 could represent a novel non-destructive treatment paradigm, providing seizure control and preserving neurocognitive function in this underserved patient population.”

“We’re excited to expand our clinical development of NRTX-1001 to address bilateral MTLE,” said Cory R. Nicholas, Ph.D., Neurona’s Chief Executive Officer and Co-Founder. “While this trial underlines the importance of advancing NRTX-1001 in clinical trials to address the needs of broader patient populations in drug-resistant epilepsy, it will also, for the first time, evaluate the safety of interneuron cell delivery into both sides of the brain, paving the way for potential future cell therapy applications in Alzheimer’s and other diseases of the nervous system.”

Dr. Nicholas added: “We would once again like to extend our gratitude to CIRM for their continued support. Refractory bilateral MTLE is among the highest unmet medical needs in epilepsy, and this grant is a big win for epilepsy patients, and their families, who are urgently in need of alternative treatment options.”

In March, the California Institute for Regenerative Medicine (CIRM) approved a $14 million award to help fund Neurona’s Phase 1/2 clinical trial of NRTX-1001 in drug-resistant bilateral MTLE.

In April, Neurona presented updated data from its unilateral MTLE trial at the AAN Annual Meeting. The results included a 92% median reduction from baseline in disabling seizures during the 7-12-month efficacy endpoint period, an 80% (4/5) responder rate with >75% seizure reduction at the 12-month endpoint, and durable seizure control in all (4/4) responders followed for 18-24 months after a single low-dose of NRTX-1001 (Cohort 1). All patients (5/5) in Cohort 1 had significantly improved quality-of-life test scores, with no patient to date experiencing persistent decline in cognition. Importantly, NRTX-1001 continues to be well-tolerated with no adverse events to date attributed to the cell therapy.

Neurona plans to present extended durability data from the first ten unilateral MTLE patients treated with low (Cohort 1) and high (Cohort 2) doses of NRTX-1001, along with new results from the eight additional patients who were enrolled in the unilateral MTLE expansion cohorts, as well as early data from the first bilateral MTLE patients at the American Epilepsy Society’s Annual Meeting in December 2025.

About Neurona Therapeutics

Neurona is developing allogeneic, off-the-shelf, regenerative neural cell therapies with the potential to provide long-term targeted repair of the nervous system following a single administration. Neurona's lead product candidate, NRTX-1001, comprising GABAergic interneurons, is currently being studied for safety and efficacy in two ongoing open label multicenter Phase 1/2 trials: NCT05135091 for drug-resistant unilateral mesial temporal lobe epilepsy (MTLE), and NCT06422923 for drug-resistant bilateral MTLE, with expansion to neocortical focal epilepsy and other indications planned in the future. The Phase 1/2 MTLE clinical trials are supported by grants from the California Institute for Regenerative Medicine (CIRM; CLIN2-13355 and CLIN2-17135). The FDA granted the Regenerative Medicine Advanced Therapy (RMAT) designation to NRTX-1001 in June 2024. Consistent with Neurona's discussion with the FDA, the Phase 3 EPIC (**EPI**lepsy **C**ell Therapy) trial is planned to start in 2H 2025 and will provide primary support for submitting a potential future Biologics License Application (BLA). For more information about Neurona, visit: www.neuronatherapeutics.com.

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