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Neurona Therapeutics Raises $120M to Advance Groundbreaking Pipeline of Regenerative Cell Therapy Candidates for Chronic Neurological Disorders

The financing, co-led by Viking Global Investors and Cormorant Asset Management with participation from The Column Group along with additional new and existing investors, follows promising early clinical data from NRTX-1001 epilepsy program

SAN FRANCISCO, Feb. 08, 2024 (GLOBE NEWSWIRE) -- Neurona Therapeutics, a clinical-stage biotherapeutics company advancing regenerative cell therapy candidates for the treatment of neurological disorders, today announced the successful completion of a $120 million financing co-led by Viking Global Investors and Cormorant Asset Management with participation from new and existing investors, including The Column Group, LYFE Capital, Schroders Capital, Willett Advisors, Ysios Capital Partners, Euclidean Capital, SymBiosis, Alexandria Venture Investments, Berkeley Frontier Fund, Sphera Biotech Master Fund LP, Spur Capital Partners, UCB Ventures, and UC Investments. Proceeds from the financing will be used to advance the company's pipeline of wholly-owned, off-the-shelf cell therapies for multiple indications, including its lead investigational candidate, NRTX-1001. NRTX-1001 is being evaluated in an ongoing open-label, single-arm Phase I/II clinical trial for treatment of drug-resistant mesial temporal lobe epilepsy (MTLE) and has potential application in Alzheimer's disease and other disorders of the nervous system.

"We are excited to co-lead this financing with Viking and other committed investors to help advance Neurona's cell therapies to address unmet needs in chronic neurological disorders," said Raymond Kelleher, M.D., Ph.D., managing director of Cormorant and a neurologist at Massachusetts General Hospital, Harvard Medical School, who will be joining the Neurona Board of Directors. "Neurona has pioneered development of a fully-differentiated cell therapy for drug-resistant focal epilepsy that is designed to be disease-modifying, repairing the affected neural network, and is yielding very promising initial clinical data. As a neurologist, I am particularly encouraged by the data generated thus far, suggesting that NRTX-1001 has the potential to provide seizure control and preserve neurocognitive function, which would be a game-changer for the field."

"This financing is a testament to the hard work and dedication of the Neurona team, commitment of our collaborators, and encouraging preliminary data from the first patients in the ongoing clinical trial of NRTX-1001 cell therapy," said Cory R. Nicholas, Ph.D., Neurona's chief executive officer and co-founder. "We are grateful for the significant investment from this reputable syndicate of new and existing investors. It signifies the conviction that Neurona's cell therapies have the potential to transform the treatment of previously refractory, devastating neurological disorders. This funding will support ongoing and planned clinical studies of NRTX-1001 for drug-resistant epilepsies and Alzheimer's disease, as well as the advancement of our other cell therapy candidates towards the clinic for additional neurological indications."

Data from the first cohort of five subjects in Neurona's ongoing clinical trial of NRTX-1001 were presented in December 2023 at the Annual Meeting of the American Epilepsy Society. The five subjects entered the study with a history of seizure activity that was not controlled by anti-seizure medications. These subjects received a one-time administration of NRTX-1001 as well as temporary immunosuppression to promote the long-term persistence of the cell therapy. The first two subjects, who had 32 and 14 seizures per month during the six-month baseline, respectively, continued to report a >95% reduction from baseline in overall seizure counts more than one year after NRTX-1001 administration, with elimination of their more severe focal impaired-awareness seizures. The first subject in the clinical trial has since discontinued the immunosuppression regimen, as planned, and has reported durable seizure reduction with no rebound in seizure frequency to date. The second subject in the clinical trial has been completely seizure-free for the past six months and recently discontinued immunosuppression, as planned. Per protocol, modality-specific cognitive tests administered at six months post-treatment, and subsequently at three-month intervals thereafter, revealed improvements on some measures, and no deterioration in performance, by the first and second subjects.

The remaining three subjects were treated with NRTX-1001 more recently and have been followed for three months post-administration. Two of the three subjects demonstrated reduced monthly seizure frequencies of 76% and 87% from baseline levels (26 and 30 seizures per month, respectively) since the first month, with elimination of their more severe focal impaired-awareness seizures. One subject, who entered the trial with baseline seizure activity of two seizures per month, had not yet responded and experienced variability in seizure count that was consistent with their disease history. Of note, NRTX-1001 required up to five to seven months from administration for maximum efficacy to become apparent in preclinical studies, reflecting the time required for the transplanted cells to functionally integrate and mature.

NRTX-1001 has been well-tolerated in all subjects to date. Adverse events have primarily been mild to moderate and typical of those associated with the temporary immunosuppression regimen, which resolved in the first subject upon discontinuation of the immunosuppressant drugs. No severe adverse events from the cell therapy, delivery procedure, or immunosuppression regimen have been reported thus far in the ongoing clinical trial. One serious adverse event, a status epilepticus seizure cluster, was reported but was consistent with the patient's medical history before treatment.

In November 2023, the Data Safety Monitoring Board overseeing the Phase I/II trial cleared continuing enrollment for a second cohort of five patients who are expected to receive a one-time administration of the higher dose level of NRTX-1001. The first patient in this open-label cohort has since received the higher dose of cells on-target and has not reported serious adverse events to date.

About NRTX-1001
NRTX-1001 is a regenerative neural cell therapy candidate derived from human pluripotent stem cells. The fully-differentiated neural cells, called interneurons, secrete the inhibitory neurotransmitter gamma-aminobutyric acid (GABA). Delivered as a one-time dose, the human interneurons are intended to integrate on-target and durably silence seizure activity in the epileptic region of the brain. NRTX-1001 is manufactured in Neurona's in-house GMP facility using proprietary methods.

About Mesial Temporal Lobe Epilepsy (MTLE)
According to the Centers for Disease Control and Prevention, an estimated 3.4 million Americans have epilepsy, and 25-35% live with ongoing seizures despite treatment with approved drugs, illustrating a substantial unmet medical need in this community. MTLE is a common type of focal epilepsy in adults and primarily affects the internal structures of the temporal lobe, where seizures often begin in a structure called the hippocampus. For people with seizures resistant to anti-seizure drugs, epilepsy surgery - where the damaged temporal lobe is surgically removed or ablated by laser - can be an option. However, the current surgical options are not available or effective for all subjects, are tissue-destructive, and can have significant adverse effects.

About Neurona's Clinical Trial of NRTX-1001 for MTLE
The ongoing clinical study is designed to evaluate the safety and efficacy of a single administration of NRTX-1001 for drug-resistant MTLE. The first stage of the trial is an open-label dose-escalation study in up to 10 people with drug-resistant MTLE, with five subjects in the first cohort treated at a starting dose and five subjects in the second cohort to be treated at a higher dose. Subjects treated

with a single infusion of NRTX-1001 cells will be monitored for safety and effects on their epilepsy disease symptoms. Subject recruitment is underway at epilepsy centers across the United States. For more information, please visit www.clinicaltrials.gov (NCT05135091). The first part of the clinical trial is supported by an $8.0 million grant from the California Institute for Regenerative Medicine (CIRM; CLIN2-13355). With $5.5 billion in funding and more than 150 active stem cell programs in its portfolio, CIRM is one of the world's largest institutions dedicated to helping people by bringing the future of cellular medicine closer to reality.

About Neurona Therapeutics
Neurona is focused on developing regenerative cell therapy candidates with single-dose curative potential. Neurona's investigational allogeneic, off-the-shelf, cell therapy candidates are designed to provide long-term repair of dysfunctional neural networks for multiple neurological disorders. For more information about Neurona, visit www.neuronatherapeutics.com.

Forward-Looking Statements
This press release includes forward-looking statements that are subject to risks and uncertainties, including risks and uncertainties inherent in the clinical development of therapeutic candidates, risks that early clinical data collected from a small number of subjects may not be predictive of data when NRTX-1001 is tested in larger clinical trials, the possibility that timelines may change, and difficulties associated with obtaining sufficient data to demonstrate safety and efficacy to support regulatory approval. NRTX-1001 is an investigational candidate and is being evaluated in ongoing clinical trials. NRTX-1001 has not been approved by any regulatory authority for commercial use or deemed to be safe or effective for any indication.

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