# Neuronascent Receives FDA Rare Pediatric Drug Designation for NNI-351 Treatment for Fragile X Syndrome

Rockville, MD, May 24, 2022 (GlobeNewswire) -- Neuronascent Inc., a clinical-stage biopharmaceutical company discovering and developing neuron-generating therapies for rare developmental disorders, today announced that the US Food and Drug Administration (FDA) granted rare pediatric designation for NNI-351 for Fragile X syndrome.

Fragile X syndrome (FXS) is a heritable monogenic disorder and is a major cause of intellectual disabilities and autism. Presently, there are no effective therapies to treat the hippocampal-related deficits, such as social anxiety, learning disability and hyperactivity as well as impaired neurogenesis.

Evaluation of NNI-351 in preclinical models of FXS (supported in part by the FRAXA organization, https://www.fraxa.org) by selectively prompting neural progenitor cells to become neurons, i.e., neurogenesis, NNI-351 reverses behavioral deficits back to normal levels. Neuronascent's oral therapy could therefore slow and potentially reverse FXS behavioral deficits in young individuals.

With a marketing application for NNI-351 for young FXS patients, Neuronascent as sponsor will request an award of a *rare pediatric disease priority voucher*. The FDA's Rare Pediatric Disease Priority Voucher Program is designed to incentivize companies to develop novel therapies to treat rare disorders of under 200,000 individuals and that primarily afflict those under the age of 18. The priority review voucher could then be used for a subsequent drug, or can be sold to another pharmaceutical company, expediting time to market and providing greater value for the rare pediatric therapeutic even with a small population pool.

"The receipt of this rare pediatric designation from the FDA allows Neuronascent to become eligible for the pediatric priority review voucher, which provides significant value to our first-in-class therapeutic NNI-351 for FXS in young individuals" stated founder and CEO, Judith Kelleher-Andersson, PhD. "With vast unmet need for novel therapies that can directly address pediatric FXS behavioral deficits, such as to improve education and social outcomes of these young patients, this designation puts a spotlight on the true potential of NNI-351."

## About NNI-351

NNI-351 is Neuronascent's lead, patented, new chemical entity aimed at reversing developmental disorders by producing new neurons postnatally to enhance neurogenesis that is slowed during early development in diseases such as FXS. NNI-351's ability to reverse behaviors in a number of models of developmental disorders, including rare pediatric disorder, FXS, suggests this novel therapy could be the first therapy to show true clinical benefit in young FXS patients.

## About Neuronascent, Inc.

Neuronascent, Inc., a privately-held, clinical-stage pharmaceutical company, was founded to discover and develop novel therapies that treat CNS disorders with high-unmet need by replacing and enhancing neuron numbers, not just connections. Through its proprietary

phenotypic discovery platform, Neuronascent has discovered a pipeline of patented small molecule neuron regenerative candidates, including clinical therapy NNI-362 for age-related disorders specifically Alzheimer's and Parkinson's disease.

Neuronascent website: https://www.neuronascent.com

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**Safe Harbor Statement**

This release contains forward-looking statements, which are made pursuant to the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995. Forward-looking statements are commonly identified by words such as "would," "may," "will," "expects," and other terms with similar meaning. Forward-looking statements are based on current beliefs, assumptions and expectations and speak only as of the date of this release and involve risks and uncertainties that could cause actual results to differ materially from current expectations.