GRIN Therapeutics Receives FDA Orphan Drug Designation for Radiprodil for the Treatment of GRIN-Related Neurodevelopmental Disorder

- Announcement follows FDA's February 2025 decision to grant radiprodil Breakthrough Therapy designation
- Company remains on track to initiate Phase 3 pivotal trial of radiprodil in mid-2025

NEW YORK, NY, March 17, 2025– GRIN Therapeutics, Inc., a leader in the development of therapies to treat serious neurodevelopmental disorders, today announced that the U.S. Food and Drug Administration (FDA) granted Orphan Drug designation for its investigational drug, radiprodil, for the treatment of GRIN-related neurodevelopmental disorder (NDD). Radiprodil is designed as a potent negative allosteric modulator selectively targeting the N-methyl-D-aspartate receptor subtype 2B (NR2B or GluN2B). The company is on track to initiate a pivotal Phase 3 trial for radiprodil in mid-2025 for the treatment of GRIN-related NDD with GoF mutations and has an ongoing global open-label clinical trial of radiprodil for the treatment of tuberous sclerosis complex (TSC) and focal cortical dysplasia (FCD) type II.

"We are making rapid progress in our efforts to bring a first-ever treatment for GRIN-related neurodevelopmental disorder to patients. Supported by our promising clinical data, the FDA's decision to grant Orphan Drug designation to radiprodil is the latest milestone in that effort," said Michael A. Panzara, MD, MPH, Chief Medical Officer at Neurvati Neurosciences and GRIN Therapeutics. "As we plan to launch our pivotal Phase 3 clinical trial to evaluate the impact of radiprodil on seizures, behavioral abnormalities and functional outcomes associated with GRIN-related NDD in the coming months, we are very grateful to the patients and their families who continue to support this important research effort."

Orphan Drug designation is designed to support innovation and research that can lead to more new treatments for diseases that affect fewer than 200,000 people in the United States. The designation grants drug developers the potential for seven years of market exclusivity for their drug after approval, during which time the FDA will generally not approve an application for the same drug targeting the same disease or condition. In February 2025, the FDA granted radiprodil Breakthrough Therapy designation for the treatment of seizures associated with GRIN-related NDD in patients with GoF mutations.

"We look forward to continuing to engage with the FDA through the means offered by Breakthrough Therapy designation, as we advance our Phase 3 program with a goal of bringing radiprodil to patients," said Anne-Marie Li-Kwai-Cheung, MChem, MTOPRA, RAPS, Senior Vice President, Development, Regulatory, and Quality at Neurvati Neurosciences and GRIN Therapeutics. "We have heard from so many clinicians and patients about the urgent unmet need in the treatment of GRIN-related disorders and our entire team is dedicated to advancing this development program through late-stage clinical research and regulatory review as rapidly as possible."

About GRIN-related neurodevelopmental disorder

GRIN-related neurodevelopmental disorder is a family of rare, genetically defined pediatric neurodevelopmental disorders caused by mutations in GRIN genes. While symptoms of GRIN-related neurodevelopmental disorder can present as early as infancy, a diagnosis is often not confirmed until age two or later when a child fails to reach developmental milestones. Individuals may experience developmental delay, intellectual disabilities, epilepsy, muscular hypotonia, movement disorders, spasticity, feeding difficulties and behavioral problems. There are currently no approved therapies for GRIN-related neurodevelopmental disorder.

About Radiprodil

Radiprodil is an investigational, selective and potent negative allosteric modulator of the N-methyl-D-aspartate (NMDA) receptor subtype 2B (NR2B or GluN2B). In nonclinical studies, radiprodil has been shown to potently and selectively modulate GluN2B. Radiprodil has also demonstrated an antiseizure effect in a number of in vitro and in vivo preclinical seizure and epilepsy models and specifically in models characterized by an enhanced GluN2B NMDA transmission, which can occur with gain-of-function (GoF) mutations in GRIN-related neurodevelopmental disorder. In vitro analysis of brain tissues extracted from both tuberous sclerosis complex (TSC) and focal cortical dysplasia (FCD) lesions has shown enhanced GluN2B NMDA expression supporting the potential ability of radiprodil to control seizures in these conditions.

About GRIN Therapeutics

GRIN Therapeutics is dedicated to the research and development of precision therapeutics for pediatric neurodevelopmental disorders with the goal of bringing hope to patients and caregivers. Late last year, GRIN Therapeutics reported promising topline data from a Phase 1b/2a clinical trial (the Honeycomb Trial) evaluating radiprodil in GRIN-related neurodevelopmental disorder in patients with GoF variants, leading to the decision to advance to a Phase 3 trial. The company has an additional ongoing clinical trial to evaluate radiprodil for the potential treatment of tuberous sclerosis complex (TSC) and focal cortical dysplasia (FCD) type II. GRIN Therapeutics is an affiliate of Neurvati Neurosciences, a portfolio company of Blackstone Life Sciences (BXLS). For more information, please visit www.grintherapeutics.com

About Neurvati Neurosciences

Neurvati Neurosciences, a portfolio company of Blackstone Life Sciences, identifies and advances the development of high-potential drug candidates across the neuroscience landscape. Neurvati employs a collaborative model that establishes fit-for-purpose affiliate companies, aligning dedicated resources with long-term strategic capital to catalyze innovative treatment options in areas of unmet need. Neurvati's team of experienced operators and drug developers seeks opportunities to challenge current treatment paradigms and make a difference for patients suffering from a wide range of neurological and psychiatric disorders. For more information, please visit www.neurvati.com

About Blackstone Life Sciences

Blackstone Life Sciences is an industry-leading private investment platform with capabilities to invest across the life cycle of companies and products within key life science sectors. By combining scale investments and hands-on operational leadership, Blackstone Life Sciences helps bring to market promising new medicines and medical technologies that improve patients' lives and currently has more than $12 billion in assets under management.

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