Tetra Discovery Partners Initiates Phase 2 Trial of BPN14770 in Fragile X Syndrome

Grand Rapids, MI (July 10, 2018): Tetra Discovery Partners today announced the initiation of a Phase 2 study of BPN14770 as a potential treatment for Fragile X Syndrome, the most common genetic form of autism. A selective small molecule inhibitor of the phosphodiesterase type-4D (PDE4D) subtype, BPN14770 has shown the ability to improve the quality of connections between neurons and to improve multiple behavioral outcomes in the Fragile X mouse model. BPN14770 has also received Orphan Drug Designation from the U.S. Food and Drug Agency for the treatment of Fragile X Syndrome.

The study, a randomized, double-blind, placebo-controlled study including two 12-week crossover periods, is being conducted in 30 adult males (ages 18 - 45 years). Endpoints for the study include preliminary cognitive and behavioral assessments of the efficacy of BPN14770 by a variety of standard tests and determinations of the experimental drug's safety and tolerability. The study will also gather pharmacokinetic and biomarker data on BPN14770. The study is being conducted at Rush University Medical Center, Chicago, Illinois by principal investigator Elizabeth M. Berry-Kravis, M.D., Ph.D. with financial support from the FRAXA Research Foundation. Additional information is available through clinicaltrials.gov (Identifier: NCT03569631).

"BPN14770 targets a basic biochemical change in how the connections between cells in the brain mature in patients with Fragile X Syndrome," said Mark E. Gurney, Ph.D., Chairman and Chief Executive Officer of Tetra Discovery Partners. "We look forward to exploring the potential therapeutic benefit of BPN14770 in Fragile X patients with Dr. Berry-Kravis, a noted expert in this disorder."

"Inhibition of PDE4 has been validated as a treatment strategy by many research groups in the Fragile X field," said Michael Tranfaglia, M.D., Medical Director and Chief Scientific Officer of the FRAXA Research Foundation. "We are very pleased to support this clinical investigation of BPN14770 in patients with Fragile X Syndrome by Dr. Berry-Kravis, whose early research was instrumental to our understanding of biochemical changes underlying the condition."

About Fragile X Syndrome

Fragile X Syndrome is a genetic condition that results from the silencing of the X-linked, Fragile X Mental Retardation-1 (FMR1) gene. Fragile X Syndrome patients display a range of behavior and other symptoms, including seizures, sleep disorders, anxiety, irritability, hyperactivity, autism, mild-to-severe cognitive impairment and intellectual disability. While Fragile X Syndrome occurs in both genders, the condition is more common and generally more severe in males. There is no cure for Fragile X Syndrome or any products approved for its treatment. Medications may be used to treat symptoms associated with Fragile X Syndrome including seizures, mood problems or other neuropsychiatric symptoms. Fragile X Syndrome occurs in approximately 1 in 4,000 males and 1 in 8,000 females.

About BPN14770

BPN14770 is a novel therapeutic agent that selectively inhibits phosphodiesterase-4D (PDE4D) to enhance early and late stages of memory formation. This unique mechanism of action has the potential to improve cognitive and memory function in devastating disorders including Fragile X Syndrome, Alzheimer's disease and other dementias, learning/developmental disabilities and schizophrenia. In preclinical studies of Fragile X Syndrome, BPN14770 improves behavioral outcomes in the Fragile X mouse model and improves the quality of connections between neurons. BPN14770 has completed three human Phase 1 clinical trials enrolling 147 subjects and has shown excellent safety, oral bioavailability, and preliminary cognitive benefit in elderly subjects. Tetra Discovery has initiated a Phase 2 study of BPN14770 in adults with Fragile X Syndrome, an indication for which BPN14770 has received Orphan Drug Designation from the U.S. Food and Drug Administration. Preparations are also under way to initiate a Phase 2 trial of BPN14770 in patients with Alzheimer's disease.

About the FRAXA Research Foundation

FRAXA's mission is to find effective treatments and ultimately a cure for Fragile X Syndrome - the most common inherited cause of autism worldwide. FRAXA funds research and clinical trials at universities all over the world. For more information please visit https://www.fraxa.org.

About Tetra Discovery Partners

Tetra Discovery Partners is a clinical stage biotechnology company developing a portfolio of therapeutic products that will bring clarity of thought to people suffering from neuro-developmental conditions such as Fragile X Syndrome, Alzheimer's disease, traumatic brain injury, and other brain disorders. Tetra uses structure-guided drug design to discover mechanistically novel, allosteric inhibitors of phosphodiesterase 4 (PDE4), an enzyme family that plays key roles in memory formation, learning, neuroinflammation, and traumatic brain injury. Tetra was a recipient of an NIH Blueprint Neurotherapeutics Program cooperative research agreement, and also receives major funding from the National Institute on Aging, the Alzheimer's Drug Discovery Foundation, the National Institute of Neurological Disorders and Stroke, and the National Institute of Mental Health through the Small Business Innovation Research (SBIR) program. Preclinical studies of Fragile X Syndrome were conducted through the FRAXA Research Foundation. Tetra Discovery Partners is headquartered in Grand Rapids, Michigan. For more information, please visit the company's website at http://www.tetradiscovery.com.

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