



Praxis Precision Medicines Receives Orphan Drug Designation for PRAX-562 for the Treatment of SCN2A-DEE

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PRAX-562 Phase 2 clinical trial for treatment of DEEs expected to initiate in first half of 2022

CAMBRIDGE, Mass., April 30, 2021 (GLOBE NEWSWIRE) -- Praxis Precision Medicines, Inc. (NASDAQ: PRAX), a clinical-stage biopharmaceutical company translating genetic insights into the development of therapies for central nervous system disorders (CNS) characterized by neuronal imbalance, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to PRAX-562 for the treatment of SCN2A development and epileptic encephalopathy (SCN2A-DEE).

"We are pleased by the FDA's decision to grant orphan drug designation to PRAX-562 for the treatment of SCN2A-DEE," said Marcio Souza, president and chief executive officer of Praxis. "As a company built on understanding the genetics of epilepsy, we are deeply committed to sodium channel research. The recognition by the FDA supports our comprehensive approach to developing treatment options for people living with mutations in the SCN2A gene. This now includes two differentiated and potentially complementary programs, PRAX-562 and PRAX-222, targeting SCN2A gain-of-function mutations in addition to a precision ASO targeting SCN2A loss-of-function mutations."

PRAX-562 is a small molecule and is the first selective persistent sodium current blocker in development for the treatment of a wide range of rare CNS disorders. Praxis plans to initiate a Phase 2 clinical trial in the first half of 2022 to explore the potential for PRAX-562 to treat a range of rare pediatric DEEs, including SCN2A-DEE and SCN8A-DEE. PRAX-562 is currently being evaluated in a Phase 1 clinical trial in adult healthy volunteers.

"Treatment with PRAX-562 has shown dose-dependent prevention of spontaneous seizures in an SCN2A animal model and complete reduction of seizures at the highest dose tested," said Bernard Ravina, M.D., chief medical officer of Praxis. "We are encouraged by these developments and the regulatory acknowledgement of SCN2A-DEE as an orphan disease, as we aim to bring forth treatments to those living with this devastating condition."

The FDA's orphan drug designation program is designed to encourage and facilitate the development of investigational treatments for rare diseases that affect fewer than 200,000 people in the United States. The designation provides various development and commercial incentives, including tax credits for clinical research costs, waiver or partial payment of application fees and market exclusivity for seven years following FDA approval.

About SCN2A-DEE

SCN2A-DEE is a rare developmental and epileptic encephalopathy caused by a variant in the SCN2A gene. The SCN2A gene is critical in the formation of sodium channel proteins in the brain, which control the flow of sodium ions into neurons. This movement of sodium ions is a major component of generating electrical signals called action potentials, the way in which the cells communicate. Many patients suffer from recurrent, typically drug-resistant seizures which can start as early as the first day of life. The seizures can be of multiple different types, up to dozens per day, with poor response to current treatment options. Patients with SCN2A-DEE have significant cognitive disabilities, ranging from moderate to severe; often movement disorders, such as dystonia or ataxia; and problems in other body systems such as gastrointestinal or ocular.

About Praxis

Praxis Precision Medicines is a clinical-stage biopharmaceutical company translating genetic insights into the development of therapies for central nervous system disorders (CNS) characterized by neuronal imbalance. Praxis is applying insights from genetic epilepsies to broader neurological and psychiatric disorders, using our understanding of shared biological targets and circuits in the brain. Praxis has established a broad portfolio, including multiple disclosed programs across CNS disorders including depression, epilepsy, movement disorders and pain syndromes, with three clinical-stage product candidates. For more information, please visit www.praxismedicines.com and follow us on [LinkedIn](#) and [Twitter](#).

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