



Praxis Precision Medicines Receives Orphan Drug Designation for PRAX-562 for the Treatment of SCN8A-DEE

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CAMBRIDGE, Mass., April 14, 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 for PRAX-562 for the treatment of SCN8A development and epileptic encephalopathy (SCN8A-DEE).

"We designed PRAX-562 with patients in mind, believing that focusing on targeting persistent sodium current rather than peak current has the potential to create a truly differentiated treatment for people living with a variety of CNS conditions," said Marcio Souza, president and chief executive officer of Praxis. "We look forward to sharing continued progress along our clinical development path for PRAX-562, as we seek to establish proof of concept in rare adult cephalgias later this year while continuing to advance toward studies in DEEs and potentially other conditions."

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 explore the potential for PRAX-562 in a range of rare pediatric DEEs, including SCN8A-DEE and SCN2A-DEE. The FDA has granted rare pediatric disease designations to PRAX-562 for the treatment of SCN8A-DEE and SCN2A-DEE. PRAX-562 is currently being evaluated in a Phase 1 clinical trial in adult healthy volunteers.

"The PRAX-562 data we have generated to date in animal models of epilepsy have been promising and it is encouraging that we have seen a favorable safety profile in our ongoing Phase 1 healthy volunteer trial," said Bernard Ravina, M.D., chief medical officer of Praxis. "In an SCN8A animal model, a clear dose response was observed with PRAX-562 resulting in complete protection of seizures in mice at the highest dose."

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 SCN8A-DEE

SCN8A-DEE is a rare developmental and epileptic encephalopathy caused by a variant in the SCN8A gene. The SCN8A 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. Patients suffer from recurrent, typically drug-resistant seizures which 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 SCN8A-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. SCN8A-DEE patients also may experience autonomic features such as increases or decreases in heart rate, abnormal breathing, and cyanosis.

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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