Praxis Precision Medicines Receives Rare Pediatric Disease and Orphan Drug Designations for Severe Pediatric Epilepsy Programs

January 7, 2021

FDA grants RPD and ODD for PRAX-222 for treatment of SCN2A-DEE

FDA grants RPD for PRAX-562 for treatment of SCN2A-DEE and SCN8A-DEE

CAMBRIDGE, Mass., Jan. 07, 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 (CNS) disorders characterized by neuronal imbalance, today announced that the U.S. Food and Drug Administration (FDA) has granted both rare pediatric disease and orphan drug designations for PRAX-222 for the treatment of SCN2A developmental and epileptic encephalopathy (SCN2A-DEE). The FDA has also granted rare pediatric disease designation for PRAX-562 for the treatment of SCN2A-DEE and for the treatment of SCN8A developmental and epileptic encephalopathy (SCN8A-DEE).

“As a company deeply rooted in the genetic drivers of severe pediatric epilepsies, these designations are especially meaningful and validating,” said Marcio Souza, president and chief executive officer of Praxis. “We are committed to drug development for both common and rare CNS diseases and look forward to progressing PRAX-562 and PRAX-222 in the clinic and ultimately to children who are in dire need of new treatment options.”

PRAX-562 is a selective small molecule and is the first persistent sodium current blocker in development for the treatment of a wide range of rare CNS disorders. The clinical development plan for PRAX-562 encompasses exploring the broad potential for the mechanism of action in rare diseases through proof-of-concept trials in two rare types of cephalgia, and then expanding into a range of rare pediatric DEEs, including SCN8A-DEE and SCN2A-DEE. PRAX-562 is currently being evaluated in a Phase 1 clinical trial in adult healthy volunteers.

PRAX-222 is an antisense oligonucleotide that is designed to lower the expression levels of the protein encoded by the SCN2A gene in patients with SCN2A gain-of-function epilepsy. The program is ongoing under a three-way collaboration with Ionis Pharmaceuticals, Inc. and RogCon, Inc. PRAX-222 is currently being evaluated in IND-enabling studies.

“The potential broad utility of PRAX-562 in DEEs and other rare CNS disorders and the precision therapy approach of PRAX-222 represent two differentiated and potentially complementary treatment paradigms inspired by human genetics,” said Steven Petrou, Ph.D., co-founder and chief scientific officer of Praxis. “The FDA granting these designations is an acknowledgement of the critical need to develop treatments for children living with these devastating diseases.”

The FDA’s rare pediatric disease designation program is designed for serious and life-threatening diseases primarily affecting children under the age of 18 with fewer than 200,000 people in the United States. Under the FDA’s rare pediatric disease and voucher programs, subject to FDA approval of a product with such designation, a company may be eligible to receive a priority review voucher that can be redeemed to obtain priority review for any subsequent marketing application or sold or transferred to other companies.

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/SCN8A-DEE
SCN2A-DEE and SCN8A-DEE are rare developmental and epileptic encephalopathies caused by variants in the SCN2A and SCN8A genes, respectively. Both the SCN2A and SCN8A genes are 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 SCN2A-DEE and 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. Those with SCN8A-DEE 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 characterized by neuronal imbalance. Praxis is applying insights into the genetic mutations that drive excitation-inhibition imbalance in diseases to select biological targets for severe pediatric epilepsies and more broadly for prevalent psychiatric diseases and neurologic disorders. Praxis has established a broad portfolio, including five disclosed programs across multiple central nervous system disorders including depression, epilepsy, movement disorders and pain syndromes, with three clinical-stage product candidates.

Investor Contact:
Alex Kane
Praxis Precision Medicines
investors@praxismedicines.com
617-300-8481

Media Contact:
Ian Stone
Canale Communications
Ian.stone@canalecomm.com
619-849-5388