



Praxis Precision Medicines to Advance PRAX-562 Phase 2 Study in Pediatric Patients with Developmental and Epileptic Encephalopathies

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BOSTON, Nov. 28, 2022 (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 excitation-inhibition imbalance, today announced plans to initiate the PRAX-562 Phase 2 EMBOLD study for the treatment of pediatric patients with developmental and epileptic encephalopathies (DEEs), following U.S. Food and Drug Administration (FDA) authorization to proceed with the study as proposed by Praxis, up to the planned maximum dose of 1.0 mg/kg/day. The EMBOLD Study is expected to initiate in the U.S in the first quarter of 2023, with two distinct cohorts in early-onset SCN2A-DEE and SCN8A-DEE patients. Topline results for both cohorts are expected in the second half of 2023.

"We designed PRAX-562 as a significantly differentiated sodium channel inhibitor specifically for the treatment of DEEs, with a goal of bringing the first precision treatment option to patients living with these devastating forms of epilepsy," said Steven Petrou, co-founder and chief scientific officer of Praxis. "Every day matters for these patients and their caregivers, and we look forward to initiating the EMBOLD study to evaluate the promise of PRAX-562 for the treatment of SCN2A, SCN8A and other DEEs."

The EMBOLD Study is a randomized, double-blind, placebo-controlled Phase 2 clinical trial to evaluate the safety, tolerability, efficacy (motor seizure frequency) and pharmacokinetics (PK) of PRAX-562 in pediatric participants aged 2 to 18 years with DEEs, followed by an open-label extension. Approximately 20 participants will be enrolled in a total of 2 distinct cohorts (n≈10 for SCN2A-DEE and n≈10 for SCN8A-DEE).

About PRAX-562

PRAX-562 is a first-in-class small molecule in development for the treatment of DEEs as a preferential inhibitor of persistent sodium current, shown to be a key driver of seizure symptoms in early onset SCN2A-DEE and SCN8A-DEE. In-vitro, PRAX-562 has demonstrated superior selectivity for disease-state NaV channel hyperexcitability and a wider therapeutic window compared to other anti-seizure medicines, with potential for enhanced efficacy and improved tolerability. In-vivo studies of PRAX-562 have demonstrated dose-dependent block of seizures up to complete inhibition of seizure activity in SCN2A, SCN8A and other DEE mouse models. PRAX-562 has been generally well-tolerated in three Phase 1 studies and has demonstrated biomarker changes indicative of NaV channel blocking effects. PRAX-562 has received Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation from the FDA, and ODD from the European Medicines Agency for the treatment of SCN2A-DEE and SCN8A-DEE respectively.

About SCN2A-DEE

SCN2A-DEE is a monogenic epilepsy disorder 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. SCN2A-DEE presents with a wide range of phenotypes. Early-onset SCN2A-DEE presents before three months and can lead to profound impact on patients, including drug-resistant seizures, significant cognitive impairment, movement disorders such as dystonia or ataxia and problems in other body systems such as gastrointestinal or ocular. Currently there are no approved treatments for SCN2A-DEE, and the standard-of-care typically involves a regimen of many concurrent anti-seizure medications as well as medications for co-morbidities. Despite these interventions, more than 70% of early-onset SCN2A-DEE patients live with uncontrolled seizures, and approximately 75% live with severe intellectual disability.

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 follow 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 CNS disorders characterized by neuronal excitation-inhibition imbalance. Praxis is applying insights from genetic epilepsies to both rare and more prevalent neurological disorders, using our understanding of shared biological targets and circuits in the brain. Praxis has established a broad portfolio including multiple programs across movement disorders and epilepsy, with four clinical-stage product candidates. For more information, please visit www.praxismedicines.com and follow us on [LinkedIn](#) and [Twitter](#).

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995 and other federal securities laws, including express or implied statements regarding Praxis' future expectations, plans and prospects, including, without limitation, statements regarding the anticipated timing of Praxis' clinical trials, our expectations, plans and timing for Praxis' clinical data and the development of

Praxis' product candidates, including the design of clinical trials and the treatment potential of Praxis' product candidates, as well as other statements containing the words "anticipate," "believe," "continue," "could," "endeavor," "estimate," "expect," "anticipate," "intend," "may," "might," "plan," "potential," "predict," "project," "seek," "should," "target," "will" or "would" and similar expressions that constitute forward-looking statements under the Private Securities Litigation Reform Act of 1995.

The express or implied forward-looking statements included in this press release are only predictions and are subject to a number of risks, uncertainties and assumptions, including, without limitation: uncertainties inherent in clinical trials; the expected timing of submissions for regulatory approval or review by governmental authorities; risks, uncertainties and assumptions regarding the impact of the continuing COVID-19 pandemic on Praxis' business, operations, strategy, goals and anticipated timelines, Praxis' ability to initiate, enroll, conduct or complete ongoing and planned clinical trials and Praxis' timelines for regulatory submissions; and other risks concerning Praxis' programs and operations as described in its Quarterly Report on Form 10-Q for the quarter ended June 30, 2022 and other filings made with the Securities and Exchange Commission. Although Praxis' forward-looking statements reflect the good faith judgment of its management, these statements are based only on information and factors currently known by Praxis. As a result, you are cautioned not to rely on these forward-looking statements. Any forward-looking statement made in this press release speaks only as of the date on which it is made. Praxis undertakes no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future developments or otherwise.

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