Praxis Precision Medicines to Present Data from Epilepsy Portfolio at the 35th International Epilepsy Congress

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BOSTON, Aug. 31, 2023 (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 that it will present data from two of its clinical-stage epilepsy programs at the upcoming 35th International Epilepsy Congress (IEC), to be held on September 2-6, 2023 in Dublin, Ireland.

“There remains a great unmet need in epilepsy for anti-seizure medications that restore quality of life to patients and their families, and we are committed to developing best-in-class treatment options for both rare and prevalent epilepsies,” said Marcio Souza, president and chief executive officer of Praxis. “Data from our Phase 1 study of PRAX-628 for focal epilepsy support a potentially best-in-class safety profile that could address the limitations of existing therapies, such as poor tolerability and dose-limiting titration requirements. We look forward to presenting data from this program along with data from our PRAX-562 program for children with SCN2A and SCN8A developmental and epileptic encephalopathies.”

Presentation Details:

PRAX-628: A Next Generation Functionally Selective Small Molecule with Potent Anticonvulsant Activity

- Session Date/Time: Sunday, September 3, 1:00 p.m. – 3:30 p.m. IST
- Poster Session: Drug Therapy
- Poster number: P031

PRAX-562 is a Well-Tolerated, Next Generation Anti-Seizure Small Molecule with Broad Anticonvulsant Activity in Multiple DEE Mouse Models

- Session Date/Time: Sunday, September 3, 1:00 p.m. – 3:30 p.m. IST
- Poster Session: Pediatric Epileptology
- Poster number: P095

PRAX-562-101: A First-in-Human Phase 1 Trial Evaluating the Safety, Tolerability, Pharmacokinetics and Food Effect of PRAX-562 in Healthy Volunteers

- Session Date/Time: Monday, September 4, 7:00 p.m. – 7:40 p.m. IST
- Poster Session: Pediatric Epileptology & Mixed
- Poster number: 408

PRAX-562-102: A Phase 1 Trial Evaluating the Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of PRAX-562 in Healthy Volunteers

- Session Date/Time: Monday, September 4, 7:00 p.m. – 7:40 p.m. IST
- Poster Session: Pediatric Epileptology & Mixed
- Poster number: 409

Safety, Tolerability and Pharmacokinetic Findings from a First-in-Human, Randomized, Double-Blinded, Placebo-Controlled Trial of Single and Multiple Ascending Doses of PRAX-628 in Healthy Participants

- Session Date/Time: Tuesday, September 5, 1:00 p.m. – 3:30 p.m. IST
- Poster Number: P292

About PRAX-562
PRAX-562 is a first-in-class small molecule in development for the treatment of developmental and epileptic encephalopathy (DEE) 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. PRAX-562’s mechanism of sodium channel block is consistent with superior selectivity for disease state sodium channel (Na_V) channel hyperexcitability. In vivo studies of PRAX-562 have demonstrated dose-dependent inhibition of seizures up to complete control 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 Na_V 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.

About PRAX-628
PRAX-628 is a next-generation, functionally selective small molecule targeting the hyperexcitable state of sodium-channels in the brain that is currently being developed as a once daily, oral treatment for adult focal onset seizures. Preclinical data demonstrates PRAX-628 is differentiated from standard of care, with the potential to be best-in-class for focal epilepsy. In vitro, PRAX-628 has demonstrated superior selectivity for disease-state Na_V channel hyperexcitability. In vivo studies of PRAX-628 have demonstrated unprecedented potency in the maximal electroshock seizure (MES) model, a highly predictive translational model for efficacy in focal epilepsy. Data from the PRAX-628-101 study demonstrated that PRAX-628 can be safely dosed in healthy subjects to greater than 15 times the predicted human equivalent of the rodent MES EC_{50}, a translational indicator that suggests a therapeutic window with unprecedented magnitude relative to approved therapies.

About Focal Epilepsy
Focal epilepsy is the most prevalent type of epilepsy, accounting for approximately 60% of all cases. In the United States alone, it is estimated that focal epilepsy affects approximately two million people. Focal epilepsy is characterized by seizures that originate in one side or area of the brain that can spread to other parts of the brain and body. Despite the plethora of approved treatments, up to 30% of patients continue to have seizures with significant impact on their quality of life. Novel compounds that specifically modulate sodium channels participating in pathological neuronal activity while sparing those participating in physiological activity may improve clinical efficacy and tolerability.

About SCN2A-DEE
SCN2A-DEE is a monogenic epilepsy disorder caused by a variation in the SCN2A gene. The SCN2A gene encodes 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 neurons communicate. SCN2A-DEE presents with a wide range of phenotypes. Early-onset SCN2A-DEE presents before three months of age 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 medications for seizures as well as 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 disabilities.

About SCN8A-DEE
SCN8A-DEE is a rare developmental and epileptic encephalopathy caused by a variation in the SCN8A gene. The SCN8A gene encodes 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 neurons 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 insights from genetic epilepsies into the development of therapies for CNS disorders characterized by neuronal excitation-inhibition imbalance. Praxis is applying genetic insights to the discovery and development of therapies for rare and more prevalent neurological disorders through our proprietary small molecule platform, Cerebrum™, and antisense oligonucleotide (ASO) platform, Solidus™, using our understanding of shared biological targets and circuits in the brain. Praxis has established a diversified, multimodal CNS 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 Facebook, LinkedIn and Twitter/X.

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