Praxis Precision Medicines to highlight their Epilepsy Portfolio at the International League Against Epilepsy 15th European Epilepsy Congress with six presentations

September 4, 2024 at 8:00 AM EDT

BOSTON, Sept. 04, 2024 (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 that it will present preclinical and clinical data from three of its epilepsy programs at the International League Against Epilepsy (ILAE) 15th European Epilepsy Congress (EEC), being held from September 7 to 11, 2024 in Rome, Italy.

“We are pleased to highlight the continued progress across our portfolio of epilepsy assets, building on the strong evidence we have already generated in the elsunersen EMBRAVE study and the relutrigine EMBOLD study,” said Steven Petrou, chief scientific officer and co-founder of Praxis. “We are currently enrolling registrational trials for elsunersen and relutrigine, and look forward to initiating our ENERGY program with PRAX-628 this year.”

Praxis at EEC 2024 | Roma Convention Center La Nuvola

Connect with members of our team at booth #441 or head to one of our presentations listed below where we will share updates across our epilepsy portfolio.

Oral Presentation

- Tuesday, September 10, 01:02 p.m. – 01:10 p.m. CEST
- Location: Caravaggio
- Session: Platform Session – Pediatric Epileptology

Significant Seizure Reduction in Pediatric Participants with Early Onset SCN2A Developmental and Epileptic Encephalopathy following Treatment with Elsunersen, a Novel Antisense Oligonucleotide: Findings from the EMBRAVE Study

Poster Presentations

- Sunday – Tuesday, September 8 – 10, 1:30 p.m. – 3:00 p.m. CEST
- Location: Forum

P083. Pharmacokinetics, Tolerability and Cardiac Safety for PRAX-628, a Precision Medicine Therapeutic Set to Initiate Registration Enabling Studies for Focal Onset Seizures

P898. A Novel Antisense Oligonucleotide for the Treatment of Early Onset SCN2A Developmental and Epileptic Encephalopathy: A First-in-Patient Report in a Preterm Infant with Refractory Status Epilepticus

P908. EMBOLD: A Clinical Trial of PRAX-562 in Subjects with Developmental and Epileptic Encephalopathies

P074. A Novel Translational Concordance Framework Identifies Preclinical Seizure Models with Highest Predictive Validity in Focal Onset Seizures

P339. A Novel Method to Define an EEG Composite for the Detection of Drug Effects of Next Generation Small Molecules for Epilepsy

About Relutrigine (PRAX-562)
Relutrigine is a first-in-class small molecule in development for the treatment of developmental and epileptic encephalopathies (DEEs) as a preferential inhibitor of persistent sodium current, shown to be a key driver of seizure symptoms in severe DEEs. Relutrigine’s mechanism of precision sodium channel (NaV) modulation is consistent with superior selectivity for disease-state NaV channel hyperexcitability. In vivo studies of relutrigine have demonstrated dose-dependent inhibition of seizures up to complete control of seizure activity in SCN2A, SCN8A and other DEE mouse models. Relutrigine has been generally well-tolerated in three Phase 1 studies and has demonstrated biomarker changes indicative of NaV channel modulation. Data from the Phase 2 EMBOLD study demonstrated in a heavily pre-treated population a well-tolerated, robust, short- and long-term improvement in motor seizures alongside maintained seizure freedom in some patients with SCN2A- and SCN8A-DEE. Relutrigine 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 NaV channels in the brain that is currently being developed as a once daily, oral treatment for adult focal onset seizures and generalized epilepsy. Preclinical data demonstrates PRAX-628 is differentiated from standard of care, with the potential to be best-in-class for focal onset seizures. In vitro, PRAX-628 has demonstrated superior

selectivity for disease-state NaV 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 EC50, a translational indicator that suggests a therapeutic window with unprecedented magnitude relative to approved therapies.

About Elsunersen (PRAX-222)
Elsunersen is an antisense oligonucleotide (ASO) designed to selectively decrease SCN2A gene expression, directly targeting the underlying cause of early- onset SCN2A-DEE to treat seizures and other symptoms in patients with gain-of-function SCN2A mutations. In vitro studies of elsunersen have demonstrated reduction in both SCN2A gene expression and protein levels. In vivo, elsunersen has demonstrated significant, dose-dependent reduction in seizures, improvement in behavioral and locomotor activity and increased survival in SCN2A mouse models. Data from the EMBRAVE study demonstrated well-tolerated, significant and sustained seizure reduction in patients with SCN2A-DEE. Elsunersen has received Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation (RPD) from the FDA, and ODD and PRIME designations from the European Medicines Agency (EMA) for the treatment of SCN2A-DEE. The Elsunersen program is ongoing under a collaboration with Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), and RogCon, Inc. To learn more about the EMBRAVE study, please visit https://www.embravestudy.com/.

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, Instagram, LinkedIn and Twitter/X.

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