# Praxis Precision Medicines to Showcase Updates from Largest Epilepsy Pipeline of Precision Epilepsy Programs at the 2024 American Epilepsy Society Annual Meeting

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BOSTON, Nov. 26, 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 American Epilepsy Society (AES) Annual Meeting, being held from December 6 to 10, 2024 in Los Angeles, California.

“At Praxis, we are on the brink of transformative change with a leading pipeline that includes relutrigine and vormatrigine, the most potent and most functionally selective anti-seizure medications developed to date,” said Steven Petrou, chief scientific officer and co-founder of Praxis. “Following promising results with the relutrigine EMBOLD study in particularly challenging childhood epilepsies, we are confident that these future therapies will redefine treatment for DEEs as well as focal and generalized epilepsy. This year at AES we will be sharing the latest advances across our epilepsy portfolio through an exciting lineup of activities.”

Praxis will have multiple options for visitors to learn more about its portfolio:

- *Exhibiting* at **booth #1235**, where visitors can interact with members of the Praxis team
- *Presenting* five unique posters covering three of its clinical-stage assets, detailed below
- *Hosting* a scientific exhibit featuring its leading portfolio of precision epilepsy programs, detailed below

**Poster Presentations**

- Saturday December 7, 12:00 p.m. – 2:00 p.m. PST
- Location: South Hall H, Level 1

**1.398. Vormatrigine Demonstrates Potent Antiseizure Activity Across Three Acute Models with Highest Predictive Validity for Focal Onset Seizures**

**1.525. Emergency Use Case of Relutrigine, a Next-Generation Sodium Channel Functional State Modulator, in an Infant with SCN2A-DEE and Refractory Seizures and Recurrent Status Epilepticus**

**1.526. Clinical Updates from the Elsunersen Emergency Use Program: A Novel ASO for Treatment of Early Onset SCN2A Developmental and Epileptic Encephalopathy**

**1.527. Establishing the Predictive Validity of Preclinical Seizure Models in Generalized Epilepsies: An Extension of the Praxis Analysis of Concordance Framework**

**1.528. Relutrigine Demonstrates Robust Seizure Reduction and Seizure Freedom in DEEs: Results from the EMBOLD Study**

Materials will be made available on the Resources page of the Praxis website following presentation at AES 2024: https://praxismedicines.com/resources/.

**Scientific Exhibit**

- **Praxis Precision Medicines: Revolutionizing Therapy in Epilepsy**

- Monday December 9, 2:00 p.m. – 5:00 p.m. PST
- Location: Room 403B, Level 2

**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. To learn more about the EMBOLD study, please visit https://www.emboldstudy.com/.

**About Vormatrigine (PRAX-628)**
Vormatrigine 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 vormatrigine is differentiated from standard of care, with the potential to be best-in-class for focal onset seizures. In vitro, vormatrigine has demonstrated superior selectivity for disease-state NaV channel hyperexcitability. In vivo studies of vormatrigine 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 vormatrigine 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.org/.

**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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