Praxis Precision Medicines to Present on Late-Stage Programs across Epilepsy and Movement Disorders at the American Academy of Neurology 2025 Annual Meeting

March 24, 2025 at 8:01 AM EDT

BOSTON, March 24, 2025 (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 data from its four late-stage programs in epilepsy and movement disorders at the American Academy of Neurology (AAN) 2025 Annual Meeting taking place from April 5 to 9, 2025 in San Diego, California.

“Praxis’s innovative pipeline continues to rapidly advance, with four late-stage programs poised to transform patient care,” said Steven Petrou, Chief Scientific Officer and Co-Founder of Praxis. “At this year’s AAN, we’re excited to present the latest developments across our diverse portfolio, including key clinical updates from the vormatrigine ENERGY program and newly initiated clinical trials actively enrolling patients across the U.S., Europe, and Latin America. We are especially pleased to host an engaging In-Booth Speaker Showcase featuring renowned experts and collaborators who share our mission of revolutionizing therapies for CNS disorders.”

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

- *Exhibiting* at **booth #2113** where visitors can interact with members of the Praxis team including a dedicated networking event, detailed below
- *Hosting* an In-Booth Speaker Showcase covering three of its clinical-stage assets, and featuring an exciting line up of leading epilepsy and movement disorders experts, detailed below
- *Presenting* four unique posters covering two of its clinical-stage assets, detailed below

Praxis Booth

Connect with members of our team at booth #2113 during exhibit hours or join us for the following:

- **In-Booth Networking Crawl** – *refreshments served*
  - Monday, April 7 | 4:00 PM – 6:00 PM PT | Booth 2113 | Exhibit Hall A-D
- **In Booth Speaker Showcase** **– *Praxis Precision Medicines: Revolutionizing CNS Therapies***
  - Sunday 6 April – Wednesday 9 April | 1:30 PM – 3:30 PM PT | Exhibit Hall A-D

Praxis Poster Presentations
P5-011: Spotlight on Essential Tremor Management and Impact: Results from an Online Survey Capturing HCP and Patient Perspectives of Disease Burden

- Saturday, April 5 | 11:45 AM – 12:45 PM PT | Exhibit Hall A

P5-012: Characteristics of Adult Essential Tremor Patients Seeking Participation in a Decentralized US Clinical Trial: Pre-screener Findings from the Essential3 Program Evaluating Efficacy and Safety of Ulixacaltamide

- Saturday, April 5 | 11:45 AM – 12:45 PM PT | Exhibit Hall A

P9-012: Epilepsy Monitoring of Prospective Seizure Observations with Electronic Records (EMPOWER): An Observational Study Designed to Better Understand the Patient Journey

- Monday, April 7 | 8:00 AM – 9:00 AM PT | Exhibit Hall A

P4-006: Updates from the First-in-human Phase 1 Clinical Trial Evaluating the Safety, Tolerability, Pharmacokinetics and Food Effect of Vormatrigine in Healthy Participants

- Wednesday, April 9 | 8:00 AM – 9:00 AM PT | Exhibit Hall A

Materials will be made available on the Resources page of the Praxis website following presentation at AAN 2025: https://praxismedicines.com/resources/.

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. epilepsy-research.com

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 Ulixacaltamide
Ulixacaltamide is a differentiated and highly selective small molecule inhibitor of T-type calcium channels designed to block abnormal neuronal burst firing in the cerebello-thalamo-cortical (CTC) circuit correlated with tremor activity. Ulixacaltamide, the most advanced program within Praxis' Cerebrum™ small molecule platform, is currently in development for the treatment of essential tremorwww.praxisessentialtremor.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 epilepsy and movement disorders, 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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