Praxis AES Wrap-up: Best-in-Class Potential Across Rare Pediatric and Adult Epilepsies

December 8, 2025

Positive Results in the EMBOLD study of relutrigine showed a 53% placebo-adjusted reduction in seizures ($p < 0.0002$), 66% increase in motor seizure-free days ($p = 0.0340$), broad and clinically meaningful functional improvements ($p \leq 0.002$)

Patients in the RADIANT study of vormatrigine showed rapid, consistent and durable improvement in seizure reduction, reaching 100% for patients continuing through 16 weeks

Expansion of the Praxis Analysis of Concordance Framework to include DEEs

Showcase of the most comprehensive epilepsy portfolio

BOSTON, Dec. 08, 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 provided clinical updates shared at the American Epilepsy Society Annual Meeting (AES) on December 6, 2025.

“The updates we shared at AES mark a pivotal moment for epilepsy treatment. The EMBOLD results highlight the long-awaited potential of relutrigine to become the first disease modifying treatment for SCN2A and SCN8A-DEE patients and show that we are positioned to deliver the same impact for the broader DEE community,” said Marcio Souza, president and chief executive officer. “Additionally, the update from the RADIANT study for focal-onset seizures and generalized epilepsy confirmed its potential as a best-in-class therapy. We are committed to accelerating these options to patients while continuing to accelerate the broad DEE study, EMERALD, our ASO programs and the FOS studies toward registration.”

Relutrigine for Developmental and Epileptic Encephalopathies (DEEs):
At AES, Praxis shared results [link to poster] of the EMBOLD study, demonstrating relutrigine was well-tolerated with rapid, significant and increasing seizure reduction over time with broad functional improvements across behavior, alertness, communication and overall status. Praxis will meet with the FDA in the coming weeks to discuss next steps for the New Drug Application (NDA). Praxis will make a determination of the timing for filing the NDA after the meeting.

- Patients receiving relutrigine (n=51) experienced a 53% placebo-adjusted reduction in seizures over 16-weeks ($p<0.0002$)
- Patients achieved a 66% increase in motor seizure-free days ($p=0.034$)
- Both clinician and caregiver global impression scores showed statistically significant improvements, with most patients improving across both scales in alertness, communication, and seizure severity.
- There were no drug-related serious adverse events and treatment-related adverse events were predominantly mild and moderate.

Vormatrigine for Focal Onset Seizures (FOS) and Generalized Epilepsy: RADIANT results [link to poster] position vormatrigine as a best-in-disease therapy: fast-acting efficacy without titration, sustained reduction over longer treatment duration, seizure-freedom potential, favorable DDI, tolerability and safety profiles with once-daily dosing.

Focal Onset Seizures (n=62)

- Patients taking vormatrigine for 8 weeks on background anti-seizure medications (ASM) saw a 54% median reduction in seizures.
- In week 1, 58% of patients achieved at least a 50% reduction in seizures, which increased to 61% by week 8.
- Increasing and sustained effect was observed, with FOS patients reaching 100% median weekly seizure reduction after 8 weeks and maintained through 16 weeks.
- Over 11% of patients experienced seizure freedom for the entire 8-week period and roughly one third of patients experienced seizure freedom for a consecutive 28-day period.

Generalized Epilepsy (n=3)

- Three patients with generalized epilepsy included in the cohort experienced a similar treatment effect as FOS patients, with rapid, durable seizure reduction.

Praxis has completed recruiting for the POWER1 pivotal study in FOS and is on track to complete the POWER2 study in the second half of 2026. The monotherapy study, POWER3, is on track to begin in the first half 2026. Additional posters presented at AES at Resources - Praxis Medicines

About Vormatrigine (PRAX-628)
Vormatrigine 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 and generalized epilepsy. Preclinical data demonstrates vormatrigine is differentiated from standard of care, with the potential to be best-in-class for focal epilepsy. 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 first cohort of patients in the RADIANT study demonstrated a robust seizure reduction and generally safe and well tolerated profile. To learn more about the POWER1 and POWER2 studies, please visit POWER1 study.

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 cohort 1 of the Phase 2 EMBOLD study demonstrated a well-tolerated, robust, short- and long-term improvement in motor seizures in a heavily pre-treated population, 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 for the treatment of SCN2A-DEE, SCN8A-DEE and Dravet syndrome; as well as Breakthrough Therapy Designation (BTD), and ODD from the European Medicines Agency for the treatment of SCN2A-DEE and SCN8A-DEE. To learn more about the EMERALD and EMBOLD studies, please visit ResilienceStudies.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, LinkedIn and Twitter/X.

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 our clinical trials, the development of our product candidates and the anticipated timing of regulatory submissions and interactions, 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; preliminary analyses from ongoing studies differing materially from final data from preclinical studies and completed clinical trials; the expected timing of clinical trials, data readouts and the results thereof, and submissions for regulatory approval or review by governmental authorities; regulatory approvals to conduct trials; and other risks concerning Praxis' programs and operations as described in its Annual Report on Form 10-K for the year ended December 31, 2024 and as updated in the Quarterly Report on Form 10-Q for the period ended June 30, 2025, as well as 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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