Praxis Precision Medicines Announces Positive, Best-in-Disease Topline Results in Patients with Focal Onset Seizures from the RADIANT Study of Vormatrigine

August 4, 2025

Dosing with vormatrigine over 8 weeks led to 56.3% median reduction in seizure frequency

Approximately 22% of patients reached 100% reduction in seizure frequency in the last 28 days on treatment

Rapid and sustained response, with over 54% of patients achieving 50% response in the first week

Vormatrigine was generally well tolerated and continues to demonstrate favorable safety profile

BOSTON, Aug. 04, 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 topline results from the Phase 2 RADIANT study evaluating vormatrigine in patients with focal onset seizures.

“We are thrilled by the best-in-disease topline results of this first cohort for the RADIANT study and for the potential to deliver a fast-acting, highly efficacious and tolerable therapy to patients living with focal epilepsy. Many of the over three million patients living with common epilepsy in the U.S. need to manage multiple anti-seizure medications, and vormatrigine is the only drug in development aiming to become first line and address the market at large.”

“These findings build on our earlier clinical data showing a differentiated profile for vormatrigine as a fast-acting, no-titration, once-daily oral drug with no requirement to be taken with food, and a favorable DDI profile, all of which are unseen in ASMs currently in the market or in development. We are well on track to complete the pivotal, 12-week POWER1 study in Q4 and, based on the results from RADIANT, expect to initiate the POWER2 study shortly. We are deeply grateful to the patients, caregivers and clinicians who are contributing to the vormatrigine trials,” said Marcio Souza, president and chief executive officer of Praxis.

“While several sodium channel therapies are already used to treat focal onset seizures, there remains substantial room for improvement within this class. A next-generation sodium channel blocker has the potential to represent an important step forward in addressing that gap. I'm encouraged by the initial results of the RADIANT study and hopeful that, in the future, we may be able to offer patients another effective treatment option,” said Jacqueline French, MD, Professor at NYU Langone Health's Comprehensive Epilepsy Center.

Scientific Presentations

Additional data will be presented at the 36th International Epilepsy Congress on August 31, 2025, in Lisbon, Portugal.

Praxis has submitted a late-breaker abstract to present the full study results at the American Epilepsy Society Annual Meeting in December 2025 in Atlanta, Georgia.

Conference Call
Praxis will discuss the study results, as well as its second quarter 2025 financial results and business highlights on a conference call taking place today, August 4, 2025 at 8:30 am ET. Individuals may register for the conference call by clicking the registration link. Once registered, participants will receive dial-in details and a unique PIN which will allow them to access the call. An audio webcast will be accessible through the Events & Presentation page under the Investor Relations section of the Company’s website.

About Vormatrigine
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 study, please visit POWER1 study.

About the RADIANT study:

Link to the RADIANT study NCT06908356

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.

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 clinical development and potential benefits of vormatrigine, the anticipated timing of our clinical trials and our participation in upcoming events and presentations, 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; 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 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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