Praxis Precision Medicines Provides Vormatrigine Program Update

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POWER1 Study in highly refractory patients with focal onset seizures did not meet its primary success measure

Secondary measure, the 50% response rate, was met and seizure reduction during the second half of the study on higher dose (30 mg) was more pronounced

Vormatrigine was generally well-tolerated; adverse event-related discontinuations were less than 10%

Approximately 90% of patients from the vormatrigine arm transitioned to and remain in the open label extension (OLE) study

Praxis is pausing enrollment in the POWER2 study to reassess the vormatrigine program and determine potential modifications

BOSTON, June 01, 2026 (GLOBE NEWSWIRE) -- Praxis Precision Medicines, Inc. (NASDAQ: PRAX), a fully integrated, leading central nervous system (CNS) precision neuroscience biopharmaceutical company, today announced results from the Phase 2/3 POWER1 study evaluating vormatrigine in patients with focal onset seizures (FOS).

“While the results for POWER1 were not what we hoped for, we are encouraged by the signal we saw on the higher dose arm, the low discontinuation rate and solid safety profile,” said Marcio Souza, President and Chief Executive Officer of Praxis. “We will take some time to review these results to ensure we have the best path forward for developing vormatrigine and the ongoing POWER2 study. We continue to focus on preparing for the planned launches for relutrigine and ulixacaltamide.”

POWER1 (NCT06999902) is a double-blind, randomized, multicenter Phase 2/3 trial that evaluated the safety and efficacy of vormatrigine in adults with FOS who are concurrently taking at least 1 but no more than 3 acceptable anti-seizure medications (ASMs). Patients were randomized to receive either once daily 20 mg/day of vormatrigine for 6 weeks and then once daily 30 mg/day of vormatrigine for another 6 weeks or once daily placebo for 12 weeks. The primary endpoint was the percent change in monthly seizure frequency from baseline.

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 demonstrate 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 patients in the RADIANT study demonstrated a robust seizure reduction and generally safe and well tolerated profile.

About Praxis
Praxis Precision Medicines is a fully integrated, leading central nervous system (CNS) precision neuroscience 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 late-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 development of our product candidates, including vormatrigine, 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, 2025 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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