Praxis Precision Medicines Announces Positive Results from EMBOLD Study for Relutrigine in SCN2A and SCN8A Developmental and Epileptic Encephalopathies

December 4, 2025

Successful interim analysis triggered early stop for efficacy

Topline study results to be shared at the American Epilepsy Society Annual Meeting on December 6, 2025

Praxis confirms an upcoming meeting with the FDA to discuss the results and NDA path

BOSTON, Dec. 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 positive results from the registrational cohort of the EMBOLD study evaluating relutrigine for the treatment of patients with SCN2A and SCN8A developmental and epileptic encephalopathies (DEEs), following a recommendation by the Data Monitoring Committee to stop the study early for efficacy.

“SCN2A and SCN8A DEEs are devastating conditions with extremely high mortality due to the debilitating seizure burden they impose on patients, and there are currently no approved treatment options. Our progress represents an important milestone towards delivering the first therapy ever designed for these children and their families” said Marcio Souza, president and chief executive officer. “We look forward to sharing the results at the American Epilepsy Society Annual Meeting.”

The FDA has confirmed a meeting to review the data and discuss next steps in the coming weeks. Praxis will make a determination on the timing for filing an NDA for relutrigine after the meeting.

The EMBOLD topline results will be presented at the American Epilepsy Society Annual Meeting at the Georgia World Congress Center, Poster Hall B2, 1.38 from 12:00-2:00 p.m. on Saturday, December 6, 2025.

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 safety profile with 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, 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 Praxis’ 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; 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 its Quarterly Report on Form 10-Q for the quarter 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.

Investor Contact: Praxis Precision Medicines investors@praxismedicines.com 857-702-9452 Media Contact: Dan Ferry Life Science Advisors Daniel@lifesciadvisors.com 617-430-7576