Praxis Precision Medicines Receives FDA Breakthrough Therapy Designation for Relutrigine for the Treatment of Seizures Associated with SCN2A and SCN8A Developmental and Epileptic Encephalopathies

July 17, 2025

The Breakthrough Therapy Designation (BTD) was granted based on the highly compelling results from the Phase 2 EMBOLD trial in SCN2A and SCN8A developmental and epileptic encephalopathies (DEEs)

The EMBOLD cohort 2 pivotal trial is on track for topline results in H1 2026 with NDA filing to follow

Praxis has recently initiated the EMERALD study investigating relutrigine broadly in DEEs

BOSTON, July 17, 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 the U.S. Food and Drug Administration (FDA) has granted BTD for relutrigine, a sodium channel functional state modulator for pediatric use for the treatment of patients with SCN2A and SCN8A DEEs.

"This BTD represents a significant milestone for our relutrigine program and further validates its potential. The EMBOLD cohort 1 study supporting our application enrolled the most severe DEE population ever studied and included patients that failed three treatments on average before joining the study. The results from the open-label extension are remarkable, showing an average of both approximately 90% reduction in seizures and over two months between seizures. These extremely encouraging clinical results translate to tangible improvement in quality of life for patients and their caregivers. We continue to execute on the registrational EMBOLD cohort 2 study and expect to share the topline results no later than the first half of 2026. We are excited that this BTD, in addition to the Rare Pediatric Disease Designation in SCN2A and SCN8A DEEs, further supports expedited development of relutrigine," commented Marcio Souza, president and chief executive officer. "In addition to this exciting update for the SCN2A and SCN8A population, we are looking forward to results from the EMERALD study which has recently been initiated for DEE patients, regardless of etiology."

The BTD enables expedited development and regulatory review for drugs that are intended to treat a serious condition, and preliminary clinical evidence indicates that relutrigine may demonstrate substantial improvement on a clinically significant endpoint(s) over existing therapies. The BTD for relutrigine was supported by the positive data from cohort 1 in the Phase 2 EMBOLD study, as well as 11-month data from the open-label extension (OLE) period of the trial. Data from the double-blind period (16 weeks of treatment) showed a placebo-adjusted monthly motor seizure reduction of 46% during the double-blind period and seizure freedom in over 30% of patients while on relutrigine. Additionally, meaningful gains were observed in alertness, communication and seizure severity as noted by both clinicians and caregivers. In May 2025, Praxis provided a data update from the ongoing OLE part of the trial during a virtual investor event. At month 11, an average of approximately 90% seizure reduction was observed in patients, and a sustained and continuous improvement in seizure-free periods was observed, with a mean of 67 days without seizures compared to 3 days in the baseline period. There were no new safety signals, drug related serious adverse events or dose reductions needed.

The EMBOLD registrational cohort 2 is currently ongoing and continues to enroll, with topline results expected no later than the first half of 2026, followed by a potential NDA filing.

Praxis has recently initiated the EMERALD study evaluating relutrigine in patients across all DEEs. EMERALD is a registrational, 16-week, placebo-controlled study evaluating seizure reduction in patients diagnosed with developmental epilepsies.

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 epilepsy and movement disorders, 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; 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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