Praxis Precision Medicines Announces FDA Acceptance and Priority Review of New Drug Application for Relutrigine in Patients with SCN2A and SCN8A DEEs

March 30, 2026

FDA assigned PDUFA target action date of September 27, 2026

BOSTON, March 30, 2026 (GLOBE NEWSWIRE) -- Praxis Precision Medicines, Inc. (NASDAQ: PRAX), a fully integrated, leading central nervous system (CNS) precision neuroscience biopharmaceutical company, today announced that the U.S. Food and Drug Administration (FDA) has accepted for priority review its New Drug Application (NDA) for relutrigine, for the treatment of SCN2A and SCN8A developmental and epileptic encephalopathies (DEEs). The FDA has set a target action date under the Prescription Drug User Fee Act (PDUFA) of September 27, 2026.

"Our first FDA acceptance of an NDA submission marks a significant milestone in our evolution to a commercial-stage company and an important step toward delivering innovative, precision neuroscience therapies to patients in need. SCN2A/8A DEEs have no currently approved targeted therapies and relutrigine, if approved, would be the first disease-modifying therapy for children suffering from these devastating and fatal conditions. We look forward to working closely with the FDA during the review process while continuing to advance our launch preparations," said Marcio Souza, president and chief executive officer.

Relutrigine for treatment of SCN2A/8A DEEs
The NDA is supported by positive results from the EMBOLD study, which was stopped early for efficacy following a successful interim analysis and recommendation from the Data Monitoring Committee. Relutrigine has an Orphan Drug Designation, as well as a Rare Pediatric Disease Designation and a Breakthrough Therapy Designation. If granted approval, relutrigine will be the first FDA-approved therapy for SCN2A/8A DEE as well as be eligible for a Pediatric Review Voucher.

Relutrigine is also being investigated in broad DEEs through the EMERALD trial, which is expected to be completed by the end of 2026.

About Relutrigine
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 study, please visit Emerald | Resilience Studies.

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, LinkedIn and X/Twitter.

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 clinical trials, the anticipated timing of regulatory submissions and interactions and potential market opportunity and commercial potential of Praxis' product candidates, 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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