Praxis Precision Medicines Announces Alignment with FDA on Simplified and Accelerated Registrational Pathway for Elsunersen in Early Onset SCN2A Developmental and Epileptic Encephalopathy

December 9, 2025

Clear recognition of high unmet need and urgency for the SCN2A community and plausibility of the mechanism of elsunersen

FDA agreed to proposed changes to the EMBRAVE3 trial design to be a single-arm, baseline-controlled study

Enrollment in EMBRAVE3 is quickly accelerating and topline results expected in 2026

Topline results from ongoing EMBRAVE study (Part A, n=9) expected in 1H 2026

BOSTON, Dec. 09, 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 the completion of a Type C meeting with the U.S. Food and Drug Administration (FDA) and agreement to immediately convert the EMBRAVE3 registrational study of elsunersen in early-onset SCN2A developmental and epileptic encephalopathy (DEE) into a single-arm study where all patients will receive elsunersen for 24 weeks, followed by an open-label extension.

Key Changes to EMBRAVE3

- The current study has been immediately converted from a double-blind, sham-controlled study to a single-arm, baseline-controlled study, enrolling 30 patients reduced from 40 patients.
- All patients currently in screening will be assigned to receive elsunersen.
- The primary analysis will be the change from baseline in countable motor seizures.

Update on EMBRAVE Study status

- The EMBRAVE Study Part A enrolled 9 patients randomized 3:1 to elsunersen or placebo/sham for 20 weeks, followed by a blinded transition to elsunersen for up to 2 years in an open-label extension.
- Praxis expects to complete Part A and disclose the topline results in the first half of 2026.

“This alignment with the FDA represents a meaningful step forward for patients and families living with SCN2A-DEE. The Agency’s recognition of both the urgency of the unmet need and the strong mechanistic rationale for elsunersen enables us to move with greater clarity and speed. Converting EMBRAVE3 to a single-arm, baseline-controlled study ensures that every child entering the trial will receive active treatment from day one, while preserving a rigorous and approvable pathway. Momentum in enrollment continues to build, and we remain focused on generating the evidence needed to bring the first targeted therapy for SCN2A gain-of-function disease to patients as quickly as possible,” said Marcio Souza, president and chief executive officer.

About Elsunersen (PRAX-222)
Elsunersen is an antisense oligonucleotide (ASO) designed to selectively decrease SCN2A gene expression, directly targeting the underlying cause of early-seizure-onset SCN2A developmental and epileptic encephalopathy (DEE) to treat seizures and other symptoms in patients with gain-of-function SCN2A mutations. In vitro studies of elsunersen have demonstrated reduction in both SCN2A gene expression and protein levels. In vivo, elsunersen has demonstrated significant, dose-dependent reduction in seizures, improvement in behavioral and locomotor activity and increased survival in SCN2A mouse models, with potential to be the first disease-modifying treatment for SCN2A-DEE. Elsunersen has received Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation (RPDD) from the FDA, and ODD and PRIME designations from the European Medicines Agency for the treatment of SCN2A-DEE. To learn more about the EMBRAVE3 study, please visit https://www.embravestudy.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 anticipated timing of clinical trials and the development 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, 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