Praxis Precision Medicines Receives FDA Breakthrough Therapy Designation for Elsunersen for the Treatment of Seizures Associated with SCN2A Developmental and Epileptic Encephalopathy Caused by Gain of Function Variants

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The Breakthrough Therapy Designation (BTD) was granted based on the positive results from the EMBRAVE Part A trial of elsunersen

Elsunersen demonstrated a 77% sham-adjusted reduction in monthly seizures (p=0.015), with benefit sustained in the open-label extension for up to one year

The pivotal EMBRAVE3 study is enrolling under a streamlined, single-arm registrational pathway aligned with the FDA

Elsunersen now holds Breakthrough Therapy, Orphan Drug and Rare Pediatric Disease Designations from the FDA, and Orphan Drug and PRIME designations from the EMA

BOSTON, June 22, 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 granted BTD for elsunersen (PRAX-222), an antisense oligonucleotide (ASO) for the treatment of seizures associated with SCN2A Developmental and Epileptic Encephalopathy (SCN2A-DEE) caused by Gain of Function (GoF) variants in SCN2A.

“Our third Breakthrough Therapy Designation for the late-stage pipeline is a defining milestone for Praxis, with immediate implications for the elsunersen program and for our Solidus™ ASO platform. It reflects the FDA’s recognition of both the urgency of the unmet need in SCN2A-DEE and the strength of our clinical data. In the EMBRAVE Part A study, elsunersen delivered a 77% sham-adjusted reduction in seizures alongside broad improvements in some of the most severely affected children, results consistent with the potential to be the first disease-modifying, targeted therapy for SCN2A gain-of-function. Together with the streamlined, single-arm EMBRAVE3 registrational pathway we aligned on with the FDA, this designation allows us to move with greater clarity and speed to bring elsunersen to patients and families as quickly as possible,” said Marcio Souza, president and chief executive officer.

The BTD enables expedited development and regulatory review for drugs that are intended to treat a serious condition, where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement on a clinically significant endpoint(s) over existing therapies. The BTD for elsunersen was supported by positive topline results [link] from the EMBRAVE Part A trial, a randomized, sham-controlled Phase 1/2 study evaluating ascending doses of elsunersen in nine pediatric patients aged 2–12 years with early-seizure-onset SCN2A-DEE. Elsunersen treatment led to a 77% sham-adjusted reduction in monthly seizures from baseline (p=0.015; 95% CI [33, 92]); 71% of elsunersen-treated patients achieved a greater than 50% reduction in seizures, and 57% achieved at least a 28-day period of seizure freedom during the six-month treatment period. Efficacy was sustained in the open-label extension for up to one year. Notably, 100% of elsunersen-treated patients, and none of those on sham, experienced additional improvements across sleep, motor function, muscle tone, attention or neuropsychomotor development. Elsunersen was well-tolerated, with no drug-related serious adverse events, no discontinuations and no neuroinflammation signals at doses up to 8 mg; most treatment-emergent adverse events were mild to moderate.

Following alignment with the FDA announced in December 2025, the pivotal EMBRAVE3 study was converted to a single-arm, baseline-controlled design in which all enrolled patients receive elsunersen for 24 weeks, followed by a treatment extension of an additional 24 weeks. EMBRAVE3 is enrolling approximately 30 patients, with the primary analysis measuring the change from baseline in countable motor seizures.

About Early-Seizure-Onset SCN2A-DEE
Early-seizure-onset SCN2A developmental and epileptic encephalopathy (SCN2A-DEE) is a rare, severe genetic epilepsy caused by gain-of-function variants in the SCN2A gene. Seizures typically begin in infancy and are associated with developmental impairment and other neurological complications. There are currently no approved therapies that address the underlying genetic cause of the disease.

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-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 Breakthrough Therapy Designation (BTD), 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. The elsunersen program is ongoing under a collaboration with Ionis Pharmaceuticals, Inc., and RogCon, Inc. To learn more about the EMBRAVE3 study, please visit Embrave | 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, 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 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," "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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