Praxis Precision Medicines Announces Positive Results from the EMBRAVE Part A Trial of Elsunersen in Patients with SCN2A Early-Onset Developmental and Epileptic Encephalopathy

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Elsunersen demonstrated placebo-adjusted seizure reduction from baseline of 77% (p=0.015)

71% of elsunersen-treated patients achieved >50% seizure reduction by period 6, with sustained benefit observed in the open-label extension for up to one year

100% of elsunersen patients - and none on placebo - had additional improvements, including sleep, motor function, muscle tone and attention

No treatment-emergent or serious adverse events related to study drug reported

BOSTON, April 06, 2026 (GLOBE NEWSWIRE) -- Praxis Precision Medicines, Inc. (NASDAQ: PRAX), a fully integrated, leading central nervous system (CNS) precision neuroscience biopharmaceutical company, today announced positive topline results from the EMBRAVE Part A trial of elsunersen in pediatric patients with early-seizure onset SCN2A developmental and epileptic encephalopathy (DEE).

“We are thrilled to see the remarkable, consistent results from EMBRAVE Part A, showing 77% reduction in monthly seizures and disease modifying improvements in children with SCN2A early-seizure onset DEE. We are well underway with our pivotal EMBRAVE3 study and look forward to sharing this placebo-controlled data from the EMBRAVE Part A study with all key stakeholders,” said Marcio Souza, president and chief executive officer.

EMBRAVE Part A is a randomized, placebo-controlled Phase 1/2 trial evaluating the safety and efficacy of ascending doses of elsunersen in patients with SCN2A DEE. Nine patients, aged 2-12 years old, were randomized 3:1 to receive either elsunersen or sham procedure every 4 weeks for 24 weeks, followed by an open-label extension (OLE); all 9 patients continued to the OLE. Patients received a starting dose of 1 mg with optional dose escalation based on observed seizure reduction and individual tolerability.

Key results from EMBRAVE Part A

Efficacy

- Elsunersen treatment led to a 77% placebo-adjusted seizure reduction from baseline (p=0.015, 95 CI [33,92]
- 57% of patients had at least a 28-day period of seizure freedom
- Efficacy was sustained in the OLE for up to one year
- 100% of elsunersen patients improved across sleep, motor function, muscle tone, attention or neuropsychomotor development compared to no improvements in placebo group

Safety

- Elsunersen was well-tolerated, with no drug-related SAEs, no discontinuations and no neuroinflammation signals at doses up to 8 mg
- Most treatment-emergent adverse events (TEAEs) were mild to moderate. TEAEs were consistent with the EMBRAVE Part 1 study

Additional results will be presented at upcoming scientific meetings.

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 ODD and 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 about previous studies and publications about elsunersen please visit the https://praxismedicines.com/resources. To learn more about the EMBRAVE3 study, please visit https://www.embravestudy.com/.

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 and the development of Praxis' product candidates, the anticipated timing of regulatory submissions and interactions, as well as other statements 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 in subsequent 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
LifeSci Advisors
Daniel@lifesciadvisors.com
617-430-7576

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