Praxis Precision Medicines Highlights DEE Clinical Program Updates at Virtual Investor Event

May 5, 2025 at 8:00 AM EDT

BOSTON, May 05, 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, hosted a virtual investor event on its clinical programs in developmental and epileptic encephalopathies (DEEs) on Friday, May 2, 2025.

“We were excited to highlight the significant opportunity in DEEs and progress Praxis is making in this field,” said Marcio Souza, president and chief executive officer of Praxis. “Relutrigine continues to show tremendous promise to broadly address the DEE market opportunity, supported by robust pre-clinical results in multiple DEE models and further strengthened by new data from the EMBOLD study showing increasing seizure control over time. We also presented the EMBRAVE3 design for elsunersen in SCN2A gain-of-function (GoF) patients. This is not only the shortest known trial for an antisense oligonucleotide (ASO) but also supports a potential regulatory pathway for early intervention with treatment starting at birth when symptoms of this devastating disease first appear. We are also thrilled to see great progress in our ASO portfolio, with PRAX-100 in mono-genetic autism on track to declare a development candidate by mid-year. This is truly an exciting period for the DEE community and Praxis, and we look forward to continue sharing our progress with multiple upcoming catalysts.”

The slide presentation and a replay of the event are available on Praxis’ website on the “ Events and Presentations” page under the investor section.

Key Event Topics and Highlights:

Relutrigine

- Praxis shared updated data from the initial cohort of patients in the EMBOLD study, highlighting results through 11 months of patient dosing. The open-label extension (OLE) includes 12 patients who continued on relutrigine following completion of the initial four-month double-blind period:
    - Patients achieved a mean seizure reduction of approximately 90% from baseline.
    - The mean longest period without seizures for patients is 67 days after 11 months of exposure, compared to 3 days at baseline. The EMBOLD registrational study for SCN2A and SCN8A-DEEs continues to enroll strongly with topline results expected no later than the first half of 2026.
- Praxis presented a comprehensive set of pre-clinical data spanning 10 different DEE disease models, providing compelling evidence that relutrigine’s anti-seizure mechanism is well suited for broad DEEs. Praxis plans to initiate the EMERALD registrational study in mid-2025.
    - EMERALD will recruit 160 patients in a randomized, placebo-controlled study with a 16-week treatment period to evaluate seizure reduction.
    - Patients will be selected based on clinical phenotype, irrespective of genetic etiology.
    - With a target population exceeding 200,000 patients in the US and precedent from other approved DEE therapies, Praxis conservatively estimates a US market potential of at least $3 billion.
- The company also shared its expectation that relutrigine could potentially be used in combination with any of its DEE ASO programs (elsunersen, PRAX-080, PRAX-090) allowing patients to receive both therapies together for broader clinical benefit.

Elsunersen

- EMBRAVE Part A is enrolling up to 16 patients on a 3:1 drug to sham ratio with once-monthly intrathecal dosing for six months. Recruitment is expected to be complete by mid-year with topline results in the first half of 2026.
- The EMBRAVE3 registrational trial, initiating in mid-2025, will initially enroll patients ages 2-18. Subsequent cohorts will support treatment initiation from birth, reflecting the early onset of SCN2A GoF DEE which can present in-utero or shortly after birth and is often diagnosed within the first three weeks of life.
- With a global addressable population estimated at 5,000 patients, and pricing analogs for other approved ASOs, Praxis conservatively estimates a global market potential of $1 billion.

Solidus pre-clinical pipeline

- Praxis plans to nominate a development candidate for its early-stage ASO program PRAX-100 in mid-2025.
  - PRAX-100 is targeting SCN2A loss-of-function (LoF) mutations, a leading cause of monogenetic autism.
  - Praxis shared pre-clinical data from four candidate ASOs showing significant increase in protein levels versus control in a humanized SCN2A mouse model, believed to be sufficient to offset the naturally occurring protein reduction seen in patients with SCN2A LoF mutations.
- Praxis remains on track to nominate development candidates for PRAX-080 and PRAX-090 by the end of 2025. PRAX-080 is targeting PCDH19 mosaic expression and PRAX-090 is designed to address SYNGAP1 LoF mutations.

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, and ODD from the European Medicines Agency for the treatment of SCN2A-DEE and SCN8A-DEE. To learn more about the EMBOLD study, please visit https://www.emboldstudy.com/.

About Elsunersen (PRAX-222)
Elsunersen is an antisense oligonucleotide (ASO) designed to selectively decrease SCN2A gene expression, directly targeting the underlying cause of early-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. Data from the Part 1 of the EMBRAVE study demonstrated well-tolerated, significant and sustained seizure reduction in patients with SCN2A-DEE. Elsunersen has received Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation (RPD) from the FDA, and ODD and PRIME designations from the European Medicines Agency (EMA) for the treatment of SCN2A-DEE. The Elsunersen program is ongoing under a collaboration with Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), and RogCon, Inc. To learn more about the EMBRAVE study, please visit https://www.embravestudy.org/.

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, 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 plans to initiate new clinical programs 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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