Praxis Precision Medicines Enters Its Next Chapter Following a Pivotal Year of Progress

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Two NDA submissions expected by mid-February 2026 for ulixacaltamide in essential tremor and relutrigine in SCN2A/8A-DEEs, both with Breakthrough Therapy Designation from the FDA

Multiple registrational readouts expected across vormatrigine, relutrigine and elsunersen in 2026

Strong balance sheet, with $1.5 billion in pro forma cash and investments

Combined peak revenue estimates for the four late-stage assets expected to exceed $20 billion

BOSTON, Jan. 12, 2026 (GLOBE NEWSWIRE) -- Praxis Precision Medicines, Inc. (NASDAQ: PRAX), a fully integrated, leading central nervous system (CNS) precision neuroscience biopharmaceutical company, today announced its business outlook and priorities for 2026.

“2025 was a year of exceptional execution for Praxis, positioning the company for multiple product launches and our transformation into a commercial-stage organization. Both ulixacaltamide and relutrigine delivered compelling late-stage results and earned Breakthrough Therapy Designation, marking the first therapies specifically designed for essential tremor and SCN2A/8A-DEEs, respectively. As we enter 2026, our focus broadens from clinical execution to commercial readiness, with a clear priority on bringing these transformative therapies to patients as efficiently as possible,” said Marcio Souza, president and chief executive officer.

Highlights of Key Clinical and Regulatory Milestones in 2025

The year 2025 was marked by exemplary performance, transformational portfolio progress and the creation of significant long-term value for both patients and shareholders.

- Throughout 2025, Praxis initiated four registration-enabling studies across the epilepsy portfolio, including POWER1 and POWER2 with vormatrigine for the treatment of focal onset seizures (FOS), the EMERALD study with relutrigine in broad developmental and epileptic encephalopathies (DEEs) and the EMBRAVE3 study with elsunersen in early-seizure-onset SCN2A DEEs.
- In July 2025, FDA granted Breakthrough Therapy Designation to relutrigine for the treatment of SCN2A and SCN8A DEEs.
- In August 2025, Praxis reported meaningful seizure reduction and seizure control in patients with FOS in the RADIANT study of vormatrigine.
- In October 2025, ulixacaltamide delivered positive results in both pivotal studies in the Essential3 program, representing the first successful Phase 3 program for a pharmacological treatment of essential tremor (ET).
- In November 2025, a pre-specified interim analysis of the EMBOLD study with relutrigine in SCN2A and SCN8A-DEEs recommended stopping early due to overwhelming efficacy. Following the planned discussion with the FDA in December 2025, Praxis confirmed plans to file an NDA for relutrigine.
- In December 2025, full RADIANT Phase 2 data were presented at the American Epilepsy Society annual meeting, with patients continuing into the open-label extension achieving a median seizure reduction of 100%, supporting vormatrigine’s potential as a best-in-disease therapy with rapid onset of efficacy without titration, durable seizure control, favorable drug-drug interaction profile, tolerability and once-daily dosing, with strong tolerability and safety.
- In December 2025, Praxis aligned with the FDA on the EMBRAVE3 registrational study with elsunersen for early-seizure-onset SCN2A DEE, and converted the design into a single-arm, baseline-controlled study, acknowledging the high unmet need and strong mechanistic rationale for elsunersen.
- In December 2025, Praxis completed a successful pre-new drug application (NDA) meeting with the FDA for ulixacaltamide for the treatment of ET.
- Praxis closed out an exceptional year of progress with the FDA granting Breakthrough Therapy Designation for ulixacaltamide in ET based on the positive Essential3 topline results. The application established that existing pharmacologic therapy is frequently ineffective, poorly tolerated or contraindicated, leaving most patients inadequately treated. Treatment with ulixacaltamide demonstrated rapid, sustained and clinically meaningful improvement in activities of daily living versus placebo, durability of response and clear added benefit when used on top of background therapy. These efficacy findings, together with a favorable and differentiated safety profile that avoids the major limitations of current treatment, supported the Breakthrough Therapy Designation.

What's Ahead in 2026 and Beyond

Praxis enters 2026 well positioned to significantly accelerate value creation as it transforms into a multi-asset commercial company. With the potential to launch multiple blockbuster medicines, the Company is advancing a differentiated pipeline of small molecules and antisense oligonucleotides (ASOs). Several pivotal readouts are expected over the next 24 months, reinforcing Praxis's leadership in CNS clinical execution.

Ulixacaltamide for ET

- NDA submission to the FDA is expected by mid-February 2026.
- Commercial infrastructure build is well underway, including key leadership hiring and the expansion and acceleration of disease awareness and medical communication programs.
- Substantial pre-launch momentum, with Praxis expecting its current US patient database of more than 200,000 patients to grow significantly ahead of launch.
- Compelling long-term commercial opportunity for ulixacaltamide, with potential to exceed $10 billion in peak revenues.

Vormatrigine for FOS and Generalized Epilepsy

- Following significant clinician and patient interest, enrollment in the POWER1 study of vormatrigine in FOS exceeded original plans, with topline results expected in the first half of 2026.
- Enrollment of POWER2, the second registrational study of vormatrigine, is expected to complete in the second half of 2026, with topline results expected in 2027.
- Clinical data from the POWER1 and POWER2 studies, if successful, would serve as the basis for an NDA submission in 2027.
- The POWER3 study to evaluate vormatrigine as monotherapy is expected to initiate in the first half of 2026.
- Peak revenues for vormatrigine in epilepsy are expected to exceed $4 billion.

Relutrigine for DEEs:

- NDA submission to the FDA is expected by mid-February 2026 for SCN2A- and SCN8A-DEE.
- Commercial preparations are underway, including significant acceleration of patient identification.
- The EMERALD study enrolling broad DEEs is expected to complete in the second half of 2026. Assuming successful NDA approval of relutrigine, the EMERALD study, if positive, would serve as the basis for an sNDA submission by 2027.
- Peak revenues for relutrigine DEEs are expected to exceed $5 billion.

Elsunersen for early-seizure-onset SCN2A DEE:

- Topline results from the ongoing EMBRAVE study are expected in the first half of 2026.
- Following FDA agreement to change the EMBRAVE3 trial design to a single-arm, baseline-controlled study, enrollment is quickly accelerating and topline results are expected in the second half of 2026.
- Clinical data from the EMBRAVE3 study, if successful, would serve as the basis for an NDA submission in 2027.
- Peak revenues for elsunersen are expected to exceed $1 billion.

Early-stage Pipeline and Lifecycle Management:

- Praxis expects to declare multiple candidates across the Cerebrum and Solidus platforms in 2026, further reinforcing its lead CNS position.
- Lifecycle focused indication expansion across the portfolio will be presented at a Praxis R&D day in the second quarter of 2026.

Corporate Updates:

- In January 2026, Praxis strengthened its leadership and governance to support its next phase of growth, appointing Jeffrey B. Kinder and Stuart Arbuckle to its Board of Directors, and promoting Megan Sniecinski to Chief Operating Officer and Dr. Steven Petrou to President of Research & Development.
- In January 2026, Praxis completed a public offering of its common stock, resulting in net proceeds of approximately $621 million. Including approximately $567 million in proceeds from the public offering in October 2025, pro forma cash, cash equivalents and marketable securities is $1.5 billion.

About Ulixacaltamide

Ulixacaltamide is a differentiated and highly selective small molecule inhibitor of T-type calcium channels designed to block abnormal neuronal burst firing in the Cerebello-Thalamo-Cortical (CTC) circuit correlated with tremor activity. Ulixacaltamide has received Breakthrough Therapy Designation from the FDA and is the most advanced program within Praxis' Cerebrum™ small molecule platform.

About Vormatrigine
Vormatrigine is a next-generation, functionally selective small molecule targeting the hyperexcitable state of sodium-channels in the brain that is currently being developed as a once daily, oral treatment for adult focal onset seizures and generalized epilepsy. Preclinical data demonstrates vormatrigine is differentiated from standard of care, with the potential to be best-in-class for focal epilepsy. In vitro, vormatrigine has demonstrated superior selectivity for disease-state NaV channel hyperexcitability. In vivo studies of vormatrigine have demonstrated unprecedented potency in the maximal electroshock seizure (MES) model, a highly predictive translational model for efficacy in focal epilepsy. Data from patients in the RADIANT study demonstrated a robust seizure reduction and generally safe and well tolerated profile. To learn more about the POWER1 and POWER2 studies, please visit POWER studies.

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 Elsunersen
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 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, 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 development of Praxis' product candidates and plans to initiate new clinical programs, the anticipated timing of regulatory submissions and interactions, potential market opportunity and commercial potential of Praxis' product candidates and our projected cash runway, 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 the Quarterly Report on Form 10-Q for the period 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.

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