Praxis Precision Medicines Provides Corporate Update and Reports First Quarter 2026 Financial Results

May 7, 2026 at 8:00 AM EDT

FDA accepted the new drug application (NDA) for ulixacaltamide in Essential Tremor with a PDUFA target action date of January 29, 2027, and the NDA for relutrigine, with priority review, in SCN2A and SCN8A developmental and epileptic encephalopathies (DEEs) with a PDUFA target action date of September 27, 2026

EMBRAVE Part A study results showed elsunersen treatment led to a 77% placebo-adjusted reduction in monthly seizures and demonstrated disease-modifying improvements in patients with early-seizure onset SCN2A-DEE

Topline results from the POWER1 study of vormatrigine in focal onset seizures expected in Q2 2026

Recruiting completed for relutrigine EMERALD study in broad DEEs, with topline results expected in Q4 2026

Cash and investments of approximately $1.4 billion as of March 31, 2026 maintains runway into 2028
Conference call today, May 7, 2026 at 8:30am

BOSTON, May 07, 2026 (GLOBE NEWSWIRE) -- Praxis Precision Medicines, Inc. (NASDAQ: PRAX), a fully integrated, leading central nervous system (CNS) precision neuroscience biopharmaceutical company, today provided a corporate update and reported financial results for the first quarter of 2026.

“This quarter marks yet another inflection point for Praxis, with FDA acceptance of NDAs for both ulixacaltamide and relutrigine, positioning us for two U.S. launches within the next eight months as we accelerate our commercial roadmap to ensure readiness and market access upon approval. Our clinical pipeline continues to deliver, with EMBRAVE Part A data showing a 77% placebo-adjusted reduction in monthly seizures and disease-modifying improvements for elsunersen in early-onset SCN2A-DEE. Looking ahead, we expect topline results from the POWER1 study of vormatrigine in focal epilepsy this quarter, followed by the EMERALD readout in broad DEEs in the fourth quarter. Importantly, we remain well-capitalized to execute on this catalyst-rich period and deliver these therapies to patients,” said Marcio Souza, president and chief executive officer.

Recent Highlights and Anticipated Milestones

Cerebrum™ Small Molecule Platform
Ulixacaltamide for Essential Tremor (ET): ET is one of the most common movement disorders, affecting approximately seven million patients in the U.S. Ulixacaltamide was the first investigational therapy to demonstrate positive results in a Phase 3 program in ET and was granted Breakthrough Therapy Designation by the FDA in December 2025.

- The FDA has accepted Praxis’ NDA for ulixacaltamide for the treatment of ET and has set a target action date under the Prescription Drug User Fee Act (PDUFA) of January 29, 2027.
- Commercial preparations and pre-launch activities continue to accelerate:
  - ESSENTIAL *to me* disease education campaign for healthcare providers was launched in April 2026 to raise awareness of Essential Tremor.
  - Commercial organization leadership hired, with build of cross-functional commercial organization and infrastructure on-track.
  - Distribution network is established, with commercial inventory build in-progress ahead of launch.
- At the recent American Academy of Neurology (AAN) Annual Meeting, Praxis shared several oral presentations and posters on ulixacaltamide. The oral plenary session on Phase 3 results from Essential3 was recognized as an Abstract of Distinction in Movement Disorders by the AAN.

Relutrigine for DEEs: Relutrigine is a sodium channel modulator designed to precisely target the hyperexcitable state of sodium-channels, with therapeutic potential across developmental epilepsies. Relutrigine has been granted Breakthrough Therapy Designation and Orphan Drug Designation by the FDA.

- The FDA has accepted with priority review the relutrigine NDA for the treatment of SCN2A and SCN8A DEEs, with a PDUFA target action date of September 27, 2026. If approved, relutrigine will be the first therapy for SCN2A/8A DEE and be eligible for a Pediatric Review Voucher.

- Preparations for the commercial launch of relutrigine are progressing well, including continued hiring within commercial and medical teams, building sufficient inventory, establishing a comprehensive patient support program and engaging with payers to ensure timely market access upon potential approval.
- Recruitment for the EMERALD study in broad DEEs is complete, with topline results expected in the fourth quarter of 2026. Assuming successful initial NDA approval of relutrigine, the EMERALD study, if positive, would serve as the basis for a supplemental NDA submission in 2027.

Vormatrigine for Focal Onset Seizures (FOS) and Generalized Epilepsy: An estimated 3.5 million people in the U.S. suffer from common epilepsies. Sodium channel therapy is the cornerstone of treatment for patients with epilepsy, yet currently approved drugs have significant safety and efficacy limitations. Vormatrigine is the most potent sodium-channel modulator ever developed for epilepsy and is designed to precisely target the hyperexcitable state of sodium-channels in adult common epilepsies.

- The POWER1 Phase 3 study for FOS is on track for topline results in the second quarter of 2026.
- POWER2, the second Phase 3 study for vormatrigine in FOS, continues to progress towards completion in the second half of 2026 with topline results anticipated in 2027.
- The POWER3 study to evaluate vormatrigine as a monotherapy remains on track to commence in the first half of 2026.

Solidus™ Antisense Oligonucleotide (ASO) Platform

- **Elsunersen for early-seizure-onset SCN2A DEE:** SCN2A early-onset DEE is a rare, genetic epilepsy characterized by early-onset seizures and severe impact on development.
    - Topline results from the EMBRAVE Part A Phase 1/2 study evaluating SCN2A early onset seizure patients were announced in April 2026 and presented at AAN:
        - Treatment with elsunersen led to a significant 77% placebo-adjusted seizure reduction from baseline (p=0.015).
        - 71% of patients treated with elsunersen achieved >50% seizure reduction by period 6, with results sustained during the open label extension for up to one year.
        - 57% of patients treated with elsunersen had at least a 28-day period of seizure freedom.
        - 100% of patients treated with elsunersen experienced improvements in sleep, motor function, muscle tone, attention or neuropsychomotor development compared to no observed improvements in placebo group.
        - Elsunersen was well-tolerated, with no drug-related SAEs, no discontinuations and no neuroinflammation signals at doses up to 8 mg.
        - Clinical updates from the elsunersen Emergency Use Program were also presented at AAN, highlighting durable seizure reduction and meaningful quality-of-life improvements across six patients treated globally, with more than 100 doses administered to date.
    - Enrollment is progressing in the EMBRAVE3 registrational trial, with topline results expected in 2027.
- Praxis remains on track to nominate a development candidate for each of its three early stage ASO therapeutic initiatives in the first half of 2026:
    - PRAX-080 is focused on targeting PCDH19 mosaic expression disorder.
    - PRAX-090 is designed to address SYNGAP1 loss-of-function (LoF) mutations, a leading cause of severe intellectual disability and epilepsy in DEEs.
    - PRAX-100 targets SCN2A LoF mutations, the predominant genetic link to de novo autism spectrum disorders.

First Quarter 2026 Financial Results:

As of March 31, 2026, Praxis had $1.4 billion in cash, cash equivalents and marketable securities, compared to $926.1 million in cash, cash equivalents and marketable securities as of December 31, 2025. This increase of $473.9 million was primarily attributable to net proceeds from Praxis' January 2026 follow-on public offering and interest income on marketable securities, partially offset by cash used in operations. The Company's cash, cash equivalents and marketable securities as of March 31, 2026 are expected to fund operations into 2028.

Research and development expenses were $78.0 million for the first quarter of 2026, compared to $60.8 million for the first quarter of 2025. The increase in research and development expenses of $17.2 million was primarily attributable to $9.2 million in increased expenses related to the Company's Cerebrum™ platform,$3.8 million in increased personnel-related expenses and $3.0 million in increased expenses related to the Company's Solidus™ platform.

General and administrative expenses were $27.9 million for the first quarter of 2026, compared to $13.9 million for the first quarter of 2025. The increase in general and administrative expenses of $14.0 million was primarily attributable to $9.8 million in increased personnel-related expenses and $3.5 million in increased professional expenses.

Praxis incurred a net loss of $92.6 million for the first quarter of 2026, including $17.1 million of stock-based compensation expense, compared to $69.3 million for the first quarter of 2025, including $8.8 million of stock-based compensation expense.

As of March 31, 2026, Praxis had 27.9 million shares of common stock outstanding.

Conference Call
Praxis will discuss first quarter 2026 financial results and business highlights on a conference call taking place today, May 7 at 8:30 am ET, which can be accessed by visiting this registration link. The live audio webcast will also be available through the Events & Presentations page of the Investors + Media section of the Company's website .

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, 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.

PRAXIS PRECISION MEDICINES, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(Amounts in thousands)
(Unaudited)

	March 31, 2026	December 31, 2025
Assets		
Cash and cash equivalents	$ 536,333	$ 357,329
Marketable securities	911,470	568,759
Prepaid expenses and other current assets	10,909	11,580
Property and equipment, net	170	147
Operating lease right-of-use assets	1,320	92
Total assets	$ 1,460,202	$ 937,907
Liabilities and stockholders' equity		
Accounts payable	$ 31,158	$ 24,628
Accrued expenses	17,648	35,033
Operating lease liabilities	1,435	110
Common stock	15	15
Additional paid-in capital	2,644,109	2,017,566
Accumulated deficit	(1,232,569)	(1,140,008)
Accumulated other comprehensive (loss) gain	(1,594)	563
Total liabilities and stockholders' equity	$ 1,460,202	$ 937,907

PRAXIS PRECISION MEDICINES, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(Amounts in thousands, except share and per share amounts)
(Unaudited)

	Three Months Ended March 31,	
	2026	2025
Operating expenses:		
Research and development	77,987	60,806
General and administrative	27,873	13,922
Total operating expenses	105,860	74,728
Loss from operations	(105,860)	(74,728)
Other income:		
Other income, net	13,299	5,432
Total other income	13,299	5,432
Net loss	$ (92,561)	$ (69,296)
Net loss per share attributable to common stockholders, basic and diluted	$ (3.20)	$ (3.29)
Weighted average common shares outstanding, basic and diluted	28,883,596	21,055,834

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