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

November 9, 2026

FDA reviews of relutrigine, with PDUFA target action date of December 27, 2026, and ulixacaltamide HCl, with PDUFA target action date of January 29, 2027, are ongoing

Topline results from the EMERALD study of relutrigine in broad developmental and epileptic encephalopathies (DEEs) expected in the fourth quarter of 2026

Cash and investments of approximately $1.3 billion as of September 30, 2026 maintain runway into 2028

Praxis to host an in-person and virtual Investor Day on November 12, 2026 and will participate in a fireside chat at the Guggenheim Biotech Summit today, November 9 at 9:30am (register here)

BOSTON, Nov. 09, 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 third quarter of 2026.

"We are entering a defining period for Praxis, with two key regulatory milestones ahead and the opportunity to become a commercial CNS company. Our teams are working to bring relutrigine and ulixacaltamide to patients who need new options. The relutrigine team is fully hired, and we expect the ulixacaltamide sales force to be in place this quarter. We continue to advance our broader pipeline, with EMERALD and EMBRAVE3 progressing toward important readouts. We look forward to discussing our commercial strategies, launch readiness and pipeline at our Investor Day later this week," 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 is the first and only 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 New Drug Application (NDA) for ulixacaltamide remains under FDA review, with a Prescription Drug User Fee Act (PDUFA) target action date of January 29, 2027.
- Commercial launch preparations continue to advance, with the commercial leadership team in place and hiring of the field sales force expected to be completed in the fourth quarter of 2026.
- Praxis has reinitiated the long-term safety study (LTSS) associated with the Essential3 program to provide an opportunity for eligible patients to participate ahead of potential commercial availability.
- Praxis initiated a strategic collaboration in July 2026 with Remagine Labs to develop a transdermal patch of ulixacaltamide for ET. The transdermal formulation is designed to expand potential treatment options for patients with ET and support the long-term development of the ulixacaltamide franchise.

Relutrigine for Developmental and Epileptic Encephalopathies (DEEs): Relutrigine is a disease state-selective voltage 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. If approved, relutrigine will be the first therapy for SCN2A and SCN8A DEEs and will be eligible for a Pediatric Review Voucher.

- The NDA for relutrigine for the treatment of SCN2A and SCN8A DEEs remains under FDA review, with a Prescription Drug User Fee Act (PDUFA) target action date of December 27, 2026.
- Preparations for the potential commercial launch of relutrigine are well advanced, with the commercial team fully hired.
- The EMERALD study in broad DEEs is in the final stages of completion.

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 POWER2 study has restarted following updates to the study design, which incorporate key learnings from the

POWER1 Phase 2/3 study.
- Praxis continues to review the study design of POWER3, its planned study of vormatrigine as a monotherapy, and expects to initiate in 2027.

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

- Enrollment in the EMBRAVE3 registrational trial continues to progress strongly. Topline results are anticipated in 2027.

Investor Day
Praxis will host an Investor Day in New York on Thursday, November 12, 2026, at 8:00 AM ET. The event will be held in person and virtually. To register, click here.

The Investor Day will include presentations from senior management on the Company's commercial strategy and launch readiness for the two programs under FDA review and on the Company's development pipeline. Additionally, key opinion leaders will join management to discuss the unmet need and current treatment landscape in ET and DEEs.

Third Quarter 2026 Financial Results:
As of September 30, 2026, Praxis had $1.3 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 $359.9 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 September 30, 2026 are expected to fund operations into 2028.

Research and development expenses were $64.2 million for the third quarter of 2026, compared to $65.8 million for the third quarter of 2025. The decrease in research and development expenses of $1.6 million was primarily attributable to $8.8 million in decreased expenses related to Cerebrum™, partially offset by $7.0 million in increased personnel-related costs.

General and administrative expenses were $42.7 million for the third quarter of 2026, compared to $12.6 million for the third quarter of 2025. The increase in general and administrative expenses of $30.1 million was primarily attributable to $17.2 million in increased personnel-related expenses, $9.4 million in increased professional expenses and $3.5 million increase in other expenses.

Praxis incurred a net loss of $94.2 million for the third quarter of 2026, including $14.9 million of stock-based compensation expense, compared to $73.9 million for the third quarter of 2025, including $7.4 million of stock-based compensation expense.

As of September 30, 2026, Praxis had 27.9 million shares of common stock outstanding.

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 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 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 POWER2 study, please visit POWER 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. The elsunersen program is ongoing under a collaboration with Ionis Pharmaceuticals, Inc., and RogCon, Inc. Elsunersen holds Breakthrough Therapy, Orphan Drug and Rare Pediatric Disease Designations from the FDA, and Orphan Drug and PRIME designations from the EMA. 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 development of Praxis' product candidates, the anticipated timing of regulatory submissions and interactions, the potential market opportunity and commercial potential of Praxis' product candidates, the anticipated timing of clinical trials, and Praxis' 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)

	September 30, 2026	December 31, 2025
Assets		
Cash and cash equivalents	$ 361,851	$ 357,329
Marketable securities	924,175	568,759
Prepaid expenses and other current assets	17,477	11,580
Property and equipment, net	1,901	147
Operating lease right-of-use assets	674	92
Other assets	5,995	—
Total assets	$ 1,312,073	$ 937,907
Liabilities and stockholders' equity		
Accounts payable	$ 15,016	$ 24,628

Accrued expenses	36,750	35,033
Operating lease liabilities	732	110
Common stock	15	15
Additional paid-in capital	2,675,810	2,017,566
Accumulated deficit	(1,410,523)	(1,140,008)
Accumulated other comprehensive (loss) gain	(5,727)	563
Total liabilities and stockholders' equity	$ 1,312,073	$ 937,907

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	**2025**	**2026**	**2025**
Operating expenses:				
Research and development	$ 64,205	$ 65,797	$ 211,608	$ 189,609
General and administrative	42,709	12,562	98,070	39,545
Total operating expenses	106,914	78,359	309,678	229,154
Loss from operations	(106,914)	(78,359)	(309,678)	(229,154)
Other income:				
Other income, net	12,680	4,425	39,163	14,797
Total other income	12,680	4,425	39,163	14,797
Net loss	$ (94,234)	$ (73,934)	$ (270,515)	$ (214,357)
Net loss per share attributable to common stockholders, basic and diluted	$ (3.23)	$ (3.36)	$ (9.31)	$ (9.97)
Weighted average common shares outstanding, basic and diluted	29,178,396	21,977,268	29,065,656	21,506,021

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