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

August 6, 2026 at 8:00 AM EDT

Mid-cycle meeting completed for ulixacaltamide HCl; FDA identified no major safety or efficacy concerns to date and does not plan to request an advisory committee meeting; PDUFA date of January 29, 2027

Mid-cycle meeting completed for relutrigine; FDA identified no major safety or efficacy concerns to date and does not plan to request an advisory committee meeting; PDUFA date of December 27, 2026

FDA Bioresearch Monitoring (BIMO) inspections of Praxis for both ulixacaltamide and relutrigine completed with no Form FDA 483 observations

Commercial infrastructure build-out accelerating for ulixacaltamide HCl in Essential Tremor and relutrigine in SCN2A and SCN8A developmental and epileptic encephalopathies (DEEs) ahead of expected approvals

Praxis received its third Breakthrough Therapy Designation following the positive results from the EMBRAVE Part A trial of elsunersen for the treatment of seizures associated with SCN2A DEE caused by gain of function variants

Cash and investments of approximately $1.4 billion as of June 30, 2026 maintains runway into 2028

Conference call today, August 6, 2026, at 8:30am

BOSTON, Aug. 06, 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 second quarter of 2026.

“This quarter reflected meaningful progress toward becoming a multi-product commercial company, with both ulixacaltamide and relutrigine advancing closer to patients through ongoing constructive dialogue with the FDA with positive mid-cycle meetings and BIMO sponsor inspections completed for both programs. We have also taken significant steps in launch preparations: our commercial leadership is in place, we are standing up our state-of-the-art commercial systems, and the momentum is tangible with field force hiring well under way,” said Marcio Souza, president and chief executive officer. “Across the rest of our portfolio, the Breakthrough Therapy Designation for elsunersen, our third in twelve months, further validates our position as a leader in CNS disease, our Solidus™ ASO platform for treating devastating CNS disorders and the depth of our CNS therapeutic development engine. Lastly, we’re excited to be continuing the POWER2 study with renewed conviction, informed by what we learned in POWER1, as we work to transform care for patients with focal epilepsy with vormatrigine.”

Recent Highlights and Anticipated Milestones
Cerebrum™ for Small Molecules

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.

- NDA review is progressing as expected, with a PDUFA date of January 29, 2027.
- The mid-cycle meeting was successfully completed and the FDA identified no major safety or efficacy concerns to date. The agency confirmed that no advisory committee meeting is planned.
- Launch preparation activities are accelerating, with commercial organization leaders hired and infrastructure, marketing and disease-education programs, medical information capabilities, physician targeting and an established distribution network with commercial inventory build in progress.
- A strategic collaboration was initiated in July 2026 with Remagine Labs to develop a transdermal patch of ulixacaltamide for ET. The transdermal formulation is designed to broaden ulixacaltamide’s addressable patient population, strengthen its competitive positioning, and support the long-term growth, durability, and value of the ulixacaltamide franchise.

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. If approved, relutrigine will be the first therapy for SCN2A and SCN8A DEEs and will be eligible for a Pediatric Review Voucher.

- Following the submission of additional sensitivity analyses of existing clinical data, which the FDA deemed collectively to be a major amendment, the FDA extended the review period for relutrigine’s NDA for the treatment of SCN2A and SCN8A

DEEs and set a new PDUFA target action date of December 27, 2026.
- The mid-cycle meeting was successfully completed and the FDA has identified no major safety or efficacy concerns to date. The agency confirmed that no advisory committee meeting is planned.
- Preparations for the commercial launch of relutrigine are gaining momentum, with commercial and medical teams hired, building sufficient inventory, establishing a comprehensive patient support program and engaging with payers to ensure timely market access upon potential approval.
- Enrollment in the EMERALD study in broad DEEs exceeded the planned target with approximately 200 patients enrolled, spanning over 50 distinct genetically defined pathological etiologies in the trial population.
- Topline results are anticipated in the fourth quarter of 2026 and assuming successful initial NDA approval of relutrigine, the EMERALD study, if positive, would serve as the basis for a supplemental NDA submission in 2027.

NDA-related activities and updates for Ulixacaltamide HCl and Relutrigine

- Praxis operations were subject to inspections by the FDA in accordance with the BIMO program related to its NDAs for ulixacaltamide HCl for Essential Tremor and relutrigine for SCN2A and SCN8A DEEs. The scope of the inspections was comprehensive, spanning overall quality and clinical operations, data integrity, including statistical and interim analyses, and safety, amongst other standard areas in the BIMO program. The inspection was completed successfully, and no form 483 was issued.
- Acknowledging the late-stage discussions with the FDA in relation to both applications, Praxis does not intend to communicate regulatory updates going forward until the expected PDUFA dates.

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.

- In June, Praxis announced topline results from the POWER1 Phase 2/3 study in highly refractory patients with FOS (link).
  - The study did not meet its primary endpoint of reduction in monthly focal seizure frequency from baseline at Week 12.
  - The study met the secondary endpoint, with a significant number of patients achieving ≥50% reduction in seizure frequency.
- Praxis is finalizing the plans to restart the POWER2 study and initiate the POWER3 study in the fourth quarter of 2026 based on the learnings from POWER1.

Solidus™ for Antisense Oligonucleotides (ASO)

- **Elsunersen for early-seizure-onset SCN2A DEE:** Early-onset SCN2A-DEE is a rare, genetic epilepsy characterized by early-onset seizures and severe impact on development.
  - The FDA granted Breakthrough Therapy Designation to elsunersen based on positive results from the EMBRAVE Part A trial. Elsunersen now holds Breakthrough Therapy, Orphan Drug and Rare Pediatric Disease Designations from the FDA, and Orphan Drug and PRIME designations from the EMA.
  - If approved, elsunersen will be eligible for a Pediatric Review Voucher.
  - Enrollment in the EMBRAVE3 registrational trial is progressing, with topline results expected in 2027.
- Praxis remains on track to nominate development candidates for several early-stage ASO therapeutic initiatives in 2026.

Second Quarter 2026 Financial Results:
As of June 30, 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 $447.8 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 June 30, 2026 are expected to fund operations into 2028.

Research and development expenses were $69.4 million for the second quarter of 2026, compared to $63.0 million for the second quarter of 2025. The increase in research and development expenses of $6.4 million was primarily attributable to $6.7 million in increased expenses related to Solidus™,$4.4 million in increased personnel-related costs and $1.8 million in increased indirect costs, partially offset by $6.5 million in decreased expenses related to Cerebrum™.

General and administrative expenses were $27.5 million for the second quarter of 2026, compared to $13.1 million for the second quarter of 2025. The increase in general and administrative expenses of $14.4 million was primarily attributable to $7.2 million in increased professional expenses and $6.3 million in increased personnel-related costs.

Praxis incurred a net loss of $83.7 million for the second quarter of 2026, including $11.3 million of stock-based compensation expense, compared to $71.1 million for the second quarter of 2025, including $7.8 million of stock-based compensation expense.

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

Conference Call
Praxis will discuss second quarter 2026 financial results and business highlights on a conference call taking place today, August 6 at 8:30 am ET, which can be accessed by dialing (800) 715-9871 with passcode 7796789 or by registering for the webcast, here. 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 of Praxis' Cerebrum™ small molecules.

About Relutrigine
Relutrigine is a first-in-class small molecule in development for the treatment of developmental and epileptic encephalopathies (DEEs). Relutrigine is a functional state-selective sodium channel (NaV) modulator that preferentially modulates NaV channels under the conditions associated with pathological neuronal hyperexcitability, including sustained depolarization, repetitive firing and increased persistent current where present. By preferentially targeting pathological NaV channel activity while sparing normal NaV function, relutrigine is designed to provide broad efficacy across DEEs with differing etiologies and improved tolerability relative to traditional sodium channel blockers. 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 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 small-molecule sodium channel modulator currently being developed as a once-daily oral treatment for adults with focal-onset seizures and generalized epilepsy. Vormatrigine is designed to preferentially inhibit the pathologically increased neuronal firing that underlies seizures while relatively preserving normal physiological activity. Preclinical data demonstrate differentiation from current standards of care and support its potential to be a best-in-class treatment for focal epilepsy. In vitro, vormatrigine has demonstrated preferential modulation of NaV channels under the sustained depolarization and repetitive-firing conditions associated with seizure activity.

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 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. Elsunersen has received BTD, 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 for small molecules through Cerebrum™, and for antisense oligonucleotides (ASOs) through Solidus™, using our understanding of shared biological targets and circuits ir 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 potential market opportunity and commercial potential of Praxis' product candidates, the anticipated timing of regulatory submissions and interactions, the anticipated timing of clinical trials, the development of Praxis' product candidates and plans to initiate new clinical programs, 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)

	June 30, 2026	December 31, 2025
Assets		
Cash and cash equivalents	$ 474,761	$ 357,329
Marketable securities	899,080	568,759
Prepaid expenses and other current assets	13,352	11,580
Property and equipment, net	492	147
Operating lease right-of-use assets	1,001	92
Internal-Use software	773	—
Other assets	643	—
Total assets	$ 1,390,102	$ 937,907
Liabilities and stockholders' equity		
Accounts payable	$ 23,386	$ 24,628
Accrued expenses	24,585	35,033
Operating lease liabilities	1,087	110
Common stock	15	15
Additional paid-in capital	2,659,990	2,017,566
Accumulated deficit	(1,316,289)	(1,140,008)
Accumulated other comprehensive (loss) gain	(2,672)	563
Total liabilities and stockholders' equity	$ 1,390,102	$ 937,907

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Operating expenses:				
Research and development	$ 69,416	$ 63,006	$ 147,403	$ 123,812
General and administrative	27,488	13,061	55,361	26,983
Total operating expenses	96,904	76,067	202,764	150,795
Loss from operations	(96,904)	(76,067)	(202,764)	(150,795)
Other income:				
Other income, net	13,184	4,940	26,483	10,372
Total other income	13,184	4,940	26,483	10,372
Net loss	$ (83,720)	$ (71,127)	$ (176,281)	$ (140,423)
Net loss per share attributable to common stockholders, basic and diluted	$ (2.87)	$ (3.31)	$ (6.08)	$ (6.60)
Weighted average common shares outstanding, basic and diluted	29,131,375	21,474,827	29,008,351	21,266,490

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