Praxis Precision Medicines Provides Corporate Update and Reports Third Quarter 2025 Financial Results

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Announced positive results from two pivotal Phase 3 Essential3 studies of ulixacaltamide HCl in essential tremor (ET); Pre-NDA meeting with FDA scheduled for Q4 2025

Plans to accelerate the development of relutrigine in SCN2A and SCN8A DEE patients after comprehensive Type B meeting with FDA

Recruitment completed for POWER1 study of vormatrigine in focal onset seizures, with topline results expected in 1H 2026

POWER2 study started, with enrollment expected to complete in 2H 2026

Pro forma cash and investments of approximately $956 million, including proceeds from October 2025 public offering, fund operations into 2028

BOSTON, Nov. 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, today provided a corporate update and reported financial results for the third quarter 2025.

“It’s been a monumental few months for us at Praxis, culminating in the first ever successful Phase 3 program of an investigational drug in ET, with both studies in the Essential3 program meeting their primary endpoints. Ulixacaltamide demonstrated significant improvements in symptoms that are not only clinically meaningful but also change patients’ lives. We have been granted a pre-NDA meeting with the FDA and look forward to discussing Praxis’ first NDA submission,” said Marcio Souza, president and chief executive officer. “Leveraging our breakthrough therapy designation for relutrigine, we completed comprehensive discussions with the FDA to perform an interim analysis of our EMBOLD study in Q4 which, if successful, supports another potential NDA submission for relutrigine in SCN2A/8A DEEs in early 2026. For our vormatrigine program, following the positive results from the first cohort of the RADIANT study, we have now completed recruitment for POWER1 with expected readout in the first half of 2026, and POWER2 is underway and planning to be fully enrolled in the second half of 2026. With the recently completed public offering, we are well-positioned to continue executing across our robust portfolio as we prepare for our first commercial launch, establishing Praxis as the leader in innovative therapies for CNS disorders.”

Recent Highlights and Anticipated Milestones

Cerebrum™ Small Molecule Platform

- **Ulixacaltamide for Essential Tremor (ET):** ET is the largest movement disorder affecting approximately seven million people in the U.S. In October 2025, Praxis announced the positive results from the Essential3 program – the first positive Phase 3 program for a drug in ET. During the recruitment phase of the trial, which started in November 2023, over 200,000 patients demonstrated interest in participating in the study, underscoring the unmet need and size of the opportunity.
  - Study 1 (placebo-controlled, parallel group) met its primary and all secondary endpoints:
    - Patients treated with ulixacaltamide in Study 1 showed a mean improvement from baseline in the modified Activities of Daily Living 11 (mADL11) at Week 8, the pre-specified primary endpoint, of 4.3 points ($p<0.0001$).
    - The improvement in mADL11 was observed as early as Week 2 and was sustained throughout Week 12.
    - All key-secondary endpoints were met, including rate of disease improvement, PGI-C and CGI-S at Week 8 showing statistically significant improvement with ulixacaltamide vs placebo ($p<0.001$).
  - Study 2 (randomized withdrawal study) met its primary endpoint and first secondary endpoint:
    - Primary endpoint of percentage of patients maintaining response was met with 55% of patients on ulixacaltamide vs 33% of patients on placebo maintaining their response ($p=0.037$).
    - The first key secondary endpoint of rate of disease improvement during the randomized withdrawal period also demonstrated superior effect in patients on ulixacaltamide vs placebo ($p=0.0042$).
  - Additional pre-specified analyses were performed, further confirming the positive effect of ulixacaltamide:
    - The first analysis combined patients treated with ulixacaltamide in Studies 1 and 2 and compared them to patients treated with placebo in Study 1. The analysis showed a 4.3 point improvement in mADL11 at Week

8 for the combined Studies 1 and 2 ulixacaltamide groups vs. Study 1 placebo ($p<0.0001$).
        - In the second analysis, there was a 4.2 point improvement in mADL11 at Week 8 for the Study 2 ulixacaltamide group vs Study 1 placebo ($p<0.0001$), respectively.
    - Ulixacaltamide was generally well tolerated with a safety profile consistent with previous trials and no drug-related serious adverse events.
    - Pre-NDA meeting with the FDA was granted and is scheduled for the fourth quarter of 2025.
- **Vormatrigine for FOS and Generalized Epilepsy:** Vormatrigine is the most potent sodium-channel modulator ever designed to precisely target the hyperexcitable state of sodium-channels in adult common epilepsies.
    - In August 2025, Praxis shared positive results from the first cohort of the RADIANT study evaluating patients with FOS:
        - Dosing with vormatrigine over 8 weeks led to 56.3% median reduction in seizure frequency.
        - Approximately 22% of patients reached 100% reduction in seizure frequency in the last 28 days.
        - There was a rapid and sustained response, with approximately 60% of patients achieving 50% response in the study.
        - Vormatrigine was generally well tolerated and continues to demonstrate a favorable safety profile.
        - Additional data were presented at the 36th International Epilepsy Congress highlighting [poster]. Praxis is planning to present additional results at the upcoming American Epilepsy Society Annual Meeting in December 2025.
    - The POWER1 Phase 2/3 registrational study for FOS has completed recruitment, with topline readout expected in the first half of 2026.
    - The POWER2 Phase 3 registrational study for FOS has started and is expected to be fully enrolled in the second half of 2026.
    - The POWER3 study to evaluate vormatrigine as a standalone agent is expected to initiate in the first half of 2026.
    - The overall ENERGY program recruitment initiative, including the EMPOWER study, has attracted approximately 20,000 patients in the US with epilepsy and continues to support patient identification for the epilepsy studies.
- **Relutrigine for Developmental and Epileptic Encephalopathies (DEEs):** Relutrigine is Praxis' second sodium channel modulator designed to precisely target the hyperexcitable state of sodium-channels, with therapeutic potential across developmental epilepsies.
    - Following a comprehensive Type B meeting, and recent meeting minutes and written advice on the protocol and statistical analysis plan, it has reached alignment with the FDA on several aspects of the relutrigine program in SCN2A and SCN8A DEEs, including the use of the ongoing EMBOLD study to serve as the basis of substantial evidence of effectiveness for the NDA submission. The FDA agreed that Praxis' proposed interim analysis, if positive, may serve as the basis of the NDA submission in early 2026. The interim analysis is planned to be conducted in the fourth quarter of 2025.Relutrigine is also being investigated in patients across all DEEs in the EMERALD trial. EMERALD is a registrational, 16-week, placebo-controlled study evaluating the efficacy and safety of relutrigine in reducing seizures in patients diagnosed with developmental epilepsies.
    - Enrollment for the EMERALD study began in the third quarter of 2025 and is expected to be complete in the second half of 2026, receiving strong interest in the geographies where it is planned. Assuming successful conclusion of the EMBOLD study and subsequent NDA approval, if positive, the EMERALD study would serve as the basis for an sNDA by 2027.

Solidus™ Antisense Oligonucleotide (ASO) Platform

- **Elsunersen for early-seizure-onset SCN2A DEE:** SCN2A GoF-DEE is a rare, genetic epilepsy characterized by early-onset seizures and severe impact on development. Elsunersen is currently being evaluated in two registrational studies:
    - The EMBRAVE Part A Phase 1/2 study is on track for topline results in the first half of 2026.
    - The EMBRAVE3 registrational study for SCN2A GoF-DEE is recruiting and expected to complete in 2026.
- Praxis remains on track to nominate a development candidate for each of its early stage ASO therapeutic initiatives in the first half of 2026:
    - PRAX-080: Focused on targeting PCDH19 mosaic expression disorder.

- PRAX-090: Designed to address SYNGAP1 loss-of-function (LoF) mutations, a leading cause of severe intellectual disability and epilepsy in DEEs.
- PRAX-100: Targeting SCN2A LoF mutations, the predominant genetic link to de novo autism spectrum disorders.

Third Quarter 2025 Financial Results:

As of September 30, 2025, Praxis had $389.2 million in cash, cash equivalents and marketable securities, compared to $469.5 million in cash, cash equivalents and marketable securities as of December 31, 2024. The decrease of $80.3 million is primarily attributable to cash used in operating activities, partially offset by net proceeds from at-the-market offerings of common stock.

In October 2025, Praxis announced the closing in October 2025 of an underwritten public offering of common stock and pre-funded warrants to purchase common stock, generating net proceeds of approximately $567.0 million, after deducting the underwriting discounts and commissions and estimated offering expenses payable by Praxis. The Company's cash, cash equivalents and marketable securities as of September 30, 2025, together with the proceeds from the October 2025 public offering, are expected to fund operations into 2028.

Praxis did not recognize any collaboration revenue during the three months ended September 30, 2025, compared to $0.3 million during the three months ended September 30, 2024. The decrease of $0.3 million is related to its Option and License Agreement with UCB. In December 2024, UCB exercised its option to in-license global development and commercialization rights for a KCNT1 small molecule development candidate, and as such, Praxis has no further research service obligations under the terms of the Option and License Agreement.

Research and development expenses were $65.8 million for the three months ended September 30, 2025, compared to $41.9 million for the three months ended September 30, 2024. The increase in research and development expenses of $23.9 million was primarily attributable to an increase of $21.7 million in Praxis' Cerebrum™ platform and an increase of $1.9 million in Praxis's Solidus™.

General and administrative expenses were $12.6 million for the three months ended September 30, 2025, compared to $15.3 million for the three months ended September 30, 2024. The decrease in general and administrative expenses of approximately $2.7 million was primarily attributable to a decrease of $2.6 million in personnel-related costs.

Praxis reported a net loss of $73.9 million for the three months ended September 30, 2025, including $7.4 million of stock-based compensation expense, compared to $51.9 million for the three months ended September 30, 2024, including $12.4 million of stock-based compensation.

As of September 30, 2025, Praxis had 21.2 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, the most advanced program within Praxis' Cerebrum™ small molecule platform is currently being evaluated in essential tremor (ET) - the largest movement disorder affecting approximately seven million people in the U.S. In October 2025, Praxis announced positive results from the Essential3 program (NCT06087276), with ulixacaltamide showing statistically significant, durable, improvement in modified activities of daily living 11 (mADL11), as well as significant improvements in rate of disease improvement over 12 weeks, PGI-C and CGI-S. A pre-NDA meeting with the FDA has been scheduled for Q4 2025.

About Vormatrigine (PRAX-628)
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 the first cohort of patients in the RADIANT study demonstrated a robust seizure reduction and generally safe and well tolerated profile. To learn more about the POWER1 study, please visit POWER1 study.

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; 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 and EMBOLD studies, please visit ResilienceStudies.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-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 EMBRAVE study, please visit https://www.embravestudy.com/.

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 movement disorders and epilepsy, with four clinical-stage product candidates. For more information, please visit www.praxismedicines.com and follow us on Facebook, 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, the anticipated timing of regulatory submissions and interactions 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; preliminary analyses from ongoing studies differing materially from final data from preclinical studies and completed 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.

PRAXIS PRECISION MEDICINES, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

(Amounts in thousands)

(Unaudited)

	September 30, 2025	December 31, 2024
Assets		
Cash and cash equivalents	$ 149,527	$ 215,372
Marketable securities	239,638	254,156
Prepaid expenses and other current assets	6,687	11,805
Property and equipment, net	174	230
Operating lease right-of-use assets	362	1,131
Other non-current assets	—	416
Total assets	$ 396,388	$ 483,110
Liabilities and stockholders' equity		
Accounts payable	$ 30,819	$ 12,528
Accrued expenses	21,628	23,763
Operating lease liabilities	436	1,369
Common stock	14	14
Additional paid-in capital	1,394,088	1,281,522
Accumulated other comprehensive gain	500	654
Accumulated deficit	(1,051,097)	(836,740)
Total liabilities and stockholders' equity	$ 396,388	$ 483,110

PRAXIS PRECISION MEDICINES, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(Amounts in thousands, except share and per share amounts)

(Unaudited)

	Three Months Ended		Nine Months Ended	
	September 30,		September 30,	
	2025	2024	2025	2024
Collaboration revenue	$ —	$ 302	$ —	$ 1,090
Operating expenses:				
Research and development	65,797	41,881	189,609	96,125
General and administrative	12,562	15,256	39,545	41,174
Total operating expenses	78,359	57,137	229,154	137,299
Loss from operations	(78,359)	(56,835)	(229,154)	(136,209)
Other income:				
Other income, net	4,425	4,925	14,797	12,069
Total other income	4,425	4,925	14,797	12,069
Net loss	$ (73,934)	$ (51,910)	$ (214,357)	$ (124,140)
Net loss per share attributable to common stockholders, basic and diluted	$ (3.36)	$ (2.75)	$ (9.97)	$ (7.21)
Weighted average common shares outstanding, basic and diluted	21,977,268	18,884,562	21,506,021	17,210,604

Investor Contact: Praxis Precision Medicines investors@praxismedicines.com 857-702-9452 Media Contact: Dan Ferry Life Science Advisors Daniel@lifesciadvisors.com 617-430-7576