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

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On track for six major study readouts across four programs over the next 12 months

Ready to initiate pivotal studies in two developmental and epileptic encephalopathy (DEE) programs in mid-year 2025: EMERALD for broad DEEs with relutrigine and EMBRAVE3 for SCN2A GoF with elsunersen

Vormatrigine continues to generate a best-in-class safety profile with new data demonstrating no food effect and higher dosing tolerability

Praxis to host a virtual investor event on Friday, May 2, 2025 to discuss its DEE portfolio [registration link]

Cash and investments of $472 million as of March 31, 2025, maintains runway into 2028

BOSTON, May 02, 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 first quarter 2025.

"We have made substantial progress in advancing our promising late-stage epilepsy portfolio and are firing on all cylinders through this unpredictable time in the industry," said Marcio Souza, president and chief executive officer of Praxis. "With multiple upcoming catalysts and data readouts, we expect a transformative year at Praxis. In the near-term, we anticipate topline results for vormatrigine from the RADIANT study in epilepsy by mid-2025, and from the POWER1 study in the second half of 2025. Vormatrigine continues to demonstrate an ideal profile and strong competitive differentiation"

Mr. Souza continued, "The registrational cohort of the EMBOLD study is recruiting strongly and is on-track for topline results no later than the first half of 2026, and we will soon initiate the registrational EMERALD study to investigate relutrigine more broadly in DEEs. The EMBRAVE study with elsunersen is progressing very well, with topline results expected in the first half of next year to support the registrational package, and we will initiate the Phase 3 EMBRAVE3 trial for elsunersen by mid-2025. While the interim analysis of Essential3 study 1 for ulixacaltamide was not what we expected, we are on track to complete both study 1 and study 2 for topline readout in the third quarter of this year. Our solid financial position ensures we have sufficient funding for all studies supporting our late-stage programs, with runway into 2028."

Recent Highlights and Anticipated Milestones
Cerebrum™ Small Molecule Platform

- **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 the currently approved drugs have significant safety and efficacy limitations. Vormatrigine is the most potent sodium-channel modulator ever designed to precisely target the hyperexcitable state of sodium-channels in adult common epilepsies.
    - At the American Association of Neurology (AAN) conference earlier this quarter, Praxis presented further data from recently completed Phase 1 studies [link], where vormatrigine continued to demonstrate an ideal profile with strong competitive differentiation.
        - Results from an additional 45 mg multiple ascending dose (MAD) cohort demonstrated a 20x exposure on the MES EC_{50} scale achieved in subjects, the most of any anti-seizure medicine, with a tolerable safety profile.
        - The Phase 1 food effect study demonstrated that food intake does not significantly impact vormatrigine absorption and therefore vormatrigine can be administered with or without food, which increases flexibility in dosing and ease of use for patients.
    - Enrollment and progress across the ENERGY program continues to advance
        - Praxis provided an update on the EMPOWER registrational study [link]at AAN, which continues to attract epilepsy patients and be a source for identifying candidate patients for the RADIANT and POWER1 studies, with over 3,000 patients registered to date
        - The RADIANT Phase 2 study in FOS and generalized epilepsy is progressing, with topline results expected mid-year 2025
        - The POWER1 Phase 2/3 registrational study for treatment resistant FOS is enrolling, with topline results expected in the second half of 2025

- The POWER2 Phase 2/3 registrational study for treatment resistant FOS is on track to initiate in the second half of 2025

- **Relutrigine for DEEs:** Relutrigine is a sodium channel modulator with therapeutic potential across developmental epilepsies. Relutrigine is currently being evaluated in the EMBOLD study in SCN2A and SCN8A DEEs, and we will be initiating the EMERALD study in a broader, pan-DEE patient population. Relutrigine has Rare Pediatric Disease Designation in three indications: SCN1A epilepsy (Dravet syndrome), SCN2A and SCN8A DEEs

    - The EMBOLD registrational cohort 2 continues to enroll strongly, with topline results expected no later than the first half of 2026, followed by potential NDA filing.
    - Praxis has completed discussions with the FDA on a trial for broad DEEs and will initiate the EMERALD registrational study in mid-2025. The study design will be shared at today's DEE virtual analyst event.

- **Ulixacaltamide for Essential Tremor (ET)**: ET is an inadequately managed, undertreated and high burden disease with a prevalence of seven million patients in the U.S. The Essential3 program is the first Phase 3 study in the disease and includes two registrational studies: Study 1 is a parallel design, placebo-controlled study (N=400) and Study 2 is a randomized withdrawal study (N=200). Since beginning recruitment in November 2023, over 200,000 patients have demonstrated interest in participating in the study.

    - On February 28, Praxis shared the results of a pre-planned interim analysis of Study 1. The Independent Data Monitoring Committee recommended that the study be stopped for futility, due to the results being unlikely to meet the primary efficacy endpoint under the parameters set by the statistical model.
    - Praxis has decided to continue the studies to completion and will share topline results in the third quarter of 2025. After reviewing the results of both studies, Praxis will make a decision if there is sufficient evidence to support an NDA filing.

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. Praxis has two studies supporting its registrational filing.
    - The EMBRAVE Part A Phase 1/2 study is continuing to enroll. Patients are randomized 3:1 drug to sham for a six-month period on a once-monthly dose, with the potential to escalate from 1 mg to 8 mg. Topline results are expected in the first half of 2026.
    - The EMBRAVE3 registrational study for SCN2A GoF-DEE is on track to initiate in mid-2025. Cohort 1 of the study will enroll up to 40 patients ages 2 to 18 years, 1:1 between drug and sham for a six-month period with once monthly intrathecal dosing of 1 mg per visit, with a primary endpoint measuring reduction in seizure frequency. Cohorts 2 and 3 will evaluate patients from ages 1 to 2 and 0 to 1 years, respectively.
    - In April 2025, the experience of an emergency-use patient dosed on elsunersen was published in Nature Medicine [linkl providing preliminary insights on the safety and efficacy of elsunersen in a preterm infant with early-onset SCN2A developmental and epileptic encephalopathy.
- Praxis remains on track to nominate a development candidate for each of its early stage ASO therapeutic initiatives in 2025
    - PRAX-080: Focused on targeting PCDH19 mosaic expression
    - 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 (ASD).

First Quarter 2025 Financial Results:

As of March 31, 2025, Praxis had $472.0 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 increase of $2.5 million is primarily attributable to net proceeds from at-the-market offerings of common stock offset by cash used in operating activities. The Company's cash, cash equivalents and marketable securities as of March 31, 2025 are expected to fund operations into 2028.

Praxis did not recognize any collaboration revenue during the three months ended March 31, 2025, compared to $0.4 million during the three months ended March 31, 2024. The decrease of $0.4 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 $60.8 million for the three months ended March 31, 2025, compared to $27.0 million for the three months ended March 31, 2024. The increase in research and development expenses of $33.8 million was primarily attributable to $29.9 million in increased expenses related to the Company's Cerebrum™ platform, $1.9 million in increased personnel expenses, and $1.9 million in increased indirect expenses.

General and administrative expenses were $13.9 million for the three months ended March 31, 2025, compared to $15.3 million for the three months ended March 31, 2024. The decrease in general and administrative expenses of approximately $1.4 million was primarily due to $2.8 million in decreased personnel-related expenses, partially offset by a $1.4 million increase in indirect expenses.

Praxis reported a net loss of $69.3 million for the three months ended March 31, 2025, including $8.8 million of stock-based compensation expense, compared to $39.6 million for the three months ended March 31, 2024, including $14.5 million of stock-based compensation.

As of March 31, 2025, Praxis had 20.3 million shares of common stock outstanding.

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 Na_V 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 study demonstrated that vormatrigine can be safely dosed in healthy subjects to greater than 20 times the predicted human equivalent of the rodent MES EC_{50}. 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 encephalopathy (DEE) as a preferential inhibitor of persistent sodium current, shown to be a key driver of seizure symptoms in early onset SCN2A-DEE and SCN8A-DEE. Relutrigine's mechanism of sodium channel blocking is consistent with superior selectivity for disease state sodium channel (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 blocking effects. Relutrigine has received ODD and RPDD from the FDA, and ODD from the European Medicines Agency for the treatment of SCN2A-DEE and SCN8A-DEE and RPDD for Dravet Syndrome from the FDA. To learn more about the EMBOLD study, please visit EMBOLD study.

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 in late-stage development for the treatment of essential tremorEssential3 study.

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 RPD 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 epilepsy and movement disorders, 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 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, 2025	December 31, 2024
Assets		
Cash and cash equivalents	$ 165,567	$ 215,372
Marketable securities	306,456	254,156
Prepaid expenses and other current assets	5,584	11,805
Property and equipment, net	247	230
Operating lease right-of-use assets	882	1,131
Other non-current assets	—	416
Total assets	$ 478,736	$ 483,110
Liabilities and stockholders' equity		
Accounts payable	$ 22,909	$ 12,528
Accrued expenses	15,547	23,763
Operating lease liabilities	1,066	1,369
Common stock	14	14
Additional paid-in capital	1,344,577	1,281,522
Accumulated other comprehensive gain	659	654
Accumulated deficit	(906,036)	(836,740)
Total liabilities and stockholders' equity	$ 478,736	$ 483,110

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

	Three Months Ended March 31,	
	2025	2024
Collaboration revenue	$ —	$ 431
Operating expenses:		
Research and development	60,806	26,984
General and administrative	13,922	15,333
Total operating expenses	74,728	42,317
Loss from operations	(74,728)	(41,886)
Other income:		
Other income, net	5,432	2,333
Total other income	5,432	2,333
Net loss	$ (69,296)	$ (39,553)
Net loss per share attributable to common stockholders, basic and diluted	$ (3.29)	$ (2.84)
Weighted average common shares outstanding, basic and diluted	21,055,834	13,904,374

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