Praxis Precision Medicines Provides Corporate Update and Reports Second Quarter 2024 Financial Results

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Up to four programs in registrational phase by 2025

Planned interim analysis for ulixacaltamide Essential3 Study 1 to be conducted in Q4 2024

Topline results for Phase 2 EMBOLD study of relutrigine (PRAX-562) in SCN2A and SCN8A developmental and epileptic encephalopathies (DEEs) expected in Q3 2024

PRAX-628 Phase 2/3 POWER1 study to initiate in Q4 2024

Initiation of additional Phase 2 study (RADIANT) for PRAX-628 in focal and generalized epilepsy in 2H 2024, with topline results expected in 1H 2025

Cash and investments of $434 million as of June 30, 2024, maintains runway into 2027

BOSTON, Aug. 13, 2024 (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 second quarter 2024.

“In the second quarter we continued to advance our entire portfolio, and we are poised to soon have up to four programs in registrational stage. The Essential3 program continues to progress well, with a pre-planned interim analysis of Study 1 to be conducted later this year. We remain on track for a successful topline readout enabling an NDA filing in 2025,” said Marcio Souza, president and chief executive officer of Praxis. “We look forward to sharing topline results of the EMBOLD study for relutrigine, formerly known as PRAX-562, in SCN2A and 8A patients later this quarter and believe these indications could be the tip of the iceberg for future application of PRAX-562 in other DEEs.”

Mr. Souza added, “We are also excited to build on the encouraging data generated to date with PRAX-628 by executing on the ENERGY program, a comprehensive set of studies, including a new Phase 2 study (RADIANT), with topline data expected in the first half of 2025 in focal and generalized epilepsy. Additionally, with elsunersen having initiated the first arm of its global confirmatory study in Brazil, we anticipate the rapid advancement of all four clinical programs in our portfolio towards regulatory registrations. Our strong cash position, which fully funds Praxis through several important readouts into 2027, enables this momentum.”

Recent Highlights and Anticipated Milestones:

Cerebrum™ Small Molecule Platform

- **Ulixacaltamide for ET**: Topline results of two studies in the Essential3 Phase 3 program for ulixacaltamide are expected in the second half of 2024 to support a planned New Drug Application (NDA) submission in 2025.
  - Since beginning recruitment in November 2023, there have been over 75,000 pre-screening forms, of which 11,000 referrals meet pre-qualifying eligibility criteria.
  - Praxis to conduct an interim analysis for Study 1 in the Essential3 program in the fourth quarter of 2024. This pre-planned analysis is part of the original statistical plan and protocol for Essential3.
  - Long-term safety study enrollment criteria have been expanded to include patients who participated in previous ET studies, including previous suvecaltamide trials.
- **PRAX-628 for Epilepsy:** Praxis expects to initiate four studies as part of the PRAX-628 ENERGY program, aiming to generate efficacy, safety and pharmacokinetics (PK) data to serve as the basis of regulatory registrations globally.
  - EMPOWER is an observational study in partnership with the Epilepsy Consortium expected to start in the third quarter of 2024, aiming to enroll patients with epilepsy to better characterize seizure burden
  - RADIANT is a Phase 2 PK, safety and efficacy study in patients with focal onset seizures (FOS) or generalized epilepsy expected to start in the second half of 2024, with topline results in the first half of 2025
  - POWER1 and POWER2 are 12-week Phase 2/3 studies in patients with FOS aiming to show efficacy of PRAX-628. POWER1 is expected to start enrolling patients in the fourth quarter of 2024, with topline results in the second half of 2025. POWER2 is expected to start enrolling patients in the first half of 2025.

- **Relutrigine (PRAX-562) for SCN2A and SCN8A DEEs:** Praxis expects topline results from the Phase 2 EMBOLD study for the treatment of pediatric patients with DEEs in the third quarter of 2024.
    - The EMBOLD study is a Phase 2 study evaluating the safety, tolerability, efficacy (motor seizure frequency) and PK of relutrigine in pediatric patients aged 2 to 18 years with DEEs, followed by an open-label extension.
    - Based on results for the EMBOLD study, Praxis expects to consider a more expansive program of relutrigine in all DEEs with high seizure burden.

Solidus™ Antisense Oligonucleotide (ASO) Platform

- **Elsunersen (PRAX-222) for early-seizure-onset SCN2A Developmental Epilepsies**
    - First arm of the global confirmatory study initiated in Brazil
    - Pivotal phase of the program remains on track, and Praxis plans to advance the program within the US and expand to Europe later in 2024.

Second Quarter 2024 Financial Results:

As of June 30, 2024, Praxis had $433.8 million in cash, cash equivalents, and marketable securities, compared to $81.3 million in cash and cash equivalents as of December 31, 2023. The increase of $352.5 million is primarily due to net proceeds from Praxis' January 2024 and April 2024 follow-on public offerings plus net proceeds from at-the-market sales of common stock, offset by cash used in operating activities.

Praxis recognized $0.4 million in collaboration revenue during the three months ended June 30, 2024, compared to $0.8 million during the three months ended June 30, 2023. The decrease of $0.4 million is associated with a decrease in revenue recorded under the UCB Collaboration Agreement.

Research and development expenses were $27.3 million for the three months ended June 30, 2024, compared to $25.6 million for the three months ended June 30, 2023. The increase in research and development expenses of $1.6 million was primarily attributable to an increase of $8.1 million in Praxis' Cerebrum™ platform and a $1.7 million increase in personnel-related costs, primarily offset by a $8.2 million decrease in costs associated with Praxis' Solidus™ platform. General and administrative expenses were $10.6 million for the three months ended June 30, 2024, compared to $10.1 million for the three months ended June 30, 2023. The increase in general and administrative expenses of approximately $0.5 million was primarily due to increased personnel-related costs.

Praxis reported a net loss of $32.7 million for the three months ended June 30, 2024, including $5.9 million of stock-based compensation expense, compared to $34.3 million for the three months ended June 30, 2023, including $5.8 million of stock-based compensation.

As of June 30, 2024, Praxis had 17.8 million shares of common stock outstanding.

Conference Call
Praxis Precision Medicines will host a conference call and webcast today at 8:00 a.m. ET to review the second quarter 2024 financial results and recent business highlights. Individuals may register for the conference call by clicking the registration link. Once registered, participants will receive dial-in details and a unique PIN which will allow them to access the call. An audio webcast will be accessible through the Investor Relations section of the company's website at www.praxismedicines.com. Following the live webcast, an archived replay will also be available.

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 tremor, www.praxisessentialtremor.com.

About PRAX-628
PRAX-628 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 epilepsy. Preclinical data demonstrates PRAX-628 is differentiated from standard of care, with the potential to be best-in-class for focal epilepsy. In vitro, PRAX-628 has demonstrated superior selectivity for disease-state Na_V channel hyperexcitability. In vivo studies of PRAX-628 have demonstrated unprecedented potency in the maximal electroshock seizure (MES) model, a highly predictive translational model for efficacy in focal epilepsy. Data from the PRAX-628-101 study demonstrated that PRAX-628 can be safely dosed in healthy subjects to greater than 15 times the predicted human equivalent of the rodent MES EC50.

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 Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation (RPD) from the FDA, and ODD and PRIME designations from the European Medicines Agency (EMA) for the treatment of SCN2A-DEE. The Elsunersen program is ongoing under a collaboration with Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), and RogCon, Inc. To learn more about the EMBRAVE study, please visit https://www.embravestudy.com/.

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 RPD from the FDA, and ODD from the European Medicines Agency for the treatment of SCN2A-DEE and SCN8A-DEE. To learn more about the EMBOLD study, please visit https://www.emboldstudy.org/.

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, 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, 2023 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, 2024	December 31, 2023
Assets		
Cash and cash equivalents	$ 145,143	$ 81,300
Marketable securities	288,688	—
Prepaid expenses and other current assets	4,837	3,580
Property and equipment, net	369	588
Operating lease right-of-use assets	1,610	2,064
Other non-current assets	416	416
Total assets	$ 441,063	$ 87,948
Liabilities and stockholders' equity		
Accounts payable	$ 8,195	$ 5,815
Accrued expenses	10,058	7,416
Operating lease liabilities	1,946	2,495

Deferred revenue	1,764	2,553
Common stock	14	13
Additional paid-in capital	1,145,308	723,577
Accumulated other comprehensive loss	(71)	—
Accumulated deficit	(726,151)	(653,921)
Total liabilities and stockholders' equity	$ 441,063	$ 87,948

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

	Three Months Ends June 30,		Six Months Ended June 30,	
	2024	**2023**	**2024**	**2023**
Collaboration revenue	$ 357	$ 781	$ 788	$ 1,464
Operating expenses:				
Research and development	27,260	25,614	54,244	51,118
General and administrative	10,585	10,127	25,918	23,397
Total operating expenses	37,845	35,741	80,162	74,515
Loss from operations	(37,488)	(34,960)	(79,374)	(73,051)
Other income:				
Other income, net	4,811	648	7,144	1,284
Total other income	4,811	648	7,144	1,284
Net loss	$ (32,677)	$ (34,312)	$ (72,230)	$ (71,767)
Net loss per share attributable to common stockholders, basic and diluted	$ (1.74)	$ (7.38)	$ (4.41)	$ (17.51)
Weighted average common shares outstanding, basic and diluted	18,824,479	4,649,371	16,364,421	4,097,833

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