Praxis Precision Medicines Provides Corporate Update and Reports Third Quarter 2023 Financial Results

November 7, 2023

Essential3 study initiated, with over 600 patients engaged in pre-recruitment observational study

Elsunersen EMBRAVE study final dose completed, Praxis seeking FDA advice on advancing development

Cash of $101.1 million as of September 30, 2023 supports runway into Q1 2025

BOSTON, Nov. 07, 2023 (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 2023.

“This quarter was momentous for Praxis as we began our first Phase 3 studies in ulixacaltamide for essential tremor,” said Marcio Souza, president and chief executive officer of Praxis. “Our epilepsy portfolio continues to advance as well, with elsunersen completing the final dosing for Part 1 of the EMBRAVE study and preliminary data shared at our 2023 R&D Day showing solid improvement to reduce seizures, while PRAX-562 and PRAX-628 are enrolling well in their respective Phase 2 programs. We look forward to sharing the PRAX-628 data in photo-paroxysmal response (PPR) in the near future.”

Recent Business Highlights and Upcoming Milestones:

Cerebrum™ Small Molecule Platform

- In November 2023, Praxis initiated Essential3, the Phase 3 program for ulixacaltamide. Essential3 is comprised of two Phase 3 studies and a long-term safety study. One study is a parallel-group, placebo-controlled design and the other is a randomized withdrawal design. Both trials will evaluate efficacy and safety in essential tremor (ET) patients at a 60 mg dose of ulixacaltamide for up to 12 weeks after a short titration period. In September 2023, Praxis initiated a pre-recruitment observational study in which over 600 ET patients expressed interest in joining a trial sponsored by Praxis. Praxis expects to complete enrollment of Essential3 in the first half of 2024.
- In October 2023 at its R&D Day, Praxis shared additional analyses from the Essential1 data set that continue to support the design of the Phase 3 program for ulixacaltamide in ET
    - Data from Essential1 showed a meaningful difference in a minimum 3-point improvement in the modified Activities of Daily Living 11-point scale (mADL11) between patients on ulixacaltamide v. placebo (55% v. 31%, p=0.023)
    - In the extension period of Essential1, which continued after Week 8 through Week 14, 64% of patients in the ulixacaltamide arm showed at least a 3-point improvement in mADL11 versus baseline. For patients transitioning from placebo at Week 8 onto ulixacaltamide, 69% achieved at least a 3-point improvement from baseline at Week 14
    - Ulixacaltamide also demonstrated incremental benefit to patients on propranolol: 48% of patients on propranolol and ulixacaltamide achieved at least a 3-point improvement in mADL11 compared to 25% for patients on propranolol and placebo
- Praxis expects topline results from the PRAX-628 Phase 2a proof-of-concept study evaluating epilepsy patients with PPR in the fourth quarter of 2023. The study evaluates the potential effect of PRAX-628 on reducing pre-seizure electroencephalogram activity for photo-sensitive patients. Upon completion of the PPR study, Praxis plans to initiate a Phase 2 study to evaluate PRAX-628 for the treatment of focal epilepsy in the first half of 2024.
- Praxis expects topline results from the PRAX-562 Phase 2 EMBOLD study for the treatment of pediatric patients with developmental and epileptic encephalopathies (DEEs) in the first half of 2024. The EMBOLD study is a randomized, double-blind, placebo-controlled Phase 2 clinical trial to evaluate the safety, tolerability, efficacy (motor seizure frequency) and pharmacokinetics of PRAX-562 in pediatric patients aged 2 to 18 years with DEEs, followed by an open-label extension. Up to 20 participants with SCN2A-DEE or SCN8A-DEE are expected to be enrolled.

Solidus™ Antisense Oligonucleotide (ASO) Platform

- In October 2023, Praxis completed dosing for Part 1 of the PRAX-222 EMBRAVE study for the treatment of pediatric patients with early-onset SCN2A-DEE. Part 1 of the EMBRAVE study is a 21-week open-label cohort in which participants

received four monthly doses of PRAX-222 (elsunersen) for approximately 13 weeks, designed to determine the safety and tolerability of intrathecal delivery of PRAX-222. The final data will be evaluated and submitted to the U.S. Food and Drug Administration to advance development.

- At the company's R&D Day in early October, preliminary analyses as of September 26, 2023, from Part 1 of the EMBRAVE study for the four patients through the first three dosing periods was shared, which showed significant seizure reduction with a favorable safety profile.

Third Quarter 2023 Financial Results:

As of September 30, 2023, Praxis had $101.1 million in cash and cash equivalents, compared to $100.5 million in cash, cash equivalents and marketable securities as of December 31, 2022. The increase of $0.6 million primarily reflects $63.4 million in net proceeds from Praxis' June 2023 underwritten public offering and $24.1 million in net proceeds from at-the-market offerings of shares of Praxis' common stock, partially offset by cash used in operations of $87.3 million during the nine months ended September 30, 2023.

Praxis recognized $0.5 million in collaboration revenue during the three months ended September 30, 2023, related to its Option and License Agreement with UCB.

Research and development expenses were $17.3 million for the three months ended September 30, 2023, compared to $30.4 million for the three months ended September 30, 2022. The decrease in research and development expenses of $13.2 million was primarily attributable to $10.5 million in decreased expenses related to Praxis' Cerebrum™ platform, $0.8 million in decreased expenses related to Praxis' Solidus™ platform and $1.9 million in decreased personnel-related expenses. General and administrative expenses were $8.7 million for the three months ended September 30, 2023, compared to $13.9 million for the three months ended September 30, 2022. The decrease in general and administrative expenses of approximately $5.1 million was primarily due to a decrease in personnel-related expenses, consulting costs and professional fees.

Praxis reported a net loss of $24.6 million for the three months ended September 30, 2023, including $5.8 million of stock-based compensation expense, compared to $43.9 million for the three months ended September 30, 2022, including $6.7 million of stock-based compensation expense.

As of September 30, 2023, Praxis had 128.5 million shares of common stock outstanding.

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.

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 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; reported interim data from ongoing studies and trials 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; Praxis' anticipated cash runway; and other risks concerning Praxis' programs and operations as described in its Annual Report on Form 10-K for the year ended December 31, 2022, its Quarterly Reports on Form 10-Q 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, 2023	December 31, 2022
Assets		
Cash and cash equivalents	$ 101,085	$ 61,615
Marketable securities	—	38,874
Prepaid expenses and other current assets	2,242	10,351
Property and equipment, net	700	971
Operating lease right-of-use assets	2,282	2,901
Other non-current assets	416	416
Total assets	$ 106,725	$ 115,128
Liabilities and stockholders' equity		
Accounts payable	$ 7,166	$ 14,672
Accrued expenses	6,979	15,850
Operating lease liabilities	2,756	3,500
Deferred revenue	3,068	5,000
Common stock	13	5
Additional paid-in capital	713,786	606,918
Accumulated other comprehensive loss	—	(173)
Accumulated deficit	(627,043)	(530,644)
Total liabilities and stockholders' equity	$ 106,725	$ 115,128

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,	
	2023	2022	2023	2022
Collaboration revenue	$ 468	$ —	$ 1,932	$ —
Operating expenses:				
Research and development	17,260	30,439	68,378	126,711
General and administrative	8,724	13,851	32,121	46,822
Total operating expenses	25,984	44,290	100,499	173,533
Loss from operations	(25,516)	(44,290)	(98,567)	(173,533)
Other income:				
Other income, net	884	345	2,168	677
Total other income	884	345	2,168	677
Net loss	$ (24,632)	$ (43,945)	$ (96,399)	$ (172,856)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.18)	$ (0.96)	$ (1.12)	$ (3.79)
Weighted average common shares outstanding, basic and diluted	135,591,429	45,774,376	86,447,174	45,591,888

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