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

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On track to initiate Phase 3 studies for ulixacaltamide in Q4 2023 after favorable End-of-Phase 2 meeting with FDA

PRAX-628 Phase 1 study showed consistent safety profile and target engagement in measures of qEEG activity at all doses with first administration

Praxis will hold an R&D Portfolio Day on October 2

Cash of $124.3 million as of June 30, 2023 expected to support runway into Q1 2025

BOSTON, Aug. 09, 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 second quarter 2023.

“Our four clinical-stage programs continue to make great progress, and we are excited to be advancing ulixacaltamide into Phase 3,” said Marcio Souza, president and chief executive officer of Praxis. “Our epilepsy portfolio continues to advance, with studies ongoing in each of our three clinical-stage programs that we expect to read out by the end of the year. We are planning to hold an R&D portfolio day on October 2 to elaborate on our science and clinical progress, including more details about the Phase 3 program for ulixacaltamide and additional data from the Essential1 study.”

Recent Business Highlights and Upcoming Milestones:

Cerebrum™ Small Molecule Platform

- In June 2023, Praxis shared the outcomes of its end-of-phase 2 (EOP2) meeting with the U.S. Food and Drug Administration (FDA) and is planning to initiate two Phase 3 studies as part of the registrational program, using mADL11 as the primary outcome measure. One study will be a parallel control design and the other using a randomized withdrawal design. Both trials will evaluate essential tremor patients at a 60 mg dose of ulixacaltamide for 12 weeks after a short titration period. The EOP2 meeting confirmed other program aspects including safety database, clinical pharmacology and toxicology requirements for registration. Praxis intends to begin enrolling patients in the fourth quarter of 2023, with read outs expected in the second half of 2024.

- In August 2023, Praxis shared the results of two additional analyses of the Essential1 dataset that showed durable effect to 14 weeks and maintenance of the safety profile seen in the Essential1 study.
    - In the open-label extension (OLE) phase, patients who continued on ulixacaltamide experienced an additional mean improvement in mADL11 of 1.7 points from week 8 to after 14 weeks of treatment, while patients who switched from placebo during the Essential1 double blind phase to ulixacaltamide during the 6-week OLE experienced mean improvement in mADL11 of 3.15 points.
    - In a randomized withdrawal sub-study, patients who switched from ulixacaltamide to placebo experienced an average loss of effect in their mADL11 per week of 47% (mean loss of effect of -1.15 points/week), compared to 6% improvement in global mean change per week (mean improvement of 0.16 points/week) for the periods receiving ulixacaltamide.

- In May 2023, Praxis announced initial results from the PRAX-628 Phase 1 safety study, which demonstrated a favorable safety and tolerability profile in healthy volunteers at concentrations more than 15-fold the Maximal Electrical Seizure model (MES EC_{50}) and predicted therapeutic range at least 3-fold wider than current market leader based on an MES model. In August 2023, Praxis announced additional data from the Phase 1 study from an analysis of EEG activity that demonstrated pharmacodynamic activity across all dose levels for study subjects who received PRAX-628 at first administration as compared with subjects who received placebo.

- In June 2023, Praxis announced it had initiated a Phase 2 proof of concept study evaluating PRAX-628 in epilepsy patients with a Photo Paroxysmal Response (PPR). The study evaluates the potential effect of PRAX-628 on reducing pre-seizure EEG activity for photo-sensitive patients. The study is expected to read out by year-end 2023 and, 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 fourth quarter of 2023. 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 participants aged 2 to 18 years with DEEs, followed by an open-label extension. Approximately 20 participants with SCN2A-DEE or SCN8A-DEE are expected to be enrolled.

Solidus™ Antisense Oligonucleotide (ASO) Platform

- Praxis is currently dosing the first dose cohort (Part 1) of the PRAX-222 EMBRAVE study for the treatment of pediatric patients with early-onset SCN2A-DEE in the U.S. Following collection of the safety and efficacy data from Part 1 of the EMBRAVE study, the data will be evaluated and submitted to the FDA to support further dose escalation. Part 1 of the EMBRAVE study is a 21-week open label cohort, in which participants will receive PRAX-222 for up to 13 weeks, designed to determine the safety and tolerability of intrathecal delivery of PRAX-222. Topline results are expected in the second half of 2023.

Corporate Update

- In June 2023, Praxis completed an underwritten public offering, which extended Praxis' cash runway into the first quarter of 2025. Praxis sold 64,449,690 shares of common stock at a public offering price of $0.95 per share, including the exercise in full by the underwriters of their option to purchase up to 9,299,690 shares of common stock, and pre-funded warrants to purchase up to an aggregate of 7,050,000 shares of common stock at a public offering price of $0.9499 per share. The net proceeds from the offering were approximately $63.4 million, after deducting underwriting discounts and commissions and other offering expenses payable by Praxis. The proceeds will be used to advance the development of ulixacaltamide into two Phase 3 studies for essential tremor, to continue clinical development of PRAX-562, PRAX-222 and PRAX-628 for various epilepsies, and for working capital and other general corporate purposes.

Second Quarter 2023 Financial Results:

As of June 30, 2023, Praxis had $124.3 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 $23.8 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 $64.1 million during the six months ended June 30, 2023.

Praxis recognized $0.8 million in collaboration revenue during the three months ended June 30, 2023 related to its Option and License Agreement with UCB.

Research and development expenses were $25.6 million for the three months ended June 30, 2023, compared to $43.6 million for the three months ended June 30, 2022. The decrease in research and development expenses of $18.0 million was primarily attributable to $19.6 million in decreased expenses related to Praxis' Cerebrum™ platform and $4.0 million in decreased personnel-related expenses, partially offset by $5.7 million in increased expenses related to the Solidus™ platform, which includes a $6.9 million one-time milestone related to the initiation of the EMBRAVE study. General and administrative expenses were $10.1 million for the three months ended June 30, 2023, compared to $16.8 million for the three months ended June 30, 2022. The decrease in general and administrative expenses of approximately $6.6 million was primarily due to a decrease in consulting costs, professional fees and personnel-related expenses.

Praxis reported a net loss of $34.3 million for the three months ended June 30, 2023, including one-time milestone expense of $6.9 million related to the PRAX-222 program, and $5.8 million of stock-based compensation expense, compared to $60.2 million for the three months ended June 30, 2022, including $7.6 million of stock-based compensation expense.

As of June 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 and the development of our product candidates, 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; 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)

	June 30, 2023	December 31, 2022
Assets		
Cash and cash equivalents	$ 124,300	$ 61,615
Marketable securities	—	38,874
Prepaid expenses and other current assets	5,529	10,351
Property and equipment, net	759	971
Operating lease right-of-use assets	2,494	2,901
Other non-current assets	416	416
Total assets	$ 133,498	$ 115,128
Liabilities and stockholders' equity		
Accounts payable	$ 8,010	$ 14,672
Accrued expenses	13,317	15,850
Operating lease liabilities	3,010	3,500
Deferred revenue	3,536	5,000
Common stock	13	5
Additional paid-in capital	708,023	606,918
Accumulated other comprehensive loss	—	(173)
Accumulated deficit	(602,411)	(530,644)
Total liabilities and stockholders' equity	$ 133,498	$ 115,128

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,	
	2023	2022	2023	2022
Collaboration revenue	$ 781	$ —	$ 1,464	$ —
Operating expenses:				
Research and development	25,614	43,620	51,118	96,272
General and administrative	10,127	16,774	23,397	32,971
Total operating expenses	35,741	60,394	74,515	129,243
Loss from operations	(34,960)	(60,394)	(73,051)	(129,243)
Other income:				
Other income, net	648	200	1,284	332
Total other income	648	200	1,284	332
Net loss	$ (34,312)	$ (60,194)	$ (71,767)	$ (128,911)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.49)	$ (1.32)	$ (1.17)	$ (2.83)
Weighted average common shares outstanding, basic and diluted	69,740,719	45,542,600	61,467,774	45,499,131

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