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

May 11, 2023

Ulixacaltamide essential tremor End-of-Phase 2 meeting with FDA scheduled for June 2023

PRAX-628 Phase 1 study results support preclinical profile indicating potential for best-in-class-efficacy for focal epilepsy

Cash and investments of $85.8 million as of March 31, 2023 supports runway into 2Q24

BOSTON, May 11, 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 first quarter 2023.

"Each of our four clinical-stage programs has made meaningful progress this year and we anticipate additional value inflecting milestones throughout the pipeline in the coming months," said Marcio Souza, president and chief executive officer of Praxis. "We shared positive topline results earlier today from the Phase 1 study of our next-generation, functional-state selective small molecule, PRAX-628, and believe that this program has the potential to change the treatment paradigm for people living with focal epilepsy. We look forward to initiating a PRAX-628 Phase 2 study in focal epilepsy later this year, and also plan to share results from our PRAX-562 and PRAX-222 programs in rare, genetic epilepsies. Finally, we eagerly anticipate the upcoming ulixacaltamide End-of-Phase 2 FDA meeting in June and intend to start a Phase 3 program in essential tremor shortly thereafter."

Recent Business Highlights and Upcoming Milestones:

Cerebrum™ Small Molecule Platform

- Praxis announced topline results from the ulixacaltamide (PRAX-944) Phase 2 Essential1 study for the treatment of moderate to severe essential tremor (ET) in March 2023. An end-of-Phase 2 meeting with the U.S. Food and Drug Administration (FDA) is scheduled for June 2023. Based upon the observed efficacy and safety profile, Praxis intends to initiate the ulixacaltamide Phase 3 program for the treatment of ET in the second half of 2023 following FDA feedback on the clinical registration plan and alignment on the overall development program.
- The Company plans to present additional results from the Essential1 study at upcoming medical conference meetings and company events:
    - Essential1 topline results poster at the World Congress on Parkinson's Disease and Related Disorders 2023 (IAPRD) in Chicago, IL on Monday, May 15, 2023 at 12:15 p.m. CDT
    - Essential1 results presentation and scientific talk at the 2nd International Tremor Conference (ITC) in New York, NY on Thursday, May 18, 2023 at 4:20 p.m. EDT
    - Essential1 results presentation and key opinion leader (KOL) company hosted event (details to follow)
- In May 2023, Praxis announced positive topline results from a Phase 1 healthy volunteer study of PRAX-628 evaluating the safety, tolerability and pharmacokinetics (PK) of PRAX-628 across single and multiple ascending dose cohorts (SAD and MAD). PRAX-628 was generally well-tolerated at all tested doses, including concentrations in the MAD that reached more than 15-fold the mouse Maximal Electroshock Seizure model (MES) EC_{50}, a highly predictive translational model for focal epilepsy. Based on the MES model, the predicted therapeutic range of PRAX-628 is at least 3-fold wider than the current market leader in focal epilepsy, indicating potential for best-in-class efficacy for PRAX-628. The Company intends to initiate a PRAX-628 Phase 2 study for the treatment of focal epilepsy in the fourth quarter of 2023.
- 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 PK 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 will be enrolled initially.
- In April 2023, Praxis presented the following posters at the 75th Annual American Academy of Neurology (AAN) meeting:
    - PRAX-562-101: A Phase 1 Trial Evaluating the Safety, Tolerability, Pharmacokinetics and Food Effect of PRAX-562 in Healthy Volunteers (Poster Session P8: 9-011)

- PRAX-562-102: A Phase 1 Trial Evaluating the Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of PRAX-562 in Healthy Volunteers (Poster Session P4: 9-011)
- PRAX-628 is a Novel, Well-tolerated, Activity Dependent Sodium Channel Blocker with Potent Anticonvulsant Activity (Poster Session P4: 9-012)
- PRAX-628: A Novel Sodium Channel Blocker with Greater Potency and Activity Dependence Compared to Standard of Care (Poster Session P8: 9-012)
- A Novel Approach to Assess the Impact of Disease in Patients with SCN8A-Related Developmental and Epileptic Encephalopathy (Poster Session P3: 9-008)
- Disease Impact and Burden in Patients with SCN2A-Related Developmental and Epileptic Encephalopathy (Poster Session P11: 9-011)

Solidus™ Antisense Oligonucleotide (ASO) Platform

- Praxis is conducting 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.

First Quarter 2023 Financial Results:

As of March 31, 2023, Praxis had $85.8 million in cash, cash equivalents and marketable securities, which is expected to fund operations into the second quarter of 2024.

Praxis recognized $0.7 million in collaboration revenue during the three months ended March 31, 2023 related to its Option and License Agreement with UCB.

Research and development expenses were $25.5 million for the three months ended March 31, 2023, compared to $52.7 million for the three months ended March 31, 2022. The decrease in research and development expenses of $27.1 million was primarily attributable to $25.3 million in decreased expenses related to the Company's Cerebrum™ and Solidus™ platforms and a $3.(million decrease in personnel-related expenses.

General and administrative expenses were $13.3 million for the three months ended March 31, 2023, compared to $16.2 million for the three months ended March 31, 2022. The decrease in general and administrative expenses of approximately $2.9 million was primarily due to a decrease in consulting and insurance-related costs as well as a decrease in personnel-related expenses.

Praxis reported a net loss of $37.5 million for the three months ended March 31, 2023, including $7.6 million of stock-based compensation expense, compared to $68.7 million for the three months ended March 31, 2022, including $7.9 million of stock-based compensation expense.

As of March 31, 2023, Praxis had 58.0 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 submissions for regulatory approval or review by governmental authorities; regulatory approvals to conduct trials; Praxis' ability to continue as a going concern; 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)

	March 31, 2023	December 31, 2022
Assets		
Cash and cash equivalents	$ 80,839	$ 61,615
Marketable securities	4,983	38,874
Prepaid expenses and other current assets	8,580	10,351
Property and equipment, net	865	971
Operating lease right-of-use assets	2,700	2,901
Other non-current assets	416	416
Total assets	$ 98,383	$ 115,128
Liabilities and stockholders' equity		
Accounts payable	$ 16,986	$ 14,672
Accrued expenses	9,352	15,850
Operating lease liabilities	3,260	3,500
Deferred revenue	4,317	5,000
Common stock	6	5
Additional paid-in capital	632,580	606,918
Accumulated other comprehensive loss	(19)	(173)
Accumulated deficit	(568,099)	(530,644)
Total liabilities and stockholders' equity	$ 98,383	$ 115,128

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

	Three Months Ended March 31,	
	2023	2022
Collaboration revenue	$ 683	$ —
Operating expenses:		
Research and development	25,504	52,652
General and administrative	13,270	16,197
Total operating expenses	38,774	68,849
Loss from operations	(38,091)	(68,849)
Other income:		
Other income, net	636	132
Total other income	636	132
Net loss	$ (37,455)	$ (68,717)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.71)	$ (1.51)
Weighted average common shares outstanding, basic and diluted	53,102,907	45,455,179

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