Praxis Precision Medicines Provides Corporate Update and Reports First Quarter 2022 Financial Results

May 9, 2022

PRAX-944 demonstrated clinically meaningful functional improvement in essential tremor patients in Part B of Phase 2a study

PRAX-114 Phase 2/3 monotherapy MDD Aria Study completed; topline results expected in June 2022

Epilepsy Day showcases largest targeted epilepsy portfolio in industry

Cash and investments of $222.5 million as of March 31, 2022 supports runway into 3Q23

BOSTON, May 09, 2022 (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, including a video highlighting recent business and pipeline progress, and reported financial results for the first quarter 2022.

“This is an incredibly exciting time at Praxis, with recent events bringing us closer to our long-term vision,” said Marcio Souza, president and chief executive officer of Praxis. “With positive topline results from our PRAX-944 Phase 2a study in essential tremor, and plans to accelerate development in the ongoing Essential1 study, we will soon have a second late-stage clinical asset along with a deep and innovative early-stage pipeline. We also recently completed our PRAX-114 Aria study, with last patient last visit achieved, and look forward to sharing those topline results in June.”

Recent Business Highlights and Upcoming Milestones:

Psychiatry

- Following the completion of the PRAX-114 Phase 2/3, placebo-controlled Aria Study for monotherapy treatment of Major Depressive Disorder (MDD), Praxis expects to report topline results in June 2022. The Aria Study is intended to serve as one of two trials required by the U.S. Food and Drug Administration (FDA) to demonstrate clinical efficacy to support registration of PRAX-114 for monotherapy treatment of MDD. Following topline results from the Aria Study, the Company intends to engage with the FDA for an end-of-Phase 2 meeting and subsequently initiate a Phase 3, placebo-controlled study in the fourth quarter of 2022.

- The Company anticipates topline results from the PRAX-114 Phase 2, placebo-controlled, dose-ranging Acapella Study for treatment of MDD in the third quarter of 2022. The Acapella Study is intended to provide additional understanding of the dose range and to evaluate the safety and efficacy of PRAX-114 at doses of 10, 20, 40 and 60 mg.

- Praxis expects topline results from the PRAX-114 Phase 2, placebo-controlled study for treatment of post-traumatic stress disorder (PTSD) in the second half of 2022. The trial is designed to evaluate the safety, tolerability and efficacy of a nightly dose of 40 mg of PRAX-114 for four weeks in approximately 80 participants with PTSD, using the CAPS-5 total score as the primary endpoint. An 8-week open-label extension period is available for all participants who complete the placebo-controlled portion of the study.

Movement Disorders

- Praxis reported positive topline results from Part B of its Phase 2a study evaluating the safety and efficacy of PRAX-944 for the treatment of essential tremor (ET). In the study, treatment with PRAX-944 resulted in clinically meaningful improvements in function, which were supported by improvements in tremor amplitude.
    - The study included both an open-label and randomized withdrawal period. In the open-label period, participants were to be titrated up to a maximum dose of 120 mg over 28-days prior to a stable period at the highest dose reached from Day 29 to Day 42. Participants who remained in the study through Day 42 were then randomized one-to-one to either active drug or placebo from Day 43 to Day 56, with a subsequent safety follow-up visit at Day 70.
    - In the open-label period through Day 42, patients treated with PRAX-944 demonstrated mean improvement from baseline of 42% in the Modified Activities of Daily Living (ADL) score (N=11, nominal $p<0.05$). Following randomization, the difference between patients who remained on treatment (N=6) through Day 56 and those

randomized to placebo (N=5) was clinically and statistically significant.
  - PRAX-944 was generally well tolerated in the study, with no new safety findings.

- The Company anticipates topline results from the PRAX-944 Phase 2b Essential1 Study for daytime treatment of ET in the second half of 2022. Following the topline results of Part B of the Phase 2a study of PRAX-944 for the treatment of ET, the Company intends to change the primary endpoint of Essential1 from safety to efficacy.

- In April 2022, Praxis presented data from PRAX-944 for ET at the 2022 American Academy of Neurology (AAN) Annual Meeting, including an oral presentation on the translational pharmacology of PRAX-944.

- Praxis initiated a Phase 2, placebo-controlled, crossover study of 10 and 20 mg of PRAX-114 for daytime treatment of ET in the first quarter of 2022 and expects topline results in the second half of 2022.

- In April 2022, the FDA cleared the Investigational New Drug (IND) submission for a Phase 2 study of PRAX-944 for the treatment of Parkinson's disease. Praxis intends to initiate a Phase 2, placebo-controlled trial to evaluate the safety, pharmacokinetics (PK) and efficacy of PRAX-944 as a non-dopaminergic treatment for the motor symptoms of Parkinson's disease in the second half of 2022.

Epilepsy

- In April 2022, Praxis hosted its 2022 Epilepsy Day, which included an overview of the Company's proprietary innovation strategy, updates on its most advanced epilepsy programs and a review of the progress in Praxis' early-stage epilepsy pipeline, including plans to declare antisense oligonucleotide (ASO) candidates for PRAX-080 for PCDH19 and PRAX-090 for SYNGAP1 in 2023.

- Praxis plans to initiate a PRAX-562 Phase 2, placebo-controlled trial for treatment of developmental epileptic encephalopathies (DEEs), including SCN2A-DEE, SCN8A-DEE and Tuberous Sclerosis Complex (TSC) in the second half of 2022.

- Praxis expects to initiate a PRAX-628 Phase 1 study in the fourth quarter of 2022 and subsequently initiate a Phase 2 study in focal epilepsy in 2023.

- In April 2022, Praxis announced that it received an email communication from the FDA that the Company's IND application for the first-in-patient study of PRAX-222, an ASO for the treatment of patients with SCN2A gain-of-function mutations, was placed on clinical hold. The letter detailing the reasons for the hold is expected to be received from the FDA within 30 days of April 28, 2022.

- In April 2022, Praxis entered into a collaboration with the University of Florida Scripps Biomedical Research Institute. As part of the agreement, Praxis will collaborate with Gavin Rumbaugh, Ph.D. and his laboratory, to leverage his phenotypic screening platform to discover and advance a novel small molecule program to treat patients with SYNGAP1.

- As a result of the Company's strategic decision to renew its focus toward epilepsy indications, Praxis has discontinued the planned PRAX-562 Phase 2 trial for treatment of rare adult cephalgias.

First Quarter 2022 Financial Results:

As of March 31, 2022, Praxis had $222.5 million in cash, cash equivalents and marketable securities, compared to $275.9 million in cash, cash equivalents and marketable securities as of December 31, 2021. This decrease of $53.4 million primarily reflects cash used in operations of $54.1 million during the three months ended March 31, 2022. The Company's cash, cash equivalents and marketable securities as of March 31, 2022 are expected to fund operations into the third quarter of 2023.

Research and development expenses were $52.7 million for the three months ended March 31, 2022, compared to $17.9 million for the three months ended March 31, 2021. The increase in research and development expenses of $34.7 million was primarily attributable to $28.1 million in increased expenses related primarily to clinical-related spend for the Company's franchises, $4.6 million in increased personnel-related costs due to increased headcount and $1.5 million in increased expenses for other exploratory CNS indications.

General and administrative expenses were $16.2 million for the three months ended March 31, 2022, compared to $9.5 million for the three months ended March 31, 2021. The increase in general and administrative expenses of $6.7 million was primarily attributable to $5.0 million in increased personnel-related costs due to increased headcount, $1.3 million in increased professional fees and a $0.4 million increase in other general and administrative expenses.

Praxis reported a net loss of $68.7 million for the three months ended March 31, 2022, including $7.9 million of stock-based compensation expense, compared to $27.4 million for the three months ended March 31, 2021, including $4.7 million of stock-based compensation expense.

As of March 31, 2022, Praxis had 45.5 million shares of common stock outstanding.

Conference Call and Webcast

Praxis will host a Q&A session focused on today's corporate update and financial results for the first quarter 2022 via a conference call and webcast today, May 9, 2022, at 4:30 p.m. ET. To access the conference call, please dial (833) 398-1037 (local) or (914) 987-7735 (international) at least 10 minutes prior to the start time and refer to conference ID 7849239. A live audio webcast of the event may also be accessed through the Events & Presentations page of the Investors + Media section of the company's website at https://investors.praxismedicines.com/events-and-presentations. A replay of the webcast will be available on Praxis' website approximately two hours after the completion of the event and will be archived for 30 days following the event.

About Praxis

Praxis Precision Medicines is a clinical-stage biopharmaceutical company translating genetic insights into the development of therapies for CNS disorders characterized by neuronal excitation-inhibition imbalance. Praxis is applying insights from genetic epilepsies to both rare and more prevalent neurological and psychiatric disorders, using our understanding of shared biological targets and circuits in the brain. Praxis has established a broad portfolio with multiple programs, including product candidates across psychiatric disorders, movement disorders and epilepsy, with three clinical-stage product candidates. For more information, please visit www.praxismedicines.com and follow us on 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 expectations, plans and timing for our clinical data, the anticipated timing of our clinical trials and regulatory filings, the development of our product candidates, including the design of our clinical trials and the treatment potential of our product candidates, and the sufficiency of our cash, cash equivalents and marketable securities, and 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; risks, uncertainties and assumptions regarding the impact of the continuing COVID-19 pandemic on Praxis' business, operations, strategy, goals and anticipated timelines, Praxis' ongoing and planned preclinical activities, Praxis' ability to initiate, enroll, conduct or complete ongoing and planned clinical trials and Praxis' timelines for regulatory submissions; and other risks concerning Praxis' programs and operations are described in additional detail in its Annual Report on Form 10-K for the year ended December 31, 2021, its Quarterly Reports on Form 10-Qand 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, 2022	December 31, 2021
Assets		
Cash and cash equivalents	$ 77,854	$ 138,704
Marketable securities	144,662	137,207
Prepaid expenses and other current assets	11,957	11,498
Property and equipment, net	1,142	1,213
Operating lease right-of-use assets	3,473	3,653
Other non-current assets	416	472
Total assets	$ 239,504	$ 292,747
Liabilities and stockholders' equity		
Accounts payable	$ 13,269	$ 10,780
Accrued expenses	30,929	26,844
Operating lease liabilities	4,284	4,311
Common stock	5	5
Additional paid-in capital	576,955	567,598
Accumulated other comprehensive loss	(606)	(176)
Accumulated deficit	(385,332)	(316,615)
Total liabilities and stockholders' equity	$ 239,504	$ 292,747

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

	Three Months Ended March 31,	
	2022	2021
Operating expenses:		
Research and development	$ 52,652	$ 17,929
General and administrative	16,197	9,490
Total operating expenses	68,849	27,419
Loss from operations	(68,849)	(27,419)
Other income:		
Other income, net	132	46
Total other income	132	46
Net loss	$ (68,717)	$ (27,373)
Net loss per share attributable to common stockholders, basic and diluted	$ (1.51)	$ (0.71)
Weighted average common shares outstanding, basic and diluted	45,455,179	38,470,710

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