Praxis Precision Medicines Provides Corporate Update and Reports Second Quarter 2021 Financial Results

August 16, 2021

Initiated PRAX-114 Phase 2 Acapella Study for adjunctive treatment of Major Depressive Disorder (MDD)

Enrollment on track for 1H22 topline results in PRAX-114 Phase 2/3 Aria Study for monotherapy treatment of MDD

PRAX-114 to advance in Phase 2b study in women with menopausal and mood symptoms

PRAX-944 Phase 2b study for treatment of essential tremor to initiate in 3Q21; Phase 2a topline results expected by the end of 2021

PRAX-562 Phase 1 healthy volunteer study completed with dose up to 150 mg and favorable safety profile

Cash and investments of $339.2 million as of June 30, 2021 supports runway into 2Q23

CAMBRIDGE, Mass., Aug. 16, 2021 (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 imbalance, today provided a corporate update and reported financial results for the second quarter ended June 30, 2021.

“The second quarter marked another inflection point for Praxis as we make progress toward being a diversified, late-stage, CNS focused company,” said Marcio Souza, president and chief executive officer of Praxis. “We expect to end the year with six controlled trials across our three clinical programs, including the ongoing registrational and Phase 2 studies in depression. As we know too well, the needs of people with CNS disorders are urgent and we are committed to bringing meaningful treatments to patients.”

Recent Business Highlights and Upcoming Milestones:

Psychiatry

- Praxis started dosing patients in the Acapella Study (Study 214), a PRAX-114 Phase 2 placebo-controlled study for adjunctive treatment of Major Depressive Disorder (MDD), in July 2021. Topline results from the Acapella Study are expected in the first half of 2022.
- Praxis expects topline results from the ongoing PRAX-114 Phase 2/3 Aria Study for monotherapy treatment of MDD in the first half of 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.
- The Company reported topline results from the PRAX-114 Phase 2a Part B proof-of-concept trial for treatment of perimenopausal depression (PMD) and consolidated safety data across all three cohorts of the now completed Phase 2a study. Praxis intends to continue investigation of PRAX-114 for treatment of women with menopausal and mood symptoms in a Phase 2b trial. Plans for the Phase 2b trial will be disclosed by the end of 2021.
    - In Part B, participants treated with a single daily dose of PRAX-114 60 mg suspension formulation (n=6) for 14 days showed improvements in menopausal and mood symptoms that were rapid, marked and maintained throughout the two-week treatment period. Results trended toward baseline following discontinuation of PRAX-114, suggesting the need for continued treatment. PRAX-114 was well tolerated, with no change in the overall safety profile.
- Praxis plans to initiate a PRAX-114 Phase 2 placebo-controlled study for treatment of post-traumatic stress disorder (PTSD) in the second half of 2021. Topline results are expected in the second half of 2022. The trial is designed to assess the efficacy of daily treatment of PRAX-114 in patients with PTSD for 4 weeks, using the CAPS-5 total score as the primary endpoint.

Movement Disorders

- The Company expects to initiate a PRAX-944 Phase 2b study for treatment of essential tremor (ET) in the US in the third quarter of 2021. Topline results are expected in the second half of 2022. The Phase 2b study is a randomized, double-blind, placebo-controlled, dose-range-finding clinical trial designed to assess the safety, tolerability and efficacy of

PRAX-944 at three dose levels up to 120 mg per day.

- Praxis is currently in the second of two cohorts of its PRAX-944 Phase 2a trial for treatment of ET, assessing patients titrated up to 120 mg/day of PRAX-944. Due to delays in recruitment in Australia, the company now expects preliminary topline open-label safety, tolerability and efficacy data by the end of 2021.

- Praxis plans to initiate a PRAX-114 Phase 2 single-dose crossover study for treatment of ET to assess safety, pharmacokinetics (PK) and efficacy in the fourth quarter of 2021. Topline results are expected in the second half of 2022.

- In the second quarter of 2021, the Company initiated a two-part PRAX-944 Phase 1 study to explore faster titration schemes for treatment of ET. Topline results are expected by the end of 2021. The Phase 1 study is intended to optimize the dosing regimen for PRAX-944 for treatment of ET and is designed to assess the safety, tolerability, and PK of titrating PRAX-944 up to 120 mg in a 10-day regimen in participants aged 18 to 54 years (Part A) and 55 to 75 years (Part B).

Rare Disease

- Praxis has completed the dosing and safety follow-up period for its single ascending dose (SAD) and multiple ascending dose (MAD) cohorts up to 150 mg and 120 mg, respectively, in its Phase 1 healthy volunteer study of PRAX-562. No dose-limiting toxicity has been observed. Dose-related changes were observed in the exploratory Auditory Steady-State Response (ASSR) electroencephalogram (EEG) translational marker, and exposures far exceeded those expected to be therapeutic in epilepsy-based animal models. Safety, tolerability, PK and preliminary biomarker data will be reported by the end of 2021. Based on the positive changes seen in the ASSR marker in this study, Praxis intends to initiate a dedicated PRAX-562 EEG study to validate the signal observed.

- The Company expects to initiate an exploratory Phase 2 trial of PRAX-562 in the fourth quarter of 2021 for treatment of patients with rare adult cephalgia, including a cohort of participants with Short-lasting Unilateral Neuralgiform headache attacks with Conjunctival injection and Tearing (SUNCT) and Short-lasting Unilateral Neuralgiform headache with Autonomic symptoms (SUNA), and a cohort of participants with Trigeminal Neuralgia (TN).

- Praxis plans to initiate a Phase 2 trial of PRAX-562 for treatment of developmental epileptic encephalopathies (DEEs), including SCN8A-DEE and SCN2A-DEE, in the first half of 2022.

- Praxis plans to complete the ongoing Investigational New Drug (IND) enabling toxicology study by the end of 2021 for its lead antisense oligonucleotide (ASO) candidate, PRAX-222, and to initiate a Phase 1/2 trial of PRAX-222 for treatment of SCN2A-DEE in the first half of 2022.

General Corporate Updates

- In June 2021, Praxis entered into a collaboration with Ligand to discover and develop novel therapies for neurological disorders using the Icagen Ion Channel Technology platform.

- In May 2021, Praxis completed a follow-on public offering of 5.75 million shares of common stock, including the exercise in full by the underwriters of their option to purchase up to 750,000 additional shares of common stock, at $18.25 per share, raising net proceeds of approximately $98.4 million after deducting underwriting discounts and commissions and other estimated offering expenses payable by Praxis.

- In May 2021, Praxis announced the appointment of Tim Kelly as chief financial officer. Mr. Kelly brings more than 20 years of experience in the life sciences, pharmaceutical and biotechnology industries. Most recently, Mr. Kelly was the chief financial officer and head of corporate management at Foundation Medicine.

Second Quarter 2021 Financial Results:

As of June 30, 2021, Praxis had $339.2 million in cash, cash equivalents and marketable securities, compared to $296.6 million in cash and cash equivalents as of December 31, 2020. This increase of $42.6 million primarily reflects the addition of $98.4 million in net proceeds from the follow-on public offering of shares of our common stock, partially offset by cash used in operations of $55.7 million in the first half of the year. The company's cash, cash equivalents and marketable securities as of June 30, 2021 are expected to fund operations into the second quarter of 2023.

Research and development expenses were $25.7 million for the three months ended June 30, 2021, compared to $9.1 million for the three months ended June 20, 2020. The increase in R&D expenses of $16.6 million was primarily attributable to $8.3 million in increased expenses related to our clinical-stage programs, $4.4 million in increased personnel-related costs due to increased headcount and $3.0 million in increased expenses related to our discovery-stage programs.

General and administrative expenses were $10.8 million for the three months ended June 30, 2021, compared to $2.5 million for the three months ended June 30, 2020. The increase in general and administrative expenses of $8.3 million was primarily attributable to $4.6 million in increased personnel-related costs due to increased headcount, $2.2 million in increased professional fees and a $1.5 million increase in other general and administrative expenses.

Praxis reported net loss of $36.4 million for the three months ended June 30, 2021, including $5.4 million of stock-based

compensation expense, compared to $11.6 million for the three months ended June 30, 2020, including $0.3 million of stock-based compensation expense.

As of June 30, 2021, Praxis had 44.7 million shares of common stock outstanding.

About Praxis
Praxis Precision Medicines is a clinical-stage biopharmaceutical company translating genetic insights into the development of therapies for central nervous system disorders (CNS) characterized by neuronal imbalance. Praxis is applying insights from genetic epilepsies to broader neurological and psychiatric disorders, using our understanding of shared biological targets and circuits in the brain. Praxis has established a broad portfolio, including multiple disclosed programs across CNS disorders including depression, epilepsy, movement disorders and pain syndromes, with three clinical-stage product candidates. For more information, please visit https://praxismedicines.com/ and follow us on LinkedIn and Twitter.

Forward-Looking Statements
This press release may contain 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 and plans for presenting pre-clinical and clinical data, projections regarding future revenues and financing performance, our long-term growth, cash, cash equivalents and marketable securities, the anticipated timing of our clinical trials and regulatory filings, the development of our product candidates and advancement of our preclinical programs, the timing, progress and success of our collaborations, 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 and in the availability and timing of data from ongoing clinical trials; whether interim results from a clinical trial will be predictive of the final results of the trial; whether results from preclinical studies or earlier clinical studies will be predictive of the results of future trials; the expected timing of submissions for regulatory approval or review by governmental authorities; regulatory approvals to conduct trials or to market products; whether Praxis' cash resources will be sufficient to fund Praxis' foreseeable and unforeseeable operating expenses and capital expenditure requirements; 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, Praxis' timelines for regulatory submissions and Praxis' financial position; 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, 2020, its Quarterly Reports on Form 10-Q and other subsequent filings made with the Securities and Exchange Commission from time to time. Although Praxis' forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts 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.

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PRAXIS PRECISION MEDICINES, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

(Amounts in thousands)

(Unaudited)

	June 30, 2021	December 31, 2020
Assets		
Cash and cash equivalents	$ 175,879	$ 296,608

Marketable securities	163,305	—
Prepaid expenses and other current assets	9,249	5,718
Property and equipment, net	182	82
Operating lease right-of-use assets	4,400	754
Other non-current assets	496	15
Total assets	$ 353,511	$ 303,177
Liabilities and stockholders' equity		
Accounts payable	$ 7,401	$ 4,088
Accrued expenses	7,826	10,869
Operating lease liabilities	4,512	763
Common stock	5	4
Additional paid-in capital	547,145	437,007
Accumulated other comprehensive loss	(50)	—
Accumulated deficit	(213,328)	(149,554)
Total liabilities and stockholders' equity	$ 353,511	$ 303,177

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,	
	2021	**2020**	**2021**	**2020**
Operating expenses:				
Research and development	$ 25,678	$ 9,050	$ 43,607	$ 15,918
General and administrative	10,805	2,520	20,295	4,121
Total operating expenses	36,483	11,570	63,902	20,039
Loss from operations	(36,483)	(11,570)	(63,902)	(20,039)
Other income:				
Other income, net	82	5	128	133
Total other income	82	5	128	133
Loss before benefit from income taxes	(36,401)	(11,565)	(63,774)	(19,906)
Benefit from (provision for) income taxes	—	(3)	—	8
Net loss	$ (36,401)	$ (11,568)	$ (63,774)	$ (19,898)
Accretion and cumulative dividends on redeemable convertible preferred stock	—	(2,039)	—	(4,103)
Gain on repurchase of redeemable convertible preferred stock	—	—	—	493
Net loss attributable to common stockholders	$ (36,401)	$ (13,607)	$ (63,774)	$ (23,508)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.88)	$ (8.28)	$ (1.59)	$ (14.37)
Weighted average common shares outstanding, basic and diluted	41,569,782	1,642,484	40,028,807	1,635,913