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

November 3, 2021

Enrollment on track for 1H22 topline results for PRAX-114 Phase 2/3 monotherapy MDD Aria Study and for PRAX-114 Phase 2 dose-ranging MDD Acapella Study

PRAX-114 Phase 2 studies in post-traumatic stress disorder and essential tremor (ET), PRAX-562 Phase 2 study in rare adult cephalgias expected to initiate in 4Q21

Preliminary results for PRAX-944 Phase 2a study for treatment of ET expected in 4Q21

First patient enrolled in PRAX-944 Phase 2b Essential1 Study for treatment of ET; topline results expected in 2H22

Cash and investments of $314.4 million as of September 30, 2021 supports runway into 2Q23

Praxis to host Movement Disorder Day on December 17, 2021

BOSTON, Nov. 03, 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 third quarter ended September 30, 2021.

“The advancements throughout our portfolio in recent months exemplify the potential of an unwavering team and an operational backbone that allows us to efficiently move a diverse set of programs forward in multiple CNS indications,” said Marcio Souza, president and chief executive officer of Praxis. “As we look back on the progress in our first year as a public company and enter a catalyst-rich period with data readouts across each of our three clinical programs, we remain as determined as ever to bring CNS drugs to those in need.”

Recent Business Highlights and Upcoming Milestones:

Psychiatry

- Praxis expects topline results from the ongoing PRAX-114 Phase 2/3, placebo-controlled Aria Study for monotherapy treatment of Major Depressive Disorder (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 expects topline results from the ongoing PRAX-114 Phase 2, placebo-controlled, dose-ranging Acapella Study in the first half 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, primarily in adjunctive MDD participants.

- Following the announcement of positive topline results from the PRAX-114 Phase 2a, proof-of-concept trial for treatment of perimenopausal depression, Praxis plans 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 in the fourth quarter of 2021.

- Praxis plans to initiate a PRAX-114 Phase 2, placebo-controlled study for treatment of post-traumatic stress disorder (PTSD) in the fourth quarter of 2021. Topline results are expected 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 4 weeks in approximately 80 participants with PTSD, using the CAPS-5 total score as the primary endpoint.

Movement Disorders

- The Company has completed dosing in a two-part PRAX-944 Phase 1 study to explore a faster titration regimen. Topline results are expected in the fourth quarter of 2021. The Phase 1 study is designed to evaluate the safety, tolerability and pharmacokinetics (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).

- Praxis is currently in the second of two cohorts of its PRAX-944 Phase 2a trial for treatment of essential tremor (ET), evaluating safety and efficacy in patients titrated up to 120 mg per day. Preliminary open-label safety, tolerability and efficacy data is expected in the fourth quarter of 2021, followed by complete open-label and placebo-controlled, randomized withdrawal results in the first half of 2022.

- Praxis initiated the PRAX-944 Phase 2b Essential1 Study for treatment of ET in the third quarter of 2021 and has started enrolling participants. Topline results are expected in the second half of 2022. Essential1 is a placebo-controlled, dose-ranging clinical trial designed to evaluate the safety, tolerability and efficacy of PRAX-944 at 20, 60 or 120 mg per day.

- Praxis plans to initiate a PRAX-114 Phase 2, placebo-controlled, crossover study for daytime treatment of ET to evaluate safety, PK and efficacy of 10 or 20 mg of PRAX-114 in the fourth quarter of 2021. Topline results are expected in the second half of 2022.

- The Company intends to initiate a Phase 2 trial to evaluate the safety, PK and efficacy of PRAX-944 as a non-dopaminergic treatment for the motor symptoms of Parkinson's disease in the first half of 2022.

- Praxis plans to host a Movement Disorder Day in New York City and virtually on Friday, December 17, 2021.

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 and 120 mg, respectively, in its PRAX-562 Phase 1, healthy volunteer study. A summary of the findings is included below and in the slide deck accompanying our third quarter 2021 corporate update in the Investors + Media section of our website.

    - PRAX-562 was well-tolerated, with no clinically significant safety findings. The most common treatment emergent adverse events (TEAEs) were headache and dizziness. Almost all TEAEs were mild in severity. No drug-related serious adverse events or severe adverse events were observed.

    - The maximum observed concentration was observed between 2 and 3 hours after dosing. There was no impact of food intake on PK. The half-life of PRAX-562 is approximately 4-5 days across the dose range evaluated.

    - Dose-related changes were observed in the exploratory Auditory Steady-State Response (ASSR) electroencephalogram (EEG) translational biomarker. In the 120 mg dose group, a reduction in ASSR of greater than 50% was observed after 14 days QD as compared to baseline.

- Based on the observed signal in the ASSR marker in the Phase 1, healthy volunteer study, Praxis has started dosing patients in the U.S. in a PRAX-562 Phase 1, placebo-controlled, two-cohort EEG study to validate the observed signal. Topline data is expected in the first half of 2022. The study is intended to evaluate ASSR as a biomarker for the PRAX-562 program to further support selection of therapeutic dose levels in Phase 2 studies.

- The Company intends to initiate a PRAX-562 Phase 2 trial in the fourth quarter of 2021 in the U.S. for treatment of patients with rare adult cephalgias, 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 PRAX-562 Phase 2 trial for treatment of developmental epileptic encephalopathies (DEEs) in the first half of 2022.

- Praxis has completed the Investigational New Drug (IND) enabling toxicology study for its lead antisense oligonucleotide (ASO) candidate, PRAX-222, and plans to initiate regulatory submissions in order to begin a PRAX-222 Phase 1/2 trial for treatment of SCN2A-DEE in the first half of 2022.

Third Quarter 2021 Financial Results:

As of September 30, 2021, Praxis had $314.4 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 $17.8 million primarily reflects $98.4 million in net proceeds from the follow-on public offering of shares of our common stock in May 2021, partially offset by cash used in operations of $79.7 million during the nine months ended September 30, 2021. The company's cash, cash equivalents and marketable securities as of September 30, 2021 are expected to fund operations into the second quarter of 2023.

Research and development expenses were $33.1 million for the three months ended September 30, 2021, compared to $12.8 million for the three months ended September 30, 2020. The increase in R&D expenses of approximately $20.4 million was primarily attributable to $10.9 million in increased expenses related to our clinical-stage programs, $5.2 million in increased personnel-related costs due to increased headcount and $2.9 million in increased expenses related to our discovery-stage programs.

General and administrative expenses were $11.6 million for the three months ended September 30, 2021, compared to $3.4 million for the three months ended September 30, 2020. The increase in general and administrative expenses of $8.2 million was primarily attributable to $4.7 million in increased personnel-related costs due to increased headcount, $1.8 million in increased professional fees and a $1.7 million increase in other general and administrative expenses.

Praxis reported net loss of $44.7 million for the three months ended September 30, 2021, including $6.5 million of stock-based compensation expense, compared to a net loss of $16.2 million for the three months ended September 30, 2020, including $1.0 million of stock-based compensation expense.

As of September 30, 2021, Praxis had 44.8 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, plans and timing for clinical data, the sufficiency of our cash, cash equivalents and marketable securities, the anticipated timing of our clinical trials and regulatory filings, and the development of our product candidates, including the design of our clinical trials and the treatment potential 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 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.

PRAXIS PRECISION MEDICINES, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(Amounts in thousands)
(Unaudited)

	September 30, 2021	December 31, 2020
Assets		
Cash and cash equivalents	$ 165,679	$ 296,608
Marketable securities	148,691	—
Prepaid expenses and other current assets	4,969	5,718
Property and equipment, net	625	82
Operating lease right-of-use assets	4,028	754
Other non-current assets	416	15
Total assets	$ 324,408	$ 303,177
Liabilities and stockholders' equity		
Accounts payable	$ 7,544	$ 4,088
Accrued expenses	16,529	10,869
Operating lease liabilities	4,413	763
Common stock	5	4
Additional paid-in capital	553,975	437,007
Accumulated other comprehensive loss	(25)	—

Accumulated deficit	(258,033)	(149,554)
Total liabilities and stockholders' equity	$ 324,408	$ 303,177

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,	
	2021	**2020**	**2021**	**2020**
Operating expenses:				
Research and development	$ 33,139	$ 12,786	$ 76,746	$ 28,704
General and administrative	11,634	3,431	31,929	7,552
Total operating expenses	44,773	16,217	108,675	36,256
Loss from operations	(44,773)	(16,217)	(108,675)	(36,256)
Other income:				
Other income, net	73	1	201	134
Total other income	73	1	201	134
Loss before benefit from income taxes	(44,700)	(16,216)	(108,474)	(36,122)
Benefit from (provision for) income taxes	(5)	—	(5)	8
Net loss	$ (44,705)	$ (16,216)	$ (108,479)	$ (36,114)
Accretion and cumulative dividends on redeemable convertible preferred stock	—	(3,943)	—	(8,046)
Gain on repurchase of redeemable convertible preferred stock	—	—	—	493
Net loss attributable to common stockholders	$ (44,705)	$ (20,159)	$ (108,479)	$ (43,667)
Net loss per share attributable to common stockholders, basic and diluted	$ (1.00)	$ (12.10)	$ (2.61)	$ (26.53)
Weighted average common shares outstanding, basic and diluted	44,714,941	1,665,902	41,608,017	1,645,982

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