Praxis Precision Medicines Provides Corporate Update and Reports Fourth Quarter and Full Year 2021 Financial Results

February 28, 2022

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

PRAX-944 Phase 2a ET topline results expected in May 2022; to include open-label and placebo-controlled withdrawal data

PRAX-222 seamless study in SCN2A-DEE expected to initiate in 2Q22

Cash and investments of $275.9 million as of December 31, 2021 supports runway into 2Q23

BOSTON, Feb. 28, 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 fourth quarter and full year 2021.

"With screening now closed in all sites in the PRAX-114 Aria Study, we are emboldened by the progress in our psychiatry franchise, and broadly across our pipeline," said Marcio Souza, president and chief executive officer of Praxis. "There is a clear unmet need for a fast-acting, durable depression treatment with a differentiated tolerability profile relative to both existing treatment options and other drugs in development, which allows patients to remain on drug throughout an episode of depression. We look forward to reporting topline results from the Aria Study in June, followed shortly thereafter by results from the Acapella Study, and intend to initiate a Phase 3 trial in MDD later this year. With additional topline data expected from PRAX-114 studies for PTSD and ET, as well as from PRAX-944 for ET, 2022 is shaping up to be a transformative year for Praxis and we are eager to share our continued progress and growth."

Recent Business Highlights and Upcoming Milestones:

Psychiatry

- Praxis expects topline results from the PRAX-114 Phase 2/3, placebo-controlled Aria Study for monotherapy treatment of Major Depressive Disorder (MDD) in the second quarter of 2022, in June. 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 PRAX-114 Phase 2, placebo-controlled, dose-ranging Acapella Study for treatment of MDD in mid-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 initiated a PRAX-114 Phase 2, placebo-controlled study for treatment of post-traumatic stress disorder (PTSD) in the fourth quarter of 2021 and has started dosing participants. 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

- In December 2021, Praxis reported preliminary open-label data from the second of two cohorts of its PRAX-944 Phase 2a trial for daytime treatment of essential tremor (ET), evaluating safety and efficacy in participants titrated up to 120 mg per day. Enrollment of study participants was subsequently completed. Topline open-label and placebo-controlled, randomized withdrawal results are expected in the second quarter of 2022, in May.

- The Company expects topline results from the PRAX-944 Phase 2b Essential1 Study for daytime treatment of ET 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 100 mg per day.

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

- Praxis intends to initiate a Phase 2, placebo-controlled 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 second quarter of 2022.

Epilepsy

- Praxis plans to initiate a PRAX-562 Phase 2, placebo-controlled trial for treatment of developmental epileptic encephalopathies (DEEs) in the second quarter of 2022.
- Praxis intends to initiate a seamless study of PRAX-222, its lead antisense oligonucleotide (ASO) candidate, for the treatment of SCN2A-DEE in the second quarter of 2022.
- In January 2022, the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) granted Orphan Drug Designation (ODD) to PRAX-222 for the treatment of SCN2A-DEE. Previously, in January 2021, the FDA granted both ODD and Rare Pediatric Disease (RPD) designation to PRAX-222 for the treatment of SCN2A-DEE.
- In December 2021, the EMA COMP granted ODD to PRAX-562 for the treatment of SCN8A-DEE and SCN2A-DEE. Previously, in January 2021, the FDA granted both ODD and RPD designation to PRAX-562 for the treatment of SCN8A-DEE and SCN2A-DEE.
- In December 2021, Praxis presented data from two of its rare epilepsy programs, PRAX-562 and its KCNT1 inhibitor, at the American Epilepsy Society 2021 Annual Meeting. Presentations on PRAX-562 focused on its potent anticonvulsant activity in SCN2A-DEE and SCN8A-DEE mouse models and its mechanistic distinction relative to standard-of-care sodium channel inhibitors, with greater potency and selectivity for persistent sodium current. The presentation on KCNT1 focused on the compound's in vitro and in vivo profiling, including its efficacy in a KCNT1 gain-of-function mouse model.
- In December 2021, Praxis entered into a research collaboration with Cerebral Therapeutics, Inc., with an exclusive option to in-license delivery technology for intracerebroventricular administration of its ASOs.
- Praxis intends to develop PRAX-628, a small molecule with unique NaV channel binding kinetics that favor inhibition of pathological neuronal activity underlying aberrant brain function, such as that seen in the initial indication of focal onset seizures. The Company anticipates use in other common forms of epilepsy and CNS excitability disorders more generally. PRAX-628 is currently in IND-enabling toxicology studies.

Other Exploratory CNS Indications

- Praxis plans to initiate a PRAX-562 Phase 2, placebo-controlled trial for treatment of rare adult cephalgias in the first quarter of 2022, 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).

General Corporate Updates

- In December 2021, Praxis announced the appointment of Megan Sniecinski as chief business officer, the promotions of Alyssa Wyant to chief regulatory and quality officer and Karl Hansen, Ph.D., to chief technical operations officer, and the decision by chief scientific officer and co-founder, Steven Petrou, Ph.D., to fully dedicate his time to Praxis upon stepping down from his role as Director of the Florey Institute of Neuroscience and Mental Health and Head of the Florey Department at The University of Melbourne.

Fourth Quarter and Full Year 2021 Financial Results:

As of December 31, 2021, Praxis had $275.9 million in cash, cash equivalents and marketable securities, compared to $296.6 million in cash and cash equivalents as of December 31, 2020. This decrease of $20.7 million primarily reflects cash used in operations of $124.6 million during the year ended December 31, 2021, partially offset by $105.7 million in net proceeds from the follow-on public offering of shares of the Company's common stock in May 2021 and at-the-market offerings during the fourth quarter of 2021. The company's cash, cash equivalents and marketable securities as of December 31, 2021 are expected to fund operations into the second quarter of 2023.

Research and development expenses were $43.5 million for the fourth quarter of 2021, compared to $16.3 million for the fourth quarter of 2020. Research and development expenses were $120.3 million for the year ended December 31, 2021, compared to $45.0 million for the year ended December 31, 2020. The increase in research and development expenses for full year 2021 of $75.3 million was primarily attributable to $43.6 million in increased expenses related to the Company's franchises, $17.6 million in increased personnel-related costs due to increased headcount and $8.8 million in increased expenses for other exploratory CNS indications.

General and administrative expenses were $15.1 million for the fourth quarter of 2021, compared to $9.4 million for the fourth quarter of 2020. General and administrative expenses were $47.1 million for the year ended December 31, 2021, compared to $17.0 million for the year ended December 31, 2020. The increase in general and administrative expenses for full year 2021 of $30.1 million was primarily attributable to $14.6 million in increased personnel-related costs due to increased headcount, $9.9 million in increased professional fees and a $5.6 million increase in other general and administrative expenses.

Praxis reported a net loss of $58.6 million for the fourth quarter of 2021, including $6.1 million of stock-based compensation

expense, compared to $25.7 million for the fourth quarter of 2020, including $3.8 million of stock-based compensation expense. Praxis reported a net loss of $167.1 million for the year ended December 31, 2021, including $22.7 million of stock-based compensation expense, compared to a net loss of $61.8 million for the year ended December 31, 2020, including $5.2 million of stock-based compensation expense.

As of December 31, 2021, Praxis had 45.3 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 fourth quarter and full year 2021 via a conference call and webcast today, February 28, 2022, at 8:30 a.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 8993704. 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, epilepsy and other exploratory CNS indications, 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 to be filed 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)

	December 31,	
	2021	**2020**
Assets		
Cash and cash equivalents	$ 138,704	$ 296,608
Marketable securities	137,207	—
Prepaid expenses and other current assets	11,498	5,718
Property and equipment, net	1,213	82
Operating lease right-of-use assets	3,653	754
Other non-current assets	472	15
Total assets	$ 292,747	$ 303,177

Liabilities and stockholders' equity		
Accounts payable	$ 10,780	$ 4,088
Accrued expenses	26,844	10,869
Operating lease liabilities	4,311	763
Common stock	5	4
Additional paid-in capital	567,598	437,007
Accumulated other comprehensive loss	(176)	—
Accumulated deficit	(316,615)	(149,554)
Total liabilities and stockholders' equity	$ 292,747	$ 303,177

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

	Three Months Ended December 31,		Year Ended December 31,	
	2021	**2020**	**2021**	**2020**
Operating expenses:				
Research and development	$ 43,511	$ 16,272	$ 120,257	$ 44,976
General and administrative	15,146	9,440	47,075	16,992
Total operating expenses	58,657	25,712	167,332	61,968
Loss from operations	(58,657)	(25,712)	(167,332)	(61,968)
Other income:				
Other income, net	70	6	271	140
Total other income	70	6	271	140
Loss before benefit from income taxes	(58,587)	(25,706)	(167,061)	(61,828)
Benefit from income taxes	5	—	—	8
Net loss	$ (58,582)	$ (25,706)	$ (167,061)	$ (61,820)
Accretion and cumulative dividends on redeemable convertible preferred stock	—	(950)	—	(8,996)
Gain on repurchase of redeemable convertible preferred stock	—	—	—	493
Net loss attributable to common stockholders	$ (58,582)	$ (26,656)	$ (167,061)	$ (70,323)
Net loss per share attributable to common stockholders, basic and diluted	$ (1.30)	$ (0.87)	$ (3.94)	$ (7.86)
Weighted average common shares outstanding, basic and diluted	44,964,580	30,703,886	42,454,055	8,950,152

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