Praxis Precision Medicines Provides Corporate Update and Reports Fourth Quarter and Full Year 2022 Financial Results

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Ulixacaltamide (PRAX-944) Phase 2b Essential1 study topline results for essential tremor expected in 1Q23; Praxis to enter quiet period following market close on Thursday, February 9

Topline results expected for each of three clinical-stage epilepsy programs in 2023 – PRAX-222 first-in-patient EMBRAVE Study safety data mid-2023, PRAX-628 first-in-human Phase 1 data mid-2023, PRAX-562 Phase 2 EMBOLD Study results in 2H23

Cash and investments of $100.5 million as of December 31, 2022 supports runway into 1Q24

BOSTON, Feb. 07, 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 fourth quarter and full year 2022.

“This year is set up to be transformative for Praxis and for the patients that we serve, with topline results for the ulixacaltamide Essential1 study imminent and data expected from each of our four clinical-stage programs in 2023,” said Marcio Souza, president and chief executive officer of Praxis. “Based on our understanding of epilepsy genetics and unique capabilities to translate these insights into therapies for patients suffering from a broad range of CNS disorders, we have built two proprietary platforms, Cerebrum™ for small molecules and Solidus™ for antisense oligonucleotides, that we expect will drive continuous innovation and value creation this year and beyond.”

Recent Business Highlights and Upcoming Milestones:

Cerebrum™ Small Molecule Platform

- Praxis expects topline results from the ongoing ulixacaltamide (PRAX-944) Essential1 study for the treatment of moderate to severe essential tremor (ET) in the first quarter of 2023. Essential1 is a randomized, double-blind, placebo-controlled, dose-range-finding Phase 2b trial evaluating the efficacy, safety and tolerability of once-daily daytime treatment of 60 or 100 mg of ulixacaltamide compared to placebo after 56 days. The primary endpoint is change from baseline to day 56 in the modified Activities of Daily Living (mADL[1]) score. Following topline results, Praxis intends to request an end-of-Phase 2 meeting with the FDA and initiate its ulixacaltamide Phase 3 development program for the treatment of ET in mid-2023.
- The Company is also conducting a randomized, double-blind, placebo-controlled Phase 2 trial to evaluate the efficacy, safety and tolerability of once-daily treatment of up to 100 mg of ulixacaltamide as a non-dopaminergic treatment for the motor symptoms of Parkinson’s disease. The primary endpoint for the study is change from baseline in the International Parkinson and Movement Disorder Society Unified Parkinson’s Disease Rating Scale (UPDRS), Part III (motor examination) score in the OFF state. Topline results are expected in the fourth quarter of 2023.
- In November 2022, Praxis announced plans to initiate the PRAX-562 Phase 2 EMBOLD study for the treatment of pediatric patients with developmental and epileptic encephalopathies (DEEs), following FDA authorization to proceed with the study as Praxis proposed, up to the planned maximum dose of 1.0 mg/kg/day. The EMBOLD study is a randomized, double-blind, placebo-controlled Phase 2 clinical trial to evaluate the safety, tolerability, efficacy (motor seizure frequency) and pharmacokinetics (PK) of PRAX-562 in pediatric participants aged 2 to 18 years with DEEs, followed by an open-label extension. Approximately 20 participants will be enrolled initially in two distinct cohorts (n≈10 for SCN2A-DEE and n≈10 for SCN8A-DEE). Topline results for both cohorts are expected in the second half of 2023.
- The Company is conducting a Phase 1 healthy volunteer study of PRAX-628 to evaluate the tolerability, PK, pharmacodynamics and food effect of PRAX-628 across single and multiple ascending dose cohorts. Topline results from the Phase 1 study are expected in mid-2023, with plans to initiate a Phase 2 study in focal epilepsy in the fourth quarter of 2023.
- In December 2022, Praxis announced that it entered into a strategic collaboration, based upon its PRAX-020 program, with UCB for the discovery of small molecule therapeutics as potential treatments for KCNT1 epilepsies. Under the terms of the collaboration, UCB retains an exclusive option to in-license global development and commercialization rights to any resulting KCNT1 small molecule development candidate. Praxis received an upfront payment from UCB, and if the option is exercised by UCB, would be eligible to receive an option fee and future success-based development and

commercialization milestone payments, for a total of up to approximately $100 million, in addition to tiered royalties on net sales of any resulting products from the collaboration.

- In December 2022, Praxis presented the following posters at the American Epilepsy Society (AES) 2022 Annual Meeting:
  - PRAX-562 is a Well-tolerated, Novel Persistent Sodium Channel Blocker with Broad Anticonvulsant Activity in Multiple DEE Mouse Models (Abstract Number: 1.281)
  - A Phase 1 Trial Evaluating the Safety, Tolerability, Pharmacokinetics and Food Effect of PRAX-562 in Healthy Volunteers (Abstract Number: 2.24)
  - A Phase 1 Trial Evaluating the Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of PRAX-562 in Healthy Volunteers (Abstract Number: 2.478)
  - Disease Impact and Burden in Patients with SCN2A-Related Developmental and Epileptic Encephalopathy (Abstract Number: 2.092)
  - A Novel Approach to Assess the Impact of Disease in Patients with SCN8A-Related Developmental and Epileptic Encephalopathy (Abstract Number: 2.096)
  - PRAX-628: A Novel Sodium Channel Blocker with Greater Potency and Activity Dependence Compared to Standard of Care (Abstract Number: 3.311)
  - PRAX-628 is a Novel, Well-tolerated, Activity Dependent Sodium Channel Blocker with Potent Anticonvulsant Activity (Abstract Number: 3.28)

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 the initial cohort of patients in 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 from Part 1 of the PRAX-222 EMBRAVE study are expected in mid-2023.
- The Company remains on track to nominate a development candidate for its most advanced preclinical ASO program, PRAX-080 for the treatment of PCDH19, in the second half of 2023.

Fourth Quarter and Full Year 2022 Financial Results:

As of December 31, 2022, Praxis had $100.5 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 $175.4 million primarily reflects cash used in operations of $185.0 million during the year ended December 31, 2022, partially offset by $9.6 million in net proceeds from at-the-market offerings of shares of the Company's common stock. The Company's cash, cash equivalents and marketable securities as of December 31, 2022 are expected to fund operations into the first quarter of 2024.

Research and development expenses were $28.3 million for the fourth quarter of 2022, compared to $43.5 million for the fourth quarter of 2021. Research and development expenses were $155.0 million for the year ended December 31, 2022, compared to $120.3 million for the year ended December 31, 2021. The increase in research and development expenses for full year 2022 of $34.7 million was primarily attributable to $29.9 million in increased expenses related to the Company's Cerebrum™ and Solidus™ platforms.

General and administrative expenses were $13.1 million for the fourth quarter of 2022, compared to $15.1 million for the fourth quarter of 2021. General and administrative expenses were $59.9 million for the year ended December 31, 2022, compared to $47.1 million for the year ended December 31, 2021. The increase in general and administrative expenses for full year 2022 of $12.8 million was primarily attributable to an increase of $11.8 million in personnel-related expenses due to changes in headcount, including an increase of $5.3 million in stock-based compensation expense.

Praxis reported a net loss of $41.2 million for the fourth quarter of 2022, including $6.4 million of stock-based compensation expense, compared to $58.6 million for the fourth quarter of 2021, including $6.1 million of stock-based compensation expense. Praxis reported a net loss of $214.0 million for the year ended December 31, 2022, including $28.6 million of stock-based compensation expense, compared to a net loss of $167.1 million for the year ended December 31, 2021, including $22.7 million of stock-based compensation expense.

As of December 31, 2022, Praxis had 49.4 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 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 interactions, 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, 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 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,	
	2022	2021
Assets		
Cash and cash equivalents	$ 61,615	$ 138,704
Marketable securities	38,874	137,207
Prepaid expenses and other current assets	10,351	11,498
Property and equipment, net	971	1,213
Operating lease right-of-use assets	2,901	3,653
Other non-current assets	416	472
Total assets	$ 115,128	$ 292,747
Liabilities and stockholders' equity		
Accounts payable	$ 14,672	$ 10,780
Accrued expenses	15,850	26,844
Operating lease liabilities	3,500	4,311
Deferred revenue	5,000	—
Common stock	5	5
Additional paid-in capital	606,918	567,598
Accumulated other comprehensive loss	(173)	(176)
Accumulated deficit	(530,644)	(316,615)
Total liabilities and stockholders' equity	$ 115,128	$ 292,747

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,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	$ 28,329	$ 43,511	$ 155,040	$ 120,257
General and administrative	13,124	15,146	59,946	47,075
Total operating expenses	41,453	58,657	214,986	167,332
Loss from operations	(41,453)	(58,657)	(214,986)	(167,332)
Other income:				
Other income, net	280	70	957	271
Total other income	280	70	957	271

Loss before benefit from income taxes	(41,173)	(58,587)	(214,029)	(167,061)
Benefit from income taxes	—	5	—	—
Net loss	$ (41,173)	$ (58,582)	$ (214,029)	$ (167,061)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.87)	$ (1.30)	$ (4.64)	$ (3.94)
Weighted average common shares outstanding, basic and diluted	47,594,823	44,964,580	46,096,737	42,454,055

[1]mADL is a composite sum of items 1 to 11 of the TETRAS-ADL subscale and items 6 (bilateral) and 7 of the TETRAS-PS; mADL score is calculated as the sum of all 13 items (item 6 of TETRAS-PS x2) and ranges from 0 to 42

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