Praxis Precision Medicines Provides Corporate Update and Reports First Quarter 2024 Financial Results

May 13, 2024

Plans to initiate two efficacy studies investigating PRAX-628 in focal onset seizures in the second half of 2024 and first half of 2025, respectively

Continued strong recruitment for the pivotal Essential3 trials in Essential Tremor (ET), with over 50,000 pre-screening forms and over 7,000 referrals received to date that meet pre-qualifying eligibility criteria; topline results expected in the second half of 2024

Randomization in PRAX-562 Phase 2 EMBOLD study completed in pediatric patients with developmental and epileptic encephalopathies (DEEs), with topline results expected in the third quarter of 2024

Completed two underwritten public offerings in 2024; Cash and investments of $451.2 million as of April 30, 2024 extends runway into 2027

BOSTON, Mass., May 13, 2024 —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 first quarter 2024.

"In the first quarter we made significant progress on all fronts. We strengthened our financial resources and reported positive photo paroxysmal response (PPR) results in PRAX-628, which we believe further de-risks our clinical development program and positions PRAX-628 as the first precision sodium channel modulator for focal onset seizures. The strength and consistency observed across both study arms, combined with a favorable tolerability and safety profile, builds confidence in our planned focal onset seizure studies for PRAX-628, with the first expected to initiate in the second half of 2024," said Marcio Souza, president and chief executive officer of Praxis. "Looking ahead, we are encouraged by the strong participation we are seeing in our Essential3 Phase 3 studies and remain on track to read out topline results in the second half of this year. These advancements, through patient-guided development strategies, will further position Praxis at the forefront of precision medicine for CNS disorders."

Recent Highlights and Anticipated Milestones:

Cerebrum™ Small Molecule Platform

- **Ulixacaltamide for ET**: Essential3, the Phase 3 program for ulixacaltamide, continues to progress through patient qualification, enrollment and trial execution, with topline results expected in the second half of 2024 to support a planned New Drug Application (NDA) submission in 2025.
    - Essential3 is comprised of two simultaneous Phase 3 studies including a 12-week, parallel design study and a 12-week randomized withdrawal study for stable responders.
    - Since beginning recruitment in November 2023, there have been over 50,000 pre-screening forms and over 7,000 referrals received to date meeting pre-qualifying eligibility criteria. Randomization pace is now dictated by appropriate patient selection and pre-randomization stability and severity criteria.
    - At the American Academy of Neurology 2024 Annual Meeting, Praxis shared additional posters on the Essential3 study design [link] and the benefits of ulixacaltamide over propranolol as analyzed from the Essential1 study [link].

- **PRAX-628 for Focal Onset Seizures:** Praxis reported positive results, with 100% of patients responding in a Phase 2a proof of concept study evaluating PRAX-628 in epilepsy patients with PPR and confirmed plans to initiate two efficacy studies in focal onset seizures.
    - The PPR study had two cohorts, where all patients responded.
        - In the 15 mg cohort (n=5), 80% of patients achieved a complete response and 20% achieved a partial response.
        - In the 45 mg cohort (n=3), 100% of patients achieved a complete response.
        - Safety was consistent with the prior dose escalation study and pharmacokinetic analysis confirmed therapeutic exposures.
    - Praxis plans to initiate two efficacy studies in focal onset seizures.
        - The first study is expected to begin in the second half of 2024, with topline results expected in 2025.
        - The second study is expected to initiate in the first half of 2025, with topline results expected in the first half of 2026.

- **PRAX-562 for SCN2A and SCN8A DEEs:** Praxis expects topline results from the PRAX-562 Phase 2 EMBOLD study for the treatment of pediatric patients with DEEs in the third quarter of 2024.

- The EMBOLD study is a randomized, double-blind, placebo-controlled Phase 2 clinical study to evaluate the safety, tolerability, efficacy (motor seizure frequency) and pharmacokinetics of PRAX-562 in pediatric patients aged 2 to 18 years with DEEs, followed by an open-label extension.

Solidus™ Antisense Oligonucleotide (ASO) Platform

- **Elsunersen (PRAX-222) for SCN2A Gain-of-Function (GoF) DEEs:** Praxis is completing multiple global regulatory interactions in the first half of 2024 in anticipation of starting the pivotal phase of the program later in 2024.

Corporate Update:

- In January 2024, Praxis completed an underwritten public offering with net proceeds of approximately $161.6 million.
- In January 2024, Praxis announced a licensing partnership with Tenacia Biotechnology to develop and commercialize ulixacaltamide for the treatment of ET in Greater China, including mainland China, Hong Kong, Macau and Taiwan, with total potential consideration of over $275 million.
- In April 2024, Praxis completed an additional underwritten public offering with net proceeds of approximately $215.8 million.
- As of April 30, 2024, Praxis had cash, cash equivalents, and marketable securities of $451.2 million.

First Quarter 2024 Financial Results:

As of March 31, 2024, Praxis had $243.3 million in cash, cash equivalents and marketable securities. Current cash, cash equivalents and marketable securities are expected to fund operations into 2027.

Research and development expenses were $27.0 million for the three months ended March 31, 2024, compared to $25.5 million for the three months ended March 31, 2023. The increase in research and development expenses of $1.5 million was primarily attributable to $2.8 million in increased personnel expenses and $1.7 million in increased expenses related to the Company's Cerebrum™ platform, partially offset by $2.4 million in decreased expenses related to the Company's Solidus™ platform.

General and administrative expenses were $15.3 million for the three months ended March 31, 2024, compared to $13.3 million for the three months ended March 31, 2023. The increase in general and administrative expenses of approximately $2.0 million was primarily due to an increase in personnel-related costs, partially offset by a decrease in consulting and professional fees.

Praxis reported a net loss of $39.6 million for the three months ended March 31, 2024, including $14.5 million of stock-based compensation expense, compared to $37.5 million for the three months ended March 31, 2023, including $7.6 million of stock-based compensation expense.

As of March 31, 2024, Praxis had 13.3 million shares of common stock outstanding.

About Ulixacaltamide

Ulixacaltamide is a differentiated and highly selective small molecule inhibitor of T-type calcium channels designed to block abnormal neuronal burst firing in the Cerebello-Thalamo-Cortical (CTC) circuit correlated with tremor activity. Ulixacaltamide, the most advanced program within Praxis' Cerebrum™ small molecule platform, is currently in late-stage development for the treatment of essential tremor, www.praxisessentialtremor.com.

About PRAX-628

PRAX-628 is a next-generation, functionally selective small molecule targeting the hyperexcitable state of voltage-gated sodium (NaV) channels in the brain that is currently being developed as a once daily, oral treatment for adult focal onset seizures. Preclinical data demonstrates PRAX-628 is differentiated from standard of care, with the potential to be best-in-class for focal onset seizures. In vitro, PRAX-628 has demonstrated superior selectivity for disease-state NaV channel hyperexcitability. In vivo studies of PRAX-628 have demonstrated unprecedented potency in the maximal electroshock seizure (MES) model, a highly predictive translational model for efficacy in focal onset seizures. Data from the PRAX-628-101 study demonstrated that PRAX-628 can be safely dosed in healthy subjects to greater than 15 times the predicted human equivalent of the rodent MES EC50.

About Elsunersen

Elsunersen is an ASO designed to selectively decrease SCN2A gene expression, directly targeting the underlying cause of early onset SCN2A-DEE to treat seizures and other symptoms in patients with GoF SCN2A mutations. In vitro studies of elsunersen have demonstrated reduction in both SCN2A gene expression and protein levels. In vivo, elsunersen has demonstrated significant, dose-dependent reduction in seizures, improvement in behavioral and locomotor activity and increased survival in SCN2A mouse models, with potential to be the first disease-modifying treatment for SCN2A-DEE. Elsunersen has received Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation (RPD) from the FDA, and ODD and PRIME designations from the European Medicines Agency (EMA) for the treatment of SCN2A-DEE. The elsunersen program is ongoing under a collaboration with Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), and RogCon, Inc. To learn more about the EMBRAVE study, please visit https://www.embravestudy.org/.

About PRAX-562

PRAX-562 is a first-in-class small molecule in development for the treatment of DEEs as a preferential inhibitor of persistent sodium current, shown to be a key driver of seizure symptoms in early onset SCN2A-DEE and SCN8A-DEE. PRAX-562's mechanism of NaV channel modulation is consistent with superior selectivity for disease-state NaV channel hyperexcitability. In vivo studies of PRAX-562 have demonstrated dose-dependent inhibition of seizures up to complete control of seizure activity in SCN2A, SCN8A and other DEE mouse models. PRAX-562 has been generally well-tolerated in three Phase 1 studies and has demonstrated biomarker changes indicative of NaV modulation. PRAX-562 has received ODD and RPD from the FDA, and ODD from the European Medicines Agency for the treatment of SCN2A-DEE and SCN8A-DEE. To learn more about the EMBOLD study, please visit https://www.emboldstudy.org/.

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 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 Facebook, LinkedIn and Twitter/X.

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 the anticipated timing of our clinical trials, the development of our product candidates, the anticipated timing of regulatory submissions and interactions and our projected cash runway, 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 clinical trials, data readouts and the results thereof, and submissions for regulatory approval or review by governmental authorities; regulatory approvals to conduct trials; and other risks concerning Praxis' programs and operations as described in its Annual Report on Form 10-K for the year ended December 31, 2023 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.

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

	March 31, 2024	December 31, 2023
Assets		
Cash and cash equivalents	$ 151,980	$ 81,300
Marketable securities	91,309	—
Prepaid expenses and other current assets	4,388	3,580
Property and equipment, net	476	588

Operating lease right-of-use assets	1,840	2,064
Other non-current assets	416	416
Total assets	$ 250,409	$ 87,948
Liabilities and stockholders' equity		
Accounts payable	$ 9,404	$ 5,815
Accrued expenses	6,976	7,416
Operating lease liabilities	2,224	2,495
Deferred revenue	2,122	2,553
Common stock	13	13
Additional paid-in capital	923,141	723,577
Accumulated other comprehensive loss	3	—
Accumulated deficit	(693,474)	(653,921)
Total liabilities and stockholders' equity	$ 250,409	$ 87,948

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

	Three Months Ended March 31,	
	2024	**2023**
Collaboration revenue	$ 431	$ 683
Operating expenses:		
Research and development	26,984	25,504
General and administrative	15,333	13,270
Total operating expenses	42,317	38,774
Loss from operations	(41,886)	(38,091)
Other income:		
Other income, net	2,333	636
Total other income	2,333	636
Net loss	$ (39,553)	$ (37,455)
Net loss per share attributable to common stockholders, basic and diluted	$ (2.84)	$ (10.58)
Weighted average common shares outstanding, basic and diluted	13,904,374	3,540,185

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