Praxis Precision Medicines Announces Positive Results of EEG Analysis of PRAX-628 Phase 1 Study

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Composite qEEG measure showed change from placebo after first dose of 5 mg

Effect in the composite qEEG measured through 10-day treatment period

Ongoing PRAX-628 Phase 2 Photo-Paroxysmal Response (PPR) expected to read out in 2H 2023

BOSTON, Aug. 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 announced the results from an analysis of EEG activity for subjects in the recent Phase 1 study that demonstrated pharmacodynamic activity across all dose levels for study subjects who received PRAX-628 at first administration as compared with subjects who received placebo.

PRAX-628 is a next generation functionally selective small molecule targeting the hyperexcitable state of sodium channels in the brain, which is currently being developed as a rapid acting, once daily, oral treatment for adult focal onset epilepsy.

"We are very pleased to see that PRAX-628 reached the brain at therapeutic levels. Combined with the PRAX-628 data we have already seen, including its solid safety results, rapid onset and wide therapeutic window, we maintain our expectations that PRAX-628 has the potential to be the best-in-class treatment for patients suffering from focal epilepsy. We look forward to sharing the results of our ongoing Phase 2 PPR study later this year, and soon after initiating a Phase 2 study in focal epilepsy," said Marcio Souza, president and chief executive officer of Praxis.

Summary of PRAX-628 Phase 1 qEEG Analysis:

- In the Phase 1 study, PRAX-628 was administered to 40 healthy participants (PRAX-628 n=30, placebo n=10). Single ascending dose (SAD) cohorts evaluated PRAX-628 doses ranging from 5 to 45 mg and multiple ascending dose (MAD) cohorts evaluated PRAX-628 doses of 20 and 30 mg.
- EEG data were collected over the course of Day 1 in the SAD cohort and over three separate days during the course of the 10-day MAD treatment period.
- qEEG analysis showed a pharmacodynamic effect at all dose levels and was significantly different from placebo at the T_{max} timepoint (~2h) for the SAD and all timepoints for the MAD portion.

Additional results from the PRAX-628 Phase 1 study will be presented at an upcoming medical conference. Praxis has begun a Phase 2 study for PRAX-628 in patients with a PPR to evaluate drug activity and dose finding, which is expected to report topline results in the second half of 2023. Based on these studies and the preclinical results, Praxis intends to advance PRAX-628 into a Phase 2 study in focal epilepsy in the first half of 2024.

About PRAX-628
PRAX-628 is a next-generation, functionally selective small molecule targeting the hyperexcitable state of sodium-channels in the brain that is currently being developed as a once daily, oral treatment for adult focal onset epilepsy. Preclinical data demonstrates PRAX-628 is differentiated from standard of care, with the potential to be best-in-class for focal epilepsy. In vitro, PRAX-628 has demonstrated superior selectivity for disease-state Na_V 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 epilepsy. 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 EC_{50}, a translational guide that suggest the therapeutic window results in unprecedented magnitude relative to approved therapies.

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 Facebook, 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 the clinical development of PRAX-628, 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, 2022, its Quarterly Reports on Form 10-Q 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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