Praxis Precision Medicines Reports Positive Topline Results from PRAX-944 Phase 2a Study in Essential Tremor Patients

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PRAX-944 demonstrated clinically meaningful functional improvement in essential tremor patients in Part B of Phase 2a study

Mean improvement at Day 42 was 42% as measured by Modified ADL, followed by statistically significant difference in the placebo-controlled randomized withdrawal phase

PRAX-944 continues to be generally well-tolerated

BOSTON, May 09, 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 announced positive topline results from Part B of its Phase 2a study evaluating the safety and efficacy of PRAX-944 for the treatment of essential tremor (ET). In the study, treatment with PRAX-944 resulted in clinically meaningful improvements in function, which were supported by improvements in tremor amplitude.

In the open-label period through Day 42, patients treated with PRAX-944 demonstrated mean improvement from baseline of 42% in the Modified Activities of Daily Living (ADL) score (N=11, nominal $p<0.05$). Following randomization, the difference between patients who remained on treatment (N=6) through Day 56 and those randomized to placebo (N=5) was clinically and statistically significant. The Modified ADL is a composite score based on TETRAS ADLs with the addition of spiral drawings and handwriting from the TETRAS performance scale.

“The data from the PRAX-944 Phase 2a study offer hope to people living with essential tremor, for a treatment developed specifically for ET,” said Bernard Ravina, M.D., chief medical officer of Praxis. “In the study, PRAX-944 demonstrated clear functional benefit while on treatment that was lost once treatment was withdrawn, with no new safety findings. Following these exciting proof-of-concept results, we intend to update the primary endpoint for the Phase 2b Essential1 study from safety to efficacy, putting us one step closer to a potential new treatment option for people living with ET.”

Part B of the Phase 2a study of PRAX-944 for the treatment of ET included both an open-label and randomized withdrawal period. In the open-label period, participants were to be titrated up to a maximum dose of 120 mg over 28-days prior to a stable period at the highest dose reached from Day 29 to Day 42. Participants who remained in the study through Day 42 were then randomized one-to-one to either active drug or placebo from Day 43 to Day 56, with a subsequent safety follow-up visit at Day 70.

“There is an enormous need for innovation in the treatment of essential tremor,” said Sheng-Han Kuo, M.D., Director, Initiative for Columbia Ataxia and Tremor (ICAT). “Today’s results highlight the potential of a new mechanism for ET, and importantly demonstrate clinically meaningful functional improvements that have a direct impact on patients’ quality of life.”

PRAX-944 was generally well tolerated in Part B of the Phase 2a study, with no new safety findings. In the study, eight of eleven participants completed the open-label period at the highest dose of 120 mg. Three evaluable participants discontinued during the open-label period due to Adverse Events (AEs), including one participant who had a pre-existing medical condition that led to a medical procedure unrelated to study drug. Treatment Emergent Adverse Events (TEAEs) were all mild to moderate, with the exception of one severe AE of essential tremor that occurred in a placebo arm patient following withdrawal of PRAX-944.

“We are emboldened by today’s results, which provide promise to those living with essential tremor that a targeted treatment is within reach,” said Patrick McCartney, executive director, International Essential Tremor Foundation. “The ET community has been underserved for decades. It’s exciting to see innovative new approaches are finally showing real progress toward safer, more effective treatments that have the potential to improve quality of life for those affected by essential tremor.”

About PRAX-944
PRAX-944 is a novel selective T-type calcium channel blocker designed to block abnormal neuron burst firing in the Cerebello-Thalamo-Cortical (CTC) circuit correlated with tremor activity. PRAX-944 is currently in clinical development for the treatment of essential tremor (ET) in the Essential1 study, a randomized, double-blind, placebo-controlled Phase 2b dose-ranging study in approximately 112 participants with TETRAS UL ≥10 at baseline, to evaluate tolerability and efficacy of PRAX-944 for treatment of adults with ET. In clinical trials to date for ET, treatment with PRAX-944 up to 120 mg/day has been generally well tolerated and showed both improvements in activities of daily living and a reduction in tremor amplitude.

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 and epilepsy, 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 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: Praxis' ability to initiate, enroll, conduct or complete ongoing and planned clinical trials and Praxis' timelines for regulatory submissions; 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; 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, 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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