Praxis Precision Medicines Announces Publication of Preclinical Data Highlighting Differentiated and Potent Antiepileptic Activity of PRAX-562

January 18, 2022

PRAX-562 demonstrated robust preclinical antiseizure activity and exhibited an improved preclinical tolerability profile compared to standard-of-care

Praxis expects to initiate a Phase 2 trial in the first half of 2022 to explore the potential for PRAX-562 to treat rare DEEs

BOSTON, Jan. 18, 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 the publication of data in the journal *Epilepsia* from a preclinical study of PRAX-562, a preferential persistent sodium channel blocker currently in development for the potential treatment of rare adult cephalgias and Developmental and Epileptic Encephalopathies (DEEs).

This preclinical study investigated the effects of PRAX-562 on intrinsic neuronal excitability and protection from induced seizures as compared to sodium channel blockers used as standard-of-care antiepileptic treatments. In the study, PRAX-562 demonstrated robust preclinical antiseizure activity and significantly improved preclinical tolerability, with improved or comparable efficacy when evaluated against currently used sodium channel blockers. The data demonstrate that by preferentially blocking persistent sodium current, PRAX-562 may represent a differentiated treatment option for disorders of hyperexcitability, such as DEEs, where standard sodium channel blockers are effective but poorly tolerated.

“Sodium channel blockers are a fundamental component of epilepsy management, but dose-limiting characteristics must be addressed for patients to realize their full benefits,” said Steven Petrou, Ph.D., co-founder and chief scientific officer of Praxis. “This study supports the potential for PRAX-562, a persistent sodium channel blocker with a favorable therapeutic window, to improve tolerability relative to marketed therapies while achieving potent antiseizure activity.”

DEEs are a group of disorders characterized by intractable seizures and other co-morbidities. Mutations in voltage-gated sodium channel (Na_V) genes are the most common causes of DEEs. Given the number of Na_V-targeting antiepileptic drugs (AEDs), and the well understood role of sodium channels in governing brain excitability, it is clear that mutations and other causes of sodium channel dysregulation contribute to the pathology of DEEs and other, more common, forms of epilepsy.[1]

“As a company deeply committed to delivering new treatment options for people suffering from complex brain disorders, we are pleased that the data highlight the potential for PRAX-562 to be a therapeutic option for people living with epilepsy,” said Marcio Souza, president and chief executive officer of Praxis. “A lot has been learned about neuronal modulation and physiology in recent years, and we are excited to initiate a study for the treatment of DEEs in the first half of this year with PRAX-562.”

Additional data supporting clinical investigation of PRAX-562 were presented at the American Epilepsy Society 2021 Annual Meeting in December.

About PRAX-562
PRAX-562 is the first preferential persistent sodium channel blocker in development for the treatment of a wide range of rare CNS disorders including rare adult cephalgias and DEEs. Praxis expects to initiate a Phase 2 trial of PRAX-562 for the 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). The Company expects to initiate a Phase 2 trial of PRAX-562 for DEEs in the first half of 2022 and anticipates reporting topline Auditory Steady-State Response (ASSR) biomarker data in the first half of 2022. PRAX-562 has received Orphan Drug and Rare Pediatric Disease Designations from the Food and Drug Administration, and Orphan Designation from the European Medicines Agency for the treatment of SCN2A-DEE and SCN8A-DEE.

About Praxis
Praxis Precision Medicines is a clinical-stage biopharmaceutical company translating genetic insights into the development of therapies for central nervous system disorders (CNS) characterized by neuronal excitation-inhibition imbalance. Praxis is applying insights from genetic epilepsies to broader neurological and psychiatric disorders, using our understanding of shared biological targets and circuits in the brain. Praxis has established a broad portfolio, including multiple disclosed programs across CNS disorders including depression, epilepsy, movement disorders and pain syndromes, 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 may contain 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 clinical data, the sufficiency of our cash, cash equivalents and marketable securities, the anticipated timing of our clinical trials and regulatory filings, and 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: 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, 2020, its Quarterly Reports on Form 10-Q and its other subsequent 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.

Investor Contact:
Alex Kane
Praxis Precision Medicines
investors@praxismedicines.com
617-300-8481

Media Contact:
Ian Stone
Canale Communications
Ian.stone@canalecomm.com

[1] Brunklaus A, Lal D. Sodium channel epilepsies and neurodevelopmental disorders: from disease mechanisms to clinical application. Dev Med Child Neurol. 2020 Jul;62(7):784-792. doi: 10.1111/dmcn.14519. Epub 2020 Mar 30. PMID: 32227486.