Praxis Precision Medicines to Present at the American Academy of Neurology 2023 Annual Meeting

April 19, 2023 at 8:00 AM EDT

BOSTON, April 19, 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 that it will deliver presentations on its clinical stage programs at the upcoming American Academy of Neurology (AAN) 2023 Annual Meeting, held April 22-27, 2023 in Boston, Massachusetts.

“Each of our clinical stage programs has made tangible progress in recent months and we look forward to sharing additional details on these programs at the upcoming AAN conference,” said Steven Petrou, Ph.D., co-founder and chief scientific officer of Praxis. “We recently released topline results from the ulixacaltamide Essential1 Phase 2 study in essential tremor and will provide perspective on these data at AAN. Also, we expect to dose the first patients in our PRAX-222 EMBRAVE study and PRAX-562 EMBOLD study within the coming weeks and are excited to share data supporting these programs. Finally, we look forward to sharing new in-vivo results for PRAX-628 that highlight this program’s potential to be a best-in-class treatment for focal epilepsy patients.”

Presentation Details:

A Novel Approach to Assess the Impact of Disease in Patients with SCN8A-Related Developmental and Epileptic Encephalopathy

- Session Date/Time: Sunday April 23, 5:30 p.m. – 6:30 p.m. EDT
- Poster Session P3: 9-008

PRAX-562-102: A Phase 1 Trial Evaluating the Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of PRAX-562 in Healthy Volunteers

- Session Date/Time: Monday April 24, 8:00 a.m. – 9:00 a.m. EDT
- Poster Session P4: 9-011

PRAX-628 is a Novel, Well-tolerated, Activity Dependent Sodium Channel Blocker with Potent Anticonvulsant Activity

- Session Date/Time: Monday April 24, 8:00 a.m. – 9:00 a.m. EDT
- Poster Session P4: 9-012

PRAX-562-101: A Phase 1 Trial Evaluating the Safety, Tolerability, Pharmacokinetics and Food Effect of PRAX-562 in Healthy Volunteers

- Session Date/Time: Tuesday April 25, 11:45 a.m. – 12:45 p.m. EDT
- Poster Session P8: 9-011

PRAX-628: A Novel Sodium Channel Blocker with Greater Potency and Activity Dependence Compared to Standard of Care

- Session Date/Time: Tuesday April 25, 11:45 a.m. – 12:45 p.m. EDT
- Poster Session P8: 9-012

Disease Impact and Burden in Patients with SCN2A-Related Developmental and Epileptic Encephalopathy

- Session Date/Time: Wednesday April 26, 11:45 a.m. – 12:45 p.m. EDT
- Poster Session P11: 9-011

PRAX-562 is a Well-tolerated, Novel Persistent Sodium Channel Blocker with Broad Anticonvulsant Activity in Multiple DEE Mouse Models

- Session Date/Time: Wednesday April 26, 2:10 p.m. EDT
- Scientific Session S34 - Child Neurology and Developmental Neurology 2: 007

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- Session Date/Time: Sunday April 23 1:00 p.m. - 1:20 p.m. EDT
- Centralized Emerging Neurologic Care Presentation Stage in the Exhibit Hall: Praxis Precision Medicines Booth #789

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 sodium channel block is consistent with superior selectivity for disease state sodium channel (Na_V) channel hyperexcitability. In vivo studies of PRAX-562 have demonstrated dose-dependent block of seizures up to complete inhibition 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 Na_V channel blocking effects. PRAX-562 has received Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation from the FDA, and ODD from the European Medicines Agency for the treatment of SCN2A-DEE and SCN8A-DEE, respectively.

About PRAX-628

PRAX-628 is a novel activity-dependent inhibitor of peak sodium current (I_{Na}) and persistent I_{Na} 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 Na_V blockers, 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 model, a translational model for efficacy in focal epilepsy.

About Ulixacaltamide
Ulixacaltamide is a differentiated and highly selective small molecule inhibitor of T-type calcium channels designed to block abnormal burst firing in neuronal circuits implicated in tremor pathology. Ulixacaltamide, the most advanced program within Praxis' Cerebrum™ small molecule platform, is currently in late-stage development for the treatment of essential tremor and is also in development as a non-dopaminergic treatment for the motor symptoms of Parkinson's disease.

About SCN2A-DEE
SCN2A-DEE is a monogenic epilepsy disorder caused by a variant in the SCN2A gene. The SCN2A gene is critical in the formation of sodium channel proteins in the brain, which control the flow of sodium ions into neurons. This movement of sodium ions is a major component of generating electrical signals called action potentials, the way in which the cells communicate. SCN2A-DEE presents with a wide range of phenotypes. Early-onset SCN2A-DEE presents before three months and can lead to profound impact on patients, including drug-resistant seizures, significant cognitive impairment, movement disorders such as dystonia or ataxia and problems in other body systems such as gastrointestinal or ocular. Currently there are no approved treatments for SCN2A-DEE, and the standard-of-care typically involves a regimen of many concurrent anti-seizure medications as well as medications for co-morbidities. Despite these interventions, more than 70% of early-onset SCN2A-DEE patients live with uncontrolled seizures, and approximately 75% live with severe intellectual disability.

About SCN8A-DEE
SCN8A-DEE is a rare developmental and epileptic encephalopathy caused by a variant in the SCN8A gene. The SCN8A gene is critical in the formation of sodium channel proteins in the brain, which control the follow of sodium ions into neurons. This movement of sodium ions is a major component of generating electrical signals called action potentials, the way in which the cells communicate. Patients suffer from recurrent, typically drug-resistant seizures which start as early as the first day of life. The seizures can be of multiple different types, up to dozens per day, with poor response to current treatment options. Patients with SCN8A-DEE have significant cognitive disabilities, ranging from moderate to severe; often movement disorders, such as dystonia or ataxia; and problems in other body systems such as gastrointestinal or ocular. SCN8A-DEE patients also may experience autonomic features such as increases or decreases in heart rate, abnormal breathing and cyanosis.

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 anticipated timing of our clinical trials and the development 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; 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 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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