Praxis Precision Medicines to Showcase Largest Pipeline of Precision Epilepsy Programs and Breadth of Commitment to Epilepsy Treatments at Upcoming Meetings

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BOSTON, Nov. 28, 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 epilepsy programs at the following upcoming meetings in Orlando, Florida. Presentations at the American Epilepsy Society (AES) Annual Meeting, as well as related scientific and patient advocacy meetings, will cover new patient data, updates on clinical progress and new paradigms for developing epilepsy therapies.

- AES Annual Meeting, being held December 1-5, 2023
- Behind the Seizure (BTS) Scientific Exhibit, being held on December 3, 2023
- Partners Against Mortality in Epilepsy (PAME) Conference, being held on November 30, 2023
- SYNGAP1 Conference, being held on November 30, 2023
- The Cute Syndrome Foundation (TCSF) SCN8A Clinician, Researcher, and Family Gathering, being held on December 1, 2023

“It’s an incredibly exciting time for Praxis and our epilepsy portfolio, as we share updates from first-in-patient and Phase 2 studies for elsunersen (PRAX-222), PRAX-562 and PRAX-628,” said Marcio Souza, president and chief executive officer of Praxis. “We look forward to presenting data spanning our Solidus and Cerebrum platforms at AES and related meetings in Florida, with presentations also set to highlight novel approaches to define efficient preclinical models and sensitive biomarkers for accelerating drug development for the patient populations we are seeking to serve. Our epilepsy programs have the potential to significantly impact the lives of patients and their families, and we are grateful for the opportunity to discuss these programs with the epilepsy community at multiple important meetings this week.”

Praxis at AES 2023 | Orange County Convention Center

- ***Meet us at booth #421 to learn about the largest pipeline of precision epilepsy programs***
- ***Head to one of our presentations listed below or visit us at the BTS Scientific Exhibit on December 3, 08:00 a.m. - 11:00 a.m. ET***

EMBRAVE: A Clinical Trial of PRAX-222, a Novel Antisense Oligonucleotide, in Pediatric Participants with Early Onset SCN2A Developmental and Epileptic Encephalopathy

- Session Date/Time: Monday, December 4, 12:00 p.m. - 2:00 p.m. ET
- Abstract number: 3.198
- Summary: Preliminary results from Part 1 of the EMBRAVE study demonstrate elsunersen tolerability and unprecedented efficacy, highlighting its potential to be the first disease-modifying treatment in early onset SCN2A developmental and epileptic encephalopathy (SCN2A-DEE).

PRAX-628 is a Next Generation, Functionally Selective Small Molecule with Potent Anti-Seizure Activity and Potential as Best-in-Class Treatment for Focal Epilepsy

- Session Date/Time: Monday, December 4, 12:00 p.m. - 2:00 p.m. ET; platform talk at 5:00 p.m. ET [Platform D | Epilepsy Therapies]
- Abstract number: 3.258
- Summary: Combined preclinical and clinical data demonstrate PRAX-628 is differentiated from standard of care, with the potential to be best-in-class for focal epilepsy. In vivo studies demonstrate unprecedented potent anticonvulsant activity in the maximal electroshock seizure (MES) model, a highly predictive translational model for efficacy in focal epilepsy, while first-in-human findings demonstrate that PRAX-628 is well tolerated at exposures >15x the predicted efficacious exposure

from mouse MES.

A Novel Method to Define an EEG Composite for the Detection of Drug Effects of Next Generation Small Molecules for Epilepsy

- Session Date/Time: Monday, December 4, 12:00 p.m. - 2:00 p.m. ET
- Abstract number: 3.455
- Summary: Application of machine learning methods to complex EEG data has the potential to accelerate drug development in epilepsy by revealing pharmacodynamic effects of novel agents that are clearly distinguishable from placebo. In this study, we demonstrate applicability of a quantitative EEG composite to three distinct next generation small molecules, with expected generalizability to any small molecule, independent of class/target.

Translational Concordance of Preclinical Seizure Models in Focal and Generalized Epilepsies

- Session Date/Time: Monday, December 4, 12:00 p.m. - 2:00 p.m. ET
- Abstract number: 3.458
- Summary: This study provides novel insights into the clinical validity of commonly used preclinical seizure models across the clinical epilepsy spectrum. Using a newly developed scoring matrix to assess translational concordance and predictability, mouse MES, audiogenic and 6-Hz 32 mA emerge as three acute seizure models with greatest predictive validity and versatility for ASM drug discovery.

Praxis at PAME 2023 | Rosen Centre Hotel

EMBRAVE: A Clinical Trial of PRAX-222, a Novel Antisense Oligonucleotide, in Pediatric Participants with Early Onset SCN2A Developmental and Epileptic Encephalopathy

- Session Date/Time: Thursday, November 30, 5:00 p.m. - 6:30 p.m. ET
- Poster number: 32
- Summary: Preliminary results from Part 1 of the EMBRAVE study demonstrate elsunersen tolerability and unprecedented efficacy, highlighting its potential to be the first disease-modifying treatment in early onset SCN2A-DEE.

EMBOLD: A Clinical Trial of PRAX-562 in Subjects with Developmental and Epileptic Encephalopathies Followed by an Open-Label Extension

- Session Date/Time: Thursday, November 30, 5:00 p.m. - 6:30 p.m. ET
- Poster number: 38
- Summary: EMBOLD is the first and only DEE trial to offer a decentralized clinical trial option, balancing clinical rigor with flexibility and convenience for participants and their families. EMBOLD will provide important findings regarding the safety, tolerability, efficacy and pharmacokinetics of PRAX-562 as a potential first- and best-in-class treatment for pediatric patients with SCN2A-DEE and SCN8A-DEE.

Praxis at SYNGAP1 2023 | Embassy Suites Hotel

Progress Toward the Discovery of an ASO for Therapeutic Upregulation of SYNGAP1

- Session Date/Time: Thursday, November 30, 1:30 p.m. - 1:45 p.m. ET | Session 4: Therapeutic Strategies to Fix SYNGAP1
- Oral presentation highlighting preclinical updates from our SYNGAP1 ASO platform at the annual scientific conference hosted by the SYNGAP Research Fund

Praxis at TCSF 2023 | Sheraton Orlando Lake Buena Vista Resort

PRAX-562: Reflection and Direction on a Next Generation Anti-Seizure Small Molecule in Development for SCN8A-DEE

- Session Date/Time: Friday, December 1, 6:15 p.m. ET
- Oral presentation highlighting clinical updates from our PRAX-562 small molecule platform at the annual clinician, researcher, and family gathering for SCN8A

About elsunersen (PRAX-222)
Elsunersen is an ASO designed to selectively decrease SCN2A gene expression, directly targeting the underlying cause of early-seizure-onset SCN2A-DEE to treat seizures and other symptoms in patients with gain-of-function 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 Priority Medicines (PRIME) designation from the European Medicines Agency (EMA) for the treatment of SCN2A-DEE. The elsunersen program is ongoing under a collaboration with Ionis Pharmaceutics, Inc., and RogCon, Inc.

About PRAX-562
PRAX-562 is a first-in-class small molecule in development for the treatment of DEE 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 (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 channel blocking effects. 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 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 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 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 EC50.

About SCN2A-DEE
SCN2A-DEE is a debilitating monogenic epilepsy disorder caused by a variant in the SCN2A gene, associated with early mortality. 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 is characterized by a broad spectrum 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 to manage 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 with patients rarely surviving beyond their teenage years.

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/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 development of our product candidates, including the design of clinical trials and the treatment potential of Praxis' 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; reported interim data from ongoing studies and trials differing materially from final data from preclinical studies and completed 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; Praxis' anticipated cash runway; 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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