Praxis Precision Medicines Announces Rare Pediatric Disease Designation Granted for Relutrigine in Dravet Syndrome

December 18, 2024

Dravet syndrome is a genetic developmental and epileptic encephalopathy (DEE) often caused by a mutation in SCN1A

This is the third Rare Pediatric Disease Designation for relutrigine, adding to those granted for SCN2A and SCN8A DEEs

Praxis plans to initiate an all-DEE trial (EMERALD), inclusive of Dravet syndrome, in 1H2025

BOSTON, Dec. 18, 2024 (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 U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation (RPDD) for relutrigine in Dravet syndrome.

“We are thrilled to have been granted rare pediatric disease designation for relutrigine in Dravet syndrome. This milestone reflects our commitment to addressing critical unmet needs in rare neurological disorders and underscores the potential of relutrigine as a meaningful new option for patients and families affected by this debilitating developmental and epileptic encephalopathy. The designation builds upon encouraging clinical data demonstrating relutrigine’s ability to reduce seizure frequency and improve overall seizure control, highlighting our dedication to advancing innovative therapies that can significantly impact quality of life for those with limited treatment options,” said Marcio Souza, president and chief executive officer.

Rare pediatric diseases are defined by the FDA as serious or life-threatening conditions primarily affecting children under 18, with fewer than 200,000 cases in the U.S. A key benefit of obtaining a RPDD is the potential to receive a priority review voucher following FDA approval of a product with RPDD if the marketing application submitted for the product satisfies certain conditions. These vouchers can significantly expedite the review process for future New Drug Applications or biologic license applications.

In September, and updated at the recent 2024 American Epilepsy Society Annual Meeting, Praxis shared results for relutrigine in SCN2A and SCN8A patients in cohort 1 of the EMBOLD study that showed:

- Placebo-adjusted monthly motor seizure reduction of 46% during the double-blind period
- Over 30% of patients achieved seizure freedom status while on relutrigine
- Meaningful gains observed in alertness, communication and seizure severity
- 77% reduction in median seizure rate seen for patients in the long-term extension

Based on the results of this study, Praxis has initiated and is enrolling patients in a second, registrational cohort for SCN2A and SCN8A patients, expecting topline results in the first half of 2026.

About Relutrigine (PRAX-562)
Relutrigine is a first-in-class small molecule in development for the treatment of developmental and epileptic encephalopathy (DEE) as a preferential inhibitor of persistent sodium current, shown to be a key driver of seizure symptoms in SCN2A-DEE and SCN8A-DEE. Relutrigine’s mechanism of sodium channel modulation is consistent with superior selectivity for disease state sodium channel (NaV) channel hyperexcitability. In vivo studies of relutrigine have demonstrated dose-dependent inhibition of seizures up to complete control of seizure activity in SCN2A, SCN8A and other DEE mouse models. Relutrigine has been generally well-tolerated in three Phase 1 studies and has demonstrated biomarker changes indicative of NaV channel blocking effects. Relutrigine has received Orphan Drug Designation (ODD) and RPDD 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 Dravet Syndrome
Dravet syndrome is a severe, progressive genetic epilepsy that typically begins within the first year of life, marked by frequent, prolonged, and treatment-resistant seizures. Beyond seizures, the condition often leads to intellectual disability, developmental delays, movement and balance difficulties, speech and language impairments, growth abnormalities, sleep disturbances, autonomic nervous system dysfunction, and mood disorders. Classified as a developmental and epileptic encephalopathy, Dravet syndrome is associated with significant cognitive and developmental impairments. Patients with this condition also face an elevated risk of early mortality, including due to sudden unexpected death in epilepsy (SUDEP).

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 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; preliminary analyses from ongoing studies 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; and other risks concerning Praxis' programs and operations as described in its Annual Report on Form 10-K for the year ended December 31, 2023, 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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