Praxis Precision Medicines Announces the FDA Has Granted Breakthrough Therapy Designation for Ulixacaltamide HCl in Essential Tremor

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The Breakthrough Therapy Designation was granted based on the positive topline results from the Essential3 Phase 3 program in essential tremor

Praxis remains on track to submit ulixacaltamide NDA in early 2026 based on recently completed pre-NDA meeting with the FDA

BOSTON, Dec. 29, 2025 (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 the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation (BTD) for ulixacaltamide, a differentiated and highly selective small molecule inhibitor of T-type calcium channels, for the treatment of patients with essential tremor (ET).

“The granting of the Breakthrough Therapy Designation for ulixacaltamide, based on the Essential3 program, further underscores its potential to address the substantial unmet need in patients with ET. We recently completed a series of positive interactions with the FDA, that, together with this BTD, are enabling us to advance this promising treatment faster to patients. We are diligently preparing for the filing of the ulixacaltamide NDA, which we expect in early 2026,” said Marcio Souza, president and chief executive officer.

The BTD enables expedited development and regulatory review for drugs that are intended to treat a serious condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement on a clinically significant endpoint(s) over available therapies. The BTD for ulixacaltamide was based on the positive topline data from the Essential3 program, consisting of two pivotal Phase 3 studies of ulixacaltamide in ET.

In December 2025, Praxis announced the successful completion of its pre-NDA meeting with the FDA, including the receipt of written feedback and an in-person meeting. The Company has aligned with the FDA on the content of the NDA and expects to submit the ulixacaltamide NDA in early 2026.

About Essential Tremor (ET)
Essential Tremor is the most common movement disorder, affecting roughly seven million people in the United States alone, representing a multi-billion dollar commercial opportunity. ET is characterized by involuntary rhythmic movement in the upper limbs, with or without tremor in other body locations such as the head, vocal cords, or legs. These tremors significantly disrupt daily living and are progressive in nature, with increases in tremor severity and amplitude commonly observed over the course of the disease. Propranolol, a beta-blocker, is the only approved pharmacotherapy for ET, offering limited efficacy and poor tolerability and is also contraindicated for comorbidities that affect a significant share of the ET population. Other beta blockers and anti-convulsants are used off-label, though similarly are characterized by limited efficacy and tolerability. The vast majority of patients are left without a treatment option, with estimated minimum of 2 million patients seeking treatment. In a patient survey, up to 77% of patients felt their ET is inadequately controlled and up to 50% of patients aren’t receiving treatment. Indeed, U.S. neurologists surveyed indicated that 85% of their visits are for patients seeking treatment, and 40% of their patients are not receiving any treatment. These findings underscore the need for more effective treatments for ET.

About Ulixacaltamide
Ulixacaltamide is a differentiated and highly selective small molecule inhibitor of T-type calcium channels designed to block abnormal neuronal burst firing in the Cerebello-Thalamo-Cortical (CTC) circuit correlated with tremor activity. Ulixacaltamide is the most advanced program within Praxis’ Cerebrum™ small molecule platform.

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 epilepsy and movement disorders, 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 Praxis’ product candidates and the anticipated timing of regulatory submissions and interactions, 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 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, 2024 and as updated in its Quarterly Report on Form 10-Q for the quarter ended June 30, 2025, as well as 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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