Praxis Precision Medicines to Present Analyses of Patient-Focused, Clinically Meaningful Endpoints from Essential1 Study of Ulixacaltamide in Essential Tremor at the 2023 MDS International Congress

August 28, 2023 at 7:00 AM EDT

BOSTON, Aug. 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 be presenting analyses highlighting patient-focused endpoints from its Essential1 study of ulixacaltamide (PRAX-944) in essential tremor (ET) at The International Parkinson and Movement Disorder Society’s (MDS) International Congress of Parkinson’s Disease and Movement Disorders®, taking place in Copenhagen, Denmark.

“Recent FDA guidance highlights the importance of patient-focused clinical outcome assessments related to treatment effect. Using data from Essential1, we define for the first time what clinically meaningful change is for adults with ET, with our results showing sustained improvement in patients’ daily functioning,” said Marcio Souza, president and chief executive officer of Praxis. “The design of our upcoming Phase 3 program for ulixacaltamide applies these insights. We look forward to presenting our analyses highlighting modified Activities of Daily Living 11 as a reliable endpoint to measure the potential efficacy and durability of ulixacaltamide at this year’s MDS Congress.”

Presentation Details:

**Patient-Focused, Clinically Meaningful Endpoints as Evidence of Improved Outcomes and Durability of Effect Following Ulixacaltamide Treatment in Adults with Essential Tremor: Findings from Essential1**

- **Viewing Dates/Times:** Monday, August 28 – Wednesday, August 30 1:00 p.m. – 3:00 p.m. CEDT
- **Poster:** LBA-19
- **Summary:** While the concept of clinically meaningful change has been extensively studied in other movement disorders and neurological conditions, this has not been evaluated in ET. This study represents the first time a Meaningful Score Difference has been defined in ET using a large patient dataset and a focus on measures determined to be most meaningful to patients. In this poster presentation, Praxis will highlight modified Activities of Daily Living 11 (mADL11) as a reliable, patient-focused clinical outcome assessment for measuring ulixacaltamide efficacy and durability of effect, with important decision-making implications for ET therapies.

**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, the most advanced program within Praxis’ Cerebrum™ small molecule platform, is currently in development for the treatment of essential tremor.

**About Essential Tremor**

Essential Tremor (ET) is the most common movement disorder, affecting roughly seven million people in the United States alone, including approximately two million diagnosed patients. 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. Other beta blockers and anti-convulsants are used off-label, though they are characterized by limited efficacy and tolerability. For these reasons, approximately 40% of patients who seek pharmacotherapy treatment for ET discontinue within two years.

**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](http://www.praxismedicines.com) and follow us on [Facebook](http://Facebook), [LinkedIn](http://LinkedIn) and [Twitter](http://Twitter).

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