



Jazz Pharmaceuticals Announces First Patient Enrolled in Phase 2b Clinical Trial Evaluating Novel Suvecaltamide (JZP385) for Once-Daily Treatment of Adults with Essential Tremor

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DUBLIN, Dec. 15, 2021 /PRNewswire/ -- Jazz Pharmaceuticals plc (Nasdaq: JAZZ) today announced that the first patient has been enrolled in a global Phase 2b clinical trial evaluating the safety and efficacy of suvecaltamide (also known as JZP385), a highly selective modulator of T-type calcium channels in development for the potential treatment of moderate to severe essential tremor (ET).¹ The multicenter, double-blind, randomized, placebo-controlled trial will evaluate adults with moderate to severe ET treated with a once-daily oral capsule of suvecaltamide or placebo. ET is the most common movement disorder, yet a new pharmacotherapy has not been approved for the underlying cause of the disorder in more than 50 years.^{2,3,4}

"Currently, patients with ET are treated with drugs that were not developed for this specific disorder. These therapies are often not well tolerated and do not fully control the frequent and involuntary tremors that patients with ET experience, leading them to discontinue treatment,"^{1,4,5} said Mark A. Goldstein, M.D., FAAN, medical director, JEM Research Institute and Phase 2b trial investigator. "We look forward to evaluating suvecaltamide to increase our understanding of its potential to manage ET in adult patients."

"The International Essential Tremor Foundation is excited that a potential therapy to address a primary cause of ET is entering a clinical trial," said Patrick McCartney, executive director, International Essential Tremor Foundation. "The lack of a targeted treatment for patients with ET means that they must rely on their perseverance to grapple with the challenges they experience in their daily lives due to the disorder."

Investigators will conduct the trial across more than 50 trial sites in the United States (U.S.), United Kingdom (U.K.) and the European Union (EU). They will enroll 400 adults aged 18 to 80 diagnosed with ET as defined by the Consensus Statement on the Classification of Tremors from the Task Force on Tremor of the International Parkinson and Movement Disorder Society.⁶ Participants also must have moderate to severe disability associated with their tremor. In this double-blind, placebo-controlled trial, patients will receive a once-daily oral dose of 10, 20 or 30 milligrams of suvecaltamide or placebo for 12 weeks.

The primary efficacy endpoint of this clinical trial is the change from baseline to Week 12 on the Tremor Research Group Essential Tremor Rating Assessment Scale (TETRAS) composite outcome score, which represents items from the TETRAS-Activities of Daily Living and TETRAS-Performance Subscale, and measures the functional impact due to tremor.

"This trial demonstrates Jazz's continued commitment to address the unmet need in people living with debilitating neurological conditions, such as ET," said Rob Iannone, M.D., M.S.C.E., executive vice president, research and development and chief medical officer of Jazz Pharmaceuticals. "Jazz's R&D organization continues to evolve and expand its capabilities across multiple mid- and late-stage programs and the advancement of suvecaltamide is the latest near-term catalyst that could potentially contribute to a pivotal package. We look forward to a topline data readout in the first half of 2024."

Additional information about the trial, including eligibility criteria and a list of clinical trial sites, can be found at www.EveresTstudy.com and www.clinicaltrials.gov (ClinicalTrials.gov Identifier: NCT05122650).

About Suvecaltamide

Suvecaltamide (also known as JZP385) is a highly selective modulator of T-type calcium channels in development for the potential treatment of moderate to severe essential tremor (ET). T-type calcium channels play a role in the brain's management of muscle movement.¹ Jazz acquired suvecaltamide, formerly known as CX-8998, through the company's August 2019 acquisition of Cavion, a clinical-stage biotechnology company that is now a wholly owned subsidiary of Jazz.⁷ In 2017, Cavion conducted a Phase 2 randomized, placebo-controlled clinical trial in adults with ET, the T-CALM (Tremor-CAv3 modulation) trial (NCT 03101241).⁸ This trial evaluated a twice-daily formulation of suvecaltamide that demonstrated proof-of-concept, and supported continued development of the molecule. The Phase 2b trial will evaluate a once-daily formulation of suvecaltamide.

About Essential Tremor

Essential tremor (ET) is the most common movement disorder affecting millions of people worldwide. In the U.S., U.K. and key countries in the EU, the prevalence of ET is estimated to be 11 million, of which 2 million patients have been diagnosed.^{9,10} ET is an abnormal and involuntary rhythmic tremor that is not a symptom of another neurological disorder or drug-induced side effect.^{4,6} ET is characterized by to-and-fro movements, called oscillations, of at least one part of the body due to involuntary rhythmic muscle movements that can occur from four to 12 times per second.^{4,6} Some patients may also experience mild degeneration in parts of the brain's cerebellum, which is responsible for voluntary movement.⁴ ET most commonly affects the hands and arms, but tremor can also occur in the head, voice and lower limbs.^{4,6} Stress, fever, physical exhaustion or low blood sugar may trigger ET or may make it worse.⁴ ET has no cure and therapies used to control symptoms of the disorder include beta blockers, anticonvulsant medicines and neurosurgical-procedures, which may not fully control tremors.^{4,6}

About Jazz Pharmaceuticals plc

Jazz Pharmaceuticals plc (NASDAQ: JAZZ) is a global biopharmaceutical company whose purpose is to innovate to transform the lives of patients and their families. We are dedicated to developing life-changing medicines for people with serious diseases—often with limited or no therapeutic options. We have a diverse portfolio of marketed medicines and novel product candidates, from early- to late-stage development, in neuroscience and oncology. Within these therapeutic areas, we are identifying new options for patients by actively exploring small molecules and biologics, and through innovative delivery technologies and cannabinoid science. Jazz is headquartered in Dublin, Ireland and has employees around the globe, serving patients in nearly 75 countries. For more information, please visit www.jazzpharmaceuticals.com and follow @JazzPharma on Twitter.

Caution Concerning Forward-Looking Statements

This press release contains forward-looking statements, including, but not limited to, statements related to the potential timing of the availability of suvecaltamide for people with essential tremor and the potential impact on that community and other statements that are not historical facts. These forward-looking statements are based on Jazz Pharmaceuticals' current plans, objectives, estimates, expectations and intentions and inherently involve significant risks and uncertainties. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of these risks and uncertainties, which include, without limitation, risks and uncertainties associated with: pharmaceutical product development; the regulatory approval process, including the risk that the company may be unable to effectively commercialize suvecaltamide; and other risks and uncertainties affecting the company and its development programs, including those described from time to time under the caption "Risk Factors" and elsewhere in Jazz Pharmaceuticals plc's Securities and Exchange Commission filings and reports (Commission File No. 001-33500), including Jazz Pharmaceuticals' Quarterly Report on Form 10-Q for the quarter ended September 30, 2021 and future filings and reports by Jazz Pharmaceuticals. Other risks and uncertainties of which Jazz Pharmaceuticals is not currently aware may also affect Jazz Pharmaceuticals' forward-looking statements and may cause actual results and the timing of events to differ materially from those anticipated. The forward-looking statements herein are made only as of the date hereof or as of the dates indicated in the forward-looking statements, even if they are subsequently made available by Jazz Pharmaceuticals on its website or otherwise. Jazz Pharmaceuticals undertakes no obligation to update or supplement any forward-looking statements to reflect actual results, new information, future events, changes in its expectations or other circumstances that exist after the date as of which the forward-looking statements were made.

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