PTC Therapeutics Announces Initiation of a Registration-Directed Clinical Trial to Evaluate Vatiquinone in Mitochondrial Epilepsy

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- Global Phase 2/3 trial now enrolling patients with highly morbid unmet medical condition -

SOUTH PLAINFIELD, N.J., Oct. 26, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT), today announced the initiation of a registration-directed Phase 2/3 clinical trial to evaluate vatiquinone (PTC743) in patients with mitochondrial epilepsy, the highly morbid condition of refractory seizures in children with inherited mitochondrial disease. It is estimated that there are approximately 11,000-13,000[1] patients with mitochondrial epilepsy in the United States, European Union, Japan and Latin America. Vatiquinone, developed from PTC's Bio-e platform, is an investigational oral small molecule that inhibits 15-Lipoxygenase, a key enzyme that regulates oxidative stress and inflammation response pathways underpinning many neurological disease pathologies, including epilepsy. The U.S. Food and Drug Administration has granted PTC743 orphan drug designation and pediatric rare disease designation for mitochondrial epilepsy.

"We are excited to initiate this registrational clinical trial to evaluate the first indication for vatiquinone that was identified from our Bio-e platform," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "As a devastating and highly fatal pediatric disorder with no approved treatment options, mitochondrial epilepsy represents an area of significant unmet need. Vatiquinone has been evaluated in over 500 patients to date, and in clinical studies it has demonstrated effects in resolving refractory status epilepticus, decreasing seizure frequency and seizure-related morbidity in certain patients. We look forward to advancing this compound for patients who are clearly in need of treatment."

The Phase 2/3 trial in mitochondrial epilepsy, named MIT-E, is a randomized, placebo-controlled study evaluating vatiquinone in approximately 60 patients with genetically confirmed mitochondrial disease and associated refractory epilepsy. Enrolled patients will participate in a one-month run-in phase to ensure a minimum number of observable motor seizures. Patients who meet that threshold will then be enrolled in a six-month parallel arm placebo-controlled phase. The study's primary endpoint is the reduction of observed motor seizure frequency in the placebo-controlled phase relative to the run-in phase. Secondary endpoints include the occurrence of status epilepticus, number of hospitalizations, rescue medication use, and caregiver burden.

About Vatiquinone (PTC743)
Vatiquinone is an investigational small molecule that inhibits 15-Lipoxygenase, an enzyme that is a key regulator of the oxidative stress and inflammation response pathways that underpin neurological disease pathology. Vatiquinone has been evaluated in a number of clinical studies in which it has been demonstrated to have an impact on mortality risk and a number of neurological and neuromuscular disease symptoms. In more than 500 patients with duration of exposure up to 10 years, vatiquinone has demonstrated a favorable safety profile.

About the Bio-e platform
The Bio-e platform is based on a family of enzyme targets, oxidoreductases, that are critical to the generation and regulation of energy key to disease pathology in the CNS and other organ systems. The platform harnesses electron-transfer chemistry to modulate key biological processes beyond the reach of current drug development approaches. The lead compounds from the Bio-e platform, vatiquinone (PTC743) and PTC857, target the enzyme 15-lipoxygenase, a key enzymatic hub that regulates the inflammation and oxidative stress that underpin mitochondrial disease and CNS pathology.

About PTC Therapeutics, Inc.
PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, on Twitter at @PTCBio, and on LinkedIn.

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Forward-Looking Statements:
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this release, other than statements of historic fact, are forward-looking statements, including statements regarding: the future expectations, plans and prospects for PTC, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses and other matters; PTC's strategy, future operations, future financial position, future revenues and projected costs; and the objectives of management. Other forward-looking statements may be identified by the words "guidance," "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: the eligible patient base and commercial potential of PTC's products and product candidates; the outcome of pricing, coverage and reimbursement negotiations with third party payors for PTC's products or product candidates that PTC commercializes or may commercialize in the future; significant business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; PTC's scientific approach and general development progress; and the factors discussed in the "Risk Factors" section of PTC's most recent Quarterly Report on Form 10-Q and Annual Report on Form 10-K, as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC. You are urged to carefully consider all such factors.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory or prove to be commercially successful.

The forward-looking statements contained herein represent PTC's views only as of the date of this press release and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this press release except as required by law.

[1]*Gorman et al. Annals of Neurology.2015; Chevallier et al. Epilepsia. 2014;*

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