PTC Therapeutics Announces Initiation of Global Phase 3 Clinical Trial to Evaluate Vatiquinone in Friedreich Ataxia

November 30, 2020

- Third trial initiated in 2020 from compounds identified from PTC's Bio-e platform -

SOUTH PLAINFIELD, N.J., Nov. 30, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT), today announced the initiation of the registration-directed Phase 3 MOVE-FA study evaluating vatiquinone (PTC743) in children and young adults with Friedreich ataxia (FA). FA is a genetic, progressive, neurodegenerative movement disorder, typically diagnosed in childhood or adolescence.[1] PTC estimates that there are 25,000 patients with FA worldwide and there are currently no approved disease modifying therapies for FA. In a previous Phase 2 trial, vatiquinone demonstrated a statistically significant effect on disease severity at 24 months relative to age and stage-matched natural history controls as assessed by the validated Friedreich ataxia rating scale (FARS) score and a favorable safety profile. Vatiquinone has been granted Orphan Drug Designation and Fast Track Designation for Friedreich ataxia by the U.S. Food and Drug Administration (FDA).

"The initiation of another pivotal trial this year is a major milestone for PTC," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "This is the third clinical trial initiated this year based on compounds using our Bio-e platform. Vatiquinone is an exciting orally bioavailable small molecule that targets 15-lipoxygenase, the key enzyme regulating signaling pathways that control neuroinflammation and oxidative stress. Both pathways are important players in determining the pathology and disease progression seen in Friedreich ataxia patients. Previous clinical trial results give us confidence in vatiquinone's potential in treating patients living with this devastating disease."

The Phase 3 MOVE-FA trial is an 18-month parallel arm, placebo-controlled study evaluating vatiquinone versus placebo in approximately 110 children and young adults with FA. The primary endpoint is the change from baseline in the modified Friedreich ataxia rating scale (mFARS), with key secondary endpoints assessing ambulation and activities of daily living. This endpoint strategy was developed in consultation with both the FDA and European Medicines Agency. The study will include sites in the U.S., E.U., Australia and Latin America.

"FARA and the FA community are grateful to PTC Therapeutics for their commitment to advancing treatments for Friedreich ataxia," stated Jennifer Farmer, Chief Executive Officer, FARA. "We are excited for the PTC743 program to reach this milestone of opening enrollment of the MOVE-FA clinical trial and we look forward to assisting PTC with recruitment in the US and internationally."

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 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 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 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; the

eligible patient base and commercial potential of PTC's products and product candidates; 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] National Organization for Rare Disorders (NORD) (2020). Friedreich's Ataxia. https://rarediseases.org/rare-diseases/friedreichs-ataxia/. Accessed November 13, 2020.

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