PTC Therapeutics Announces Topline Results from Vatiquinone MOVE-FA Registration-Directed Trial

May 23, 2023

- Conference call and webcast to be held at 5:00 pm EDT -

SOUTH PLAINFIELD, N.J., May 23, 2023 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today reported topline results from the MOVE-FA trial of vatiquinone in patients with Friedreich ataxia. The study did not meet its primary endpoint of statistically significant change in mFARS score at 72 weeks in the primary analysis population. However, vatiquinone treatment did demonstrate significant benefit on key disease subscales and secondary endpoints. In addition, in the population of subjects that completed the study protocol, significance was reached in the mFARS endpoint and several secondary endpoints.

"While we are disappointed that the study did not achieve its primary endpoint, we are encouraged by the findings of meaningful impact on several different aspects of FA disease progression and morbidity over 72 weeks," said Matthew B. Klein, M.D., Chief Executive Officer, PTC Therapeutics. "Given the signals of clinical benefit, vatiquinone's well-established safety profile in children, and the unmet medical need for pediatric patients with FA, we look forward to discussing a potential path to registration with regulatory authorities."

The MOVE-FA trial enrolled 146 pediatric and adult patients, the majority of which were under 18 years of age. The mean placebo corrected change in the mFARS score in the primary analysis population was 1.6 (p=0.14). Notably, there was significant benefit recorded in the bulbar and upright stability subscales (nominal p values of 0.044 and 0.021, respectively) which are regarded as reflective of key aspects of disease morbidity and predictive of loss of time to loss of ambulation. In addition, a statistically significant difference was recorded on the Modified Fatigue Scale, which captures one of the most impactful sources of disease morbidity (nominal p value of 0.025). On a prespecified sensitivity analysis of subjects who completed 72 weeks on assigned therapy, the placebo corrected difference was 2.31, which represents a 75% slowing in disease progression over 72 weeks. Overall, vatiquinone was demonstrated to be well tolerated, adding to the large volume of safety data collected in other pediatric clinical studies.

Conference Call and Webcast Details:
PTC will hold a conference call at 5:00 pm EDT today to discuss this news. To access the call by phone, please click here to register and you will be provided with dial-in details. To avoid delays, we recommend participants dial in to the conference call 15 minutes prior to the start of the call. The webcast conference call can be accessed on the Investor section of the PTC website at https://ir.ptcbio.com/events-presentations. A replay of the call will be available approximately two hours after completion of the call and will be archived on the company's website for 30 days following the call.

About the MOVE-FA Clinical Trial
The Phase 3 registration-directed trial in Friedreich Ataxia patients, called MOVE-FA, is a randomized, placebo-controlled 72-week trial with the primary endpoint being change in the modified Friedreich Ataxia Rating Scale (mFARS) score. The mFARS is a clinical assessment that measures disease progression, namely swallowing and speech, upper and lower limb coordination, and upright stability. The key secondary endpoint is the change from baseline in activities of daily living as assessed by the FA-Activities of Daily Living (ADL) scale. Patients who completed the placebo portion of the trial will be trial are eligible to enroll in an open label 24-week extension.

About Vatiquinone
PTC is developing vatiquinone, a potential treatment for Friedreich ataxia based on our Bio-e platform. Vatiquinone is a small molecule, first-in-class selective inhibitor of 15-Lipoxygenase (15-LO), an enzyme that is a key regulator of the energetic and oxidative stress pathways that are disrupted in Friedreich ataxia. Inhibition of 15-LO helps to alleviate the consequences of mitochondrial dysfunction and oxidative stress, ultimately preventing ferroptosis and aiding neuronal survival.[1,2,3] Vatiquinone has been evaluated in a number of clinical studies and has demonstrated an impact on mortality risk and a number of neurological and neuromuscular disease symptoms.

About Friedreich Ataxia
Friedreich ataxia (FA) is a rare, physically debilitating, life-shortening, neuromuscular disorder that mainly affects the central nervous system and the heart.[4] It is the most common hereditary ataxia (abnormal, uncoordinated movements) and is usually caused by a single genetic defect in the frataxin (FXN) gene that leads to reduced production of frataxin, a mitochondrial protein that is important for cellular metabolism and energy production.[4,5] Decreased frataxin levels are associated with mitochondrial iron accumulation and increased oxidative stress, which can lead to cell death through ferroptosis.[6,7,8]

Symptoms include progressive loss of coordination and muscle strength leading to poor balance and coordination, difficulty speaking, swallowing, and breathing, curvature of the spine, serious heart conditions, diabetes, and hearing and vision impairment.[9,10] The severity of symptoms and speed of progression varies between people and some symptoms may not be evident in all. Friedreich ataxia is usually diagnosed in childhood or adolescence.[5,11] Approximately 25,000 people have Friedreich ataxia globally.

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 innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. PTC's mission is to provide access to best-in-class treatments for patients who have little to no treatment options. PTC's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. PTC believes this allows it to maximize value for all its stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, Instagram, LinkedIn and Twitter at @PTCBio.

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Forward-Looking Statement

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this press release, other than statements of historic fact, are forward-looking statements, including statements with respect to 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, future operations, future financial position, future revenues, 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; expectations with respect to the COVID-19 pandemic and related response measures and their effects on PTC's business, operations, clinical trials, regulatory submissions and approvals, and PTC's collaborators, contract research organizations, suppliers and manufacturers; 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; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; and the factors discussed in the "Risk Factors" section of PTC's most recent 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.

References:

1. Hinman A, et al. PLoS one. 2018;13:e0201369.
2. PTC Therapeutics. EPI-743 Pre-Clinical Data Deck.
3. Shrader WD, et al. Bioorg Med Chem Lett. 2011;21:3693–3698.
4. Lynch DR, Farmer JM, Balcer LJ, et al. Arch Neurol 2002;59(5):743–747.
5. Campuzano V, Montermini L, Lutz Y, et al. Hum Mol Genet 1997;11(6):1771–1780.
6. Campuzano V, et al. Hum Mol Genet. 1997;6:1771–1780.
7. Cook A, Giunti P. Br Med Bull. 2017;124:19–30.
8. Pandolfo M, Hausmann L. J Neurochem. 2013;126:142–146.
9. Bürk K. Cerebellum Ataxias 2017;4:4.
10. Cook A, Giunti P. Br Med Bull 2017;124(1):19–30.
11. Delatycki MB, Williamson R, Forrest SM. J Med Genet 2000;37(1):1–8.