Results Add to Body of Evidence Confirming EMFLAZA®'s Benefit Over Prednisone

June 24, 2021

- Data presented at the 2021 Parent Project Muscular Dystrophy Annual Conference -

SOUTH PLAINFIELD, N.J., June 24, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today presented pooled placebo data from multiple studies in which the results confirmed the clinical benefit of EMFLAZA® (deflazacort) over prednisone for the treatment of Duchenne muscular dystrophy (DMD). The results demonstrated that DMD patients on daily EMFLAZA performed better on key measures of physical function including the six-minute-walk test, key timed function tests, and the North Star Ambulatory Assessment than patients taking daily prednisone.

"The results of this analysis adds to the body of evidence and clearly demonstrates the clinical benefit of EMFLAZA over prednisone," stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "Importantly, these results also reflect the benefit that patients experience in the real-world setting."

Dr. Craig McDonald, University of California Davis School of Medicine added, "Importantly, the early initiation of EMFLAZA translates to better overall outcomes for patients which is more evident once patients have entered into the decline phase of the disease."

PTC also presented oral and poster presentations reporting long-term, real-world and clinical data, including its role in slowing DMD disease progression and improving ambulatory function over time. Additional data comparing the efficacy of EMFLAZA to prednisone on selected markers of disease progression in subgroups of DMD patients were presented. Also highlighted were results from a real-world study of patients who switched from prednisone to EMFLAZA following the Food and Drug Administration (FDA) approval.

About EMFLAZA®(deflazacort)

EMFLAZA® is indicated for the treatment of Duchenne muscular dystrophy in patients two years of age and older.

IMPORTANT SAFETY INFORMATION

Contraindication: Do not use if you are allergic to deflazacort or any of the inactive ingredients in EMFLAZA.

Do not stop taking EMFLAZA, or change the amount you are taking, without first checking with your healthcare provider, as there may be a need for gradual dose reduction to decrease the risk of adrenal insufficiency and steroid "withdrawal syndrome". Acute adrenal insufficiency can occur if corticosteroids are withdrawn abruptly and can be fatal. A steroid "withdrawal syndrome," seemingly unrelated to adrenocortical insufficiency, may also occur following abrupt discontinuance of corticosteroids. For patients already taking corticosteroids during times of stress, the dosage may need to be increased.

- Hyperglycemia: Corticosteroids can increase blood glucose, worsen pre-existing diabetes, predispose those on long-term treatment to diabetes mellitus, and may reduce the effect of anti-diabetic drugs. Monitor blood glucose at regular intervals. For patients with hyperglycemia, anti-diabetic treatment should be initiated or adjusted accordingly.
- Increased Risk of Infection: Tell your healthcare provider if you have had recent or ongoing infections or if you have recently received a vaccine or are scheduled for a vaccination. Seek medical advice at once should you develop fever or other signs of infection, as some infections can potentially be severe and fatal. Avoid exposure to chickenpox or measles, but if you are exposed, medical advice should be sought without delay.
- Alterations in Cardiovascular/Kidney Function: EMFLAZA can cause an increase in blood pressure, salt and water retention, or a decrease in your potassium and calcium levels. If this occurs, dietary salt restriction and potassium supplementation may be needed.
- Behavioral and Mood Disturbances: There is a potential for severe behavioral and mood changes with EMFLAZA and you should seek medical attention if psychiatric symptoms develop.
- Effects on Bones: There is a risk of osteoporosis or decrease in bone mineral density with prolonged use of EMFLAZA, which can potentially lead to vertebral and long bone fractures.
- Effects on Growth and Development: Long-term use of corticosteroids, including EMFLAZA may slow growth and development in children.
- Ophthalmic Effects: EMFLAZA may cause cataracts or glaucoma and you should be monitored if corticosteroid therapy is

continued for more than 6 weeks.
- Vaccination: The administration of live or live attenuated vaccines is not recommended. Killed or inactivated vaccines may be administered, but the responses cannot be predicted.
- Serious Skin Rashes: Seek medical attention at the first sign of a rash.
- Drug Interactions: Certain medications can cause an interaction with EMFLAZA. Tell your healthcare provider of all the medicines you are taking, including over-the-counter medicines (such as insulin, aspirin or other NSAIDS), dietary supplements, and herbal products. Alternate treatment, dosage adjustment, and/or special test(s) may be needed during the treatment.

Common side effects that could occur with EMFLAZA include: Facial puffiness or Cushingoid appearance, weight increased, increased appetite, upper respiratory tract infection, cough, frequent daytime urination, unwanted hair growth, central obesity, and colds.

Full Prescribing Information can be found here.

You may report side effects to ProPharma Group at 1–866–562–4620 or drugsafety@propharmagroup.com.

You may report side effects to FDA at 1–800–FDA–1088 or www.fda.gov/medwatch.

About Duchenne muscular dystrophy
Primarily affecting males, Duchenne muscular dystrophy (Duchenne) is a rare and fatal genetic disorder that results in progressive muscle weakness from early childhood and leads to premature death in the mid-twenties due to heart and respiratory failure. It is a progressive muscle disorder caused by the lack of functional dystrophin protein. Dystrophin is critical to the structural stability of all muscles, including skeletal, diaphragm, and heart muscles. Patients with Duchenne can lose the ability to walk (loss of ambulation) as early as age ten, followed by loss of the use of their arms. Duchenne patients subsequently experience life-threatening lung complications, requiring the need for ventilation support, and heart complications in their late teens and twenties.

More information regarding Duchenne is available through the Muscular Dystrophy Association and the Parent Project Muscular Dystrophy. Additionally, information and resources are available at www.duchenneandyou.com.

About PTC
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. The Company's strategy is to leverage its strong scientific expertise and global commercial infrastructure to maximize value for its patients and other stakeholders. 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 commercialization of its products and product candidates; PTC's strategy, 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; 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 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, including Emflaza.

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.

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