PTC Presents Results from a Real-World Study of Steroid Switching in the Treatment of Patients with Dystrophinopathies

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- Desire to slow disease progression and tolerability issues were the primary reasons for switching from prednisone to EMFLAZA® (deflazacort) -

- Delayed disease progression and improved benefit-risk reported during the 6-month average follow up after switching to EMFLAZA -

SOUTH PLAINFIELD, N.J., March 15, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced results from a real-world study of patients with Duchenne muscular dystrophy (DMD) and Becker muscular dystrophy (BMD) who switched from prednisone to EMFLAZA® (deflazacort) following the FDA approval. The primary reason the majority of patients chose to switch to EMFLAZA was a desire to delay disease progression to improve the benefit and tolerability. During the 6-months average follow-up after switching, physician-reported outcomes were consistent with EMFLAZA addressing the primary reasons for switching. Data were presented at the 2021 Muscular Dystrophy Association (MDA) Virtual Clinical and Scientific Conference.

"We're encouraged by the clinical benefit exhibited by patients taking EMFLAZA in this real-world analysis," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics. "This data supports the results that we saw in patients on corticosteroids in the placebo arms of multiple Duchenne clinical trials and what we have heard from the Duchenne community. We are committed to providing access to clinically differentiated treatments for patients with high unmet need."

The real-world chart review was conducted between February 2017 and December 2018 by 55 neurologists who contributed data for 92 male patients with DMD or BMD that switched from prednisone to EMFLAZA. Physicians ranked desire to slow disease progression, tolerability and caregiver or patient request as the most common motivations for switching from prednisone to EMFLAZA. Of the 92 patients, 62 individuals had DMD (mean age of 6.2 years at switch) and 30 individuals had BMD (mean age of 20.1 years at switch). To the extent recorded in medical records, Clinical Global Impression (CGI) of Improvement scores were collected at the clinic visit closest to steroid switching during prednisone treatment, and the last available clinic visit during EMFLAZA treatment. The average treatment duration was 3.3 years for prednisone and 6 months for EMFLAZA prior to the chart extraction.

Results found that during the 6-month average follow-up after switching, among charts with CGI recorded, most patients' disease progression improved or stabilized during steroid treatment, with a shift towards improvement after switching. Switching was reported as "very" or "somewhat" effective at addressing primary reasons in 95% of patients with DMD and 90% of patients with BMD.

Commonly recorded adverse events during prednisone and EMFLAZA treatment included weight gain, Cushingoid appearance, increased appetite, central obesity, and fluid retention.

"Results from the real-world chart review presented today support the potential of EMFLAZA to alter the natural history of Duchenne muscular dystrophy, demonstrating its capability to slow progression of the disease and improve benefit-risk," said Dr. Susan Apkon, Investigator, Chief Pediatric Rehabilitation, Vice-Chair Department of Physical Medicine and Rehabilitation, Fischahs Chair in Pediatric Rehabilitation, Children's Hospital of Colorado. "We believe that EMFLAZA will continue to provide DMD patients a safe and effective treatment option."

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.

Please see the accompanying full Prescribing Information

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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