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PTC Therapeutics Completes Acquisition of Emflaza™ for the Treatment of Duchenne Muscular Dystrophy in the U.S.

SOUTH PLAINFIELD, N.J., April 20, 2017 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced it has completed its acquisition of all rights to Emflaza™ (deflazacort) for the treatment of Duchenne muscular dystrophy (DMD) in the U.S. Execution of the asset purchase agreement setting forth the terms of the acquisition was announced on March 16, 2017.

"We are pleased the acquisition was completed ahead of schedule, following early conclusion of the antitrust review period," stated Stuart Peltz, Ph.D., chief executive officer, PTC Therapeutics, Inc. "We've been engaging with key stakeholders in the DMD community to understand their needs and are working to make Emflaza commercially available as soon as possible. PTC is committed to bringing this important therapy to patients with DMD. Our goal is to enable access for eligible patients irrespective of insurance status, and we look forward to discussing commercial launch details on our upcoming quarterly earnings call."

Financial terms of the acquisition include a total upfront consideration of \$140 million paid to Marathon Pharmaceuticals. Marathon is also entitled to receive payments from PTC based on annual net sales of Emflaza beginning in 2018, which PTC expects will range as a percentage of net sales between the low to mid-20s on a blended average basis. In addition, Marathon has the opportunity to receive a single \$50 million sales-based milestone.

About Duchenne Muscular Dystrophy

Primarily affecting males, Duchenne muscular dystrophy (DMD) 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 skeletal, diaphragm, and heart muscles. Patients with DMD can lose the ability to walk as early as age ten, followed by loss of the use of their arms. DMD 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 DMD 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 Therapeutics

PTC is a global biopharmaceutical company focused on the discovery, development, and commercialization of novel medicines using our expertise in RNA biology. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders and oncology. PTC has discovered all of its compounds currently under development using its proprietary technologies. Since its founding nearly 20 years ago, PTC's mission has focused on developing treatments to fundamentally change the lives of patients living with rare genetic disorders. The company was founded in 1998 and is headquartered in South Plainfield, New Jersey. For more information on the company, please visit our website www.ptcbio.com.

About Emflaza

EMFLAZA™ is indicated for the treatment of Duchenne™ muscular dystrophy in patients 5 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.

- 1 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.

- 1 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.
- 1 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.
- 1 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.
- 1 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.
- 1 Effects on Growth and Development: Long-term use of corticosteroids, including EMFLAZA may slow growth and development in children.
- 1 Ophthalmic Effects: EMFLAZA may cause cataracts or glaucoma and you should be monitored if corticosteroid therapy is continued for more than 6 weeks.
- 1 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.
- 1 Serious Skin Rashes: Seek medical attention at the first sign of a rash.
- 1 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.

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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, other than those of historical fact, contained in this release are forward-looking statements, including statements related to PTC's expectations with respect to the future commercial availability of, and access to, Emflaza; PTC's expectations with respect to contingent payments to Marathon based on annual net sales; the future expectations, plans and prospects for PTC; PTC's strategy, future operations, future financial position, future revenues or projected costs; and the objectives of management. Other forward-looking statements may be identified by the words "look forward", "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: PTC's preparations for a commercial launch of Emflaza; PTC's ability to realize the anticipated benefits of the acquisition of Emflaza, including the possibility that the expected benefits from the acquisition will not be realized or will not be realized within the expected time period; negative effects of this announcement on the market price of PTC's common stock; significant transaction costs, unknown liabilities, the risk of litigation and/or regulatory actions related to the acquisition of Emflaza, as well as other 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 Translarna™ (ataluren) and Emflaza; 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 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 Translarna or Emflaza.

The forward-looking statements contained herein represents 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.

To view the original version on PR Newswire, visit:<http://www.prnewswire.com/news-releases/ptc-therapeutics-completes-acquisition-of-emflaza-for-the-treatment-of-duchenne-muscular-dystrophy-in-the-us-300443091.html>

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