March 16, 2017

PTC Therapeutics Announces Agreement to Acquire Emflaza™ for the Treatment of Duchenne Muscular Dystrophy in U.S.

- Emflaza is the first treatment approved in the U.S. for DMD patients 5 years and older, regardless of genetic mutation
- Aligns with PTC's mission and commitment to the DMD community
- $140 million upfront consideration (comprised of cash and PTC common stock) plus potential contingent payments based on net sales and single milestone payment
- PTC to host investor conference call today, March 16, 2017 at 8:30 am ET

SOUTH PLAINFIELD, N.J., March 16, 2017 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced it has entered into an asset purchase agreement with Marathon Pharmaceuticals, LLC to acquire all rights to Emflaza™ (deflazacort). Emflaza is the first treatment approved in the United States for all Duchenne muscular dystrophy (DMD) patients five years and older, regardless of their genetic mutation. DMD is a rare and fatal genetic disorder that results in progressive muscle weakness from early childhood and leads to premature death due to heart and respiratory failure. Emflaza aligns with PTC's mission to bring therapies to patients who have rare diseases with limited or no treatment options.

"With our nearly 20-year commitment to the Duchenne community, it is deeply meaningful for us to bring this critical therapy to U.S. patients," said Stuart W. Peltz, Ph.D., chief executive officer of PTC Therapeutics, Inc. "We believe Emflaza is a disease-modifying therapy that has been shown to slow disease progression. In keeping with PTC's mission, we are excited to work with the community to raise the standard of care for DMD patients."

DMD treatment guidelines recommend steroids as a foundational component of the standard of care. Emflaza reduces inflammation, which is critical to preserving muscle function and delaying disease progression. It received FDA approval on February 9, 2017 and has the potential to benefit many DMD patients in the U.S.

"Our goal has always been to ensure Emflaza is studied, understood and available to any Duchenne patient who needs it, and we determined that this transaction is the best path for ensuring that will happen," said Jeff Aronin, president and CEO, Marathon Pharmaceuticals, LLC. "Now that we have achieved FDA approval of Emflaza, the focus can turn to ensuring patients have access to this important therapy. PTC is well known by the Duchenne community and is ideally positioned to achieve this shared goal."

"Based on our long-standing experience with DMD and strong partnership with the community, we believe PTC is uniquely positioned to launch Emflaza in the U.S.," added Mark Rothera, PTC's chief commercial officer. "We are finalizing our commercialization plans and intend to share more information after the transaction closes."

Transaction Overview
Under the terms of the asset purchase agreement, Marathon will receive total upfront consideration of $140 million upon closing of the transaction, comprised of approximately $75 million in cash and approximately $65 million in PTC common stock, subject to a maximum 6.9 million share limit (with any shortfall to be made whole with additional cash consideration). 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.

The transaction is expected to be accretive to both earnings and cash flow beginning in 2018. The transaction is expected to close in the second quarter of 2017, subject to customary closing conditions, including receipt of clearance under the Hart-Scott-Rodino Act.

Advisors
Evercore is acting as financial advisor to PTC on the transaction. Wilmer Hale is acting as legal advisor to PTC.

Conference Call and Webcast
PTC will host a call today at 8:30 am ET, which can be accessed by dialing (877) 303-9216 (domestic) or (973) 935-8152

(international) and providing the passcode 61526711. A live, listen-only webcast of the conference call can be accessed on the investor relations section of the PTC website at www.ptcbio.com. An accompanying slide presentation will be posted at 8:15 AM on the investor relations section of the PTC website at www.ptcbio.com. A webcast 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 two weeks.

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' 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 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, the more severe form of the disorder, 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.

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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 closing of its planned acquisition of all rights to Emflaza™ (deflazacort), or the "planned acquisition"; the potential financial impact and benefits to PTC of the planned acquisition, including with respect to a potential launch of Emflaza and PTC's expectations with respect to contingent payments to Marathon based on annual net sales; the potential advantages of Emflaza; 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: satisfaction of the conditions to closing the planned acquisition (including the failure to obtain necessary regulatory approvals) in the anticipated timeframe or at all; PTC's ability to realize the anticipated benefits of the planned acquisition, including the possibility that the expected benefits from the planned 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 planned acquisition; 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 most recent Quarterly Report on Form 10-Q or 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 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.

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/ptc-therapeutics-announces-agreement-to-acquire-emflaza-for-the-treatment-of-duchenne-muscular-dystrophy-in-us-300424705.html

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