

**UNITED STATES**  
**SECURITIES AND EXCHANGE COMMISSION**  
WASHINGTON, D.C. 20549

**FORM 8-K**

**CURRENT REPORT**

**Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934**

Date of Report (Date of earliest event reported): **April 20, 2017**

**PTC THERAPEUTICS, INC.**

(Exact Name of Company as Specified in Charter)

**Delaware**  
(State or Other Jurisdiction  
of Incorporation)

**001-35969**  
(Commission  
File Number)

**04-3416587**  
(IRS Employer  
Identification No.)

**100 Corporate Court**  
**South Plainfield, NJ**  
(Address of Principal Executive Offices)

**07080**  
(Zip Code)

Company's telephone number, including area code: **(908) 222-7000**

**Not applicable**

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

## Item 2.01. Completion of Acquisition or Disposition of Assets.

On April 20, 2017, PTC Therapeutics, Inc. (the “Company”), completed the previously announced acquisition of all rights to Emflaza™ (deflazacort) (the “Transaction”). The Transaction was completed pursuant to an asset purchase agreement, dated March 15, 2017, as amended on April 20, 2017 to amend certain post-closing obligations (as amended, the “Asset Purchase Agreement”) by and between the Company and Marathon Pharmaceuticals, LLC (“Marathon”).

The assets (the “Assets”) acquired by the Company in the Transaction include intellectual property rights related to Emflaza, inventories of Emflaza, certain contractual rights related to Emflaza, including the contractual rights described herein, among others, and certain other assets related to Emflaza.

The Company assumed certain liabilities and obligations in the Transaction, including the contractual obligations described herein and various other liabilities and obligations arising out of, or relating to, the Assets.

Upon the closing of the Transaction, the Company paid to Marathon total upfront consideration of approximately \$140 million. The total upfront consideration was comprised of \$75 million in cash, funded through cash on hand, and 6,683,598 shares of the Company’s common stock. The number of shares of common stock issued at closing was determined by dividing \$65 million by the volume weighted average price per share of the Company’s common stock on the Nasdaq Stock Market for the 15 trading day period ending on the third trading day immediately preceding the closing. As previously disclosed, Marathon will be entitled to receive contingent payments from the Company based on annual net sales of Emflaza beginning in 2018, up to a specified aggregate maximum amount for such payments, and a single \$50 million sales-based milestone, in each case subject to the terms and conditions of the Asset Purchase Agreement. The Company expects that the contingent payments will, on a blended average basis, range in percentages of net sales between the low to mid-twenties.

The above description of the Asset Purchase Agreement is a summary only and is qualified in its entirety by reference to the terms of the Asset Purchase Agreement. A copy of the Asset Purchase Agreement was previously filed as Exhibit 2.1 to the Company’s Current Report on Form 8-K filed on March 16, 2017. A copy of the amendment to the Asset Purchase Agreement is attached hereto as Exhibit 2.1.

*Faes Agreement.* Upon the closing of the Transaction, Marathon assigned to the Company all rights, and the Company assumed all obligations, under the Exclusive License and Supply Agreement, dated as of May 12, 2015 and amended as of November 1, 2015, between Marathon and Faes Farma, S.A. (“Faes”) (the “Faes Agreement”).

Pursuant to the assignment and assumption of the Faes Agreement, Faes grants the Company a license in, to and under certain intellectual property, know-how and technology of Faes (as per the definition of “Licensed Assets” in the Faes Agreement) related to the deflazacort oral suspension pharmaceutical product as owned and currently supplied by Faes in certain markets in the world (the “Faes Product”, together with the Licensed Assets, the “Faes Assets”) to research, develop, obtain regulatory approval for, market, promote, distribute, sell, use, commercialize and, solely as expressly permitted under the Faes Agreement, manufacture the deflazacort oral suspension pharmaceutical products developed by the Company (or developed by Marathon prior to the closing of the Transaction) based upon and utilizing the Faes Assets and approved by the FDA for the treatment in humans of Duchenne muscular dystrophy or other indications in and for the territory of the United States (the “Deflazacort Suspension Product”) as set forth in the Faes Agreement. The license is exclusive during the Manufacturing Term and non-exclusive thereafter. The Manufacturing Term is defined as (a) the Initial Manufacturing Term, commencing on the date of the Faes Agreement and ending on the 20th anniversary thereof, together with (b) any Renewed Manufacturing Terms, which are automatic renewals of the Manufacturing Term for 10-year periods, subject to earlier termination by the parties in accordance with the terms of the Faes Agreement.

Subject to the terms and conditions of the Faes Agreement, Faes reserves all rights under the Licensed Assets to research, develop and manufacture the Faes Product for any and all purposes both inside the United States (with the Company’s prior written consent, not to be unreasonably withheld) or outside the United States. Faes also retains the right under the Faes Agreement to research, develop, make and have made, use, market, distribute, offer for sale, sell and import the Faes Product for any and all purposes outside of the United States.

Pursuant to the Faes Agreement, the parties agree to work collaboratively to conduct manufacturing research and development work relating to Deflazacort Suspension Products in accordance with the terms of the Faes Agreement.

Pursuant to the terms of the Faes Agreement, during the initial period commencing on the FDA approval date of Emflaza and continuing until the seventh anniversary of such date, Faes shall be the exclusive supplier to the Company of any finished Deflazacort Suspension Product for use in the United States. During the Manufacturing Term, Faes and its affiliates may only supply deflazacort oral suspension products for use in the United States to the Company. The Faes Agreement provides that Faes shall supply finished Deflazacort Suspension Products to the Company at a specified per unit supply price.

Pursuant to the terms of the Faes Agreement, during the initial period commencing on the FDA approval date of Emflaza and continuing until the seventh anniversary of such date, the Company is obligated to pay to Faes royalty payments, on a quarterly basis, based on a percentage (ranging from low to middle-low double digits) of, or a fixed payment with respect to, the Company's annual net sales of Deflazacort Suspension Product in the United States as specified in the Faes Agreement, subject to reduction in accordance with the terms of the agreement. The royalty payments during such initial period are subject to a minimum aggregate annual payment ranging from €0.5 million to €1.5 million per year.

The Faes Agreement may be terminated at any time upon the mutual agreement of the parties or upon a party's material breach of its material obligations under the Faes Agreement, subject to notice and cure periods and other procedures set forth in the Faes Agreement. The Faes Agreement also contains provisions relating to, among other things, representations and warranties of the parties, purchase orders, pricing, payment terms, costs and expenses, regulatory matters, audit and reporting rights, intellectual property matters, limitations on assignment, confidentiality, indemnification and dispute resolution.

*Alcami Agreement.* Upon the closing of the Transaction, Marathon assigned to the Company all rights, and the Company assumed all obligations, under the Commercial Manufacturing Agreement, dated as of September 18, 2015 and amended as of September 18, 2016 and January 6, 2017, between Marathon and Alcami Corporation, f/k/a AAI Pharma Services Corp. ("Alcami") (the "Manufacturing Agreement").

Pursuant to the assignment and assumption of the Manufacturing Agreement, the Company agrees to exclusively purchase from Alcami, and Alcami agrees to exclusively manufacture and supply, all of the Company's requirements for deflazacort tablets as well as secondary packaging of pre-filled deflazacort oral suspension bottles (the "Manufacturing Products") for the commercialization of such Manufacturing Products in the United States, and any other jurisdiction in which the Company may commercialize the Manufacturing Products, pursuant to the terms of the Manufacturing Agreement. The initial term of the Manufacturing Agreement continues for a period of five years commencing on the first date of shipment of commercial product from Alcami's site with respect to each Manufacturing Product, subject to automatic two-year renewal periods with respect to each Manufacturing Product unless terminated by the parties pursuant to the terms of the Manufacturing Agreement. Pursuant to the terms of the Manufacturing Agreement, the Company is required to supply to Alcami the active pharmaceutical ingredient for Manufacturing Products, which shall remain the sole property of the Company.

The Manufacturing Agreement may be terminated as described above or upon a party's material breach of its obligations under the Manufacturing Agreement in addition to other specified events, including with respect to governmental actions, certain circumstances relating to individual products, force majeure or bankruptcy proceedings, in each case subject to notice, cure periods and other conditions set forth in the Manufacturing Agreement. The Manufacturing Agreement also contains provisions relating to, among other things, representations and warranties of the parties, purchase orders, pricing (including price changes), payment terms, costs and expenses, regulatory matters, non-conforming products, recalls, intellectual property matters, limitations on assignment, confidentiality, indemnification and dispute resolution provisions.

### **Item 3.02. Unregistered Sales of Equity Securities.**

The description of the common stock consideration set forth in Item 2.01 above is incorporated herein by reference. In connection with the closing of the Transaction, the Company issued to Marathon the common stock consideration pursuant to an exemption from registration afforded by Section 4(a)(2) of the Securities Act of 1933, as amended (the "Securities Act"), and/or Regulation D promulgated thereunder, based in part on Marathon's representations to the Company that it is an "accredited investor" as that term is defined under Rule 501(a) under the Securities Act.

### **Item 7.01. Regulation FD Disclosure.**

On April 20, 2017, the Company issued a press release in which it announced the closing of the Transaction. A copy of the press release is attached to this Current Report on Form 8-K (this "Report") as Exhibit 99.1 and is incorporated by reference into this Item 7.01.

The information set forth in or incorporated by reference into this Item 7.01, including Exhibit 99.1, shall not be deemed to be "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liability of that section, and shall not be incorporated by reference into any registration statement or other document filed under the Securities Act or the Exchange Act, except as shall be expressly set forth by specific reference in such filing.

### **Item 9.01. Financial Statements and Exhibits.**

#### **(a) Financial Statements of Business Acquired**

The financial statements of the acquired business required by this item have not been filed on this Report but will be filed by amendment not later than 71 calendar days after the date that this Report was required to be filed.

#### **(b) Pro Forma Financial Information**

The pro forma financial information required by this item has not been filed on this Report but will be filed by amendment not later than 71 calendar days after the date that this Report was required to be filed.

#### **(d) Exhibits**

See Exhibit Index attached hereto.

### **Cautionary Statement Concerning Forward Looking Statements**

This Report contains forward-looking statements addressing the Transaction and the other transactions contemplated in the Asset Purchase Agreement and other statements about future expectations, prospects, estimates and other matters that are dependent upon future events or developments. All statements, other than those of historical fact, contained in this Report are forward-looking statements, including statements related to the Company's expectations with respect to the future commercial availability of, and access to, Emflaza; the Company's expectations with respect to contingent payments to Marathon based on annual net sales; the future expectations, plans and prospects for the Company; the Company'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.

The Company'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 Company's preparations for a commercial launch of Emflaza; the Company's ability to realize the anticipated benefits of the Transaction, including the possibility that the expected benefits from the Transaction will not be realized or will not be realized within the expected time period; negative effects of the announcement of the Asset Purchase Agreement or the closing of the Transaction on the market price of the Company's common stock; significant transaction costs, unknown liabilities, the risk of litigation and/or regulatory actions related to the Transaction, 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 the Company'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 the Company's Annual Report on Form 10-K as well as any updates to these risk factors filed from time to time in the Company's other filings with the SEC. 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 the Company's views only as of the date of this Report and the Company does not undertake or plan to update or revise any such forward-looking statements to reflect actual



**AMENDMENT NO. 1 TO ASSET PURCHASE AGREEMENT**

This Amendment No. 1, dated as of April 20, 2017 (this "Amendment"), to the Asset Purchase Agreement (the "Agreement"), dated as of March 15, 2017, is made and entered into by and between PTC Therapeutics, Inc., a Delaware corporation ("Buyer"), and Marathon Pharmaceuticals, LLC, a Delaware limited liability company ("Seller"). Capitalized terms used, but not otherwise defined, in this Amendment shall have the meanings ascribed to them in the Agreement. Buyer and Seller may be referred to herein, together, as the "Parties" and, individually, as a "Party".

WHEREAS, Buyer and Seller have previously entered into the Agreement pursuant to which, among other things, Seller has agreed to sell, assign, transfer and deliver (or cause to be sold, assigned, transferred or delivered) to Buyer, and Buyer has agreed to purchase from Seller, the Acquired Assets, upon the terms and subject to the conditions set forth therein; and

WHEREAS, pursuant to Section 10.10 of the Agreement, Buyer and Seller desire to amend the Agreement upon the terms and subject to the conditions set forth herein.

**NOW, THEREFORE**, in consideration of the foregoing and other good and valuable consideration, the adequacy and receipt of which hereby are acknowledged, the Parties hereby agree as follows:

1. Section 1.4(xi) of the Agreement is hereby amended and restated to read in its entirety as follows:

“(xi) Books and Records. Copies of all Product Books and Records (to be delivered at Closing or within two (2) Business Days thereafter); and”

2. Sections 5.16(b) and (c) of the Agreement are hereby amended to read in their entirety as follows:

“(b) Product Returns. Buyer shall be financially responsible for all costs associated with any customer or wholesaler returns of all Products, regardless of whether they bear Seller’s NDC numbers or Buyer’s NDC numbers, including, but not limited to, Product included in the Seller Labeled Inventory, Retained Inventory sold by Seller after the Closing Date under and in accordance with Section 5.17 of this Agreement and Product bearing Buyer’s NDC numbers, which returns shall be made in accordance with Buyer’s returned goods policy at the time of such return, unless otherwise mutually agreed by Buyer and Seller; and Buyer shall be responsible for processing all such returns.

(c) Government Rebates. Buyer shall be solely responsible for and with respect to all rebates relating to the sale of the Products pursuant to any government (federal or state) drug rebate programs (including, but not limited to, Medicaid, 340B Drug Discount Program, and Medicare gap coverage plans) ("Government Rebates"), regardless of

whether they bear Seller's NDC numbers or Buyer's NDC numbers (including, but not limited to, Retained Inventory sold by Seller after the Closing Date under and in accordance with Section 5.17 of this Agreement), which includes, but is not limited to, all obligations to make any and all submissions, certifications, and payments (including, but not limited to, any penalties or late fees) related to such Government Rebates. Buyer shall: (A) ensure that Seller receives all Government Rebate-related correspondence or other notifications from the applicable Governmental Authority related to such Government Rebates; and (B) indemnify and hold harmless Seller from all liability arising from or with respect to such Government Rebates."

3. Section 5.16(e) of the Agreement is hereby amended and restated to read in its entirety as follows:

"(e) Chargeback Claims.

(i) Buyer shall be solely responsible for and with respect to all chargeback claims (the "Chargeback Claims") relating to the sale of the Products, regardless of whether they bear Seller's NDC numbers or Buyer's NDC numbers (including, but not limited to, Retained Inventory sold by Seller after the Closing Date under and in accordance with Section 5.17 of this Agreement).

(ii) Within thirty (30) Days after the Closing Date, Buyer shall (1) (aa) apply to the VA to approve the addition of the Products (including Seller Labeled Inventory) to Buyer's Master Agreement/Pharmaceutical Pricing Agreement/Federal Supply Schedule Contract ("FSS") or (bb) apply to obtain an interim FSS contract in Buyer's (or its Affiliate's) name for the Products (including Seller Labeled Inventory); (2) as applicable consistent with Buyer's commercialization plans, apply to obtain an interim Medicaid Drug Rebate Program Agreement in Buyer's (or its Affiliates') name with the Centers for Medicare and Medicaid Services; and (3) as applicable consistent with Buyer's commercialization plans, apply to obtain a 340B Pharmaceutical Pricing Agreement in Buyer's (or its Affiliates') name with the Health Resources and Services Administration."

4. Section 5.17 of the Agreement is hereby amended and restated to read in its entirety as follows:

"5.17 Buyer Launch. Buyer agrees that it will Launch the Products in the United States, and commercialize and sell the Products into the commercial channel, on or prior to May 12, 2017 (the "Agreed Upon Buyer Launch Date"); provided, however, that, in the event Buyer fails to so Launch the Products (and commercialize and sell the Products into the commercial channel) in the United States on or prior to the Agreed Upon Buyer Launch Date, Buyer acknowledges and agrees that Seller shall be expressly authorized and empowered hereunder to Launch the Products in the United States using the Retained Inventory until such time as Buyer Launches the Products in the United States, and, in connection therewith, Buyer hereby grants to Seller, and Seller hereby accepts, a non-exclusive, non-transferable, non-sublicensable, license in the United States under the Acquired Assets and the Assigned Contracts to so Launch the Products in the United States

and to commercialize and sell the Retained Inventory for its own account in the United States, including, the right to sell the Retained Inventory to US Bioservices Corporation or another specialty distributor selected by Seller (“US Bio”) for distribution into the commercial channel; provided, further, that Seller shall pay to Buyer with respect to such Product sales an amount equal to sixty percent (60%) of the Profit Share. For purposes of this Section 5.17, “Profit Share” is defined as the gross sales amount invoiced by Seller with respect to the sale of the Product to US Bio hereunder, less a standard and customary “prompt pay” discount, less the applicable cost of goods sold and less any out-of-pocket expenses. Except for such Profit Share, such license shall be fully-paid up and royalty free.”

5. Section 5.18 of the Agreement is hereby amended by deleting the phrase “[W]ithin twenty (20) days following the Closing,” in the first and last sentence thereof and replacing it with the phrase “[O]n or prior to May 15, 2017,”.

6. Section 9.1 of the Agreement is hereby amended by inserting the following definition in alphabetical order therein:

“Retained Inventory” means the Seller Labeled Inventory listed on Schedule 5 attached hereto.”

7. Section 9.1(a)(ii) of the Agreement is hereby amended and restated to read in its entirety as follows:

“all Inventories, wherever located, including consignment Inventory and Inventory held or order or in transit, but expressly excluding the Retained Inventory; provided, however, (xx) that in the event Buyer Launches the Products in the United States (and commercializes and sells the Products into the commercial channel in the United States) on or prior to the Agreed Upon Buyer Launch Date as provided for in Section 5.17, the Retained Inventory shall thereafter be deemed to constitute an “Acquired Asset” for purposes of this Agreement, and (yy) that in the event Seller Launches the Products in the United States (and commercializes and sells the Products into the commercial channel in the United States) after the Agreed Upon Buyer Launch Date as provided for in Section 5.17, and Buyer subsequently Launches the Products in the United States (and commercializes and sells the Products into the commercial channel in the United States), any remaining Retained Inventory held by Seller shall thereafter be deemed to constitute an “Acquired Asset” for purposes of this Agreement;”

8. The reference to “Section 2.10(a)(i) of the Seller Disclosure Schedule” in Section 9.1(e) of the Agreement (“Assigned Contracts”) is hereby deleted and replaced with “Section 2.11(a)(i) of the Seller Disclosure Schedule” and the Contracts listed on Schedule A attached hereto are added as “Assigned Contracts” for purposes of the Agreement, with the Parties expressly agreeing that (a) if the assignment of any such additional Assigned Contract requires the consent of a Third Party thereto, the Parties shall attempt to obtain such Third Party consent on or prior to the Closing, but obtaining such Third Party consent shall not constitute a closing condition for purposes of this Agreement, including, but not limited to, Sections 1.4(a)(vi) and 6.1(b) of the Agreement, and (b) if any such Third Party consent is not obtained on or prior to the Closing,

such applicable additional Assigned Contract shall constitute a “Deferred Item” as defined in, and subject to, Section 1.1(c) of the Agreement.

9. Schedule I to the Agreement is hereby amended and restated in its entirety to read in its entirety as set forth on Schedule I-1 or Schedule I-2 attached hereto, as applicable.

10. Set forth on Schedule 2 attached hereto is a list of domain names included in the Acquired Assets.

11. A new Schedule 5 (“Retained Inventory”) shall be attached to the Agreement, which Schedule 5 is attached hereto as Schedule 5 to this Amendment.

12. Notwithstanding anything to the contrary set forth herein or in the Agreement, including, without limitation, Section 1.4(b)(vi) thereof, the Parties hereby agree that Buyer’s delivery of a resale certificate with respect to the Inventories, in form and substance reasonably acceptable to Buyer, shall not be a condition to Closing; provided, that the Parties shall use their commercially reasonable efforts to cause such resale certificate to be delivered as soon as reasonably practicable following the Closing

13. This Amendment shall be governed in all respects, including validity, interpretation, and effect, by and construed in accordance with the internal Laws of the State of Delaware (including in respect of the statute of limitations or other limitations period applicable to any claim, controversy or dispute) without giving effect to any choice or conflict of Law provision or rule (whether of the State of Delaware or any other jurisdiction) that would cause the application of Laws of any jurisdictions other than those of the State of Delaware.

14. This Amendment may be signed in any number of counterparts, each of which shall be an original, with the same effect as if the signatures hereto were upon the same instrument. This Amendment shall become effective when each Party shall have received a counterpart hereof signed by the other Party. Until and unless each Party has received a counterpart hereof signed by the other Party hereto, this Amendment shall have no effect, and no Party shall have any right or obligation hereunder (whether by virtue of any other oral or written agreement or other communication). The exchange of a fully executed Amendment (in counterparts or otherwise) by electronic transmission in .PDF format or by facsimile shall be sufficient to bind the Parties to the terms and conditions of this Amendment.

15. Except as otherwise provided herein, the Agreement shall remain unchanged and in full force and effect.

16. From and after the execution of this Amendment by the Parties, any reference to the Agreement shall be deemed to be a reference to the Agreement as amended by this Amendment.

**[SIGNATURE PAGE FOLLOWS]**

IN WITNESS WHEREOF, the Parties have executed this Amendment as of the day and year first written above.

**PTC THERAPEUTICS, INC.**

By: /s/ Mark E. Boulding

Name: Mark E. Boulding

Title: Executive Vice President and Chief Legal Officer

**MARATHON PHARMACEUTICALS, LLC**

By: /s/ Patrick J. Morris

Name: Patrick J. Morris

Title: Executive Vice President, Legal Affairs, Mergers, Acquisitions and General Counsel

*[Signature Page to Amendment No. 1 to Asset Purchase Agreement]*



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

**SOUTH PLAINFIELD, N.J., April 20, 2017** – 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](http://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](http://www.ptcbio.com).

### **About Emlaza**

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.

- **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](mailto:drugsafety@propharmagroup.com).

You may report side effects to FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).

#### **For More Information:**

##### **Investors:**

Emily Hill

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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.