# UNITED STATES SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

## FORM 8-K/A

(Amendment No. 1)

## 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)

Delaware001-3596904-3416587(State or Other Jurisdiction<br/>of Incorporation)(Commission<br/>File Number)(IRS Employer<br/>Identification No.)

100 Corporate Court South Plainfield, NJ

07080

(Address of Principal Executive Offices) (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):

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

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. o

#### EXPLANATORY NOTE

This Amendment No. 1 on Form 8-K/A (this "Amendment") is being filed to provide the financial statements and pro forma financial information required by Item 9.01 (a) and (b) of Form 8-K with respect to the acquisition by PTC Therapeutics, Inc. (the "Company") of all rights to EMFLAZA<sup>TM</sup> (deflazacort) (the "EMFLAZA Business") from Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), which was completed on April 20, 2017, as disclosed in the Current Report on Form 8-K filed by the Company with the Securities and Exchange Commission on April 20, 2017 (the "Original 8-K"), which is hereby amended and supplemented by this Amendment. In accordance with the requirements of Item 9.01 (a)(4) and (b)(2) of Form 8-K, this Amendment is being filed within 71 calendar days of the date that the Original 8-K was required to be filed.

Except as provided herein, the disclosures contained in this Amendment have not been updated to reflect events, results or developments that have occurred since the filing of the Original 8-K. This Amendment should be read in conjunction with the Original 8-K.

## Item 9.01. Financial Statements and Exhibits.

- (a) Financial Statements of Business Acquired
  - (i) The audited financial statements of the EMFLAZA Business as of and for the years ended December 31, 2016 and 2015 and the independent auditors' report thereon are filed as Exhibit 99.2 hereto and are incorporated into this Item 9.01(a) by reference.
  - (ii) The unaudited financial statements of the EMFLAZA Business as of and for the three months ended March 31, 2017 are filed as Exhibit 99.3 hereto and are incorporated into this Item 9.01(a) by reference.
- (b) Pro Forma Financial Information

The unaudited pro forma combined financial statements of the Company are filed as Exhibit 99.4 hereto and are incorporated into this Item 9.01(b) by reference.

(d) Exhibits

See Exhibit Index attached hereto.

#### Signature

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this Amendment No. 1 on Form 8-K/A to be signed on its behalf by the undersigned hereunto duly authorized.

## PTC Therapeutics, Inc.

Date: July 3, 2017 By: /s/ Christine Utter

Name: Christine Utter

Title: Principal Financial Officer

## EXHIBIT INDEX

Exhibit No.	Description
2.1	Amendment to the Asset Purchase Agreement dated April 20, 2017 (1)
23.1	Consent of Deloitte & Touche LLP
99.1	Press Release, dated April 20, 2017, issued by PTC Therapeutics, Inc. (1)
99.2	Audited financial statements of the EMFLAZA Business as of and for the years ended December 31, 2016 and 2015 and the independent auditors' report thereon
99.3	Unaudited financial statements of the EMFLAZA Business as of and for the three months ended March 31, 2017
99.4	Unaudited pro forma combined statements of operations the year ended December 31, 2016 and for the three months ended March 31, 2017 and unaudited pro forma combined balance sheet as of March 31, 2017

(1) Previously filed.

## CONSENT OF INDEPENDENT AUDITORS

We consent to the incorporation by reference in Registration Statement No. 333-194323, No. 333-189962, No. 333-203485, No. 333-208830, No. 333-211997, and 333-215407 on Form S-8 of PTC Therapeutics, Inc. of our report dated May 10, 2017, relating to the financial statements of the Emflaza Business appearing in this Current Report on Form 8-K/A dated July 3, 2017 of PTC Therapeutics, Inc.

/s/ Deloitte & Touche LLP Chicago, Illinois July 3, 2017

## **Emflaza Business**

Financial Statements as of and for the Years Ended December 31, 2016 and 2015, and Independent Auditors' Report

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#### INDEPENDENT AUDITORS' REPORT

To the Board of Managers of Marathon Pharmaceutical Holdings, LLC:

We have audited the accompanying financial statements of the Emflaza Business (the "Business") (a wholly owned business of Marathon Pharmaceutical Holdings, LLC), which comprise the balance sheets as of December 31, 2016 and 2015, and the related statements of operations, comprehensive loss, net parent company deficit, and cash flows for the years then ended, and the related notes to the financial statements

## Management's Responsibility for the Financial Statements

Management is responsible for the preparation and fair presentation of these financial statements in accordance with accounting principles generally accepted in the United States of America; this includes the design, implementation, and maintenance of internal control relevant to the preparation and fair presentation of financial statements that are free from material misstatement, whether due to fraud or error.

## Auditors' Responsibility

Our responsibility is to express an opinion on these financial statements based on our audits. We conducted our audits in accordance with auditing standards generally accepted in the United States of America. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free from material misstatement.

An audit involves performing procedures to obtain audit evidence about the amounts and disclosures in the financial statements. The procedures selected depend on the auditor's judgment, including the assessment of the risks of material misstatement of the financial statements, whether due to fraud or error. In making those risk assessments, the auditor considers internal control relevant to the Business' preparation and fair presentation of the financial statements in order to design audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Business' internal control. Accordingly, we express no such opinion. An audit also includes evaluating the appropriateness of accounting policies used and the reasonableness of significant accounting estimates made by management, as well as evaluating the overall presentation of the financial statements.

We believe that the audit evidence we have obtained is sufficient and appropriate to provide a basis for our audit opinion.

#### Opinion

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of the Emflaza Business as of December 31, 2016 and 2015, and the results of its operations and its cash flows for the years then ended in accordance with accounting principles generally accepted in the United States of America.

## **Emphasis of Matter**

As described in Note 1, the Emflaza Business' financial statements have been derived from the financial statements and accounting records of Marathon Pharmaceutical Holdings, LLC. The carve-out financial statements include the allocation of certain assets, liabilities, expenses, and income that have historically

been held at the Marathon Pharmaceutical Holdings, LLC corporate level but which are specifically identifiable or attributable to the Emflaza Business.

/s/ Deloitte & Touche LLP Chicago, Illinois May 10, 2017

BALANCE SHEETS
AS OF DECEMBER 31, 2016 AND 2015
(In thousands of dollars)

	2016	2015
ASSETS	2016	2015
CURRENT ASSETS: Prepaid expenses	\$ 1,565	\$ 733
PROPERTY AND EQUIPMENT—Net	470	305
OTHER ASSETS	117	93
TOTAL ASSETS	\$ 2,152	\$ 1,131
LIABILITIES AND NET PARENT COMPANY DEFICIT		
CURRENT LIABILITIES:		
Accounts payable	\$ 3,953	\$ 1,588
Accrued compensation	2,605	2,096
Accrued expenses	753	756
Total current liabilities	7,311	4,440
OTHER LONG-TERM LIABILITIES	434	379
COMMITMENTS AND CONTINGENCIES (Note 5)		
Total liabilities	7,745	4,819
NET PARENT COMPANY DEFICIT	(5,593)	(3,688)
TOTAL LIABILITIES AND NET PARENT COMPANY DEFICIT	\$ 2,152	\$ 1,131

## STATEMENTS OF OPERATIONS FOR THE YEARS ENDED DECEMBER 31, 2016 AND 2015 (In thousands of dollars)

	2016	2015
OPERATING EXPENSES:		
Salaries and benefits	\$ 15,387	\$ 11,820
Selling and distribution	7,416	1,279
Research and development	12,957	14,335
General and administrative	6,438	5,124
Depreciation	122	56
Total operating expenses	42,320	32,614
NET LOSS	\$ 42,320	\$ 32,614

STATEMENTS OF COMPREHENSIVE LOSS FOR THE YEARS ENDED DECEMBER 31, 2016 AND 2015 (In thousands of dollars)

	2016	2015
NET LOSS	\$ 42,320	\$ 32,614
OTHER COMPREHENSIVE INCOME		
COMPREHENSIVE LOSS	\$ 42,320	\$ 32,614

# STATEMENTS OF NET PARENT COMPANY DEFICIT FOR THE YEARS ENDED DECEMBER 31, 2016 AND 2015 (In thousands of dollars)

BALANCE—December 31, 2014	\$ (2,098)
Net loss	(32,614)
Net transactions with Marathon Pharmaceuticals, LLC	31,024
BALANCE—December 31, 2015	(3,688)
Net loss	(42,320)
Net transactions with Marathon Pharmaceuticals, LLC	40,415
BALANCE—December 31, 2016	\$ (5,593)

## STATEMENTS OF CASH FLOWS FOR THE YEARS ENDED DECEMBER 31, 2016 AND 2015 (In thousands of dollars)

	2016	2015
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	\$ (42,320)	\$ (32,614)
Adjustments to reconcile net loss to net cash	, ,	, ,
used in operating activities:		
Depreciation	122	56
Deferred rent and other noncash items	55	259
(Increase) decrease in assets:		
Prepaid expenses	(856)	(566)
Increase (decrease) in liabilities:		
Accounts payable	2,365	547
Accrued expenses and other current liabilities	506	1,528
Net cash used in operating activities	(40,128)	(30,790)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchase of property and equipment	(287)	(234)
Net cash used in investing activities	(287)	(234)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Net transactions with Marathon Pharmaceuticals, LLC	40,415	31,024
Net cash provided by financing activities	40,415	31,024
NET CHANGE IN CASH AND CASH EQUIVALENTS	_	_
CASH AND CASH EQUIVALENTS—Beginning of year		
CACH AND CACH FOUNTAL ENTO. For Late	Φ.	Φ.
CASH AND CASH EQUIVALENTS—End of year	<u> </u>	<u> </u>
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SUPPLEMENTAL DISCLOSURE OF CASH FLOW	Φ.	Φ.
INFORMATION—Cash paid for interest and income taxes	<u> </u>	<u> </u>

NOTES TO FINANCIAL STATEMENTS AS OF AND FOR THE YEARS ENDED DECEMBER 31, 2016 AND 2015 (In thousands of dollars)

#### 1. BASIS OF PRESENTATION

Basis of Presentation—Marathon Pharmaceuticals, LLC ("Marathon") develops specialty pharmaceuticals for high-need populations, including patients who have limited or no other treatment options. On March 16, 2017, Marathon entered into an asset sale agreement with PTC Therapeutics, Inc. (PTC) for all rights to Marathon's product Emflaza™ (the "Company" or the "Emflaza Business") contingent on customary closing procedures. This sale closed on April 20, 2017. Proceeds from the sale are comprised of up-front payments of \$75 million in cash and \$65 million in common shares of PTC, and contingent future sales-based milestone and royalty payments.

These financial statements present the results of the Emflaza Business, which consists of all resources, personnel and otherwise, devoted to the research, development, manufacture, distribution, sale, and use of Emflaza, including trademarks. Emflaza™ was approved by the U.S. Food and Drug Administration on February 9, 2017 for the treatment of Duchenne muscular dystrophy in patients 5 and older. Emflaza™ had not been commercialized during the periods reflected in these financial statements, as such no revenues have been allocated to the Emflaza Business.

The accompanying financial statements have been prepared on a stand-alone basis and are derived from Marathon's accounting records. The financial statements reflect the Emflaza Business' historical financial position, results of operations, comprehensive loss and cash flows prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP").

Cash and cash equivalents held by Marathon were not allocated to the Emflaza Business. All intercompany transactions and accounts with Marathon have been eliminated. All intercompany transactions between Marathon and the Company are considered to be effectively settled in the financial statements at the time the transaction is recorded. The total net effect of the settlement of these intercompany transactions is reflected in the Statements of Cash Flows as a financing activity and in the balance sheets as net parent company deficit. These amounts have been reflected as an investment by Marathon as it was not intended that these amounts would be settled at any time

The financial statements include allocations of expenses related to certain corporate functions, including management, legal, finance, human resource, information technology, risk management and unit-based compensation. These expenses have been allocated to the Emflaza Business based on direct usage or benefit where identifiable, with the remainder allocated on pro rata basis of headcount and other measures. Marathon considers the expense allocation methodology and results to be reasonable for all periods presented. However, the allocations may not be indicative of the actual expense that would have been incurred had the Emflaza Business been operated as an independent company for the periods presented.

## 2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

**Use of Estimates**—The financial statements have been prepared in conformity with GAAP. These accounting principles require management to make certain estimates and assumptions that can affect the reported amount of assets and liabilities and contingent liabilities at the date of the financial

statements, as well as the reported amounts of expenses during the periods presented. Significant estimates include unit-based compensation expense and allocation of corporate expenses and liabilities. Actual amounts could differ materially from those estimates.

Research and Development Expenses—Research and development costs are expensed as incurred. Payments due to third parties in connection with research and development collaborations prior to regulatory approval are expensed as incurred.

Fair Value of Financial Instruments—The financial statements include accounts payable and accrued liabilities, all of which are short term in nature and, accordingly, approximate fair value.

It is the Company's policy, in general, to measure nonfinancial assets and liabilities at fair value on a nonrecurring basis. The instruments are not measured at fair value on an ongoing basis, but are subject to fair value adjustments in certain circumstances (such as evidence of impairment), which, if material, are disclosed in the accompanying footnotes.

Property and Equipment—Property and equipment are recorded at historical cost. These costs are depreciated over the estimated useful lives of the individual assets using the straight-line method. The Company depreciates leasehold improvements over the life of the respective leases or the service lives of the improvements, whichever is shorter. Any gains and losses from the disposition of property and equipment are included in the results of operations as incurred. The following table shows estimated useful lives of property and equipment:

7 years

3 years

**Useful Life** Description

Furniture and fixtures Computer equipment and software Leasehold improvements Lesser of asset life or remaining lease term

Income Taxes—Marathon is treated as a partnership for federal and state income tax purposes. Income is allocated to members in accordance with the terms of Marathon's operating agreement and is included in their individual income tax returns. Therefore, no liability or provision for federal income taxes has been allocated to the Emflaza Business' financial statements.

Equity Awards—Certain employees of Marathon have been granted value appreciation rights (VARs) in an affiliated company, MP Investor, LLC ("Investor, LLC"), an entity with a 67% ownership interest in the parent of Marathon. Certain other employees of Marathon have been granted value appreciation rights and unit options in Marathon Pharmaceutical Holdings, LLC ("Holdings LLC"), the parent of Marathon. Accounting Standards Codification (ASC) 718—Compensation—Stock Compensation, specifies that awards of the parent company to employees of consolidated subsidiaries are reported as a compensation expense with an offset to capital contributions, provided no consideration is paid from the subsidiary to the parent. Marathon has not provided any consideration to Investor, LLC or Holdings, LLC for such awards.

Accordingly, compensation expense incurred by Marathon related to such awards has been allocated to the Emflaza Business' statements of operations on a specific identification basis by recipient. The expense is allocated to the Emflaza Business based on the recipients' level of support of the Emflaza Business (see Note 6—"Equity Awards").

Recently Issued Accounting Pronouncements—In March 2016, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2016-09, Compensation-Stock

Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting. ASU No. 2016-09 provides amended guidance that simplifies several aspects of the accounting for employee share-based payment transactions, including the accounting for forfeitures, and statutory tax withholding requirements, as well as classification in the statements of cash flows. The new standard will become effective for the Company's fiscal year ending December 31, 2018. Early adoption is permitted and the application of the guidance requires various transition methods depending on the specific amendment. The Company is currently assessing the impact of this amended guidance and the timing of adoption.

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)* which provides amended guidance to increase transparency and comparability among organizations by recognizing lease assets and lease liabilities on the balance sheet and disclosing key information about leasing arrangements. The new guidance clarifies the criteria for distinguishing between a finance lease and operating lease, as well as classification between the two types of leases, which is substantially unchanged from the previous lease guidance. Further, the new guidance requires a lessee to recognize in the statements of financial position, a liability to make lease payments (the "lease liability") and a right-of-use asset, initially measured at the present value of the lease payments. For finance leases, a lessee should recognize interest on the lease liability separately from amortization of the right-of-use asset. For operating leases, a lessee should recognize a single lease cost, calculated so that the cost of the lease is allocated over the lease term on a generally straight-line basis. For leases with a term of 12 months or less, a lessee is permitted to make an accounting policy election not to recognize lease assets and lease liabilities. The new standard will become effective for the Company's fiscal year ending December 31, 2020. The Company is currently assessing the impact of this amended guidance and the timing of adoption.

In August 2016, the FASB issued ASU No. 2016-15, Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments. ASU No. 2016-15 provides specific guidance on eight cash flow issues where current guidance is unclear or does not include any specifics on classification, including contingent consideration payments made after a business combination and distributions received from equity method investees, among other items. The new standard will become effective for Company's fiscal year ending December 31, 2019. The Company is currently evaluating the impact of this ASU on the financial statements and related disclosures.

## 3. PROPERTY, PLANT, AND EQUIPMENT

Property, plant, and equipment at December 31, 2016 and 2015, consist of the following:

	2016	2015
Leasehold improvements	\$ 478	\$ 241
Computer equipment and software	180	99
Furniture and fixtures	54	54
	712	394
Less accumulated depreciation	(242)	(89)
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	\$ 470	\$ 305

Depreciation expense was \$122 and \$56 in 2016 and 2015, respectively.

## 4. ACCRUED EXPENSES

Accrued expenses at December 31, 2016 and 2015, consist of the following:

	2016	2015
Research and development services	\$ 360	\$ 490
Professional fees	240	198
Consulting and other services	153	68
	\$ 753	\$ 756

## 5. COMMITMENTS AND CONTINGENCIES

**Lease Commitment**—The Company has various operations in leased facilities. Rent expense allocated to the Emflaza Business and included in the statements of operations for 2016 and 2015 was \$452 and \$469, respectively.

The following summary presents, in the aggregate and for the indicated years, future minimum lease payments required under the terms of the present operating leases:

## Years Ending

## **December 31**

2017	\$ 511
2018	522
2019	523
2020	33
Minimum operating lease payments	\$ 1,589

**Litigation**—From time to time, the Company is subject to claims and suits arising in the ordinary course of business. The Company accrues for such liabilities when they are known if they are deemed probable and can be reasonably estimated. There were no such liabilities reflected in either of the balance sheets as of December 31, 2016 or December 31, 2015.

## 6. EQUITY AWARDS

Employees of Marathon are eligible to receive unit options, VARs and Restricted Incentive Units under a variety of incentive plans. Awards under these plans have a 10-year contractual term and vest over the period specified in the applicable award agreement (generally four or five years from the date of grant), at achievement of a performance requirement or upon a change of control (as defined in the applicable plan).

Unit-based compensation expense allocated to the Emflaza Business was \$2,372 and \$3,345 for the years ended December 31, 2016 and 2015, respectively.

## 7. RELATED-PARTY TRANSACTIONS

Marathon is party to a management agreement for professional services provided by a related party. The related party is an entity that shares common ownership with Marathon. In addition, Marathon's chief executive officer is the president and owner of the entity. For the years ended December 31, 2016 and 2015, the Emflaza Business was allocated \$1,712 and \$1,800, respectively, in management fee expenses to this related party that is included in general and administrative expenses within the statements of operations. As of December 31, 2016 and 2015, there were management fees payable of \$402 and \$483, respectively, reflected as a component of accounts payable.

The operations of the Company are funded by Marathon. All such funding is accounted for through parent company investment. Marathon's funding of the Emflaza business was \$40,415 and \$31,024 for the years ended December 31, 2016 and 2015, respectively.

## 8. SUBSEQUENT EVENTS

The Company has evaluated and, as necessary, made changes to these financial statements for subsequent events through May 10, 2017, the date these financial statements were available to be issued.

On February 9, 2017 the US Food and Drug Administration approved the Company's New Drug Applications for Emflaza oral tablets and oral suspension for the treatment of Duchenne Muscular Dystrophy in patients of five years of age and older. This approval triggered a milestone payment of \$2 million, which was capitalized as an intangible asset.

On March 16, 2017, Marathon entered into an asset sale agreement with PTC Therapeutics, Inc. ("PTC") for all rights to Marathon's product Emflaza<sup>TM</sup>, contingent on customary closing procedures. Assets transferred under this agreement exclude the Company's Priority Review Voucher. This sale closed on April 20, 2017. Proceeds from the sale are comprised of up-front payments of \$75 million in cash and \$65 million in common shares of PTC, and contingent future sales-based milestone and royalty payments.

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## **Emflaza Business**

Balance Sheets as of March 31, 2017 and December 31, 2016, and Statement of Net Parent Company Deficit for the Three Months Ended March 31, 2017 and Statements of Operations, Comprehensive Loss, and Cash Flows for the Three Months Ended March 31, 2017 and 2016

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BALANCE SHEETS
AS OF MARCH 31, 2017 AND DECEMBER 31, 2016
(In thousands of dollars)

ASSETS	2017	2016
ASSETS		
CURRENT ASSETS:		
Prepaid expenses	\$ 1,356	\$ 1,565
Inventories	 232	 
Total current assets	1,588	1,565
PROPERTY AND EQUIPMENT—Net	445	470
INTANGIBLE ASSETS	1,952	
OTHER ASSETS	 127	 117
TOTAL ASSETS	\$ 4,112	\$ 2,152
LIABILITIES AND NET PARENT COMPANY DEFICIT		
CURRENT LIABILITIES:		
Accounts payable	\$ 4,127	\$ 3,953
Accrued compensation	969	2,605
Accrued expenses	 1,109	 753
Total current liabilities	6,205	7,311
OTHER LONG-TERM LIABILITIES	 402	 434
COMMITMENTS AND CONTINGENCIES (Note 8)		
Total liabilities	6,607	7,745
NET PARENT COMPANY DEFICIT	 (2,495)	 (5,593)
TOTAL LIABILITIES AND NET PARENT COMPANY DEFICIT	\$ 4,112	\$ 2,152

## STATEMENTS OF OPERATIONS FOR THE THREE MONTHS ENDED MARCH 31, 2017 AND 2016 (In thousands of dollars)

	2017		2016
OPERATING EXPENSES:			
Salaries and benefits	\$ 9,557	\$	2,676
Selling and distribution	4,207		623
Research and development	3,236		2,402
General and administrative	3,210		1,072
Depreciation and amortization	 97		24
Total operating expenses	20,307		6,797
NET LOSS	\$ 20,307	\$	6,797

STATEMENTS OF COMPREHENSIVE LOSS FOR THE THREE MONTHS ENDED MARCH 31, 2017 AND 2016 (In thousands of dollars)

	2017	2016
NET LOSS	\$ 20,307	\$ 6,797
OTHER COMPREHENSIVE INCOME		 
COMPREHENSIVE LOSS	\$ 20,307	\$ 6,797

# STATEMENTS OF NET PARENT COMPANY DEFICIT FOR THE PERIOD ENDED MARCH 31, 2017 (In thousands of dollars)

BALANCE—December 31, 2016	\$ (5,593)
Net loss	(20,307)
Net transactions with Marathon Pharmaceuticals, LLC	 23,405
BALANCE—March 31, 2017	\$ (2,495)

## STATEMENTS OF CASH FLOWS FOR THE THREE MONTHS ENDED MARCH 31, 2017 AND 2016 (In thousands of dollars)

	2017	2016
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	\$ (20,307)	\$ (6,797)
Adjustments to reconcile net loss to net cash	· · · · ·	
used in operating activities:		
Depreciation and amortization	97	24
Deferred rent and other noncash items	(32)	(19)
(Increase) decrease in assets:	,	,
Prepaid expenses	199	(25)
Inventories	(232)	,
Increase (decrease) in liabilities:	,	
Accounts payable	174	(401)
Accrued expenses and other current liabilities	(1,280)	(1,538)
	(1,-22)	(1,000)
Net cash used in operating activities	(21,381)	(8,756)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchase of property and equipment	(24)	(141)
Acquisition of intangible assets	(2,000)	
Net cash used in investing activities	(2,024)	(141)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Net transactions with Marathon Pharmaceuticals, LLC	23,405	8,897
Net cash provided by financing activities	23,405	8,897
NET CHANGE IN CASH AND CASH EQUIVALENTS	_	_
CASH AND CASH EQUIVALENTS—Beginning of period		
CASH AND CASH EQUIVALENTS—End of period	<u>\$</u>	<u> </u>
SUPPLEMENTAL DISCLOSURE OF CASH FLOW		
INFORMATION—Cash paid for interest and income taxes	\$ —	\$ —

## NOTES TO FINANCIAL STATEMENTS (In thousands of dollars)

#### 1. BASIS OF PRESENTATION

Basis of Presentation—Marathon Pharmaceuticals, LLC ("Marathon") develops specialty pharmaceuticals for high-need populations, including patients who have limited or no other treatment options. On March 16, 2017, Marathon entered into an asset sale agreement with PTC Therapeutics, Inc. (PTC) for all rights to Marathon's product Emflaza<sup>TM</sup> (the "Company" or the "Emflaza Business") contingent on customary closing procedures. This sale closed on April 20, 2017. Proceeds from the sale are comprised of up-front payments of \$75 million in cash and \$65 million in common shares of PTC, and contingent future sales-based milestone and royalty payments.

These unaudited condensed interim financial statements reflect all normal and recurring adjustments that are, in the opinion of management, necessary for a fair presentation of the results for the interim periods. The financial statements present the results of the Emflaza Business, which consists of all resources, personnel and otherwise, devoted to the research, development, manufacture, distribution, sale, and use of Emflaza, including trademarks. Emflaza™ was approved by the U.S. Food and Drug Administration on February 9, 2017 for the treatment of Duchenne muscular dystrophy in patients 5 and older. Emflaza™ has not been commercialized during the periods reflected in these financial statements, as such no revenues have been allocated to the Emflaza Business.

The accompanying financial statements have been prepared on a stand-alone basis and are derived from Marathon's accounting records. The financial statements reflect the Emflaza Business' historical financial position, results of operations, comprehensive loss and cash flows prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP").

Cash and cash equivalents held by Marathon were not allocated to the Emflaza Business. All intercompany transactions and accounts with Marathon have been eliminated. All intercompany transactions between Marathon and the Company are considered to be effectively settled in the financial statements at the time the transaction is recorded. The total net effect of the settlement of these intercompany transactions is reflected in the Statements of Cash Flows as a financing activity and in the balance sheets as net parent company deficit. These amounts have been reflected as an investment by Marathon as it was not intended that these amounts would be settled at any time.

The financial statements include allocations of expenses related to certain corporate functions, including management, legal, finance, human resource, information technology, risk management and unit-based compensation. These expenses have been allocated to the Emflaza Business based on direct usage or benefit where identifiable, with the remainder allocated on pro rata basis of headcount and other measures. Marathon considers the expense allocation methodology and results to be reasonable for all periods presented. However, the allocations may not be indicative of the actual expense that would have been incurred had the Emflaza Business been operated as an independent company for the periods presented.

## 2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

**Use of Estimates**—The financial statements have been prepared in conformity with GAAP. These accounting principles require management to make certain estimates and assumptions that can affect the reported amount of assets and liabilities and contingent liabilities at the date of the financial statements, as well as the reported amounts of expenses during the periods presented. Significant estimates include unit-based compensation expense and allocation of corporate expenses and liabilities. Actual amounts could differ materially from those estimates.

**Research and Development Expenses**—Research and development costs are expensed as incurred. Payments due to third parties in connection with research and development collaborations prior to regulatory approval are expensed as incurred.

**Fair Value of Financial Instruments**—The financial statements include accounts payable and accrued liabilities, all of which are short term in nature and, accordingly, approximate fair value.

It is the Company's policy, in general, to measure nonfinancial assets and liabilities at fair value on a nonrecurring basis. The instruments are not measured at fair value on an ongoing basis, but are subject to fair value adjustments in certain circumstances (such as evidence of impairment), which, if material, are disclosed in the accompanying footnotes.

**Inventories**—Inventory costs are capitalized once regulatory approval for commercial sale has been obtained. Inventories are stated at the lower of cost or market, with market based on the lower of replacement cost or net realizable value. A provision for slow-moving and obsolete inventory is made if the amount of inventory recorded exceeds the Company's estimates of inventory that may be sold prior to its expiration based on projected sales levels.

Intangible Assets—Intangible assets consist of product rights which are capitalized and amortized over their estimated useful life of 7 years.

**Property and Equipment**—Property and equipment are recorded at historical cost. These costs are depreciated over the estimated useful lives of the individual assets using the straight-line method. The Company depreciates leasehold improvements over the life of the respective leases or the service lives of the improvements, whichever is shorter. Any gains and losses from the disposition of property and equipment are included in the results of operations as incurred. The following table shows estimated useful lives of property and equipment:

Description Useful Life

Furniture and fixtures

Computer equipment and software

Leasehold improvements

7 years 3 years

Lesser of asset life or remaining lease term

**Income Taxes**—Marathon is treated as a partnership for federal and state income tax purposes. Income is allocated to members in accordance with the terms of Marathon's operating agreement and is included in their individual income tax returns. Therefore, no liability or provision for federal income taxes has been allocated to the Emflaza Business' financial statements.

**Equity Awards**—Certain employees of Marathon have been granted value appreciation rights (VARs) in an affiliated company, MP Investor, LLC ("Investor, LLC"), an entity with a 67% ownership interest in the parent of Marathon. Certain other employees of Marathon have been granted value appreciation rights and unit options in Marathon Pharmaceutical Holdings, LLC ("Holdings LLC"), the parent of Marathon. Accounting Standards Codification (ASC) 718 —*Compensation—Stock Compensation*, specifies that awards of the parent company to employees of consolidated subsidiaries are reported as a compensation expense with an offset to capital contributions, provided no consideration is paid from the subsidiary to the parent. Marathon has not provided any consideration to Investor, LLC or Holdings, LLC for such awards.

Accordingly, compensation expense incurred by Marathon related to such awards has been allocated to the Emflaza Business' statements of operations on a specific identification basis by recipient. The expense is allocated to the Emflaza Business based on the recipients' level of support of the Emflaza Business (see Note 9—"Equity Awards").

## **Recently Issued Accounting Pronouncements**

In March 2016, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2016-09, Compensation-Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting. ASU No. 2016-09 provides amended guidance that simplifies several aspects of the accounting for employee share-based payment transactions, including the accounting for forfeitures, and statutory tax withholding requirements, as well as classification in the statements of cash flows. The new standard will become effective for the Company's fiscal year ending December 31, 2018. Early adoption is permitted and the application of the guidance requires various transition methods depending on the specific amendment. The Company is currently assessing the impact of this amended guidance and the timing of adoption.

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)*, which provides amended guidance to increase transparency and comparability among organizations by recognizing lease assets and lease liabilities on the balance sheet and disclosing key information about leasing arrangements. The new guidance clarifies the criteria for distinguishing between a finance lease and operating lease, as well as classification between the two types of leases, which is substantially unchanged from the previous lease guidance. Further, the new guidance requires a lessee to recognize in the statements of financial position, a liability to make lease payments (the "lease liability") and a right-of-use asset, initially measured at the present value of the lease payments. For finance leases, a lessee should recognize interest on the lease liability separately from amortization of the right-of-use asset. For operating leases, a lessee should recognize a single lease cost, calculated so that the cost of the lease is allocated over the lease term on a generally straight-line basis. For leases with a term of 12 months or less, a lessee is permitted to make an accounting policy election not to recognize lease assets and lease liabilities. The new standard will become effective for the Company's fiscal year ending December 31, 2020. The Company is currently assessing the impact of this amended guidance and the timing of adoption.

In August 2016, the FASB issued ASU No. 2016-15, *Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments*. ASU No. 2016-15 provides specific guidance on eight cash flow issues where current guidance is unclear or does not include any specifics on classification, including contingent consideration payments made after a business combination and distributions received from equity method investees, among other items. The new standard will become effective for the Company's fiscal year ending December 31, 2019. The Company is currently evaluating the impact of this ASU on the financial statements and related disclosures.

## 3. PROPERTY, PLANT, AND EQUIPMENT

Property, plant, and equipment at March 31, 2017 and December 31, 2016, consist of the following:

	;	2017	2016
Leasehold improvements	\$	495	\$ 478
Computer equipment and software		200	180
Furniture and fixtures		54	 54
		749	712
Less accumulated depreciation		(304)	 (242)
	\$	445	\$ 470

Depreciation expense was \$49 and \$24 for the three months ended March 31, 2017 and 2016, respectively.

#### 4. INVENTORIES

Inventories of \$232 as of March 31, 2017 consist of purchased finished goods. No provision has been made for slow-moving or obsolete inventory as of March 31, 2017 and no current period charges for obsolescence have occurred in the three months ended March 31, 2017 and 2016. There were no inventories as of December 31, 2016.

## 5. ACQUISITION

In January of 2013, Marathon entered into an asset acquisition agreement with Norphan Pharmaceuticals, Inc. whereby Marathon acquired the right, title and interest in four pharmaceutical products including deflazacort. At the time of acquisition, none of the pharmaceutical products had been approved for commercial sale by the U.S. Food and Drug Administration (FDA). The agreement called for an initial payment of \$250 and contingent payments including a regulatory approval milestone and post-approval sales-based royalty payments. The initial payment was recorded to in-process research and development expenses. No external transaction costs were incurred.

On February 9, 2017 deflazacort (Emflaza<sup>™</sup>) was approved by the FDA for the treatment of Duchenne muscular dystrophy, triggering a \$2 million milestone payment. This payment was capitalized and recorded as an intangible asset in the balance sheet, with an associated estimated useful life of 7 years.

## 6. INTANGIBLE ASSETS

Intangible assets at March 31, 2017 and December 31, 2016 consist of the following:

	2017		2016
Product rights Less accumulated amortization	\$	2,000 (48)	\$
	\$	1,952	\$

Product rights are amortized over their useful life of 7 years. Amortization expense was \$48 and \$0 for the three month periods ended March 31, 2017 and 2016, respectively. Amortization expense of \$286 will occur in each of the next five successive fiscal years.

## 7. ACCRUED EXPENSES

Accrued expenses at March 31, 2017 and December 31, 2016, consist of the following:

	2017		2016
Professional fees	\$ 542	\$	240
Consulting and other services	319		153
Supply chain services	188		
Research and development services	 60		360
	\$ 1,109	\$	753

#### 8. COMMITMENTS AND CONTINGENCIES

**Lease Commitment**—The Company has various operations in leased facilities. Rent expense allocated to the Emflaza Business and included in the statements of operations for 2017 and 2016 was \$136 and \$108, respectively.

The following summary presents, in the aggregate and for the indicated years, future minimum lease payments required under the terms of the present operating leases:

## **Years Ending**

#### **December 31**

2017 (remaining nine months)	\$ 386
2018	522
2019	523
2020	 33
Minimum operating lease payments	\$ 1,464

**Litigation**—From time to time, the Company is subject to claims and suits arising in the ordinary course of business. The Company accrues for such liabilities when they are known if they are deemed probable and can be reasonably estimated. There were no such liabilities reflected in either of the balance sheets as of March 31, 2017 or December 31, 2016.

#### 9. EQUITY AWARDS

Employees of Marathon are eligible to receive unit options, VARs and Restricted Incentive Units under a variety of incentive plans. Awards under these plans have a 10-year contractual term and vest over the period specified in the applicable award agreement (generally four or five years from the date of grant), at achievement of a performance requirement or upon a change of control (as defined in the applicable plan).

Unit-based compensation expense allocated to the Emflaza Business was \$4,451 and \$594 for the three months ended March 31, 2017 and 2016, respectively.

#### 10. RELATED-PARTY TRANSACTIONS

Marathon is party to a management agreement for professional services provided by a related party. The related party is an entity that shares common ownership with Marathon. In addition, Marathon's chief executive officer is the president and owner of the entity. For each of the three months ended March 31, 2017 and 2016, the Emflaza Business was allocated \$425 in management fee expenses to this related party that is included in general and administrative expenses within the statements of operations. As of March 31, 2017 and December 31, 2016, there were management fees payable of \$81 and \$402, respectively, reflected as a component of accounts payable.

The operations of the Company are funded by Marathon. All such funding is accounted for through parent company investment. Marathon's funding of the Emflaza business was \$23,405 and \$8,897 for the three months ended March 31, 2017 and 2016, respectively.

## 11. SUBSEQUENT EVENTS

The Company has evaluated and, as necessary, made changes to these financial statements for subsequent events through May 10, 2017, the date these financial statements were available to be issued.

On March 16, 2017, Marathon entered into an asset sale agreement with PTC Therapeutics, Inc. ("PTC") for all rights to Marathon's product Emflaza<sup>TM</sup>, contingent on customary closing procedures. Assets transferred under this agreement exclude the Company's Priority Review Voucher. This sale closed on April 20, 2017. Proceeds from the sale are comprised of up-front payments of \$75 million in cash and \$65 million in common shares of PTC, and contingent future sales-based milestone and royalty payments.

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#### UNAUDITED PRO FORMA COMBINED FINANCIAL STATEMENTS

On April 20, 2017, PTC Therapeutics, Inc. ("PTC" or the "Company") completed its acquisition of all rights to EMFLAZA<sup>TM</sup> (deflazacort) (the "Transaction") pursuant to an asset purchase agreement (the "Asset Purchase Agreement"), dated March 15, 2017, and amended on April 20, 2017, by and between the Company and Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC) ("Marathon"), for total upfront consideration comprised of \$75.0 million in cash and 6,683,598 shares of the Company's common stock, which was determined by dividing \$65.0 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. The assets (the "Assets") acquired by the Company in the Transaction include intellectual property rights related to EMFLAZA, inventories of EMFLAZA, certain contractual rights to EMFLAZA, and certain other assets related to EMFLAZA in addition to assuming certain liabilities and obligations, including contractual obligations and various other liabilities and obligations arising out of, or relating to, the Assets. 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 \$50.0 million sales-based milestone payment, in each case subject to the terms and conditions of the Asset Purchase Agreement.

The Company expects to account for the Transaction as an asset acquisition pursuant to the adoption of the recently issued guidance in Accounting Standards Update (ASU) No. 2017-01, "Business Combinations (Topic 805): Clarifying the Definition of a Business." Under ASU No. 2017-01, if substantially all of the fair value of the gross assets acquired is concentrated in a single identifiable asset or group of similar identifiable assets, the set of transferred assets is not a business. The Company has determined that substantially all of the fair value of the Transaction is related to the rights to EMFLAZA and meets the threshold for applying asset acquisition accounting treatment.

The following unaudited pro forma combined financial statements were prepared in accordance with Article 11 of the Securities and Exchange Commission's ("SEC") Regulation S-X and reflect adjustments to the extent they are directly attributable to the acquisition, factually supportable, expected to have a continuing impact and, for balance sheet purposes, are nonrecurring. The unaudited pro forma combined financial statements present the combination of the historical consolidated financial statements of the Company and stand-alone financial statements pertaining to EMFLAZA (the "Emflaza Business") which have been derived from Marathon's accounting records adjusted to give effect to the April 20, 2017 acquisition of EMFLAZA by the Company. The unaudited pro forma combined balance sheet as of March 31, 2017 combines the unaudited historical consolidated balance sheet of the Emflaza Business as of March 31, 2017, giving effect to the Transaction as if it had occurred on March 31, 2017. The unaudited pro forma combined statements of operations for the year ended December 31, 2016 and for the period ended March 31, 2017 combine the audited historical consolidated statement of operations of the Company and the unaudited historical statement of operations of the Emflaza Business for the year ended December 31, 2016, and the unaudited historical consolidated statement of operations of the Company and the unaudited historical statement of operations of the Emflaza Business for the period ended March 31, 2017, giving effect to the Transaction as if it had occurred on January 1, 2016.

The unaudited pro forma combined financial statements are presented for informational purposes only and are not intended to represent or be indicative of what the financial position or results of operations would have been had the Transaction occurred on the dates indicated. The unaudited pro forma combined financial statements are not intended to represent or be indicative of the financial position or results of operations for any future periods.

The "Historical PTC" column in the unaudited pro forma combined financial statements reflects the Company's historical financial statements as of and for the period ended March 31, 2017 and historical statement of operations for the year ended December 31, 2016 and does not reflect any adjustments related to the Transaction.

The "Historical Emflaza" column in the unaudited pro forma combined financial statements reflects the Emflaza Business' historical financial statements as of and for the period ended March 31, 2017 and historical statement of operations for the year ended December 31, 2016 and does not reflect any adjustments related to the Transaction.

The "Pro Forma Adjustments" are based on the Company's assumptions and estimates that the Company believes are reasonable and which are described in the accompanying notes to the unaudited pro forma combined financial statements.

PTC Therapeutics, Inc. Unaudited Pro Forma Combined Balance Sheet In thousands

		March 31, 2017							
	Hi	Historical PTC Historic		orical Emflaza		ro Forma djustments	Notes		Pro Forma Combined
Assets						_			
Current assets:									
Cash and cash equivalents	\$	54,106	\$	_	\$	_		\$	54,106
Marketable securities		148,471		_		(75,000)	(A)		73,471
Inventory		1,354		232		1,451	(B)		3,037
Prepaid expenses and other current assets		4,623		1,356		(1,356)	(C)		4,623
Trade receivables, net		32,173		_		_			32,173
Total current assets		240,727		1,588		(74,905)			167,410
Fixed assets, net		7,059		445		(445)	(C)		7,059
Intangible assets, net		_		1,952		147,979	(C), (D)		149,931
Deposits and other assets		859		127		(127)	(C)		859
Total assets	\$	248,645	\$	4,112	\$	72,502		\$	325,259
Liabilities and stockholders' equity	_								
Current liabilities:									
Accounts payable and accrued expenses	\$	45,153	\$	6,205	\$	(4,781)	(C), (E)	\$	46,577
Other current liabilities		265		_		_			265
Total current liabilities		45,418		6,205		(4,781)			46,842
Deferred revenue - long-term		2,194		_		_			2,194
Long-term debt		99,895		_		_			99,895
Other long-term liabilities		311		402		(402)	(C)		311
Total liabilities		147,818		6,607		(5,183)			149,242
Stockholders' equity:									
Common stock		34		_		7	(A)		41
Additional paid-in capital		865,834		_		75,183	(A)		941,017
Accumulated other comprehensive loss		(876)		_		_			(876)
Accumulated deficit		(764,165)		(2,495)		2,495	(C)		(764,165)
Total stockholders' equity		100,827		(2,495)		77,685			176,017
Total liabilities and stockholders' equity	\$	248,645	\$	4,112	\$	72,502		\$	325,259

See accompanying unaudited notes.

PTC Therapeutics, Inc. Unaudited Pro Forma Combined Statement of Operations In thousands (except per share data)

		Year ended December 31, 2016									
	]	Historical PTC		Historical Emflaza		Pro Forma Adjustments	Notes		Pro Forma Combined		
Revenues:											
Net product revenue	\$	81,447	\$	_	\$	_		\$	81,447		
Collaboration and grant revenue		1,258		_		_			1,258		
Total revenues	<u> </u>	82,705		_		_		<u></u>	82,705		
Operating expenses:											
Research and development		117,633		19,781		(6,824)	(F)		130,590		
Selling, general and administrative		97,130		22,539		12,734	(G), (H)		132,403		
Total operating expenses		214,763		42,320		5,910			262,993		
Loss from operations		(132,058)		(42,320)		(5,910)			(180,288)		
Interest expense, net		(8,276)		_		_			(8,276)		
Other expense, net		(1,207)		_		_			(1,207)		
Loss before income tax expense		(141,541)		(42,320)		(5,910)			(189,771)		
Income tax expense		(569)		_		_			(569)		
Net loss attributable to common stockholders	\$	(142,110)	\$	(42,320)	\$	(5,910)		\$	(190,340)		
Weighted-average shares outstanding:											
Basic and diluted (in shares)		34,044,584				6,683,598	(A)		40,728,182		
Net loss per share—basic and diluted (in dollars per share)	\$	(4.17)						\$	(4.67)		

See accompanying unaudited notes.

## PTC Therapeutics, Inc. Unaudited Pro Forma Combined Statement of Operations In thousands (except per share data)

	Three months ended March 31, 2017									
	I	Historical PTC		Historical Emflaza		Pro Forma Adjustments	Notes		Pro Forma Combined	
Revenues:										
Net product revenue	\$	26,442	\$	_	\$	_		\$	26,442	
Collaboration and grant revenue		105		_		_			105	
Total revenues		26,547				_			26,547	
Operating expenses:										
Cost of product sales		39		_		_			39	
Research and development		27,363		7,463		(4,227)	(F)		30,599	
Selling, general and administrative		25,500		12,844		(24)	(G), (H)		38,320	
Total operating expenses		52,902		20,307		(4,251)			68,958	
Loss from operations		(26,355)		(20,307)		4,251			(42,411)	
Interest expense, net		(2,219)		_		_			(2,219)	
Other expense, net		(318)		_		_			(318)	
Loss before income tax expense		(28,892)		(20,307)		4,251			(44,948)	
Income tax expense		(165)		_		_			(165)	
Net loss attributable to common stockholders	\$	(29,057)	\$	(20,307)	\$	4,251		\$	(45,113)	
Weighted-average shares outstanding:										
Basic and diluted (in shares)		34,305,948				6,683,598	(A)		40,989,546	
Net loss per share—basic and diluted (in dollars per share)	\$	(0.85)					. ,	\$	(1.10)	

See accompanying unaudited notes.

# PTC Therapeutics, Inc. Notes to Unaudited Pro Forma Combined Financial Statements In thousands (except per share data unless otherwise noted)

#### 1. Preliminary estimate of consideration transferred

The following is a preliminary estimate of the components of the consideration transferred as part of the acquisition:

Cash consideration (upfront payment to Marathon)	\$ 75,000
Fair value of PTC common stock issued to Marathon (6,683,598 shares)	75,190
Acquisition costs	1,424
Total preliminary consideration transferred	\$ 151,614

## 2. Purchase price

The following is a summary of the preliminary allocation of the purchase price reconciled to the estimate of net consideration transferred. The purchase price allocation assumes that the acquisition occurred on March 31, 2017:

Purchase price	\$ 151,614
Total fair value of tangible assets acquired and liabilities assumed:	
Inventory	 1,683
EMFLAZA rights	\$ 149,931

#### 3. Reclassifications to unaudited pro forma combined financial statements

For purposes of the unaudited pro forma combined financial statements, the following captions from the Emflaza Business' historical balance sheet, which is included in Exhibit 99.3 of this filing, have been reclassified to conform to the presentation of the Company:

• \$4.1 million from "accounts payable", \$1.0 million from "accrued compensation" and \$1.1 million from "accrued expenses" was reclassified into "accounts payable and accrued expenses."

For purposes of the unaudited pro forma combined financial statements, the following captions from the Emflaza Business' historical statement of operations for the year ended December 31, 2016, which is included in Exhibit 99.2 of this filing, have been reclassified to conform to the presentation of the Company:

- \$6.8 million from "salaries and benefits" and \$0.05 million from "depreciation" was reclassified into "research and development" expense.
- \$8.6 million from "salaries and benefits", \$7.4 million from "selling and distribution" and \$0.07 million from "depreciation" was reclassified into "selling, general, and administrative" expense.

For purposes of the unaudited pro forma combined financial statements, the following captions from the Emflaza Business' historical statement of operations for the period ended March 31, 2017, which is included in Exhibit 99.3 of this filing, have been reclassified to conform to the presentation of the Company:

- \$4.2 million from "salaries and benefits" and \$0.02 million from "depreciation and amortization" was reclassified into "research and development" expense.
- \$5.4 million from "salaries and benefits", \$4.2 million from "selling and distribution" and \$0.08 million from "depreciation and amortization" was reclassified into "selling, general, and administrative" expense.

#### 4. Pro forma adjustments

The pro forma adjustments are based on preliminary estimates and assumptions that are subject to change. The following adjustments have been reflected in the unaudited pro forma combined financial statements:

- A. Reflects the adjustment to record the upfront cash payment to Marathon of \$75.0 million and the issuance of 6,683,598 shares of common stock of the Company transferred to Marathon at the close of the acquisition valued at \$75.2 million.
- B. Reflects the adjustment to the historical inventory acquired by the Company to its estimated fair value based on the preliminary purchase price allocation (see Note 2).
- C. Reflects the adjustment to record the removal of the assets and liabilities allocated to the Emflaza Business that were not acquired by PTC as part of the Transaction.
- D. Reflects the adjustment to record the intangible asset related to the EMFLAZA product rights based on the allocation of the purchase price as if the Transaction had occurred on March 31, 2017 (see Note 2).
- E. Reflects the adjustment to record unpaid transaction costs directly related to the asset acquisition of \$1.4 million in accounts payable and accrued expenses.
- F. Reflects the adjustment to remove employee compensation costs and depreciation expense recorded in "research and development" expense in the "Historical Emflaza" column due to no assembled workforce or property, plant, or equipment being included as part of the Acquired Assets.
- G. Reflects the adjustment to remove employee compensation costs and depreciation expense recorded in "selling, general, and administrative" expense in the "Historical Emflaza" column due to no assembled workforce or property, plant, or equipment being included as part of the Acquired Assets.
- H. Reflects the adjustment to record the historical intangible asset acquired by the Company to their estimated fair values. The following table summarizes the estimated fair value of the Emflaza Business' identifiable intangible asset and the estimated useful life and uses a straight line method of amortization:

	Estimated Fair Value		Estimated Useful Life in Years	Year ended December 31, 2016 Amortization Expense		Period ended March 31, 2017 Amortization Expense	
EMFLAZA rights	\$	149,931	7.0	\$	21,419	\$	5,355
Pro forma adjustments				\$	21,419	\$	5,355