

PTC THERAPEUTICS, INC.

FORM 8-K (Current report filing)

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Address	100 CORPORATE COURT SOUTH PLAINFIELD, NJ 07080-2449
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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): **August 7, 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.)
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100 Corporate Court South Plainfield, NJ (Address of Principal Executive Offices)	07080 (Zip Code)
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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.

EXHIBIT INDEX

Exhibit No.	Description
10.1	Consulting Services Agreement between the Registrant and Mark A. Rothera
99.1	Press Release, dated August 8, 2017

PTC CONSULTING SERVICES AGREEMENT

This Consulting Services Agreement (together with all attachments hereto, this "Agreement"), effective as of August 10, 2017 (the "Effective Date"), is made by and between PTC Therapeutics, Inc. ("PTC" or the "Company") and Mark Rothera (the "Consultant").

WHEREAS, the Consultant has served as an employee of PTC, advising on marketing/commercial and strategic global commercial and product portfolio matters relating to the building and expansion of PTC's commercial business in the rare disease area (the "Field"), under the Employment Agreement dated as of May 24, 2013 between PTC and the Consultant (the "Employment Agreement"), until the Effective Date;

WHEREAS, the Consultant and PTC are parties to a letter agreement dated August 10, 2017 (the "Separation Agreement"), which, *inter alia*, reaffirms the Consultant's continuing contractual and legal obligations to PTC with respect to confidential information, non-competition and non-solicitation; and

WHEREAS, the purpose of this Agreement is to set forth the terms and conditions governing (i) Consultant's rendering consulting services to PTC in the Field in exchange for the consideration as further described in this Agreement, and (ii) various related matters, including without limitation Consultant's obligations to protect information and property which is confidential and proprietary to PTC and its affiliated entities.

NOW THEREFORE, PTC and Consultant agree as follows:

SERVICES AND NATURE OF ENGAGEMENT

Services. Consultant shall perform the services for PTC in the Field as reasonably requested from time to time, as set forth in Exhibit 1 hereto (the "Services"). Consultant shall also provide such other Services as the parties may mutually agree. Consultant represents that he is capable of properly performing the Services.

Independent Contractor Status. The Consultant understands and agrees that he is engaged herein as an independent contractor, and not an employee of the Company. The Consultant shall retain control over the manner and method by which he provides the Services and shall not be subject to the control or direction of the Company, except that the Company may specify the general services that the Company engages Consultant to perform. Consultant specifically acknowledges and agrees that he has the requisite knowledge, expertise, experience, training and, if applicable, license(s) and/or certification(s), to perform the Services, and that the Company will not provide Consultant with any training concerning the manner or methods of performance of the Services.

No Employment Relationship. Nothing in this Agreement shall create any contract or relationship of employment between the Consultant and the Company or render the Consultant an employee of the Company. The Consultant shall be deemed an independent contractor and not an employee of the Company for all purposes, including all federal/national, state/regional and local laws pertaining to income taxes, withholding taxes, social security, unemployment compensation, workers compensation/employers' liability or any other rights, benefits, or obligations relating to employment. The Consultant specifically understands and agrees that he: (i) shall not receive a salary or any health, welfare, or other benefits provided by the Company to its employees; (ii) is not entitled to submit any claim for injury or illness either directly to the Company or under any workers' compensation/employers' liability coverage maintained by the Company; and (iii) shall, in relation to the Services rendered pursuant to this Agreement, receive an IRS Form 1099 (or equivalent) from the Company with respect to his compensation for the Services, and it shall be the Consultant's sole responsibility to report and to pay all applicable income taxes on all payments made to the Consultant by the Company under this Agreement, and the Company shall not withhold any taxes except to the extent required by applicable law. The Consultant shall not, at any time, represent to others that he is, nor will he hold himself out to be, an employee of the Company. The Consultant is not authorized to bind the Company or to incur any obligation or liability on behalf of the Company.

COMPENSATION

As Consultant's total cash compensation for Consultant's Services hereunder, PTC shall pay Consultant a fixed amount of \$6,349.00 for August 2017 and an additional \$9,500.00 for each additional full calendar month (in each case, pro-rated for any partial quarter/month), for an estimated four 8-hour days per month (or equivalent) month. Consultant shall send all invoices for

Services via email to both accountspayable@ptcbio.com and mrexroad@ptcbio.com. Such invoices shall contain a general description of the Services performed, the dates of performance, and the amount of time spent performing the Services.

In addition, the outstanding options to purchase common stock, restricted stock and restricted stock units of PTC issued/granted to Consultant prior to the Effective Date based on his prior services as an employee of PTC shall be treated as set forth on Schedule A hereto (the "Equity Awards"), *provided that*, Consultant shall be prohibited from engaging in any transaction related to PTC's stock, including exercising any stock options which have vested or will vest, from the Effective Date through the day that PTC's general 'quarterly blackout period' for the 2017 third quarter ends, which date is two business days following PTC's investor call to discuss quarterly results for the third quarter.

In addition to the foregoing amount, PTC shall pay Consultant for (i) all reasonable and necessary travel expenses (other than ordinary commuting expenses) incurred by Consultant at the prior request of PTC in providing the Services, and (ii) other expenses pre-approved in writing by an authorized PTC representative that are necessary to performance of the Services. Payment for such expenses shall be made to Consultant within 45 calendar days of receipt by PTC of invoices and receipts substantiating such expenses and detailing the services to which they relate, and PTC shall then have no further obligation to Consultant for such expenses.

Consultant shall keep or cause to be kept full, timely and accurate records in reasonable form and detail and to which PTC and its designated employees, agents or representatives shall have access at any reasonable time for auditing purposes. When requested by PTC, Consultant shall be required to report on the status of its work on the Services in a manner satisfactory to PTC.

NO CONFLICTS

Throughout the term of this Agreement the parties hereto agree that the type of services to be rendered hereunder by Consultant are not exclusive to PTC. Consultant, during the term of this Agreement, may accept from others, concurrent consulting work provided that such consulting work does not in any way interfere with the Services Consultant is required to perform under this Agreement and is not a breach of any of Consultant's obligations under this Agreement. Notwithstanding the foregoing, Consultant represents that (i) he has not, and will not during the term of this Agreement, enter into any agreement or relationship that would interfere with or prevent his performance under this Agreement, (ii) by entering into and performing this Agreement, he is not and will not be violating any agreement or arrangement with any third parties, and (iii) that during the term of this Agreement he will not solicit or divert business, customers or employees of PTC on behalf of Consultant, himself or any other business.

CONFIDENTIALITY

(a) In providing consulting services to PTC pursuant to this Agreement, Consultant may have continued access to or acquire PTC's confidential or proprietary information, including without limitation information that pertains to PTC's employees, products, sales/marketing/distribution activities, processes, equipment, programs, development efforts, therapeutic targets, compounds, assays, know-how, or plans ("Proprietary Information"). Consultant agrees not to disclose any Proprietary Information to third parties or to use any Proprietary Information for any purpose other than performance of the Services.

(b) Proprietary Information subject to the foregoing paragraph does not include information Consultant can demonstrate: (i) is or later becomes available to the public through no breach of this Agreement by Consultant; (ii) is obtained by Consultant from a third party who Consultant had a commercially reasonable basis to believe had the legal right to disclose the information to Consultant; (iii) is already in the possession of Consultant on the Effective Date; or (iv) is required to be disclosed by law, government regulation, or court order; provided, however, that Consultant shall use his best efforts to provide PTC with notice and an opportunity to oppose or limit such disclosure.

(c) Consultant shall not disclose to PTC any confidential information of other parties without the prior written consent of PTC.

(d) Consultant agrees to promptly return, upon termination of this Agreement or at any other time, upon request by PTC, all Proprietary Information and all other materials in Consultant's possession that were either (i) supplied by PTC or its representatives in conjunction with the Services or (ii) generated by Consultant in the performance of the Services and contain or reference Proprietary Information. This obligation of return of materials is in addition to, and shall not be construed to limit, any deliverables with respect to the Services.

INTELLECTUAL PROPERTY

(a) Consultant hereby assigns to PTC any right, title, and interest Consultant may have in any know-how, invention, discovery, improvement, or other intellectual

property which Consultant develops during the course of and as a direct result of performing the Services. Any intellectual property assignable to PTC pursuant to the preceding sentence is hereinafter referred to as "PTC Intellectual Property". Upon the request of PTC, Consultant shall (i) provide such documentation relating to any PTC Intellectual Property, and (ii) execute such further assignments, documents, and other instruments, as may be necessary to assign PTC Intellectual Property to PTC and to assist PTC in applying for, obtaining and enforcing patents or other rights in the United States and in any foreign country with respect to any PTC Intellectual Property. PTC will bear the cost of preparation of all patent or other applications and assignments, and the cost of obtaining and enforcing all patents and other rights to PTC Intellectual Property. Consultant hereby designates PTC as agent, and grants to PTC a power of attorney with full power of substitution, for

the purpose of effecting the foregoing assignments. Consultant agrees not to publish any PTC Intellectual Property without the prior written consent of PTC.

TERM AND TERMINATION

(a) Unless terminated earlier pursuant to the following paragraphs, this Agreement shall terminate on September 30, 2018 (the Effective Date through such termination date being the "Term") or such lesser period as PTC and the Consultant may mutually agree. The parties may extend the Term by mutual written agreement.

(b) PTC may terminate this Agreement immediately on written notice in the case of: (A) a material breach by Consultant (i) of any obligation hereunder, and/or (ii) of any obligation under the Separation Agreement; or (B) Consultant's Misconduct. For purposes of this Agreement, "Misconduct" means: (i) material failure to perform the Services; (ii) any action or omission by Consultant involving willful misconduct or gross negligence with regard to the Company, which has a detrimental effect on the Company; (iii) any action or omission by Consultant which materially adversely affects the business activities, reputation, goodwill or image of the Company; (iv) Consultant's misappropriation of the funds of the Company; or (v) Consultant's disqualification, bar or suspension by any governmental authority from performing any of the Services.

(c) Termination of this Agreement under this section shall not affect (i) PTC's obligation to pay for services previously performed by Consultant or expenses reasonably incurred by Consultant for which Consultant is entitled to reimbursement under this Agreement, (ii) Consultant's rights relating to the Equity Awards which survive termination per their express terms and the express terms of this Agreement or (iii) Consultant's continuing obligations to PTC under the Confidentiality, Intellectual Property, Term and Termination, and Miscellaneous sections of this Agreement. However, except as specifically set forth in this paragraph, Consultant shall not be entitled to any form of continuing compensation or other remuneration of any nature following the termination of this Agreement.

MISCELLANEOUS

(a) Failure of any party to insist, in one or more instances, on performance by the other in strict accordance with the terms and conditions of this Agreement shall not be deemed a waiver or relinquishment of any right granted hereunder or of the future performance of any such term or condition or of any other term or condition of this Agreement, unless such waiver is contained in a writing signed by or on behalf of the waiving party.

(b) This Agreement shall be governed by and construed and enforced in accordance with the laws of the State of New Jersey, U.S.A., without application of the conflicts of law provisions thereof.

(c) PTC may assign its rights and obligations hereunder (i) to any person or entity who succeeds to all or substantially all of PTC's business or that aspect of PTC's business in which Consultant is principally involved, or (ii) to any affiliate wholly-owned by or under common control with PTC. Consultant's rights and obligations under this Agreement are of a personal nature and therefore may not be assigned without the prior written consent of PTC.

(d) This Agreement shall inure to the benefit of and be binding upon the respective heirs, executors, successors, representatives, and authorized assigns of the parties, as the case may be.

(e) The relationship created by this Agreement shall be that of third party contractor, and, as such, the Consultant shall be responsible for the payment of all taxes including, but not limited to, social security and income tax relating to the rendering of the Services and compensation paid to the Consultant pursuant to the terms of this Agreement. Consultant shall not be entitled to any rights, benefits, or privileges of PTC employees, nor shall Consultant have any authority to bind or act as agent for PTC or its employees for any purpose.

(f) Consultant acknowledges that any breach or threatened breach of the terms or conditions of this Agreement will result in substantial, continuing and irreparable injury to PTC. Therefore, Consultant agrees that, in addition to any other remedy that may be available to PTC, PTC shall be entitled to injunctive or other equitable relief by a court of appropriate jurisdiction in the event of any breach or threatened breach of this Agreement, and Consultant waives any requirements relating to the posting of bond or other surety in connection with such injunctive or equitable relief.

(g) Notice or payments given by one party to the other hereunder shall be in writing and deemed to have been properly given or paid if deposited with the United States Postal Service, registered or certified mail, addressed as follows:

If to PTC:

PTC Therapeutics
Attention: Legal Department
100 Corporate Court

with an email copy to: legal@ptcbio.com

If to Consultant:

Mark Rothera
337 Woodmont Circle
Palmer Twp, PA 18045

(h) Consultant represents and warrants that he has never been: (i) excluded, barred from participation in, or sanctioned by any state or federal health care program, including Medicare or Medicaid in the United States, or any similar programs in any other country; (ii) the recipient of a criminal conviction related to any such health care program; or (iii) Debarred (as defined below) or under investigation by any regulatory authority for Debarment action. Consultant will not knowingly use the services of any person who has been Debarred, in any capacity, in connection with the Services. Consultant shall notify PTC in the event that he shall become subject to any of the conditions set forth in this subparagraph (h) during or after the term of this Agreement, and provide PTC upon request with a list of the full names of all persons who have or will supervise, administer or perform any Services. The foregoing obligation shall survive the termination or expiration of this Agreement. “Debarred” or “Debarment” in relation to a person or an entity means, as applicable, a person or entity subject to limitations or any form of endorsement (x) under the Generic Drug Enforcement Act or by the FDA (including persons or entities required to be listed under Section 306(k)(2) of the U.S. Food, Drug and Cosmetic Act), or (y) under any competent regulatory authority or other recognized national, multi-national or industry body.

(i) Except as expressly set forth herein (and except for the Separation Agreement), this Agreement replaces all previous agreements and the discussions relating to the subject matters hereof and constitutes the entire agreement between PTC and Consultant with respect to the subject matters of this Agreement; provided, however, that the Equity Awards shall be governed exclusively by the terms of a separate stock option or equity certificate/agreement and the applicable PTC equity plan documents, as amended or otherwise modified by this Agreement. This Agreement may not be modified in any respect by any verbal statement, representation, or agreement made by any employee, officer, or representative of PTC, or by any written documents unless it is signed by an officer of PTC and by Consultant.

(j) For the avoidance of doubt, Consultant’s post-employment obligations to PTC, as set forth under the Employment Agreement and reaffirmed in the Separation Agreement, remain valid and binding and shall continue on in full force and effect.

(k) If any term or provision of this Agreement is deemed invalid, contrary to, or prohibited under applicable laws or regulations of any jurisdiction, the remainder of this Agreement shall remain in effect, and the relevant term or provision shall be limited to the maximum permissible extent.

JURY TRIAL WAIVER

The parties agree to waive any right to a trial by jury regarding any dispute, claim or cause of action arising out of, concerning, or related to, this Agreement or the Services.

[Signature Page Follows]

IN WITNESS WHEREOF, the parties have executed this Agreement effective as of the Effective Date.

CONSULTANT:

/s/ Mark Rothera
Mark Rothera
Date: August 7, 2017

PTC THERAPEUTICS, INC.

/s/ Mark E. Boulding
Name: Mark E. Boulding
Title: CLO & EVP
Date: August 7, 2017

Exhibit 1

Services

Consultant's consulting services shall include but not be limited to:

- (1) Leveraging personal sector network to problem solve around specific strategic topics or tactical initiatives;**
- (2) Advice relating to geographical expansion plans;**
- (3) Support messaging and insights into investor community;**
- (4) Portfolio strategy evaluations and insights; and**
- (5) Ad-hoc issue resolution or information sharing in fields/topics relating to his previous work with PTC**

Schedule A

RESTRICTED SHARES, RESTRICTED STOCK UNITS, AND STOCK OPTIONS

A. Restricted Shares:

Complete List of Consultant's Restricted Shares of Stock ("Restricted Shares"):

9,600 restricted shares granted on January 3, 2017, of which 9,600 are currently unvested.

Treatment of Restricted Shares:

From the Effective Date through and including the date this Agreement is terminated, the 2,400 Restricted Shares scheduled to vest from the Effective Date through September 30, 2018 shall continue to vest and become unrestricted per the regular schedule based on Consultant's continuity of services; and the remaining 7,200 shall immediately cease vesting and be cancelled effective as of the Effective Date, and Consultant shall have no further rights with respect thereto.

When and if the Restricted Shares vest (solely with respect to the Restricted Shares that are not subject to a timely and properly filed election under Section 83 of the Internal Revenue Code, if applicable), the Company shall require the Consultant to satisfy any applicable income and employment tax withholding obligations prior to the Company's issuance of any shares with respect to the release of any such shares.

B. Stock Options Granted Since January 2014:

Complete List of Stock Options Granted in January 2014 or after ("Stock Options Granted Since January 2014") and their Vesting Treatment:

(i) stock options to purchase 56,000 shares granted on January 3, 2017 with an exercise price of \$11.23 per share, comprised of:

(x) 0 which have vested but remained unexercised;

(y) 0 which have vested and been exercised; and

(z) 56,000 which remain unvested, of which (i) the 21,000 scheduled to vest from the Effective Date through September 30, 2018 shall continue to vest per the regular schedule set forth in the applicable stock option agreement from the Effective Date through and including the date this Agreement is terminated in accordance with its terms, based on Consultant's continuity of services under this Agreement; and (ii) the remaining 35,000 shall immediately cease vesting and be cancelled effective as of the Effective Date, and Consultant shall have no further rights with respect thereto.

(ii) stock options to purchase 70,000 shares granted on January 4, 2016 with an exercise price of \$30.86 per share, comprised of:

(x) 26,250 which have vested but remained unexercised;

(y) 0 which have vested and been exercised; and

(z) 43,750 which shall immediately cease vesting effective as of the Effective Date and shall be cancelled and Consultant shall have no further rights with respect thereto.

- (iii) stock options to purchase 69,550 shares granted on January 2, 2015 with an exercise price of \$51.00 per share, comprised of:
 - (x) 43,468 which have vested but remained unexercised;
 - (y) 0 which have vested and been exercised; and
 - (z) 26,082 which remain unvested, shall immediately cease vesting effective as of the Effective Date and shall be cancelled and Consultant shall have no further rights with respect thereto.
- (iv) stock options to purchase 65,000 shares granted on January 28, 2014, with an exercise price of \$27.05 per share, comprised of:
 - (x) 56,875 which have vested but remained unexercised;
 - (y) 0 which have vested and been exercised; and
 - (z) 8,125 which remain unvested, shall continue to vest per the regular schedule set forth in the applicable stock option agreement from the Effective Date through and including the date this Agreement is terminated in accordance with its terms, based on Consultant's continuity of services under this Agreement.

Exercisability of Stock Options Granted Since January 2014:

(ii) *exercise period* :

During the Term and until three (3) months immediately following termination of this Agreement, consistent with and subject to the terms of the applicable stock option agreement/certificate and related documentation, Consultant shall have the right to exercise the vested portion of the Stock Options Granted Since 2014, except those Stock Options which are being cancelled per the terms above; provided, however, that in no event may Consultant exercise any such options beyond the date that is ten (10) years from the initial grant date of any such options as set forth in the applicable option agreement or certificate.

(iii) *change to Non-Qualified Options* :

As a result of Consultant's termination from employment with PTC, any vested Incentive Stock Options exercised after the date that is three (3) months following his last day of employment (i.e., three (3) months following the Effective Date) with PTC shall, by operation of law, be treated as Non-Qualified Stock Options.

(iv) *withholding obligations* :

When and if Consultant exercises any Stock Options, PTC shall require him to satisfy any applicable income and employment tax withholding obligations prior to PTC's issuance of any shares with respect to the options.

C. Stock Options Granted Prior to January 2014:

Complete List of Stock Options Granted prior to January 2014 ("Stock Options Granted Prior to January 2014") :

stock options to purchase 165,000 shares granted on May 15, 2013, with an exercise price of \$10.85 per share, comprised of:

- (x) 75,352 which have vested but remained unexercised;
- (y) 89,648 which have vested and been exercised; and

(z) 0 which remain unvested.

Exercisability of Stock Options Granted Prior to January 2014:

(i) *exercise period* :

During the Term and until three (3) months immediately following termination of this Agreement, consistent with and subject to the terms of the applicable stock option agreement/certificate and related documentation, Consultant shall have the right to exercise the vested portion of the Stock Options Granted Prior to January 2014; provided, however, that in no event may Consultant exercise any of such options beyond the date that is ten (10) years from the initial grant date of any such options as set forth in the applicable option agreement or certificate.

(ii) *withholding obligations* :

When and if Consultant exercises any Stock Options, PTC shall require him to satisfy any applicable income and employment tax withholding obligations prior to PTC's issuance of any shares with respect to the options.

Certification of Insider Trading Policy:

Consultant will read and execute PTC's current "Consultant Certification Regarding Insider Trading Policy".



PTC Therapeutics Reports Second Quarter 2017 Financial Results and Provides Corporate Update

- *Translarna™ second quarter sales of \$45.8 M representing 197% growth over 2Q2016 -*
- *Over 1,200 patients on EMFLAZA™ 12 weeks into launch -*
- *~400% dose-dependent increase in full length SMN2 mRNA shown in SUNFISH trial interim analysis -*
- *2017 revenue guidance increased to \$155-180 M -*

SOUTH PLAINFIELD, N.J., – Aug. 8, 2017 – PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the second quarter ending June 30, 2017.

“Since our founding nearly 20 years ago, it has been our mission to provide treatments to patients living with rare diseases who have limited treatment options,” said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. “I am proud to have introduced Translarna, the first therapy for nonsense mutation Duchenne muscular dystrophy patients and we remain dedicated to working with patients and their families to bring Translarna to the U.S. We are also pleased with the successful early launch of EMFLAZA. Consistent with our mission, we are working to ensure broad access to all eligible EMFLAZA Duchenne patients regardless of financial or insurance status.”

Second Quarter Financial Highlights:

- Translarna net product sales were \$45.8 million for the second quarter of 2017, representing 197% growth over \$15.4 million reported in the second quarter of 2016.
 - EMFLAZA net product sales were \$2.1 million for the second quarter of 2017.
 - Total revenues for the second quarter of 2017 were \$48.0 million compared to \$15.6 million in the same period of 2016. The change in total revenue was a result of the expanded commercial launch of Translarna and the successful U.S. EMFLAZA launch.
 - GAAP R&D expenses were \$30.8 million for the second quarter of 2017 compared to \$28.8 million for the same period in 2016. Non-GAAP R&D expenses were \$26.9 million for the second quarter of 2017, excluding \$3.9 million in non-cash, stock-based compensation expense, compared to \$24.6 million for the same period in 2016, excluding \$4.1 million in non-cash, stock-based compensation expense and one-time restructuring costs of \$0.1 million. The increase in R&D expense for the second quarter of 2017 as compared to the prior year period was primarily due to start-up of clinical activities and
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- regulatory spend, partially offset by the decreased costs due to the completion of our CF program at the end of 2016.
- GAAP SG&A expenses were \$28.9 million for the second quarter of 2017 compared to \$23.4 million for the same period in 2016. Non-GAAP SG&A expenses were \$24.9 million for the second quarter of 2017, excluding \$4.0 million in non-cash, stock-based compensation expense, compared to \$18.3 million for the same period in 2016, excluding \$4.6 million in non-cash, stock-based compensation expense and one-time restructuring costs of \$0.4 million. The increase in SG&A expenses primarily related to the expansion of the U.S. commercial sales team in support of the launch of EMFLAZA.
 - Net interest expense for the second quarter of 2017 was \$3.0 million compared to net interest expense of \$2.1 million in the same period in 2016. The increase in net interest expense is primarily a result of increased interest expense related to the \$40 million secured loan facility which we closed during the quarter partially offset by reduced interest income from investments.
 - Net loss for the second quarter of 2017 was \$17.5 million compared to a net loss of \$38.9 million for the same period in 2016.
 - Cash, cash equivalents, and marketable securities totaled approximately \$181.1 million at June 30, 2017 compared to approximately \$231.7 million at December 31, 2016.
 - Shares issued and outstanding as of June 30, 2017, were 41.3 million, which includes 0.1 million shares of unvested restricted stock awards.

2017 Guidance:

- Translarna net sales for 2017 are now anticipated to be between \$120 and \$140 million, an increase from prior guidance of \$115 to \$130 million. PTC anticipates EMFLAZA net sales for 2017 to be between \$15 and \$20 million. PTC also anticipates a \$20 million milestone payment in 2017 related to the SMA program for total 2017 revenues between \$155 and \$180 million.
- GAAP operating expenses for the full year 2017 are anticipated to be between \$250 to \$260 million. Excluding estimated non-cash stock-based compensation expense of approximately \$40 million, full year 2017 non-GAAP operating expenses are anticipated to be between \$210 million and \$220 million. These expenses will be primarily in support of the commercial availability of Translarna globally, the commercial launch of EMFLAZA in the U.S. and the continued research and clinical development of other product pipeline candidates.
- PTC expects to end 2017 with over \$120 million of cash and cash equivalents.

Key Second Quarter and Other Corporate Highlights:

- **EMFLAZA™ for the treatment of Duchenne muscular dystrophy successfully launched in the U.S. with establishment of EMFLAZACares Program.** PTC has successfully launched EMFLAZA in the U.S. with over 1200 patients receiving therapy only 12 weeks into the launch. We estimate that there are 9,000 Duchenne patients in the U.S. over the age of five. EMFLAZACares is a program designed to enable all eligible patients to have access to EMFLAZA regardless of financial or insurance status. Based on progress to date, we are raising our 2017 guidance to \$15-20M from \$5-10M.
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- **Translarna™ revenue of \$45.8 M in second quarter, which represents a 197% growth over 2Q2016.** PTC continues to expand on its strong global footprint in Duchenne muscular dystrophy, with sales generated in over 25 countries. Market access discussions regarding funding on a country-by-country basis are ongoing. This strong performance reflects continued uptake, sustainable pricing levels, and high (> 90%) compliance to treatment.
- **NDA for Translarna under FDA review with PDUFA date of October 24, 2017.** The FDA has assigned the New Drug Application (NDA) for ataluren (Translarna™) a Prescription Drug User Fee Act (PDUFA) date of October 24, 2017. The company is preparing for the Advisory Committee Meeting, which is tentatively scheduled for September 28, 2017.
- **SMA clinical program on track to advance to a pivotal phase in the second half of 2017.** The spinal muscular atrophy (SMA) program, a joint collaboration with Roche and the SMA Foundation, is expected to advance into pivotal studies in the second half of 2017. Commencement of the pivotal portion of either study will trigger a \$20 million milestone payment to PTC from Roche. Preliminary data from the first cohort of the SUNFISH trial was presented at the CureSMA Conference and demonstrated a dose dependent increase up to 400% increase in SMN2 transcript. In addition, no toxicities requiring patients' withdrawal had been observed in the clinic to date.

Non-GAAP Financial Measures:

In this press release, the financial results and financial guidance of PTC are provided in accordance with accounting principles generally accepted in the United States (GAAP) and using certain non-GAAP financial measures. In particular, non-GAAP financial measures exclude stock-based compensation expense and one-time restructuring expenses relating to the reorganization of operations intended to improve efficiency and better align costs and employment structure with PTC's strategic plans. These non-GAAP financial measures are provided as a complement to results reported in GAAP because management uses these non-GAAP financial measures when assessing and identifying operational trends. In management's opinion, these non-GAAP financial measures are useful to investors and other users of PTC's financial statements by providing greater transparency into the operating performance at PTC and the company's future outlook. Quantitative reconciliations of GAAP financial measures are included in the tables below.

PTC Therapeutics, Inc.
Consolidated Statements of Operations
(In thousands, except per share data)
Unaudited

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Revenues:				
Net product revenue	\$ 47,891	\$ 15,437	\$ 74,334	\$ 34,314
Collaboration and grant revenue	71	196	176	214
Total revenues	47,962	15,633	74,510	34,528
Operating expenses:				
Cost of product sales	758	—	797	—
Research and development (1)	30,835	28,827	58,198	60,226
Selling, general and administrative (2)	28,866	23,366	54,365	49,304
Total operating expenses	60,459	52,193	113,360	109,530
Loss from operations	(12,497)	(36,560)	(38,850)	(75,002)
Interest expense, net	(3,008)	(2,060)	(5,227)	(4,016)
Other expense, net	(1,820)	(387)	(2,139)	(1,107)
Loss before income tax expense	(17,325)	(39,007)	(46,216)	(80,125)
Income tax (expense) benefit	(150)	93	(316)	(22)
Net loss attributable to common stockholders	\$ (17,475)	\$ (38,914)	\$ (46,532)	\$ (80,147)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	39,621,738	34,000,333	36,978,528	33,959,751
Net loss per share—basic and diluted (in dollars per share)	\$ (0.44)	\$ (1.14)	\$ (1.26)	\$ (2.36)
(1) Research and development reconciliation				
GAAP research and development	\$ 30,835	\$ 28,827	\$ 58,198	\$ 60,226
Less: share-based compensation expense	3,895	4,087	8,362	8,415
Less: one-time restructuring cost	—	118	—	834
Non-GAAP research and development	\$ 26,940	\$ 24,622	\$ 49,836	\$ 50,977
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	\$ 28,866	\$ 23,366	\$ 54,365	\$ 49,304
Less: share-based compensation expense	3,990	4,649	8,552	9,236
Less: one-time restructuring cost	—	430	—	1,617
Non-GAAP selling, general and administrative	\$ 24,876	\$ 18,287	\$ 45,813	\$ 38,451

PTC Therapeutics, Inc.
Summary Consolidated Balance Sheets
(In thousands, except per share data)
Unaudited

	June 30, 2017	December 31, 2016
Cash, cash equivalents and marketable securities	\$ 181,069	\$ 231,666
Total assets	\$ 383,078	\$ 269,345
Total debt	\$ 141,242	\$ 98,216
Total deferred revenue	6,430	1,587
Total liabilities	\$ 213,347	\$ 149,762
Total stockholders' equity (41,304,008 and 34,169,410 common shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively)	169,731	119,583
Total liabilities and stockholders' equity	\$ 383,078	\$ 269,345

Upcoming Events:

PTC management will present a company update at the upcoming Citi 12th Annual Biotech Conference on Thursday, September 7th. The presentation will be webcast live on the Events and Presentations page under the Investor Relations section of PTC Therapeutics website at www.ptcbio.com and will be archived for 2 weeks following the presentation. It is recommended that users connect to PTC's website several minutes prior to the start of the webcast to ensure a timely connection. PTC's current Investor Presentation is available at the same website location.

Today's Conference Call and Webcast Reminder:

Today's conference call will take place at 4:30 PM ET and can be accessed by dialing (877) 303-9216 (domestic) or (973) 935-8152 (international) five minutes prior to the start of the call and providing the passcode 58898313. A live, listen-only webcast of the conference call can be accessed on the Investor Relations section of the PTC website at www.ptcbio.com. A webcast replay of the call will be available approximately two hours after completion of the call and will be archived on the company's website for two weeks.

About PTC Therapeutics

PTC is a global biopharmaceutical company focused on the discovery, development, and commercialization of novel medicines using our expertise in RNA biology. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders and oncology. PTC has discovered all of its compounds currently under development using its proprietary technologies. Since its founding nearly 20 years ago, PTC'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.

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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 the information provided under the heading "2017 Guidance" and statements regarding: the future expectations, plans and prospects for PTC; the size of the DMD patient population eligible for EMFLAZA treatment in the U.S.; the PDUFA date and FDA advisory committee meeting date for the Translarna NDA; advancement of PTC's joint collaboration program in SMA, including whether and when Sunfish or Firefish may transition into the pivotal part of the applicable study and whether and when a milestone payment to PTC from Roche may be triggered; 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 "guidance", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: 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; 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 outcome of pricing, coverage and reimbursement negotiations with third party payors for EMFLAZA and Translarna; whether, and to what extent, third party payors impose additional requirements before approving EMFLAZA prescription reimbursement; PTC's

ability to resolve the matters set forth in the Refuse to File letter it received from the FDA in connection with its NDA for Translarna for the treatment of nmDMD, including whether PTC's filing of the NDA over protest with the FDA will result in a timely or successful review of the NDA, and whether PTC will be required to perform additional clinical and non-clinical trials or analyses at significant cost, which, if successful, could potentially support the approval of the NDA filed over protest or a new NDA submission; the recommendation the advisory committee provides to the FDA for Translarna for the treatment of nmDMD; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area (EEA), including whether the European Medicines Agency (EMA) determines in future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; PTC's ability to enroll, fund, complete and timely submit to the EMA the results of Study 041, a randomized, 18-month, placebo-controlled clinical trial of Translarna for the treatment of nmDMD followed by an 18-month open label extension, which is a specific obligation to continued marketing authorization in the EEA; the eligible patient base and commercial potential of Translarna, EMFLAZA and PTC's other product candidates; the enrollment and conduct of studies under the SMA collaboration and events during, or as a result of, the studies that could delay or prevent further development of under the program; PTC's scientific approach and general development progress; PTC's ability to satisfy its obligations under the terms of the senior secured term loan facility with MidCap Financial; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; and the factors discussed in the "Risk Factors" section of PTC's most recent Quarterly Report on Form 10-Q as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC. You are urged to carefully consider all such factors.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Translarna or EMFLAZA.

The forward-looking statements contained herein represent PTC's views only as of the date of this press release and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this press release except as required by law.