PTC THERAPEUTICS, INC.

FORM 8-K
(Current report filing)

Filed 10/25/17 for the Period Ending 10/25/17

Address	100 CORPORATE COURT
	SOUTH PLAINFIELD, NJ, 07080-2449
Telephone	9082227000
CIK	0001070081
Symbol	PTCT
SIC Code	2834 - Pharmaceutical Preparations
Industry	Biotechnology & Medical Research
Sector	Healthcare
Fiscal Year	12/31

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): **October 25, 2017**

PTC THERAPEUTICS, INC.

(Exact Name of Company as Specified in Charter)

Delaware	**001-35969**	**04-3416587**
(State or Other Jurisdiction of Incorporation)	(Commission File Number)	(IRS Employer 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):

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

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

Emerging growth company ☐

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

Item 8.01. Other Events

On October 25, 2017, PTC Therapeutics, Inc. issued a press release announcing that it received a Complete Response letter from the U.S. Food and Drug Administration (FDA) regarding its New Drug Application (NDA) for Translarna (ataluren).

The press release is attached hereto as Exhibit 99.1 and is incorporated herein by reference.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press Release, dated October 25, 2017

Signature

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this Report to be signed on its behalf by the undersigned hereunto duly authorized.

PTC Therapeutics, Inc.

Date: October 25, 2017

By: /s/ Christine Utter
Name: Christine Utter
Title: Principal Accounting Officer

PTC Therapeutics Receives Complete Response Letter for Ataluren's NDA

SOUTH PLAINFIELD, N.J., October 25, 2017 – PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the Office of Drug Evaluation I of the U.S. Food and Drug Administration (FDA) has issued a complete response letter (CRL) for the New Drug Application (NDA) of the investigational medicine ataluren for the treatment of nonsense mutation dystrophinopathies.

"We are extremely disappointed for the Duchenne community and strongly disagree with the agency's conclusions," said Stuart W. Peltz, Ph.D., chief executive officer of PTC Therapeutics. "We believe that this decision fails to consider the benefit-risk of ataluren and the high unmet medical need. Therefore, we plan to file a formal dispute resolution request next week."

The letter from the Office of Drug Evaluation I of the FDA stated that it is unable to approve the application in its current form. Specifically, the letter indicated that evidence of effectiveness from an additional adequate and well-controlled clinical trial(s) will be necessary at a minimum to provide substantial evidence of effectiveness. The letter also mentioned other nonclinical and CMC matters that PTC is in the process of addressing.

About ataluren (Translarna™)
Ataluren, discovered and developed by PTC Therapeutics, Inc., is a protein restoration therapy designed to enable the formation of a functioning protein in patients with genetic disorders caused by a nonsense mutation. A nonsense mutation is an alteration in the genetic code that prematurely halts the synthesis of an essential protein. The resulting disorder is determined by which protein cannot be expressed in its entirety and is no longer functional, such as dystrophin in Duchenne muscular dystrophy. Translarna, tradename of ataluren, is licensed in the European Economic Area for the treatment of nonsense mutation Duchenne muscular dystrophy in ambulatory patients aged five years and older. Ataluren is an investigational new drug in the United States.

About Duchenne Muscular Dystrophy
Primarily affecting males, Duchenne muscular dystrophy (DMD) is a rare and fatal genetic disorder that results in progressive muscle weakness from early childhood and leads to premature death in the mid-twenties due to heart and respiratory failure. It is a progressive muscle disorder caused by the lack of functional dystrophin protein. Dystrophin is critical to the structural stability of skeletal, diaphragm, and heart muscles. Patients with DMD can lose the ability to walk as early as age ten, followed by loss of the use of their arms. DMD patients subsequently experience life-threatening lung complications, requiring the need for ventilation support, and heart complications in their late teens and twenties. It is estimated that a nonsense mutation is the cause of DMD in approximately 13 percent of patients.

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 contained in this release are forward-looking statements, including statements regarding the future expectations, plans and prospects for PTC; PTC's plans for further interactions with the FDA; the outcome of any formal dispute resolution request; the clinical utility and potential advantages of Translarna (ataluren); PTC's strategy, future operations, future financial position, future revenues or projected costs; and the objectives of management. Other forward-looking statements may be identified by the words "look forward", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including PTC's ability to resolve the matters set forth in the Complete Response letter it received from the FDA in connection with its NDA for Translarna for the treatment of nmDMD either via outcome of any formal dispute resolution request or other interactions with the FDA, and PTC’s ability to perform additional clinical trials, non-clinical studies, and CMC assessments or analyses at significant cost; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area, 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; the eligible patient base and commercial potential of Translarna and PTC's other product candidates; PTC's ability to commercialize and commercially manufacture Translarna in general and specifically as a treatment for nmDMD; the outcome of pricing and reimbursement negotiations in those territories in which PTC is authorized to sell Translarna for the treatment of nmDMD; the outcome of ongoing or future clinical studies in Translarna; expectations for regulatory approvals; PTC's ability to meet existing or future regulatory standards with respect to Translarna; 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.

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.