PTC THERAPEUTICS, INC.
Form 8-K
October 15, 2015

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 15, 2015**

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):

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))

Item 7.01. Regulation FD Disclosure.

On October 15, 2015, PTC Therapeutics, Inc. (PTC or the Company) issued a press release announcing the results from ACT DMD, its Phase 3, double-blind, placebo-controlled, 48-week clinical trial to evaluate the efficacy and safety of Translarna (ataluren) in patients with Duchenne muscular dystrophy caused by nonsense mutations, or nmDMD. PTC will host a conference call Thursday, October 15, 2015, at 5:00 p.m. ET to discuss the results of ACT DMD.

The full text of the press release announcing the results of ACT DMD as well as information on how to access the conference call and accompanying slide presentation is attached as Exhibit 99.1 hereto and is incorporated by reference into this Item 7.01.

The information set forth in or incorporated by reference into this Item 7.01 shall not be deemed to be filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the Exchange Act), or otherwise subject to the liability of that section, and shall not be incorporated by reference into any registration statement or other document filed under the Securities Act of 1933, as amended, or the Exchange Act, except as shall be expressly set forth by specific reference in such filing.

Item 8.01. Other Events.

On October 15, 2015, the Company announced results from ACT DMD. The ACT DMD trial results showed clinically meaningful benefits for Translarna-treated patients. In the overall intent-to-treat study population, the primary endpoint of change from baseline in the 6-minute walk test, or 6MWT, demonstrated a 15 meter benefit (p=0.213), which was not statistically significant. A highly significant benefit of 47 meters (p=0.007) was demonstrated in the pre-specified patient population of 300-400 meters at baseline as measured by the 6MWT, which is in line with the Company s prior experience in its randomized, double-blind, placebo controlled, dose ranging Phase 2b clinical trial evaluating the long term efficacy and safety of Translarna in patients with nmDMD, or the Phase 2b trial, and consistent with the evolving understanding of the 6MWT. Importantly, no patients in this group lost ambulation (0/47) versus four patients in the placebo group (4/52). Translarna showed a benefit over placebo across key secondary and tertiary endpoints, including timed function tests (10 meter Run/Walk, 4 Stair Climb, 4 Stair Descend) and the North Star Ambulatory Assessment test. In addition, a pre-specified meta-analysis of the combined placebo-controlled ACT DMD and Phase 2b trials demonstrated a statistically significant benefit of Translarna across the primary (p=0.015) and key secondary endpoints.

After the completion of ACT DMD, both placebo and treated patients were given the opportunity to continue on Translarna in an open-label extension study. Ninety-seven percent of the patients who completed ACT DMD enrolled in the extension study.

ACT DMD, the largest placebo-controlled study ever conducted in patients with DMD, is a multi-center, randomized, double-blind, Phase 3 clinical trial involving 228 patients in 53 sites across 18 countries. Patients between the ages of 7 and 16 with nmDMD were randomized to receive either Translarna 40mg/kg per day (n=114) or placebo (n=114) over 48 weeks. The primary endpoint was change from baseline in the 6MWT. Analyses of data from pre-specified subgroups, including the pre-specified subgroup of patients with baseline 6-minute walk distance (6MWD) of 300 400 meters, was also completed. Key secondary outcome measures were timed-function tests, including time to run or walk 10 meters and the time to ascend or descend four stairs. Tertiary endpoints included the North Star Ambulatory Assessment test, a functional scale designed for ambulant boys affected by DMD, and the Pediatric Outcomes Data Collection Instrument (PODCI), a validated tool for measuring quality of life in pediatric patients with orthopedic conditions. Supportive analyses of ambulation were conducted, including the proportion of patients with at least 10% worsening in 6MWD. A pre-specified meta-analysis of combined data from the ACT DMD and Phase 2b (ambulatory decline phase) studies was also performed.

The ACT DMD study confirmed the favorable safety profile of Translarna, which was generally well-tolerated, consistent with results from previous studies. More than 500 nmDMD patients have now received Translarna, the largest population to be treated with a disease-modifying agent in DMD.

Item 9.01. Financial Statements and Exhibits.

99.1	Press Release dated October 15, 2015

SIGNATURES

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 15, 2015

By: /s/ Shane Kovacs
Shane Kovacs
Chief Financial Officer

EXHIBIT INDEX

Exhibit No.	Description
99.1	Press Release dated October 15, 2015