

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): **January 10, 2018**

PTC THERAPEUTICS, INC.

(Exact Name of Company as Specified in Charter)

Delaware

(State or Other Jurisdiction
of Incorporation)

001-35969

(Commission
File Number)

04-3416587

(IRS Employer
Identification No.)

100 Corporate Court

South Plainfield, NJ

(Address of Principal Executive Offices)

07080

(Zip Code)

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

Not applicable

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

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

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

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

Emerging growth company

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

Item 7.01. Regulation FD Disclosure.

On Wednesday, January 10th at 2:30 pm PT at the 36th Annual J.P. Morgan Healthcare Conference (the "Conference"), PTC Therapeutics, Inc. (the "Company") will highlight the Company's 20-year commitment to bring best-in-class therapies with differentiated clinical benefit to patients affected by rare disorders, the company's 2018 strategic priorities, preliminary 2017 financial results and 2018 financial guidance. The presentation will be webcast live and the accompanying slide deck has been posted on the Events and Presentations page under the Investors section of the Company's website. A copy of the slide deck, which the Company intends to utilize in various meetings at the Conference, is also attached as Exhibit 99.1.

The information in this Current Report on Form 8-K, including Exhibit 99.1, shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

<u>Exhibit No.</u>	<u>Description</u>
99.1	Corporate Presentation - 36th Annual J.P. Morgan Healthcare Conference

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: January 10, 2018

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



**PTC Therapeutics:
20 years of commitment to bringing
new treatments to patients with rare disorders**

JANUARY 2018



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 presentation, are forward-looking statements, including statements regarding: the future expectations, plans and prospects for PTC; PTC's preliminary 2017 unaudited results; PTC's financial guidance for 2018; PTC's plans for further interactions with the FDA regarding the Translarna NDA; the outcome of any formal dispute resolution request filed with the FDA; the size of the DMD patient population eligible for Emlaza treatment in the U.S.; expansion of Translarna globally; the timing of, and PTC's ability to, expand the approved product label of Translarna for the treatment of nMDMD in the EEA, in pediatric patients, or otherwise; advancement of PTC's studies of Translarna for the treatment of other indications and its other pipeline programs; advancement of PTC's joint collaboration program in SMA; the clinical utility and potential advantages of Translarna (ataluren) and Emlaza™ (deflazacort); 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 "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 Emlaza, 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 Emlaza, 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 Emlaza and Translarna; whether, and to what extent, third party payors impose additional requirements before approving Emlaza prescription reimbursement; 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 (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, Emlaza 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 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.

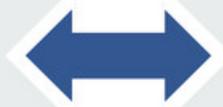
As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including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

The forward-looking statements contained herein represent PTC's views only as of the date of this presentation 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 presentation except as required by law.

Our vision

Our mission

To bring best in-class therapies with **clinical benefit** to **patients** affected by **rare disorders**



Our strategy

To leverage our global commercial infrastructure to **maximize value for patients and all stakeholders**

Sustainable, growing DMD business enables continued innovation & growth

Total 2017 unaudited revenue of \$195 million

1) Growing global DMD franchise with unaudited 2017 revenue of \$174 million

- Translarna™ strong continued patient uptake reflected in annual growth
- Emflaza™ impressive launch; opportunity to establish standard of care

(\$ million)	2017 Unaudited Revenue	2018 Financial Guidance
Translarna	\$145	\$170 - \$185
Emflaza	\$29	\$90 - \$110
DMD Franchise	\$174	\$260 - \$295

2) Leveraging internal splicing technology platform

- Spinal Muscular Atrophy program in pivotal stage; triggered \$20 million milestone in Q4:17
- Huntington's Disease program developing

Survival data on SMA Type 1 babies will be presented at EU SMA congress in January 2018

3) Pursuing internal and in-licensing value-creation opportunities

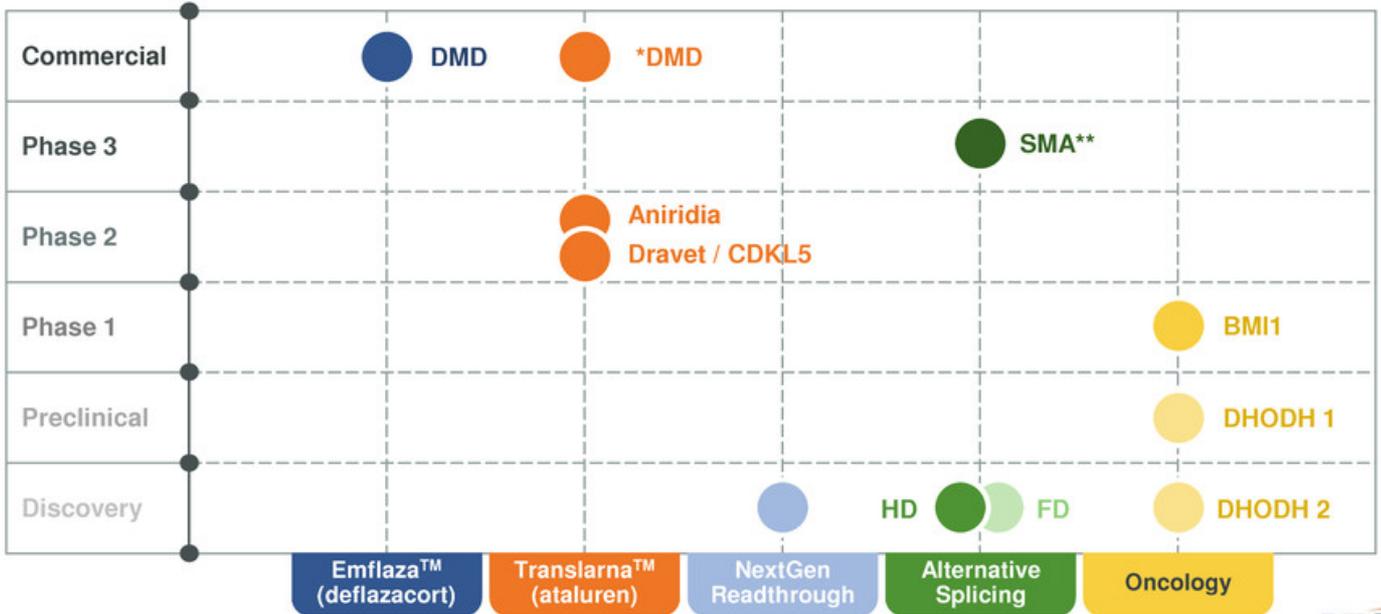
- Leverage our strong global commercial infrastructure

Poised to pursue new assets and maximize value

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Expanding pipeline through in-house innovation



* MA requires annual renewal following reassessment by the European Medicines Agency (EMA)

** Phase 3 Sunfish clinical study of RG7916 in SMA type 2/3 patients began in Nov 2016, Firefish pivotal stage in SMA type 1 patients to begin in coming months

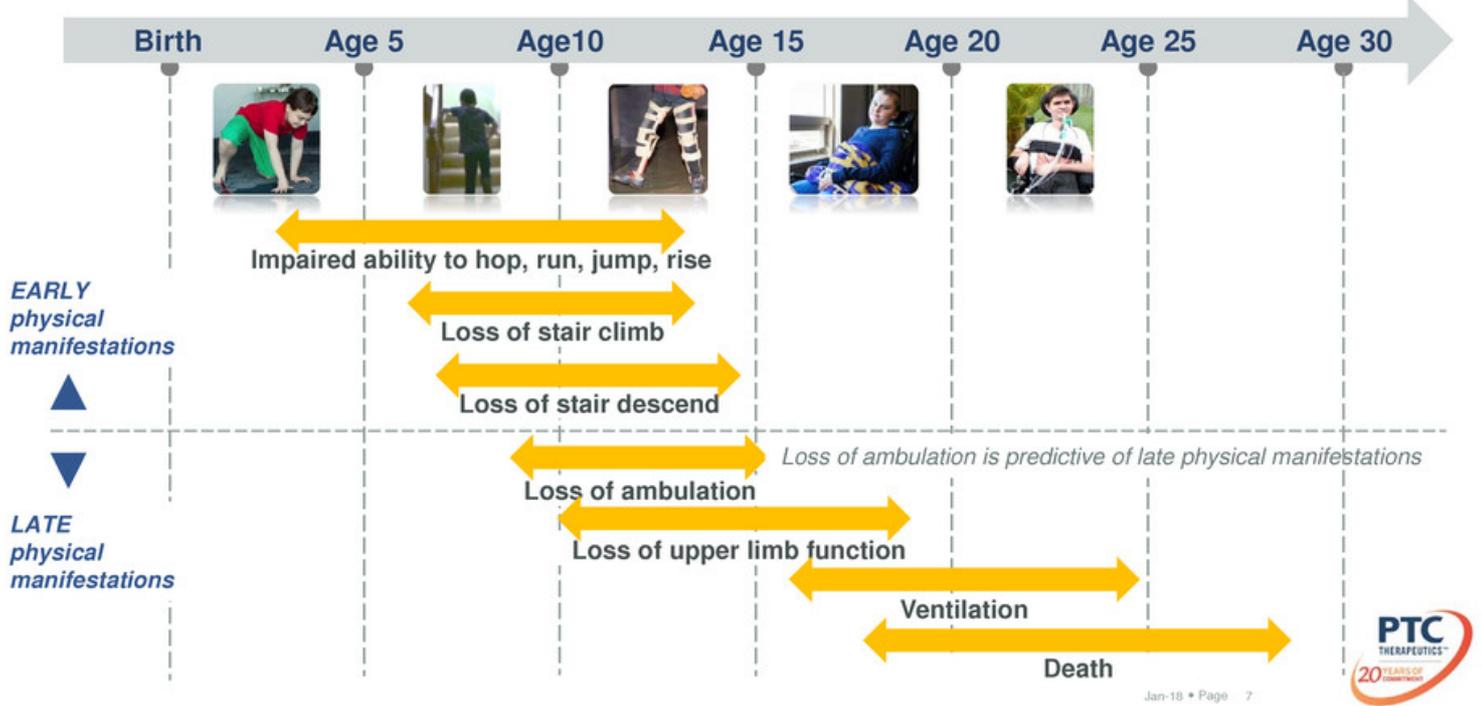




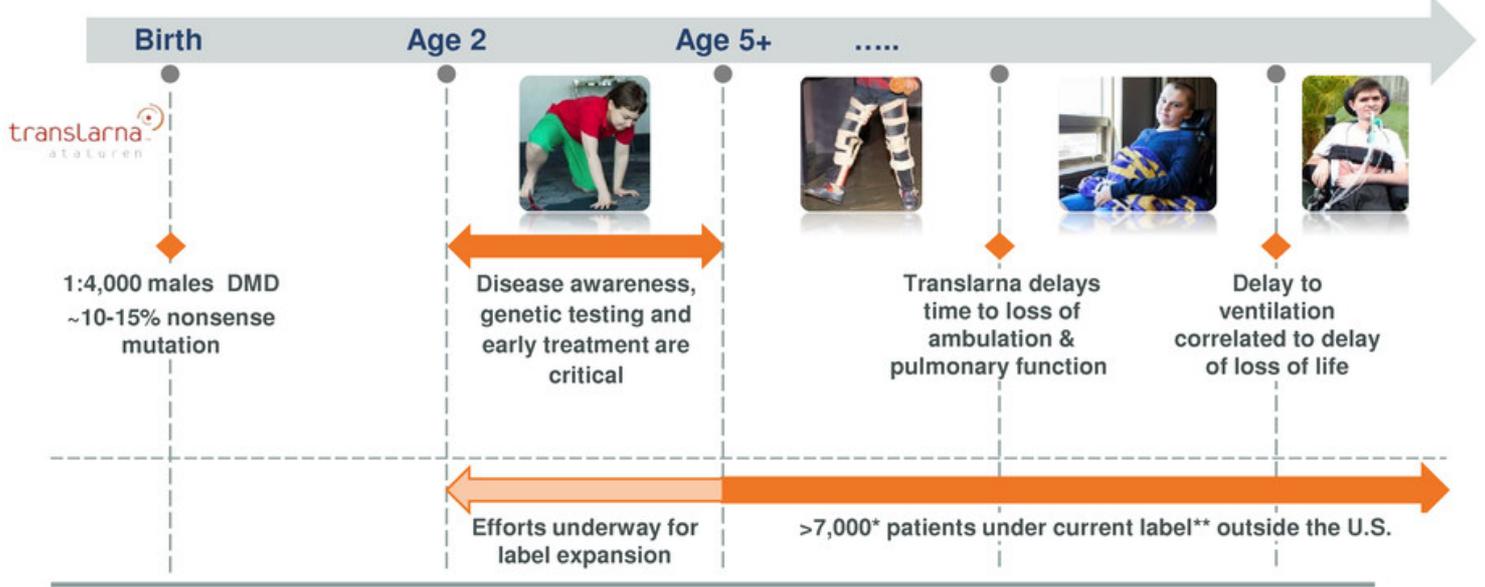
Translarna™ & Emflaza™

Transforming the standard of care for DMD patients

DMD progression is sequential, non-linear and irreversible



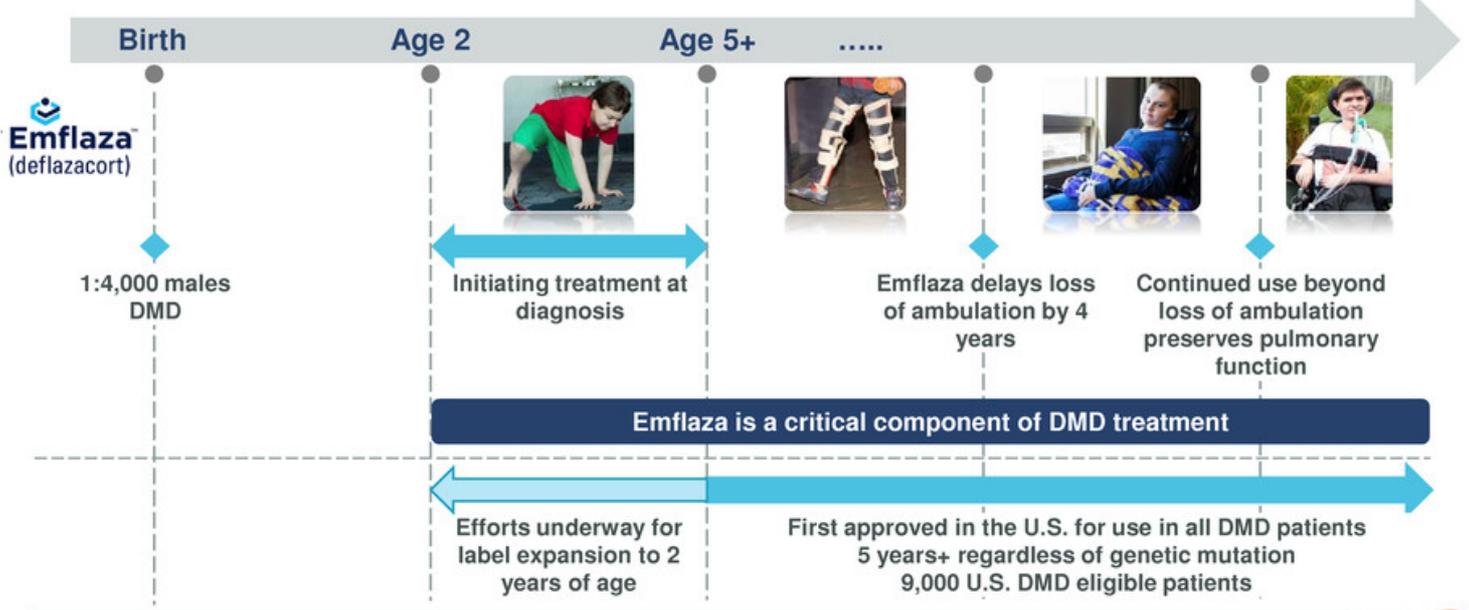
Translarna™: Addressing the underlying cause of nonsense mutation Duchenne Muscular Dystrophy



**Patients are diagnosed worldwide far too late
Label expansion efforts underway for earlier treatment**



Establishing Emflaza™ : Establishing as part of standard of care for U.S patients



Earlier / longer treatment extends time to loss of function



Translarna™: Proven track record of successful global sales

- Unaudited 2017 net revenue of \$145 M
- Guidance for 2018 of \$170 - \$185 M
- Global sales outside of the U.S. since 2014
- Pediatric expansion under EMA review
- U.S. appeal process underway, expect update in Q1

~15%

Expected 5 year
(12/31/17-12/31/22)
CAGR



Emflaza™: Establishing standard of care for all DMD patients in the U.S.



- 2017 Emflaza unaudited net sales of \$29 M
- Guidance for 2018 of \$90 - \$110 M
- Results from Lancet publication reinforce Emflaza efficacy differentiation

Articles

Long-term effects of glucocorticoids on function, quality of life, and survival in patients with Duchenne muscular dystrophy: a prospective cohort study

Craig M McDonald, Erik K Herrickson, Richard F Albrecht, Tina Dang, Namita C Joyce, Fengming He, Paula R Clemens, Eric P Hoffmann, Arvid Crans, Heather Gerlich-Drossner, and the CMDC Investigators*

Summary

Background Glucocorticoid treatment is recommended as a standard of care in Duchenne muscular dystrophy; however, few studies have assessed the long-term benefits of this treatment. We examined the long-term effects of glucocorticoids on milestone-related disease progression across the lifespan and survival in patients with Duchenne muscular dystrophy.

Methods For this prospective cohort study, we enrolled male patients aged 2–25 years with Duchenne muscular dystrophy at 20 centers in nine countries. Patients were followed up for 10 years. We compared no glucocorticoid treatment or cumulative treatment duration of less than 1 month versus treatment of 1 year or longer with regard to progression of nine disease-related and clinically meaningful mobility and upper limb milestones. We used Kaplan-Meier analyses to compare glucocorticoid treatment groups for time to stand from supine of 5 s or longer and 10 s or longer, and loss of stand from supine, four-stair climb, ambulation, full overhead reach, hand-to-mouth function, and hand function. Risk of death was also assessed. This study is registered with ClinicalTrials.gov, number NCT01046832.

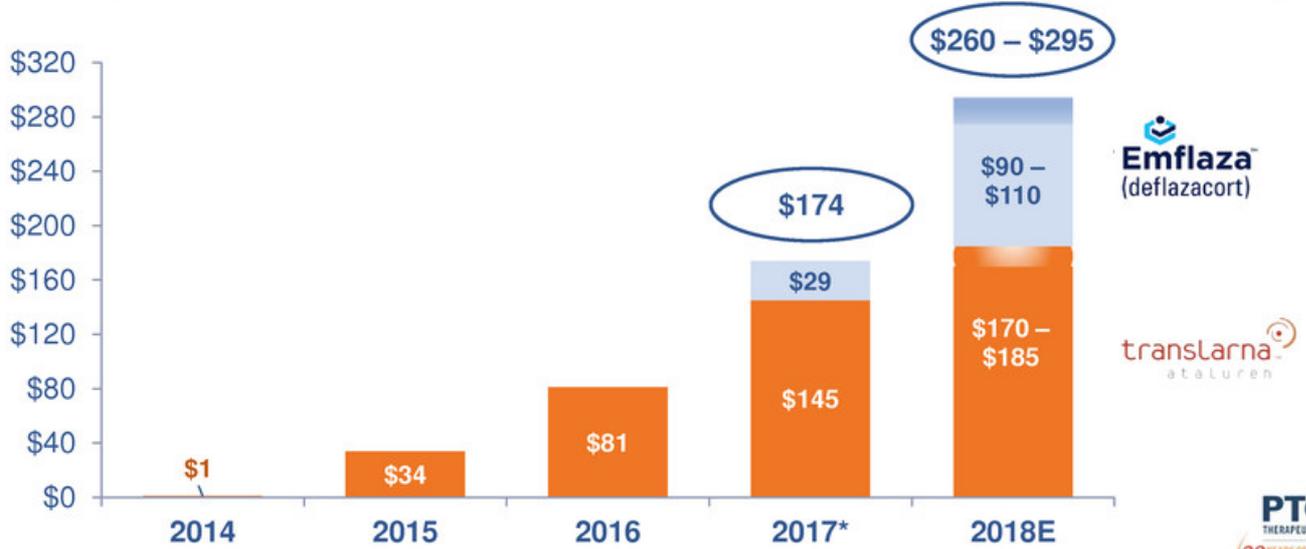
Published online
November 22, 2017
[http://dx.doi.org/10.1016/S1473-3099\(17\)33400-4](http://dx.doi.org/10.1016/S1473-3099(17)33400-4)
See related Comment
[http://dx.doi.org/10.1016/S1473-3099\(17\)33400-4](http://dx.doi.org/10.1016/S1473-3099(17)33400-4)
*See appendix pp 21–28 for a full list of study investigators
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SPL, MEd, Assoc. Prof., Vancouver

milestones by 2.8–8.0 years compared with treatment for less than 1 month. Deflazacort was associated with increased median age at loss of three milestones by 2.1–2.7 years in comparison with prednisone or prednisolone (log-rank $p < 0.012$). 45 patients died during the 10-year follow-up. 39 (87%) of these deaths were attributable to Duchenne-related

Growing global DMD franchise

Translarna™ & Emflaza™ DMD Net Sales

(\$ in millions)

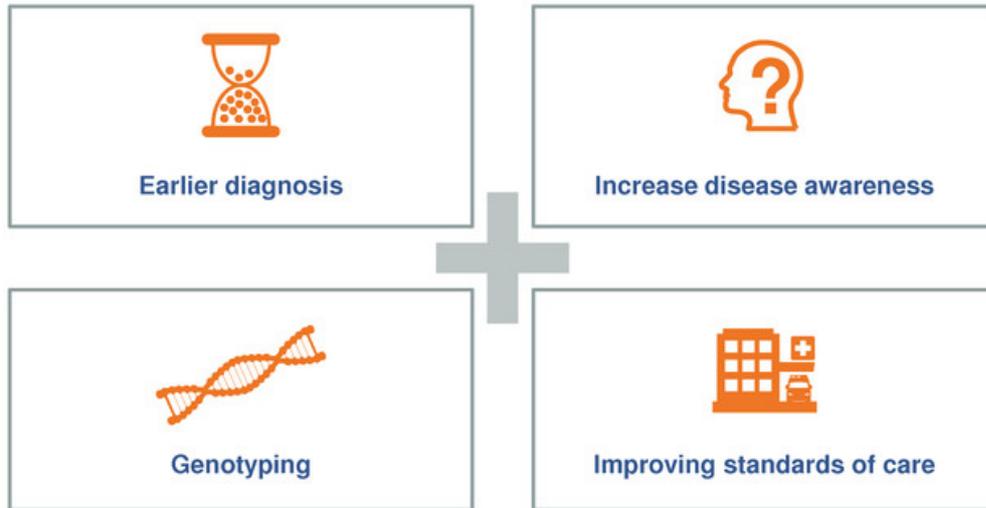


• Unaudited

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Improving patient outcome by delivering best in class therapy earlier to preserve function



Pursuing label expansions to bring Emflaza™ & Translarna™ to younger patients to ultimately improve patient outcome

An efficient, scalable commercialization engine

- 2017 unaudited product net sales of \$174 M
- Established footprint in 47 countries worldwide
- Experienced commercial team in orphan disease
- Fully integrated global commercial infrastructure



South Plainfield,
New Jersey



Zug Switzerland,
Marketing, Medical and
Regulatory Hub

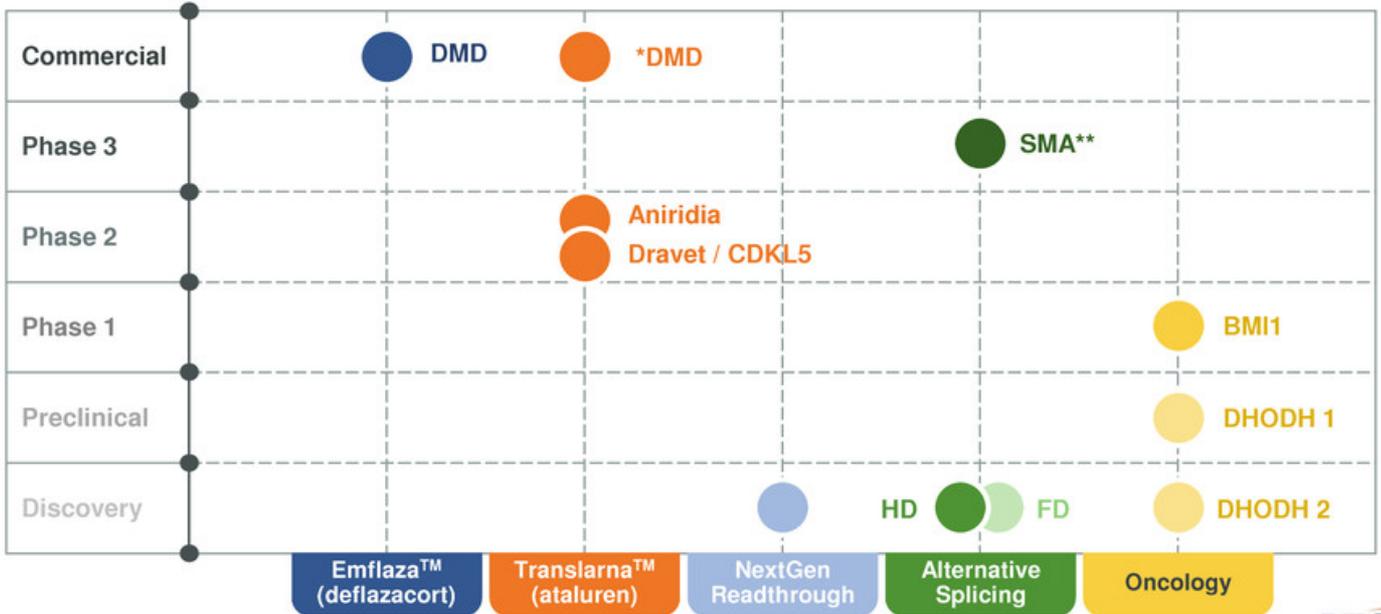


Dublin, Ireland
International HQ



Latam Regional Office, Sao
Paulo, Brazil

Expanding pipeline through in-house innovation



• MA requires annual renewal following reassessment by the European Medicines Agency (EMA)

** Phase 3 Sunfish clinical study of RG7916 in SMA type 2/3 patients began in Nov 2016, Firefish pivotal stage in SMA type 1 patients to begin in coming months

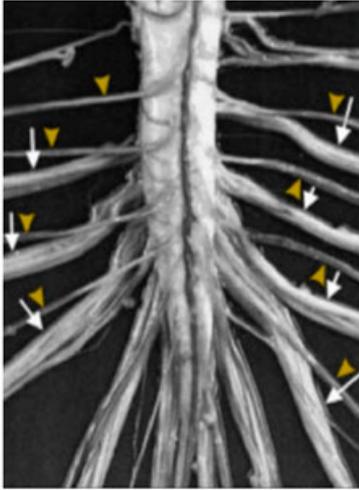




Small molecule splicing technology

Broad platform addressing rare disorders

Spinal Muscular Atrophy: The leading genetic cause of mortality in infants

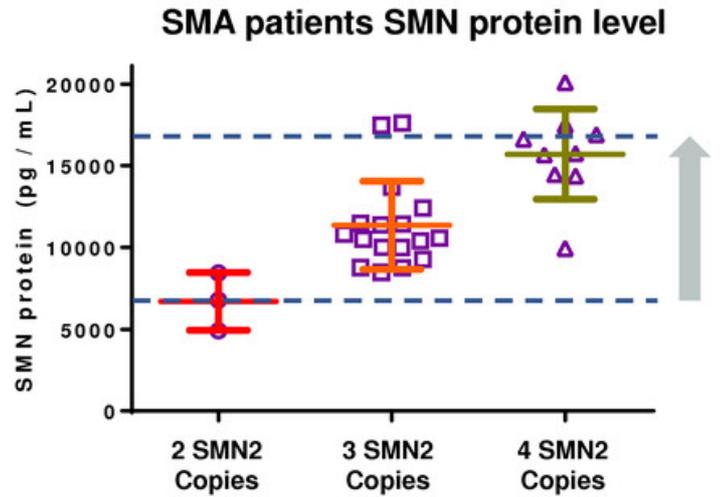


⇨ Dorsal Root
▶ Ventral Root

- Spinal muscular atrophy (SMA) is caused by the loss of SMN1 gene
- Low expression of SMN protein leads to the loss of motor neurons in the spinal cord
- One in every 10,000 children born is affected with the disorder
- Publications demonstrate involvement of muscle, liver, bones and other peripheral tissues in addition to CNS
- SMA program partnered with Roche and the SMA Foundation

RG7916 has the opportunity to be best in class

- Demonstrated complete restoration of SMN RNA in SMA patients
- RG7916 has shown 2.5 fold median increase in protein in patients
- Small molecule has potential for broad tissue distribution key to SMA treatment
- Oral administration



RG7916 has been well tolerated with no drug related safety findings leading to withdrawals

SUNFISH

- Clinical study in SMA type 2/3 patients between 2 and 25 years old
 - Ongoing pivotal part will enroll 168 patients, placebo controlled 2:1, endpoint of total motor function measure (MFM-32) at 12 months

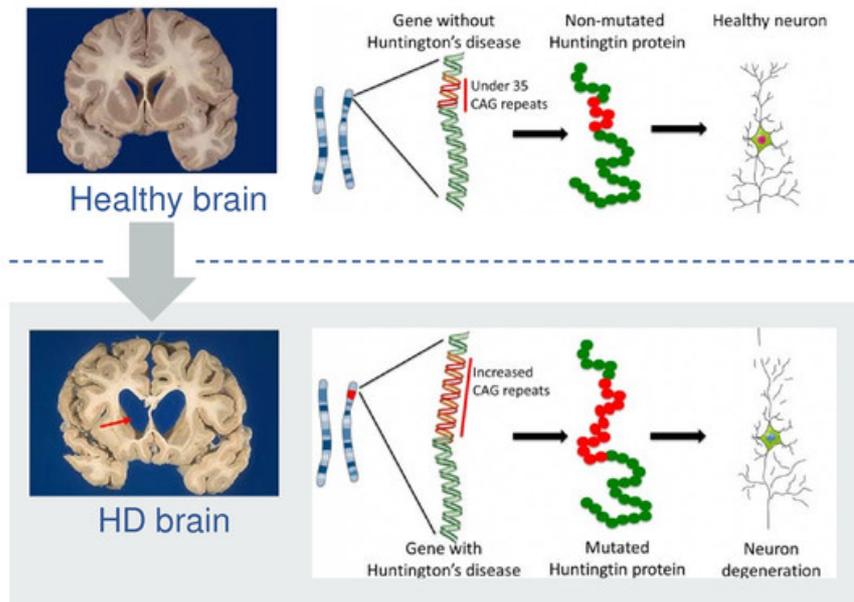
FIREFISH

- Clinical study in SMA type 1 patients between 1 and 7 months old
 - Open label study, completing dose finding part
 - Pivotal part expected to start in coming months, will enroll 40 babies, complete enrollment during 2018
 - Endpoint of sitting unsupported as measured by Bayley infant scales

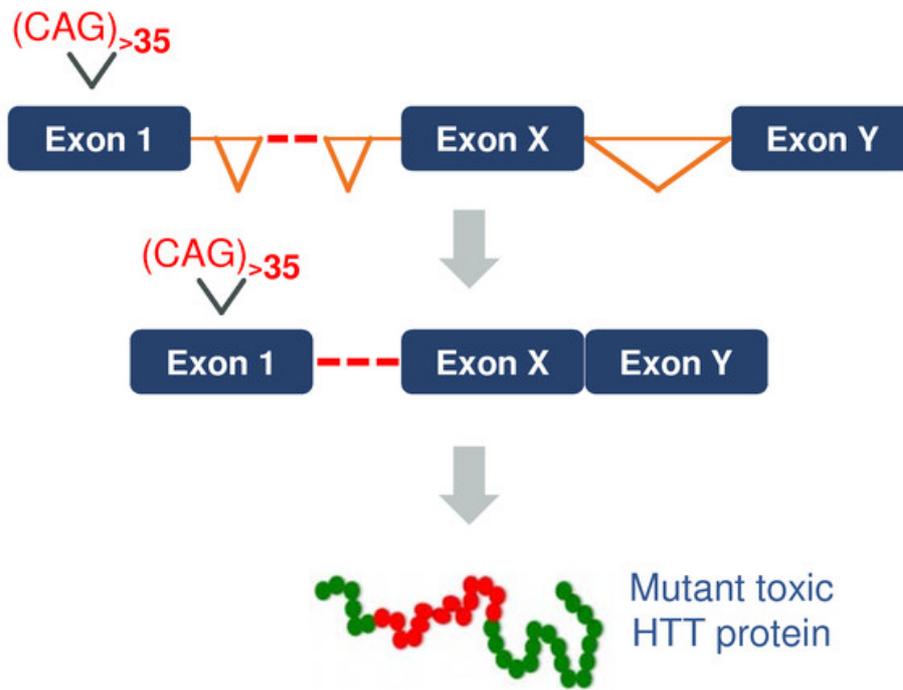
**Firefish dose escalation survival data expected
at EU SMA congress**

Huntington's Disease is caused by a CAG repeat expansion in the HTT gene

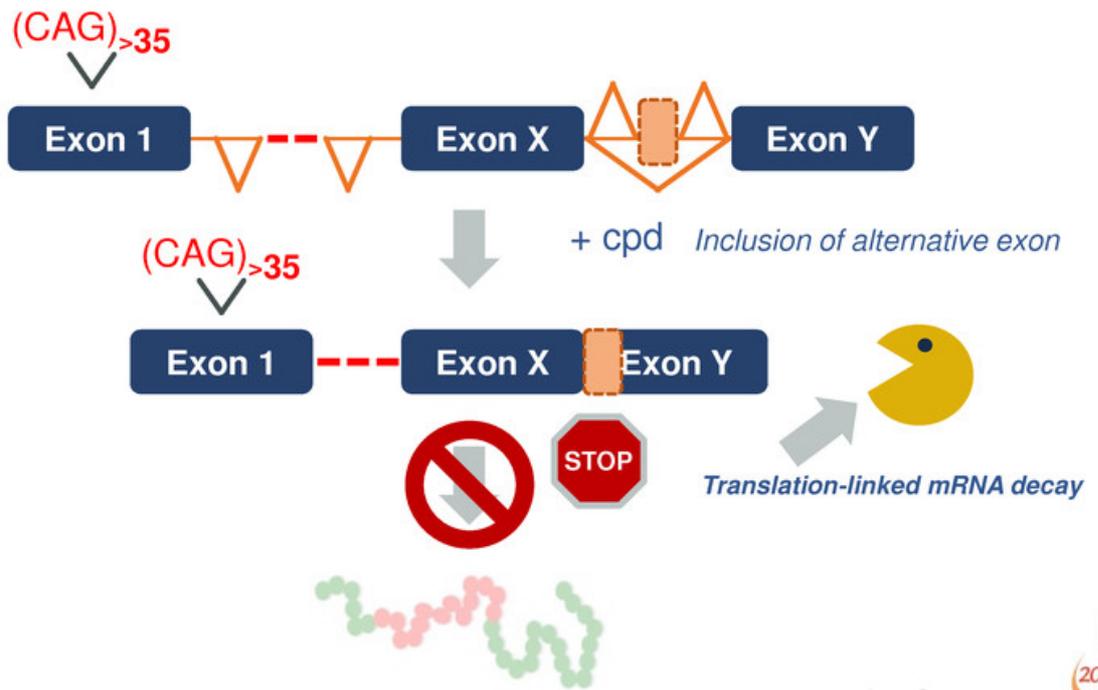
- HD is caused by expression of mutant Huntingtin (HTT) protein due to the CAG repeat expansion
- Neuron degeneration predominantly in the striatum and cerebral cortex
- High unmet medical need:
 - No approved disease modifying treatment



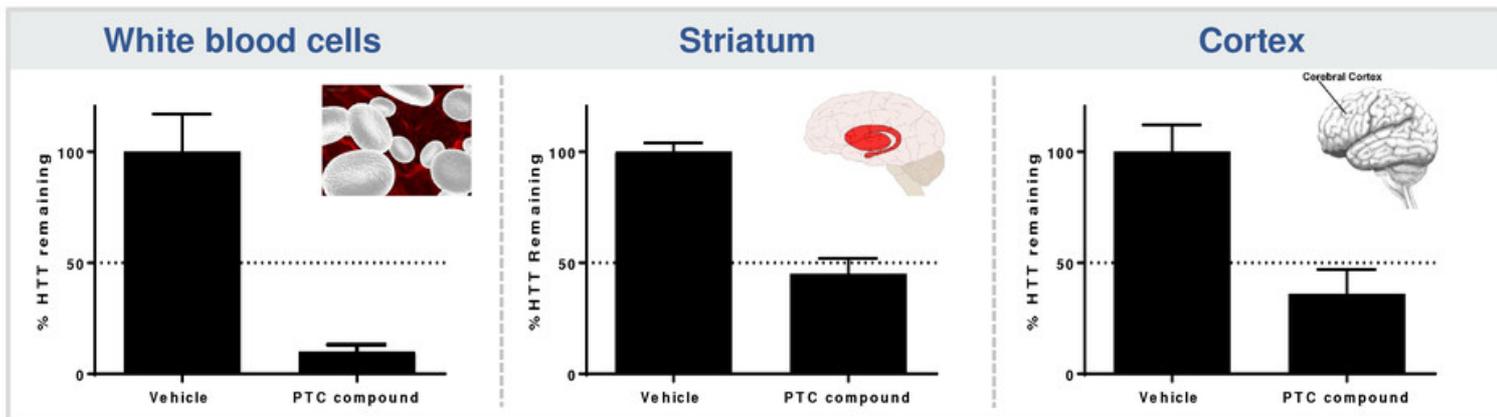
Treating HD by modifying HTT splicing



Opportunity to address HTT by leveraging PTC's splicing platform



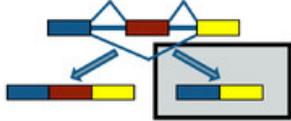
PTC compounds lower human HTT in key regions of the brain and tissues in a mouse model of HD



Optimization has led to the discovery of several classes of small molecules

- ✓ Potent and selective to lowering of HTT mRNA and protein
- ✓ Widely distributed in animals, demonstrating HTT protein lowering in the brain and periphery

PTC's platform technologies target RNA biology to modulate gene expression with small molecules

Platform	Mechanism Targeted	Programs
Splicing	 <p>Target splicing event to restore or reduce protein</p>	SMA – SMN2 FD – IKBKAP HD – HTT



20 years of targeting RNA biology for drug discovery and development



Cutting edge platform technology discovered and developed by PTC



Advancement of SMA program in pivotal clinical trials



Several splicing-targeting programs amendable to several targets

- Currently targeting HD, FD, and others



PTC: Discovering, developing and commercializing clinically differentiated therapies for rare disease

PTC's future events and milestones



SMA RG7916 data at EU SMA



U.S. FDA Translarna™ decision



Analyst Day highlighting pipeline



Establish Emflaza™ as U.S. DMD standard of care



15% CAGR Translarna revenue through 2022

Sustainable, growing DMD business enables continued innovation & growth

Total 2017 unaudited revenue of ~\$195 million

1) Growing global DMD franchise with unaudited 2017 revenue of \$174 million

(\$ million)	2017 Unaudited Revenue	2018 Financial Guidance
Translarna	\$145	\$170 - \$185
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DMD Franchise	\$174	\$260 - \$295

2) Strong sustainable financial position

- 2018 GAAP R&D + SG&A \$280-\$290M
- 2018 non-GAAP R&D +SG&A \$250 - \$260 million (excludes \$30M non-cash stock-based compensation expense)
- 12/31/17 cash position of ~\$191

3) Pursuing internal and in-licensing value-creation opportunities

- Leverage our strong global commercial infrastructure

