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 9, 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** (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):

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 2.02. Results of Operations and Financial Condition.

On January 9, 2017, PTC Therapeutics, Inc. (the “Company”) issued a press release (the “press release”) announcing certain preliminary (unaudited) financial information for its fourth quarter and fiscal year ending December 31, 2016, including that the Company expects to report (i) Translarna™ (ataluren) net sales for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) of approximately $81 million and (ii) ending cash and cash equivalents of approximately $230 million. Final results are subject to completion of the Company’s year-end audit.

Item 7.01. Regulation FD Disclosure.

The Company also announced financial guidance for its fiscal year ending December 31, 2017 in the press release, including that the Company expects (i) ex-U.S. Translarna nmDMD net sales of between $105 and $125 million, assuming current exchange rates; (ii) non-GAAP operating expenses $190 and $200 million, excluding estimated non-cash stock-based compensation expense of approximately $35 million, for total GAAP operating expenses of approximately $225 to $235 million; and (iii) ending cash and cash equivalents of approximately $160 million.

The Company announced that on Wednesday, January 11th at 7:30 am PT at the 35th Annual J.P. Morgan Healthcare Conference (the "Conference"), the Company will present its 2017 strategic priorities, preliminary 2016 financial results, and 2017 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 99.2.

This Current Report on Form 8-K and Exhibits 99.1 and 99.2 include a forward-looking financial measure that was not prepared in accordance with accounting principles generally accepted in the United States (GAAP), non-GAAP operating expenses (which excludes stock-based compensation expense). Management uses this measure to assess its operations and, in management’s opinion, this non-GAAP measure is useful to investors and other users of its financial statements by providing greater transparency into the expected operating performance at PTC and the Company’s future outlook. Non-GAAP financial measures are not an alternative for financial measures prepared in accordance with GAAP.

The information in this Current Report on Form 8-K, including Exhibits 99.1 and 99.2, 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.

Forward Looking Statements: All statements, other than those of historical fact, contained in this Current Report on Form 8-K, are forward-looking statements, including preliminary (unaudited) financial information for 2016 and financial guidance for 2017. The Company'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 the preliminary nature of the Company's 2016 financial information, which is subject to completion of the Company's year-end audit; the assumptions underlying the Company's financial guidance for 2017; and the factors discussed in the “Risk Factors” section of the Company's most recent Quarterly Report on Form 10-Q as well as any updates to these risk factors filed from time to time in the Company's other filings with the SEC. You are urged to carefully consider all such factors. The forward-looking statements contained herein represent the Company's views only as of the date of this Current Report on Form 8-K and the Company 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 Current Report on Form 8-K except as required by law.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits:

99.1	Press Release dated January 9, 2017
99.2	Corporate Presentation - 35th Annual J.P. Morgan Healthcare Conference

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: January 9, 2017

By: /s/ Shane Kovacs

Shane Kovacs

Chief Financial Officer

EXHIBIT INDEX

Exhibit No.	Description
99.1	Press Release dated January 9, 2017
99.2	Corporate Presentation - 35th Annual J.P. Morgan Healthcare Conference

Exhibit 99.1

PTC Therapeutics Provides Corporate Update and Outlines 2017 Strategic Priorities to Maximize the Global Value of Translarna™ and Advance its Innovative Pipeline

– Preliminary 2016 Translarna unaudited net sales of approximately $81M, a 140% increase vs. 2015 –
– Strategic update for Translarna regulatory path in the U.S. –
– Topline ACT CF data anticipated late first quarter 2017 –
– SMA program actively enrolling patients across SUNFISH and FIREFISH trials –
– RG7916 granted orphan-drug designation for the treatment of SMA –
– Translarna 2017 net sales guidance of $105M to $125M –

SOUTH PLAINFIELD, N.J., Jan. 9, 2017 - PTC Therapeutics, Inc. (NASDAQ: PTCT) today provided a corporate update, which will be detailed as part of the company presentation at the 35th Annual
J.P. Morgan Healthcare Conference on Wednesday, January 11th at 7:30 am PT. Stuart W. Peltz, Ph.D., PTC's Chief Executive Officer, will present the company's 2017 strategic priorities, preliminary 2016 financial results and 2017 financial guidance. The presentation will be webcast live and available with the related slide deck on the Events and Presentations page under the investors section of PTC Therapeutics' website at www.ptcbio.com.

Commercial Highlights, Preliminary 2016 Unaudited Financial Results, and 2017 Guidance

- PTC expects to report Translarna (ataluren) net sales for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) of approximately $81 million for 2016, an increase of 140% over the prior year and achieving the upper-end of guidance. This strong performance reflects rapid uptake, sustainable pricing, and high (>90%) compliance to treatment.
- PTC expects to report year-end 2016 cash and cash equivalents of approximately $230 million.
- For 2017, PTC expects to achieve ex-U.S. Translarna nmDMD net sales of between $105 and $125 million, assuming current exchange rates, representing continued strong growth year-over-year of its sustainable DMD business. This is driven by both increased penetration into the over 25 countries where Translarna is currently available as well as continued geographic expansion into new territories.
- Non-GAAP operating expenses for 2017 are expected to be between $190 and $200 million excluding estimated non-cash stock-based compensation expense of approximately $35 million, for total operating expenses of approximately $225 to $235 million.
- PTC expects to finish 2017 with approximately $160 million of cash and cash equivalents.

Clinical and Regulatory Highlights

- Following multiple interactions with U.S. FDA officials and PTC's advisors, PTC plans to file the Translarna New Drug Application (NDA) for nmDMD over protest with the U.S. FDA in the first quarter of 2017. Feedback indicated this process, rather than continued appeal, is the best path forward for the current Translarna NDA to receive a full and fair review. Filing over protest is a procedural path permitted by U.S. FDA regulations that allows a company to have its NDA filed and reviewed when there is a disagreement with regulators over the acceptability of the NDA submission. PTC plans to supplement the current NDA with additional efficacy analyses utilized by the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) in their recent renewal recommendation.

- The EMA’s CHMP recommended the renewal of the conditional marketing authorization for Translarna (ataluren) for the treatment of nmDMD based on a continued positive benefit-risk assessment. As a specific obligation of the renewal, PTC will conduct an additional trial of Translarna in nmDMD.
- Top-line results of ACT CF are anticipated late in the first quarter of 2017. ACT CF is a Phase 3, international, multicenter, randomized, double-blind, placebo-controlled trial that is evaluating the absolute change in percent predicted forced expiratory volume in one second (FEV1) in patients with nonsense mutation cystic fibrosis (nmCF).
- The spinal muscular atrophy (SMA) program, a joint collaboration with Roche and the SMA Foundation, is expected to advance into two pivotal studies in 2017. SUNFISH and FIREFISH are both two part studies in childhood onset (Type 2/3) and infant onset (Type 1) SMA patients, respectively. Both studies are enrolling the initial dose escalation part of the study which will then transition to the pivotal part of the study evaluating efficacy. Commencement of the pivotal portion of either study will trigger a $20 million milestone payment to PTC from Roche. RG7916 was recently granted orphan-drug designation by the U.S. FDA.

Pipeline Highlights:

- Phase 2 proof-of-concept studies of Translarna in additional rare disease indications, including aniridia, MPS I, and Dravet/CDKL5, continue to progress. Proof-of-concept from these studies would further validate Translarna’s potential as a precision medicine for a number of rare genetic disorders caused by a nonsense mutation.
- Clinical development of PTC596 is expected to progress into additional clinical studies in 2017. PTC596 is a novel, oral investigational drug that reduces the levels of BMI1, a protein required for cancer stem cell survival. An ongoing Phase 1 dose escalating study confirms that PTC596 is generally well tolerated at doses that achieved or exceed plasma concentrations in preclinical models.
- PTC’s genetic disorders research organization is actively advancing lead optimization programs from its splicing platform focused on Huntington’s disease and Familial Dysautonomia.

About PTC Therapeutics, Inc.
PTC is a global biopharmaceutical company focused on the discovery, development and commercialization of orally administered, proprietary small molecule drugs targeting an area of RNA biology we refer to as post-transcriptional control. Post-transcriptional control processes are the regulatory events that occur in cells during and after a messenger RNA, or mRNA, molecule is copied from DNA through the transcription process. 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. PTC plans to continue to develop these compounds both on its own and through selective collaboration arrangements with leading pharmaceutical and biotechnology companies. For more information on the company, please visit our website www.ptcbio.com.

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Forward Looking Statements:

All statements, other than those of historical fact, contained in this press release, are forward-looking statements, including statements regarding: the future expectations, plans and prospects for PTC; PTC's preliminary 2016 unaudited results, including (i) 2016 net sales of Translarna for the treatment of nmDMD and (ii) year-end 2016 cash and cash equivalents; PTC's financial guidance for 2017, including (i) net sales, (ii) non-GAAP and GAAP operating expenses, and (iii) ending cash and cash equivalents; the timing and outcome of PTC's regulatory process including, (i) PTC's ability to resolve the matters set forth in the Refuse to File letter from the FDA or otherwise advance Translarna for the treatment of nmDMD in the U.S., including its ability to supplement the NDA with additional efficacy analyses and submit its NDA for nmDMD with the FDA via the file over protest process in the first quarter of 2017 and (ii) the final determination by the European Commission with respect to renewal of the marketing authorization in the European Economic Area (EEA) for Translarna for the treatment of nmDMD and PTC's plan to conduct an additional Phase 3 randomized trial of Translarna in nmDMD; the clinical utility and potential advantages of Translarna; when top-line results of ACT CF will be available and reported; the timing, results and conduct of PTC's clinical studies of PTC596 and Translarna for the treatment of other indications, including statements regarding the timing of initiation, evaluation, enrollment and completion of the studies; any further advancement of either or both of the FIREFISH and SUNFISH studies under the joint SMA collaboration, including transition into the pivotal part of either study; the timing of a milestone payment, if any, to PTC from Roche; PTC's ability to continue to supply Translarna to patients, increase commercial penetration in countries where Translarna is currently available, and continue commercial expansion into new territories; 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 "preliminary," "guidance," "will," "plan," "expect," "target," "anticipate," "believe," "estimate," "intend," "may," "potential," "project," "possible," "potential," "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: the preliminary nature of PTC's 2016 financial results, which are subject to completion of its year-end audit; the assumptions underlying PTC's financial guidance for 2017; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the EEA, including whether the European Commission determines to approve the renewal of such authorization and whether the EMA determines in future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; the final design of the new nmDMD trial that PTC will undertake pursuant to the specific obligation associated with the marketing authorization (if renewed) and PTC's ability to enroll, fund and conduct such trial; the timing and outcome of future interactions PTC has with the FDA with respect to Translarna for the treatment of nmDMD, including PTC's ability to resolve the matters set forth in the Refuse to File letter from the FDA or otherwise advance Translarna for the treatment of nmDMD in the United States (whether pursuant to the file over protest process or otherwise), including whether PTC is required to perform additional clinical and non-clinical trials at significant cost and whether such trials, if successful, may enable FDA review of a NDA; the outcome of ongoing or future clinical trials or studies in Translarna, in particular ACT CF; events during, or as a result of, the SUNFISH or FIREFISH studies that could delay or prevent further advancement of the SMA program, including future actions or activities under the SMA joint development program; the eligible patient base and commercial potential of Translarna and PTC's other product candidates; the EMA's determinations with respect to PTC's variation submission which seeks to add Translarna for the treatment of nmCF to PTC's marketing authorization in the EEA; the scope of regulatory

approvals or authorizations for Translarna (if any), including labeling and other matters that could affect the availability or commercial potential of Translarna; PTC's ability to commercialize and commercially manufacture Translarna in general and specifically as a treatment for nmDMD, including its ability to establish and maintain arrangements with manufacturers, suppliers, distributors and production and collaboration partners on favorable terms; the outcome of pricing and reimbursement negotiations in those territories in which PTC is authorized to sell Translarna; expectations for regulatory approvals, including PTC's ability to make regulatory submissions in a timely manner (or at all), the period during which the outcome of regulatory reviews will become available, adverse decisions by regulatory authorities (or other delay or deceleration of the regulatory process), and PTC's ability to meet existing or future regulatory standards with respect to Translarna; PTC's scientific approach and general development progress; 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 Translarna will receive full regulatory approval in any territory or maintain its current marketing authorization in the EEA, or prove to be commercially successful in general, or specifically with respect to the treatment of nmDMD.

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.

Exhibit 99.2

PTC Therapeutics: A Precision Medicine Platform

35th Annual JP Morgan Healthcare Conference

PTC THERAPEUTICS

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 the information appearing under the headings "2016 Preliminary Results" and "2017 Guidance" as well as statements regarding: the future expectations, plans and prospects for PTC; the timing and outcome of PTC's regulatory process in the U.S., including as related to a planned file over protest of the new drug application for Translarna™ for the treatment of nmDMD, and in the European Economic Area (EEA), including as related to the European Commission's determination as to renewal of the marketing authorization for Translarna for the treatment of nmDMD and PTC's plan to conduct an additional Phase 3 randomized trial of Translarna in nmDMD; the clinical utility and potential advantages of Translarna; when top-line results of ACT CF will be available and reported and any statements related to the potential results of such trial; PTC's estimates regarding the potential market opportunity for Translarna, including the size of eligible patient populations and PTC's ability to identify such patients; PTC's ability to maintain the current label under the marketing authorization in the EEA; the timing of, and PTC's ability to, expand the approved product label of Translarna for the treatment of nmDMD in the EEA, whether pursuant to its ongoing Phase 2 study of Translarna for nmDMD in pediatric patients, or otherwise; the timing of, and PTC's ability to, obtain additional marketing authorizations for Translarna in other territories, including the U.S., or for additional indications, including nmCF; the timing, results and conduct of PTC's clinical studies of PTC596 and Translarna for the treatment of other indications; further advancement of the FIREFISH and SUNFISH studies under the joint SMA collaboration, including transition into the pivotal part of either study; the timing of a milestone payment, if any, to PTC from Roche; PTC's ability to increase commercial presence in countries where Translarna is currently available and commercially expand into new territories; 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 "estimate," "outlook," "will," "plan," "expect," "anticipate," "believe," "estimate," "intend," "may," "potential," "possible," "potential," "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: the completion of PTC's year-end audit; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the EEA, including whether the European Commission determines to approve the renewal of such authorization and whether the EMA determines in future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; the final design of the new nmDMD trial that PTC will undertake pursuant to the specific obligation associated with the marketing authorization (if renewed) and PTC's ability to enroll, fund and conduct such trial; the timing and outcome of future interactions PTC has with the FDA with respect to Translarna for the treatment of nmDMD, including PTC's ability to resolve the matters set forth in the Refuse to File letter from the FDA or otherwise advance Translarna for the treatment of nmDMD in the United States (whether pursuant to the file over protest process or otherwise), including whether PTC is required to perform additional clinical and non-clinical trials at significant cost; the outcome of ongoing or future clinical trials or studies in Translarna, in particular ACT CF and the Translarna nmDMD pediatric study; events during, or as a result of, the SUNFISH or FIREFISH studies that could delay or prevent further advancement of the SMA program, including future actions or activities under the SMA joint development program; the eligible patient base and commercial potential of Translarna and PTC's other product candidates; the scope of regulatory approvals or authorizations for Translarna (if any), including labeling and other matters that could affect the availability or commercial potential of Translarna; 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; PTC's scientific approach and general development progress; 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.

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PTC THERAPEUTICS

Our mission

To leverage our knowledge of **RNA biology** to bring **novel therapeutics** to patients affected by **rare and neglected disorders**

Sustainable, growing DMD business enables innovative research & development engine

- Translarna™: Strong commercial growth for DMD ex-US
- Development and regulatory milestones for Translarna
  - ✓ CHMP recommended renewal of approval of DMD*
  - ✓ File NDA over protest for DMD with the U.S. FDA in Q1 2017
  - ✓ ACT CF Phase 3 clinical trial results in Q1 2017
  - ✓ Proof-of-concept trials advance: MPS I, Aniridia & Dravet / CDKL5
- Expanding pipeline
  - ✓ SMA: advancing into two pivotal studies
  - ✓ BMI1, Huntington's and FD programs
- Strong financial position with healthy balance sheet

*Annual renewal subject to approval by the European Commission

PTC THERAPEUTICS

Translarna™

Precision medicine platform: Realizing Translarna's full value through multiple indications

PTC THERAPEUTICS

Translarna™ : Sustainable, growing DMD business supporting long term success

- Preliminary 2016 Translarna unaudited net sales of ~$81M*
- Established footprint in 47 countries worldwide
- EMA renewal establishes sustainability of ex-US business
- Experienced commercial team in orphan disease

25+ **Countries worldwide with Translarna commercial therapy***

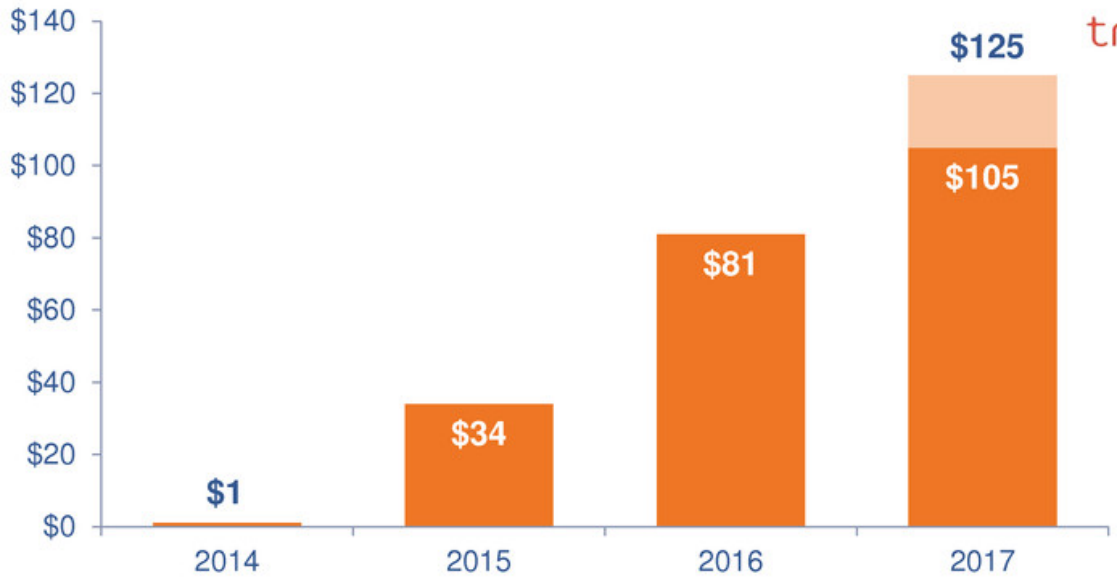
*Commercial sales through commercial or early access programs

PTC THERAPEUTICS

Strong demand drives substantial year-over-year net sales growth

Translarna™ ex-US DMD Net Sales

($ in millions)

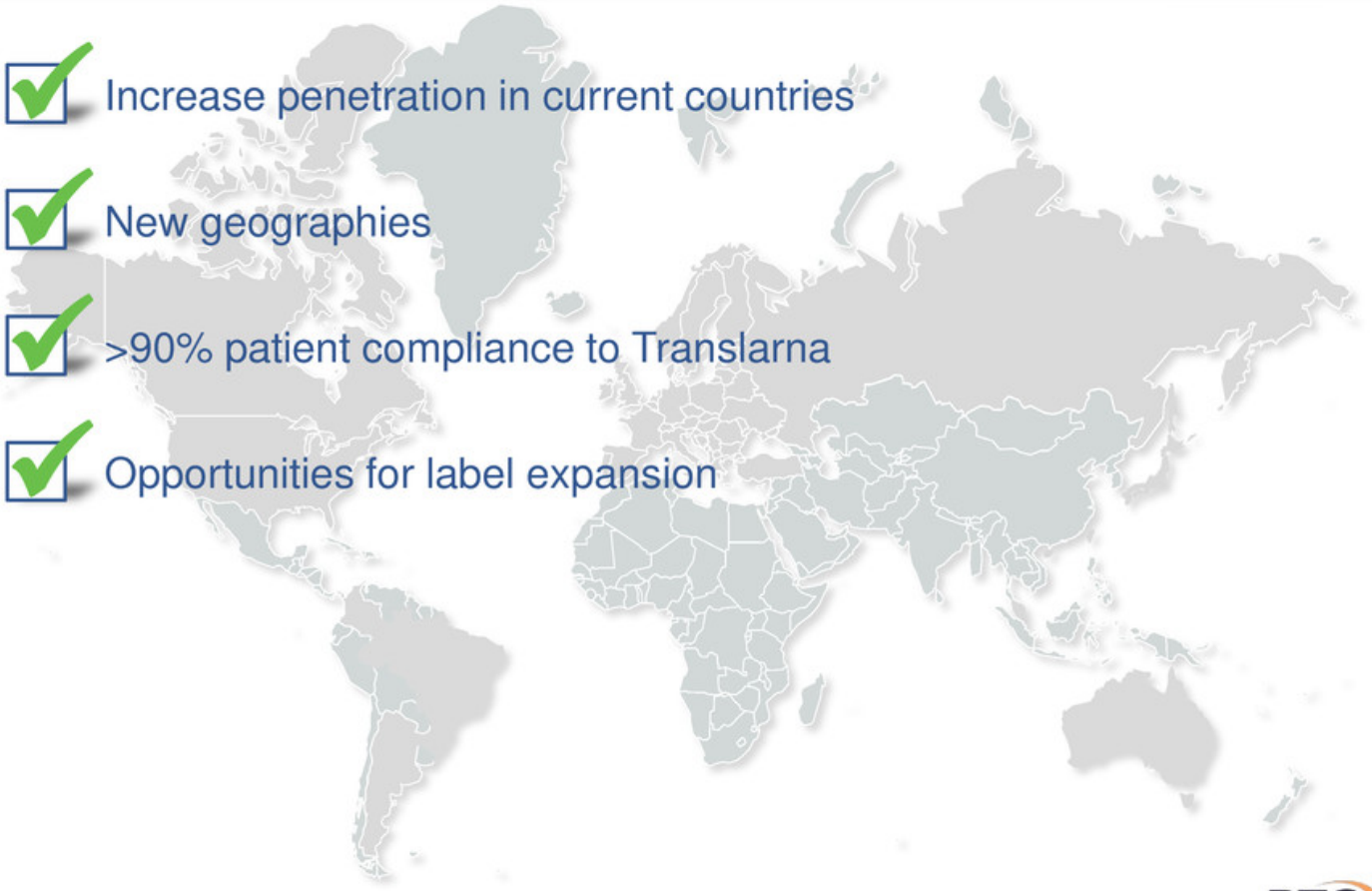


translarna™ ataluren

2017 Translarna net sales guidance of $105 - $125M

PTC THERAPEUTICS

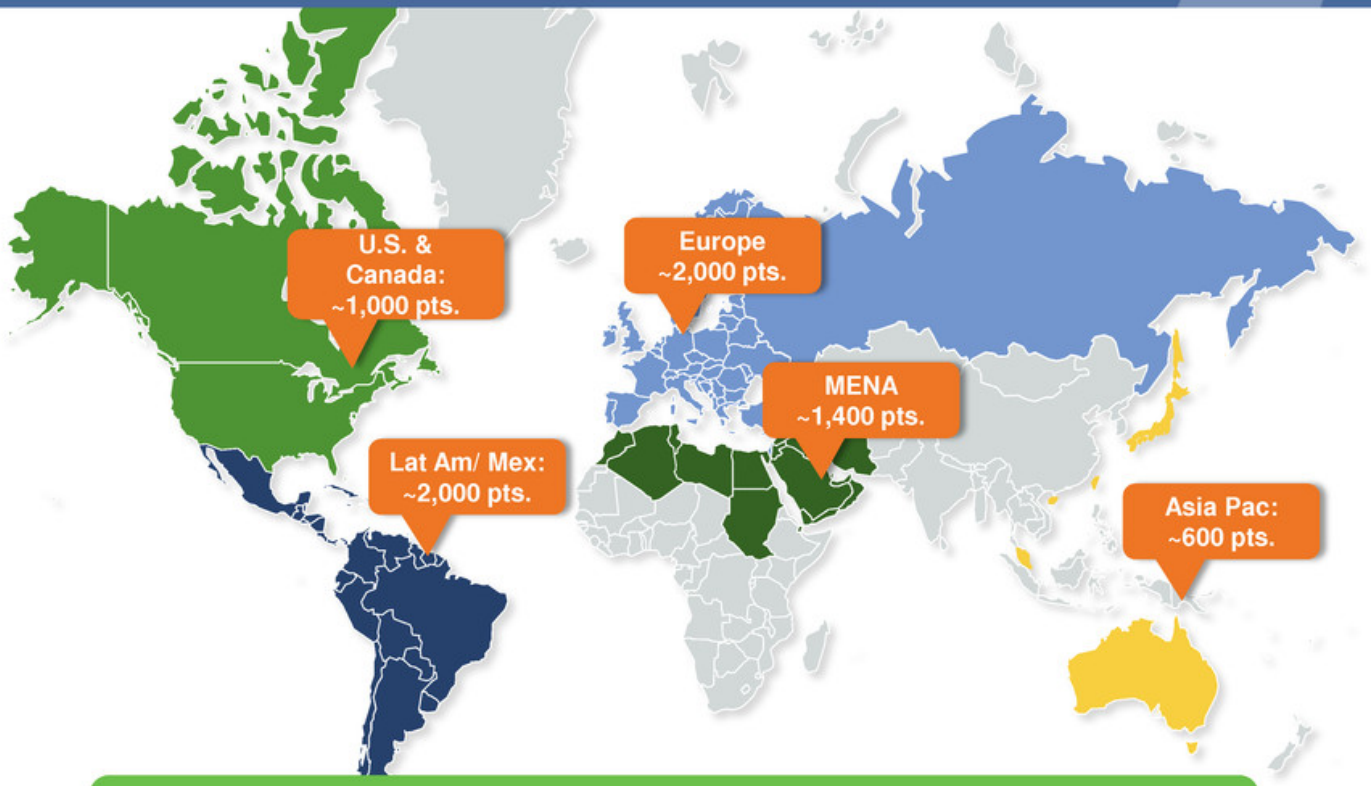
Key Drivers of Continued Market Growth



- Increase penetration in current countries
- New geographies
- >90% patient compliance to Translarna
- Opportunities for label expansion

PTC THERAPEUTICS

>7,000 addressable nmDMD patients worldwide

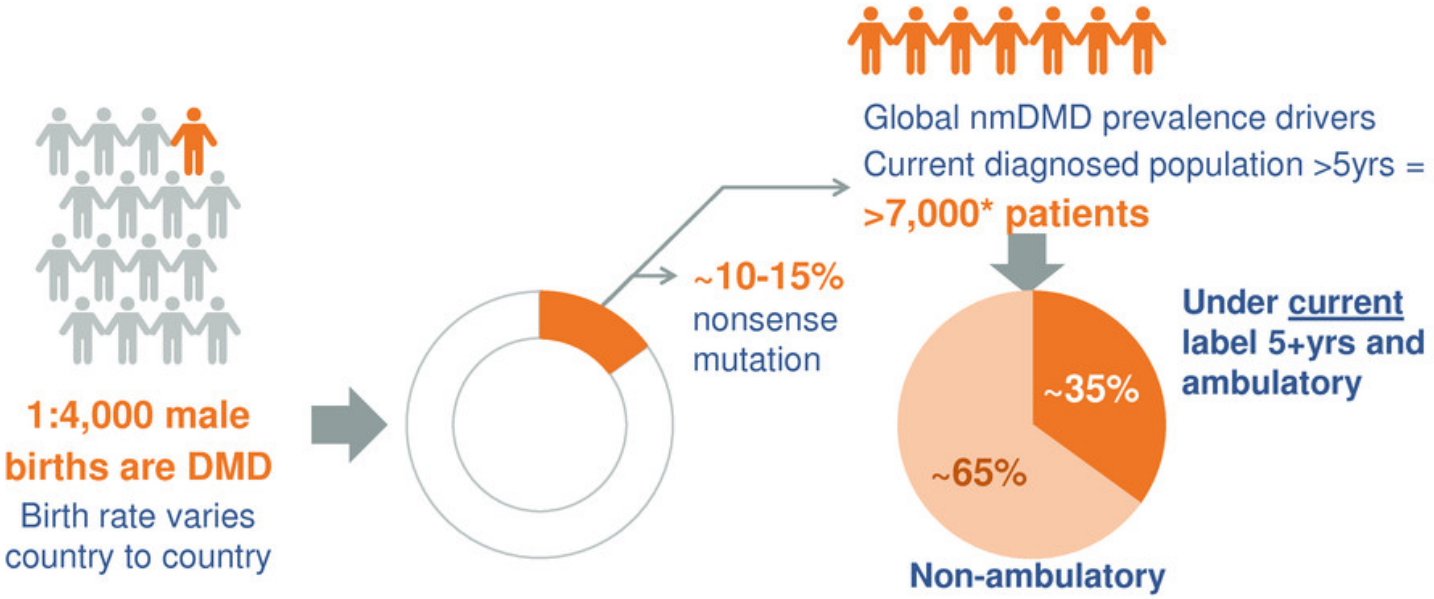


~85% of patients outside the U.S.

Internal estimates of potential patient population based on data derived from a variety of sources and are subject to change

PTC THERAPEUTICS

Developing insight on patient prevalence

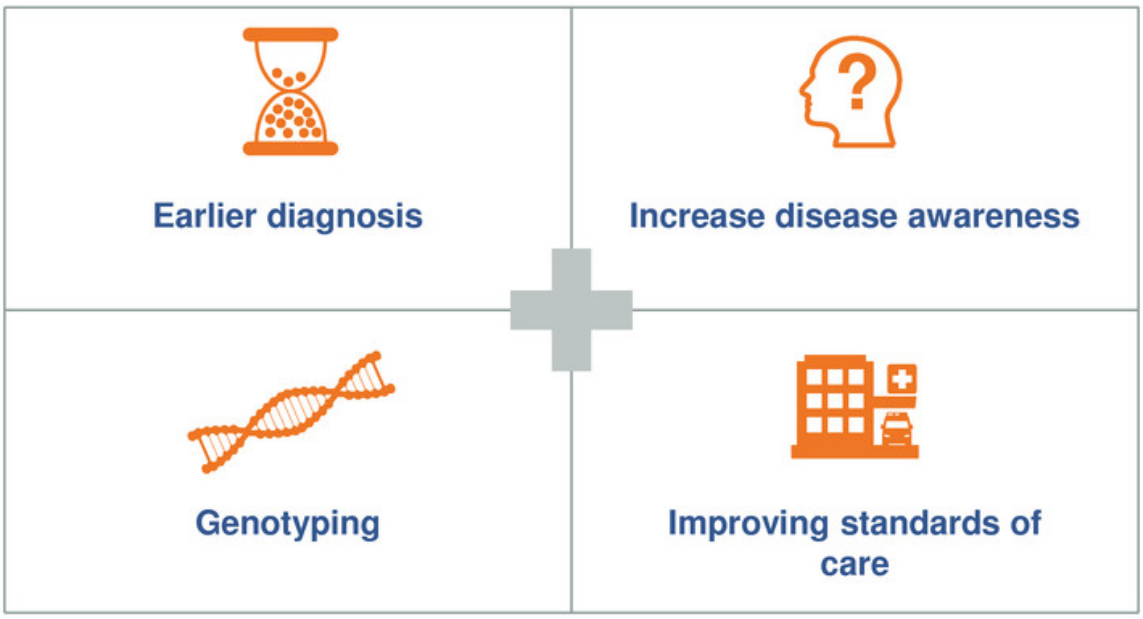


Typically patients not diagnosed <5 years of age

*Internal estimates of potential patient population based on data derived from a variety of sources and are subject to change

PTC THERAPEUTICS

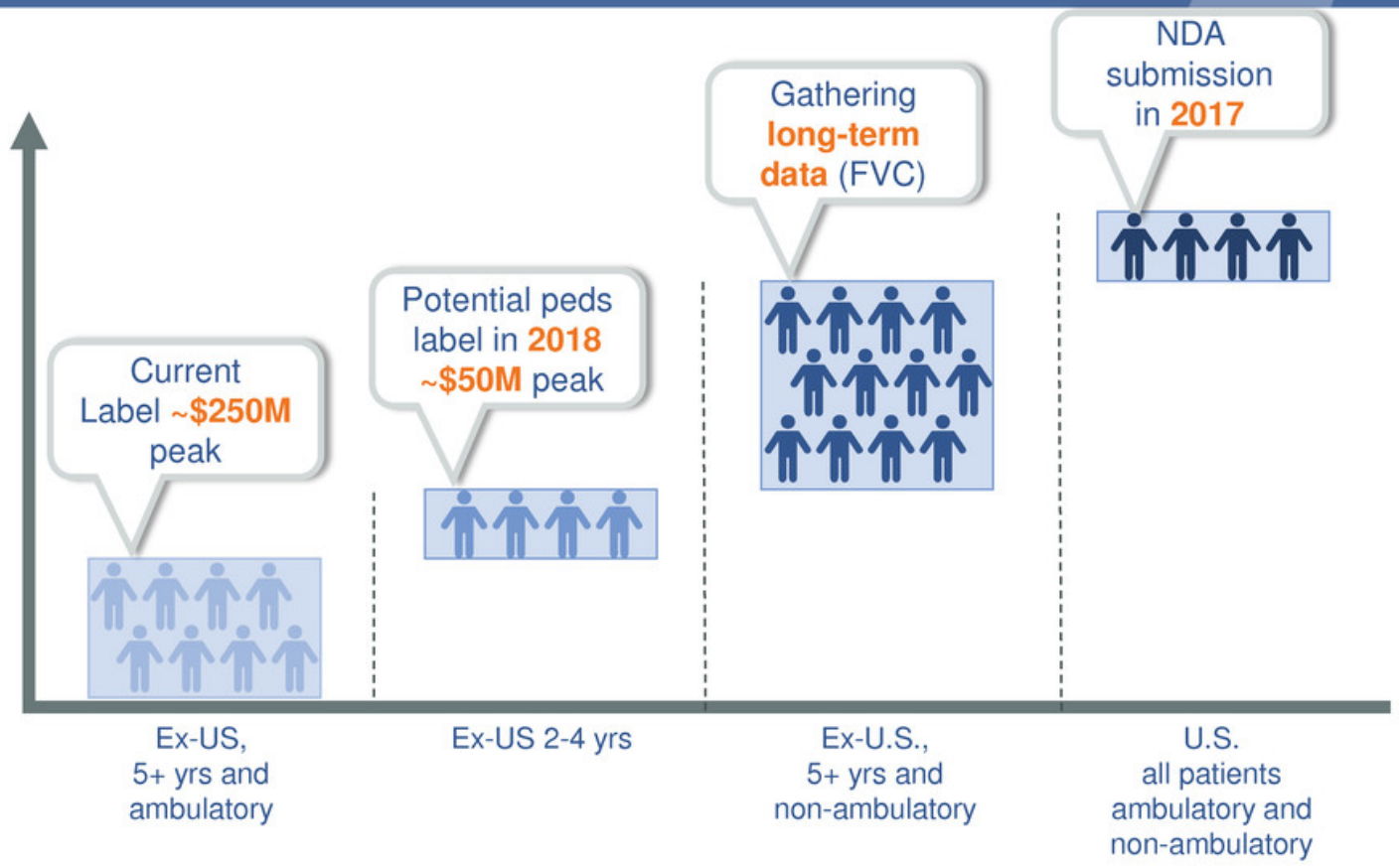
Current efforts to grow addressable DMD population



Identifying 2-4 year olds increases potential addressable population by ~20%

PTC THERAPEUTICS

Potential market opportunity for Translarna™ in DMD



Translarna™ regulatory update: CHMP recommends renewal of approval, NDA to be reviewed by FDA

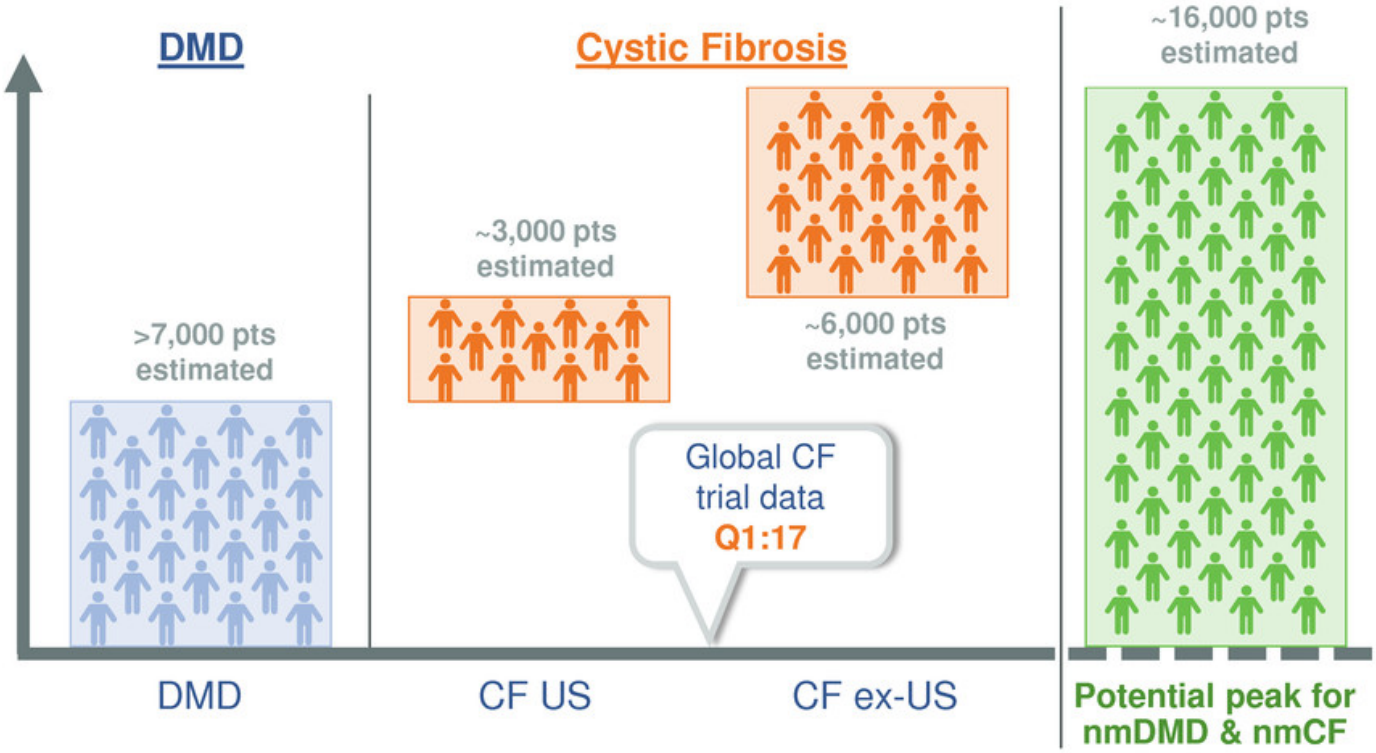
EMA

- Renewal of Translarna DMD approval recommended by CHMP
  - Commitment to conduct post marketing DMD trial

FDA

- Multiple interactions with FDA officials and advisors over the last several months
- Feedback indicated best path forward for full and fair review of the current Translarna NDA is to file over protest rather than continue appeal of RTF
- NDA to be supplemented with additional efficacy data utilized by CHMP in renewal recommendation

Potential Market opportunity for Translarna™ in nmCF: >9,000 patients worldwide

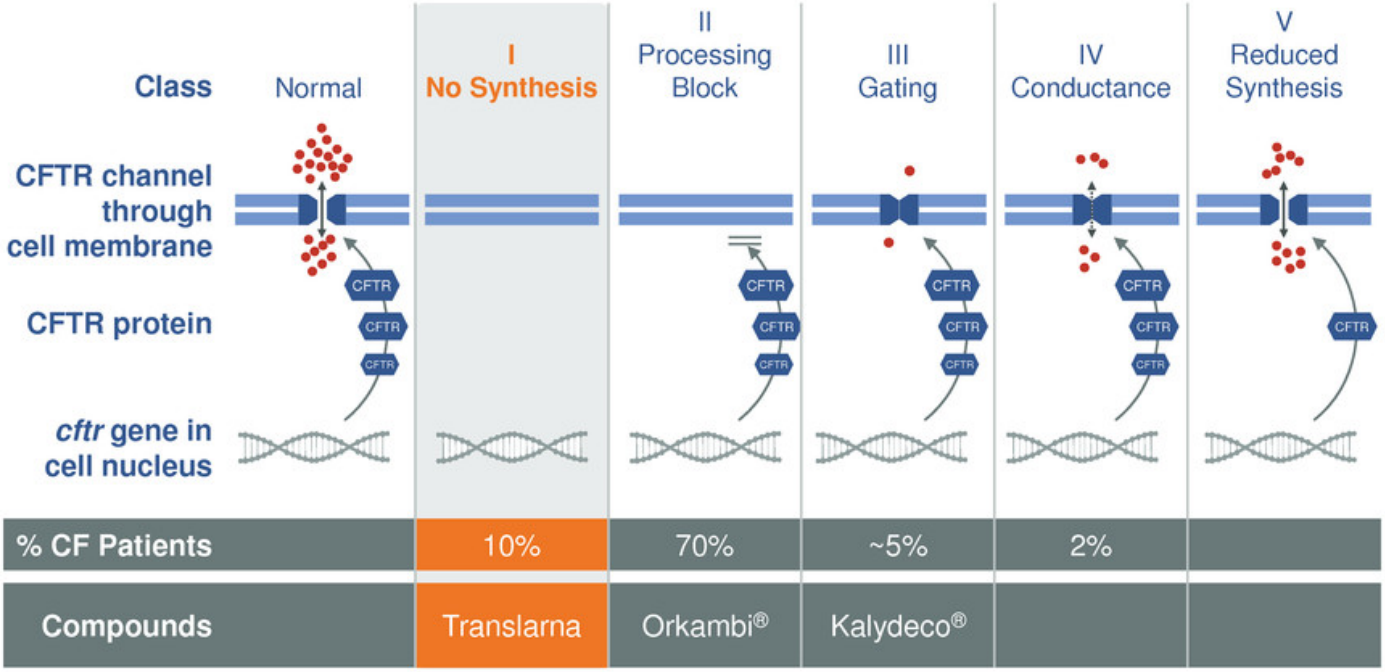


nmCF represents a significant commercial opportunity

Internal estimates of potential patient population based on data derived from a variety of sources and are subject to change

PTC THERAPEUTICS

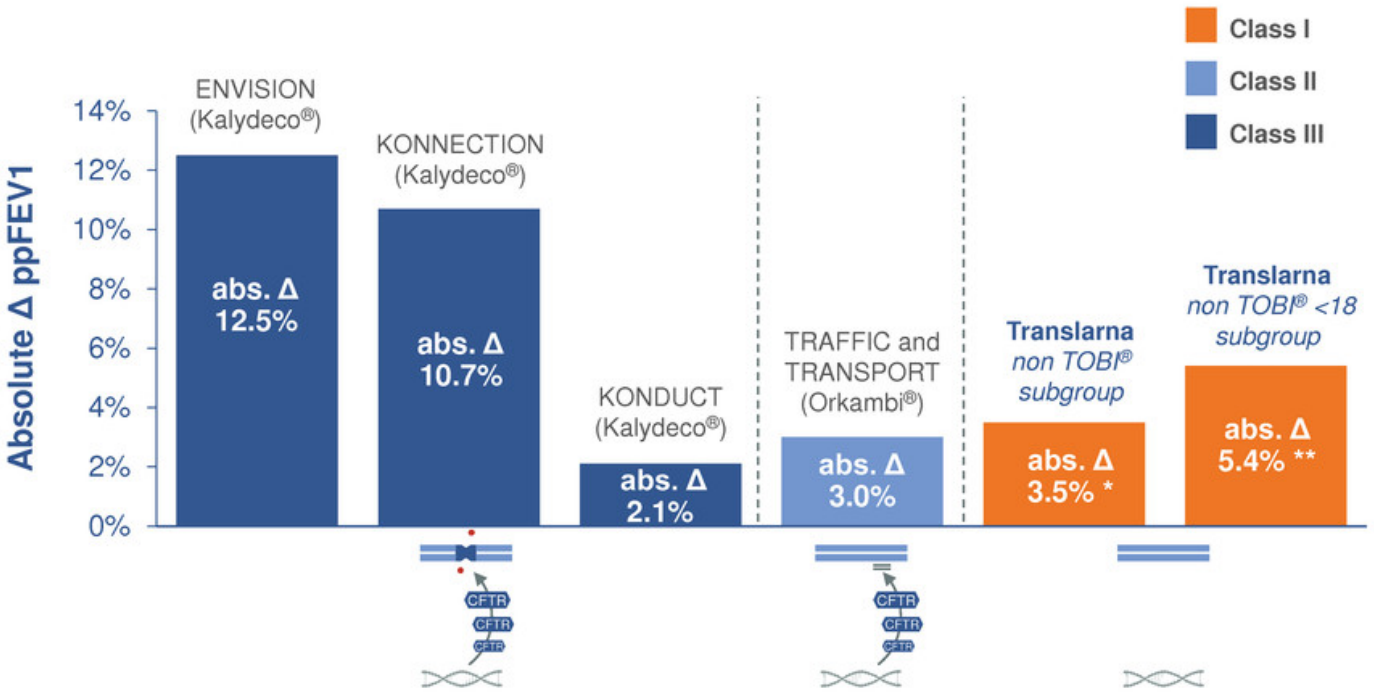
Cystic fibrosis is caused by a malfunction of the CFTR protein due to different mutations



Translarna™ is only compound in development targeting Class I CF

PTC THERAPEUTICS

Translarna™ CF data is comparable to other approved CF correctors***

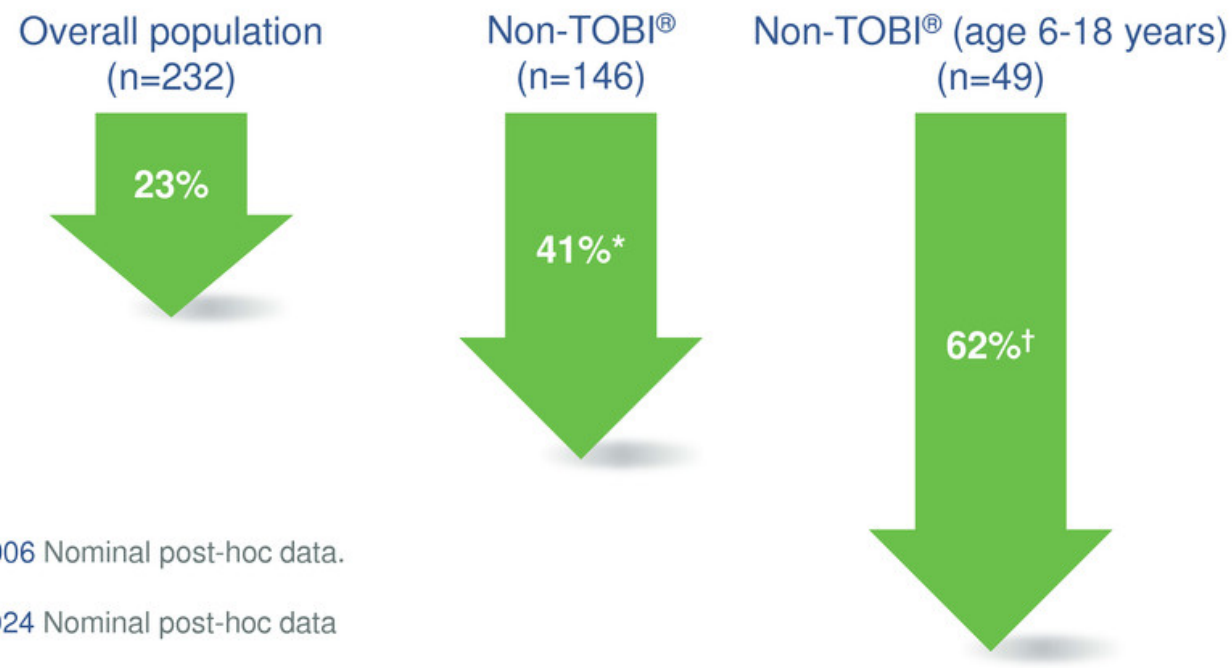


Slowing disease progression provides clinically meaningful benefits to CF patients over the long-term

* Post hoc analysis; relative Δ 5.7% ** Post hoc analysis; relative Δ 8.4% *** Tested in separate trials

PTC THERAPEUTICS

Translarna™ reduced exacerbations



*p=.006 Nominal post-hoc data.

†p=.024 Nominal post-hoc data

De Boeck K, et al. 39th European Cystic Fibrosis Conference, Basel, Switzerland, June 8-11, 2016. Abstract WS13.1.

ACT CF (Ataluren Confirmatory Trial) study: Topline data expected late Q1 2017

Length of Trial	Translarna™ (n)	Placebo (n)
48 weeks	140	140

Primary outcome measure:

- % predicted FEV1

Secondary outcome measure:

- Pulmonary exacerbation rate
- CFQ-R
- BMI

Eligibility Criteria	Stratification
• Nonsense mutation CF • ≥6 years • FEV1 ≥40% and ≤90% predicted • No chronic inhaled TOBI	• Age: <18 vs ≥18 years • Inhaled antibiotic use (yes vs no) • Baseline FEV1: ≥65% vs <65% of predicted

Trial powered with 90% confidence for 3% absolute Δ FEV1

PTC THERAPEUTICS

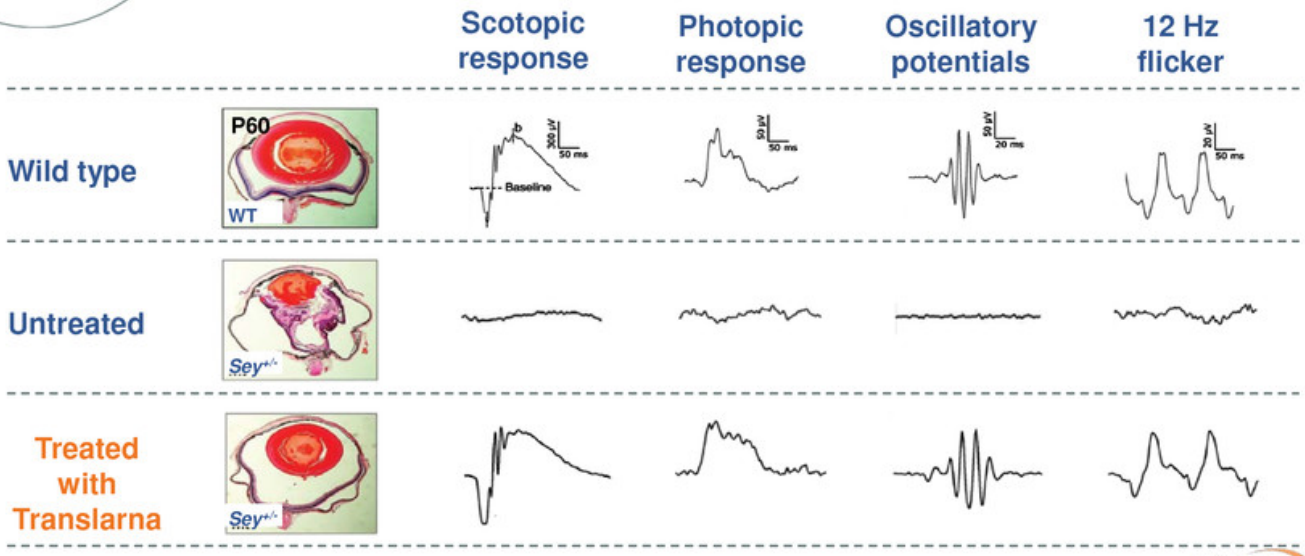
Translarna™: Realizing a new paradigm for the treatment of rare diseases, progressing proof of concept studies

Ion channel disorders Publications: 1	**Genetically defined epilepsy**	**Metabolic disorders** Publications: 10	Muscle disorders Publications: 6
Eye disorders Publications: 3	Skin disorders Publications: 2	Neurological disorders Publications: 8	Pulmonary disorders Publications: 10

~ 40 publications in many disease models

Translarna™ restores morphology and sight in Aniridia mouse model

- **20-40%** nonsense mutation
  - 12 month placebo controlled trial
  - Primary endpoint PAX6 levels, eye form and function
  - Enrolling well, targeting up to 40 patients



Gregory-Evans 2014 JCI

PTC THERAPEUTICS

Progressing proof of concept studies: MPS I and Dravet / CDKL5

60-80%
MPS I
nonsense mutation

- **MPS I:** Metabolic enzyme disorder
  - 3 month open label trial currently enrolling naïve patients
  - Primary endpoint change in urinary & CNS GAG levels
  - Challenges in enrolling, targeting 8 patients

50% Dravet
10% CDKL5
nonsense mutation

- **Dravet / CDKL5:** Genetic epilepsy
  - 32 weeks placebo controlled trials
  - Primary endpoint number of monthly seizures
  - Targeting up to 16 patients (8 for Dravet and 8 for CDKL5)

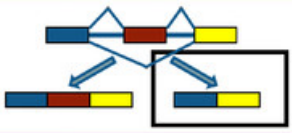
PTC THERAPEUTICS

Small molecule splicing technology platform

Multiple on-going programs

The spinal muscular atrophy program validates PTC's small molecule splicing platform

Splicing



Target splicing event to restore or reduce protein

SMA - SMN2
FD – IKBKAP
HD – HTT

Tumor Resistance – MCL1

MOTOR NEURON DISEASE

SMN2 splicing modifiers improve motor function and longevity in mice with spinal muscular atrophy

"The combination of structural and morphing capabilities that enhances or alters a particular material characteristic is likely where the most innovative applications of origami engineering lie."

MOLECULAR BIOLOGY

A splicing magic bullet

Drugs that modulate RNA splicing are potential

NEUROMUSCULAR DISORDERS

Beefing up the right splice variant to treat spinal muscular atrophy

Spinal muscular atrophy (SMA), the leading genetic cause of infant mortality, is characterized by progressive degeneration of motor neurons in the anterior horn of the spinal cord and atrophy of skeletal muscles. SMA results from mutations in survival of motor neuron 1 (*SMN1*). Naryshkin *et al.* have now identified orally available small-molecule compounds that alter the splicing of the nearly identical *SMN2*, which can improve motor function and extend lifespan in mouse models of SMA.

compounds that alter *SMN2* splicing. SMN-C3, protein levels were partially

The NEW ENGLAND JOURNAL of MEDICINE

CLINICAL IMPLICATIONS OF BASIC RESEARCH

Elizabeth G. Phimister, Ph.D., *Editor*

Romancing the Spliceosome to Fight Spinal Muscular Atrophy

Kathryn J. Swoboda, M.D.

Every scientist, clinician, and patient seeks that holy grail that will cure disease. Nowhere is this need more acute than in the field of neurodegeneration. In neurogenetic diseases, in the large majority of cases, aberrant code is embedded in the DNA in every cell, and the blood–brain bar-

cies from a single pre-mRNA template. Alternative splicing results in the exclusion of one or more exons from the mature mRNA and facilitates the expression of different protein isoforms (all derived from the same gene) at different stages of development, in different tissues, and in

Pivotal portion of both Sunfish & Firefish trials expected to begin in 2017

SUNFISH

- Clinical study in SMA type 2/3 patients initiated in November
  - Enrolling 36 patients for dose escalation phase, placebo controlled 2:1
  - Pivotal phase will be 150 patients, placebo controlled 1:1, endpoint of total motor function measure (MFM-32) at 12 months

FIREFISH

- Clinical study in SMA type 1 patients <7 months old
  - Enrolling 8 patients for dose escalation phase
  - Pivotal phase will enroll 40 babies, open label endpoint of sitting as measured by Bayley infant scale

Roche

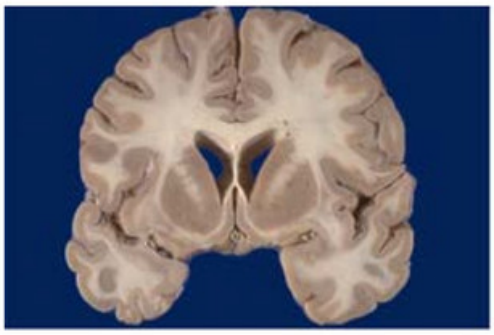
SMA FOUNDATION

Two pivotal studies expected to begin in 2017

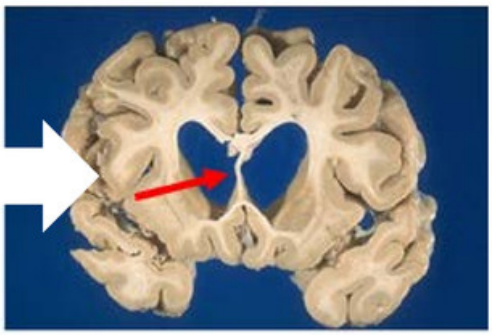
PTC THERAPEUTICS

Huntington's disorder is a progressive, inherited neurodegenerative disorder

Healthy



HD

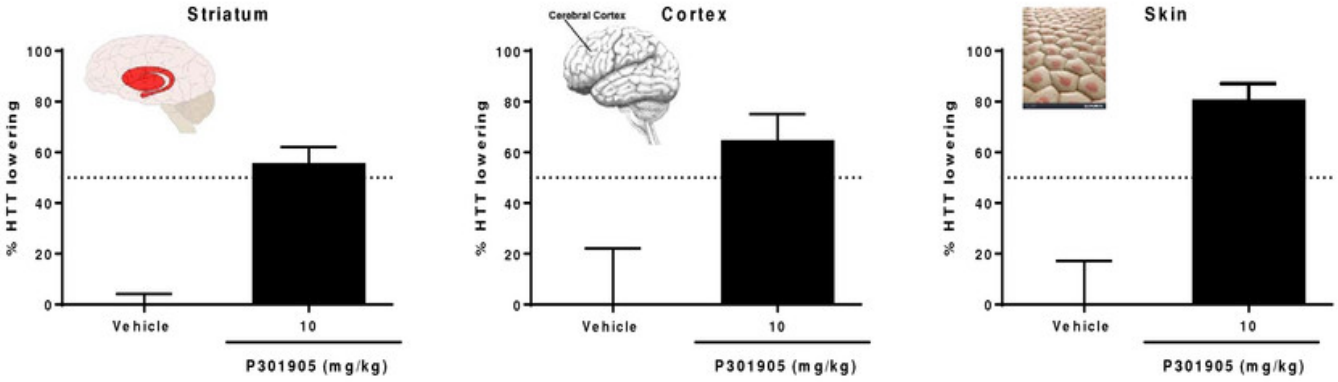


- HD is caused by expression of mutant Huntingtin (HTT) protein
- Causes selective and devastating neuronal loss
  - Predominantly in the striatum and cerebral cortex
- Adult onset at ~30-50 years
- Unmet medical need
  - Patients: 30,000 US, 100,000 WW

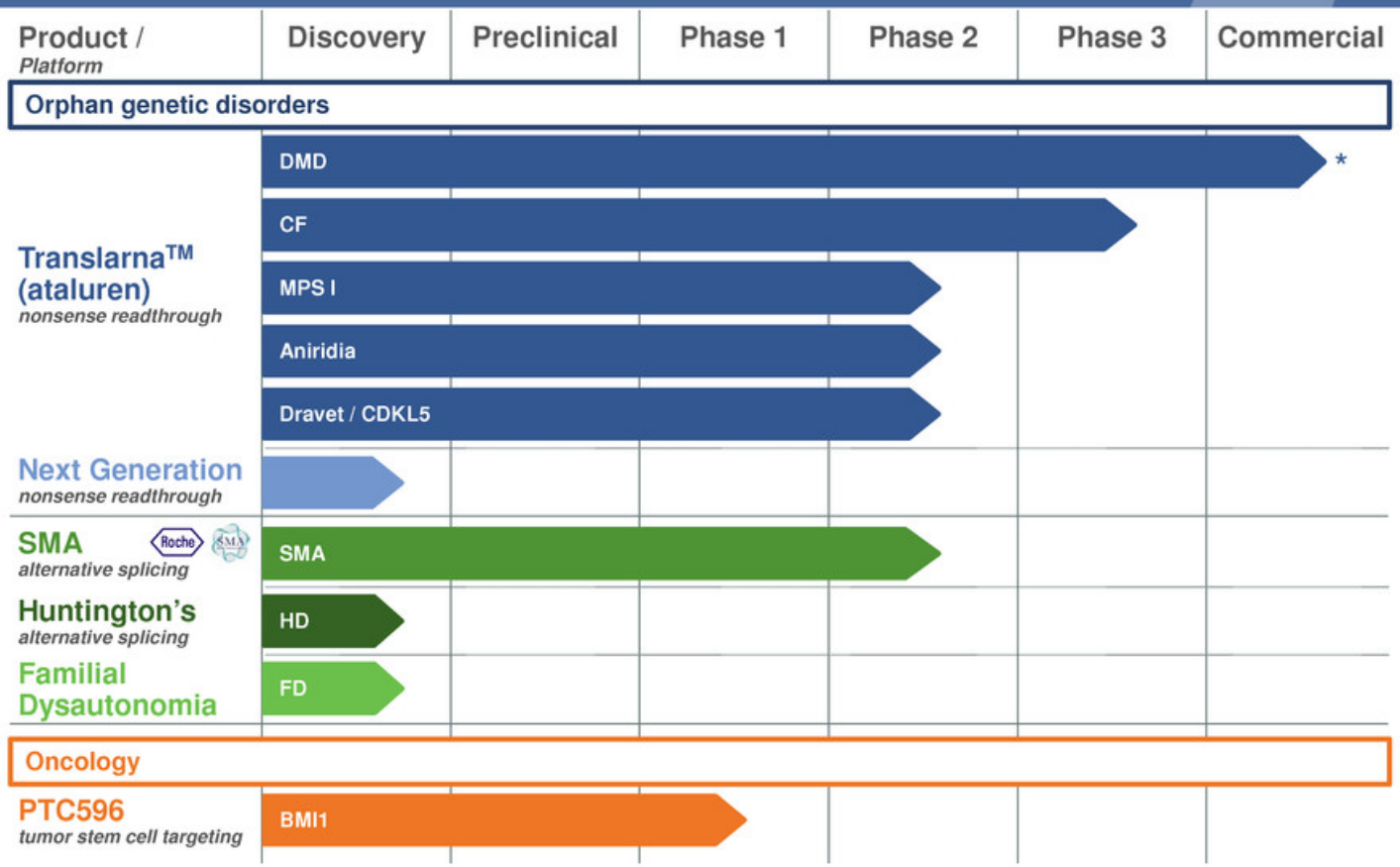
PTC THERAPEUTICS

HD program in Lead Optimization – showcases PTC's alternative splicing platform

- Multiple orally bioavailable, brain penetrant compounds being optimized
  - Lowers HTT by altering splicing
- Opportunity to provide first-in-class disease modifying, HTT lowering small molecule therapy
  - Potential to circumvent delivery and distribution challenges seen with the current modalities



Expanding pipeline through in-house innovation



* Marketing authorization (MA) recommended by CHMP in the European Economic Area, which is subject to approval by the European Commission; MA requires annual renewal following reassessment by the European Medicines Agency (EMA)

PTC THERAPEUTICS

Strong financial position with sustainable, growing DMD business

2016 Preliminary Results

- Preliminary 2016 Translarna™ unaudited net sales of ~$81 million
- Commercial business cash flow positive 2yrs post launch
- 12/31/16 year-end cash position of ~$230 million

2017 Guidance

- 2017 Translarna net sales guidance of $105 – $125 million*
- $20 million SMA milestone payment expected mid-2017
- 2017 non-GAAP operating expenses of $190 - $200 million (excludes ~$35M in non-cash stock based compensation)
- 12/31/17 year-end cash position anticipated to be ~$160 million

- ex-US DMD sales
- Based on current exchange rates.

PTC THERAPEUTICS

PTC Near-term milestones