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 7, 2019**

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)

Registrant'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))

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 o

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. o

Item 2.02. Results of Operations and Financial Condition.

On January 7, 2019, PTC Therapeutics, Inc. (the "Company") issued a press release (the "press release") announcing certain preliminary (unaudited) financial information for its fiscal year ending December 31, 2018, including that the Company expects to report (i) Translarna™ (ataluren) net product revenue for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) of approximately $171 million; (ii) EMFLAZA™ (deflazacort) net product revenue for the treatment of Duchenne muscular dystrophy (DMD) of approximately $91 million; and (iii) ending cash and cash equivalents of approximately $227 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, 2019 in the press release, including that the Company anticipates (i) full-year net product revenues to be between $285 and $305 million and (ii) GAAP R&D and SG&A expense for the full year 2019 to be between $395 and $405 million with non-GAAP R&D and SG&A expense for the full year 2019 to be between $360 and $370 million, excluding estimated non-cash, stock-based compensation expense of approximately $35 million.

The Company announced that on Tuesday, January 8th at 5:00 pm PT at the 37th Annual J.P. Morgan Healthcare Conference (the "Conference"), the Company will present its 2019 strategic priorities, preliminary 2018 financial results, and 2019 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 R&D and SGA expenses (which excludes non-cash stock-based compensation expense). Management uses this measure when assessing and identifying operational trends 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 historical and projected operating performance of 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 fiscal year 2018 and financial guidance for fiscal year 2019. 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 2018 financial information, which is subject to completion of the Company's year-end audit; the assumptions underlying the Company's financial guidance for 2019; and the factors discussed in the "Risk Factors" section of the Company's Annual Report on Form 10-K for the year ended December 31, 2017, Quarterly Reports on Form 10-Q for the periods ended March 31, 2018, June 30, 2018 and September 30, 2018 and Exhibit 99.2 to PTC's Current Report on Form 8-K filed on August 24, 2018 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 and the exhibits hereto 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. All website addresses given in this Current Report on Form 8-K or incorporated herein by reference are for information only and are not intended to be an active link or to incorporate any website information into this Report.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press Release, dated January 7, 2019 issued by PTC Therapeutics, Inc.
99.2	Corporate Presentation - 37th 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 7, 2019

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

PTC Therapeutics Provides Corporate Update and Outlines 5-year Strategic Plan at 2019 J.P. Morgan Healthcare Conference

- Multiple products to be launched by 2023 potentially generating ~$1.5 billion revenue annually- - Plan to submit BLA in 2H 2019 for AADC gene therapy program -
- Risdiplam regulatory filing targeted for 2H 2019 -
- TEGSEDI™ filed in Brazil for expected approval in 2019 -
- Duchenne franchise continuing to expand -

SOUTH PLAINFIELD, N.J., – Jan. 7, 2019 – PTC Therapeutics, Inc. (NASDAQ: PTCT) today provided a corporate update, which will be detailed as part of the company's presentation at the 37th Annual J.P. Morgan Healthcare Conference on Tuesday, January 8th at 5:00 p.m. PT. Stuart W. Peltz, Ph.D., PTC's Chief Executive Officer, will highlight the company's growth and diversification focused on bringing clinically differentiated, life-changing treatments to patients affected by rare disorders. Additionally, an overview of the company's strategic vision will be provided as well as preliminary 2018 financial results and 2019 financial guidance. The presentation will be webcast live on the Events and Presentations page under the investors' section of PTC Therapeutics' website at www.ptcbio.com.

Corporate Highlights

Advancing gene therapy portfolio

- PTC plans to submit a BLA with the FDA followed by an MAA in Europe for the AADC deficiency gene therapy program in 2H 2019 with expected launch in 2020. Identification of patients with AADC deficiency has been a priority for the Company, with approximately 100 patients identified to date in the U.S. and Europe. PTC expects to screen about 100,000 patients who are at risk for AADC deficiency before the regulatory approval to maximize patient benefit at time of launch.
- Friedreich's ataxia program is advancing with an expected IND filing and entry into the clinic in 2019.
- PTC is enhancing its internal research and in-house manufacturing capabilities with fully dedicated resources to maximize current and future programs.

Risdiplam regulatory filing progress. The SMA program is a collaboration between PTC, Roche and SMA Foundation.

- Successfully completed enrollment of pivotal portion of FIREFISH trial in Type 1 SMA babies in 2018.
- Regulatory submission targeted for the second half of 2019 based on recent feedback from the FDA & national health authorities in Europe that Part 1 of FIREFISH and SUNFISH may be sufficient to file NDA/MAA.
- The goal of the clinical development program is to support the approval of a broad label so risdiplam can potentially benefit as many SMA patients as possible.
- Short- and long-term cash milestones to PTC expected upon regulatory approval and commercial launches. PTC expects the peak annual royalties/milestones from risdiplam to exceed $200M.

Expanding commercial platform

- TEGSEDI™ application filed with ANVISA- Brazilian regulatory authority. ANVISA granted priority review. PTC expects approval in Brazil by year end 2019. TEGSEDI has the potential to generate peak revenues of approximately $150M in LATAM.
- Duchenne franchise expected to continue to grow over the next 5 years. Translarna ex-U.S. launch in patients 2 to 5 years of age now initiated. Non-ambulatory label expansion is currently under EMA regulatory review. Emflaza® continues to accrue new patients in the U.S. with expected increased market share gained via differentiation and improved market access.
- In recent interactions, the FDA invited PTC to submit a supplementary NDA (sNDA) for Emflaza for patients 2 to 5 years of age on the basis that existing data support its safety and efficacy in this population. PTC recently submitted the sNDA for potential approval in 2019. The previous written request from the FDA that a trial in patients 2 to 5 years of age be performed has been officially withdrawn and the trial will no longer be conducted. PTC now expects to launch Emflaza in this younger population before the end of 2019.

Growing pipeline and R&D capabilities

- PTC’s alternative splicing platform has generated another development candidate. PTC258 was selected as a development candidate for Familial dysautonomia (FD), a rare genetic neurological disorder that effects the sensory and autonomic nervous systems, causing life-threatening medical complications from birth. PTC258 is advancing to IND enabling studies with the goal to enter the clinic in late 2019. This program is in collaboration with MGH and NYU.
- Translarna’s dystrophin study was initiated in 4Q 2018 for potential U.S. regulatory submission in early 2020.
- PTC’s oncology portfolio continues to advance with the initiation of a study in AML with PTC299 and a DIPG study for PTC596. PTC expects these studies to move to the expanded cohort stage in 2020. PTC596 is also being developed for the treatment of patients with Leiomyosarcoma (LMS) with the first patient in that trial expected to be dosed in Q1 2019.

Preliminary Unaudited 2018 Financial Results

- PTC expects to report Translarna™ (ataluren) net product revenue for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) of approximately $171 million for full year 2018, an increase of 18% over the prior year.
- PTC expects to report EMFLAZA® (deflazacort) net product revenue for the treatment of Duchenne muscular dystrophy (DMD) of approximately $91 million for full year 2018, an increase from $28.8 million in the prior year.
- PTC expects to report year-end cash and cash equivalents of approximately $227 million.

PTC is currently in the process of finalizing its financial results for the 2018 fiscal year. The above information is based on preliminary unaudited information and management estimates for the full year 2018, subject to the completion of PTC's financial closing procedures. In addition, the above information is subject to revision as PTC completes its financial closing procedures for fiscal 2018.

2019 Guidance

- PTC anticipates full year net product revenues to be between $285 and $305 million.
- PTC anticipates GAAP R&D and SG&A expense for the full year 2019 to be between $395 and $405 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2019 to be between $360 and $370 million, excluding estimated non-cash, stock-based compensation expense of approximately $35 million.

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, the non-GAAP financial measure excludes non-cash, stock-based compensation expense. This non-GAAP financial measure is provided as a complement to financial measures reported in GAAP because management uses this non-GAAP financial measure when assessing and identifying operational trends. In management's opinion, this non-GAAP financial measure is useful to investors and other users of PTC's financial statements by providing greater transparency into the historical and projected operating performance of PTC and the company's future outlook. Non-GAAP financial measures are not an alternative for financial measures prepared in accordance with GAAP. A quantitative reconciliation of the non-GAAP financial measure to its closest equivalent GAAP financial measure is included in the table below.

PTC Therapeutics, Inc.
Reconciliation of GAAP to Non-GAAP 2019 Projected Full Year R&D and SG&A Expense
(In thousands)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A expense	$ 395,000	$ 405,000
Less: projected non-cash stock-based compensation expense	35,000	35,000
Total projected non-GAAP R&D and SG&A expense	**$ 360,000**	**$ 370,000**

SMA Milestones/Royalties supporting information

- SMA program Royalties to PTC from Roche based on net sales:

Tier of Calendar Year Worldwide Net Sales in $US million	Percent (%) of Net Sales
0 – 500	8
> 500 – 1,000	11
> 1,000 – 2,000	14
> 2,000	16

- SMA program Sales-threshold-based payments to PTC from Roche:

Event Total Calendar Year Net Sales ($US)	Payment ($US)
> $ 500,000,000	$25,000,000
> $ 750,000,000	$50,000,000
> $ 1,500,000,000	$100,000,000
> $ 2,500,000,000	$150,000,000
Total Remaining	$325,000,000

- SMA program Milestone-based payments to PTC from Roche:

Event	Payment ($US)
Filing of an NDA in the US	$15,000,000
Filing of an NDA in an EU country or with the EMA	$15,000,000
Filing of an NDA in Japan	$7,500,000
First Commercial Sale in US	$20,000,000
First Commercial Sale in the EU	$20,000,000
First Commercial Sale in Japan	$10,000,000
Total Remaining	$87,500,000

About PTC Therapeutics, Inc.
PTC is a science-led, global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to globally commercialize products is the foundation that drives investment in a robust pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need.

For More Information:
Investors:
Emily Hill
+ 1 (908) 912-9327
ehill@ptcbio.com

Media:
Jane Baj
+1 (908) 912-9167
jbaj@ptcbio.com

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, other than statements of historic fact, are forward-looking statements, including the information provided under the headings "Preliminary Unaudited 2018 Financial Results", including with respect to (i) 2018 net product revenue of Translarna for the treatment of nmDMD and EMFLAZA for the treatment of Duchenne muscular dystrophy and (ii) year-end 2018 cash and cash equivalents, and "2019 Guidance", including with respect to (i) 2019 net product revenue guidance and (ii) 2019 GAAP and non-GAAP R&D and SG&A expense guidance, and statements regarding: the future expectations, plans and prospects for PTC; expectations with respect to PTC's gene therapy platform, including any potential regulatory submissions; PTC's expectations with respect to the licensing and potential commercialization of TEGSEDI and Waylivra; expansion of commercialization of Translarna and Emflaza; advancement of PTC's joint collaboration program in SMA, including any potential regulatory submissions; PTC's strategy, future operations, future financial position, future revenues, 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: the outcome of pricing, coverage and reimbursement negotiations with third party payors for Emflaza and Translarna and any other product candidates that PTC may commercialize in the future; whether, and to what extent, third party payors impose additional requirements before approving Emflaza prescription reimbursement; PTC's ability to complete any dystrophin study necessary in order to resolve the matters set forth in the denial to the Complete Response letter it received from the FDA in connection with its new drug application for Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD), 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; expectations with respect to the potential financial impact or PTC's ability to realize the anticipated benefits of the acquisition of Agilis and its gene therapy platform, including with respect to the business of Agilis and expectations with respect to the potential achievement of development, regulatory and sales milestones and contingent payments to the former Agilis equityholders with respect thereto and PTC's ability to obtain marketing approval of PTC-AADC and other product candidates acquired from Agilis, will not be realized or will not be realized within the expected time period; expectations with respect to the potential financial impact and benefits of the collaboration and licensing agreement with Akcea Therapeutics, Inc., including with respect to the timing of regulatory approval of TEGSEDI and Waylivra in countries in LATAM and the Caribbean, the commercialization of TEGSEDI and Waylivra, and PTC's expectations with respect to contingent payments to Akcea based on net sales and the potential achievement of regulatory milestones; the enrollment, conduct, and results 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, including any potential regulatory submissions with regards to Risdiplam; 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 or the acquisition of its gene therapy pipeline, 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 eligible patient base and commercial potential of Translarna, Emflaza, PTC-AADC, TEGSEDI, Waylivra, Risdiplam or any of PTC's other product candidates; 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 Annual Report on Form 10-K for the year ended December 31, 2017, Quarterly Reports on Form 10-Q for the

periods ended March 31, 2018, June 30, 2018 and September 30, 2018 and Exhibit 99.2 to PTC's Current Report on Form 8-K filed on August 24, 2018, 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, Emflaza, PTC-AADC, TEGSEDI, Waylivra or Risdiplam.

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.


PTC 2019
JP Morgan Healthcare Conference
Stuart Peltz, CEO

Forward looking statement

All statements contained in this presentation, other than statements of historic fact, are forward-looking statements, including statements related to preliminary unaudited 2018 financial information with respect to 2018 net product revenue of Translarna for the treatment of nmDMD and EMFLAZA for the treatment of Duchenne muscular dystrophy, statements with respect to 2019 net product revenue guidance and statements regarding: the future expectations, plans and prospects for PTC; expectations with respect to PTC's gene therapy platform, including any potential regulatory submissions; PTC's expectations with respect to the licensing and potential commercialization of Tegsedi and Waylivra; expansion of commercialization of Translarna and Emflaza; advancement of PTC's joint collaboration program in SMA, including any potential regulatory submissions; PTC's strategy, future operations, future financial position, future revenues, 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: the outcome of pricing, coverage and reimbursement negotiations with third party payors for Emflaza and Translarna and any other product candidates that PTC may commercialize in the future; whether, and to what extent, third party payors impose additional requirements before approving Emflaza prescription reimbursement; PTC's ability to complete any dystrophin study necessary in order to resolve the matters set forth in the denial to the Complete Response letter it received from the FDA in connection with its new drug application for Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD), 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; expectations with respect to the potential financial impact or PTC's ability to realize the anticipated benefits of the acquisition of Agilis and its gene therapy platform, including with respect to the business of Agilis and expectations with respect to the potential achievement of development, regulatory and sales milestones and contingent payments to the former Agilis equityholders with respect thereto and PTC's ability to obtain marketing approval of PTC-AADC and other product candidates acquired from Agilis, will not be realized or will not be realized within the expected time period; expectations with respect to the potential financial impact and benefits of the collaboration and licensing agreement with Akcea Therapeutics, Inc., including with respect to the timing of regulatory approval of Tegsedi and Waylivra in countries in LATAM and the Caribbean, the commercialization of Tegsedi and Waylivra, and PTC's expectations with respect to contingent payments to Akcea based on net sales and the potential achievement of regulatory milestones; the enrollment, conduct, and results 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, including any potential regulatory submissions with regards to Risdiplam; 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 or the acquisition of its gene therapy pipeline, 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 eligible patient base and commercial potential of Translarna, Emflaza, PTC-AADC, Tegsedi, Waylivra, Risdiplam or any of PTC's other product candidates; 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 Annual Report on Form 10-K for the year ended December 31, 2017, Quarterly Reports on Form 10-Q for the periods ended March 31, 2018, June 30, 2018 and September 30, 2018 and Exhibit 99.2 to PTC's Current Report on Form 8-K filed on August 24, 2018, 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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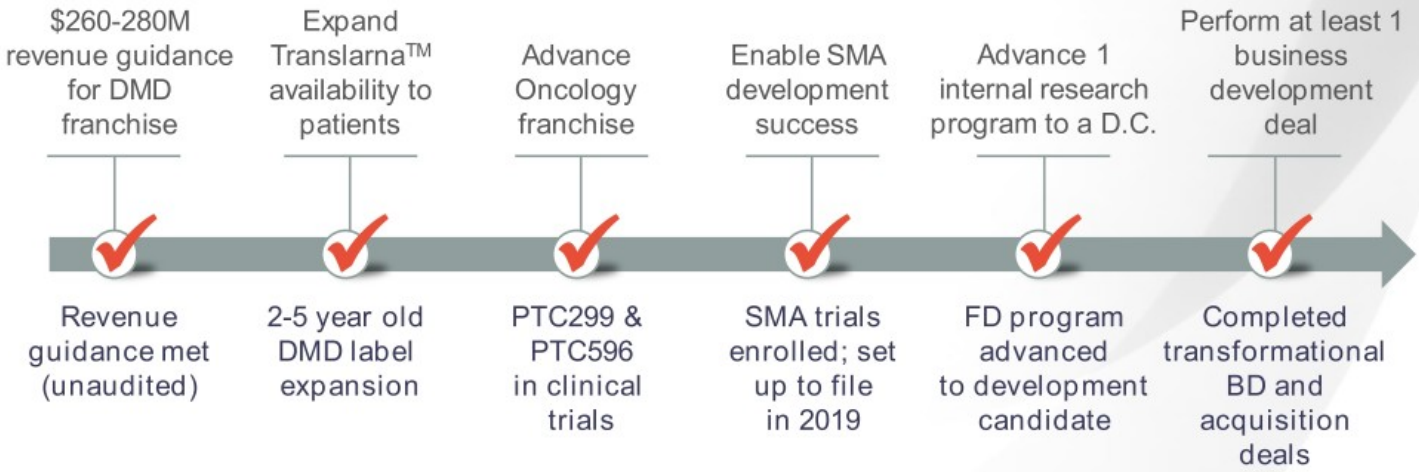
The forward-looking statements contained herein represent PTC's views only as of the date of this presentation and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this presentation except as required by law.

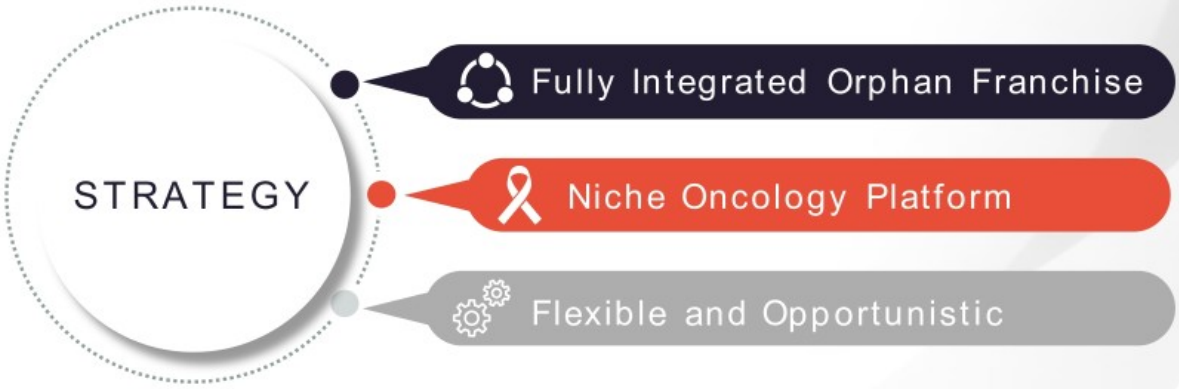
2018: a transformational year



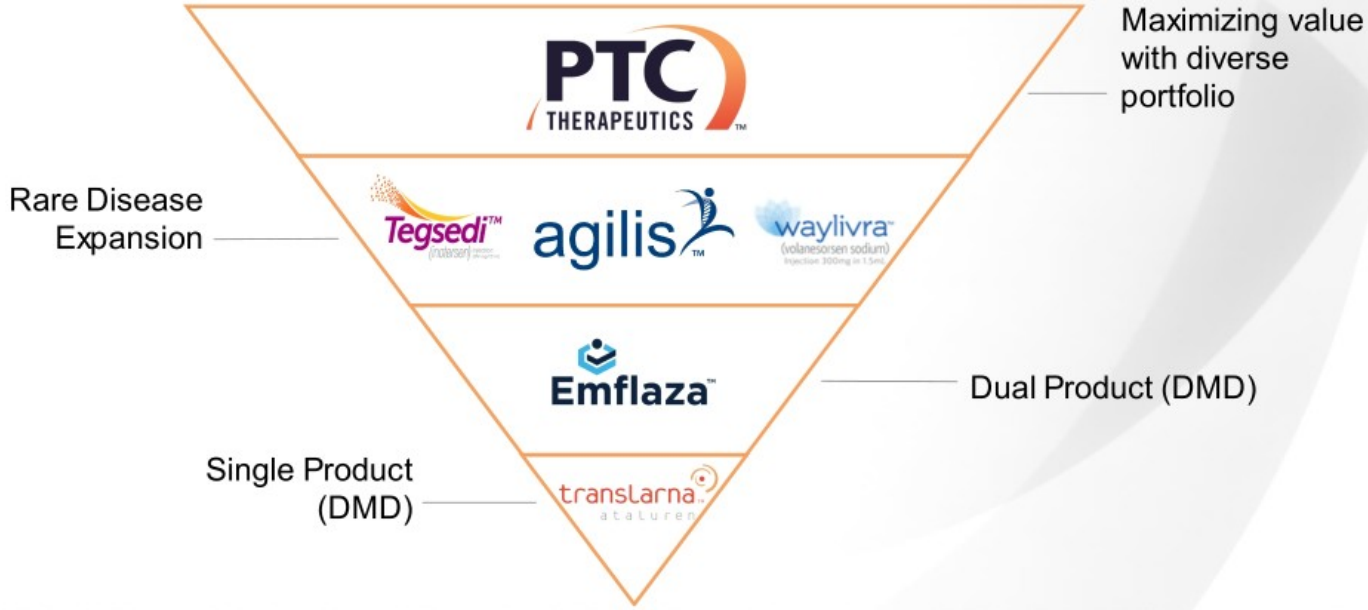
Started 2018 with a clear 3-year vision

VISION

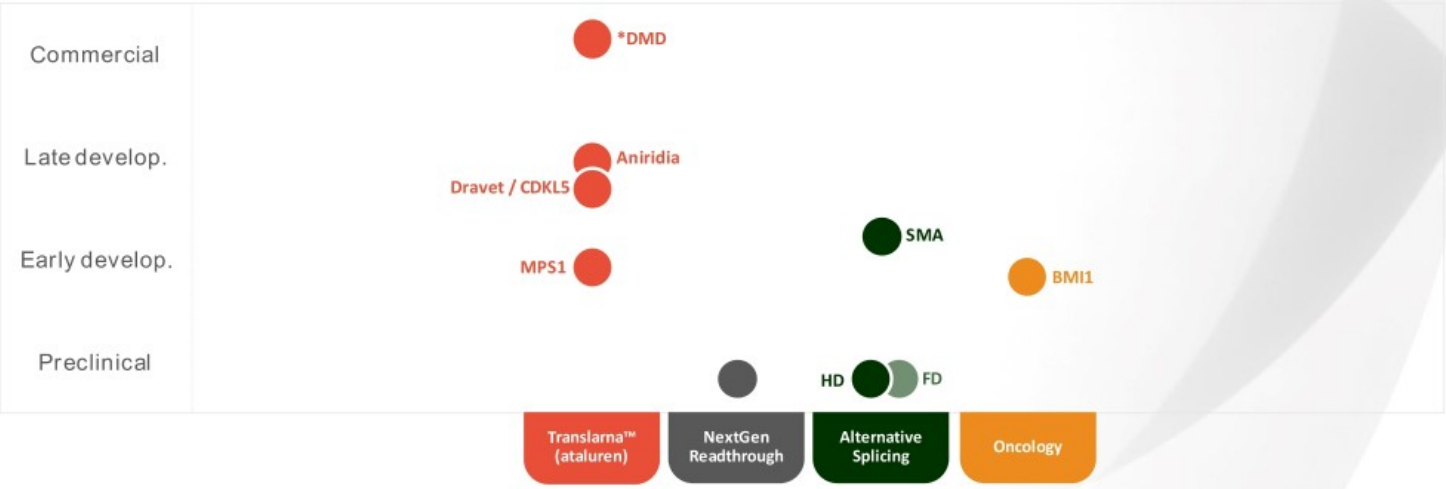
PTC is a fully integrated, innovative rare disorder company leveraging research capabilities and core technology platforms, building out world-class commercial capabilities, and being an ideal partner for late-stage, ultra-orphan disorders for which there is high unmet medical need.



Building a diverse leading rare disorder biotech: Delivering on our vision

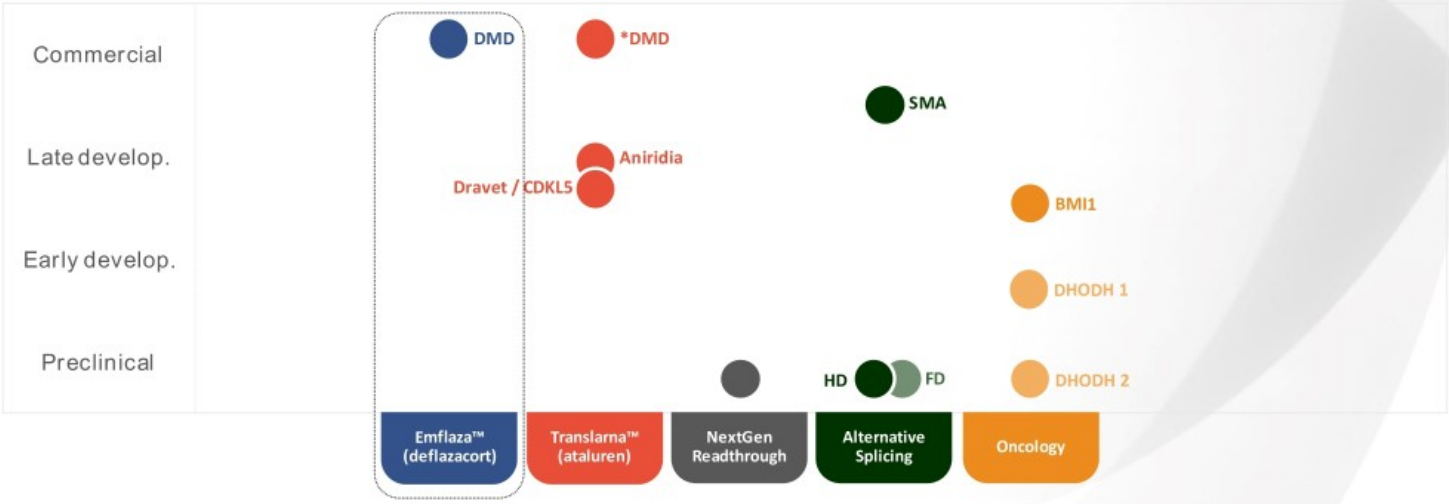


Pipeline evolution: January 2017



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

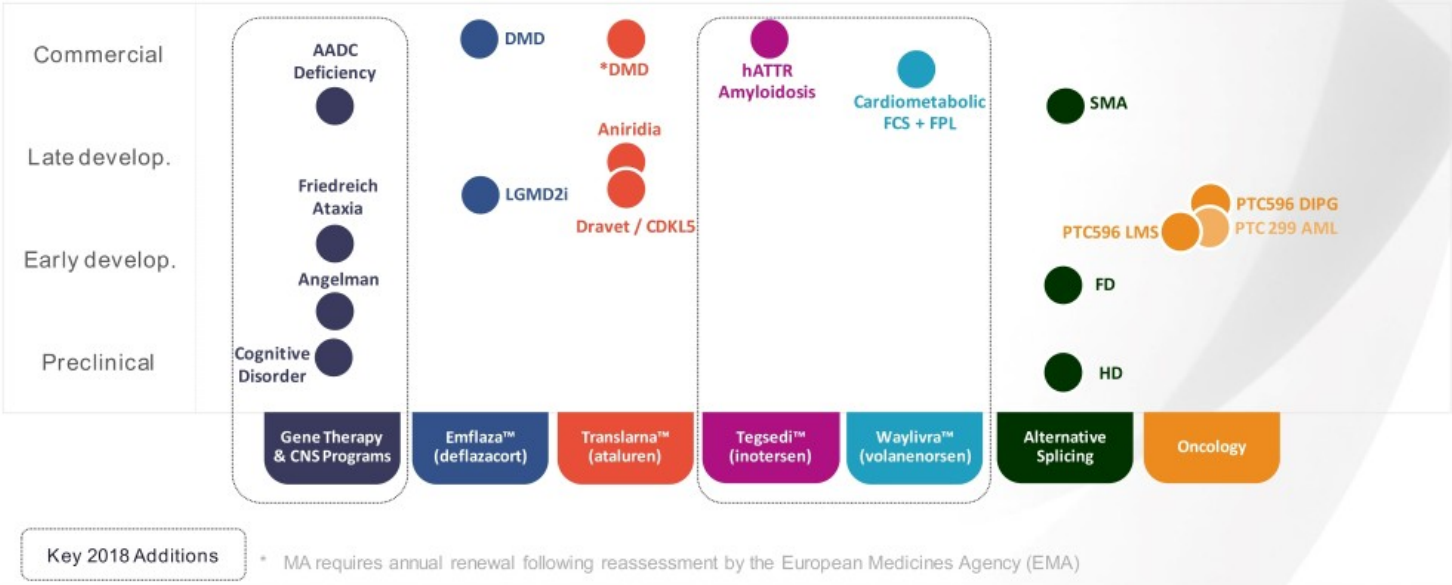
Pipeline evolution: January 2018



Key 2017 Additions

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

Pipeline evolution: January 2019



Key 2018 Additions

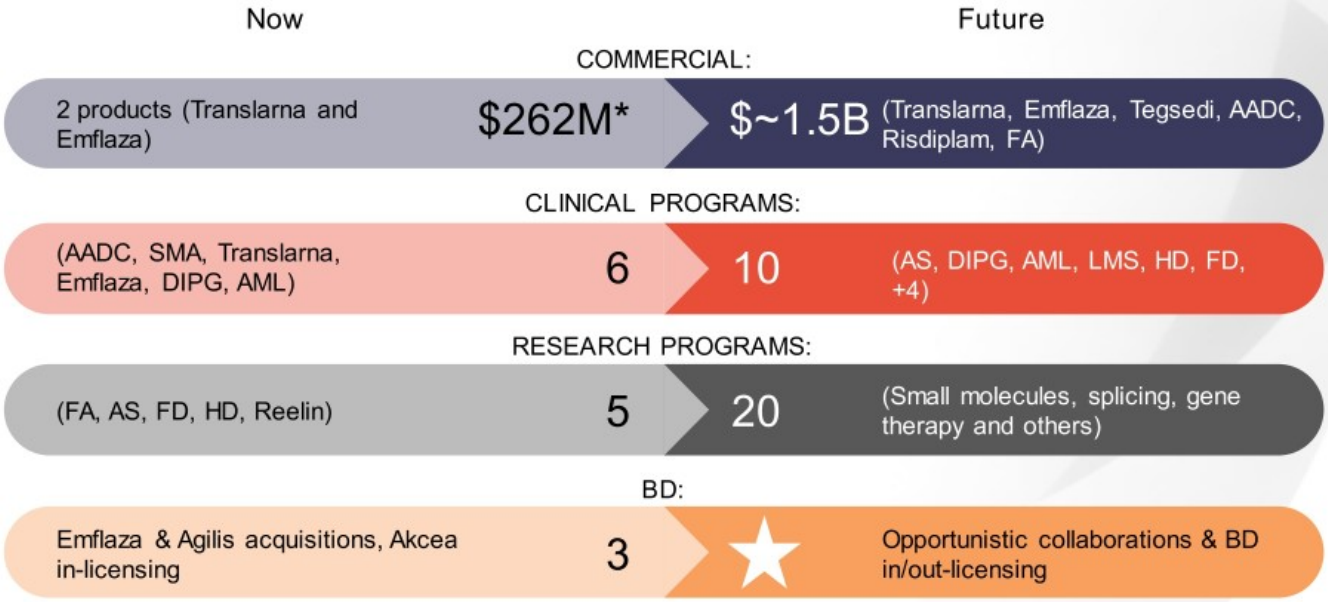
* MA requires annual renewal following reassessment by the European Medicines Agency (EMA)

Delivering on our 3-year vision

VISION

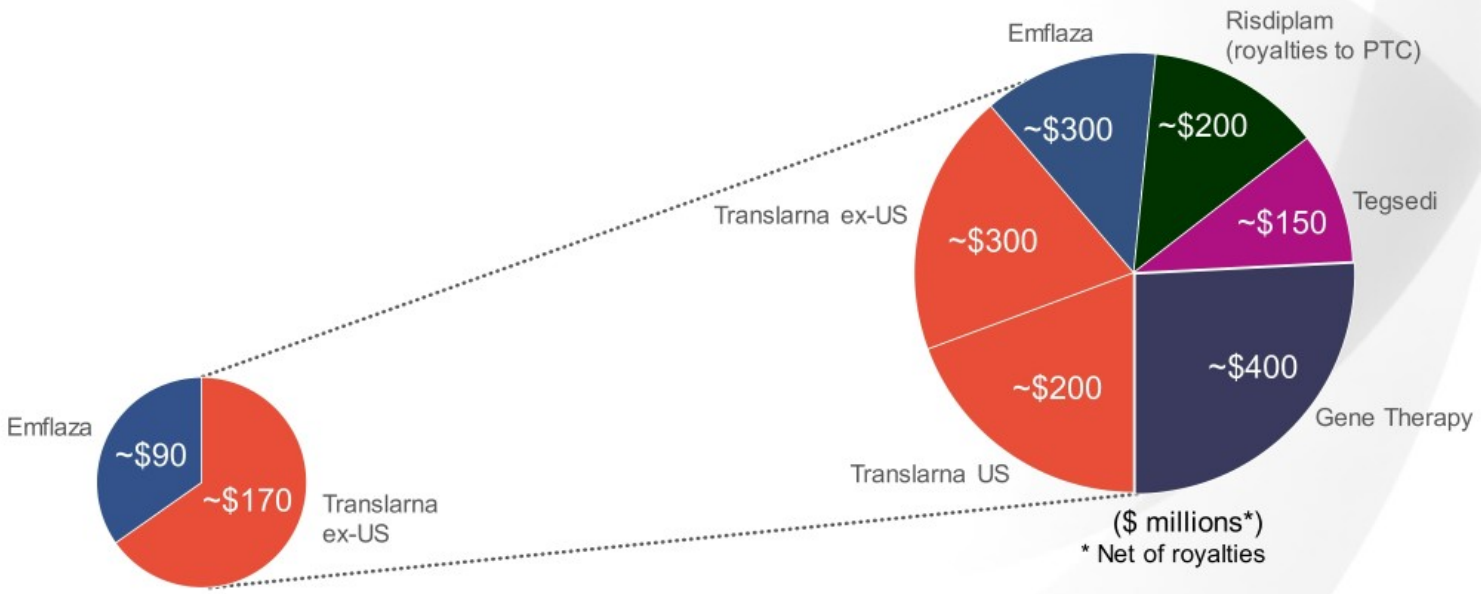
PTC is a fully integrated, innovative rare disorder company leveraging research capabilities and core technology platforms, building out world-class commercial capabilities, and being an ideal partner for late-stage, ultra-orphan disorders for which there is high unmet medical need.

Looking forward:
PTC growth vision for the next 5 years



* Unaudited 2018 revenue

~$1.5B potential revenues to PTC by 2023



* Revenue based on PTC current assumptions and estimates


Building a Leading Rare Disorder Biotech
Global DMD Franchise
PTC

PTC is the leader in DMD treatment
2 of 3 approved products

- Translarna is the first-ever targeted therapeutic approved for DMD anywhere in the world (EMA, 2014)
- Translarna is now available in >40 countries worldwide ex-US and in trials for US potential approval in 2020
- Emflaza is the first-and-only corticosteroid approved specifically for DMD anywhere in the world (US, 2017)
- Emflaza data demonstrates best-in-class corticosteroid
- PTC DMD franchise is now helping many thousands of families living with Duchenne around the world

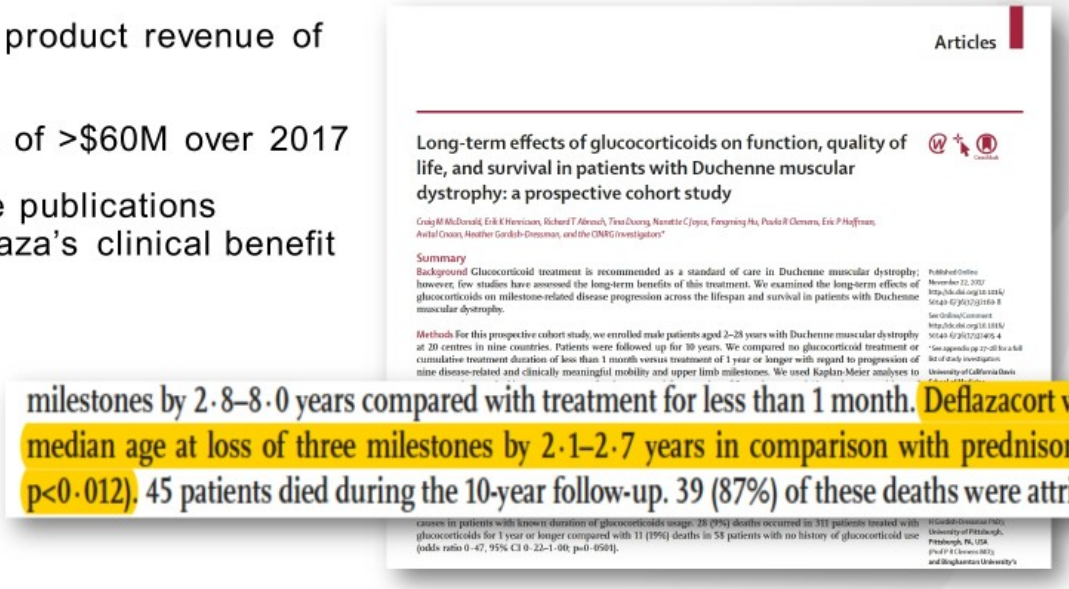
Translarna™: proven track record of performance

- Unaudited 2018 net product revenue of $171M, an 18% increase over 2017
- Global sales outside of the U.S.
- Pediatric expansion approved in 2018
- Label expansion for non-ambulatory patients under review
- U.S. dystrophin study underway, completion YE:19

Emflaza®: Establishing standard of care for all DMD patients in the US

Emflaza® (deflazacort)

- 2018 Emflaza net product revenue of $91M (unaudited)
- Revenue increase of >$60M over 2017
- Data from multiple publications demonstrate Emflaza's clinical benefit over prednisone



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 Henricson, Richard T Abresch, Tina Duong, Nanette C Joyce, Fangming Hu, Paula R Clemens, Eric P Hoffman, Awital Cnaan, Heather Gordish-Dressman, and the CINRG 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.

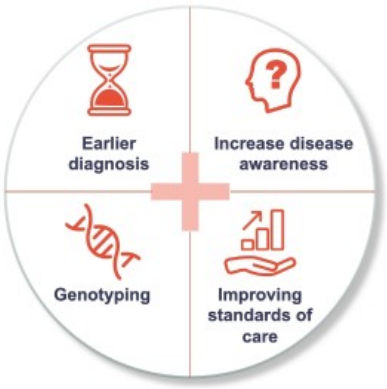
Methods For this prospective cohort study, we enrolled male patients aged 2–28 years with Duchenne muscular dystrophy at 20 centres in nine countries. Patients were followed up for 10 years. We compared no glucocorticoid treatment or cumulative treatment duration of less than 1 month versus treatment 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

milestones by 2·8–8·0 years compared with treatment for less than 1 month. Deflazacort was as
median age at loss of three milestones by 2·1–2·7 years in comparison with prednisone or
p<0·012). 45 patients died during the 10-year follow-up. 39 (87%) of these deaths were attributal

causes in patients with known duration of glucocorticoids usage. 28 (9%) deaths occurred in 311 patients treated with glucocorticoids for 1 year or longer compared with 11 (19%) deaths in 58 patients with no history of glucocorticoid use (odds ratio 0·47, 95% CI 0·22–1·00; p=0·0501).

PTC THERAPEUTICS

Continuing to drive long-term growth of DMD franchise



translarna™
ataluren

Label expansion under review for Translarna™ in non-ambulatory patients by the EMA

sNDA for Emflaza® 2-5 year old U.S. patients submitted with potential approval in '19

Emflaza™
(deflazacort)

An efficient, scalable business engine

- 2018 unaudited product net revenue of $262M
- 2019 DMD franchise revenue guidance of $285 - $305M
- Established footprint in >40 countries worldwide
- Experienced commercial and medical teams in orphan disease
- Fully integrated global infrastructure


Building a Leading Rare Disorder Biotech
Leveraging our Global Commercial Franchise
PTC

Preparing for successful launch

Tegsedi™ (inotersen)

Tegsedi best fit for Latin American hATTR market

hATTR polyneuropathy most prevalent phenotype in Latin America ~6,000 patients

Sub-cutaneous self administration preferable to infusions in the region

Diversifies our rare disease portfolio and revenues

All key hiring completed in Latam

Regulatory dossier filed with ANVISA and rare-disease priority review granted

Expected approval YE:19

PTC THERAPEUTICS

Two potential assets in Latin America

Tegsedi™ (inotersen)

Tegsedi best fit for Latin American hATTR market

hATTR polyneuropathy most prevalent phenotype in Latin America ~6,000 patients

Sub-cutaneous self administration preferable to infusions in the region

Diversifies our rare disease portfolio and revenues

waylivra™ (volanesorsen sodium) Injection 300mg in 1.5mL

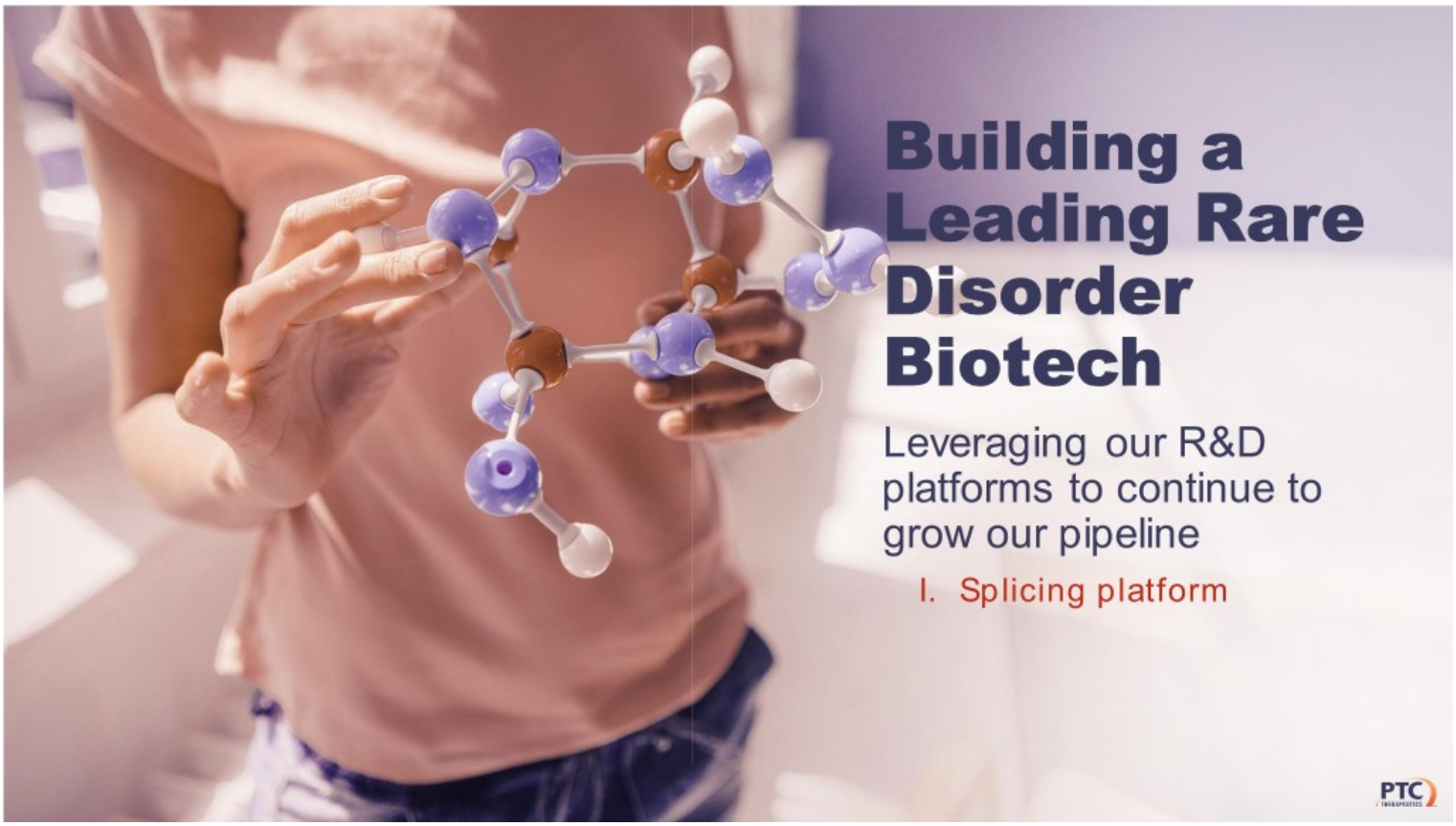
Waylivra: could utilize our patient support in Latin America

Similar economic opportunity to Translarna in Latin America

No other treatments available to treat FCS

Under regulatory review in EU

FCS = familial chylomicronemia-syndrome
FPL = familial partial lipodystrophy


Building a Leading Rare Disorder Biotech
Leveraging our R&D platforms to continue to grow our pipeline
I. Splicing platform
PTC

Leaders in small molecule RNA-splicing technology

Development of SMA candidate as potential best-in-class treatment

13 years of discovering and developing drugs that target pre-mRNA splicing

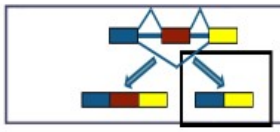
Cutting-edge tech platform discovered and developed by PTC

2nd Splicing Compound: A Development Candidate to treat Familial Dysautonomia

Continue to exploit Splicing platform; addressing additional areas of unmet need

Risdiplam in development for Spinal Muscular Atrophy (SMA)

- Primary genetic cause of infant mortality
- Small molecule promotes the correct splicing of the mutant RNA
- Small molecule has potential for best in class therapy
- Broad tissue distribution and protein restoration



Risdiplam targets-splicing events to restore SMN protein levels

Risdiplam has potential to be a $2B product

- Revenue > $1B subject to mid-teens* royalty to PTC from Roche
- Potential to PTC to exceed $200M/ year; including competitive assumptions for SMA gene therapy
- Firefish & Sunfish fully enrolled
- Risdiplam well tolerated at all doses, no ocular toxicity found in humans

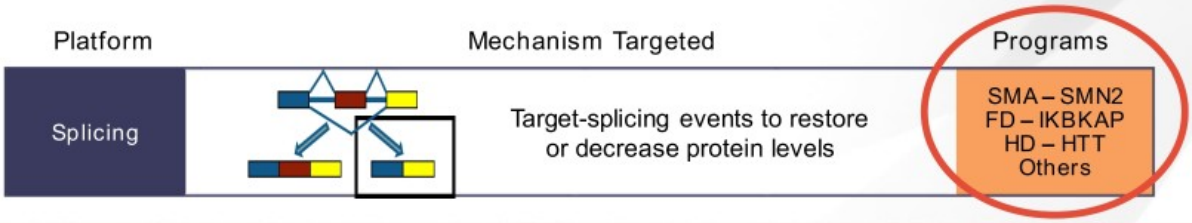
SMA FOUNDATION

Roche

* Revenue estimates based on PTC solely on assumptions
Full tiered royalty table in press release

The splicing technology is a proven platform to identify new therapeutics

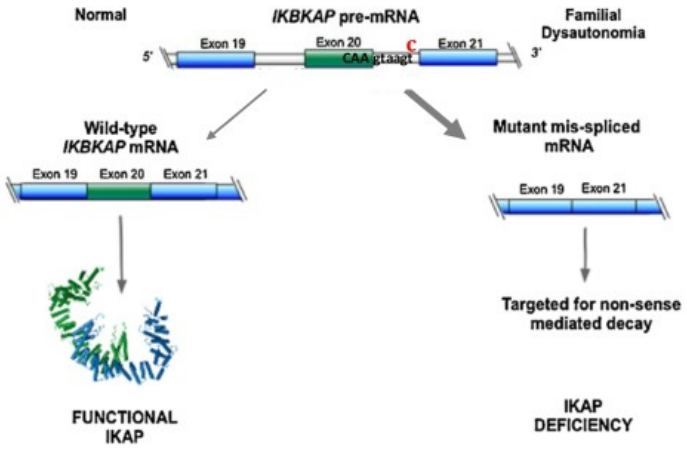
- Development of SMA candidate as potential best-in-class treatment
- 13 years of discovering and developing drugs that target pre-mRNA splicing
- Cutting-edge tech platform discovered and developed by PTC
- 2nd splicing compound: A development candidate to treat Familial Dysautonomia
- Continue to exploit splicing platform; addressing additional areas of unmet need



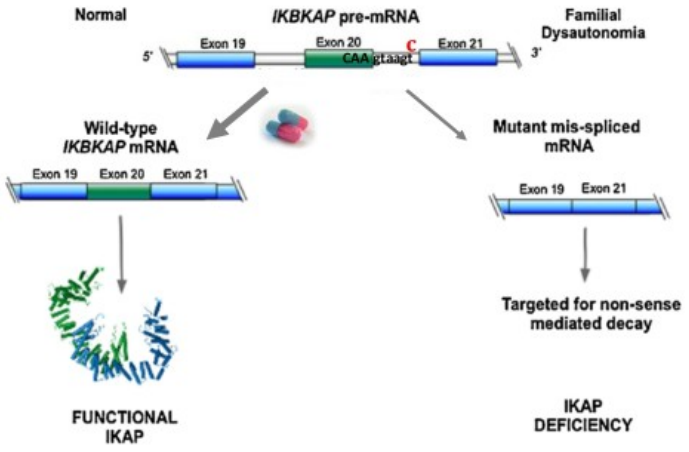
Familial dysautonomia:

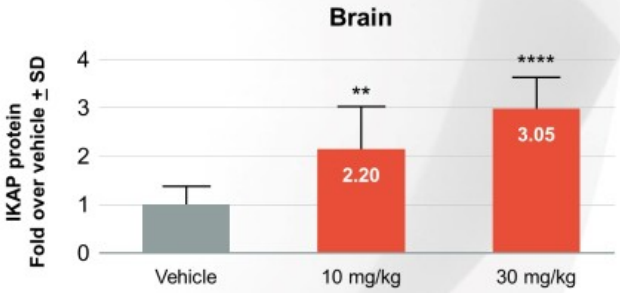
- Genetic disorder primarily affecting the sensory and autonomic neurons
- Caused by a splicing-altering mutation in the IKBKAP (ELP1) gene resulting in low levels of IKAP protein
- Ashkenazi Jewish ancestry, carrier frequency is ~1:30
- No therapies are currently available for FD, only supportive treatments
- PTC is collaborating with MGH and NYU to advance treatments for FD

The splicing mutation in IKBKAP gene leads to IKAP protein deficiency resulting in FD

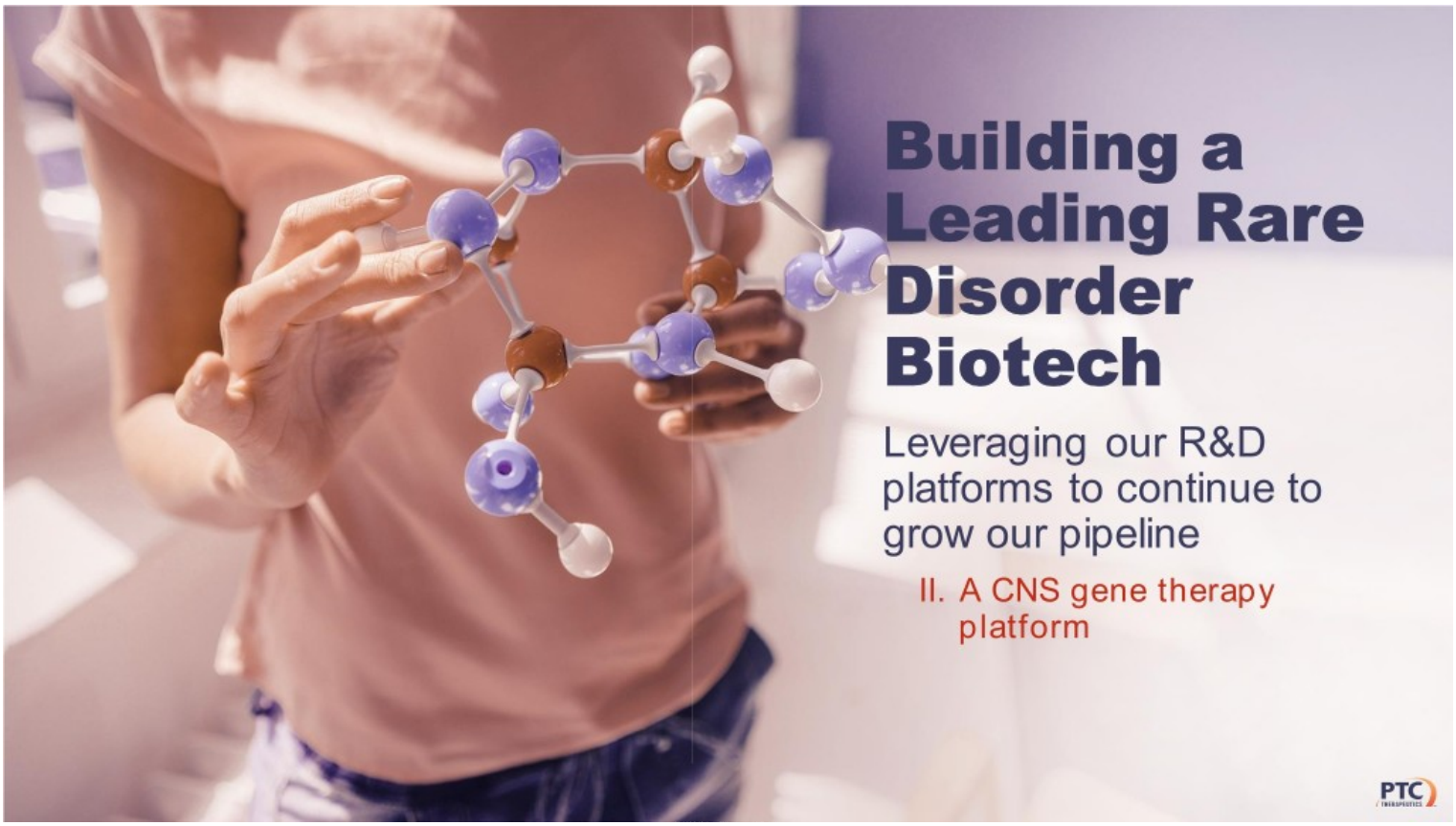


PTC-258 splicing modifiers restore IKAP levels





Development candidate PTC-258 selected YE:18
Scheduled to enter the clinic in 2019


Building a Leading Rare Disorder Biotech
Leveraging our R&D platforms to continue to grow our pipeline
II. A CNS gene therapy platform
PTC

Gene therapy development strategy

Execute on current programs

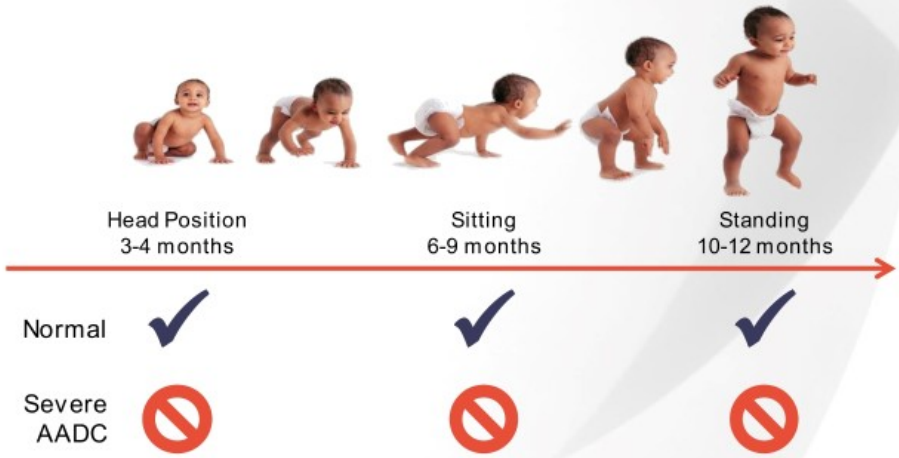
Target dates:
- AADC Launch 2020
- Friedreich Launch 2023
- Angelman IND 2020
- Reelin IND 2020

Priority to secure in-house manufacturing capabilities to support long-term capacity

Expand the pipeline with internal research and external collaboration

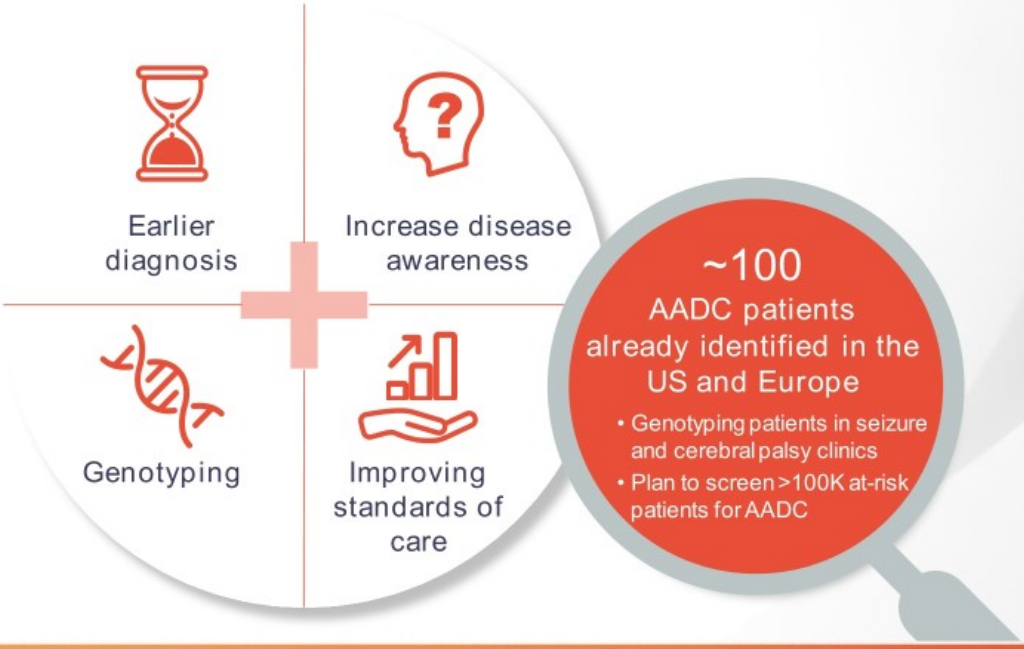
AADC deficiency is a devastating disease with high unmet need

- Rare progressive childhood disease, affecting approximately 5,000 patients globally
- Children with severe AADC deficiency never achieve motor development milestones
- Profound development failure with shortened life expectancy in severe forms (4 - 8yrs)



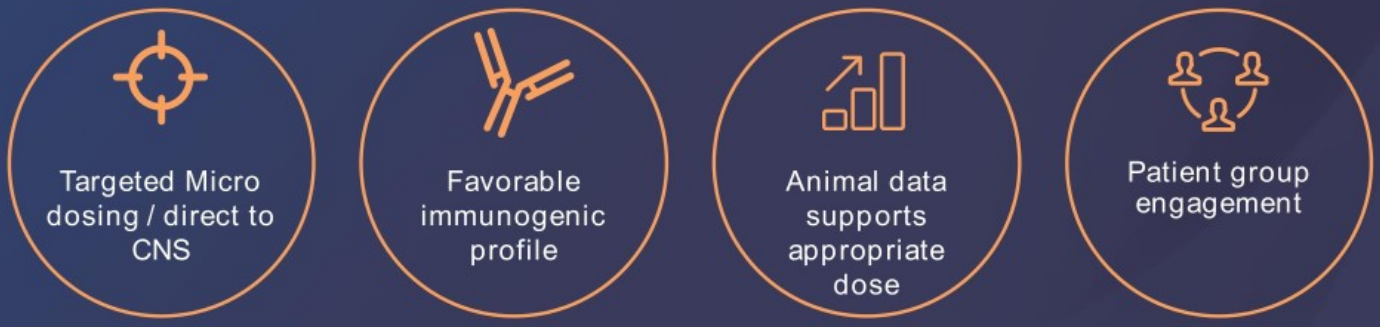
Wassenberg T et al. Orphanet J Rare Dis. 2017;12(1):12

Patient identification is our expertise



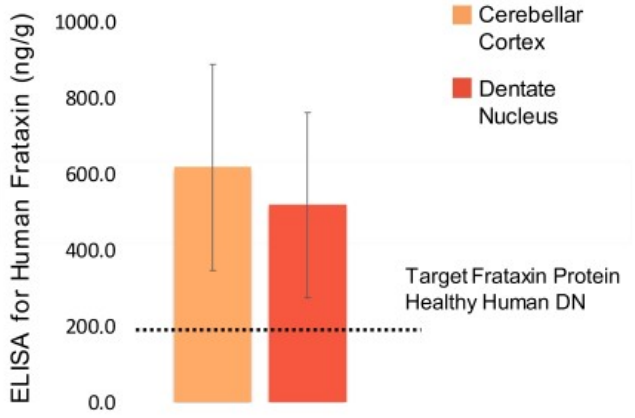
Most advanced FA gene therapy program

PTC plans to file IND in 2019



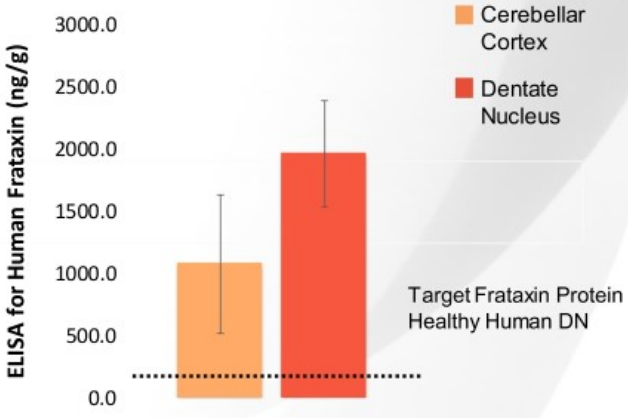
Moving toward IND filing in 2019

PTC-FA Intracerebellar Dosing in Porcine Model*



Unilateral dose of 3.0 x 10^{12} vg total - Day 28 Mean (SEM)
*Human-specific detection

PTC-FA Intracerebellar Dosing in NHP Model*

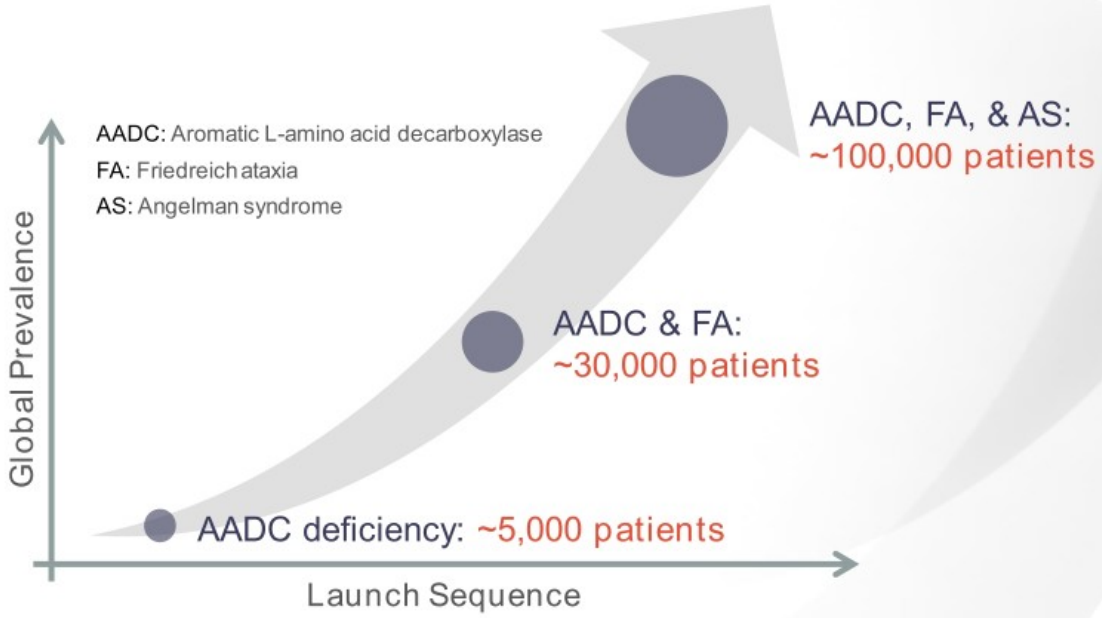


Bi-lateral Dose of 2.4 x 10^{12} vg total - Day 28 - Mean (SEM)
*NHP background subtracted

2019 goals: file an AADC BLA & FA IND

Immediate clinical manufacturing capabilities as well as the plan to expand to commercial scale

Potential addressable market in excess of $5B

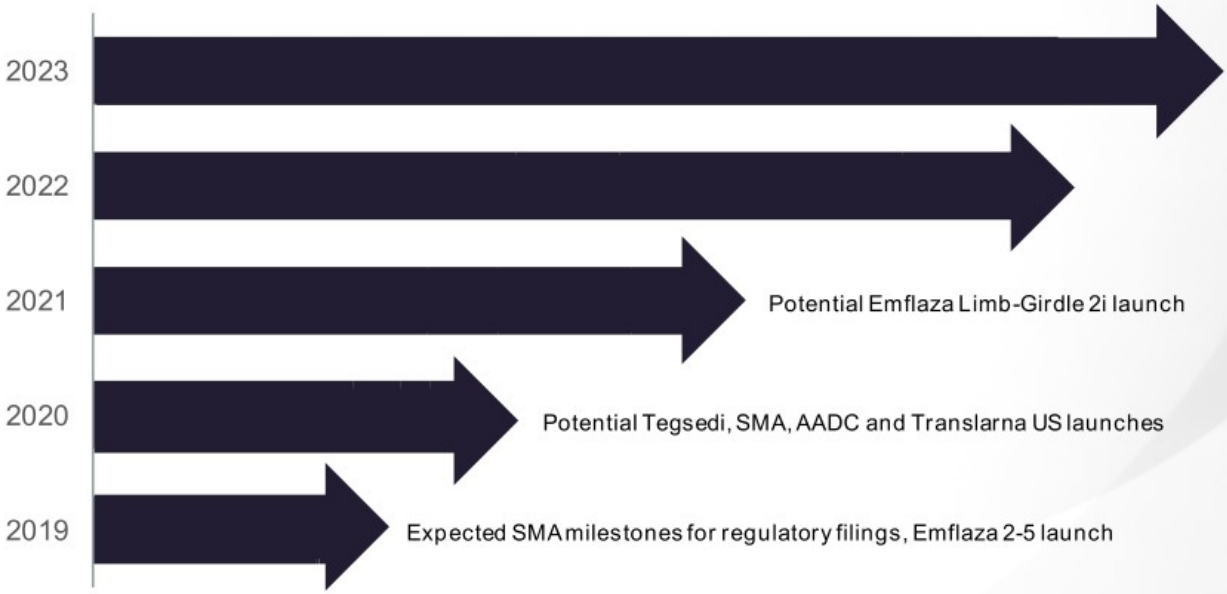


Niche oncology strategy prioritizes value creation

Internal research	Use of current platforms to add new targets to portfolio with focus on splicing
Solid tumors	PTC596 in pediatric brain tumor (DIPG) phase 1/2 trial and Leiomyosarcoma (LMS)
Hematologic malignancies	PTC299 AML dose escalation trial initiated in 2018
Business development	Assess out-licensing opportunities

Sustainable growth expected over next 5 years

Potential revenues to PTC from DMD franchise, Gene therapy programs, Tegsedi and Risdiplam



measured by moments

Everyone has a different definition of progress. For the last 20 years, we've measured our progress researching rare disease in moments. Smiling ones and crying ones. Moments spent with our boys' families and ones with their friends. We know that every step forward comes after several steps backward, because we've lived it—whether spending time with families in their homes or with our scientists researching in our labs.

It can be easy to lose yourself as you progress further. Although we've grown, our heart remains in the same place, because we've never measured ourselves like larger companies do. Our biggest accomplishment has always been the time we can give to all of our families. Whether it's hours, days, months, or years, every small moment is a big win.

PTC THERAPEUTICS