PTC Q3:18 Earnings call

Nov 5, 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 related to PTC's future expectations, plans and prospects; expectations with respect to PTC's recently acquired 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; PTC's strategy, future operations, future financial position, future revenues, projected costs; or intended use of proceeds from its public offering of common stock; 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 for which 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 Biotherapeutics, Inc. ("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; 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 or any of PTC's other product candidates; 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; 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 or Waylivra.

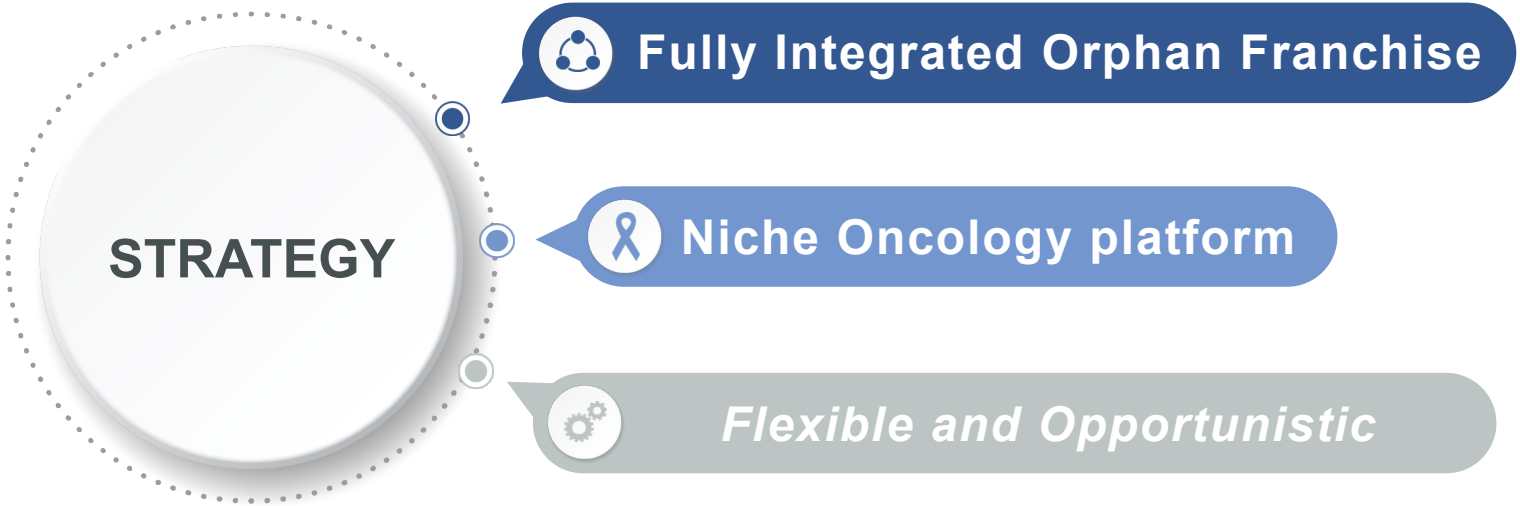
The forward-looking statements contained herein represent PTC's views only as of the date of this presentation and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this presentation except as required by law.

PTC THERAPEUTICS™ 20 YEARS OF COMMITMENT

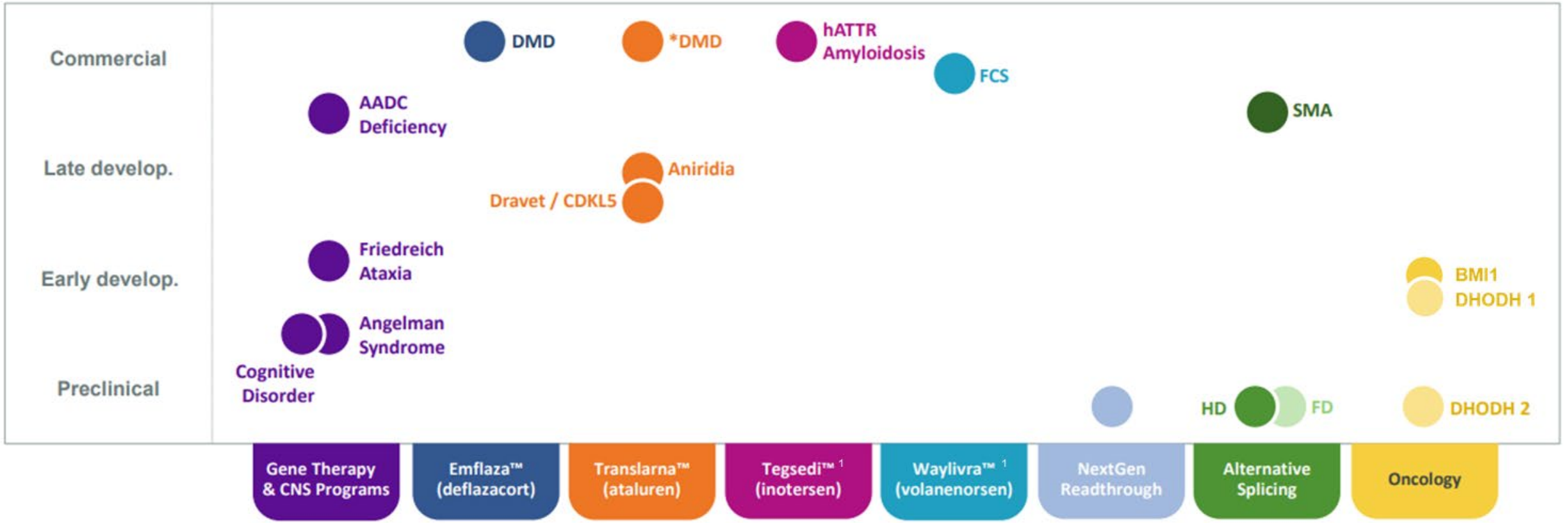
Delivering on our strategic vision

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.



PTC Product Pipeline

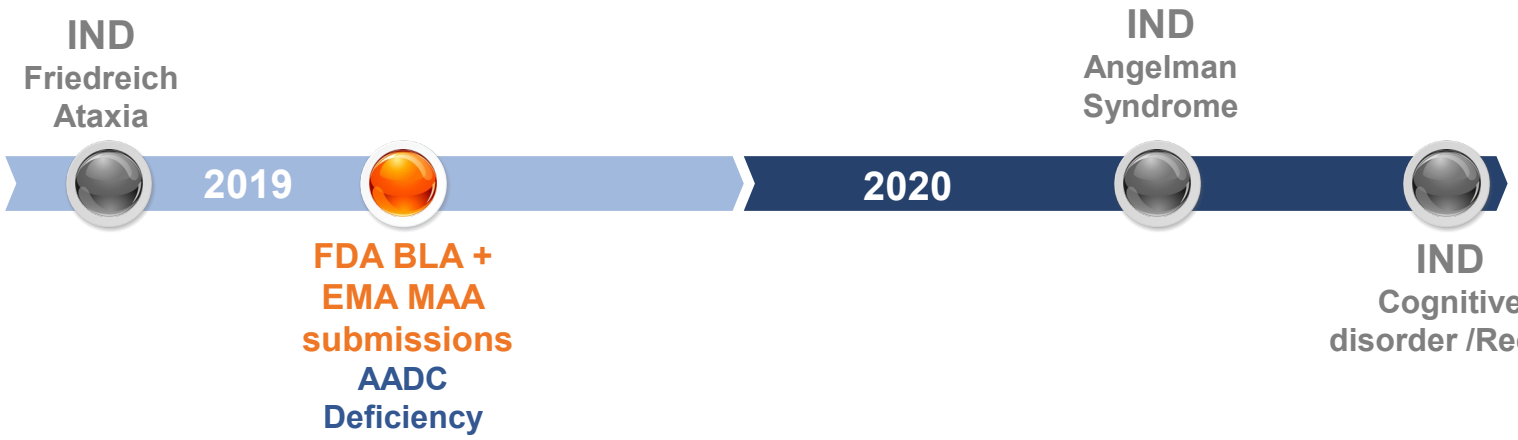


* MA requires annual renewal following reassessment by the European Medicines Agency (EMA), confirmatory study 041 for conditional approval ongoing

[1] LATAM & Caribbean rights licensed from Akcea Therapeutics

Note: Cardiometabolic diseases due to elevated triglyceride level: FCS = Familial Chylomicronemia Syndrome

Potential gene therapy development milestones



Platform gene therapy manufacturing advantages

Targeted micro-dosing

- Low doses of vector required
- Efficient, scalable manufacturing
- Low manufacturing hurdles using existing systems

Strategic partnership with **MassBiologics Laboratories**

Immediate clinical manufacturing capabilities as well as the **potential to expand** to commercial scale

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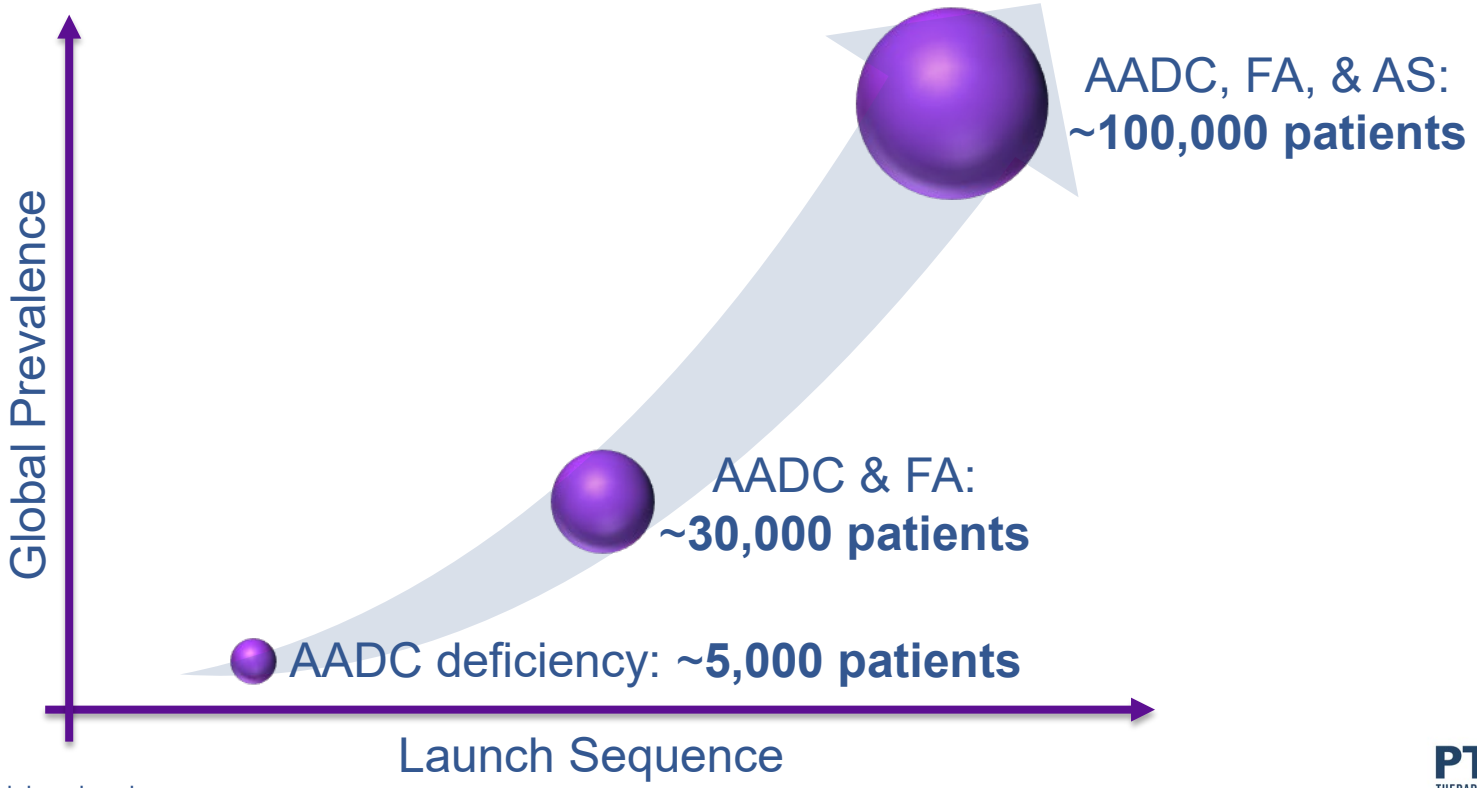
Potential addressable market in excess of $5B



AADC: Aromatic L-amino acid decarboxylase
FA: Friedreich ataxia
AS: Angelman syndrome

Acquisition fits perfectly within PTC's strategy

20
years
rare genetic disease focus

Global
development and business infrastructure

State of art
patient finding & reimbursement

Market Access Expertise

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 and require care throughout their lifetimes
- Profound development failure with shortened life expectancy in severe forms (4 - 8yrs)

	Head Position 3-4 months	Sitting 6-9 months	Standing 10-12 months
Normal	✓	✓	✓
Severe AADC	✗	✗	✗

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

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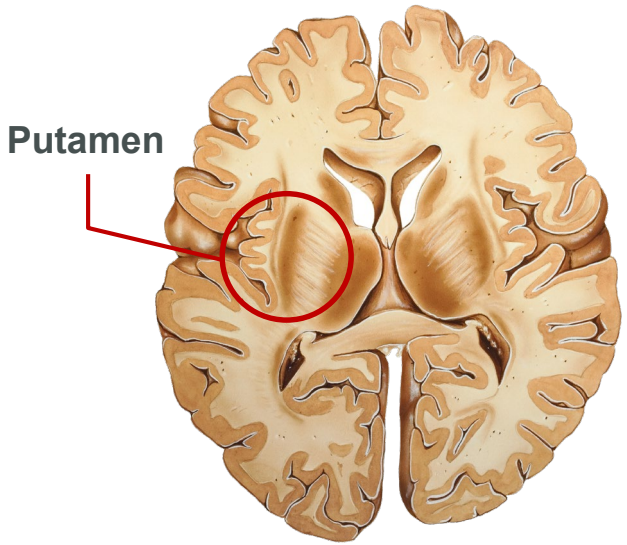
PTC- AADC:
Advanced CNS-delivered gene therapy program



- **Target-delivered gene therapy**
  - Single administration of AAV2-hAADC
  - Low dose (1.8 x 10^{11} vg total)
  - Direct delivery using established stereotactic surgery
- **Clinically durable effect in patients**
  - First patients treated in 2010
  - Three clinical studies with safety data in 26 patients
  - Functional improvements on validated scales
  - Significant and durable gains in major motor development milestones

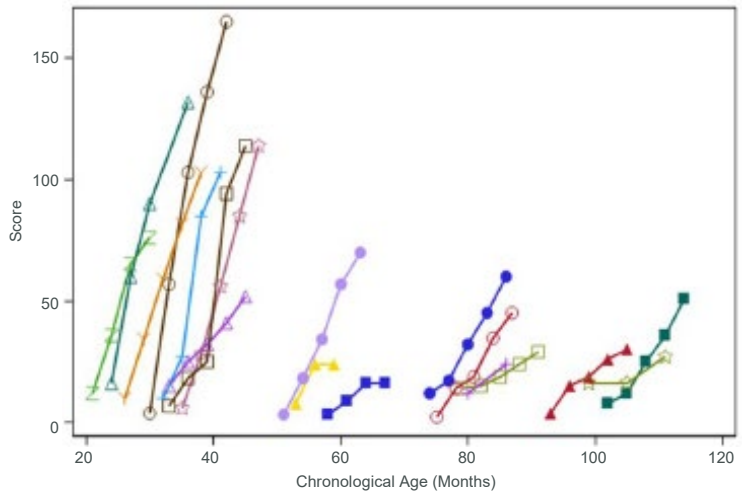
Hwu W et al. Sci Transl Med. 2012;4(134):134ra61
Hwu W et al. Lancet Child Adolesc Health 2017;1: 265–73

PTC THERAPEUTICS™ 20 YEARS OF COMMITMENT

PTC-AADC:
Significant & durable motor improvements

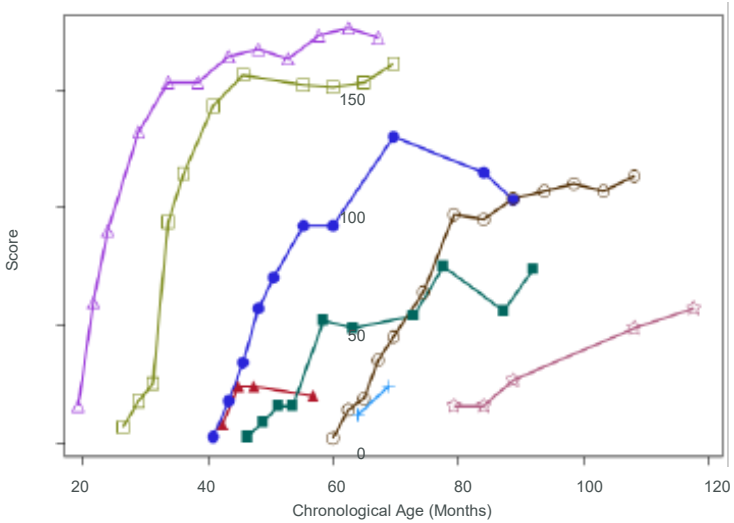
1-Year Results of Motor Development

PDMS-2[1] through 12 months – Studies 1 & 2



5-Year Results of Motor Development

PDMS-2[1] through 60 months - Study 1



1 Peabody Developmental Motor Scale

AADC deficiency patient
Before and after treatment with PTC-AADC

Video Link

Case 5 T0 (2 yo)

In part: Science Translational Medicine 16 May 2012 Vol 4 Issue 134 134ra61

PTC THERAPEUTICS™ 20 YEARS OF COMMITMENT

AADC deficiency patient
Before and after treatment with PTC-AADC

Video Link

Case 5 *T0 (2 yo)* *T13 (3 yo)*

In part: Science Translational Medicine 16 May 2012 Vol 4 Issue 134 134ra61

PTC THERAPEUTICS™ 20 YEARS OF COMMITMENT

AADC deficiency patient
Before and after treatment with PTC-AADC

Video Link

Case 5 *T0 (2 yo)* *T13 (3 yo)* *T29 (4 yo)*

In part: Science Translational Medicine 16 May 2012 Vol 4 Issue 134 134ra61

Friedreich Ataxia (FA) is a severe neuromuscular disorder amenable to gene therapy

- Inherited, monogenic disease arising from triplet repeat expansion
- Mutation in frataxin gene limits protein production

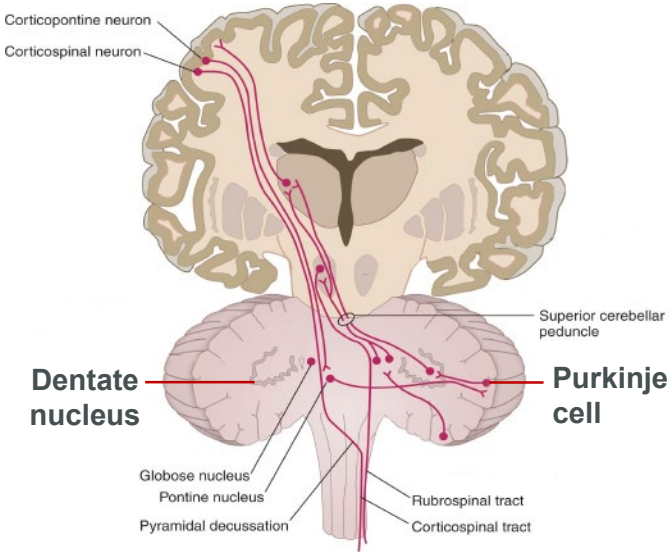
Larger repeat expansion

↓

Lower protein levels

↓

Reduced mitochondrial function



- ✓ **Most common hereditary ataxia (~25,000 patients globally)**
- ✓ **Childhood onset**
- ✓ **Debilitating, life shortening neuromuscular disorder**
- ✓ **Only palliative treatments available currently**

Koeppen A. J Neurol Sci. 2011 April 15; 303(1-2): 1–12

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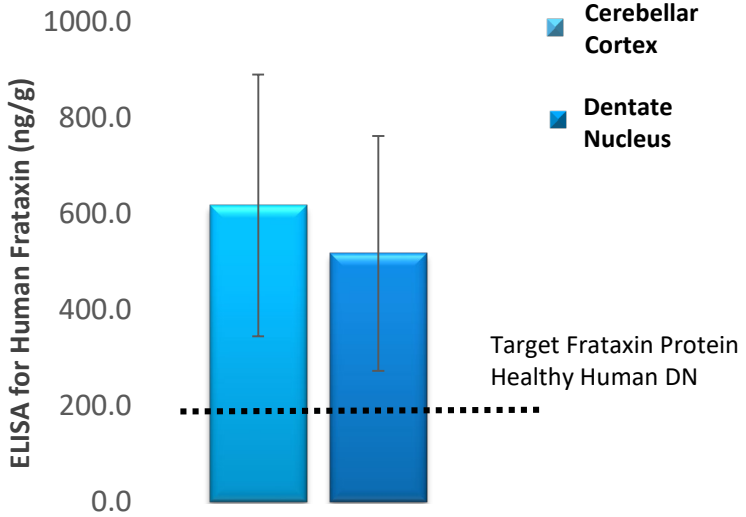
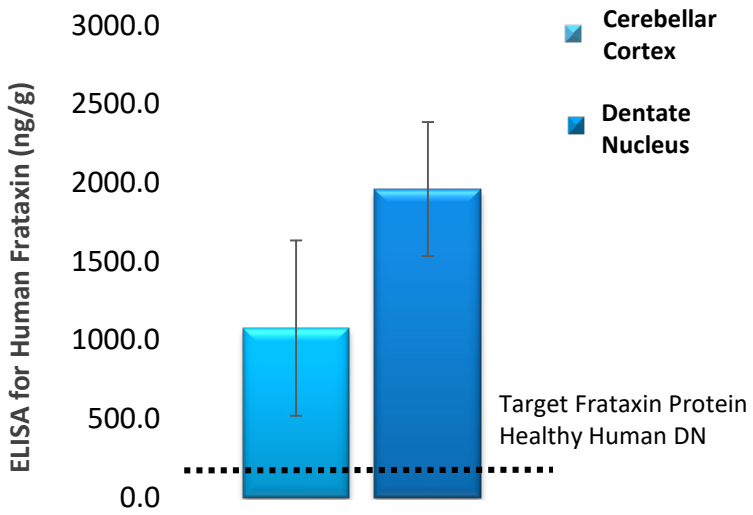
Moving toward IND filing in 2019

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

Most advanced FA gene therapy program

PTC plans to file IND in 2019

- Targeted Micro dosing / direct to CNS
- Favorable immunogenic profile
- Animal data supports appropriate dose
- Patient group engagement

Potential gene therapy development milestones