



PTC Acquisition of Agilis Biotherapeutics

July 19, 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 expectations with respect to the closing of its planned acquisition of Agilis and the other transactions contemplated in conjunction with the acquisition; the potential financial impact and benefits to PTC of the acquisition, including with respect to the business of Agilis to be acquired and PTC's expectations with respect to contingent payments to the Agilis equityholders based on net sales and the potential achievement of development, regulatory and sales milestones and contingent payments to the Agilis equityholders with respect thereto; the future expectations, plans and prospects for PTC; 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 "look forward", "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: satisfaction of the conditions to closing the acquisition (including the failure to obtain necessary Agilis stockholder and regulatory approvals) in the anticipated timeframe or at all; PTC's ability to realize the anticipated benefits of the acquisition, including the possibility that the expected benefits from the acquisition will not be realized or will not be realized within the expected time period; negative effects of the announcement of the acquisition on the market price of PTC's common stock; significant transaction costs; unknown liabilities; the risk of litigation and/or regulatory actions related to the acquisition; 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™ (ataluren) and Emflaza; 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 or Annual Report on Form 10-K as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC.

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 candidate will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Translarna or Emflaza, or any product candidates acquired in the transaction from Agilis.

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.

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.

STRATEGY



Fully Integrated Orphan Franchise

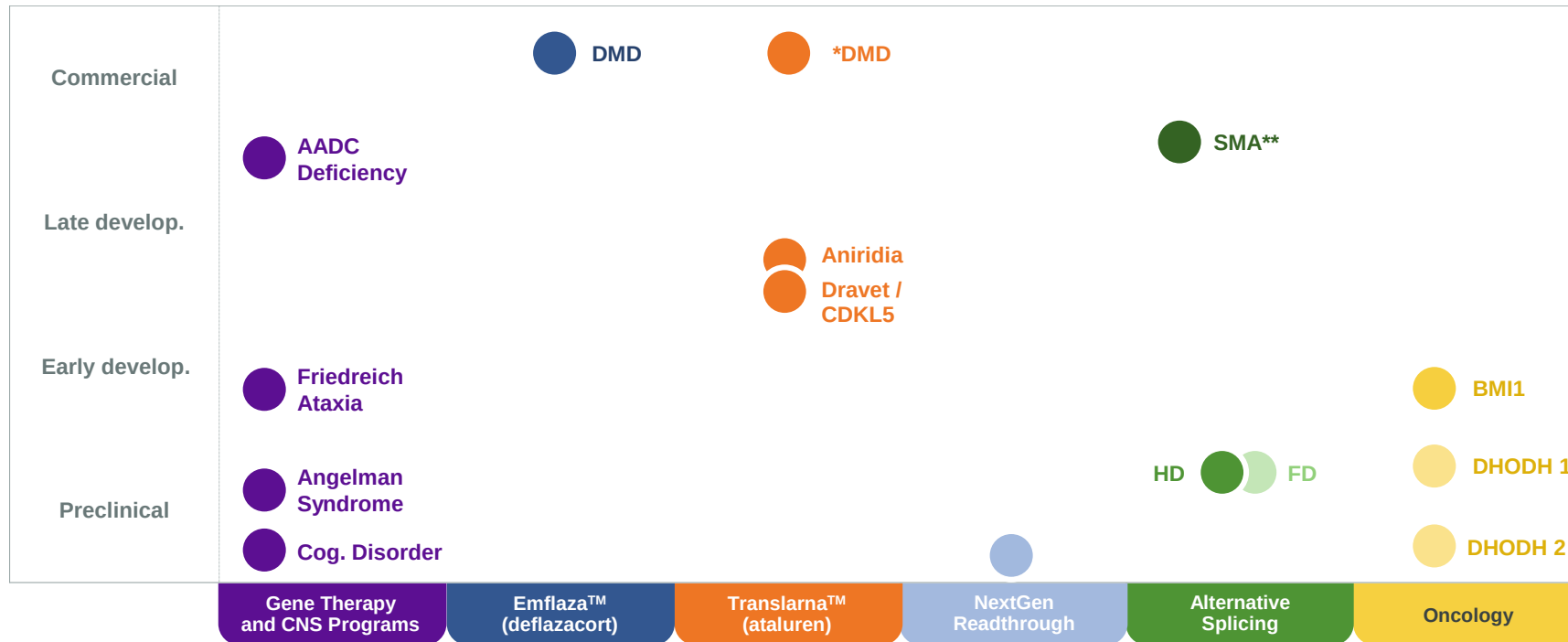


Niche Oncology platform



Flexible and Opportunistic

Strong rare disease combined pipeline



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

** Sunfish clinical study of RG7916 in SMA type 2/3 patients transitioned to its phase 3 second stage in Oct 2017, Firefish in SMA type 1 patients transitioned to its phase 3 second stage in March 2018

Acquisition fits perfectly within PTC's strategy



20

years

rare genetic
disease focus



Global
development
and business
infrastructure

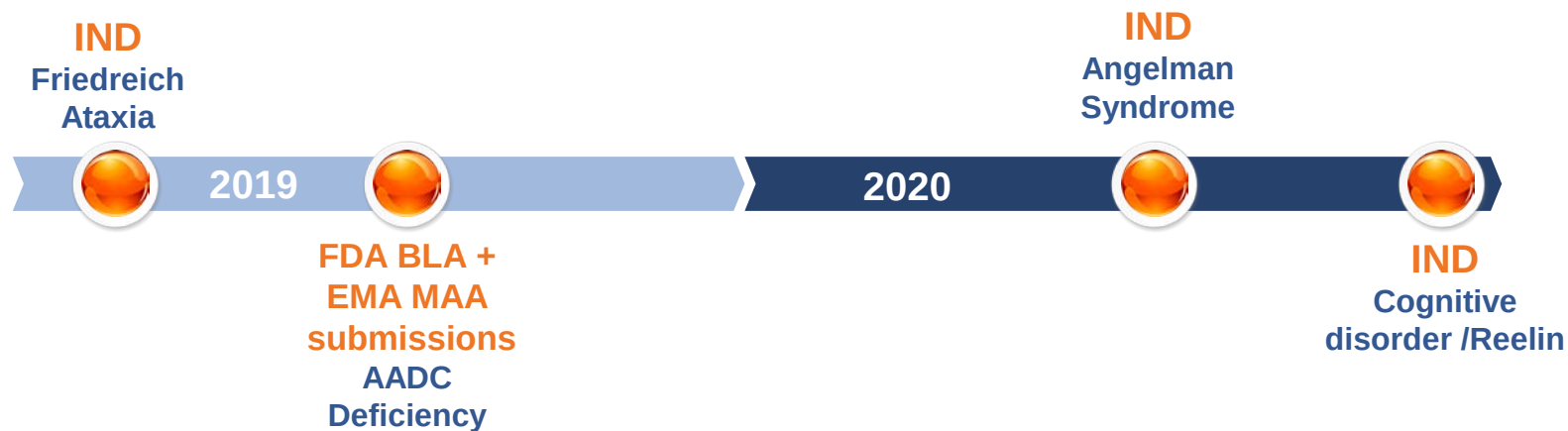


State of art
patient finding &
reimbursement



Market
Access
Expertise

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

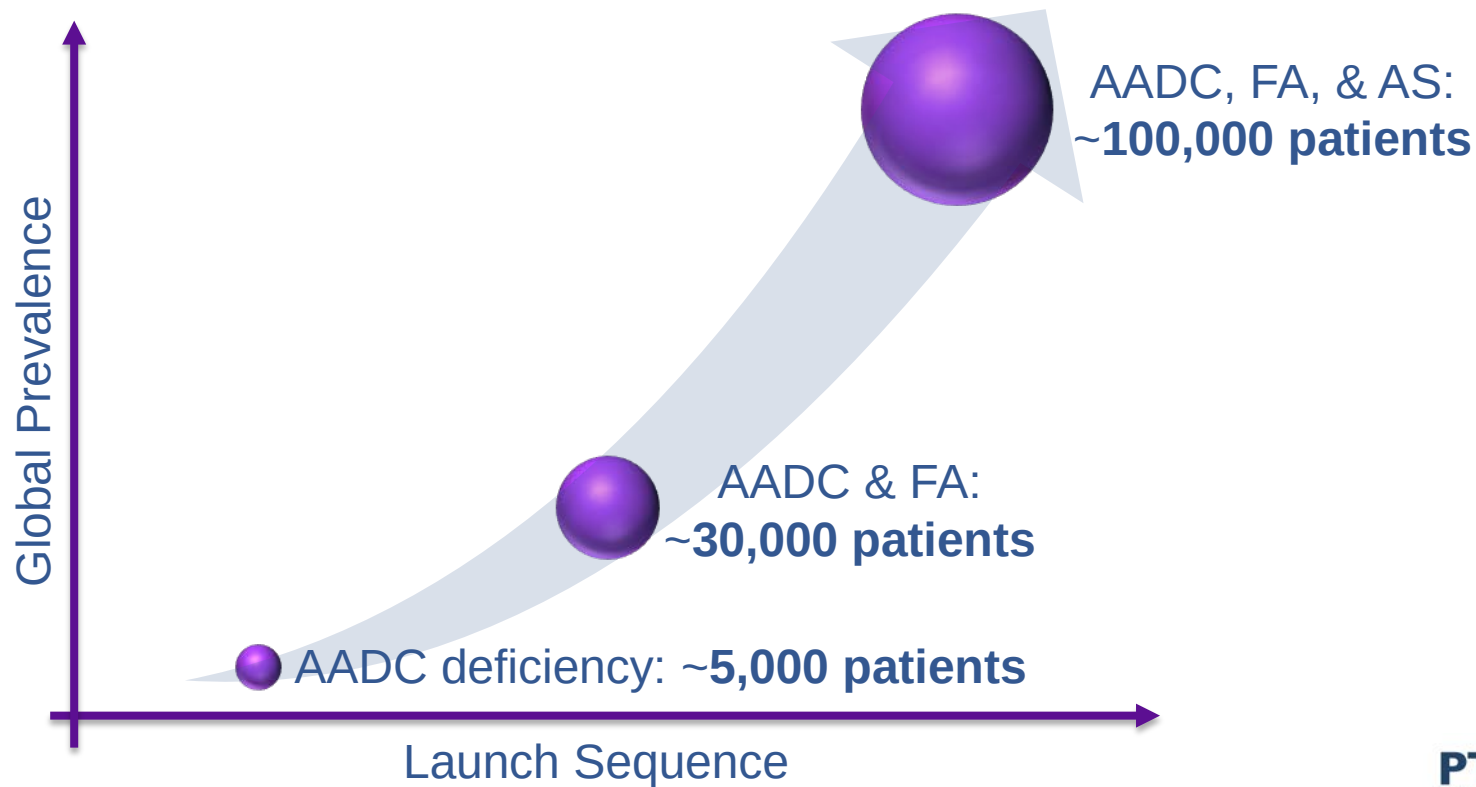
**MassBiologics
of UMMS***



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

*MassBiologics of University of Massachusetts Medical School

Potential addressable market in excess of \$5B



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

Strengthening leadership team and adding experience in gene therapy

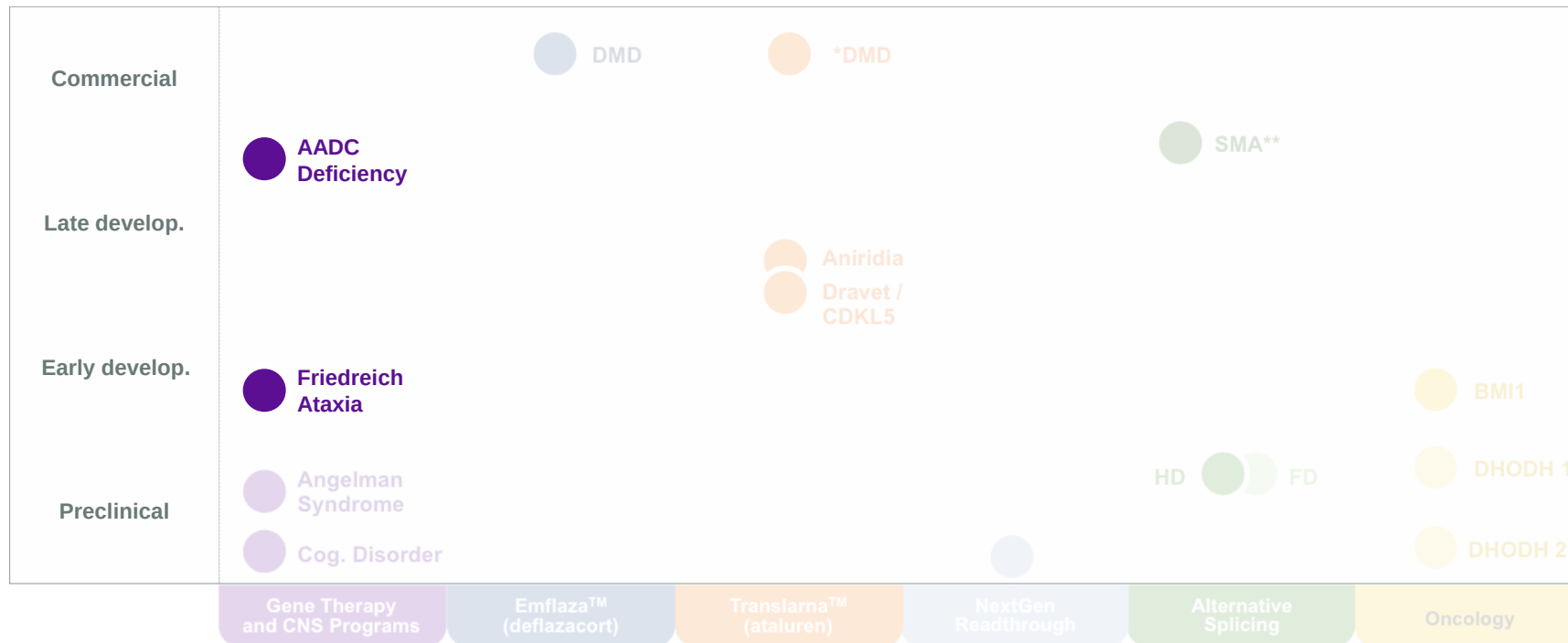


Dr. Pykett
Agilis President and
Chief Executive Officer



Dr. Cook
Agilis Chief Operating Officer

Focus on the most advanced gene therapy programs

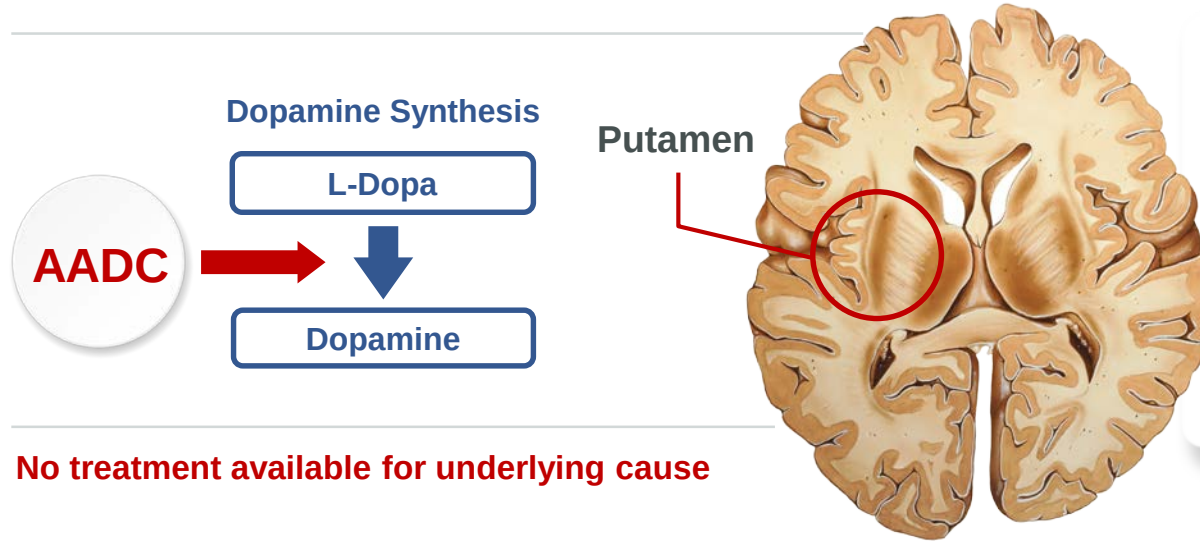


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AADC deficiency is a well-defined monogenic disorder amenable to gene therapy

- Mutation of the DDC gene causes deficiency of the AADC enzyme
- AADC makes dopamine via well-defined biochemical pathway

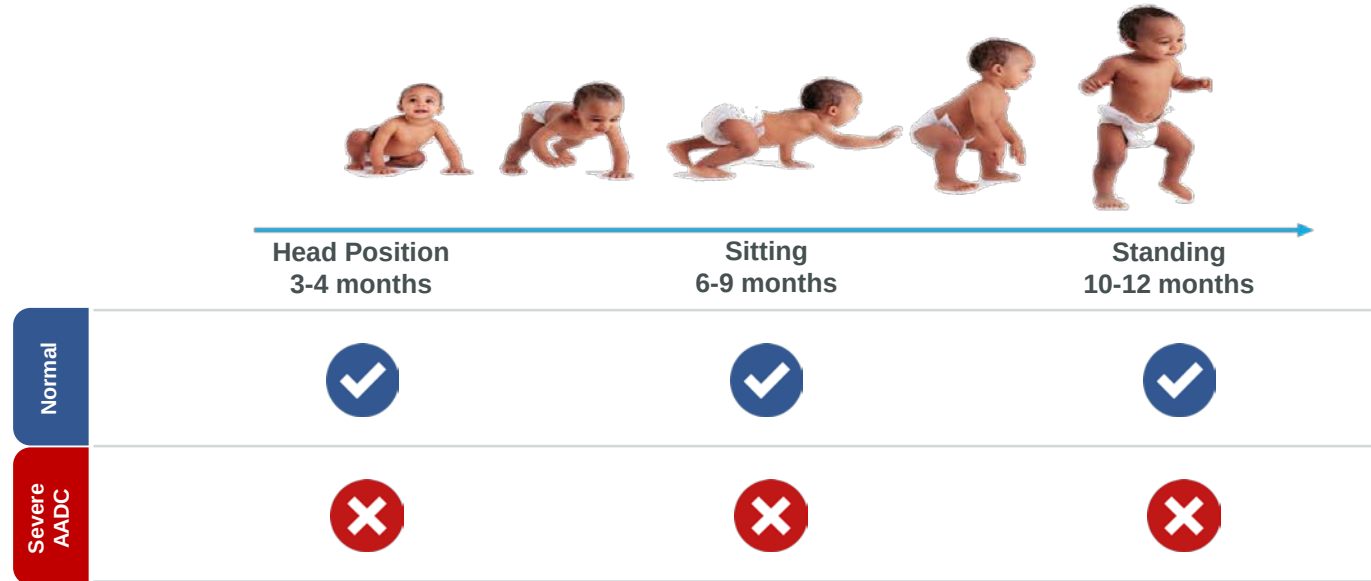


No treatment available for underlying cause

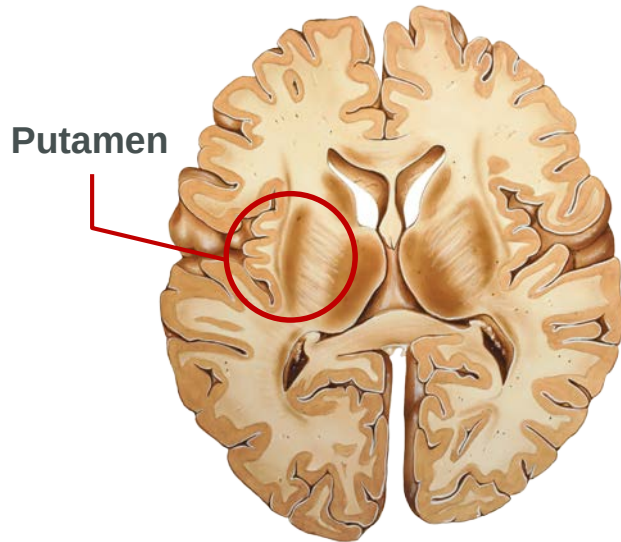
- ✓ Non-dividing cells
- ✓ Affected cells remain intact for long periods
- ✓ Limited, focal number of affected cells
- ✓ Amenable to direct delivery

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)



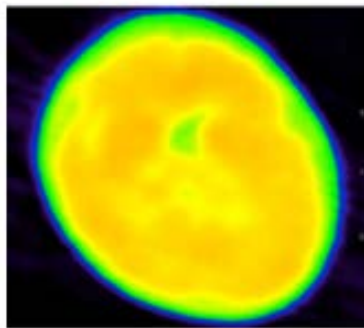
GT- AADC: Advanced CNS-delivered gene therapy program



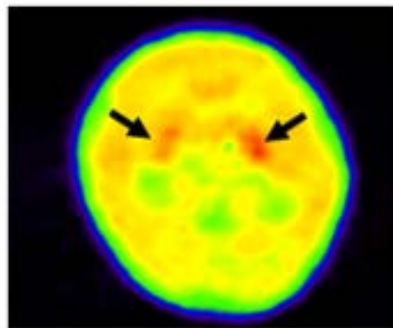
- **Target-delivered gene therapy**
 - Single administration of AAV2-hAADC
 - Low dose (1.8×10^{11} vg total)
 - Direct delivery using established stereotactic surgery
- **Clinically durable effect in patients**
 - First patients treated in 2010
 - Three clinical studies with safety data in 26 patients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

GT-AADC: Durable restoration of dopamine in patients

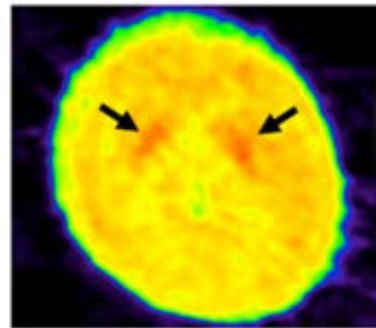
Pharmacodynamic PET imaging highlights significant de novo dopamine production



Pre-treated Patient



Patient A: at 1 year



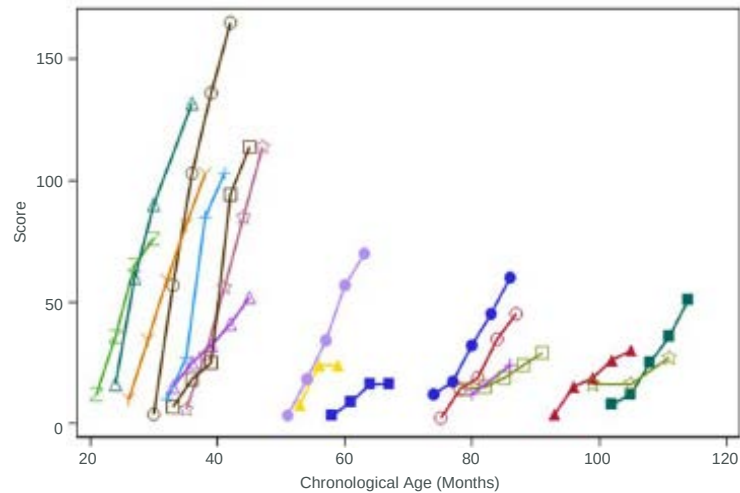
Patient B: at 5 years

Significant change
from baseline in
patients observed,
independent of
chronological age
(n=13)

GT-AADC: Significant & durable motor improvements

1-Year Results of Motor Development

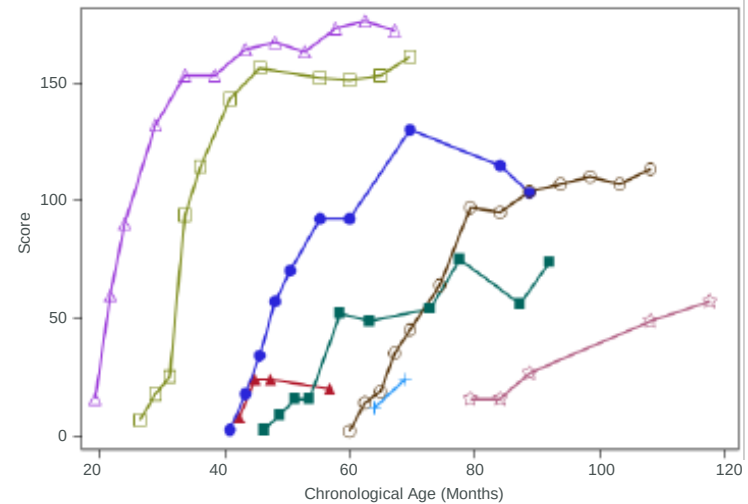
PDMS-2¹ through 12 months – Studies 1 & 2



1 Peabody Developmental Motor Scale

5-Year Results of Motor Development

PDMS-2¹ through 60 months - Study 1

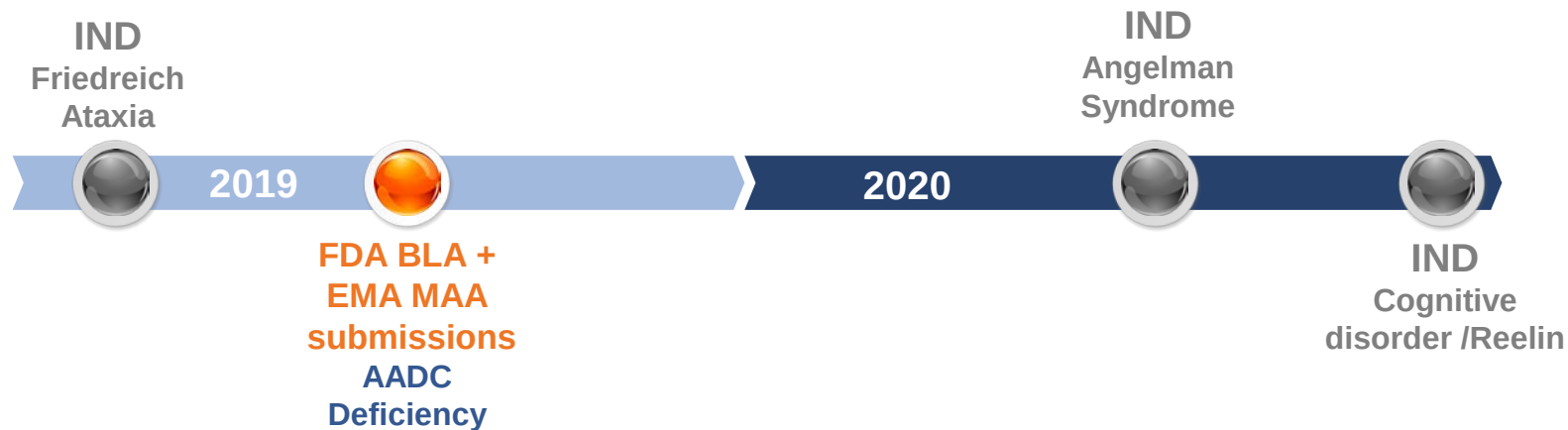


AADC deficiency patient Before and after treatment with GT-AADC

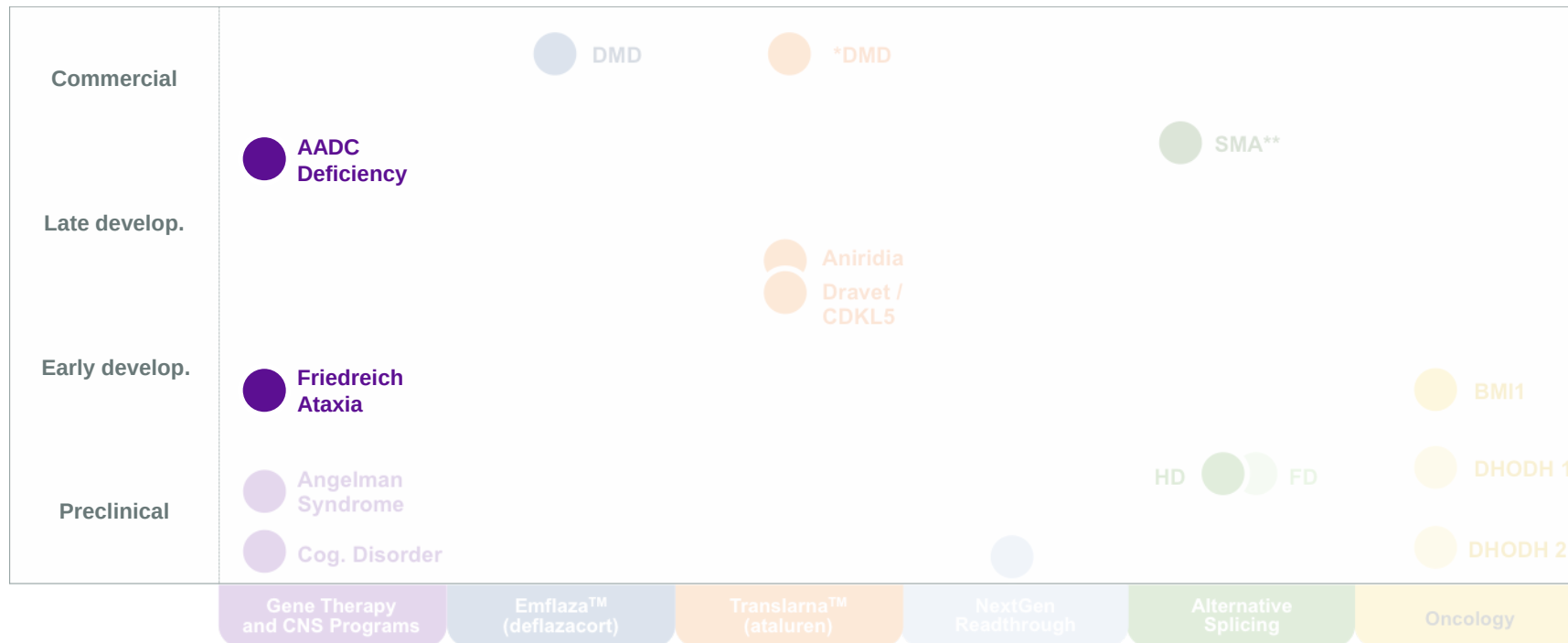


<https://www.ptcbio.com/en/aadc/>

Potential gene therapy development milestones



Focus on the most advanced gene therapy programs

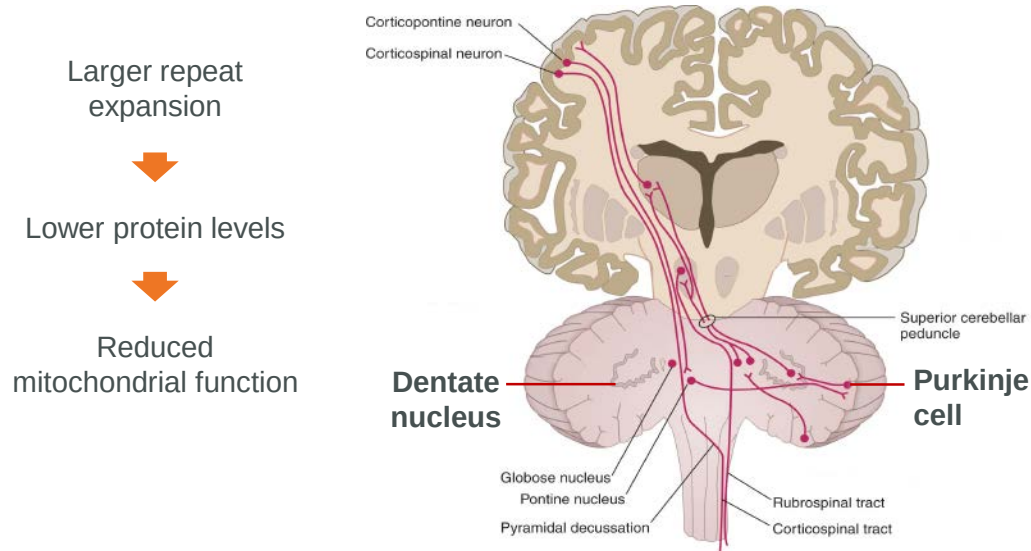


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Friedreich Ataxia (FA) is a severe neuromuscular disorder amenable to gene therapy

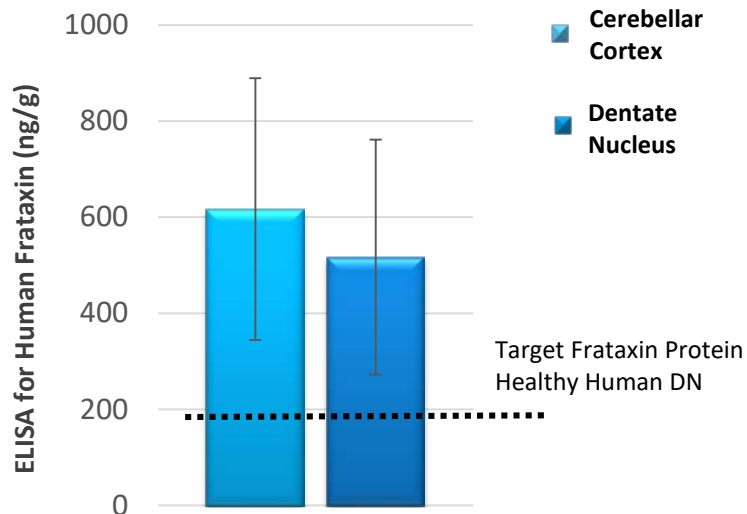
- Inherited, monogenic disease arising from triplet repeat expansion
- Mutation in frataxin gene limits protein production



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

Moving toward IND filing in 2019

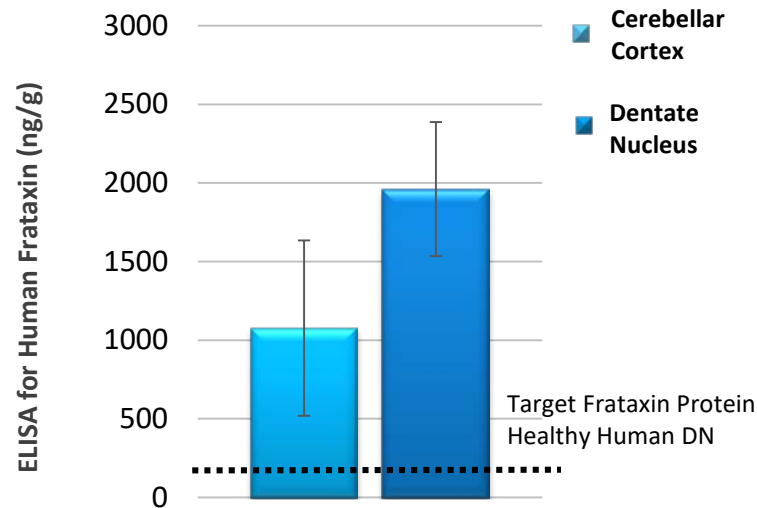
GT-FA Intracerebellar Dosing in Porcine Model*



Unilateral dose of 3.0×10^{12} vg total - Day 28 Mean (SEM)

*Human-specific detection

GT-FA Intracerebellar Dosing in NHP Model*



Bi-lateral Dose of 2.4×10^{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**

Transaction highlights



Consideration

- \$200M upfront (~ \$150M stock / \$50M cash)
- \$60M potential future development milestones in the next 2-years, including BLA acceptance for GT-AADC
- Up to \$535M related to regulatory approvals for AADC, FA and Angelman + one PRV
- Up to \$150M in tiered commercial milestones
- 2-6% of net sales for FA and Angelman



Financing

- Funding using cash on hand



Timing

- Subject to customary closing, expected to be finalized in Q3 2018

Significant value creation opportunities over next two years



Gene Therapy platform

- BLA submission and potential approval of first CNS-delivered gene therapy
- IND submission of FA gene therapy next year
- Preclinical development of AS and cognitive disorder programs advancing



Duchenne franchise

- Launch of pediatric label expansion for Translarna™
- Translarna Non-ambulatory label expansion submission in 2018
- Translarna dystrophin study for U.S. approval in 2019
- Continue to establish Emflaza® as standard of care
- Continue to expand Translarna business globally



Splicing platform

- SMA Firefish & Sunfish pivotal data for potential registration
- Huntington's and FD programs choose DC and enter clinical development



Niche Oncology platform

- Initiation of PTC299 and PTC596 clinical trials