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 15, 2020**

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):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Trading Symbol(s)**	**Name of each exchange on which registered**
Common Stock, $0.001 par value per share	PTCT	Nasdaq Global Select Market

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

Emerging growth company ☐

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

Item 7.01. Regulation FD Disclosure.

On Wednesday, January 15, 2020 at 8:30 am PT at the 38th Annual J.P. Morgan Healthcare Conference (the "Conference"), PTC Therapeutics, Inc. (the "Company") presented pursuant to advance notice its 2020 strategic priorities, preliminary 2019 financial results, and 2020 financial guidance. The Company's presentation has been made available on the Events and Presentations page under the Investors section of the Company's website. A copy of the slide deck, which the Company presented to investors at the Conference, is attached as Exhibit 99.1.

The information in this Item 7.01 of this Current Report on Form 8-K, including Exhibit 99.1, shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing.

Item 8.01 Other Events

At the Conference, the Company announced certain preliminary (unaudited) financial information for its fiscal year ending December 31, 2019, including that the Company expects to report (i) total unaudited revenue of approximately $306 million, including net product revenue for the Duchenne muscular dystrophy franchise of approximately $291 million and a $15 million milestone payment from F. Hoffmann La Roche Ltd and Hoffmann La Roche Inc. for the risdiplam New Drug Application ("NDA") acceptance; (ii) net product revenue for Translarna™ of approximately $190 million, (iii) net product revenue for Emflaza® of approximately $101 million and (iv) ending cash and cash equivalents of approximately $686 million. Final results are subject to completion of the Company's year-end audit and are subject to revision.

The Company also announced certain corporate updates, including that (i) the Company submitted a Marketing Authorization Application for the potential approval of a gene therapy treatment, PTC-AADC, for AADC deficiency with the European Medicines Agency and the Company expects the Committee for Medicinal Products for Human Use opinion in the second half of 2020, (ii) in a recent interaction with the U.S. Food and Drug Administration (the "FDA") there was a request for additional information concerning the use of the commercial delivery system for PTC-AADC in young patients and the Company anticipates the submission of a Biologics License Application to the FDA in the second quarter of 2020, (iii) the Prescription Drug User Fee Act date for a decision by the FDA on the risdiplam NDA is May 24, 2020 and (iv) in 2020, the Company plans to initiate three clinical trials in its newly acquired redox platform including potential registrational trials in mitochondrial epilepsy and Friedreich ataxia with PTC743 and a Phase 1 trial with PTC857 targeting GBA Parkinson's disease.

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 2019, expectations with respect to future financial results and statements regarding: expectations with respect to the Company's gene therapy platform, including any potential regulatory submissions and manufacturing capabilities; and advancement of the Company's joint collaboration program in spinal muscular atrophy ("SMA"), including any potential regulatory submissions, commercialization or royalty or milestone payments. 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 2019 financial information, which is subject to completion of the Company's year-end audit; expectations with respect to the Company's gene therapy platform, including any potential regulatory submissions and potential approvals, manufacturing capabilities and the potential financial impact and benefits of its leased biologics facility and the potential achievement of development, regulatory and sales milestones and contingent payments that the Company may be obligated to make; 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 and potential commercialization with regards to risdiplam; 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, 2018, Quarterly Reports on Form 10-Q for the periods ended March 31, 2019, June 30, 2019 and September 30, 2019 as well as any updates to these risk factors filed from time to time in the Company's other filings with the Securities and Exchange Commission. You are urged to carefully consider all such factors. The forward-looking statements contained herein and the exhibit 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	Corporate Presentation - 38th Annual J.P. Morgan Healthcare Conference
104	The cover page from this Current Report on Form 8-K, formatted in Inline XBRL

Signature

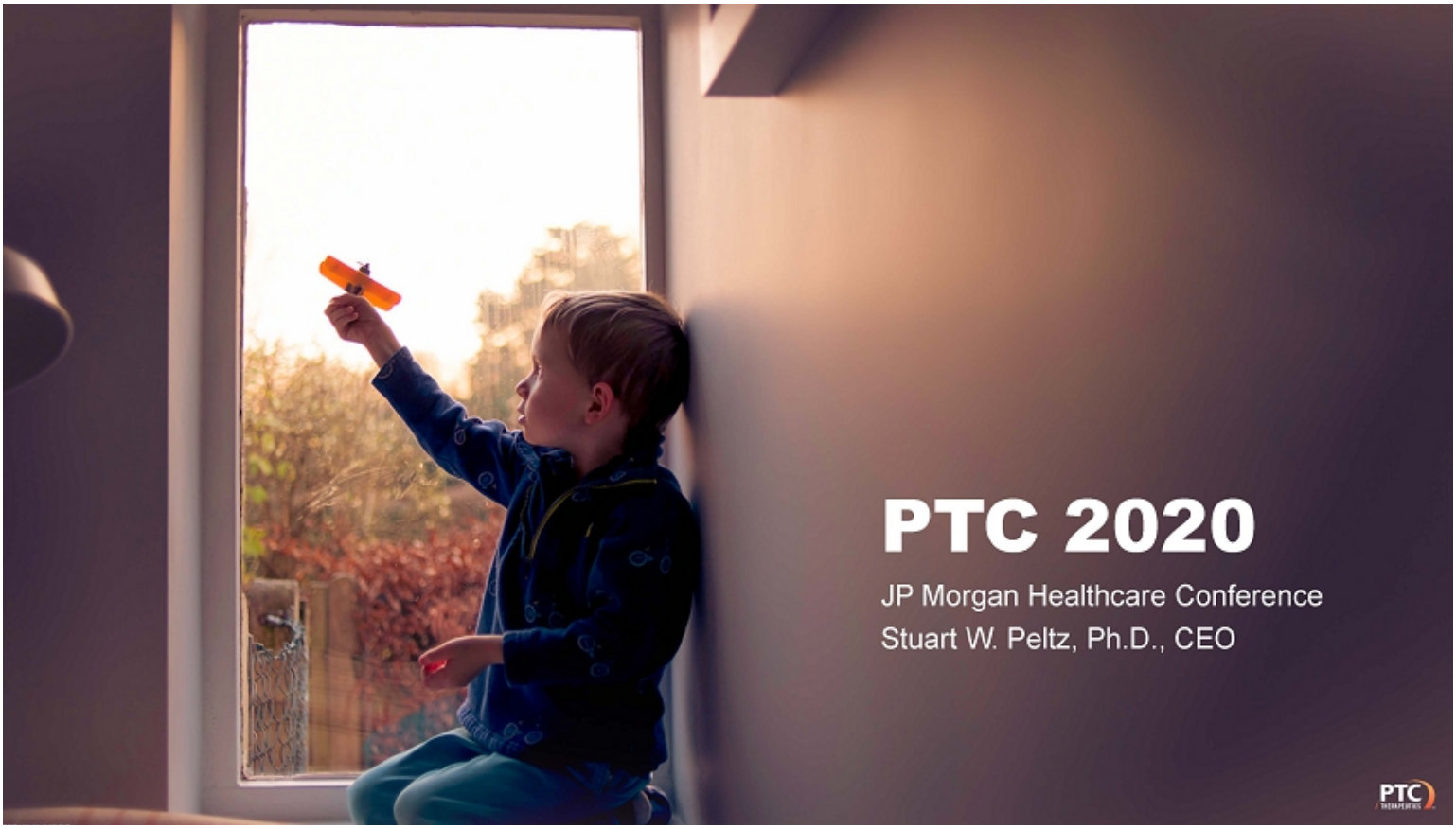
Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this Report to be signed on its behalf by the undersigned hereunto duly authorized.

PTC Therapeutics, Inc.

Date: January 17, 2020

By: /s/ Emily Hill
Name: Emily Hill
Title: Chief Financial Officer

Exhibit 99.1


PTC 2020
JP Morgan Healthcare Conference
Stuart W. Peltz, Ph.D., CEO
PTC

Forward looking statement

This presentation contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this presentation, other than statements of historic fact, are forward-looking statements, including statements related to preliminary unaudited 2019 financial information with respect to 2019 net product revenue of Translarna for the treatment of nmDMD and EMFLAZA for the treatment of Duchenne muscular dystrophy, statements with respect to 2020 net product revenue guidance, statements with respect to the 2020 operating expenditure 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 and manufacturing capabilities; advancement of PTC's joint collaboration program in SMA, including any potential regulatory submissions, commercialization or royalty or milestone payments; PTC's expectations with respect to the licensing, regulatory submissions and potential commercialization of Tegsedi and Waylivra; expansion of commercialization of Translarna and Emflaza and related 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 PTC's products or product candidates that PTC commercializes or may commercialize in the future; expectations with respect to PTC's gene therapy platform, including any potential regulatory submissions and potential approvals, manufacturing capabilities and the potential financial impact and benefits of its leased biologics facility and the potential achievement of development, regulatory and sales milestones and contingent payments that PTC may be obligated to make; 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 and potential commercialization with regards to risdiplam; PTC's ability to complete a dystrophin study necessary to support a re-submission of its Translarna NDA for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) to the FDA, and PTC's ability to perform any necessary 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 commercialization of Tegsedi and Waylivra; significant 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 lease agreement for its leased biologics facility; PTC's ability to satisfy its obligations under the terms of the senior secured term loan facility with MidCap Financial; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; and the factors discussed in the "Risk Factors" section of PTC's most recent Quarterly Report on Form 10-Q and 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. 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 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.

A global, commercial,
diversified, biopharmaceutical company focused on
innovative therapies for rare genetic disorders

PTC THERAPEUTICS

Global commercial capabilities & infrastructure

Strong financial performance supports future growth

~$306M
2019 Unaudited Net Revenue

$291M
2019 DMD Franchise Net Product Unaudited Net Revenue

$320 – $340M
2020 DMD Franchise Net Product Revenue Guidance

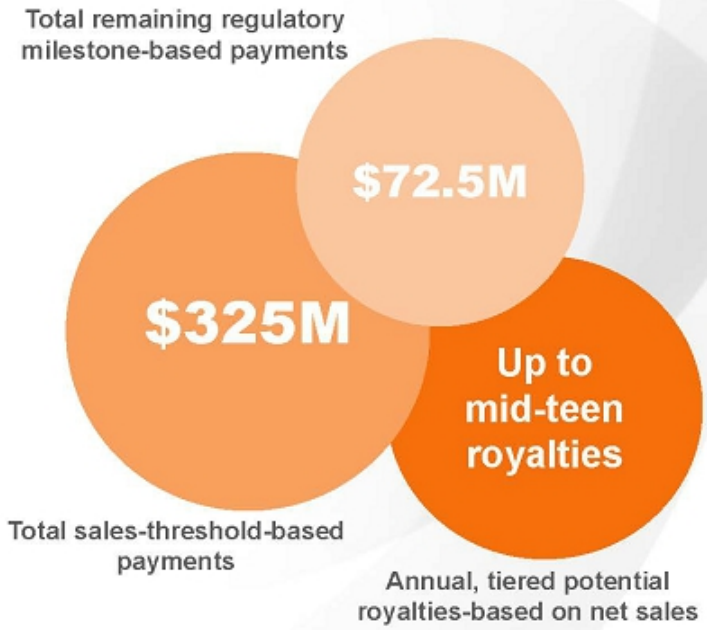
New product launches and approvals could also contribute to 2020 revenue (e.g.,Tegsedi, commercial Waylivra EAP, PTC-AADC and risdiplam)

$545 – $575M
2020 Opex Guidance

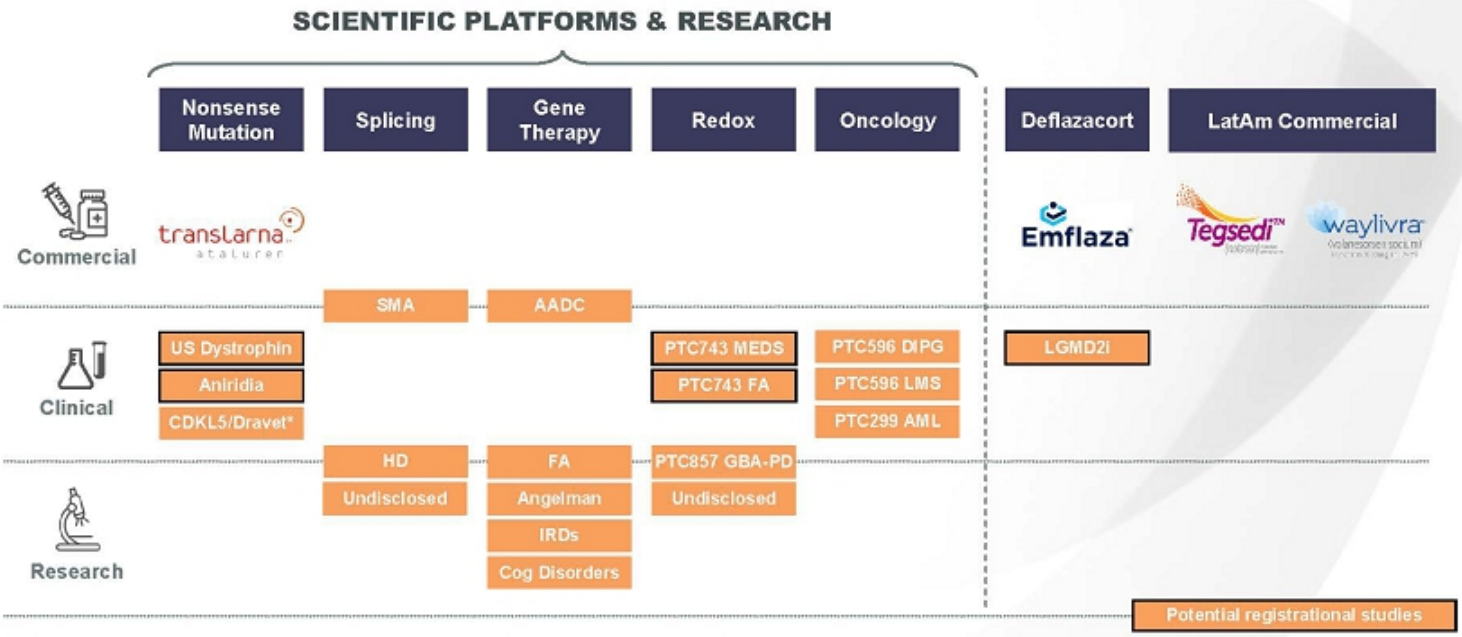
Significant success-based revenue through remaining risdiplam milestones and royalties

Potential 2020 risdiplam milestone payments to PTC

Milestone	Payment
MAA Filing with EMA	$ 15,000,000
NDA Filing in Japan	$ 7,500,000
First US Commercial Sale	$ 20,000,000
Total	**$ 42,500,000**

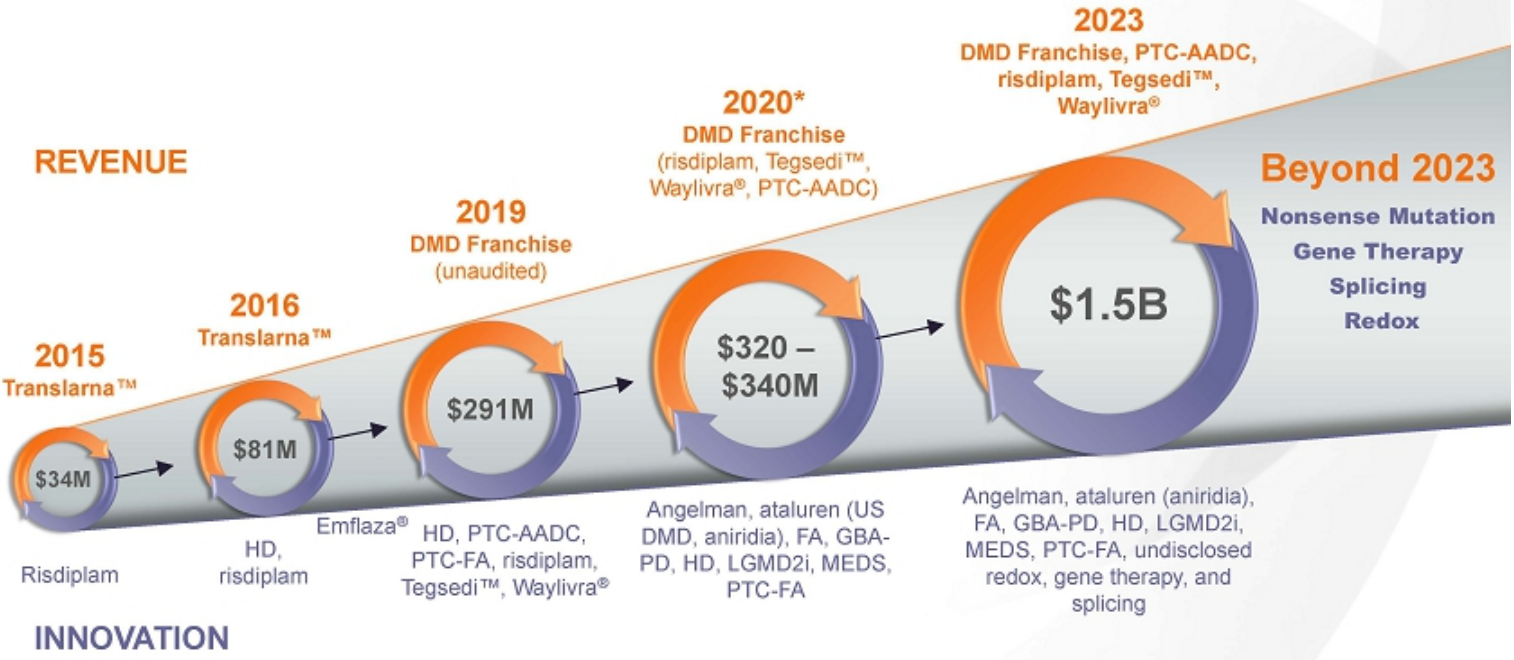


Multiplatform approach builds diversified pipeline

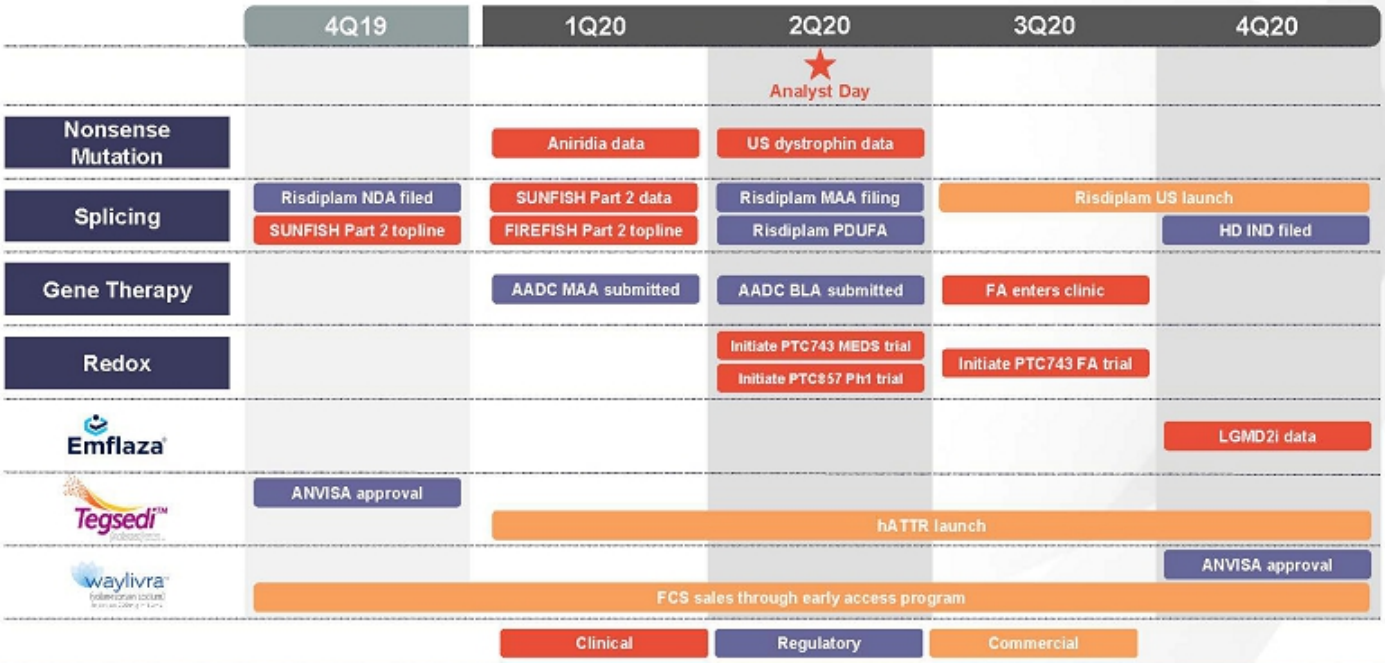


* Investigator-initiated study with NYU

Sustainable innovation drives continuous value creation



Multiple potential value driving events in 2020



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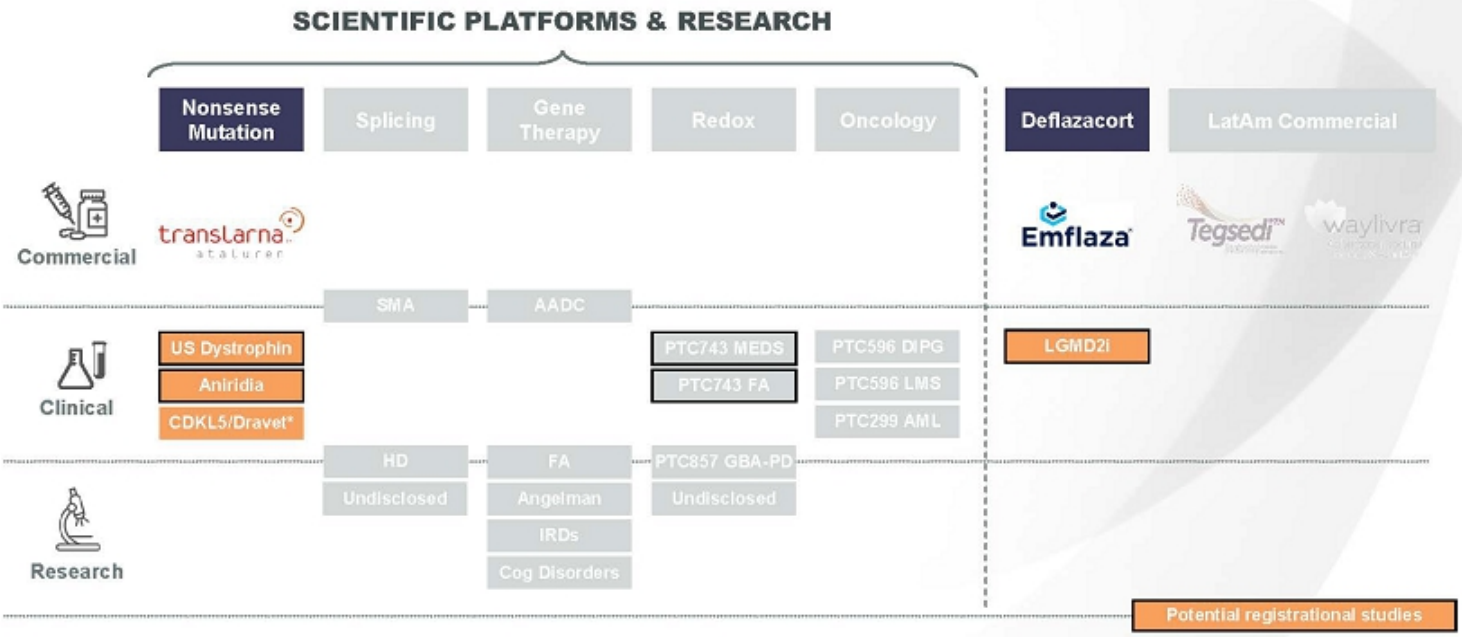


* Investigator-initiated study with NYU

Translarna™ demonstrates long-term benefit in DMD patients

~90%
EU5 nmDMD patients treated with Translarna

~85%
Compliance

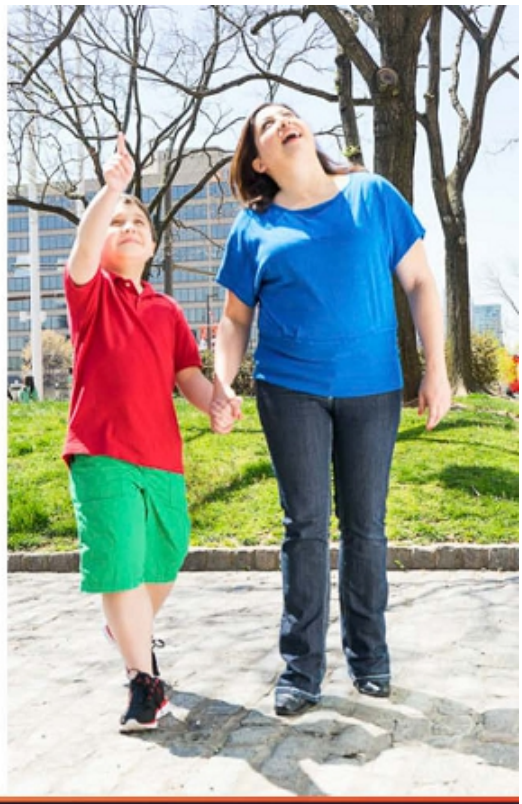
STRIDE is a real-world, long-term registry of patients receiving Translarna

Translarna treatment slowed disease progression in nmDMD compared to matched natural history patients

3.5 years
Delay in loss of ambulation

3 years
Extension in ability to stand from supine in less than 5 seconds

2.2%
Translarna-treated pts had an FVC<50% compared to 32.1%



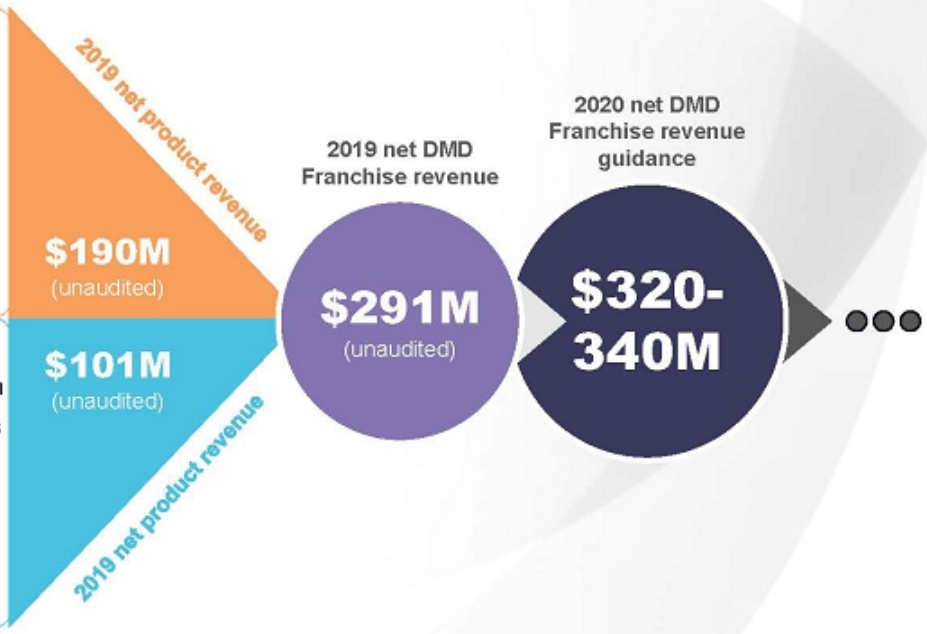
Growth opportunities in DMD franchise

Translarna

- Increased penetration in existing territories
- Geographic expansion into new territories
- Increased awareness and earlier diagnosis
- Potential US NDA submission in 2020

Emflaza

- Growth in 2-5 year olds from label expansion
- Optimize dosing in both new and existing pts
- Publications showing the benefit of Emflaza over prednisone
  - Reduced payer restrictions
  - Benefit of switching



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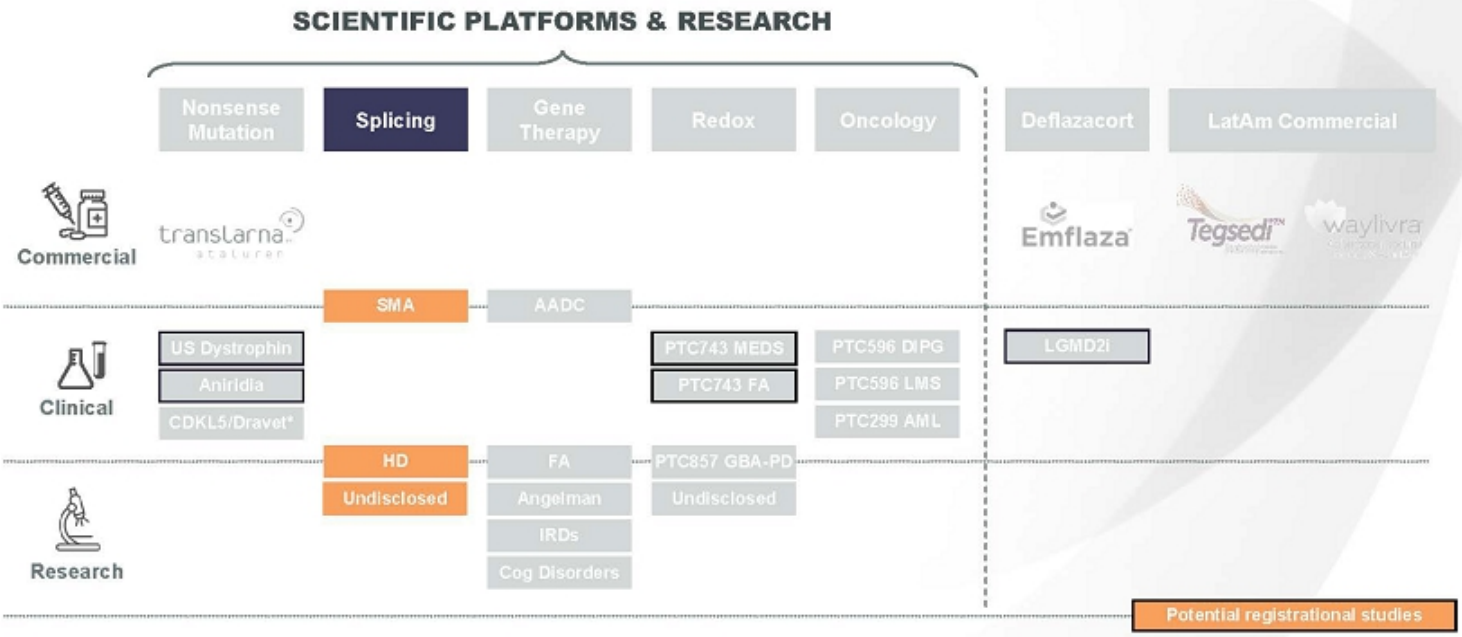
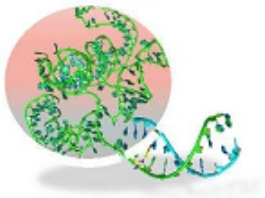


* Investigator-initiated study with NYU

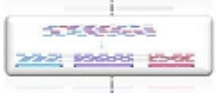
Splicing is highly selective with broad applicability



Exploiting U1-noncanonical exons

- Alternative splicing
- Exon skipping
- Stop exon inclusion

Isoform plex



HTSpliceseq

Transcriptome mRNA isoform detection platforms

2019

- **Risdiplam** *SMN2 exon 7 inclusion*
- **Familial dysautonomia** *ELP1 exon 20 inclusion*
- **Huntington disease** *Stop exon inclusion*

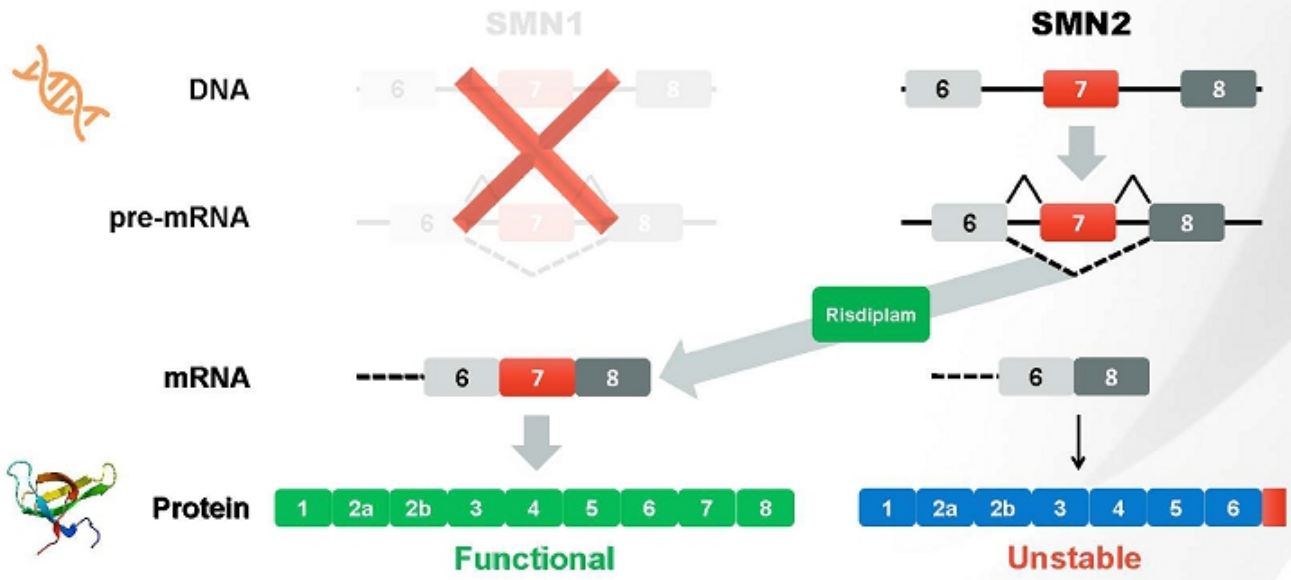
2020 and beyond

Proprietary RNA-targeted small molecule library

>5 additional undisclosed targets

PTC

Risdiplam: An oral small molecule selective SMA splicing modifier



Risdiplam – Most competitive commercial profile across broadest population

FIREFISH – Type 1 SMA

82%
on high dose had a CHOP-INTEND ≥ 40

48%
of all infants were able to maintain head control

33%
of all infants were able to sit independently

12%
on high dose achieved bouncing, the first HINE-2 walking milestone

FIREFISH Part 2 topline data expected in Q1 2020

SUNFISH – Type 2 and 3 SMA

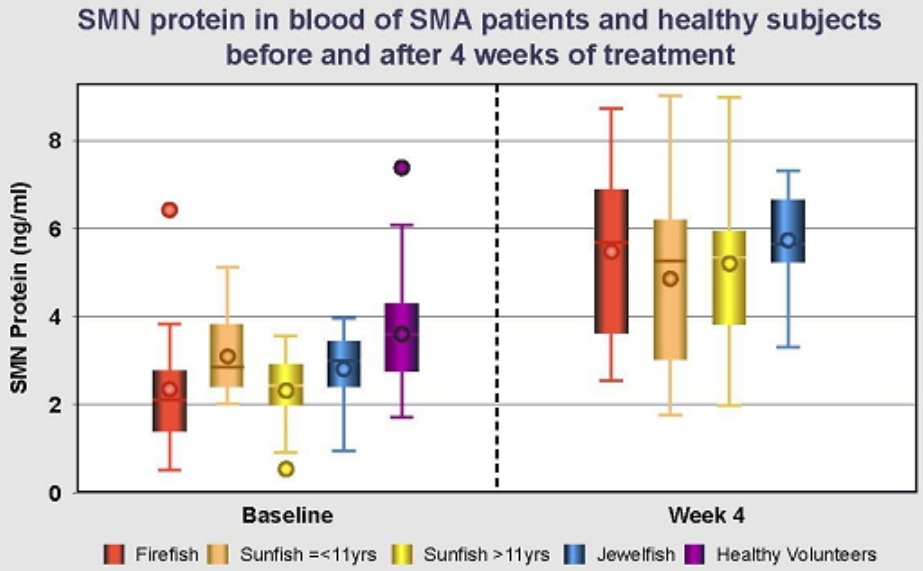
58%
achieved improvements of ≥ 3 in MFM32 scores

Part 2 study demonstrated statistically significant improvement for patients

SUNFISH Part 2 data presentation expected in Q1 2020

- Studied in Type 1,2,3 patients from newborns to 60 years of age
- No treatment-related safety findings leading to withdrawal
- Systemic and central mode of action
- Demonstrated increases in full-length SMN2 mRNA
- Ability to measure full-length mRNA and protein in blood
- **PDUFA: May 24, 2020**

Risdiplam – Most competitive commercial profile across broadest population



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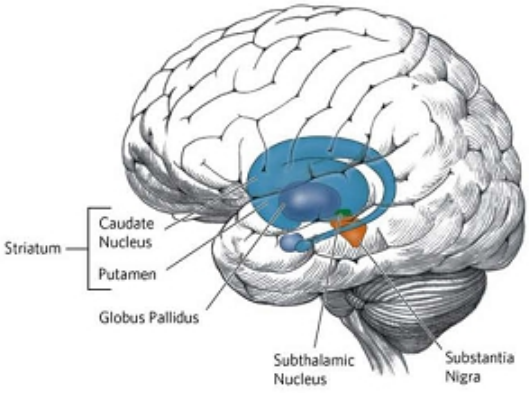
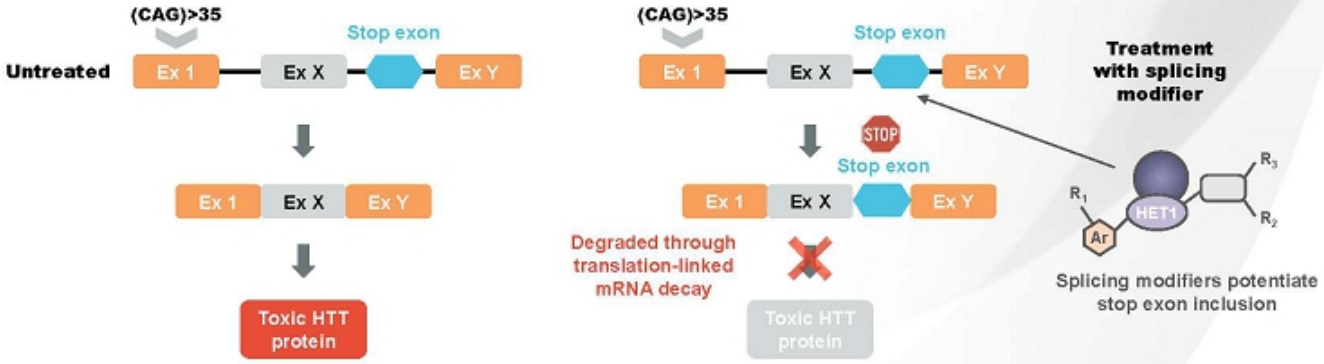
Toxic HTT protein aggregation causes extensive neuronal cell death



Goh et al. *Aus Psychiatry*. 2018

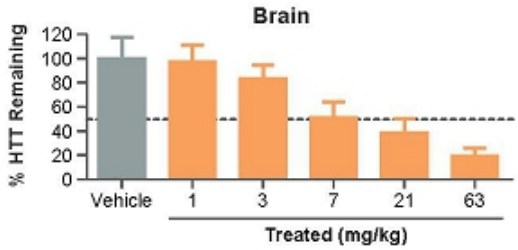
Splicing modifiers reduce HTT protein in Huntington disease

HD is a neurodegenerative disease caused by a toxic gain-of-function triplet repeat (CAG) expansion in the huntingtin gene



HD splicing small molecules have broad tissue distribution

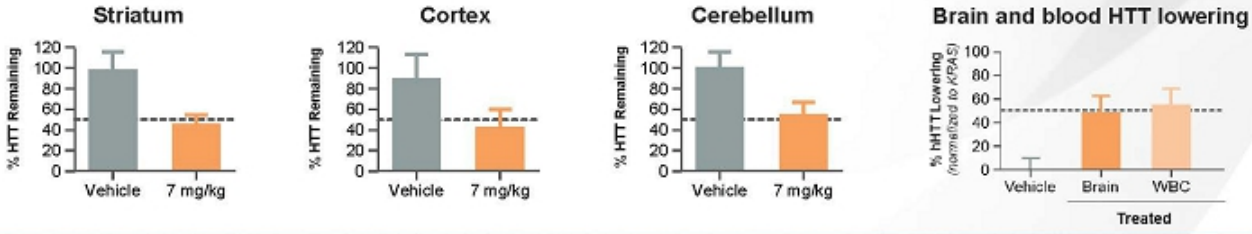
Dose dependent HTT lowering in the brain in BACHD mice



HD IND expected late 2020

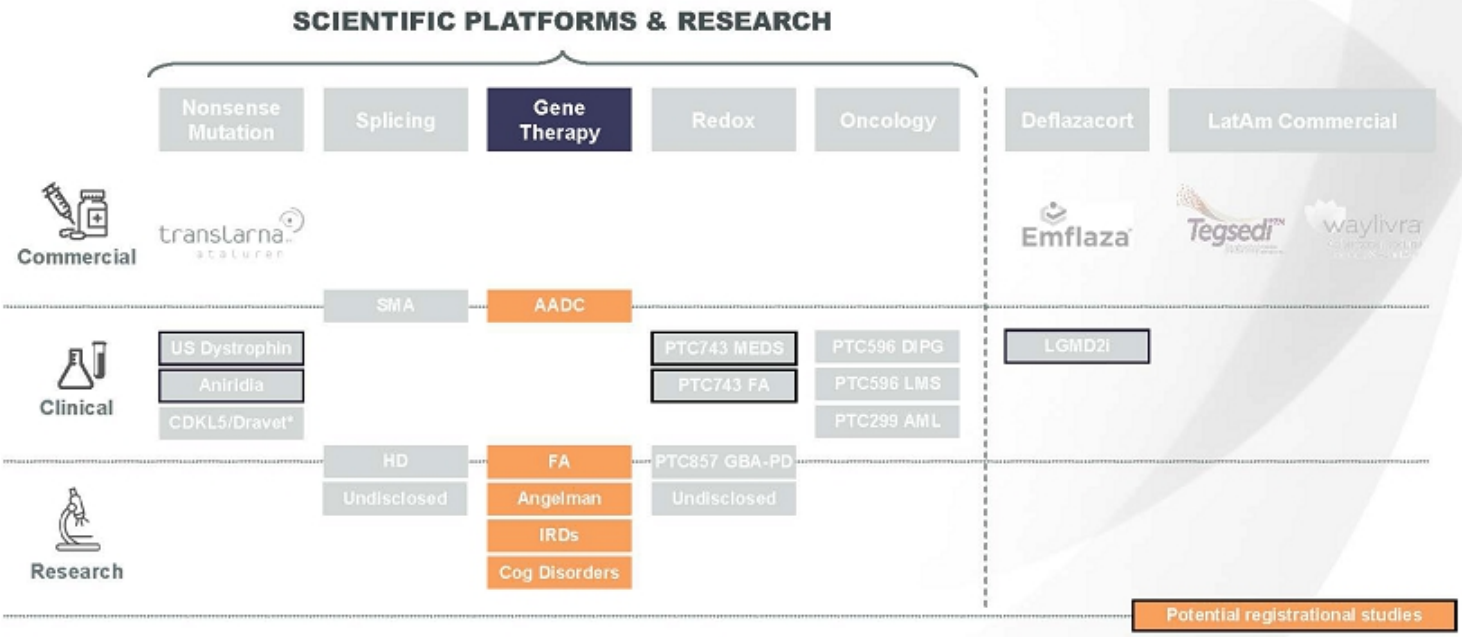
- Oral, crosses BBB
- Titratable
- Development candidate selected
- Ability to measure mRNA and protein in blood in healthy volunteers

Uniform HTT lowering with ~1:1 brain and blood concentrations*



*Data on file from multiple studies

Multiplatform approach builds diversified pipeline



* Investigator-initiated study with NYU

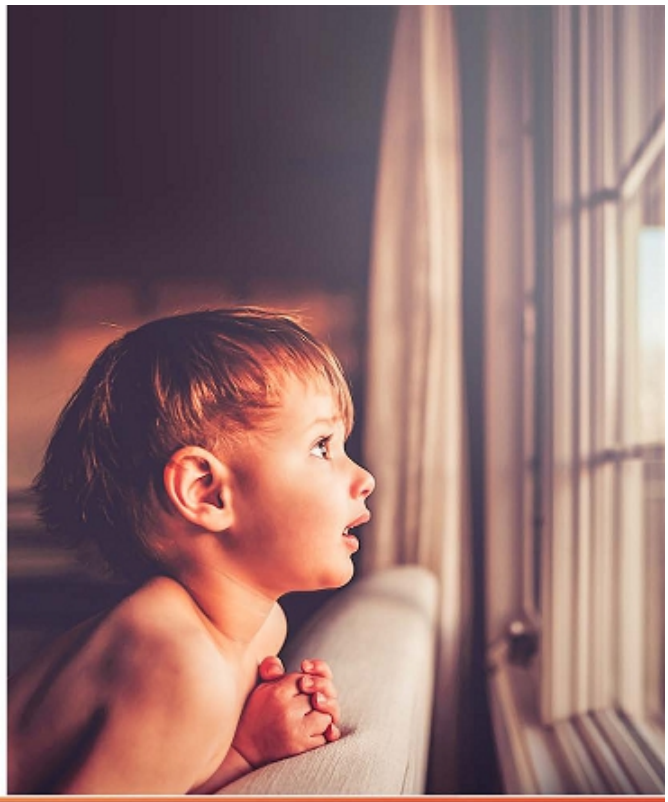
Treating rare monogenic disorders with targeted gene therapy

Potential advantages of targeted therapy

- Local administration lowers systemic immunogenicity and exposure
- Low turnover cells may lead to improved durability
- Micro-dosing lowers manufacturing and patient burden

Pipeline

- PTC-AADC MAA submitted
- PTC-AADC BLA now expected in 2Q20
- PTC-FA expected to enter clinic in 3Q20
- Angelman syndrome IND anticipated 1H 2021
- >5 nonclinical development candidates



Internal gene therapy manufacturing capabilities

- In-house manufacturing begins in 2020
- 15-year lease on ~185,000 sq. ft. which includes a state-of-the-art biologics production facility with supporting research and operations buildings in NJ
- Highly qualified staff in biologics manufacturing joining PTC
- Facility to support gene therapy production & continued development of investigational medicines

AADC deficiency – Rare disorder with significant unmet need

	Normal	AADC
Head Position Up *3-4 months*	✓	⊘
Sitting *6-9 months*	✓	⊘
Standing *10-12 months*	✓	⊘

- 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 - 8 yrs)
- Patients identified in Asia, US, Europe and LatAm
- > 50 disease causing variants described in AADC deficiency

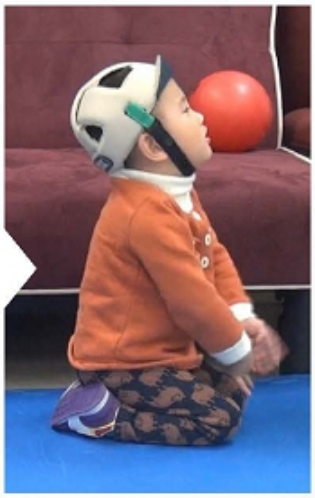
Wassenberg T et al. *Orphanet J Rare Dis*. 2017;12(1):12

PTC-AADC patients make significant and sustainable progress

Untreated

Age 2

Post-Treatment



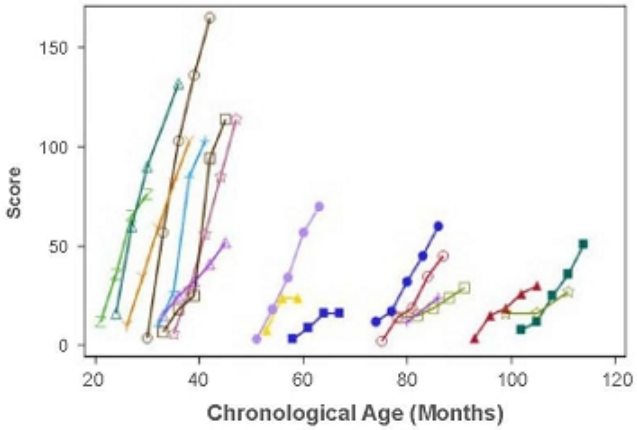
Age 3

Age 4.5

PTC-AADC demonstrates significant and durable motor improvements across patient population

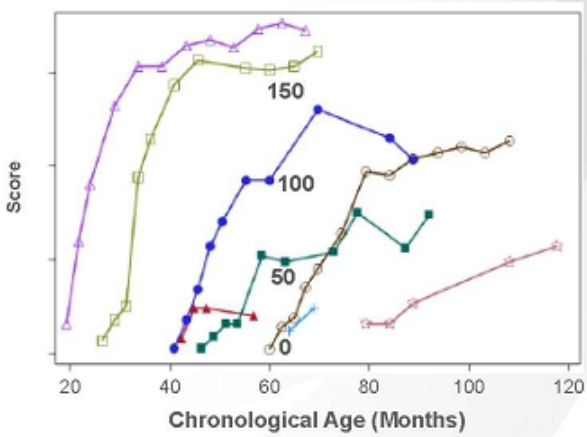
1-Year Results of Motor Development

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



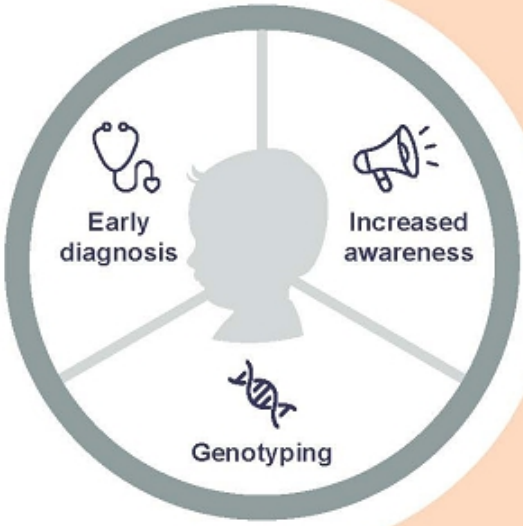
5-Year Results of Motor Development

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



[1] Peabody Developmental Motor Scale
Chien et al. Presented at Child Neurology Society October 23-26 2019, Charlotte, NC

Expertise in patient identification



AADC US efforts:
No-cost testing via whole blood
3-OMD newborn screening test; reflex to AADC enzyme activity and DDC gene sequencing

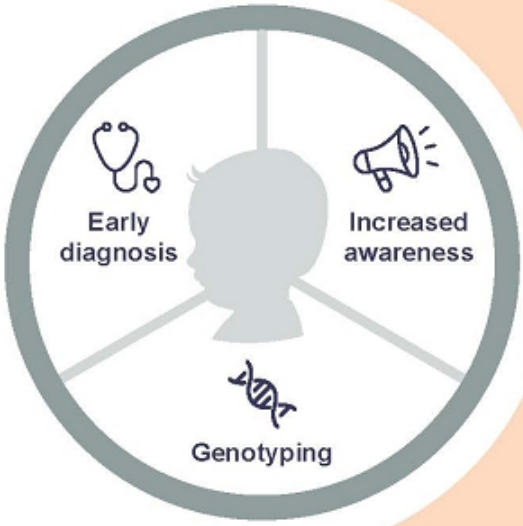
AADC Ex-US efforts:
No-cost testing via dried blood spot cards
3-OMD screening; reflex to DDC gene sequencing

200 AADC patients identified

Prevalence Screening Study in Patients with Cerebral Palsy (CP)
Protocol driven study to identify prevalence of AADC in CP patients of unknown etiology

Newborn Screening
More than 1 million newborns expected to be screened by the end of 2021 in the US

Expertise in patient identification



AADC US efforts:
No-cost testing via whole blood
3-OMD newborn screening test; reflex to AADC enzyme activity and DDC gene sequencing

AADC Ex-US efforts:
No-cost testing via dried blood spot cards
3-OMD screening; reflex to DDC gene sequencing

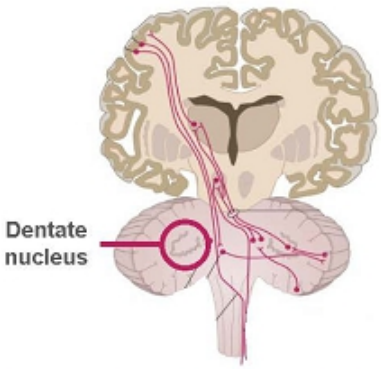
300+ AADC patients by launch

Prevalence Screening Study in Patients with Cerebral Palsy (CP)
Protocol driven study to identify prevalence of AADC in CP patients of unknown etiology

Newborn Screening
More than 1 million newborns expected to be screened by the end of 2021 in the US

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

Larger repeat expansion → Lower protein levels



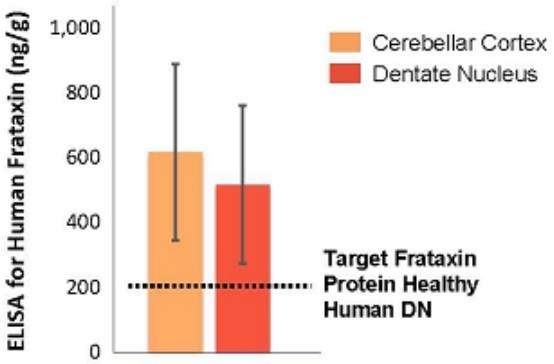
Reduced mitochondrial function

- 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

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

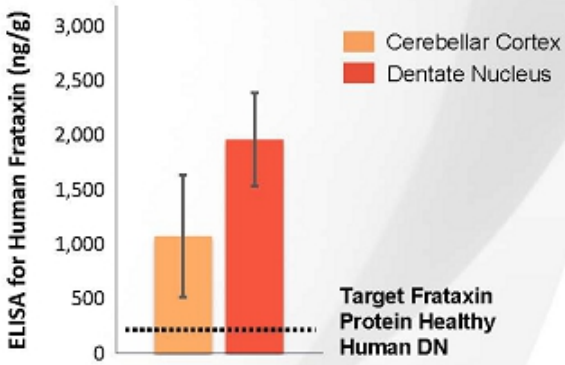
Frataxin expression seen in large animal models

PTC-FA Intracerebellar Dosing in Porcine Model*



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

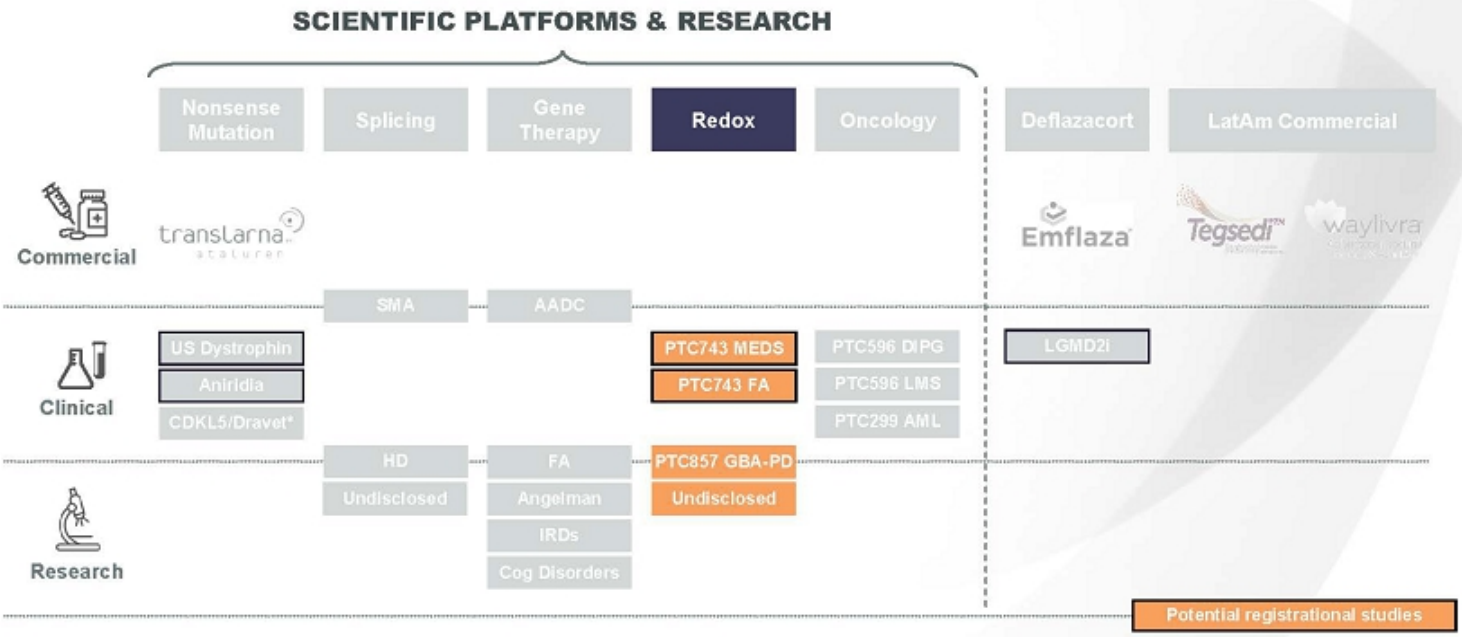
PTC-FA Intracerebellar Dosing in NHP Model*



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

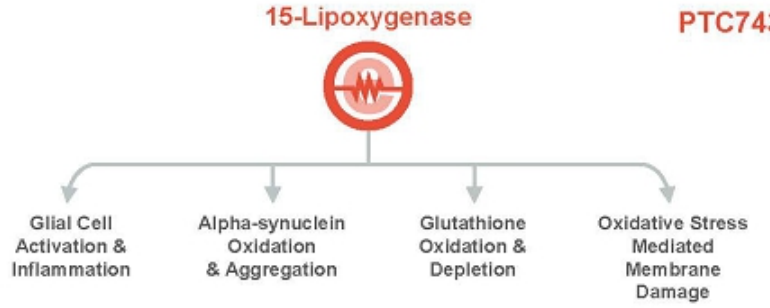
PTC-FA to enter clinic 3Q20

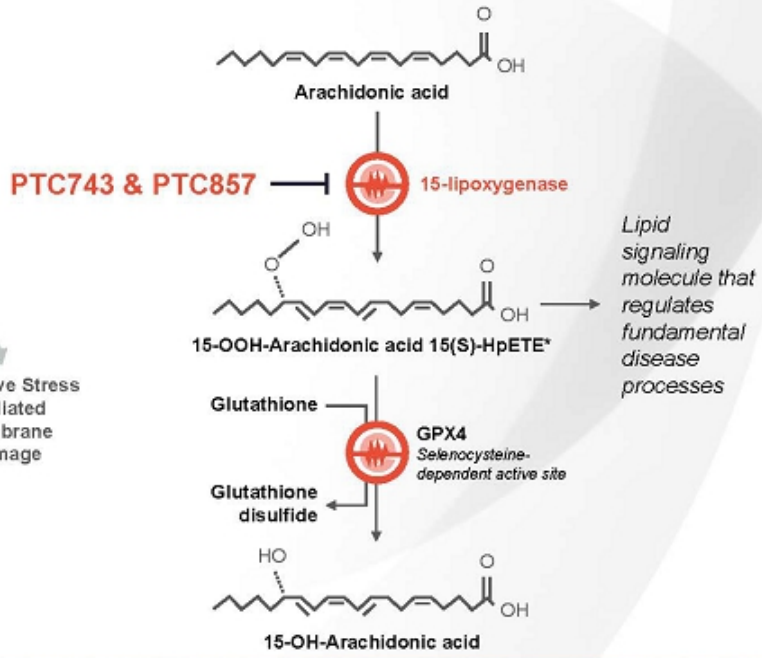
Multiplatform approach builds diversified pipeline



* Investigator-initiated study with NYU

Redox platform is a novel approach focusing on inflammation and oxidative stress





Initiating three redox clinical trials in 2020

PTC743

Mitochondrial Epilepsy Trial

Trial Starting 2Q20

- Proof-of-concept in dozens of patients
- Clinical trials demonstrated reduction in hospitalizations and mortality risk in MEDS patients
- To enroll patients with 4 most common sub-types of MEDS

5-6K
patients in the US and EU

PTC743

Friedreich Ataxia Trial

Trial Starting 3Q20

- Mechanism linked to FA pathology
- >60 subjects treated; Improvement in FARS compared to natural history
- Potentially complementary with FA gene therapy

25K
patients WW

PTC857

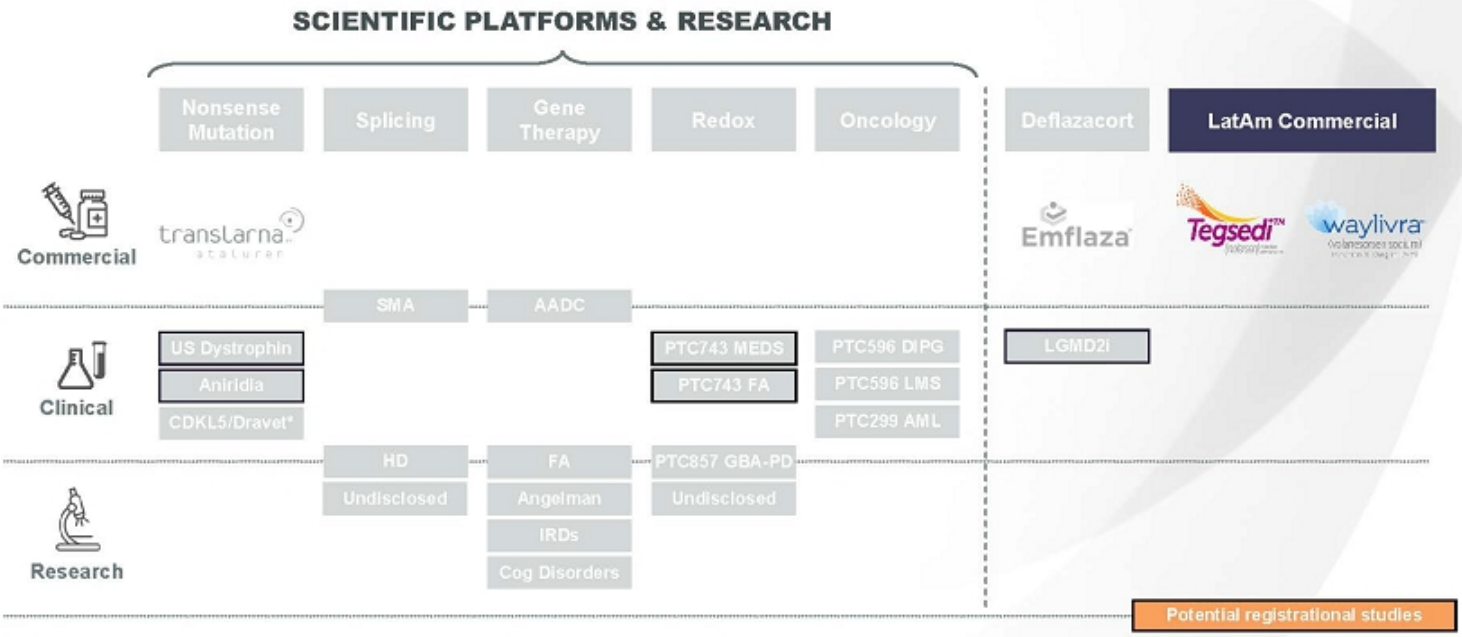
Phase 1 Trial

Trial Starting 2Q20

- Targeting GBA Parkinson's disease as first indication
- Inhibits alpha-synuclein oxidation and aggregate intensity in preclinical trials
- Protects dopamine-related motor function in MPTP mouse

~50 – 90K
patients in the US

Multiplatform approach builds diversified pipeline



* Investigator-initiated study with NYU

Leveraging our existing LatAm infrastructure to commercialize Tegsedi & Waylivra

Best fit for Latin American hATTR market

ANVISA approval granted in 2019

Only approved hATTR indication to include improvements in QoL

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

Waylivra to utilize our patient support in Latin America

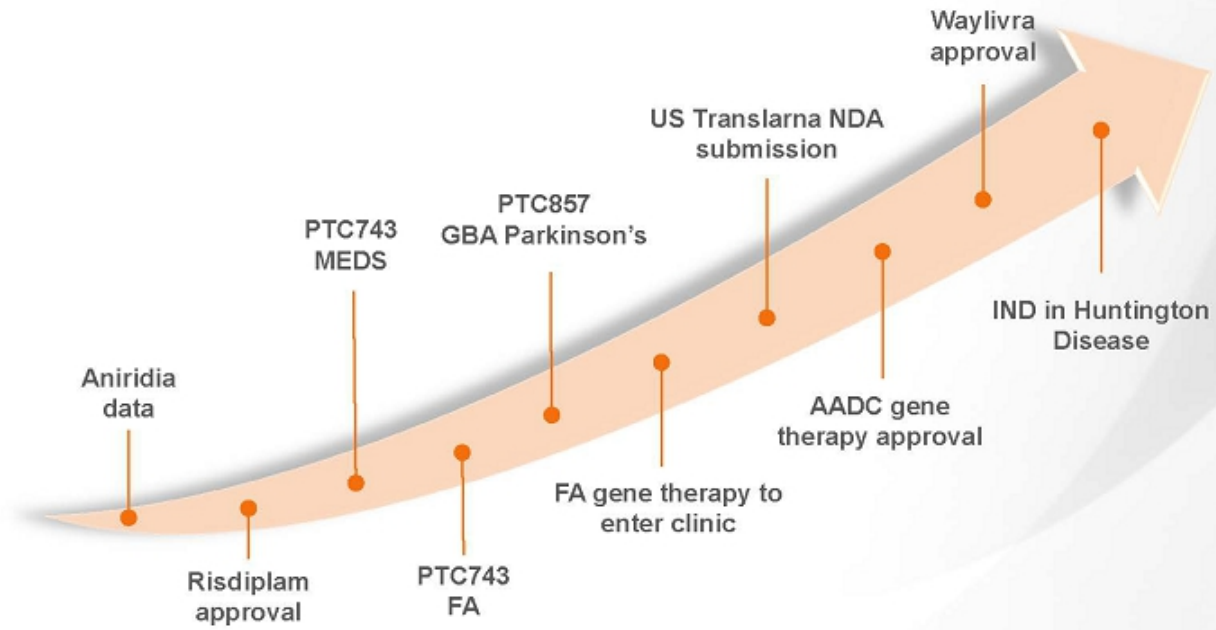
Potential ANVISA approval expected in 4Q20

Potential first FCS treatment

Received EU conditional marketing approval

35 FCS = familial chylomicronemia-syndrome

Potential 2020 milestones to generate value



A global, commercial,
diversified, biopharmaceutical company focused on
innovative therapies for rare genetic disorders

PTC THERAPEUTICS