PTC Therapeutics

Jefferies Virtual Healthcare Conference

Stuart W. Peltz, Ph.D., CEO

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 with respect to future revenue and statements regarding: the future expectations, plans and prospects for PTC, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses and other matters; 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 commercialization of its products and product candidates; 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 manufacturing 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 PTC's products and 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 manufacturing 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.

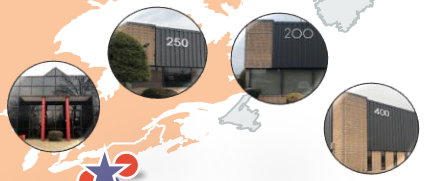
As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including 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



Offices in
20 countries

Footprint in
>50 countries

850+
employees

Strong Commercial Performance & Capital Position

$291M
Total 2019 DMD Franchise Revenue

$68.2M
Total 1Q20 Net Product Revenue

+28%
1Q20 YoY Total Net Product Revenue Growth

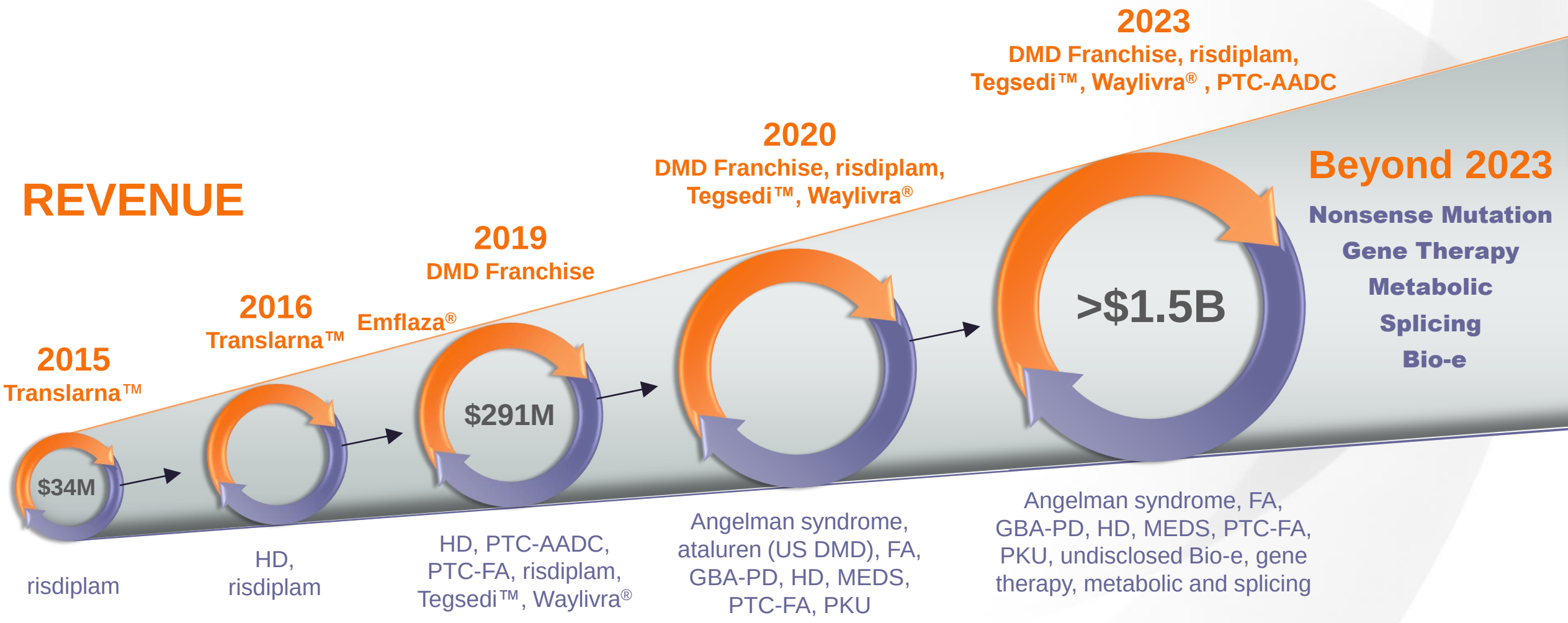
$596M
1Q20 Ending Cash Position

Multiplatform approach builds diversified pipeline

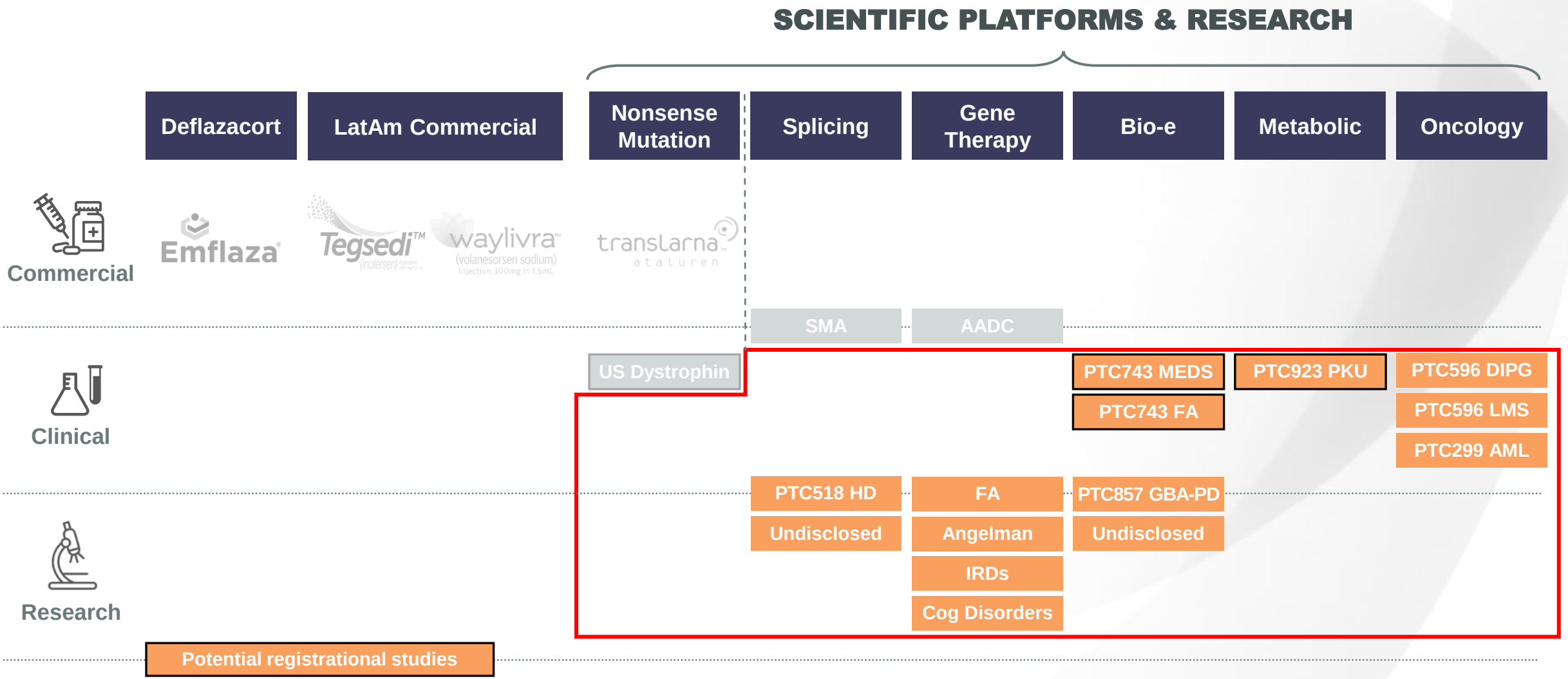
SCIENTIFIC PLATFORMS & RESEARCH

	Deflazacort	LatAm Commercial	Nonsense Mutation	Splicing	Gene Therapy	Bio-e	Metabolic	Oncology
Commercial	Emflaza®	Tegsedi™ (inotersen) injection 284 mg/1.5 mL	waylivra™ (volanesorsen sodium) Injection 300mg in 1.5mL					
			translarna™ ataluren					
Clinical			US Dystrophin	SMA	AADC	PTC743 MEDS	PTC923 PKU	PTC596 DIPG
						PTC743 FA		PTC596 LMS
								PTC299 AML
Research				PTC518 HD	FA	PTC857 GBA-PD		
				Undisclosed	Angelman	Undisclosed		
					IRDs			
					Cog Disorders			
	Potential registrational studies							

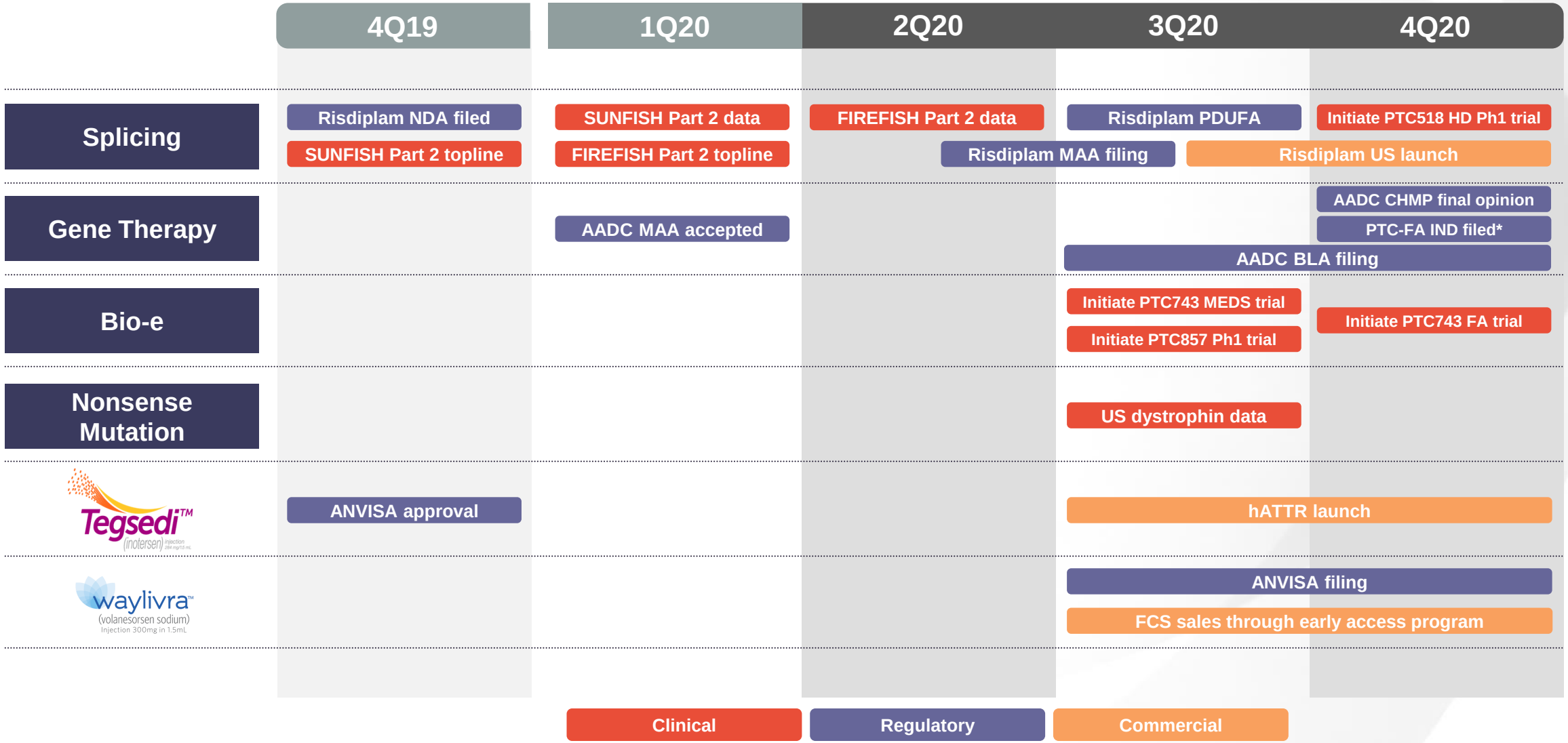
Scientific platforms & strategic business development drive sustainable innovation & continuous value creation



Majority of pipeline not represented in >$1.5B revenue target

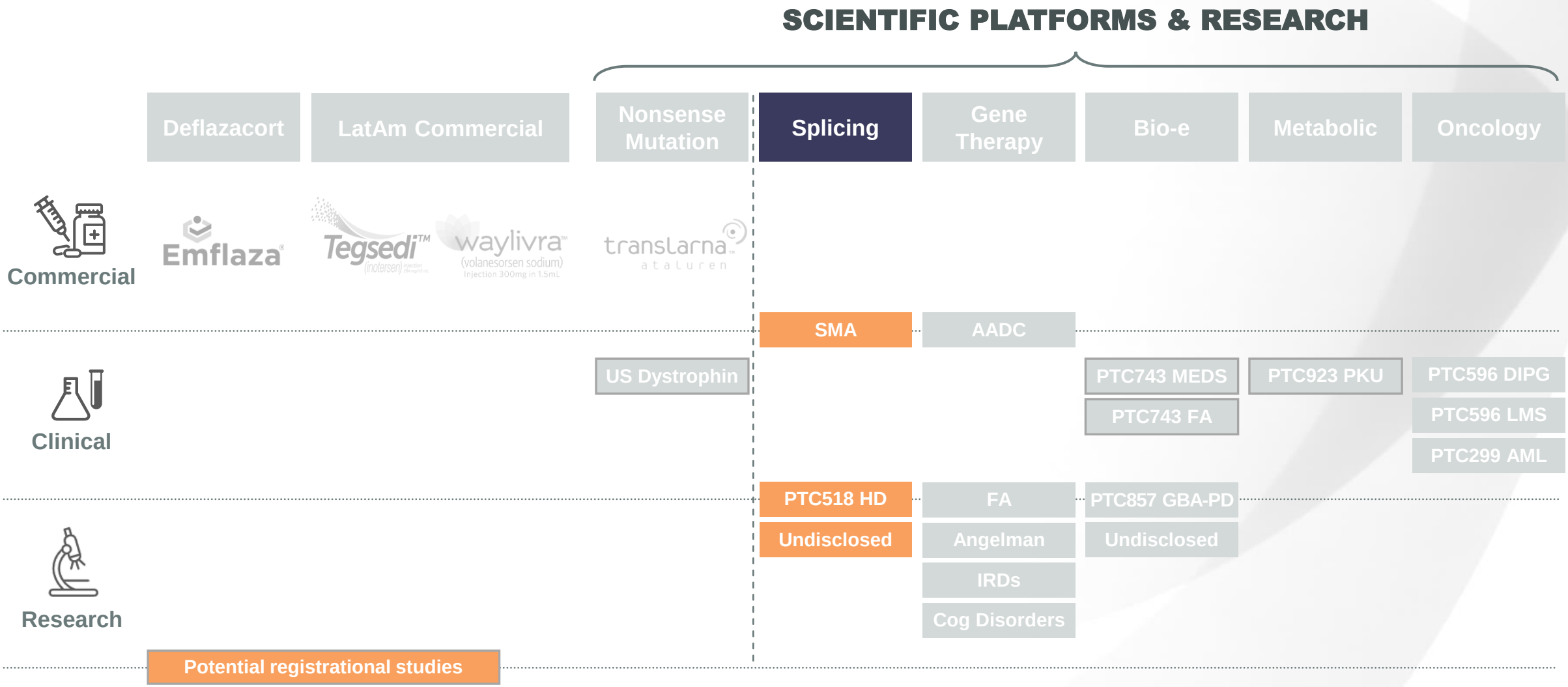


Multiple potential value driving events in 2020

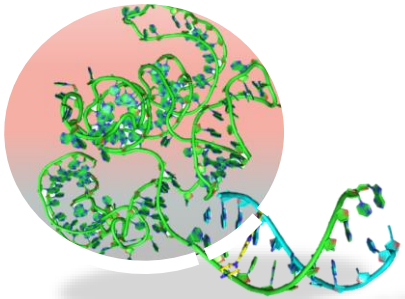


*PTC-FA IND filing delayed at least one quarter from 3Q20

Multiplatform approach builds diversified pipeline

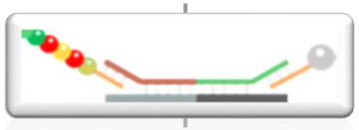


Splicing is highly selective with broad applicability

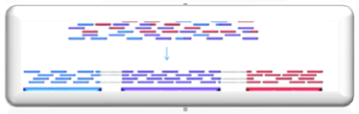


Exploiting U1-noncanonical exons

- Alternative splicing
- Exon skipping
- Stop exon inclusion



Isoform plex



HTSpliceseq

Transcriptome mRNA isoform detection platforms

2019

- **SMA (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

Risdiplam – Most competitive commercial profile across broadest population

FIREFISH – Type 1 SMA

FIREFISH Part 2 demonstrated statistically significant improvement in proportion of infants sitting for at least 5 seconds at 12 months

29%

12 of 41 infants were able to sit for at least 5 seconds without support at month 12; p<0.0001

85%

35 of 41 infants were event-free at month 12

95%

of infants alive maintained the ability to swallow after 12 months

Results confirm risdiplam's clinically meaningful efficacy in infants with advanced and difficult to treat disease

FIREFISH Part 2 met primary & key secondary endpoints

SUNFISH – Type 2 and 3 SMA

Part 2 pivotal study demonstrated statistically significant improvement in MFM-32 scores compared to placebo

1.55

point change compared to placebo (p=0.0156) in MFM-32 scores

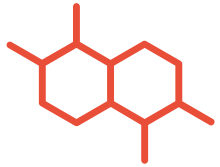
1.59

Point change compared to placebo (p=0.0028) in RULM scores

Included broadest group of SMA patients studied, age 2-25, representative of real-world spectrum of people living with SMA

SUNFISH Part 2 met primary & key secondary endpoints

Risdiplam – Most competitive commercial profile across broadest population



Small Molecule with systemic mode of action

Oral, at home-administration

Full target engagement - SMN2 full-length ↑, Δ7 mRNA ↓

Durably increases SMN throughout CNS and periphery

Studied in type 1,2,3 patients from newborns to 60 years of age

Clinically meaningful efficacy in real world patient population

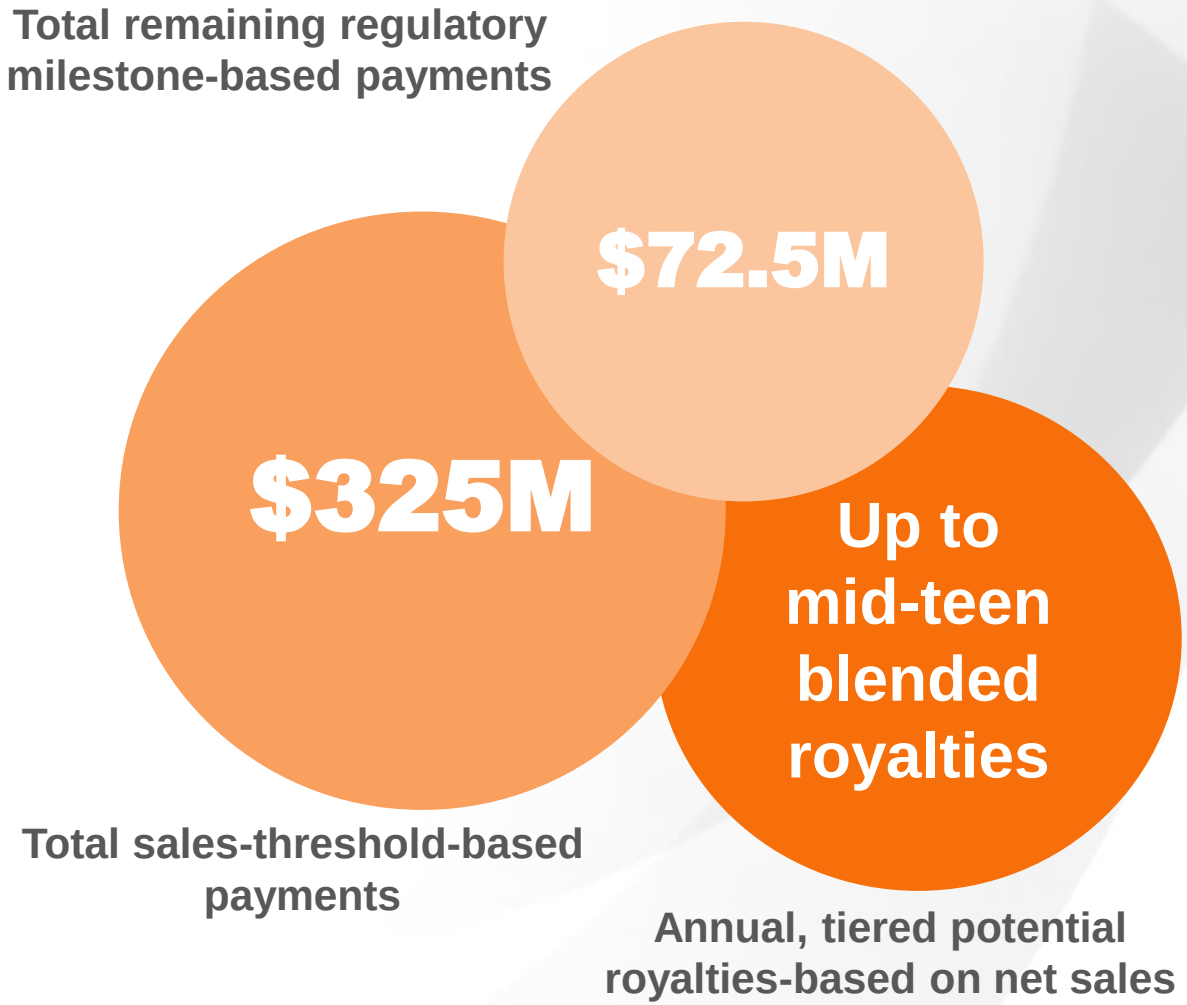
Strong safety profile

PDUFA date: August 24, 2020

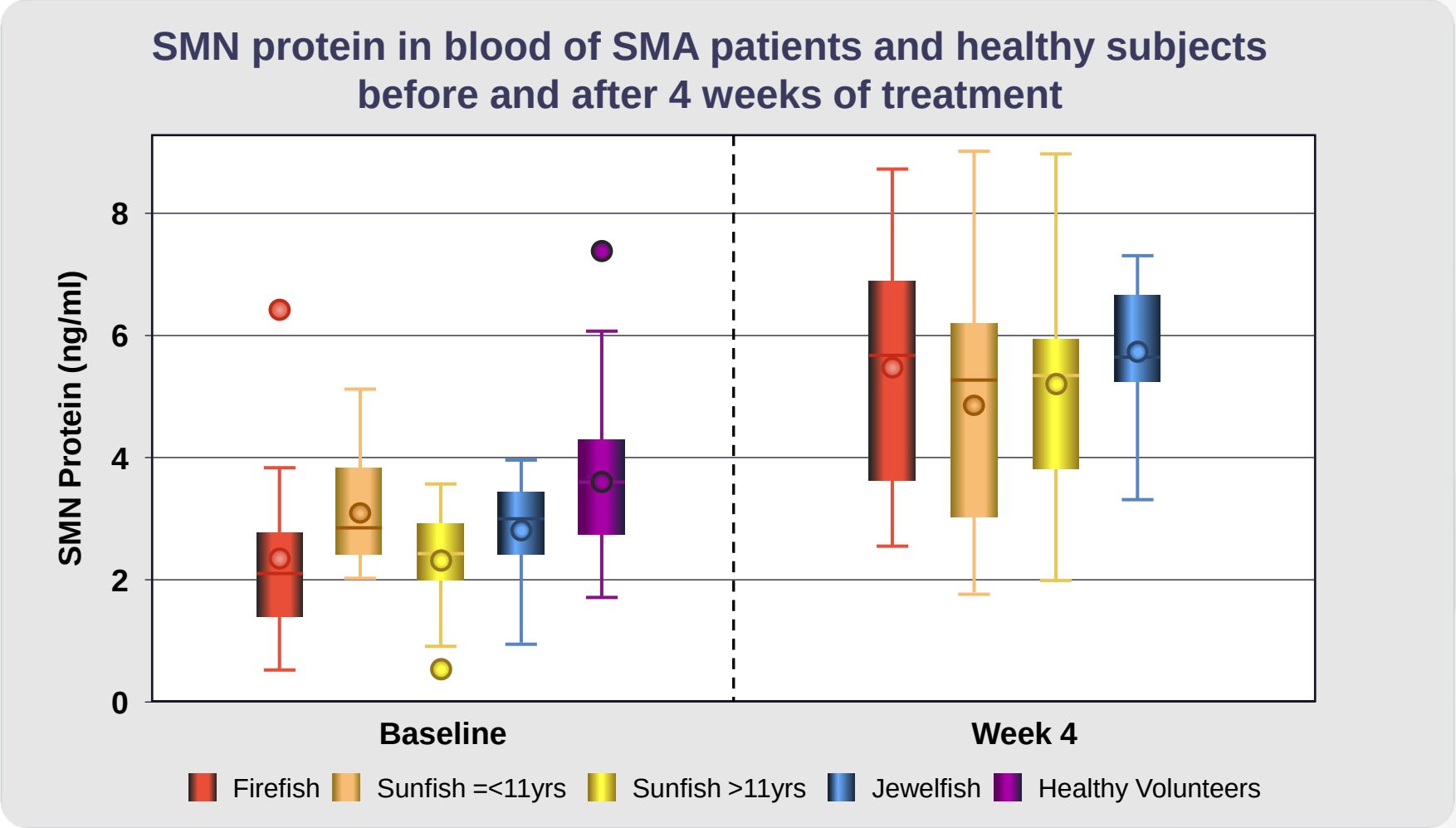
Significant success-based revenue through remaining risdiplam milestones and royalties

Potential 2020 risdiplam milestone payments to PTC

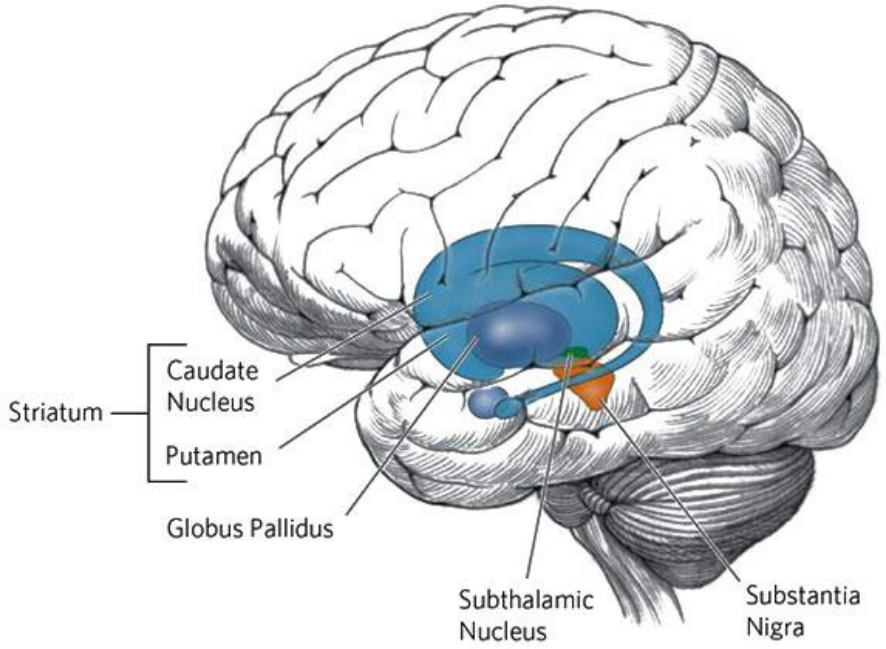
Milestone	Payment
MAA Filing with EMA	$ 15,000,000
NDA Filing in Japan	$ 7,500,000
First US Commercial Sale	$ 20,000,000
Total	**$ 42,500,000**



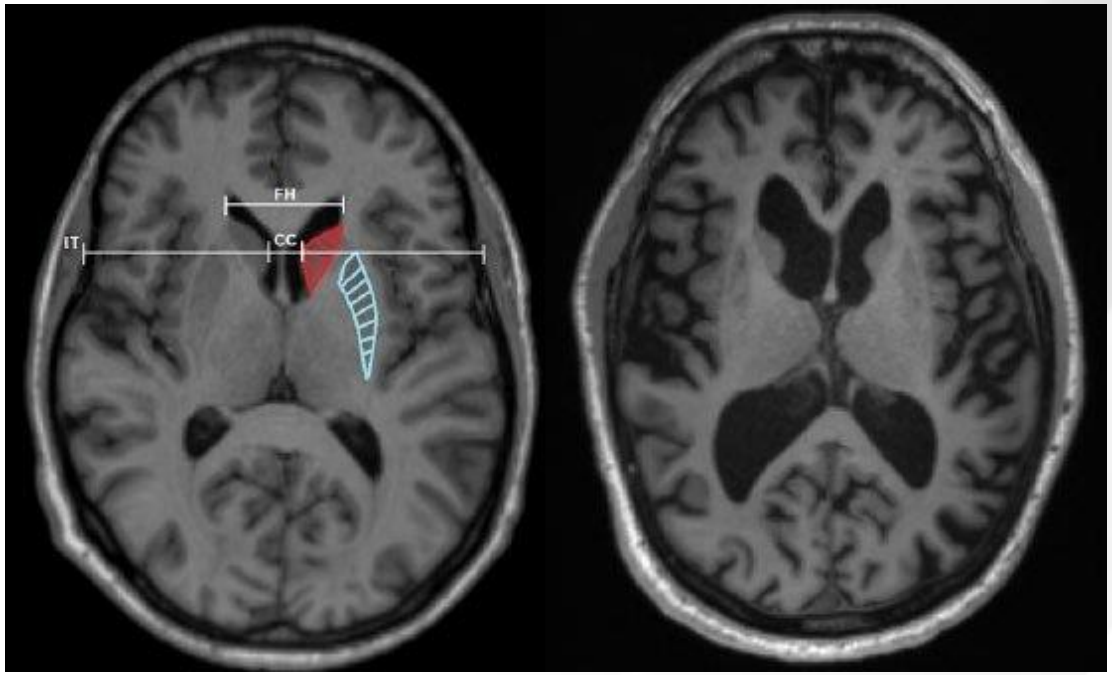
Activity of systemically distributed splicing drugs can be measured in blood



Toxic HTT protein aggregates cause extensive neuronal cell death



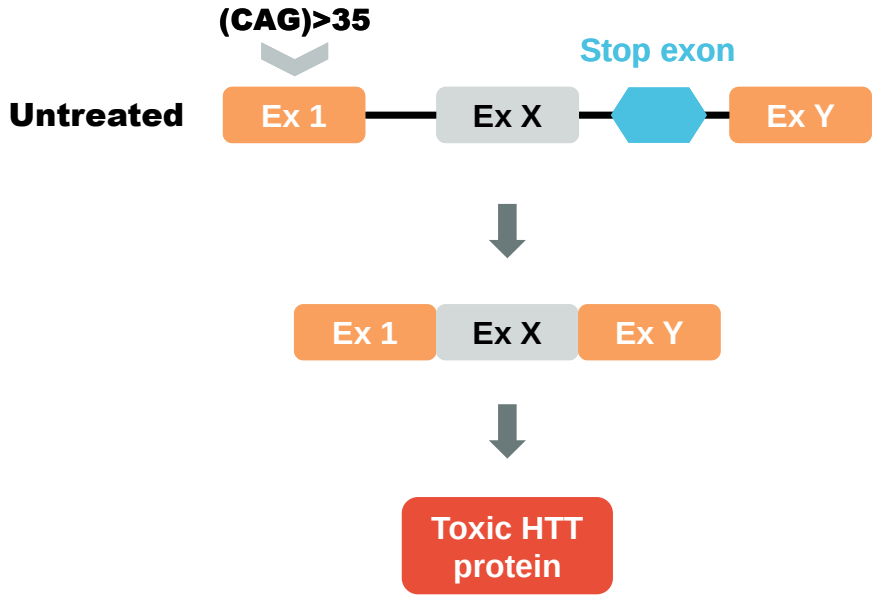
Healthy HD



Goh et al. *Aus Psychiatry*. 2018

Splicing modifiers reduce HTT protein levels in Huntington disease

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



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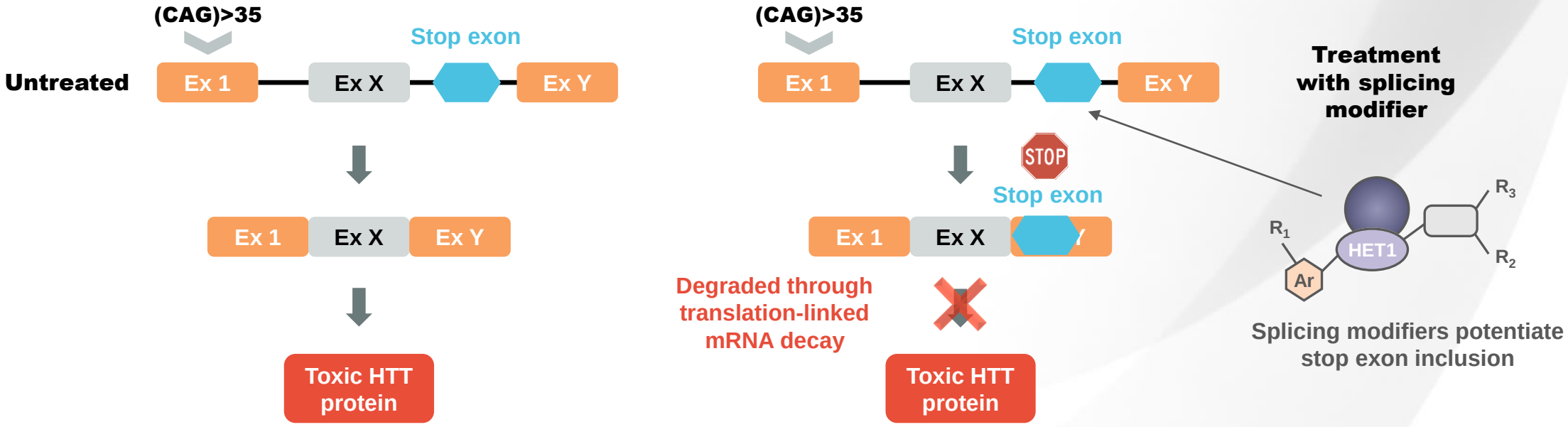
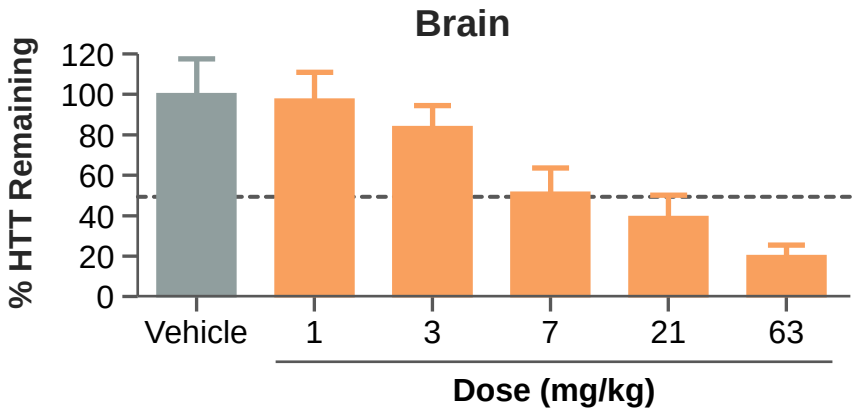


HD splicing small molecules with broad tissue distribution

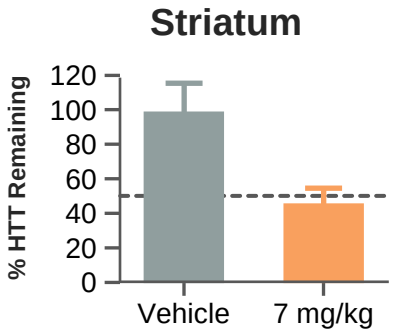
Dose dependent HTT lowering in the brain in BACHD mice

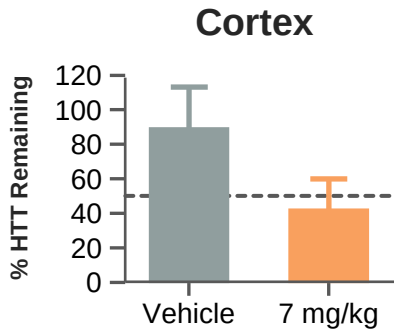


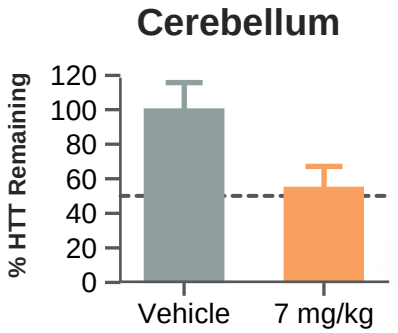
Ph1 trial planned for 4Q 2020

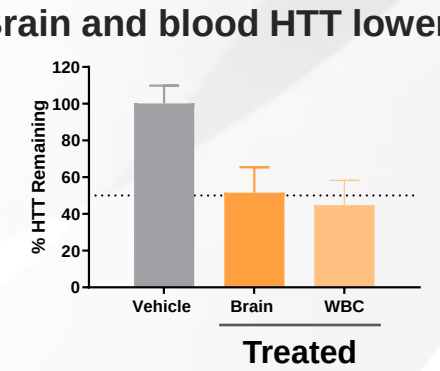
- Oral, crosses BBB
- Titratable
- IND toxicology studies ongoing
- Ability to measure mRNA and protein in blood in healthy volunteers

Measurements demonstrate uniform HTT lowering across brain regions with ~1:1 brain and blood concentrations*



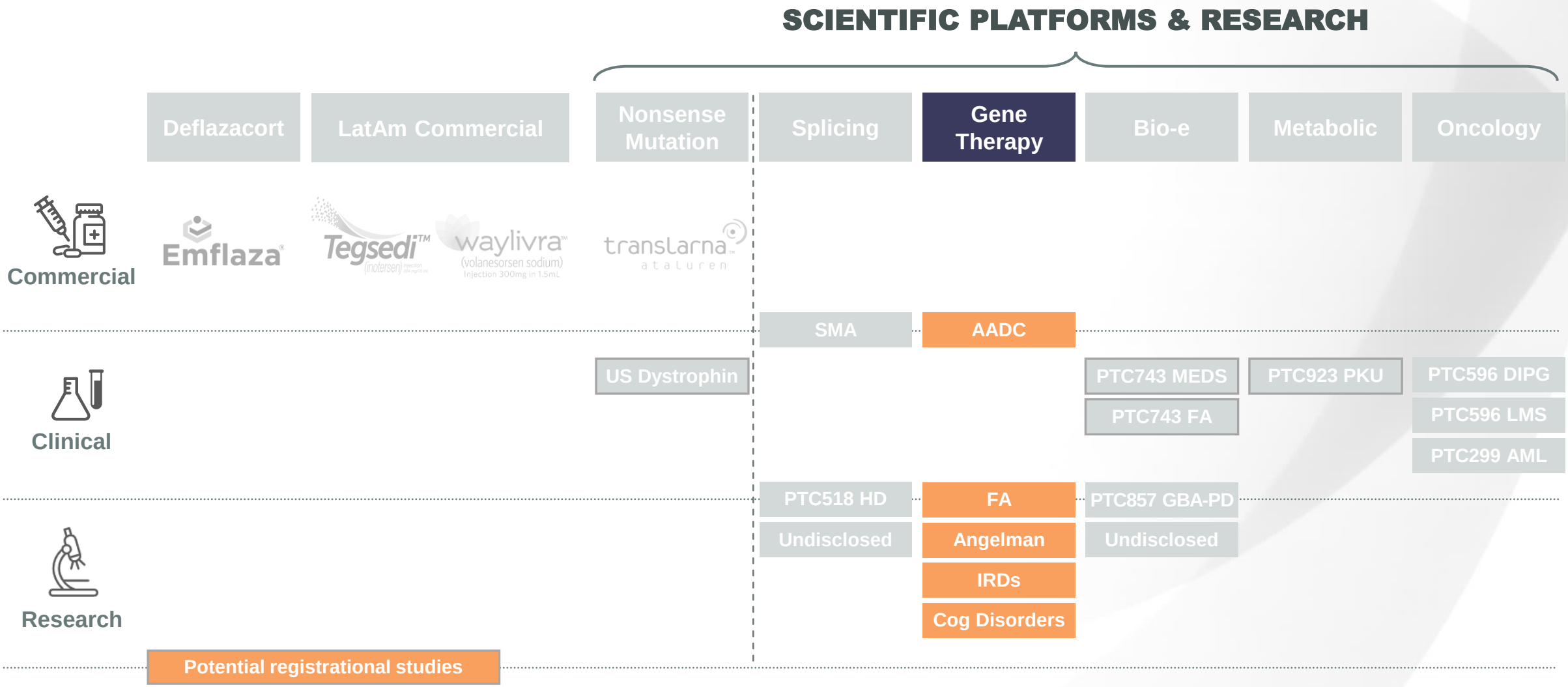






*Data on file from multiple studies

Multiplatform approach builds diversified pipeline



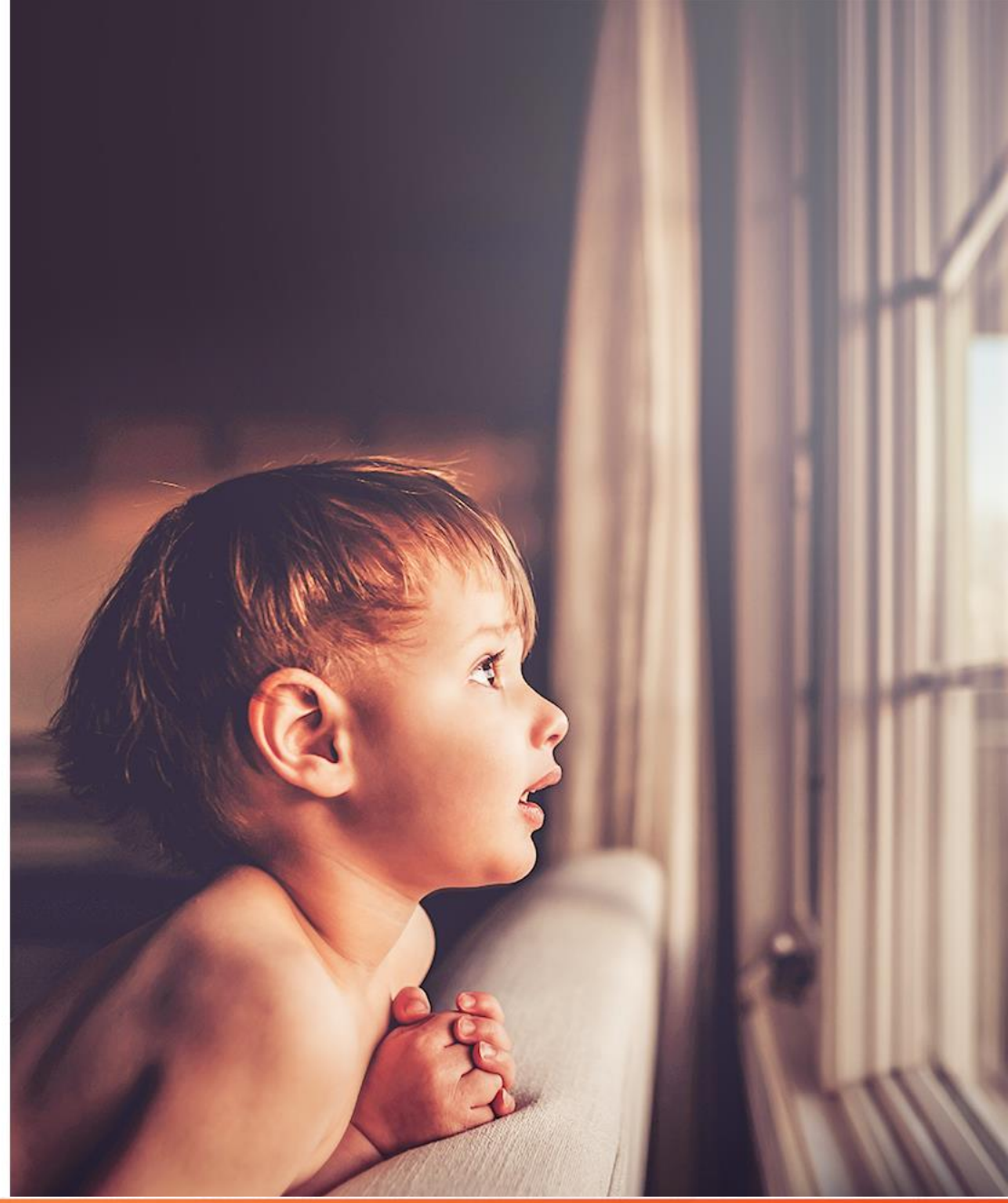
Treating rare monogenic disorders with targeted gene therapy

Potential advantages of targeted therapy

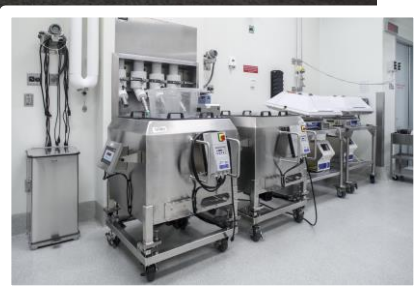
- Local administration lowers systemic immunogenicity and exposure
- Low turnover cells may lead to improved durability
- Micro-dosing lowers manufacturing and patient burden

Pipeline

- PTC-AADC MAA submitted
- PTC-AADC BLA expected in 2H20
- PTC-FA and Angelman syndrome IND enabling activities progressing
- >5 nonclinical development candidates



Internal gene therapy manufacturing capabilities



- GMP manufacturing of clinical material to begin in early 2021
- 15-year lease on ~220,000 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

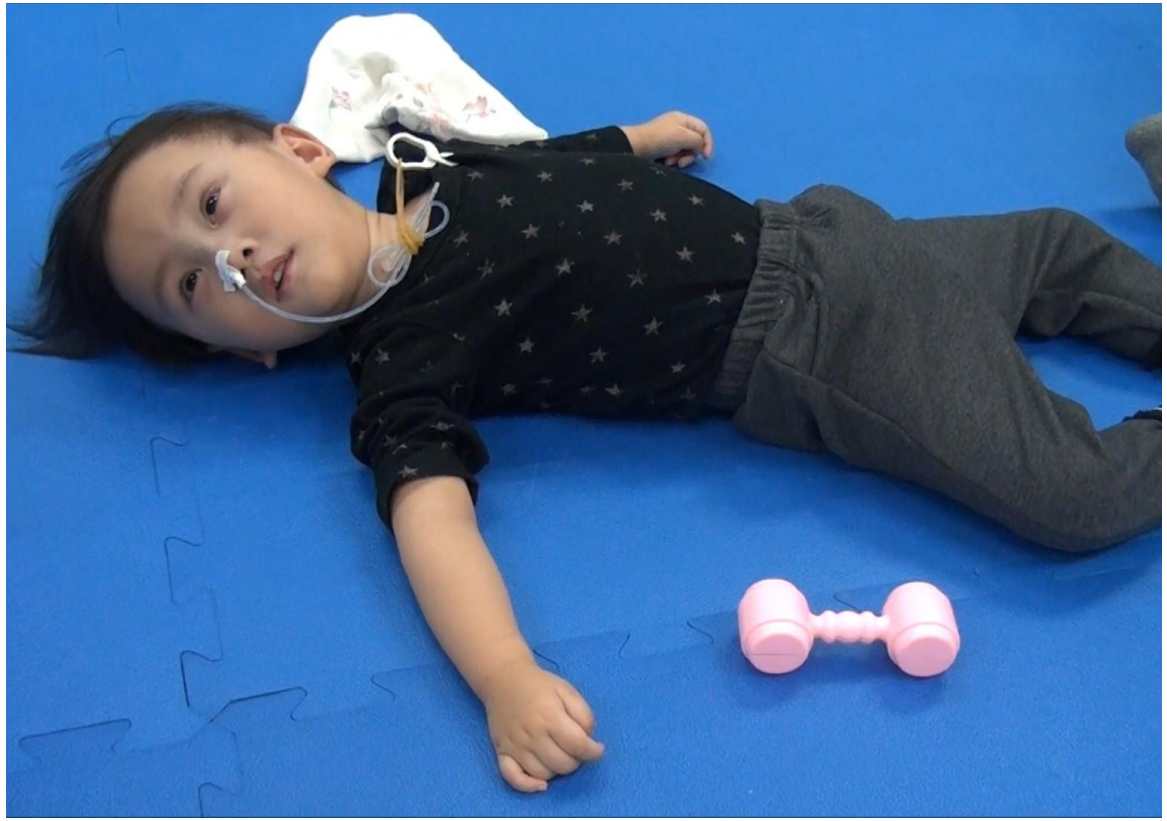
AADC deficiency – Rare disorder with significant unmet need

- 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
- ~80 disease causing variants described in AADC deficiency

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

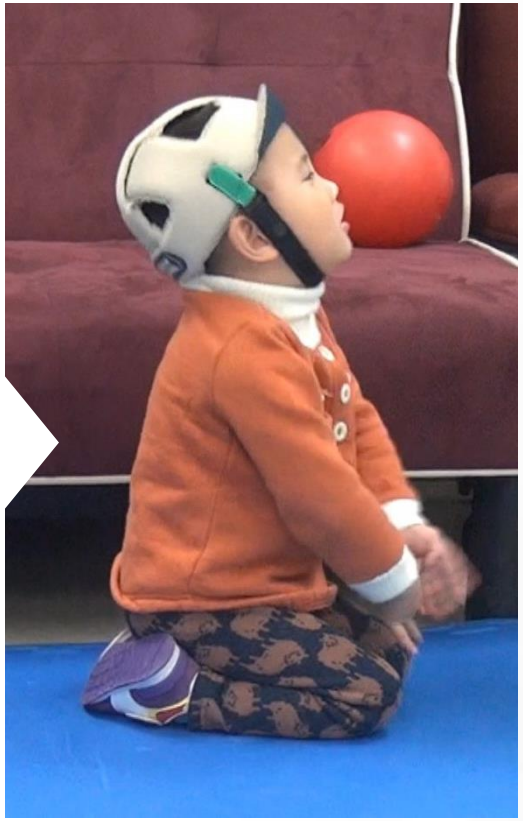
PTC-AADC patients make significant and sustainable progress

Untreated



Age 2

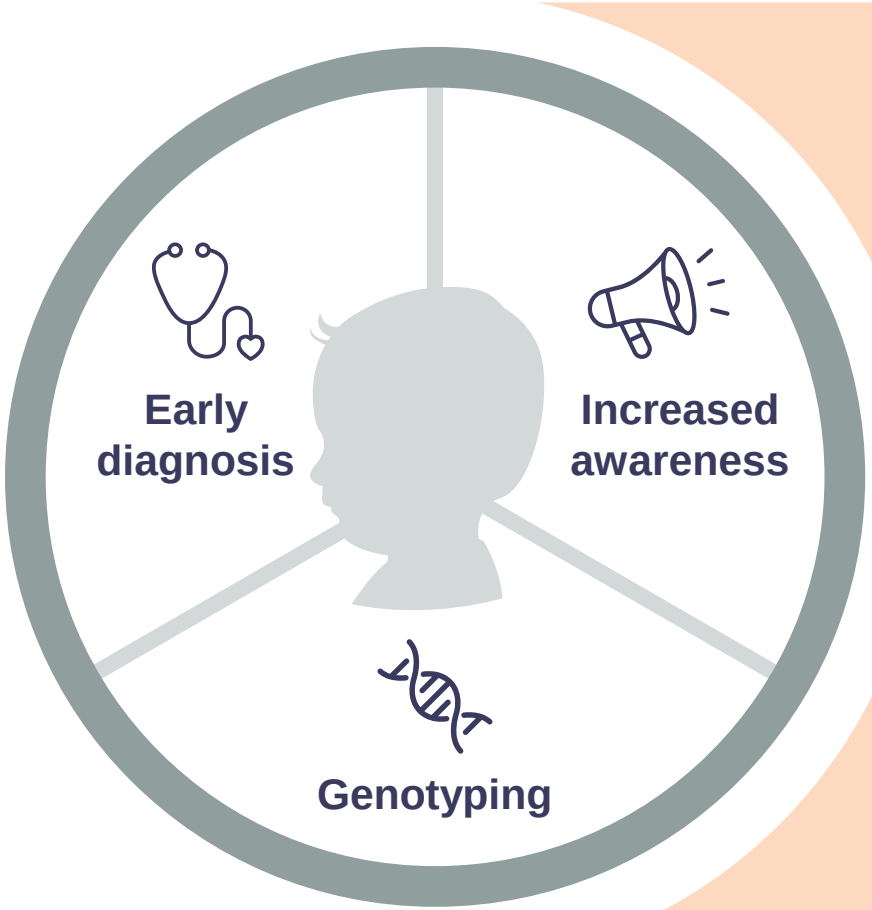
Post-Treatment



Age 3

Age 4.5

Adapting patient identification efforts due to COVID-19



Implemented virtual education & patient finding initiatives

Conducted master class with >200 HCPs from >20 countries

Held multiple European AADC steering committee meetings

Virtual HCP meetings continue to support diagnosis of new patients in cerebral palsy & epilepsy clinics

300+ AADC patients by launch

Focused on education supporting early patient diagnosis

Rolled out 'The Road Less Traveled' program for finding a path towards early patient diagnosis

Launched social media campaigns

Leveraging expert videos focusing on symptoms and directing viewers to disease state websites

Multiplatform approach builds diversified pipeline

SCIENTIFIC PLATFORMS & RESEARCH

	Deflazacort	LatAm Commercial	Nonsense Mutation	Splicing	Gene Therapy	Bio-e	Metabolic	Oncology
Commercial	Emflaza®	Tegsedi™ (inotersen) / waylivra™ (volanesorsen sodium) Injection 300mg in 1.5mL	translarna™ ataluren					
Clinical			US Dystrophin	SMA	AADC	PTC743 MEDS PTC743 FA	PTC923 PKU	PTC596 DIPG PTC596 LMS PTC299 AML
Research				PTC518 HD Undisclosed	FA Angelman IRDs Cog Disorders	PTC857 GBA-PD Undisclosed		
	Potential registrational studies							

Multiplatform approach builds diversified pipeline

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Research				PTC518 HD Undisclosed	FA Angelman IRDs Cog Disorders	PTC857 GBA-PD Undisclosed		

Potential registrational studies

Bio-e Platform Overview

Platform capabilities:

Based on a family of oxidoreductase enzyme targets critical to generation and regulation of energy key to disease pathology

Novel approach:

Intersection of electron-transfer chemistry and biology

Validated target and mechanism of action:

Lead compound PTC743 targets the enzyme 15-lipoxygenase – a key enzyme hub that regulates inflammation and oxidative stress

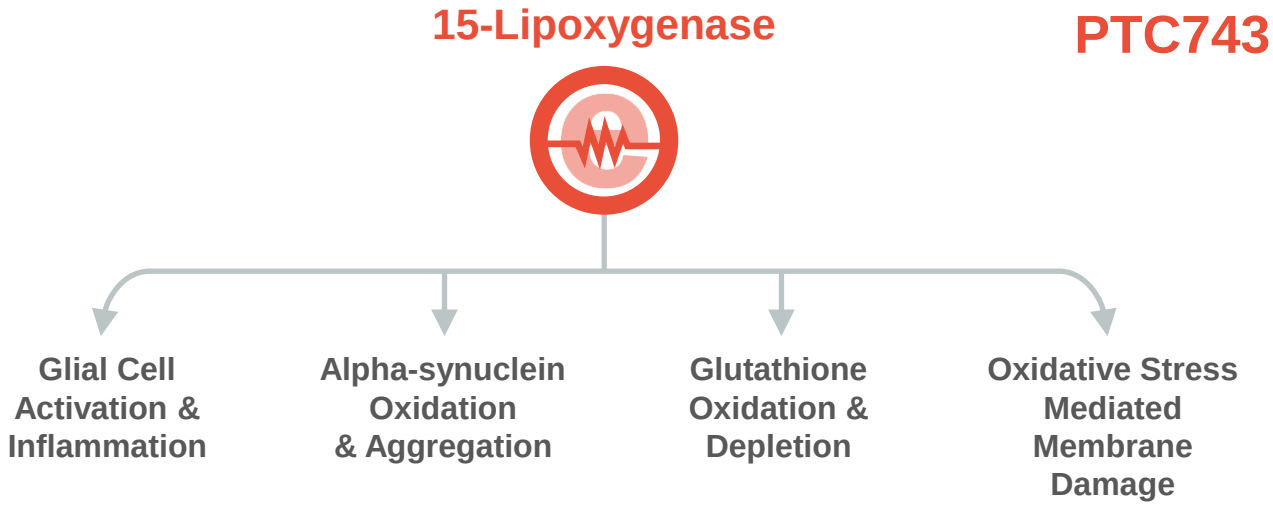
Extensive pediatric safety and exposure history:

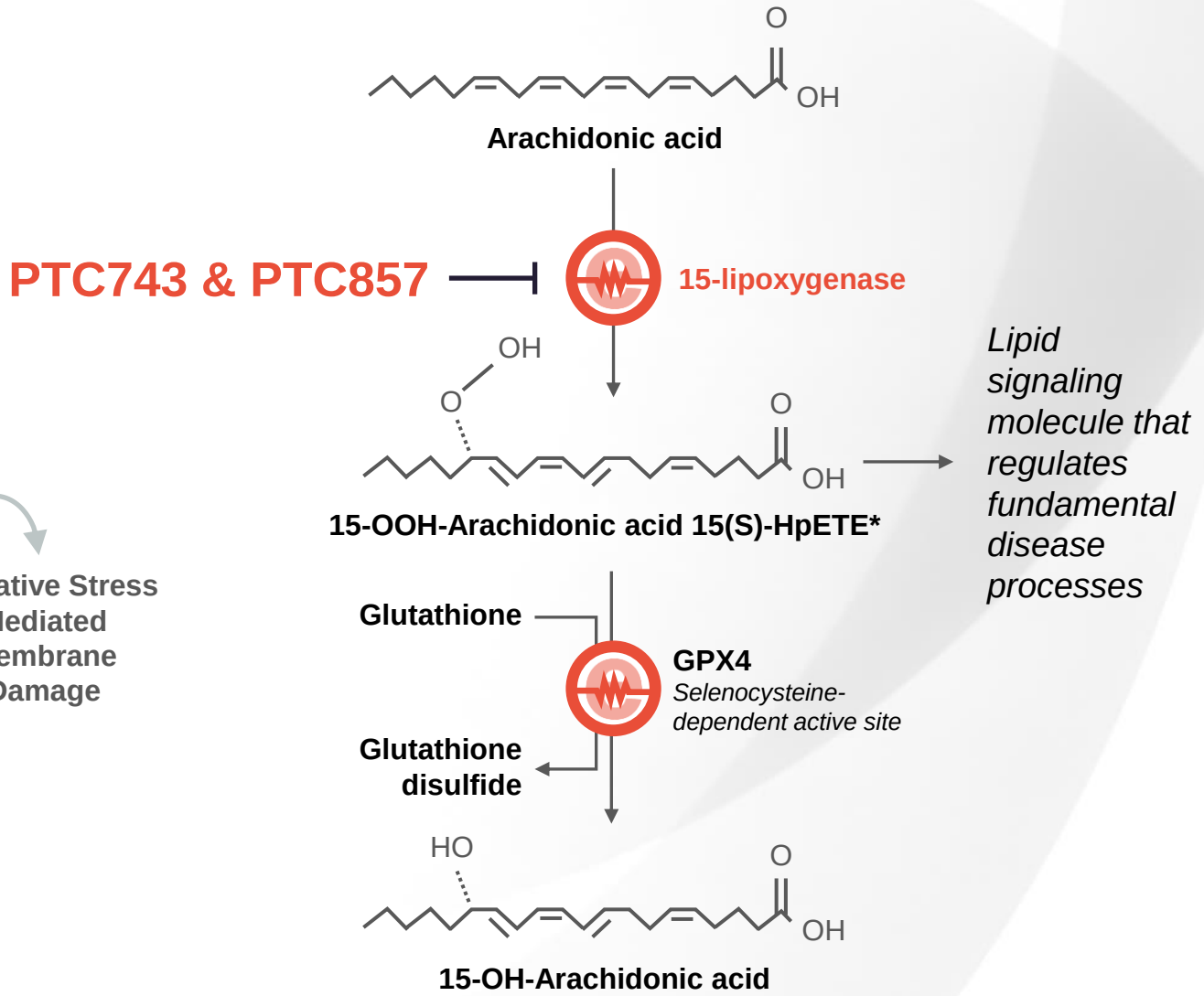
PTC743 has been evaluated in over 500 patients – mostly children – with duration of exposure up to 10 years, and has been safe and well-tolerated

Pipeline potential:

Large number of oxidoreductase targets with known biological significance (>100), and diverse redox small molecule library

Initial target is 15-lipoxygenase — key regulator of inflammation and oxidative stress pathways in CNS diseases





Initiating Three Bio-e Clinical Trials in 2020

PTC743

Mitochondrial Epilepsy Trial

Trial Starting 3Q20

- Proof-of-concept established in dozens of patients
- Clinical trials demonstrated reduction in hospitalizations and mortality risk in mitochondrial epilepsy patients
- Enrolling patients with 4 most common sub-types of mitochondrial epilepsy

5-6K

patients in the US and EU

PTC743

Friedreich Ataxia Trial

Trial Starting 4Q20

- 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

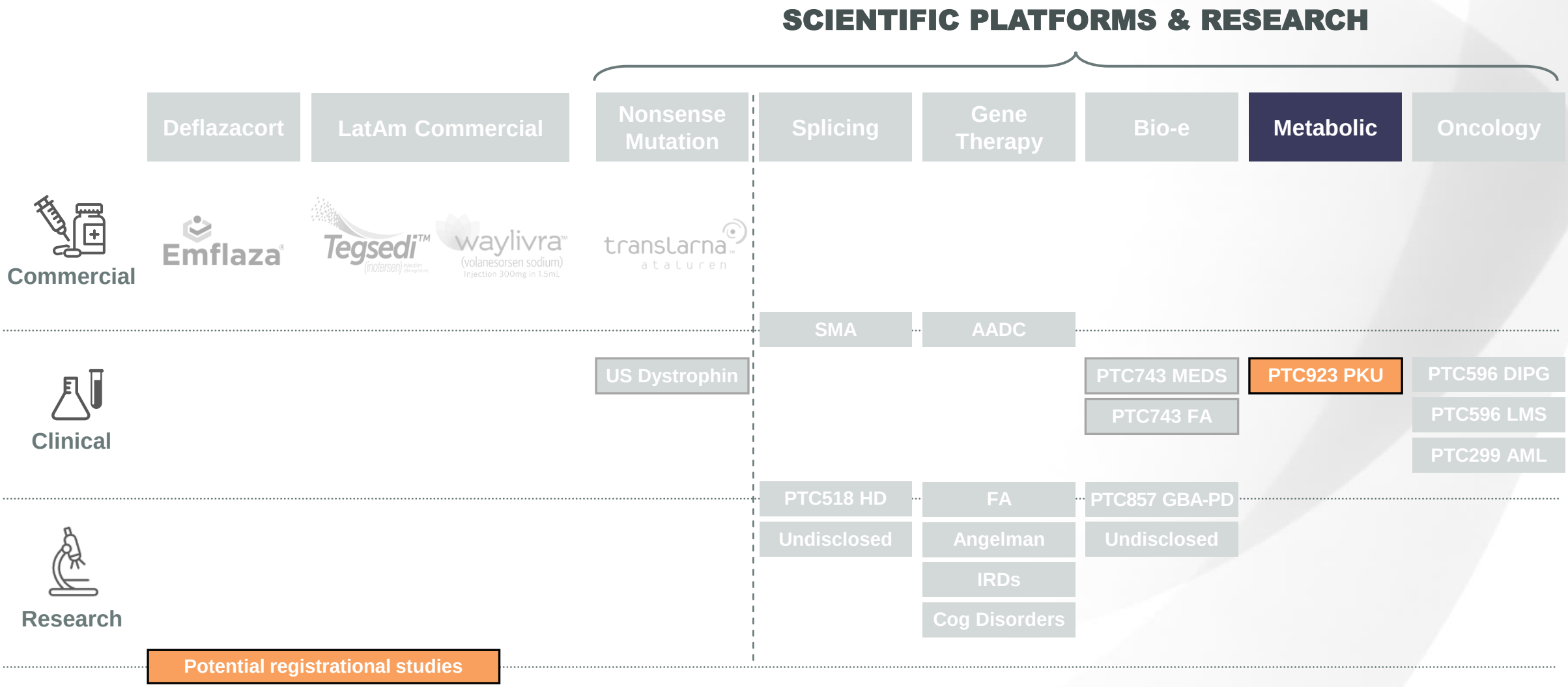
Trial Starting 3Q20

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

~50 – 90K

patients in the US

Multiplatform approach builds diversified pipeline

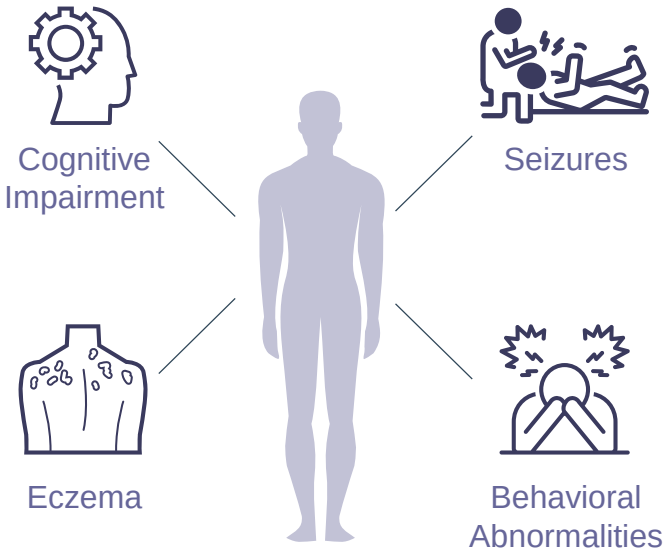


Diversifying and strengthening our rare disease portfolio

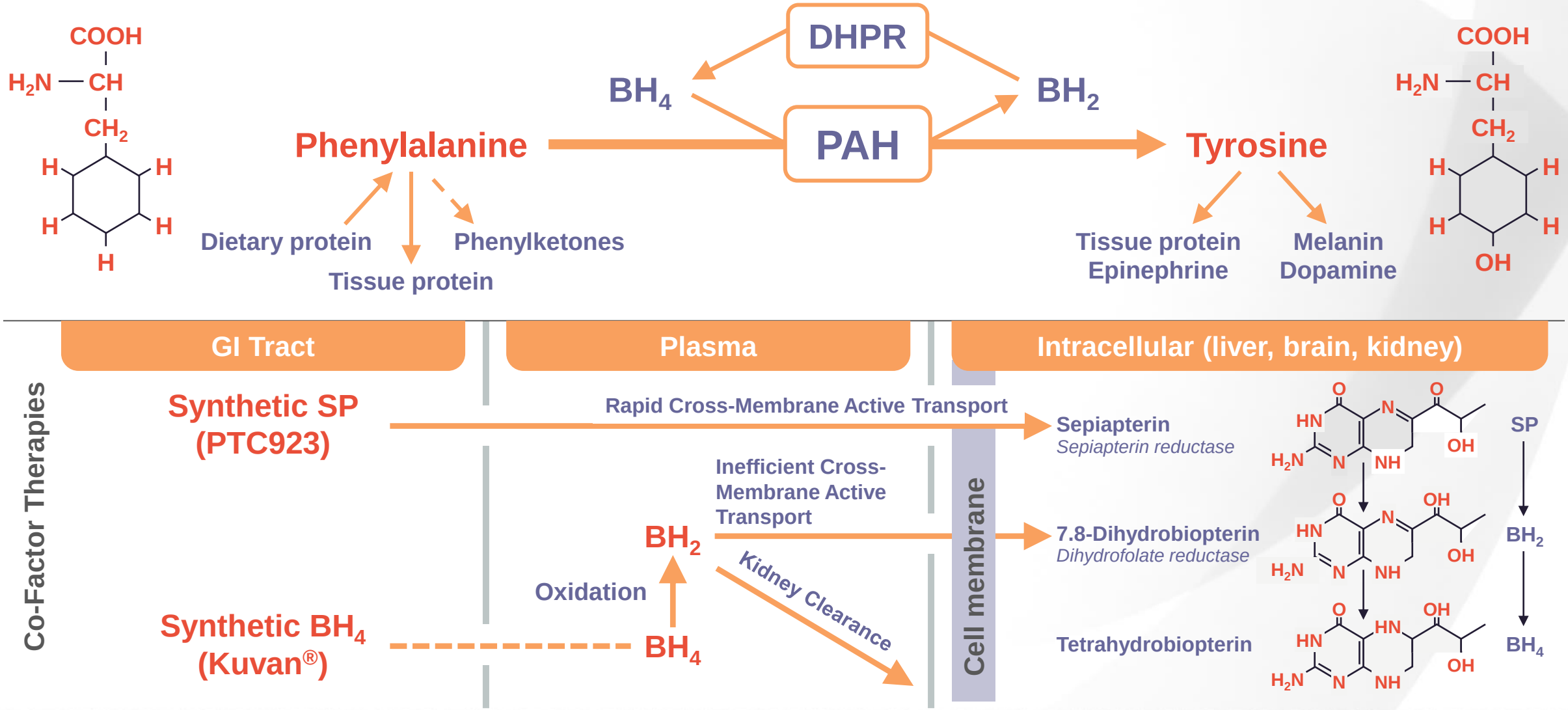
PTC923 Phase 3 Ready for Phenylketoneuria (PKU)

- High unmet need in PKU; 60-70% untreated
- Significant commercial opportunity; ~58,000 PKU patients globally
- Clearly defined market with newborn screening & established centers of specialists in place
- PTC923 differentiated relative to existing treatment options
- Fits with both clinical development and commercial expertise

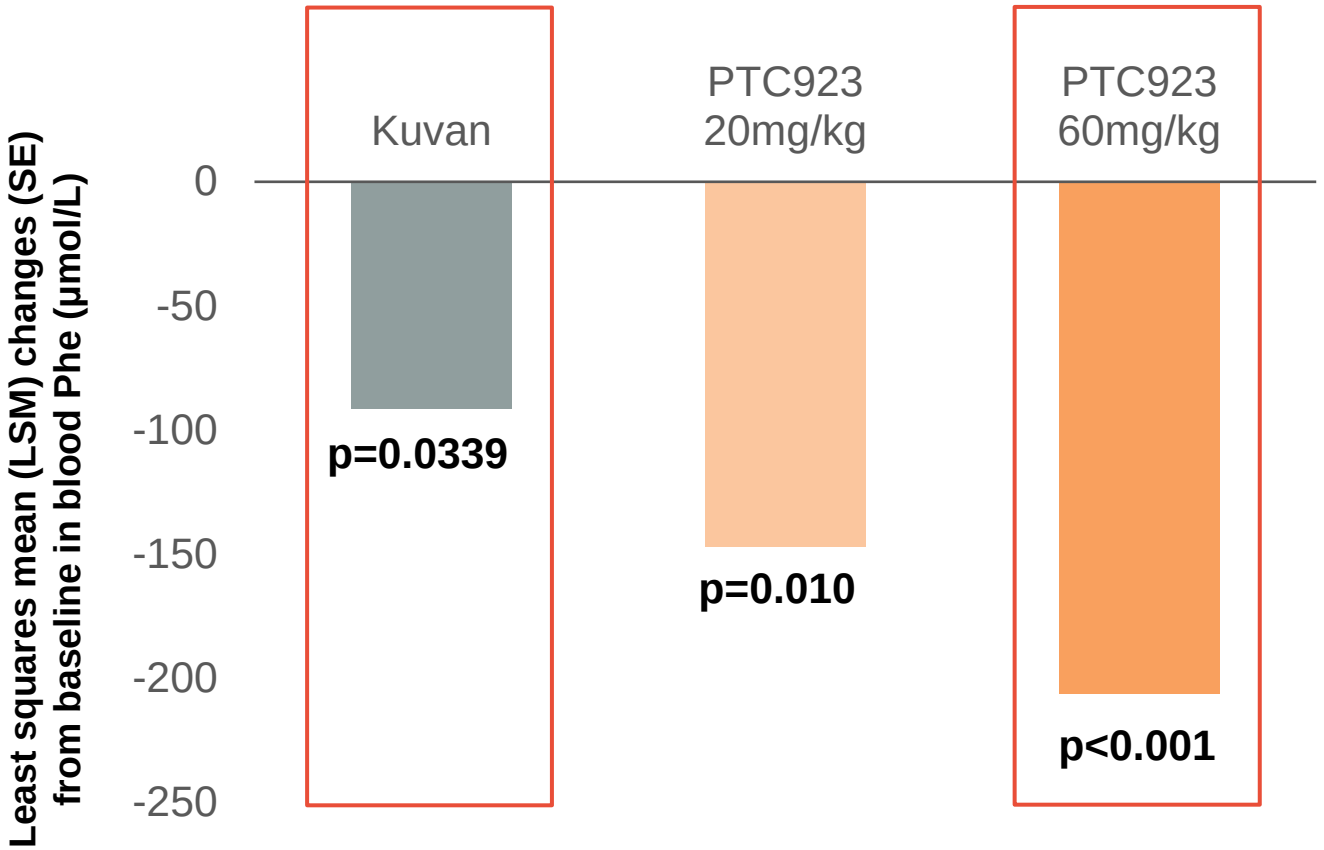
Symptoms of PKU can be managed well by maintaining low Phe levels in the body



Differentiated mechanism of action leads to greater intracellular bioavailability

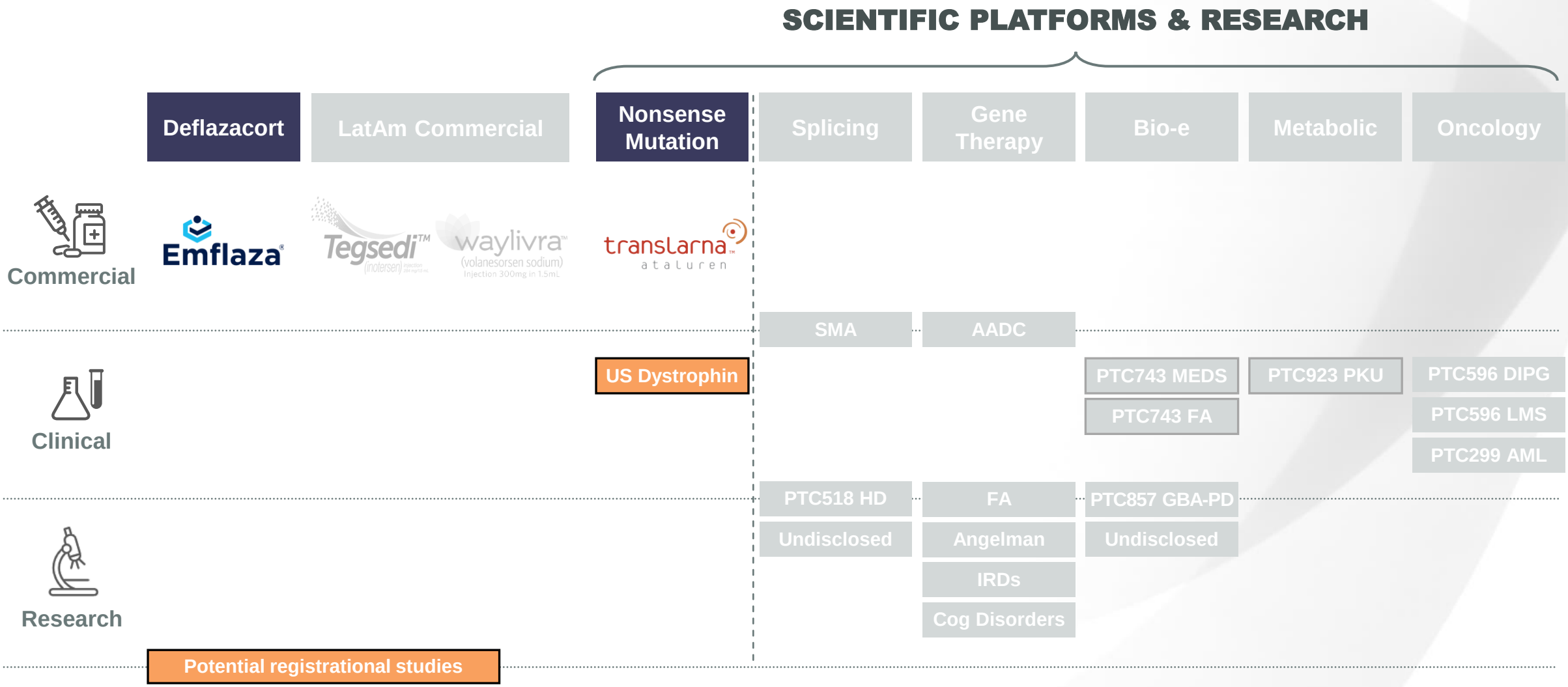


Demonstrated statistically significant differences in reduction of Phe relative to existing treatment options in Phase 2 study



- 60 mg/kg/day most effective dose
- 114.9 greater µmol/L reduction of Phe with 60 mg/kg/day PTC923 relative to Kuvan; p=0.0098
- 50% increased responder rate with PTC923 as compared to Kuvan (12/19 vs. 8/19)

Multiplatform approach builds diversified pipeline



Translarna™ demonstrates long-term benefit in DMD patients

~90%
EU5 nmDMD patients treated with Translarna

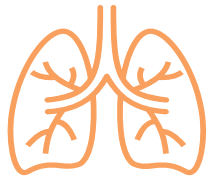
~85%
Compliance

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

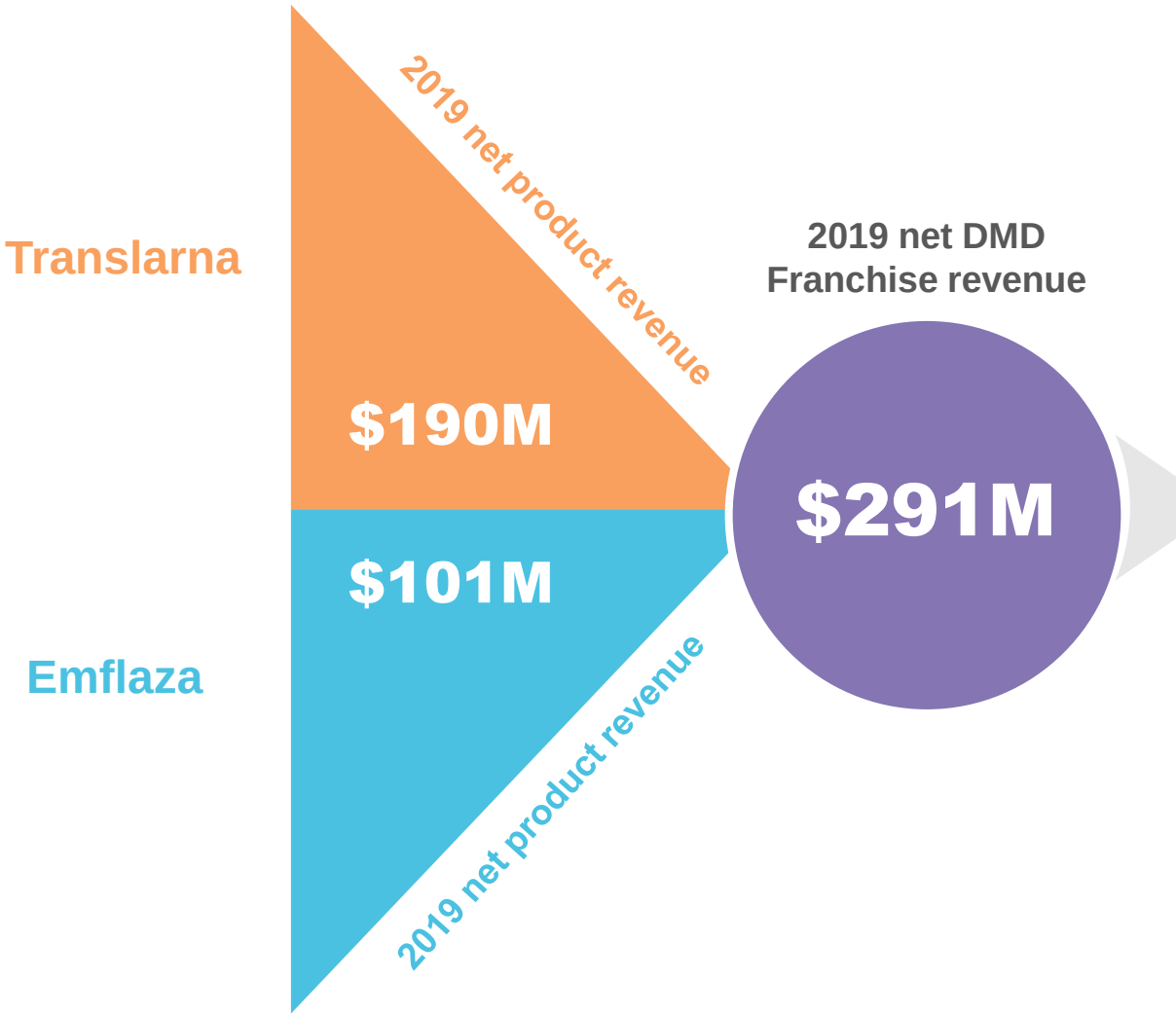
3.5 years
Delay in loss of ambulation

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%

Strong DMD franchise performance with continued growth opportunities



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

Ataluren US dystrophin trial data expected 3Q20

Open-label dystrophin study

Ataluren naïve patients

Nonsense mutation DMD boys ranging in age from 2-7

- Length: 40 weeks; N=20 patients
- Biopsies taken at baseline and 40 weeks after treatment
- Single site
- All samples analyzed together at the end of the study
- Endpoint: % dystrophin change from baseline as measured by ECL

Dystrophin levels measured using ECL assay

- Validated with FDA
- More sensitive than western blot to the full-length dystrophin protein
- Biopsies taken using less-invasive needle biopsy
- Biopsies taken from two muscles to improve sample quality

Multiplatform approach builds diversified pipeline

SCIENTIFIC PLATFORMS & RESEARCH

	Deflazacort	LatAm Commercial	Nonsense Mutation	Splicing	Gene Therapy	Bio-e	Metabolic	Oncology
Commercial	Emflaza®	Tegsedi™ (inotersen) injection 284 mg/1.5 mL; waylivra™ (volanesorsen sodium) Injection 300mg in 1.5mL	translarna™ ataluren					
Clinical			US Dystrophin	SMA	AADC	PTC743 MEDS PTC743 FA	PTC923 PKU	PTC596 DIPG PTC596 LMS PTC299 AML
Research				PTC518 HD Undisclosed	FA Angelman IRDs Cog Disorders	PTC857 GBA-PD Undisclosed		

Potential registrational studies

Leveraging our existing LatAm infrastructure to commercialize Tegsedi & Waylivra

Tegsedi™ (inotersen) injection 284 mg/1.5 mL

Best fit for Latin American hATTR market

ANVISA approval granted in 2019

First & only approved at-home therapy in Brazil with slowing disease progression and QoL indicated in label

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

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

Waylivra to utilize our patient support in Latin America

ANVISA submission expected in 2H20

Potential first FCS treatment

Received EU conditional marketing approval

FCS = familial chylomicronemia-syndrome

Potential 2020 remaining milestones to generate value