Fourth Quarter and Full Year 2019 Financial Results & Corporate Update

PTC Therapeutics, Inc.

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 2020 net product revenue guidance, statements with respect to the 2020 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 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 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, regulatory approvals 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.

Multiplatform approach builds diversified pipeline

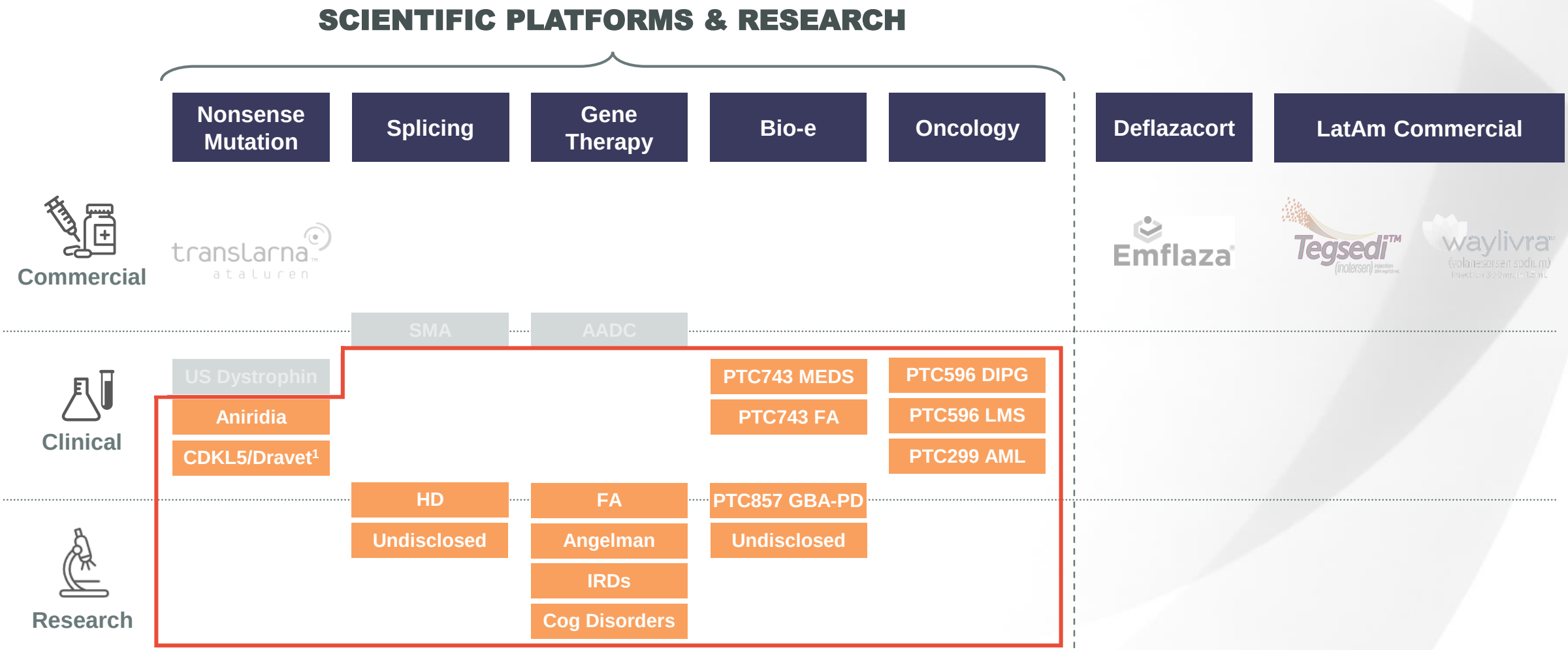
SCIENTIFIC PLATFORMS & RESEARCH

	Nonsense Mutation	Splicing	Gene Therapy	Bio-e	Oncology	Deflazacort	LatAm Commercial
Commercial	translarna™ ataluren					Emflaza®	Tegsedi™ (inotersen) injection 284 mg/1.5 mL; waylivra™ (volanesorsen sodium) Injection 300mg in 1.5mL
Clinical	US Dystrophin Aniridia CDKL5/Dravet[1]	SMA	AADC	PTC743 MEDS PTC743 FA	PTC596 DIPG PTC596 LMS PTC299 AML		
Research		HD Undisclosed	FA Angelman IRDs Cog Disorders	PTC857 GBA-PD Undisclosed			

Potential registrational studies

[1] Investigator-initiated study with NYU

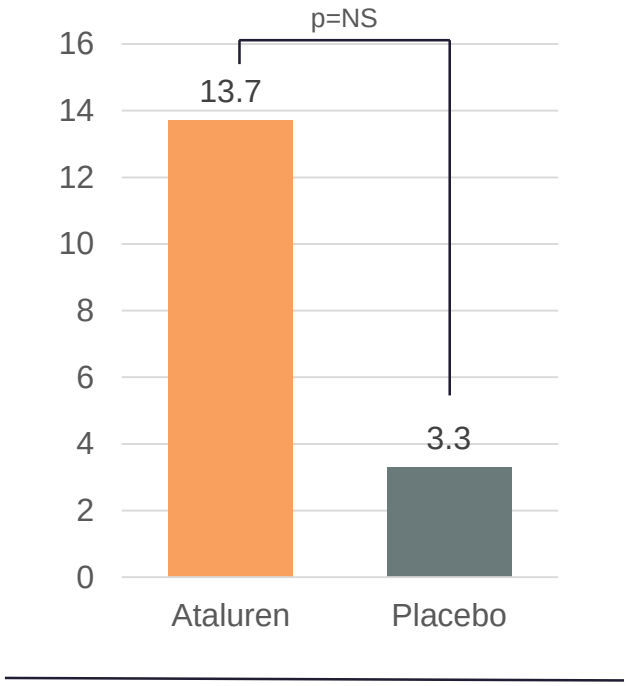
Majority of pipeline not represented in >$1.5B revenue target



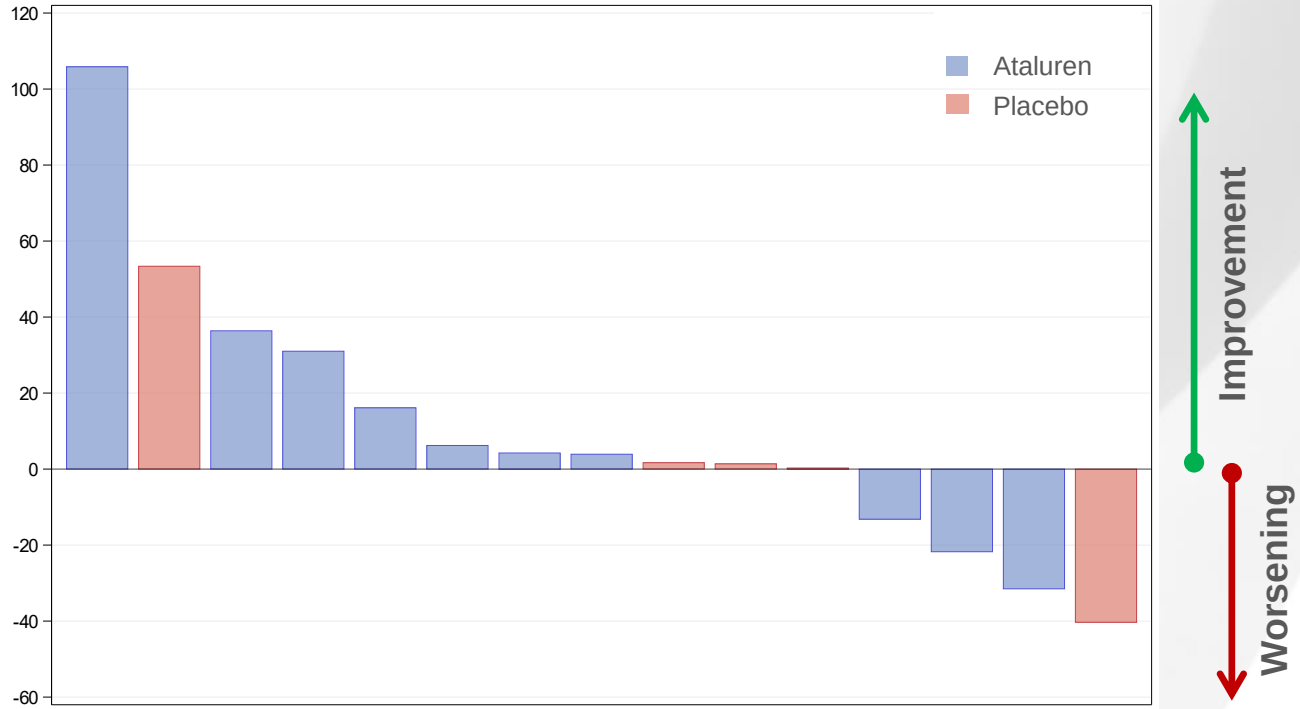
[1] Investigator-initiated study with NYU

Treatment with ataluren showed trend towards improvement in maximum reading speed in nonsense mutation aniridia patients

Percent change in maximum reading speed in both eyes over 48 weeks

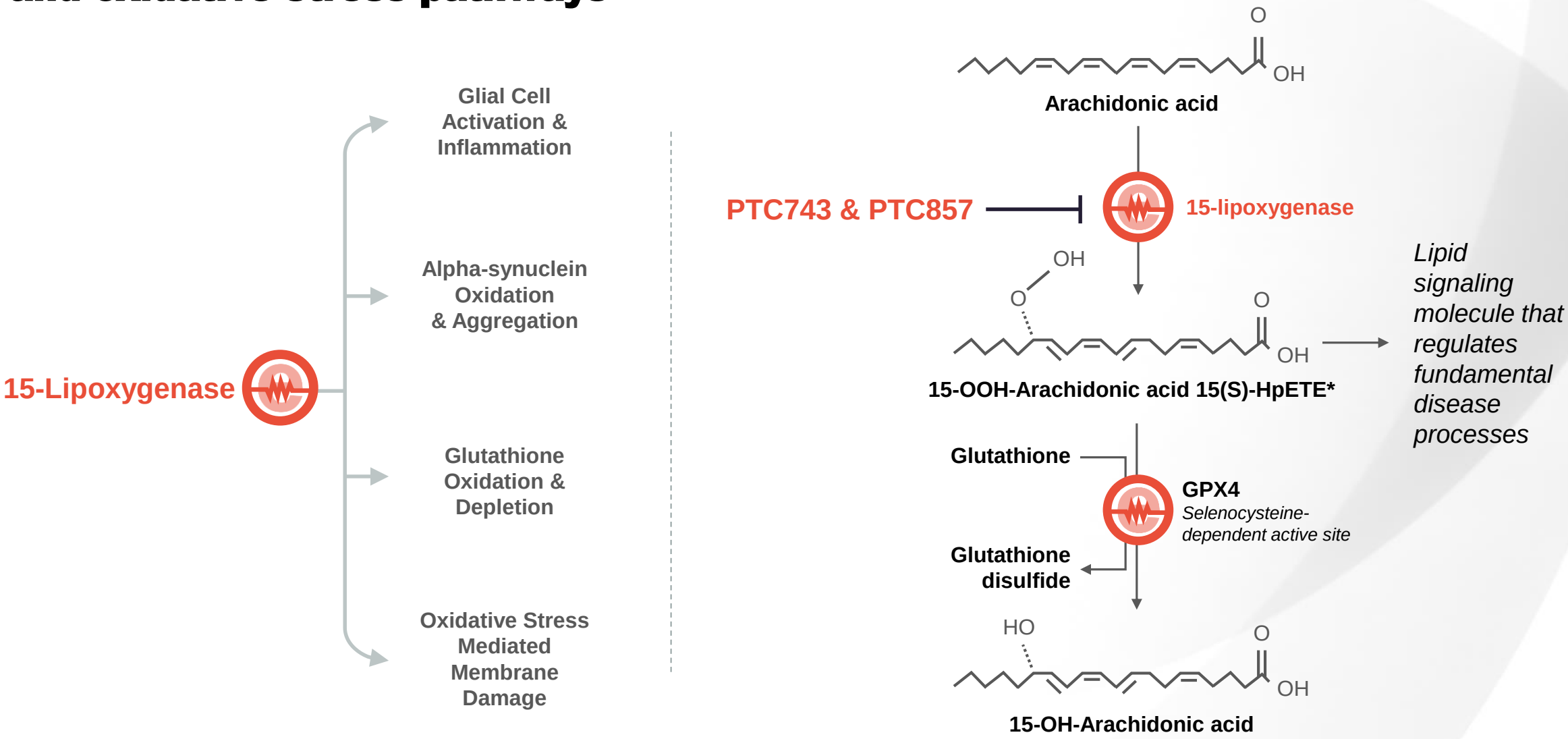


Percent change from baseline in maximal reading speed in the ITT population of patients aged ≥ 8 years over 48 weeks



NS= not significant

PTC743 and PTC857 target 15-lipoxygenase — a key regulator of inflammation and oxidative stress pathways



Initiating three clinical trials in 2020 in Bio-e platform

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
- Potential registrational trial

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
- Potential registrational trial

25K

patients WW

PTC857

Phase 1 Trial

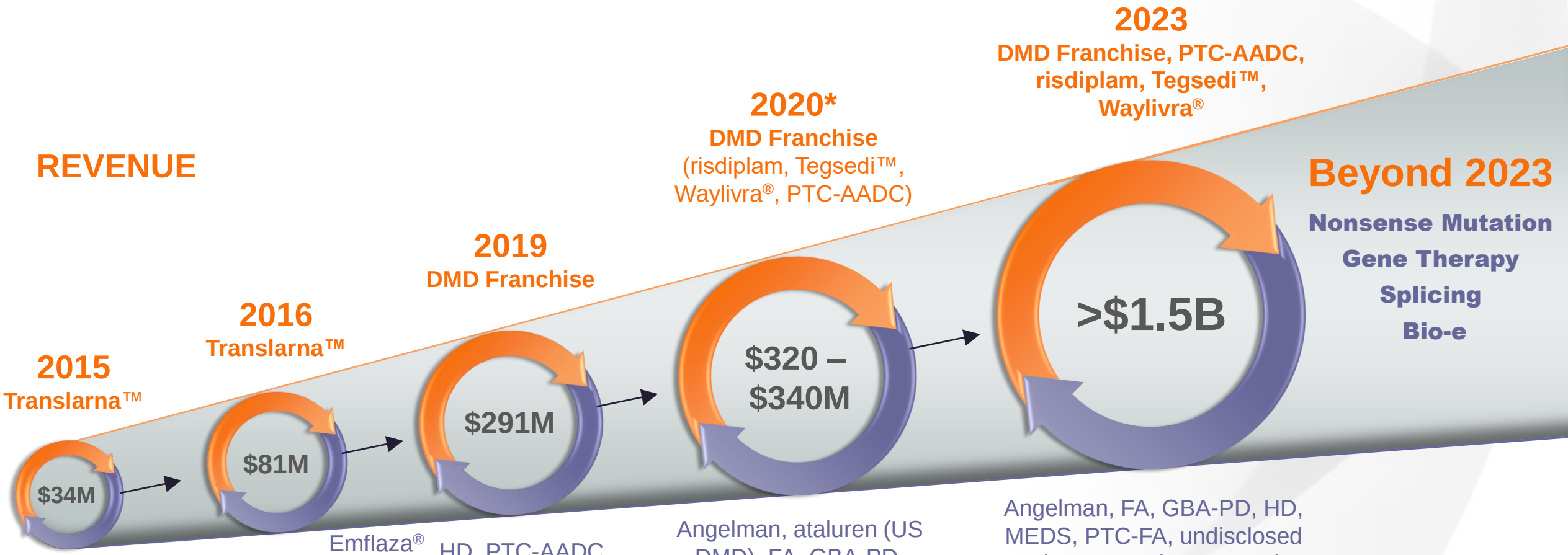
Trial Starting 3Q20

- 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

Sustainable innovation drives continuous value creation



* Revenue guidance does not include new product launches

Strong financial performance supports future growth

$307M
2019 Combined Net Revenue

$291M
2019 DMD Franchise Net Product 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 non-GAAP R&D and SG&A expense

SMA Milestones and Royalties

Royalties to PTC based on net sales

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

Sales-based milestones to PTC

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**

Milestone-based payments to PTC

Anticipated 2020

Event	Payment ($US)
Filing of a MAA in an EU country or with the EMA	**$ 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	**$ 72,500,000**