PTC Q2 2018 Earnings and Corporate Update

August 7, 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 the information provided relating to our "2018 Guidance", including with respect to (i) 2018 net product revenue and net sales guidance for Translarna and Emflaza and (ii) 2018 GAAP and non-GAAP R&D and SG&A expense guidance, and statements regarding: the future expectations, plans and prospects for PTC; our expectations with respect to the closing of our planned acquisition of Agilis Biotherapeutics, Inc. and potential subsequent regulatory submissions of any product candidates acquired upon closing; our expectations with respect to the licensing and potential commercialization of Tegsedi and Waylivra; expansion of commercialization of Translarna & 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; 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 NDA 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; PTC’s expectations with respect to the closing of its planned acquisition of Agilis, and the other transactions contemplated in conjunction with the acquisition, including with respect to matters of timing, including the satisfaction of closing conditions, the anticipated financial impact and potential benefits to PTC, integration of Agilis into PTC’s business and any product candidates PTC may acquire from Agilis into its business strategy assuming completion of the acquisition and other matters related to the acquisition; 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 planned acquisition of Agilis, 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, Tegsedi, Waylivra, PTC's other product candidates and any product candidates PTC may acquire upon completion of our planned acquisition of Agilis; 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 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.

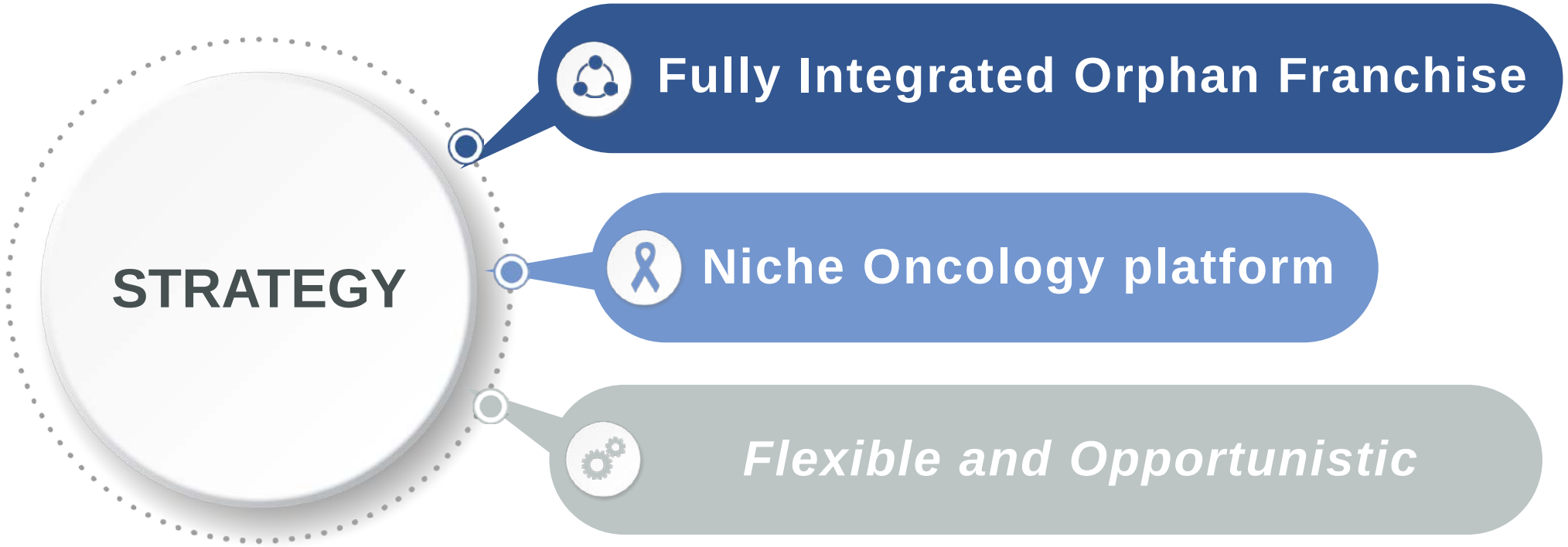
As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including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

Delivering on our strategic vision

VISION

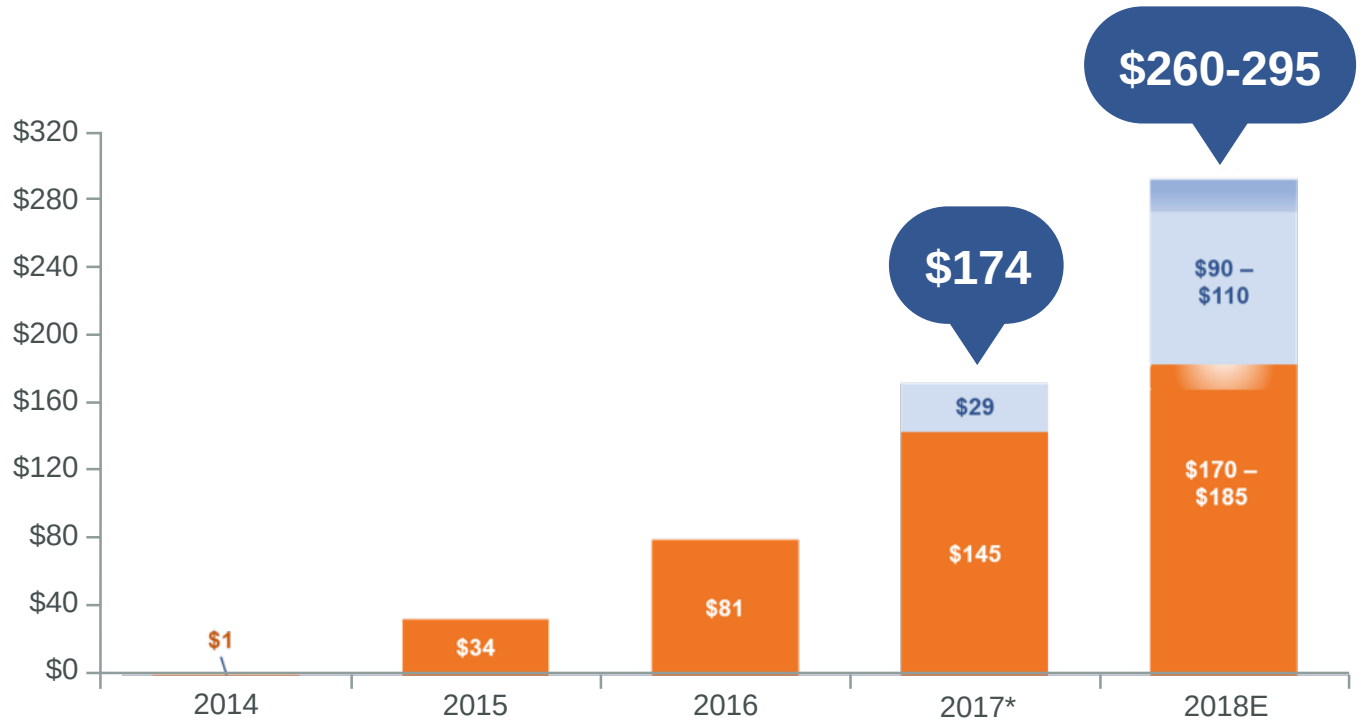
PTC is a fully integrated, innovative rare disorder company leveraging research capabilities and core technology platforms, building out world-class commercial capabilities, and being an ideal partner for late-stage, ultra-orphan disorders for which there is high unmet medical need.



Growing global DMD franchise

Translarna™ & **Emflaza®** DMD Net Sales

($ in millions)



Emflaza® (deflazacort)

translarna™ ataluren

In Q2 DMD franchise grew 17% year over year

Continuing to drive long term growth of DMD franchise

Earlier diagnosis

Increase disease awareness

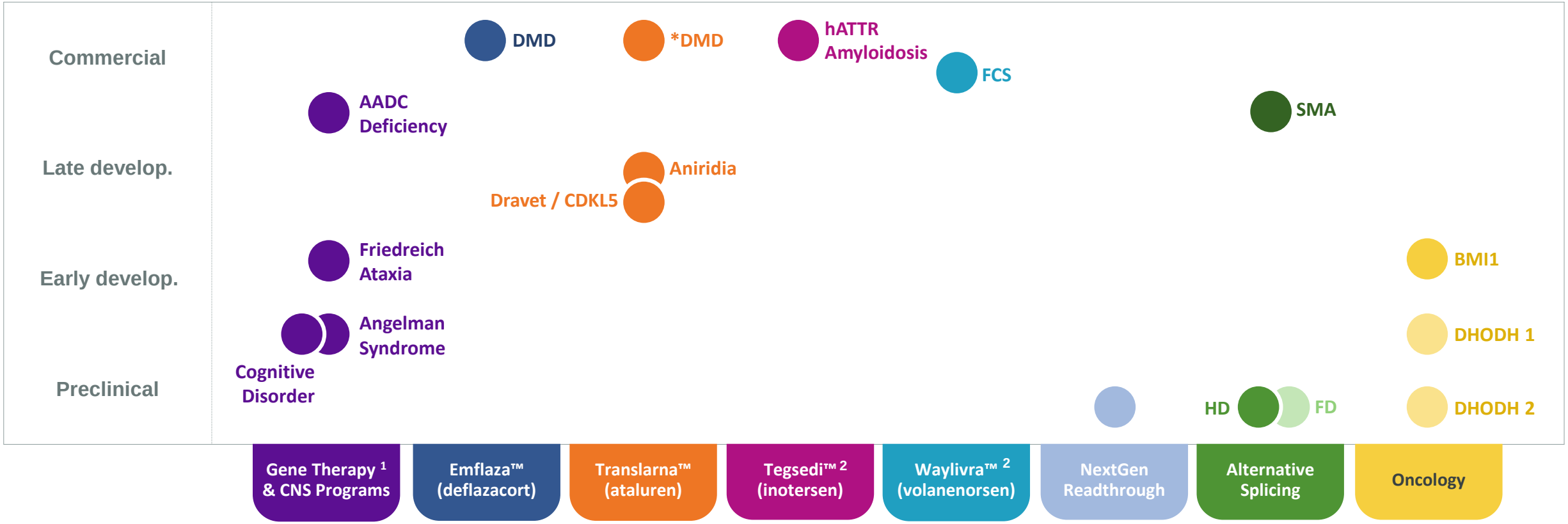
Genotyping

Improving standards of care

Approved EU label expansion for Translarna™ in 2-5 year-old patients

Strategy to bring Emflaza® to younger patients in the U.S.

Transformative transactions build out late stage and commercial pipeline



1 Gene therapy assets subject to closing of planned Agilis acquisition
2 LATAM & Caribbean rights licensed from Akcea Therapeutis
* MA requires annual renewal following reassessment by the European Medicines Agency (EMA)
Cardiometabolic diseases due to elevated triglyceride level: FCS = Familial Chylomicronemia Syndrome

Agilis acquisition highlights

CNS micro-dosing

- Low doses of vector required
- Efficient, scalable manufacturing
- Low manufacturing hurdles using existing systems
- **Cells with low turnover**

Strategic partnership with **MassBiologics Laboratories**

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

IND
Friedreich Ataxia

FDA BLA + EMA MAA submissions
AADC Deficiency

IND
Angelman Syndrome

IND
Cognitive disorder / Reelin

In-licensing opportunity of Tegsedi™ and Waylivra™ Leverages PTC's Latin America commercial infrastructure

Diversifies our rare disease portfolio and revenues

Tegsedi™ (inotersen) injection 284 mg/1.5 mL

Tegsedi best fit for Latin American hATTR market

hATTR polyneuropathy most prevalent phenotype in Latin America

~6,000 patients

Sub-cutaneous delivery preferable to infusions in the region

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

Waylivra launch: will utilize our patient support in Latin America

Similar economic opportunity to Translarna in Latin America

No other treatments available to treat FCS

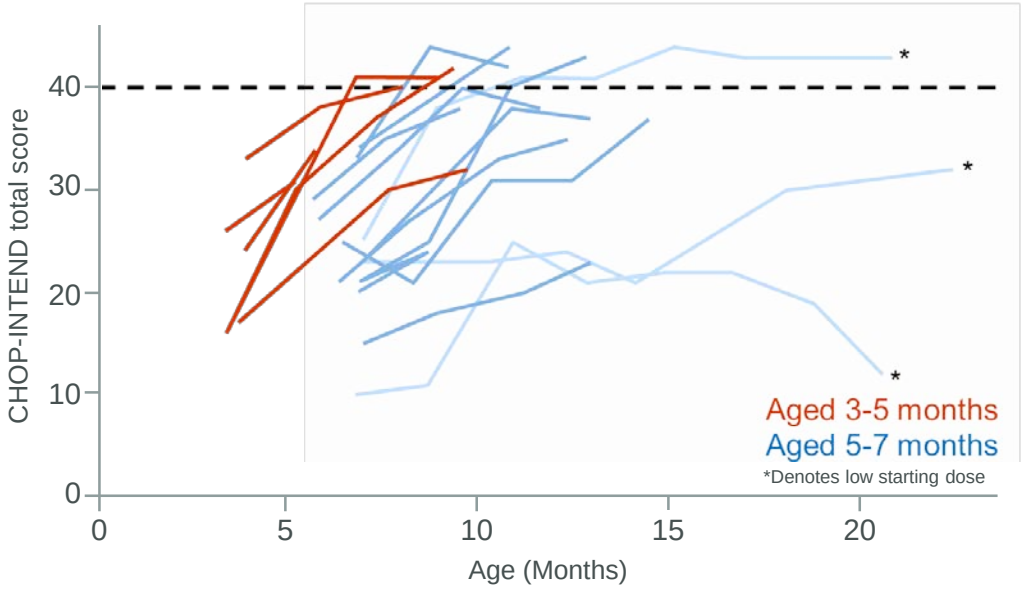
Potential for expansion in FPL

FCS = familial chylomicronemia-syndrome
FPL= familial partial lipodystrophy

PTC THERAPEUTICS™ 20 YEARS OF COMMITMENT

Cure SMA presentation showed motor muscle improvement and included video of 3 sitting SMN1 babies

CHOP-INTEND Score: Individual Patient Plots Show Continuous Improvement from Baseline



Median change from baseline in CHOP-INTEND score was:

	Aged 3 - 5 months	Aged 5 - 7 months	Overall
Day 56	**8.0** (n=6)	**4.5** (n=14)	**5.5** (n=20)
Day 119	**13.5** (n=4)	**11.0** (n=12)	**12.5** (n=16)
Day 182	**20.5** (n=2)	**11.0** (n=9)	**14.0** (n=11)

Updated SMA data including additional sitting babies expected at WMS in October

Intent-to-treat patients from FIREFISH Part 1. Data cut-off: 24th May 2018.
CHOP-INTEND. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders

PTC's value creation opportunities

- Establish Emflaza® as standard of care & grow Translarna™ globally
- Initiate launch activities for Tegsedi™ & Waylivra™ in Latin America
- SMA Firefish & Sunfish continued data for potential registration
- File BLA for AADC and IND for FA gene therapy programs
- Niche oncology programs enter clinical stage development
- Splicing platform development, Huntington's and FD to enter clinic