Translarna™ Study 045 and Clinical Update

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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 regarding: the future expectations, plans and prospects for PTC, including with respect to the commercialization of its products and product candidates; PTC's plans for interactions with the FDA; the clinical utility and potential advantages of Translarna; 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; PTC's ability 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 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; whether regulators will agree with PTC’s characterization of the results of PTC’s clinical trials including demonstration of meaningful clinical benefit of its products and product candidates; 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; 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.

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.

Study 045 Design*

Key Eligibility Criteria

- Ambulatory nmDMD pts aged ≥2 to <8 years

Enrollment

- 20 subjects enrolled (biopsies performed at UCLA Medical Center, Los Angeles, California)

Study Objectives

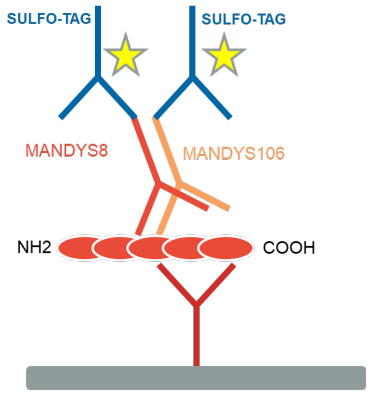
- Primary
  - Change from baseline to Week 40 in dystrophin expression, electrochemiluminescence (ECL) assay
- Secondary
  - Change from baseline to Week 40 in dystrophin expression, immunohistochemistry (IHC) assay

*Designed specifically for FDA regulatory process

Analytical Methods – ECL and IHC Assays

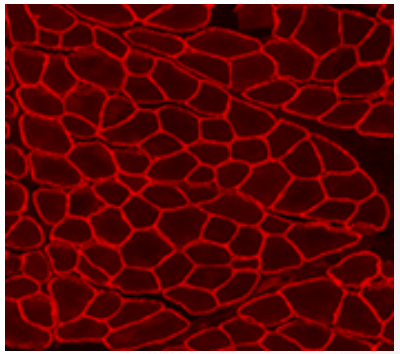
ECL Assay

- Quantitative measurement of total dystrophin protein levels
- Utilizes electrochemiluminescence technology from Meso Scale Diagnostics



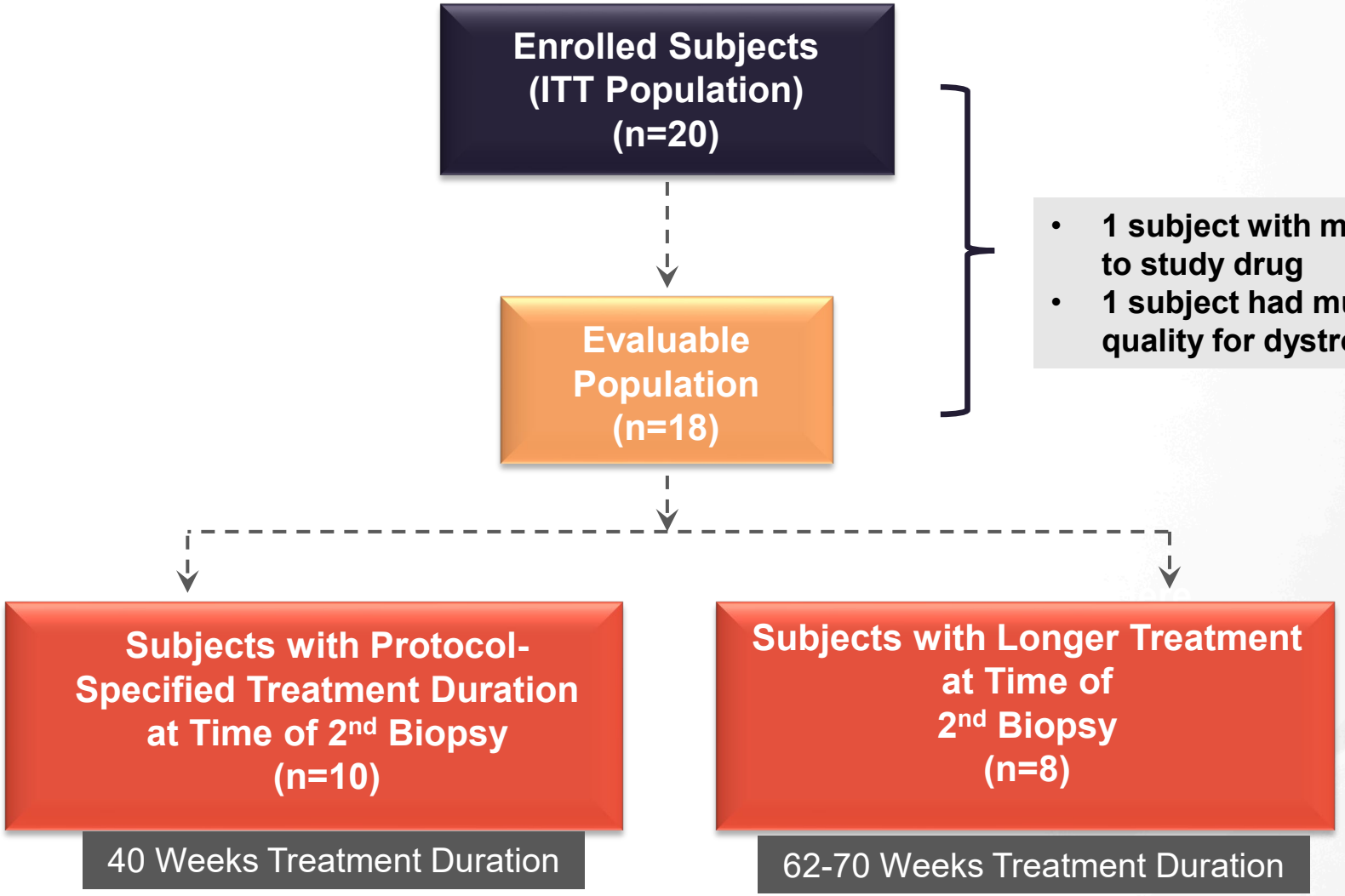
IHC Assay

- Provides quantifiable measurement of dystrophin localized to the membrane of the muscle cell (consistent with a functional protein)
- Utilizes immunohistochemistry to assess dystrophin protein levels



- ECL and IHC assays are fully validated and were reviewed by the FDA
- Both assays are sensitive at low levels of dystrophin

Study Population Summary



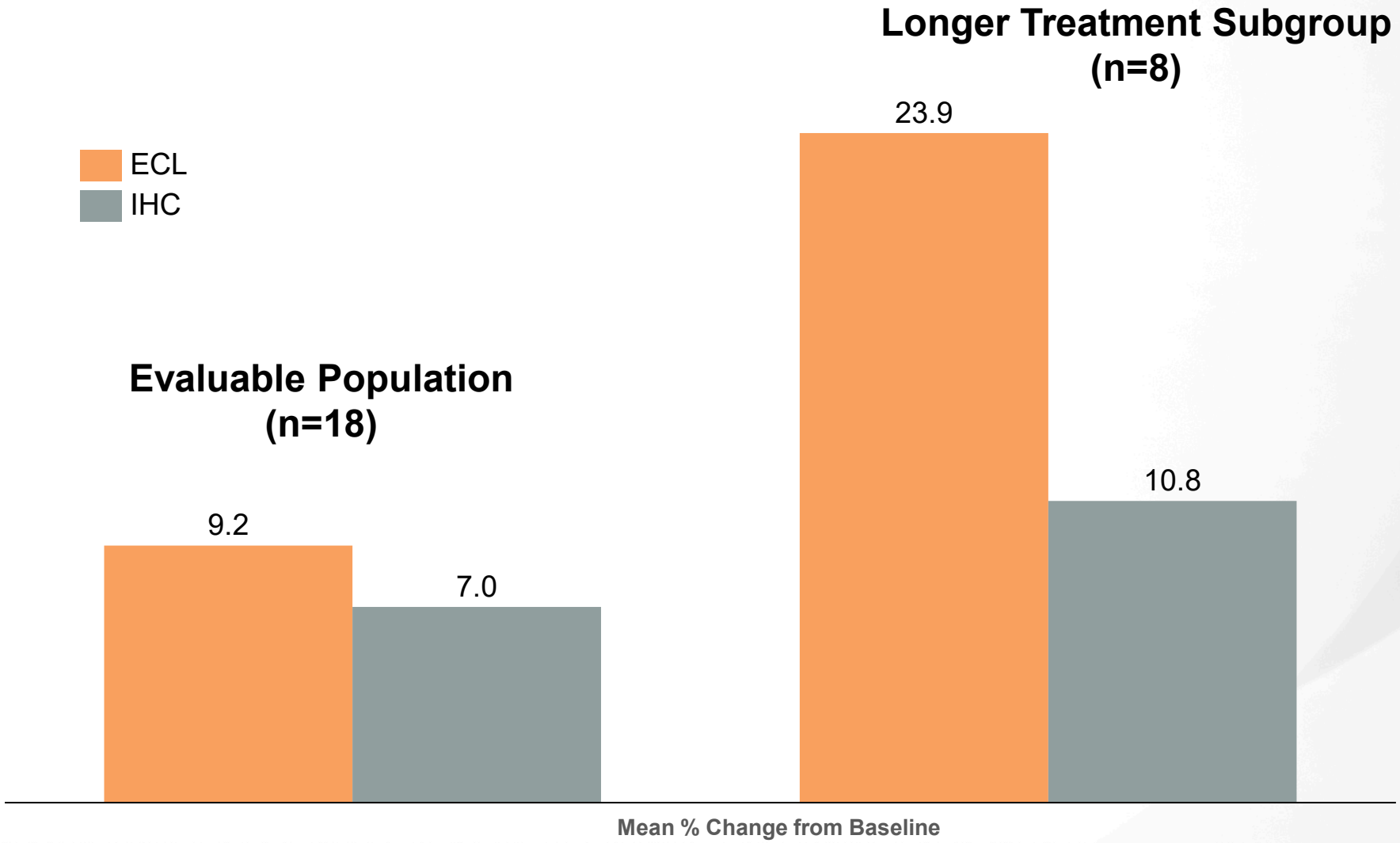
Translarna™ Treatment Resulted in Increased Dystrophin Expression on both ECL and IHC Assays

Mean % Change from Baseline			
ITT Population (n=20)		Evaluable Population (n=18)	
ECL Assay	IHC Assay	ECL Assay*	IHC Assay*
6.56% (p=0.24)	4.91% (p=0.11)	9.20% (p=0.19)	7.00% (p=0.04)

Primary endpoint – ECL Assay ITT Population
Secondary endpoint – IHC Assay ITT Population

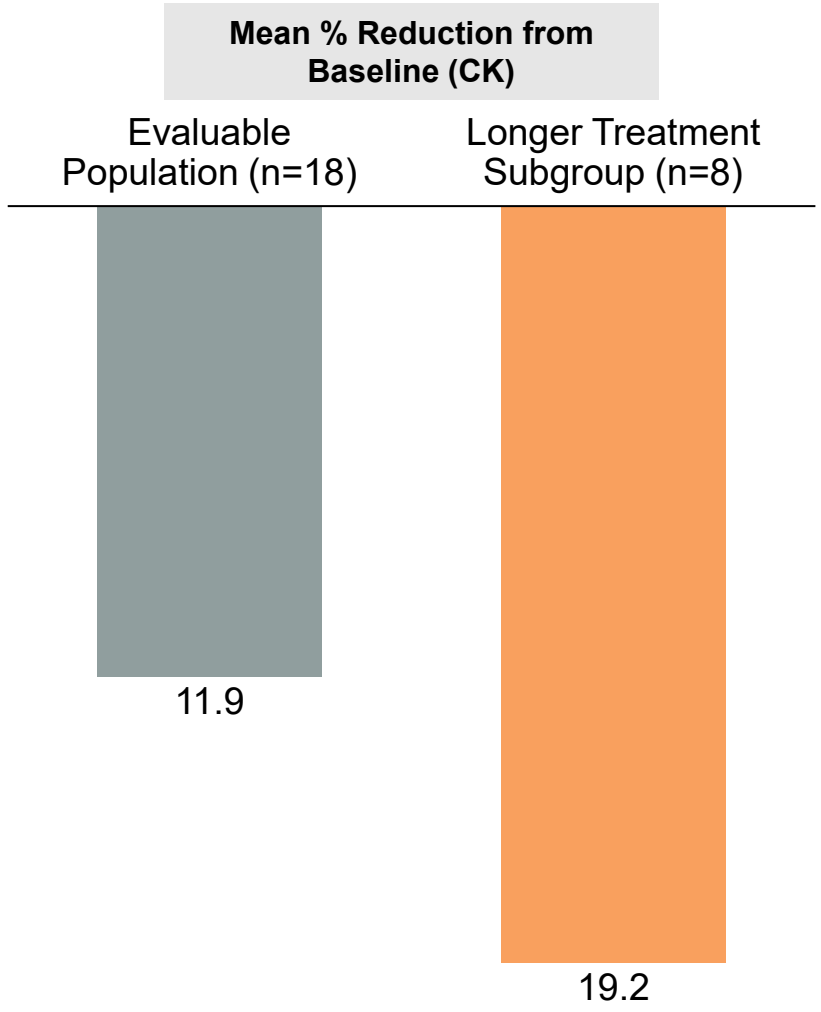
*Exploratory; not adjusted for multiplicity

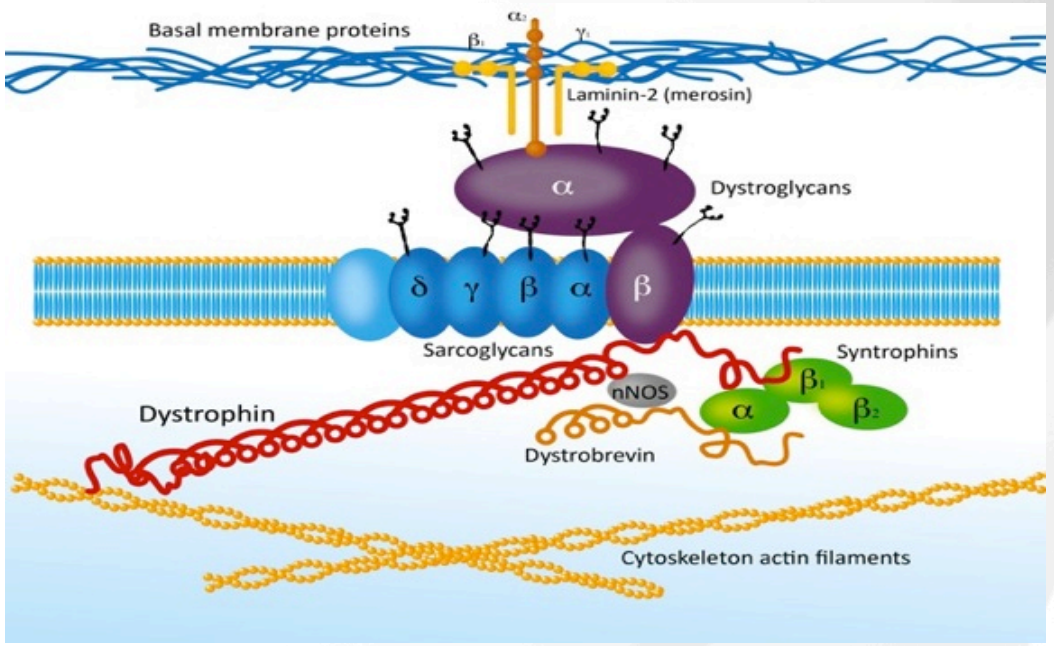
Longer Treatment Duration Resulted in Greater Dystrophin Expression Increase



Exploratory; not adjusted for multiplicity

Translarna Treatment Resulted in Reduction in Creatine Kinase (CK) Levels



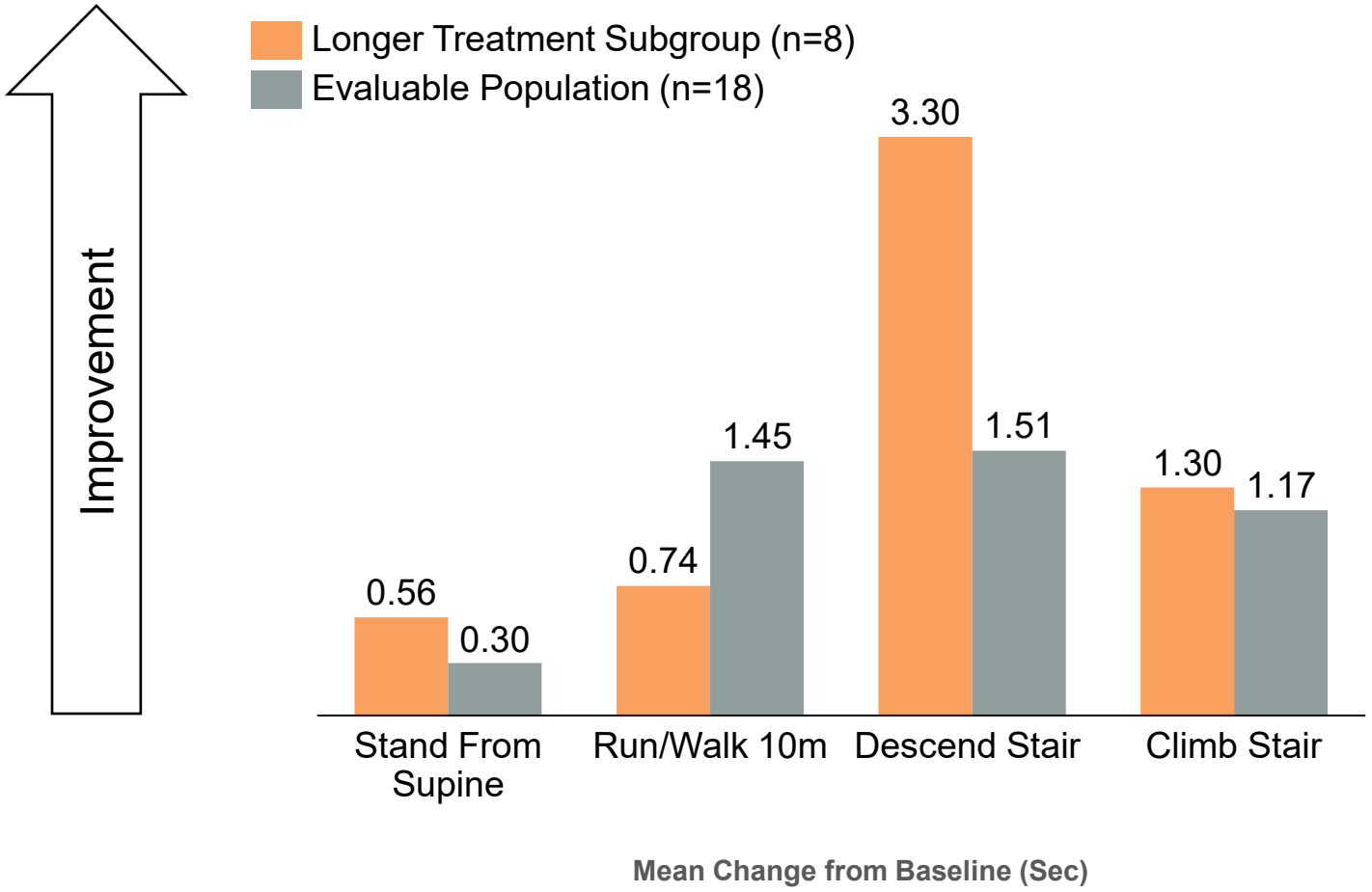


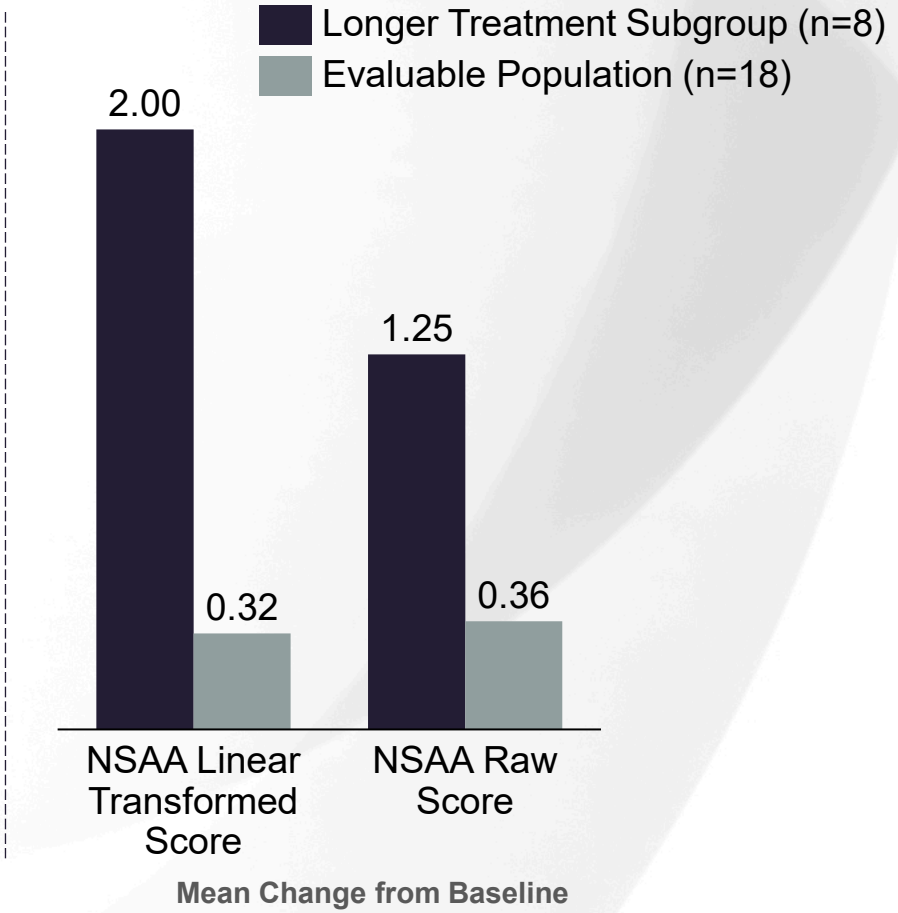
Adapted from Goemans N, *et al.* 2014[4]

Exploratory; not adjusted for multiplicity

Translarna Treatment Resulted in Clinical Improvement

Timed Function Tests (TFTs) and North Star Ambulatory Assessment





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Study 045 Trends Support Translarna Benefit

Increased Dystrophin Expression

Translarna treatment resulted in increased dystrophin expression as assessed by both ECL and IHC assays

Longer Treatment Greater Increase

Longer treatment with Translarna was associated with a greater increase in dystrophin expression

Improvement in CK and Clinical Measures

Translarna treatment resulted in a reduction in CK levels and an improvement in all clinical measures

Exploratory; not adjusted for multiplicity

Study 045 Adds to Existing Totality of Evidence Supporting Clinical Benefit of Translarna

Study 004 (Dystrophin Study)
28 days
N=38

Production of full-length dystrophin

Study 007 (RCT)
48 weeks
N=174

Improvement across multiple clinical endpoints

Study 020 (RCT)
48 weeks
N=228

Improvement across multiple clinical endpoints

Study 019 (Long-term Study)
~3.5 years*
N=94

Preservation of pulmonary function

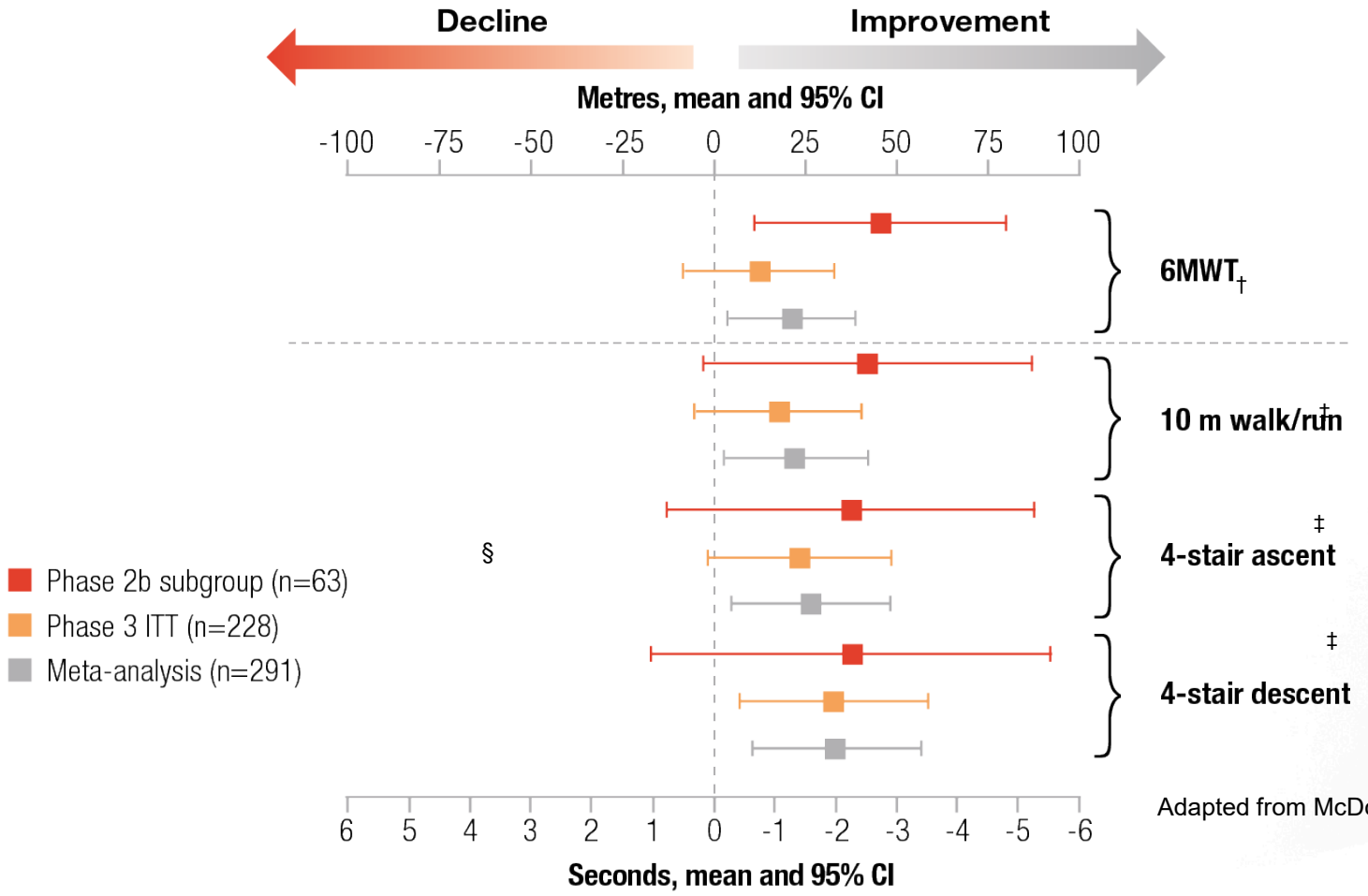
Study 025 (STRIDE Registry)
~5 years
N=250

Long term preservation of ambulation

Long term preservation of pulmonary function

*Median exposure

Totality of Evidence Shows Consistent Treatment Effect for Translarna Across Multiple Clinical Endpoints



Meta-analysis LS mean difference (SEM)

6MWT†	21.1 (9.0) m
10 m walk/run‡	–1.4 (0.6) s
4-stair ascent‡	–1.6 (0.7) s
4-stair descent‡	–2.0 (0.7) s

Adapted from McDonald CM, *et al*. 2017[1]

Phase 2b and 3 studies did not meet their primary endpoints. *In children aged ≥7 to ≤16 years old. † Primary endpoint. ‡ Secondary endpoint. § Patients from the Phase 2b study that met ACT DMD inclusion criteria. The meta-analysis included patients from the Phase 2b subgroup matching the entry criteria for the Phase 3 trial. 6MWD, 6-minute walk distance; CI, confidence interval; LS, least-squares; SEM, standard error of the mean. 1. McDonald CM, *et al. Lancet*. 2017;390:1489–1498.

STRIDE Registry Provides Long-term Real-world Evidence of Translarna Benefit

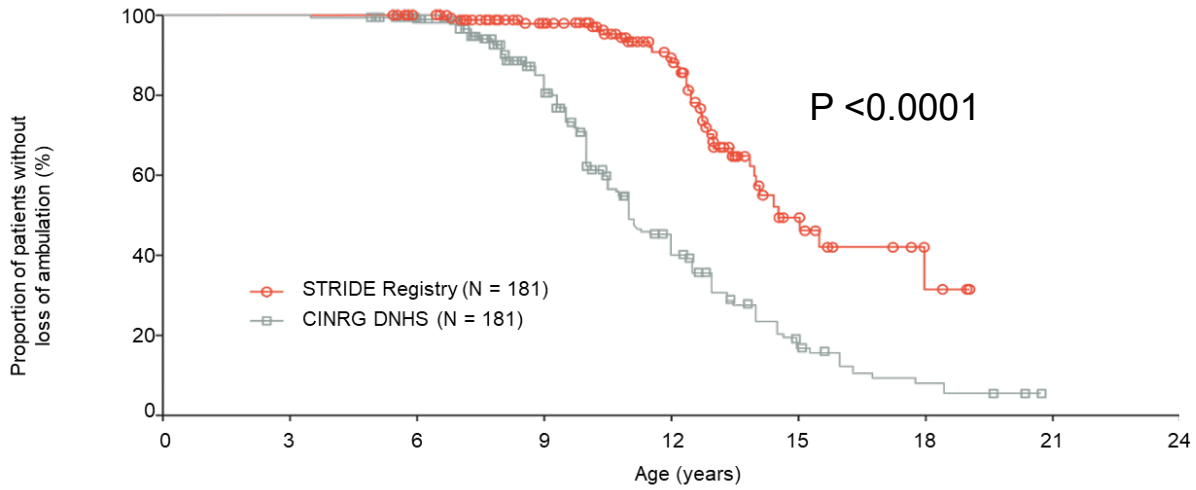
Multicenter registry study, evaluating the long-term safety and efficacy in a real-world setting

Includes nmDMD patients with varied demographics, providing data that is representative of real-world patient experiences

Over 250 patients being followed for 5 years and over 1000 patient years of experience

STRIDE Data Demonstrates Translarna Preserves Ambulation and Pulmonary Function

Delay of loss of ambulation by 3.5 years



Prevention of loss of pulmonary function

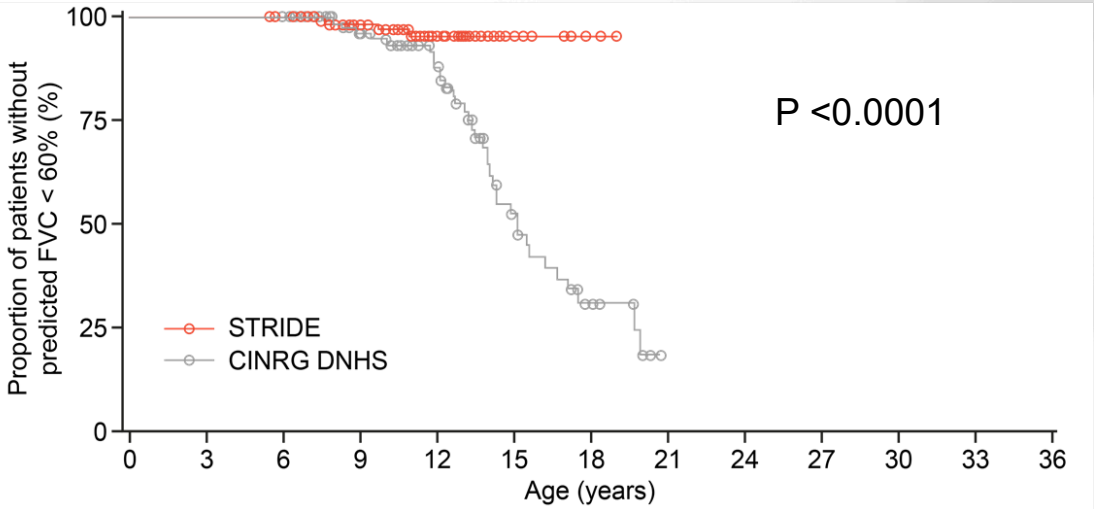


Figure adapted from Mercuri E, et al. *J Comp Eff Res*. 2020; 9(5):341-360.
Data cut off 9 July 2018

Next Steps: Discussion with FDA on Potential Approval Pathways

Biological Results

Increased full-length dystrophin production

Clinical Results

Clinical benefit across multiple endpoints in several placebo-controlled trials

Real-World Evidence

STRIDE registry of long-term clinical benefit on key aspects of disease progression

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Ongoing Clinical Study

Study 041
Results 3Q 2022