# 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): **February 4, 2021**

# 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**<br>**South Plainfield, NJ**<br>(Address of Principal Executive Offices) | **07080**<br>(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 February 4, 2021, PTC Therapeutics, Inc. (the "Company") issued a press release announcing that the Company will host a conference call on February 4, 2021 at 5:30 p.m. eastern time. During this call, the Company expects to review results from its clinical study 045 of Translarna™ (ataluren) in patients with nonsense mutation Duchenne muscular dystrophy.

Directions on how to access the conference call are included in the press release attached as Exhibit 99.1. A copy of the slide deck that will be presented on the conference call is attached as Exhibit 99.2

The information in this Current Report on Form 8-K, including Exhibits 99.1 and 99.2, 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 9.01. Financial Statements and Exhibits.**

(d) Exhibits

| Exhibit No. | Description |
|---|---|
| 99.1 | Press Release, dated February 4, 2021 issued by PTC Therapeutics, Inc. |
| 99.2 | Corporate Presentation – Clinical study 045 of Translarna in patients with nonsense mutation Duchenne muscular dystrophy |
| 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: February 4, 2021

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

# PTC Therapeutics to Host Call to Review Results from its Study of Translarna™ (ataluren) in Patients with Nonsense Mutation Duchenne Muscular Dystrophy

**SOUTH PLAINFIELD, N.J., Feb. 4, 2021** -- PTC Therapeutics, Inc. (NASDAQ: PTCT) will host a conference call today at 5:30 p.m. E.T. to review results from its clinical study 045 of Translarna™ (ataluren) in patients with nonsense mutation Duchenne muscular dystrophy.

The webinar can be accessed by dialing (877) 303-9216 (domestic) or (973) 935-8152 (international) five minutes prior to the start of the webinar and providing the passcode 3495760. A live, listen-only webcast can be accessed on the Events and Presentations page under the investor relations section of PTC Therapeutics' website at www.ptcbio.com. The accompanying slide presentation will be posted on the investor relations section of the PTC website. A webcast replay will be available approximately two hours after completion of the webinar and will be archived for 30 days following the webinar.

**About Duchenne muscular dystrophy**
Primarily affecting males, Duchenne muscular dystrophy (Duchenne) is a rare and fatal genetic disorder that results in progressive muscle weakness from early childhood and leads to premature death in the mid-twenties due to heart and respiratory failure. It is a progressive muscle disorder caused by the lack of functional dystrophin protein. Dystrophin is critical to the structural stability of all muscles, including skeletal, diaphragm, and heart muscles. Patients with Duchenne can lose the ability to walk (loss of ambulation) as early as age ten, followed by loss of the use of their arms. Duchenne patients subsequently experience life-threatening lung complications, requiring the need for ventilation support, and heart complications in their late teens and twenties.

More information regarding Duchenne is available through the Muscular Dystrophy Association and the Parent Project Muscular Dystrophy. Additionally, information and resources are available at www.duchenneandyou.com.

**About Translarna™ (ataluren)**
Translarna (ataluren), discovered and developed by PTC Therapeutics, Inc., is a protein restoration therapy designed to enable the formation of a functioning protein in patients with genetic disorders caused by a nonsense mutation. A nonsense mutation is an alteration in the genetic code that prematurely halts the synthesis of an essential protein. The resulting disorder is determined by which protein cannot be expressed in its entirety and is no longer functional, such as dystrophin in Duchenne muscular dystrophy. Translarna, the tradename of ataluren, is licensed in the European Economic Area for the treatment of nonsense mutation Duchenne muscular dystrophy in ambulatory patients aged two years and older. Ataluren is an investigational new drug in the United States.

**About PTC Therapeutics, Inc.**
PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and

commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need.

**For More Information:**

**Media & Investors:**

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Translarna™
Study 045 and
Clinical Update
Stuart W. Peltz, Ph.D.
Chief Executive Officer

# 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 update 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, 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**

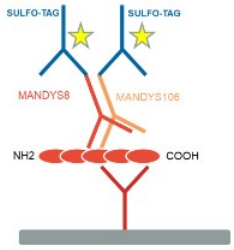
- Primary
  - Change from baseline to Week 40 in dystrophin expression, electrochemiluminescence (ECL) assay
- Secondary
  - Change from baseline to Week 40 in dystrophin expression, immunohistochemistry (IHC) assay

*Designed specifically for FDA regulatory process

# Analytical Methods – ECL and IHC Assays

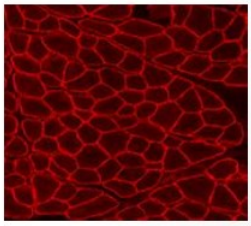
## ECL Assay

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



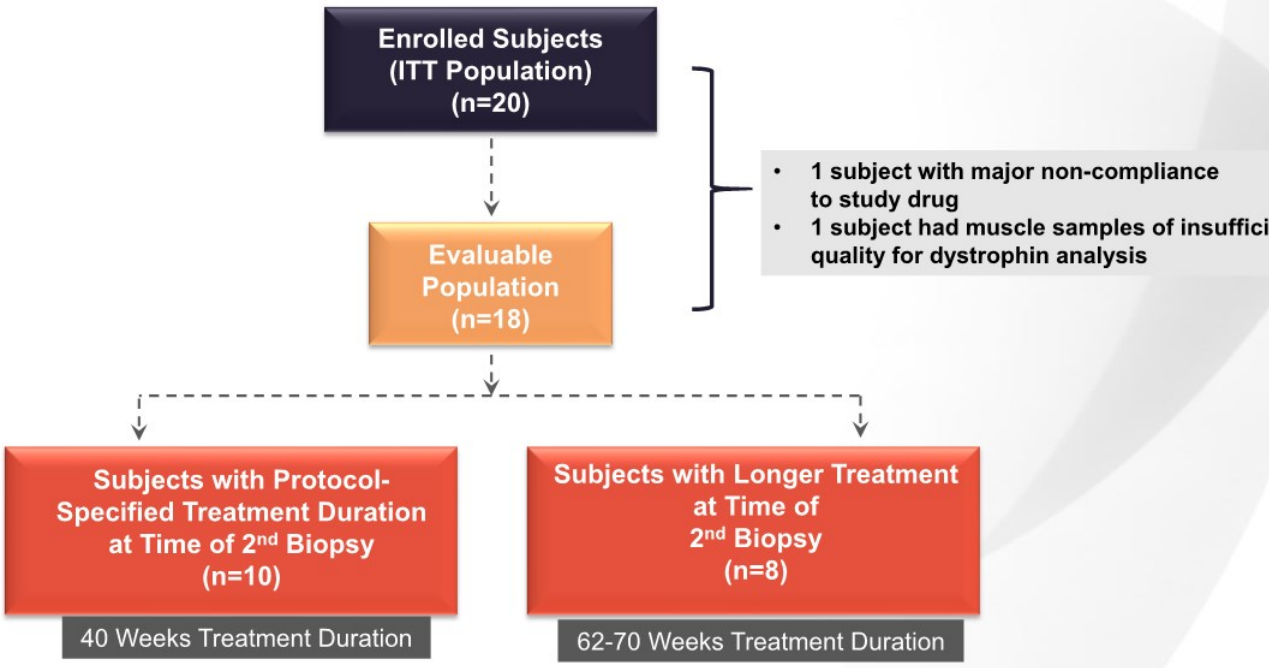

## IHC Assay

- 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



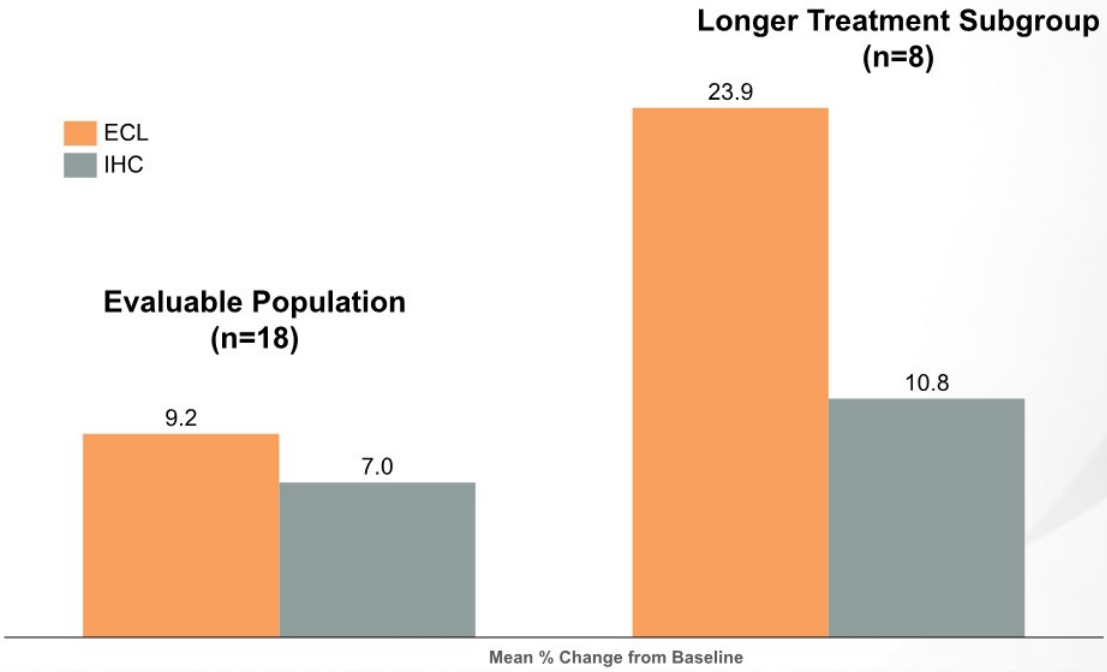
# Translarna™ Treatment Resulted in Increased Dystrophin Expression on both ECL and IHC Assays

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

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

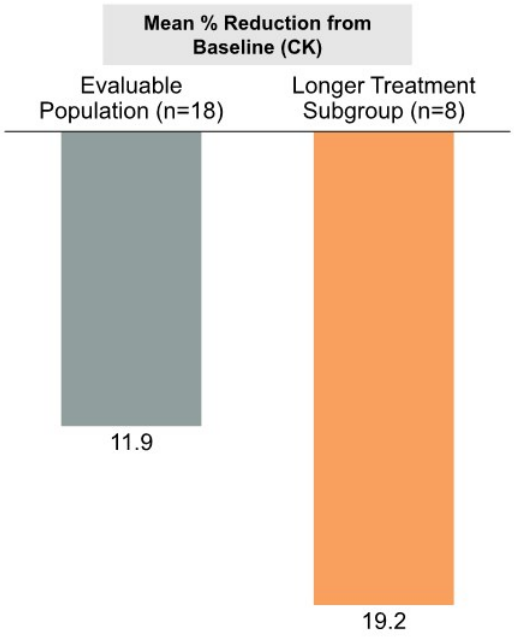

*Exploratory; not adjusted for multiplicity

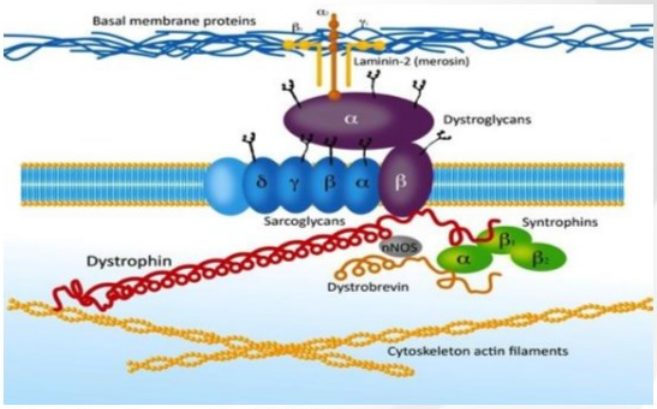
# Longer Treatment Duration Resulted in Greater Dystrophin Expression Increase




7 Exploratory; not adjusted for multiplicity

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



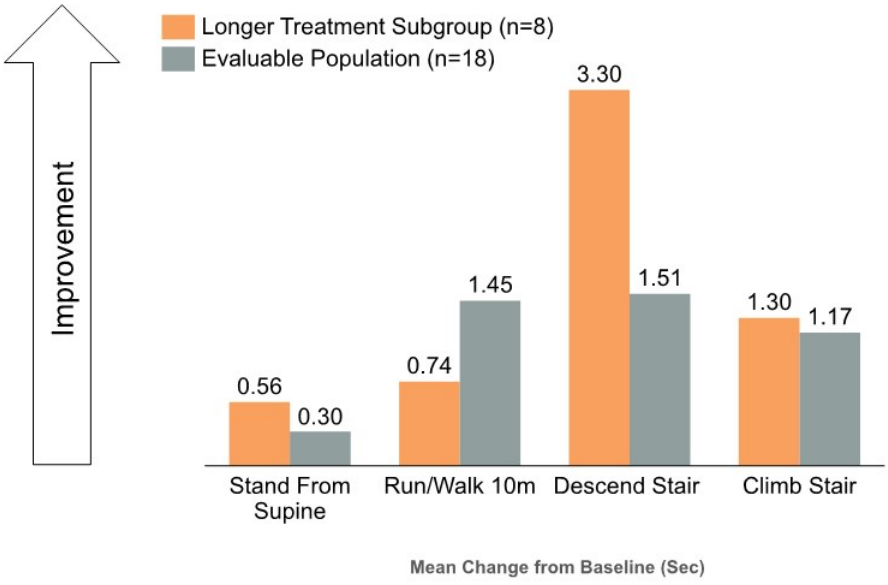

Adapted from Goemans N, *et al.* 2014[4]



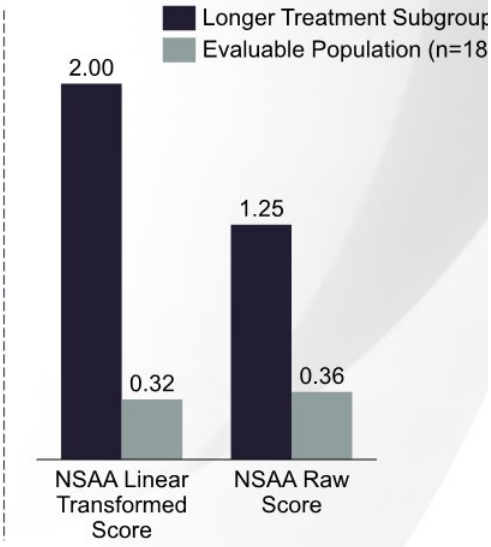
Exploratory; not adjusted for multiplicity

# Translarna Treatment Resulted in Clinical Improvement

**Timed Function Tests (TFTs) and North Star Ambulatory Assessment**




Mean Change from Baseline (Sec)




Mean Change from Baseline



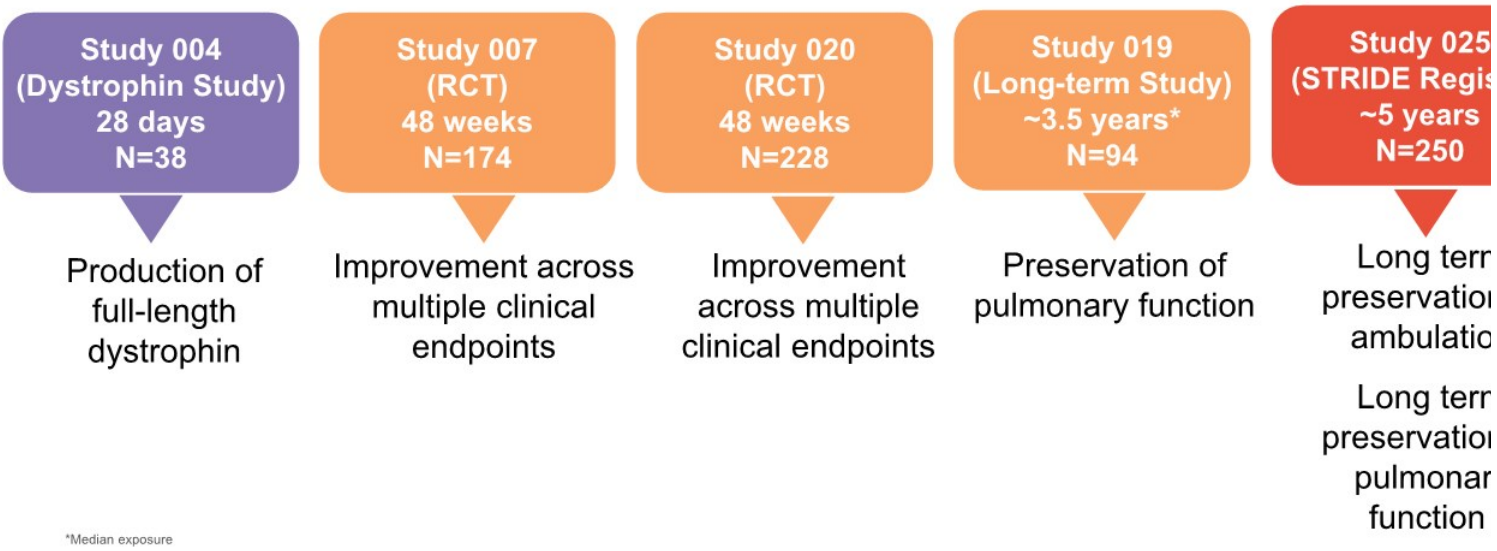
Exploratory; not adjusted for multiplicity

# Study 045 Trends Support Translarna Benefit

| Increased Dystrophin Expression | Longer Treatment Greater Increase | Improvement in CK and Clinical Measures |
|---|---|---|
| Translarna treatment resulted in increased dystrophin expression as assessed by both ECL and IHC assays | Longer treatment with Translarna was associated with a greater increase in dystrophin expression | Translarna treatment resulted in a reduction in CK levels and an improvement in all clinical measures |

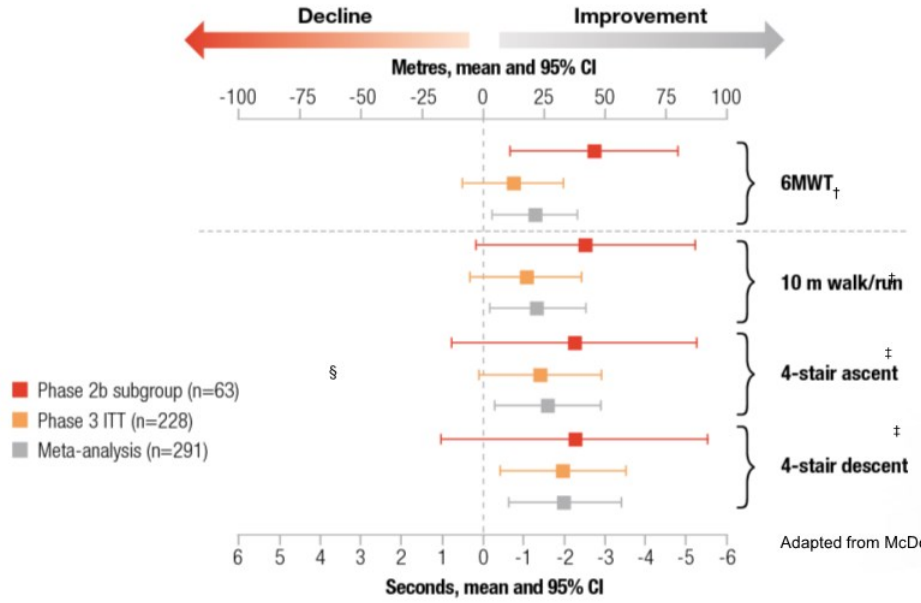
 Exploratory; not adjusted for multiplicity

# Study 045 Adds to Existing Totality of Evidence Supportin Clinical Benefit of Translarna




*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

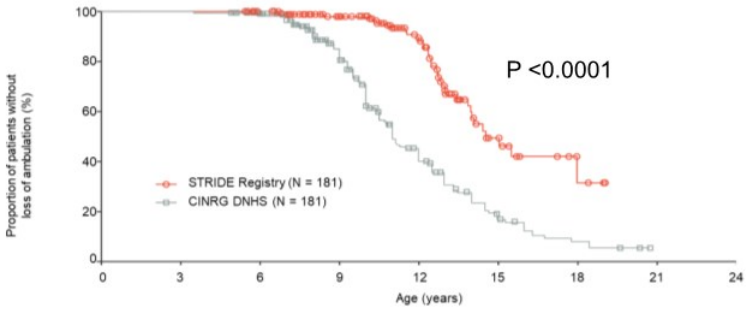
Multicenter registry study, evaluating the long-term safety and efficacy in a real-world setting


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

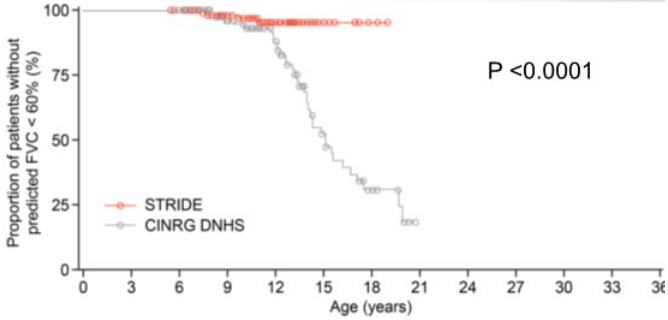





Figure adapted from Mercuri E, et al. *J Comp Eff Res*. 2020; 9(5):341-360.
Data cut off 9 July 2018

# Next Steps: Discussion with FDA on Potential Approval Pathway

# Next Steps: Discussion with FDA on Potential Approval Pathway