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): **June 21, 2022**

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 South Plainfield, NJ	**07080**
(Address of Principal Executive Offices)	(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.

As previously announced, PTC Therapeutics, Inc. (the "Company") hosted a conference call on June 21, 2022 at 8:00 a.m. eastern time. During this conference call, the Company discussed the top-line results from the placebo-controlled trial of Study 041 for Translarna. A copy of the slide deck that was presented during the conference call is attached as Exhibit 99.1.

The information in this Current Report on Form 8-K ("Report") (including Item 7.01 and Exhibit 99.1) 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. All website addresses given in this Report or incorporated herein by reference are for information only and are not intended to be an active link or to incorporate any website information into this Report.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Corporate Presentation – Study 041 Update
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: June 21, 2022

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


Translarna™
(Ataluren)
Study 041
Topline
Results
21st June 2022
PTC

Forward looking statements

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 EMA and 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 maintain its marketing authorization of Translarna for the treatment of nmDMD in Brazil, Russia, the European Economic Area (EEA) and other regions, 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 ability to utilize results from Study 041 to support a marketing approval for Translarna for the treatment of nmDMD in the United States; whether investigators agree with PTC's interpretation of the results of clinical trials and the totality of clinical data from our trials in Translarna; 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 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.

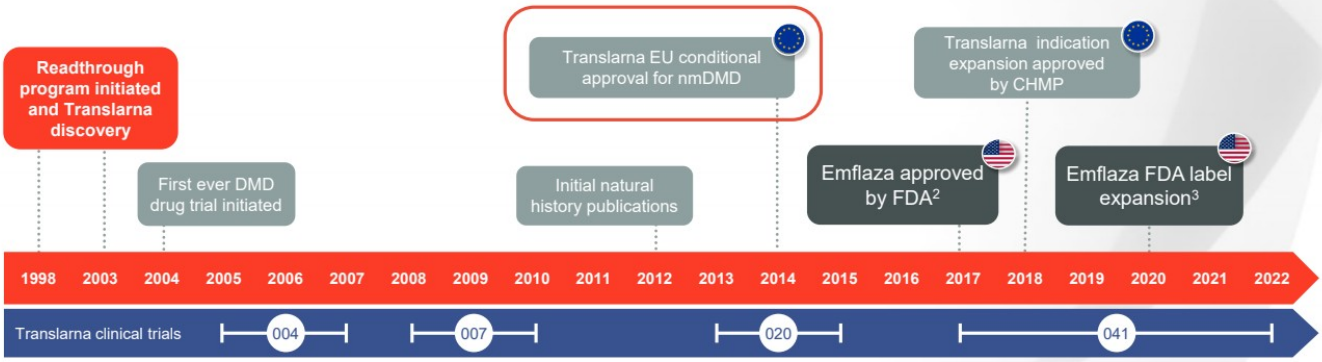
The forward-looking statements contained herein represent PTC's views only as of the date of this presentation and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this presenation except as required by law.

Agenda

1. Overview of DMD and Translarna
2. STRIDE Registry
3. Study 041 Topline Results

Overview of DMD and Translarna

PTC has pioneered development of therapies for DMD patients for 25 years[1]



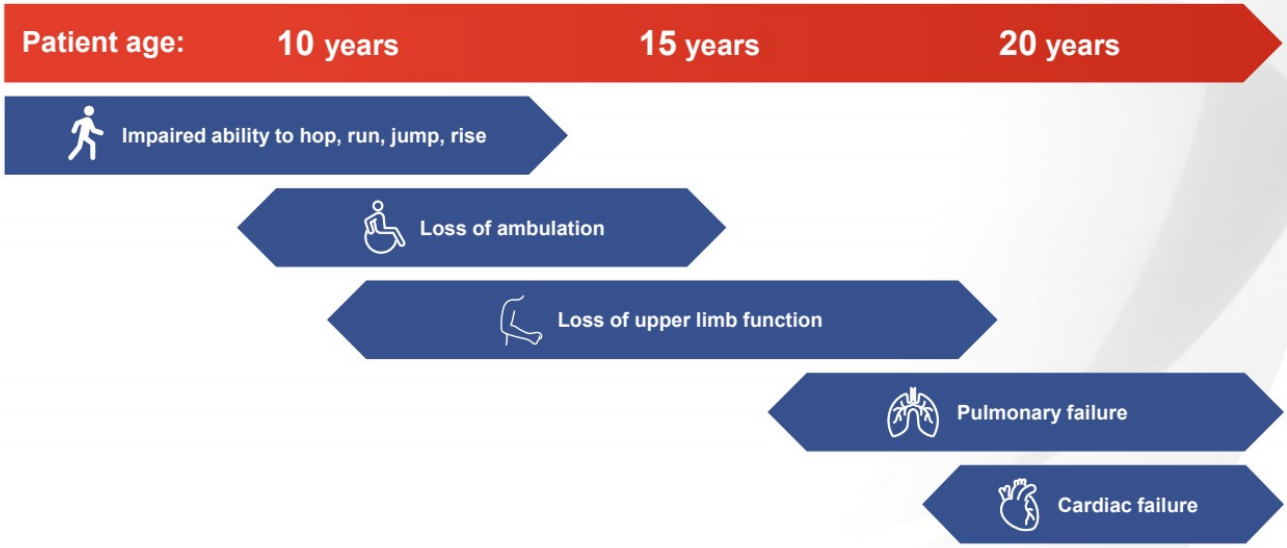
CHMP, Committee for Medicinal Products for Human Use; DMD, Duchenne muscular dystrophy; FDA, Food and Drug Administration; nmDMD, nonsense mutation Duchenne muscular dystrophy. 1. Data on file. PTC Therapeutics. South Plainfield, NJ. November 2020; 2. Drugs.com [Internet]. Available from: https://www.drugs.com/nda/emflaza_170420.html (Accessed May 2022); 3. Drugs.com [Internet]. Approval for the label expansion of deflazacort, c2019. Available from: https://www.drugs.com/newdrugs/ptc-therapeutics-receives-fda-approval-expansion-emflaza-deflazacort-labeling-include-patients-2-5-4989.html (Accessed May 2022)

DMD is a relentlessly progressive, fatal, genetic disorder

- Debilitating X-linked genetic disorder leading to death in early adulthood
- 1 in every 3,500-5,000 live male births with ~13% caused by nonsense mutations[1-7]
- Caused by the lack of dystrophin protein due to mutations that prevent its production

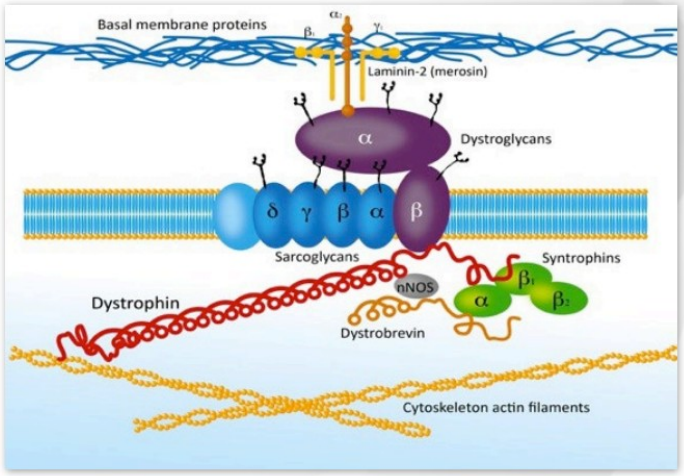
1. van Ruiten HJ,et al.Arch Dis Child. 2014;99:1074–1077. 2. Bushby K, et al. Lancet Neurol. 2010;9:77–93. 3. Aartsma-Rus A, et al. J Med Genet. 2016;53(3):145–151. 4. Crisafulli S, et al. Orphanet J Rare Dis. 2020;15(1):141
5. Pichavant C, et al. Mol Ther. 2011;19:830–840. 6. Kalman L, et al. J Mol Diagn. 2011;13:167–174. 7. Bladen CL, et al. Hum Mutat. 2015;36:395–402

DMD follows well-established clinical progression



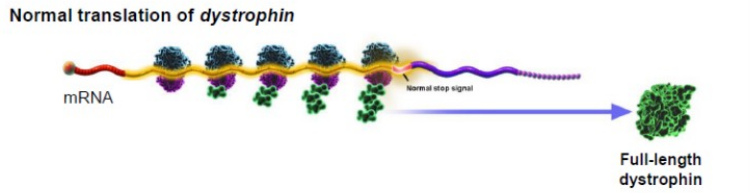
DMD pathology results from lack of full-length dystrophin

- Dystrophin connects the cytoskeleton to extracellular matrix and is critical during muscle contractions
- Muscles lacking dystrophin are more susceptible to damage
- Damaged dystrophin is replaced by fatty and connective tissue
- Dystrophin restoration is expected to prevent muscle damage



Goemans, Nathalie et al. "New Perspectives in the Management of Duchenne Muscular Dystrophy." European neurological review 9 (2014): 78.

Translarna enables the production of full-length, functional dystrophin



- mRNA, messenger ribonucleic acid. Peltz SW, *et al. Annu Rev Med*. 2013;64:407–425
- Translarna, Summary of Product Characteristics.
- Haas M *et al. Neuromuscul Disord*. 2015;25:5–13.

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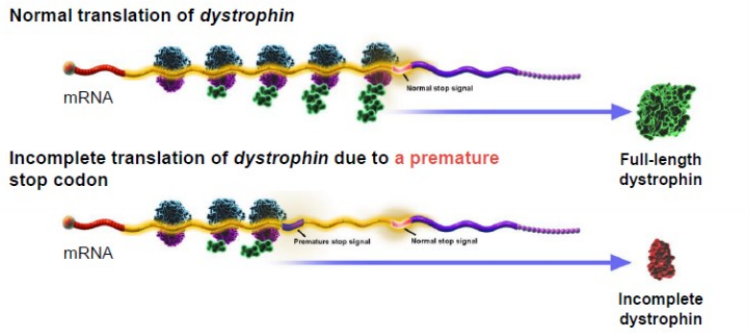

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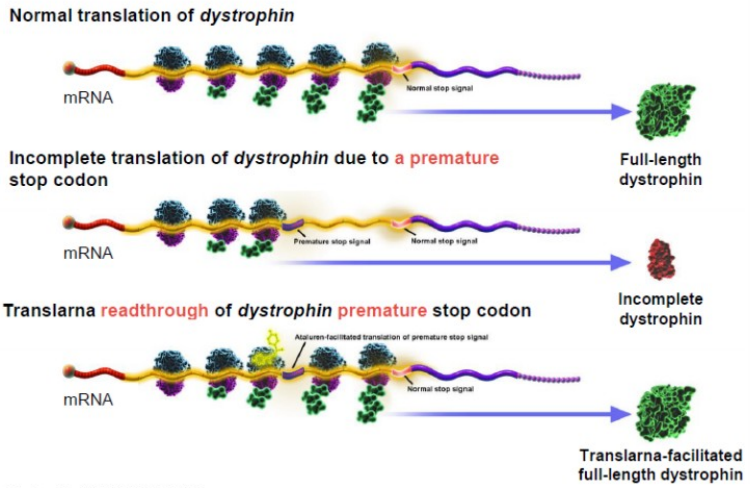

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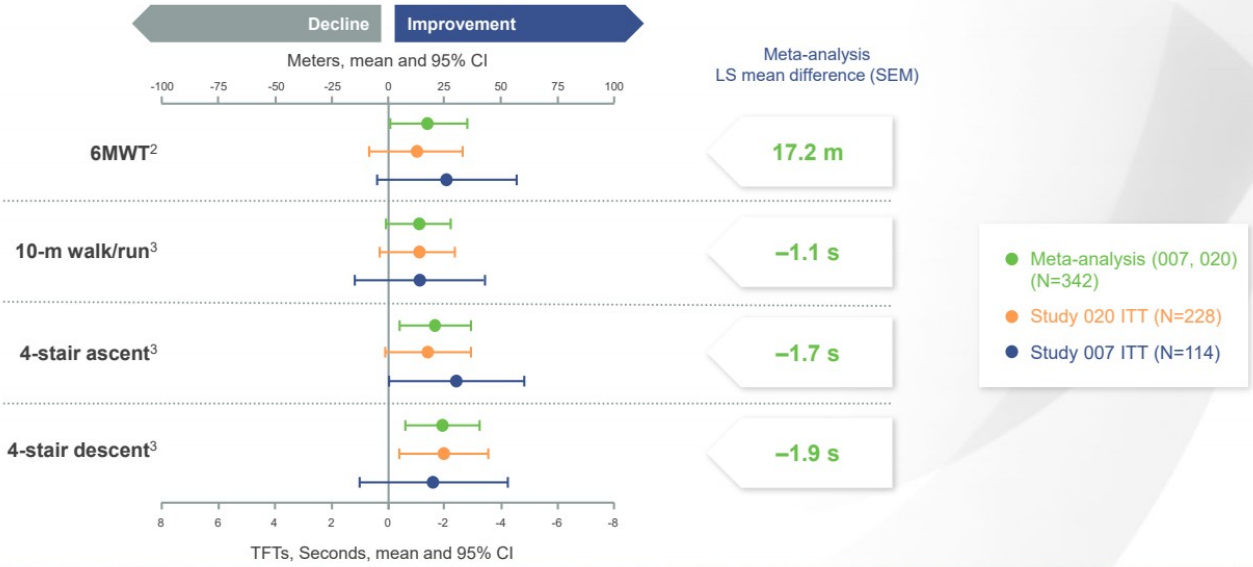
Translarna demonstrated consistent treatment effect across multiple clinical endpoints in previous trials[1]



1. Campbell, *et al. Journal of Comparative Effectiveness Research.* 2020; 9(14), 973–984. **Meta-analyses of ataluren randomized controlled trials in nonsense mutation Duchenne muscular dystrophy.** 2. Primary endpoint. 3. Secondary endpoint.

STRIDE Registry

STRIDE registry captures real-world evidence of Translarna benefit

- Patient registry collecting **long-term real-world evidence**
- Collecting information on the **characteristics of large numbers of patients** receiving Translarna with nmDMD

Aims to understand:

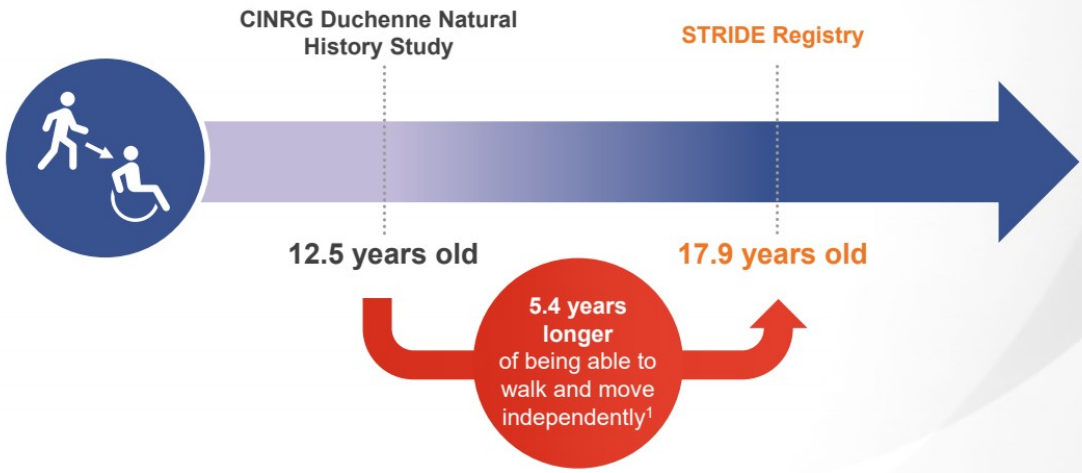
- **the safety profile of Translarna** over a long period of time
- how **effective long-term treatment** is in **preserving the ability to walk**, move independently, as well as lung and heart function

- STRIDE includes data from:[1,2]
  - **13** countries
  - **64** sites
  - **288** patients

Patients in the STRIDE Registry are followed up for at least 5 years to monitor disease progression

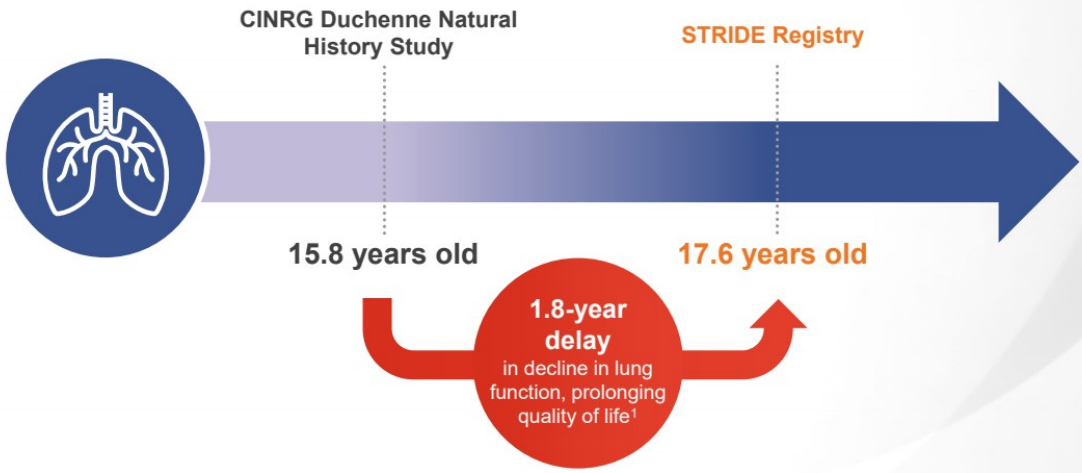
STRIDE, Strategic Targeting of Registries and International Database of Excellence
NCT02369731. 1. Muntoni F *et al. J Comp Eff Res* 2019;8:1187–200; 2. Muntoni F *et al.* Poster presented at the 14th European Paediatric Neurology Society Congress, 28 April – 2 May 2022, Glasgow

STRIDE registry demonstrates Translarna treatment significantly preserves ambulation



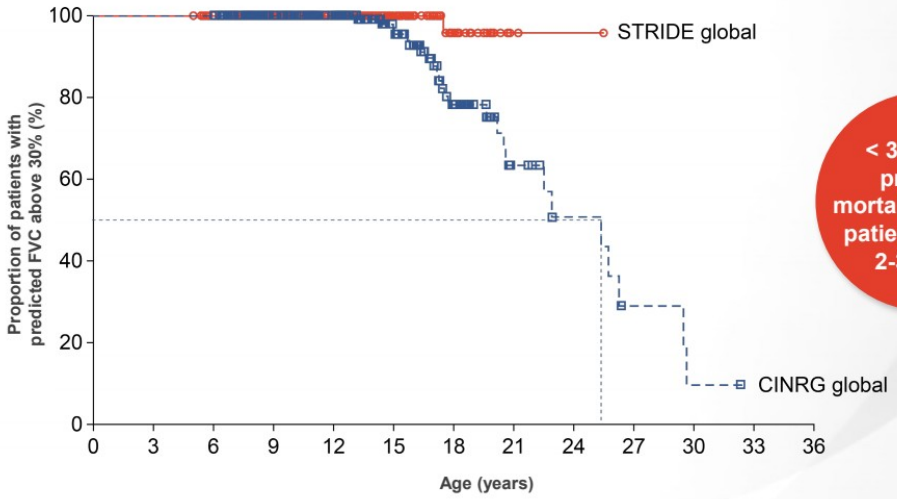
[a]The median (95% CI) ages at loss of ambulation were: 17.9 (14.4, NA) years for STRIDE and 12.5 (11.6, 13.5) years for the CINRG DNHS; p < 0.0001.
CINRG DNHS, Cooperative International Neuromuscular Research Group Duchenne Natural History Study;
1. Mercuri E *et al*. Poster presented at the 14th European Paediatric Neurology Society Congress, 28 April – 2 May 2022, Glasgow

STRIDE registry demonstrates Translarna treatment significantly preserves pulmonary function



[a]The median (95% CI) ages at predicted FVC < 60% were: 17.6 (16.2, NA) years for STRIDE and 15.8 (15.1, 16.5) years for the CINRG DNHS; p < 0.0051
CINRG DNHS, Cooperative International Neuromuscular Research Group Duchenne Natural History Study;
1. Tulinius M et al. Poster presented at the 14th European Paediatric Neurology Society Congress, 28 April – 2 May 2022, Glasgow

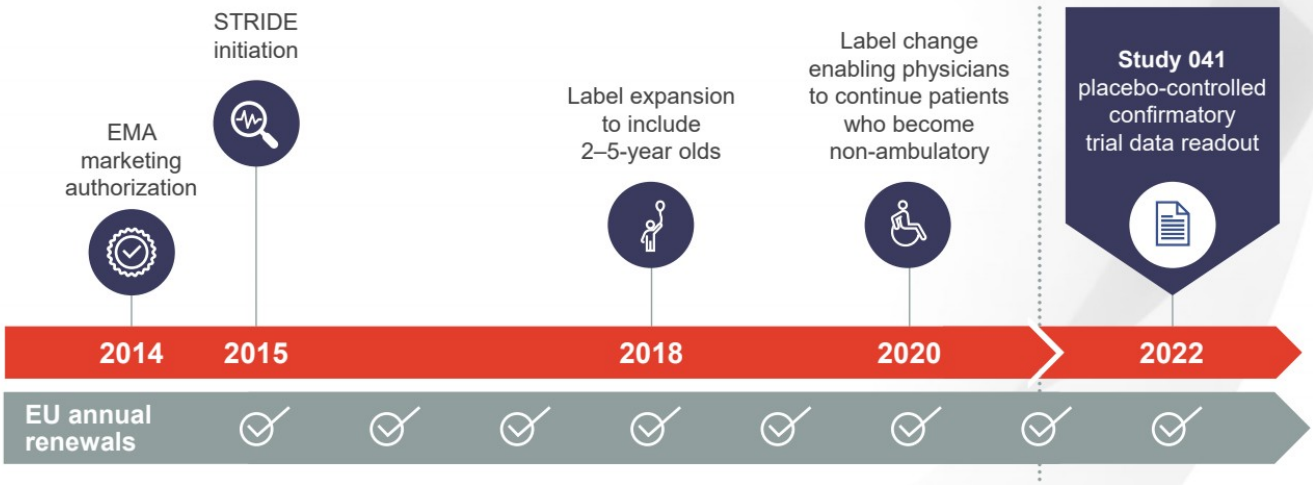
Translarna protects against loss of pulmonary function



< 30% FVC predicts mortality in DMD patients within 2-3 years

PTC Therapeutics, data on file

EMA regulatory history continues to support the positive risk-benefit of Translarna



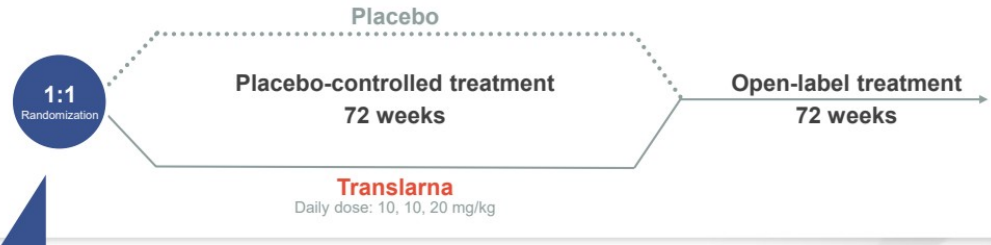
Study 041: Overview and study design

Eligibility Criteria

- Age ≥5 years old
- 6MWD ≥150 meters
- Stable steroid regimen

Key Outcome Measures

- Primary endpoint: 6MWT
- Secondary endpoints: Northstar Ambulatory Assessment (NSAA), Timed Function Tests (TFTs)



Stratification Factors

- Steroid use at baseline
- 6MWD at baseline
- Time to stand from supine at baseline

Broad population with comprehensive endpoints and 144 week trial duration

Study 041: Intent-to-Treat (ITT) population

Baseline Characteristic	Translarna (N=183)	Placebo (N=176)	Overall (N=359)
Mean age at enrollment, yrs [min, max]	8.1 [5.0, 14.0]	8.2 [5.0, 14.0]	8.1 [5.0, 14.0]
Baseline 6MWD, n (%)			
<300m	21 (11.5)	21 (11.9)	42 (11.7)
300 to <350m	40 (21.9)	37 (21.0)	77 (21.4)
350 to <400m	46 (25.1)	46 (26.1)	92 (25.6)
≥400m	76 (41.5)	72 (40.9)	148 (41.2)
Steroid use at baseline, n (%)	183 (100)	176 (100)	359 (100)
Deflazacort	79 (43.2)	73 (41.5)	152 (42.3)
prednisone/prednisolone	104 (56.8)	103 (58.5)	207 (57.7)
Baseline time to stand from supine, n (%)			
<5 seconds	62 (33.9)	55 (31.3)	117 (32.6)
≥5 seconds	121 (66.1)	121 (68.8)	242 (67.4)

Study 041: Subject disposition

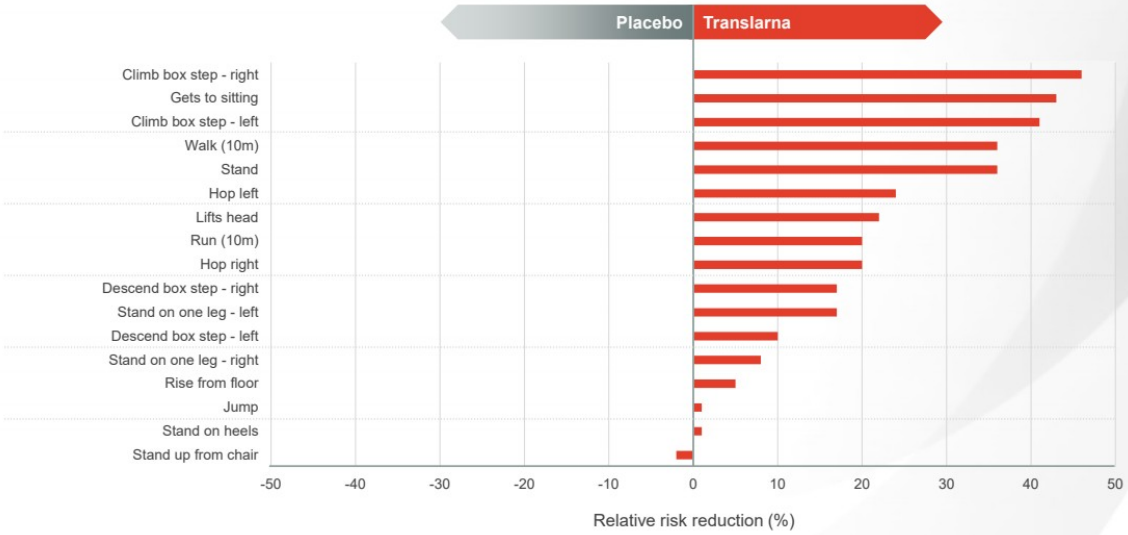
	ITT population	
	Translarna (N=183)	Placebo (N=176)
ITT Population	183	176
Completed placebo controlled portion (%)	179 (97.8)	172 (97.7)
Early termination (%)	4 (2.2)	4 (2.3)
Adverse event	0	0
COVID	0	0
Loss to follow-up	1 (0.5)	0
Withdrew consent	0	3 (1.7)
Protocol noncompliance	2 (1.1)	0
Other	1 (0.5)	1(0.6)

Translarna treatment resulted in significant benefit across key endpoints in ITT population

Endpoint Assessment (Change from baseline)	Translarna (N=183)	Placebo (N=176)	Difference
6MWD (m)	-53.0	-67.4	**14.4 (p=0.0248)**
Rate of change (m/wk)	-0.74	-0.94	**0.20 (p=0.0248)**
Loss of ambulation, N (%)	12 (6.6%)	20 (11.4%)	--
NSAA			
Total score	-3.7	-4.5	**0.9 (p=0.0235)**
Linear score	-9.6	-11.9	**2.3 (p=0.0246)**
Timed function tests			
10m walk	3.04	3.82	**-0.78 (p=0.0422)**
Stair ascend	4.98	6.04	**-1.06 (p=0.0293)**
Stair descend	4.96	5.25	-0.29 (p=0.5749)

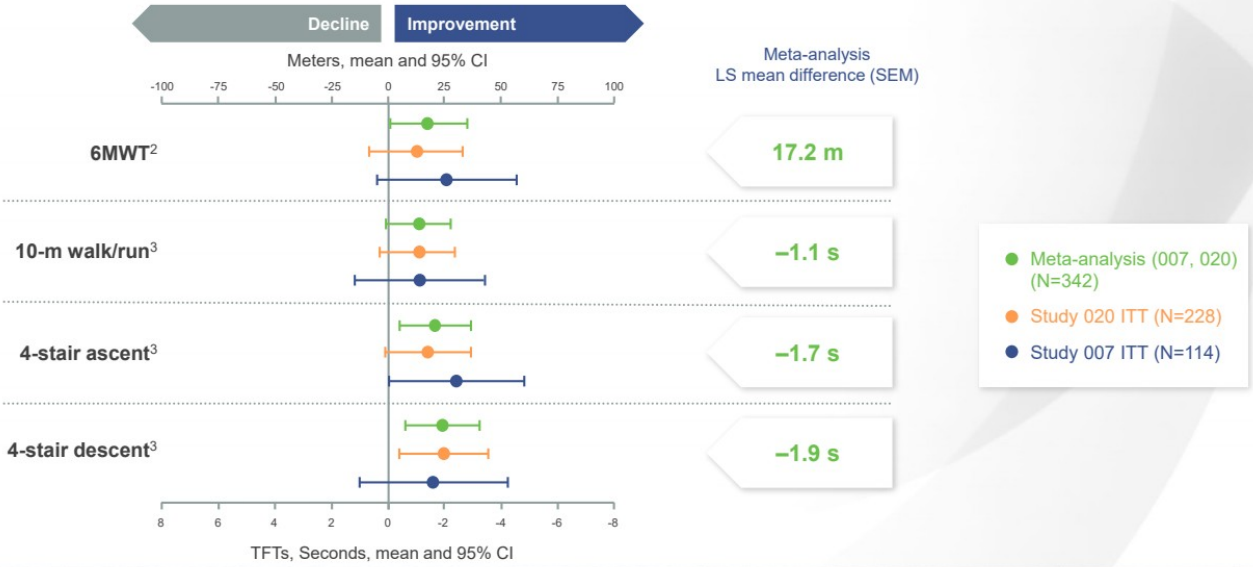
2 2 The ITT population was not the primary analysis population

NSAA results confirm Translarna treatment preserves functional abilities in ITT population



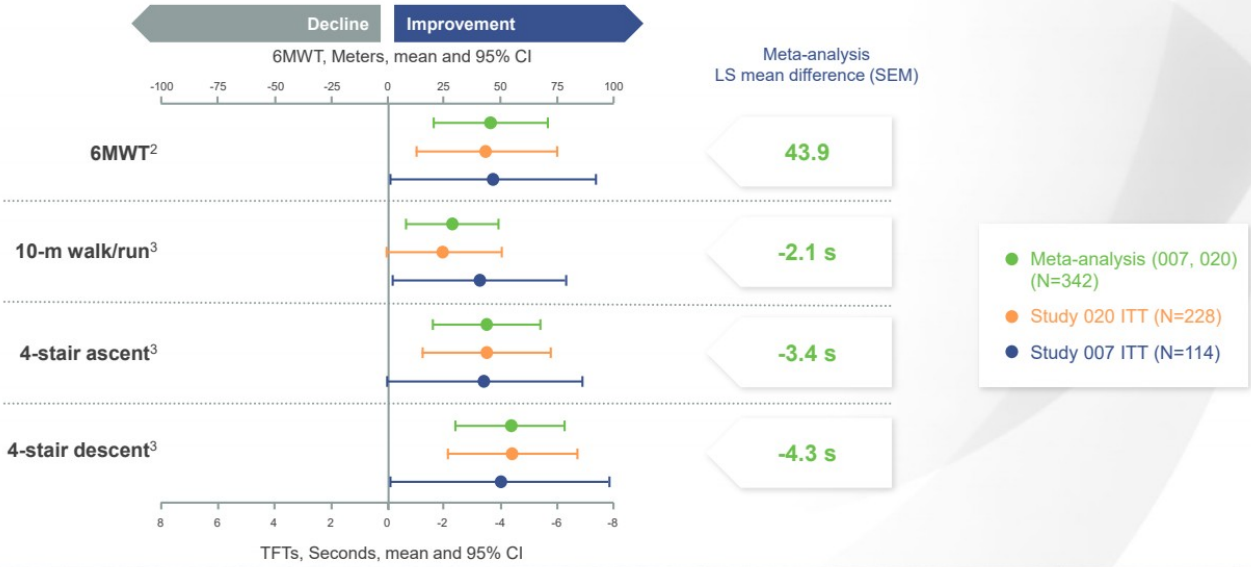
The ITT population was not the primary analysis population

Translarna demonstrated consistent treatment effect across multiple clinical endpoints in previous trials[1]



1. Campbell, *et al. Journal of Comparative Effectiveness Research.* 2020; 9(14). 973–984. **Meta-analyses of ataluren randomized controlled trials in nonsense mutation Duchenne muscular dystrophy.** 2. Primary endpoint. 3. Secondary endpoint.

Translarna demonstrated greatest effects in patients with baseline 6MWD of 300-400 meters in previous trials[1]

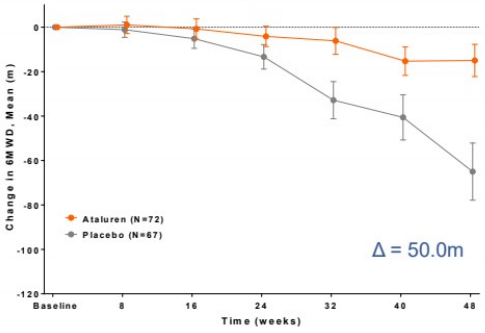


1. Campbell, *et al. Journal of Comparative Effectiveness Research*. 2020; 9(14), 973–984. **Meta-analyses of ataluren randomized controlled trials in nonsense mutation Duchenne muscular dystrophy**. 2. Primary endpoint. 3. Secondary endpoint.

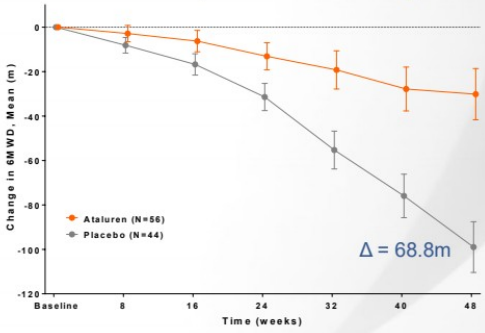
Clinically meaningful, significant treatment effects on 6MWT seen in subpopulations in previous trials

- 6MWD <400m (left panel) or stand from supine ≥5s (right panel) both exclude stable patients

≥300m 6MWD, <400m 6MWD



≥300m 6MWD, ≥5s stand from supine



Data from Studies 007 and 020

Primary analysis population for 041: ≥300m 6MWD, ≥5s stand from supine

Study 041: Subgroup analyses

	6MWD 300-400m		6MWD ≥300m and ≥5 sec supine to stand*	
Baseline Characteristic	**Translarna (N=86)**	**Placebo (N=83)**	**Translarna (N=92)**	**Placebo (N=93)**
Mean age at enrollment (yrs) [min, max]	8.1 [5, 14]	8.5 [5, 14]	8.9 [7, 14]	8.9 [6, 14]
Baseline 6MWD, n (%)				
<300m	0 (0)	0 (0)	0 (0)	0 (0)
300 to <350m	40 (46.5)	37 (44.6)	29 (31.5)	32 (34.4)
350 to <400m	46 (53.5)	46 (55.4)	33 (35.9)	31 (33.3)
>400m	0 (0)	0 (0)	30 (32.6)	30 (32.3)
Steroid use at baseline, n (%)	86 (100)	83 (100)	92 (100)	93 (100)
Deflazacort	37 (43.0)	35 (42.2)	41 (44.6)	41 (44.1)
prednisone/prednisolone	49 (57.0)	48 (57.8)	51 (55.4)	52 (55.9)
Baseline time to stand from supine, n (%)				
<5 seconds	16 (18.6)	15 (18.1)	0	0
≥5 seconds	70 (81.4)	68 (81.9)	92 (100)	93 (100)

*primary analysis population

Translarna treatment resulted in significant benefit in 300-400m subgroup

	6MWD 300m – 400m			6MWD 300m and ≥5 sec supine to stand*		
Endpoint Assessment	**Translarna (N=86)**	**Placebo (N=83)**	**Difference**	**Translarna (N=92)**	**Placebo (N=93)**	**Difference**
6MWD (m)						
Change from baseline	-55.8	-80.0	**24.2 (p=0.0310)**	-81.8	-90.1	8.3 (p=0.3626)
Rate of change (m/wk)	-0.77	-1.11	**0.34 (p=0.0310)**	-1.14	-1.25	0.11 (p=0.3626)
Loss of ambulation	5 (5.7%)	10 (12.0%)	--	5 (5.4%)	9 (9.7%)	--
NSAA						
Total score	-4.4	-5.5	1.1 (p=0.0837)	-5.2	-6.1	0.9 (p=0.1258)
Linear score	-10.0	-13.3	**3.3 (p=0.0419)**	-11.4	-14.0	2.5 (p=0.0656)
Timed function tests						
10m walk	2.99	4.28	**-1.29 (p=0.0429)**	3.06	3.79	-0.73 (p=0.1877)
Stair ascend	5.26	7.55	**-2.29 (p=0.0050)**	5.19	6.96	**-1.76 (p=0.0155)**
Stair descend	4.62	5.59	-0.97 (p=0.2714)	4.58	4.78	-0.19 (p=0.7997)

*primary analysis population

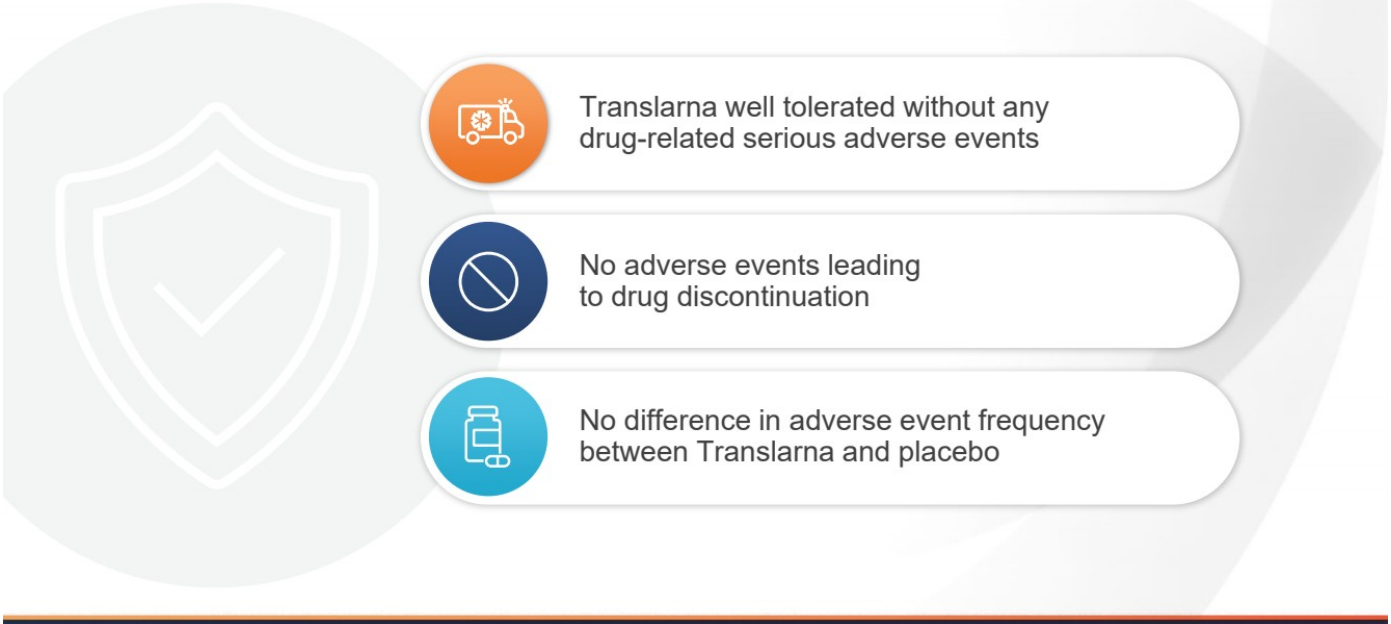
Pooled analysis from three placebo-controlled trials confirms significant treatment benefit

	Overall study population Study 007, 020 and 041	
	Translarna (N=354)	Placebo (N=347)
6MWD (48 weeks)	-28.1	-47.4
	19.3 (p=0.0002)	
NSAA*		
Total Score	1.01 (p=0.002)	
Linear Score	2.28 (p=0.005)	
TFTs		
10m walk	-1.30 (p=0.0001)	
Stair ascend	-1.43 (p=0.0004)	
Stair descend	-1.51 (p=0.0004)	

*Study 020 & 041 only

6MWD 300-400m - Difference **32.1** (p=0.0005)

Study 041: Safety summary



Study 041 confirms Translarna treatment benefit in nmDMD patients

First disease modifying treatment for DMD to show a statistically significant treatment benefit across the entire ITT population

Totality of evidence across multiple DMD clinical studies and real-world data demonstrate clinical benefit

Translarna demonstrates a favorable safety profile with more than 3000 patients treated

3 1 The ITT population was not the primary analysis population

Alternative analysis method (ANCOVA)

Translarna treatment demonstrated benefit across key endpoints in ITT population

Endpoint Assessment (Change from baseline)	Difference - MMRM (p value)	Difference - ANCOVA (p value)
6MWD (m)	**14.4 (p=0.0248)**	15.3 (p=0.0805)
Rate of change (m/wk)	**0.20 (p=0.0248)**	0.21 (p=0.0805)
NSAA		
Total score	**0.9 (p=0.0235)**	0.9 (p=0.0695)
Linear score	**2.3 (p=0.0246)**	2.5 (p=0.0648)
Timed function tests		
10m walk	**-0.78 (p=0.0422)**	-0.90 (p=0.0972)
Stair ascend	**-1.06 (p=0.0293)**	-1.23 (p=0.0605)
Stair descend	-0.29 (p=0.5749)	-0.31 (p=0.6485)

Analysis in subgroups demonstrate similar trends

The ITT population was not the primary analysis population

Pooled analysis from three placebo-controlled trials confirms significant treatment benefit

	Overall study population Study 007, 020 and 041	
	Translarna (N=354)	Placebo (N=347)
6MWD (48 weeks)	-28.1	-47.4
	19.3 (p=0.0002)	
NSAA*		
Total Score	1.01 (p=0.002)	
Linear Score	2.28 (p=0.005)	
TFTs		
10m walk	-1.30 (p=0.0001)	
Stair ascend	-1.43 (p=0.0004)	
Stair descend	-1.51 (p=0.0004)	

*Study 020 & 041 only

6MWD 300-400m - Difference **32.1** (p=0.0005)

Study 041 confirms Translarna treatment benefit in nmDMD patients

First disease modifying treatment for DMD to show a statistically significant treatment benefit across the entire ITT population

Study 041 adds to existing evidence of clinical benefit from STRIDE real-world registry and previous DMD clinical studies

Submit results confirming favorable Translarna risk-benefit to EMA

Utilize 041 results to align with the FDA on NDA approval pathway

3 5 The ITT population was not the primary analysis population

Questions?