Precision Genetic Medicine for Neuromuscular Diseases

23rd International Congress of the World Muscle Society

Mendoza, Argentina
4 October 2018

SAREPTA THERAPEUTICS

NATIONWIDE CHILDREN'S
When your child needs a hospital, everything matters.

Forward-Looking Statements

This presentation contains "forward-looking statements." Any statements that are not statements of historical fact may be deemed to be forward-looking statements. Words such as “believe,” “anticipate,” “plan,” “expect,” “will,” “may,” “intend,” “prepare,” “look,” “potential,” “possible” and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to the goal of the AAVrh74.MHCK7 micro-dystrophin study 1, its design and endpoints; and the expectations from the study, including AAVrh74 efficient transduction to all muscle types, MHCK7 selective for cardiac and skeletal transgene muscle expression, widespread micro-dystrophin expression in all biopsied muscles, reduction in CK levels and favorable safety profile with no unexpected immunological responses.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta’s control. Actual results could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties. Known risk factors include the following: success in preclinical testing and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and initial results from a clinical trial do not necessarily predict final results; different methodologies, assumptions and applications Sarepta utilizes to assess particular safety or efficacy parameters may yield different statistical results, and even if Sarepta believes the data collected from clinical trials of its product candidates are promising, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities; Sarepta’s ongoing research and development efforts may not result in any viable treatments suitable for clinical research or commercialization due to a variety of reasons, some of which may be outside of Sarepta’s control, including possible limitations of Company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, and regulatory, court or agency decisions, such as decisions by the United States Patent and Trademark Office with respect to patents that cover our product candidates; and even if Sarepta’s programs result in new commercialized products, Sarepta may not achieve any significant revenues from the sale of such products; if the actual number of patients suffering from DMD is smaller than estimated, Sarepta’s revenue and ability to achieve profitability may be adversely affected; and those risks identified under the heading “Risk Factors” in Sarepta’s most recent Annual Report on Form 10-K for the year ended December 31, 2017 or most recently filed Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by the Company which you are encouraged to review.

Any of the foregoing risks could materially and adversely affect the Company’s business, results of operations and the trading price of Sarepta’s common stock. You should not place undue reliance on forward-looking statements. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof, except to the extent required by applicable law or SEC rules.

OUR VISION FOR THE FUTURE OF

Precision Genetic Medicine

23rd International Congress of the World Muscle Society

Welcome and Introduction

Doug Ingram

President and CEO

Sarepta Therapeutics, Inc.

SAREPTA THERAPEUTICS

NATIONWIDE CHILDREN'S

When your child needs a hospital, everything matters.

OUR VISION FOR THE FUTURE OF

Precision Genetic Medicine

23rd International Congress of the World Muscle Society

Clinical Update: AAVrh74.MHCK7.Micro-dystrophin Program

Jerry Mendell, MD

The Ohio State University College of Medicine

Paul D Wellstone Muscular Dystrophy Cooperative Research Center

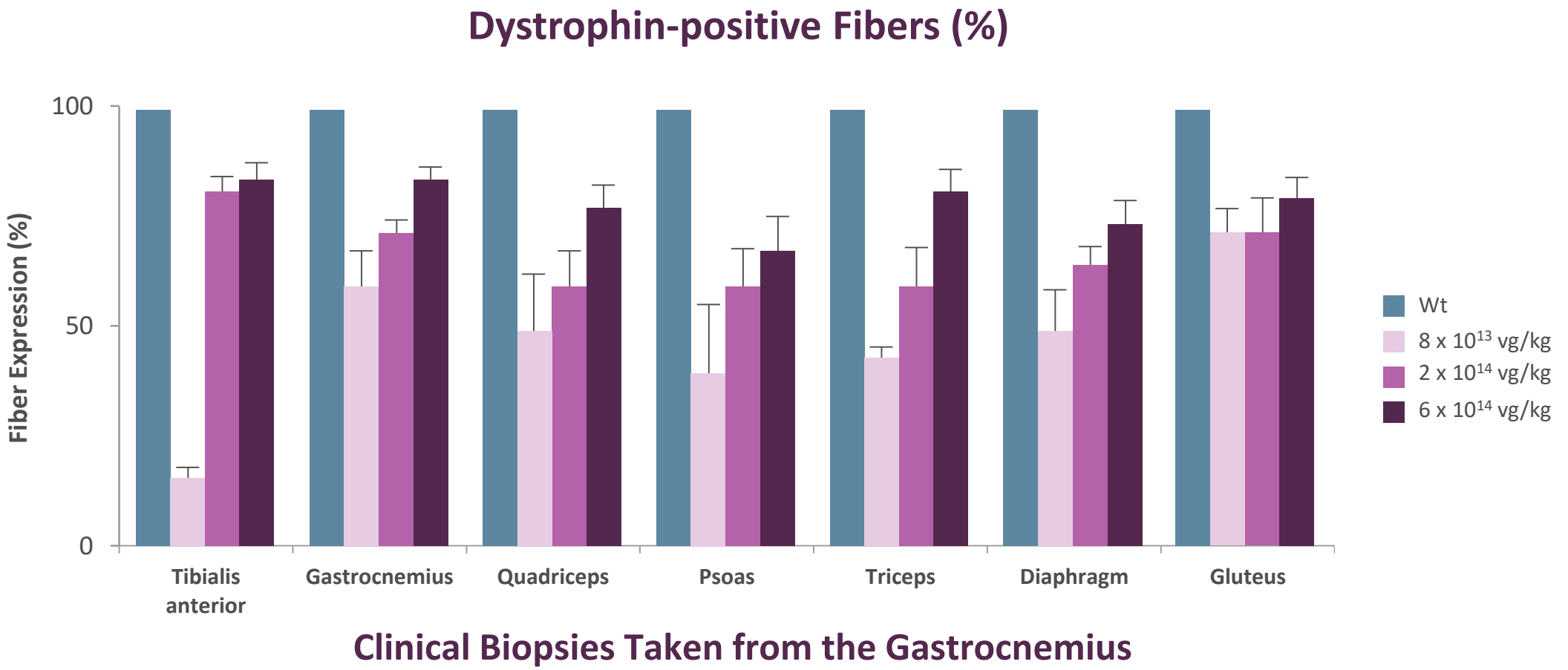
Nationwide Children's Hospital

Columbus, OH

AAVrh74.MHCK7.Micro-dystrophin: Goal of Study 1 was to Validate Pre-clinical Results

Expectations based on pre-clinical models
AAVrh74 efficient transduction to all muscle types
MHCK7 selective for cardiac and skeletal transgene muscle expression
Widespread micro-dystrophin expression in all biopsied muscles
Reduction in creatine kinase (CK)
Favorable safety profile with no unexpected immunological responses

AAVrh74.MHCK7.Micro-dystrophin Widespread Expression after Gene Delivery in *mdx* Mice



Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Micro-dystrophin Clinical Trial Design

SAREPTA THERAPEUTICS

NATIONWIDE CHILDREN'S
When your child needs a hospital, everything matters.℠

Open-Label Trial Design

- **Cohort B**
  - 4 subjects
  - 4-7 years of age
- **Inclusion criteria**
  - Confirmed *DMD* mutation
  - Negative for AAVrh74 antibodies

ClinicalTrials.gov Identifier: NCT03375164.

Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Cohort B (4-7 Years of Age) Endpoints

- **Primary endpoint**
  - Safety
- **Secondary endpoints**
  - Change in micro-dystrophin expression pre- vs post-treatment
  - Decrease in CK
  - 100-meter timed test (100 m)
  - North Star Ambulatory Assessment (NSAA; 10-meter timed test included)
  - Timed up and go (TUG)
  - Ascend and descend 4 steps
  - Hand-held dynamometry (HHD)
  - Cardiac magnetic resonance imaging (at 1 year)

ClinicalTrials.gov Identifier: NCT03375164.

Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Subject Demographics at Baseline

Subject	Age (years)	CK Levels at Baseline (U/L)
1	5	20,691
2	4	23,414
3	6	34,942
4	4	29,210

ClinicalTrials.gov Identifier: NCT03375164.

Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Subject 4:
Micro-dystrophin Data

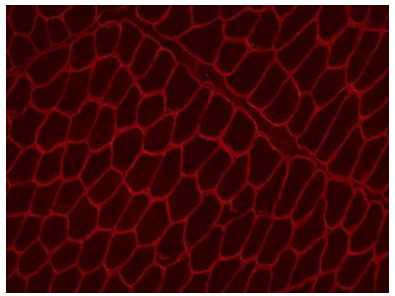
SAREPTA THERAPEUTICS

NATIONWIDE CHILDREN'S
When your child needs a hospital, everything matters.℠

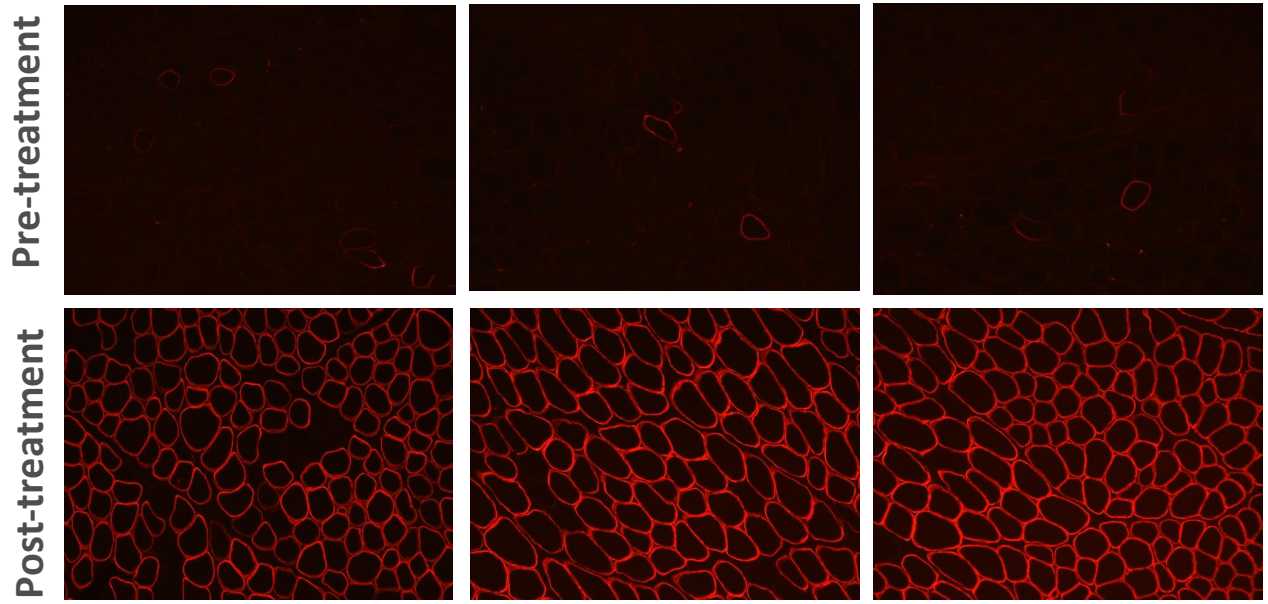
Robust Micro-dystrophin Expression in Muscle Fibers From the Gastrocnemius in Subject 4

Micro-dystrophin Expression (IHC)

Normal Control



Subject 4



Subject	Mean Intensity	Percentage of Dystrophin-positive Fibers
4	**160.0%**	**96.2%**

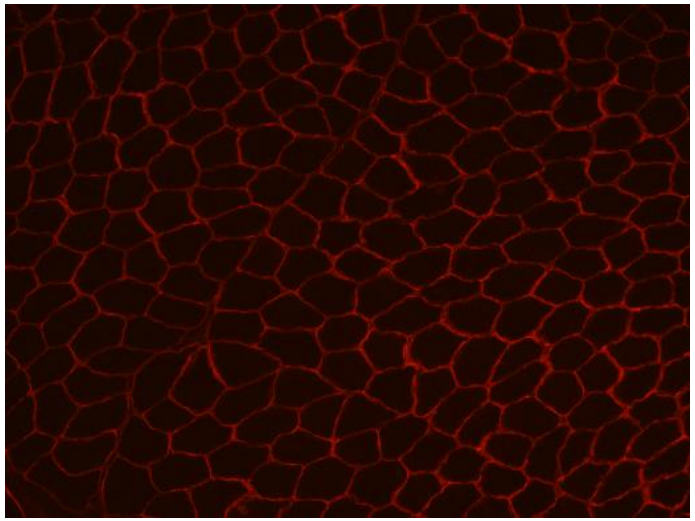
ClinicalTrials.gov Identifier: NCT03375164.

Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

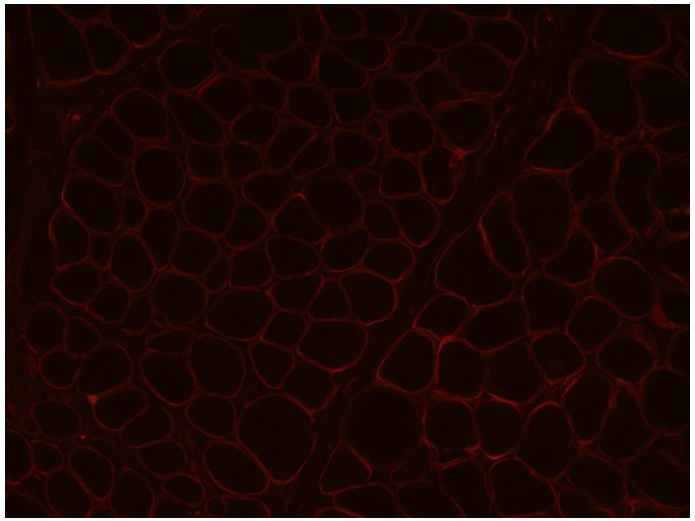
Micro-dystrophin Gene Therapy Upregulates DAPC Proteins in Subject 4

Expression of β-sarcoglycan in Muscle Fibers From the Gastrocnemius of Subject 4 (IHC)

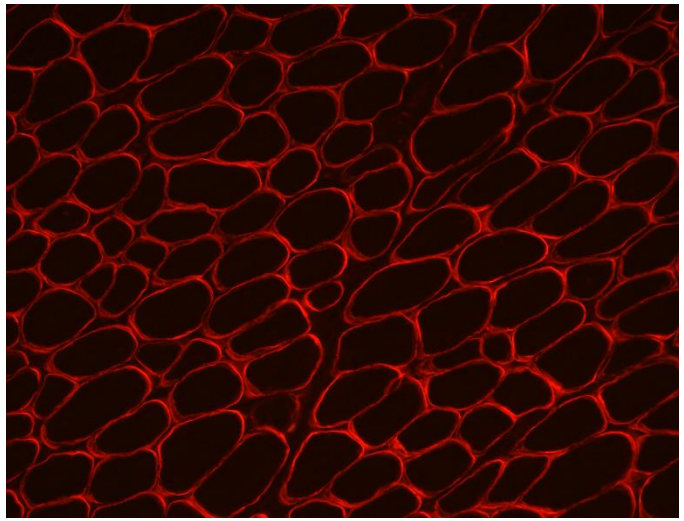
Normal Control



Pre-treatment



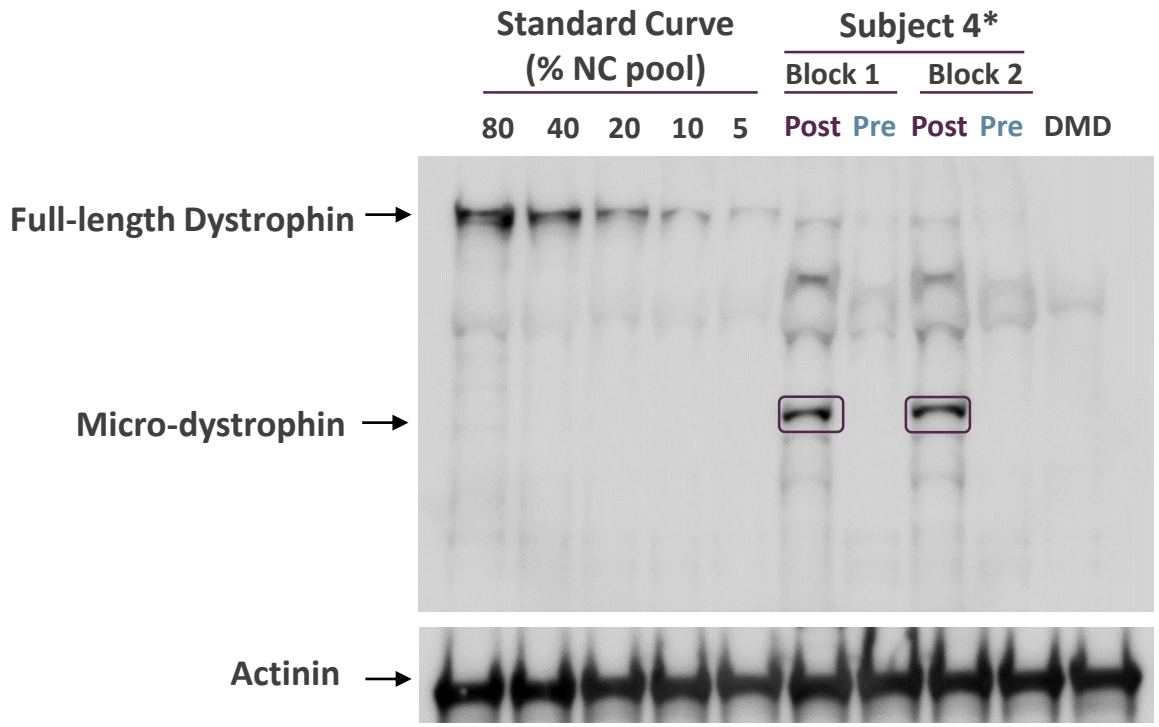
Post-treatment



ClinicalTrials.gov Identifier: NCT03375164.

Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Detection of Micro-dystrophin Expression by Western Blot Post-treatment in Subject 4



*Samples diluted 1:4 as ULOQ (>80%) exceeded in initial analysis.

Mean values multiplied by correction factor for final value compared to normal.

Western Quantitation Method	Mean Micro-dystrophin Expression vs Normal
Sarepta	**182.7%** (not adjusted for fat and fibrotic tissue)
Nationwide	**222.0%** (adjusted for fat and fibrotic tissue)

ClinicalTrials.gov Identifier: NCT03375164.

Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

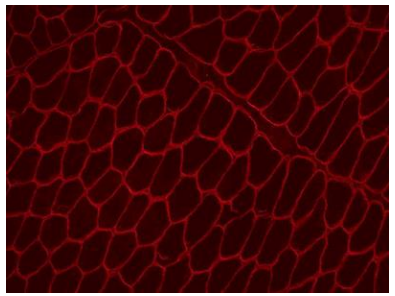
Micro-dystrophin Summary: All Subjects (n=4)

SAREPTA THERAPEUTICS

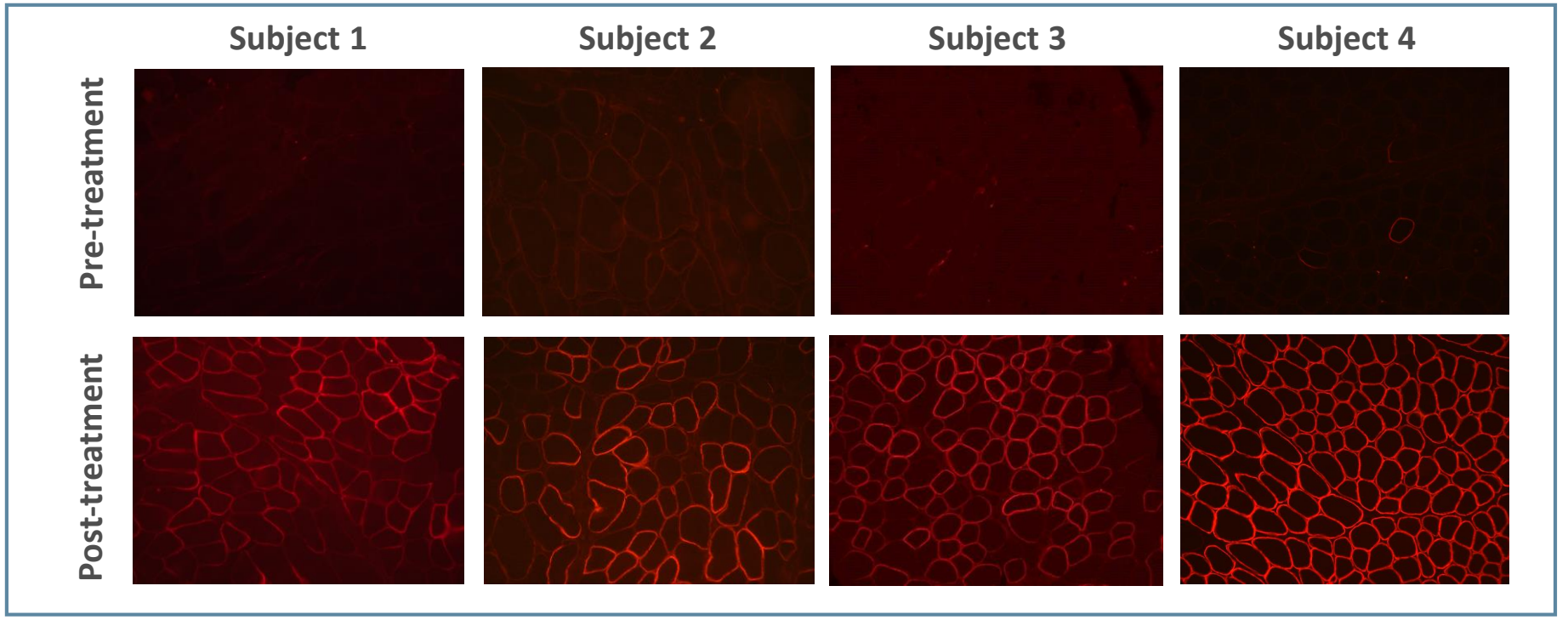
NATIONWIDE CHILDREN'S
When your child needs a hospital, everything matters.℠

Robust Micro-dystrophin Expression in Muscle Fibers from the Gastrocnemius in All 4 Subjects

Normal Control



Micro-dystrophin Expression (IHC)

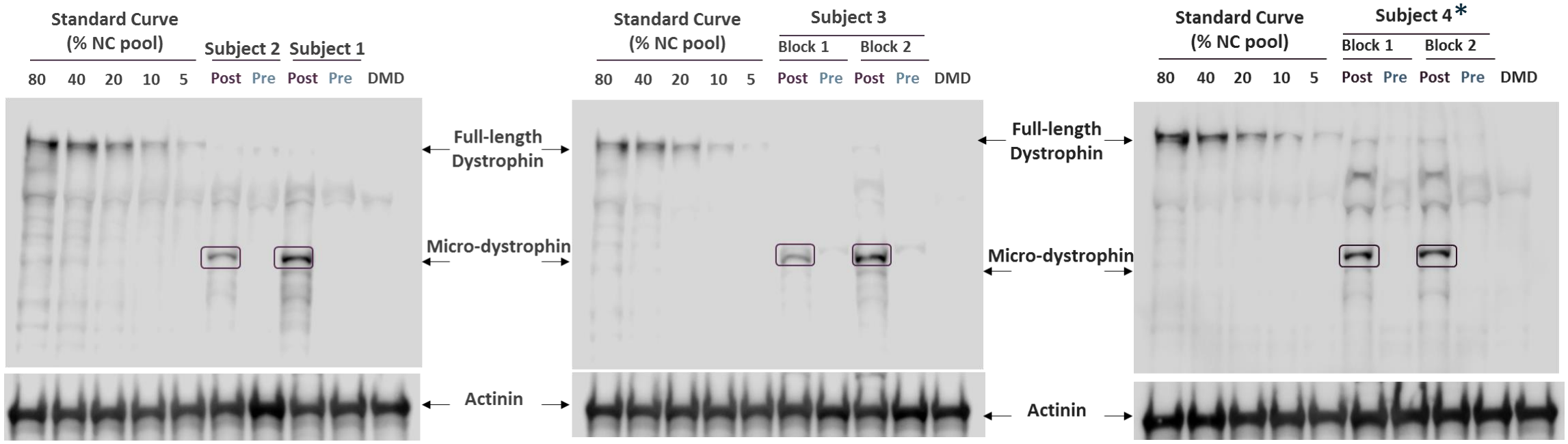


	Intensity	Percentage of Dystrophin-positive Fibers
Mean (n=4)	96.0%	81.2%

ClinicalTrials.gov Identifier: NCT03375164.

Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Detection of Micro-dystrophin Expression by Western Blot Post-treatment in All 4 Subjects



Western Quantitation Method	Mean Micro-dystrophin Expression (N=4) vs Normal
Sarepta	**74.3%** (not adjusted for fat and fibrotic tissue)
Nationwide	**95.8%** (adjusted for fat and fibrotic tissue)

p-Tyr phosphotyrosine; ULOQ, upper limit of quantification.
*Samples diluted 1:4 due to sample above ULOQ.
ClinicalTrials.gov Identifier: NCT03375164.
Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Robust Micro-dystrophin Expression is Supported by Vector Genome Count

Micro-dystrophin Expression (IHC)

	Intensity	Percentage of Dystrophin-positive Fibers
Mean (n=4)	**96.0%**	**81.2%**

Micro-dystrophin Expression (Western Blot)

	Sarepta (not adjusted for fat/fibrosis)	**Nationwide** (adjusted for fat/fibrosis)
Mean (n=4)	**74.3%**	**95.8%**

Vector Genome Number

	Vector Copies/µg DNA	Copies per Nucleus
Mean (n=4)	**>10^5**	**3.3**

ClinicalTrials.gov Identifier: NCT03375164.

Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

AAVrh74.MHCK7.Micro-dystrophin: Clinical Data Summary (n=4)

SAREPTA THERAPEUTICS

NATIONWIDE CHILDREN'S
When your child needs a hospital, everything matters.℠

Summary of Clinical Data

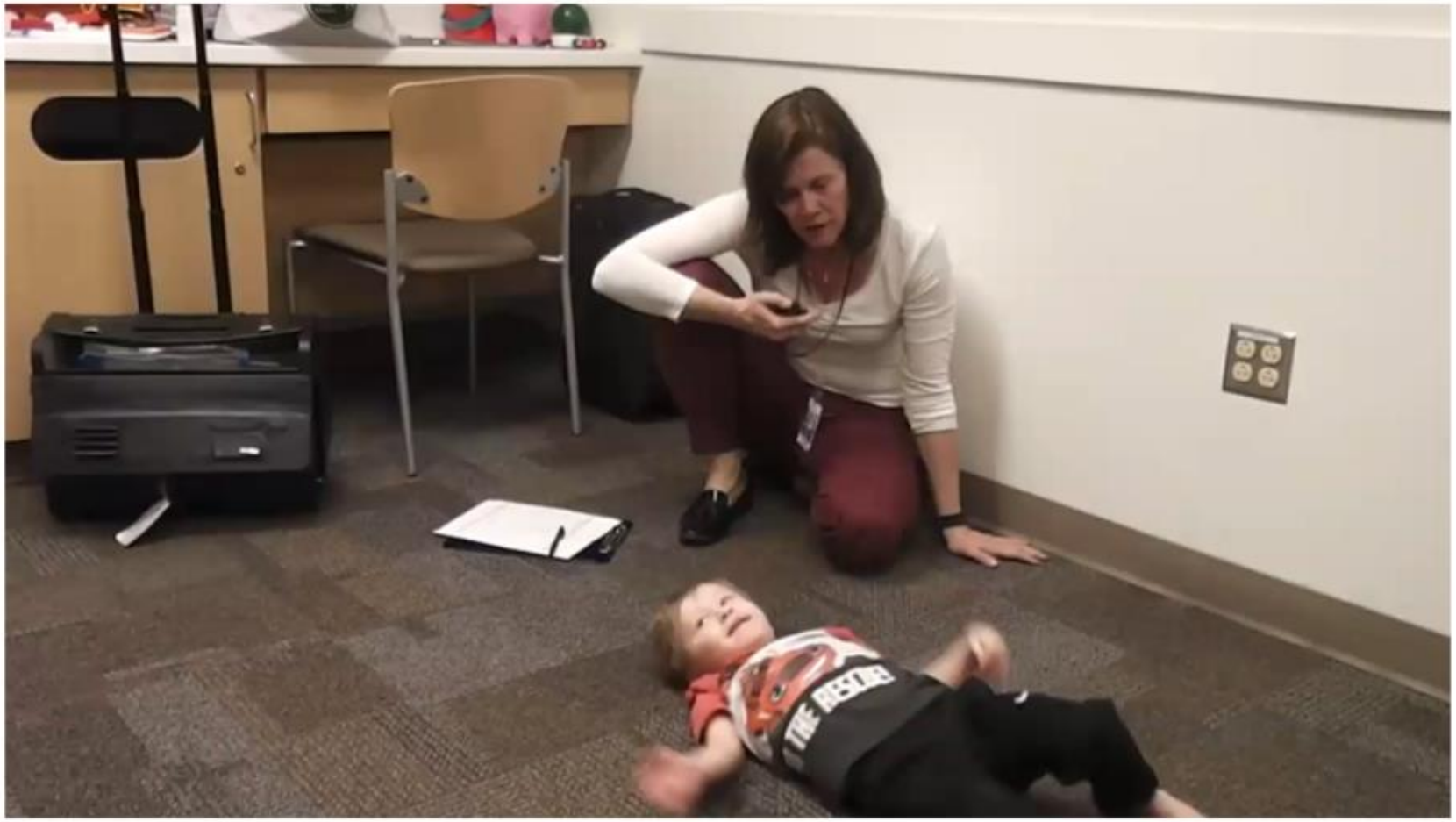
Change from Baseline to Last Assessment

Subject	Assessment	NSAA (Δ)	Time to Rise (sec)	4 Stairs Up (sec)	100 m (sec)	10 m (sec)	CK (U/L)
1	Baseline	18	3.7	3.4	49.3	5.1	20,691
	Last Visit (Day 270)	26 (+8)	3.0	2.3	43.2	4.3	6,317
2	Baseline	19	3.0	3.8	49.9	4.3	23,414
	Last Visit (Day 180)	27 (+8)	3.7	2.6	48.6	3.9	6,209
3	Baseline	26	3.9	1.9	59.3	4.7	34,942
	Last Visit (Day 180)	30 (+4)	3.4	1.8	48.4	4.1	9,650
4	Baseline	19	4.1	4.8	67.2	5.4	29,210
	Last Visit (Day 90)	25 (+6)	2.3	2.2	50.7	4.4	1,382
Average	% Change From Baseline	33% Improvement	13% Improvement	31% Improvement	14% Improvement	14% Improvement	78% Improvement

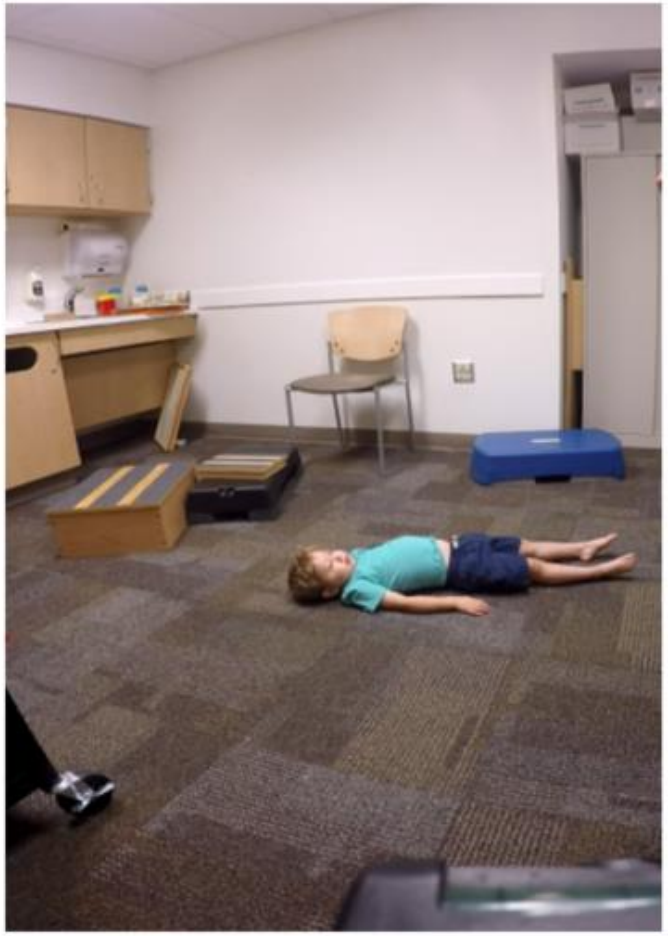
ClinicalTrials.gov Identifier: NCT03375164.

Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Patient Video: Rise From Floor – Subject 4



Baseline



90 days post-treatment

Patient Video: 4-stair Climb – Subject 1

Baseline

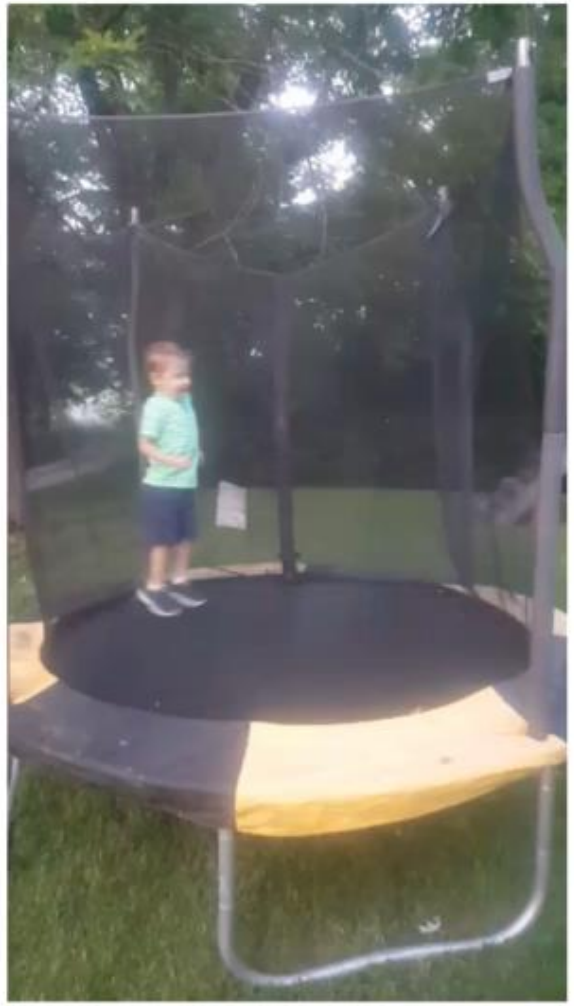
270 days post-treatment

Patient Home Videos: Activities of Daily Living

60 days post-treatment (Pt 1)

30 days post-treatment (Pt 2)

60 days post-treatment (Pt 3)



75 days post-treatment (Pt 4)

Safety (n=4)

- No serious adverse events in this study
- 3 subjects had elevated γ-glutamyl transpeptidase, which resolved with steroid treatment within a week
- No other clinically significant laboratory findings
- Subjects had transient nausea generally within the first week coincident with increased steroid dosing
  - Did not correlate with liver enzyme elevations or any other abnormality

ClinicalTrials.gov Identifier: NCT03375164.

Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Summary

- All 4 treated subjects are doing well
  - Biomarkers show large magnitude of effect within 3 months (CK and dystrophin)
  - “Very early days" but initial functional data show improvement consistent with biomarker data
  - Early results show these boys performing in a manner unexpected for the typical boy with DMD
  - Favorable safety profile to date with up to 9 months of follow-up

Question and Answer

SAREPTA THERAPEUTICS

NATIONWIDE CHILDREN'S
When your child needs a hospital, everything matters.℠

Thank You

SAREPTA THERAPEUTICS

NATIONWIDE CHILDREN'S
When your child needs a hospital, everything matters.℠