Sarepta Therapeutics Investor Update

October 1, 2015

Presenter

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Forward-Looking Statement

- *This presentation, contains forward-looking statements. These forward-looking statements generally can be identified by the use of words such as "believes" or belief," "anticipates," "plans," "expects," "will," "intends," "potential," "possible," "advance" and similar expressions. These forward-looking statements include statements about the safety and efficacy of eteplirsen, analysis of eteplirsen and control cohort data and their implications, and eteplirsen's potential as treatment for Duchenne Muscular Dystrophy, the potential market for our exon skipping product candidates and Sarepta's mission, commitments and business plans and strategies. Forward-looking statements also include those made during the presentation regarding future business developments and actions and the timing of the same.*

- *Each forward-looking statement contained in this presentation is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statement. Applicable risks and uncertainties include, among others: the results of our ongoing research and development efforts and clinical trials for eteplirsen and our other product candidates may not be positive or consistent with prior results or demonstrate a safe treatment benefit; there may be delays in our projected timelines or our product candidates, chemistries or technologies may never become commercially available for regulatory or other reasons including a negative decision on our NDA for eteplirsen by an advisory committee or the FDA; agency or court decisions with respect to our patents or those of third parties may negatively impact our business; our product candidates and or the use of or application of our chemistries and technology may fail in the research, development or commercialization process for various other reasons including the possibility that we may not be able to comply with all regulatory requests and requirements for the research, development and commercialization of our product candidates; and those risks identified under the heading "Risk Factors" in Sarepta's Annual Report on Form 10-K for the year ended December 31, 2014 filed with the Securities and Exchange Commission (SEC), Sarepta's Quarterly Report on Form 10-Q for the quarter ended June 30, 2015 and Sarepta's other filings with the SEC, which we encourage investors to review at www.sec.gov, for a more detailed discussion on risks and uncertainties relating to our business.*

- *Any of the foregoing risks could materially and adversely affect Sarepta's business, results of operations and the trading price of Sarepta's common stock. We caution investors not to place considerable reliance on the forward-looking statements contained in this presentation. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.*

Panelists

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All panel members either advise or consult for Sarepta Therapeutics.

Summary of Accomplishments

- Confirmed clinical activity of eteplirsen in an intention-to-treat (ITT) analysis of 6-minute walk test (6MWT) for Study 201/202 vs external control
- Dystrophin production confirmed by each methodology:
  - 4th biopsy
    - Reverse-transcription–polymerase chain reaction (RT-PCR)
    - Dystrophin intensity
    - % dystrophin-positive fibers
    - Western blot
- Expanded clinical program
- Expanded safety database
- Eteplirsen New Drug Application (NDA) filed for accelerated approval

Key Data Included in NDA Filing

- ITT analysis of 6MWT or Study 201/202 vs external control
  - Intermediate clinical endpoint upon which the eteplirsen NDA filed with FDA
- Pulmonary function
- 4^{th} biopsy
- Rescore of % dystrophin-positive fibers
- Safety

ITT Analysis of 6MWT for Study 201/202 vs External Control

Establishing an Appropriate External Control Cohort Per FDA Request

- Only two Duchenne muscular dystrophy (DMD) registries contained longitudinal 6MWT data up to 36 months and included:
  - Genetic mutation data
  - Equivalent care standards including steroids
- Prospectively defined filters were used to identify external controls from these registries
  - Age, steroid use, and mutation type
  - External control patients would have been eligible for eteplirsen 201 trial based on baseline characteristics

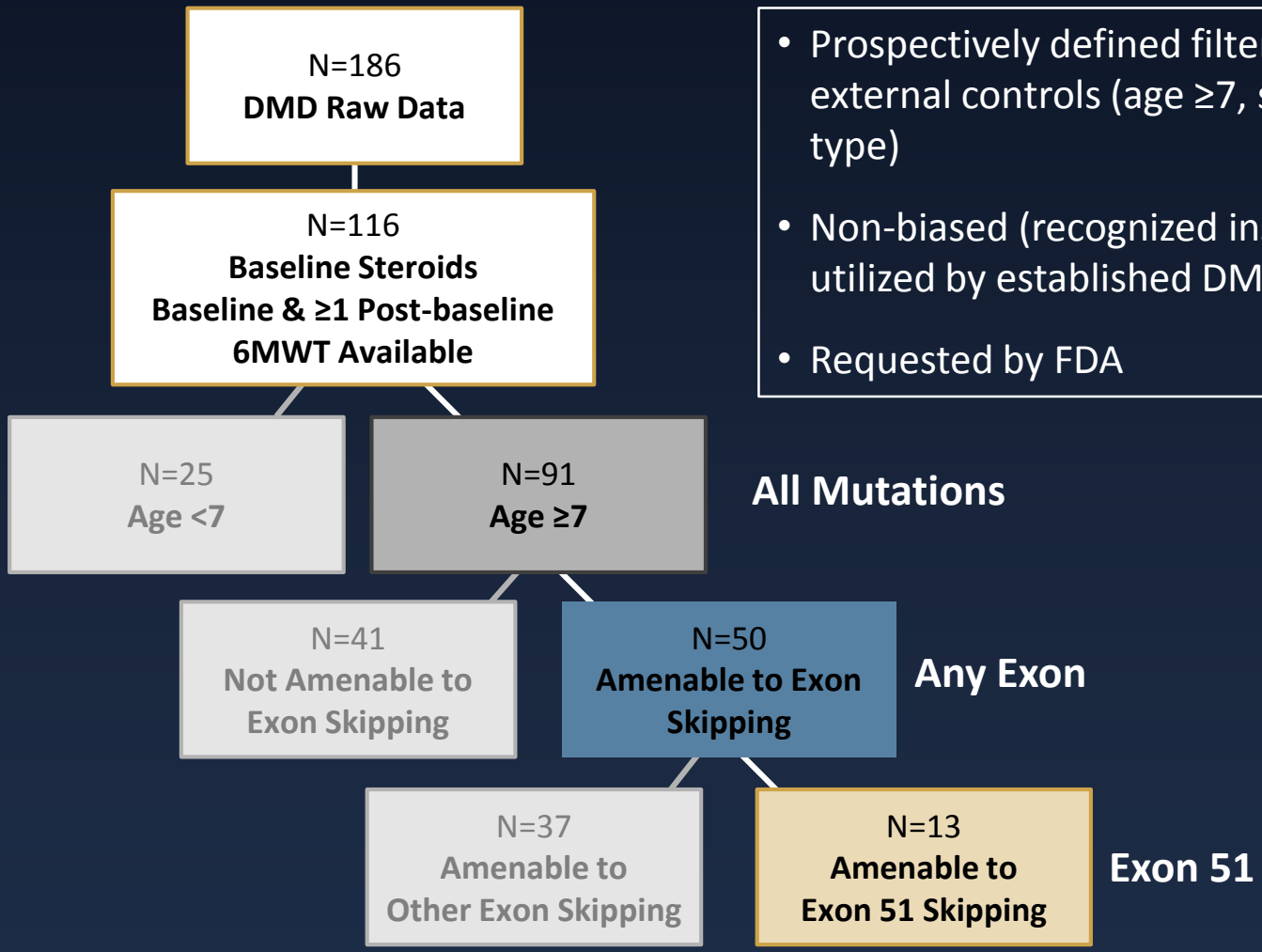
	Italian DMD Telethon N=97	Leuven Neuromuscular Research Center (NMRC), Belgium N=89	Eteplirsen N=12 (ITT)
Clinical Outcomes	6MWT	6MWT	6MWT, PFT

6MWT, 6-minute walk test; NSAA, North Star Ambulation Assessment; PFT, pulmonary function tests.

Italian Telethon & Leuven NMRC DMD Natural History Registries

- Investigator-initiated studies, independent of sponsor
- Patients treated according to CDC/TREAT-NMD care standards
  - Steroid use recorded
- Enrolled all patients who met eligibility criteria
  - Attending a participating neuromuscular clinic
  - Genetically confirmed diagnosis of DMD
  - No cognitive impairment that could affect 6MWT performance
- 6MWT administered by trained physical therapists according to modified American Thoracic Society procedure
- Published data in peer-reviewed articles

Derivation of External Control Groups by Prospectively Defined Filters



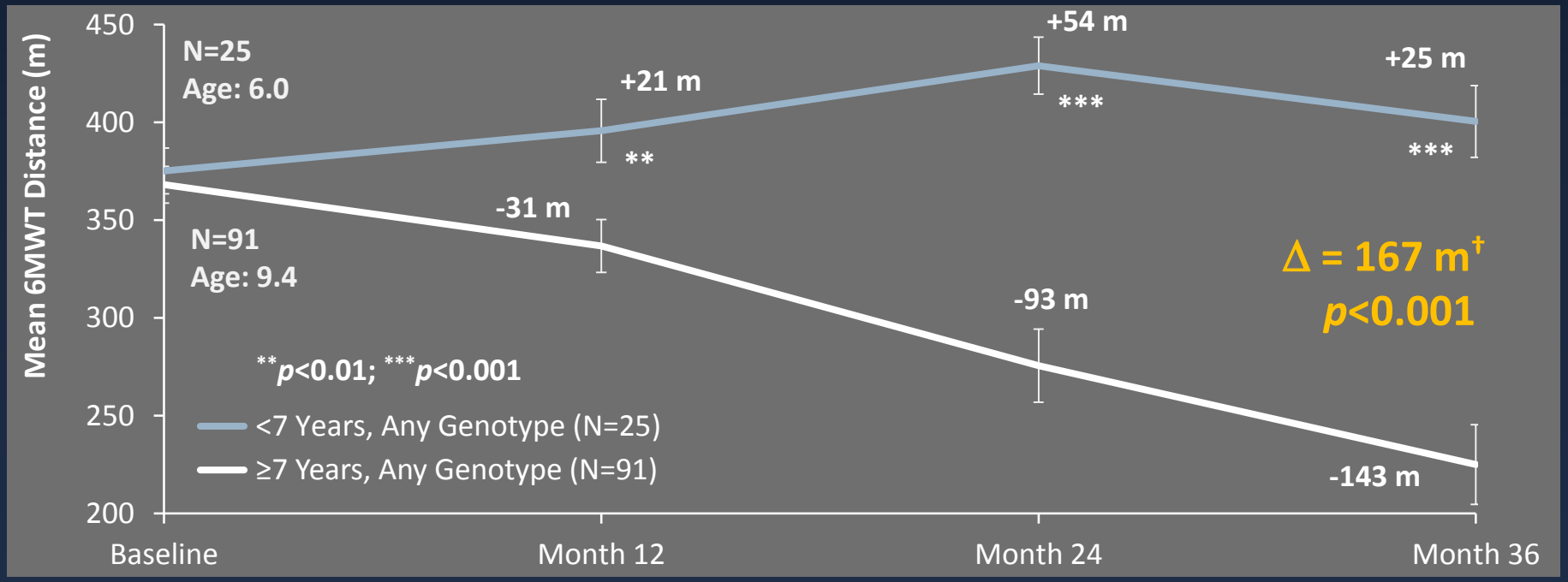
- Prospectively defined filters applied to identify external controls (age ≥7, steroids, mutation type)
- Non-biased (recognized institutions, databases utilized by established DMD companies)
- Requested by FDA

N indicates participants at baseline; some patients did not contribute data through 36 months.

Impact of Baseline Age (<7 or ≥7) on 6MWT Trends for Any Genotype

PATIENTS <7 SHOW IMPROVED 6MWT PERFORMANCE WHILE PATIENTS ≥7 SHOW DECLINE

- Patients <7 initially improve in walking ability through 24 months and maintain 6MWT above baseline through 36 months
  - 54-meter increase observed in the first 24 months
- It is challenging to show a benefit in 6MWT in ages <7 as patients are improving due to growth & development where growth outpaces the disease

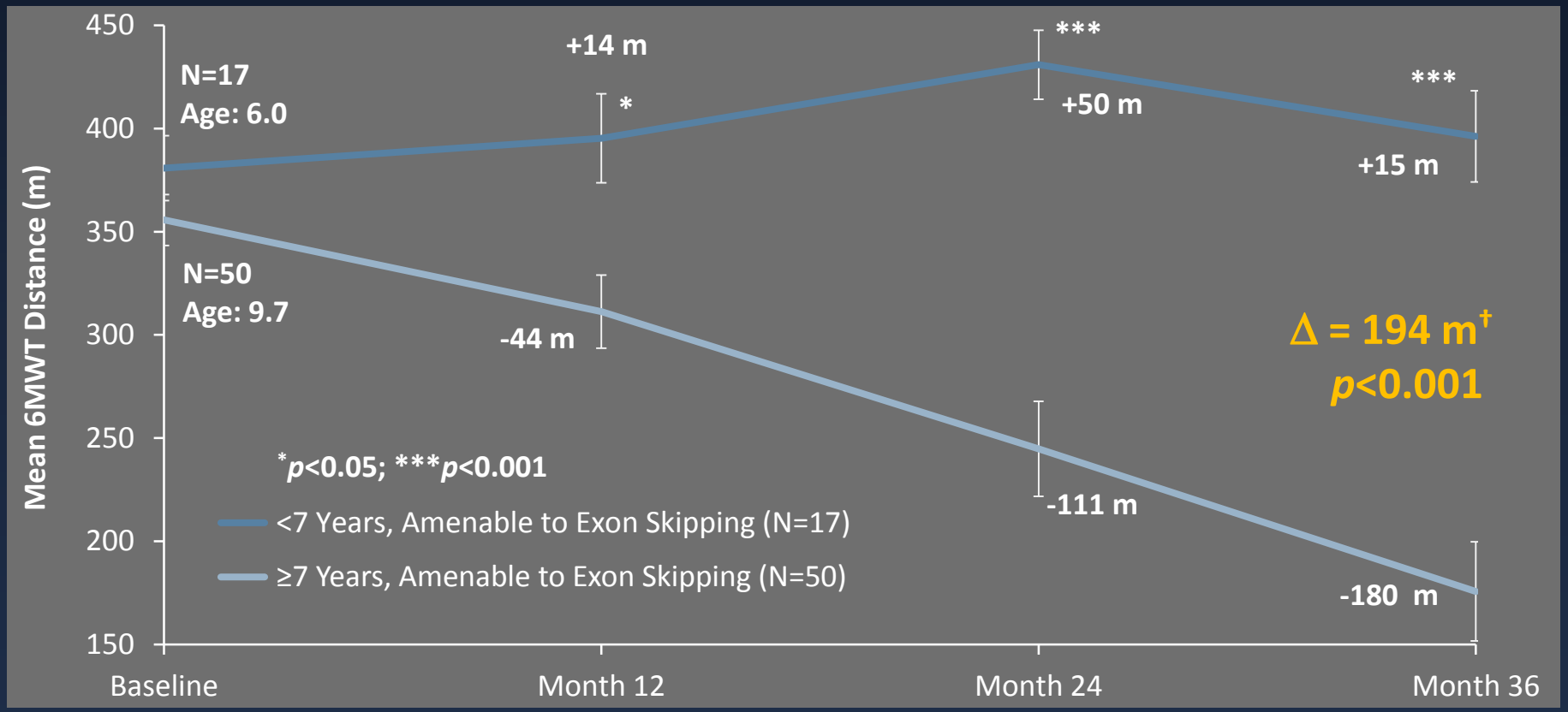


†Difference in mean change from baseline.

Impact of Baseline Age (<7 or ≥7) on 6MWT Trends in Patients Amenable to Exon Skipping

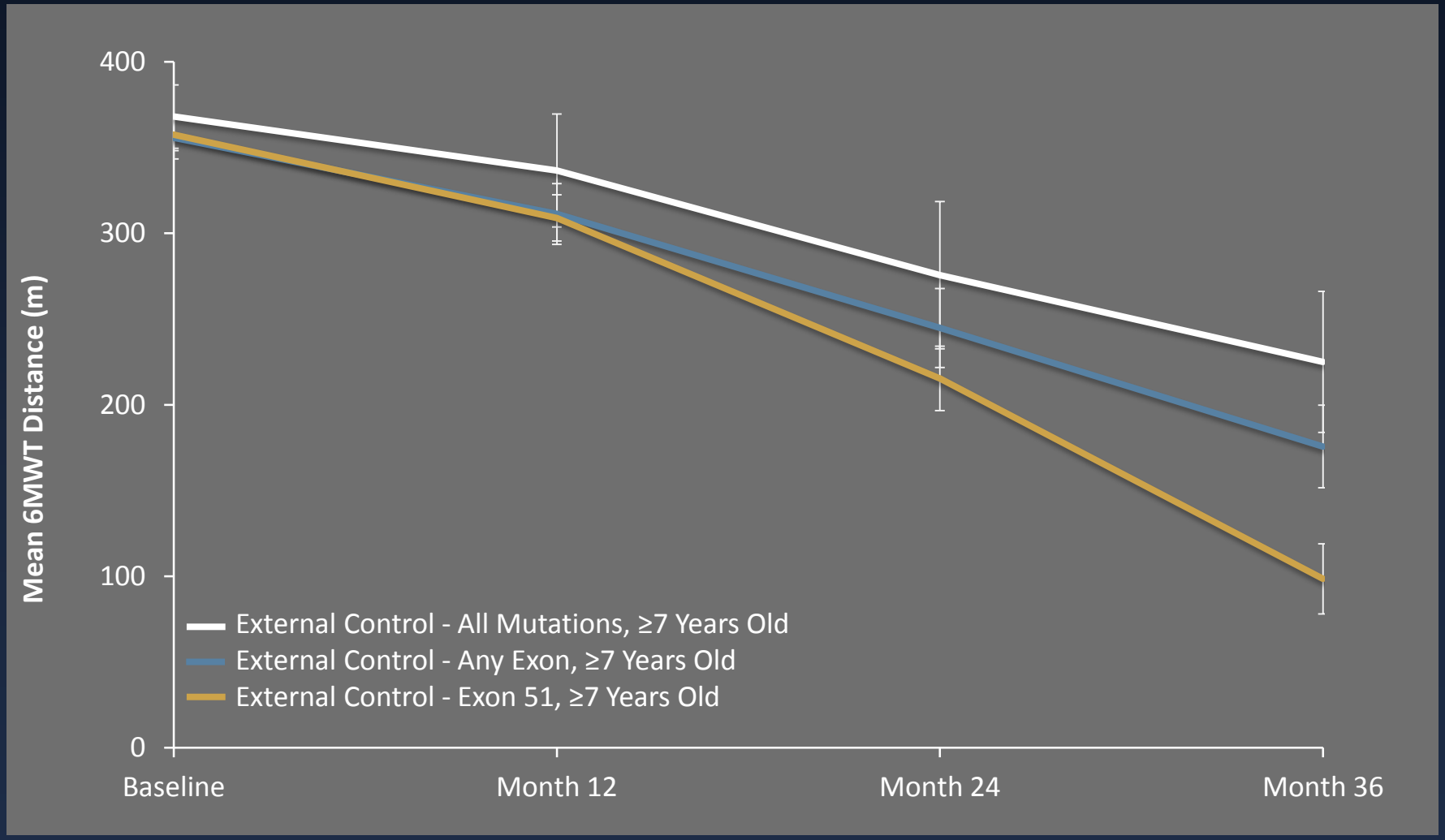
SIMILAR TREND OBSERVED IN EXON SKIP AMENABLE PATIENTS

- Patients <7 initially improve in walking ability through 24 months and maintain 6MWT above baseline through 36 months



†Difference in mean change from baseline.

Exon 51 Declines More Rapidly Than Other Genotypes



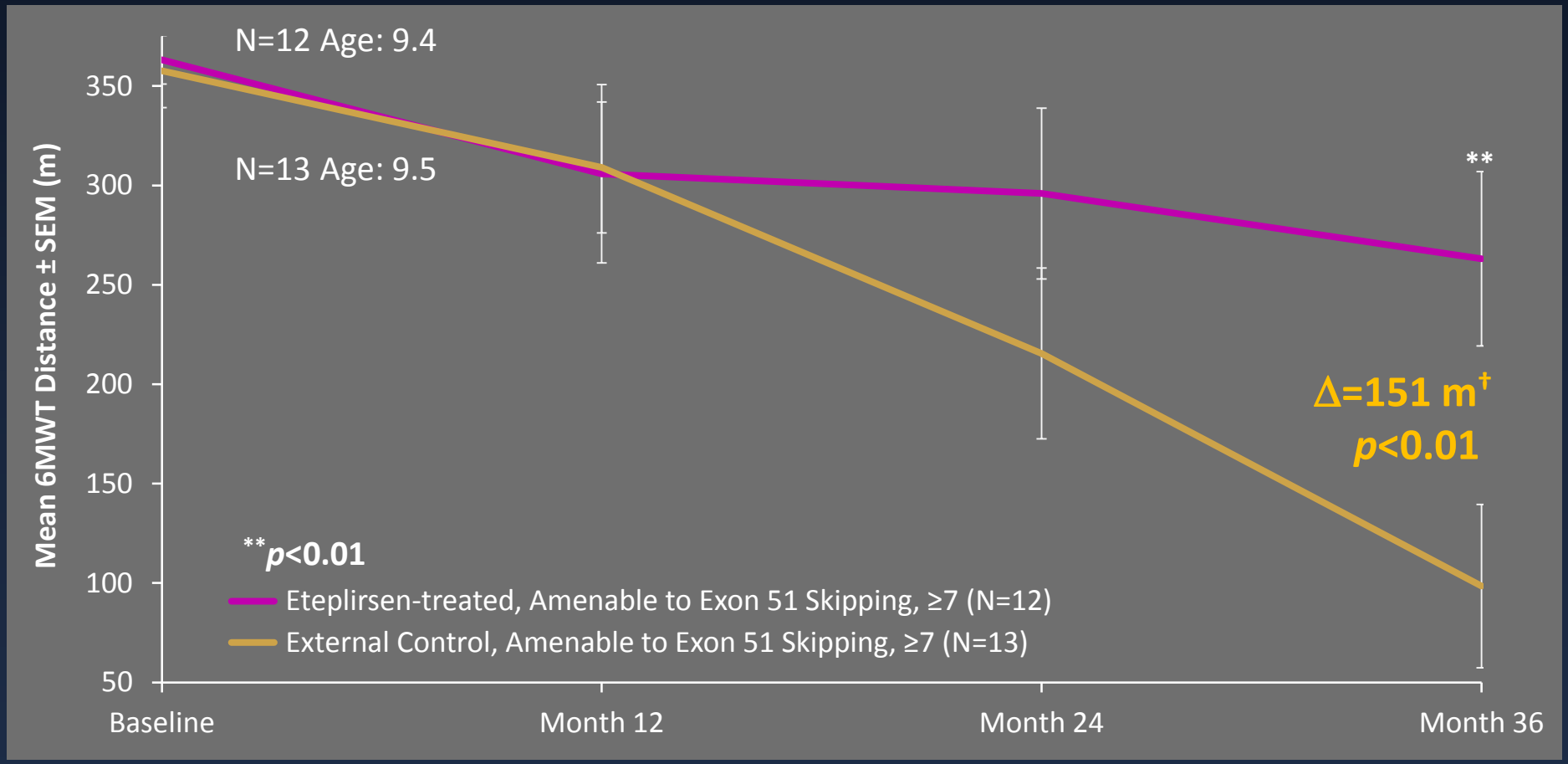
Patient Characteristics at Baseline: Eteplirsen and External Control Groups Were Well Matched

Parameter	Pivotal Study	6MWT External Control Groups	
Number of patients	Study 201/202 N=12	Exon 51 N=13	Any Exon N=50
Age, years Mean (SD)	9.4 (1.18)	9.5 (1.45)	9.7 (1.52)
6MWT distance, m Mean (SD)	363.2 (42.19)	357.6 (66.75)	355.7 (87.28)
Deletion mutations represented:	45–50, 48–50, 49–50, 50, 52	45–50, 48–50, 49–50, 50, 52	Skippable mutations
Steroid use	100%	100%	100%

151-Meter Difference Between Eteplirsen-treated vs Matched External Controls at 3 Years

ITT ANALYSIS, N=12 FOR ETEPLIRSEN-TREATED PATIENTS AT BASELINE, 12, 24, AND 36 MONTHS

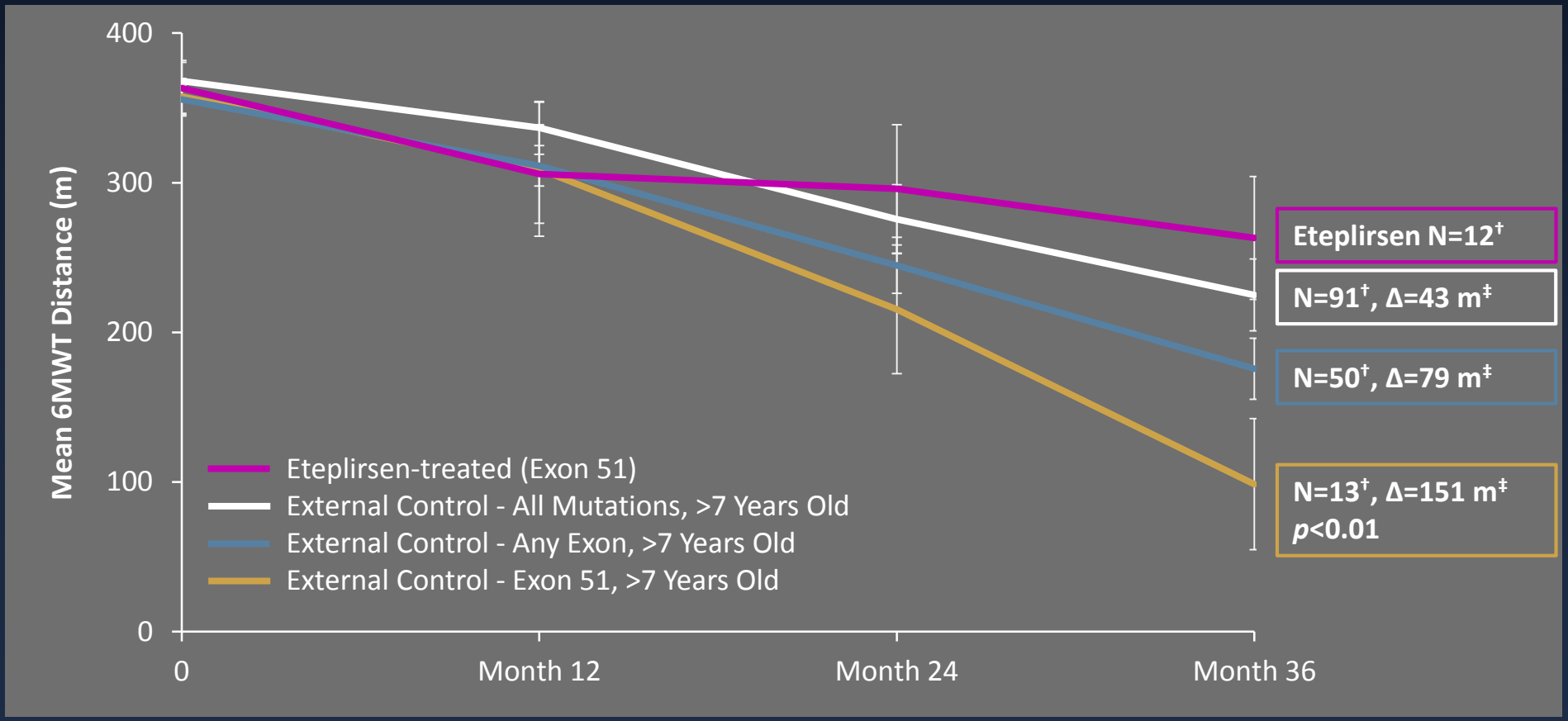
- External controls were steroid-treated, aged ≥7, and amenable to exon 51 skipping



- 2 patients in the historical group did not contribute data to the Month 36 timepoint

†Difference in mean change from baseline.

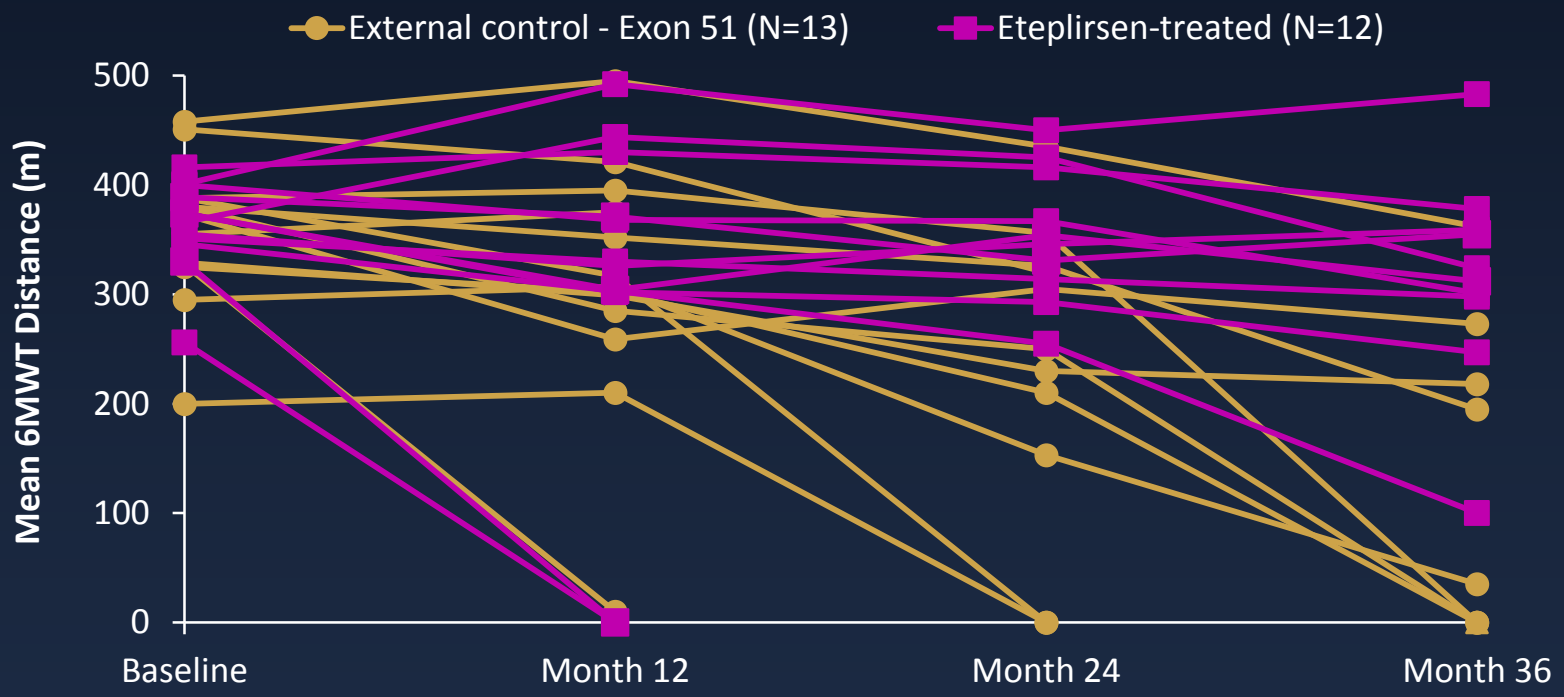
Analysis Shows Slower Rate of 6 MWT Decline in Eteplirsen Treated Patients Compared to Multiple Controls



†Sample size at baseline.
‡Difference at 36 months in mean change from baseline.

Individual Patient Data for Eteplirsen (N=12) vs External Control (EC)

DIFFERENCE IN RATE OF DECLINE OBSERVED IN THE MAJORITY OF PATIENTS

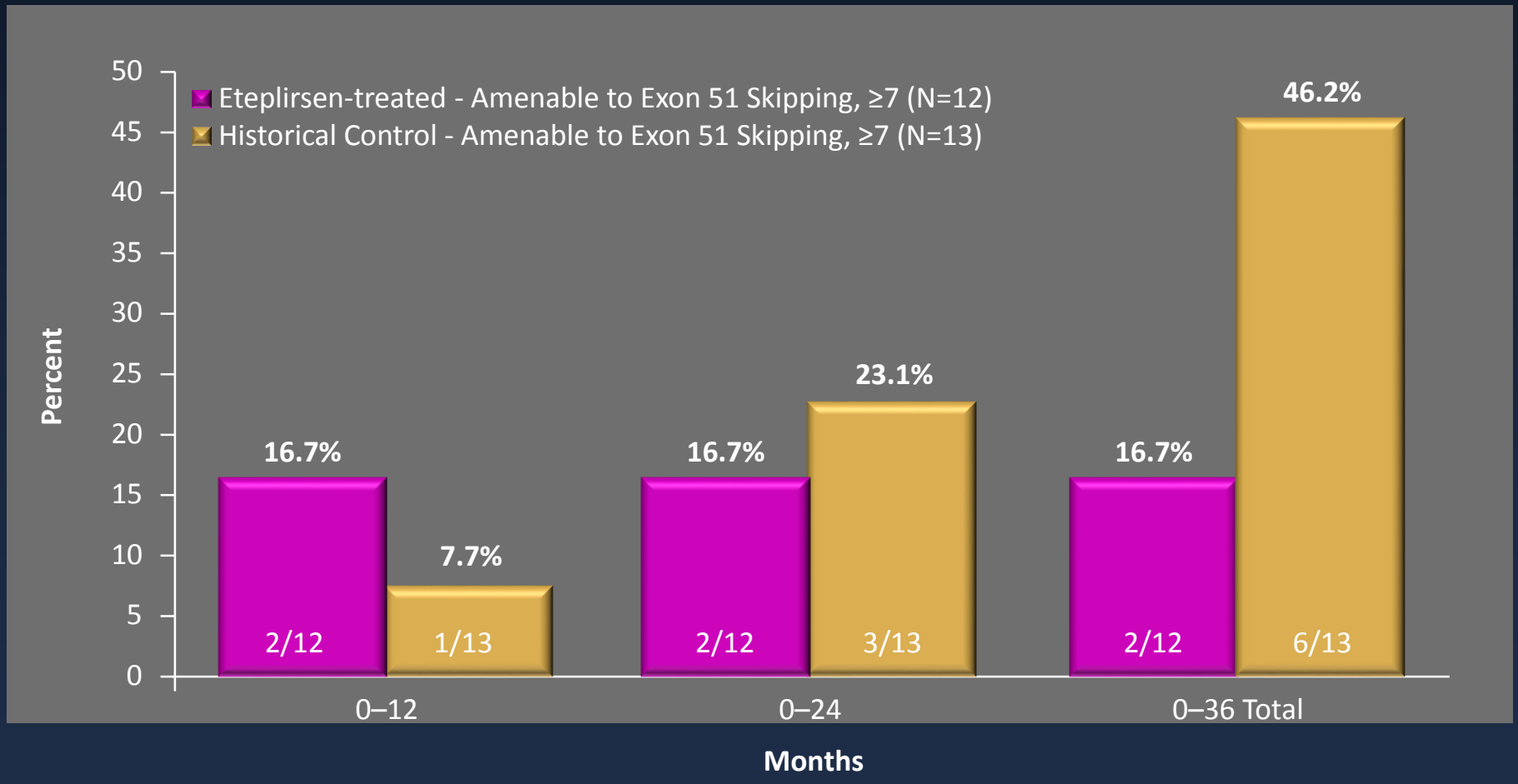


	Baseline		12 Months		24 Months		36 Months	
	EC	Etep	EC	Etep	EC	Etep	EC	Etep
% Walking >300 m	85%	92%	62%	83%	46%	67%	23%	58%
% Walking >150 m	100%	100%	92%	83%	77%	83%	46%	75%
% Non-ambulant	0%	0%	8%	17%	23%	17%	46%	17%

External control (age ≥7, on steroids, amenable to exon 51 skipping)

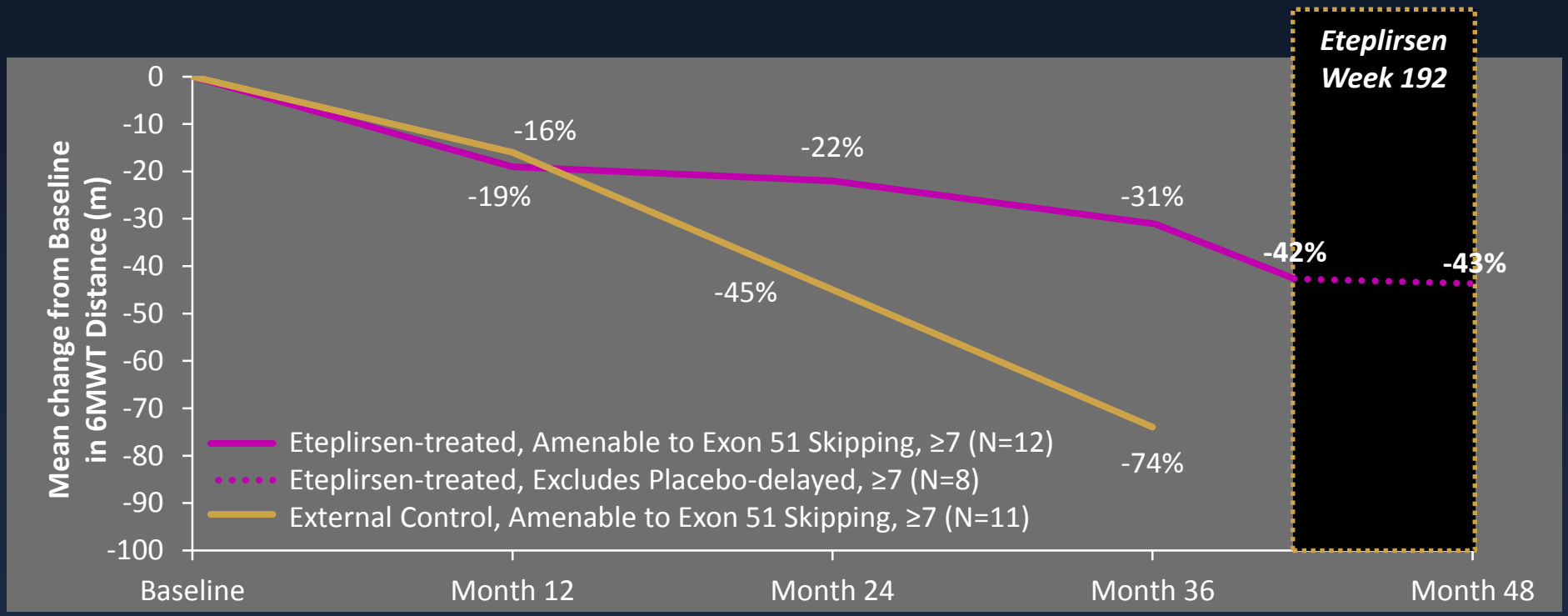
Eteplirsen-treated Patients (N=12) Showed a Lower Rate of Loss of Ambulation Than External Control

- 6/13 (46%) untreated external controls lost ambulation over 3 years
- 2/12 (17%; all in year 1) eteplirsen patients lost ambulation over 3 years



Eteplirsen-treated Cohort Maintains Benefit Through Week 192

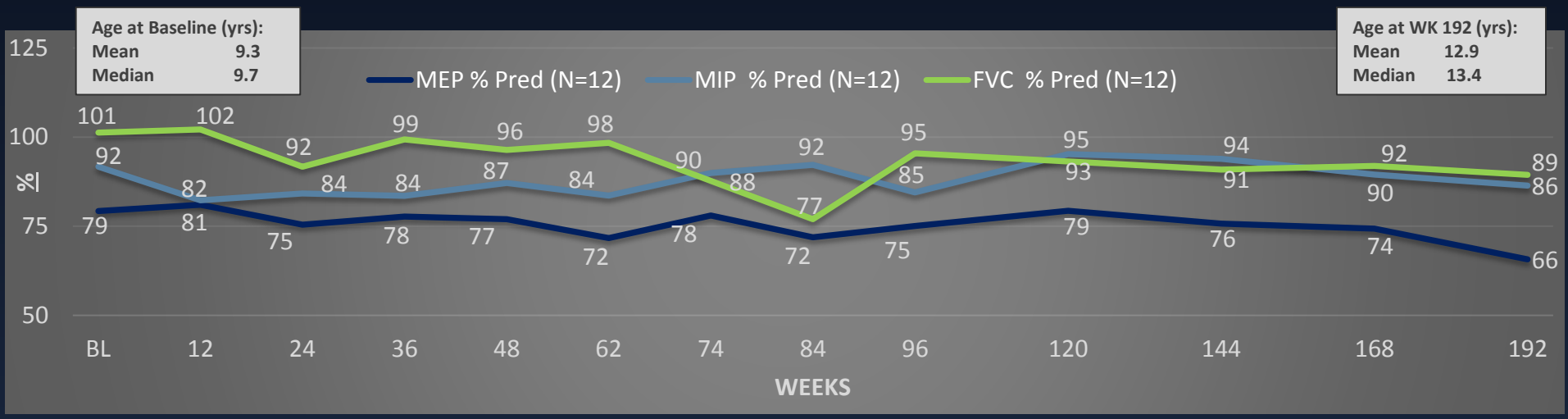
10/12 ETEPLIRSEN-TREATED BOYS REMAIN AMBULANT AT WEEK 192 (MEAN AGE 12.9) – ~160 WEEKS SINCE AN ETEPLIRSEN-TREATED BOY LOST AMBULATION



- N=8 at month 48 as the placebo crossover patients (N=4) began treatment at week 25 (wk 25-192) and have not reached 48 months of treatment as other subjects did (wk 1-192) and have only completed 168 weeks of treatment included above. All patients are included (N=12) above in the eteplirsen values from when they started therapy.
  No new non-ambulant patients or discontinuations.

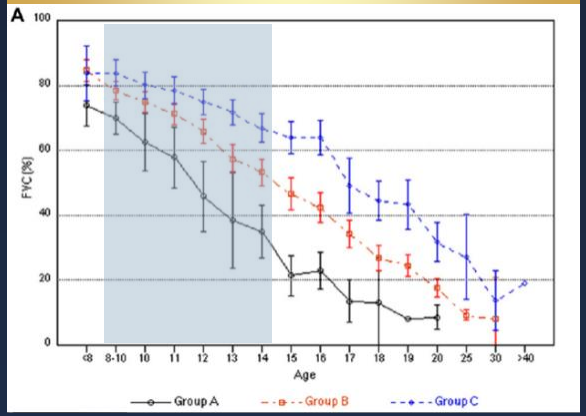
Pulmonary Function

Pulmonary Function: Eteplirsen-treated Patients (N=12) Remain Relatively Stable through Week 192

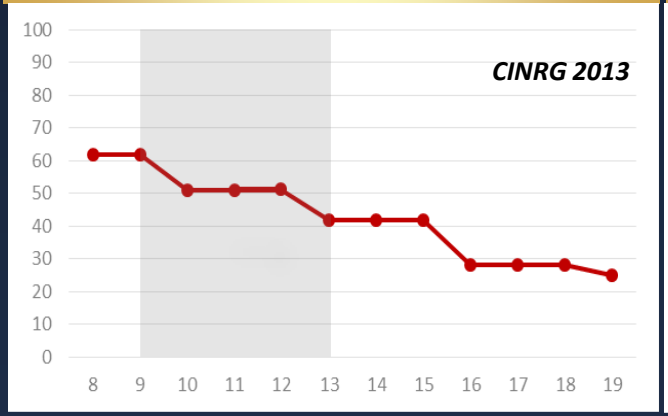


NATURAL HISTORY SHOWS STEADY DECLINES OVER TIME IN PULMONARY FUNCTION IN DMD PATIENTS

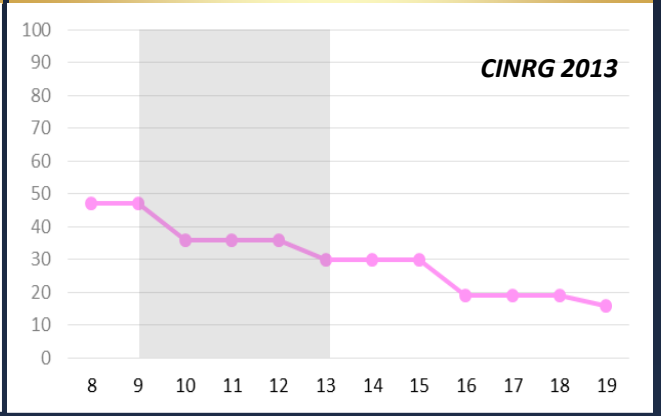
FVC % Predicted



MIP % Predicted



MEP % Predicted



MEP, maximum expiratory pressure; MIP, maximum inspiratory pressure; FVC, forced vital capacity; BL, baseline.
*Wilson et al. 1984 equations.

Efficacy Summary

SLOWER RATE OF DMD PROGRESSION AT 3 YEARS OBSERVED IN STUDY 201/202 ETEPLIRSEN TREATED PATIENTS AS MEASURED BY MULTIPLE FUNCTIONAL ENDPOINTS

- Slowed disease progression at 3 years compared to external controls amenable to exon 51 skipping
  - At 3 years 6MWT Δ = 151 m, $p < 0.01$
  - Decrease in proportion losing ambulation (17% vs 46%)
- Relative stability in % predicted MIP and MEP over 3 years compared to data from the scientific literature

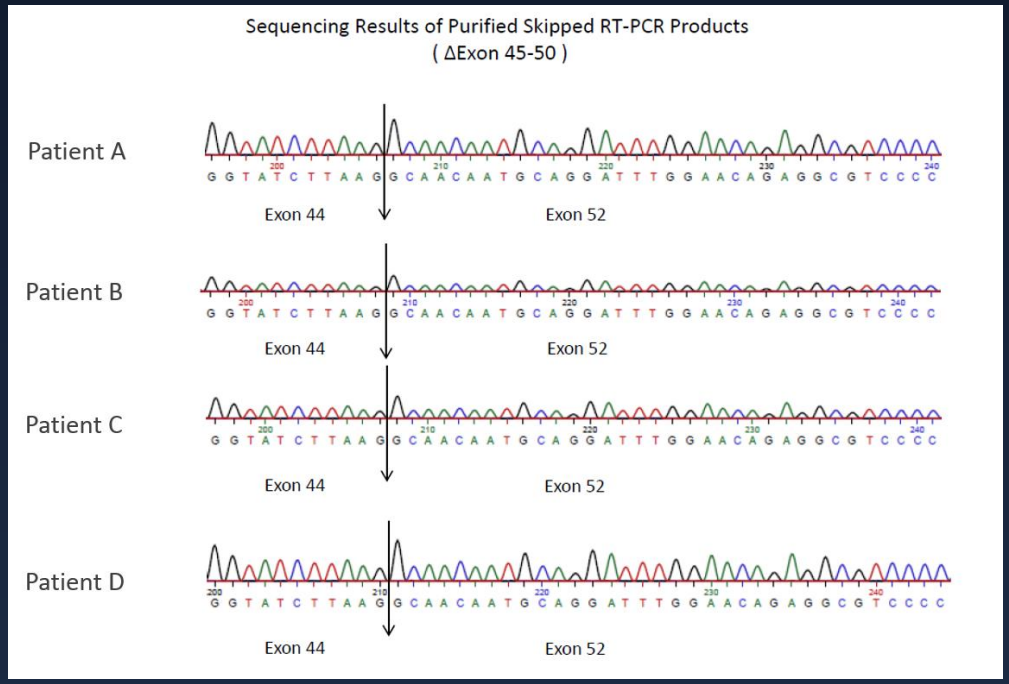
FOURTH BIOPSY

Background on Fourth Biopsy

- 11/12 patients volunteered for surgical biopsy
  - All protocols designed in collaboration with FDA
- Measurements of mechanism of action
  - RT-PCR
- Measurements of dystrophin expression
  - Percent dystrophin-positive fibers
  - Dystrophin signal intensity
  - Western blot
- Measurements were blinded, randomized, and analyzed by 4 independent pathologists

Demonstration of Exon Skipping by RT-PCR and Confirmed by Sequencing

- 4th Biopsy: All (100%) patients (N=11*) demonstrated exon 51 skipped mRNA product after 180 weeks of treatment was present
- In-frame mRNA transcripts as a result of exon 51 skipping confirmed by sequencing in all patients



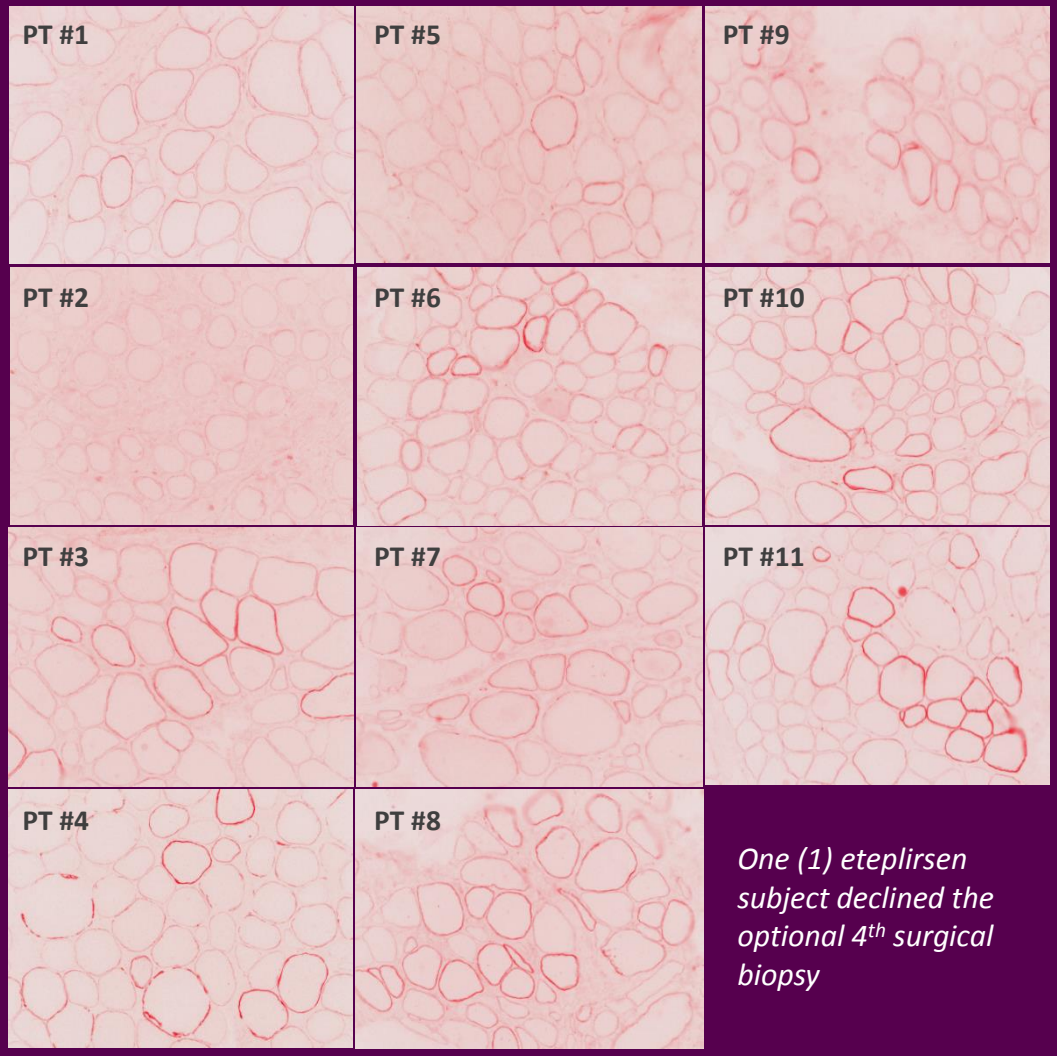
100% of patients dosed with eteplirsen in completed clinical studies to date demonstrated exon skipping (N=36)

*1 boy opted out of the voluntary surgical biopsy.

Dystrophin-positive Fibers Visibly Present in Eteplirsen-treated Patients' 4th Biopsies Compared to DMD Exon 51 Skip Amenable Control Biopsies

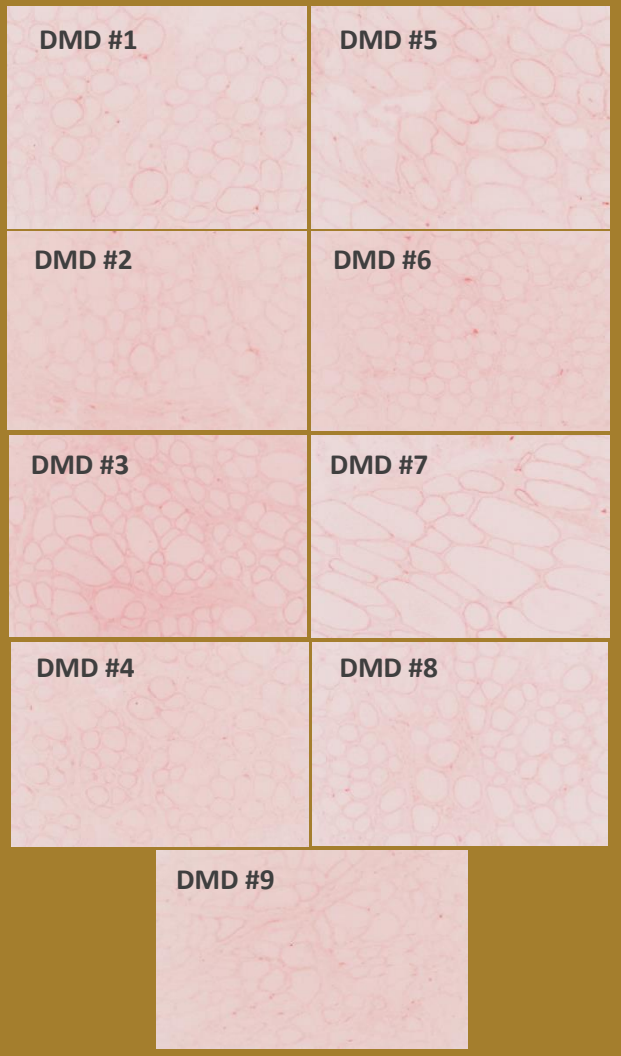
DYSTROPHIN DETECTED IN 10/11 BIOPSIES vs NO DYSTROPHIN-POSITIVE FIBERS IN ANY DMD 51 CONTROLS

Eteplirsen-Treated Week 180



One (1) eteplirsen subject declined the optional 4th surgical biopsy

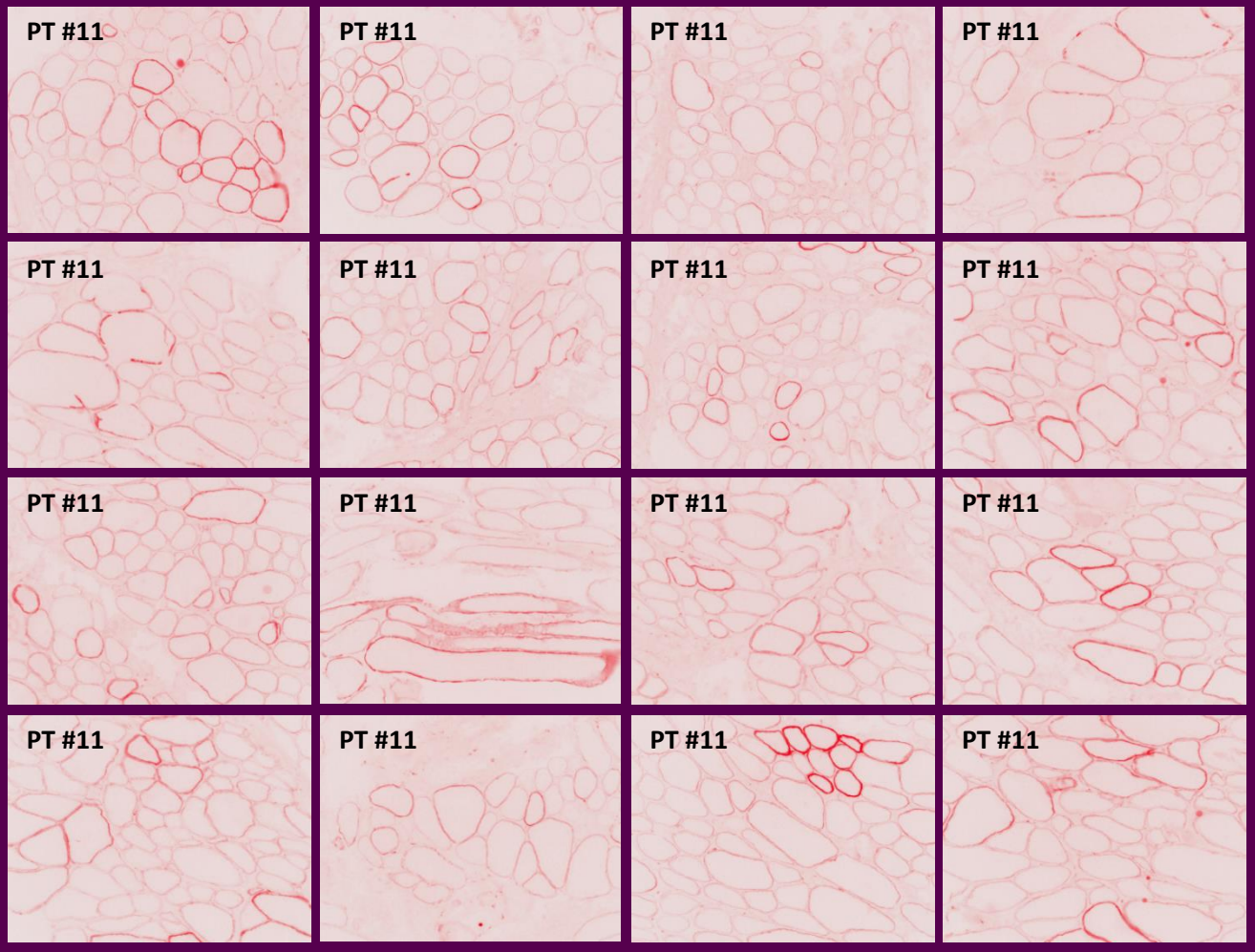
Untreated Exon 51 DMD Controls



Patient #11: All 4th Biopsy Images Compared to Exon 51 DMD Skip Amenable Controls at Week 180

SUBJECT 11: EVERY IMAGE OF 4TH BIOPSY SHOWN BELOW TO SHOW MULTIPLE LAYERS OF BIOPSY

Eteplirsen-Treated Week 180: Every Biopsy Image Patient #11

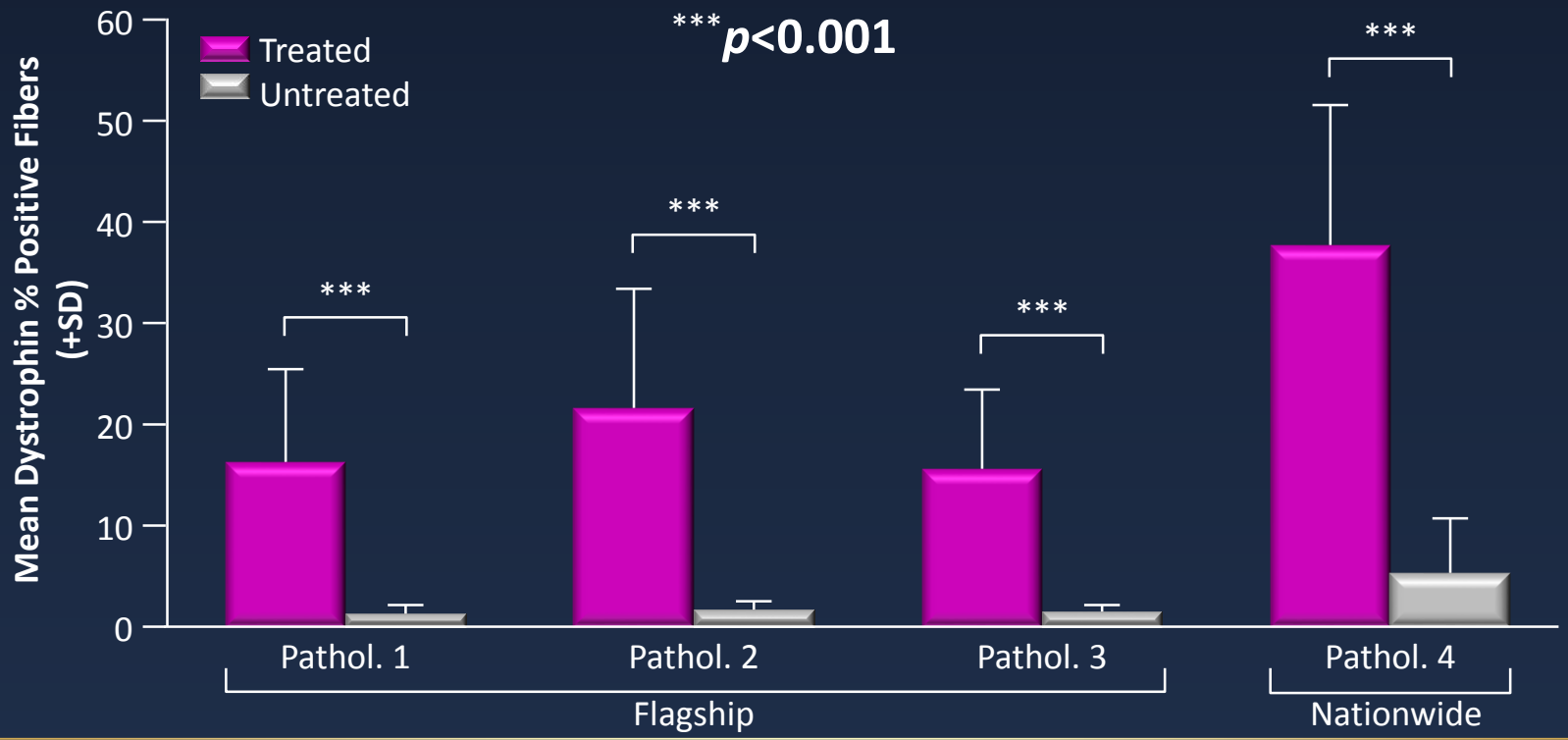


Untreated DMD 51 Controls

Statistically Significant Increase in Percent Positive Fibers Observed in Treated vs. Control

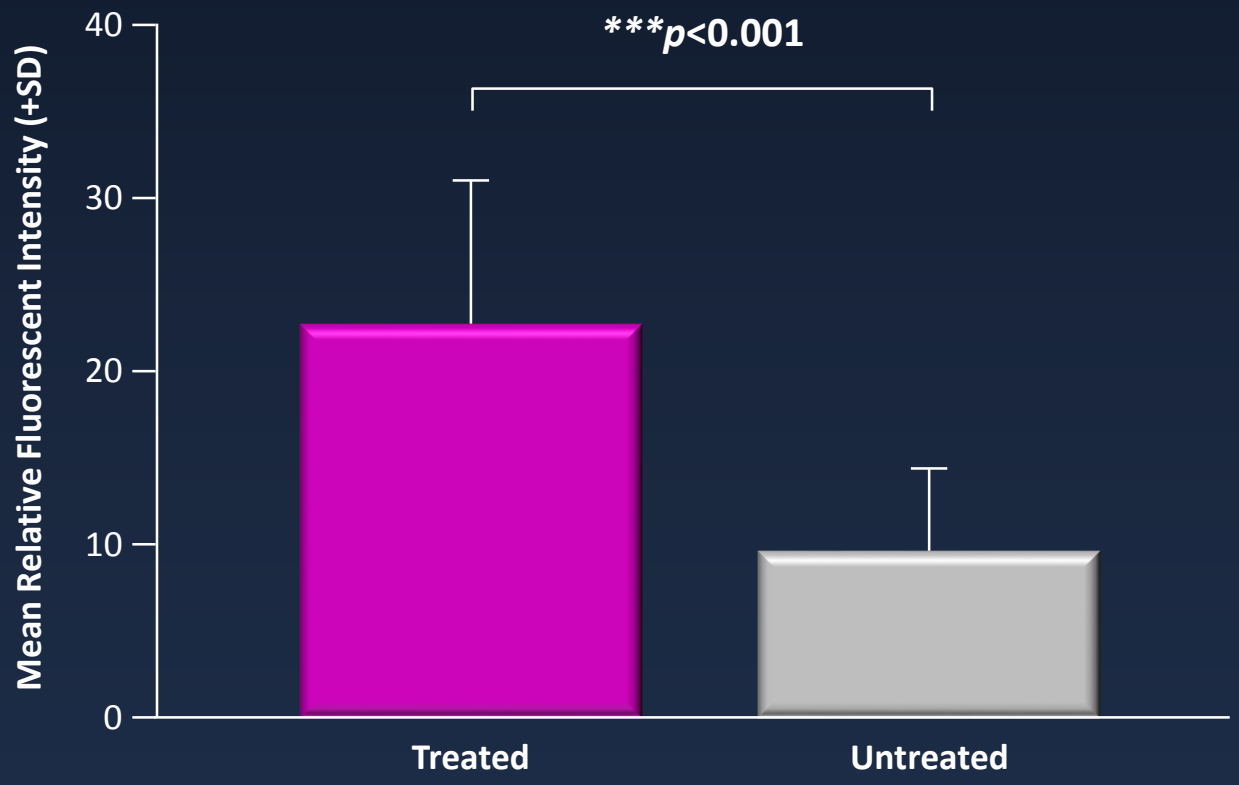
PROTOCOL REVIEWED BY FDA PRIOR TO EVALUATION OF TISSUE BY 4 BLINDED PATHOLOGISTS

	Flagship	Nationwide
Increase in Detected Dystrophin Positive Fibers Treated vs Untreated	1453%	641%



Mean Fluorescence Intensity Demonstrated a Statistically Significant Increase in Treated vs Controls

Increase in Detected Dystrophin Treated vs Untreated	140%



Role of Western Blot

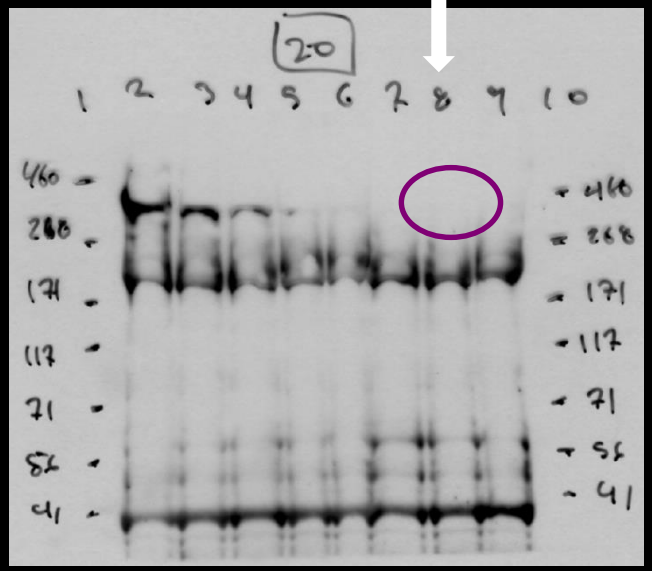
- Primary diagnostic tool prior to the introduction of genetic testing
  - Absence of band confirmed DMD
  - Presence of band confirmed BMD
- Nine of 11 eteplirsen-treated patients had an observable dystrophin band
- Nine untreated DMD controls amenable to skipping exon 51 used as comparator group
- One out of 9 controls had an observable band

DMD, Duchenne muscular dystrophy; BMD, Becker muscular dystrophy.

Western Blot Results vs Baseline Patient A

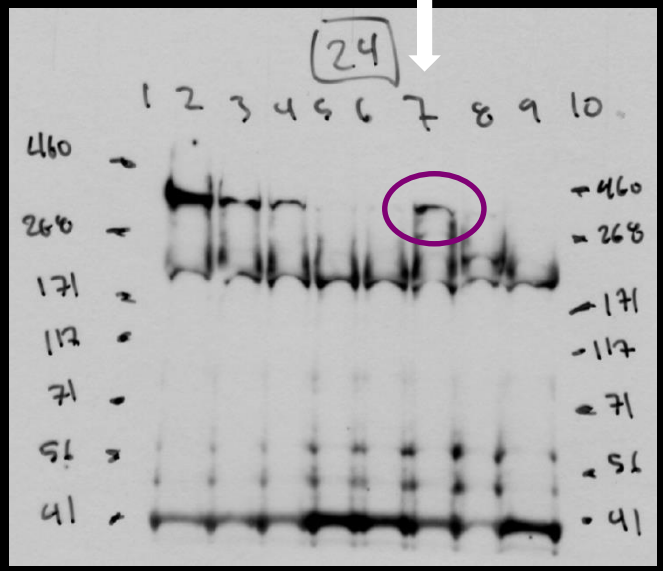
WESTERN BLOT

Patient A Baseline **(Untreated)**



Dystrophin Band (DYS1): Absent

Patient A Week 180 **(Treated)**



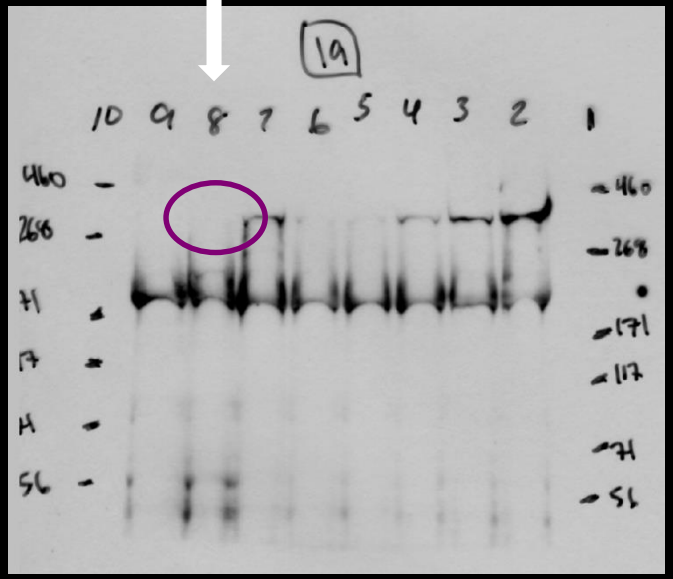
Dystrophin Band (DYS1): Present

Consistent 50 µg total protein per lane loaded
Exposure time 30 minutes per gel

Western Blot Results vs Baseline Patient B

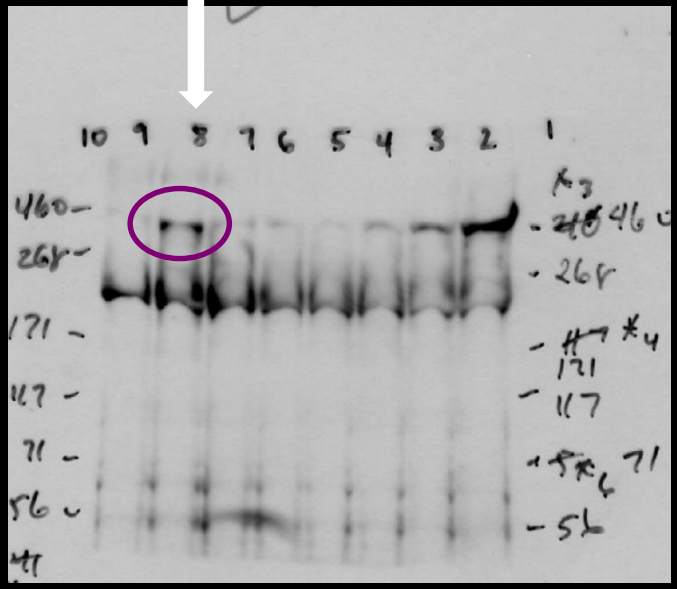
WESTERN BLOT

Patient B Baseline **(Untreated)**



Dystrophin Band (DYS1): Absent

Patient B Week 180 **(Treated)**



Dystrophin Band (DYS1): Present

Consistent 50 µg total protein per lane loaded
Exposure time 30 minutes per gel

Fourth Biopsy Summary: Following Eteplirsen Treatment, Increased Dystrophin Expression Confirmed by All Quantification Methods

- RT-PCR - Exon skipping in 100% of patients and all confirmed by sequencing

Eteplirsen-treated vs Untreated Exon 51-amenable DMD Controls:

- Dystrophin Intensity ($p<0.001$)
  - Automated quantification of dystrophin intensity at the sarcolemma using BIOQUANT® software confirmed statistical significant increases
- % Dystrophin-positive Fibers ($p<0.001$)
  - 4 blinded pathologists independently scored and confirmed statistically significant increases in dystrophin-positive fibers compared to DMD control biopsies
- Western Blot
  - Presence of dystrophin protein confirmed in 9 of 11 (82%) of eteplirsen patients at Week 180 vs 1 of 9 (11%) in the DMD control biopsies

Rescore of Percent Dystrophin-positive Fibers in Study 201

Background on Rescore

Original assessment of dystrophin-positive fibers in Study 4658-201:

- Scored by one blinded pathologist
- Met primary endpoint
  - Statistically significant increase in % dystrophin-positive fibers of eteplirsen-treated patients at Week 24 compared to baseline

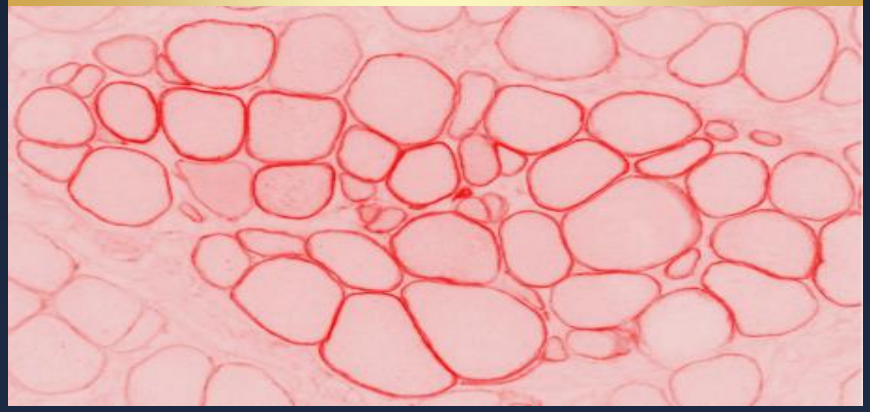
Re-assessment of dystrophin-positive fibers in Study 4658-201:

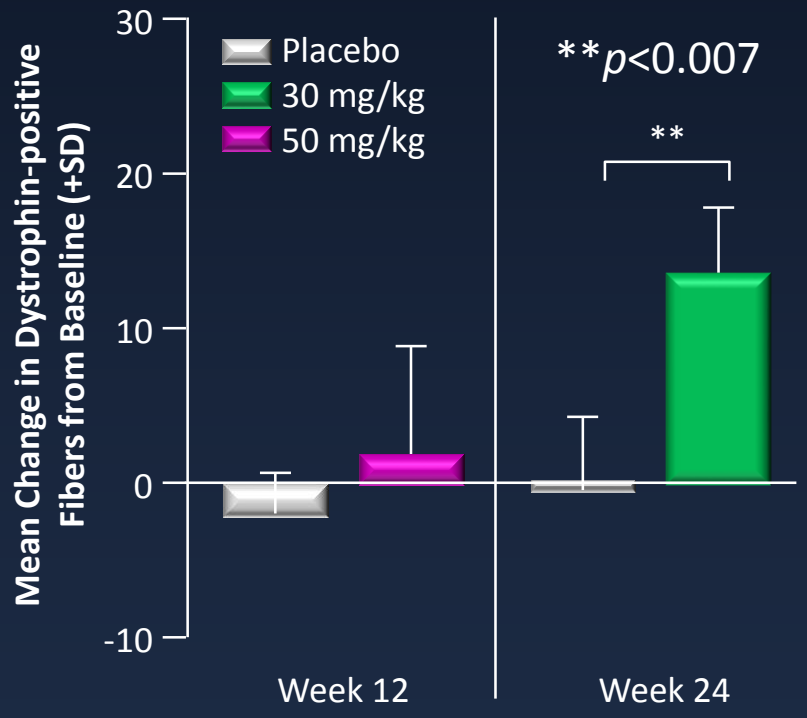
- Scored by 3 independent blinded pathologists, plus blinded nationwide pathologist (total 4)

3 Independent Pathologists Confirmed Statistically Significant Change in Dystrophin-positive Fibers From Baseline

- 30 mg/kg: Statistically significant increase of dystrophin-positive fibers from baseline at Week 24, which confirms previously announced result
- 50 mg/kg: No significant change from baseline at Week 12, which demonstrates a delay in production as expected
- Placebo: No significant change from baseline at Weeks 12 and 24, which confirms previously announced result

Patient on 30 mg/kg at Week 24





Inter-rater reliability (ICC = 0.793) and intra-rater reliability (ICC = 0.944) showed excellent level of concordance among 3 independent pathologists for all treatment groups

Safety

Eteplirsen Safety Database in the NDA (N=72)

Study & Description	Dose (mg/kg)	Route	Duration of Dosing	N
Study 33 *Proof-of-concept*	0.09, 0.9	IM	Single dose	7
Study 28 *Dose ranging*	0.5, 1, 2, 4, 10, 20	IV†	12 weeks	19
Study 201/202 *Double-blind, placebo-controlled/ open-label extension*	30, 50	IV†	~3 years	12
Studies 301 & 204 *Recently initiated*	30	IV†	12-24 weeks	12
	30	IV†	<12 weeks	22
ALL ETEPLIRSEN TREATED PATIENTS				***72***

- 46 patients (46 patient-years) exposed to ≥30 mg/kg proposed clinical dose

†Dose administered once weekly

Enhanced Safety Database Submitted in the NDA

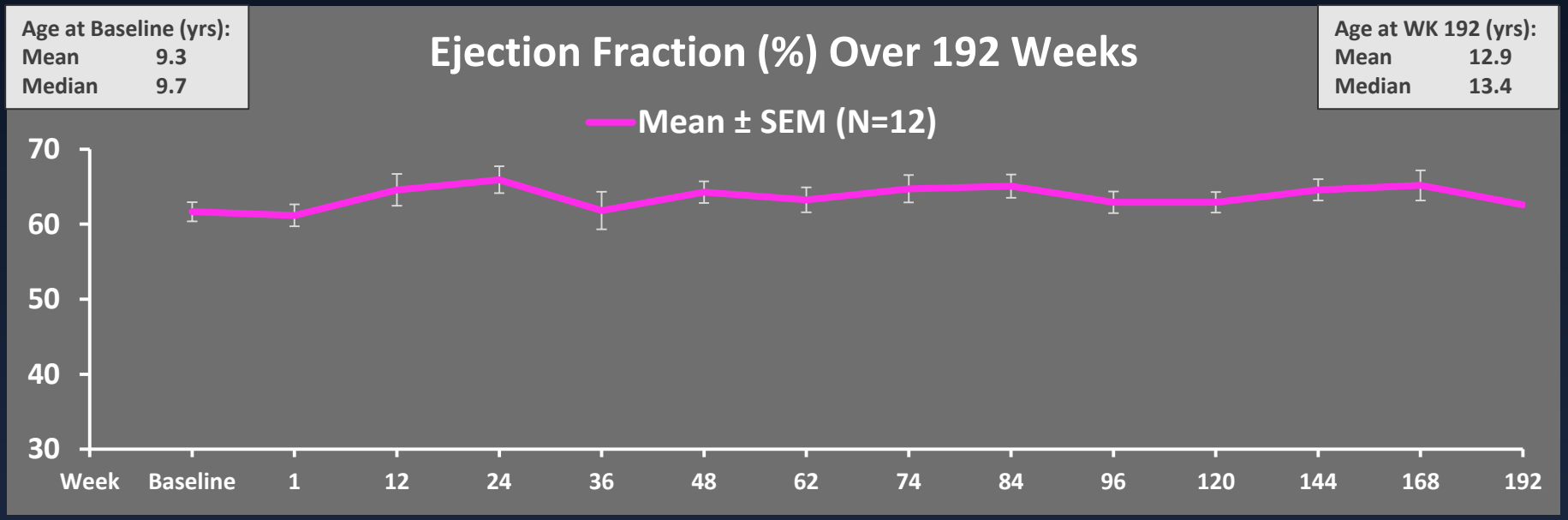
- NDA submitted with 72 total patients with 46 patient years of experience at ≥30mg/kg with >2600 doses provided in the NDA
  - 12 patients treated for over 3 years
  - 12 patients treated for 3–6 months
  - 114 patients to be included in next safety data cut (120-day update)
- Most common adverse events were mild and unrelated to study drug similar to 201/202
- Adverse drug reactions include flushing, erythema, and mild temperature elevation
- No drug-related serious adverse events
  - No evidence of drug-related renal, hepatic, coagulation, or severe cutaneous AESIs*
    - No elevated GGT, glomerular nephritis, hepatocellular injuries
    - No clinically significant infusion-site reactions with associated ulcers
    - No thrombocytopenia, intra cranial venous sinus thrombosis, intracranial hypertension
    - No drug related alopecia

*AESI, adverse event of special interest observed with phosphorothioate anti-sense oligonucleotides

Week 192 Safety Update: No Missed Doses Due to Drug-related Adverse Events Through Week 192

- Total doses administered >2300 at Week 192
- Majority of missed doses due to planned family vacations
- No missed doses due to eteplirsen-related adverse events (AEs)
- No eteplirsen-related serious AEs
- No intermittent dosing needed
- No drug holidays, no dose reductions, no discontinuations
- No hospitalizations due to drug-related AEs
- Most common AEs were mild and unrelated to study drug (201/202)
- Adverse drug reactions to Eteplirsen include flushing, erythema, and mild temperature elevation

Mean Ejection Fraction Through 192 Weeks (ITT; N=12)



NATURAL HISTORY SHOWS CARDIAC HEALTH DECREASES OVER TIME AS BOY AGES AND DISEASE PROGRESSES

Mean Change From Baseline to Week 192	
Mean All Groups	1.49%

No evidence of declining left ventricular ejection fraction at Week 192 of eteplirsen treatment

Summary of Key Results: Totality of the Data

Clinical Efficacy	• 151-meter advantage in 6MWT of eteplirsen-treated patients compared to external control at 3 yrs ($p<0.01$) • Pulmonary stability
Biochemical Efficacy (Supportive Efficacy)	• 4th Biopsy (Voluntary: 11 patients) at week 180 – RT-PCR: 100% of patients demonstrated exon skipping, mechanism of action confirmed – Dystrophin intensity: $p<0.001$ compared to DMD controls – % dystrophin-positive fibers: $p<0.001$ vs DMD controls – Western blot: protein production confirmed in 9 of 11 patients • Rescore of Weeks 12 & 24 – 30 mg/kg: Statistically significant increase of dystrophin-positive fibers from baseline at Week 24, which confirms previously announced result $p<0.007$
Safety	• Drug continues to remain well tolerated after >2300 doses in 201/202 • No injection-site reactions, thrombocytopenia, coagulation, pulmonary embolisms, or renal and hepatic impairment • No hospitalizations due to drug-related adverse events • No decrease in ejection fraction

Sarepta's Vision

OUR MISSION IS TO FIND A TREATMENT FOR EVERY BOY WITH DMD: EVERY MINUTE MATTERS

- We are committed to developing therapies for patients with DMD regardless of underlying mutation
  - Eteplirsen for exon 51 skipping under FDA review and is the key for PMO exon skipping in DMD
    - Four clinical trials ongoing, over 100 patients will be receiving eteplirsen once trials fully enroll
      - Meeting with EMA and hiring key personnel in 2016 for EU strategy
- Exons 53 & 45
  - Clinical trials underway in US and EU, will enroll over 100 patients
- Our goal is to develop treatments for 8 exons by 2018
  - Working collaboratively with the FDA & EMA to determine an approval path in rarer mutations
  - Working internally on exons 55, 52, 50, 35, 8, and beginning collaboration on exon-2 duplication
- Evaluating approaches beyond exon skipping to bring disease-modifying treatments to all patients with DMD

PMO, phosphorodiamidate morpholino oligomer; EMA, European Medicines Agency.

Panel Discussion

Thank you

Line open for questions