SAREPTA THERAPEUTICS

REALIZING THE POTENTIAL OF RNA-BASED TECHNOLOGY

STIFEL HEALTHCARE CONFERENCE 2014

NOVEMBER 19, 2014



FORWARD LOOKING STATEMENTS

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 being well capitalized; our manufacturing capabilities, including the use of our new manufacturing facility; advancing the regulatory and clinical pathway on the eteplirsen program; the potential market and percentage of patients that could be treated with our DMD product candidates; our plans and ability to comply with FDA requirements to consider an NDA submission for eteplirsen complete including conducting additional eteplirsen clinical trials and providing additional data, analysis and other information to the FDA; the potential timing of a submission by us and a filing and acceptance of an NDA for eteplirsen by the FDA and other planned pre-approval and approval activities on an accelerated or other pathways; advancing follow-on exons into and within human clinical trials; the broad potential of and our plans to continue advancing our PMO technology and chemistries in DMD and into additional disease areas, including through collaborations; our preparedness and plans to allow for a global response to Ebola and conduct well controlled clinical trials; our plans for and programs underway for infections of highest medical need and focus on bacterial strains and efficacy of PPMOs; our plans to continue progressing our chemistry platform into multiple programs showing promise including myostatin, pompe and progeria; plans for our program focused on toll like receptors and its potential broad range of applicability in multiple diseases; our beliefs regarding the potential of and safety and efficacy of our product candidates in DMD and rare and infectious diseases; and the timing of and the expected or planned research, development, clinical and regulatory progress for our product candidates. Forward-looking statements also include those made during the presentation regarding future business developments and actions and the timing of the same, including our ability to establish and protect intellectual property rights and commercialize our product candidates without claims of infringement and the potential use of Sarepta's Ebola drug candidate to treat patients.

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: we may not have sufficient funds to execute our business plans; our product candidates may fail in the research, development or commercialization process for various reasons; we may not be able to comply with all regulatory requests and requirements for the research, development and commercialization of our product candidates; the FDA may determine that substantial additional data is required for accelerated or other approval of eteplirsen or that our NDA submission for eteplirsen does not qualify for filing, even with additional information; the results of our ongoing research and development efforts and clinical trials may not be positive or consistent with prior results; there may be delays in timelines relating to an NDA submission, initiating clinical trials, or making a product commercially available for regulatory or internal reasons; we may not be able to manufacture sufficient drug supply for our studies or commercialization; agency or court decisions with respect to our patents or those of third parties may negatively impact our business; our Ebola drug candidate may not be effective in humans and those risk identified under the heading "Risk Factors" in Sarepta's Quarterly Report on Form 10-Q for the quarter ended September 30, 2014 filed with the Securities and Exchange Commission (SEC), and Sarepta's other filings with the SEC.

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.

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CORPORATE HIGHLIGHTS

- Well capitalized with ~$240 million in cash as of 9/30/2014



- Corporate growth in 2014
  - Opened corporate HQ and research lab in Cambridge, MA
  - Acquired manufacturing facility on 26 acres of land in MA
  - Research & synthesis capabilities expanded in MA & OR
  - ~200 employees; ~40 with PhD and/or MD degrees
- Pioneering pathway in lead DMD clinical programs
  - Focus remains on DMD – advancing eteplirsen towards regulatory approval, bringing follow-on exons into human clinical trials
- Broad potential with PMO technology and chemistry
  - Significant progress made advancing chemistry platform into additional indications in genetic diseases and against viral and bacterial infections

DUCHENNE MUSCULAR DYSTROPHY

DEVASTATING RARE DISEASE WITH HIGH UNMET NEED

- Affects approximately 1 in 3,500 boys worldwide
- 25,000- 30,000 patients in the U.S. and Europe
- Sarepta's lead program (Exon 51) estimated to benefit ~13% of DMD patients
- Follow-on Exon-Skipping Drugs have potential to treat 60-80% of DMD patients



Relentless Progression of DMD

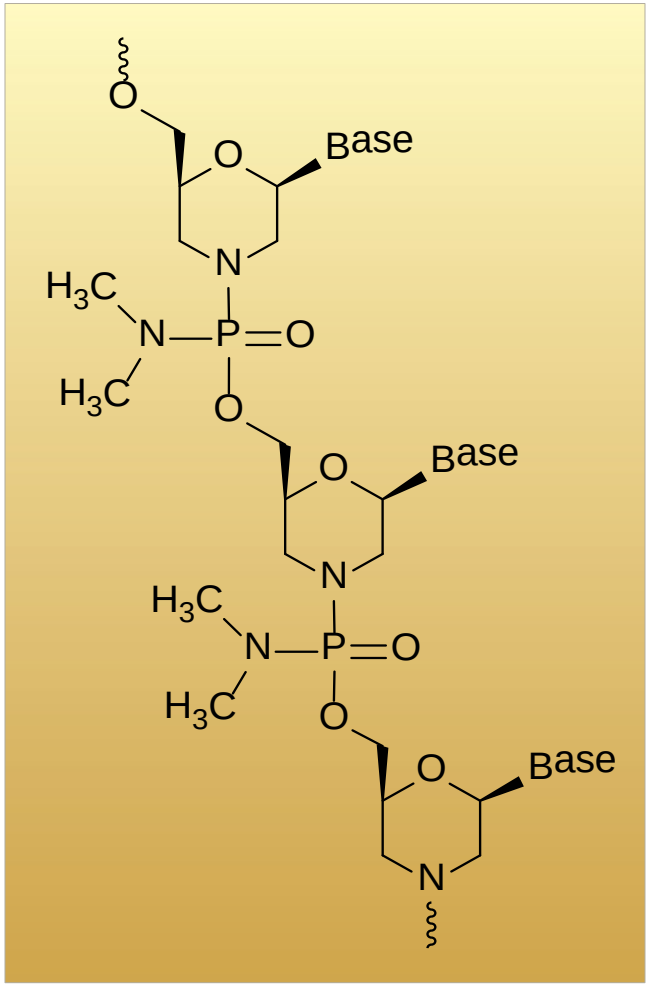
Delayed Milestones in Early Years	DMD Patients Begin Decline Around Age 7	Pulmonary Function Begins to Decline	Loss of Ambulation Age 10 - 15	Decline in Upper Body Muscle and Respiratory Function	Death in Mid/Late 20's

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PMO FOR THE TREATMENT OF DMD

CHRONIC LIFELONG THERAPY DEMANDS SAFETY

- >1,800 doses, representing ~44 patient years across all studies, given to DMD boys with doses up to 50 mg/kg/wk for nearly 3 years without clinically significant treatment-related adverse events
- Does not activate innate immune system through Toll-like receptor (TLR) binding
- Charge neutral PMO chemistry minimizes protein binding to prevent off-target effects
- Plasma half-life of 2 to 6 hours
- Cleared through the kidney
- Sequence specific binding to pre-mRNA directs alternative splicing



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DMD CLINICAL AND REGULATORY UPDATE

Dosing Initiated in Eteplirsen Studies

- Nov 17: First patient dosed in Study 301 (Ambulatory Patient Confirmatory Study)
- Nov 12: First patient dosed in Study 204 (Advanced/Non-Ambulatory Patients)
- 1Q2015: First patient to be dosed in Study 203 (Younger Ambulatory Patients – 4 to 6 year olds)

FDA Feedback Received on Dystrophin Reassessment Protocol

- Rescoring of Dystrophin-Positive Fibers by 3 Independent Pathologists Underway

FDA Feedback Received on Master Protocol for Exon 53 and Exon 45 Drugs

- Agreement to proceed with primary endpoint of 6MWT in combined population
- Placebo-controlled study with SRP-4045 and SRP-4053 (2:1 randomization of Tx:Pbo within each population) to begin dosing patients with 30mg/kg in 1H2015
- Study will enroll ambulatory patients 7-16 years and capture similar outcomes as Eteplirsen confirmatory study (dystrophin, other clinical endpoints, safety)

Clinical data to be generated and incorporated into NDA submission (mid-year 2015)

- 168-week clinical data from study 201/202: Topline 6MWT Results planned for 1Q2015
- Safety data in newly exposed eteplirsen patients (subset with at least 3-month safety data)
- Results from 4th Biopsy is expected from subset of patients in Study 201/202

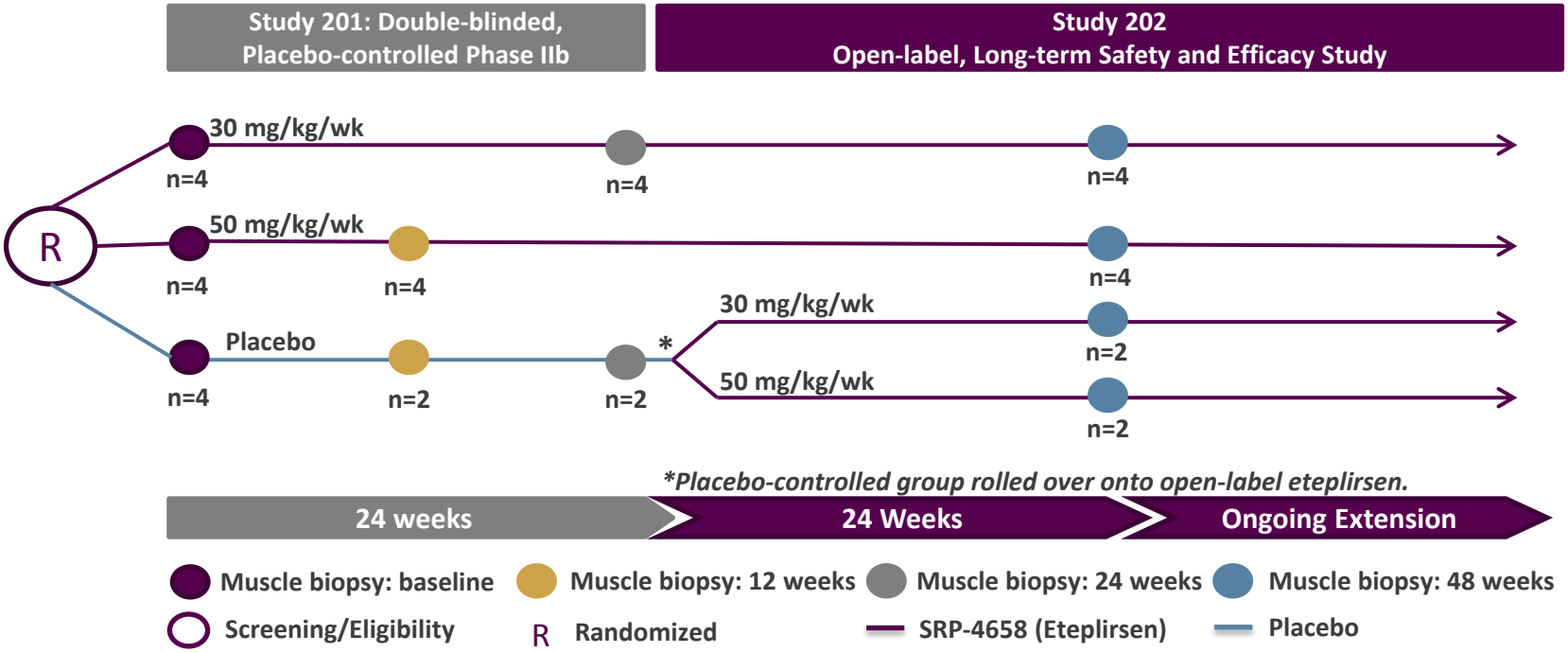
DMD CLINICAL PROGRAM (US AND EU):

ETEPLIRSEN AND FOLLOW-ON EXON-SKIPPING DRUGS (EXONS 45 AND 53)

Study	Duration (weeks)	n	Status	Exon Target Treatment	DMD Population	1^0 Outcome Measure
4658-uk-33	Single Dose	7	Complete	Exon 51		Safety; Dystrophin
4658-uk-28	12	19	Complete	Exon 51	5-15 yrs, amb	Safety; Dystrophin
4658-us-201	24	12	Complete	Exon 51	7-13 yrs, amb	Safety; Dystrophin
4658-us-202	120	12	Ongoing	Exon 51	7-13 yrs, amb	6MWT; Dystrophin
4658-us-301	48	120	Dosing: Active Enrollment	Exon 51	7-16 yrs, amb	6MWT; Dystrophin
4658-us-204	96	20	Dosing: Active Enrollment	Exon 51	7-21 yrs, non-amb	Safety
4658-us-203	96	20	Final Protocol: Dosing 1Q2015	Exon 51	4-6 yrs, amb	Safety; Dystrophin
4045-us-301	48	90	FDA Agreement on Master Protocol	Exon 45 and Exon 53	7-16 yrs, amb	6MWT; Dystrophin
4053-eu-101	48	36	Enrolling; Dosing 1Q2015	Exon 53	7-16 yrs, amb	6MWT, Dystrophin
4045-eu-301	TBD	TBD	TBD	Exon 45 and Exon 53	7-16 yrs, amb	6MWT; Dystrophin

ETEPLIRSEN PHASE IIb STUDY DESIGN

STUDY 201: RANDOMIZED, DOUBLE-BLIND, PLACEBO-CONTROLLED
STUDY 202: OPEN-LABEL, LONG-TERM SAFETY AND EFFICACY



KEY INCLUSION CRITERIA

- Out-of-frame deletion(s) that may be corrected by exon 51 skipping
- Between the ages of 7 and 13 years
- Between 200 and 400 meters (±10%) on 6MWT at Baseline
- Receiving treatment with a stable dose of oral corticosteroids for at least 24 weeks before study entry

KEY ENDPOINTS

- 6MWT[2]
- % Dystrophin positive fibers[1]
- Pulmonary function tests[3]
- Safety and tolerability
- PK

[1] Primary Endpoint (201/202); [2] Primary Endpoint (202) & Secondary Endpoint (201); [3]Exploratory Endpoint

PATIENT CHARACTERISTICS AT BASELINE

STUDY 201: RANDOMIZED, DOUBLE-BLIND, PLACEBO-CONTROLLED
STUDY 202: OPEN-LABEL, LONG-TERM SAFETY AND EFFICACY

	Cohort	N	Age (yrs) mean	Weight (kg) mean	Height (cm) mean	BMI (kg/m²) mean	6 MWT (m) mean**
mITT† (n=10)	**Eteplirsen**	6	**9.4**	29.4	122.2	19.5	388.6
	PBO/Delayed-Tx*	4	**8.8**	30.7	119.3	21.5	380.3
ITT (n=12)	**30 mg/kg**	4	**9.8**	34.9	130.5	20.3	347.3
	50 mg/kg	4	**9.1**	29.1	121.3	19.6	384.8
	Total *(Min, Max)*	12	***9.3 (7.3, 11.0)***	*31.5 (22.1, 39.8)*	*123.7 (116, 138)*	*20.5 (16.4, 25.6)*	*370.8 (259, 437)*

* Placebo/delayed-treatment cohort at 36 weeks had mean age of 9.5 years and mean 6MWT of 327.5 meters.

** 6MWT baseline values per patient were collected on 2 consecutive days, mean is based on average of both values.

† The Modified-Intent-To-Treat (mITT, n=10) patient population excluded two patients in the 30-mg/kg eteplirsen treated cohort who showed rapid disease progression upon enrollment and lost ambulation proximate to Week 24.

ROBUST BIOLOGIC RESPONSE OBSERVED ACROSS ALL STUDIES WITH ETEPLIRSEN

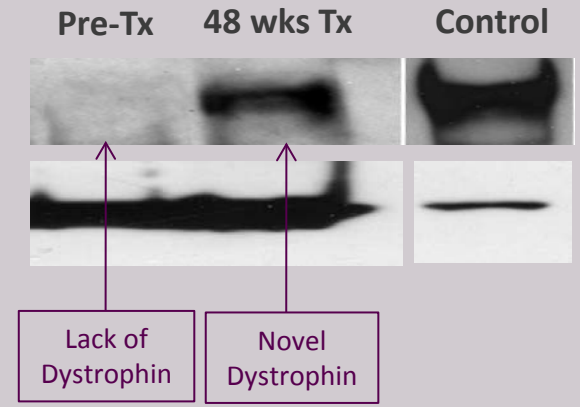
EXON SKIPPING CONFIRMED via RT-PCR; DYSTROPHIN INCREASE via IMMUNOFLUORESCENCE; WESTERN BLOT (w/ DYS1 ANTIBODY) TESTED ON ONE SUBJECT WITH CLEAR PROTEIN EXPRESSION

201/202

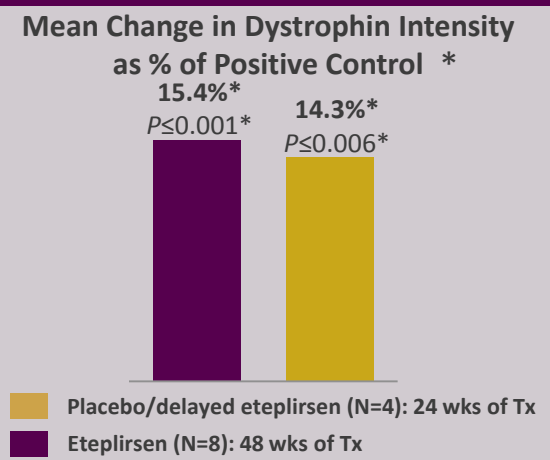
RT-PCR



Western Blot



Immunofluorescence



Eteplirsen Treatment resulted in:	Study 33 (N=7)	Study 28 (N=17)	Study 201 (N=12)	Study 202 (N=12)
Exon 51 skipping as measured by RT-PCR	✓	✓	✓	✓
Novel dystrophin production as measured by IF and/or WB	✓	✓	✓	✓

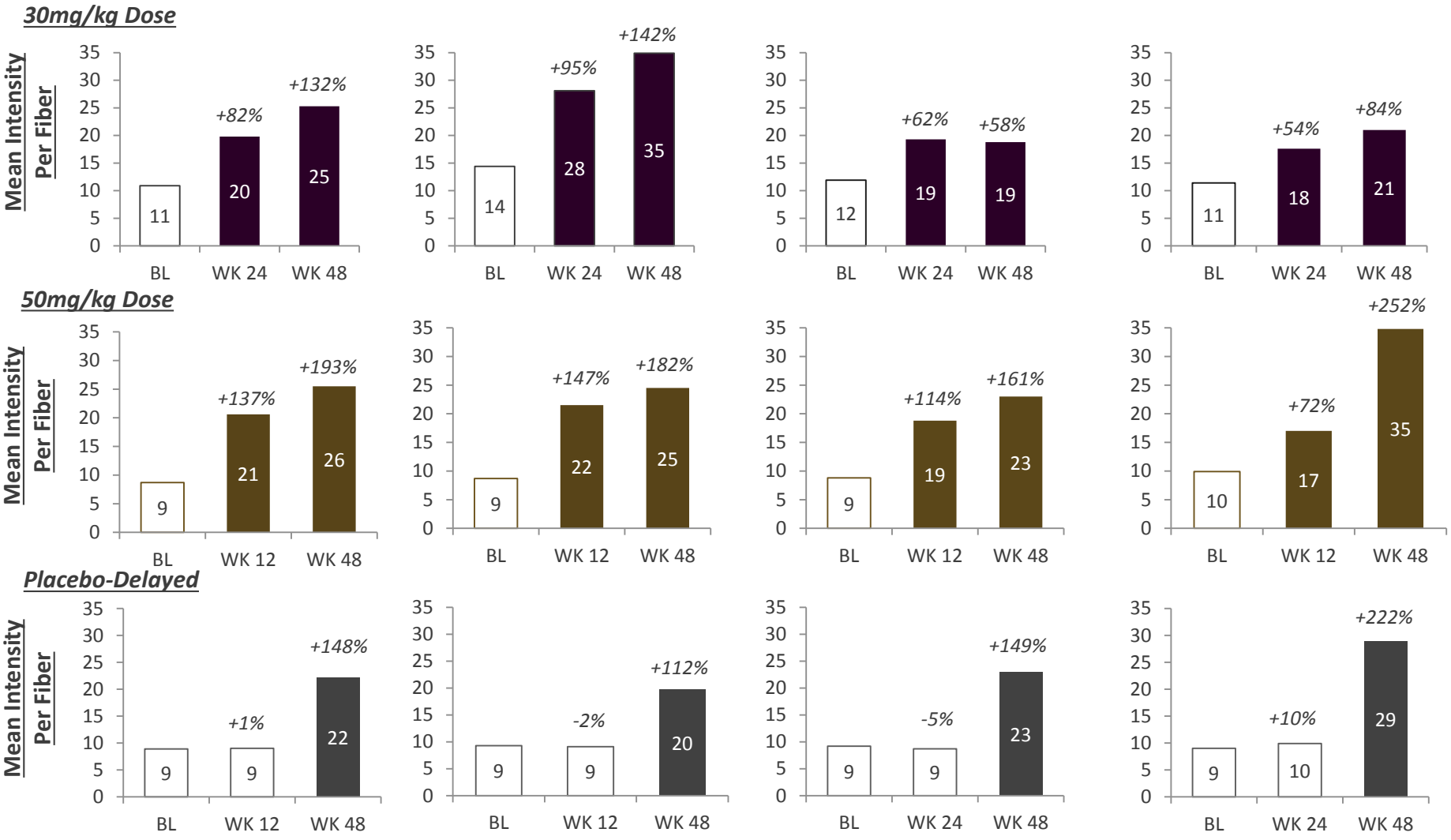
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Mendell JR, et al. *Ann Neurol*. 2013;74(5):637-647.

*Results based on 40x magnification of image using BioQuant Software

OBJECTIVE MEASURE OF DYSTROPHIN INTENSITY INCREASED WITH ETEPLIRSEN TREATMENT IN ALL PATIENTS

RELATIVE INCREASES IN DYSTROPHIN OF ~100%-200% OVER BASELINE



*Results based on 40x magnification of image using BioQuant Software; No significant increase using 20x magnification, which was only used at BL, 12 and 24 wks.

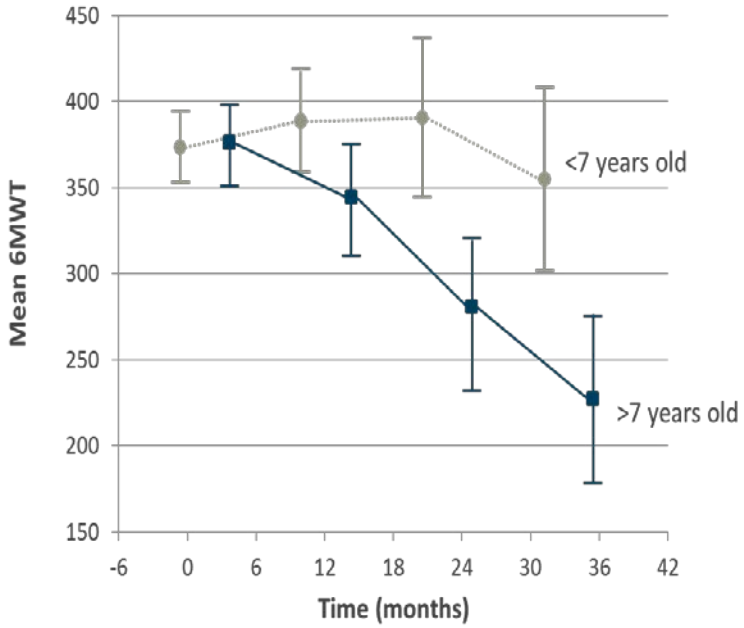
SIX MINUTE WALK TEST (6MWT) IS A WELL ESTABLISHED OUTCOME MEASURE

DMD SHOWS PROGRESSIVE DECLINE IN PATIENTS OLDER THAN 7 YEARS

- 6MWT is an integrated assessment of cardiac, respiratory, and circulatory functions along with muscular capacity
- Natural history studies indicate progressive functional decline in boys over 7 years of age[1,2]

"Those above the age of 7 years showed a progressive deterioration that was much more marked with each increasing year after baseline" [3]

"The sharper progression with each found in our cohort, especially in the older boys >7 suggest that the relatively stable results on these measures over two or three years, as reported in some of these studies, may be related to the beneficial efficacy of the drug as this is not common in untreated boys"[3]



[3]6MWT, 6-minute walk test; DMD, Duchenne muscular dystrophy. Mercuri E, et al. 13th International Congress on Neuromuscular Diseases (ICNMD), July 5-10, 2014, Nice, France.

INCLUDED AS FUNCTIONAL ASSESSMENT IN MANY APPROVED DRUGS

Included as a functional assessment in multiple clinical trials[4]

- Aldurazyme® for MPS I (Hurler, Hurler-Scheie)
- Elaprase® for MPS II (Hunter Syndrome)
- Myozyme® for Pompe disease
- Vimizim® for MPS IVA (Morquio A Syndrome)

Served as the basis for regulatory approval of drugs for a number of rare diseases, with mean changes ranging from 23 to 44 meters[5,6,7,8]

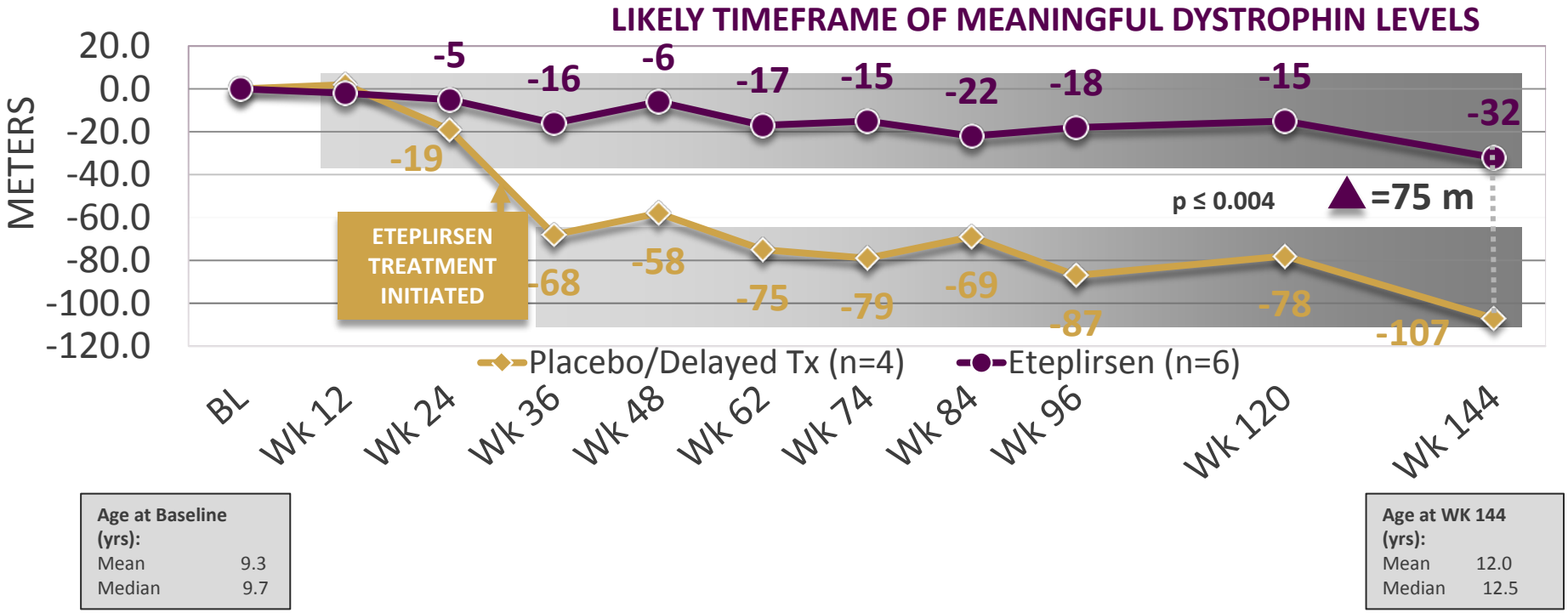
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Note: Aldurazyme and Myozyme are registered trademarks of Biomarin/Genzyme LLC. Elaprase is a registered trademark of Shire Human Genetic Therapies, Inc.

[1]McDonald, Henricson, et al 2013
[2]Mazzone, *et al* 2013
[4]Information obtained from www.clinicaltrials.gov on 24Sep2014
[5]Muenzer et al 2006
[6]Rubin et al, 2002
[7]Wraith et al 2004
[8]van der Ploeg et al,2010

6MWT CHANGE FROM BASELINE TO WEEK 144 IN STUDY 201/202

DATA BASED ON MAXIMUM 6MWT SCORE WHEN TEST WAS REPEATED



Age at Baseline (yrs):	
Mean	9.3
Median	9.7

Age at WK 144 (yrs):	
Mean	12.0
Median	12.5

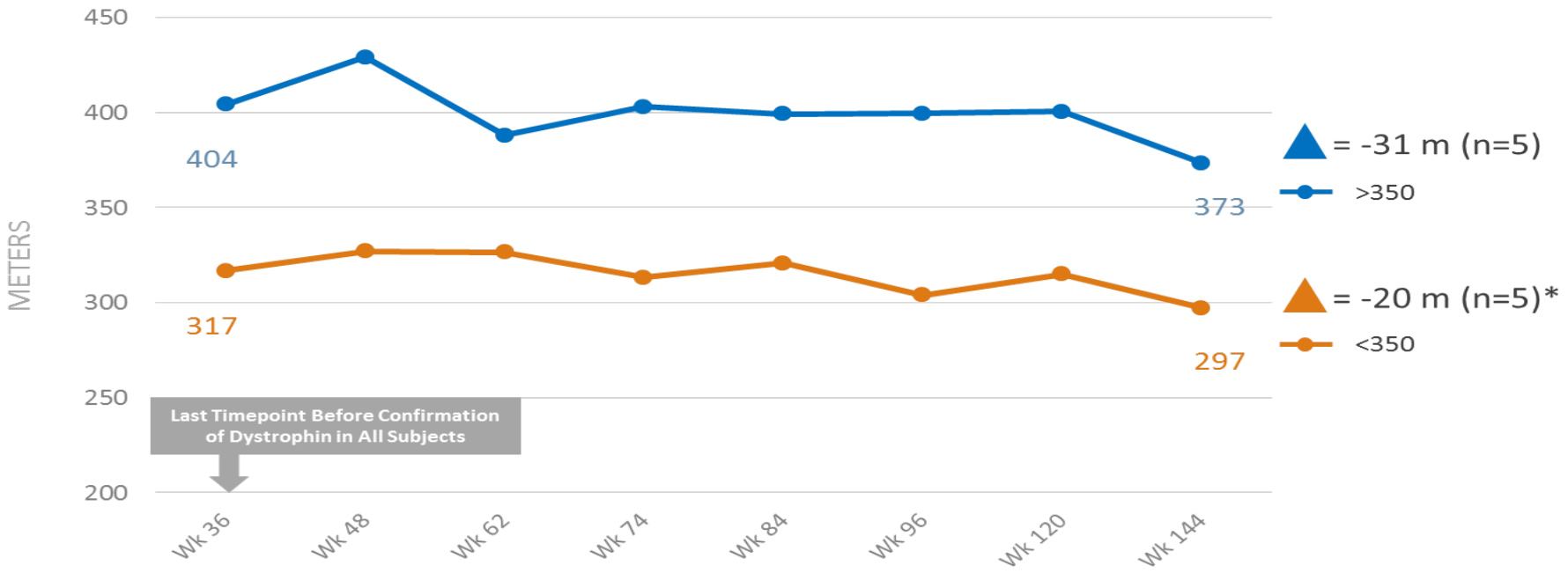
- The eteplirsen treated arm (N=6) lost 30 meters in the 6MWT from week 12 to 144 (2.5 years) once dystrophin production was confirmed
- The placebo delayed treatment arm (N=4) lost 39 meters in the 6MWT from week 36 to 144 (2.0 years) once dystrophin production was confirmed

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Note: Statistical analysis based on modified Intent-To-Treat (mITT, n=10, excludes two patients who experienced rapid decline and lost ambulation early in the study) Population using MMRM Test

6MWT OUTCOMES STRATIFIED BY DISTANCE (<350m v. >350m) AT 36 WEEKS

ETEPLIRSEN TREATED PATIENTS (mITT, n=10) CONTINUE TO BE AMBULATORY AND DEMONSTRATE **SLOWER DECLINE IN WALKING ABILITY THAN NATURAL HISTORY WOULD PREDICT**

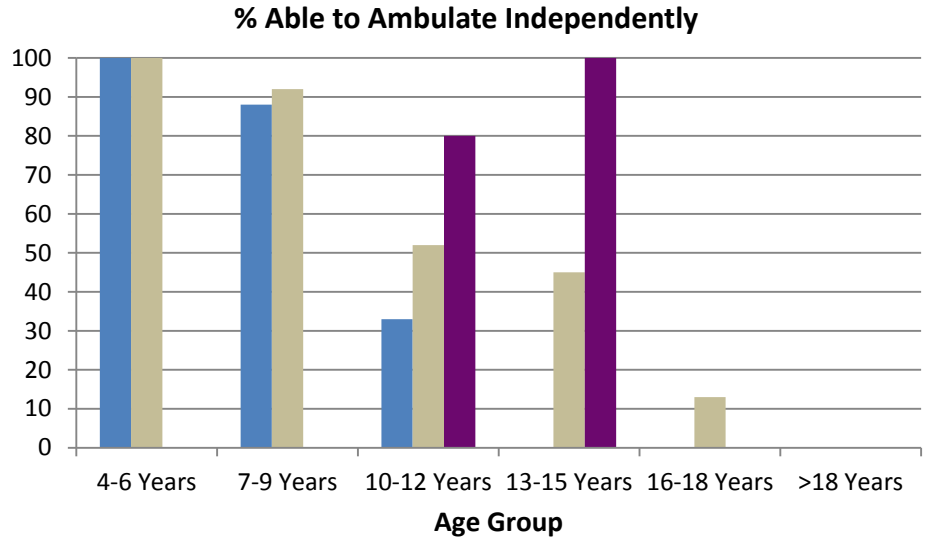


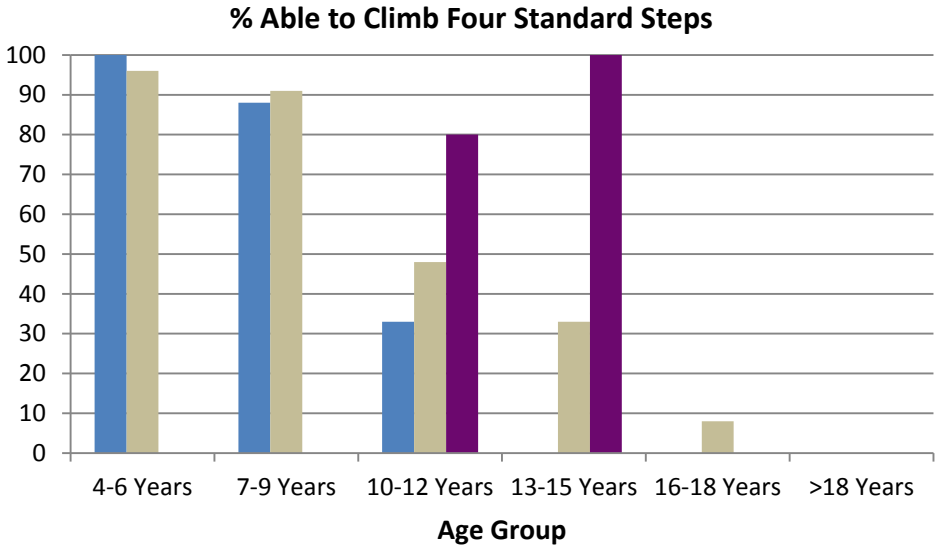
Outcomes with 6MWT was demonstrated for more than two years regardless of the subject walking above or below 350 meters after dystrophin production was confirmed

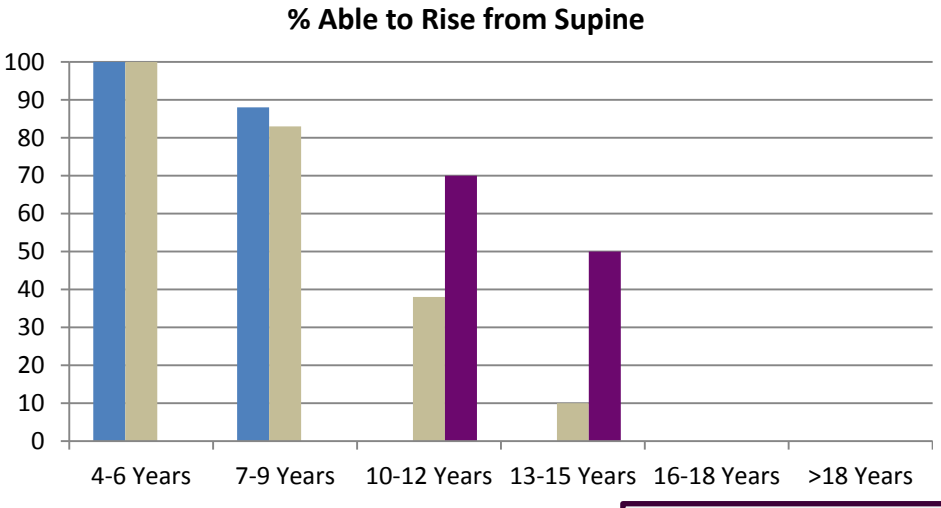
Note: Mean age: 9.9 years at week 36; 12.0 years at Week 144; Includes modified Intent-to-Treat (mITT) Population
**n=4 at week 84 due to a patient recovering from a broken ankle who was unable to participate at this time point*

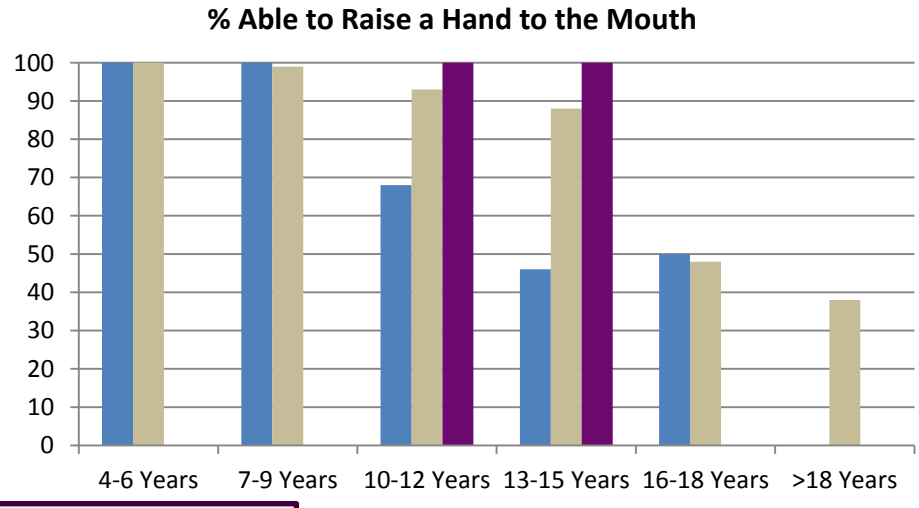
SUBJECTS ON ETEPLIRSEN COMPARED TO PUBLISHED NATURAL HISTORY* (N=12 AT WEEK 144)

TEN BOYS WERE AGED 10-12 (AVG. AGE 11.75) AND TWO WERE AGED 13-15 (AVG. AGE 13.5) AT WEEK 144 (N=12)







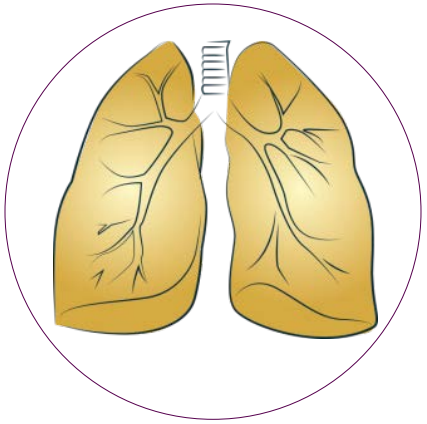


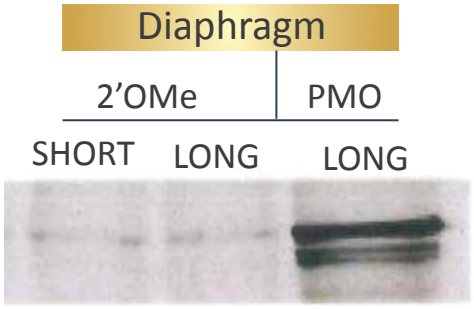


*Henricson, et al. 2013

PULMONARY STABILITY AS AN OUTCOME MEASURE IN DMD

PULMONARY FUNCTION TESTS: PHASE IIB EXPLORATORY EFFICACY ENDPOINTS





RESPIRATORY FUNCTION DECLINE IN DMD

- Respiratory decline begins early in DMD leading to a high morbidity and mortality in late-stage DMD
- Majority of respiratory failures due to ineffective cough and impaired airway clearance

MAXIMUM INSPIRATORY AND EXPIRATORY PRESSURE (MIP AND MEP) AND FORCED VITAL CAPACITY (FVC)

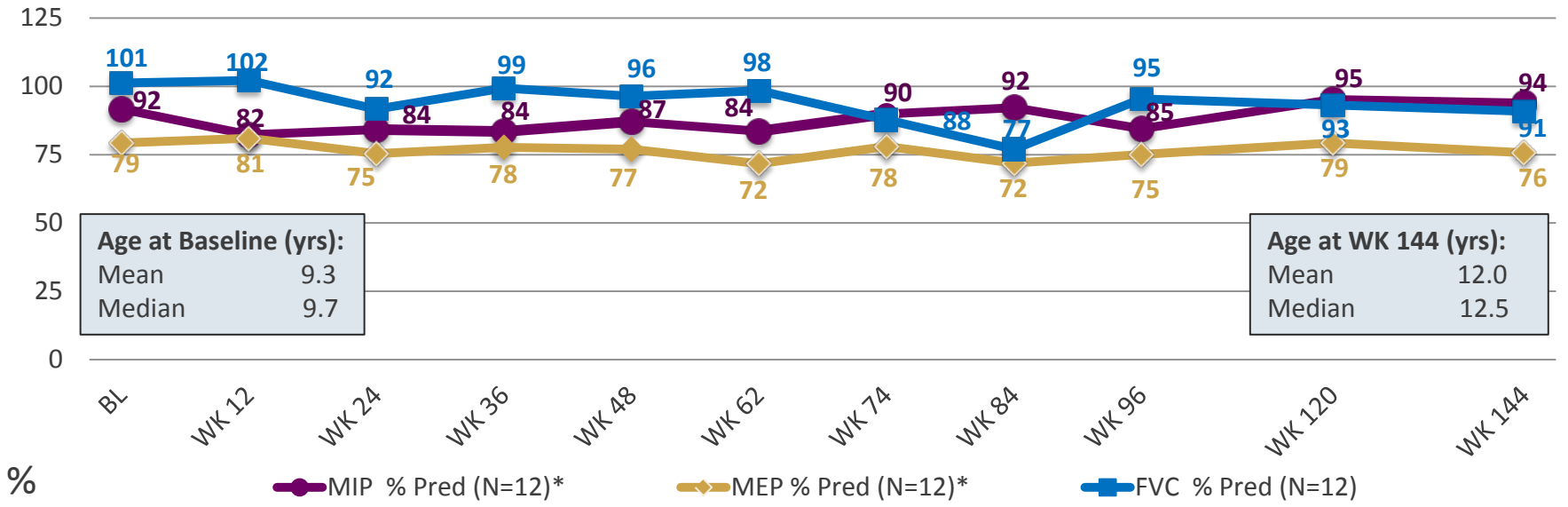
- Sensitive measures of respiratory muscle strength well characterized in the disease natural history
- MEP typically deteriorates before MIP and FVC in DMD patients
- Declines in MIP and MEP correlate with decreases in voluntary cough capacity

Significant increase of dystrophin expression achieved in diaphragm muscle of mdx mouse

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Heemskerk et al, 2009; Braverman et al; Smith et al, 1987; Galasko et al, 1992; Hahn et al, 1997; Kang et al, 2000; Phillips et al, 2001

MIP, MEP & FVC % PREDICTED TO WEEK 144 DEMONSTRATE STABILITY IN INTENT-TO-TREAT POPULATION



PFT	Mean Baseline PFT Value	Mean Week-144 Value	% Change From Baseline
MIP	63.1 cm H_2O	72.4 cm H_2O	+14.7%
MEP	68.1 cm H_2O	76.8 cm H_2O	+12.8%
FVC	1.73 liters	1.92 liters	+11.0%
MIP % Predicted	91.7%	93.9%	+2.4%
MEP % Predicted	79.3%	75.7%	-4.5%
FVC % Predicted	101.3%	90.9%	-10.3%

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* Wilson et al. 1984 equations

LONG-TERM SAFETY PROFILE OF ETEPLIRSEN

STUDY 201/202

- **No clinically significant treatment-related adverse events observed through 144 weeks**
  - One treatment-unrelated serious adverse event: distal femur fracture
  - Two instances of changes to coagulation due to thrombosis in device: port not flushed adequately of heparin
  - Reported cases of transient urine protein elevation resolved without intervention and resulted in no clinical symptoms or other laboratory kidney marker changes
- **No clinically significant treatment-related changes detected on any monitored safety laboratory parameter**
  - Liver-specific enzymes, kidney function, coagulation profiles, or platelet counts
- **No hospitalizations, discontinuations, or treatment interruptions**
- **Well tolerated with >1,600 doses (~42 patient years) administered in study 201/202**
  - No subject missed more than two consecutive doses
    - Missed doses primarily due to vacation and/or summer camp
  - PBO/Delayed-Tx cohort (n=4) completed on average 118.8 out of 120 possible doses
  - Eteplirsen cohorts (n=8) completed on average 142.5 out of 144 possible doses
- **No signs or symptoms of immune activation, including lack of infusion reactions, lack of treatment related hypersensitivity, and no flu-like symptoms**
  - Only one instance of injection site pain reported over nearly three years of weekly infusions
  - No reported incidents of erythema, induration or discoloration at injection sites

LONG-TERM SAFETY PROFILE OF ETEPLIRSEN

ETEPLIRSEN IS WELL TOLERATED WITH >1,600 DOSES (~42 PATIENT YEARS) ADMINISTERED in 201/202

TREATMENT-EMERGENT ADVERSE EVENT	PLACEBO FOR 24 WKS N=4 (%)	ETEPLIRSEN FOR 24 WKS N=8 (%)	ETEPLIRSEN FOR 120 WKS N=4 (%)	ETEPLIRSEN FOR 144 WKS N=8 (%)
Procedural pain	3 (75)	4 (50)	1 (25)	6 (75)
Vomiting	0	3 (38)	2 (50)	4 (50)
Hypokalaemia	2 (50)	4 (50)	0	4 (50)
Cough	2 (50)	2 (25)	1 (25)	3 (38)
Back pain	2 (50)	1 (12)	1 (25)	4 (50)
Fall	1 (25)	1 (12)	0	1 (12)
Headache	2 (50)	1 (12)	4 (100)	4 (50)
Balance disorder	0	3 (38)	0	3 (38)
Diarrhoea	1 (25)	1 (12)	1 (25)	2 (25)
Dermatitis Contact	0	2 (25)	0	3 (38)
Pyrexia	2 (50)	1 (12)	0	1 (12)
Haematoma	1 (25)	2 (25)	0	2 (25)
Abdominal pain	2 (50)	0	1 (25)	1 (12)
Nausea	1 (25)	1 (12)	2 (50)	1 (12)
Rhinitis	1 (25)	1 (12)	0	1 (12)
Polyuria	0	1 (12)	0	1 (12)
Muscle Spasms	0	1 (12)	2 (50)	1 (12)
Musculoskeletal Pain	0	1 (12)	1 (25)	1 (12)
Proteinuria†	1 (25)	0	1 (25)	5 (62)
Injection Site Pain	0	1 (12)	0	1 (12)

Through 144 weeks of trial, 97% (514/530) of assessments of protein in urine were negative

- 3% total positive assessments through 144 weeks (includes placebo)
- 1.2% of all assessments for subjects on eteplirsen (6/486) classified as proteinuria
  - Majority of cases determined unrelated to treatment†
  - Cases mild and transient
- 2.3% (1/44) of assessments exhibited background proteinuria in subjects on placebo

Only one subject reported injection site pain over 144 weeks of treatment

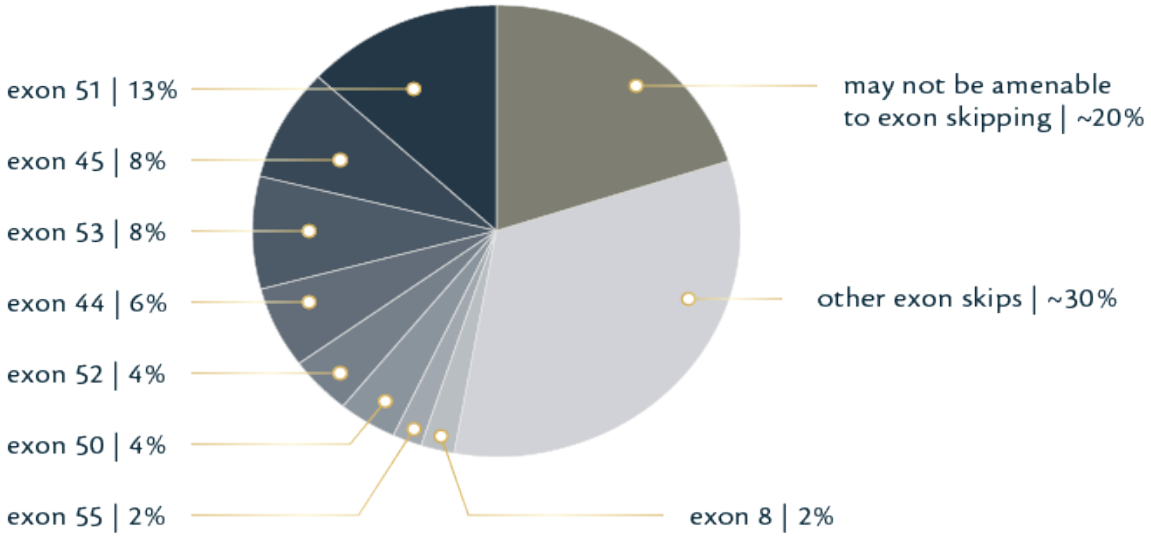
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†5 of the 7 cases of proteinuria were determined to be unrelated to treatment

CURRENTLY TOP 3 LEAD CANDIDATES PROGRESSING TO CLINICAL TRIALS

SAREPTA'S EXON SKIPPING PLATFORM FOR DMD

CLINICAL PROGRAMS		DISCOVERY	PRE-CLINICAL	CLINICAL DEVELOPMENT
Rare Diseases	DMD	Eteplirsen (AVI-4658)		
	DMD	SRP-4053		
	DMD	SRP-4045		
	DMD	SRP-4050		
	DMD	SRP-4044		
	DMD	SRP-4052		
	DMD	SRP-4055		
	DMD	SRP-4008		



About 13% of DMD patients may be treated with an exon 51-skipping therapy.

Available data suggest up to 80 percent of DMD patients have genotypes amenable to exon skipping.

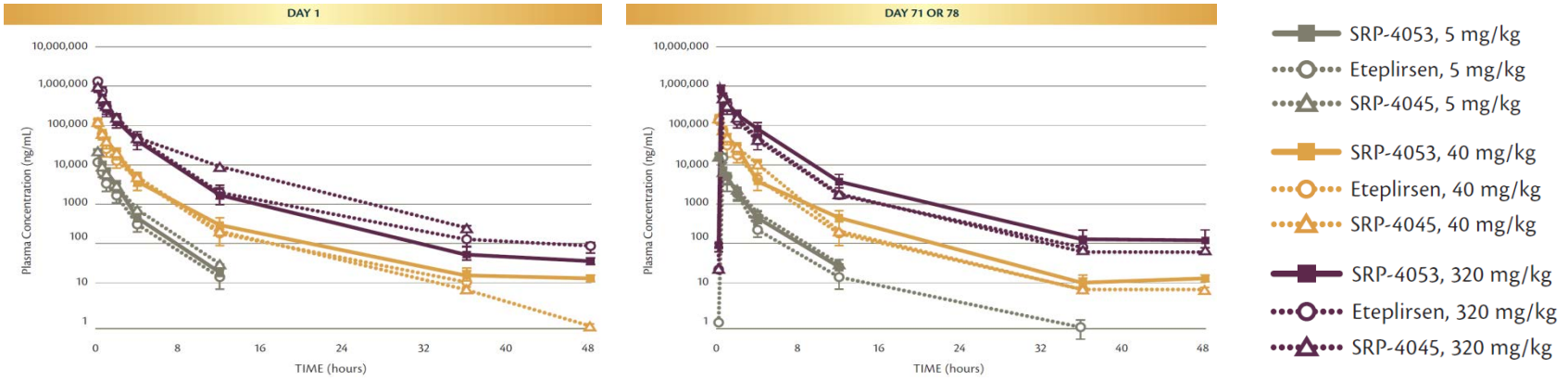
*Percent of DMD boys amenable to skipping each exon listed. Source: Annemieke Aartsma-Rus, et al. Hum Mutat. 2009 Mar;30(3):293-9.

THREE PMO DRUG CANDIDATES DEMONSTRATED REPRODUCIBLE PRECLINICAL SAFETY PROFILES

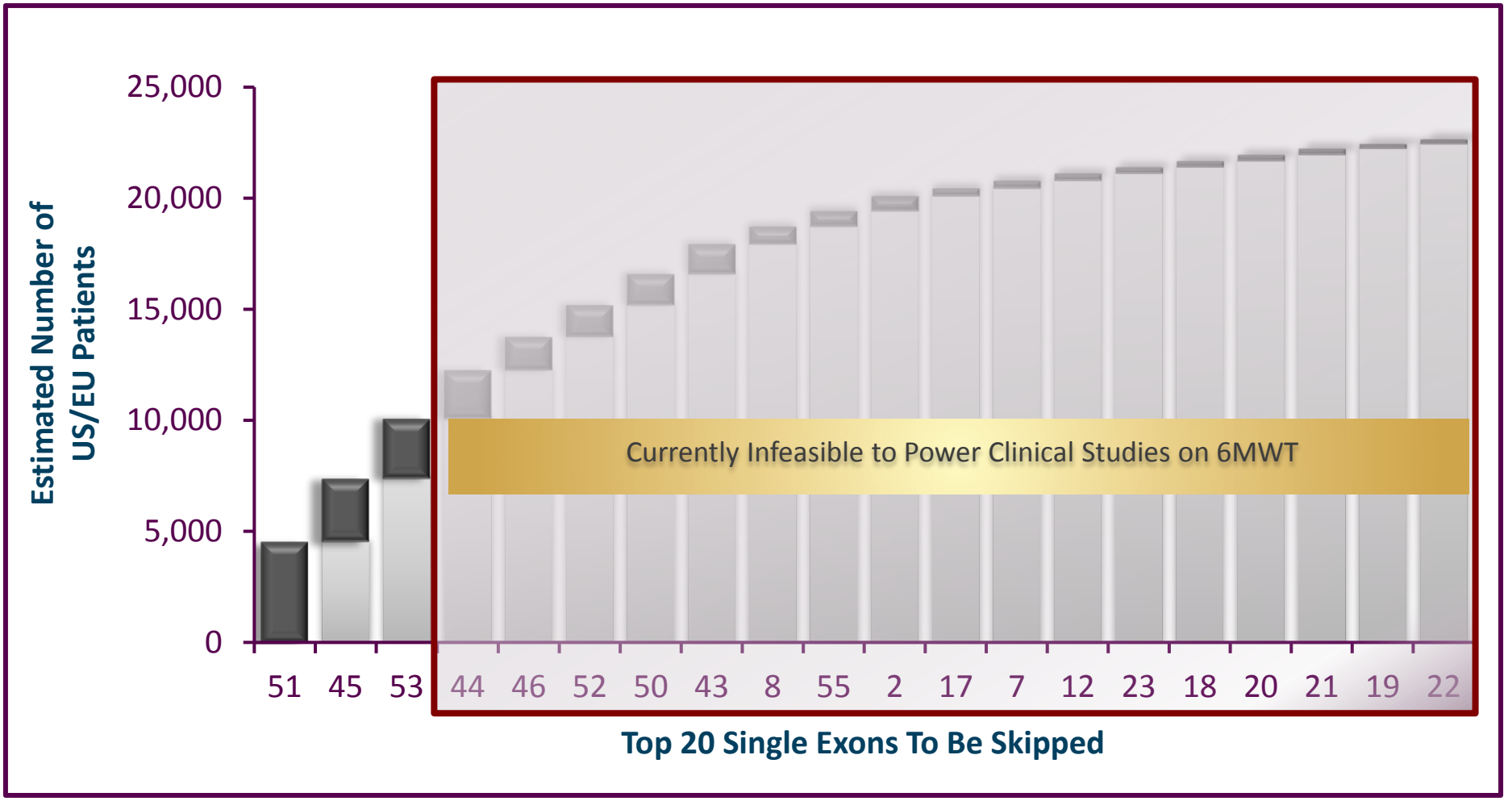
NO ADVERSE EFFECTS IN REPEAT DOSE TOXICITY EVALUATIONS UP TO 320 MG/KG

STUDY	DRUG	SPECIES	ROUTE/DOSE	N	RESULTS
GENOTOXICITY *Bacterial Reverse Mutation* *Chromosomal Ab.* *Micronucleus*	**Eteplirsen** **SRP-4045** **SRP-4053**	N/A	N/A	N/A	Negative: mutagenic potential, induction of chromosomal aberrations, induction of micronuclei
SAFETY PHARMACOLOGY *Vitals* *CNS* *Pulmonary* *Cardiac*	**Eteplirsen**	Cynomolgus Monkey	IV, SC : 0, 40, 160, 320 mg/kg	6	No biologically relevant findings on vital signs, CNS, or cardiopulmonary activity
	SRP-4045	Cynomolgus Monkey	IV: 0, 40, 160, 320 mg/kg	4	
	SRP-4053	Cynomolgus Monkey	IV: 0, 40, 160, 320 mg/kg	4	
REPEAT DOSE TOXICITY *Kidney Pathology* *Cardiovascular* *Reproductive* *Immunotoxicity* *Complement Activation*	**Eteplirsen**	Cynomolgus Monkey	IV weekly: 0, 5, 40, 320 mg/kg	6	NOAEL= MFD; 320 mg/kg
	SRP-4045	Cynomolgus Monkey	IV weekly for 12 weeks: 0, 5, 40, 320 mg/kg	9	NOAEL= MFD; 320 mg/kg
	SRP-4053	Cynomolgus Monkey	IV weekly: 0, 5, 40, 320 mg/kg	9	NOAEL= MFD; 320 mg/kg

Toxicokinetic Profiles are comparable following the first and last weekly dose of 5, 40, or 320 mg/kg



SAREPTA IS COMMITTED TO THE DMD COMMUNITY AND WILL WORK WITH REGULATORS TO GET DRUG CANDIDATES TO ALL BOYS WHO CAN BENEFIT FROM EXON SKIPPING THERAPIES

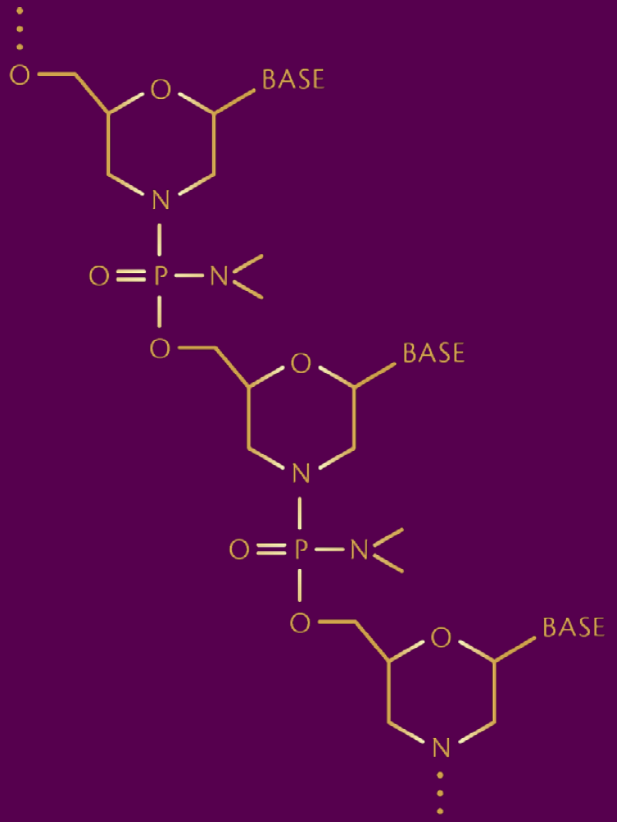


SAREPTA THERAPEUTICS

Aartsma-Rus A, et al. *Hum Mutat.* 2009;30(3):293-299.

SAREPTA
THERAPEUTICS

PIPELINE BEYOND DMD



NEXT GENERATION PMO-BASED CHEMISTRIES

ENHANCED TISSUE TARGETING, INTRACELLULAR DELIVERY AND DRUG POTENCY

IP PROTECTED NEXT GENERATION PMO CHEMISTRIES

PMO*plus*®



- Incorporates selective, position specific positive charges
- Enhances efficacy with demonstrated human safety as an anti-infective treatment for viral pathogens
  - Ebola, Marburg, Influenza and other diseases

PPMO



- Restoration of antibiotic susceptibility demonstrated for multi-drug resistant bacteria *in vitro*
- 100% survival in a mouse model of *E. coli* infection
- Biofilm reduction impressive in *Burkholderia*

PMO-X®



- Increased cellular uptake & tissue-specific targeting
- Oncology & immunology applications
- Enhances activity and duration in CNS application

SAREPTA THERAPEUTICS

BUILDING THE FUTURE OF SAREPTA

EXPANDING PIPELINE BEYOND DMD AND EBOLA/MARBURG/FLU VIRUSES
MORPHOLINO CHEMISTRY HAS DEMONSTRATED BROAD-BASED PROOF OF CONCEPT

- 15,000 sq./ft. additional lab space opened in Cambridge in 2014
- Acquired manufacturing facility on 26 acres of land in MA in 2014
- Research & synthesis capabilities expanded in both Oregon and Cambridge in 2014

PMO CHEMISTRY EXPERIENCE

- 38 total DMD patients dosed with PMO with no dose limiting toxicities or discontinuations
- PMO*plus*® dosed at 16 mg/kg daily for 14 consecutive days (112 mg/kg/wk) in healthy adults daily without adverse effects
- Over 100 healthy humans dosed with PMO*plus*® chemistry
- Continuing to expand our PMO-based platform
- Successful GMP reproducible mid-scale capability with multiple successful batch runs with quality drug product
- Multiple CMOs engaged to produce drug for clinical trials and pipeline products

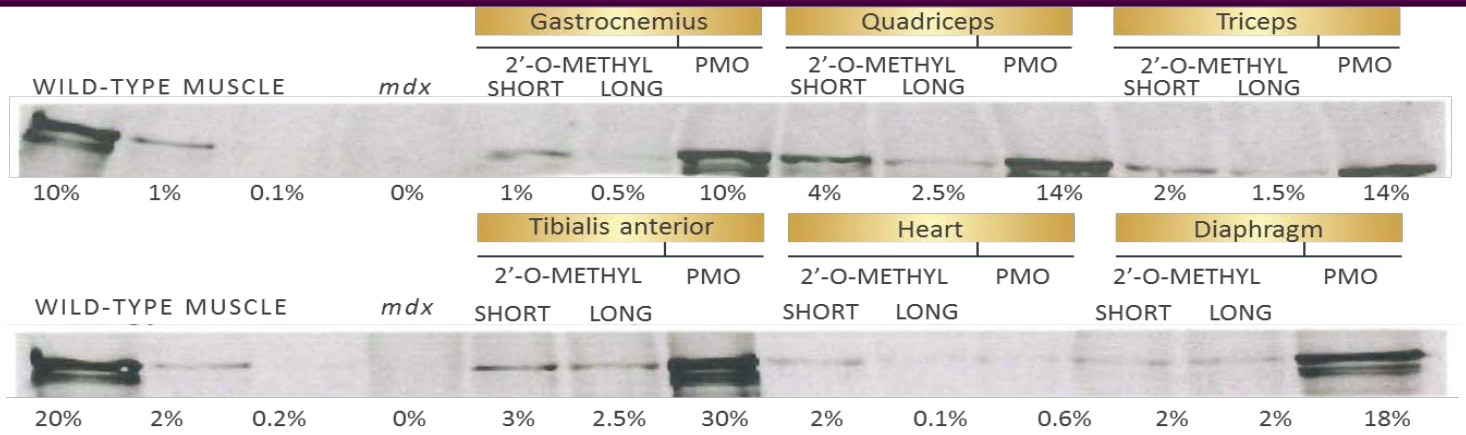
RESEARCH MOVING FORWARD

- 7 Research collaborations with universities in rare or infectious diseases outside of DMD using PMO, PMO-X® or PMO*plus*®
- 3 Research collaborations utilizing CROs focused on rare disease outside DMD using PMO*plus*®, PMO-X® and PMO
- 6 internal research programs outside of DMD
- Cellular data complete in 5 collaborations, patent applications filed, moving into animal studies
- Ongoing discussions with 9 government agencies around Ebola program

DIFFERENTIATED CHEMISTRY AND SEQUENCE POTENCY FOR ETEPLIRSEN

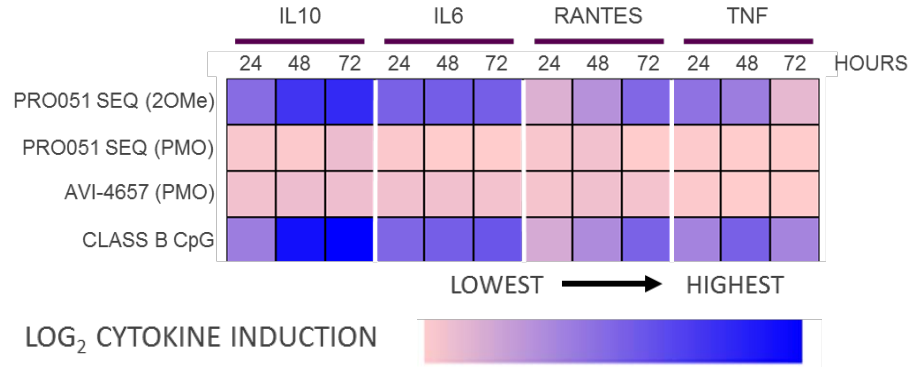
CHEMISTRY, SEQUENCE, AND SAFETY ADVANTAGES IN DMD

LEIDEN RESEARCHERS SHOWED THAT PMO CHEMISTRY HAS UP TO 10-FOLD HIGHER DYSTROPHIN PRODUCTION IN A MDX MOUSE MODEL ACROSS VARIOUS MUSCLE GROUPS



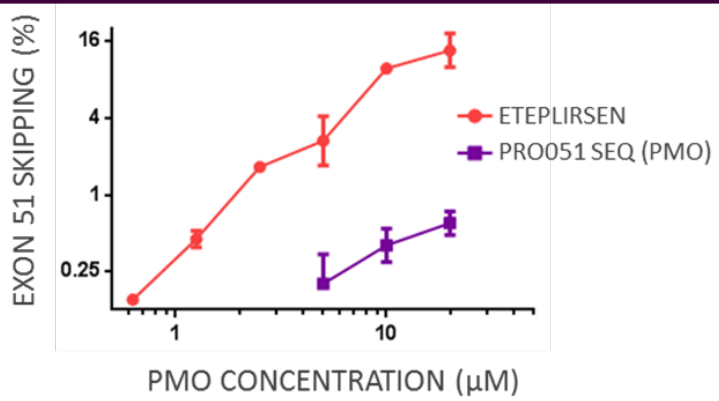
Source: Heemskerk, et al, 2009

CYTOKINE SCREENING DEMONSTRATED CLEAR DIFFERENTIATION BETWEEN PMOS AND 2'OME CHEMISTRIES



Source: Sarepta Internal Data

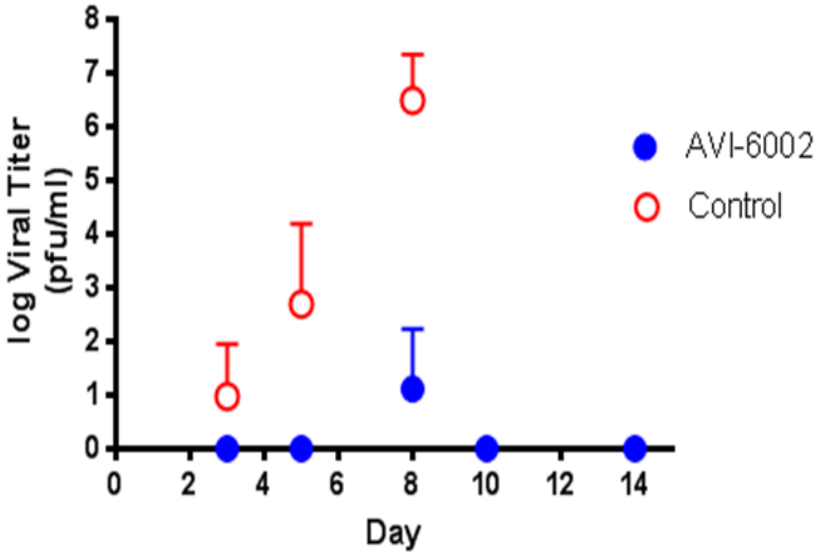
SAREPTA COMPARISON STUDIES OF ETEPLIRSEN SEQUENCE VS DRISAPERSEN SEQUENCE SHOWED UP TO 10-FOLD HIGHER EXON-SKIPPING ACTIVITY



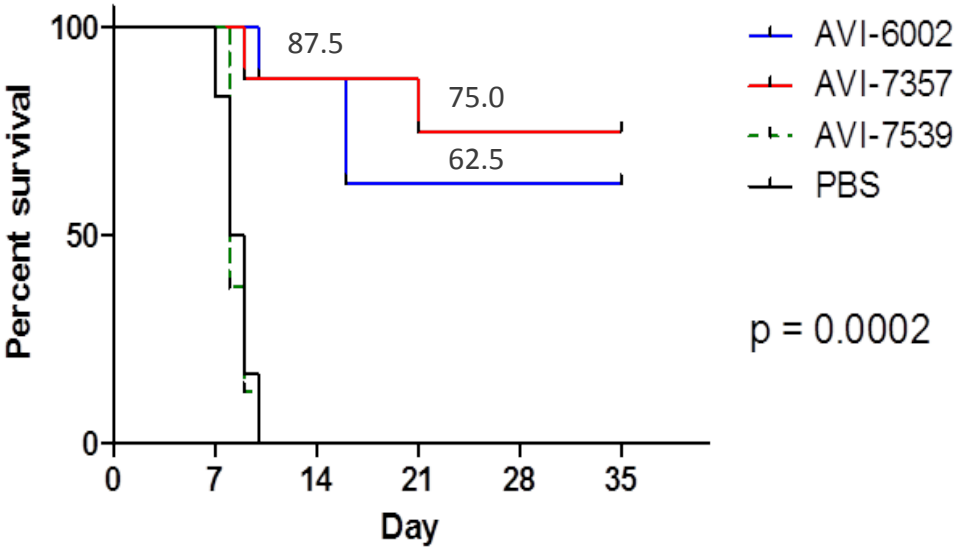
Source: Sarepta Internal Data

PMO*PLUS*® EFFICACY AGAINST EBOLA: POTENT ANTIVIRAL EFFECT AND IMPROVED SURVIVAL IN NHPs

DEMONSTRATION OF VIRAL LOAD SUPPRESSION AND SURVIVAL BENEFIT[1,2,3]



- Design: 40 mg/kg/day IM/IP x 14 days
- N= 4 (2M/2F) treated, 1 control (5 additional control animals from contemporaneous studies included)
- Peak recoverable viable virus at Day 8 was 5 log lower than control



- Component vs Combination vs Placebo
- Design: 40 mg/kg/day x 14 day AVI-6002 vs AVI-7537 vs AVI-7539

AVI-6002 is a combination of AVI-7537 & AVI-7539
AVI-7537 is demonstrated to be active component

SAREPTA THERAPEUTICS

[1]Iversen, TIDES Las Vegas, NV; [2]Iversen, Viruses 2012; [3]Wong OTS 2014; Internal Sarepta data

AVI-7537 PREPAREDNESS: NHP EFFICACY AND HUMAN SAFETY DEMONSTRATED, DRUG SUPPLY AVAILABLE

NHP survival against Ebola lethal challenge[1,2]

- Targets Ebola VP24 –well conserved; no base pair mismatches compared to reported clinical isolates from 2014 outbreak
- 5 studies, 67 Rhesus macaques infected with 1000 pfu (highly lethal dose) Ebola-Zaire
- 73.5% (17/25) overall survival (range 60-80%) of NHPs receiving 20-40 mg/kg/day x14 days
- 100% (2/2) of surviving monkeys re-challenged at day 50 survived without retreatment
- 0/27 (0%) controls survived – controls died by day 11 post-infection, median mortality by day 8

Human safety testing: no observed safety concerns at any dose tested

- SAD study tested AVI-7537 up to 9.0 mg/kg[3]
- MAD study tested AVI-7288[4] up to 16 mg/kg/day[5] x 14 days (identical backbone chemistry as AVI-7537)

Drug supply readiness for global response & well-controlled clinical trials

- Emergency use protocol, ICF and fact sheets available for healthcare institutions
- ~20 courses fill/finished and available immediately
- ~250 additional courses available in 3-6 months (if funding is obtained)
- ~750 additional courses available in additional 6-9 months (if funding is obtained)

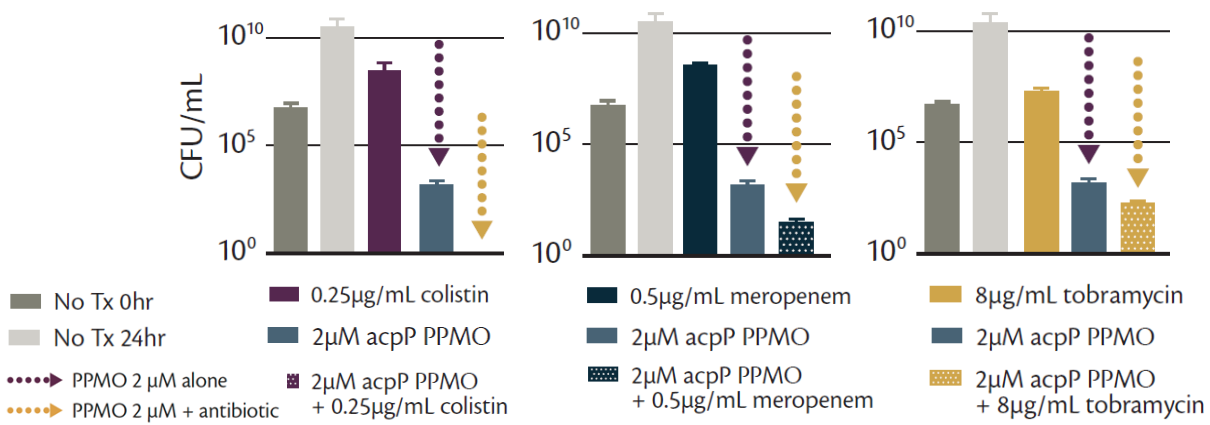
[1]Iversen, TIDES Las Vegas, NV; [2]Wong, OTS 2014 San Diego; [3]Clinical Study Report, 6002-us-101, NCT01353027; [4]AVI-7288 for Marburg has same backbone chemistry as AVI-7537; [5]Estimated human efficacious dose = 10 mg/kg/day, Heald A et al. ECCMID, 2014 Barcelona

ANTIBIOTIC-RESISTANT BACTERIAL INFECTIONS ARE A SERIOUS GLOBAL HEALTH CONCERN

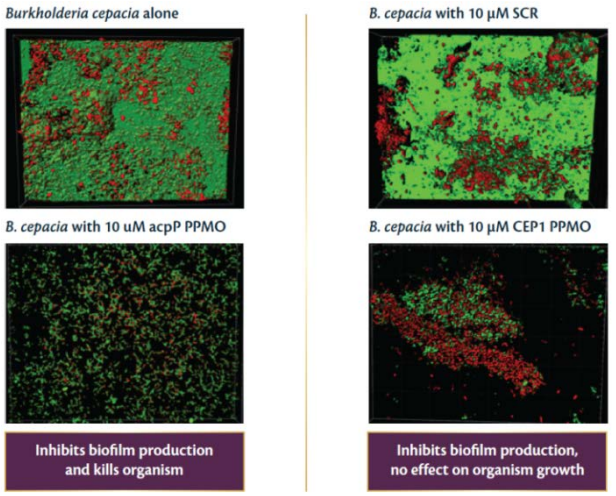
MILLIONS OF RESISTANT INFECTIONS AND ~50,000 DEATHS EACH YEAR ACROSS US AND EU[1,2]

- Sarepta is focused on infections of highest medical need and large hospital-based opportunities
  - Six programs identified and underway
- Additional focus on major bacterial infections affecting children with Cystic Fibrosis
  - Pseudomonas & Burkholderia
- Results demonstrate PPMO alone reduced bacterial colony forming units (CFU) greater than antibiotics
- Additional reduction of CFUs seen when PPMO and antibiotics were co-administered

Colistin, Meropenem, and Tobramycin susceptibility restored with co-administration of PPMO in *A.Baumannii*[3]



PPMO inhibits bacterial biofilm production and kills organism[4]

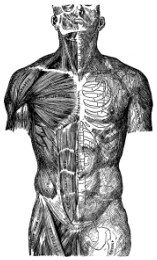


Red: *Burkholderia cepacia*; Green: biofilm

1. *CDC; Antibiotic Resistance Threats in the United States, 2013*
2. *EMA and ECDC; The Bacterial Challenge: Time to React, 2009*
3. *Harbour L, et al. ICAAC 2014. Washington, DC; Abstract F-1553*
4. *Smith B, et al. ICAAC 2014. Washington, DC; Abstract F-1552*

R&D FOCUSED ON HIGH-VALUE TARGETS

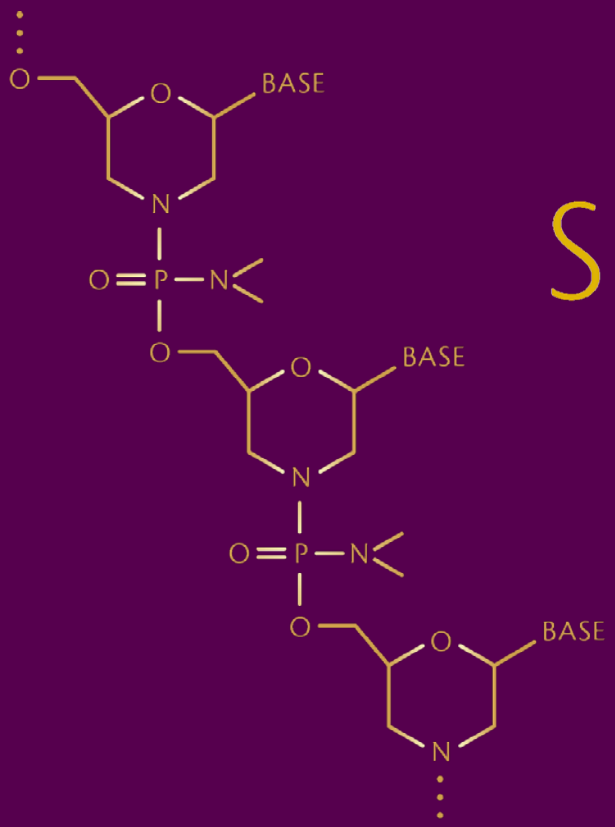
SAREPTA IS MAKING CONSIDERABLE PROGRESS APPLYING PMO TECHNOLOGY IN ADDITIONAL THERAPEUTIC AREAS



	CLINICAL PROGRAMS	DISCOVERY	PRE-CLINICAL	CLINICAL
Rare Diseases	DMD Exon 51	Eteplirsen (AVI-4658)		
	DMD Exon 53	SRP-4053		
	DMD Exon 45	SRP-4045		
	DMD Exon 50	SRP-4050		
	DMD Exon 44	SRP-4044		
	DMD Exon 52	SRP-4052		
	DMD Exon 55	SRP-4055		
	DMD Exon 8	SRP-4008		
	DMD & Becker MD	Myostatin Inhibition		
	Progeria	Progerin		
	Adult Onset Pompe Disease	Alpha-glucosidase		
	Lupus & Graft vs. Host Disease	Toll Like Receptors (TLR)		
Anti-Infective	Marburg Virus	AVI-7288		
	Ebola Virus	AVI-7537		
	Influenza	AVI-7100		
	Drug-Resistant Bacteria	Burkholderia Cepacia		
	Drug-Resistant Bacteria	Pseudomonas Aeruginosa		
	Drug-Resistant Bacteria	Klebsiella pneumoniae		
	Drug-Resistant Bacteria	Acinetobacter baumannii		
	Drug-Resistant Bacteria	Staphylococcus aureus		
	Drug-Resistant Bacteria	Neisseria gonorrhea		

FINANCIAL OVERVIEW

SHARES OUTSTANDING	41.3 million
RECENT CLOSING PRICE	$16.12 as of 11/7/14
TRADING VOLUME	1.6 million shares daily (90 day average volume)
MARKET CAPITALIZATION	~$670 million
CASH & OTHER INVESTMENTS (UNAUDITED)	~$240 million as of 9/30/14
CURRENT 2014 NON-GAAP OPERATING LOSS GUIDANCE	$110 - $120 million



SAREPTA
THERAPEUTICS

THANK YOU