SAREPTA THERAPEUTICS

REALIZING THE POTENTIAL OF RNA-BASED TECHNOLOGY

CREDIT SUISSE 2014 HEALTHCARE CONFERENCE

NOVEMBER 11, 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.

SAREPTA THERAPEUTICS

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 3500- 5000 boys worldwide
- There are approximately 25,000- 30,000 patients in the U.S. and Europe
- Sarepta's lead program (Exon 51) could benefit ~13% of DMD patients
- Follow-on Exon-Skipping Drugs have potential to treat 60-80% of DMD patients

Relentless Progression of DMD

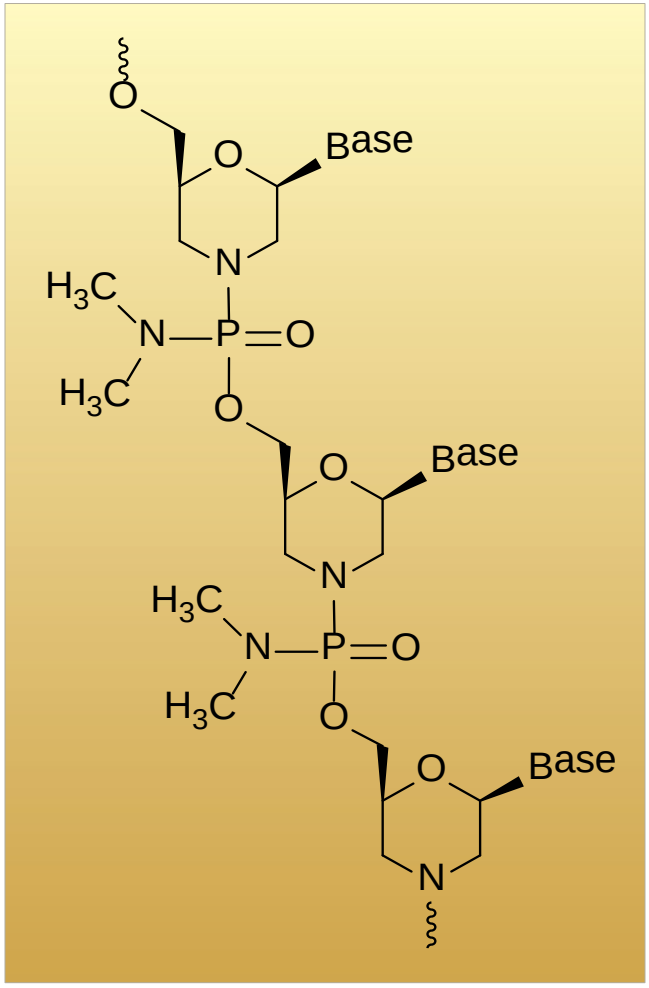
Delayed Milestones in Early Years	DMD Patients Begin Decline Around Age 7	Pulmonary Function Begins to Decline	Loss of Ambulation Age 10 - 15	Decline in Upper Body Muscle and Respiratory Function	Death in Mid/Late 20's

SAREPTA THERAPEUTICS

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



SAREPTA THERAPEUTICS

PRE-NDA FDA MEETING SUMMARY

ADDITIONAL BIOCHEMICAL AND CLINICAL DATA REQUESTED

Recent guidance requires additional data be included in the NDA submission

Biochemical data requested for inclusion in NDA submission

- Independent assessment of biopsy images to score dystrophin-positive fibers by three pathologists

Clinical data requested for inclusion in NDA or at time of NDA submission

- 168-week data from study 201/202 added in the NDA
- 3-month data from at least 12 to 24 newly exposed patients
- Available data from new eteplirsen studies, even if exposure is <3 months
- Patient-level natural history data from independent academic institutions
- MRI data with natural history controls from independent academic stud

US BASED CLINICAL TRIALS FOR ETEPLIRSEN

ONGOING EFFORTS TO MEET FDA REQUIREMENTS FOR APPROVAL

Study	Phase	Duration (weeks)	n	Status	Dystrophin Produced	RT-PCR: Exon Skipping	1⁰ Outcome Measure
4658-uk-33	I/II	Single Dose	7	Complete	✓	✓	Safety; Dystrophin
4658-uk-28	I/II	12	19	Complete	✓	✓	Safety; Dystrophin
4658-us-201	IIa	24	12	Complete	✓	✓	Safety; Dystrophin
4658-us-202	IIb	120	12	Ongoing	✓	✓	6MWT; Dystrophin
4658-us-301	III	48	120	Enrolling: FPD 4Q2014			6MWT; Dystrophin
4658-us-204	II	96	20	Enrolling: FPD 4Q2014			Safety
4658-us-203	II	96	20	Final Protocol: FPD 1Q2015			Safety; Dystrophin
4045-us-301	III	48	90	FDA Meeting 4Q2015			6MWT; Dystrophin

Phase III Confirmatory Study (4658-us-301)

Study sites: IRB approved

- **WA**: *Seattle Children's Hospital**
- **IA**: *U of Iowa Children's Hospital**
- **AZ**: *Neuromuscular Research Center**
- **PA**: *CHOP of UPMC*
- **TN**: *U of TN Health Science Center (MDA Clinic)*
- **FL**: *NW Florida Clinical Research Group, LLC*
- **CA**: *UC Davis, Davis Medical Center, Dept Physical Medicine & Rehabilitation*
- **OH**: *Cinncinati Children's Hospital Medical Center*
- ***MD:*** *Kennedy Krieger Institute, Center for Genetic Muscle Disorders*

Potential sites: IRB approval pending

- **UT**: *U of Utah, Department of Neurology*
- **CA**: *Stanford U School of Medicine/Medical Center*
- **MN**: *U of Minnesota*
- **OH**: *Nationwide Children's Hospital Neuromuscular Div.*
- **GA**: *Children's Hospital of Atlanta*
- **CA**: *David Geffen School of Medicine at UCLA*
- **PA**: *Penn State Hershey Medical Center*
- **NH**: *Dartmouth- Hitchcock Medical Center*

*Fully contracted and actively recruiting; 22 additional potential future site locations not included above

EU BASED TRIALS

EXPANDING ACCESS GLOBALLY

Exon	Study	Phase	n	Status	1^0 Outcome Measures
51	Pending discussions with the EMA				
53	4053-101	I/II	48	Enrolling: FPD 4Q2014	Part 1: Safety, Tolerability Part 2: 6MWT, Dystrophin
45/53	4045-301	TBD	TBD	Planning Global Study	6MWT, Dystrophin, Safety

4053-101: Study Start 4Q2014

Clinical Sites

- University College London (UK)
- University of Newcastle (UK)
- Universitá Cattolica del Sacro Cuore (IT)
- Association Institut de Myologie (FR)

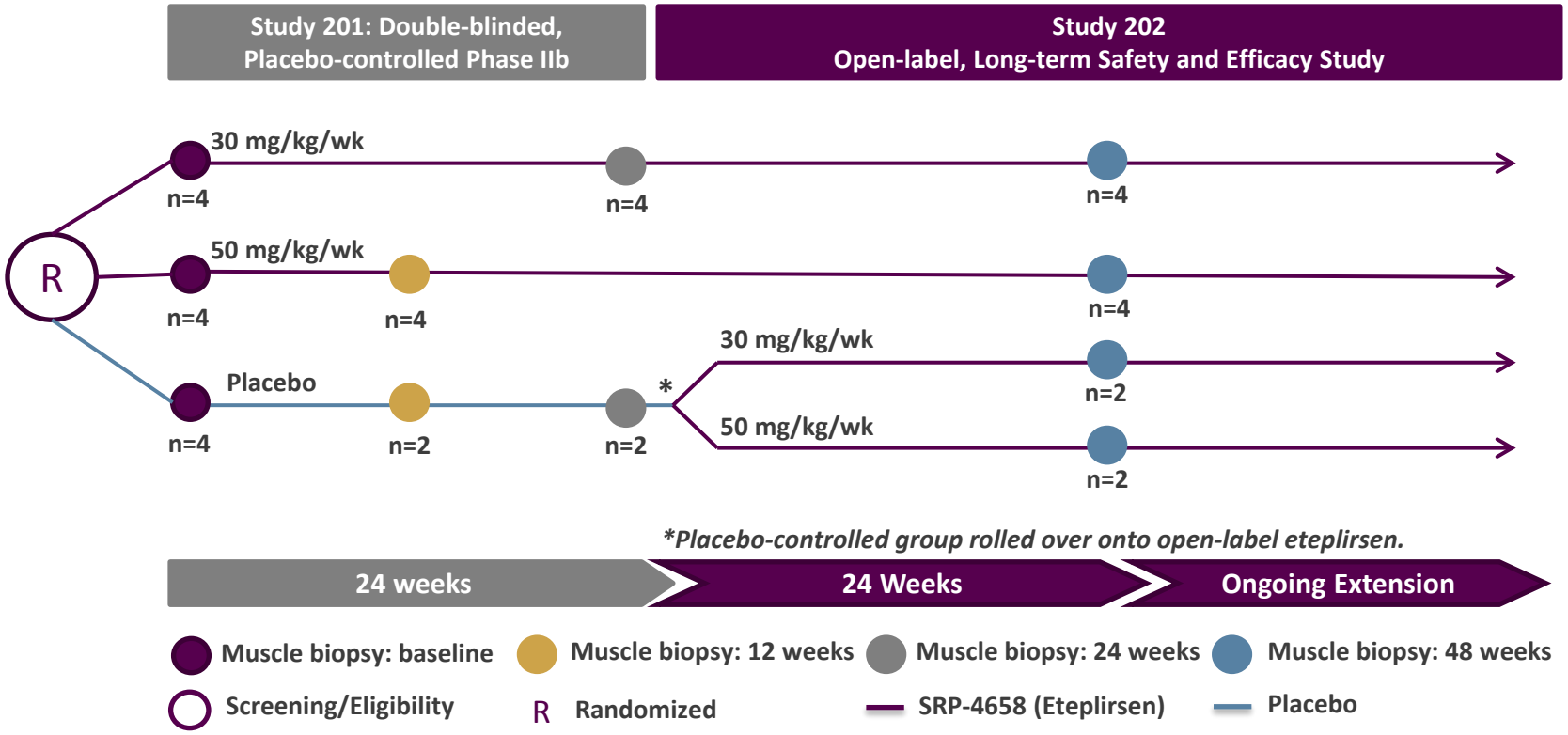
Experience with the EMA:
Voluntary Harmonization Process
Individual country filings

SAREPTA THERAPEUTICS

4053-101 has received funding from the European Union's Seventh Programme for research, technological development and demonstration under grant agreement No 305370.

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.

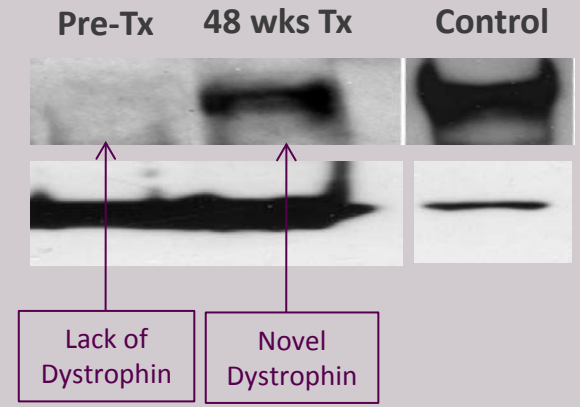
ROBUST BIOLOGIC RESPONSE OBSERVED ACROSS ALL STUDIES WITH ETEPLIRSEN

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

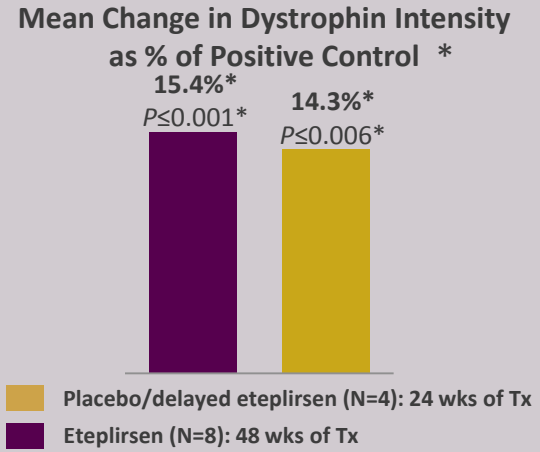
201/202

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	✓	✓	✓	✓

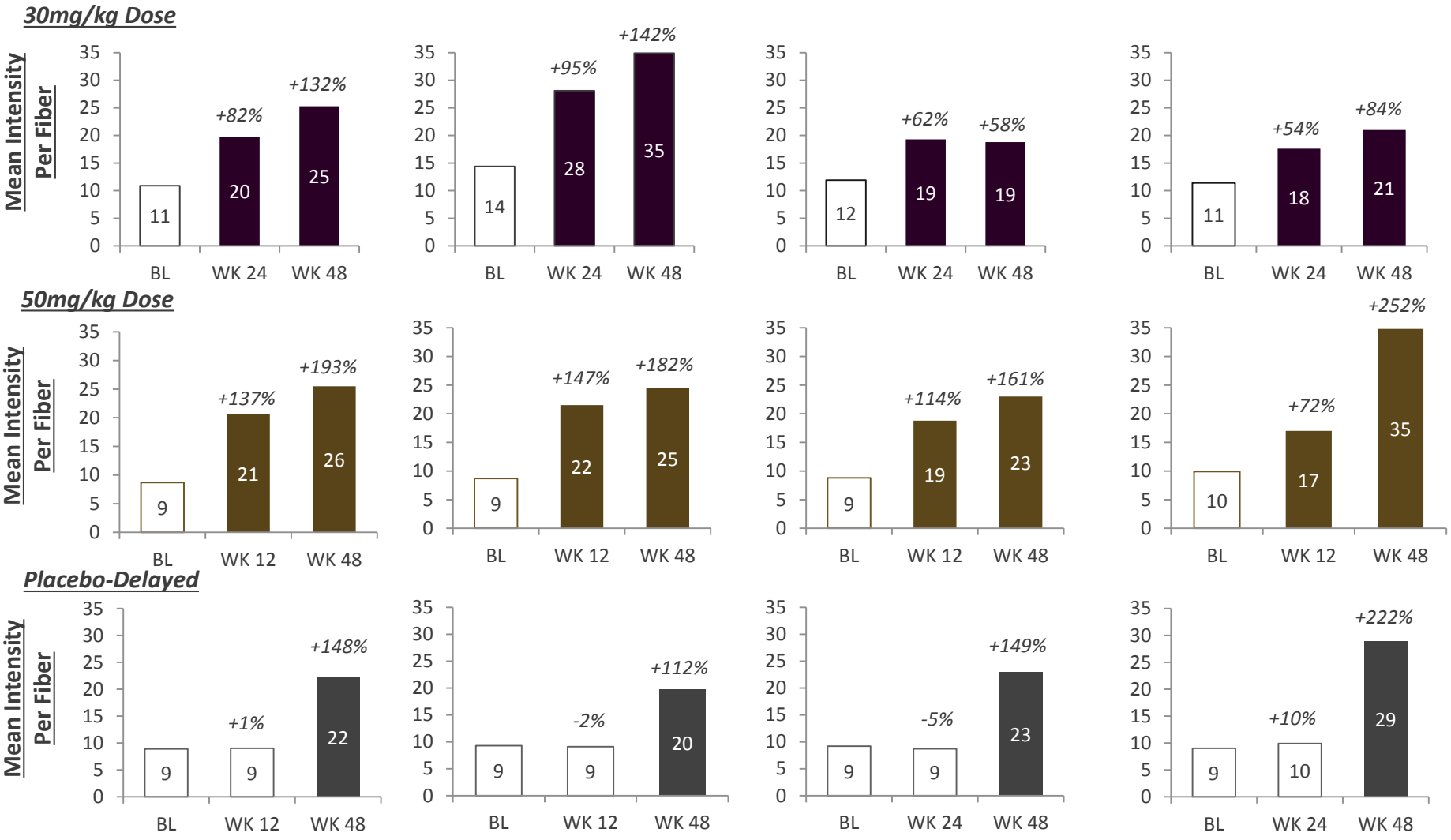
SAREPTA THERAPEUTICS

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

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

DYSTROPHIN INTENSITY INCREASED WITH ETEPLIRSEN TREATMENT VS BASELINE AND PLACEBO IN ALL SUBJECTS BY 48 WEEKS

INTENSITY INCREASE OBSERVED ONCE DYSTROPHIN CONFIRMED (N=12)



*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.

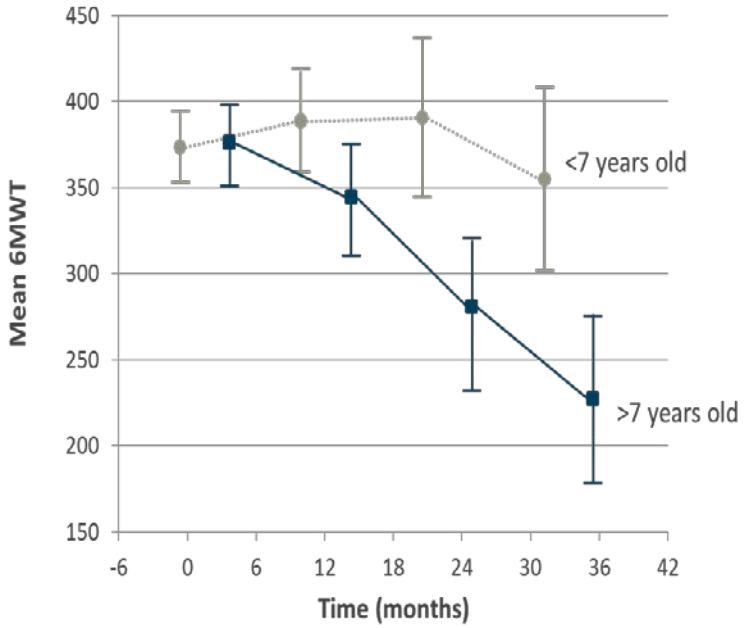
SIX MINUTE WALK TEST (6MWT) IS A WELL ESTABLISHED OUTCOME MEASURE

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" [8]

"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"[8]



[8]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[3]

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

Served as the basis for regulatory approval of drugs for a number of rare diseases, with mean changes ranging from 28 to 44 meters[4,5,6,7]

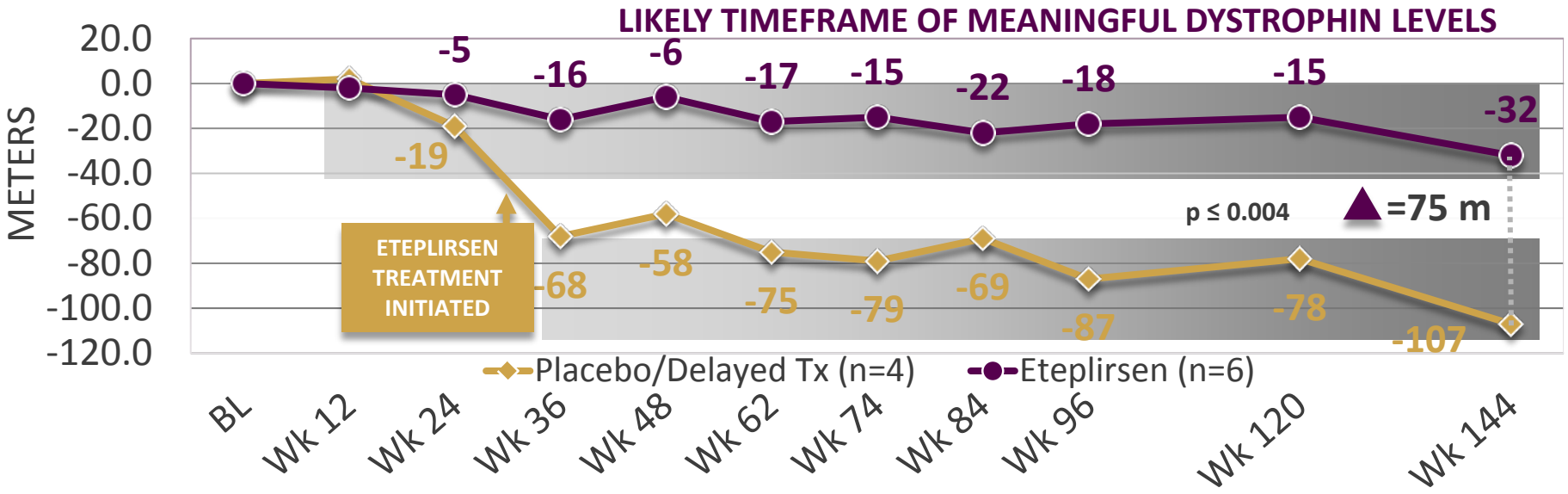
SAREPTA THERAPEUTICS

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
[3]Information obtained from www.clinicaltrials.gov on 24Sep2014
[4]Muenzer et al 2006
[5]Rubin et al, 2002
[6]Wraith et al 2004
[7]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

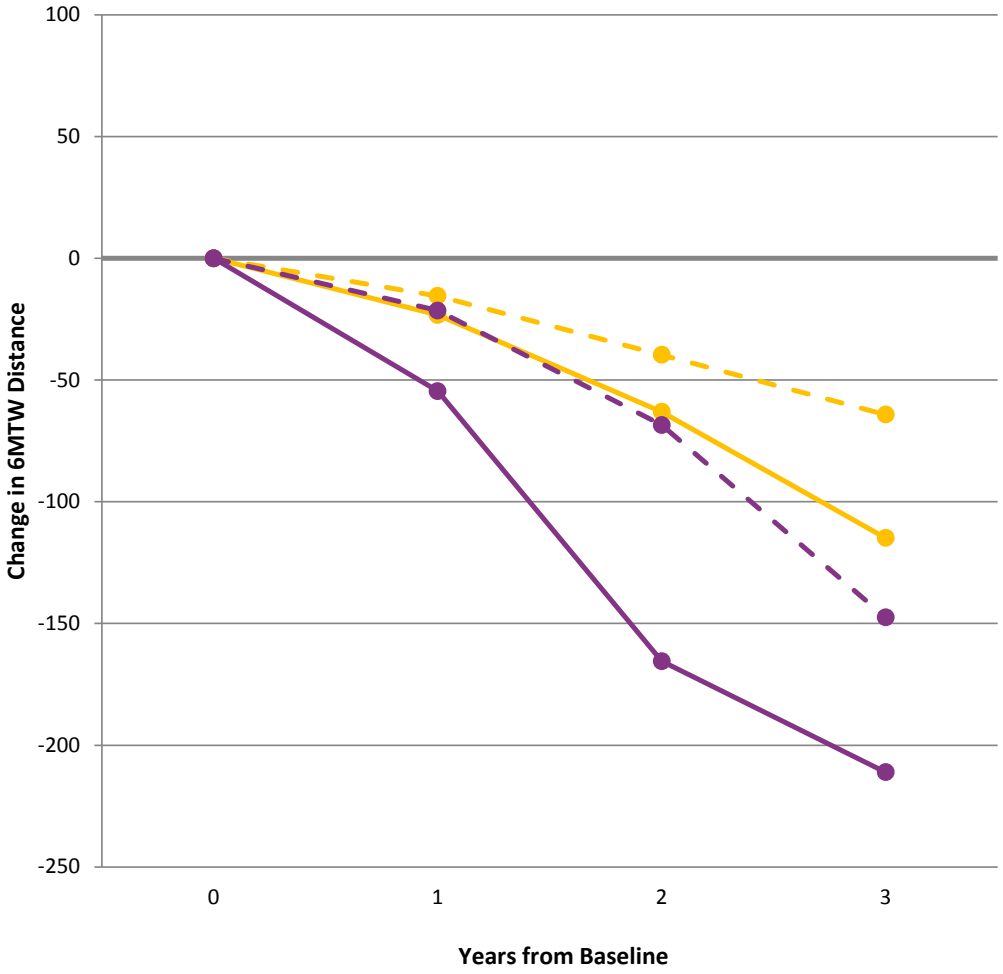
[1]6MWT, 6-minute walk test; DMD, Duchenne muscular dystrophy. Mercuri E, et al. 13th International Congress on Neuromuscular Diseases (ICNMD), July 5-10, 2014, Nice, France.

SAREPTA THERAPEUTICS

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

6-MINUTE WALK TEST 3-YEAR NATURAL HISTORY DATA

DECLINE OBSERVED IN SUBJECTS AGED 7 AND ABOVE, WITH A MORE PRONOUNCED DECLINE IN THOSE WITH BASELINE 6MWT DISTANCES BELOW 350M[1]



Age ≥ 7
Baseline 6MWD ≥ 350M
N=42, N=40, N=36
Excludes n=1, n=3 and n=7 with loss of ambulation by 1, 2 and 3 years respectively

Age ≥ 7
Baseline 6MWD ≥ 350M
N=43
Includes those with loss of ambulation

Age ≥ 7
Baseline 6MWD < 350M
N=22, N=12, N=11
Excludes n=3, n=13 and n=14 with loss of ambulation by 1, 2 and 3 years respectively

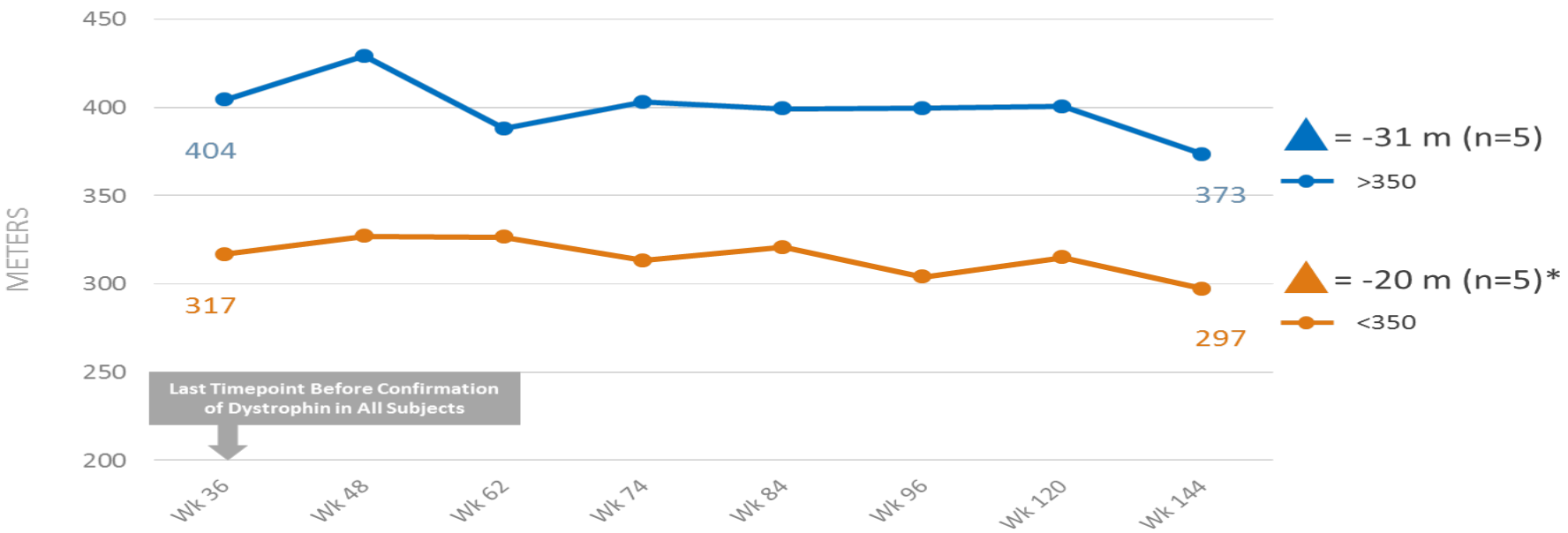
Age ≥ 7
Baseline 6MWD < 350M
N=25
Includes those with loss of ambulation

SAREPTA THERAPEUTICS

1. Pane et al, 2014

STRATIFICATION OF SUBJECTS BY 6MWT DISTANCE AT 36 WEEKS

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



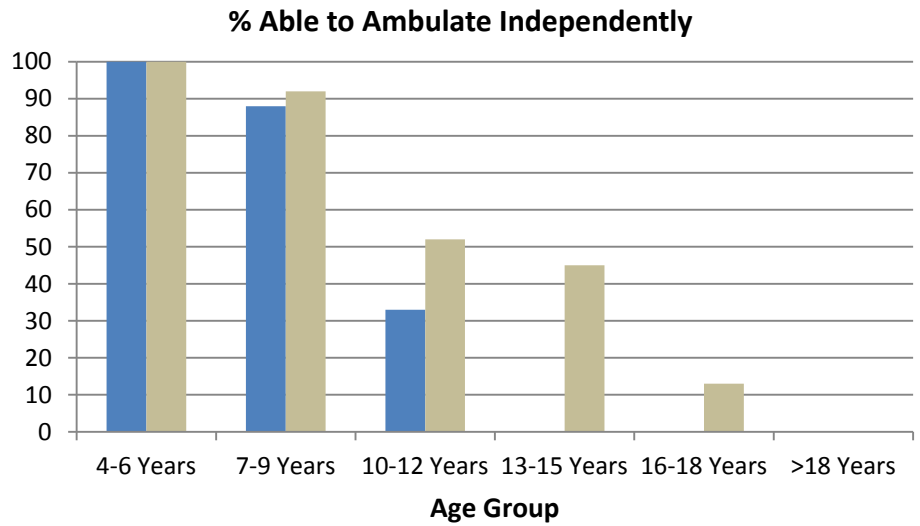
Stability in the 6MWT was demonstrated for more than two years regardless of the subject walking above or below 350 Meters after dystrophin production was confirmed

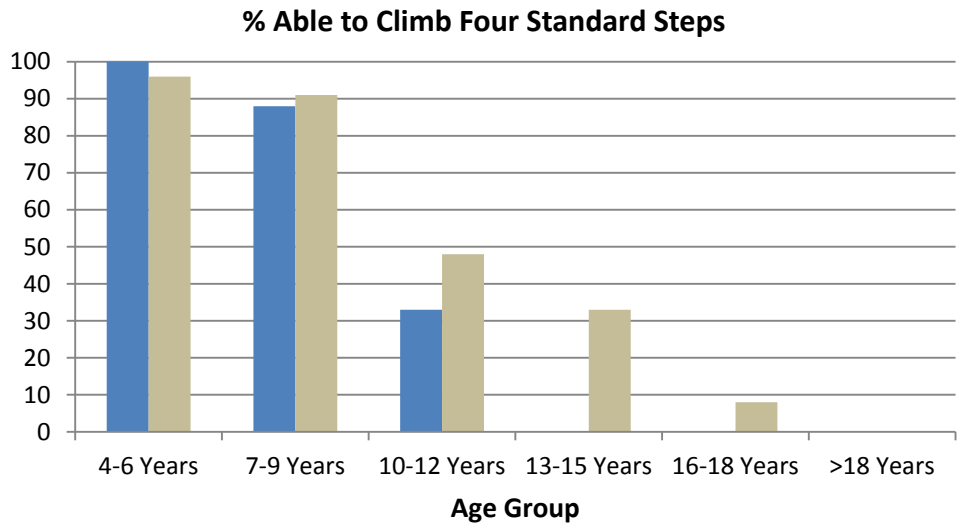
SAREPTA THERAPEUTICS

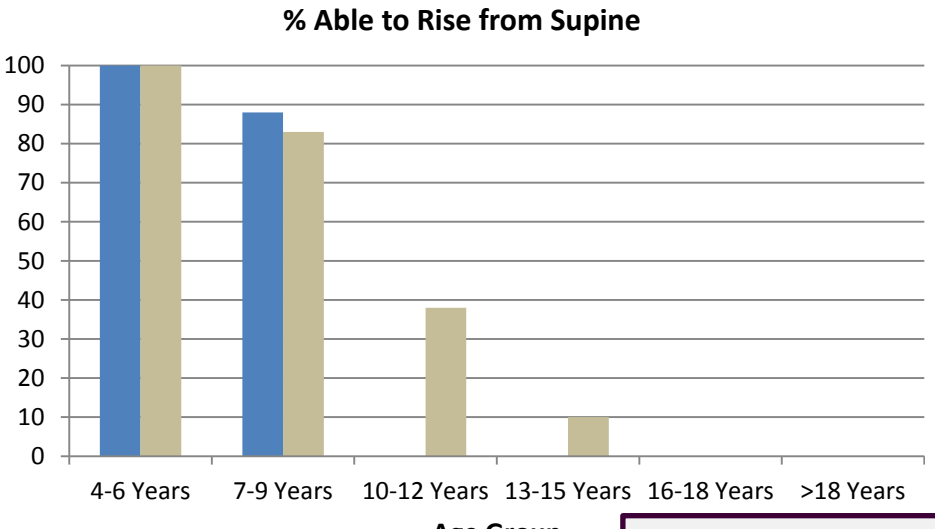
Note: Mean age: 9.9 years at week 36; 12.0 years at Week 144; Includes modified Intent-to-Treat (mITT) Population
**n=4 at week 84 due to a patient recovering from a broken ankle who was unable to participate at this time point*

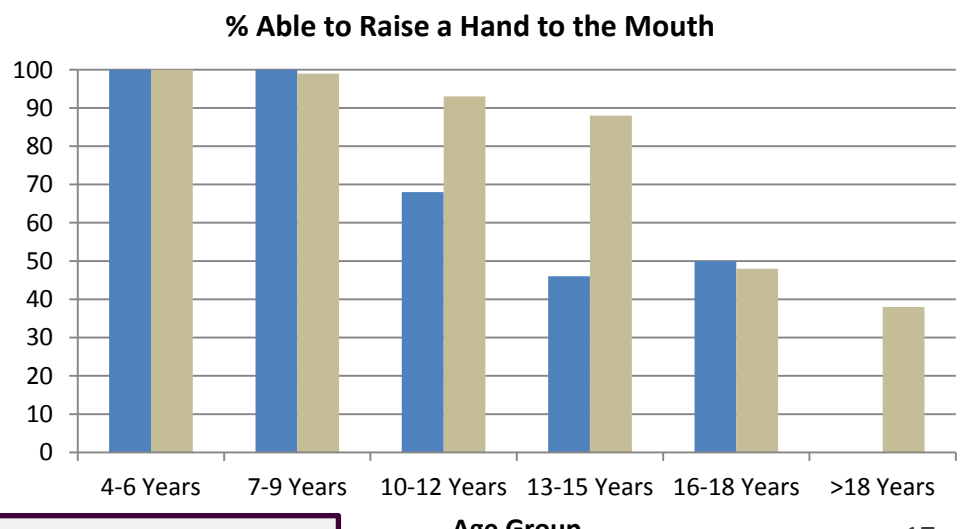
PUBLISHED NATURAL HISTORY* BY AGE AND STEROID STATUS (GC); STUDY SUGGEST GC BENEFIT BUT DECLINES PERSIST

347-SUBJECT STUDY ON GC-TREATED VS. GC-NAÏVE PATIENTS*





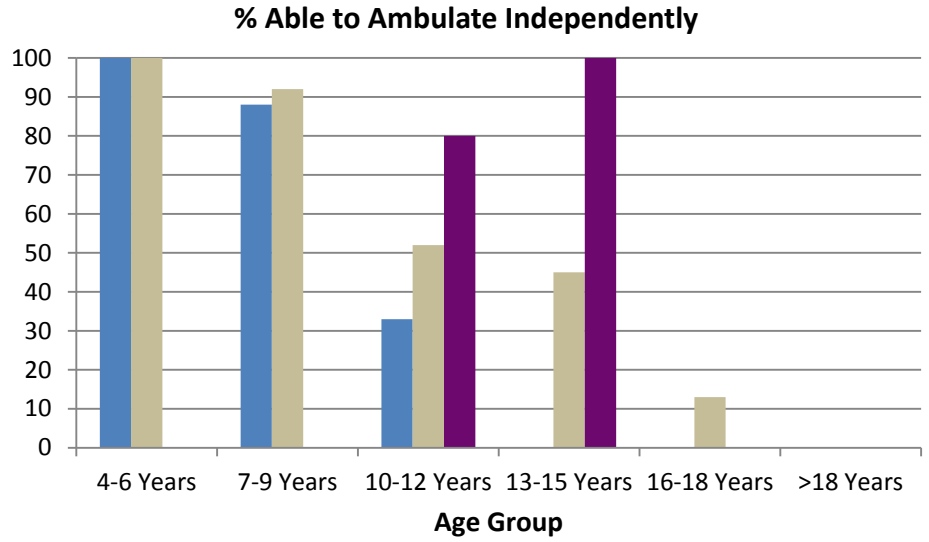


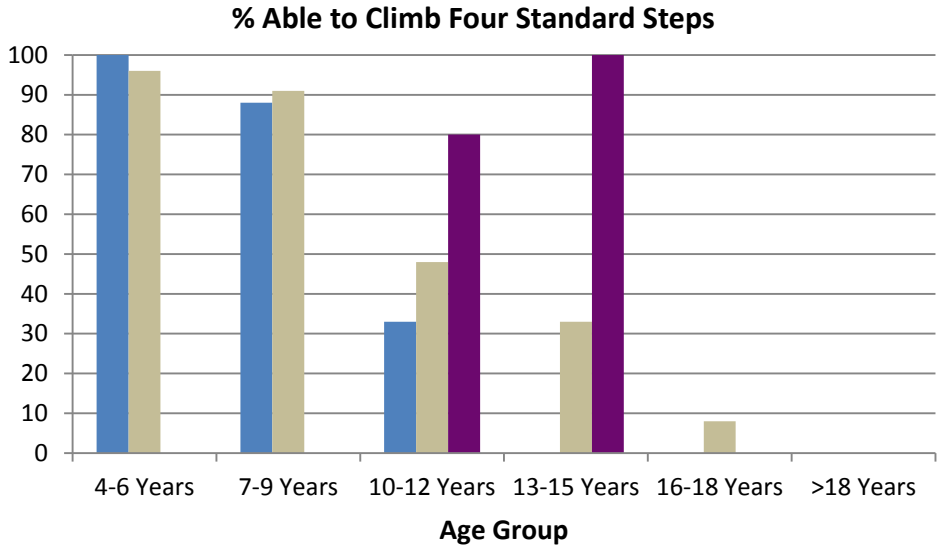


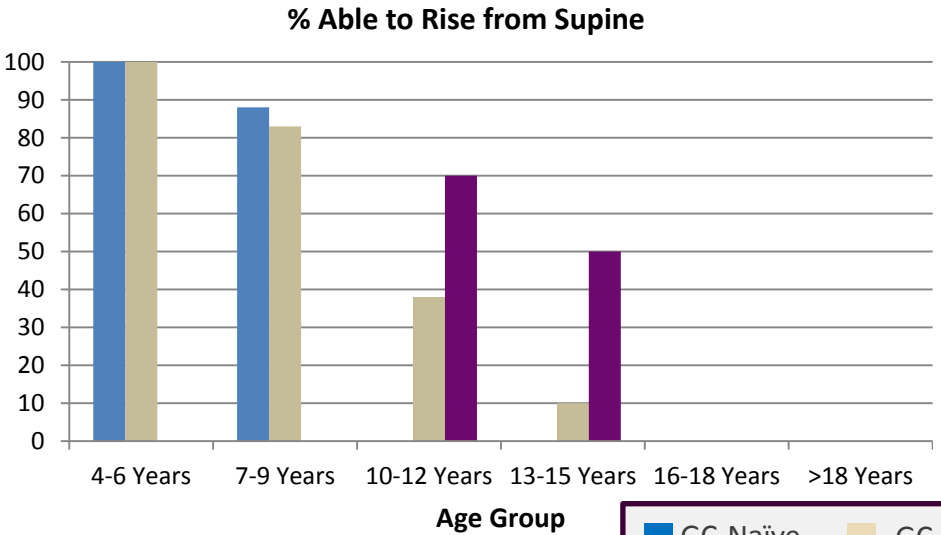
*Henricson, et al. 2013

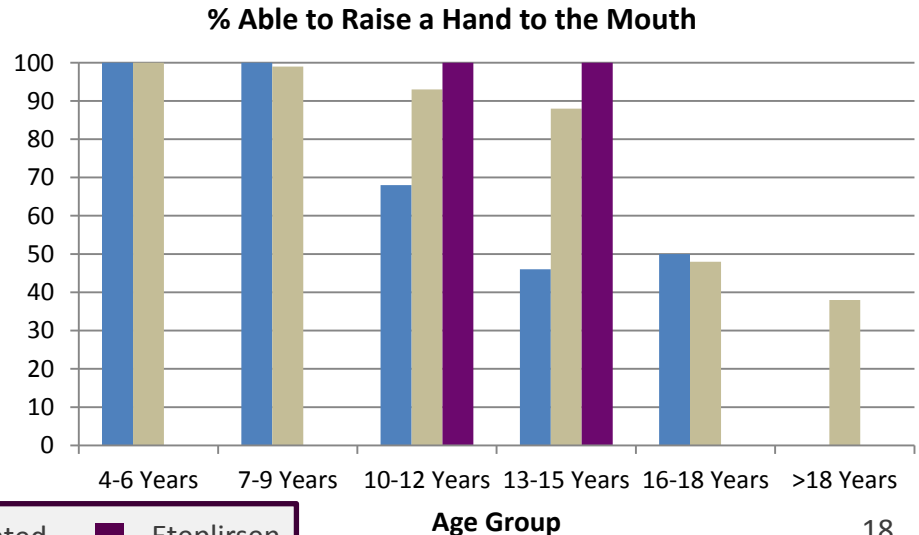
SUBJECTS ON ETEPLIRSEN COMPARED TO PUBLISHED NATURAL HISTORY* (N=12 AT WEEK 144)

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





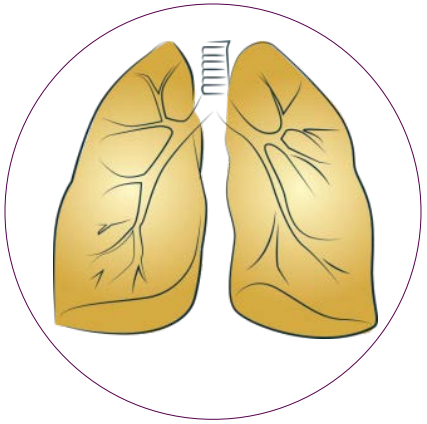


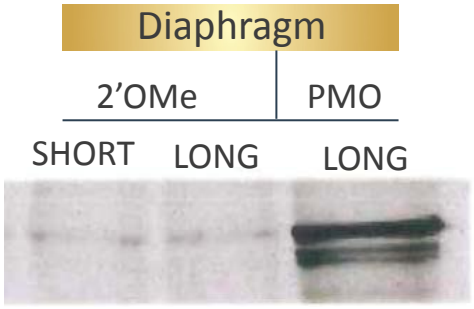


*Henricson, et al. 2013

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)

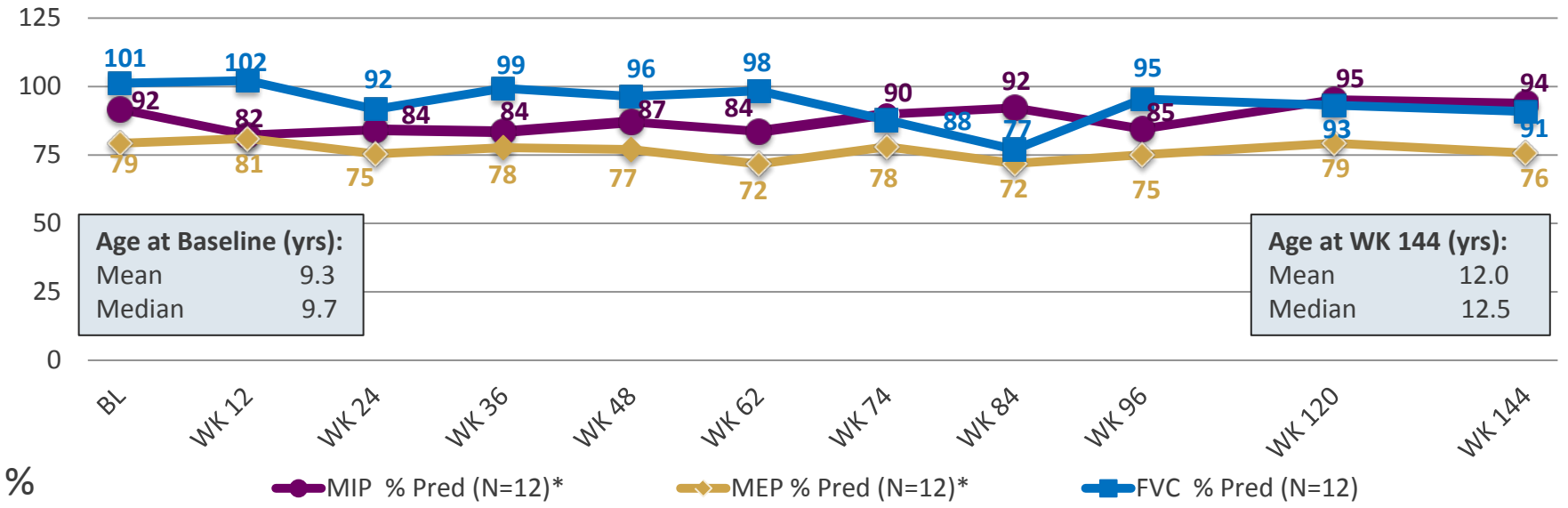
- Sensitive measures of respiratory muscle strength well characterized in the disease natural history
- MEP typically deteriorates before MIP and FVC in DMD patients
- Declines in MIP and MEP correlate with decreases in voluntary cough capacity

Significant increase of dystrophin expression achieved in diaphragm muscle of mdx mouse following a single 100 mg/kg dose of PMO

SAREPTA THERAPEUTICS

[1]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%

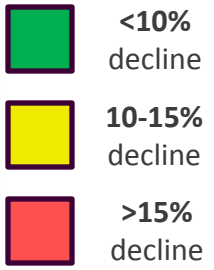
SAREPTA THERAPEUTICS

* Wilson et al. 1984 equations

PERCENT CHANGE FROM BASELINE AT WEEK 144

mITT: INDIVIDUAL PATIENT DATA: 6MWT, MIP AND MEP % PREDICTED

Ambulatory Patients	Age at Baseline	% Change in 6MWT Max Distance (m) at 36 wks	Age at WK 144	% Change in Outcome Measures FROM WK 36 THROUGH WK 144¥		
				6MWT (max)	MIP % Predicted	MEP % Predicted
ETEPLIRSEN	9.03	-4.5	11.80	-10.4	+4.4	-7.3
ETEPLIRSEN	10.53	-10.8	13.30	+8.1	+11.7	+2.7
ETEPLIRSEN *	7.29	+14.2	10.06	-29.3	+19.8	-21.3
ETEPLIRSEN	8.79	-7.5	11.56	-1.4	+8.9	-0.7
ETEPLIRSEN **	10.95	-14.5	13.72	-3.8	+9.2	-31.2
ETEPLIRSEN	9.60	-4.2	12.37	+17.5	+82.7	+16.9
PBO/Delayed Tx	7.56	-15.4	10.33	+10.2	-11.0	-12.8
PBO/Delayed Tx †	10.03	-17.0	12.80	-25.2	+27.2	+17.8
PBO/Delayed Tx	7.55	-16.2	10.32	-21.2	+2.7	-28.2
PBO/Delayed Tx	10.12	-18.9	12.89	-11.6	+63.2	+0.8



Average Age at WK 144: Eteplirsen = 12.1 yrs; Placebo/Delayed Tx = 11.6 yrs

¥ From last time point before dystrophin confirmed in placebo patients

** Youngest patient in Study; ** Oldest patient in Study; † Patient recovering from broken left ankle and was unable to perform test at Week 84*

6MWT Percentages based on maximum value (predefined primary analysis) of two measures taken at baseline

PARENTS-REPORTED QUALITY-OF-LIFE-RELATED STATEMENTS IN STUDY 201/202

PROVIDED TO THE FDA AT THE AGENCY'S REQUEST

Age (yr)	Specific Parent Comments
10.75	He used to be tired after school – now comes home and plays constantly. PE teacher is 'impressed with his stamina.'
12.25	PE teacher noticed he was more active and participating in more activities than before. The child ran around with his friends 3 days in a row without fatigue (typically this is exhausting for him).
12.67	Used to be 'couch potato,' now he wants to play with friends after school, go outside, play basketball. Used to go to sleep immediately after school Friday night due to fatigue and sleep until 11am the next day. Now he goes to bed and gets up at a normal hour.
11.25	Mother received feedback that the child was running around getting into trouble at school and the teachers had difficulty catching him.
11.25	Prior to treatment, used a chair most of the day. During a recent family trip to D.C., he didn't use a chair at all. The child completed a 3-mile walk in October without fatigue.
9.00	Child walked throughout entire trip to Busch Gardens without fatigue
12.25	Opens bottles of soda independently; no reports of hand pain with handwriting tasks
11.75	Used to use a computer at school – handwriting has improved so he can handwrite more assignments
10.75	No longer needs help from mother to do math homework. He's understanding he can do new things and enjoys showing them off.
12.67	Mother reported he 'seems happier' and 'feels better.'
11.25	Reading better; quicker processing – answers questions quicker
10.75	Plays basketball and football in gym class with peers. The child isn't tired after short running activities. Much more active in the pool – swimming and diving for objects on the bottom.
12.67	Joined the Dodgeball club and loves his PE class. Child also 'danced his butt off' for 3 hours at a school event without fatigue.
11.25	Used to be pushed around in chair in gym class – now runs around playing games with peers
12.25	Ability to jump off of and over things without falling (i.e. stairs, short wall at playground, jumped over bike rack). Mother reports he now 'gets a running start and takes a flying jump' onto parent's bed – never attempted this previously.
11.83	Previously used a rolling backpack; now carries a backpack to school. Can now step up onto a curb while carrying backpack.
11.25	Jumping; learning to skip/gallop; can open the car trunk, take out his backpack, and carry it into the house; can lift his chair onto his desk at school
9.25	Improved grip strength to swing independently at the park.
11.25	Used to have to ride the wheelchair accessible bus to school. Now his IEP has been changed to allow him to ride the bus with his peers.
10.75	Mother reported the child wants to do everything himself, because he realizes he can do more now.

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

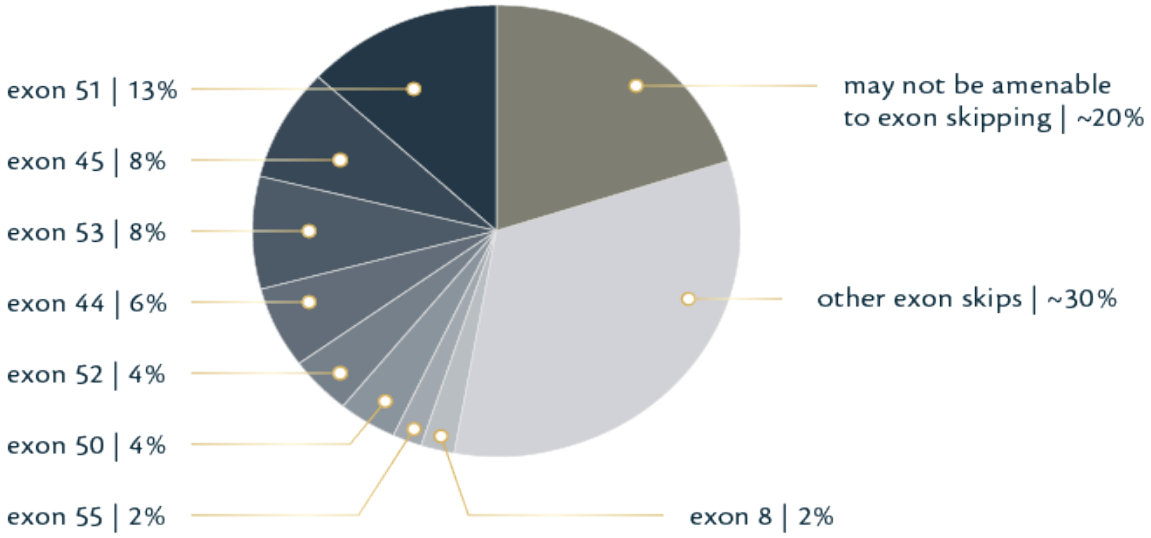
SAREPTA THERAPEUTICS

†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.

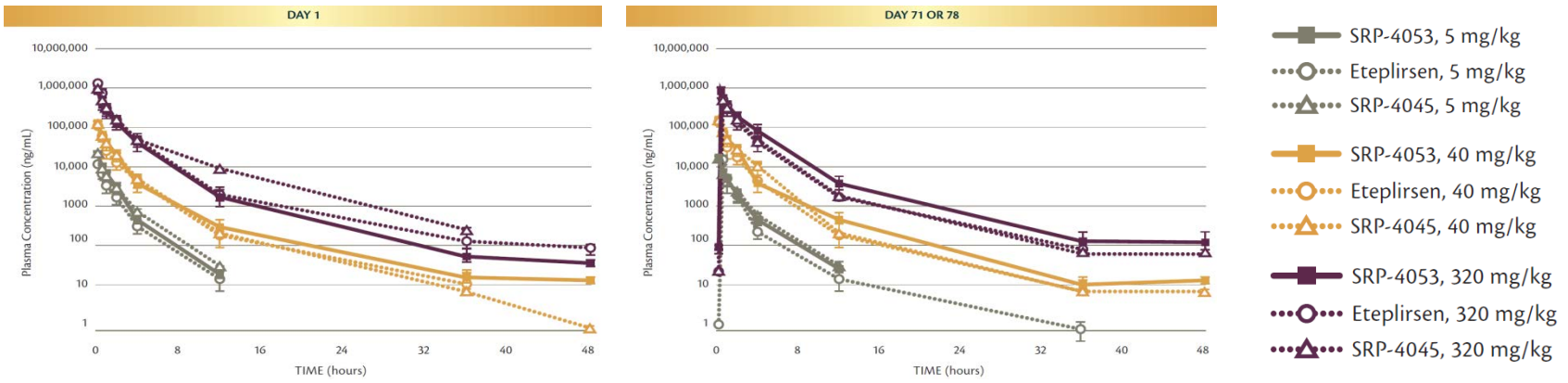
*Percent of DMD boys amenable to skipping each exon listed. Source: Annemieke Aartsma-Rus, et al. Hum Mutat. 2009 Mar;30(3):293-9.

THREE PMO DRUG CANDIDATES DEMONSTRATED REPRODUCIBLE PRECLINICAL SAFETY PROFILES

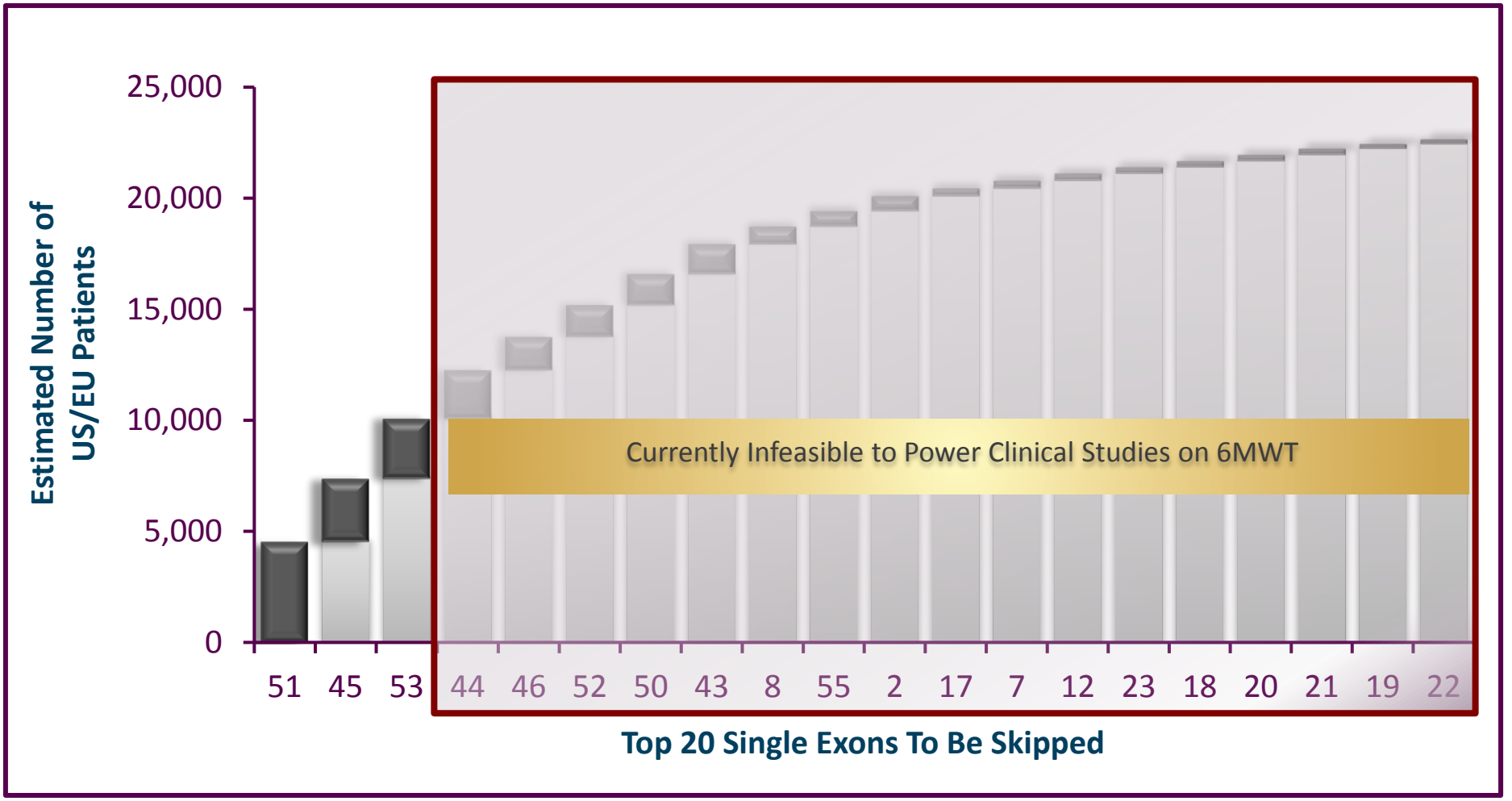
NO ADVERSE EFFECTS IN REPEAT DOSE TOXICITY EVALUATIONS UP TO 320 MG/KG

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

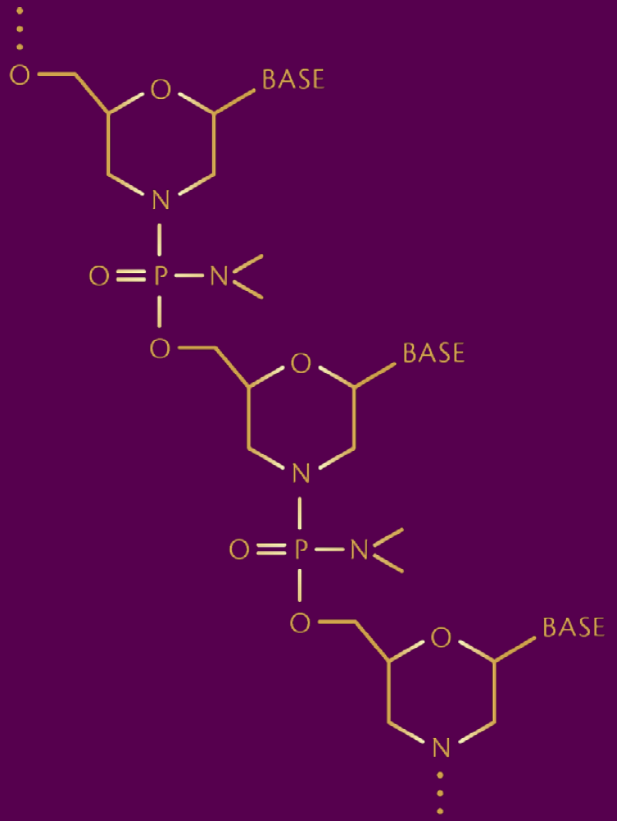


SAREPTA THERAPEUTICS

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

SAREPTA
THERAPEUTICS

RESEARCH PIPELINE



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

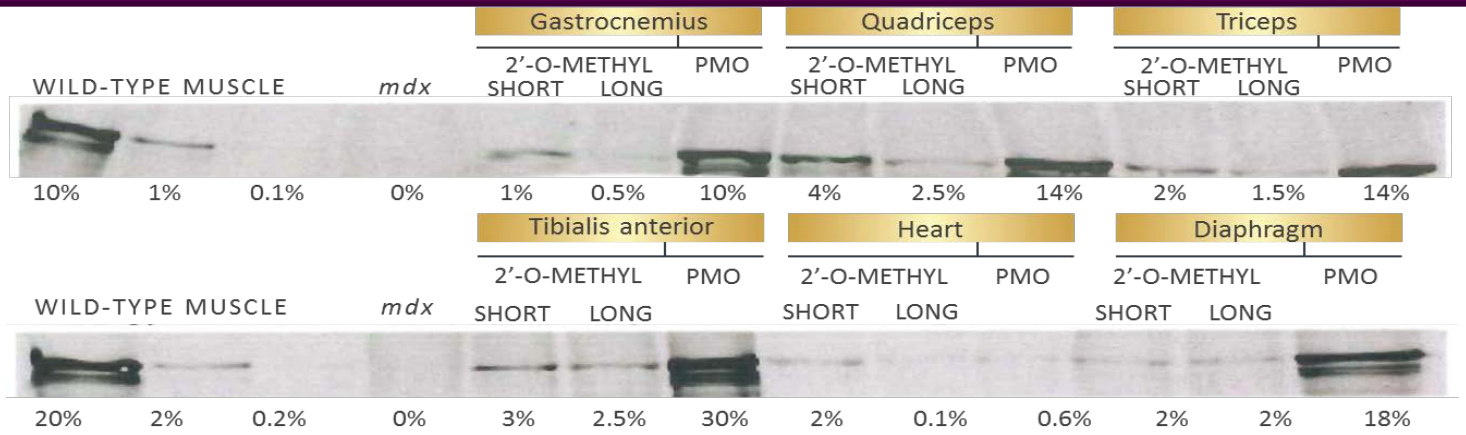
RESEARCH MOVING FORWARD

- 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

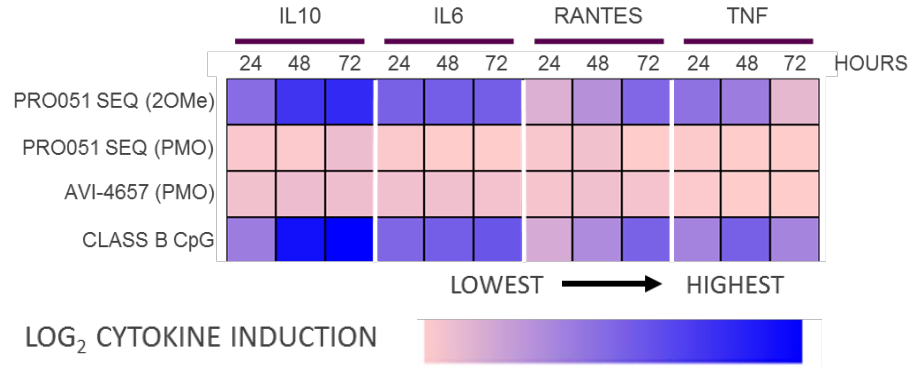
CHEMISTRY, SEQUENCE, AND SAFETY ADVANTAGES IN DMD

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



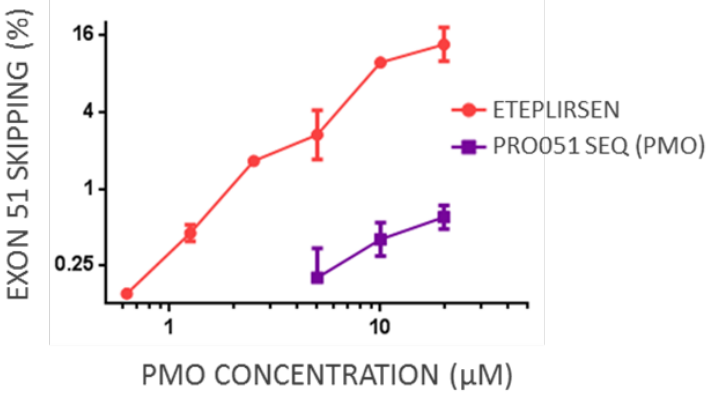
Source: Heemskerk, et al, 2009

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

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

EBOLA DRUG (AVI-7537) DEVELOPMENT: HISTORY

Extensive target optimization and library developed → **2004 USAMRIID request for real-life laboratory event** → **Extensive collaborative effort culminating in 2010 JPM/TMT award**

Date	Milestone	Collaborators/Partners
2002	Evaluation of PMO chemistry as an antisense oligomer to target negative sense single strand viruses; proof of concept as an anti-infective	UTMB, USAMRIID
2004	USAMRIID lab accident leads to request for rapid response for potential high-risk Ebola exposure; optimized target and synthesis of agent delivered to USAMRIID in 7 days.	USAMRIID, NMRL
2005-2009	Discovery and target optimization studies begin; more than 60 mouse and 14 guinea pig studies performed. Cell line toxicity studies explored	NIAID and USAMRIID
2010	JPMS/TMT awards AVI BioPharma (renamed Sarepta Therapeutics in 2011) MCM platform award to develop PMOplus® countermeasures for ZEBOV and MARV	USAMRIID, DOD/MCS
2012	Successful completion of 18 NHP studies including 5 pivotal studies with AVI-7537 or AVI-6002; completion of Phase 1 SAD in healthy adults; completion of toxicity assays. Program stopped due to funding constraints.	USAMRIID, DOD/MCS
2014	Ebola outbreak and Public Health Emergency of Global Importance. DOD purposes AVI-7537 back to Sarepta; fill/finish completed; subunit testing underway for >250 treatment courses	

75% NHP SURVIVAL AGAINST EBOLA LETHAL CHALLENGE WITH PMO*PLUS*® TREATMENT [1,2]

0/27 (0%) controls survived – All controls died by day 11 after infection

AVI-7537 EBOLA VIRUS

- Targets Ebola VP24 – appears well conserved and has no base pair mismatches compared to reported clinical isolates from 2014 outbreak
- 5 studies, 77 Rhesus macaques infected with 1000 pfu (highly lethal dose) Ebola-Zaire
  - 27 controls, 50 exposed to drug
- **73.5% (17/25**) overall survival (range 60-80%) of those receiving 20-40 mg/kg/day of AVI-7537 for 14 days
- **100% (2/2**) of surviving monkeys re-challenged with highly lethal dose of virus at day 50 survived without retreatment
  - Treatment did not interfere with development of antibodies against Ebola virus
- **0/27 (0%)** controls survived - <u>all controls died by day 11 after infection, median mortality seen by day 8</u>

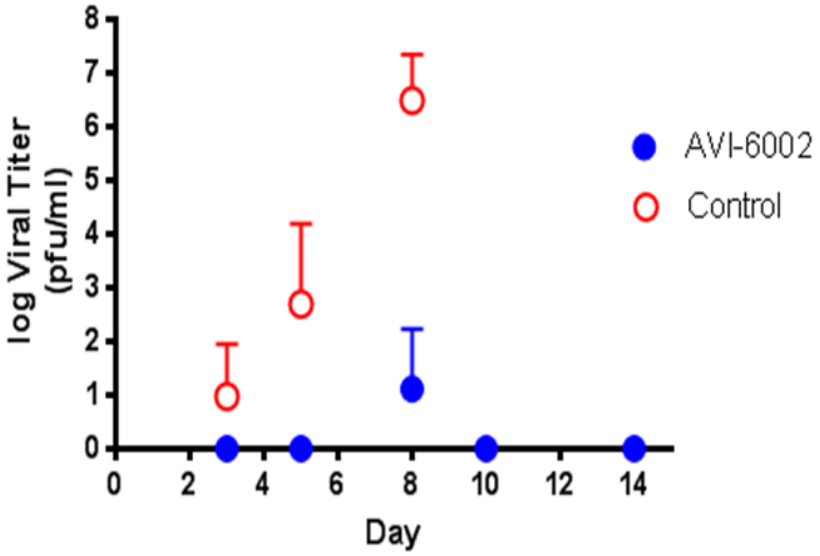
SAREPTA THERAPEUTICS

[1]Iverson, TIDES Las Vegas, NV [2]Wong, OTS 2014 San Diego Internal Sarepta data

AVI-7537: POTENT ANTIVIRAL EFFECT IN NHPs

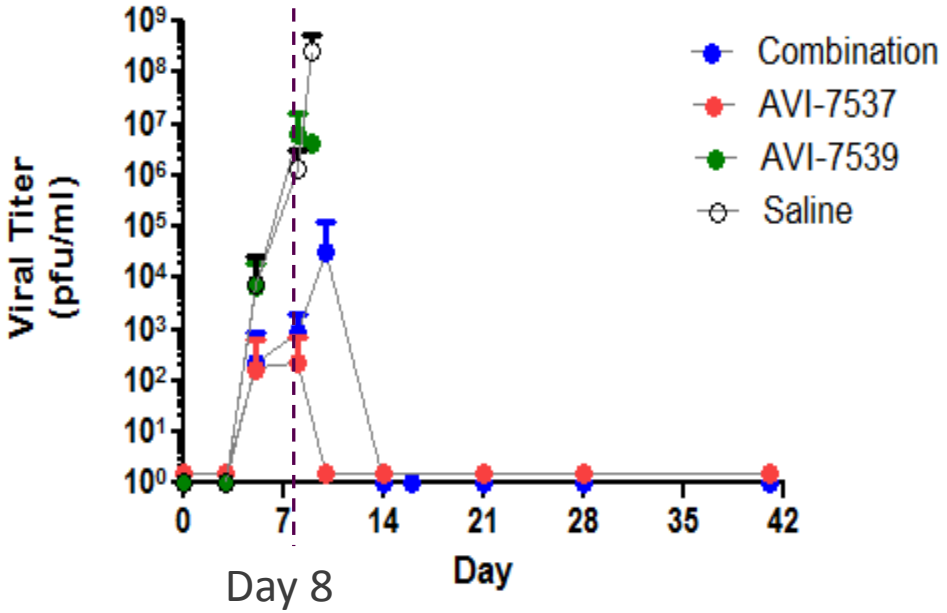
CLEAR DOSE AND SEQUENCE DEPENDENT VIRAL LOAD SUPPRESSION BY PLAQUE ASSAY AND QRT-PCR[1,2]

Study 6, Proof-of-Concept



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 5 log lower than control.

Study 15, Component vs. Combo



Day 8

Design: 40 mg/kg/day IV x 14 days,
AVI-6002 vs. AVI-7537 vs. AVI-7539 vs. control
N= 8 (4M/4F) each treatment group; 6 (3/3) control
Peak recoverable viable virus at Day 8 in AVI-7537 treated group ~5 log lower than control or AVI-7539 treated animals.

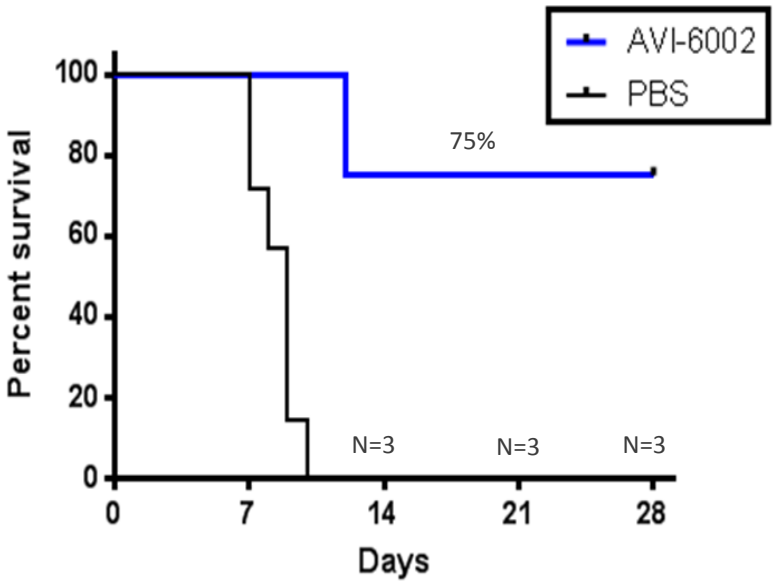
SAREPTA THERAPEUTICS

[1]Iverson, TIDES Las Vegas, NV [2]Iverson, Viruses 2012 Internal Sarepta data

AVI-7537: SIGNIFICANTLY IMPROVED SURVIVAL IN NHPs

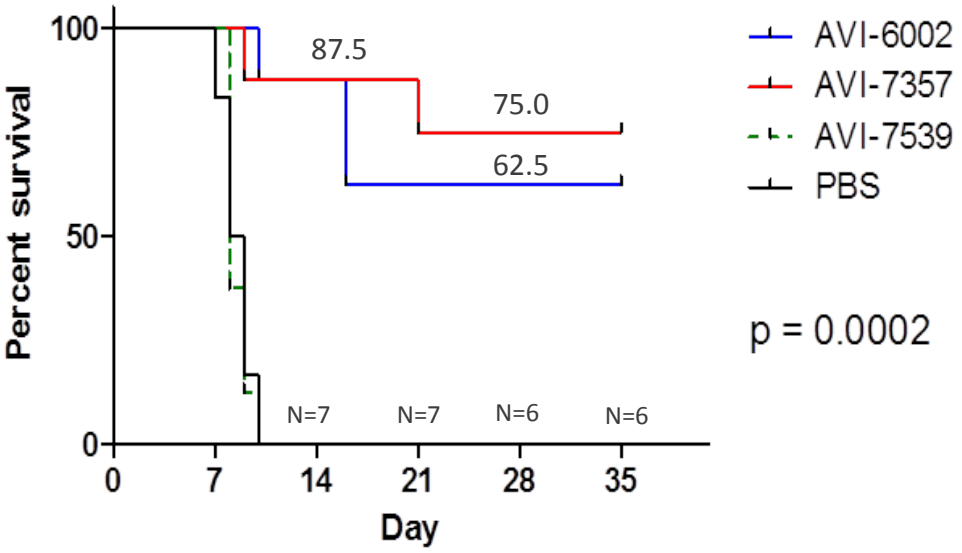
NHP SURVIVAL IS DOSE DEPENDENT[1,2]

Study 6, Proof-of-Concept



Proof of Concept
Design: 40 mg/kg/day IM/IP x 14 days
4:1 AVI-6002 to placebo

Study 15, Component vs. Combo



Component vs. Combination vs. Placebo
Design: 40 mg/kg/day x 14 day AVI-6002 vs. AVI-7537 vs. AVI-7539
8 animals per treatment group; 6 animals saline controls
All animals (6 saline, 8 AVI-7539) died by day 10.

SAREPTA THERAPEUTICS

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

AVI-6002 (w AVI-7537):
SINGLE ASCENDING DOSE SAFETY STUDY IN HEALTHY HUMAN SUBJECTS

Dose Cohort	Dose Level 1:1 AVI 7537 and AVI 7539	N = (study subjects and placebo)	DSMB Review
1	0.01mg/kg	4+1	No observed safety concerns – proceed to next dose level
2	0.1 mg/kg	4+1	No observed safety concerns – proceed to next dose level
3	1.0 mg/kg	4+1	No observed safety concerns – proceed to next dose level
4	3.0 mg/kg	4+1	No observed safety concerns – proceed to next dose level
5	6.0 mg/kg	4+1	No observed safety concerns – proceed to next dose level
6	9.0 mg/kg	4+1	No observed safety concerns- proceed with development

SAREPTA THERAPEUTICS

Clinical Study Report, 6002-us-101, NCT01353027, 22 March 2011

AVI-7288 (SAME BACKBONE CHEMISTRY AS AVI-7537)

MULTIPLE ASCENDING DOSE SAFETY STUDY IN HEALTHY HUMAN SUBJECTS

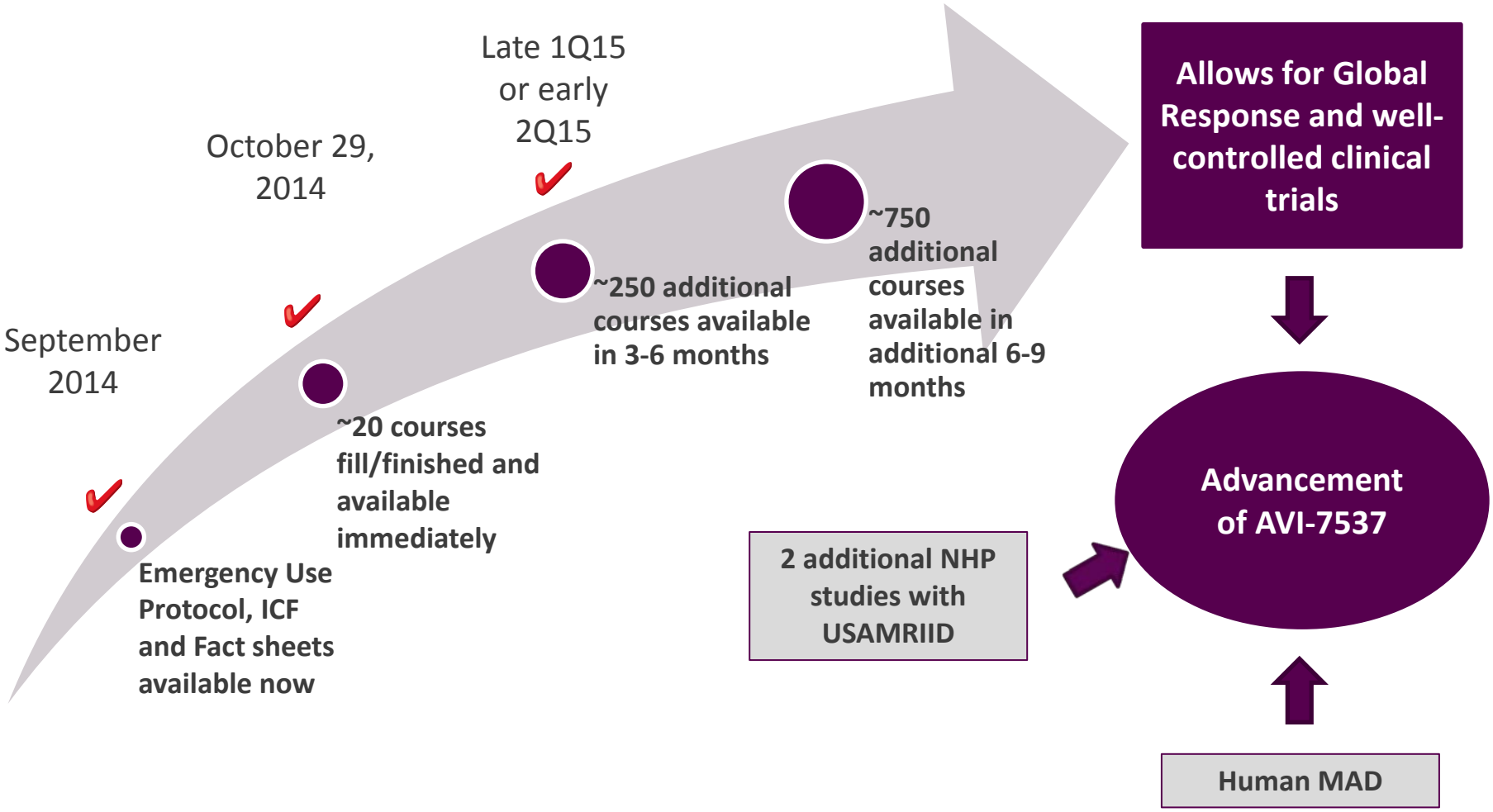
Dose Cohort	Dose Level mg/kg	N = (3:1 active: placebo)	Total Weekly Dose	Mean Total Dose Received per Subject	DSMB Review
1	1	8 (6:2)	7 mg/kg	153 mg	No observed safety concerns – proceed to next dose level
2	4	8 (6:2)	28 mg/kg	793 mg	No observed safety concerns – proceed to next dose level
3	8	8 (6:2)	56 mg/kg	1366 mg	No observed safety concerns – proceed to next dose level
4	12	8 (6:2)	84 mg/kg	1810 mg	No observed safety concerns – proceed to next dose level
5	16	8 (6:2)	112 mg/kg	3000 mg	No observed safety concerns – continue development of AVI-7288

- No observed safety concerns up to 16 mg/kg/day x 14 day dosing (highest mean total exposure 3000 mg per subject)
- Higher than the estimated human efficacious dose of 10 mg/kg/day

SAREPTA THERAPEUTICS

*Heald A et al. ECCMID, Barcelona 2014, ABSTR # ECCMI-1265

AVI-7537: SAREPTA'S EBOLA PREPAREDNESS AND DEVELOPMENT PROGRAM



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 focused on infections of highest medical need and large hospital-based opportunities
  - Six programs identified and underway

CDC Designated Antibiotic Resistance Threats, US, 2013 (Sarepta programs bold)

Urgent

- *Clostridium difficile*
- **Carbapenem-resistant *Enterobacteraceae***
- **Drug-resistant *Neisseria gonorrhoeae***

Serious

- **Multi-drug resistant *Acinetobacter***
- **Extended spectrum β–lactamase producing *Enterobacteraceae* (ESBL)s**
- **Multi-drug resistant *Pseudomonas aeruginosa***
- Drug-resistant *Campylobacter*
- Drug resistant *Salmonella* spp.
- Drug resistant *Shigella*
- **Methicillin resistant *Staphylococcus aureus***
- Drug resistant *Streptococcus pneumoniae*

Infectious Diseases Society of America designated MDR-bacterial threats, 2013:
ESKAPE: *Enterococcus faecium*, ***Staphylococcus aureus, Klebsiella pneumoniae, Acinetobacter baumannii, Pseudomonas aeruginosa, Enterbacter spp***

SAREPTA THERAPEUTICS

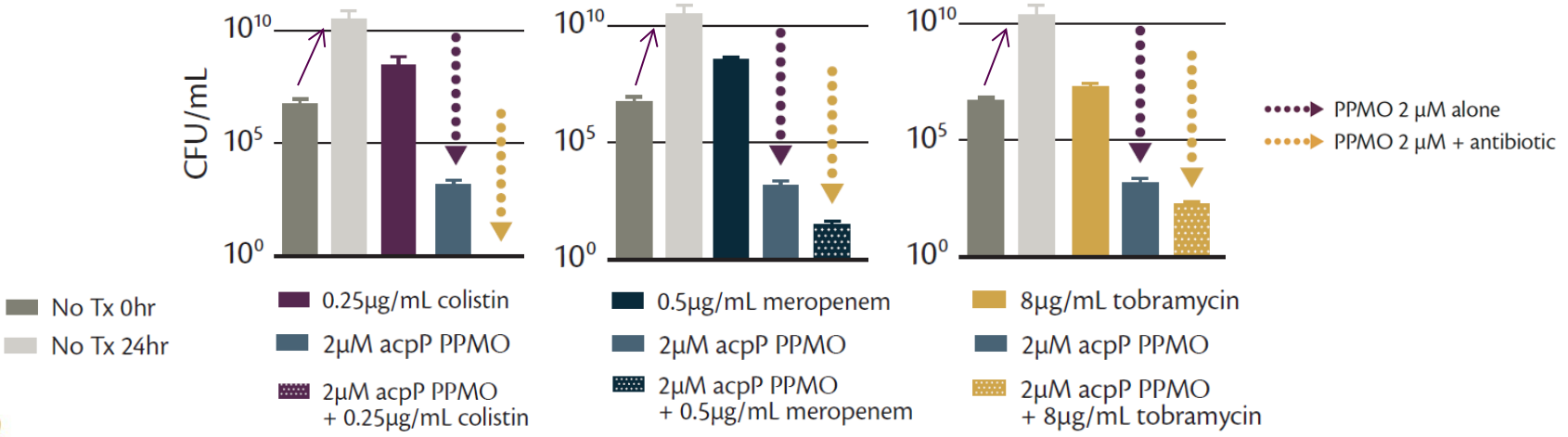
1. CDC; *Antibiotic Resistance Threats in the United States, 2013*
2. *EMA and ECDC; The Bacterial Challenge: Time to React, 2009*

PPMO RESTORES ANTIBIOTIC SUSCEPTIBILITY IN RESISTANT STRAINS

PPMO ALONE REDUCED BACTERIAL COLONY FORMING UNITS GREATER THAN ANTIBIOTICS; CO-ADMINISTRATION OF PPMO AND ANTIBIOTICS RESULTED IN SIGNIFICANT KNOCKDOWN

- Sarepta focusing efforts on a total of 6 bacterial strains and seeing positive results
- Additional focus on major bacterial infections affecting children with Cystic Fibrosis
  - Pseudomonas & Burkholderia focus on infection in patients with CF
- 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*



SAREPTA THERAPEUTICS

PPMO INHIBITS BIOFILM PRODUCTION AND BACTERIAL GROWTH AND DEMONSTRATES SURVIVAL *IN VIVO*

PPMO AFFORDED 100% SURVIVAL IN *E. COLI* INFECTED MICE AT DOSES AS LOW AS 30 MG

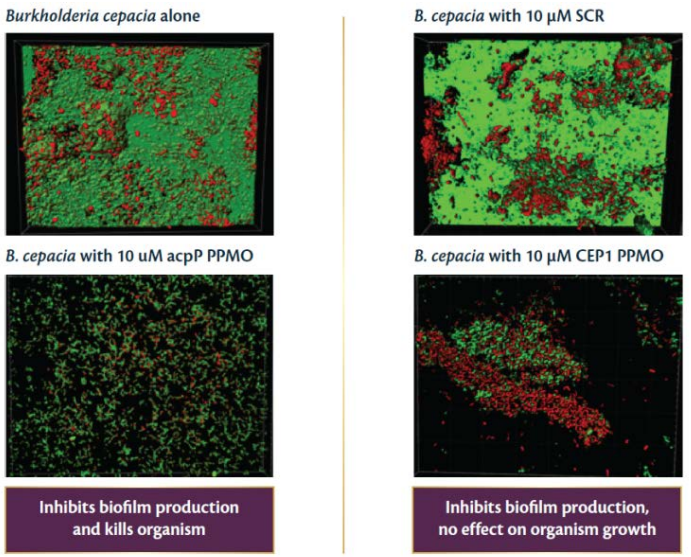
PPMOs as potent multi-organism anti-bacterial agents for hospital infections

- Antibiotic susceptibility restored in *A. baumannii* and *E. coli*
- Bacterial biofilm production & growth inhibited in Burkholderia Cepacia
- Bacterial infection reduced & survival promoted in *E. coli* infected mice
- 100% survival in treated mice; 0% survival for untreated mice

Red: *Burkholderia cepacia*; Green: biofilm

CONCLUSIONS FROM ICAAC POSTER PRESENTATIONS

- "PPMO's that target the *cepI* and *acpP* genes of *Burkholderia cenocepacia* J2315 were able to both prevent biofilm formation and breakdown existing biofilm"
- "NDM-1 PPMO's can be used in combination with carbapenems to kill carbapenem-resistant bacterial pathogens. This is a new strategy to combat pathogens that express NDM-1"
- "A single PPMO was effective against 3 different pathogens"



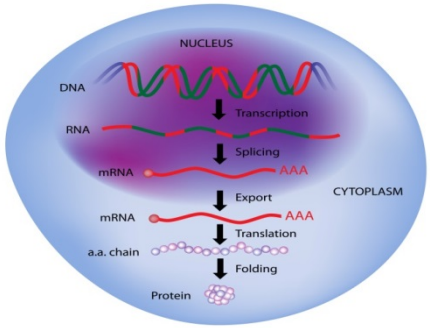
Sarepta can target the organism and biofilm or biofilm only

SAREPTA THERAPEUTICS

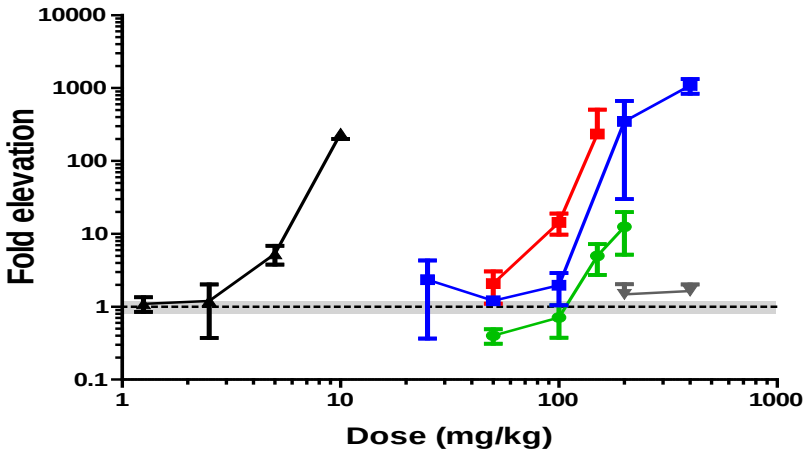
Smith B, et al. ICAAC 2014. Washington, DC. Abstract F-1552 | Mellbye BL, et al. 2009
Departments of Medicine and Microbiology, University of Texas Southwestern Medical Center, Dallas, TX, USA
Department of Microbiology, Oregon State University, Corvallis, OR, USA

SAREPTA'S CHEMISTRY PLATFORM PROGRESSING INTO MULTIPLE PROGRAMS

- >1600 PMO doses in DMD with no drug-associated AEs or safety signals (> 3 years in children) in 201/202
- NHP safety studies confirm safety profile (exon 51 & 53) with similar PK compared to Eteplirsen
- PMO*PLUS* dosed at 16 mg/kg in healthy adults daily (112 mg/kg) for 14 consecutive days without adverse effects
- PMO-X and PPMO show dramatically increased in vivo activity with wide therapeutic index

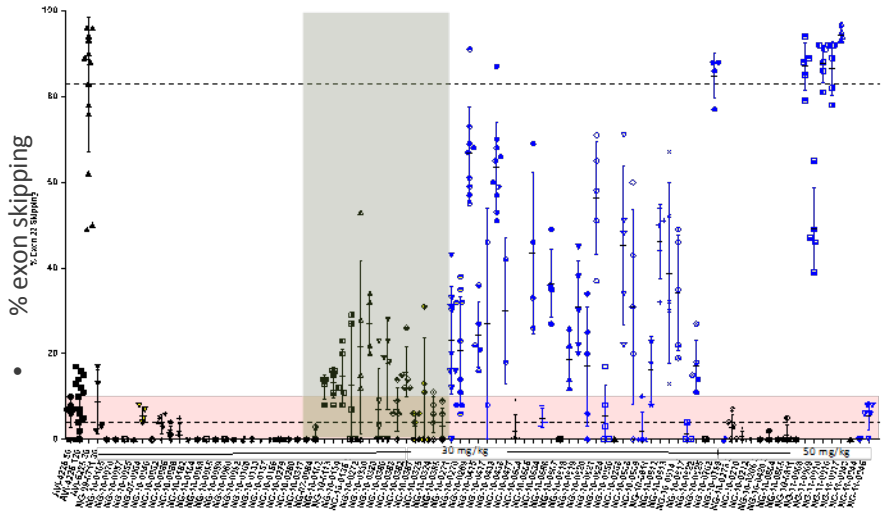


NEW CHEMISTRIES SHOWING PROMISE INDUCTION OF KIM-1



SAREPTA'S NEW CHEMISTRIES MOVING INTO ANIMAL STUDIES WITHIN MULTIPLE PROGRAMS

SAREPTA TESTING MULTIPLE NEW GENERATION CHEMISTRIES



SELECT CHEMISTRIES IN HEART MUSCLE

SAREPTA HAS INITIATED PROGRAMS IN MYOSTATIN, ADULT-ONSET POMPE AND PROGERIA

RESEARCH ONGOING UTILIZING MULTIPLE CHEMISTRIES FROM SAREPTA'S PLATFORM

Myostatin Inhibition

- Inhibition of myostatin at the pre-mRNA level should allow increases in muscle mass and strength
  - Different MOA utilized than other approaches which had off target affects
  - Research ongoing using PMO*plus*, PMO and PMO-X
- Major markets outside of DMD as follow-on indications
  - Other application outside DMD being explored
- Research ongoing and collaboration with Dr. George Dickson at Royal Holloway

Pompe Disease

- Sarepta's technology can be utilized in exons associated with Pompe Disease
- Potential to treat many adult onset patients at the pre-mRNA level
- Potential for off target affects likely limited based on PMO chemistry experience in DMD
  - Research ongoing with PMO and PMO-X
- Collaboration with Murdoch University, Steve Wilton and Sue Fletcher

Progeria

- Ultra-orphan disease state of the highest unmet need
- Sarepta's technology being applied to alter the aging affect seen in Progeria
  - Potential to apply technology to broader application if successful in Progeria
- Program initiated in collaboration with the NIH and University of Maryland

PROPRIETARY PROGRAM FOCUSED ON TOLL LIKE RECEPTORS (TLR) WITH A BROAD RANGE OF APPLICABILITY

RESEARCH PROGRAM INTIATED IN LUPUS AND GVHD; ASSET PROVIDES SAREPTA TARGETS IN MULTIPLE OTHER DISEASE STATES

TLR program may be applicable in a number of conditions that involve an innate immune response or a Th1-like immune response, including:

- Graft-versus Host Disease
- Autoimmune Diseases
- Inflammation
- Allograft rejection
- Cancer
- Infection
- Sepsis

TLR program can be used in the prevention of autoimmune disorders, an airway inflammation, inflammatory disorders, infectious diseases, skin disorders(e.g. psoriasis), allergy, and asthma including:

- Systemic Lupus Erythematosus (SLE)
- Inflammatory bowel disease
- Crohn's Disease
- Ulcerative Colitis
- Rheumatoid Arthritis
- Multiple Sclerosis
- Diabetes mellitus.

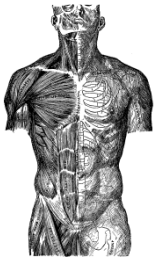
TLR signaling has also been linked to neurogenesis and was found to be involved in the pathogenesis of neurodegenerative diseases and could be used to prevent or treat neurodegenerative diseases such as:

- Alzheimer's disease
- Prion diseases
- Amyotrophic Lateral Sclerosis
- Parkinson's disease

SAREPTA THERAPEUTICS

R&D FOCUSED ON HIGH-VALUE TARGETS

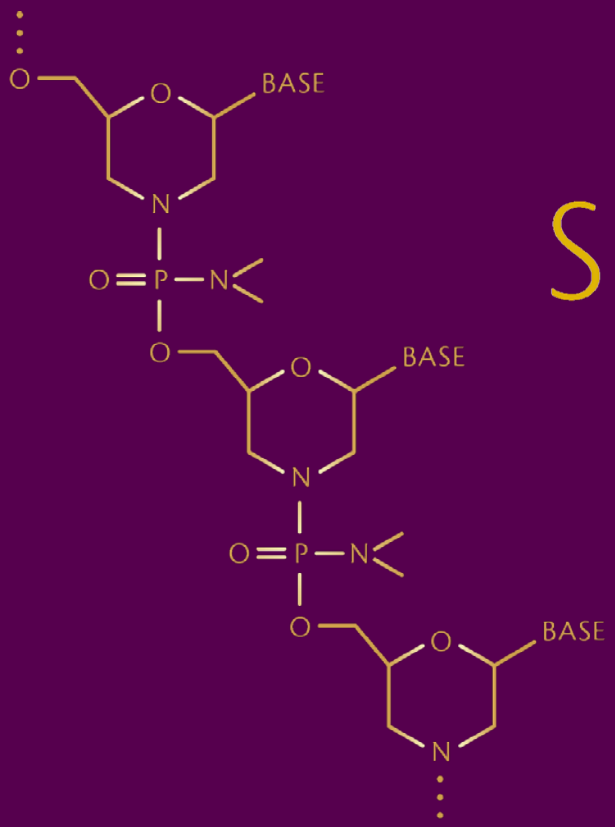
SAREPTA IS MAKING CONSIDERABLE PROGRESS IN APPLYING TECHNOLOGY INTO ADDITIONAL THERAPEUTIC AREAS



	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