UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): January 9, 2015

Sarepta Therapeutics, Inc.
(Exact name of registrant as specified in its charter)

Delaware	001-14895	93-0797222
(State or other jurisdiction of incorporation)	(Commission File Number)	(IRS Employer Identification No.)

215 First Street
Suite 415
Cambridge, MA 02142
(Address of principal executive offices, including zip code)

(617) 274-4000
(Registrant's telephone number, including area code)

(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Item 2.02 Results of Operations and Financial Condition.

Chris Garabedian, President and Chief Executive Officer of Sarepta Therapeutics, Inc. (the "Company"), will be conducting meetings with several investors attending the 33nd Annual J.P. Morgan Healthcare Conference (the "Conference") in San Francisco from January12, 2015 through January 15, 2015. At these meetings, Mr. Garabedian will disclose that the Company had cash and other investments of $211 million as of December 31, 2014 (unaudited).

Item 5.02 Departure of Directors or Certain Officers; Election of Directors; Appointment of Certain Officers; Compensatory Arrangements of Certain Officers.

On January 9, 2015, after a tenure of more than a decade on the Company's Board of Directors (the "Board"), John Hodgman notified the Company that he will be participating in an extended religious mission and, as result, has decided to resign from his positions as Director, Interim Chairman of the Board, Chairman of the Audit Committee and member of the Board's audit and compensation committees effective on the date of the next annual meeting of stockholders, which the Company anticipates will be held in June of this year. There were no known disagreements between Mr. Hodgman and the Company or any officer or director of the Company which led to Mr. Hodgman's resignation.

"We at Sarepta value John's sage advice and will miss his insightful guidance as a director and in his role as interim chair. We thank him for more than a decade of service to the Company and wish him all the best in his missionary assignment and future endeavors" said Chris Garabedian, President and Chief Executive Officer of the Company.

Item 7.01 Regulation FD Disclosure.

The disclosure in Item 2.02 above is hereby incorporated by reference into this Item 7.01.

As part of the meetings at the Conference, on January 15, 2015, Mr. Garabedian will deliver the slide presentation attached to this report as Exhibit 99.1, which is incorporated herein by reference.

The information in this report and Exhibit 99.1 to this report is furnished pursuant to Items 2.02 and 7.01 and shall not be deemed "filed" for the purposes of Section 18 of the Securities and Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section. It may only be incorporated by reference in another filing under the Exchange Act or the Securities Act of 1933, as amended, if such subsequent filing specifically references the information furnished pursuant to Items 2.02 and 7.01 of this report.

Item 9.01. Financial Statements and Exhibits.

(d) *Exhibits*. The following exhibit is furnished as part of this report.

Exhibit No.	Description
99.1	Sarepta Therapeutics, Inc. slide presentation to be presented at the 33rd Annual J.P. Morgan Healthcare Conference on January 15, 2015.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Sarepta Therapeutics, Inc.

By: /s/ Christopher Garabedian
Christopher Garabedian
President and Chief Executive Officer

Date: January 12, 2015

Exhibit 99.1


SAREPTA
THERAPEUTICS
REALIZING THE POTENTIAL OF
RNA-BASED TECHNOLOGY
JP MORGAN HEALTHCARE CONFERENCE
JANUARY 15, 2015

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 our use of unique PMOs to develop treatments in areas of high unmet need; our plans to continue advancing eteplirsen towards regulatory approval and moving follow-on exons into human clinical trials; the potential broad application of our chemistries and technologies across a variety of disease areas; our strategic research focus and plans to continue advancing our chemistry platform and technologies, including our Toll-like Receptor Antagonist Program, into additional indications in rare or other diseases and against antibiotic-resistant bacterial infections, including through collaborations; our capitalization status; the potential market for eteplirsen and follow-on exon skipping drugs; our plans and ability to comply with the U.S. Food and Drug Administration (FDA) requirements to consider a new drug application (NDA) submission for eteplirsen complete; our plans and ability to successfully initiate and complete additional clinical trials for eteplirsen and other follow-on product candidates in Duchenne Muscular Dystrophy (DMD) and providing additional data, analysis and other information requested by the FDA and potential timing of the same; the potential of and timing of an NDA submission by us, which will continue to be evaluated based on FDA discussions and as additional data become available, and a potential filing and acceptance of an NDA for eteplirsen by the FDA on an accelerated or other pathway; our continuing discussions with the EMA regarding a potential approval pathway in Europe; our beliefs regarding the potential of and safety and efficacy of our product candidates, chemistries and technologies in DMD, rare and infectious diseases and other disease areas; 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 potentially commercialize our product candidates without claims of infringement.

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 and or the use of or application of our chemistries and technology 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 potential 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 or demonstrate a treatment benefit ; there may be delays in timelines or we may not make an NDA submission orsuccessfully initiate, conduct or complete clinical trials, or make eteplirsen or any of our product candidates, chemistries or technologies commercially available for regulatory or other 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; and those risks 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, 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.

SAREPTA THERAPEUTICS

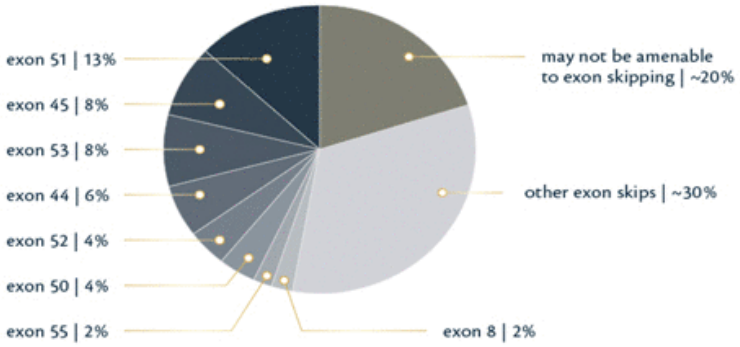
CORPORATE HIGHLIGHTS

- Sarepta is a late-stage development company utilizing RNA-based technology with unique phosphorodiamidate morpholino oligomers (PMOs) to develop treatments for areas of high unmet need
- Priority remains on DMD – advancing eteplirsen towards regulatory approval, moving follow-on exons into human clinical trials
- Differentiated PMO chemistries with broad application across variety of disease areas
- Significant progress made advancing chemistry platform into additional indications in rare diseases and against antibiotic-resistant bacterial infections
- 5 DMD clinical trials underway
- More than 10 new research programs underway
- 2 Chemistries tested in humans (PMO & PMO*plus*®)
- Well-capitalized with ~$211 million in cash and other investments as of 12/31/2014

DUCHENNE MUSCULAR DYSTROPHY

DEVASTATING RARE DISEASE WITH SIGNIFICANT UNMET NEED

- Affects approximately 1 in 3,500[1] boys worldwide
- 25,000- 30,000 patients in the U.S. and Europe
- Sarepta's lead program (Exon 51) estimated to target ~13% of DMD patients[2]
- Follow-on Exon-Skipping Drugs have potential to treat 60-80% of DMD patients



SAREPTA THERAPEUTICS

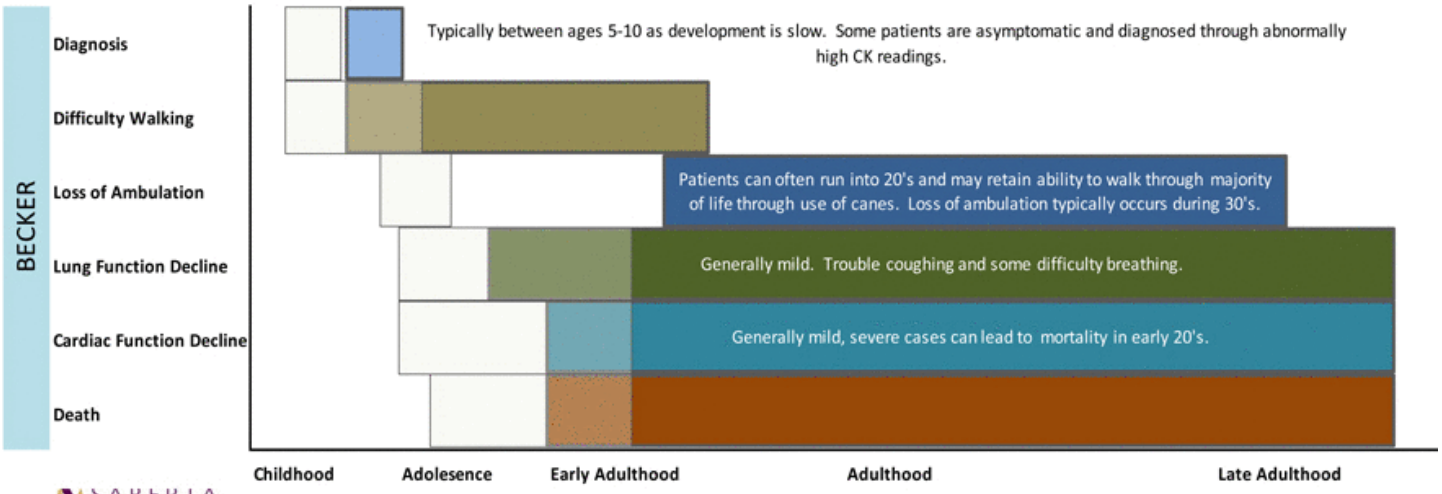
1: National Human Genome Research Institute

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

DMD COMPARED TO BECKER MUSCULAR DYSTROPHY (BMD)

ANY SHIFT FROM DUCHENNE TOWARDS BMD WOULD REPRESENT A MEANINGFUL DIFFERENCE IN PATIENT LIFE SPAN AND QUALITY OF LIFE

- BMD is a milder phenotype with a more gradual progression[1]
- The majority of patients with BMD remain ambulant significantly longer than patients living with DMD[1]



1. Muscular Dystrophy Association

REGULATORY UPDATE

DMD GLOBAL CLINICAL AND REGULATORY UPDATE

168 Week Data Released

- All evaluable patients remained ambulant
- Continued decline across all patients from week 144
- Ongoing stability of respiratory function
- NDA still planned for mid-2015
- NDA Submission will continue to be evaluated based on FDA discussions and as additional data become available

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 to begin dosing patients with 30mg/kg in 1H2015

FDA communicated additional data requirements for NDA submission now planned for mid-year 2015

- 168-week clinical data from study 201/202
- Safety data in newly exposed eteplirsen patients (subset with at least 3-month safety data); Dosing expected in 12-24 pts this month
- Results from 4th Biopsy are expected for subset of patients in Study 201/202; Scheduling of biopsies underway
- Dystrophin Rescore underway
- Patient level natural history data being collected

EMA Update

- Meeting in December provided preliminary guidance that additional clinical data will be needed for conditional approval
- EMA encouraged continued discussions as additional data is compiled from current and new studies

DMD CLINICAL PROGRAM STATUS (US AND EU):

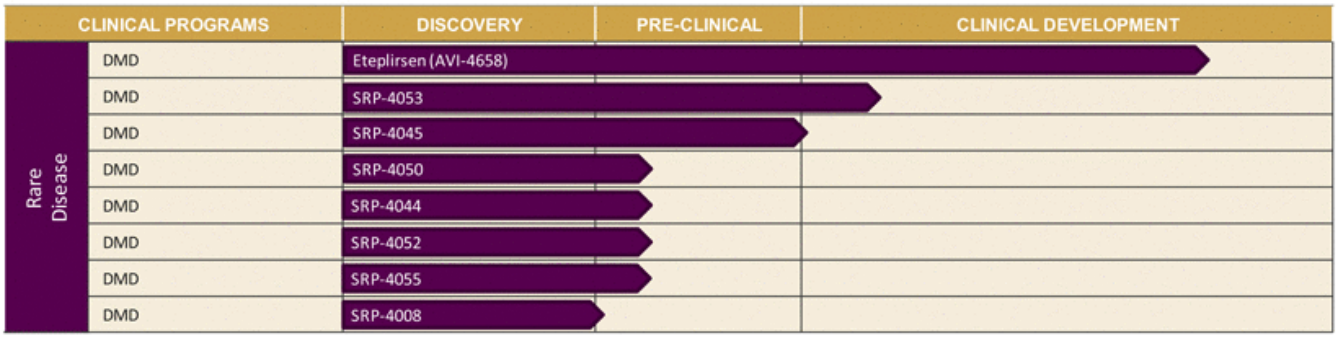
ETEPLIRSEN AND FOLLOW-ON EXON-SKIPPING DRUGS (EXONS 45 AND 53)

Study	Duration (weeks)	US/EU	n	Status	Exon Target Treatment	DMD Population
4658-33	Single Dose	EU	7	**Completed**	Exon 51	10-17 yrs, non-amb
4658-28	12	EU	19	**Completed**	Exon 51	5-15 yrs, amb
4658-201	28	US	12	**Completed**	Exon 51	7-13 yrs, amb
4658-202	240[1]	US	12	**Data through 168 Weeks**	Exon 51	7-13 yrs, amb
4658-301	48	US	120	**Dosing**	Exon 51	7-16 yrs, amb
4658-204	96	US	20	**Dosing**	Exon 51	7-21 yrs, non-amb
4658-203	96	US	20	**Dosing 1Q2015**	Exon 51	4-6 yrs, amb
4053-101	48	EU	36	**Dosing Jan 2015**	Exon 53	6-16 yrs, amb
4045-301	TBD	EU/US	90	TBD	Exon 45/53	7-16 yrs, amb

1. 212 weeks in extension phase for a total 201/202 duration of 240 weeks.

DMD PIPELINE UPDATE

SAREPTA'S EXON SKIPPING PLATFORM FOR DMD



DMD Clinical Development Status

PROMOVI 4658-301 – 39 Sites

- 2 of 2 Surgical Sites fully initiated
- 11 of 14 Hub Sites fully initiated
- 18 of 39 Infusion Sites fully initiated
- Partially enrolled with patients receiving eteplirsen
- Data potentially available late 2016 – early 2017

4658-204 – 4+ Sites

- Sites enrolling

4658-203 – 7 Sites Selected

- Enrolling Q1 2015

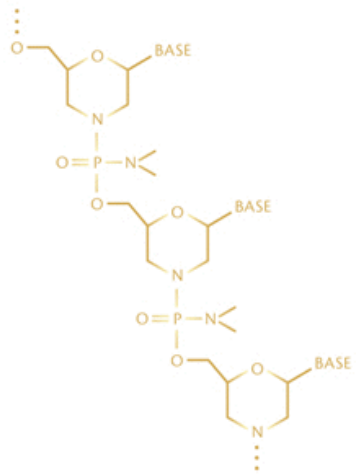
4053-101 (Europe) – 4 Sites

- Sites enrolling

PMO FOR THE TREATMENT OF DMD

CHRONIC LIFELONG THERAPY DEMANDS SAFETY

- >2080 doses, representing ~50 patient years across all studies
- Given to DMD boys with doses up to 50 mg/kg/wk for more than 3 years without clinically-significant treatment-related adverse events, dose-limiting toxicities or discontinuations
- 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 3 to 5 hours*
- Cleared through the kidney*
- Sequence-specific binding to pre-mRNA directs alternative splicing*

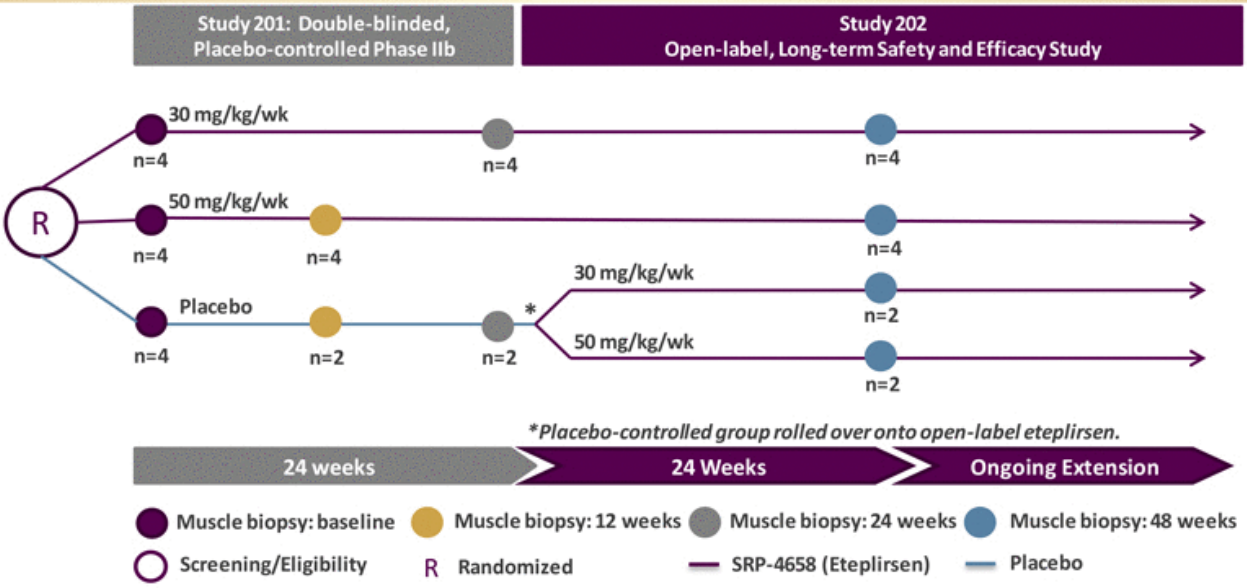


SAREPTA THERAPEUTICS

*Sarepta internal data on file

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.

ETEPLIRSEN STUDY 201/202 DESIGNED TO ENROLL MORE ADVANCED PATIENTS (EXPECTED TO DECLINE WITHIN 48 WEEKS)

STUDY	Baseline Age	Baseline 6MWT	<7 Excluded	Healthier Patients Excluded	Evaluation Time Period
Eteplirsen 201/202 Phase I/II	9.3	371	Yes	>440m (400 + 10%)	3.2 Years
DMD114117 (DEMAND II) Phase II	7.3	409	No	No	48 Weeks
DMD114876 (DEMAND V) Phase II	7.8	409	No	No	25 Weeks
DMD114044 (DEMAND III) Phase III	8.2	341	No	No	48 Weeks
Ataluren Phase IIb	8.5	357	No	No	48 Weeks

- Selected for an enriched population of patients expected to decline over 48 weeks
- Study screened out healthier subjects (defined by >440 meters at baseline)
- Controlled/prospective study in DMD without any treatment interruption or dose adjustment

SAREPTA THERAPEUTICS

* Per sponsor clinical study reports

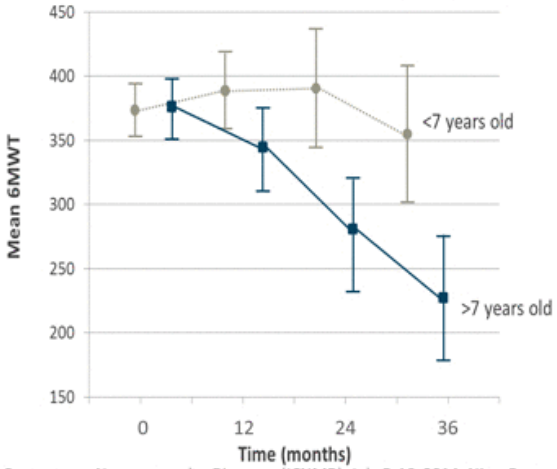
SIX MINUTE WALK TEST (6MWT) IS A WELL ESTABLISHED OUTCOME MEASURE

NATURAL HISTORY 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.
Note: Study imputed zero values for patients who lost ambulation during follow up periods

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]

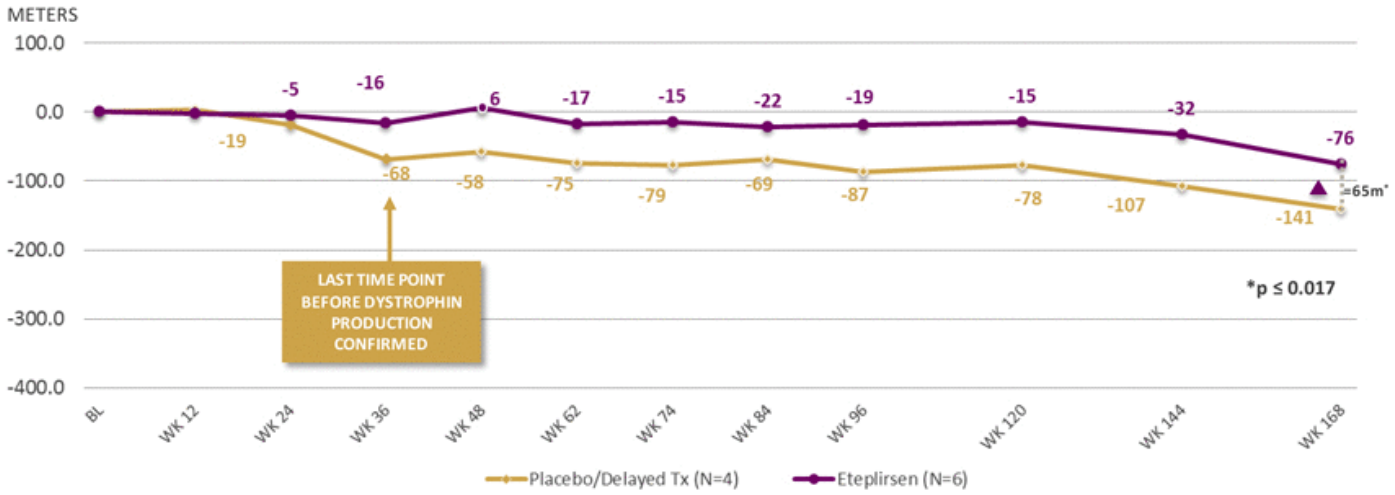
SAREPTA THERAPEUTICS

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
[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

LESS THAN 80 METERS LOST OVER 3.2 YEARS IN CONTINUOUS ETEPLIRSEN COHORT (mITT; n=6)

LESS THAN 80 METERS LOST OVER 2.5 YEARS IN PLACEBO/DELAYED ETEPLIRSEN COHORT FROM LAST TIME POINT BEFORE DYSTROPHIN CONFIRMED (n=4)



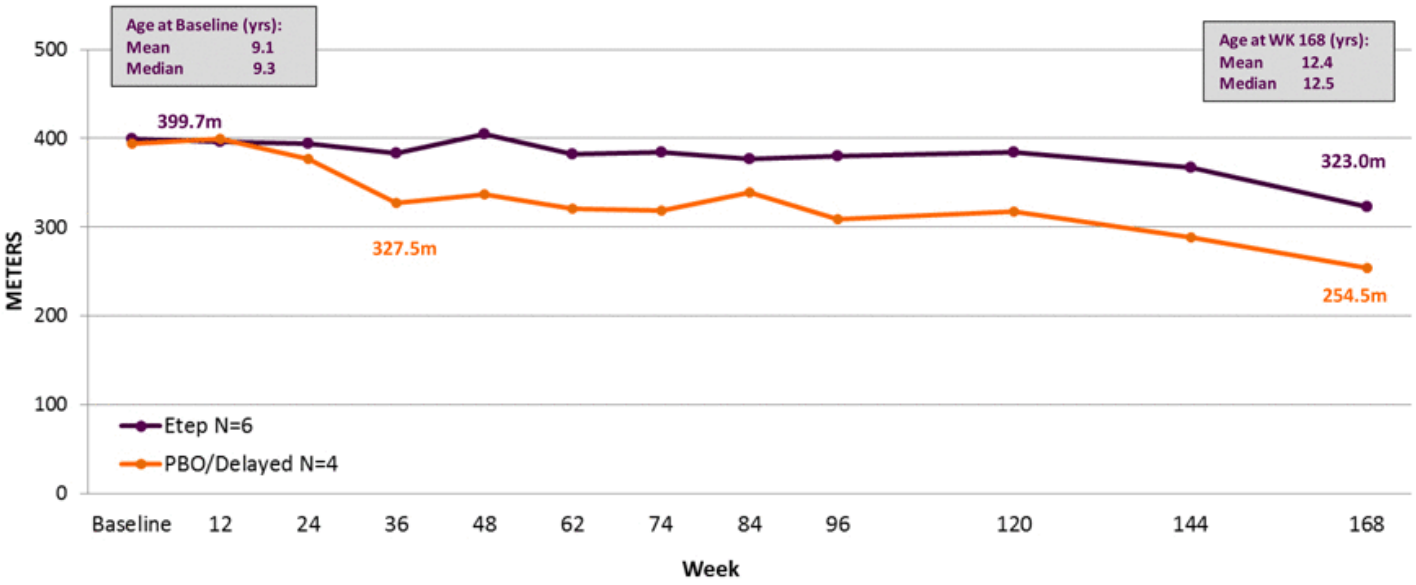
- After general stability on the 6MWT through 120 weeks, similar declines of walking distance were observed from week 120 through week 144 with 61 meter and 63 meter declines in the continuous eteplirsen and delayed eteplirsen treatment groups, respectively
- All patients showed declines in walking distance from week 144 to 168, including one patient in each arm that declined by more than 75 meters (highest 6MWT performer in continuous group; lowest 6MWT performer in delayed group)
- After 3.2 years of therapy the mean age of the boys in the continuous eteplirsen arm (mITT) was 12.6 years (median age 12.9)
- After 168 weeks of continuous eteplirsen treatment the mITT cohort (n=6) walked an average of 323 meters

SAREPTA THERAPEUTICS

Note: 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

PATIENTS MAINTAINED AVERAGE 6MWT DISTANCE OF MORE THAN 300 METERS THROUGH 168 WEEKS IN CONTINUOUS ETEPLIRSEN COHORT (mITT n=6)

PLACEBO-DELAYED PATIENTS DECLINED AT SIMILAR RATE AFTER DYSTROPHIN CONFIRMED

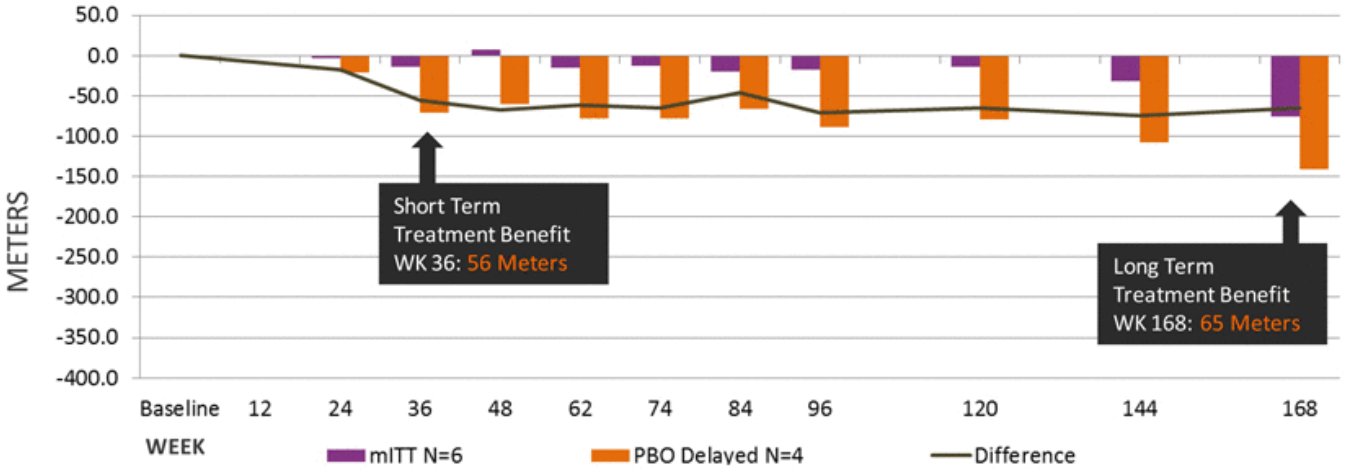


- 6MWT is a measure of total endurance and how well the entire body accomplishes the task. It is a test to help measure not only how muscles perform, but also lungs, and heart.

Note: 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

AFTER > 2.5 YEARS OF THERAPY (WEEK 36 – 168), THE OBSERVED TREATMENT DIFFERENCE BETWEEN PLACEBO-DELAYED AND CONTINUOUSLY-TREATED ARM REMAINS INTACT (mITT n=10)

6MWT CHANGE FROM BASELINE TO WEEK 168 IN STUDY 201/202



- After 3.2 years of therapy the mean age of the boys in the continuous eteplirsen arm (mITT) was 12.6 years (median age 12.9)
- Baseline ambulation for the continuous eteplirsen treatment cohort (mITT) was 399.7 meters and the placebo rollover cohort was 394.5 meters using maximum score of two measures

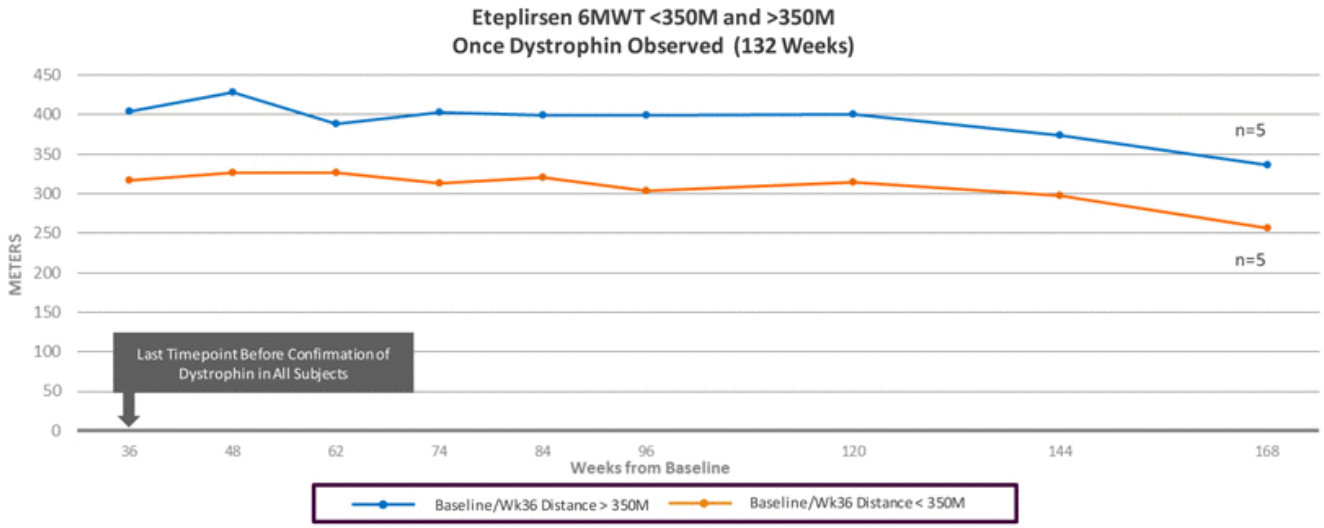
SAREPTA THERAPEUTICS

DATA BASED ON MAXIMUM 6MWT SCORE WHEN TEST WAS REPEATED

Note: 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

WALKING DISTANCE IN POPULATION ABOVE AND BELOW 350 METERS FROM LAST TIME POINT BEFORE DYSTROPHIN CONFIRMED (WEEKS 36-168), mITT POPULATION (n=10)

DATA BASED ON MAXIMUM 6MWT SCORE WHEN TEST REPEATED, ALL SUBJECTS > AGE 7 AT BASELINE



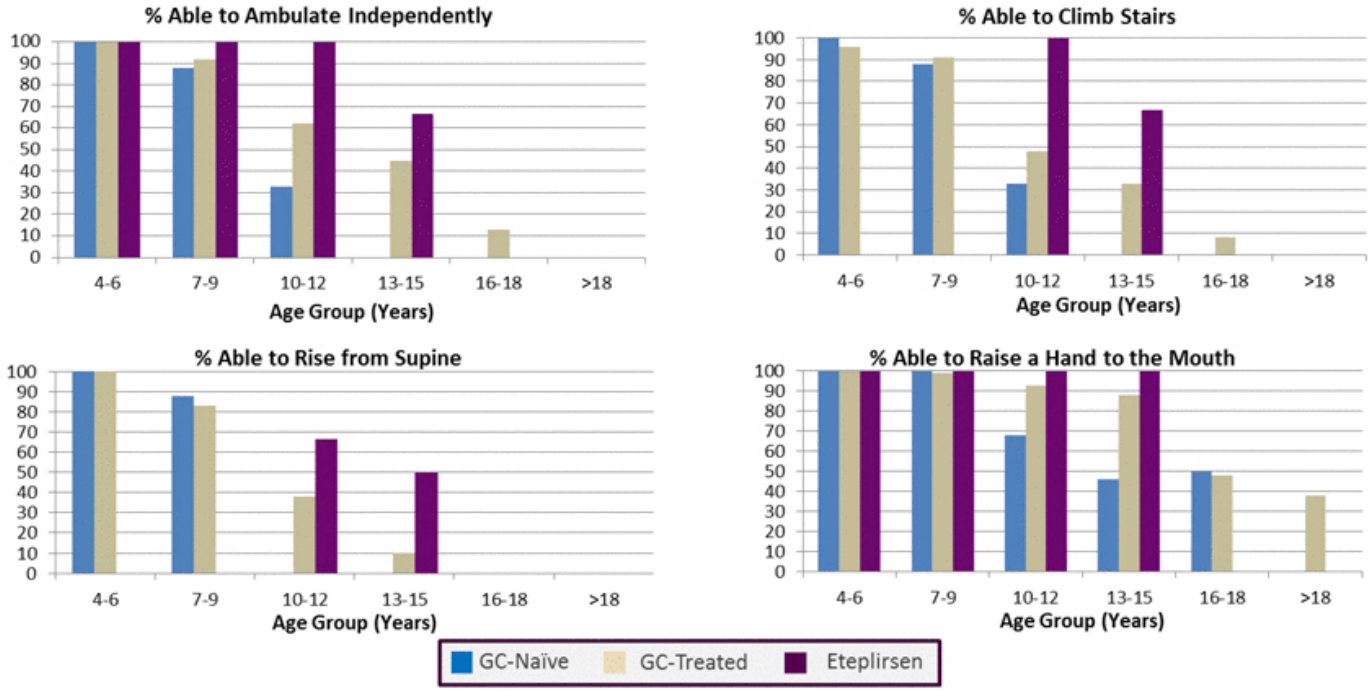
- The study group which walked <350m at week 36 (n=5) lost 61 meters in ambulation from the potential time of dystrophin production (Week 36) through Week 168 compared to loss of 68 meters in >350m group.

Note: Mean age: 9.8 years at week 36; 12.4 years at Week 168; 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 excludes two patients who experienced rapid decline and lost ambulation early in the study) Population using MMRM Test*

ETEPLIRSEN TREATED BOYS FUNCTION STATUS AT WEEK 168 (n=12)

SIX BOYS AGED 10-12 (AVG. AGE 11.5) AND SIX AGED 13-15 (AVG. AGE 13.4) AT WEEK 168 (n=12)

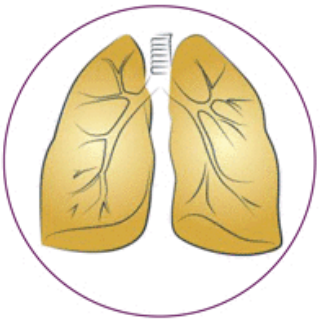
NATURAL HISTORY SUBJECT STUDY ON GC-TREATED VS. GC-NAÏVE PATIENTS* (n=347)

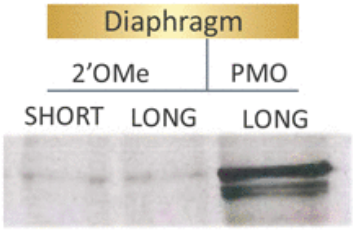


*Henricson, et al. 2013

PULMONARY STABILITY AS AN OUTCOME MEASURE IN DMD

PULMONARY FUNCTION TESTS: PHASE IIB 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
- FVC has been shown to decline at an average rate of 8 to 8.5 percent per year after 10 to 12 years of age
- Declines in MIP and MEP correlate with decreases in voluntary cough capacity

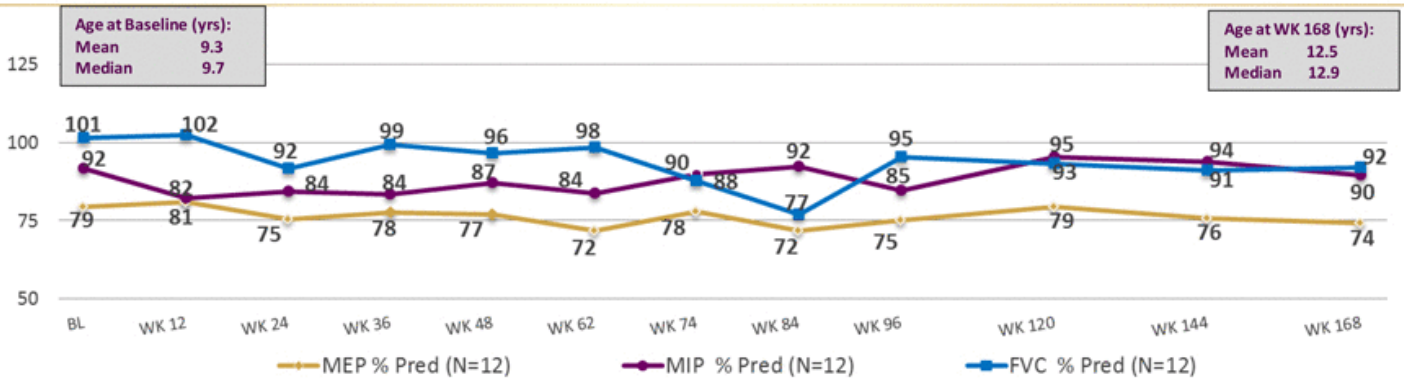
Significant increase of dystrophin expression achieved in diaphragm muscle of mdx mouse following PMO dosing

SAREPTA THERAPEUTICS

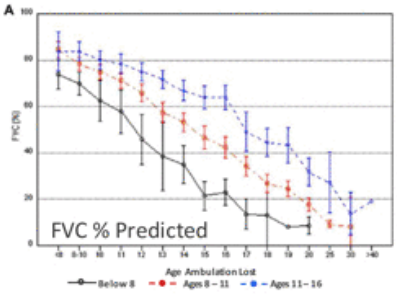
Braverman et al; Smith et al, 1987; Galasko et al, 1992; Hahn et al, 1997; Kang et al, 2000; Phillips et al, 2001; McDonald et al, 1995

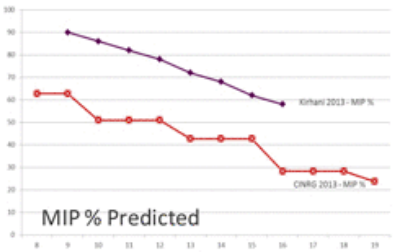
STABILITY OF PULMONARY FUNCTION IN ETEPLIRSEN-TREATED PATIENTS (ITT n=12)

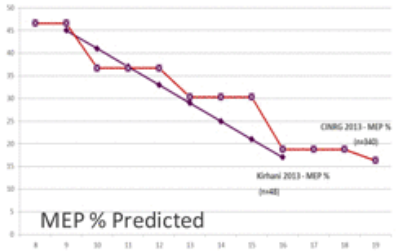
MIP & MEP RESPIRATORY FUNCTION DECLINES IN DMD ACCORDING TO NATURAL HISTORY



NATURAL HISTORY SHOWS DECLINES OVER TIME IN FVC, MIP & MEP % PREDICTED IN BOYS LIVING WITH DMD







Humbertclaude et al. European Journal of Pediatric Neurology 16 (2012) 149 e160

SAREPTA THERAPEUTICS

* Wilson et al. 1984 equations

ETEPLIRSEN SAFETY PROFILE

SAFETY PROFILE OF ETEPLIRSEN FOR LONG TERM USE

- **No clinically significant treatment-related adverse events observed through 168 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 >1890 doses (~50 patient years) administered in studies 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 142.3 out of 144 possible doses
  - Eteplirsen cohorts (n=8) completed on average 165.9 out of 168 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 greater than three years of weekly infusions
  - No reported incidents of erythema, induration or discoloration at injection sites

LONG-TERM SAFETY PROFILE OF ETEPLIRSEN (168 WEEKS)

SAFETY RESULTS OBSERVED WITH >1,890 DOSES OF ETEPLIRSEN (~50 PATIENT YEARS) ADMINISTERED in 201/202

TREATMENT-EMERGENT ADVERSE EVENT	PLACEBO FOR 24 WKS n=4 (%)	ETEPLIRSEN FOR 24 WKS n=8 (%)	ETEPLIRSEN FOR 144 WKS n=4 (%)	ETEPLIRSEN FOR 168 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	4 (50)
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 168 weeks, 97% (590/609) of assessments of protein in urine were negative

- 3% total positive assessments through 168 weeks (includes placebo)
- 1.1% of all assessments for subjects on eteplirsen (6/565) 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 168 weeks of treatment

SAREPTA THERAPEUTICS

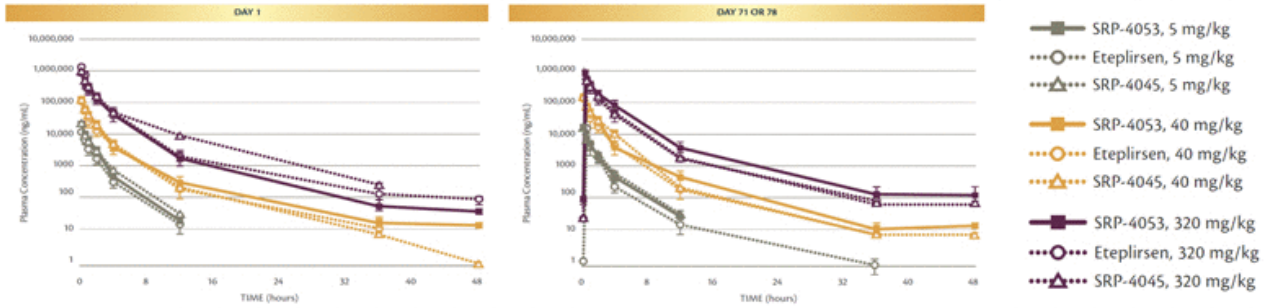
†5 of the 7 cases of proteinuria were determined to be unrelated to treatment

PRECLINICAL SAFETY PROFILES IN NON HUMAN PRIMATES IN FIRST THREE PMO DRUG CANDIDATES

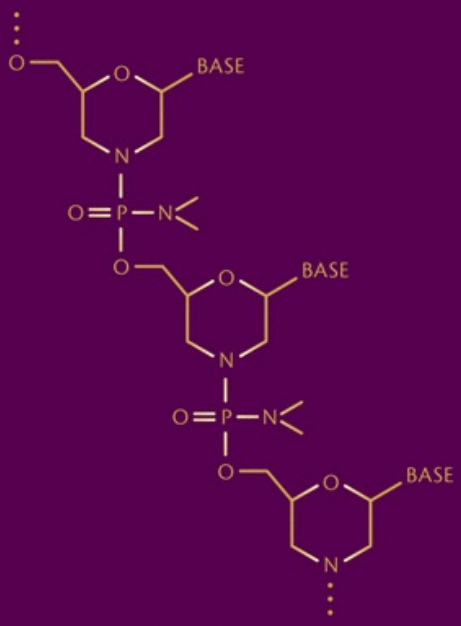
NO ADVERSE EFFECTS IN REPEAT DOSE TOXICITY EVALUATIONS UP TO 320 MG/KG

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



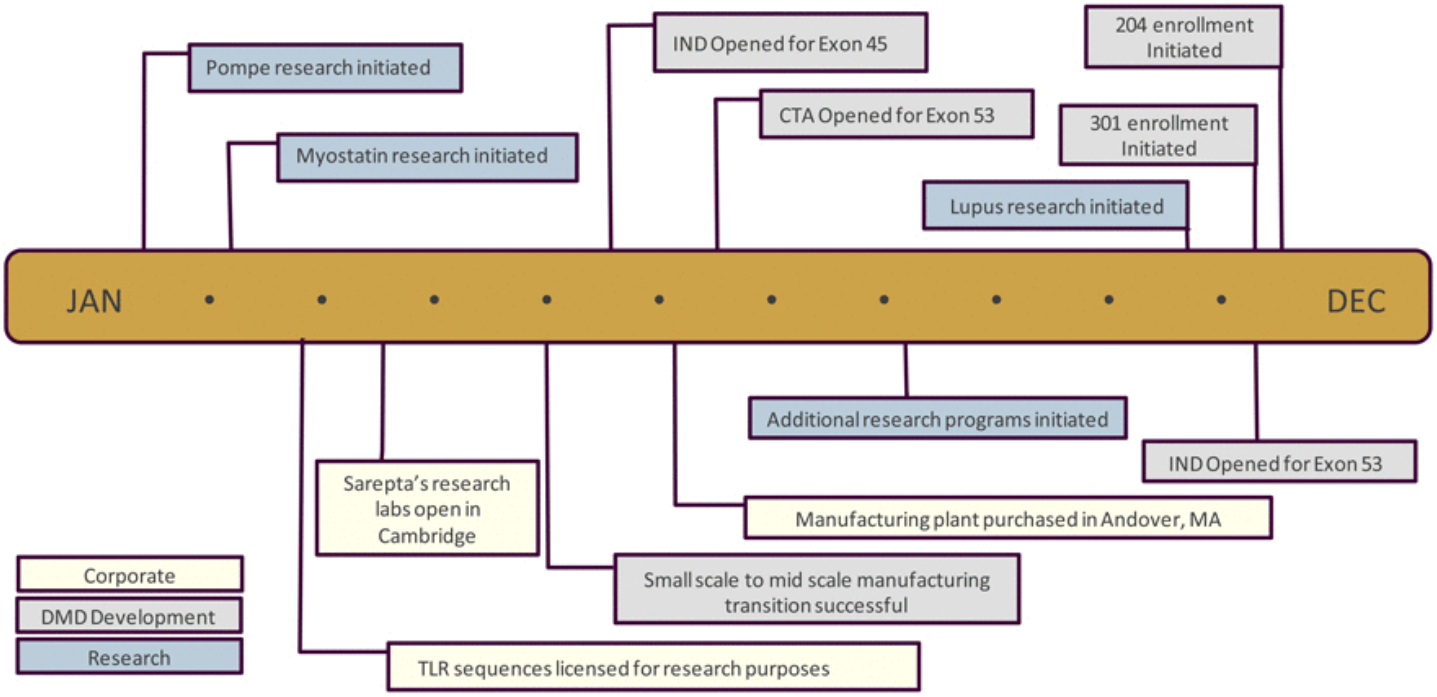
SAREPTA
THERAPEUTICS



RESEARCH PIPELINE

2014 ACCOMPLISHMENTS: PROGRESS TOWARDS BUILDING A FULLY INTEGRATED COMPANY

SETTING A STRONG FOUNDATION TO BUILD UPON IN 2015

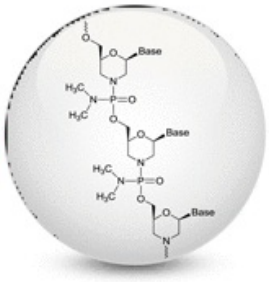


WHAT MAKES ETEPLIRSEN DIFFERENT?

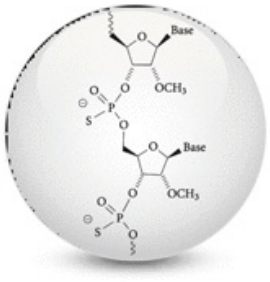
SEQUENCE, CHEMISTRY AND DOSE ARE ALL IMPORTANT FACTORS

DMD EXON-SKIPPING CHEMISTRIES

PHOSPHORODIAMIDATE MORPHOLINO OLIGOMER (PMO)



2'-O-METHYL PHOSPHOROTHIOATE

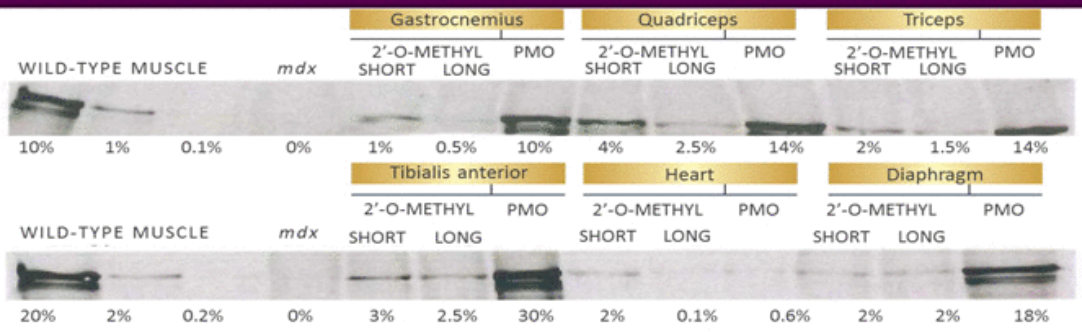


SEQUENCE	Drug-specific optimized sequence	
CHEMISTRY	Charge neutral Plasma half-life of 2-6 hours	Charge negative Plasma half-life of 19-56 days
DOSE	Up to 50 mg/kg intravenous	Up to 9 mg/kg subcutaneous

DIFFERENTIATED CHEMISTRY AND SEQUENCE POTENCY FOR ETEPLIRSEN

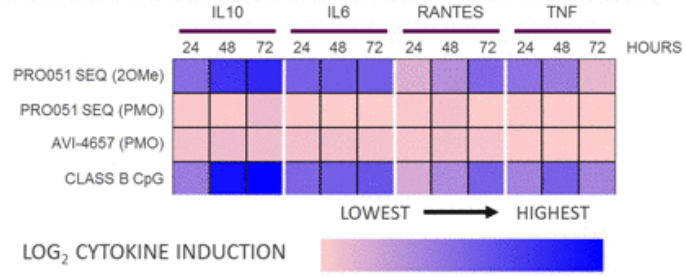
CHEMISTRY, SEQUENCE, AND SAFETY ADVANTAGES IN DMD

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



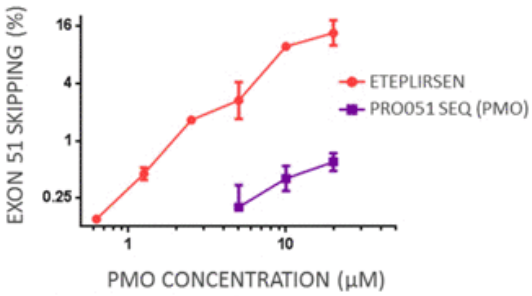
Source: Heemskerk, et al, 2009

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



Source: Sarepta Internal Data

SAREPTA COMPARISON STUDIES OF ETEPLIRSEN SEQUENCE VS PRO051 SEQUENCE SHOWED UP TO 10-FOLD HIGHER EXON-SKIPPING ACTIVITY



Source: Sarepta Internal Data

NUMEROUS CHEMISTRIES CURRENTLY BEING TESTED IN MULTIPLE RESEARCH PROJECTS

POTENTIALLY ENHANCED TISSUE TARGETING, INTRACELLULAR DELIVERY AND DRUG POTENCY

NEXT GENERATION PMO CHEMISTRIES

PMO-X® **PMO*plus*®** **PPMO**

VERSATILITY

PMOs are highly adaptable molecules and, minor modifications, potentially can be rapidly designed to target specific tissues, genetic sequences, or pathogens

SPECIFICITY

PMOs are charge neutral which may limit interactions with proteins in the body other than the target RNA*

STABILITY

PMOs are highly resistant to degradation by enzymes, potentially enabling drug activity*

A UNIVERSE OF POSSIBILITIES WITH PMO-BASED RNA MODULATION

* Hudziak, et al. 1996

PMO-BASED CHEMISTRY PLATFORM CLINICALLY EXPERIENCED

SAREPTA HAS MULTIPLE RESEARCH PROGRAMS IN HIGH VALUE DISEASE STATES

HUMAN SAFETY DATA GENERATED IN 2 CHEMISTRIES (PMO AND PMO*PLUS*®)

- Promising chemistry platform - exon skipping & inclusion approaches in cell, animal, and humans
  - 2 unique chemistries dosed in humans
    - PMO program: >3 years of dosing demonstrate that PMO is safe and well tolerated
    - PMO*plus*: dosed up to 112mg/kg/week with no observed adverse events
      - Ongoing evaluation in multiple research programs
- Opportunity to upregulate or downregulate proteins via exon skipping or exon inclusion

SAREPTA'S PMO PLATFORM HAS DEMONSTRATED:

- Inhibition of mRNA function (Antisense, RNAi)
- Inhibition of miRNA or lncRNA function (Antagomirs)
- Control of mRNA function (Splice switching)
- Alteration of mRNA function (mRNA Re-Engineering)

STRATEGIC AREAS OF RESEARCH FOCUS

SAREPTA FOCUSING ON A REPRODUCIBLE PATH WITH A VISION OF CREATING RNA THERAPEUTICS IN RARE GENETIC, ANTI-INFECTIVE, NEUROMUSCULAR, AND CNS DISEASES

RESEARCH MOVING FORWARD IN 2015

- 7 Research collaborations with universities in Rare Genetic, CNS, NM or Infectious Diseases outside of DMD using PMO, PMO-X or PMO*plus*
- 6 internal research programs ongoing outside of DMD (CNS, NM, RGD)
- In vitro data complete in 5 collaborations, patent applications filed, moving into animal studies
- Multiple opportunities for data generation and research progress in 2015

Rare Genetic Disease

- High unmet needs in focused areas
- Validated using exon skipping/inclusion approach
- IV dosing
- Sarepta or strategic partner to commercialize globally

Neuromuscular/CNS

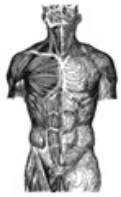
- Augment our DMD pipeline
- Potential for single therapy to address multiple indications
- IV dosing
- Genetically validated targets
- Sarepta or strategic partner to commercialize globally

Anti-Infective

- Multiple indications
- High unmet medical need
- Opportunity to reverse resistance
- IV dosing; inhaled dosing
- Defined opportunity
- Opportunity for a strategic partner

10 NEW RESEARCH PROJECTS STARTED IN 2014

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		

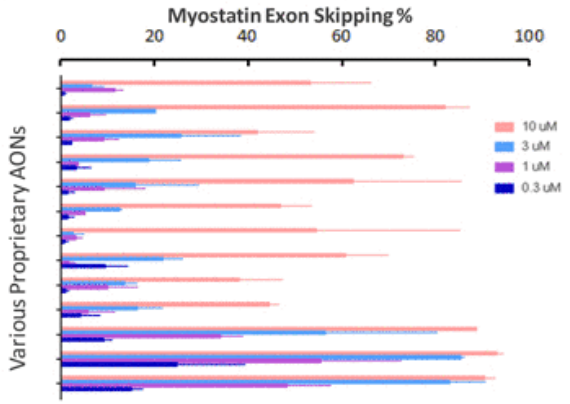
MYOSTATIN INHIBITION

APPROACH PROVIDES SAREPTA MULTIPLE OPPORTUNITIES IN DISEASE STATES OUTSIDE BECKER AND DMD

SPLICE-ALTERING APPROACH UTILIZED TO INHIBIT MYOSTATIN PROTEIN PRODUCTION AT THE mRNA LEVEL

- Inhibition of myostatin at the pre-mRNA level potentially allows increased muscle mass and strength
  - Different MOA utilized than other approaches which had off target effects
  - Research ongoing using PMO*plus*®, PMO and PMO-X®
- PMO's restored mdx weight to normal mouse levels in a 10 week mouse model
- Sarepta PMO's designed to inhibit myostatin lead to increased mobility in mdx mouse model

PMO's IN DEVELOPMENT GENERATING HIGH LEVELS OF EXON SKIPPING IN CELL MODELS



RATIONALE FOR SAREPTA'S APPROACH IN MYOSTATIN INHIBITION

- Sarepta's PMO chemistry has demonstrated affinity to penetrate muscle cells
- Sarepta technology has demonstrated exon and inclusion skipping in cells, animals, and humans
- PMO or PMO*plus*® chemistries provide a shorter regulatory path due to human safety data

SEVERAL POTENTIAL INDICATIONS – HIGH UNMET MEDICAL NEED IN MUSCLE WASTING DISEASES

- Duchenne and Becker Muscular Dystrophy
- Muscle loss caused by ALS, SMA, etc.
- Cachexia caused by Cancer, HIV/AIDS, MS, etc.
- Open to collaborations

ADULT ONSET POMPE DISEASE

POTENTIAL STAND-ALONE OR COMBINATION WITH EXISTING ENZYME REPLACEMENT THERAPIES – RESTORES BODY'S ABILITY TO PRODUCE ACID ALPHA-GLUCOSIDASE

SAREPTA'S TECHNOLOGY SUCCESSFULLY ABLE TO INDUCE EXON INCLUSION AND GENERATE ENZYME ACTIVITY

- Exon inclusion approach restores read-through: up-regulating enzyme production
- Sarepta's technology has demonstrated utility as an exon inclusion approach
- Approach similar to DMD with multiple underlying mutations requiring unique exon-inclusion drugs
- PMO or PMO*plus* chemistries provide a shorter regulatory path due to existing human safety data
- Adult onset Pompe Disease, while rare, is an established market
- Has potential to provide benefit that exceeds currently-marketed enzyme replacement therapy
- Enzyme replacement could restore ability to produce enough alpha-glucosidase to become asymptomatic
- Demonstrated partial restoration of enzyme production compared to no enzyme production in Pompe wild type cells

TOLL-LIKE RECEPTOR (TLR) ANTAGONIST PROGRAM: LICENSED IN 2014

SUPPORTS PIPELINE APPROACH BEYOND RARE DISEASE AND OPEN TO PARTNERSHIPS

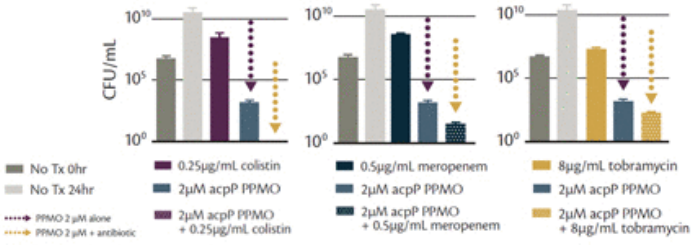
- Sarepta has proprietary rights to AONs that antagonize TLR 7/8/9 with a high degree of activity compared to potentially competing AONs
- Research program initiated in Lupus and GvHD
  - Provides Sarepta targets in multiple other disease states
- TLR program may be applicable in a number of conditions that involve an innate immune response or a Th1-like immune response
- 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
- 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
- IP position established based on novel AONs; long lifecycle potential

PPMO RESTORES ANTIBIOTIC SUSCEPTIBILITY IN RESISTANT STRAINS AND INHIBITS BIOFILM PRODUCTION (2 UNIQUE POC APPROACHES CONFIRMED)

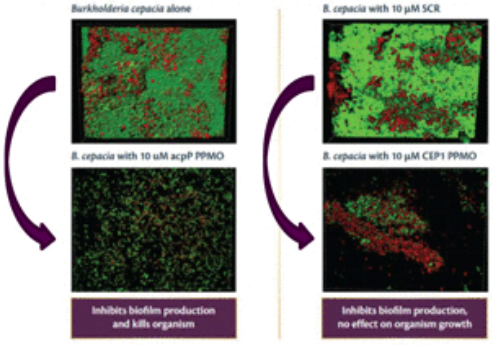
OPPORTUNITY TO CAPITALIZE ON EMERGING ANTIBACTERIAL DATA

- Sarepta is focused on infections of highest medical need and large hospital-based opportunities
  - Six programs identified and underway
- NDM-1 PPMO's can be used in combination with carbapenem to kill carbapenem-resistant bacterial pathogens
  - This is a new strategy to combat pathogens that express NDM-1
- PPMO's that target the cepI and acpP genes of Burkholderia cenocepacia J2315 were able to both prevent biofilm formation and breakdown existing biofilm
  - Broad commercial applicability
  - Opportunities for partnerships

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*

FINANCIAL OVERVIEW

SHARES OUTSTANDING	41.3 million
RECENT CLOSING PRICE	$14.93 as of 1/7/15
TRADING VOLUME	1.5 million shares daily (90 day average volume)
MARKET CAPITALIZATION	~$617 million
CASH & OTHER INVESTMENTS (UNAUDITED)	~$211 million as of 12/31/14


BASE
BASE
BASE
SAREPTA
THERAPEUTICS
THANK YOU