REALIZING THE POTENTIAL OF RNA-BASED TECHNOLOGY

STIFEL HEALTHCARE CONFERENCE 2014

NOVEMBER 19, 2014
FORWARD LOOKING STATEMENTS

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

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

Any of the foregoing risks could materially and adversely affect Sarepta’s business, results of operations and the trading price of Sarepta’s common stock. We caution investors not to place considerable reliance on the forward-looking statements contained in this presentation. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.
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 3,500 boys worldwide
- 25,000-30,000 patients in the U.S. and Europe
- Sarepta’s lead program (Exon 51) estimated to benefit ~13% of DMD patients
- Follow-on Exon-Skipping Drugs have potential to treat 60-80% of DMD patients

Relentless Progression of DMD

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

Dosing Initiated in Eteplirsen Studies

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

FDA Feedback Received on Dystrophin Reassessment Protocol

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

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

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

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

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

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

<table>
<thead>
<tr>
<th>Study</th>
<th>Duration (weeks)</th>
<th>n</th>
<th>Status</th>
<th>Exon Target Treatment</th>
<th>DMD Population</th>
<th>1° Outcome Measure</th>
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<tbody>
<tr>
<td>4658-uk-33</td>
<td>Single Dose</td>
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<td>Complete</td>
<td>Exon 51</td>
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<td>Safety; Dystrophin</td>
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<td>4658-uk-28</td>
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<td>19</td>
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<td>Exon 51</td>
<td>5-15 yrs, amb</td>
<td>Safety; Dystrophin</td>
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<tr>
<td>4658-us-201</td>
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<td>12</td>
<td>Complete</td>
<td>Exon 51</td>
<td>7-13 yrs, amb</td>
<td>Safety; Dystrophin</td>
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<td>Ongoing</td>
<td>Exon 51</td>
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<td>6MWT; Dystrophin</td>
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<tr>
<td>4658-us-301</td>
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<td>120</td>
<td>Dosing: Active Enrollment</td>
<td>Exon 51</td>
<td>7-16 yrs, amb</td>
<td>6MWT; Dystrophin</td>
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<tr>
<td>4658-us-204</td>
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<td>20</td>
<td>Dosing: Active Enrollment</td>
<td>Exon 51</td>
<td>7-21 yrs, non-amb</td>
<td>Safety</td>
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<tr>
<td>4658-us-203</td>
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<td>20</td>
<td>Final Protocol: Dosing 1Q2015</td>
<td>Exon 51</td>
<td>4-6 yrs, amb</td>
<td>Safety; Dystrophin</td>
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<td>4045-us-301</td>
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<td>90</td>
<td>FDA Agreement on Master Protocol</td>
<td>Exon 45 and Exon 53</td>
<td>7-16 yrs, amb</td>
<td>6MWT; Dystrophin</td>
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<tr>
<td>4053-eu-101</td>
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<td>36</td>
<td>Enrolling; Dosing 1Q2015</td>
<td>Exon 53</td>
<td>7-16 yrs, amb</td>
<td>6MWT, Dystrophin</td>
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<td>4045-eu-301</td>
<td>TBD</td>
<td>TBD</td>
<td>TBD</td>
<td>Exon 45 and Exon 53</td>
<td>7-16 yrs, amb</td>
<td>6MWT; Dystrophin</td>
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ETEPLIRSEN PHASE IIb STUDY DESIGN

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

Study 201: Double-blinded, Placebo-controlled Phase IIb

- 30 mg/kg/wk
  - n=4
- 50 mg/kg/wk
  - n=4
- Placebo
  - n=4

Study 202: Open-label, Long-term Safety and Efficacy Study

- 30 mg/kg/wk
  - n=4
- 50 mg/kg/wk
  - n=2

*Placebo-controlled group rolled over onto open-label eteplirsen.

24 weeks

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
- % Dystrophin positive fibers
- Pulmonary function tests
- 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**

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

* 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

**Eteplirsen Treatment resulted in:**

<table>
<thead>
<tr>
<th></th>
<th>Study 33 (N=7)</th>
<th>Study 28 (N=17)</th>
<th>Study 201 (N=12)</th>
<th>Study 202 (N=12)</th>
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<tbody>
<tr>
<td>Exon 51 skipping</td>
<td>✔️</td>
<td>✔️</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>Novel dystrophin</td>
<td>✔️</td>
<td>✔️</td>
<td>✔️</td>
<td>✔️</td>
</tr>
</tbody>
</table>


*Results based on 40x magnification of image using BioQuant Software*
OBJECTIVE MEASURE OF DYSTROPHIN INTENSITY INCREASED WITH ETËPLIRSEN TREATMENT IN ALL PATIENTS

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

*Results based on 40x magnification of image using BioQuant Software; No significant increase using 20x magnification, which was only used at BL, 12 and 24 wks.
SIX MINUTE WALK TEST (6MWT) IS A WELL ESTABLISHED OUTCOME MEASURE

DMD SHOWS PROGRESSIVE DECLINE IN PATIENTS OLDER THAN 7 YEARS

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

“Those above the age of 7 years showed a progressive deterioration that was much more marked with each increasing year after baseline”

“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”

INCLUDED AS FUNCTIONAL ASSESSMENT IN MANY APPROVED DRUGS

Included as a functional assessment in multiple clinical trials
- 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

Note: Aldurazyme and Myozyme are registered trademarks of Biomarin/Genzyme LLC. Elaprase is a registered trademark of Shire Human Genetic Therapies, Inc.
6MWT CHANGE FROM BASELINE TO WEEK 144 IN STUDY 201/202
DATA BASED ON MAXIMUM 6MWT SCORE WHEN TEST WAS REPEATED

• 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

Note: Statistical analysis based on modified Intent-To-Treat (mITT, n=10, excludes two patients who experienced rapid decline and lost ambulation early in the study) Population using MMRM Test
6MWT OUTCOMES STRATIFIED BY DISTANCE (<350m v. >350m) AT 36 WEEKS

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

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

Note: Mean age: 9.9 years at week 36; 12.0 years at Week 144; Includes modified Intent-to-Treat (mITT) Population
*n=4 at week 84 due to a patient recovering from a broken ankle who was unable to participate at this time point
SUBJECTS ON ETEPLIRSEN COMPARED TO PUBLISHED NATURAL HISTORY* (N=12 AT WEEK 144)

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

*Henricson, et al. 2013
PULMONARY 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

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 H₂O | 72.4 cm H₂O | +14.7%
MEP | 68.1 cm H₂O | 76.8 cm H₂O | +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%

* Wilson et al. 1984 equations

**Age at Baseline (yrs):**
- Mean: 9.3
- Median: 9.7

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

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**Sarepta Therapeutics**
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

<table>
<thead>
<tr>
<th>TREATMENT-EMERGENT ADVERSE EVENT</th>
<th>PLACEBO FOR 24 WKS N=4 (%)</th>
<th>ETEPLIRSEN FOR 24 WKS N=8 (%)</th>
<th>ETEPLIRSEN FOR 120 WKS N=4 (%)</th>
<th>ETEPLIRSEN FOR 144 WKS N=8 (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Procedural pain</td>
<td>3 (75)</td>
<td>4 (50)</td>
<td>1 (25)</td>
<td>6 (75)</td>
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<tr>
<td>Vomiting</td>
<td>0</td>
<td>3 (38)</td>
<td>2 (50)</td>
<td>4 (50)</td>
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<tr>
<td>Hypokalaemia</td>
<td>2 (50)</td>
<td>4 (50)</td>
<td>0</td>
<td>4 (50)</td>
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<tr>
<td>Cough</td>
<td>2 (50)</td>
<td>2 (25)</td>
<td>1 (25)</td>
<td>3 (38)</td>
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<tr>
<td>Back pain</td>
<td>2 (50)</td>
<td>1 (12)</td>
<td>1 (25)</td>
<td>4 (50)</td>
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<td>Fall</td>
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<td>Muscle Spasms</td>
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<td>2 (50)</td>
<td>1 (12)</td>
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<tr>
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<td>0</td>
<td>1 (25)</td>
<td>5 (62)</td>
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<tr>
<td>Injection Site Pain</td>
<td>0</td>
<td>1 (12)</td>
<td>0</td>
<td>1 (12)</td>
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</table>

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

†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

<table>
<thead>
<tr>
<th>CLINICAL PROGRAMS</th>
<th>DISCOVERY</th>
<th>PRE-CLINICAL</th>
<th>CLINICAL DEVELOPMENT</th>
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<tr>
<td>DMD</td>
<td>Eteplirsen (AVI-4658)</td>
<td>SRP-4053</td>
<td>SRP-4050</td>
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</tbody>
</table>


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.
THREE PMO DRUG CANDIDATES DEMONSTRATED REPRODUCIBLE PRECLINICAL SAFETY PROFILES
NO ADVERSE EFFECTS IN REPEAT DOSE TOXICITY EVALUATIONS UP TO 320 MG/KG

<table>
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<tr>
<th>STUDY</th>
<th>DRUG</th>
<th>SPECIES</th>
<th>ROUTE/DOSE</th>
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<th>RESULTS</th>
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<td>N/A</td>
<td>N/A</td>
<td>Negative: mutagenic potential, induction of chromosomal aberrations, induction of micronuclei</td>
</tr>
<tr>
<td></td>
<td>SRP-4045</td>
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<td></td>
<td>SRP-4053</td>
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<td></td>
</tr>
<tr>
<td>SAFETY PHARMACOLOGY</td>
<td>Eteplirsen</td>
<td>Cynomolgus Monkey</td>
<td>IV, SC : 0, 40, 160, 320 mg/kg</td>
<td>6</td>
<td>No biologically relevant findings on vital signs, CNS, or cardiopulmonary activity</td>
</tr>
<tr>
<td></td>
<td>SRP-4045</td>
<td>Cynomolgus Monkey</td>
<td>IV: 0, 40, 160, 320 mg/kg</td>
<td>4</td>
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</tr>
<tr>
<td></td>
<td>SRP-4053</td>
<td>Cynomolgus Monkey</td>
<td>IV: 0, 40, 160, 320 mg/kg</td>
<td>4</td>
<td></td>
</tr>
<tr>
<td>REPEAT DOSE TOXICITY</td>
<td>Eteplirsen</td>
<td>Cynomolgus Monkey</td>
<td>IV weekly: 0, 5, 40, 320 mg/kg</td>
<td>6</td>
<td>NOAEL= MFD; 320 mg/kg</td>
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<tr>
<td></td>
<td>SRP-4045</td>
<td>Cynomolgus Monkey</td>
<td>IV weekly for 12 weeks: 0, 5, 40, 320 mg/kg</td>
<td>9</td>
<td>NOAEL= MFD; 320 mg/kg</td>
</tr>
<tr>
<td></td>
<td>SRP-4053</td>
<td>Cynomolgus Monkey</td>
<td>IV weekly: 0, 5, 40, 320 mg/kg</td>
<td>9</td>
<td>NOAEL= MFD; 320 mg/kg</td>
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</tbody>
</table>

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.

---

Top 20 Single Exons To Be Skipped

Currently Infeasible to Power Clinical Studies on 6MWT

PIPELINE BEYOND DMD
NEXT GENERATION PMO-BASED CHEMISTRIES
ENHANCED TISSUE TARGETING, INTRACELLULAR DELIVERY AND DRUG POTENCY

IP PROTECTED NEXT GENERATION PMO CHEMISTRIES

**PMOplus®**
- 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
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
- PMOplus® 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 PMOplus® 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 PMOplus®
- 3 Research collaborations utilizing CROs focused on rare disease outside DMD using PMOplus®, 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
**PMOPLUS® EFFICACY AGAINST EBOLA: POTENT ANTIVIRAL EFFECT AND IMPROVED SURVIVAL IN NHPs**

DEMONSTRATION OF VIRAL LOAD SUPPRESSION AND SURVIVAL BENEFIT\(^1,2,3\)

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

\(^1\)Iversen, TIDES Las Vegas, NV; \(^2\)Iversen, Viruses 2012; \(^3\)Wong OTS 2014; Internal Sarepta data

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AVI-6002 is a combination of AVI-7537 & AVI-7539

AVI-7537 is demonstrated to be active component

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\[^1\]Iversen, TIDES Las Vegas, NV; \[^2\]Iversen, Viruses 2012; \[^3\]Wong OTS 2014; Internal Sarepta data
AVI-7537 PREPAREDNESS: NHP EFFICACY AND HUMAN SAFETY DEMONSTRATED, DRUG SUPPLY AVAILABLE

NHP survival against Ebola lethal challenge\(^1,2\)

- Targets Ebola VP24 –well conserved; no base pair mismatches compared to reported clinical isolates from 2014 outbreak
- 5 studies, 67 Rhesus macaques infected with 1000 pfu (highly lethal dose) Ebola-Zaire
- 73.5% (17/25) overall survival (range 60-80%) of NHPs receiving 20-40 mg/kg/day \(\times 14\) days
- 100% (2/2) of surviving monkeys re-challenged at day 50 survived without retreatment
- 0/27 (0%) controls survived – controls died by day 11 post-infection, median mortality by day 8

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

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

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

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

\(^1\)Iversen, TIDES Las Vegas, NV; \(^2\)Wong, OTS 2014 San Diego; \(^3\)Clinical Study Report, 6002-us-101, NCT01353027; \(^4\)AVI-7288 for Marburg has same backbone chemistry as AVI-7537; \(^5\)Estimated human efficacious dose = 10 mg/kg/day, Heald A et al. ECCMID, 2014 Barcelona
ANTIBIOTIC-RESISTANT BACTERIAL INFECTIONS ARE A SERIOUS GLOBAL HEALTH CONCERN

MILLIONS OF RESISTANT INFECTIONS AND ~50,000 DEATHS EACH YEAR ACROSS US AND EU\(^1,2\)

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

Colistin, Meropenem, and Tobramycin susceptibility restored with co-administration of PPMO in \textit{A. Baumannii}\(^3\)

PPMO inhibits bacterial biofilm production and kills organism\(^4\)

Red: \textit{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
<table>
<thead>
<tr>
<th>R&amp;D FOCUSED ON HIGH-VALUE TARGETS</th>
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</thead>
<tbody>
<tr>
<td>SAREPTA IS MAKING CONSIDERABLE PROGRESS APPLYING PMO TECHNOLOGY IN ADDITIONAL THERAPEUTIC AREAS</td>
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<table>
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<tr>
<th>CLINICAL PROGRAMS</th>
<th>DISCOVERY</th>
<th>PRE-CLINICAL</th>
<th>CLINICAL</th>
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<tr>
<td>Rare Diseases</td>
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<tr>
<td>DMD Exon 51</td>
<td>Eteplirsen (AVI-4658)</td>
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<td>DMD Exon 53</td>
<td>SRP-4053</td>
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<td>DMD &amp; Becker MD</td>
<td>Myostatin Inhibition</td>
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<td>Progeria</td>
<td>Progerin</td>
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<td>Adult Onset Pompe Disease</td>
<td>Alpha-glucosidase</td>
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<td>Lupus &amp; Graft vs. Host Disease</td>
<td>Toll Like Receptors (TLR)</td>
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<td>Anti-Infective</td>
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<td>Marburg Virus</td>
<td>AVI-7288</td>
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<td>Ebola Virus</td>
<td>AVI-7537</td>
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<td>Influenza</td>
<td>AVI-7100</td>
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<td>Drug-Resistant Bacteria</td>
<td>Burkholderia Cepacia</td>
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<td>Pseudomonas Aeruginosa</td>
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<td>Klebsiella pneumoniae</td>
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<td>Acinetobacter baumannii</td>
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<tr>
<td>Drug-Resistant Bacteria</td>
<td>Neisseria gonorrhoea</td>
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## FINANCIAL OVERVIEW

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