EXON SKIPPING PROGRAM UPDATE FOR THE DUCHENNE MUSCULAR DYSTROPHY COMMUNITY

with

CURE DUCHENNE
DUCHENNE ALLIANCE
MUSCULAR DYSTROPHY ASSOCIATION (MDA)
PARENT PROJECT MUSCULAR DYSTROPHY (PPMD)
ACTION DUCHENNE, JOINING JACK AND HARRISON’S FUND
UNITED PARENT PROJECTS MUSCULAR DYSTROPHY

MAY 13, 2014
This presentation contains forward-looking statements. These forward-looking statements generally can be identified by the use of words such as “believes or belief,” “anticipates,” “plans,” “expects,” “will,” “intends,” “potential,” “possible,” “advance” and similar expressions. These forward-looking statements include statements about the timing of an NDA submission for eteplirsen in the treatment of DMD; the potential filing and acceptance of an NDA for eteplirsen by the FDA; the timing and submission of additional data, analysis and other information to the FDA necessary for the FDA to make regulatory determinations; the timing and design of and ability to initiate additional studies for eteplirsen and other follow-on exons; the potential regulatory approval of eteplirsen on an accelerated pathway; and our ability to establish and protect intellectual property rights and 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 be able to comply with all FDA requests; 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 and new clinical trials may not be positive; 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 supply for clinical trials or commercialization; Agency or court decisions with respect to our patents may negatively impact our business; and those identified under the heading “Risk Factors” in Sarepta’s Annual Report on Form 10-K for the year ended December 31, 2013 and Quarterly Report on Form 10-Q for the quarter ended March 31, 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.
OUR GOALS FOR TODAY

• Discuss recent FDA guidance regarding eteplirsen and follow-on exon skipping therapies

• Review critical next steps for eteplirsen and DMD development program:
  – Planned submission of New Drug Application to U.S. FDA by year end
  – Expanded clinical development program including 7 new studies

• Review additional resources:
  – Let’s Skip Ahead, an online resource center for the DMD community from Sarepta
  – Decode Duchenne, a genetic testing program administered by PPMD and supported by Sarepta

• Question and answer session with the DMD Advocacy Organizations
SUMMARY OF RECENT FDA FEEDBACK

SAREPTA PLANS TO SUBMIT A NEW DRUG APPLICATION FOR ETEPLIRSEN BY YEAR END

1. POTENTIAL ETEPLIRSEN ACCELERATED APPROVAL PATHWAY
   • An accelerated approval for eteplirsen could be considered using dystrophin or the 6-minute walk test
   • FDA identified additional safety and efficacy data that, if positive, may increase the chance of a successful New Drug Application filing decision by the FDA

2. ETEPLIRSEN CONFIRMATORY STUDY REQUIRED FOR ACCELERATED APPROVAL
   • If an accelerated approval is granted, Sarepta will be required to confirm the safety and efficacy of eteplirsen with positive data from one of the following studies:
     – Open-label historically controlled clinical study of eteplirsen (no placebo)
     – Placebo-controlled study of one or more follow-on exon skipping therapies

3. EXON SKIPPING PIPELINE
   • FDA provided initial guidance on the development of follow-on exon skipping therapies
**KEY REGULATORY ACTIVITIES AND POTENTIAL TIMELINE**

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<tbody>
<tr>
<td></td>
<td>Collaboration with FDA on dystrophin quantification methods</td>
<td>NDA Submission</td>
<td>NDA Filing</td>
<td>Advisory Committee</td>
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<td>Sarepta to seek scientific advice from European Medicines Agency</td>
<td>144wk data</td>
<td>Possible 4th biopsy</td>
<td>168wk data</td>
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</tbody>
</table>

*U.S. regulatory timeline and potential outcomes are for illustration only and assume additional positive safety and efficacy information, an eteplirsen NDA is accepted by FDA for filing within 60 days of submission, and the FDA grants a 6-month priority review*
TYPICAL NDA SUBMISSION AND REVIEW PROCESS

- **NDA submitted by sponsor**: 60 days
- **NDA accepted for review by FDA**: Formal review begins upon acceptance of NDA
- **Day 74 letter**
- **Advisory committee**: FDA solicits non-binding recommendations from experts
- **Approval decision**: 6 months (priority) or 10 months (standard)
### POTENTIAL TIMELINE FOR ETEPLIRSENN AND FOLLOW-ON EXON CLINICAL STUDIES

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<td></td>
<td><strong>ETEPLIRSENN STUDIES (EXON 51)</strong></td>
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<td>144wk data</td>
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<td>168wk data</td>
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<td>Ongoing Phase IIb extension study</td>
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<td>Open-label historically controlled confirmatory study in ambulatory patients</td>
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<td>Open-label study in patients 4-6 years old</td>
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<td>Open-label study in patients with limited or no walking ability</td>
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<td></td>
<td><strong>FOLLOW-ON EXON STUDIES (EXONS 53 &amp; 45)</strong></td>
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<td>Open-label study of SRP-4053</td>
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<td>Placebo-controlled study of SRP-4045 and SRP-4053</td>
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<td><strong>NATURAL HISTORY STUDY</strong></td>
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<td>Natural history study (timing TBD)</td>
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### Clinical Milestones

- **CRITICAL STUDY START-UP ACTIVITIES**
WHAT IS GENERALLY INVOLVED IN STARTING A CLINICAL STUDY?

MULTI-STEP PROCESS NOW UNDERWAY TO INITIATE NEW EXON SKIPPING STUDIES

CURRENTLY IN PROCESS

• Develop a study protocol (details how the study will be conducted)
• Identify clinical sites who will participate
• Select a clinical research organization to help conduct the study
• Obtain approval from the FDA and clinical site institutional review boards
• Train investigators and clinical site staff on study procedures
• Prepare and release drug supply

PLANNED

• Begin patient screening and informed consent process
WHAT DOES PARTICIPATING IN A CLINICAL TRIAL TYPICALLY INVOLVE FOR PATIENTS AND FAMILIES?

Contact clinical site
- Clinical sites will be posted on ClinicalTrials.gov and Let’s Skip Ahead once available
- Families can reach out directly to a site to obtain more information

Informed consent
- Patients and families review the potential risks and benefits of participation with their clinical investigator

Screening visit
- The clinical investigator determines the patient’s eligibility to participate in the study

Study enrollment
- If all eligibility criteria are met, the patient starts participation in the study

On study
- Required study assessments are conducted at regional centers
- Patients may receive weekly infusions at a local infusion site
## SUMMARY OF ETEPLIRSEN STUDIES

### 3 NEW TRIALS PLANNED TO BEGIN IN 2014 IN BROADER DMD PATIENT POPULATION

<table>
<thead>
<tr>
<th>Study</th>
<th>Major Eligibility Criteria*</th>
<th>Key Endpoints</th>
<th>Clinical Sites</th>
<th>Timeline</th>
</tr>
</thead>
</table>
| Open-label historically controlled confirmatory study | • Boys aged 7-16 years  
• Able to walk a minimum distance  
• Deletion mutation amenable to exon 51 skipping (treated cohort)  
• Deletion mutation not amenable to exon 51 skipping (untreated cohort)  
• Stable corticosteroid regimen | • 6-minute walk test  
• Dystrophin  
• Safety  
• Other measures | • United States | 3Q 2014 |
| Open-label study in patients less than 7 years old | • Boys aged 4-6 years  
• Deletion mutation amenable to exon 51 skipping | • Dystrophin  
• Safety  
• Other measures | • United States | 4Q 2014 |
| Open-label study in older patients not able to walk minimum distance | • Boys aged 20 years or less  
• Unable to walk a minimum distance or non-ambulatory  
• Deletion mutation amenable to exon 51 skipping | • Safety  
• Other measures | • United States | 4Q 2014 |

*Additional study eligibility criteria to be posted on ClinicalTrials.gov and Let’s Skip Ahead online resource center.
# SUMMARY OF FOLLOW-ON EXON STUDIES

2 NEW CLINICAL TRIALS PLANNED TO BEGIN IN 2014 / EARLY 2015

<table>
<thead>
<tr>
<th>Study</th>
<th>Major Eligibility Criteria*</th>
<th>Key Endpoints</th>
<th>Clinical Sites</th>
<th>Timeline</th>
</tr>
</thead>
</table>
| Open-label study of SRP-4053 | • Boys aged 6-15 years  
• Able to walk a minimum distance  
• Deletion mutation amenable to exon 53 skipping  
• Stable corticosteroid regimen | • 6-minute walk test  
• Dystrophin  
• Safety  
• Other measures | • United Kingdom  
• France  
• Italy | 3Q 2014 |
| Placebo-controlled study of SRP-4045 and/or SRP-4053 [Design to be confirmed with FDA] | • Boys aged 7-16 years  
• Able to walk a minimum distance  
• Deletion mutation amenable to exon 53 or exon 45 skipping  
• Stable corticosteroid regimen | • 6-minute walk test  
• Dystrophin  
• Safety  
• Other measures | • North America  
• Europe | 4Q 2014 / 1Q 2015 |

*Additional study eligibility criteria to be posted on ClinicalTrials.gov and Let’s Skip Ahead online resource center
MULTIPLE STUDIES TO BEGIN ENROLLMENT SOON
OPPORTUNITY FOR BROAD POPULATION OF DMD PATIENTS TO PARTICIPATE

• Patients previously treated with drisapersen may be eligible to participate in eteplirsen clinical studies
  – Required minimum 24-week (6 month) wash-out period

• Concurrent participation in other clinical studies of investigational drugs for DMD is not permitted

• Opportunity for patients with multiple genotypes to participate

Clinical site selection is currently underway...

Sign up on Let’s Skip Ahead to provide information that may help us identify and select clinical site locations for upcoming studies.
## SAREPTA EXON SKIPPING PLATFORM FOR DMD

### LEAD CANDIDATES PROGRESSING TO CLINICAL TRIALS

<table>
<thead>
<tr>
<th>Target</th>
<th>Discovery</th>
<th>Preclinical</th>
<th>Clinical</th>
<th>Status</th>
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<tbody>
<tr>
<td>Eteplirsen</td>
<td></td>
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<td>120-week data reported, NDA submission targeted by end of year 2014,</td>
<td>Additional studies expected to be initiated in 2014</td>
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<tr>
<td>(Exon 51)</td>
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<tr>
<td>Exon 45</td>
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<td>Pre-IND guidance received, IND submission planned in Q3, Study expected</td>
<td>Study expected to be initiated in 2014/2015</td>
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<tr>
<td>Exon 53</td>
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<td>Pre-IND guidance received, IND submission planned in Q3, Two studies</td>
<td>Two studies expected to be initiated in 2014/2015</td>
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<tr>
<td>Exon 50</td>
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<td>Lead sequence identified</td>
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<td>Exon 44</td>
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<td>Lead sequence selection underway</td>
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<td>Exon 52</td>
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<td>Lead sequence selection underway</td>
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<td>Exon 55</td>
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<td>Lead sequence selection underway</td>
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<tr>
<td>Exon 8</td>
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<td>Lead sequence selection underway</td>
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SAREPTA EXON SKIPPING PLATFORM FOR DMD

ABOUT HALF OF DMD POPULATION IS ADDRESSABLE WITH 8 DRUG CANDIDATES

LET’S SKIP AHEAD ONLINE RESOURCE CENTER

STUDY INFORMATION TO BE POSTED ON WEBSITE ONCE AVAILABLE

- An online resource center for patients with DMD, their families and healthcare providers
  - Information and resources on exon skipping, genetic testing and clinical trials
- An opportunity to sign up for updates about upcoming clinical trials and other important information from Sarepta
- Visit www.SkipAhead.com to sign up
“DECODE DUCHENNE” PROGRAM NOW AVAILABLE

PPMD AND SAREPTA JOIN FORCES TO IMPROVE ACCESS TO GENETIC TESTING

• Program offers genetic testing at no cost to eligible patients in the United States who are unable to access testing due to barriers such as cost or lack of insurance coverage

• Contact the DuchenneConnect coordinator to learn about eligibility criteria
  – Email: coordinator@duchenneconnect.org
  – Phone: (201) 937-1408
  – Website: www.DuchenneConnect.org

• Administered by PPMD and DuchenneConnect; supported by Sarepta
INNOVATIVE DRUG DEVELOPMENT REQUIRES COLLABORATION
Thank you to all of the patients and families involved in our exon skipping clinical studies

ETEPLIRSEN PHASE IIB STUDY GROUP

• Jerry Mendell, Principal Investigator
• Hoda Abdel Hamid
• Barry J. Byrne
• Anne M. Connolly
• Robert A. Dracker
• L. Matthew Frank
• Peter T. Heydemann
• Fawn Lee
• Keven C. O’Brien
• Susan E. Sparks
• Linda A. Specht
• Nationwide Children’s Hospital neuromuscular clinic team and research lab

SKIP-NMD COLLABORATION (EU EXON 53 STUDY)

• Francesco Muntoni, Principal Investigator
• George Dickson
• Raymond Gilles
• Edward Kaye
• Earnest Larnach
• Eugenio Mercuri
• Volker Straub
• William van’t Hoff
• David Vissière
• Thomas Voit
QUESTIONS AND ANSWERS

For additional questions, please contact us:

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  – SkipAhead@sarepta.com

• Phone (toll free)
  – 1-855-DMD-SKIP (855-363-7547)