Sarepta is dedicated to helping DMD patients and their families, and we are committed to working as fast as possible to move our eteplirsen and follow-on DMD programs forward. We know the patient community is interested in our regulatory and clinical progress, so we have outlined our latest developments below. We have multiple studies that we plan to enroll soon, and we remain steadfast in our efforts to address this devastating disease.

Regulatory Progress

- We are on track to submit a New Drug Application (NDA) for eteplirsen by the end of the year.
- We have continued to have productive dialogue with the FDA regarding our dystrophin methodology. The agency recently completed a site visit with Nationwide Children’s Hospital in Columbus, Ohio where dystrophin analysis and quantification were conducted for our eteplirsen Phase IIb study.
- We plan to conduct a 4th biopsy in patients in our ongoing Phase IIb extension study for eteplirsen and believe more than half of the patients will agree to participate. These data will be submitted for consideration in connection with an NDA.
- We successfully submitted the investigational new drug (IND) application for our Exon 45-skipping drug candidate, SRP-4045, and currently have an open IND for this candidate to conduct a dosing study.
- We anticipate submitting an IND for our Exon 53-skipping candidate, SRP-4053, later this year.
- We plan to meet with the FDA later this year to discuss the design of our trial with SRP-4045 and SRP-4053, including potential use of a single Master Protocol, which may allow for speedier enrollment and faster access to our drug candidates by combining multiple exon-skipping drugs, that use the same chemistry, same standard dose, and show similar pharmacokinetics and safety, as part of a larger study.
- In Europe, we have submitted our Clinical Trial Application for SRP-4053 to the EMA and plan to initiate dosing in our EU dose-escalation study in the coming months.
- We plan to have our first guidance meeting with the EMA later this year on the feasibility of expedited approval of eteplirsen, such as under the conditional approval mechanism.
Clinical Progress

- **Eteplirsen Confirmatory Study (Open-label, historically controlled)**
  - **Major eligibility criteria:** Boys aged 7-16 yrs; 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
  - **Key endpoints:** 6MWT; pulmonary function tests; dystrophin; safety; other measures
  - **Target enrollment:** 60-80 patients (treated cohort)
  - **Clinical Sites:** United States
  - **Update:** The final protocol for this study was reviewed by the FDA in early June and has been sent to the clinical trial sites, along with informed consent documentation and contracts. Right now, the final protocol is making its way through either local, institutional IRBs or central IRBs depending on each site’s standard operating procedures. In total, 39 sites have been approached for potential participation, with as many as 14 regional hub sites, which will conduct clinical outcome measures, including 6MWT and pulmonary function tests. We hope this approach of having many more infusion sites for dosing on a weekly basis will minimize the burden on families. Clinical outcome measures will be obtained every 12 weeks for the first year.

- **Eteplirsen Study in Patients with Limited Ambulation (Open-label)**
  - **Major eligibility criteria:** Boys up to 21 yrs; unable to walk a minimum distance or non-ambulatory; deletion mutation amenable to exon 51 skipping
  - **Key endpoints:** safety; pulmonary function tests; other measures
  - **Target enrollment:** 20 patients
  - **Clinical Sites:** United States
  - **Update:** The protocol for this study has been submitted to the FDA. We have received no comments to date after a more than 30-day review period, so we expect to get sites up and running in order to start dosing in the November timeframe.

- **Eteplirsen Study in Younger Patients (Open-label)**
  - **Major eligibility criteria:** Boys aged 4-6 yrs; deletion mutation amenable to exon 51 skipping
  - **Key endpoints:** dystrophin; safety; other measures
  - **Target enrollment:** 20 patients
  - **Clinical Sites:** United States
  - **Update:** We have finalized the protocol for this study and plan to submit it soon to the FDA for review.

- **Exon 53 Skipping (SRP-4053) Study (Open-label, Preceded by 12 wk Placebo-Controlled Dose Titration)**
  - **Major eligibility criteria:** Boys aged 6-15 yrs; able to walk a minimum distance; deletion mutation amenable to exon 53 skipping (treated cohort); deletion mutation not amenable to exon 53 skipping (untreated cohort); stable corticosteroid regimen
Key endpoints: 6MWT; pulmonary function tests; dystrophin; safety; other measures

Target enrollment: 24 patients (treated cohort)

Clinical Sites: UK; France; Italy

Update: We expect this study, which is part of a European Commission FP7 Innovation Grant, to include 4 sites in Europe, including London, Newcastle, Paris and Rome. We have submitted clinical trial applications to all of the countries and are in the process of Ethics Committee reviews. It is expected that enrollment will begin in September, with dosing to begin in October or November.

Exon 45 Skipping (SRP-4045) and Exon 53 Skipping (SRP-4053) Study (Placebo-Controlled)

Update: We are preparing to meet with the FDA later this year on a combined SRP-4045 and SRP-4053 protocol, and expect to get feedback in the fourth quarter of this year, in time to initiate this study in the early part of next year.

Ongoing Eteplirsen Phase IIb extension study – 144-week Data

We continue to be encouraged, after nearly 3 years of follow-up, by the performance of the 12 boys in the ongoing eteplirsen Phase IIb open-label extension study, all of whom are showing unexpected outcomes in comparison to the natural history of DMD.

- Results on the 6MWT showed a decline in walking ability at a rate slower than would be expected based on available DMD natural history data.
- We continue to observe remarkable stabilization of respiratory muscle function as assessed by pulmonary function tests.
- Eteplirsen continues to be well-tolerated, with no reported clinically significant treatment-related adverse events, treatment-related serious adverse events, or treatment-related hospitalizations or discontinuations.

Updates on our clinical programs and other developments will be accessible through Let’s Skip Ahead by visiting www.skipahead.com.

If you have any questions, please email skipahead@sarepta.com or contact Kara Boniface at 617-274-3919.

Forward Looking-Statements:

This patient community update contains various forward-looking statements regarding regulatory, clinical and other Sarepta matters. Each forward-looking statement contained in this update 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 those identified under the heading “Risk Factors” in Sarepta’s Quarterly Report on Form 10-Q for the Quarter ended June 30, 2014 (http://www.sec.gov/Archives/edgar/data/873303/000119312514299374/d759218d10q.htm) and other documents filed by Sarepta with the Securities and Exchange Commission.