January 7, 2021

Dear Duchenne Community,

Today, Sarepta announced topline results from Study 102 (Part 1) of SRP-9001, an investigational micro-dystrophin gene therapy designed for the treatment of people with Duchenne muscular dystrophy (NCT03769116).

These results reinforce our determination to bring potentially transformative treatments, including SRP-9001, to people living with Duchenne. This dataset is incredibly valuable and rich, and it has already added to our understanding of the safety and efficacy of this investigational therapy. We will be working to analyze the finer details of this data in the coming weeks and months.

The body of data for SRP-9001 is growing, and the combination of insights gained across studies will inform our ongoing clinical development program, including how we shape and advance future studies.

As you have requested, we are pleased to share some insights into the study and data as highlighted in today's press release.

Study 102 is an ongoing, randomized, double blind, placebo-controlled trial. Forty-one boys with Duchenne (aged 4-7) were enrolled in this study. The data we shared today relate to the experience of 20 boys in Part 1 of the study who received investigational micro-dystrophin gene therapy compared to 21 boys treated with placebo. Here is a summary of information shared today:

- No new safety signals were observed for SRP-9001
- Study met the primary biological endpoint of micro-dystrophin protein expression at 12 weeks post-treatment, as measured by western blot, in SRP-9001-treated participants versus placebo
- SRP-9001-treated participants showed an increase in NSAA total score compared to placebo at 48 weeks; however, the study did not achieve statistical significance on the primary functional endpoint of improvement in NSAA total score compared to placebo at 48 weeks post-treatment
- It has been noted that the baseline functional characteristics are not well matched between the treatment and placebo group. In clinical trials this is undesirable and may contribute to a lack of statistical significance seen in a functional endpoint.

Some may wonder about the primary functional endpoint used to evaluate functional outcomes. NSAA is a 17-item rating scale that is used to measure functional motor abilities in people with Duchenne who are ambulant. The 17 tasks in NSAA are evaluated for 1) the amount of time it takes for an individual to complete a task, 2) whether assistance was needed to complete the task and 3) whether or not an individual is able to perform the task. It is used to monitor the progression of the disease and treatment effects which makes it suitable as an endpoint in Duchenne clinical trials.

Study 102 is ongoing and very valuable data is still being collected. The study remains blinded to all: participants, investigators, study staff, and sponsor. By now, all 41 boys have completed their 48-week assessment and have entered the Part 2 crossover phase (during which boys who previously received placebo are treated with SRP-9001). Participants will continue to be monitored for safety, and will undergo biopsies at week 12 in Part 2 to assess expression and biological markers, in addition to longer-term assessments of functional outcomes.

Importantly, we will continue to collect data from an established and growing number of trials in this clinical development program: 9001-101, 9001-102, and 9001-103, and beyond. Each piece of data increases understanding of the short and long-term impact of this investigational therapy.

We are thankful for those who participate and support participation in all clinical studies in Duchenne, and want to recognize that your contribution will inform the care and treatment of Duchenne for many years to come.

Our mission remains unchanged. Advancing the development of SRP-9001 remains a critical priority, and we will move forward with diligence and urgency to bring SRP-9001 to people with Duchenne around the world.

If you have any questions please contact your treating Physician or you may reach out to Sarepta Patient Affairs by emailing Advocacy@sarepta.com.

Warm regards,

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