Duchenne Community Bulletin: SRP-9001 Clinical Trial Updates (June 2021)

Over the past few months, we have been able to update the community on several of our ongoing SRP-9001 clinical trials. We recognize there are many questions on study timing, eligibility criteria, and anticipated trial site locations for future trials. We will not have detailed information on these questions until we have worked with regulators to finalize our plans and we look forward to communicating with the Duchenne community around more details later this year. However, in the meantime, we would like to share a status update of each currently ongoing trial. We hope this resource provides clarity and if you have additional questions or would like to connect with Sarepta, you may reach out to Advocacy@sarepta.com.

SRP-9001 Update:

We continue to advance our ongoing SRP-9001 clinical studies and to analyze and publish data as it becomes available. We have included below a brief status update on the currently ongoing SRP-9001 trials.

- 9001-101 (NCT03375164): Dosing is complete, and long-term follow-up of the four participants continues.
- 9001-102 (NCT03769116): All dosing is complete. We have shared data from Part 1 with the community. Part 2 of this study is continuing and will be complete by the end of 2021.
- 9001-103 ENDEAVOR (NCT04626674): We continue to advance this study. SRP-9001 Study 103 is the first study to use commercially representative material. This material is representative of the manufacturing process Sarepta intends to use for all future clinical trials, and if approved, for commercial supply as well. We have shared data from the first 11 participants in this study. We are working on expanding this study to include non-ambulatory and older ambulatory study participants. Please note that these cohorts are planned to be very small.

We remain committed to future clinical development of SRP-9001. In addition to conducting in-depth analyses of all data available, we look forward to working with regulators to map next steps for SRP-9001 clinical development. Our next steps are as follows: we plan to meet with the U.S. Food and Drug Administration (FDA) by the middle of this year to discuss plans for future studies, including 9001-301, EMBARK.

Learning more about upcoming 9001 clinical trials and eligibility criteria:

We understand that many families have questions about how they can learn about upcoming clinical trials. Our primary recommendation for families interested in learning about clinical trials being conducted in Duchenne muscular dystrophy is to speak with their physician about their interest. We also recommend connecting with Duchenne patient advocacy organizations.

Families may also find more information about ongoing clinical trials by searching on the clinicaltrials.gov website. Clinicaltrials.gov postings will include some general information about the trial, as well as trial site contact information. The contact information for sites will be available on the clinicaltrials.gov page (under the "Contacts and Locations" section) for each individual trial.

If you are looking for a Sarepta clinical trial, you can search on clinicaltrials.gov in "other terms" for "Sarepta". If you have questions about a Sarepta clinical trial you can email Clinicaltrials@sarepta.com and you are also welcome to contact our Patient Affairs team at Advocacy@sarepta.com.

Information about the ExploreDMD seroprevalence study:

ExploreDMD is a study to evaluate anti-AAV antibody prevalence for rAAVrh74 seropositivity in individuals living with Duchenne muscular dystrophy. For more information, please visit ExploreDMD.com[1].

1. https://clinicaltrials.sarepta.com/exploredmd