COMMUNITY BULLETIN UPDATE

June 9 2022

Addressing Questions about Status of Sarepta Clinical Trial Programs

In response to questions we have received from the community about Sarepta's clinical trials, we are happy to provide an update on our actively recruiting clinical trial programs for Duchenne muscular dystrophy as of June 9, 2022. For the most up to date information on clinical trials, please refer to clinicaltrials.gov.

1. What Sarepta clinical trials are currently recruiting?

Below is a brief review of Sarepta clinical trials currently recruiting in our Duchenne muscular dystrophy pipeline. The information below is not comprehensive but intended to give an overview. Those interested in pursuing a clinical trial should discuss it with their physician. More information can be found on clinicaltrials.gov using the NCT (national clinical trial) numbers indicated below.

Trial Name	NCT #	Trial Overview	Intervention/ treatment	Treatment Modality	Phase	Age (in years) of Participants	Site information
ESSENCE	NCT02500381	A trial evaluating the efficacy and safety of casimersen and golodirsen in individuals living with Duchenne who are amenable to exon 45 skipping and exon 53 skipping, respectively.	SRP-4045 (casimersen) and SRP-4053 (golodirsen)	PMO Exon Skipping	3	6-13	Not currently recruiting in U.S. but recruiting in 19 other countries. Please see clinicaltrials.gov for a list of enrolling countries and sites.
MIS51ON	NCT03992430	A 2-part trial comparing the safety and efficacy of a high dose of eteplirsen to the approved 30 mg/kg dose in individuals living with Duchenne with deletion mutations amenable to exon 51 skipping. We are currently in Part 2 of the trial.	AVI-4658 (eteplirsen)	PMO Exon Skipping	3	4-13	Sites active in United States, Greece, Korea, New Zealand, Spain, Taiwan and Turkey. As addditional sites activate, they will be added to clinicaltrials.gov. You may find more information about the trial here.
MOMENTUM	NCT04004065	A two-part, multiple ascending dose trial to evaluate the safety and tolerability of SRP-5051 in individuals living with Duchenne amenable to exon 51 skipping. We are currently in Part B of the trial.	SRP-5051 (vesleteplirsen)	PPMO Exon Skipping	2	7-21	Sites active in United States, Belgium, Canada, Germany, Spain and United Kingdom. As additional sites activate, they will be added to clinicaltrials.gov. You may find more information about the trial here
EMBARK	NCT05096221	A randomized, double-blind, placebo-controlled study evaluating the safety and efficacy of SRP-9001 in individuals living with Duchenne.	SRP-9001 (delandistrogene moxeparvovec)	Gene transfer therapy	3	4-7	Sites active in United States, Spain and Taiwan. Additional sites are planned for United States, Europe and Asia and will be added to clinicaltrials.gov as they activate. You may find more information here.

2. Are you planning a gene therapy trial for an expanded population?
   We are planning a trial for older ambulatory and non-ambulatory individuals and will share details of this trial and others as we draw nearer to start dates.

3. Why is there a study evaluating eteplirsen when it has been approved in the United States?
   As part of an FDA postmarketing study requirement, MIS51ON is evaluating a higher dose of eteplirsen and comparing it to the approved 30 mg/kg dose. There is no placebo group in this study, rather, participants will receive a higher dose of eteplirsen or the approved 30 mg/kg dose.

4. Once enrolled in a Sarepta clinical trial, what support can I expect?
   We are committed to providing a positive experience for the individuals living with Duchenne and their families who participate in our trials. We regularly incorporate community feedback into the design of our trials in order to reduce study participant burden and to make trials more inclusive and accessible to all. In general, reasonable travel, accommodation and meals while traveling to and from the site are financially covered by Sarepta, and Sarepta provides support staff to help with travel co-ordination. Please note, country relocation is not supported. We recommend discussing this with your physician and reaching out to study sites if you have questions about participation.

5. Will the COVID 19 vaccine affect my/my child's potential participation in a gene therapy trial?
   For more information about COVID 19 vaccine and clinical trials, please refer to our Community Bulletin: COVID-19 Vaccination and Gene Therapy.

6. How do I learn more about the clinical trials listed above?
   Each clinical trial has a NCT # associated with it. You may go to clinicaltrials.gov and search in "other terms" for the NCT #. There, you will find more information about the trial and contact information for sites that are currently recruiting. You may also reach out to SareptAlly@Sarepta.com for any specific questions about Sarepta clinical trials.

7. Where can I find additional information about clinical trials in general?
   Please visit Duchenne.com for additional information about clinical trial considerations. Additionally, feel free to reach out to advocacy@sarepta.com for other questions.