



Sarepta plans to have study sites in the following countries:

United States
Belgium
Canada
Czech Republic
France
Germany
Italy
Israel
Spain
Sweden
United Kingdom

Visit www.clinicaltrials.gov and search NCT02500381 for a list of sites that are currently enrolling patients.

For more information,
please visit
www.EssenceTrial.com

Visit www.sarepta.com for updates on
Sarepta's clinical studies



4045-301

CLINICAL TRIAL

ESSENCE

For Duchenne
muscular dystrophy (DMD) patients
with deletion mutations amenable
to exon 45 or exon 53 skipping

Information on site locations and
contact information for Sarepta Therapeutics, Inc.
(Sarepta) can be found on the back of this brochure

The ESSENCE Study

The purpose of this Phase III research study is to evaluate the safety and effectiveness of SRP-4045 and SRP-4053, Sarepta's exon 45- and exon 53-skipping investigational drugs

Who may be able to participate in the ESSENCE study?

- A boy with DMD, 7 to 13 years old who can walk
- Has a genetic test that shows he has a deletion that may be treated by skipping exon 45 or 53*. Talk to your doctor if you are unsure.
- Has been on a stable dose of corticosteroids (eg, prednisone or deflazacort) for at least 6 months
- Stable lung (breathing) and heart function

There are additional requirements for participation and these will be reviewed with patients and their families during the screening process.

Where are study sites located?

We plan to have study sites in cities throughout the United States as well as in the countries listed on the back of this brochure. For a list of sites now enrolling patients, visit www.clinicaltrials.gov and search NCT02500381. You can find the closest active study site at EssenceTrial.com

Why should I consider participating in this study?

The potential benefits of SRP-4045 and SRP-4053 in patients with DMD are unknown. Even if your son does not benefit from being in this study, we might learn something that could advance research and help others.

What risks are associated with this study?

As with all research studies, there can be risks associated with possible side effects of taking the study drug and with the medical tests carried out as part of the study. Information on the possible risks your son may face in this study is available in the consent form, and should be discussed with your study doctor.

* Deletions amenable to exon 45 skipping include, but are not limited to, deletions of exons 12-44, 18-44, 44, 46-47, 46-48, 46-49, 46-51, 46-53 or 46-55.
Deletions amenable to exon 53 skipping include, but are not limited to, deletions of exons 42-52, 45-52, 47-52, 48-52, 49-52, 50-52, 52, or 54-58.

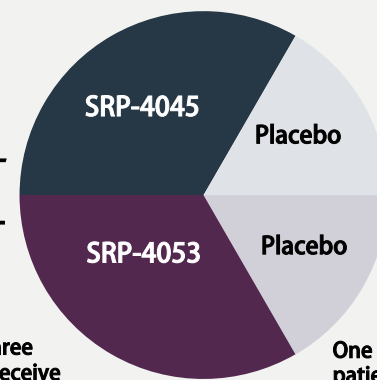
SRP-4045

DMD patients with deletions potentially responsive to, or amenable to exon-45 skipping

SRP-4053

DMD patients with deletions potentially amenable to exon-53 skipping

Two out of three patients will receive active study drug.



Placebo

Patients are randomly assigned to receive either active study drug or inactive placebo

One out of three patients will receive inactive placebo

What is a randomized placebo-controlled study and why is Sarepta running one?

Randomized, placebo-controlled means that each study participant will be picked randomly, by chance (like tossing a coin) to receive either active study drug (SRP-4045 or SRP-4053, depending on their deletion type) or "placebo." Placebo is made to look just like the study drug, but it will not contain any active drug. If your son takes part in this study, he will have a 2 in 3 chance of receiving study drug and a 1 in 3 chance of receiving placebo.

Researchers use a placebo to see if the study drug works and to see how safe it is compared to not taking anything. A placebo-controlled trial design is the type most commonly used for Phase III clinical trials that are used to get approval for drugs from agencies like the FDA and the European Medicines Agency.

Patients who complete the 2-year placebo-controlled part of the study will be eligible to participate in the 2-year open-label extension period. Open-label means all patients will receive active study drug and you and the study team will know that your son is receiving it.

What is involved in participating in this study?

- Weekly visits to your nearest study site, where your son will receive an infusion of active study drug or placebo (depending on which treatment group your son is assigned to, and what part of the study he's in). Your son will also periodically have blood drawn and physical examinations done at your local site.
- Approximately every 12 weeks there will be functional assessments which include tests of walking distance, other walking-related activities, muscle strength, breathing and heart function. You may need to travel to a site other than your nearest clinical trial site to do these functional assessments.
- There are also 2 muscle biopsies, 1 at the beginning of the study and 1 after approximately one year. Depending on where you live it may be necessary to travel for the biopsy surgery.
- You can learn more about all of the requirements and activities in this research study from the study doctor.

Will I be compensated for participating?

Generally, reasonable costs associated with participation in the study will be prepaid or reimbursed by Sarepta in accordance with the approved travel policy for the procedures performed as part of the study. Aside from travel costs, no additional compensation will be provided to participants. Additional information will be provided by the study site.