



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?

Key inclusion criteria include:

- 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.

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## Where will study sites be 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](http://www.clinicaltrials.gov) and search NCT02500381. You may also email [trialinfo@sarepta.com](mailto:trialinfo@sarepta.com) for more information.

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## Who determines eligibility to participate?

The principal investigator (doctor) at a study site determines whether or not a patient meets all of the inclusion criteria and none of the exclusion criteria for the study, and is therefore eligible to participate in the study. Evaluations and tests will be performed during the screening visit, and the study doctor will review and assess eligibility. Ultimately, the decision to participate in this study should be made between your family and your child's physician.

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## Why should I consider participating in this study?

While no benefit can be guaranteed from participation in any clinical study, we believe some benefits may include:

- Access to an investigational therapy
- Access to highly experienced clinicians with strong expertise in treating DMD
- Better understanding of your son's disease
- Opportunity to become more familiar with what participation in a clinical study entails
- Opportunity to contribute to what is known about DMD progression
- Opportunity to help others by contributing to medical research that may accelerate the development of DMD therapies

\* 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.

**This randomized, placebo-controlled study includes 3 arms:**

- DMD patients with deletions amenable to exon 45 skipping, who will receive SRP-4045
- DMD patients with deletions amenable to exon 53 skipping, who will receive SRP-4053
- DMD patients with deletions amenable to exon 45 or 53 skipping, who will receive placebo

### **What risks are associated with this study?**

As with all clinical studies, there can be risks associated with possible side effects of taking the study drug and with the standard medical tests carried out as part of the study at each visit. Information on the possible side effects you may experience in this study is available in the consent form and should be discussed with your study doctor.

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### **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.

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### **What is involved in participating in this study?**

Enrolled patients will visit study sites for dosing, functional assessments, medical testing, and 2 biopsies over the course of the initial 2-year placebo-controlled portion of the study. This will be followed by the 2-year open-label period, during which all patients will be able to receive active study drug. Dosing visits will occur weekly and functional assessments will be conducted every 3 months in the placebo-controlled period and less frequently in the open-label period. The study doctor and his or her staff will review the study requirements during the informed consent process.

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### **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 study procedures performed as part of the study. Information will be provided by the study site.

Sarepta plans to have clinical study sites  
in the following countries:

**United States**  
**Belgium**  
**Canada**  
**France**  
**Germany**  
**Italy**  
**Netherlands**  
**Sweden**  
**United Kingdom**

Visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov) and search NCT02400381  
for a list of sites that are currently enrolling patients.

**For more information,  
contact:  
[trialinfo@sarepta.com](mailto:trialinfo@sarepta.com)**

Visit [www.sarepta.com](http://www.sarepta.com) for updates on  
Sarepta's clinical studies

