UNIVERSITY OF CALIFORNIA LOS ANGELES
PARENT PERMISSION/YOUTH (13-17) ASSENT TO PARTICIPATE IN RESEARCH

A Double-Blind, Placebo-Controlled, Multi-Center Study With an Open-Label Extension to Evaluate the Efficacy and Safety of SRP-4045 and SRP-4053 in Patients with Duchenne Muscular Dystrophy
Protocol Number: 4045-301
Protocol Version Date: June 07, 2016

INTRODUCTION

Study Sponsor: Sarepta Therapeutics
215 First St.
Cambridge, MA 02142

Investigator: [redacted]

Research Address: David Geffen School of Medicine at UCLA
UCLA Neurology
[redacted]

[redacted] and associates from the Department of Neurology at the University of California, Los Angeles are conducting a research study. Your child is being invited to participate in a research study. The purpose of this "Parent Permission/Youth Assent to Participate in Research" is to give you the information that you will need to help you decide whether or not to allow your child to participate in the research study. *Please read this form carefully*.

The study team will explain the research study to you, and answer your questions about anything you do not understand in connection with your decision. You should ask questions about what your child will be asked to do, the risks, the benefits, your child's rights as a subject, or anything else about the research or this form that is not clear to you. When all of your questions have been answered to your satisfaction, you can decide if you want your child to take part in this study or not. This process is called "informed consent."

Participation in the research study is voluntary. You may refuse to allow your child to be in this study or your child may quit this study at any time, and standard medical care will still be available here or from a doctor of your choice without a penalty or loss of benefits.

If you agree to have your child participate in this research study, you will be asked to sign this Parent Permission Form. If your child is 13 years old, he will also be asked to sign this form to give assent.If he is 12 years or younger, he will be asked to sign a shorter Assent Form more appropriate to his age. You will be given a signed and dated

copy of this Parent Permission Form and the Assent Form (if applicable) for your records.

WHY IS THIS STUDY BEING DONE?
The study is being conducted for Sarepta Therapeutics, Inc. (called the Sponsor). The Sponsor is studying two new investigational drugs, SRP-4045 and SRP-4053, for the treatment of Duchenne Muscular Dystrophy (DMD).

We are doing this research study to find out if SRP-4045 and SRP-4053 can help people with DMD. We also want to find out if SRP-4045 and SRP-4053 are safe to take without causing too many side effects. SRP-4045 and SRP4053 are not approved by the U.S. Food and Drug Administration (FDA), the European Medicines Agency (EMA), or similar government agencies abroad. This means that SRP-4045 and SRP-4053 are investigational (experimental) drugs and can only be used in research studies.

DMD is caused by a mutation (a change) in the gene (the part of cells that contain the instructions which tell our bodies how to grow and work) that makes dystrophin (a protein). Dystrophin is important for protecting muscles from stress and damage during activity. If a person has DMD, his body is not able to make enough working dystrophin to protect his muscles.

Some DMD patients are missing certain parts or “exons” of the dystrophin gene so their bodies can’t make full-length dystrophin protein. For these people with so called deletion mutations, “skipping” over an addition exon may allow the body to produce a shortened, but still working, form of the dystrophin protein. This research study will test the effects of these drugs on muscle function in people with DMD who have deletions that may be treated by skipping exon 45 or 53.

HOW MANY PEOPLE WILL TAKE PART IN THIS STUDY?
This study will include approximately 99 male patients. There will be approximately 40 different sites (locations) participating in this study in the U.S and Europe. UCLA will likely enroll 12 subjects in this study.

WHAT WILL HAPPEN IF I TAKE PART IN THIS STUDY?
You are being asked to allow your child to participate in this research study because your child has DMD and prior genetic testing has indicated that he has a deletion that may be treated by skipping exon 45 or 53. After the Screening portion of the study, if your child is eligible to join the study, he may be enrolled in one of the following groups:

- **SRP- 4045 Randomized Group** – This means your child has a genetic deletion that may be treated by SRP-4045. He will receive an intravenous (IV) infusion of SRP-4045 at a dose of 30 mg/kg once a week for up to 96 weeks during the double-blind, placebo-controlled part of the study. He will then continue to receive infusions at the same dosage of the study drug in the open-label period of the study.

- **SRP- 4053 Randomized Group** – This means your child has a genetic deletion that may be treated by SRP-4053. He will receive an intravenous (IV) infusion of SRP-4053 at a dose of 30 mg/kg once a week for up to 96 weeks during the double-blind, placebo-controlled part of the study. He will then continue to receive infusions at the same dosage of the study drug in the open-label period of the study.

- **Placebo group** - This means your child has a genetic deletion that may be treated by SRP-4045 or SRP-4053. He will receive an intravenous (IV) infusion of placebo once a week for up to96 weeks during the double-blind, placebo-controlled part of the study. After the double-blind period is over he will move into the open-label period and receive the study drug, SRP-4045 or SRP-4053 depending on his genetic deletion, at a dose of 30 mg/kg by weekly

This study is a **double-blind, randomized placebo-controlled** research study followed by an **open-label extension**.

Double-blind means that the study doctor, nurses, caregivers, Sponsor, you, and your child will not know if your child is receiving active study drug or placebo.

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 deletion type) or “placebo”. Placebo is made to look like the study drug, but it will not contain any active drug. Your child will have a 2 in 3 chance of receiving active study drug and a 1 in 3 chance of receiving placebo in the double-blind placebo-controlled part of the study.

Researchers use a placebo to see if the study drug works and how safe it is compared to not taking anything. While participating in this study, your son will still be able to receive standard treatment for his disease.

Open-label means that all patients will receive the study medicine. The study doctor, nurses, caregivers, Sponsor, you and your child will all know that your child is receiving study drug.

Your child’s expected participation in the full study will last up to 204 weeks: up to [redacted] weeks in the screening period, up to 96 weeks in the double-blind placebo-controlled period, up to 96 weeks in the open-label period and [redacted] weeks in the follow-up period. During this time, your child will need to come in for study visits on a set schedule and have the examinations described later in this form.

When approximately 75 patients have been in the trial for 48 weeks a group of independent experts will review key study results and will make a determination on whether patients will roll into the open-label period of the study and receive active drug or continue in the placebo-controlled period out to Week 96 as planned. No one directly involved in the study will see these results or take part in this expert review.

At the completion of this study, your son will be eligible for an open-label extension study, where active study drug (SRP-4045 or SRP4053) will be provided for the duration of that study.

Please keep in mind how the study examinations and visits described will affect your work and family schedules. You may find that these examinations and visits need some planning. Ask the study doctor or study staff if you have any questions about the examinations and procedures for the study.

Study Procedures -Screening Phase

The study doctor will perform the following examinations and tests to make sure that this study is right for your child. [redacted]

At Your [redacted] Site [redacted]:

1. **Informed Consent and Assent**

2. **Review of Inclusion/Exclusion Criteria –** If your child does not meet all of the inclusion criteria and/or if your child meets any of the exclusion criteria, your child will not be allowed to take part in this research study and will not continue in the remainder of the Screening phase.

3. **Review of Medical History and Current Medications / Treatment –** You and your child will be asked questions about other medicines that he may be taking. This will include all prescribed and over-the-counter medications as well as other treatments that your child has taken, including herbal supplements, vitamins, minerals, homeopathic preparations and physical therapy.

4. **Physical Examination –** Examination of general appearance including head, eyes, ears, nose, throat, heart, chest, abdomen, and skin, as well as lymph nodes, muscles, bones, and nervous system will be conducted.

5. **Weight and height**

6. **Vital Signs –** Blood pressure, heart rate, breathing rate, and oral temperature will be measured.

7. **Blood and Urine Collection for Genetic Testing and Laboratory Assessments –** About 18 mL of blood (about 4 teaspoons) will be taken from your child's arm. A topical anesthetic (numbing) cream (e.g., lidocaine 2.5%, prilocaine 2.5%, or LMX4 cream) may be applied to your child's skin prior to the needle stick, if needed. Your child will also be asked to collect his urine in a provided container during this visit. It is fine for your child to eat and drink before these tests.

8. **Adverse Events –** You and your child will be asked about how he is feeling and about any changes in his health.

9. [redacted]

AtYour [redacted] Site: [redacted] you and your child will [redacted] be required to complete [redacted] assessment visits at UCLA, [redacted] The [redacted] site will assess your child's lung, muscle, and heart function by performing the tests described below.

The first [redacted] visit occurs soon after you have met with your [redacted] Site doctor, and there will be another [redacted] visit (the Baseline visit) that will occur [redacted] weeks later. [redacted]

The following assessments will be conducted [redacted] at the [redacted] Site.

1. **Informed Consent and Assent**

2. **Review of Inclusion/Exclusion Criteria –** If your child does not meet all of the inclusion criteria and/or if your child meets any of the exclusion criteria, your child will not be allowed to take part in this research study and will not continue in the remainder of the Screening phase. [redacted]

3. **Vital Signs** – Blood pressure, heart rate, breathing rate, and oral temperature will be measured. [redacted]

4. **Pulmonary Function Tests (PFT)** – These tests measure how well the lungs work. Your child will be asked to blow in a tube attached to a computer to see how much air he can inhale (breathe in) and exhale (breathe out) from his lungs. [redacted]

5. **6 Minute Walk Test (6MWT)** – Your child will be asked to walk for 6 minutes without help. [redacted]

6. **Height -** [redacted]

7. [redacted]

[redacted]

8. **North Star Ambulatory Assessment (NSAA)** – Your child will be asked to perform some exercises like getting up from the floor and stepping up on a box step.[redacted]

9. [redacted]

[redacted]

[redacted]

[redacted]

13. **Electrocardiogram (ECG)** – This test checks the electrical activity of the heart. Wires will be placed on your child's chest to measure how well his heart beats. [redacted]

14. **Echocardiogram (ECHO)** – This test uses sound waves to create a picture of the heart. A standard 2-dimensional (2D) ECHO will be obtained. In this test, your child will be lying on a bed and a person will move a probe on your child's chest and look at a screen to see a picture of how well your child's heart beats and moves inside his chest. [redacted]

[redacted]

Confirmation of Eligibility:

The [redacted] study physician and Sponsor will review all results of the screening and baseline assessments described above and confirm your son's eligibility. Your study doctor will inform you if your child is eligible to be enrolled in this study. If he is eligible, he will continue onto the assessments below.

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At UCLA, [redacted]
You will be required to sign a separate Informed Consent Form for the procedures that will be performed [redacted]. [redacted]

1. **Informed Consent and Assent**

2. **Muscle Biopsy** - In order to learn if SRP-4045 or SRP-4053 can help a DMD patient's body make dystrophin, your son will need to undergo two muscle biopsy procedures during the course of the study, where a small piece of tissue is taken from a muscle in his arm. The first biopsy will take place prior to receiving the first infusion of study drug or placebo.

   Your child will be given anesthesia (sleep medicine) prior to the biopsy, which will make him sleep through the procedure. The biopsy takes approximately 30 to 60 minutes to complete. The surgeon will make a small cut in the skin of your child's upper arm and two samples of your child's muscle will be taken out to be studied. Each piece will not be larger than the size of a pencil eraser. The skin will be closed with stitches that usually disappear by themselves and the biopsy area will be covered with a bandage to keep it clean and dry.

   Your child will stay at the hospital for a few hours after the procedure while he wakes up from the sleep medicine. You and your child will be given instructions and have the opportunity to discuss how to take care of the wound. He should avoid strenuous activity with that arm for a week.

3. **Blood Collection -** A blood sample will be collected so the study doctor can make sure your child is healthy enough to receive active study drug or placebo at the [redacted] Site a few days later. This sample will also be used for research about changes in the blood that happen with DMD and that might be affected by treatment. About 14mL or 3 tsp of your son's blood will be taken at this visit.

4. Weight

Study Procedures – Placebo-Controlled Period
During this part of the study, your child will receive either SRP-4045 or SRP-4053 (depending on your child's deletion) at a dose of 30 mg/kg, or placebo as an intravenous (IV) infusion (meaning the study drug or placebo will be given through a vein directly into the bloodstream) once a week for up to 96 weeks.

[redacted] You and your child will visit:

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(a) your [redacted] Site every week to receive study drug or placebo infusions, answer questions about how your child has been feeling and perform routine assessments;

(b) Your [redacted] Site every 12 weeks for study-specific tests that measure his muscle, lung and heart function; and

(c) Your [redacted] site for a muscle biopsy at the beginning of the study as described above and again at 48 weeks (about 1 year) into the study.

Blood and urine will be collected at some of these visits (approximately once every week for the first 8 weeks of the study, once at Week 12, and then every 4 to 12 weeks) for laboratory tests for safety and to learn more about the study drug and about DMD. Blood samples will be taken [redacted] during 21 of the study visits in the Screening and placebo-controlled periods and at 15 visits during the open-label period. [redacted]

In the placebo-controlled period of the study additional blood will be collected at 9 of these [redacted] Site visits before and after the infusion to learn more about how the body processes the study drug. In the open-label period this additional blood will be collected at 4 visits. [redacted]
The maximum amount of blood that will be taken at a single visit is 22 mL (about 4 ½ teaspoons). No more than approximately 265 mL (a little more than 1 cup) will be collected over the course of the placebo-controlled period of the study.

Your child will need to undergo a second muscle biopsy procedure after 48 weeks (about 1 year) of being in the study.

The study doctor will schedule the visits and it is important that your child follows this schedule closely and is seen on those days the doctor schedules in order to remain enrolled in this research study. The procedures and tests that will take place at each of the study visits are presented below.

At Your [redacted] Site:
<u>Weeks 1 to 96: Visits occur once a week for 96 weeks</u>

The assessments below will be conducted at your [redacted] Site. Each visit will last approximately 2-3 hours.

1. **Adverse Events** – *Weekly*
   You and your child will be asked about how he is feeling and about any changes in his health.

2. **Review of Concomitant Medications** (Use of drugs at the same time) and **Physical Therapy Management** [redacted]

These will be the same kinds of questions that will be asked during the Screening phase of the study.

3. **Physical Examination** – About once every 4 weeks
   At some visits your child will have a full physical examination including general appearance, head, eyes, ears, nose, and throat, heart, chest, abdomen, skin, lymph nodes, muscles, bones, and nervous system. At other visits your child will have a brief physical examination where only general appearance, head, eyes, ears, nose, throat, heart, chest, abdomen, and skin will be checked.

4. **Blood and Urine Collection for Laboratory Assessments** – *Starting at Week 1 for the first 8 weeks of the study, once at Week 12, and every 12 weeks thereafter* [redacted]
   A maximum of 22 mL of blood (about 5 teaspoons) will be drawn during a single visit. This total includes samples collected to measure levels of study medication in the blood (PK collection) as noted in number 7 below. [For sites not doing PK: A maximum of 14 mL of blood (about 3 teaspoons) will be drawn in a single visit. Your child will also be asked to collect his urine in a provided container during these visits. It is fine for your child to eat and drink before these tests.

5. **Vital signs and Weight** – *Weekly*
   Blood pressure, heart rate, breathing rate, and oral temperature will be measured [redacted]

6. **Active Study Drug or Placebo Infusion** – *Weekly*
   SRP-4045 or SRP-4053, or placebo will be given once a week via an IV infusion that takes approximately 35 to 60 minutes. A topical anesthetic (numbing) cream may be applied to your child's skin prior to the infusion, if needed. After the infusion ends, your child will be observed for at least 1 hour to make sure he is doing okay. As long as he is okay and all other tests and procedures have been completed, the study doctor will then let you and your child go home.

7. **PK Collection** [redacted]
   At select sites, small blood samples will be taken to help us understand how the body processes the study drug and how it is distributed in the body. [redacted] Approximately 8 mL or ½ tablespoon in total will be collected at each PK collection visit.

At Your [redacted] Site:
Functional Assessments: [redacted]

In addition to the previously mentioned visits at your [redacted] Site, you and your child will also be required to complete an assessment visit [redacted] at the specific weeks

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listed above. [redacted]

The study doctors at the [redacted] Site will assess your child's lung, muscle, and heart function by performing the tests described below. These are the same tests that are done during the Screening phase of the study. [redacted]

The following assessments will be conducted at the [redacted] Site:

1. **Pulmonary Function Tests (PFT) –** [redacted]

2. **6 Minute Walk Test (6MWT) –** [redacted]

[redacted]

4. **Electrocardiogram (ECG)** [redacted]

5. **Height** [redacted]

6. **North Star Ambulatory Assessment (NSAA)** – [redacted]

[redacted]

[redacted]

9. **Echocardiogram (ECHO)** [redacted]

[redacted]

[redacted]

At Your [redacted] Site:

Muscle Biopsy: Week 48

In addition to the previously mentioned visits [redacted], you and your child will also be required to visit [redacted] at Week 48 for a second muscle biopsy. This is the same procedure that is done during the Screening part of the study.

[redacted]

Study Procedures – Open-Label Period

At [redacted] Site:
Weeks Open Label 1 to 96: Visits occur once a week for 96 weeks

The assessments below will be conducted at your [redacted] Site. Each visit will last approximately 2-3 hours.

1. Adverse Events – Weekly

2. Review of Concomitant Medications (Use of medications at the same time) and Physical Therapy Management [redacted]

3. Physical Examination [redacted]

4. Blood and Urine Collection for Laboratory Assessments – [redacted]
A maximum of 20 mL of blood (about 4 teaspoons) will be drawn during a single visit. This total includes samples collected to measure levels of study medication in the blood (PK collection) as noted in number 7 below. [For sites not doing PK: A maximum of 11 ml of blood (about 2 teaspoons) will be drawn during a single visit) Your child will also be asked to collect his urine in a provided container during these visits. It is fine for your child to eat and drink before these tests.

5. Vital Signs and Weight – Weekly. Weight will be measured once every 4 weeks.

6. Active Study Drug Infusion – Weekly
SRP-4045 or SRP-4053 will be given once a week via an IV infusion that takes approximately 35 to 60 minutes.

7. [PK Site only: PK Collection [redacted]
At select sites, small blood samples will be taken to help us understand how the body processes the study drug and how it is distributed in the body. A blood sample will be taken from your child's arm [redacted] Approximately 8 mL or ½ tablespoon in total will be collected at each PK collection visit.]

8. Electrocardiogram (ECG) [redacted]

At [redacted] Site:
Functional Assessments: Open Label Weeks [redacted]

In addition to the previously mentioned visits [redacted] you and your child will also be required to complete an assessment visit [redacted] at the specific weeks

listed above. [redacted] The study doctors [redacted] will assess your child's lung, muscle, and heart function by performing the tests described below. These are the same tests that will be done during the Placebo-Controlled Period of the study. [redacted]

The following assessments will be conducted [redacted]

1. Pulmonary Function Tests (PFT)
[redacted]

2. 6 Minute Walk Test (6MWT)
[redacted]

3. [redacted]
[redacted]

4. Electrocardiogram (ECG) [redacted]
[redacted]

5. Height
[redacted]

6. North Star Ambulatory Assessment (NSAA)
[redacted]

7. [redacted]
[redacted]

[redacted]

9. Echocardiogram (ECHO) [redacted]
[redacted]

[redacted]

Study Procedures – Follow-up period

At [redacted] Site:
Open Label [redacted] (End of Study Visit): Approximately [redacted] weeks after the last infusion of study drug

1. **Adverse Events**

2. **Review of Concomitant Medications** (Use of medications at the same time) **and Physical Therapy Management**

3. **Full Physical Examination**

4. **Vital Signs**

5. **Blood and Urine Collection for Laboratory Assessments** –
   About 7 mL of blood (about 1½ teaspoons) will be collected. Your child will also be asked to collect his urine in a provided container. It is fine for your child to eat and drink before these tests.

[redacted]

WHAT HAPPENS IF I DO NOT FINISH THIS STUDY?
It is your choice to be in this study or to stop at any time. If you decide to stop your child from being in this study, you need to tell the study doctor or the study coordinator. The notice of withdrawal should be in writing and sent to the following address:

[redacted]

If you stop your child from being in the study, there will not be a penalty or loss of benefits to which your child is otherwise entitled. It may be helpful if you could explain your reasons. Your child may receive standard treatment and no prejudice will be shown towards you/your child for medical care or participation in future research.

CAN THE RESEARCHERS REMOVE ME FROM THIS STUDY?
If at any time the study doctor or sponsor believes participating in this study is not the best choice of care for your child, his participation may be stopped and other care prescribed by the study doctor. If you or your child fail to follow study instructions, your

child's participation in the study may also be stopped. If unexpected medical problems come up during this clinical trial, your study doctor or the Sponsor may decide that it is best to stop your child's participation in the study. His participation may also be stopped if the Sponsor cancels or stops the research study.

If your child's participation in this study ends before completing all of the study activities, your child will be asked to visit the study doctor (unless you have withdrawn consent) and you will be asked to return to the [redacted] Site [redacted] so that the following tests can be completed:

- PFT, 6-MWT, NSAA, [redacted] height, ECHO, and ECG

Your study doctor will ask you and your child to return to your [redacted] Site [redacted] so that the following study procedures can be done:

- Collection of any change in medications or physical therapy
- Review of adverse events (how your child has been feeling)
- Physical examination and vital signs
- Blood and urine collection for laboratory assessments – 7 mL of blood (about 1.5 teaspoons) will be taken from your child's arm. Your child will also be asked to collect his urine in a provided container during this visit. It is fine for your child to eat and drink before these tests.

If you withdraw your child from the study because he is not feeling well, you will be encouraged to come back for check-ups until he is feeling better.

WHAT KINDS OF RISKS OR DISCOMFORTS COULD I EXPECT?

There are risks to taking part in this research study. One risk is that your child may have side effects from taking SRP-4045 or SRP-4053 or from participating in the other study procedures.

SRP-4045

In animal studies of SRP-4045, kidney damage was observed. Because of this potential risk, we will monitor kidney function carefully during the clinical trial.

The first human study of SRP-4045 is taking place in the US in boys and young men with advanced-stage DMD. While some information about SRP-4045 is available now, some risks remain unknown. If any new findings about the risks of SRP-4045 become available, we will share them with you.

As of 01 March 2016, 12 patients had received SRP-4045 in the study. The following health issues were reported in more than 1 patient: headache and vomiting (3 patients each); musculoskeletal pain (pain in joints or muscles), nasopharyngitis (inflammation of

the nose and throat), nausea, neck pain, pain in extremity (pain in arms or legs) and procedural pain (2 patients each). All other health issues were experienced by 1 patient each. The majority of health issues were mild, non-serious and considered unrelated to SRP-4045.

SRP-4053

[redacted]

Several boys are currently receiving SRP-4053 in a European clinical trial similar to this one. While some information about SRP-4053 is available now, some of the risks remain unknown. If any new findings about the risks of SRP-4053 become available, we will share them with you.

As of 22February 2016, 25 patients with DMD had received SRP-4053 in the study.

The most commonly reported health issues were cough (10 patients), headache (8 patients), fever (7 patients), nasopharyngitis (inflammation of the nose and throat), rhinitis (inflammation and irritation of the lining of the nose) and upper abdominal pain (6 patients each). Some patients experienced fever on days of study infusions that was considered related to SRP-4053. Most of these patients recovered the same day and the fever was considered mild. The majority of health issues were mild, non-serious and considered unrelated to SRP-4053.

Blood Draw/IV Risk
Drawing blood and starting IVs by placing a needle in a vein may cause pain, lightheadedness and fainting, bleeding, bruising, or swelling at the puncture site. Infection is a rare possibility. Topical anesthetic or numbing cream may be used on the skin to decrease the discomfort, if needed. Although there are no known major side effects from the numbing cream, some side effects like skin irritation or an allergic reaction are possible.

Electrocardiogram (ECG) Risk
The test uses sticky pads placed on your child's body which are not painful, but the pads may make the skin itchy or cause redness or a rash, and removing them may feel like pulling off a sticky adhesive bandage.

Muscle Biopsy Risk
The common side effects of muscle biopsies may include pain, scarring, infection, bruise, numbness near the biopsy site, or delayed wound healing. Although infection is rare, signs of infection include discharge (oozing), redness, and an increase in pain over time. If you or your child see any of these signs, you should report this to the study doctor immediately.

Sedation/General Anesthesia Risk

Risks from any local anesthetic (numbing medicine) that might be used during the muscle biopsy procedure may include pain with injection, and (very rarely) allergic reactions. It is possible that your child will have a reaction to the sedative or general anesthetic (medications that help your child relax and fall asleep for the biopsy). Rarely, sedation or general anesthesia may compromise breathing. Other side effects of sedation or anesthesia include nausea, vomiting, drowsiness, dizziness, sore throat, shivering, aches and pains, discomfort, and agitation upon awakening from anesthesia. Your child will be carefully monitored before, during and after sedation or anesthesia for signs of breathing issues and other side effects and may need to stay in the hospital longer than expected if they happen. Minor discomfort after biopsy surgery is common, but the likelihood of other risks is low.

DMD patients are more susceptible to rhabdomyolysis, a condition in which muscle cells break down; proteins from the muscle can then enter the bloodstream and possibly injure the kidneys. One DMD patient in another study was hospitalized for rhabdomyolysis prior to receiving any study drug; in this case, the rhabdomyolysis was thought to be caused by a specific kind of anesthesia he received prior to muscle biopsy. In the present study, the doctors performing biopsies and giving anesthesia will be reminded about the special considerations regarding anesthetic use in DMD patients.

If any of the symptoms listed above are severe, your child must get medical help immediately. If you are worried about anything while in this study, please call the study doctor or study nurse at the telephone number on the first page.

Reproductive Risks

There are no results from any studies to indicate whether or not SRP-4045 or SRP-4053 may have an effect on an unborn child. There have not been any studies on human or animal pregnancy while on these drugs. Since the risk is unknown and your child may have already gone through puberty or may go through puberty during the course of the study, he should not father a baby while on infusions during the study and must agree to a medically acceptable method of birth control for him and his partner for the entire length of the study and for 90 days after the last infusion.

Unforeseen Risks

There may be other risks of being in this research study that are not known at this time. There is no way to predict if your child will experience any side effects. Side effects that are not yet known may also occur.

WHAT WILL HAPPEN IF NEW INFORMATION IS FOUND OUT ABOUT THE DRUG OR TREATMENT?

If new information is found out during this study that might change your mind about taking part or might affect your child's health, your study doctor will discuss it with you as soon as possible.

USE OF SAMPLES AND DATA FOR FUTURE RESEARCH PURPOSES

During the research study, we will be collecting blood and tissue samples ("samples") from your child. Some of these samples may not be fully used so that there are excess

or leftover portions. The Sponsor would like to store the leftover samples for research at a later time, perhaps even years from now. These samples may be stored by the Sponsor for up to 15 years. This future research may be for purposes other than those defined in this study. The future research may be performed by the Sponsor, or the samples may be shared with outside researchers selected by the Sponsor to perform research. You and your child will not be told the results of any future research. It is possible that the samples might be used to develop products or tests that could be patented and licensed. Should this occur, you or your child will not receive financial compensation

If you do not agree to have your child's samples stored and used for future research, your child will still be able to participate in the research study. Your decision will in no way affect your child's ability to receive other care, the payment for healthcare, or healthcare benefits.

You are free to withdraw your consent for your child's samples to be stored for future research at any time. If you decide to withdraw your consent, you may contact the study doctor or study nurse. Upon receipt of your request to withdraw, any remaining identifiable samples will be destroyed.

Please initial next to YES or NO below whether you give permission for the research described above:

_____ **YES** I agree to allow blood and/or tissue/data to be stored and used for future research (including extra blood drawn specifically for future research). I understand that they may be used for testing/research unrelated to this study, ***including genetic analysis***, and that I will not be told the results.

_____ **NO** I do not want leftover blood and/or tissue/data to be stored or used for future research, and I do not agree that other assessments may be used for research other than for the purpose of this study.

Genetic Information Nondiscrimination Act (GINA)

A Federal law, called the Genetic Information Nondiscrimination Act (GINA), generally makes it illegal for health insurance companies, group health plans, and most employers to discriminate against your child based on his genetic information. This law generally will protect you in the following ways:

- Health insurance companies and group health plans may not request your child's genetic information that we get from this research.
- Health insurance companies and group health plans may not use your child's genetic information when making decisions regarding your child's eligibility or premiums.
- Employers with 15 or more employees may not use your child's genetic information that we get from this research when making a decision to hire, promote, or fire your child or when setting the terms of his employment.

This Federal law does not protect your child against genetic discrimination by companies that sell life insurance, disability insurance, or long-term care insurance.

OTHER IMPORTANT INFORMATION
Parents or other family of a child participating in the research study may not hold stock nor any other interest with the potential for financial gain in the Sponsor, Sarepta Therapeutics, Inc. By signing this consent form, you agree that neither you nor any of your family have stock or an interest with the potential for financial gain in Sarepta Therapeutics, Inc.

It is important that your child's healthcare providers, including primary care physicians and specialists, know about all physical therapy regimens and medicines your child is taking. This includes the study drug being tested in this research study.

The study doctor and study team are being paid by Sarepta Therapeutics, Inc. for the time and knowledge needed to do this study. If applicable, your study doctor will disclose to you any financial links or other interests that he/she may have to the Sponsor.

Being in more than one research study at the same time is not permitted. Please tell the study doctor if your child has been involved in any research studies in the past, including any observational studies that did not include drug treatment.

In order to avoid any possibility that the research study or the interpretation of results from the research study could be influenced by statements made about the research while the study is ongoing, parents are asked to refrain from communicating their child's participation in the study publicly so that researchers and government agencies can have an unbiased interpretation of the information collected.

ARE THERE ANY BENEFITS IF I PARTICIPATE?
The potential benefits of SRP-4045 and SRP-4053 in participants with DMD are unknown. Your child may experience some benefits from taking part in this study, but we cannot and do not promise that there will be any benefit. Your child will be assessed very intensively and this care may lead to earlier detection of a potential issue compared to standard of care. Even if your child does not benefit from being in this study, we might learn something that could help others.

WHAT OTHER CHOICES DO I HAVE IF I DON'T WANT TO PARTICIPATE?
Your child's participation in this study is voluntary. It is not necessary to be in this study to get care for this condition. If you decide not to allow your child to be in this study, the study doctor will talk to you about other possible treatments or refer you to your child's regular doctor for care.

HOW WILL INFORMATION ABOUT ME AND MY PARTICIPATION BE KEPT CONFIDENTIAL?
The researchers will do their best to make sure that your child's private information is kept confidential. Information about your child will be handled as confidentially as possible, but participating in research may involve a loss of privacy and the potential for

a breach in confidentiality. Study data will be physically and electronically secured. As with any use of electronic means to store data, there is a risk of breach of data security.

Use of personal information that can identify your child:

The researchers will make every attempt to protect your child's confidentiality and to make sure that his personal identity does not become known. This signed consent form will be stored in a locked file that will be accessible only to a very small number of authorized people involved in this project. The research team will carefully follow the coding, storage, and data sharing plan explained below.

You may need to sign a separate medical information release form so that those medical records can be transferred to [redacted]

People and agencies that will have access to your child's information:

The research team, authorized UCLA personnel, the study sponsor Sarepta Therapeutics Inc., the sponsor's designees, representatives, and agents (including third parties who are providing services to the sponsor related to the study), health insurance or other third party payers that may provide payment for care received in connection with the research study and regulatory agencies such as the Food and Drug Administration (FDA), may have access to study data and records to monitor the study. You will be asked to sign an additional form called the Permission to Use Personal Health Information for Research. This form lists additional people and agencies that may have access to your information and the permitted uses for that information. Research records provided to authorized, non-UCLA personnel will not contain identifiable information about you child. Publications and/or presentations that result from this study will not identify your child by name.

WILL I BE PAID FOR MY PARTICIPATION?

You will not be paid to be in this study. [redacted] You will be responsible for any medical care for your child's needs that is not provided as part of the research study.

ARE THERE ANY COSTS FOR TAKING PART IN THIS STUDY?

The study sponsor will supply and pay for the cost of supplying and administering the study drug and all research related laboratory tests and procedures.

However, your child and his insurer will be billed for the costs of any standard medical care he receives during his participation in the study and you will be responsible for any associated co-payments and deductibles. There is a possibility that your medical insurance company may not cover these costs because he is in a research study. If this happens you might have unexpected expenses from being in this study, such as the costs associated with treating side effects. Financial counseling and itemized cost estimates are available upon request.

UCLA Office of the Human Research Protection Program (OHRPP):
If you have questions about your rights while taking part in this study, or you have concerns or suggestions and you want to talk to someone other than the researchers about the study, you may contact the UCLA OHRPP by phone: [redacted]; by email: [redacted] or U.S. mail: [redacted].

Public Information about this Study:
ClinicalTrials.gov is a website that provides information about federally and privately supported clinical trials.

A description of this clinical trial will be available on http://www.ClinicalTrials.gov, as required by U.S. Law. This Web site will not include information that can identify you. At most, the Web site will include a summary of the results. You can search this Web site at any time.

WHAT HAPPENS IF I BELIEVE I AM INJURED BECAUSE I TOOK PART IN THIS STUDY?
It is important that you promptly tell the researchers if you believe that your child has been injured because of taking part in this study. You can tell the researcher in person or call him/her at the number listed above.

If your child is injured as a result of being in this study, UCLA will provide necessary medical treatment. The costs of the treatment may be covered by the University of California or the study sponsor Sarepta Therapeutics or billed to you or his insurer just like other medical costs, depending on a number of factors. The University and the study sponsor do not normally provide any other form of compensation for injury. For more information about this, you may call the UCLA Office of the Human Research Protection Program at [redacted] or send an email to [redacted].

WHO CAN I CONTACT IF I HAVE QUESTIONS ABOUT THIS STUDY?
You may contact [redacted] with any questions or concerns about the research or your participation in this study.

WHAT ARE MY RIGHTS IF I TAKE PART IN THIS STUDY?
Taking part in this study is your choice. You can choose whether or not you want your child to participate. Whatever decision you make, there will be no penalty to your child and he will not lose any of his regular benefits.

- You have a right to have all of your questions answered before deciding whether to take part.
- Your decision will not affect the medical care your child receives from UCLA.
- If you decide to allow your child take part, he can leave the study at anytime.
- If you decide to stop allowing him to participate in this study you should notify the research team right away. The researchers may ask you to complete some procedures in order to protect his safety.
- If you decide not to take part, your child can still get medical care from UCLA.

HOW DO I INDICATE MY AGREEMENT TO PARTICIPATE?
If you agree to allow your child participate in this study you should sign and date below. You have been given a copy of this consent form and the Research Participant's Bill of Rights to keep. You will be asked to sign a separate form authorizing access, use, creation, or disclosure of health information.

SIGNATURE OF THE PARTICIPANT (FOR AGES 13-17)

Name of Participant

Signature of Participant Date

SIGNATURE OF THE PARENT/GUARDIAN

Name of Parent/Guardian #1

Signature of Parent/Guardian #1 Date

Name of Parent/Guardian #2

Signature of Parent/Guardian #2 Date

SIGNATURE OF PERSON OBTAINING PERMISSION

Name of Person Obtaining Permission Contact Number

Signature of Person Obtaining Permission Date