EXONDYS 51 (eteplirsen)

AN FDA-APPROVED TREATMENT FOR DUCHENNE MUSCULAR DYSTROPHY

EXONDYS 51 is used to treat Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation in the dystrophin gene that can be treated by skipping exon 51. EXONDYS 51 was approved under accelerated approval. Accelerated approval allows for drugs to be approved based on a marker that is considered reasonably likely to predict a clinical benefit. EXONDYS 51 treatment increased the marker, dystrophin, in skeletal muscle in some patients. Verification of a clinical benefit may be needed for EXONDYS 51 to continue to be approved.

IMPORTANT RISK INFORMATION

Allergic reactions, including wheezing, chest pain, cough, rapid heart rate, and hives have occurred in patients who were treated with EXONDYS 51. Seek immediate medical care if signs and symptoms of allergic reactions occur.

Side effects that happened at least 25% more often in 8 patients treated with EXONDYS 51 by intravenous infusion than in 4 patients treated with an inactive intravenous infusion were problems with balance (38%, 0%), vomiting (38%, 0%), and skin irritation (25%, 0%). The most common side effects were problems with balance and vomiting.

The most common side effects seen in greater than 10% of patients receiving EXONDYS 51 (N=163) in other clinical trials were headache, cough, rash, and vomiting.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You may also report side effects to Sarepta Therapeutics at 1-888-SAREPTA (1-888-727-3782).

Please see the Important Risk Information above and the accompanying full Prescribing Information for EXONDYS 51 (eteplirsen).

DUCHENNE MUSCULAR DYSTROPHY: A PROGRESSIVE, MUSCLE-WASTING DISEASE

Duchenne muscular dystrophy, sometimes shortened to DMD or just Duchenne, is a rare genetic disease that affects mainly boys.

Duchenne is caused by a genetic mutation, or change, in the dystrophin gene. This change in the gene can either be inherited or occur spontaneously.

This mutation prevents the body from producing enough or any dystrophin, a protein that muscles need to work properly.

Without dystrophin, muscle cells become damaged and weaken over time.

Most muscles are affected, including those responsible for walking and raising arms.

Duchenne is progressive and irreversible.

Please see the Important Risk Information on the front cover and the accompanying full Prescribing Information for EXONDYS 51 (eteplirsen).

EXONDYS 51 HELPS THE BODY MAKE A SHORTER FORM OF THE DYSTROPHIN PROTEIN

EXONDYS 51 is an FDA-approved treatment for Duchenne muscular dystrophy in patients who have a genetic mutation of the dystrophin gene that is amenable to exon 51 skipping. Approximately 13% of people diagnosed with Duchenne have this type of mutation.

EXONDYS 51 was approved based on clinical trials that showed an increase in the amount of dystrophin protein found in the skeletal muscle of some patients. The increase in dystrophin is considered reasonably likely to predict a clinical benefit. Verification of a clinical benefit may be needed for EXONDYS 51 to continue to be approved.

MEET GRAYSEN.
GRAYSEN IS A BOY WITH DMD
(DELETION OF EXONS 48-50)

EXONDYS 51 has more than **5 years of real-world experience**

EXONDYS 51
(eteplirsen) Injection

HOW IT WORKS

EXONDYS 51 is an exon-skipping therapy. The goal of exon skipping is to allow the body to make a shorter form of the dystrophin protein. Let's take a closer look at how:

The dystrophin gene is the largest gene in the body, made up of 79 exons (portions of a gene) that are linked together to form the instructions for making dystrophin — a protein muscles need to work properly.

Think of the exons like toy train cars, each with a special connection that allows one car to connect to another. In order for all the cars to move together as a train, the connections between cars must match so that they can connect to one another.

Duchenne is caused by a genetic mutation, or change, in the dystrophin gene. Most commonly, one or more exons are missing. This causes errors in the instructions for making dystrophin, and the body is not able to produce enough or any working dystrophin protein.

Imagining the toy train, one or more cars would be missing, leaving the remaining cars not connected. In this example, we can see that car 50 is missing. This results in cars 49 and 51 not being able to connect.

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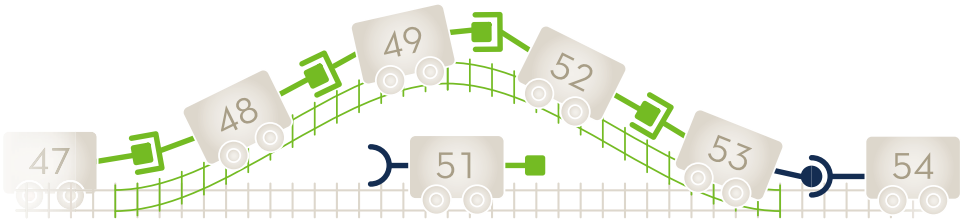
Exon-skipping technology allows the body to make dystrophin protein by skipping over a specific exon. EXONDYS 51 works using exon skipping, and the result is a shorter form of the dystrophin protein.

So with our train, we would move certain cars aside to "skip over" them so we could find a car with the right connection to allow the remaining cars to connect. In our example, car 51 would be skipped over to allow car 49 to connect to car 52.

This new train would be shorter, but all the cars would still be connected.



In some boys, weekly infusions of EXONDYS 51 have been shown to help the body make a shorter form of the dystrophin protein.

EXONDYS 51: APPROVED BY FDA UNDER ACCELERATED APPROVAL

When studying a new medicine, it can sometimes take many years to see whether it actually has an effect on how a patient survives, feels, or functions. There is a regulation called Accelerated Approval, which allows the FDA to approve medicines on a faster timeline based on what's called a "surrogate endpoint."

A surrogate endpoint is a marker of some kind, for instance, a laboratory measurement, specific test, physical sign, or radiographic image (like an X-ray). This marker is thought to predict a clinical benefit, but is not itself a measure of clinical benefit.

Accelerated approval applies to medicines that have been studied for safety and effectiveness in treating serious or life-threatening illnesses, and that provide a meaningful benefit to patients over existing medicines.

The FDA may grant accelerated approval for a medication based on clinical trials that are considered "adequate and well controlled," and show that the product has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit.

Accelerated approval also requires that, after approval, additional adequate and well-controlled studies, called confirmatory trials, are conducted to verify and describe the clinical benefit.

- **The increase of dystrophin protein in the skeletal muscle of some patients was the surrogate endpoint that supported accelerated approval of EXONDYS 51.**
- **The FDA concluded that an increase in dystrophin in skeletal muscle in some patients treated with EXONDYS 51 was reasonably likely to predict a clinical benefit.**

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POSSIBLE SIDE EFFECTS OF EXONDYS 51

Allergic reactions, including wheezing, chest pain, cough, rapid heart rate, and hives have occurred in patients who were treated with EXONDYS 51. Seek immediate medical care if signs and symptoms of allergic reactions occur.

The side effects that occurred at least 25% more often in 8 patients treated with EXONDYS 51 by intravenous infusion than in 4 patients who received a placebo were:

- Problems with balance (38%, 0%)
- Vomiting (38%, 0%)
- Skin irritation (25%, 0%)

The most common side effects were problems with balance and vomiting.

The most common side effects seen in greater than 10% of patients receiving EXONDYS 51 (N=163) in other clinical trials were headache, cough, rash, and vomiting.

Talk to your doctor if you experience any side effects. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You may also report side effects to Sarepta Therapeutics at 1-888-SAREPTA (1-888-727-3782).

TREATMENT WITH EXONDYS 51

As you get ready to start treatment with EXONDYS 51, be sure to discuss any questions you may have with your doctor. In addition, the information below will provide you with answers to some common questions you may have.

What is EXONDYS 51?

EXONDYS 51 is used to treat Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation in the dystrophin gene that can be treated by skipping exon 51. EXONDYS 51 was approved under accelerated approval. Accelerated approval allows for drugs to be approved based on a marker that is considered reasonably likely to predict a clinical benefit. EXONDYS 51 treatment increased the marker, dystrophin, in skeletal muscle in some patients. Verification of a clinical benefit may be needed for EXONDYS 51 to continue to be approved.

Who can take EXONDYS 51?

Patients who receive EXONDYS 51 must have a genetic test that shows a mutation in the dystrophin gene that can be treated by skipping exon 51. A healthcare provider is needed to interpret your genetic test to determine whether you can take EXONDYS 51.

Should I continue taking my other medications while on EXONDYS 51?

You should talk with your doctor about all the medications you are taking. Your doctor is the best person to advise you about your medicines.

How is EXONDYS 51 administered?

EXONDYS 51 is given by intravenous (IV) infusion once a week via an in-line 0.2 micron filter. An IV infusion is a way of delivering medicine directly into your bloodstream through a vein. Your doctor may discuss the use of a port, which is a device installed under the skin for repeat use in delivering IV medications. EXONDYS 51 infusion is always given and monitored by a healthcare provider.

How much EXONDYS 51 will I receive?

The amount of EXONDYS 51 you will be given is based upon how much you weigh. The recommended dosage of EXONDYS 51 is 30 milligrams per kilogram of body weight, once weekly.

Please see the Important Risk Information on the front cover and the accompanying full Prescribing Information for EXONDYS 51 (eteplirsen).

How long will my infusion last?

EXONDYS 51 will be intravenously infused over 35-60 minutes.

What happens if I miss an infusion?

If a dose of EXONDYS 51 is missed, it may be administered as soon as possible after the scheduled time. Talk to your doctor if you miss a dose.

Where will I get my infusion?

You may receive your infusions at your doctor's office, an infusion center, or your home. You and your doctor may need to discuss these options, including whether home therapy is an option for you.

QUESTIONS FOR MY DOCTOR

SAREPTASSIST

SUPPORT BY YOUR SIDE

PERSONALIZED SUPPORT STARTS HERE.

SareptAssist is a support program designed to help patients seeking information on EXONDYS 51 (eteplirsen). Our dedicated team will provide information on:

- Insurance benefits
- Financial assistance options
- Treatment logistics
- Options for weekly infusions
- Ongoing education and support

GET STARTED

For more information or to enroll in the program, call 1-888-SAREPTA (1-888-727-3782) or visit SareptAssist.com.

Case Managers are available Monday through Friday, 8:30 am – 6:30 pm ET.

Spanish-speaking Case Managers and interpreters for other languages are available.

Please see the Important Risk Information on the front cover and the accompanying full Prescribing Information for EXONDYS 51 (eteplirsen).

NAVIGATING THE PROCESS

Enrollment in SareptAssist

With your consent and signature, your doctor will complete and submit the SareptAssist START Form, which will begin the SareptAssist process. The START Form authorizes your Case Manager to start a benefits investigation to understand your current insurance benefits.

Welcome Call

Your dedicated Case Manager will reach out to welcome you and explain how they can help.

Benefits Investigation.

Your Case Manager will work with you to help you understand your insurance benefits and next steps. Depending on the type of insurance you have and your other relevant circumstances, your Case Manager will provide information on financial assistance options that you may be eligible for.

Treatment Location Options

You may receive your infusions at your doctor's office, an infusion center, or your home. You and your doctor may need to discuss these options, including whether home therapy is an option for you.

Starting EXONDYS 51

Once your insurance benefits have been confirmed, your Case Manager will work closely with the providing pharmacy (specialty or hospital pharmacy) to facilitate treatment access and coordinate drug delivery to your treatment location. The pharmacy will call you to schedule shipments of EXONDYS 51.

Ongoing Support

Your Case Manager is committed to working with you during your treatment journey, and will check in with you periodically. As your needs change (eg, you have new insurance, a change of address, are planning a vacation, etc.), your Case Manager can keep you informed of your options to help avoid treatment interruptions.

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SareptAssist Patient Support Program

Experienced and dedicated Case Managers who are here to help you during your treatment journey.

1-888-SAREPTA (1-888-727-3782)
Visit SareptAssist.com
Case Managers are available Monday through Friday, 8:30 am – 6:30 pm ET

Spanish-speaking Case Managers and interpreters for other languages are available. SareptAssist is a resource available only to those who have been prescribed EXONDYS 51. SareptAssist is only available in the US.

Please see the Important Risk Information on the front cover and the accompanying full Prescribing Information for EXONDYS 51 (eteplirsen).

SAREPTA THERAPEUTICS