VYONDYS 53
(golodirsen) 100 mg/2 mL injection

Luke, AGE 12
AMENABLE TO
EXON 53 SKIPPING

GETTING STARTED ON VYONDYS 53

VYONDYS 53 (golodirsen)
AN FDA-APPROVED TREATMENT

VYONDYS 53 is used to treat patients with Duchenne muscular dystrophy (DMD) who have a confirmed mutation in the dystrophin gene that can be treated by skipping exon 53.

This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with VYONDYS 53. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

IMPORTANT RISK INFORMATION

Allergic reactions, including rash, fever, itching, hives, and inflammation and/or peeling of the skin have occurred in patients who were treated with VYONDYS 53. Seek immediate medical care if signs and symptoms of allergic reactions occur.

Damage to the kidneys was seen in animals who received golodirsen. Although damage to the kidneys was not seen in clinical studies with VYONDYS 53, potentially fatal kidney damage has occurred with other drugs that work in a similar way. Your doctor may recommend urine and blood testing before starting treatment followed by urine testing every month and a blood test every 3 months to monitor your kidneys.

Adverse reactions that have occurred in at least 20% of patients treated with VYONDYS 53 and more often than in patients who received an inactive intravenous (IV) infusion were headache (41%, 10%), fever (41%, 14%), fall (29%, 19%), pain in the abdomen (27%, 10%), infection of the nose and throat (27%, 14%), cough (27%, 19%), vomiting (27%, 19%), and nausea (20%, 10%).

Other adverse reactions that occurred in greater than 5% of patients treated with VYONDYS 53 and more often than in patients who received an inactive IV infusion were pain at the IV site, back pain, pain, diarrhea, dizziness, stretch or tear in a ligament, bruising, flu, pain in the mouth and throat, stuffy or runny nose, scrapes or scratches of the skin, ear infection, seasonal allergy, fast heartbeat, reactions related to the IV catheter site, constipation, and broken bones.

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 full Prescribing Information for VYONDYS 53 (golodirsen).

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.

Duchenne is progressive and irreversible.

Please see the Indication and Important Risk Information on cover and on pages 8 and 9, and the accompanying full Prescribing Information for VYONDYS 53 (golodirsen).

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THE ROLE OF DYSTROPHIN PROTEIN

Dystrophin is a protein found in muscle cells

It acts as a shock absorber to the muscle

When there are mutations in the dystrophin gene, muscle cells are replaced with scar tissue and fat in a process called fibrosis

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

Nicholas, AGE 17
AMENABLE TO EXON 53 SKIPPING

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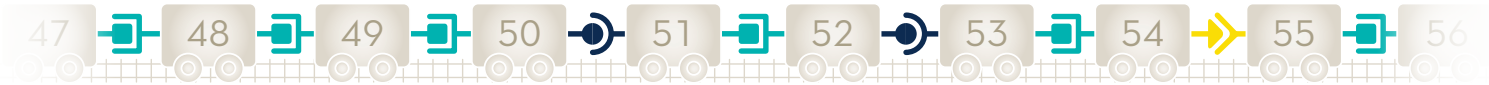
HOW IT WORKS

VYONDYS 53 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 cars 50–52 are missing. This results in cars 49 and 53 not being able to connect.

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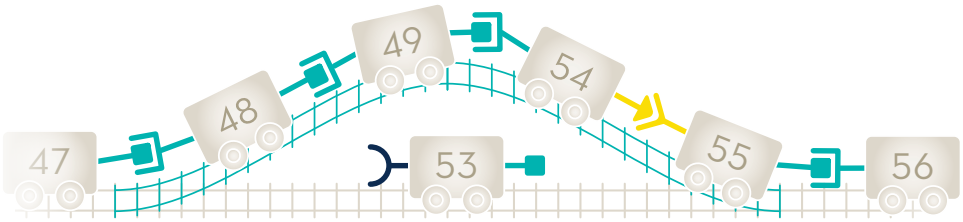
Exon skipping technology allows the body to make dystrophin protein by skipping over a specific exon. VYONDYS 53 works using exon skipping and the result is a shorter form of the dystrophin protein.

So with our train, we would move a certain car aside to"skip over" it so we could find a car with the right connection to allow the remaining cars to connect. In our example, car 53 would be skipped over to allow car 49 to connect to car 54.

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



Clinical studies of VYONDYS 53 tested whether exon skipping happened on the dystrophin gene of boys treated with the drug. In those studies, exon skipping occurred in all 25 evaluated study participants.

Boys who received VYONDYS 53 had variable responses in the amount of dystrophin production.

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VYONDYS 53:
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 new 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 to be conducted to verify and describe the clinical benefit.

- The presence of dystrophin protein in the skeletal muscle of patients was the surrogate endpoint that supported accelerated approval of VYONDYS 53.
- FDA approval under accelerated approval means that the surrogate, increased dystrophin in skeletal muscle, is reasonably likely to predict a clinical benefit.
- VYONDYS 53 has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

Elijah, AGE 8
AMENABLE TO EXON 53 SKIPPING

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POSSIBLE SIDE EFFECTS OF VYONDYS 53

Allergic reactions, including rash, fever, itching, hives, and inflammation and/or peeling of the skin have occurred in patients who were treated with VYONDYS 53. Seek immediate medical care if signs and symptoms of allergic reactions occur.

Damage to the kidneys was seen in animals who received golodirsen. Although damage to the kidneys was not seen in clinical studies with VYONDYS 53, potentially fatal kidney damage has occurred with other drugs that work in a similar way. Your doctor may recommend urine and blood testing before starting treatment followed by urine testing every month and a blood test every 3 months to monitor your kidneys.

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).

TABLE 1 ADVERSE REACTIONS OBSERVED IN AT LEAST 20% OF TREATED PATIENTS AND GREATER THAN PLACEBO WERE (VYONDYS 53, PLACEBO)

Adverse Reaction (%)	VYONDYS 53 | n=41	Placebo | n=21
Headache	41%	10%
Fever	41%	14%
Fall	29%	19%
Pain in abdomen	27%	10%
Infection of nose and throat	27%	14%
Cough	27%	19%
Vomiting	27%	19%
Nausea	20%	10%

Other adverse reactions that occurred in greater than 5% of patients treated with VYONDYS 53 and more often than in patients who received an inactive IV infusion were

- pain at the IV site
- back pain
- pain
- diarrhea
- dizziness
- stretch or tear in a ligament
- bruising
- flu
- pain in the mouth and throat
- stuffy or runny nose
- scrapes or scratches of the skin
- ear infection
- seasonal allergy
- fast heartbeat
- reactions related to the IV catheter site
- constipation
- broken bones

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TREATMENT WITH VYONDYS 53

As you get ready to start treatment with VYONDYS 53, 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 VYONDYS 53?

VYONDYS 53 is used to treat patients with Duchenne muscular dystrophy (DMD) who have a confirmed mutation in the dystrophin gene that can be treated by skipping exon 53.

This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with VYONDYS 53. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

Who can take VYONDYS 53?

Patients who receive VYONDYS 53 must have a genetic test that shows a mutation in the dystrophin gene that can be treated by skipping exon 53. A healthcare provider is needed to interpret your genetic test to determine whether you can take VYONDYS 53.

What allergic reactions have been reported with VYONDYS 53?

Allergic reactions, including rash, fever, itching, hives, and inflammation and/or peeling of the skin have occurred in patients who were treated with VYONDYS 53. Seek immediate medical care if signs and symptoms of allergic reactions occur.

Is there concern for kidney damage while on VYONDYS 53?

Damage to the kidneys was seen in animals who received golodirsen. Although damage to the kidneys was not seen in clinical studies with VYONDYS 53, potentially fatal kidney damage has occurred with other drugs that work in a similar way. Your doctor may recommend urine and blood testing before starting treatment followed by urine testing every month and a blood test every 3 months to monitor your kidneys.

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What are the most common side effects reported with VYONDYS 53?

Adverse reactions that have occurred in at least 20% of patients treated with VYONDYS 53 and more often than in patients who received an inactive intravenous (IV) infusion were headache (41%, 10%), fever (41%, 14%), fall (29%, 19%), pain in the abdomen (27%, 10%), infection of the nose and throat (27%, 14%), cough (27%, 19%), vomiting (27%, 19%), and nausea (20%, 10%).

Should I continue taking my other medications while on VYONDYS 53?

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 VYONDYS 53 supplied?

VYONDYS 53 is supplied in 2 mL single-dose vials containing 100 mg golodirsen (50 mg /mL). The solution is a clear to slightly opalescent, colorless liquid, and may contain trace amounts of small, white to off-white amorphous particles.

How much VYONDYS 53 will I receive?

The amount of VYONDYS 53 you will be given is based upon how much you weigh. The recommended dosage of VYONDYS 53 is 30 milligrams per kilogram of body weight, once weekly over 35-60 minutes intravenous (IV) infusion via in-line 0.2 micron filter.

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How long will my infusion last?

VYONDYS 53 will be intravenously infused over 35–60 minutes via an in-line 0.2 micron filter.

What happens if I miss an infusion?

If a dose of VYONDYS 53 is missed, it may be administered as soon as possible after the scheduled dose. 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.

How is VYONDYS 53 administered?

VYONDYS 53 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. VYONDYS 53 infusion is always given and monitored by a healthcare provider.

Are there any special considerations when using a port?*

Ask your doctor for any patient instructions provided by the maker of your port. Carefully follow these or other instructions provided by your doctor for care of your port site to reduce the risk of complications, including infections.

What kind of care is required when using a port?*

Your doctor or the maker of your port may recommend hygiene measures after placement of the port and after each use of the port. Carefully follow these and other instructions provided by your doctor.

When should I contact my doctor about my port?*

Follow instructions from the maker of your port and your doctor regarding when to contact your doctor. Always contact your doctor:

- If you notice any redness, tenderness, bruising, swelling, warmth or drainage at or near the injection site
- For a fever
- If there is swelling, tingling or pain at or near the port injection site or in the arm closest to the port.

**Always refer to the manufacturers instruction for use (IFU) guide for more information on safety and precautions and ask your health care provider to review the relevant instruction for use of your port with you.*

QUESTIONS FOR MY DOCTOR

For more information, visit VYONDYS53.com

SAREPTASSIST

SUPPORT, BY YOUR SIDE

PERSONALIZED SUPPORT STARTS HERE.

SareptAssist is a support program designed to help patients seeking information on VYONDYS 53 (golodirsen). 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.

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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

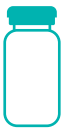
Your dedicated Case Manager will reach out to welcome you and explain how they can help.

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 VYONDYS 53

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 VYONDYS 53.

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 (e.g., 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 VYONDYS 53.
SareptAssist is only available in the U.S.

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SAREPTA THERAPEUTICS

08/21 C-SRP-53-US-0018