AMONDYS 45

(casimersen) 100 mg/2 mL injection

AN FDA-APPROVED TREATMENT

Colin, AGE 11
DELETION OF EXONS 12-44

GETTING STARTED ON AMONDYS 45

What is AMONDYS 45 (casimersen)?

AMONDYS 45 is used to treat patients with Duchenne muscular dystrophy (DMD) who have a confirmed mutation of the dystrophin gene that can be treated by skipping exon 45.

This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with AMONDYS 45. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

IMPORTANT SAFETY INFORMATION

Contraindications: Do not receive AMONDYS 45 if you are allergic to casimersen or any of the ingredients in AMONDYS 45. Serious allergic reactions to casimersen have included anaphylaxis, which may include difficulty breathing, tightness in the chest, and angioedema which may include swelling of the mouth, face, lips, or tongue.

Hypersensitivity Reactions: Serious allergic reactions, including angioedema and anaphylaxis, have occurred in patients who were treated with AMONDYS 45. Patients should seek immediate medical care should they experience signs and symptoms of allergic reactions. Your doctor will institute appropriate medical treatment which may include slowing, interrupting, or discontinuing the AMONDYS 45 infusion. Your doctor will monitor you until the condition resolves.

Kidney Toxicity and Monitoring: Damage to the kidneys was seen in animals who received casimersen. Although damage to the kidneys was not seen in clinical studies with AMONDYS 45, 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: Side effects occurring in at least 20% of patients treated with AMONDYS 45 and at least 5% more frequently than in patients who received an inactive intravenous (IV) infusion were (AMONDYS 45, placebo): upper respiratory tract infection (65%, 55%), cough (33%, 26%), fever (33%, 23%), headache (32%, 19%), joint pain (21%, 10%), and pain in mouth and throat (21%, 7%).

Other side effects that occurred in at least 10% of patients treated with AMONDYS 45 and at least 5% more frequently than patients who received an inactive IV infusion were: ear pain, nausea, ear infection, pain after injury, and dizziness and light-headedness.

What do I do if I have side effects?

Ask your healthcare provider for advice about any side effects that concern you.

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).

The information provided here does not include all that is known about AMONDYS 45. To learn more, talk with your healthcare provider.

Before receiving this infusion, please see the full Prescribing Information for AMONDYS 45 (casimersen).

DUCHENNE MUSCULAR DYSTROPHY: A PROGRESSIVE, MUSCLE-WEAKENING DISEASE

Duchenne muscular dystrophy, sometimes shortened to DMD or just Duchenne, is a rare genetic disease that can either be inherited or occur spontaneously.

Duchenne is caused by a genetic mutation, or change, in the dystrophin gene. This mutation prevents the body from producing enough or any dystrophin, a protein that muscles need to work properly.

Without dystrophin, muscle cells are damaged, and eventually, are replaced with scar tissue and fat in a process called fibrosis.

Levi, AGE 10
DELETION OF EXONS 46-51

Please see the Indication and Important Safety Information on cover and on pages 8 and 9, and the accompanying full Prescribing Information for AMONDYS 45 (casimersen).

AMONDYS 45
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Duchenne weakens the body's muscles over time. Once muscle tissue is weak or gone, it cannot be "fixed," which is why Duchenne is considered irreversible.

Early identification of DMD allows for **early intervention.**

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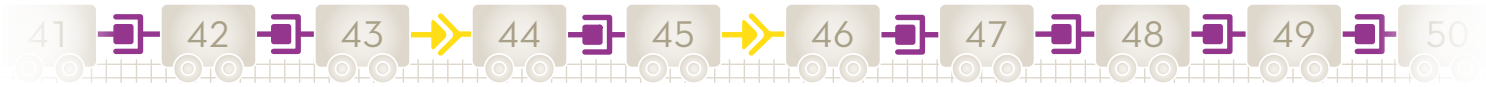
HOW IT WORKS

AMONDYS 45 is an exon-skipping therapy. The goal of exon skipping is to allow the body to make a shorter form of the dystrophin protein.

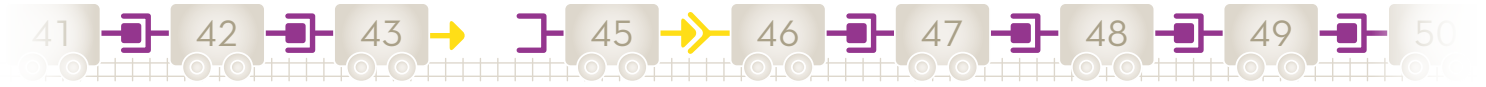
The dystrophin gene is the largest gene in the body, made up of 79 exons (portions of a gene) that are linked together to form the instructions for making dystrophin—a protein muscles need to work properly.

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 they can connect to one another.



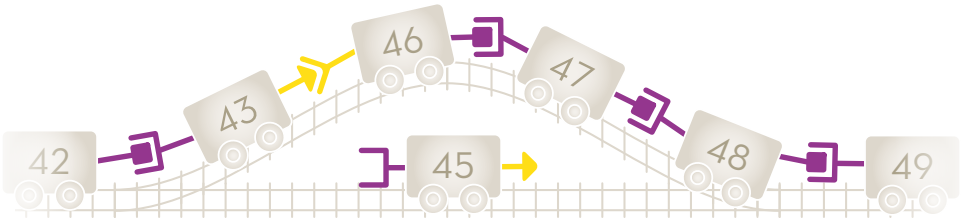
Duchenne is caused by a genetic mutation, or change, in the dystrophin gene. Most commonly, one or more exons are missing. This causes errors in the instructions for making dystrophin, and the body is not able to produce enough or any working dystrophin protein.

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 44 is missing. This results in cars 43 and 45 not being able to connect.



Exon-skipping technology allows the body to make dystrophin protein by skipping over a specific exon. AMONDYS 45 works using exon skipping, and the result is a shorter form of the dystrophin protein.

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 45 would be skipped over to allow car 43 to connect to car 46. This new train would be shorter, but all the cars would still be connected.



Boys who received AMONDYS 45 had variable responses in the amount of increased dystrophin production after 48 weeks of treatment. Data from an ongoing clinical study showed 27 boys (median age 9 years) who received AMONDYS 45 had an average increase of 1.74% of normal dystrophin production compared to 0.76% of normal production for boys who received a placebo infusion (n=16).

Exon skipping is intended to allow the body to make a **shorter form** of the **dystrophin protein.**

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AMONDYS 45 (casimersen) 100 mg/2 mL injection

WHAT IS AMONDYS 45?

AMONDYS 45 was approved by the FDA under accelerated approval for the treatment of Duchenne muscular dystrophy in patients who have a genetic mutation of the dystrophin gene that is amenable to exon 45 skipping. **Approximately 9% of people diagnosed with Duchenne have this type of mutation.**

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. Accelerated Approval allows the FDA to approve medicines on a faster timeline based on a "surrogate endpoint," which is a marker of some kind (eg, a laboratory measurement or specific test) 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 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.

Dystrophin in skeletal muscle

- The presence of dystrophin protein in the skeletal muscle of patients was the surrogate endpoint that supported accelerated approval of AMONDYS 45.
- FDA approval under accelerated approval means that increased dystrophin in skeletal muscle (surrogate endpoint) is reasonably likely to predict a clinical benefit.
- AMONDYS 45 has met the full statutory standards for safety and effectiveness and, as such, is not considered investigational or experimental.

Nicholas, AGE 12
DELETION OF EXON 44

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POSSIBLE SIDE EFFECTS OF AMONDYS 45

- Serious allergic reactions, including angioedema (swelling under the skin, which may include mouth, face, lips, or tongue) and anaphylaxis (a serious, potentially life-threatening allergic reaction), have occurred in patients who were treated with AMONDYS 45.
- Seek immediate medical care if signs and symptoms of allergic reactions occur. Your doctor will institute appropriate medical treatment which may include slowing, interrupting, or discontinuing the AMONDYS 45 infusion. Your doctor will monitor you until the condition resolves.
- Damage to the kidneys was seen in animals who received casimersen.
- Although damage to the kidneys was not seen in clinical studies with AMONDYS 45, 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.
- Infusion-related reactions including rash, headache, cough, abdominal pain (including upper abdominal pain), and vomiting occurred within 24 hours from the start of an infusion of AMONDYS 45.

Talk with your doctor about any side effects you may experience.

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SIDE EFFECTS OBSERVED IN AT LEAST 20% OF PATIENTS TREATED WITH AMONDYS 45 AND AT LEAST 5% MORE FREQUENTLY THAN IN THE PLACEBO GROUP

Adverse Reaction (%)	AMONDYS 45 (N=57)	Placebo (N=31)
Upper respiratory tract infections*	**65%**	**55%**
Cough	**33%**	**26%**
Fever	**33%**	**23%**
Headache	**32%**	**19%**
Joint pain	**21%**	**10%**
Pain in mouth and throat	**21%**	**7%**

*Includes upper respiratory infection, infection of nose and/or throat, and stuffy or runny nose

Other adverse reactions that occurred in at least 10% of patients treated with AMONDYS 45 and at least 5% more frequently than patients who received an inactive intravenous (IV) infusion were:

- Ear pain
- Nausea
- Ear infection
- Pain after injury
- Dizziness and light-headedness

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).

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TREATMENT WITH AMONDYS 45

As you prepare to start treatment with AMONDYS 45, be sure to discuss any questions you may have with your doctor. Below are answers to some commonly asked questions.

What is AMONDYS 45?

AMONDYS 45 is used to treat patients with Duchenne muscular dystrophy (DMD) who have a confirmed mutation of the dystrophin gene that can be treated by skipping exon 45.

This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with AMONDYS 45. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

Who can take AMONDYS 45?

Patients who receive AMONDYS 45 must have a genetic test that shows a mutation in the dystrophin gene that can be treated by skipping exon 45. A healthcare provider is needed to interpret your genetic test to determine whether you can take AMONDYS 45.

Who should not receive AMONDYS 45?

Do not receive AMONDYS 45 if you are allergic to casimersen or any of the ingredients in AMONDYS 45. Serious allergic reactions to casimersen have included anaphylaxis, which may include difficulty breathing and tightness in the chest, and angioedema, which may include swelling of the mouth, face, lips, or tongue.

What allergic reactions have been reported with AMONDYS 45?

Serious allergic reactions, including angioedema (swelling under the skin, which may include mouth, face, lips, or tongue) and anaphylaxis (a serious, potentially life-threatening allergic reaction), have occurred in patients who were treated with AMONDYS 45. Seek immediate medical care if signs and symptoms of allergic reactions occur. Your doctor will institute appropriate medical treatment which may include slowing, interrupting, or discontinuing the AMONDYS 45 infusion. Your doctor will monitor you until the condition resolves.

Is there concern for kidney damage while on AMONDYS 45?

Damage to the kidneys was seen in animals who received casimersen. Although damage to the kidneys was not seen in clinical studies with AMONDYS 45, 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.

What are the most common side effects reported with AMONDYS 45?

Side effects occurring in at least 20% of patients treated with AMONDYS 45 and at least 5% more frequently than in patients who received an inactive intravenous (IV) infusion were (AMONDYS 45, placebo): upper respiratory tract infection (65%, 55%), cough (33%, 26%), fever (33%, 23%), headache (32%, 19%), joint pain (21%, 10%), and pain in mouth and throat (21%, 7%).

Should other medications be continued while on AMONDYS 45?

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

How is AMONDYS 45 supplied?

AMONDYS 45 is supplied in 2 mL single-dose vials containing 100 mg casimersen (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 AMONDYS 45 will I receive?

The amount of AMONDYS 45 you will be given is based upon how much you weigh. The recommended dosage of AMONDYS 45 is 30 mg/kg of body weight, once weekly as a 35 to 60-minute intravenous (IV) infusion via an in-line 0.2 micron filter.

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How is AMONDYS 45 administered?

AMONDYS 45 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. AMONDYS 45 infusion is always given and monitored by a healthcare provider.

How long will my infusion last?

AMONDYS 45 will be intravenously infused over 35-60 minutes via an in-line 0.2 micron filter.

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.

What happens if I miss an infusion?

If a dose of AMONDYS 45 is missed, it may be administered as soon as possible after the scheduled dose. Talk to your doctor if you miss a dose.

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.

Always refer to the port manufacturer's instruction for use (IFU) guide for more information on safety and precautions, and ask your healthcare provider to review the relevant IFU of your port with you.

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 infusion site
- You experience swelling, tingling or pain at or near the port infusion site or in the arm closest to the port
- You develop a fever

**Always refer to the port manufacturer's instruction for use (IFU) guide for more information on safety and precautions, and ask your healthcare provider to review the relevant IFU of your port with you.*

QUESTIONS FOR MY DOCTOR

For more information, visit **AMONDYS45.com**

SAREPTASSIST

SUPPORT, BY YOUR SIDE

PERSONALIZED SUPPORT STARTS HERE.

SareptAssist is a support program designed to help patients seeking information on AMONDYS 45 (casimersen). 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 Indication and Important Safety Information on cover and on pages 8 and 9, and the accompanying full Prescribing Information for AMONDYS 45 (casimersen).

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NAVIGATING THE PROCESS

Enrollment in SareptAssist

With your consent and signature, your doctor will complete and submit the SareptAssist Enrollment Form, which will begin the SareptAssist process. The Enrollment 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 AMONDYS 45

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 AMONDYS 45.

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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AMONDYS 45

(casimersen) 100 mg/2 mL injection

Watch Beck's video to learn more about his experience with AMONDYS 45.

Meet Beck, age 14

Amenable to exon 45 skipping and receiving AMONDYS 45

GET STARTED TODAY

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 AMONDYS 45.
SareptAssist is only available in the US.

Please see the Indication and Important Safety Information on cover and on pages 8 and 9, and the accompanying full Prescribing Information for AMONDYS 45 (casimersen).

SAREPTA THERAPEUTICS

10/24 C-SRP-45-US-0034-V4