MDA Muscular Dystrophy Association

Treatment Fact Sheets For the Neuromuscular Community

The treatment landscape for neuromuscular disease is rapidly changing, with more than 20 new treatments approved in less than a decade and many more being researched. It is important for people living with neuromuscular diseases to understand the types of medications and treatments available so they can make informed decisions about their care.

This resource provides fact sheets about medications and treatments. They are meant to familiarize patients, caregivers, and families with medications and/or treatments their healthcare providers may recommend. The information provided is not exhaustive.

How to use this resource

Healthcare providers can print out relevant pages for a patient and use the "Notes From Your Healthcare Provider" section to provide individualized information.

Similarly, patients may print specific pages and take them to their healthcare provider to discuss. The decision to start a certain medication or pursue a specific treatment is individualized and should always be discussed with a qualified healthcare provider.

Additional Support

MDA's Resource Center provides education, one-on-one support, and resources for people living with neuromuscular diseases. Our Resource Specialists are available Monday through Friday, 9 a.m. to 5 p.m. CT, to answer questions and connect you with resources.

Phone: 833-ASK-MDA1 (833-275-6321) Email: ResourceCenter@mdausa.org

Duchenne Muscular Dystrophy (DMD)

Name: aGamree® (vamorolone)

Pronunciation: *ah-gahm-ree*

How does it work? It works to reduce inflammation by blocking the production of proteins (cytokines) that cause inflammation.

Ages treated: 2 and older

What does it look like? It is an oral suspension.

How is it given? The usual dosage is 6 mg/kg taken orally once daily, preferably with a meal, up to a maximum daily dosage of 300 mg for patients weighing more than 50 kg.

Possible side effects*: Cushingoid features, psychiatric disorders, vomiting, weight gain, vitamin D deficiency, immunosuppression, increased risk of infection, change in cardiovascular and kidney function, increase in blood pressure and water retention, osteoporosis, cataracts, glaucoma, muscle weakness, blood clots, Kaposi's sarcoma

Patient assistance program information:
Catalyst Pathway Program
Visit **yourcatalystpathways.com** or call **833-422-8259**.

Notes From Your Healthcare Provider

Prescriber: _____

Contact info: _____

Specific instructions: _____

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Duchenne Muscular Dystrophy (DMD)

Name: Amondys 45 (casimersen)

Pronunciation: *ah-mon-dis 45*

How does it work?

- Exon skipping therapy that can help the body make a shorter form of the dystrophin protein
- For individuals who have a confirmed genetic mutation in the dystrophin gene that can be treated by skipping exon 45
- Can decrease muscle weakness

Ages treated: Patients of any age who have a confirmed mutation of the dystrophin gene that is amenable to exon 45 skipping

What does it look like? It is a liquid in a plastic bag, which is connected to a small tube to deliver it directly into a patient's bloodstream through a vein.

How is it given? Amondys 45 is given via intravenous (IV) infusion. Some patients may have a device called a port installed under their skin for repeat infusions.

Possible side effects*: Upper respiratory tract infection, cough, fever, headache, joint pain, pain in mouth and throat

Patient assistance program information:
SareptAssist
Visit **sarepta.com/sareptassist** or call **888-SAREPTA (888-727-3782)**.

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Duchenne Muscular Dystrophy (DMD)

Name: Elevidys (delandistrogene moxeparvovec-rokl)

Pronunciation: *el-ev-e-dis*

How does it work?

- First gene therapy for DMD designed to target the underlying cause of the disease
- Uses an adeno-associated virus (AAVrh74) to introduce a shortened version of the dystrophin gene (mini-dystrophin) into muscle tissue of boys with DMD, partially compensating for their lack of a functional dystrophin gene and addressing the underlying genetic defect that causes DMD

Ages treated: For information regarding eligibility criteria, visit elevidys.com/treatment-journey/eligibility.

What does it look like? It is a liquid in a plastic bag, which is connected to a small tube to deliver it directly into a patient's bloodstream through a vein.

How is it given? Elevidys is given as a single-dose intravenous (IV) infusion. Several steps are required before and after the infusion day, including ongoing monitoring.

Possible side effects*: Vomiting, acute serious liver injury, immune-mediated myositis (inflammation of muscles) and myocarditis (inflammation of the heart), nausea, increased liver function tests, fever, decreased platelet counts

Patient assistance program information:
SareptAssist
Visit **sarepta.com/sareptassist** or call **888-SAREPTA (888-727-3782)**.

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Duchenne Muscular Dystrophy (DMD)

Name: Emflaza™ (deflazacourt)

Pronunciation: *em-flah-zah*

How does it work?

- Acts as an anti-inflammatory and immunosuppresive agent
- Converted by the body into an active metabolite called 21-desDFZ
- Active form of the drug helps decrease inflammation and suppress the immune system

Ages treated: 2 years and older

What does it look like? It can be prescribed as an oral tablet or an oral suspension.

How is it given? In tablet or liquid form, Emflaza can be taken with or without food. Dosing is based on the patient's weight. When taking liquid Emflaza, it should be mixed with 3-4 oz of milk or juice. Do not mix Emflaza with grapefruit or grapefruit juice.

Possible side effects*: Cushingoid appearance, weight gain, increased appetite, cough, frequent urination, upper respiratory tract infection, unwanted hair growth, weight gain, skin redness, abdominal discomfort

Patient assistance program information:
PTC Cares
Visit ptccares.com or call 844-478-2227.

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Duchenne Muscular Dystrophy (DMD)

Name: Exondys 51 (eteplirsen)

Pronunciation: *ex-on-dis 51*

How does it work?

- Exon skipping therapy that can help the body make a shorter form of the dystrophin protein
- For individuals who have a confirmed genetic mutation in the dystrophin gene that can be treated by skipping exon 51
- Can decrease muscle weakness

Ages treated: Any age with a confirmed mutation in the dystrophin gene that can be treated by skipping exon 51

What does it look like? It is a liquid in a plastic bag, which is connected to a small tube to deliver it directly into a patient's bloodstream through a vein.

How is it given? Exondys 51 is given via weekly intravenous (IV) infusions, monitored by a medical professional. It is typically infused at home, but can be administered at an infusion center.

Possible side effects*: Allergic reactions, including wheezing, chest pain, cough, rapid heart rate, hives

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Duchenne Muscular Dystrophy (DMD)

Name: Prednisone (prednisolone)

Pronunciation: *pred-nih-zone*

How does it work?

- Corticosteroid medicine, which can calm an overactive immune system
- When prescribed in certain doses, can help reduce and ease symptoms of inflammation

Ages treated: Consult with your doctor.

What does it look like? Prednisone can be prescribed in a tablet, liquid, or other form. Appearance differs depending on the method of delivery, dosage, vendor, and other factors.

How is it given? Prednisone can be taken in a variety of ways. Take prednisone exactly as prescribed by your doctor, and follow all directions on your prescription label. Take prednisone with food. Your dosage needs may change based on health needs and/or provider adjustments.

Possible side effects*: Sleep problems (insomnia), mood changes, increased appetite, gradual weight gain, acne, increased sweating, dry or thinning skin, bruising or discoloration, slow wound healing, headache, dizziness, nausea, stomach pain

Patient assistance program information:
Prednisone is available as a generic drug and is typically covered by most Medicare and insurance plans.

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Duchenne Muscular Dystrophy (DMD)

Name: Viltepso® (viltolarsen)

Pronunciation: *vil-tep-so*

How does it work?

- Exon skipping therapy that can help the body make its own, shortened dystrophin protein
- For individuals who have a confirmed genetic mutation in the dystrophin gene that can be treated by skipping exon 53
- Helps the body make dystrophin, which can decrease muscle weakness and help a person move and flex their muscles

Ages treated: Patients of any age who have a confirmed mutation of the dystrophin gene that is amenable to exon 53 skipping

What does it look like? It is a liquid in a plastic bag, which is connected to a small tube to deliver it directly into a patient's bloodstream through a vein.

How is it given? Viltepso is given via weekly intravenous (IV) infusions, monitored by a medical professional. It is typically infused at home but can be administered at an infusion center.

Possible side effects*: Side effects may include upper respiratory tract infection, injection site reaction, cough, and fever. In clinical studies, no patients experienced kidney toxicity during treatment, however, kidney toxicity may be possible. Your doctor may monitor the health of your kidneys before and during treatment.

Patient assistance program information:
NSSupport
Visit **viltepso.com/support** or call **833-NSSUPRT (833-677-8778)**.

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Duchenne Muscular Dystrophy (DMD)

Name: Vyondys 53 (golodirsen)

Pronunciation: *vie-on-dis 53*

How does it work?

- Exon skipping therapy that can help the body make a shorter form of the dystrophin protein
- Can decrease muscle weakness and extend the length of time individuals with DMD can walk, eat independently, and breathe without assistance

Ages treated: All patients who have a confirmed genetic mutation in the dystrophin gene that can be treated by skipping exon 53

What does it look like? It is a liquid in a plastic bag, which is connected to a small tube to deliver it directly into a patient's bloodstream through a vein.

How is it given? Vyondys 53 is given via weekly intravenous (IV) infusions, monitored by a medical professional. It is typically adinistered at an infusion center. Home infusions can be done under the supervision of a medical professional.

Possible side effects*: Allergic reactions, including rash, fever, itching, hives, inflammation and/or peeling of the skin

Patient assistance program information:
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