

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 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

MDA aims to make the information in these fact sheets available for informational purposes only. MDA does not endorse any brands, services, or products, and the inclusion of any therapy in these fact sheets does not constitute an endorsement by MDA. Please talk to your medical advisor to obtain more information about these treatments, as a healthcare provider should administer any therapy or practice described in these fact sheets in accordance with professional standards of care in light of the unique circumstances of each patient’s situation.

MDA has sought to make these fact sheets as accurate and up-to-date as possible. However, the information in them was extracted from manufacturers’ guidelines, and MDA is not responsible for any errors in such guidelines. Furthermore, as new scientific information becomes available, recommendations regarding treatments and therapies may change.

Spinal Muscular Atrophy (SMA)

Name: Evrysdi® (risdiplam)

Pronunciation: ev-ris-dee

How does it work?

- Designed to help make and maintain more SMN protein via effects on mRNA alternative splicing
- May help improve or preserve motor function

Ages treated: Adults, children, and infants aged 2 months and older

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

How is it given? It is taken once a day by mouth or feeding tube after a meal. It can be administered at home.

Possible side effects*: Side effects may include fever, diarrhea, and rash. For infantile-onset SMA, they may also include runny nose, sneezing, sore throat (upper respiratory infection), lung infection (lower respiratory infection), constipation, vomiting, or cough.

Patient assistance program information:

MySMA Support

Visit evrysdi.com/resources/support-for-you.html or call **833-387-9734**.

Notes From Your Healthcare Provider

Prescriber: _____

Contact info: _____

Specific instructions: _____

*Not all of the possible side effects of this medicine and precautions related to taking it are covered in this information sheet. For a complete list of side effects and precautions, ask your healthcare professional (doctor, nurse, pharmacist) for a manufacturer's package insert or another reference. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit fda.gov/medwatch or call **800-FDA-1088**.

DISCLAIMER: This document is meant to inform and educate the community. The information presented is not intended to replace discussions with your healthcare provider and should not be considered medical advice. Please consult with your healthcare team, medication manufacturer, and insurance company for information specific to you.

Spinal Muscular Atrophy (SMA)

Name: Evrysdi® (risdiplam) tablet

Pronunciation: ev-ris-dee

How does it work?

- Designed to help make and maintain more SMN protein via effects on mRNA alternative splicing
- May help improve or preserve motor function

Ages treated: For people 2 years of age and older who weigh more than 44 lbs.

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

How is it given? It is taken by mouth, swallowed whole or dispersed in non-chlorinated drinking water. In April 2026, Evrysdi was approved to be administered via nasogastric (NG) or gastrostomy (G) feeding tubes (after dispersion in water).

Possible side effects*: Side effects may include fever, diarrhea, and rash. For infantile-onset SMA, they may also include runny nose, sneezing, sore throat (upper respiratory infection), lung infection (lower respiratory infection), constipation, vomiting, or cough.

Patient assistance program information:

MySMA Support

Visit evrysdi.com/resources/support-for-you.html or call **833-387-9734**.

Notes From Your Healthcare Provider

Prescriber: _____

Contact info: _____

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Spinal Muscular Atrophy (SMA)

Name: ITVISMA[®] (onasemnogene abeparvovec-brve)

Pronunciation: zohl-jenz-muh

How does it work?

- Gene therapy designed to deliver a new copy of the survival of motor neuron 1 (SMN1) gene to produce SMN protein
- Results in long-term production of the SMN protein
- Improves muscle function and survival

Ages treated: Children 2 years and older, teens, and adults with a confirmed mutation in the SMN1 gene.

What does it look like? Liquid in a vial

How is it given? ITVISMA[®] is delivered directly to the fluid surrounding the spinal cord and brain using a spinal tap (intrathecal injection).

Possible side effects*: Most common adverse reactions include upper respiratory tract infection, fever, upper gastrointestinal symptoms, increased liver enzymes, headache, dizziness, pain in extremity, low platelet counts, and sensory disturbance. ITVISMA[®] can increase liver enzymes and cause hepatotoxicity. Frequent monitoring is important.

Patient assistance program information:

Novartis Patient Support

Visit itvisma.com/support/novartis-patient-support or call **855-441-4363**.

Notes From Your Healthcare Provider

Prescriber: _____

Contact info: _____

Specific instructions: _____

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Spinal Muscular Atrophy (SMA)

Name: SPINRAZA® (nusinersen)

Pronunciation: spin-rah-zah

How does it work?

- Antisense oligonucleotide (ASO) that binds to a specific sequence of the SMN2 gene to increase production of functional SMN protein
- May help improve or preserve motor function by targeting the underlying cause of muscle weakness

Ages treated: All ages

What does it look like? It is a clear liquid.

How is it given? Spinraza® is delivered directly to the fluid surrounding the spinal cord and brain using a spinal tap (intrathecal injection). After four initial loading doses, Spinraza is given three times a year. In March 2026, the FDA approved a high-dose regimen for Spinraza®.

Possible side effects*: Lower respiratory infection, fever, constipation, headache, vomiting, back pain, and post-lumbar puncture headache

Patient assistance program information:

SMA360°

Visit spinraza.com/en_us/home/support-and-events/sma360.html.

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Spinal Muscular Atrophy (SMA)

Name: ZOLGENSMA® (onasemnogene abeparvovec-xioi)

Pronunciation: zohl-jenz-muh

How does it work?

- Gene therapy designed to deliver a new copy of the survival of motor neuron (SMN) gene to produce SMN protein
- Results in long-term production of the SMN protein
- Improves muscle function and survival

Ages treated: Children under 2 years old with genetically confirmed SMA and up to four copies of the SMN2 gene.

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? Zolgensma is delivered via a one-time intravenous (IV) infusion

Possible side effects*: Side effects may include nausea, vomiting, and liver damage. Other immune reactions have led to several reported fatalities.

Patient assistance program information:

OneGene Program

Visit zolgensma.com/onegene-program or call **855-441-GENE (855-441-4363)**.

Notes From Your Healthcare Provider

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Contact info: _____

Specific instructions: _____

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