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

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: _____

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*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**.

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.

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: _____

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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. After four initial loading doses, Spinraza® is given three times a year.

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**.

Notes From Your Healthcare Provider

Prescriber: _____

Contact info: _____

Specific instructions: _____

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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

Prescriber: _____

Contact info: _____

Specific instructions: _____

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