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

Amyotrophic Lateral Sclerosis (ALS)

Name: Exservan™ (riluzole) oral film

Pronunciation: *ex-ser-van*

How does it work?

- Prolongs survival by blocking the release of glutamate (neurotransmitter that, in excess levels, can damage nerve cells)
- Acts to increase survival duration

What does it look like? The oral film form is rectangular with a honey lemon flavor. Each film is individually packaged in a foil pouch.

How is it given? The oral film dissolves on top of the tongue. It does not require water, making it an option for those who have difficulty swallowing. It should be taken at least one hour before or two hours after a meal.

Possible side effects*: Liver problems, serious lung problems (interstitial lung disease), numbness in the mouth or tongue, muscle weakness, nausea, lung problems, high blood pressure, stomach (abdominal) pain

Patient assistance program information:
Visit **exservan.com**.

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**.

Amyotrophic Lateral Sclerosis (ALS)

Name: Qalsody® (tofersen)

Pronunciation: *cal-sod-ee*

How does it work?

- Works with ALS caused by a mutation in the superoxide dismutase 1 (*SOD1*) gene (called SOD1-ALS)
- Antisense oligonucleotide (ASO) designed to bind to *SOD1* RNA to prevent the body from producing new SOD1 protein

What does it look like? It is a clear solution in a syringe prepared at the time of dosing.

How is it given? It is administered by an injection into the fluid that surrounds the spinal cord and brain (cerebrospinal fluid) using a spinal tap (lumbar puncture) in the lower back. After an initial dosing period, Qalsody® is administered approximately every 28 days.

Possible side effects*: Pain at the injection site, pain or numbness in arms or legs, persistent headache, increased white blood cell count, increased protein in the cerebrospinal fluid

Patient assistance program information:
Biogen Support Services
Visit **qalsody.com/en-us/home/support-and-resources.html** or call **877-725-7639**.

Notes From Your Healthcare Provider

Prescriber: _____

Contact info: _____

Specific instructions: _____

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Amyotrophic Lateral Sclerosis (ALS)

Name: Radicava ORS® (edaravone) oral suspension

Pronunciation: *rah-dih-cah-vah*

How does it work?

- Reported to work by relieving the effects of oxidative stress, which may be related to the death of motor neurons (nerve cells) in people with ALS
- Is thought to neutralize free radicals (unstable molecules) that can damage nerve cells

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

How is it given? Radicava ORS® is a liquid. Typical dosing is 5 ml, given by mouth or feeding tube. Patients or caregivers should dose the medication with a syringe to ensure the correct amount. It should be taken in the morning with water on an empty stomach, waiting one hour before consuming food. After an initial dosing period, it is given for 10 consecutive days every 28 days.

Possible side effects*: Allergic reaction, bruising, headache, problems balancing or walking, bronchospasm (tightening of the airways), lightheadedness/fainting

Patient assistance program information:
JourneyMate Support Program
Visit **radicava.com/patient/journeymate/** or call **855-457-6968**.

Notes From Your Healthcare Provider

Prescriber: _____

Contact info: _____

Specific instructions: _____

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Amyotrophic Lateral Sclerosis (ALS)

Name: Rilutek® (riluzole)

Pronunciation: *ril-yoo-tek*

How does it work? It prolongs survival by blocking the release of glutamate (a neurotransmitter that, in excess levels, can damage nerve cells).

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

How is it given? Take this medication by mouth on an empty stomach (at least one hour before or two hours after a meal) as directed by your doctor, usually twice daily.

Possible side effects*: Dizziness, drowsiness, nausea, vomiting, numbness/tingling around the mouth

Patient assistance program information:
People diagnosed with ALS typically have insurance coverage for generic Rilutek® through their prescription drug benefit plan. Copays or other pharmacy fees may not be covered. Visit **GoodRx.com** or **healthwellfoundation.org** for support.

Notes From Your Healthcare Provider

Prescriber: _____

Contact info: _____

Specific instructions: _____

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Amyotrophic Lateral Sclerosis (ALS)

Name: Tiglutik® (riluzole) oral suspension

Pronunciation: *tig-la-tick*

How does it work?

- Prolongs survival by blocking the release of glutamate (a neurotransmitter that, in excess levels, can damage nerve cells)
- Active ingredient (riluzole) has been shown to prolong survival

What does it look like? It is a mildly thick oral liquid.

How is it given? Tiglutik is administered twice daily by mouth or through a feeding tube and given on an empty stomach. Gently shake the bottle for at least 30 seconds by continuously turning the bottle up and down until the Tiglutik suspension is mixed well and you do not see any clear liquid at the top of the suspension or any particles at the bottom of the bottle. Using a syringe is recommended to ensure accurate dosing.

Possible side effects*: Numbness/tingling around the mouth, weakness, nausea, decreased lung function, high blood pressure, abdominal pain

Patient assistance program information:
Visit **tiglutik.com/helping-patients-get-tiglutik/** or call **901-201-5470**.

Notes From Your Healthcare Provider

Prescriber: _____

Contact info: _____

Specific instructions: _____

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