

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

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.

Friedreich Ataxia (FRDA or FA)

Name: Skyclarys (omaveloxolone)

Pronunciation: *sky-clair-is*

How does it work?

- Contains omaveloxolone, an activator of the nuclear factor-erythroid 2 related factor 2 (Nrf2) pathway, which is suppressed in people with FRDA
- Is thought to reduce the inflammatory response and improve mitochondrial and neurological function by activating the Nrf2 pathway

Ages treated: Adults and children aged 16 years and older

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

How is it given? Skyclarys is an oral medication given once daily.

Possible side effects*: Increase in blood liver enzymes, increase in a blood protein called B-type natriuretic peptide (BNP), changes in cholesterol levels, headache, nausea, stomach pain, tiredness, diarrhea, muscle pain

Patient assistance program information:

REACH

Visit biogenreach.com or call **844-98-REACH**.

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.