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.

Dermatomyositis (DM)

Name: Octagam® 10% (immune globulin intravenous human)

Pronunciation: *oct-ah-gam*

How does it work?

- Sterilized solution made from human plasma that contains antibodies to help the body protect itself against infection from various diseases
- Helps improve muscle function and skin rash

Ages treated: Adults (18 years and older)

What does it look like? Octagam® 10% is a clear to pale yellow, colorless-to-pale-yellow liquid solution supplied in a glass vial.

How is it given? Octagam® 10% is given by IV infusion through a needle inserted into your vein once daily over 2 to 5 consecutive days every 4 weeks. Each infusion takes roughly 4-5 hours. This may be done at home, in a hospital, or at an infusion center, but it is always done by a healthcare professional.

Possible side effects*: Common side effects may include headache, fever, nausea, vomiting, chills, muscle or joint pain, increased blood pressure or heart rate, shortness of breath, and infusion-site reactions.Octagam® 10% may increase the risk of blood clots and kidney problems, including acute kidney failure. Serious allergic reactions and other complications can also occur. Your healthcare team may monitor your kidney function and assess your risk factors before and during treatment.

Patient assistance program information: Octagam® 10% Co-Pay Program Website: PfizerIGuide.com or Call 1-844-448-4337. The free Ig Companion App helps users navigate infusions, track and export infusion information, access educational content, and receive event reminders.

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.

Dermatomyositis (DM)

Name: Lisraya® (brepocitinib)

Pronunciation: liz-RAY-uh (brep-oh-SIH-ti-nib)

How does it work?

- Lisraya® is a targeted dual TYK2 and JAK1 inhibitor that blocks key inflammatory pathways involved in autoimmune inflammation. It may help improve muscle strength, overall physical function, and skin rash while reducing the need for systemic steroids.

Ages treated: Adults (18 years and older)

What does it look like? Lisraya® is a pink, capsule-shaped tablet. The 30 mg tablet is film-coated and has “BT30” printed on one side.

How is it given? Lisraya® is taken orally (by mouth) as a once-daily tablet, with or without food. Follow all directions on your prescription label and take it consistently at the same time each day.

Possible side effects*: Side effects may include upper respiratory tract infections, headache, nausea, acne, elevated liver enzymes, or diarrhea. Because it affects the immune system, it may increase the risk of serious infections.

Patient assistance program information:
My Compass Support To enroll visit their website: https://lisraya.com/patient-support-enrollment, text LISRAYA to 37538 or call 1-888-736-9788

Notes From Your Healthcare Provider

Prescriber: _____

Contact info: _____

Specific instructions: _____

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