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.

Generalized Myasthenia Gravis (gMG)

Name: IMAAVY™ (nipocalimab-aahu)

Pronunciation: im-ah-vee

How does it work?

- Decreases levels of immunoglobin G (IgG), including harmful IgG autoantibodies, by blocking the FcRn receptor
- May improve essential daily functions such as chewing, swallowing, speaking, and breathing

Ages treated: People 12 years of age and older with gMG who are anti-acetylcholine receptor (AChR) or anti-muscle-specific tyrosine kinase (MuSK) antibody positive

What does it look like? It is a liquid in a single-dose vial.

How is it given? It is administered via intravenous (IV) infusion over about 30 minutes. It is usually administered every two weeks.

Possible side effects*: Respiratory tract infections, peripheral edema (swelling in hands, ankles, or feet), and muscle spasms. People who are being treated with Imaavy should not receive live vaccines.

Patient assistance program information:
Imaavy With Me
Visit **imaavy.com/generalized-myasthenia-gravis/** or call **844-4withMe (844-494-8463)**.

Notes From Your Healthcare Provider

Prescriber: _____

Contact info: _____

Specific instructions: _____

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Generalized Myasthenia Gravis (gMG)

Name: RYSTIGGO® (rozanolixizumab-noli)

Pronunciation: rye-stig-oh

How does it work?

- It targets neonatal Fc receptor (FcRn) and blocks it from extending the life of these harmful antibodies.
- These antibodies are broken down in the cell and sent to the body's disposal system.

Ages treated: Adult patients diagnosed with gMG who are anti-acetylcholine receptor (AChR) or anti-muscle-specific tyrosine kinase (MuSK) antibody positive

What does it look like? It is a liquid in a syringe.

How is it given? Subcutaneous (under the skin) injections are given once weekly in six-week cycles. It is administered by a medical professional at an infusion center or doctor's office. Some individuals may be able to receive treatment at home with nursing assistance.

Possible side effects*: Headache, infections, diarrhea, fever, hypersensitivity reactions, nausea

Patient assistance program information:
ONWARD
Visit **ucbonward.com/RYSTIGGO** or call **844-669-2731.**

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Generalized Myasthenia Gravis (gMG)

Name: Soliris (eculizamab)

Pronunciation: so-leer-is

How does it work?

- In gMG, muscle function is lost when a faulty immune response mistakenly attacks muscle cells. This is activated in part by a key protein called C5.
- Soliris blocks the C5 protein.

Ages treated: People 6 years of age and older with gMG who are anti-acetylcholine receptor (AChR) antibody positive

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? A healthcare provider will administer Soliris through an intravenous (IV) infusion, usually over 35 minutes in adults and one to four hours in children. Adults will usually receive an infusion weekly for five weeks, then every two weeks. For children under 18, their healthcare provider will decide how often they will receive Soliris depending on their age and body weight.

Possible side effects*: Common side effects include muscle and joint pain. It may lower the immune system's ability to fight infections, and it increases the chance of getting serious meningococcal infections caused by Neisseria meningitidis bacteria. Meningococcal infections may quickly become life-threatening or cause death if not recognized and treated early.

Patient assistance program information: Visit **alexiononesource.com/soliris,** call **888-765-4747,** or email **OneSource@Alexion.com**

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Generalized Myasthenia Gravis (gMG)

Name: ULTOMIRIS® (ravulizumab-cwvz)

Pronunciation: ult-oh-meer-is

How does it work?

- In gMG, muscle function is lost when a faulty immune response mistakenly attacks muscle cells. This is activated in part by a key protein called C5.
- Ultomiris blocks the C5 protein.

Ages treated: Adults with anti-acetylcholine receptor (AChR) antibody-positive gMG

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? Typically given once every eight weeks (six to seven times per year) via intravenous (IV) infusion. Each infusion typically takes less than one hour.

Possible side effects*: Common side effects include diarrhea and upper respiratory tract infections. Other possible side effects include increased risk of infection and infusion-related reactions, such as lower back pain, abdominal pain, muscle spasms, changes in blood pressure, tiredness, feeling faint, shaking chills (rigors), discomfort in your arms or legs, bad taste, or drowsiness.

Patient assistance program information:
Alexion OneSource
Visit **alexiononesource.com/ultomiris,** call **888-765-4747,** or email **OneSource@Alexion.com.**

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Generalized Myasthenia Gravis (gMG)

Name: UPLIZNA® (inebilizumab-cdon)

Pronunciation: up-liz-na

How does it work?

- CD19-Targeted B-cell therapy
- It works by targeting and depleting CD19 B+ cells, reducing harmful antibodies.

Ages treated: Adults with gMG who are anti-acetylcholine receptor (AChR) antibody positive and anti-muscle specific tyrosine kinase (MuSK) antibody positive.

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? Uplizna is given via intravenous (IV) infusion twice per year after two initial loading doses.

Possible side effects*: Most common side effects in patients with gMG were headaches and infusion-related reactions. Infusion-related reactions may include headache, nausea, sleepiness, shortness of breath, muscle aches, rash, palpitations, and even severe reactions like anaphylaxis. Uplizna may cause low blood cell counts and may increase the risk of infection.

Patient assistance program information:
Amgen By Your Side
Visit **amgenbyyourside.com** or call **844-469-4297**

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Generalized Myasthenia Gravis (gMG)

Name: VYVGART ® (efgartigimod alfa-fcab)

Pronunciation: viv-gart

How does it work?

- It binds to FcRn and prevents the recycling of the antibodies that cause MG.
- These antibodies are broken down in the cell and sent to the body's disposal system.

Ages treated: Adults with anti-acetylcholine receptor (AChR) antibody-positive gMG

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? Vyvgart is administered via intravenous (IV) infusion. Treatment cycles are typically one treatment each week for four weeks with a personalized break between cycles.

Possible side effects*: Increased risk of infection, such as urinary tract and respiratory tract infections, allergic reactions, infusion-related reactions, headache

Patient assistance program information:
My VYVGART Path
Visit **vyvgart.com/gmg/support-and-resources/intro-to-myvyvgartpath** or call **833-697-2841.**

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Generalized Myasthenia Gravis (gMG)

Name: VYVGART Hytrulo® (efgartigimod alfa and hyaluronidase-qvfc)

Pronunciation: viv-gart hi-true-loh

How does it work?

- It binds to FcRn and prevents the recycling of the antibodies that cause MG.
- These antibodies are broken down in the cell and sent to the body's disposal system.

Ages treated: Adults with anti-acetylcholine receptor (AChR) antibody-positive gMG

What does it look like? It is a liquid in a syringe.

How is it given? Administered through a subcutaneous (under the skin) injection into an area of pinched skin on your abdomen over 30-90 seconds. Treatment cycles consist of one injection each week for four weeks with a personalized break between cycles.

Possible side effects*: Respiratory tract infection; headache; urinary tract infection; injection site reactions, including rash, redness of the skin, itching sensation, bruising, pain, hives

Patient assistance program information:
My VYVGART Path
Visit **vyvgart.com/gmg/support-and-resources/intro-to-myvyvgartpath** or call **833-697-2841.**

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Generalized Myasthenia Gravis (gMG)

Name: ZILBRYSQ® (zilucoplan injection)

Pronunciation: zil-brisk

How does it work?

- It blocks C5, a protein involved in muscle cell damage.
- Muscle cell damage can interrupt the signals between nerves and muscles, a known cause of gMG.

Ages treated: Adults with gMG who are anti-acetylcholine receptor (AChR) antibody positive

What does it look like? It is a liquid in a syringe.

How is it given? Zilbrysq is can be self-administered once daily using pre-filled syringes for subcutaneous (under the skin) injections.

Possible side effects*: Increased risk of meningococcal infections and other types of serious infections, inflammation of the pancreas (pancreatitis) and other pancreatic problems, injection site reactions, upper respiratory tract infections, diarrhea

Patient assistance program information:
ONWARD
Visit **ucbonward.com/ZILBRYSQ** or call **844-669-2731**

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