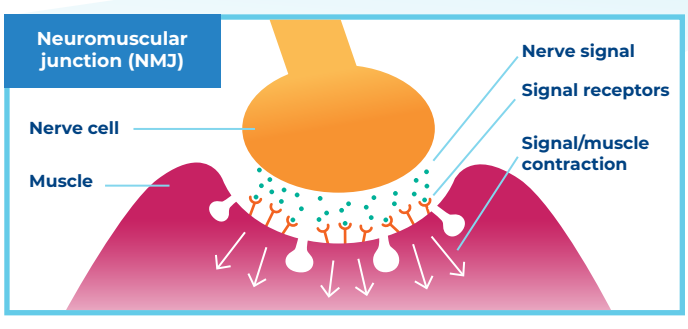
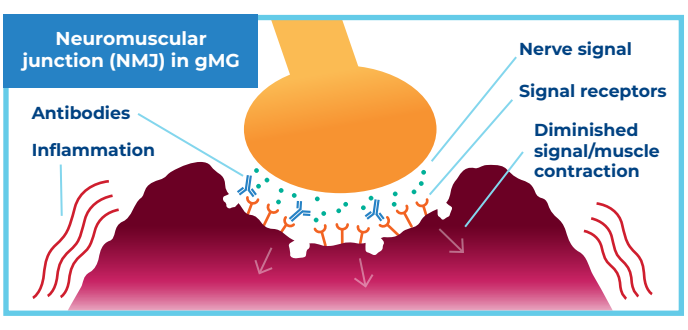
Generalized myasthenia gravis (gMG)

ALEXION
AstraZeneca Rare Disease

WHAT IS GENERALIZED MYASTHENIA GRAVIS?

Generalized myasthenia gravis (gMG) is a **rare autoimmune disorder** characterized by loss of muscle function and severe muscle weakness.[1]





The **neuromuscular junction (NMJ)** is the connection point between **nerve cells** and the **muscles** they control.[1]

85% of people with gMG are AChR+, meaning they produce specific antibodies (anti-AChR) that bind to signal receptors at the NMJ. This binding activates the complement system, causing the immune system to attack the NMJ. This leads to inflammation and a **breakdown in communication** between the **brain** and the **muscles**.[1]

Diagnosed prevalence in adults is[2-14]

~64K ~80.5K ~15K

Most commonly begins for **women before the age of 40** and for **men after the age of 60.**[15-17]

Initial symptoms may include[18,19]

- Slurred speech
- Double vision
- Droopy eyelids
- Lack of balance

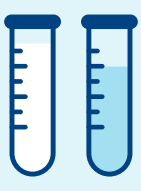
which can often lead to more severe symptoms as the disease progresses[18,19]

- Impaired swallowing
- Choking
- Extreme fatigue
- Respiratory failure

HOW IS gMG DIAGNOSED?[18]

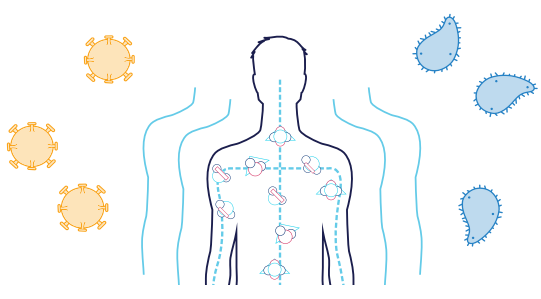
gMG is typically diagnosed with a **physical examination** to evaluate muscle function.

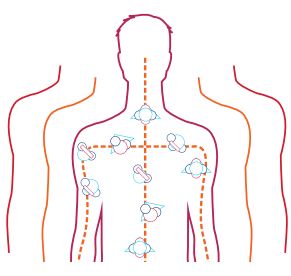
Blood tests for certain antibodies, including anti-acetylcholine receptor (anti-AChR), are also used



as well as **nerve and muscle stimulation** and **chest computed tomography** or **magnetic resonance imaging (MRI)**.

THE COMPLEMENT SYSTEM





The complement system is a part of the immune system and is **essential to the body's defense against infection**.[20]

When the system is **thrown out of balance**, or dysregulated, these proteins can **trigger a dangerous, uncontrolled cascade of reactions** that attack cells and tissues resulting in **harmful inflammation** and the **destruction of healthy cells**.[21]

WHAT ROLE DOES COMPLEMENT INHIBITION PLAY IN TREATING gMG?

Alexion's clinical studies in gMG have shown that **inhibiting the complement system** (by blocking the C5 protein) prevents the body's attack on the NMJ.

This **reduces the damage** and helps prevent the breakdown in communication between the brain and the muscles.

Alexion's leadership in complement inhibition has set the course for the continued study and development of innovative treatments for certain rare complement-mediated neurological diseases, including gMG.

WHAT TREATMENT APPROACH IS BEING STUDIED BY ALEXION?

In addition to **our development of a therapy that is approved for adults with AChR+ gMG**, we continue to advance research and other clinical trial programs in the disease, including an ongoing **Phase 3 study involving our long-acting complement inhibitor**.

We remain focused on **accelerating the discovery and development of new**, **life-changing therapies** for people living with gMG.

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