FAQ for Efgartigimod alfa in the treatment of myasthenia gravis (gMG) – EAMS agreement

1. What is the Early Access to Medicines Scheme?

The aim of the Early Access to Medicines Scheme (EAMS) is to provide earlier availability of promising medicines to patients in the UK where there is high unmet clinical need, before the normal product licensing process is completed. The medicinal products included in the scheme are those that are intended to treat, diagnose or prevent seriously debilitating or life-threatening conditions where there are no adequate treatment options. The scheme is regulated by the Medicines and Healthcare Regulatory Agency (MHRA) in the UK.

More information about the scheme can be found on its website here.

2. What is the EAMS indication?

Efgartigimod alfa is used to treat adults with a disease-causing muscle weakness, called generalised myasthenia gravis (gMG). The full EAMS indication is….efgartigimod alfa is indicated for the treatment of adult patients with AChR-antibody seropositive generalised myasthenia gravis (gMG), including patients with refractory gMG who have failed, not tolerated or are ineligible for licensed treatment.

3. What patients are eligible for the scheme?

Patients with gMG who have AChR-Ab's may be eligible for the scheme. Patients who may be appropriate for inclusion into EAMS will be identified by clinicians in Specialist MG Centres across the UK.

4. How do patients get onto the scheme?

Patients should ask their MG clinical team about possible participation in the scheme.

5. How long will the EAMS be open?

An EAMS is reviewed by the Commission on Human Medicines (CHM) and considered for renewal each year; ArgenX intend to apply for annual renewals of EAMS if NICE approval hasn't come through by the end of the EAMS.

An EAMS programme is only open to include new patients until a product gets a marketing authorisation (sometimes described as a 'product license'). This can be extended, in a programme called EAMS+, until the time that NICE (or other devolved nations decision makers) make a decision about funding efgartigimod. After this point, however, no new patients can be added to the scheme.

6. How does the EAMS work?

The EAMS allows a medicine to be prescribed by UK specialists to treat people before it has a marketing authorisation. This is not a clinical trial; rather, it reflects how the medicine will be used in real life once it has a license.

Treatment will only be provided in specialist centres that are experienced in managing Myasthenia Gravis and there is the requirement for careful monitoring to ensure the safety of patients during the EAMS.

7. Where is the EAMS available?

This EAMS is available in the UK National Health Service in England, Scotland, Northern Ireland and Wales.

8. What is efgartigimod alfa?

Efgartigimod alfa binds to and blocks a protein in the body called neonatal Fc receptor (FcRn). By blocking FcRn, efgartigimod alfa decreases the level of IgG autoantibodies which are proteins of the immune system that attack parts of a person's own body by mistake.

In patients with gMG, IgG autoantibodies attack and damage proteins on nerves called acetylcholine receptors. Because of this damage, the nerves are not able to make the muscles contract as well as normal, leading to muscle weakness and difficulty moving. By binding to the FcRn protein and reducing autoantibody levels, efgartigimod alfa can improve the ability of muscles to contract and reduce the symptoms of the disease and their impact on daily activities.

9. Is efgartigimod alfa available for patients who are taking steroids?

Efgartigimod alfa can be taken with most existing gMG treatments.

10. What are the possible side effects?

Like all medicines, efgartigimod alfa can cause side effects, although not everyone gets them.

The most frequent side effects, affecting at least 10% of patients treated with efgartigimod alfa were upper respiratory infections, which commonly included nasal obstruction, sore throat, tonsilitis, pharyngitis or the common cold.

Other common side effects including at least 1% of patients were urinary tract infections, bronchitis (inflammation of the airway passages), muscle pains or headache during or after administration of efgartigimod alfa.

Infusion reactions (allergic reactions) are also possible; these and serious infections, may require that treatment is stopped.

Specialist doctors will also be able to explain this further.

11. Are extra tests and hospital visits required for EAMS?

The treatment will be given by your doctor or other healthcare provider.

The dose you receive will depend on your bodyweight and will be administered in cycles of one infusion per week for 4 weeks. Your doctor will determine when further treatment cycles are needed.