Medicines & Healthcare products Regulatory Agency

MHRA

Early Access to Medicines Scheme – Treatment protocol – Information for patients

Introduction

The aim of the Early Access to Medicines Scheme (EAMS) is to provide earlier availability of promising new unlicensed medicines (medicines that do not have a marketing authorisation or are used outside their licence) to UK patients that have a high unmet clinical need. The medicines included in the scheme after they have received a positive scientific opinion are those that are intended to treat, diagnose or prevent seriously debilitating or life-threatening conditions where there are no adequate treatment options. More information about the scheme can be found here: http://www.mhra.gov.uk/Howweregulate/Innovation/EarlyaccesstomedicinesschemeEAMS/index.htm

The information below is intended for you, the patient, and is provided by the pharmaceutical company (called scientific opinion holder) that manufactures the EAMS medicine. This medicine, which does not yet have a drug licence or is used outside its licence, may also be used in combination with other medicines. More information about medicines licensing can be found here: http://www.nhs.uk/conditions/medicines-information

This medicine can be prescribed for individual patients to meet specific needs provided they are given sufficient information about the medicine to make an informed decision. Your physician will be responsible for giving you all the information you need to make this decision and for obtaining informed consent from you prior to treatment. You will be asked to sign a form to confirm that you are providing informed consent to receiving the EAMS treatment. Information on consent can be found here:
https://www.nhs.uk/conditions/Consent-to-treatment

The information below is provided to help you decide with your physician on whether to use the EAMS medicine and helps explain how to use it in accordance with the pharmaceutical company's instructions for safe and proper use. A positive scientific opinion is not a recommendation for use of the medicine and should not be interpreted as such. Under EAMS, the risk and legal responsibility for prescribing the medicine remains with the physician, and the opinion and EAMS documentation published by the MHRA are intended only to inform physicians' decision making and not to recommend use. An EAMS scientific opinion does not affect the civil liability of the manufacturer or any physician in relation to the product.

The information below may change during the time you are using the medicine if more data become available. Your physician will highlight to you any changes that you need to be aware of.

Whilst you are using this medicine, data will be collected on the use and safety profile of the medicine, to ensure that the benefits of taking the medicine continue to outweigh any potential risks. Your physician will answer all your questions during and after the treatment and will provide you with contact details that you should use in case of any events or problems.

Each patient enrolled in the scheme will continue to receive the EAMS product until the end of the treatment in line with prescribing and NHS guidance and as long as benefit is seen. In rare cases where the EAMS treatment may not be available anymore, your physician will discuss other options with you.

Information for the patient

Efgartigimod alfa 20 mg/ml concentrate for solution for infusion
Efgartigimod alfa

Read all of this leaflet carefully before you are given this medicine because it contains important information for you.
- Keep this leaflet. You may need to read it again.
- If you have any further questions, ask your doctor or nurse.
- If you get any side effects, talk to your doctor or nurse. This includes any possible side effects not listed in this leaflet. See section 4.

What is in this leaflet
1. What efgartigimod alfa is and what it is used for
2. What you need to know before you are given efgartigimod alfa
3. How efgartigimod alfa is given
4. Possible side effects
5. How to store efgartigimod alfa
6. Contents of the pack and other information

1. What efgartigimod alfa is and what it is used for

What efgartigimod alfa is
Efgartigimod alfa contains the active substance 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.

What efgartigimod alfa is used for
Efgartigimod alfa is used to treat adults with generalised Myasthenia Gravis (gMG), an autoimmune disease that causes muscle weakness. gMG can affect multiple muscle groups throughout the body. The condition can also lead to shortness of breath, extreme fatigue and difficulties swallowing.

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.

2. What you need to know before you are given efgartigimod alfa

You must not be given efgartigimod alfa
- if you are allergic to efgartigimod alfa or any of the other ingredients of this medicine (listed in section 6).

Warnings and precautions
Talk to your doctor before using efgartigimod alfa.

<u>Infections</u>
Before starting efgartigimod alfa, inform your doctor if you have any infections.

<u>Infusion reactions (allergic reactions)</u>
Efgartigimod alfa contains a protein that can cause allergic reactions in some people. You will be monitored for signs of an infusion reaction during and for 1 hour after treatment.

Immunisations (vaccinations)
Please inform your doctor if you have received a vaccine in the last 4 weeks, or if you plan to be vaccinated in the near future.

Children and adolescents
Do not give this medicine to children below 18 years of age because the safety and efficacy of efgartigimod alfa have not been established.

Elderly
There are no special precautions needed for the treatment of patients who are older than 65 years of age.

Other medicines and efgartigimod alfa
Tell your doctor if you are using, have recently used or might use any other medicines.

Contraception, pregnancy and breast-feeding
If you are pregnant or breastfeeding, think you may be pregnant or are planning to have a baby, ask your doctor or healthcare provider for advice before taking this medicine.

Driving, cycling and using machines
Efgartigimod alfa is not expected to influence the ability to drive or use machines.

Efgartigimod alfa contains sodium
This medicine contains 67.2 mg sodium (main component of cooking/table salt) in each vial. This is equivalent to 3.4% of the recommended maximum daily dietary intake of sodium for an adult.

3. How efgartigimod alfa is given

The treatment will be given by your doctor or other health care provider. Your healthcare provider will first dilute the product. The dilution will be administered from a drip bag through a tube directly into one of your veins.

What dose of efgartigimod alfa you will receive and how often
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. Instructions for proper use of this medicine is provided at the end of this document.

If you receive more efgartigimod alfa than you should
If you suspect that you have been accidentally administered a higher dose of efgartigimod alfa than prescribed, please contact your doctor for advice.

If you miss a dose of efgartigimod alfa
If you forget an appointment to receive efgartigimod alfa, please contact your doctor immediately for advice and see section below “If you stop using efgartigimod alfa”.

If you stop receiving efgartigimod alfa
Interrupting or stopping treatment with efgartigimod alfa may cause your gMG symptoms to come back. Please speak to your doctor before stopping efgartigimod alfa. Your doctor will discuss the possible side effects and risks with you. Your doctor will also want to monitor you closely.

If you have any further questions on the use of this medicine, ask your doctor.

4. Possible side effects

Like all medicines, this medicine can cause side effects, although not everybody gets them. The following side effects have been reported with this medicine:

Very common: may affect more than 1 in 10 people:
- upper respiratory tract infections (commonly includes nasal obstruction, sore throat, tonsillitis, pharyngitis, laryngitis, sinusitis, otitis media, and the common cold).

Common: may affect up to 1 in 10 people:

- urinary tract infections (pain or a burning sensation during urination)
- bronchitis (inflammation of the airways passages)
- myalgia (muscles pain)
- headache during or after the administration of efgartigimod alfa

If you experience any side effects, talk to your doctor. This includes any possible side effects not listed in this leaflet.

5. How to store efgartigimod alfa

Your doctor is responsible for disposing of any unused product correctly in line with the information that is intended for healthcare professionals.

Keep this medicine out of the sight and reach of children.

Do not use this medicine after the expiry date which is stated on the carton and on the label after “EXP”. The expiry date refers to the last day of that month.

Store in a refrigerator (2 °C to 8 °C).

Do not freeze.

Store in the original package in order to protect from light.

Do not use this medicine if visible particles are observed and/or the liquid in the vial is discoloured.

After dilution the product should be used immediately and the infusion (drip) should be completed within 4 hours of dilution. If this is not possible, the diluted solution can be stored at 2 °C to 8 °C for up to 8 hours, and infusion should be completed within 4 hours of removal from the refrigerator.

Do not throw away any medicines via wastewater or household waste. Ask your pharmacist how to throw away medicines you no longer use. These measures will help protect the environment.

6. Contents of the pack and other information

What does this medicine contain?

- The active substance is efgartigimod alfa 400 mg in a 20 ml vial (20 mg/ml)..
- Each 20 ml vial contains 400 mg efgartigimod alfa (20 mg/ml).
- The other ingredients are:
  - sodium dihydrogen phosphate, monohydrate
  - sodium disodium hydrogen phosphate, anhydrous
  - sodium chloride
  - L-arginine hydrochloride
  - polysorbate 80
  - water for injections

What efgartigimod alfa looks like and contents of the pack

Efgartigimod alfa is presented as a sterile concentrate for intravenous infusion (20 ml in a vial – pack size of 1).

Efgartigimod alfa is a liquid. It is colourless to slightly yellow, clear to almost clear.

Scientific Opinion Holder and manufacturer

Scientific Opinion Holder
argenx BV
Industriepark-Zwijnaarde 7
9052 Gent
Belgium

Manufacturer
Clinigen Healthcare Ltd
Unit 3, Canada Rd
West Byfleet
Surrey
KT14 74L
UK

This protocol was revised in May 2022

Additional information

Informed Consent/Assent Form

All patients will have the Early Access to Medicines Scheme explained to them using the informed consent form. The patient will be asked to sign this form and a copy will be given to them to keep. You will also be asked to give permission to have some data collected to help understand efgartigimod alfa treatment within the Early Access to Medicines Scheme

Patient Alert Card

Each patient must be given a Patient Alert Card before they start treatment with efgartigimod alfa. The patient must keep this alert card with them at all times during the treatment and for at least 1 month after completing their treatment. The card summarises that they are currently receiving efgartigimod alfa, the important side effects for which they need to seek assistance should they occur, details of the patient's treating physician, out of hours contact details and the company contact details.

Patient data to be collected

Patient data collected during the scheme are mostly used for safety surveillance and cannot replace a proper clinical trial to support a marketing authorisation. These data are required by the MHRA to help verify that the patient's condition complies with the EAMS indication and help interpret the side effects and other events occurring during and after the EAMS treatment. These data include patient age; patient gender; efgartigimod alfa dose, number of infusions, when doses are given; any other conditions a patient has; medications being given, including treatments for gMG; adverse events; hospital visits and any procedures related to gMG.

Additional data will be collected on how effective the treatment is on a voluntary basis. These data include a measure of gMG symptoms (using the patient reported MG-ADL scale.

Contact information

Contact details for medical information

Email: medinfo@argenx.com

Contact information for safety reporting

Adverse events must be reported to: drugsafety@clinigengroup.com, phone 01932 824084 or via fax: 01932 824284

Adverse events must also be reported to Yellow Card: https://yellowcard.mhra.gov.uk; alternatively, you may call Freephone 0808 100 3352 (available between 10am-2pm Monday – Friday).