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

Sebetralstat 300 mg film-coated tablets

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 sebetralstat is and what it is used for

Sebetralstat is a medicine that contains the active substance sebetralstat.

Sebetralstat is used to treat attacks of hereditary angioedema in adults and adolescents aged from 12 years.

What Hereditary Angioedema is

Hereditary angioedema is a condition that often runs in families. In hereditary angioedema your blood does not have enough of a protein called C1 inhibitor, or the protein does not work properly. This leads to too much of the enzyme 'plasma kallikrein', which in turn increases the levels of 'bradykinin' in your bloodstream. Too much bradykinin leads to attacks of swelling and pain in different parts of your body.

How sebetralstat works

Sebetralstat works by blocking the activity of plasma kallikrein and helps reduce the levels of bradykinin. When taken at the start, or during an attack, this will stop further progression of the attack and the symptoms of swelling and pain.

2. What you need to know before you are given sebetralstat

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

Warnings and precautions

After taking sebetralstat for a laryngeal attack it is important to seek immediate medical attention.

If you have severely reduced liver function talk to your doctor before taking sebetralstat as this medicine may not be suitable for you.

Children and adolescents

Sebetralstat is not recommended in children under 12 years. This is because it has not been studied in this age group.

Other medicines and sebetralstat

Tell your doctor or pharmacist if you are taking, have recently taken or might take any other medicines. This is because some other medicines can affect the way sebetralstat works.

In particular, tell your doctor or pharmacist if you are taking the following:
- antibiotic medicines (e.g. rifampicin, erythromycin, clarithromycin)
- some medicines for fungal infections (e.g. itraconazole, ketoconazole)
- medicines that affect your heart (such as beta blockers and calcium channel blockers e.g.verapamil and diltiazem)
- antiviral medicines (e.g. efavirenz, ritonavir, saquinavir)
- some medicines used for epilespsy (e.g. carbamazipine, phenytoin, phenobarbital).

If you are not sure, talk to your doctor or pharmacist before taking sebetralstat.

Contraception, pregnancy and breast-feeding

Sebetralstat is not recommended if you are pregnant or breast-feeding, think you may be pregnant or are planning to have a baby.

Driving, cycling and using machines

Sebetralstat does not influence the ability to drive, cycle or use machines.

Sebetralstat contains croscarmellose sodium

This medicine contains less than 1 mmol sodium (23 mg) per tablet, that is to say essentially ‘sodium-free’.

3. How sebetralstat is given

Always take this medicine exactly as your doctor has told you. Check with your doctor if you are not sure.

Adults and adolescents aged 12 years and over: The recommended dose is **ONE tablet** to be taken at the earliest sign that you are having an attack. Swallow the tablet whole with some water, if required. The tablet can be taken with or without food.

An additional dose of ONE tablet may be taken if symptoms persist.

Use in children

Sebetralstat is not recommended for use in children under 12 years of age because it has not been studied in these patients.

If you take more sebetralstat than you should

Tell your doctor immediately if you have taken too many sebetralstat tablets.

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:

Common side effects (may affect up to 1 in 10 people):
- Indigestion
- Fatigue (a lack of energy).

Reporting of side effects
If you get any side effects, talk to your doctor or pharmacist. This includes any possible side effects not listed in this leaflet. You can also report side effects directly via the Yellow Card Scheme at www.mhra.gov.uk/yellowcard or search for MHRA Yellow Card in the Google Play or Apple App Store. By reporting side effects you can help provide more information on the safety of this medicine.

5. How to store sebetralstat

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 blister wallet after EXP. The expiry date refers to the last day of that month.

This medicine does not require any special storage conditions.

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 sebetralstat contains

- The active substance is sebetralstat.
- The other ingredients are:

  Tablet core: microcrystalline cellulose; croscarmellose sodium; povidone K30; magnesium stearate.

  Film-coatings: Macrogol poly(vinyl alcohol) grafted copolymer; talc; titanium dioxide (E171); glycerol monocaprylocaprate (Type 1); poly(vinyl alcohol); iron oxide yellow (E172); iron oxide black (E172); maltodextrin; guar galactomannan; hypromellose; triglycerides, medium chain.

What sebetralstat looks like and contents of the pack

Sebetralstat 300 mg tablets are yellow, oval, biconvex tablets debossed with KalVista logo “K” on one side and “300” on the other side. The tablets are packed in a blister encased in a child-resistant cardboard wallet. The wallets are contained in a cardboard box. The pack contains 6 tablets.

Scientific Opinion Holder and manufacturer

Scientific Opinion Holder:
KalVista Pharmaceuticals Ltd.
Porton Science Park
Bybrook Road, Porton Down
Salisbury SP4 0BF

Manufacturer:
Almac Clinical Services Limited
Seagoe Industrial Estate
9 Charlestown Road
Craigavon
Northern Ireland
BT63 5PW
United Kingdom

This protocol was revised in March 2025

Additional information

Informed Consent/Assent Form

All patients will have the Early Access to Medicines Scheme explained to them using the informed consent/assent form. The patient will be asked to sign this form and a copy will be given to them to keep.

Your physician will not undertake any investigation specifically required for the EAMS until valid consent/assent has been obtained.

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:

- Age
- Height
- Weight
- Birth gender
- Race/ethnicity (where permitted)
- Clinical background

The following information regarding each patient's clinical history will be captured:

- Age at diagnosis/first attack
- HAE type and how confirmed
- Family history details
- Number of attacks experienced/treated in the last 12 months
- Details of any preventative treatments prescribed
- Details of most recent attack, including date, duration, symptoms/severity, location(s) affected
- Details of on-demand treatment(s) employed for most recent attack (drug name, dosing, when initiated, time to resolution)
- Diagnosed comorbidities
- Details of concomitant medications
- Angioedema-control test (AECT-4 instrument)
- EQ-Visual Analogue Scale (EQ-VAS)

During enrolment, patients will be offered the opportunity to provide additional patient-reported data via a separate ePatient portal. This is optional and patients will give their consent to provide further data as part of the portal registration process. Data collected via the ePatient portal will be truly anonymized as the HCP will not have access to the ePatient portal and no other identifiable information will be collected on this platform.

Patients choosing to register for access to the ePatient portal will be asked to report details of attacks in real time and in the post-attack follow-up period, including:

- Details of the attacks experienced (such as symptoms/severity; location; duration)
- Details of sebetralstat and outcomes (time to initial improvement; time to resolution)
- Following each attack and 6-weeks post attack, patients may complete an EQ-5D-5L health-related quality of life instrument.

The reason for collecting these additional data are to learn more about the use of the medicine in real life.

Contact information

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