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:

Lumasiran 189 mg/mL solution for injection
lumasiran

▼This medicine is subject to additional monitoring. This will allow quick identification of new safety information. You can help by reporting any side effects you may get. See the end of section 4 for how to report side effects.

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

1. What lumasiran is and what it is used for

What lumasiran is
Lumasiran contains the active substance 'lumasiran'.

What lumasiran is used for
Lumasiran is used to treat an illness called 'Primary Hyperoxaluria Type 1' (PH1). It is used in adults and children of all ages.

What PH1 is
PH1 is a rare illness that makes the liver make too much of something called 'oxalate'.
- Oxalate is removed by the kidneys and through your urine.
- In people with PH1, the extra oxalate can cause kidney stones and kidney failure.
- Oxalate can also build up, and damage other parts of the body such as eyes, heart, skin, and bone. This is called 'oxalosis'.

How lumasiran works
This medicine works by lowering the amount of 'glycolate oxidase' (GO) that the liver makes.
- GO is one of the proteins the liver uses to make oxalate.
- By lowering the amount of GO in the liver, less oxalate is made.
- This leads to lower levels of oxalate in the urine and blood.
- This can help to reduce the effects of this illness.

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

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

Warnings and precautions
Talk to your doctor before being given this medicine.

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

Pregnancy
If you are pregnant, think you may be pregnant, or are planning to have a baby, ask your doctor or nurse for advice before using this medicine.

Breast-feeding
This medicine may pass into breast milk. If you are breast-feeding, ask your doctor for advice before taking this medicine. Your doctor will then help you decide whether to stop breast-feeding or to stop treatment. This will take into account the benefit of breast-feeding for your child and the benefit of the treatment for you.

Driving and using machines
This medicine is unlikely to have any effect on your ability to drive or use machines.

Lumasiran contains sodium
This medicine contains less than 1 mmol sodium (23 mg) per mL – so we can say that it is 'sodium-free'.

3. How lumasiran is given

How much lumasiran is given
Your doctor will work out how much medicine to give you. The amount will depend on how much you weigh. Your doctor will adjust your dosing regimen as your weight changes.

You will receive your first doses (loading doses) once a month for 3 months. You will then start maintenance dosing one month after the last loading dose.

Body weight less than 10 kg
- Loading doses: 6 mg for every kg of your weight, given once a month for 3 months.
- Maintenance dosing: 3 mg for every kg of your weight, given once every month.

Body weight from 10 kg to less than 20 kg
- Loading doses: 6 mg for every kg of your weight, given once a month for 3 months.
- Maintenance dosing: 6 mg for every kg of your weight, given once every 3 months.

Body weight 20 kg or more
- Loading doses: 3 mg for every kg of your weight, given once a month for 3 months.
- Maintenance dosing: 3 mg for every kg of your weight, given once every 3 months.

How lumasiran is given
This medicine will be given to you by a doctor or nurse.
- It is given as an injection under the skin ('sub-cutaneously') into your stomach area (abdomen), or in some cases, your upper arm or thigh. The site of injection will be rotated.
- Depending on your dose, more than one sub-cutaneous injection may need to be given.

If you are given too much lumasiran
In the unlikely event that your doctor or nurse gives you too much (an overdose) they will check you for side effects.

If you miss your dose of lumasiran
If you miss a dose of lumasiran, talk to your doctor or nurse as soon as possible about when to get your next dose.

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

4. Possible side effects

Like all medicines, this medicine can cause side effects, although not everybody gets them.

The following side effects may occur when taking lumasiran:

Very common: may affect more than 1 in 10 people
- Redness, pain, itching, or swelling at the site of the injection (injection site reaction).

Tell your doctor or nurse if you notice any of the side-effects listed above.

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

You should also report any side effects directly via the Yellow Card Scheme at: www.mhra.gov.uk/yellowcard or call freephone 0800 731 6789 (10am to 2pm Monday-Friday only).

Your doctor or pharmacist will complete an EAMS Safety Information Reporting Form, and a Discontinuation Form if you stop treatment.

5. How to store lumasiran

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

This medicine is for single-use only. Once the product is opened, use immediately.

Store at 2-8 °C (Refrigerate, do not freeze).

Keep vial in the outer carton to protect from light.

Do not throw away any medicines via wastewater or household waste. Your doctor or nurse will throw away any medicines that are no longer being used. These measures will help protect the environment.

6. Contents of the pack and other information

What lumasiran contains
- The active substance is lumasiran.
- Each mL contains lumasiran sodium equivalent to 189 mg lumasiran.
- The other ingredients are water for injections, sodium hydroxide, and phosphoric acid.

What lumasiran looks like and contents of the pack
This medicine is a clear, colourless-to-yellow solution for sub-cutaneous injection.

Each pack contains one single-use vial containing 0.5 mL solution for sub-cutaneous injection.

Scientific Opinion Holder
Alnylam UK Limited
Braywick Gate
Braywick Road
Maidenhead
SL6 1DA
United Kingdom

Manufacturer
Alnylam Netherlands B.V.
Strawinskylaan 3051
1077 ZX Amsterdam
Netherlands

This leaflet was last revised in June 2020

EAMS number: 43942/0002

Additional information:

Prior to requesting access to lumasiran, you will have the scheme explained to you using this leaflet. You will be requested to sign an Informed Consent Form and will be given a copy to keep.

In addition to pharmacovigilance reporting, data may be collected on clinical efficacy on a voluntary basis. These tests can include: blood and urinary tests, renal stone and physical assessments. This data may be shared with the EAMS Scientific Opinion Holder, and regulatory authorities.

Contact details:

EAMS queries
To request access to EAMS or EAMS related enquiries: EAP@alnylam.com

Pharmacovigilance reporting
All reporting forms should be submitted to Clinigen at: drugsafety@clinigengroup.com

Alnylam Medical Information Hotline
Toll: +44 162 88 78592
Toll-free: 08001412569
medinfo@alnylam.com