



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

Voxelotor 500 mg film-coated tablets

Read all of this leaflet carefully before you start taking 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.
- This medicine has been prescribed for you only. Do not pass it on to others. It may harm them, even if their signs of illness are the same as yours.
- If you get any side effects, talk to your doctor. This includes any possible side effects not listed in this leaflet. See section 4.

What is in this leaflet

What voxelotor is and what it is used for
What you need to know before you take voxelotor
How to take voxelotor
Possible side effects
How to store voxelotor
Contents of the pack and other information

1. What voxelotor is and what it is used for

What voxelotor is and how it works

Voxelotor contains the active substance voxelotor. Voxelotor works on a protein in red blood cells called haemoglobin to help it take up oxygen that red blood cells can deliver throughout the body.

Patients with the condition called sickle cell disease have an altered form of haemoglobin called sickle haemoglobin which is different from the normal haemoglobin. When the sickle haemoglobin gives up oxygen to the tissues, it sticks together to form long rods and causes red blood cells to alter their shape to that of a crescent moon making these cells rigid and sickled shape. Sick red blood cells cannot deliver oxygen as well as healthy red blood cells and are also broken down more quickly, leading to lowered levels of red blood cells (haemolytic anaemia). By improving the way the altered haemoglobin holds onto oxygen, voxelotor improves the function of red blood cells and prolongs their lifespan.

What voxelotor is used for

Voxelotor, alone or together with hydroxycarbamide (also known as hydroxyurea), is used to treat **haemolytic anaemia** in adults and children from 12 years with **sickle cell disease**.

2. What you need to know before you take voxelotor

Do not take voxelotor

- if you are allergic to voxelotor or any of the other ingredients of this medicine (listed in section 6)
- If you have severe kidney problems
- If you have severe liver problems
- If you are pregnant or breastfeeding
- If you are in a clinical study or have participated in a clinical study in the last 30 days.
- If you have severe depression, anxiety, schizophrenia or trauma-related disorders

Warnings and precautions

If you get any symptoms of allergic reactions, stop taking voxelotor and talk to your doctor or get emergency medical help immediately. Symptoms are for example rash, including nettle rash (hives), shortness of the breath and swelling of the face.

If you are receiving blood transfusions, talk to your doctor about possible difficulties with the interpretation of certain blood tests when taking this medicine.

Children under 12 years

This medicine is not recommended for children under 12 years due to lack of data in this age group.

Other medicines and voxelotor

Tell your doctor if you are taking, have recently taken or might take any other medicines.

Some medicines can affect how voxelotor works or may make side effects more likely. In particular, tell your doctor if you take any of the following medicines:

- rifampicin (used to treat bacterial infections)
- phenobarbital, carbamazepine, phenytoin (used to treat epilepsy and other illnesses)
- sirolimus, tacrolimus (used to prevent organ rejection after transplantation)
- St John's wort (a herbal medicine to treat depression)
- alfentanil (a painkiller used during an operation with anaesthetics)

Tell your doctor that you are taking voxelotor if you are having a medical procedure or surgery.

Pregnancy and breast-feeding

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

- **Pregnancy**
Do not take voxelotor during pregnancy.
- **Breast-feeding**
Do not breast-feed while taking voxelotor because it is not known if voxelotor passes into breast milk and could affect the baby.

Driving and using machines

Voxelotor has no or negligible influence on the ability to drive and use machines.

Voxelotor contains sodium

This medicine contains less than 1 mmol sodium (23 mg) per dose of three tablets, that is to say essentially 'sodium-free'.

3. How to take voxelotor

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

The recommended dose for adults and children from 12 years is:

Three 500 mg tablets taken once daily by mouth.

Swallow the tablets whole with one glass of water, with or without food. Do not cut, crush or chew the tablets because of the unpleasant taste.

If you take more voxelotor than you should

Contact your doctor immediately.

If you forget to take voxelotor

Continue with your normal dosing schedule on the next day. Do not take a double dose to make up for a forgotten dose.

If you stop taking voxelotor

Do not stop taking this medicine without your doctor's advice. It is important to take voxelotor daily.

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.

Stop taking voxelotor and inform your doctor or get emergency medical help immediately if you experience any of the following serious side effects:

Uncommon (may affect up to 1 in 100 people)

- **allergic reactions**
Symptoms are for example rash, including nettle rash (hives), shortness of breath and swelling of the face.

Other side effects may occur with the following frequency:

Very common (may affect more than 1 in 10 people)

- headache
- diarrhoea
- abdominal (belly) pain
- nausea
- rash

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 can also report side effects directly via 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 voxelotor

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 bottle and carton after EXP. The expiry date refers to the last day of that month.

Store at or below 30° C.

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 voxelotor contains

- The active substance is voxelotor. One tablet contains 500 mg voxelotor.

- The other ingredients are:
 - microcrystalline cellulose (E460)
 - croscarmellose sodium (E468)
 - sodium laurilsulfate (E487)
 - silica, colloidal anhydrous (E551)
 - magnesium stearate (E470b)
 - polyvinyl alcohol (E1203)
 - titanium dioxide (E171)
 - polyethylene glycol (E1521)
 - talc (E553b)
 - iron oxide yellow (E172)

What voxelotor looks like and contents of the pack

Light yellow to yellow, oval-shaped, biconvex, film-coated tablets, debossed with “GBT 500” on one side. Tablet dimensions: approximately 18 mm x 10 mm.

Voxelotor is packaged in a plastic bottle with a child-resistant cap. Each bottle contains 90 film-coated tablets. The bottle also contains coil and a silica gel desiccant canister to help keep your medicine dry.

SCIENTIFIC OPINION HOLDER

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