



## 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) to UK patients that have a high unmet clinical need. The medicines 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. 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 that manufactures the medicine. This medicine does not yet have a drug licence (also called a marketing authorisation). More information about medicines licensing can be found here:

<http://www.mhra.gov.uk/Safetyinformation/Generalsafetyinformationandadvice/Adviceandinformationforconsumers/MymedicineFromlaboratorytopharmacyshelf/Licensingmarketingauthorisation/index.htm>

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. Informed consent should be obtained from you prior to treatment.

This information is provided to help you decide with your doctor on whether to use the medicine and helps explain how to use the medicine in accordance with the pharmaceutical company's instructions for safe and proper use. 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 outweigh any potential risks.

### Information for the patient:

#### **Raxone ▼ 150 mg film-coated tablets** idebenone

▼ 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 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 or pharmacist.
- 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 or pharmacist. This includes any possible side effects not listed in this leaflet. See section 4 for more details.
- It is important that you keep the **EAMS Patient Card** (which will fit in a wallet) with you at all times during treatment
- Always tell any doctor or other health care professional that is treating you that you are taking Raxone and show them the **EAMS Patient card** (this includes your GP, dentist, nurse, pharmacist)

### **What is in this leaflet**

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

#### **1. What Raxone is and what it is used for**

Raxone contains a substance called idebenone.

#### **Idebenone is currently used to:**

- treat vision impairment in adults and adolescents with an eye disease called Leber's Hereditary Optic Neuropathy (LHON).

The pack of medicine you are given may include information about this condition.

#### **In the Early Access to Medicines Scheme (EAMS) idebenone is used to**

- slow the rate of decline of breathing ability in patients with Duchenne muscular dystrophy (DMD) not taking glucocorticoids.

DMD is caused by a problem with some of your genes (called a "genetic mutation").

- Muscles are often damaged and repaired during normal life. In DMD, there is a genetic mutation that means an important protein called dystrophin is not produced. This protein helps to keep muscle cells intact and when this is missing muscle cells fail to work properly and this causes damage.
- Over time, muscle cells are lost and muscles become weaker. This affects all muscles that are needed to move the body.
- The muscles affected include those needed for breathing (respiration) – causing a problem called "respiratory insufficiency". This means that patients with DMD may at some time need support to breathe and may have more frequent lung infections.
- Treatment with Raxone can protect cells and helps them to produce energy again. This can slow down the gradual increase in breathing problems.

#### **2. What you need to know before you take Raxone**

##### **Do not take Raxone:**

- if you are allergic to idebenone or any of the other ingredients of this medicine (listed in section 6).
- if you currently are using glucocorticoids

##### **Warnings and precautions**

Talk to your doctor or pharmacist before taking Raxone if:

- you have any blood, liver or kidney problems.

##### Change in urine colour

Raxone may make your urine become reddish brown. This change in colour is harmless – it does not mean your treatment needs to change. However, the change in colour could mean that you have problems with your kidneys or bladder.

- Tell your doctor if your urine changes colour.
- He or she may do a urine check to make sure the change in colour is not hiding other problems.

##### Use of glucocorticoids

There is not enough information from clinical trials on the use of Raxone in patients with DMD taking glucocorticoids. Therefore Raxone should not be used by people who are taking glucocorticoids.

Any decision to stop glucocorticoids should be separate from the decision to start treatment with Raxone and you should discuss this with your doctor carefully.

### **Children and adolescents**

Children with DMD who are under 10 years of age should not use this medicine. This is because it is not known if Raxone is safe or works in these patients.

### **Other medicines and Raxone**

Some other medicines can affect the way Raxone works and Raxone can affect the way some other medicines work. Tell your doctor if you are taking, have recently taken or might take any other medicines, especially any of the following:

- antihistamines to treat allergies (astemizole, terfenadine)
- to treat heartburn (cisapride)
- to treat muscle and speech tics associated with Tourette's syndrome (pimozide)
- to treat heart rhythm disorders (quinidine)
- to treat migraine (dihydroergotamine, ergotamine)
- to put you to sleep called "anaesthetics" (alfentanil)
- to treat inflammation in rheumatoid arthritis and psoriasis (cyclosporine)
- to prevent the rejection of an organ transplant (sirolimus, tacrolimus)
- to treat strong pain called "opioids" (fentanyl)

### **Driving and using machines**

Raxone is not expected to affect your ability to drive or use machines.

### **Raxone contains lactose and sunset yellow (E110)**

- Raxone contains lactose (a type of sugar). If you have been told by your doctor that you have lactose intolerance or that you cannot tolerate or digest some sugars, talk to your doctor before taking this medicine.
- Raxone contains a colourant called "sunset yellow" (also called E110). This may cause allergic reactions.

## **3. How to take Raxone**

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

### **How much to take**

The recommended dose is 2 tablets three times a day - this is a total of 6 tablets per day.

### **Taking this medicine**

- Take the tablets with food - this helps to get more of the medicine from your stomach into your blood. The medicine needs to get into your blood to then reach your muscles.
- Swallow the tablets whole with a glass of liquid. If you cannot swallow the tablets, they may be crushed as recommended by your doctor
- Take the tablets at the same time each day. For example, with breakfast in the morning, with lunch at midday and with dinner in the evening.

### **If you take more Raxone than you should**

If you take more Raxone than you should, talk to your doctor straight away.

### **If you forget to take Raxone**

If you forget a dose, skip the missed dose. Take the next dose at the usual time.

Do not take a double dose to make up for a forgotten dose.

### **If you stop taking Raxone**

Talk to your doctor before you stop taking this medicine.

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

#### 4. Possible side effects

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

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

- cold (nasopharyngitis)
- cough

**Common** (may affect up to 1 in 10 people):

- diarrhoea\* (mild-to-moderate and usually does not require that you stop treatment)
- back pain

**Unknown frequency** (frequency cannot be estimated from the available data):

- bronchitis
- changes in blood test results: low level of white blood cells, or low level of red blood cells, or low level of platelets
- increased cholesterol or fat in the blood – shown in tests
- fits, feeling confused, seeing or hearing things that are not real (hallucinations), feeling excited, movements that you cannot control, a tendency to wander away, feeling dizzy, headache, feeling restless, lacking energy
- nausea\*, vomiting\*, loss of appetite†, stomach ache\*
- high levels of some liver enzymes in the body which mean you have liver problems – shown in tests, high levels of “bilirubin” – this can make your skin and the whites of your eyes look yellow, hepatitis
- rash†, itching†
- pain in hands or feet\*
- high levels of nitrogen in the blood\* - shown in tests change in urine colour
- generally feeling unwell

\*Side effects that may be caused by Raxone

†Side effects medically relevant in case of lactose intolerance and allergic reactions

#### 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](http://www.mhra.gov.uk/yellowcard) and you can also telephone 03303 328102.

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

#### 5. How to store Raxone

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 the bottle after “EXP”. The expiry date refers to the last day of that month.

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 Raxone contains

- The active substance is idebenone. Each film-coated tablet contains 150 mg of idebenone.
- The other ingredients are:
  - Tablet core: lactose monohydrate, microcrystalline cellulose, croscarmellose sodium, povidone K25, magnesium stearate and colloidal silica.
  - Tablet film-coating: macrogol, poly(vinyl alcohol), talc, titanium dioxide, sunset yellow (E110).

### What Raxone looks like and contents of the pack

- Raxone film-coated tablets are orange, round tablets of 10 mm diameter, engraved with the Santhera logo on one side and "150" on the other side.
- Raxone is supplied in white plastic bottles. Each bottle contains 180 tablets.

### Scientific opinion holder (for EAMS in DMD)

Santhera (UK) Ltd  
26-28 Hammersmith Grove  
London W6 7HA

### Marketing authorisation holder and manufacturer

Santhera Pharmaceuticals (Deutschland) GmbH  
Marie-Curie Strasse 8  
79539 Lörrach  
Germany

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**EAMS number (for DMD):** 46555/0001

**Marketing authorisation number (for LHON):** EU/1/15/1020/001

### Additional information:

Before treatment starts, you, the patient, and, if relevant, your parent or legal guardian, will have the scheme explained to you using this leaflet and the **EAMS consent form**. You, and/or your parent or legal guardian (where needed), will be asked to sign this document and will be given a copy to keep.

You will also be given an **EAMS patient card**, which is a wallet-sized card that you should carry at all times. It alerts any other healthcare professional that may treat you that you are receiving idebenone through an early access scheme, and has contact details of your own specialist and the company.

This medicinal product has been authorised under 'exceptional circumstances'. This means that due to the rarity of the disease it has not been possible to obtain complete information on this medicinal product.

The European Medicines Agency will review any new information which may become available every year and this leaflet will be updated as necessary.

### Contact information:

#### Medical Information and Adverse events

Tel: 03303 328102  
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