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) 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
Vyndaqel 61 mg soft capsules
tafamidis

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, pharmacist or nurse.
- 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, pharmacist or nurse. This includes any possible side effects not listed in this leaflet. See section 4.

What is in this leaflet

1. What Vyndaqel is and what it is used for

Vyndaqel contains the active substance tafamidis.

Vyndaqel is a medicine which treats a disease called transthyretin amyloidosis. Transthyretin amyloidosis is caused by a protein called transthyretin (TTR) that does not work properly. TTR is a protein that carries other substances, such as hormones, through the body.

In patients with this disease, TTR breaks up and may form fibres called amyloid. Amyloid can build up between cells in your heart (known as transthyretin amyloid cardiomyopathy or ATTR-CM) and in other places in your body. The amyloid causes the symptoms of this disease. When this occurs in your heart, it prevents your heart from working normally.

Vyndaqel, can prevent TTR from breaking up and forming amyloid. This medicine is used to treat adult patients whose heart has been affected (people with symptomatic cardiomyopathy).

2. What you need to know before you take Vyndaqel

Do not take Vyndaqel

If you are allergic to tafamidis or any of the other ingredients of this medicine (listed in section 6).

Warnings and precautions

Talk to your doctor, pharmacist or nurse before taking Vyndaqel.

- Women that can become pregnant should use birth control while taking Vyndaqel and should continue using birth control for one month after stopping treatment with Vyndaqel. There are no data on the use of Vyndaqel in pregnant women.

Children and adolescents

Children and adolescents do not have the symptoms of transthyretin amyloidosis. Vyndaqel is therefore not used for children and adolescents.

Other medicines and Vyndaqel

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

You should inform your doctor or pharmacist if you are taking any of the following:

- non-steroidal anti-inflammatory drugs
- diuretic medicines (e.g. furosemide, bumetanide)
- anti-cancer medicines (e.g. methotrexate, imatinib)
- statins (e.g. rosuvastatin)
- anti-viral medicines (e.g. oseltamivir, tenofovir, ganciclovir, adefovir, cidofovir, lamivudine, zidovudine, zalcitabine)
- patisiran or inotersen

Pregnancy, breast-feeding and fertility

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

- You should not take Vyndaqel if you are pregnant or breast-feeding.
- If you are able to become pregnant, you must use birth control during treatment and for one month after stopping treatment.

Driving and using machines

Vyndaqel is believed to have no or negligible influence on the ability to drive and use machines.

Vyndaqel contains sorbitol

This medicine contains 44 mg sorbitol in each capsule.

3. How to take Vyndaqel

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

The recommended dose is one Vyndaqel 61 mg (tafamidis) capsule taken once a day.

If you vomit after taking this medicine and can identify the intact Vyndaqel capsule, then an additional dose of Vyndaqel should be taken in the same day; if you cannot identify the Vyndaqel capsule, then no additional dose of Vyndaqel is necessary, and you can resume taking Vyndaqel the next day as usual.
Method of administration

Vyndaqel is for oral use.
The soft capsule should be swallowed whole, not crushed or cut.
The capsule may be taken with or without food.

If you take more Vyndaqel than you should

You should not take more capsules than your doctor tells you to. If you take more capsules than you have been told to take, contact your doctor.

If you forget to take Vyndaqel

If you forget to take a dose, take your capsules as soon as you remember. If it is within 6 hours before your next dose, skip the missed dose and 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 Vyndaqel

Do not stop taking Vyndaqel without first speaking to your doctor. As Vyndaqel works by stabilising the TTR protein, if you stop taking Vyndaqel, the protein will no longer be stabilised, and your disease may progress.

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.

In clinical studies, the frequency of adverse events in patients treated with Vyndaqel was similar and comparable to placebo. No adverse events were identified as adverse drug reactions associated with Vyndaqel administration.

The most frequent side effects reported in patients with ATTR-CM who received tafamidis in research studies.
Very Common (reported in at least 10% of patients):

- Fall
- Heart failure
- Hypotension (low blood pressure)
- Dyspnoea (shortness of breath)
- Atrial fibrillation (irregular, rapid heart rate that may cause symptoms like heart palpitations)heart palpitations)
- Peripheral oedema (fluid accumulation most commonly causing swelling of lower legs)
- Fatigue (tiredness)
- Dizziness

- Bronchitis and pneumonia (infections of the respiratory tract and lung)
- Cough
- Gout
- Insomnia
- Acute kidney injury (your kidneys suddenly stop working properly)

Tell your doctor immediately if you get any side effects. Do not try to treat your symptoms with other medicines on your own.

Changes in test results

Changes in thyroid function can occur in patients treated with tafamidis. You may be told that test results for thyroid function have altered, however no corresponding symptoms of thyroid dysfunction were observed in clinical studies.

5. How to store Vyndaqel

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 label. The expiry date refers to the last day of that month.

Do not store above 25°C.

Any unused medicine or waste material should be disposed of in accordance with local requirements.

6. Contents of the pack and other information

What Vyndaqel contains

- The active substance is tafamidis. Each capsule contains 61 mg micronized tafamidis.

- The other ingredients are: gelatine, glycerine, sorbitol [see section 2], mannitol, sorbitan, red iron oxide, purified water, polyethylene glycol 400, polysorbate 20, povidone (K-value 90), butylated hydroxytoluene (E321), and White Opacode ink (comprises of: macrogol 400, polyvinyl acetate phthalate, propylene glycol, titanium dioxide, ammonium hydroxide).

What Vyndaqel looks like and contents of the pack

Vyndaqel soft capsules are reddish brown, oblong shaped printed with “VYN 61” in white ink. They are supplied in high-density polyethylene (HDPE) bottles with a closure. A pack of 40 soft capsules is provided.

Scientific Opinion Holder
Pfizer Limited
Ramsgate Road
Sandwich, Kent
CT13 9NJ

United Kingdom

Manufacturer
Pfizer Limited
Ramsgate Road
Sandwich, Kent
CT13 9NJ
United Kingdom

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Additional information:

Informed Consent Form

Before your treatment starts, you will have the scheme explained to you using this leaflet and the EAMS patient consent form. You will be asked to sign this document and will be given a copy to keep.

Contact Details for Medical Information
Pfizer Limited Medical Information, tel +44 (0) 1304 616161