Package leaflet: Information for the patient

Opfolda 65 mg hard capsules
miglustat

Read all of this leaflet carefully before you take 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 Opfolda is and what it is used for

What Opfolda is
Opfolda is a medicine that is used in the treatment of late-onset Pompe disease in adults. This medicine contains the active substance 'miglustat'.

What it is used for
Opfolda is always used with another medicine called 'cipaglucosidase alfa', a type of enzyme replacement therapy (ERT). It is therefore very important that you also read the package leaflet of cipaglucosidase alfa.

If you have any questions about your medicines, please ask your doctor or pharmacist.

How Opfolda works
People with Pompe disease have low levels of the enzyme acid alpha-glucosidase (GAA). This enzyme helps control levels of glycogen (a type of carbohydrate) in the body.

In Pompe disease, high levels of glycogen build up in the muscles of the body. This keeps muscles, such as the muscles that help you walk, the muscles under the lungs that help you breathe, and the heart muscle, from working properly.

Opfolda binds to cipaglucosidase alfa during treatment. This makes the shape of cipaglucosidase alfa more stable, so it can be more easily absorbed from the blood by the muscle cells that are affected by Pompe disease. When in the cells, cipaglucosidase alfa works like GAA to help break down glycogen and control its levels.

2. What you need to know before you take Opfolda

Do not use Opfolda
- If you are allergic to miglustat or any of the other ingredients of this medicine (listed in section 6).
- If you are allergic to cipaglucosidase alfa.

Warnings and precautions
Talk to your doctor, pharmacist, or nurse before taking Opfolda.

Look out for serious side effects
Opfolda is used together with cipaglucosidase alfa, a type of enzyme replacement therapy (ERT), so you should also read the package leaflet of cipaglucosidase alfa. These medicines can cause side effects that you need to tell your doctor about straight away. This includes allergic reactions. Signs of allergic reactions are listed in section 4 'Allergic reactions'. These can be severe and may happen when you are being given the medicine or during the hours after.

Tell a doctor or nurse immediately if you are experiencing infusion-related or allergic reactions or think you may be experiencing them. Inform your doctor or nurse if you have ever had any such reaction with another ERT before you are given Opfolda.

Children and adolescents
This medicine should not be given to patients under the age of 18 years old. This is because the effects of Opfolda in combination with cipaglucosidase alfa in this age group are not known.

Other medicines and Opfolda
Tell a doctor or nurse if you are using, have recently used, or will be using any other medicines. This includes medicines obtained without a prescription, including herbal medicines.

Pregnancy and breast-feeding
If you are pregnant or breast-feeding, think you may be pregnant, or are planning to have a baby, do not take this medicine but talk to your doctor or pharmacist immediately for advice.

There is no experience with the use of Opfolda in combination with cipaglucosidase alfa during pregnancy. Your doctor will discuss with you the risks and benefits of taking these medicines.

- Do not take Opfolda and / or receive cipaglucosidase alfa if you are pregnant. Be sure to tell your doctor immediately if you get pregnant, think that you may be pregnant, or if you are planning to become pregnant. There may be risks to the unborn baby.
- Opfolda in combination with cipaglucosidase alfa should not be given to women who are breast-feeding. A decision will need to be made whether to stop treatment or to stop breast-feeding.

Contraception and fertility
Female patients of childbearing potential must use reliable birth control methods during and for 4 weeks after stopping both medicines.

Driving and using machines
Opfolda has no or negligible influence on your ability to drive or use machines. You should also read the package leaflet of cipaglucosidase alfa, as that medicine may have an impact.

3. How to take Opfolda

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

How much Opfolda to take
- Opfolda (miglustat) capsules must be used with cipaglucosidase alfa. See also the package leaflet of cipaglucosidase alfa.
- If you weigh 50 kg or more, the recommended dose is 4 capsules containing each 65 mg of miglustat.
- If you weigh between 40 kg and 50 kg, the recommended dose is 3 capsules.

How often to take Opfolda

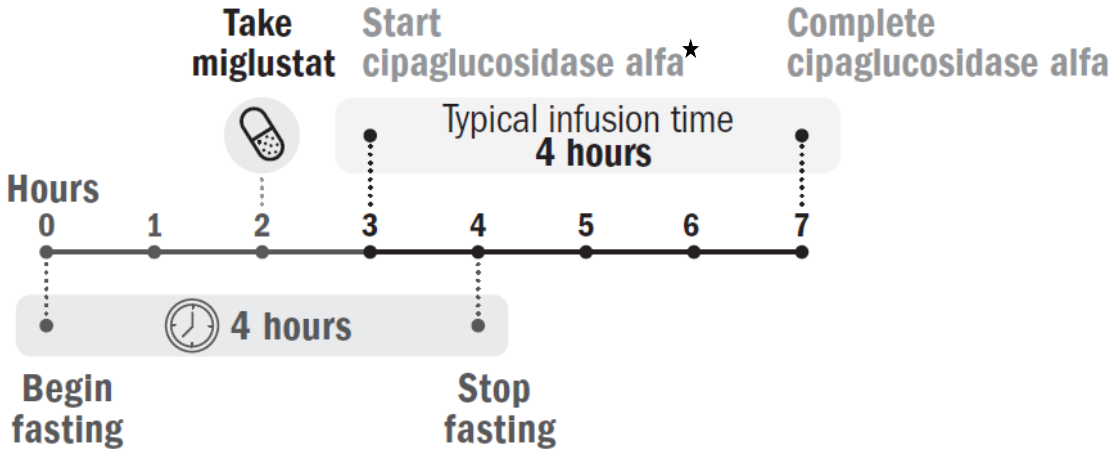
- You will receive Opfolda and cipaglucosidase alfa once every other week. Both are used on the same day.
- Take both medicines exactly as you have been told to by your doctor, see Figure 1. This is so your treatment can work as well as possible.

Opfolda with food

You must take Opfolda by mouth on an empty stomach.

- Fast for 2 hours before and 2 hours after taking this medicine.
- During this 4-hour fasting period, water, fat-free (skimmed) cow's milk, and tea or coffee can be consumed. Do not use cream, whole/semi-skimmed cow's milk, non-dairy milks, sugar, or sweeteners. You can have fat-free (skimmed) cow's milk with your tea or coffee.
- Two hours after taking Opfolda, you can resume normal eating and drinking.

Figure 1. Dose timeline



★Miglustat 65 mg hard capsules should be taken approximately 1 hour but no more than 3 hours before the start of the cipaglucosidase alfa infusion.

Switching from another enzyme replacement therapy (ERT)

If you are currently being treated with another ERT:

- Your doctor will tell you when to stop the other ERT before starting Opfolda.
- Tell your doctor when you completed your last dose.

If you take more Opfolda than you should

Tell your doctor immediately or go to the hospital if you accidentally take more capsules than you were prescribed. You may be at increased risk of experiencing side effects with this medicine (see section 4). Your doctor will provide appropriate supportive care.

If you forget to take Opfolda

If you miss a dose of Opfolda, please speak to your doctor or nurse. Contact your doctor or nurse immediately to reschedule miglustat in combination with cipaglucosidase alfa as soon as possible.

If you stop taking Opfolda

Speak to your doctor if you wish to stop Opfolda treatment. The symptoms of your disease may worsen if you stop treatment.

4. Possible side effects

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

Opfolda is used with cipaglucosidase alfa, and side effects can occur with either of these medicines.

The following side effects may occur:

Allergic reactions
Allergic reactions may include symptoms such as rash anywhere on the body, puffy eyes, prolonged difficulty breathing, cough, swelling of the lip, tongue, or throat, itchy skin, and hives.

Tell a doctor or nurse immediately if you are experiencing or think you may be experiencing allergic reactions. Inform your doctor or nurse if you have ever had any such reaction.

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

Common (may affect up to 1 in 10 people)
- Shortness of breath (dyspnoea)
- Sudden reddening of the face, neck, or upper chest
- Rise in blood pressure
- Stomach pain
- Bloating
- Passing gas or wind
- Loose, runny stools
- Trouble passing stools
- Vomiting
- Fatigue
- Nausea
- Fever
- Very itchy hives (urticaria)
- Itchy rash, wanting to scratch (pruritis)
- Chills
- Muscle cramps, muscle pain, muscle weakness
- Involuntary shaking of one or more parts of the body
- Increased sweating
- Pain
- Altered sense of taste

Uncommon (may affect up to 1 in 100 people)
- Asthma
- Allergic reaction
- Uneasy stomach
- Indigestion
- Sore or irritated throat
- Painful and abnormal contractions of the throat
- Feeling of uneasiness, overall feeling of being sluggish
- Feeling jittery
- Swelling in the hands, feet, ankles, legs
- Constant feeling of being tired
- Unusual paleness of the skin

- Low blood pressure
- Decrease in platelets or a type of white blood cell – shown in tests
- Pain in joints
- Pain in the area between the hip and rib
- Muscle fatigue
- Increased rigidity of muscles
- Feeling drowsy
- Pain in one or both sides of the head, throbbing pain, aura, eye pain, sensitivity to light (migraine)
- Skin discolouration
- Balance disorder

Reporting of side effects

If you get any side effects, talk to your doctor or nurse. This includes any possible side effects not listed in this leaflet. You can also report side effects directly (see details below). By reporting side effects you can help provide more information on the safety of this medicine.

United Kingdom

Yellow Card Scheme

Website: www.mhra.gov.uk/yellowcard

or search for MHRA Yellow Card in the Google Play or Apple App Store

5. How to store Opfolda

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 the letters "EXP". The expiry date refers to the last date 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 that you no longer use. These measures will help protect the environment.

6. Contents of the pack and other information

What Opfolda contains

- The active substance is miglustat. Each hard capsule contains 65 mg of miglustat.

- The other ingredients are:

Capsule contents
Pregelatinised starch (maize)
Magnesium stearate (E470b)
Microcrystalline cellulose (E460i)
Sucralose (E955)
Colloidal silicon dioxide

Capsule shell
Gelatin
Titanium dioxide (E171)
Black iron oxide (E172)

Edible printing ink
Black iron oxide (E172)
Potassium hydroxide (E525)
Propylene glycol (E1520)
Strong ammonia solution (E527)
Shellac (E904)

What Opfolda looks like and contents of the pack

Bottles of 4 and 24 capsules.
Not all pack sizes may be marketed.
Size 2 hard capsule with a grey opaque cap and white opaque body with "AT2221" printed in black on the body, containing white to off-white powder.

Marketing Authorisation Holder

Amicus Therapeutics UK Limited
One Globeside
Fieldhouse Lane
Marlow, Buckinghamshire
SL7 1HZ

Manufacturer
Manufacturing Packaging Farmaca (MPF) B.V.
Neptunus 12, Heerenveen, 8448CN, Netherlands

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