

## **B. PACKAGE LEAFLET**

## Package leaflet: Information for the user

### Miglustat Gen.Orph 100 mg hard capsules miglustat

**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.

#### What is in this leaflet

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

#### 1. What Miglustat Gen.Orph is and what it is used for

Miglustat Gen.Orph contains the active substance miglustat which belongs to a group of medicines that affect metabolism. It is used to treat two conditions:

- **Miglustat Gen.Orph is used to treat mild to moderate type 1 Gaucher disease in adults.**

In type 1 Gaucher disease, a substance called glucosylceramide is not removed from your body. It starts to build up in certain cells of the body's immune system. This can result in liver and spleen enlargement, changes in the blood and bone disease.

The usual treatment for type 1 Gaucher disease is enzyme replacement therapy. Miglustat Gen.Orph is only used when a patient is considered unsuitable for treatment with enzyme replacement therapy.

- **Miglustat Gen.Orph is also used to treat progressive neurological symptoms in Niemann-Pick type C disease in adults and in children.**

If you have Niemann-Pick type C disease, fats such as glycosphingolipids build up in the cells of your brain. This can result in disturbances in neurological functions such as slow eye movements, balance, swallowing, and memory, and in seizures.

Miglustat Gen.Orph works by inhibiting the enzyme called 'glucosylceramide synthase' which is responsible for the first step in the synthesis of most glycosphingolipids.

#### 2. What you need to know before you take Miglustat Gen.Orph

##### Do not take Miglustat Gen.Orph

- if you are allergic to miglustat or any of the other ingredients of this medicine (listed in section 6)

##### Warnings and precautions

Talk to your doctor or pharmacist before taking Miglustat Gen.Orph

- if you suffer from kidney disease

- if you suffer from liver disease

Your doctor will perform the following tests before treatment and during treatment with Miglustat Gen.Orph:

- an examination to check the nerves in your arms and legs
- measurement of vitamin B12 levels
- monitoring growth if you are a child or adolescent with Niemann-Pick type C disease
- monitoring of blood platelet counts

The reason for these tests is that some patients have had tingling or numbness in the hands and feet, or a decrease in body weight, while taking Miglustat Gen.Orph. The tests will help the doctor decide whether these effects are due to your disease or other existing conditions, or due to side effects of Miglustat Gen.Orph (see section 4 for further details).

If you have diarrhoea, your doctor may ask you to change your diet to reduce your lactose and carbohydrate intake such as sucrose (cane sugar), or not to take Miglustat Gen.Orph together with food, or to temporarily reduce your dose. In some cases the doctor may prescribe anti-diarrhoeal medicines such as loperamide. If your diarrhoea does not respond to these measures, or if you have any other abdominal complaint, consult your doctor. In such case, your doctor may decide to conduct further investigations.

Male patients should use reliable birth control methods during their treatment with Miglustat Gen.Orph, and for 3 months after finishing treatment.

#### **Children and adolescents**

Do not give this medicine to children and adolescents (below 18 years old) with type 1 Gaucher disease because it is not known if it works in this disease.

#### **Other medicines and Miglustat Gen.Orph**

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

Tell your doctor if you are taking medicines containing imiglucerase, which are sometimes used at the same time as Miglustat Gen.Orph. They may lower the amount of Miglustat Gen.Orph in your body.

#### **Pregnancy, breast-feeding and fertility**

You should not take Miglustat Gen.Orph if you are pregnant or thinking of becoming pregnant. Your doctor can give you more information. You must use effective birth control while taking Miglustat Gen.Orph. Do not breastfeed while you are taking Miglustat Gen.Orph.

Male patients should use reliable birth control methods during their treatment with Miglustat Gen.Orph, and for 3 months after finishing treatment.

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

#### **Driving and using machines**

Miglustat Gen.Orph may make you feel dizzy. Do not drive or use any tools or machines if you feel dizzy.

#### **Miglustat Gen.Orph contains sodium**

This medicine contains less than 1 mmol sodium (23 mg) per hard capsule, that is to say essentially 'sodium-free'.

### **3. How to take Miglustat Gen.Orph**

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

- **For type 1 Gaucher disease:** for adults, the usual dose is one capsule (100 mg) three times a day (morning, afternoon and evening). This means a daily maximum of three capsules (300 mg).
- **For Niemann-Pick type C disease:** For adults and adolescents (over 12 years old), the usual dose is two capsules (200 mg) three times a day (morning, afternoon and evening). This means a daily maximum of six capsules (600 mg).

For children **less than 12 years old**, your doctor will adjust the dose for Niemann-Pick type C disease.

If you have a problem with your kidneys you may receive a lower starting dose. Your doctor may reduce your dose, e.g., to one capsule (100 mg) once or twice a day, if you suffer from diarrhoea when taking Miglustat Gen.Orph (see section 4). Your doctor will tell you how long your treatment will last.

Miglustat Gen.Orph can be taken with or without food. You should swallow the whole capsule with a glass of water.

#### **If you take more Miglustat Gen.Orph than you should**

If you take more capsules than you were told to, consult your doctor immediately. Miglustat has been used in clinical studies at doses up to 3000 mg: this caused decreases in white blood cells and other side effects similar to those described in section 4.

#### **If you forget to take Miglustat Gen.Orph**

Take the next capsule at the usual time. Do not take a double dose to make up for a forgotten dose.

#### **If you stop taking Miglustat Gen.Orph**

Don't stop taking Miglustat Gen.Orph without talking to your doctor.

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.

#### Most serious side effects:

**Some patients have had tingling or numbness in the hands and feet (seen commonly).** They could be signs of peripheral neuropathy, due to side effects of Miglustat Gen.Orph or they could be due to existing conditions. Your doctor will perform some tests before and during treatment with Miglustat Gen.Orph to assess this (see section 2).

**If you do get any of these effects, please seek medical advice from your doctor as soon as possible.**

**If you get a slight tremor, usually trembling hands, seek medical advice from your doctor** as soon as possible. The tremor often disappears without needing to stop the treatment. Sometimes your doctor will need to reduce the dose or stop Miglustat Gen.Orph treatment to stop the tremor.

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

The most common side effects are diarrhoea, flatulence (wind), abdominal (stomach) pain, weight loss and decreased appetite.

**If you do lose some weight** when you start treatment with Miglustat Gen.Orph don't worry. People usually stop losing weight as treatment goes on.

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

Common side effects of treatment include headache, dizziness, paraesthesia (tingling or numbness), abnormal coordination, hypoaesthesia (reduced sensation to touch), dyspepsia (heartburn), nausea (feeling sick), constipation and vomiting, swelling or discomfort in the abdomen (stomach) and thrombocytopenia (reduced levels of blood platelets). The neurological symptoms and thrombocytopenia could be due to the underlying disease.

**Other possible side effects** are muscular spasms or weakness, fatigue, chills and malaise, depression, difficulty sleeping, forgetfulness and less libido.

Most patients get one or more of these side effects, usually at the start of treatment or at intervals during treatment. Most cases are mild and disappear quite quickly. If any of these side effects cause problems, consult your doctor. He or she may reduce the dose of Miglustat Gen.Orph or recommend other medicines to help control side effects.

### **Reporting of side effects**

If you get any side effects, talk to your doctor or pharmacist. This includes any possible side effects not listed in this leaflet. You can also report side effects directly via Yellow Card Scheme Website: [www.mhra.gov.uk/yellowcard](http://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 Miglustat Gen.Orph**

Keep this medicine out of the sight and reach of children.

Do not take this medicine after the expiry date which is stated on the carton after 'EXP'. The expiry date refers to the last day 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 you no longer use. These measures will help protect the environment.

## **6. Contents of the pack and other information**

### **What Miglustat Gen.Orph contains**

- The active substance is miglustat 100 mg.
- The other ingredients are sodium starch glycolate (type A), povidone (K30), magnesium stearate, gelatin, titanium dioxide (E171). See section 2 "Miglustat Gen.Orph contains sodium".

### **What Miglustat Gen.Orph looks like and contents of the pack**

Miglustat Gen.Orph 100 mg hard capsules are white opaque cap and body, hard gelatin capsules size 4 of 14. mm length.

Pack size of 84 hard capsules in non-perforated blisters and 84x1 hard capsules in perforated unit dose blisters.

### **Marketing Authorisation Holder**

Gen.Orph  
185 Bureaux de la Colline  
92213 Saint Cloud Cedex  
France

### **Manufacturers**

Delpharm Reims  
10 rue Colonel Charbonneaux

51100 Reims  
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or  
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**This leaflet was last revised in December 2023**