

Package Leaflet: Information for the user

Miglustat Bluefish 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

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1. What Miglustat Bluefish is and what it is used for

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

- **Miglustat Bluefish 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 Bluefish is only used when a patient is considered unsuitable for treatment with enzyme replacement therapy.

- **Miglustat Bluefish 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 Bluefish 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 Bluefish

Do not take Miglustat Bluefish

- 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 Bluefish

- 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 Bluefish:

- 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 Bluefish. 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 Bluefish (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 Bluefish 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 Bluefish, 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 Bluefish

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 Bluefish. They may lower the amount of miglustat in your body.

Pregnancy, breast-feeding and fertility

You should not take Miglustat Bluefish 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 Bluefish. Do not breast-feed while you are taking Miglustat Bluefish.

Male patients should use reliable birth control methods during their treatment with Miglustat Bluefish, 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 Bluefish may make you feel dizzy. Do not drive or use any tools or machines if you feel dizzy.

Miglustat Bluefish 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 Bluefish

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 hard capsule (100 mg) three times a day (morning, afternoon and evening). This means a daily maximum of three hard 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 hard capsule (100 mg) once or twice a day, if you suffer from diarrhoea when taking Miglustat Bluefish (see section 4). Your doctor will tell you how long your treatment will last.

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

If you take more Miglustat Bluefish than you should

If you take more hard capsules than you were told to, consult your doctor immediately. Miglustat has been used in clinical trials 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 Bluefish

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

If you stop taking Miglustat Bluefish

Don't stop taking Miglustat Bluefish 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 Bluefish or they could be due to existing conditions. Your doctor will perform some tests before and during treatment with Miglustat Bluefish 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 Bluefish 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 Bluefish 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 Bluefish or recommend other medicines to help control side effects.

Reporting of side effects

If you get any side effects, talk to your doctor, pharmacist 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.

[To be completed nationally]

5. How to store Miglustat Bluefish

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 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 Miglustat Bluefish contains

- The active substances is miglustat. Each hard capsule contains 100 mg Miglustat.

The other ingredients are:

Capsule contents: sodium starch glycolate type A, povidone, magnesium stearate.

Capsule shell: titanium dioxide (E171), gelatin.

What Miglustat Bluefish looks like and contents of the pack

Miglustat Bluefish are white hard gelatin capsules, filled with a homogenous, white to off-white granulate.

Miglustat Bluefish is available in pack sizes of 84 hard capsules in perforated Aclar-Alu (PVC/PE/PCTFE-Alu) blisters.

Not all pack sizes may be marketed.

Marketing Authorisation Holder and Manufacturer

[To be completed nationally]

This medicinal product is authorised in the Member States of the EEA under the following names:

Germany	Miglustat Bluefish 100 mg Hartkapseln
Denmark	Miglustat Bluefish
France	Miglustat Bluefish 100 mg gélule
Norway	Miglustat Bluefish 100 mg kapsler, harde
Sweden	Miglustat Bluefish 100 mg hårda kapslar

This leaflet was last revised in 2023-07-07.