Patient leaflet in accordance with the Pharmacists' Regulations (Preparations) - 1986

This medicine is dispensed with a doctor's prescription only

Miglustat G.L. 100 mg
Capsules

Active ingredient and quantity
Each capsule contains: miglustat 100 mg

For a list of the inactive ingredients in this medicine: see section 2 under 'Important information about some of this medicine's ingredients', and section 6 'Additional information'.

Read the entire leaflet carefully before you start using this medicine. This leaflet contains concise information about this medicine.
If you have any further questions, consult your doctor or pharmacist.
This medicine has been prescribed to treat your illness. Do not pass it on to others. It may harm them, even if it seems to you that their medical condition is similar to yours.

This medicine is not intended for children and adolescents under the age of 18 years with type 1 Gaucher disease.

1. What is this medicine intended for?

- Treatment of mild to moderate type 1 Gaucher disease in patients who have been found unsuitable for enzyme replacement therapy.
- Treatment of progressive neurological symptoms in adults and children who suffer from Niemann-Pick type C disease.

Therapeutic group: An enzyme inhibitor that affects metabolism.

2. Before using this medicine

Do not use this medicine if:

- You are sensitive (allergic) to the active ingredient (miglustat) or to any of the other ingredients in this medicine (see section 6 – 'Additional information').
- You are pregnant or breastfeeding.

Special warnings about using this medicine

- **Before using Miglustat G.L. 100 mg, tell your doctor if:**
  - You suffer from kidney disease.
  - You suffer from liver disease.
  - You are sensitive to any food or medicine.

- If you suffer from diarrhea, your doctor may recommend a change in your diet to lower the amount of lactose and carbohydrates consumed (such as sucrose), or not to take Miglustat G.L. 100 mg together with food, or to temporarily reduce your dose. In some cases, your doctor may consider giving an anti-diarrheal medicine such as loperamide. If the diarrhea is not relieved by these measures, or if you suffer from another problem in your digestive system, consult your doctor. In such case, your doctor may decide to conduct further investigation.
- Male patients must use reliable birth control methods during the course of treatment with Miglustat G.L. 100 mg, and for 3 months after finishing treatment.

Children and adolescents
This medicine is not intended for children and adolescents under the age of 18 years with type 1 Gaucher disease.

Tests and follow-up
Before and during treatment with this medicine, the doctor will refer you to perform the following tests:

- An examination of nerves in the hands and feet
- Measurement of vitamin B_{12} levels
- Monitoring growth in children and adolescents with Niemann-Pick type C disease
- Monitoring of blood platelet count

The reason for these tests is that some patients may experience numbness or tingling in the hands and feet, or weight loss, during the course of treatment with the medicine. The tests will help the doctor decide whether these effects are due to your disease, due to other existing problems, or due to side effects of Miglustat G.L. 100 mg (see section 4 'Side effects').

Interactions with other medicines
If you are taking or have recently taken other medicines, including nonprescription medications and dietary supplements, tell your doctor or pharmacist. Particularly if you are taking:
- Medicines containing imiglucerase, which are sometimes given together with Miglustat G.L. 100 mg, may lower the amount of Miglustat G.L. 100 mg in your body.

Using this medicine and food
The medicine may be taken with or without food.

Pregnancy, breastfeeding and fertility
Do not use Miglustat G.L. 100 mg if you are pregnant or planning to become pregnant. You must use effective birth control methods during the course of treatment with Miglustat G.L. 100 mg.

Do not breastfeed during treatment with Miglustat G.L. 100 mg.

Male patients must use reliable birth control methods during the course of treatment with Miglustat G.L. 100 mg, and for 3 months after finishing the treatment.

If you are pregnant, breastfeeding, think you may be pregnant or planning to become pregnant, do not use this medicine before you consult your doctor. Your doctor will be able to give you more information.

Driving and using machines
Using this medicine may cause dizziness. Therefore, do not drive or operate dangerous machines if you feel dizzy.

Important information about some of this medicine's ingredients
Miglustat G.L. 100 mg contains sodium
This medicine contains less than 1 mmol sodium (23 mg) per capsule, that is to say essentially 'sodium free'.

3. How to use this medicine?

Always use this medicine according to your doctor's instructions. Check with your doctor or pharmacist if you are not sure about your dose or about how to take this medicine. Only your doctor will determine your dose and how you should take this medicine. The recommended dosage is usually:

- For type 1 Gaucher disease
  Adults: The dose is generally one capsule (100 mg) three times a day (morning, afternoon and evening). The maximum amount is three capsules (300 mg) per day.
- For Niemann-Pick type C disease
  Adults and adolescents (over 12 years of age): The dose is generally two capsules (200 mg) three times a day (morning, afternoon and evening). The maximum amount is six capsules (600 mg).
  Children less than 12 years of age: Your doctor will adjust the dose.

If you suffer from a problem with your kidneys, you may be prescribed a lower starting dose. If you suffer from diarrhea while taking Miglustat G.L. 100 mg, your doctor may consider reducing your dose, e.g., to one capsule (100 mg) once or twice a day (see section 2 'Special warnings about using this medicine'). Your doctor will explain to you how long your treatment will last.

Do not exceed the recommended dose.

- The medicine may be taken with or without food.
- Swallow the capsules whole with a glass of water.

If you have accidentally taken a higher dose
If you have taken an overdose, or if a child has accidentally swallowed some medicine, immediately see a doctor or go to a hospital emergency room and bring the medicine package with you.

During the use of miglustat in clinical trials at doses up to 3000 mg, a decrease in white blood cells was observed, as well as other side effects similar to those described in section 4 of this leaflet.

If you forget to take the medicine at the required time, take the next dose at the regular time and consult your doctor. Do not take a double dose to make up for a forgotten dose. Adhere to the treatment as recommended by your doctor.

If you stop taking this medicine
Do not stop taking the medicine without consulting your doctor.

Do not take medicines in the dark! Check the label and dose every time you take medicine. Wear glasses if you need them.
If you have any further questions about using this medicine, consult your doctor or pharmacist.

4. Side effects

As with any medicine, using Miglustat G.L. 100 mg may cause side effects in some users. Do not be alarmed by this list of side effects. You may not experience any of them.

Refer to the doctor immediately if any of the following side effects occurs:

- The most serious side effects:
  Some patients may have tingling or a pins-and-needles sensation in the hands and feet (seen commonly). These effects may be signs of peripheral nerve disease (neuropathy), due to side effects of Miglustat G.L. 100 mg or due to existing diseases. Your doctor will refer you to perform tests before and during treatment with Miglustat G.L. 100 mg to assess this (see section 2 'Tests and follow-up').
- If you feel a slight tremor, usually in the hands, refer to your doctor as soon as possible. In most cases, the tremor disappears without needing to stop the treatment, but sometimes your doctor will consider reducing the dose or stopping Miglustat G.L. 100 mg treatment to stop the tremor.

Additional side effects

Very common side effects – affect more than 1 in 10 patients:
Diarrhea, bloated abdomen (flatulence), abdominal pain, weight loss, decreased appetite.
If you lose weight when starting Miglustat G.L. 100 mg treatment, do not worry. This effect usually stops in patients as treatment goes on.

Common side effects – affect up to 1 in 10 patients:
Headaches, dizziness, pins-and-needles sensation or tingling, impaired coordination, reduced sense of touch, heartburn, nausea, constipation, vomiting, bloating or discomfort in the stomach, reduced level of blood platelets (thrombocytopenia). The neurological symptoms and the reduced level of blood platelets could be caused by the disease itself.

Other side effects include muscle spasms, muscle weakness, fatigue, chills and malaise, depression, insomnia, memory loss, reduced libido.

Most patients experience one or more of the effects mentioned above, usually at the start of treatment or at intervals during treatment. In most cases, these effects are mild and disappear relatively quickly. If these effects cause problems, refer to your doctor. Your doctor will consider reducing the dose of Miglustat G.L. 100 mg, or alternatively, recommending other medicines to treat these side effects.

If you experience any side effect, if any side effect gets worse, or if you experience a side effect not mentioned in this leaflet, consult your doctor.

You can report side effects to the Ministry of Health by following the 'Reporting Side Effects of Drug Treatment' link on the Ministry of Health home page (www.health.gov.il), which opens an online form for reporting side effects, or you can also use this link: https://sideeffects.health.gov.il

5. How to store the medicine?

- Prevent poisoning! To prevent poisoning, keep this and all other medicines in a closed place, out of the reach and sight of children and/or infants. Do not induce vomiting unless explicitly instructed to do so by your doctor.
- Do not use the medicine after the expiry date (exp. date), which is stated on the package. The expiry date refers to the last day of that month.
- Store below 25˚C.
- Do not throw away medicines via wastewater or household waste. Ask the pharmacist how to dispose of medicines you no longer use. These measures will

help protect the environment.

6. Additional information

In addition to the active ingredient, this medicine also contains:
Gelatin, sodium starch glycolate type A, povidone, titanium dioxide (E 171), magnesium stearate.

What the medicine looks like and contents of the pack:
Hard opaque white gelatin capsules.
The medicine is packaged in boxes of 4 trays (blisters); each tray contains 21 capsules – a total of 84 capsules.

Registration holder's name and address: A.L. Medi-Market Ltd., 3 Hakatif St., Emek Hefer Industrial Park, 3877701

Manufacturer's name and address: G.L. Pharma GmbH, Industriestrasse 1, A-8502 Lannach, Austria

Registration number of the medicine in the Ministry of Health's National Drug Registry: 173-42-36671-99

Revised in November 2023 according to Ministry of Health guidelines.