

What is Miglustat?

Miglustat is a prescription medicine. Globally, it is used alone or, in the U.S., in combination with arimoclomol to address neurological problems caused by Niemann-Pick Disease Type C (NPC) in patients 2 years and older. It helps slow down disease progression. ZAVESCA is manufactured by Johnson & Johnson and YARGESA is manufactured by Dexcel Pharma.

How It Works

Miglustat influences Niemann-Pick disease type C (NPC) in several ways. One mechanism of action involves blocking the body's production of specific fatty substances called glycosphingolipids. This particular approach is known as substrate reduction therapy. Additionally, it's thought that miglustat has other, more intricate effects that are not yet fully understood.

Why It Matters

Miglustat can stabilize or delay the progression of neurological symptoms. In long-term studies, miglustat has been associated with decreasing the risk of dying from NPC.

How to Take Miglustat

Form: Oral capsules

Route: By mouth

Food: Can be taken with or without food

Dosing Schedule

Your doctor will determine your specific dose. Clinicians may prescribe miglustat in combination with MIPLYFFA (arimoclomol) as per the FDA-approved MIPLYFFA prescribing information. Since miglustat is not formally approved in the US for NPC as a stand-alone therapy, clinicians may prescribe it "off label" as a monotherapy. Since miglustat is not approved in the U.S. for NPC, clinicians can prescribe doses based on the prescribing information in Europe where miglustat is approved for NPC by the European Medicines Agency (EMA). Alternatively, the FDA approved prescribing information in the US for Gaucher Disease type 1. Both are outlined below.

European Medicines Agency (EMA) Dosing for NPC:

Patient Group	Dose	Frequency	Total Daily Dose
Adults and Adolescents (≥12 years)	200 mg	3× per day	600 mg/day
Pediatric Patients (<12 years)	Based on BSA*	3× per day	Calculated dose

*Consult Summary of Product Characteristics (SmPC) for specific pediatric calculations

FDA-Approved Dosing for Gaucher Disease:

Patient Group	Dose	Frequency	Total Daily Dose
Adults with Type 1 Gaucher Disease	100 mg	3× per day	300 mg/day

What If You Miss a Dose?

- Take the next dose at the scheduled time
- Do not double-dose to make up for a missed one

Common Side Effects

- Diarrhea • Weight loss • Abdominal pain • Flatulence • Tremor (shaking) • Nausea • Headache
- Dizziness

Important Safety Information

- **Peripheral Neuropathy:** Miglustat can cause nerve damage, leading to numbness, tingling, or weakness. Your doctor will monitor for this
- **Tremor:** New or worsening tremor may occur
- **Gastrointestinal Effects:** Diarrhea and other GI issues are common
- **Kidney Function:** Not recommended for patients with severe kidney problems. Your doctor will monitor kidney function
- **Pregnancy:** May cause harm to an unborn baby. Do not take if pregnant or planning to become pregnant

Who Should NOT Take It?

- Anyone with a known allergy to miglustat or its ingredients
- Pregnant women or those planning to become pregnant
- Patients with severe kidney impairment
- Always inform your doctor about all health conditions and medicines

Storage & Safety Tips

- Store at room temperature
- Keep out of reach of children
- Follow your doctor's dosing schedule
- Keep all follow-up appointments for monitoring

Reporting Side Effects

In addition to reporting any side effects to your healthcare providers, you are encouraged to report side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088.

Questions?

Always talk to your doctor or pharmacist if you have questions about your medicine.

If you ever have questions about miglustat - how to take it, side effects, or anything else - please call your doctor or pharmacist.