Miglusca 100

Miglustat 100mg Capsule

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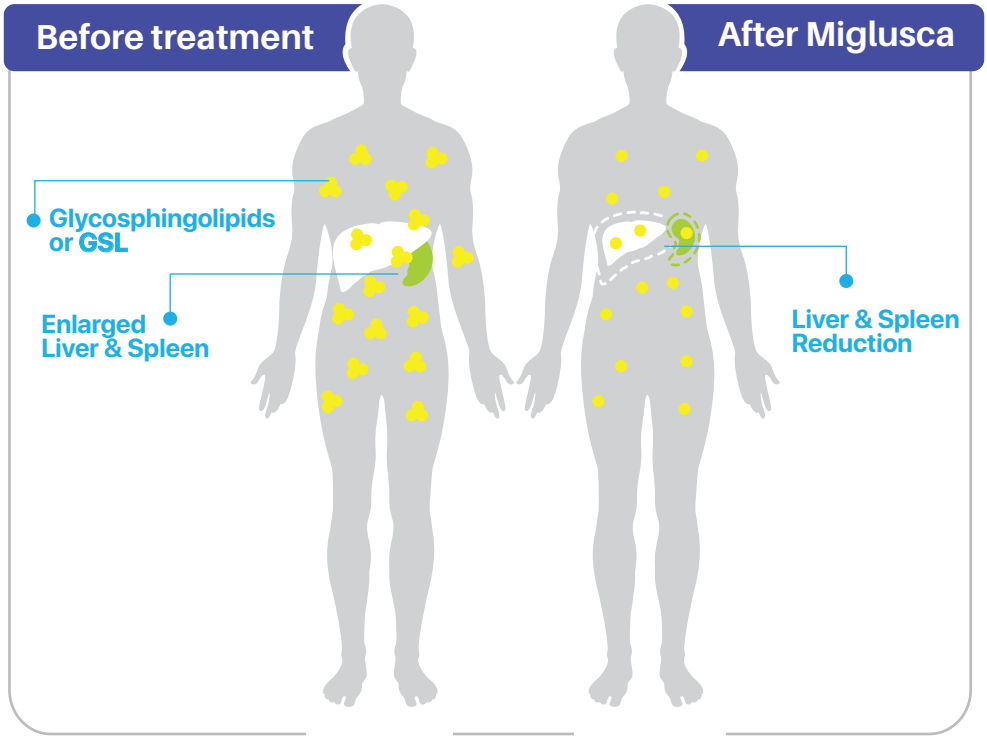
TODAY

Miglusca is a glucosylceramide synthase inhibitor; indicated as monotherapy for treatment of adult patients with mild to moderate type 1 Gaucher disease and also indicated for the treatment of progressive neurological manifestations in adult and pediatric patients with Niemann-Pick type C disease [1]. **Gaucher disease is equally prevalent in males and females, with a worldwide prevalence of 0.70 to 1.75 per 100,000 individuals** [2].

MORE THAN 90% Of GAUCHER DISEASE PATIENTS HAVE TYPE 1

There are 3 types of Gaucher disease.

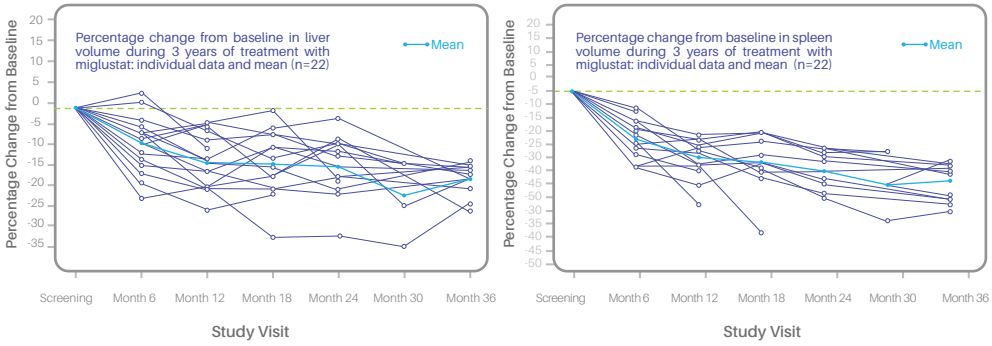
Gaucher disease type 1 can be effectively managed once a diagnosis is made.



- **Reduced swelling of the liver**
- **Reduced swelling of the spleen**

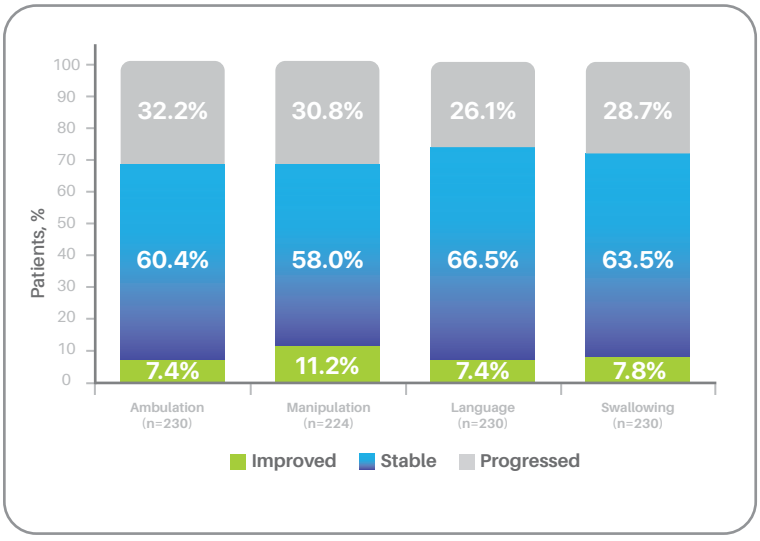
long-term therapeutic study in type I Gaucher disease [3]

The improvement in key clinical features of Gaucher disease was observed after 12 months of treatment with Miglustat. In this study, it observed continued and increasing efficacy, with sustained safety of Miglustat after 36 months of treatment. Liver and spleen volumes continued to decline during the second and third years of the study. No new cases of peripheral neuropathy were reported during this extended study.



Treatment outcomes following continuous Miglustat therapy in patients with Niemann-Pick disease Type C [4]

70.5% (n = 153/217) of the continuous Miglustat therapy population had improved or stable disease. Stable scores were observed in the majority of patients in all domains. Stable or decreased scores were observed for all domains: ambulation (n = 156/230; 67.8%), manipulation (n = 155/224; 69.2%), language (n = 170/230; 73.9%), and swallowing (n = 164/230; 71.3%).



Dosage and Administration [1]:

- Recommended dosage is 100mg administered orally three times a day at regular intervals.
- May reduce dosage to 100mg once or twice a day in some patients due to tremor or diarrhea.
- Adjust in patients with renal impairment:

Renal impairment	Adjusted Creatinine Clearance (in mL/min/1.73m2)	Recommendations
Mild	50-70	Start dose at 100mg twice a day
Moderate	30-50	Start dose at 100mg once a day
Severe	<30	Use is not recommended

Contraindications: None. **Warnings and Precautions:** •Peripheral neuropathy: Perform baseline and follow-up neurological evaluations at 6-month intervals in all patients. •Tremor: Reduce dose to ameliorate tremor or discontinue treatment if tremor does not resolve within days of dose reduction. •Diarrhea and weight loss: Evaluate for underlying gastrointestinal disease in patients who do not respond to usual interventions (e.g., diet modification). •Reductions in Platelet Count: Mild reductions in platelet counts without association with bleeding were observed in some patients. Monitoring of platelet counts is recommended. •Pregnancy and Lactation: may cause fetal harm and also Breastfeeding is not recommended. **Adverse reactions:** The most common adverse reactions (incidence ≥5%) are: diarrhea, weight loss, stomach pain, gas, nausea and vomiting, headache including migraine, tremor, leg cramps, dizziness, weakness, vision problems, thrombocytopenia, muscle cramps, back pain, constipation, dry mouth, heaviness in arms and legs, memory loss, unsteady walking, anorexia, indigestion, paresthesia, stomach bloating, stomach pain not related to food, and menstrual changes. **Drug interactions:** Co-administration of Miglustat and imiglucerase may lead to increased clearance of imiglucerase.

Rx Code: 71212

References:

1.FDA prescribing information, Miglustat. 2.Nalysnyk L, Rotella P, Simeone JC, et al. Gaucher disease epidemiology and natural history: a comprehensive review of the literature. Hematology 2017; 22: 65-73. 3. Elstein D, Hollak C, Aerts JM, van Weely S, Maas M, Cox TM, Lachmann RH, Hrebicek M, Platt FM, Butters TD, Dwek RA, Zimran A. Sustained therapeutic effects of oral miglustat (Zavesca, N-butyldeoxynojirimycin, OGT 918) in type I Gaucher disease. J Inherit Metab Dis. 2004;27(6):757-66. doi: 10.1023/B: BOLI.0000045756.54006.17. PMID: 15505381. 4.Patterson MC, Mengel E, Vanier MT, Moneuse P, Rosenberg D, Pineda M. Treatment outcomes following continuous miglustat therapy in patients with Niemann-Pick disease Type C: a final report of the NPC Registry. Orphanet J Rare Dis. 2020 Apr 25;15(1):104. doi: 10.1186/s13023-020-01363-2. PMID: 32334605; PMCID: PMC7183679.