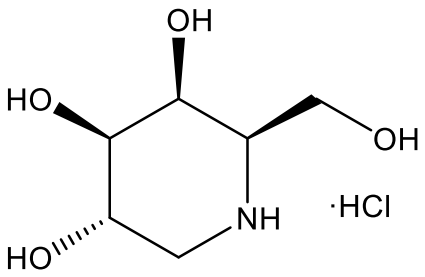
Catalog # 10-2632

Migalastat HCl

CAS# 75172-81-5

1-Deoxygalactonojirimycin HCl; (2R,3S,4R,5S)-2-(Hydroxymethyl)-3,4,5-piperidinetriol HCl; DGJ; GR181413; AT1001

Lot # E106438



A potent, selective, and orally available inhibitor of α-galactosidase A (IC_{50}= 40 nM).[1] It binds to mutant misfolded α-GalA shifting the folding behavior toward functional active conformation (pharmacological chaperone activity) followed by trafficking to the lysosomes.[2] Migalastat is active broadly across species from *Drosophila*[3] to human[4]. Clinically useful agent for Fabry disease.[5]

1) Asano *et al.* (2000), *In vitro inhibition and intracellular enhancement of lysosomal α-galactosidase A activity in Fabry lymphoblasts by 1-deoxygalactonojirimycin and its derivatives*; Eur. J. Biochem. **267** 4179
2) Siekierska *et al.* (2012), *α-Galactosidase aggregation is a determinant of pharmacological chaperone efficacy on Fabry disease mutants*; J. Biol. Chem. **287** 28386
3) Braunstein *et al.* (2020), *Misfolding of Lysosomal α-Galactosidase a in a Fly Model and Its Alleviation by the Pharmacological Chaperone Migalastat*; Int. J. Mol. Sci. **21** 7397
4) Benjamin *et al*. (2009), *The pharmacological chaperone 1-deoxygalactonojirimycin increases alpha-galactosidase A levels in Fabry patient cell lines*; J. Inherit. Metab. Dis. **32** 424
5) McCafferty and Scott (2019), *Migalastat: A Review in Fabry Disease*; Drugs **79** 543

PHYSICAL DATA

Molecular Weight:	199.63
Molecular Formula:	$C_6H_{13}O_4 \cdot HCl$
Purity:	>98% by TLC
	NMR: (Conforms)
Solubility:	Water (50 mg/ml)
Physical Description:	White solid
Storage and Stability:	Store as supplied at -20C for up to 2 years from the date of purchase. Solutions in water may be stored at -20°C for up to 2 months.

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