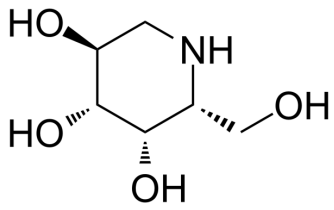
Migalastat

Cat. No.:	HY-14929
CAS No.:	108147-54-2
Molecular Formula:	$C_6H_{13}NO_4$
Molecular Weight:	163.17
Target:	Others
Pathway:	Others
Storage:	Please store the product under the recommended conditions in the Certificate of Analysis.



BIOLOGICAL ACTIVITY

Description	Migalastat (GR181413A free base) is an orally active and competitive inhibitor of α-galactosidase A (α-Gal A) with an IC_{50} of 0.04 μM for human α-Gal A[1].
IC_{50} & Target	IC50: 0.04 μM (human α-Gal A)[1]; Ki: 0.04 μM (human α-Gal A)[1]

In Vitro

Migalastat inhibits human lysosomal a-Gal A with IC_{50} and K_i values of 0.04 μM[1].

MCE has not independently confirmed the accuracy of these methods. They are for reference only.

Cell Viability Assay[4]

Cell Line:	EHK cells mutated α-Gal A
Concentration:	10 μM
Incubation Time:	9 days
Result:	Reduced Gb3 accumulation and lysosome volume.

In Vivo

Fabry disease is an X-linked recessive disorder caused by the deficient activity of α-galactosidase A[2].

Migalastat (oral gavage, 3 mg/kg daily for 4 weeks) increases α-Gal A activity in heart, kidney, spleen, and liver in a dose- and time-dependently in transgenic mice that express human mutant alpha-Gal A (TgM)[2].

Migalastat shows the half-life of less than 1 day in all major issues in TgM for 2 weeks pretreatment[2].

Migalastat (oral gavage, 100 mg/kg daily for 28 days) to transgenic mice reduces lyso-Gb3 levels up to 64%, 59%, and 81% in kidney, heart, and skin, respectively[3].

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Animal Model:	Male nontransgenic (Non-Tg) C57BL/6 mice; transgenic mice expressing human mutant R301Q α-Gal A (TgM), α-Gal A knockout mice (KO), mice express human R301Q α-Gal A in a null background (TgM/KO)[2]
Dosage:	3 mg/kg
Administration:	Oral gavage; every day for 4 weeks

Result:	Reduced Globotriaosylceramide (Gb3) storage remarkably in kidney of mice.

REFERENCES

[1]. Asano N, et al. In vitro inhibition and intracellular enhancement of lysosomal alpha-galactosidase A activity in Fabry lymphoblasts by 1-deoxygalactonojirimycin and its derivatives. Eur J Biochem. 2000 Jul;267(13):4179-86.

[2]. Ishii S, et al. Preclinical efficacy and safety of 1-deoxygalactonojirimycin in mice for Fabry disease. J Pharmacol Exp Ther. 2009 Mar;328(3):723-31.

[3]. Young-Gqamana B, et al. Migalastat HCl reduces globotriaosylsphingosine (lyso-Gb3) in Fabry transgenic mice and in the plasma of Fabry patients. PLoS One. 2013;8(3):e57631.

[4]. Welford RWD, et al. Glucosylceramide synthase inhibition with lucerastat lowers globotriaosylceramide and lysosome staining in cultured fibroblasts from Fabry patients with different mutation types. Hum Mol Genet. 2018 Oct. 27(19):3392-3403.

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