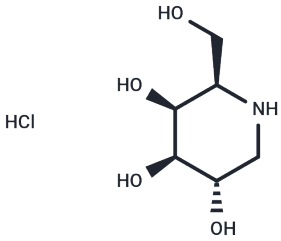
Data Sheet (Cat.No.T12038)

TargetMol

Migalastat hydrochloride

Chemical Properties



CAS No. : 75172-81-5

Formula: C6H14ClNO4

Molecular Weight: 199.63

Storage: Store at low temperature
Powder: -20°C for 3 years | In solvent: -80°C for 1 year
Actual storage temperature shall be subject to the COA.

Biological Description

Description	Migalastat hydrochloride (GR181413A) is an orally available, potent and competitive inhibitor of alpha-galactosidase A. It promotes the transport of alpha-galactosidase A to the lysosome and can be used in the study of Fabry disease.
Targets(IC50)	Others,glycosidase
In vitro	Migalastat hydrochloride (GR181413A) demonstrates IC50 and Ki values of 0.04 μM for human lysosomal alpha-Gal A[4].
In vivo	Fabry disease is an X-linked recessive disorder caused by deficient activity of alpha-galactosidase A (α-Gal A)[2]. In transgenic mice expressing the mutant human α-Gal A (TgM), oral administration of Migalastat hydrochloride (3 mg/kg per day for 4 consecutive weeks) results in a dose- and time-dependent increase in α-Gal A activity in the heart, kidneys, spleen, and liver[2].After a 2-week pretreatment with Migalastat hydrochloride, the half-life for all major issues is less than 1 day[2]. Administration of Migalastat hydrochloride (100 mg/kg per day orally for 28 days) in transgenic mice leads to a reduction of 64%, 59%, and 81% in globotriaosylceramide (Gb3) levels in the kidneys, heart, and skin, respectively[3].

Solubility Information

Solubility	H2O: 50 mg/mL (250.46 mM),Sonication is recommended. (< 1 mg/ml refers to the product slightly soluble or insoluble)

Preparing Stock Solutions

	1mg	5mg	10mg
1 mM	5.0093 mL	25.0463 mL	50.0927 mL
5 mM	1.0019 mL	5.0093 mL	10.0185 mL
10 mM	0.5009 mL	2.5046 mL	5.0093 mL
50 mM	0.1002 mL	0.5009 mL	1.0019 mL

Please select the appropriate solvent to prepare the stock solution, according to the solubility of the product in different solvents. Please use it as soon as possible.

Note: The dilution table applies only to solid products. For liquid products, please calculate the stock solution based on the stated concentration and/or density.

Reference

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.

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.

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.

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.

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