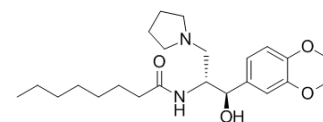


Eliglustat

Cat. No.:	HY-14885		
CAS No.:	491833-29-5		
Molecular Formula:	C ₂₃ H ₃₆ N ₂ O ₄		
Molecular Weight:	404.54		
Target:	Others		
Pathway:	Others		
Storage:	Powder	-20°C	3 years
		4°C	2 years
	In solvent	-80°C	6 months
		-20°C	1 month



Solvent & Solubility

In Vitro

DMSO : ≥ 100 mg/mL (247.19 mM)

* "≥" means soluble, but saturation unknown.

Preparing Stock Solutions	Solvent	Mass	1 mg	5 mg	10 mg
	Concentration				
	1 mM		2.4719 mL	12.3597 mL	24.7194 mL
	5 mM		0.4944 mL	2.4719 mL	4.9439 mL
	10 mM		0.2472 mL	1.2360 mL	2.4719 mL

Please refer to the solubility information to select the appropriate solvent.

BIOLOGICAL ACTIVITY

Description	Eliglustat is an specific, potent and orally active glucocerebrosidase inhibitor with an IC ₅₀ of 24 nM.
IC ₅₀ & Target	IC50: 24 nM (glucocerebrosidase) ^[1]
In Vitro	Eliglustat tartrate shows good potency with an IC ₅₀ of 24 nM and specificity against the target enzyme ^[1] . Incubating K562 or B16/F10 cells for 72 h with increasing amounts of Genz-112638 (0.6-1000 nM) results in a dose-dependent reduction of cell surface levels of both GM1 and GM3. The mean IC ₅₀ value for inhibiting the cell surface presentation of GM1 in K562 cells was 24 nM (range 14-34 nM) and that for GM3 in B16/F10 cells was 29 nM (range 12-48 nM) ^[1] .
In Vivo	Mice that received drug prior to significant accumulation of substrate (10 weeks of age) showed reduced levels of glucosylceramide and number of Gaucher cells in the spleen, lung and liver when compared to age-matched control animals ^[1] .

PROTOCOL

Cell Assay ^[1]

The inhibitory activity of Genz-112638 is determined indirectly by measuring its effect on the cell surface levels of the gangliosides GM1 and GM3 on either K562 or B16/F10 cells. GM1 levels on the K562 cells are determined by incubating the cells with increasing amounts of Genz-112638 (0.6-1000 nM) for 72 h after which the cells are harvested and stained using 10 µg of recombinant cholera toxin-FITC in 100 µL phosphate buffered saline (PBS) containing 0.5% bovine serum albumin (BSA) for 30 min on ice. Cells are washed, resuspended in PBS containing 0.5% BSA and the fluorescence quantitated^[1].

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

Animal Administration ^[1]

Mice: Eliglustat hemitartrate is dissolved in water for injection and administered in a dose escalation from 75 mg/kg/day to 150 mg/kg/day over the course of nine days, with three days at each dose and increments of 25 mg/kg/day. Mice are weighed three times per week to monitor the potential impact of the drug on their overall health. Animals are killed by carbon dioxide inhalation^[1].

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

REFERENCES

[1]. McEachern KA, et al. A specific and potent inhibitor of glucosylceramide synthase for substrate inhibition therapy of Gaucher disease. *Mol Genet Metab.* 2007 Jul;91(3):259-68.

Caution: Product has not been fully validated for medical applications. For research use only.

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