Lucerastat

Cat. No.: HY-106392

CAS No.: 141206-42-0

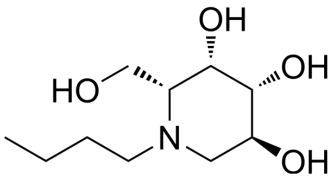
Molecular Formula: $C_{10}H_{21}NO_4$

Molecular Weight: 219.28

Target: Glucosylceramide Synthase (GCS)

Pathway: Neuronal Signaling

Storage: -20°C, sealed storage, away from moisture
* In solvent : -80°C, 6 months; -20°C, 1 month (sealed storage, away from moisture)



SOLVENT & SOLUBILITY

In Vitro

H_2O : 24 mg/mL (109.45 mM; ultrasonic and warming and heat to 80°C)
DMSO : 22 mg/mL (100.33 mM; ultrasonic and warming and heat to 80°C)

Preparing Stock Solutions

Concentration / Solvent / Mass	1 mg	5 mg	10 mg
1 mM	4.5604 mL	22.8019 mL	45.6038 mL
5 mM	0.9121 mL	4.5604 mL	9.1208 mL
10 mM	0.4560 mL	2.2802 mL	4.5604 mL

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

BIOLOGICAL ACTIVITY

Description

Lucerastat, the galactose form of Miglustat, is an orally-available inhibitor of glucosylceramide synthase (GCS). Lucerastat has the potential for Fabry disease study[1][2].

In Vitro

Fabry patient-derived fibroblasts with the genotypes R301G (residual -GalA activity; 20%) R220X (<3%) and W162X (<1%).
MCE has not independently confirmed the accuracy of these methods. They are for reference only.
Cell Viability Assay[2].

Cell Line:	Fabry patient-derived fibroblasts with the genotypes R301G (residual-GalA activity; 20%) R220X (<3%) and W162X (<1%).
Concentration:	
Incubation Time:	9 days.
Result:	Dose-dependently inhibited GCS, reducing glucosylceramide and increasing sphingomyelin.

In Vivo

Lucerastat (1200 mg/kg/day food admix), a GCS inhibitor, reduces Gb3 in the absence of residual-GalA activity[2].

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

Animal Model:	Fabry mice ($Gla^{-/0}$ and $Gla^{-/-}$, n = 5 or 6 for each gender)[2].
Dosage:	1200 mg/kg/day food admix.
Administration:	Food admix for 20 weeks.
Result:	Reduced lipid storage in two major organs affected by FD: mean Gb3 in the kidneys (-33%, $p<0.01$). and α-galactose- terminated glycosphingolipids in the dorsal root ganglia (-48%, $p<0.05$). In the liver of the Fabry mice, mean glucosylceramide (GlcCer (24:0)) was reduced (-59%, $p<0.001$) in addition to Gb3 (24:1) (-37%, $p<0.05$) demonstrating substrate reduction through GCS inhibition.

REFERENCES

[1]. Sanne J van der Veen, et al. Developments in the Treatment of Fabry Disease. J Inherit Metab Dis. 2020 Feb 21.

[2]. R.W.D. Welford, et al. Lucerastat, an Iminosugar for Substrate Reduction Therapy in Fabry Disease: Preclinical Evidence.

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