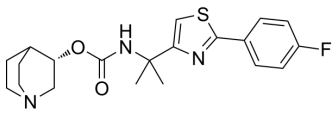
Ibiglustat

Cat. No.:	HY-16743
CAS No.:	1401090-53-6
Molecular Formula:	$C_{20}H_{24}FN_3O_2S$
Molecular Weight:	389.49
Target:	Glucosylceramide Synthase (GCS)
Pathway:	Neuronal Signaling
Storage:	Powder -20°C 3 years 4°C 2 years In solvent -80°C 1 year -20°C 6 months



SOLVENT & SOLUBILITY

In Vitro

DMSO : 50 mg/mL (128.37 mM; Need ultrasonic)

Preparing Stock Solutions

Solvent / Mass / Concentration	1 mg	5 mg	10 mg
1 mM	2.5675 mL	12.8373 mL	25.6746 mL
5 mM	0.5135 mL	2.5675 mL	5.1349 mL
10 mM	0.2567 mL	1.2837 mL	2.5675 mL

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

In Vivo

1. Add each solvent one by one: 10% DMSO >> 40% PEG300 >> 5% Tween-80 >> 45% saline
   Solubility: ≥ 2.5 mg/mL (6.42 mM); Clear solution
2. Add each solvent one by one: 10% DMSO >> 90% (20% SBE-β-CD in saline)
   Solubility: ≥ 2.5 mg/mL (6.42 mM); Clear solution
3. Add each solvent one by one: 10% DMSO >> 90% corn oil
   Solubility: ≥ 2.5 mg/mL (6.42 mM); Clear solution

BIOLOGICAL ACTIVITY

Description

Ibiglustat (Venglustat) is an orally active, brain-penetrant glucosylceramide synthase (GCS) inhibitor. Ibiglustat can be used for the research of Gaucher disease type 3, Parkinson's disease associated with GBA mutations, Fabry disease, GM2 gangliosidosis, and autosomal dominant polycystic kidney disease[1][2].

IC_{50} & Target

Glucosylceramide synthase[1].

In Vitro

Ibiglustat (SAR402671) (1 μM, 15 days; Fabry disease (FD) cells) is close to the physiological level in untreated WT cells in GL-

3 levels, suggesting that Ibiglustat can prevent additional GL-3 accumulation and could serve to ameliorate the abundant levels of this sphingolipid in FD cardiomyocytes[4]

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

CUSTOMER VALIDATION

- FASEB J. 2020 Dec;34(12):15922-15945.

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REFERENCES

[1]. Stojkovska I, et al. Molecular mechanisms of α-synuclein and GBA1 in Parkinson's disease. Cell Tissue Res. 2018;373(1):51-60. doi:10.1007/s00441-017-2704-y

[2]. Itier JM, et al. Effective clearance of GL-3 in a human iPSC-derived cardiomyocyte model of Fabry disease. J Inherit Metab Dis. 2014 Nov;37(6):1013-22.

[3]. Viel C, et al. Preclinical pharmacology of glucosylceramide synthase inhibitor venglustat in a GBA-related synucleinopathy model. Sci Rep. 2021;11(1):20945. Published 2021 Oct 22.

[4]. Peterschmitt MJ, et al. Pharmacokinetics, Pharmacodynamics, Safety, and Tolerability of Oral Venglustat in Healthy Volunteers. Clin Pharmacol Drug Dev. 2021;10(1):86-98.

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