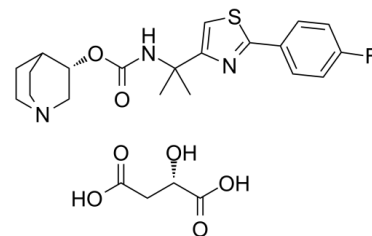


Data Sheet

Product Name:	Ibiglustat (L-Malic acid)
Cat. No.:	CS-0069906
CAS No.:	1629063-78-0
Molecular Formula:	C ₂₄ H ₃₀ FN ₃ O ₇ S
Molecular Weight:	523.57
Target:	Others
Pathway:	Others
Solubility:	DMSO : ≥ 100 mg/mL (191.00 mM)



BIOLOGICAL ACTIVITY:

Ibiglustat L-Malic acid (Venglustat L-Malic acid), a potential therapy for PD Parkinson's disease, SRT in Fabry's and Gaucher's, is a selective, allosteric inhibitor of **glucosylceramide synthase (GCS)** with ability to cross the blood-brain barrier^{[1][2][3]}. IC₅₀ & Target: Glucosylceramide synthase^[1]. **In Vitro:** Ibiglustat (SAR402671) (1 μM, 15 days) treated FD cells are 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].

References:

- [1]. WO 2015089067 A1.
- [2]. Iva Stojkovska, et al. Molecular mechanisms of α-synuclein and GBA1 in Parkinson's disease. Cell Tissue Res. 2017.
- [3]. Christoph Arenz, et al. Recent advances and novel treatments for sphingolipidoses. Future Med. Chem. (2017) 9(14), 1687–1700.
- [4]. 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.

CAIndexNames:

Butanedioic acid, 2-hydroxy-, (2S)-, compd. with (3S)-1-azabicyclo[2.2.2]oct-3-yl N-[1-[2-(4-fluorophenyl)-4-thiazolyl]-1-methylethyl]carbamate (1:1)

SMILES:

O=C(O[C@@H]1CN2CCC1CC2)NC(C)(C3=CSC(C4=CC=C(F)C=C4)=N3)C.OC(C[C@H](O)C(O)=O)=O

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

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