

# Product data sheet



MedKoo Cat#: 319731 Name: Lucerastat CAS: 141206-42-0 Chemical Formula: C <sub>10</sub> H <sub>21</sub> NO <sub>4</sub> Exact Mass: 219.1471 Molecular Weight: 219.281		
Product supplied as:	Powder	
Purity (by HPLC):	≥ 98%	
Shipping conditions	Ambient temperature	
Storage conditions:	Powder: -20°C 3 years; 4°C 2 years. In solvent: -80°C 3 months; -20°C 2 weeks.	

## 1. Product description:

Lucerastat is an extremely potent and selective  $\alpha$ -gal A (a-D-galactosidase) inhibitor for the treatment of lipid storage disorders and Fabry's disease.

## 2. CoA, QC data, SDS, and handling instruction

SDS and handling instruction, CoA with copies of QC data (NMR, HPLC and MS analytical spectra) can be downloaded from the product web page under "QC And Documents" section. Note: copies of analytical spectra may not be available if the product is being supplied by MedKoo partners. Whether the product was made by MedKoo or provided by its partners, the quality is 100% guaranteed.

## 3. Solubility data

Solvent	Max Conc. mg/mL	Max Conc. mM
DMF	20.0	91.21
DMSO	26.0	118.57
Ethanol	5.0	22.80
PBS (pH 7.2)	10.0	45.60
Water	24.0	109.45

## 4. Stock solution preparation table:

Concentration / Solvent Volume / Mass	1 mg	5 mg	10 mg
1 mM	4.56 mL	22.80 mL	45.60 mL
5 mM	0.91 mL	4.56 mL	9.12 mL
10 mM	0.46 mL	2.28 mL	4.56 mL
50 mM	0.09 mL	0.46 mL	0.91 mL

## 5. Molarity Calculator, Reconstitution Calculator, Dilution Calculator

Please refer the product web page under section of "Calculator"

## 6. Recommended literature which reported protocols for in vitro and in vivo study

In vitro study

1. Welford RWD, Mühlemann A, Garzotti M, Rickert V, Groenen PMA, Morand O, Üçeyler N, Probst MR. 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 1;27(19):3392-3403. doi: 10.1093/hmg/ddy248. PMID: 29982630; PMCID: PMC6140777.

In vivo study

TBD

## 7. Bioactivity

Biological target:

Lucerastat, the galactose form of Miglustat, is an orally-available inhibitor of glucosylceramide synthase (GCS).

In vitro activity

# Product data sheet



Lucerastat dose dependently reduced Gb3 in all cell lines. For 13 cell lines the Gb3 data could be fit to an IC<sub>50</sub> curve, giving a median IC<sub>50</sub> [interquartile range (IQR)] = 11  $\mu$ M (8.2-18); the median percent reduction (IQR) in Gb3 was 77% (70-83). Lucerastat treatment also dose dependently reduced LysoTracker Red staining of acidic compartments. Lucerastat's effects in the cell lines were compared to those with current treatments-agalsidase alfa and migalastat.

Reference: Hum Mol Genet. 2018 Oct 1;27(19):3392-3403. <https://pubmed.ncbi.nlm.nih.gov/29982630/>

In vivo activity

---

TBD

*Note: The information listed here was extracted from literature. MedKoo has not independently retested and confirmed the accuracy of these methods. Customer should use it just for a reference only.*