# **Product data sheet**



MedKoo Cat#: 314252				
Name: Eliglustat free base				
CAS#: 491833-29-5 (free base)				
Chemical Formula: C <sub>23</sub> H <sub>36</sub> N <sub>2</sub> O <sub>4</sub>				
Exact Mass: 404.2675				
Molecular Weight: 404.55				
Product supplied as:	Powder			
Purity (by HPLC):	$\geq 98\%$			
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:

Eliglustat, also known as GENZ-112638, (trade name Cerdelga) is a treatment for Gaucher's disease developed by Genzyme Corp that was approved by the FDA August 2014. Commonly used as the tartrate salt, Eliglustat is believed to work by inhibition of glucosylceramide synthase.

#### 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		
DMSO	90.0	222.47		
Ethanol	80.0	197.75		

#### 4. Stock solution preparation table:

Concentration / Solvent Volume / Mass	1 mg	5 mg	10 mg
1 mM	2.47 mL	12.36 mL	24.72 mL
5 mM	0.49 mL	2.47 mL	4.94 mL
10 mM	0.25 mL	1.24 mL	2.47 mL
50 mM	0.05 mL	0.25 mL	0.49 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. Wingerter A, El Malki K, Sandhoff R, Seidmann L, Wagner DC, Lehmann N, Vewinger N, Frauenknecht KBM, Sommer CJ, Traub F, Kindler T, Russo A, Otto H, Lollert A, Staatz G, Roth L, Paret C, Faber J. Exploiting Gangliosides for the Therapy of Ewing's Sarcoma and H3K27M-Mutant Diffuse Midline Glioma. Cancers (Basel). 2021 Jan 29;13(3):520. doi: 10.3390/cancers13030520. PMID: 33572900; PMCID: PMC7866294.

#### In vivo study

1. Cox TM. Eliglustat tartrate, an orally active glucocerebroside synthase inhibitor for the potential treatment of Gaucher disease and other lysosomal storage diseases. Curr Opin Investig Drugs. 2010 Oct;11(10):1169-81. PMID: 20872320.

#### 7. Bioactivity

Biological target: Eliglustat is a glucocerebroside synthase inhibitor with an IC50 of 24 nM.

#### In vitro activity

In order to target the ganglioside GD2 in H3K27M-mutant DMG (diffuse midline glioma), glycoysphingolipids synthesis was blocked in vitro with eliglustat, an inhibitor of the ceramide synthase (IC50 = 24 nM). Eliglustat acts as a substrate reduction therapy by

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reducing the production of glucosylceramide (Figure 1C). Eliglustat completely inhibited cell growth at a concentration of about 45–61.5  $\mu$ M (IC50) as demonstrated by impedance analysis (Figure 9), while cell growth at lower concentration ( $\leq 10 \mu$ M) was still observed.

Reference: Cancers (Basel). 2021 Jan 29;13(3):520. https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7866294/

#### In vivo activity

Eliglustat tartrate (Genz-112638) is a glucocerebroside (glucosylceramide) synthase inhibitor for the treatment of Gaucher disease and other lysosomal storage disorders. Gaucher disease is an inherited defect of lysosomal functions caused by mutations in the GBA1 gene leading to accumulation of glucocerebroside, primarily in macrophages. In vivo, eliglustat tartrate administered to Asp409Val/null mice lowered the concentrations of glucocerebroside in the liver, lung and spleen and reduced the number of Gaucher cells in the liver.

Reference: Curr Opin Investig Drugs. 2010 Oct;11(10):1169-81. https://pubmed.ncbi.nlm.nih.gov/20872320/

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.