PRODUCT INFORMATION



Eliglustat (hemitartrate)

Item No. 21487

CAS Registry No.: 928659-70-5

Formal Name: N-[(1R,2R)-2-(2,3-dihydro-1,4-

> benzodioxin-6-yl)-2-hydroxy-1-(1-pyrrolidinylmethyl) ethyl]-octanamide, 2R,3R-

dihydroxybutanedioate (2:1)

Synonym: Genz-112638

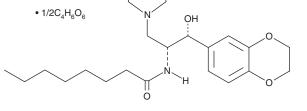
MF: $C_{23}H_{36}N_2O_4 \bullet 1/2C_4H_6O_6$

FW: 479.6 **Purity:** UV/Vis.:

 λ_{max} : 286 nm Supplied as: A crystalline solid

-20°C Storage: Stability: ≥4 years

Information represents the product specifications. Batch specific analytical results are provided on each certificate of analysis.



Laboratory Procedures

Eliglustat (hemitartrate) is supplied as a crystalline solid. A stock solution may be made by dissolving the eliglustat (hemitartrate) in the solvent of choice, which should be purged with an inert gas. Eliglustat (hemitartrate) is soluble in organic solvents such as ethanol, DMSO, and dimethyl formamide. The solubility of eliglustat (hemitartrate) in these solvents is approximately 20 mg/ml.

Eliglustat (hemitartrate) is sparingly soluble in aqueous buffers. For maximum solubility in aqueous buffers, eliglustat (hemitartrate) should first be dissolved in ethanol and then diluted with the aqueous buffer of choice. Eliglustat (hemitartrate) has a solubility of approximately 0.03 mg/ml in a 1:30 solution of ethanol:PBS (pH 7.2) using this method. We do not recommend storing the aqueous solution for more than one day.

Description

Eliglustat is an inhibitor of glucosylceramide synthase (IC50 = 40 nM for inhibition of glucosylceramide production in K562 cells). It is selective for glucosylceramide synthase over α-glucosidase I and II, α-1,6-glucosidase, lysosomal glucocerebrosidase, non-lysosomal glucosylceramidase, sucrase, and maltase $(IC_{50}s = >10 \mu M \text{ for all})$. It decreases cell surface levels of the gangliosides GM_1 and GM_3 in K562 and B16/F10 cells with IC50 values of 24 and 29 nM, respectively. Eliglustat (150 mg/kg per day) decreases glucosylceramide levels in the liver and lungs of D409V/null mice, a model of Gaucher disease. Formulations containing eliglustat have been used in the treatment of type 1 Gaucher disease.

Reference

1. McEachern, K.A., Fung, J., Komarnitsky, S., et al. A specific and potent inhibitor of glucosylceramide synthase for substrate inhibition therapy of Gaucher disease. Mol. Genet. Metab. 91(3), 259-267 (2007).

WARNING
THIS PRODUCT IS FOR RESEARCH ONLY - NOT FOR HUMAN OR VETERINARY DIAGNOSTIC OR THERAPEUTIC USE.

This material should be considered hazardous until further information becomes available. Do not ingest, inhale, get in eyes, on skin, or on clothing. Wash thoroughly after handling. Before use, the user must review the complete Safety Data Sheet, which has been sent via email to your institution.

WARRANTY AND LIMITATION OF REMEDY

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CAYMAN CHEMICAL

1180 EAST ELLSWORTH RD ANN ARBOR, MI 48108 · USA PHONE: [800] 364-9897

[734] 971-3335

FAX: [734] 971-3640 CUSTSERV@CAYMANCHEM.COM WWW.**CAYMANCHEM**.COM