Exaluren disulfate

Cat. No.: HY-114231B

CAS No.: 2244622-33-9

Molecular Formula: $C_{19}H_{42}N_4O_{18}S_2$

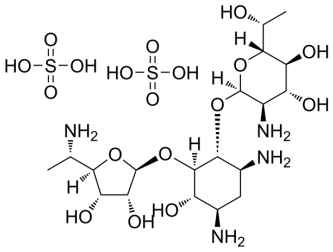
Molecular Weight: 678.68

Target: Others

Pathway: Others

Storage: -20°C, stored under nitrogen

* In solvent : -80°C, 6 months; -20°C, 1 month (stored under nitrogen)



SOLVENT & SOLUBILITY

In Vitro

H_2O : 100 mg/mL (147.34 mM; Need ultrasonic)

DMSO : < 1 mg/mL (insoluble or slightly soluble)

Preparing Stock Solutions

Solvent / Mass / Concentration	1 mg	5 mg	10 mg
1 mM	1.4734 mL	7.3672 mL	14.7345 mL
5 mM	0.2947 mL	1.4734 mL	2.9469 mL
10 mM	0.1473 mL	0.7367 mL	1.4734 mL

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

In Vivo

1. Add each solvent one by one: PBS
   Solubility: 100 mg/mL (147.34 mM); Clear solution; Need ultrasonic

BIOLOGICAL ACTIVITY

Description

Exaluren (ELX-02) disulfate is an investigational, advanced synthetic eukaryotic ribosome selective glycoside (ERSG). Exaluren disulfate is being developed as a therapy for genetic diseases caused by nonsense mutations[1].

In Vitro

Exaluren (ELX-02) disulfate (100-400 μg/mL) is not toxic, and permits read-through of nonsense mutations in human cells[1].

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

Cell Cytotoxicity Assay[1]

Cell Line:	Wildtype human proximal tubule cells (HK-2)
Concentration:	0, 100 and 400 μg/mL
Incubation Time:	0, 24, 48 and 72 hours

Result:	Cytotoxicity assay in wildtype human proximal tubule cells (HK-2) showing no toxic effect of 400 μg/mL at 0, 24, 48 and 72 h.

In Vivo

Exaluren (ELX-02) disulfate (10 and 30 mg/kg; repeat subcutaneous administration; twice weekly, total of 8 doses) shows accumulation in tissues that is dose dependent without gender difference[1].

In plasma Exaluren (ELX-02) disulfate is rapidly absorbed with a T_{max} of 0.25 h after both single (a single subcutaneous injection at 10 mg/kg at dose volume of 5 mL/kg) and repeated administration (twice weekly with 10 mg/kg/dose for 21 days; total of 7 administrations). Exaluren (ELX-02) disulfate is rapidly eliminated from plasma in a biphasic manner with the terminal half-life ($T_{1/2}$) of 0.5 h[1].

In a $Ctns^{Y226X}$ nonsense mutant mouse, subcutaneous Exaluren (ELX-02) disulfate accumulates in kidney tissue without overt renal toxicity and that Exaluren (ELX-02) disulfate (10 mg/kg X2/week for 3 weeks) reduces renal cystine accumulation in vivo[1].

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

Animal Model:	Twenty-nine $Ctns^{Y226X/Y226X}$ mice 5-7 month old[1]
Dosage:	10 and 30 mg/kg
Administration:	Subcutaneous injection, at dose volume of 5 mL/kg, twice weekly for a period of 28 days (total of 8 doses)
Result:	Highest levels were measured in the kidney, followed by spleen and liver, with lower levels in other tissues (lung, heart, cochlea and brain).

CUSTOMER VALIDATION

- Mol Ther. 2023 Jan 13;S1525-0016(23)00014-X.

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REFERENCES

[1]. Leubitz A, et al. Safety, Tolerability, and Pharmacokinetics of Single Ascending Doses of ELX-02, a Potential Treatment for Genetic Disorders Caused by Nonsense Mutations, in Healthy Volunteers. Clin Pharmacol Drug Dev. 2019 Jan 16.

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