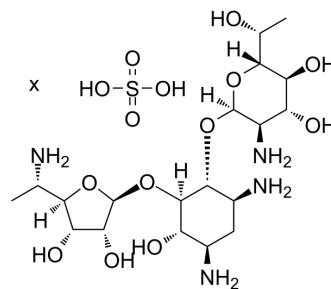


Exaluren sulfate

Cat. No.:	HY-114231C
CAS No.:	1375073-94-1
Molecular Formula:	C ₁₉ H ₄₀ N ₄ O ₁₄ S
Molecular Weight:	580.6
Target:	Others
Pathway:	Others
Storage:	Please store the product under the recommended conditions in the Certificate of Analysis.



BIOLOGICAL ACTIVITY

Description	Exaluren (ELX-02) sulfate is an investigational, advanced synthetic eukaryotic ribosome selective glycoside (ERSG). Exaluren sulfate is being developed as a therapy for genetic diseases caused by nonsense mutations ^[1] .								
In Vitro	<p>Exaluren (ELX-02) sulfate (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.</p> <p>Cell Cytotoxicity Assay^[1]</p> <table border="1"> <tr> <td>Cell Line:</td> <td>Wildtype human proximal tubule cells (HK-2)</td> </tr> <tr> <td>Concentration:</td> <td>0, 100 and 400 µg/mL</td> </tr> <tr> <td>Incubation Time:</td> <td>0, 24, 48 and 72 h</td> </tr> <tr> <td>Result:</td> <td>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.</td> </tr> </table>	Cell Line:	Wildtype human proximal tubule cells (HK-2)	Concentration:	0, 100 and 400 µg/mL	Incubation Time:	0, 24, 48 and 72 h	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.
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In Vivo	<p>Exaluren (ELX-02) sulfate (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].</p> <p>In plasma Exaluren (ELX-02) sulfate 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) sulfate is rapidly eliminated from plasma in a biphasic manner with the terminal half-life (T_{1/2}) of 0.5 h^[1].</p> <p>In a Ctns^{Y226X} nonsense mutant mouse, subcutaneous Exaluren (ELX-02) sulfate accumulates in kidney tissue without overt renal toxicity and that Exaluren (ELX-02) sulfate (10 mg/kg X2/week for 3 weeks) reduces renal cystine accumulation in vivo^[1].</p> <p>MCE has not independently confirmed the accuracy of these methods. They are for reference only.</p> <table border="1"> <tr> <td>Animal Model:</td> <td>Twenty-nine CtnsY226X/Y226X mice 5-7 month old^[1]</td> </tr> <tr> <td>Dosage:</td> <td>10 and 30 mg/kg</td> </tr> <tr> <td>Administration:</td> <td>Subcutaneous injection, at dose volume of 5 mL/kg, twice weekly for a period of 28 days (total of 8 doses)</td> </tr> </table>	Animal Model:	Twenty-nine CtnsY226X/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)		
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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.

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

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