

Givosiran

Cat. No.:	HY-132610
CAS No.:	1639325-43-1
Sequence:	RNA, (Cm-sp-Am-sp-Gm-Am-Am-Am-(2'-deoxy-2'-fluoro)G-Am-(2'-deoxy-2'-fluoro)G-U m-(2'-deoxy-2'-fluoro)G-Um-(2'-deoxy-2'-fluoro)C-Um-(2'-deoxy-2'-fluoro)C-Am-Um-Cm-Um-Um-Am), 3'-[[[(2S,4R)-1-[29-[[2-(acetylamino)-2-deoxy-β-D-galactopyranosyl]oxy]-14,14-bis[[3-[[3-[[5-[[2-(acetylamino)-2-deoxy-β-D-galactopyranosyl]oxy]-1-oxo-pentyl]amino]propyl]amino]-3-oxopropoxy]methyl]-1,12,19,25-tetraoxo-16-oxa13,20,24-triazanone-1-yl]-4-hydroxy-2-pyrrolidinyl]methyl hydrogen phosphate], complex with RNA (Um-sp-(2'-deoxy-2'-fluoro)A-sp-(2'-deoxy-2'-fluoro)A-(2'-deoxy-2'-fluoro)G-Am-(2'-deoxy-2'-fluoro)U-Gm-(2'-deoxy-2'-fluoro)A-Gm-(2'-deoxy-2'-fluoro)A-Cm-(2'-deoxy-2'-fluoro)A-Cm-(2'-deoxy-2'-fluoro)U-Cm-(2'-deoxy-2'-fluoro)U-Um-(2'-deoxy-2'-fluoro)U-Cm-(2'-deoxy-2'-fluoro)U-Gm-sp-Gm-sp-Um) (1:1)
Target:	Small Interfering RNA (siRNA)
Pathway:	Epigenetics
Storage:	-20°C, protect from light * In solvent : -80°C, 6 months; -20°C, 1 month (protect from light)

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BIOLOGICAL ACTIVITY

Description	Givosiran (ALN-AS1) is a small interfering RNA that targets hepatic aminolevulinate synthase 1 (ALAS1) messenger RNA. Givosiran downregulates ALAS1 mRNA and prevents accumulation of neurotoxic δ-aminolevulinic acid and porphobilinogen levels. Givosiran can be used for the research of acute intermittent porphyria ^{[1][2]} .
In Vitro	Givosiran is conjugated to a trivalent N-acetylgalactosamine ligand, which binds specifically to the asialoglycoprotein receptor, enabling targeted delivery to hepatocytes ^[2] . MCE has not independently confirmed the accuracy of these methods. They are for reference only.

REFERENCES

- [1]. Scott LJ. Givosiran: First Approval. *Drugs*. 2020 Feb;80(3):335-339.
- [2]. Balwani M, et, al. Phase 3 Trial of RNAi Therapeutic Givosiran for Acute Intermittent Porphyria. *N Engl J Med*. 2020 Jun 11;382(24):2289-2301.

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

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