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## Fazirsiran

Cat. No.:	HY-132604
CAS No.:	2175009-08-0
Molecular Weight:	15587.6
Sequence:	$ \begin{array}{l} RNA, ([1'-de(6-amino-9H-purin-9-yl)]dA-(5'\rightarrow 5')-sp-Am-Gm-Cm-Gm-Um-Um-Um-Am-(} \\ ARO-AAAT \\ ARO-AAAT \\ Um-Am-Am-Cm-Am-(3'\rightarrow 3')-sp-[1'-de(6-amino-9H-purin-9-yl)]dA),3'-[O-[cis-4-[(3S,8S)-17-[[2-(acetylamino)-2-deoxy-\beta-D-galactopyranosyl]oxy]-3,8-bis[[[2-[2-[[2-(acetylamino)-2-deoxy-\beta-D-galactopyranosyl]oxy]ethox]ethyl]amino]carbonyl]-1,6,11-trioxo-1 \\ \mathsf{5-oxa-2,7,12-triazaheptadec-1-yl]cyclohexyl] hydrogen phosphorothioate], complex \\ with \mathsf{RNA (Um-sp-(2'-deoxy-2'-fluoro)G-sp-Um-(2'-deoxy-2'-fluoro)U-Am-(2'-deoxy-2'-fluoro)U-Am-(2'-deoxy-2'-fluoro)A-Am-Cm-Am-Um-Gm-(2'-deoxy-2'-fluoro)C-Cm-(2'-deoxy-2'-fluoro)U-Am-(2'-deoxy-2'-fluoro)A-Am-(2'-deoxy-2'-fluoro)C-Gm-(2'-deoxy-2'-fluoro)C-sp-Um) (1:1) \\ \end{array}$
Target:	Small Interfering RNA (siRNA)
Pathway:	Epigenetics
Storage:	-20°C, sealed storage, away from moisture

BIOLOGICAL ACTIVITY		
Description	Fazirsiran (ARO-AAT) is a second-generation RNAi agent. Fazirsiran consistes of a cholesterol-conjugated RNAi trigger (chol- RNAi) to selectively degrade Alpha1-antitrypsin (AAT) mRNA by RNAi and a melittin-derived peptide conjugated to N- acetylgalactosamine (NAG) formulated as the excipient EX1 to promote endosomal escape of the chol-RNAi in hepatocytes <sup>[1]</sup> . Fazirsiran can be used in the study of Alpha-1 Antitrypsin Deficiency (AATD) liver disease.	
In Vivo	Fazirsiran (ARO-AAT; sc; 4 mg/kg; on 1, 15, 29, 43 days during 57 days) deeply reduces Z-AAT mRNA and protein in five-week- old male PiZ mice <sup>[1]</sup> . MCE has not independently confirmed the accuracy of these methods. They are for reference only.	

## REFERENCES

[1]. Christine I Wooddell, et al. Development of an RNAi therapeutic for alpha-1-antitrypsin liver disease. JCI Insight. 2020 Jun 18;5(12):e135348.

\* In solvent : -80°C, 6 months; -20°C, 1 month (sealed storage, away from moisture)

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

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