

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (GH-148)

EPLONTERSEN

PRONUNCIATION

ep" lon ter' sen

THERAPEUTIC CLAIM

Treatment of transthyretin-mediated amyloidosis (ATTR)

CHEMICAL NAMES

1. DNA, d([2'-O-(2-methoxyethyl)]m⁵rU-s^p-[2'-O-(2-methoxyethyl)]m⁵rC-[2'-O-(2-methoxyethyl)]m⁵rU-[2'-O-(2-methoxyethyl)]m⁵rU-[2'-O-(2-methoxyethyl)]rG-G-s^p-T-s^p-T-s^p-A-s^p-m⁵C-s^p-A-s^p-T-s^p-G-s^p-A-s^p-A-s^p-[2'-O-(2-methoxyethyl)]rA-[2'-O-(2-methoxyethyl)]m⁵rU-[2'-O-(2-methoxyethyl)]m⁵rC-s^p-[2'-O-(2-methoxyethyl)]m⁵rC-s^p-[2'-O-(2-methoxyethyl)]m⁵rC), 5'-[26-[[2-(acetamino)-2-deoxy-β-D-galactopyranosyl]oxy]-14,14-bis[[3-[[6-[[2-(acetamino)-2-deoxy-β-D-galactopyranosyl]oxy]hexyl]amino]-3-oxopropoxy]methyl]-8,12,19-trioxo-16-oxa-7,13,20-triazahexacos-1-yl hydrogen phosphate]
2. *all-P-ambo-5'-O-}{[(6-{5-[(tris{3-[6-(2-acetamido-2-deoxy-β-D-galactopyranosyloxy)hexylamino]-3-oxopropoxymethyl})methyl]amino-5-oxopentanamido}hexoxy)]phospho}-2'-O-(2-methoxyethyl)-5-methyl-P-thiouridylyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)-5-methylcytidylyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)-5-methyluridylyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)-5-methyluridylyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)guanylyl-(3'-O→5'-O)-2'-deoxy-P-thioguanilyl-(3'-O→5'-O)-P-thiothymidylyl-(3'-O→5'-O)-P-thiothymidylyl-(3'-O→5'-O)-2'-deoxy-P-thioadenilyl-(3'-O→5'-O)-2'-deoxy-5-methyl-P-thiocytidylyl-(3'-O→5'-O)-2'-deoxy-P-thioadenilyl-(3'-O→5'-O)-P-thiothymidylyl-(3'-O→5'-O)-2'-deoxy-P-thioguanilyl-(3'-O→5'-O)-2'-deoxy-P-thioadenilyl-(3'-O→5'-O)-2'-deoxy-P-thioadenilyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)adenilyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)-5-methyluridylyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)-5-methyl-P-thiocytidylyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)-5-methyl-P-thiocytidylyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)-5-methylcytidine*

STRUCTURAL FORMULA

(3'-5') R1-U=C-U-U-G-d(G=T=A=C=A=T=G=A=A)A-U-C=C=C

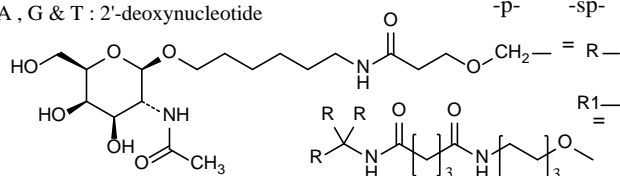
Legend:

C & U : 2'-O-(2-methoxyethyl)-5-methylnucleotide

A & G : 2'-O-(2-methoxyethyl)nucleotide

C & U : 2'-deoxy-5-methylnucleotide

A, G & T : 2'-deoxynucleotide



MOLECULAR FORMULA	C ₂₉₆ H ₄₃₇ N ₇₇ O ₁₅₆ P ₂₀ S ₁₃
MOLECULAR WEIGHT	8606.5 Da
TRADEMARK	None as yet
SPONSOR	Akcea Therapeutics
CODE DESIGNATIONS	ION-682884, AKCEA-TTR-L _{Rx} , IONIS-TTR-L _{Rx}
<u>CAS</u> REGISTRY NUMBER	1637600-16-8
UNII	0GRZ0F5XJ6
WHO NUMBER	11482

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