

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (HI-06)

SAPABLURSEN

PRONUNCIATION

sap" a blur' sen

THERAPEUTIC CLAIM

Treatment of beta thalassemia (β -thalassemia)

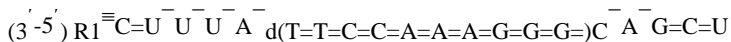
CHEMICAL NAMES

1. DNA, d([2'-O-(2-methoxyethyl)]rG-s^p-[2'-O-(2-methoxyethyl)]rG-[2'-O-(2-methoxyethyl)]rA-[2'-O-(2-methoxyethyl)]m⁵rC-[2'-O-(2-methoxyethyl)]rA-T-s^p-T-s^p-G-s^p-m⁵C-s^p-m⁵C-s^p-A-s^p-G-s^p-T-s^p-A-s^p-A-s^p-[2'-O-(2-methoxyethyl)]m⁵rU-[2'-O-(2-methoxyethyl)]m⁵rC-[2'-O-(2-methoxyethyl)]rG-s^p-[2'-O-(2-methoxyethyl)]m⁵rC-s^p-[2'-O-(2-methoxyethyl)]rA), 5'-[26-[[2-(acetylamino)-2-deoxy- β -D-galactopyranosyl]oxy]-14,14-bis[[3-[[6-[[2-(acetylamino)-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-(28-[(2-acetamido-2-deoxy- β -D-galactopyranosyl)oxy]-16,16-bis{[3-((6-[(2-acetamido-2-deoxy- β -D-galactopyranosyl)oxy]hexyl)amino)-3-oxopropoxy]methyl}-1-hydroxy-1,10,14,21-tetraoxo-2,18-dioxa-9,15,22-triaza-1 λ ⁵-phosphaoctacosan-1-yl)-2'-O-(2-methoxyethyl)-5-methyl-P-thiocytidylyl-(3'→5')-2'-O-(2-methoxyethyl)-5-methyluridylyl-(3'→5')-2'-O-(2-methoxyethyl)-5-methyluridylyl-(3'→5')-2'-O-(2-methoxyethyl)-5-methyluridylyl-(3'→5')-2'-O-(2-methoxyethyl)adenylyl-(3'→5')-P-thiothymidylyl-(3'→5')-P-thiothymidylyl-(3'→5')-2'-deoxy-5-methyl-P-thiocytidylyl-(3'→5')-2'-deoxy-5-methyl-P-thiocytidylyl-(3'→5')-2'-deoxy-P-thioadenylyl-(3'→5')-2'-deoxy-P-thioadenylyl-(3'→5')-2'-deoxy-P-thioadenylyl-(3'→5')-2'-deoxy-P-thioguanylyl-(3'→5')-2'-deoxy-P-thioguanylyl-(3'→5')-2'-O-(2-methoxyethyl)-5-methylcytidylyl-(3'→5')-2'-O-(2-methoxyethyl)adenylyl-(3'→5')-2'-O-(2-methoxyethyl)-P-thioguanylyl-(3'→5')-2'-O-(2-methoxyethyl)-5-methyl-P-thiocytidylyl-(3'→5')-2'-O-(2-methoxyethyl)-5-methyluridine*
3. *all-P-ambo-5'-O-[(6-{5-[(tris{3-[6-(2-acetamido-2-deoxy- β -D-galactopyranosyloxy)hexylamino]-3-oxopropoxymethyl})methyl]amino-5-oxopentanamido}hexyl)]phospho}-2'-O-(2-methoxyethyl)-5-methyl-P-thiocytidylyl-(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)-5-methyluridylyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)adenylyl-(3'-O→5'-O)-P-thiothymidylyl-(3'-O→5'-O)-P-thiothymidylyl-(3'-O→5'-O)-2'-deoxy-5-methyl-P-thiocytidylyl-(3'-O→5'-O)-2'-deoxy-5-methyl-P-thiocytidylyl-(3'-O→5'-O)-2'-deoxy-P-thioadenylyl-(3'-O→5'-O)-2'-deoxy-P-thioadenylyl-(3'-O→5'-O)-2'-deoxy-P-thioadenylyl-(3'-O→5'-O)-2'-deoxy-P-thioguanylyl-(3'-O→5'-O)-2'-deoxy-P-thioguanylyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)-5-methylcytidylyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)adenylyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)-P-thioguanylyl-(3'-O→5'-O)-2'-O-(2-*

methoxyethyl)-5-methyl-*P*-thiocytidylyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)-5-methyluridine

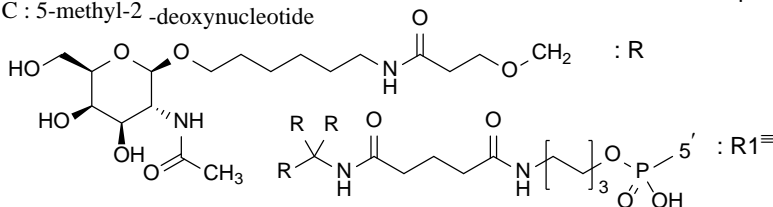
STRUCTURAL FORMULA

SEQ 1 cuuuattcca aaggcagcu



Legend:

C & U : 2'-*O*-(2-methoxyethyl)-5-methylnucleotide =: HS O - : HO O
A & G : 2'-*O*-(2-methoxyethyl)nucleotide 3' / \ 5' 3' / \ 5'
A, G, & T : 2'-deoxynucleotide -sp- -p-
C : 5-methyl-2'-deoxynucleotide



MOLECULAR FORMULA

$C_{296}H_{436}N_{80}O_{155}P_{20}S_{13}$

MOLECULAR WEIGHT

8631.5 Da

TRADEMARK

None as yet

SPONSOR

Ionis Pharmaceuticals Inc.

CODE DESIGNATIONS

ISIS 702843, IONIS-TMPRSS6-LR_x

CAS REGISTRY NUMBER

2273007-95-5

UNII

DXS4HJC32N

WHO NUMBER

11518

gbk