

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (hi-07)

SAPABLURSEN SODIUM

PRONUNCIATION

sap" a blur' sen

THERAPEUTIC CLAIM

Treatment of beta thalassemia (β -thalassemia)

CHEMICAL NAMES

1. DNA, d([2'-O-(2-methoxyethyl)]m⁵rC-s^p-[2'-O-(2-methoxyethyl)]m⁵rU-[2'-O-(2-methoxyethyl)]m⁵rU-[2'-O-(2-methoxyethyl)]m⁵rU-[2'-O-(2-methoxyethyl)]rA-T-s^p-T-s^p-m⁵C-s^p-m⁵C-s^p-A-s^p-A-s^p-A-s^p-G-s^p-G-s^p-G-s^p-[2'-O-(2-methoxyethyl)]m⁵rC-[2'-O-(2-methoxyethyl)]rA-[2'-O-(2-methoxyethyl)]rG-s^p-[2'-O-(2-methoxyethyl)]m⁵rC-s^p-[2'-O-(2-methoxyethyl)]m⁵rU), 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], sodium salt (1:20)
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, sodium salt (1:20)
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-methylcytidyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)adenyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)-P-thioguanyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)-5-methyl-P-thiocytidyl-(3'-O→5'-O)-2'-O-(2-methoxyethyl)-5-methyluridine, icosasodium salt

STRUCTURAL FORMULA

SEQ 1 cuuuattcca aagggcagcu

(3'-5') [R1≡C=U-U-U-A-d(T=T=C=C=A=A=A=G=G=G=C-A-G=C=U)]²⁰⁻ 20 Na⁺

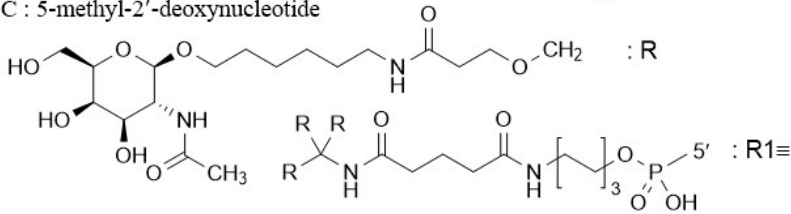
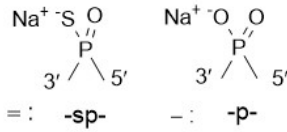
Legend:

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

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

A, G, & T : 2'-deoxynucleotide

C : 5-methyl-2'-deoxynucleotide



MOLECULAR FORMULA	C ₂₉₆ H ₄₁₆ N ₈₀ Na ₂₀ O ₁₅₅ P ₂₀ S ₁₃
MOLECULAR WEIGHT	9071.1 Da
TRADEMARK	None as yet
SPONSOR	Ionis Pharmaceuticals Inc.
CODE DESIGNATIONS	ISIS 702843 sodium
CAS REGISTRY NUMBER	2273008-00-5
UNII	6BGP5P5P2C

gbk