

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (FG-138)

FITUSIRAN

PRONUNCIATION

fit' ue sir' an

THERAPEUTIC CLAIM

Prevention or reduction of bleeding episodes in patients with hemophilia A or B

CHEMICAL NAMES

1. RNA, ((2'-deoxy-2'-fluoro)G-s^P-Gm-s^P-(2'-deoxy-2'-fluoro)U-Um-(2'-deoxy-2'-fluoro)A-Am-(2'-deoxy-2'-fluoro)C-Am-(2'-deoxy-2'-fluoro)C-(2'-deoxy-2'-fluoro)C-(2'-deoxy-2'-fluoro)A-Um-(2'-deoxy-2'-fluoro)U-Um-(2'-deoxy-2'-fluoro)A-Cm-(2'-deoxy-2'-fluoro)U-Um-(2'-deoxy-2'-fluoro)C-Am-(2'-deoxy-2'-fluoro)A), 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-oxopentyl]amino]propyl]amino]-3-oxopropoxy]methyl]-1,12,19,25-tetraoxo-16-oxa-13,20,24-triazanonacos-1-yl]-4-hydroxy-2-pyrrolidinyl]methyl hydrogen phosphate], complex with RNA (Um-s^P-(2'-deoxy-2'-fluoro)U-s^P-Gm-(2'-deoxy-2'-fluoro)A-Am-(2'-deoxy-2'-fluoro)G-Um-(2'-deoxy-2'-fluoro)A-Am-(2'-deoxy-2'-fluoro)A-Um-Gm-Gm-(2'-deoxy-2'-fluoro)U-Gm-(2'-deoxy-2'-fluoro)U-Um-(2'-deoxy-2'-fluoro)A-Am-(2'-deoxy-2'-fluoro)C-Cm-s^P-Am-s^P-Gm)
2. small interfering RNA (siRNA) inhibiting antithrombin liver production:
duplex of [(2S,4R)-1-{1-[(2-acetamido-2-deoxy-β-D-galactopyranosyl)oxy]-16,16-bis({3-[(3-{5-[(2-acetamido-2-deoxy-β-D-galactopyranosyl)oxy]pentanamido}propyl)amino]-3-oxopropoxy}methyl)-5,11,18-trioxo-14-oxa-6,10,17-triazanonacosan-29-oyl)-4-hydroxypyrrolidin-2-yl]methyl hydrogen (*all-P-ambo*)-2'-deoxy-2'-fluoro-*P*-thioguanlyl-(3'→5')-2'-O-methyl-*P*-thioguanlyl-(3'→5')-2'-deoxy-2'-fluorouridylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-deoxy-2'-fluoroadenylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-deoxy-2'-fluorocytidylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-deoxy-2'-fluorocytidylyl-(3'→5')-2'-deoxy-2'-fluorocytidylyl-(3'→5')-2'-deoxy-2'-fluoroadenylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-deoxy-2'-fluorouridylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-deoxy-2'-fluorouridylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-deoxy-2'-fluoroadenylyl-(3'→5')-2'-O-methylcytidylyl-(3'→5')-2'-deoxy-2'-fluorouridylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-deoxy-2'-fluorocytidylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-deoxy-2'-fluoroadenylate and (*all-P-ambo*)-2'-O-methyl-*P*-thiouridylyl-(3'→5')-2'-deoxy-2'-fluoro-*P*-thiouridylyl-(3'→5')-2'-O-methylguanylyl-(3'→5')-2'-deoxy-2'-fluoroadenylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-deoxy-2'-fluoroguanlylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-deoxy-2'-fluoroadenylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-deoxy-2'-fluoroadenylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-O-methylguanylyl-(3'→5')-2'-O-methylguanylyl-(3'→5')-2'-deoxy-2'-fluorouridylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-O-methylguanylyl-(3'→5')-2'-deoxy-2'-fluorouridylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-deoxy-2'-fluoroadenylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-deoxy-2'-fluorocytidylyl-(3'→5')-2'-O-methyl-*P*-thiocytidylyl-(3'→5')-2'-O-methyl-*P*-thioadenylyl-(3'→5')-2'-O-methylguanosine

STRUCTURAL FORMULA

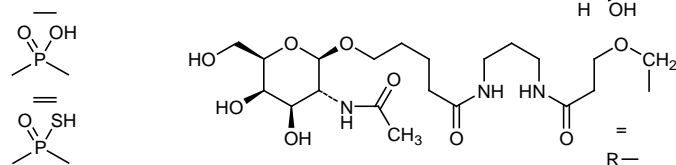
(3'-5') G=G-U-U-A-A-C-A-C-C-A-U-U-U-A-C-U-U-C-A-A-R1

(5'-3') G=A=C-C-A-A-U-U-G-U-G-U-A-A-U-G-A-A-G=U=U

Legend

X : 2'-deoxyx-2'-fluoro

X : 2'-O-methyl



MOLECULAR FORMULA

C₅₂₀H₆₇₉F₂₁N₁₇₅O₃₀₉P₄₃S₆

MOLECULAR WEIGHT

16,248 Da

TRADEMARK

None as yet

SPONSOR

Genzyme Corporation

CODE DESIGNATION

ALN-AT3SC

CAS REGISTRY NUMBER

1499251-18-1

UNII

SV9W47ZLE1

WHO NUMBER

10177

SCS