

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (HI-147)

VUTRISIRAN

PRONUNCIATION

vue" tri sir' an

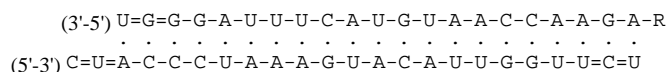
THERAPEUTIC CLAIM

Treatment of transthyretin (TTR)-mediated amyloidosis (ATTR amyloidosis)

CHEMICAL NAMES

1. RNA, (Um-s^p-(2'-deoxy-2'-fluoro)C-s^p-Um-Um-Gm-(2'-deoxy-2'-fluoro)G-Um-Um-(2'-deoxy-2'-fluoro)A-Cm-Am-Um-Gm-(2'-deoxy-2'-fluoro)A-Am-(2'-deoxy-2'-fluoro)A-Um-Cm-Cm-Cm-Am-s^p-Um-s^p-Cm), complex with RNA (Um-s^p-Gm-s^p-Gm-Gm-Am-Um-(2'-deoxy-2'-fluoro)U-Um-(2'-deoxy-2'-fluoro)C-(2'-deoxy-2'-fluoro)A-(2'-deoxy-2'-fluoro)U-Gm-Um-Am-Am-Cm-Cm-Am-Am-Gm-Am) 3'-[[[(2*S*,4*R*)-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] (1:1)
2. {(2*S*,4*R*)-1-[(2-acetamido-2-deoxy-β-D-galactopyranosyl)-17,17-bis-({3-[(3-{5-[(2-acetamido-2-deoxy-β-D-galactopyranosyl]oxy]-pentanamido}propyl)amino]-3-oxopropoxy}methyl)-6,12,19-trioxo-1,15-dioxa-7,11,18-triazatriacontan-30-oyl]-4-hydroxypyrrolidin-2-yl}methyl hydrogen *all-P-ambo*-2'-O-methyl-*P*-thiouridylyl-(3'→5')-2'-O-methyl-*P*-thioguanilyl-(3'→5')-2'-O-methylguanylyl-(3'→5')-2'-O-methylguanylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-deoxy-2'-fluorouridylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-deoxy-2'-fluorocytidylyl-(3'→5')-2'-deoxy-2'-fluoroadenylyl-(3'→5')-2'-deoxy-2'-fluorouridylyl-(3'→5')-2'-O-methylguanylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-O-methylcytidylyl-(3'→5')-2'-O-methylcytidylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-O-methylguanylyl-(3'→5')-2'-O-methyl-3'-adenylate duplex with *all-P-ambo*-2'-O-methyl-*P*-thiocytidylyl-(5'→3')-2'-O-methyl-*P*-thiouridylyl-(5'→3')-2'-O-methyladenylyl-(5'→3')-2'-O-methylcytidylyl-(5'→3')-2'-O-methylcytidylyl-(5'→3')-2'-O-methylcytidylyl-(5'→3')-2'-O-methyluridylyl-(5'→3')-2'-deoxy-2'-fluoroadenylyl-(5'→3')-2'-O-methyladenylyl-(5'→3')-2'-deoxy-2'-fluoroadenylyl-(5'→3')-2'-O-methylguanylyl-(5'→3')-2'-O-methyluridylyl-(5'→3')-2'-O-methyladenylyl-(5'→3')-2'-O-methylcytidylyl-(5'→3')-2'-deoxy-2'-fluoroadenylyl-(5'→3')-2'-O-methyluridylyl-(5'→3')-2'-O-methyluridylyl-(5'→3')-2'-deoxy-2'-fluoroguanilyl-(5'→3')-2'-O-methylguanylyl-(5'→3')-2'-O-methyluridylyl-(5'→3')-2'-O-methyl-*P*-thiouridylyl-(5'→3')-2'-deoxy-2'-fluoro-*P*-thiocytidylyl-(5'→3')-2'-O-methyluridine

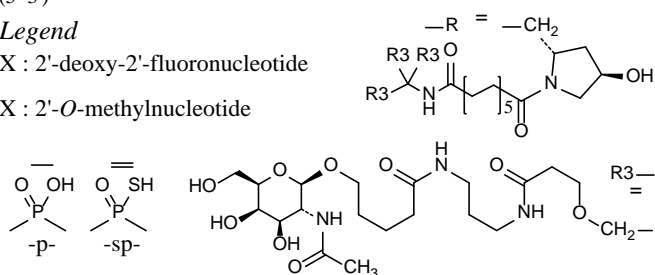
STRUCTURAL FORMULA



Legend

X : 2'-deoxy-2'-fluoronucleotide

X : 2'-O-methylnucleotide



MOLECULAR FORMULA

C₅₃₀H₇₁₅F₉N₁₇₁O₃₂₃P₄₃S₆

MOLECULAR WEIGHT

16345

TRADEMARK

None as yet

SPONSOR

Alnylam Pharmaceuticals, Inc.

CODE DESIGNATIONS

ALN-65492

CAS REGISTRY NUMBER

1867157-35-4

UNII

GB4I2JI8UI

WHO NUMBER

10579

gbk