

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (GH-49)

LUMASIRAN

PRONUNCIATION

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THERAPEUTIC CLAIM

Treatment of primary hyperoxaluria type 1

CHEMICAL NAMES

1. RNA, (Gm-s^P-Am-s^P-Cm-Um-Um-Um-(2'-deoxy-2'-fluoro)C-Am-(2'-deoxy-2'-fluoro)U-(2'-deoxy-2'-fluoro)C-(2'-deoxy-2'-fluoro)C-Um-Gm-Gm-Am-Am-Am-Um-Am-Um-Am), 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)A-s^P-Um-Am-Um-(2'-deoxy-2'-fluoro)U-Um-(2'-deoxy-2'-fluoro)C-(2'-deoxy-2'-fluoro)C-Am-Gm-Gm-Am-(2'-deoxy-2'-fluoro)U-Gm-(2'-deoxy-2'-fluoro)A-Am-Am-Gm-Um-Cm-s^P-Cm-s^P-Am) (1:1)
2. [(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'-O-methyl-*P*-thioguanlyl-(3'→5')-2'-O-methyl-*P*-thioadenyl-(3'→5')-2'-O-methylcytidyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-deoxy-2'-fluorocytidyl-(3'→5')-2'-O-methyladenyl-(3'→5')-2'-deoxy-2'-fluorouridylyl-(3'→5')-2'-deoxy-2'-fluorocytidyl-(3'→5')-2'-deoxy-2'-fluorocytidyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-O-methylguanylyl-(3'→5')-2'-O-methylguanylyl-(3'→5')-2'-O-methyladenyl-(3'→5')-2'-O-methyladenyl-(3'→5')-2'-O-methyladenyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-O-methyladenyl-(3'→5')-2'-O-methyladenylate duplex with *all-P-ambo*-2'-O-methyl-*P*-thioadenyl-(5'→3')-2'-O-methyl-*P*-thiocytidyl-(5'→3')-2'-O-methylcytidyl-(5'→3')-2'-O-methyluridylyl-(5'→3')-2'-O-methylguanylyl-(5'→3')-2'-O-methyladenyl-(5'→3')-2'-O-methyladenyl-(5'→3')-2'-deoxy-2'-fluoroadenyl-(5'→3')-2'-O-methylguanylyl-(5'→3')-2'-deoxy-2'-fluorouridylyl-(5'→3')-2'-O-methyladenyl-(5'→3')-2'-O-methylguanylyl-(5'→3')-2'-O-methylguanylyl-(5'→3')-2'-O-methyladenyl-(5'→3')-2'-deoxy-2'-fluorocytidyl-(5'→3')-2'-deoxy-2'-fluorocytidyl-(5'→3')-2'-O-methyluridylyl-(5'→3')-2'-deoxy-2'-fluorouridylyl-(5'→3')-2'-O-methyluridylyl-(5'→3')-2'-O-methyladenyl-(5'→3')-2'-O-methyl-*P*-thiouridylyl-(5'→3')-2'-deoxy-2'-fluoro-*P*-thioadenyl-(5'→3')-2'-O-methyluridine

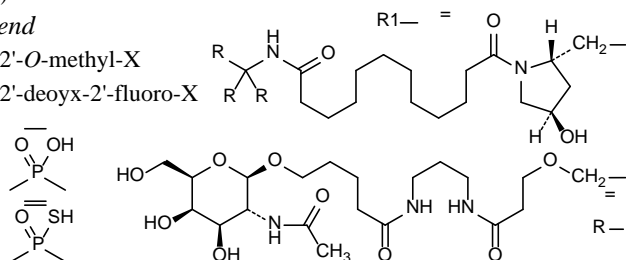
STRUCTURAL FORMULA

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

Legend

X : 2'-O-methyl-X

X : 2'-deoxyx-2'-fluoro-X



MOLECULAR FORMULA

C₅₃₀H₇₁₂F₁₀N₁₇₃O₃₂₀P₄₃S₆

MOLECULAR WEIGHT

16.34 kDa

TRADEMARK

None as yet

SPONSOR

Alnylam Pharmaceuticals Inc.

CODE DESIGNATIONS

ALN-G01, ALN-65585, AD-65585

CAS REGISTRY NUMBER

1834610-13-7

UNII

RZT8C352O1

WHO NUMBER

10684

gbk