

# STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (FG-124)

GIVOSIRAN

PRONUNCIATION

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THERAPEUTIC CLAIM

Treatment of Acute Hepatic Porphyria (AHP)

## CHEMICAL NAMES

1. RNA, (Cm-s<sup>P</sup>-Am-s<sup>P</sup>-Gm-Am-Am-Am-(2'-deoxy-2'-fluoro)G-Am-(2'-deoxy-2'-fluoro)G-Um-(2'-deoxy-2'-fluoro)G-Um-(2'-deoxy-2'-fluoro)C-Um-(2'-deoxy-2'-fluoro)C-Am-Um-Cm-Um-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<sup>P</sup>-(2'-deoxy-2'-fluoro)A-s<sup>P</sup>-(2'-deoxy-2'-fluoro)A-(2'-deoxy-2'-fluoro)G-Am-(2'-deoxy-2'-fluoro)U-Gm-(2'-deoxy-2'-fluoro)A-Gm-(2'-deoxy-2'-fluoro)A-Cm-(2'-deoxy-2'-fluoro)A-Cm-(2'-deoxy-2'-fluoro)U-Cm-(2'-deoxy-2'-fluoro)U-Um-(2'-deoxy-2'-fluoro)U-Cm-(2'-deoxy-2'-fluoro)U-Gm-s<sup>P</sup>-Gm-s<sup>P</sup>-Um) (1:1)
2. 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'-O-methyl-*P*-thiocytidylyl-(3'→5')-2'-O-methyl-*P*-thioadenylyl-(3'→5')-2'-O-methylguanylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-deoxy-2'-fluoroguanlylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-deoxy-2'-fluoroguanlylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-deoxy-2'-fluoroguanlylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-deoxy-2'-fluorocytidylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-deoxy-2'-fluorocytidylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-O-methylcytidylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-O-methyladenylate and (*all-P-ambo*)-2'-O-methyl-*P*-thiouridylyl-(5'→3')-2'-O-methyl-*P*-thioguanlylyl-(5'→3')-2'-O-methylguanylyl-(5'→3')-2'-deoxy-2'-fluorouridylyl-(5'→3')-2'-O-methylcytidylyl-(5'→3')-2'-deoxy-2'-fluorouridylyl-(5'→3')-2'-O-methyluridylyl-(5'→3')-2'-deoxy-2'-fluorouridylyl-(5'→3')-2'-O-methylcytidylyl-(5'→3')-2'-deoxy-2'-fluoroadenylyl-(5'→3')-2'-O-methylcytidylyl-(5'→3')-2'-deoxy-2'-fluoroadenylyl-(5'→3')-2'-O-methylguanylyl-(5'→3')-2'-deoxy-2'-fluoroadenylyl-(5'→3')-2'-O-methylguanylyl-(5'→3')-2'-deoxy-2'-fluorouridylyl-(5'→3')-2'-O-methyladenylyl-(5'→3')-2'-deoxy-2'-fluoroguanlylyl-(5'→3')-2'-deoxy-2'-fluoro-*P*-thioadenylyl-(5'→3')-2'-deoxy-2'-fluoro-*P*-thioadenylyl-(5'→3')-2'-O-methyluridine

## STRUCTURAL FORMULA

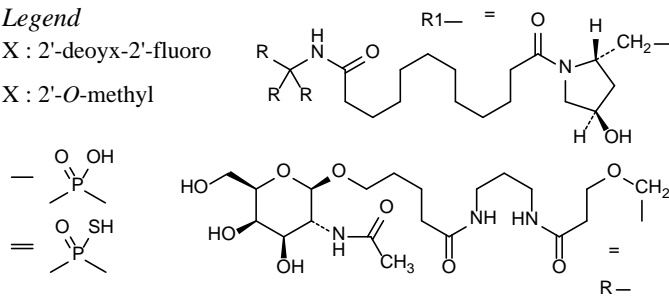
(3'-5') C=A-G-A-A-G-A-G-U-G-U-C-U-C-A-U-C-U-U-A-R1

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

### Legend

X : 2'-deoxyx-2'-fluoro

X : 2'-O-methyl



MOLECULAR FORMULA

C<sub>524</sub>H<sub>694</sub>F<sub>16</sub>N<sub>173</sub>O<sub>316</sub>P<sub>43</sub>S<sub>6</sub>

MOLECULAR WEIGHT

16.30 kDa

TRADEMARK

None as yet

SPONSOR

Anylam Pharmaceuticals, Inc.

CODE DESIGNATION

ALN-AS1

CAS REGISTRY NUMBER

1639325-43-1

UNII

ROV204583W

WHO NUMBER

10280

SCS