STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (OP-88)	AGAZISIRAN
PRONUNCIATION	ah gaz" i sir’ an
THERAPEUTIC CLAIM	Treatment of paroxysmal nocturnal hemoglobinuria (PNH)

CHEMICAL NAMES

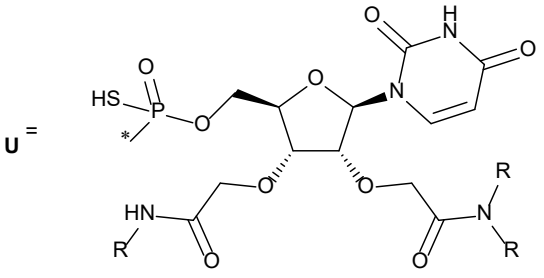
([3′-*O*-[2-[[[1-[17-[[2-(acetylamino)-2-deoxy-β-D-galactopyranosyl]oxy]-3,6,9,12,15-pentaoxaheptadec-1-yl]-1*H*-1,2,3-triazol-4-yl]methyl]amino]-2-oxoethyl]-2′-*O*-[2-[bis[[1-[17-[[2-(acetylamino)-2-deoxy-β-D-galactopyranosyl]oxy]-3,6,9,12,15-pentaoxaheptadec-1-yl]-1*H*-1,2,3-triazol-4-yl]methyl]amino]-2-oxoethyl]]U-(5′→5′)-sp-Gm-sp-Gm-Am-Am-Gm-Gm-Am-(2′-deoxy-2′-fluoro)U-Um-(2′-deoxy-2′-fluoro)A-(2′-deoxy-2′-fluoro)U-(2′-deoxy-2′-fluoro)C-(2′-deoxy-2′-fluoro)U-Gm-Gm-Am-Um-Gm-Um-Cm-Um-sp-Am-(3′→3′)-sp-[1′-de(6-amino-9*H*-purin-9-yl)]A), complex with RNA (Um-sp-(2′-deoxy-2′-fluoro)A-sp-Gm-(2′-deoxy-2′-fluoro)A-Cm-(2′-deoxy-2′-fluoro)A-Um-(2′-deoxy-2′-fluoro)C-Cm-(2′-deoxy-2′-fluoro)A-Gm-(2′-deoxy-2′-fluoro)A-Um-(2′-deoxy-2′-fluoro)A-Am-(2′-deoxy-2′-fluoro)U-Cm-(2′-deoxy-2′-fluoro)C-Um-(2′-deoxy-2′-fluoro)U-sp-Cm-sp-Cm) (1:1)

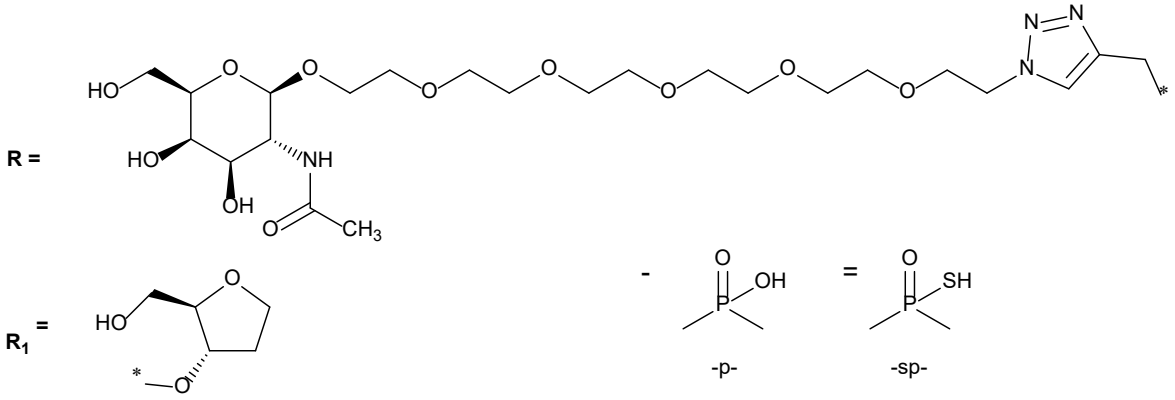
O-[(2*R*,3*S*)-2-(hydroxymethyl)oxolan-3-yl] hydrogen *all-P-ambo*-3'-*O*-(2-{[(1-{17-[(2-acetamido-2-deoxy-β-D-galactopyranosyl)oxy]-3,6,9,12,15-pentaoxaheptadecan-1-yl}-1*H*-1,2,3-triazol-4-yl)methyl]amino}-2-oxoethyl)-2'-*O*-(2-{bis[(1-{17-[(2-acetamido-2-deoxy-β-D-galactopyranosyl)oxy]-3,6,9,12,15-pentaoxaheptadecan-1-yl}-1*H*-1,2,3-triazol-4-yl)methyl]amino}-2-oxoethyl)-*P*-thiouridylyl-(5'→5')-2'-*O*-methyl-*P*-thioguanylyl-(3'→5')-2'-*O*-methylguanylyl-(3'→5')-2'-*O*-methyladenylyl-(3'→5')-2'-*O*-methyladenylyl-(3'→5')-2'-*O*-methylguanylyl-(3'→5')-2'-*O*-methylguanylyl-(3'→5')-2'-*O*-methyladenylyl-(3'→5')-2'-deoxy-2'-fluorouridylyl-(3'→5')-2'-*O*-methyluridylyl-(3'→5')-2'-deoxy-2'-fluoroadenylyl-(3'→5')-2'-deoxy-2'-fluorouridylyl-(3'→5')-2'-deoxy-2'-fluorocytidylyl-(3'→5')-2'-deoxy-2'-fluorouridylyl-(3'→5')-2'-*O*-methylguanylyl-(3'→5')-2'-*O*-methylguanylyl-(3'→5')-2'-*O*-methyladenylyl-(3'→5')-2'-*O*-methyluridylyl-(3'→5')-2'-*O*-methylguanylyl-(3'→5')-2'-*O*-methyluridylyl-(3'→5')-2'-*O*-methylcytidylyl-(3'→5')-2'-*O*-methyl-*P*-thiouridylyl-(3'→5')-2'-*O*-methyl-*P*-thio-3'-adenylate duplex with *all-P-ambo*-2'-*O*-methyl-*P*-thiocytidylyl-(5'→3')-2'-*O*-methyl-*P*-thiocytidylyl-(5'→3')-2'-deoxy-2'-fluorouridylyl-(5'→3')-2'-*O*-methyluridylyl-(5'→3')-2'-deoxy-2'-fluorocytidylyl-(5'→3')-2'-*O*-methylcytidylyl-(5'→3')-2'-deoxy-2'-fluorouridylyl-(5'→3')-2'-*O*-methyladenylyl-(5'→3')-2'-deoxy-2'-fluoroadenylyl-(5'→3')-2'-*O*-methyluridylyl-(5'→3')-2'-deoxy-2'-fluoroadenylyl-(5'→3')-2'-*O*-methylguanylyl-(5'→3')-2'-deoxy-2'-fluoroadenylyl-(5'→3')-2'-*O*-methylcytidylyl-(5'→3')-2'-deoxy-2'-fluorocytidylyl-(5'→3')-2'-*O*-methyluridylyl-(5'→3')-2'-deoxy-2'-fluoroadenylyl-(5'→3')-2'-*O*-methylcytidylyl-(5'→3')-2'-deoxy-2'-fluoroadenylyl-(5'→3')-2'-*O*-methyl-*P*-thioguanylyl-(5'→3')-2'-deoxy-2'-fluoro-*P*-thioadenylyl-(5'→3')-2'-*O*-methyluridine

STRUCTURAL FORMULA (see next page.)

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

X = 2'-O-methylnucleotide
X = 2'-deoxy-2'-fluoronucleotide





Sequence Length: 46 (24, 22)
13 a, 9 c, 9 g, 14 u, 1 x
multistranded (2); modified

Sequence Details

Sequence 1: Length 24; RNA; linear
1 uggaaggauu aucuggaugu cuax - -
Sequence 2: Length 22; RNA; linear

MOLECULAR FORMULA	$C_{534}H_{706}F_{15}N_{179}O_{329}P_{44}S_8$
MOLECULAR WEIGHT	16800.75
TRADEMARKS	None
SPONSOR	ADARx Pharmaceuticals, Inc.
DESIGNATION CODES	ADX-038, RD2830
CAS REGISTRY NUMBER	2985646-78-2
UNII	RHG8LAD9CP
WHO NUMBER	13364

SCS