

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (KL-191)

RAPIROSIRAN

PRONUNCIATION

ra peer" oh seer'an

THERAPEUTIC CLAIM

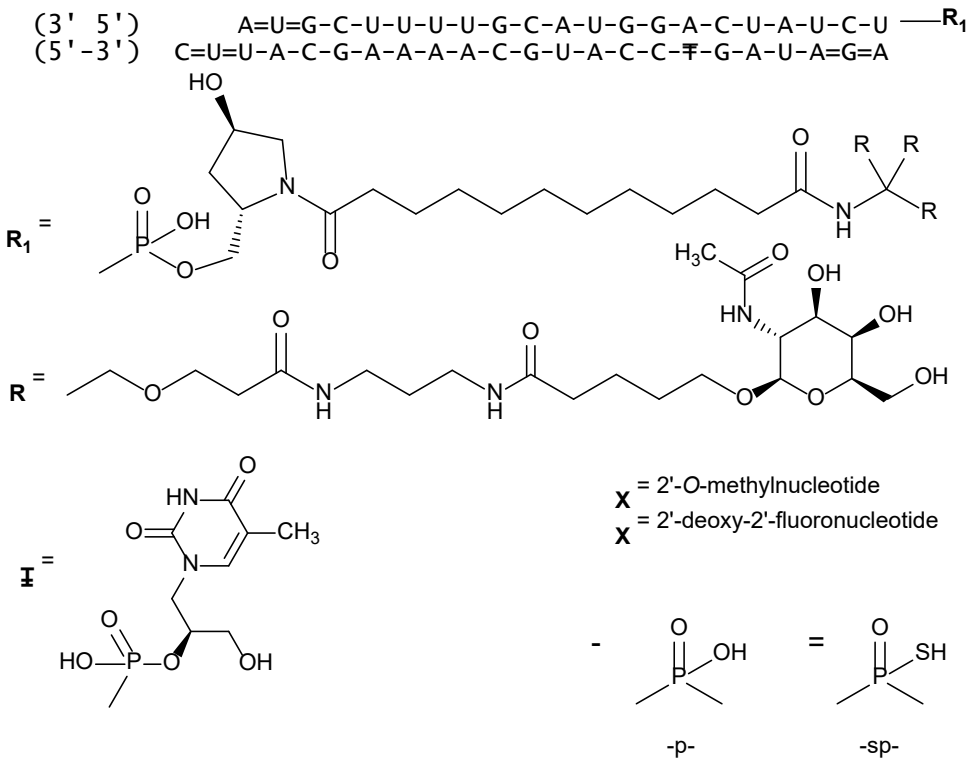
Treatment of nonalcoholic steatohepatitis (NASH)

CHEMICAL NAMES

RNA, (Am-s^P-(2'-deoxy-2'-fluoro)G-s^P-Am-Um-Am-Gm-(3'→3')-[1-de-β-D-ribofuranosyl-1-[(2S)-2,3-dihydroxypropyl]]m⁵U-(2'→5')-Cm-Cm-Am-Um-Gm-Cm-(2'-deoxy-2'-fluoro)A-Am-(2'-deoxy-2'-fluoro)A-Am-Gm-Cm-Am-Um-s^P-Um-s^P-Cm), complex with RNA (Am-s^P-Um-s^P-Gm-Cm-Um-Um-(2'-deoxy-2'-fluoro)U-Um-(2'-deoxy-2'-fluoro)G-(2'-deoxy-2'-fluoro)C-(2'-deoxy-2'-fluoro)A-Um-Gm-Gm-Am-Cm-Um-Am-Um-Cm-Um) 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] (1:1)

[(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 *all-P-ambo*-2'-O-methyl-*P*-thioadenylyl-(3'→5')-2'-O-methyl-*P*-thiouridylyl-(3'→5')-2'-O-methylguanylyl-(3'→5')-2'-O-methylcytidylyl 3'→5')-2'-O-methyluridylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-deoxy-2'-fluorouridylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-deoxy-2'-fluoroguanilyl-(3'→5')-2'-deoxy-2'-fluorocytidylyl-(3'→5')-2'-deoxy-2'-fluoroadenylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-O-methylguanylyl-(3'→5')-2'-O-methylguanylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-deoxy-2'-fluorocytidylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-O-methyladenylyl-(3'→5')-2'-O-methyluridylyl-(3'→5')-2'-O-methylcytidylyl-(3'→5')-2'-O-methyl-3'-uridylyl, duplex with *all-P-ambo*-2'-O-methyl-*P*-thiocytidylyl-(5'→3')-2'-O-methyl-*P*-thiouridylyl-(5'→3')-2'-O-methyluridylyl-(5'→3')-2'-O-methyladenylyl-(5'→3')-2'-O-methylcytidylyl-(5'→3')-2'-O-methylguanylyl-(5'→3')-2'-O-methyladenylyl-(5'→3')-2'-deoxy-2'-fluoroadenylyl-(5'→3')-2'-O-methyladenylyl-(5'→3')-2'-deoxy-2'-fluoroadenylyl-(5'→3')-2'-O-methylcytidylyl-(5'→3')-2'-O-methylguanylyl-(5'→3')-2'-O-methyluridylyl-(5'→3')-2'-O-methyladenylyl-(5'→3')-2'-O-methylcytidylyl-(5'→3')-2'-O-methylcytidylyl-(5'→2')-1-de-β-D-ribofuranosyl-1-[(2S)-2,3-dihydroxypropyl]-5-methylguanylyl-(3'→3')-2'-O-methyladenylyl-(5'→3')-2'-O-methyluridylyl-(5'→3')-2'-O-methyl-*P*-thioadenylyl-(5'→3')-2'-deoxy-2'-fluoro-*P*-thioguanilyl-(5'→3')-2'-O-methyladenosine

STRUCTURAL FORMULA (See next page.)



MOLECULAR FORMULA	C ₅₃₀ H ₇₁₉ F ₇ N ₁₇₁ O ₃₂₃ P ₄₃ S ₆
MOLECULAR WEIGHT	16307.22
TRADEMARKS	None
SPONSOR	Regeneron Pharmaceuticals, Inc.
DESIGNATION CODES	ALN-HSD; ALN-288996; AD-288996
CAS REGISTRY NUMBER	2755196-97-3
UNII	F3NU7CDG3E
WHO NUMBER	12805

SCS