

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (HI-160) SEPOFARSEN
PRONUNCIATION sep" oh far' sen
THERAPEUTIC CLAIM Treatment of Leber's congenital amaurosis type 10 (LCA10)
CHEMICAL NAMES

1. RNA, (*P*-thio)(Gm-Gm-Um-Gm-Gm-Am-Um-Cm-Am-Cm-Gm-Am-Gm-Um-Um-Cm-Am)
2. all-*P*-ambo-2'-*O*-methyl-*P*-thioguanlyl-(3'→5')-2'-*O*-methyl-*P*-thioguanlyl-(3'→5')-2'-*O*-methyl-*P*-thiouridylyl-(3'→5')-2'-*O*-methyl-*P*-thioguanlyl-(3'→5')-2'-*O*-methyl-*P*-thiouridylyl-(3'→5')-2'-*O*-methyl-*P*-thioadenylyl-(3'→5')-2'-*O*-methyl-*P*-thiouridylyl-(3'→5')-2'-*O*-methyl-*P*-thiocytidylyl-(3'→5')-2'-*O*-methyl-*P*-thioadenylyl-(3'→5')-2'-*O*-methyl-*P*-thiocytidylyl-(3'→5')-2'-*O*-methyl-*P*-thioguanlyl-(3'→5')-2'-*O*-methyl-*P*-thioadenylyl-(3'→5')-2'-*O*-methyl-*P*-thioguanlyl-(3'→5')-2'-*O*-methyl-*P*-thiouridylyl-(3'→5')-2'-*O*-methyl-*P*-thiouridylyl-(3'→5')-2'-*O*-methyl-*P*-thiocytidylyl-(3'→5')-2'-*O*-methyladenosine

STRUCTURAL FORMULA

(5'- 3') (2'-*O*-methyl) (*P*-thio) (mG-mG-mU-mG-mG-mA-mU-mC-mA-mC-mG-mA-mG-mU-mU-mC-mA)

MOLECULAR FORMULA C₁₈₀H₂₃₅N₆₇O₁₀₁P₁₆S₁₆
MOLECULAR WEIGHT 5962
TRADEMARK None as yet
SPONSOR ProQR Therapeutics
CODE DESIGNATIONS EW9I3PJC3R, QR-110
CAS REGISTRY NUMBER 2227173-68-2
UNII 7CAZ46X8EL
WHO NUMBER 11149

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