

May 26, 2016

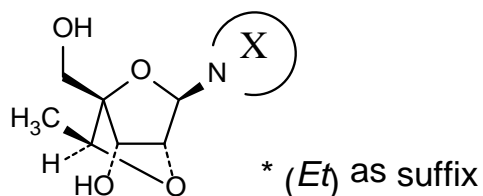
STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (CD-183)	BALIFORSEN
PRONUNCIATION	bal' i for' sen
THERAPEUTIC CLAIM	Treatment of Myotonic Dystrophy Type I

CHEMICAL NAMES

1. DNA, d(*P*-thio)([2'-*O*-(2-methoxyethyl)]m⁵rU-[2'-*O*-(2-methoxyethyl)]m⁵rC-(3'→4')-[2',5'-anhydro-6'-deoxy-4'-*C*-(hydroxymethyl)-α-*L*-mannofurano]m⁵C-(3'→4')-[2',5'-anhydro-6'-deoxy-4'-*C*-(hydroxymethyl)-α-*L*-mannofurano]m⁵C-G-A-A-T-G-T-m⁵C-m⁵C-(3'→4')-[2',5'-anhydro-6'-deoxy-4'-*C*-(hydroxymethyl)-α-*L*-mannofurano]G-(3'→4')-[2',5'-anhydro-6'-deoxy-4'-*C*-(hydroxymethyl)-α-*L*-mannofurano]A-[2'-*O*-(2-methoxyethyl)]m⁵rC-[2'-*O*-(2-methoxyethyl)]rA)
2. (*all-P-ambo*)-2'-*O*-(2-methoxyethyl)-5-methyl-*P*-thiouridylyl-(3'→5')-2'-*O*-(2-methoxyethyl)-5-methyl-*P*-thiocytidylyl-(3'→5')-2'-*O*,4'-*C*-[(1*S*)-ethane-1,1-diyl]-5-methyl-*P*-thiocytidylyl-(3'→5')-2'-*O*,4'-*C*-[(1*S*)-ethane-1,1-diyl]-5-methyl-*P*-thiocytidylyl-(3'→5')-2'-deoxy-*P*-thioguanylyl-(3'→5')-2'-deoxy-*P*-thioadenylyl-(3'→5')-2'-deoxy-*P*-thioadenylyl-(3'→5')-*P*-thiothymidylyl-(3'→5')-2'-deoxy-*P*-thioguanylyl-(3'→5')-*P*-thiothymidylyl-(3'→5')-2'-deoxy-5-methyl-*P*-thiocytidylyl-(3'→5')-2'-deoxy-5-methyl-*P*-thiocytidylyl-(3'→5')-2'-*O*,4'-*C*-[(1*S*)-ethane-1,1-diyl]-*P*-thioguanylyl-(3'→5')-2'-*O*,4'-*C*-[(1*S*)-ethane-1,1-diyl]-*P*-thioadenylyl-(3'→5')-2'-*O*-(2-methoxyethyl)-5-methyl-*P*-thiocytidylyl-(3'→5')-2'-*O*-(2-methoxyethyl)adenosine

STRUCTURAL FORMULA



(3'-5')-(*P*-thio)(m⁵Umoe-m⁵Cmoe-m⁵C(*Et*)-m⁵C(*Et*)-dG-dA-dA-dT-dG-dT-m⁵dC-m⁵dC-G(*Et*)-A(*Et*)-m⁵Cmoe-Amoe)

Legend:

d as prefix = 2'-deoxy
(*Et*) as suffix = 2'-*O*,4'-*C*-[(1*S*)-ethane-1,1-diyl]
m⁵ as prefix = 5-methyl
moe as suffix = 2'-*O*-(2-methoxyethyl)

MOLECULAR FORMULA	$C_{180}H_{240}N_{59}O_{90}P_{15}S_{15}$
MOLECULAR WEIGHT	5616
TRADEMARK	None as yet
SPONSOR	Ionis Pharmaceuticals
CODE DESIGNATIONS	IONIS 598769
<u>CAS</u> REGISTRY NUMBER	1698048-23-5
UNII	DR9CF3915M
WHO NUMBER	10433
SCS	