

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN

ETEPLIRSEN

PRONUNCIATION

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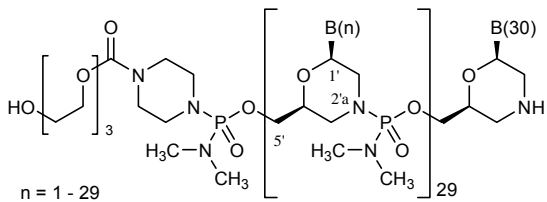
THERAPEUTIC CLAIM

Treatment of Duchenne muscular dystrophy

CHEMICAL NAMES

1. RNA, [*P*-deoxy-*P*-(dimethylamino)](2',3'-dideoxy-2',3'-imino-2',3'-seco)(2'a→5')(C-m5U-C-C-A-A-C-A-m5U-C-A-A-G-G-A-A-G-A-m5U-G-G-C-A-m5U-m5U-m5U-C-m5U-A-G), 5'-[*P*-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl]-*N,N*-dimethylphosphonamidate]
2. *P*,2',3'-trideoxy-*P*-(dimethylamino)-5'-*O*-{*P*-[4-(10-hydroxy-2,5,8-trioxadecanoyl)piperazin-1-yl]-*N,N*-dimethylphosphonamidoyl]-2',3'-imino-2',3'-secocytidylyl-(2'a→5')-*P*,3'-dideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secothymidylyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secocytidylyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secocytidylyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secoadenylyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secoadenylyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secocytidylyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secoadenylyl-(2'a→5')-*P*,3'-dideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secothymidylyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secocytidylyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secoadenylyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secoadenylyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secoguanilyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secoguanilyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secoadenylyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secoadenylyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secoguanilyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secoadenylyl-(2'a→5')-*P*,3'-dideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secothymidylyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secoguanilyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secocytidylyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secoadenylyl-(2'a→5')-*P*,3'-dideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secothymidylyl-(2'a→5')-*P*,3'-dideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secothymidylyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secocytidylyl-(2'a→5')-*P*,3'-dideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secothymidylyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secocytidylyl-(2'a→5')-*P*,3'-dideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secothymidylyl-(2'a→5')-*P*,2',3'-trideoxy-*P*-(dimethylamino)-2',3'-imino-2',3'-secoadenylyl-(2'a→5')-2',3'-dideoxy-2',3'-imino-2',3'-secoguanosine

STRUCTURAL FORMULA



B(1-30):
C-T-C-C-A-A-C-A-T-C-A-A-G-G-A-A-G-A-T-G-G-C-A-T-T-C-T-A-G

MOLECULAR FORMULA

$C_{364}H_{569}N_{177}O_{122}P_{30}$

MOLECULAR WEIGHT

10306

TRADEMARK

None as yet

SPONSOR

AVI BioPharma, Inc.

CODE DESIGNATION

AVI-4658

CAS REGISTRY NUMBER

1173755-55-9