

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (hi-94)

VESLETEPLIRSEN HYDROCHLORIDE

PRONUNCIATION

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THERAPEUTIC CLAIM

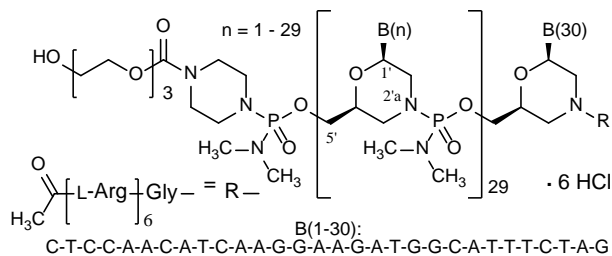
Treatment of Duchenne muscular dystrophy in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 51 skipping

CHEMICAL NAME

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-[2'a-(*N*2-acetyl-L-arginyl-L-arginyl-L-arginyl-L-arginyl-L-arginyl-L-arginylglycyl)]G), 5'-[*P*-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl]-*N,N*-dimethylphosphoramidate], hydrochloride (1:6) (Source: CAS)
2. all-*P*-ambo-2'-*N*-(*N*2-acetyl-L-arginyl-L-arginyl-L-arginyl-L-arginyl-L-arginyl-L-arginylglycyl)[2',3'-azanediy]-*P*-(dimethylamino)-*P*,2',3'-trideoxy-2',3'-seco](2'-*N*→5')(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-T-C-T-A-G) 5'-{*P*-[4-({2-[2-(2-hydroxyethoxy)ethoxy]ethoxy}carbonyl)piperazin-1-yl]-*N,N*-dimethylphosphoramidate} hydrochloride (1:6)
3. *P*,2',3'-trideoxy-*P*-(dimethylamino)-5'-O-{*P*-[4-(10-hydroxy-2,5,8-trioxadecanoyl)piperazin-1-yl]-*N,N*-dimethylphosphoramidoyl}-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'-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,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'a-(N²-acetyl-L-arginyl-L-arginyl-L-arginyl-L-arginyl-L-
 arginyl-L-arginylglycyl)-2',3'-dideoxy-2',3'-imino-2',3'-secoguanosine,
 hydrochloride (1:6)

STRUCTURAL FORMULA



MOLECULAR FORMULA	C ₄₀₄ H ₆₅₂ Cl ₆ N ₂₀₂ O ₁₃₀ P ₃₀
MOLECULAR WEIGHT	11561
TRADEMARK	None as yet
SPONSOR	Sarepta Therapeutics, Inc.
CODE DESIGNATIONS	SRP-5051
<u>CAS</u> REGISTRY NUMBER	2101569-97-3
UNII	6LN2JH1CPI

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