

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (FG-219)

GOLODIRSEN

PRONUNCIATION

goe" loe dir' sen

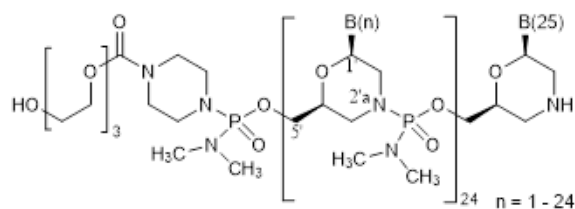
THERAPEUTIC CLAIM

Treatment of Duchenne muscular dystrophy

CHEMICAL NAMES

1. RNA, [*P*-deoxy-*P*-(dimethylamino)](2',3'-dideoxy-2',3'-seco)(2'a→5')(G-m⁵U-m⁵U-G-C-C-m⁵U-C-C-G-G-m⁵U-m⁵U-C-m⁵U-G-A-A-G-G-m⁵U-G-m⁵U-m⁵U-C), 5'-[*P*-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl]-*N,N*-dimethylphosphonamidate]
2. all-*P*-ambo-[2',3'-azanediy]-*P*-(dimethylamino)-*P*-2',3'-trideoxy-2',3'-seco] (2'-*N*→5')(G-T-T-G-C-C-T-C-C-G-G-T-T-C-T-G-A-A-G-G-T-G-T-T-C) 5'-{*P*-[4-({2-[2-(2-hydroxyethoxy)ethoxy]ethoxy}carbonyl)piperazin-1-yl]-*N,N*-dimethylphosphonamidate}

STRUCTURAL FORMULA



B(1-25): G-T-T-G-C-C-T-C-C-G-G-T-T-C-T-G-A-A-G-G-T-G-T-T-C

MOLECULAR FORMULA

C₃₀₅H₄₈₁N₁₃₈O₁₁₂P₂₅

MOLECULAR WEIGHT

8647 daltons

TRADEMARK

None as yet

SPONSOR

Sarepta Therapeutics, Inc.

CODE DESIGNATION

SRP-4053

CAS REGISTRY NUMBER

1422959-91-8

UNII

033072U4MZ

WHO NUMBER

10355

SCS