

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (GH-67)

FORDADISTROGENE MOVAPARVOVEC

PRONUNCIATION

for" da dis' troe jeen moe" va par' voe vek

THERAPEUTIC CLAIM

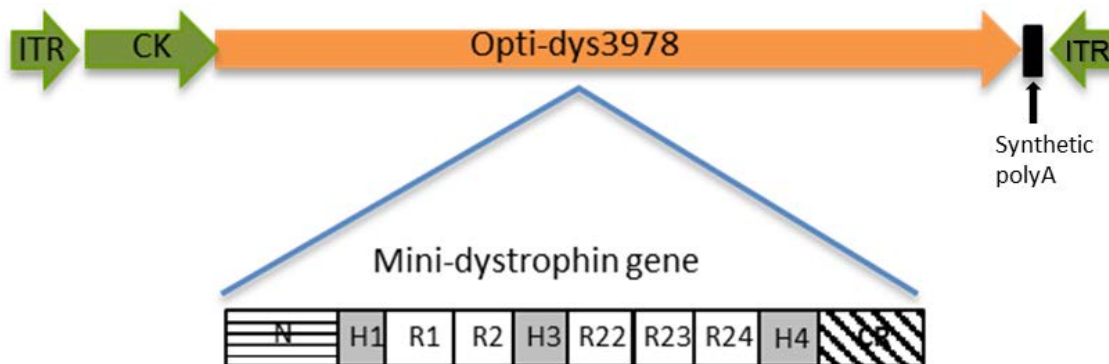
Treatment of Duchenne muscular dystrophy

DESCRIPTION

PF-06939926 is a recombinant AAV vector containing the AAV serotype 9 (AAV9) capsid packaging a miniaturized codon-optimized version of the *DMD* gene (called opti-dys Δ 3978) which encodes the domains minimally required for functionality of the dystrophin protein (mini-dystrophin). A synthetic polyadenylation (spolyA) sequence follows the opti-dys Δ 3978 gene. Cardiac and skeletal muscle-specific transgene expression is mediated by a synthetic enhancer and promoter. The expression construct is flanked by the AAV2 inverted terminal repeats (ITRs). The 3978 basepair mini-dystrophin open reading frame encodes the N-terminus; hinge regions H1, H3 and H4; spectrin-like repeats R1, R2 and R22-24; and the C-terminal cysteine-rich region of the dystrophin protein.

SCHEMATIC MAP

Schematic Structure of the Recombinant AAV Serotype 9 Vector Containing the Human Mini-Dystrophin Gene



ITR= AAV2 Inverted Terminal Repeats; CK= synthetic muscle-specific enhancer and promoter

TRADEMARK

None as yet

SPONSOR

Pfizer Inc.

CODE DESIGNATION

PF-06939926

CAS REGISTRY NUMBER

2248111-37-5

UNII

H4T32JIH01

WHO NUMBER

11362

SCS