

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (HI-105)

COBNABEXAGENE ANVUPARVOVEC

PRONUNCIATION

kob na bex' a jeen An vu par' voe vek

THERAPEUTIC USE

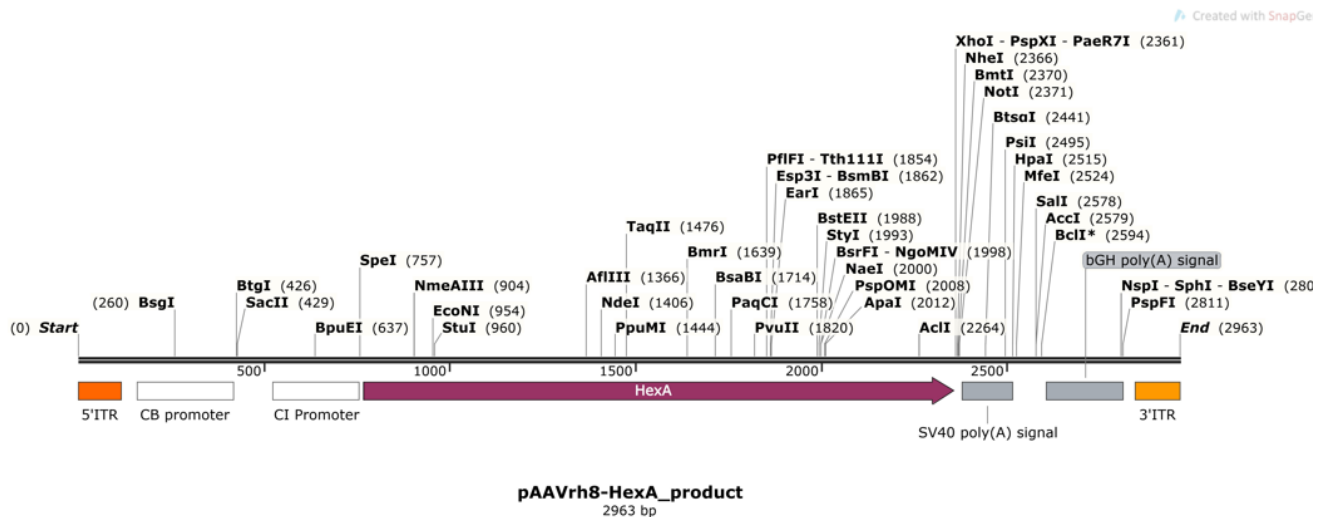
Treatment of Tay-Sachs and Sandhoff disease.

DESCRIPTION

DNA (recombinant adeno-associated virus serotype rh.8 vector AAVrh8-CB-ci-HEXA chicken β -actin promoter/chimeric chicken β -actin and rabbit β -globin intron/human β -acetylhexosaminidase α -subunit transgene wtHexA/SV40 polyadenylation signal/bovine growth hormone polyadenylation signal plus adeno-associated virus serotype 2 inverted terminal repeat flanks)

SCHEMATIC MAP

Figure 1: Structure of AAVrh8-CB-ci-HEXA and AAVrh8-CB-ci-HEXB



TRADEMARK

None as yet

SPONSOR

Axovant

CODE DESIGNATION

AXO-AAV-GM2 (rAAVrh8-HEXA/B)

CAS REGISTRY NUMBER

2364477-18-7

UNII

Y92LXG5D7W (HEXA) D35L53F823 (HEXB)

WHO NUMBER

11854

SCS