STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (JK-189)	GILAVEBEXAGENE ANVUPARVOVEC
PRONUNCIATION	gi la" ve bex’ a jeen an" u par’ voe vek
THERAPEUTIC USE	Treatment of Tay-Sachs and Sandhoff disease

DESCRIPTION

AXO-AAV-GM2 (rAAVrh8-HEXA/B) is a one-time, adeno-associated virus (AAV) in vivo gene therapy for the treatment of Tay-Sachs and Sandhoff disease. The therapy is designed to provide a functional copy of both HEXA and HEXB genes through a 1:1 formulation of two monocistronic vectors carrying either gene. Each vector utilizes a replication-deficient AAV serotype rh.8 (AAVrh.8) capsid containing a viral genome consisting of AAV2 inverted terminal repeats (ITRs), a chicken β-actin promoter, a chimeric chicken β-actin/rabbit β-globin intron, the HEXA or HEXB cDNA transgene, and a bovine growth hormone polyadenylation signal.

CAS NAME

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

SEQUENCE

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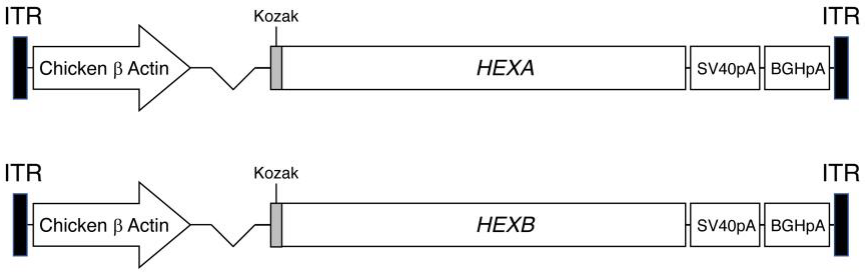
SCHEMATIC MAP

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



TRADEMARK	None yet
SPONSOR	Axovant Gene Therapies
CODE DESIGNATION	AXO-AAV-GM2

CAS REGISTRY NUMBER	2364477-18-7
UNII	CUC7FB5YWE
WHO NUMBER	12326

SCS