

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (HI-104)	BOMTABEGAGENE BAVOPARVOVEC
PRONUNCIATION	bom" ta beg' a jeen bav" oh par' voe vek
THERAPEUTIC USE	Treatment of GM1 gangliosidosis

DESCRIPTION

DNA (recombinant adeno-associated virus serotype 9 vector AAV9-GLB1 cytomegalovirus immediate early enhancer fusion with chicken β -actin promoter/donor and acceptor splice site-containing intron/human lysosomal acid β -galactosidase transgene hGLB1/SV40 polyadenylation signal plus adeno-associated virus serotype 2 inverted terminal repeat flanks) (CAS INDEX NAME)

The investigational product is a non-replicating, single stranded adeno-associated virus vector AAV9-GLB1, an AAV serotype expressing human GLB1 cDNAs encoding lysosomal acid beta-galactosidase (β -gal), formulated in a buffered aqueous suspension for IV delivery. The single stranded DNA genome of AAV9-GLB1 consists of the inverted terminal repeats from AAV2 flanking an expression cassette consisting of a cytomegalovirus immediate early enhancer fused to a chicken β -actin promoter, an intron containing splice donor and splice acceptor sites, the human β gal cDNA, and a poly A site from SV40.

The vector plasmid for AAV9-GLB1 vector contains the transgene hGLB1 which encodes the lysosomal acid β -D-galactosidase protein. Expression of the transgene cassette is under the control of CBA promoter. The biologic product will be administered to enable the in vivo expression of β -D-galactosidase

SCHEMATIC MAP



Schematic structure of AAV9-GLB1 viral vector genome.

ITR: AAV2 terminal repeat; Promoter: CBA; Transgene: hGLB1 transgene; Poly A: SV40 polyadenylation signal.

Figure 1: AAV9-GLB1 Vector Genome

TRADEMARK	None as yet
SPONSOR	Sio Gene Therapies
CODE DESIGNATION	AXO-AAV-GM1
<u>CAS</u> REGISTRY NUMBER	2364477-20-1
UNII	ZMG9G56THB

WHO NUMBER

11901

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