

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (JK-78)

BOTARETIGENE SPAROPARVOVEC

PRONUNCIATION

boe ta ret' i jeen spa roe par' voe vek

THERAPEUTIC USE

Treatment of retinitis pigmentosa (XLRP)

DESCRIPTION

DNA (recombinant pseudotyped adeno-associated virus 5 vector AAV-RPGR human rhodopsin kinase promoter fragment-regulated human retinitis pigmentosa GTPase regulator RPGR-ORF15 isoform gene variant plus adeno-associated virus 2 inverted terminal repeat flanks)

SEQUENCE

Numbered sequence

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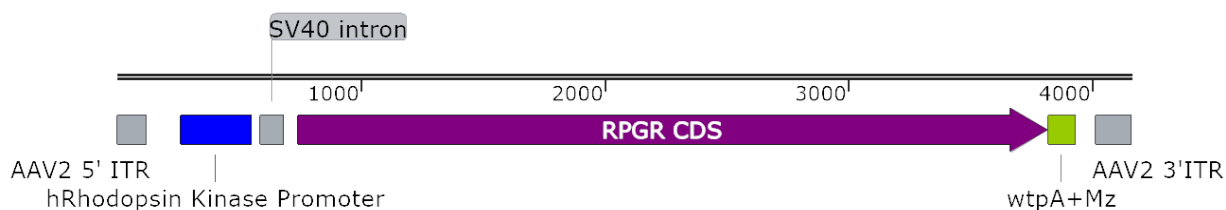
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Table of Features of AAV-RPGR

Feature	Location	Function
5'ITR	1-122bp	The AAV2 ITR sequences function as both the origin of viral DNA replication and the packaging signal of the viral genome. The ITRs also allow for the formation of T-shaped structurally stable forms flanking the transgene cassette that persist in the cells of interest.
hRKp Promoter	260-551 bp	The human rhodopsin kinase promoter (hRKp) drives cell-type specific expression of the therapeutic gene in rod and cone photoreceptors.
SV40 Intron	589-685bp	The intron upstream of the therapeutic gene enhances expression of the therapeutic gene.
Therapeutic gene	743-3823 bp	A complementary deoxyribonucleic acid (cDNA) encoding a engineered form of the human retinitis pigmentosa guanosine triphosphatase regulator open reading frame 15 (hRPGR.ORF15), containing an in-frame deletion of 378 bp in the highly repetitive purine-rich linker region.
wtPA+MZ	3824-3995	Polyadenylation sequence, aids in transcription termination.
3'ITR	4015-4159bp	The AAV2 ITR sequences function as both the origin of viral DNA replication and the packaging signal of the viral genome.

SCHEMATIC MAP



Feature	Position within viral genome	Description
5' AAV2 ITR	1-122	Inverted terminal repeat (ITR) derived from AAV2
hRhodopsin Kinase Promoter	260-551	Human rhodopsin kinase promoter
SV40 intron	589-685	Intronic region derived from simian virus 40
RPGR CDS	743-3823	Open reading frame encoding retinitis pigmentosa GTPase regulator
wtpA+Mz	3824-3935	Polyadenylation sequence
3' AAV2 ITR	4015-4159	Inverted terminal repeat (ITR) derived from AAV2

TRADEMARK None yet

SPONSOR Meira GTx

CODE DESIGNATION AAV-RPGR

CAS REGISTRY NUMBER 2417551-39-2

UNII IIO8Q82SD1

WHO NUMBER 12076

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