

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (GH-110)	COTORETIGENE TOLIPARVOVEC
PRONUNCIATION	koe" toe ret' i jeen tol" i par' voe vek
THERAPEUTIC CLAIM	Treatment of X-linked retinitis pigmentosa (XLRP) due to mutations in the RPGR gene

DESCRIPTION

AAV8-RPGR is a purified recombinant serotype 8 adeno-associated viral vector (AAV8) encoding a codon optimised (CO) copy of the cDNA encoding human Retinitis Pigmentosa GTPase Regulator (coRPGR). Each 20 nm AAV virion contains a single stranded DNA insert sequence of 4450 bp in length (plus short non-coding and cloning sequences) comprising:

- 119 bp 5' AAV2 inverted terminal repeat (ITR)
- 199 bp human rhodopsin kinase (RK) promoter
- 3459 bp human coRPGR cDNA
- 263 bp Bovine growth hormone polyadenylation sequence (bGH-polyA)
- 130 bp 3' AAV2 ITR

The ITRs are the only sequences retained from the wild-type serotype 2 AAV genome.

SCHEMATIC MAP

Structural Organization of AAV8-RPGR



TRADEMARK	None as yet
SPONSOR	Biogen
CODE DESIGNATION	AAV8-RPGR
<u>CAS</u> REGISTRY NUMBER	2246424-95-1
UNII	V1R4T1S1M0

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