

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (GH -111)

CEVARETIGENE RITOPARVOVEC

PRONUNCIATION

sev" a ret' i jeen rit" oh par' voe vek

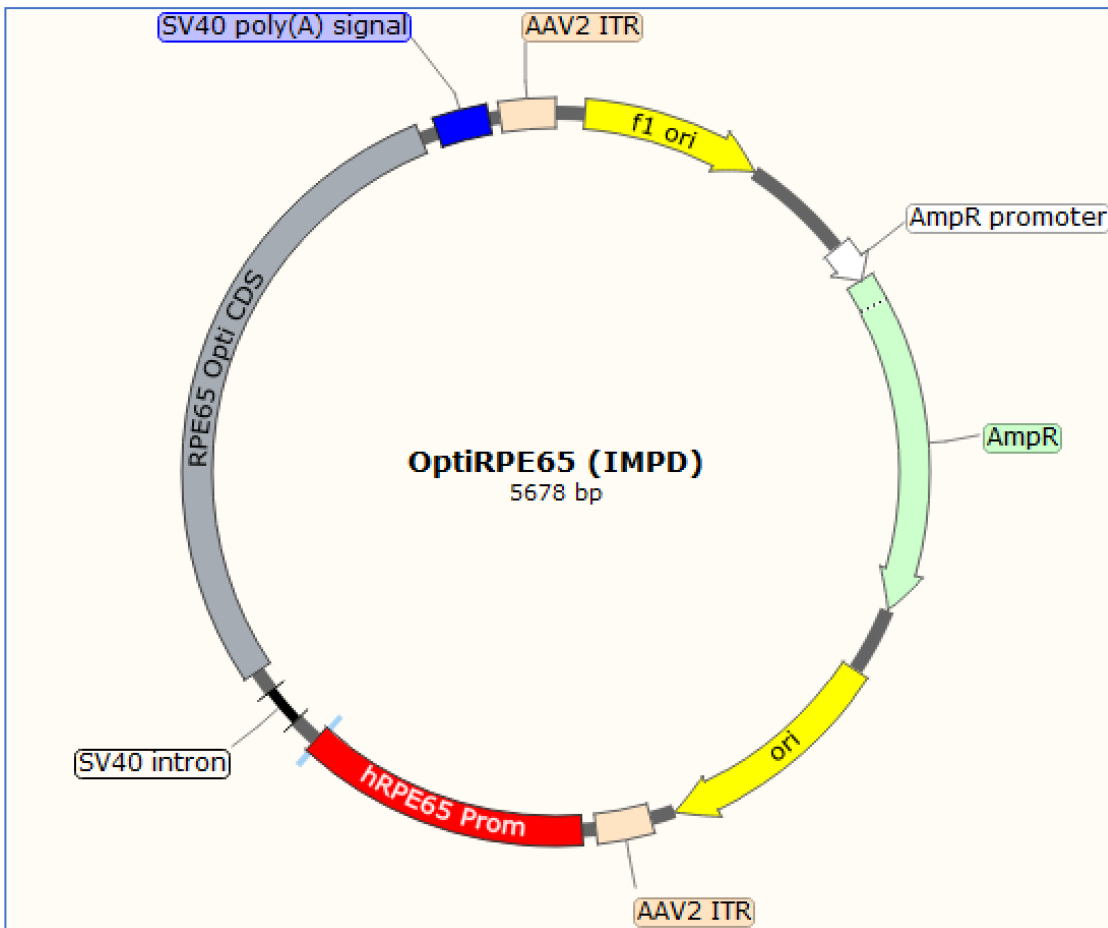
THERAPEUTIC CLAIM

Treatment of Leber congenital amaurosis (retinal dystrophy) associated with defects in RPE65 (LCA2)

DESCRIPTION

DNA (recombinant adeno-associated virus pseudotyped 2/5 vector MV2/5-OPTIRPE65, which harbors a human all-trans-retinyl ester isomerohydrolase codon-optimized gene RPE65 driven by an endogenous human RPE65 native promoter, an sv40 intron embedded between promoter and gene ORF and terminated by an sv40 polyA signal, all flanked by adeno-associated virus serotype 2 inverted terminal repeats (ITRs))

SCHEMATIC MAP



TRADEMARK	None as yet
SPONSOR	Meira GTx
CODE DESIGNATION	A001
<u>CAS</u> REGISTRY NUMBER	2247971-00-0
UNII	KVF2UY1OR4
WHO NUMBER	11368

SCS