STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (GH-43)	BEREMAGENE GEPERPAVEC
PRONUNCIATION	ber em' a jeen jep er' pa vek
THERAPEUTIC CLAIM	Treatment of epidermolysis bullosa dystrophica

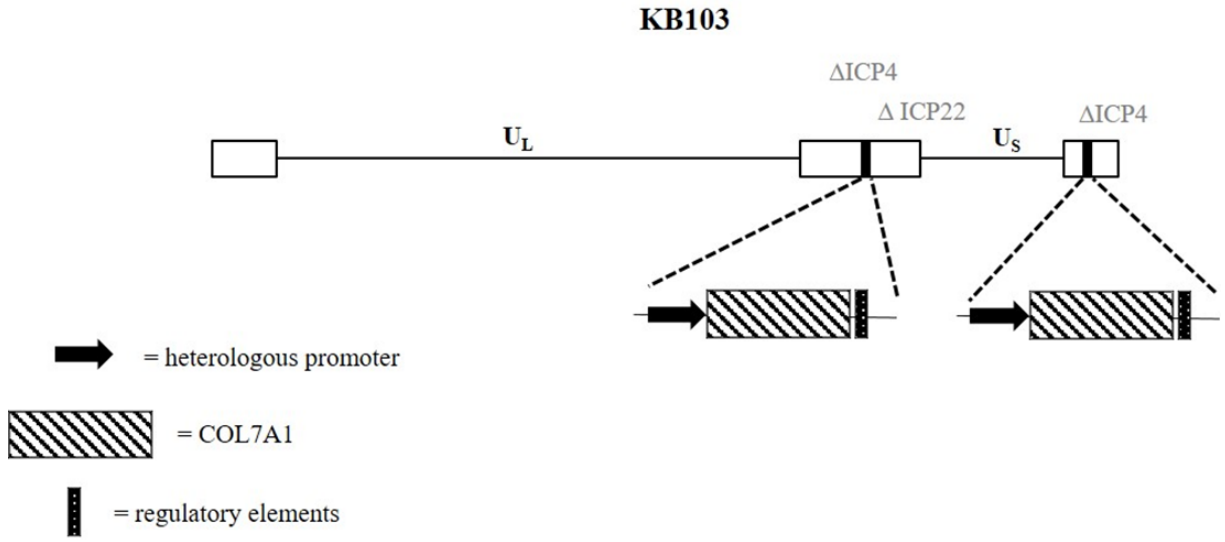
DESCRIPTION

DNA (recombinant herpes simplex virus type 1 strain KOS plasmid vector KB103 human collagen COL7A1-specifying) CAS NAME

KB103 is a replication-deficient, non-integrating type-1 herpes simplex virus (HSV-1) vector expressing human COL7A1. The HSV-1 strain KOS was employed to produce KB103, as the KOS strain is less virulent than other commonly used HSV-1 strains. The vector was generated by first deleting both copies of the viral Immediate Early (IE) gene ICP4, and then inserting a copy of COL7A1 into each ICP4 locus. These copies of COL7A1 are expressed from the human CMV (hCMV) promoter to allow for constitutive expression of COL7A1 upon cellular infection. In addition, KB103 is completely deleted for the IE gene ICP22 in order to further reduce any potential cytotoxic effects of the engineered virus. To enhance persistence in host cells (via inhibition of MHC class I antigen presentation), the function of ICP47 was retained. Because ICP4 is deleted, KB103 is non-replicating and shows no growth in non-complementing cells lacking exogenous ICP4, confirming the complete inactivation of both copies of the viral ICP4 gene. Complete abrogation of ICP22 expression and retention of ICP47 expression was confirmed by quantitative reverse transcription PCR (qRT-PCR) analysis.

SCHEMATIC MAP

Figure 1 - KB103 Vector Map



TRADEMARK	None as yet
SPONSOR	Krystal Biotech, Inc.
CODE DESIGNATION	KB103
CAS REGISTRY NUMBER	2241888-62-8
UNII	AQN7K24KQU
WHO NUMBER	11345

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