

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (HI-198)

MARNETEGRAGENE AUTOTEMCEL

PRONUNCIATION

mar" ne teg' ra jeen aw" toe tem' sel

THERAPEUTIC CLAIM

Treatment of severe leukocyte adhesion deficiency-1

PRODUCT DESCRIPTION

RP-L201 is a cell therapy product which consists of autologous genetically modified CD34+ hematopoietic cells. The product is manufactured from the patient's own CD34+ cells which are transduced with a lentiviral vector (LV) (Chim-CD18-WPRE LV) that encodes for the CD18  $\beta$ -subunit of human  $\beta$ 2 Integrins (ITGB2 gene product)

SCHEMATIC

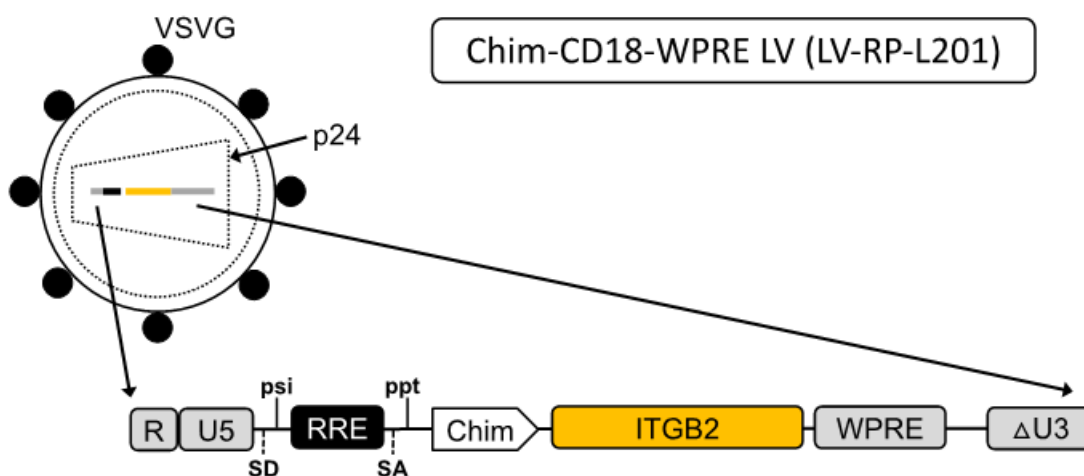


Table of features:

Name	Type	Minimum position in reference sequence (nucleotide)	Maximum position in reference sequence (nucleotide)	Length (base pairs)
5' LTR (R-U5)	LTR	0	180	180
PBS SL123	CDS	181	334	154
psi	packaging signal	226	351	126
RRE	Regulatory	845	1085	241
Cppt/CTS	Polypurine tract/Terminator	1,573	1,690	118
CatG	Eukaryotic promoter	1,714	2,078	365
cFes	Eukaryotic promoter	2,108	2,568	461
<b>ITGB2</b>	<b>CDS</b>	<b>2,677</b>	<b>4,986</b>	<b>2,310</b>
WPRE	Regulatory	5,021	5,616	596

