

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (HI-197)

MOZAFANCOGENE AUTOTEMCEL

PRONUNCIATION

moe" za fan koe' jeen aw" toe tem' sel

THERAPEUTIC CLAIM

Treatment Fanconi anemia (FA) Complementation Group A

PRODUCT DESCRIPTION

RP-L102 is a cell therapy product that consists of autologous genetically modified CD34+ hematopoietic cells. The product is manufactured from the patient's own CD34+ cells that are transduced with a lentiviral vector (LV) that encodes the human *FANCA* gene.

SCHEMATIC

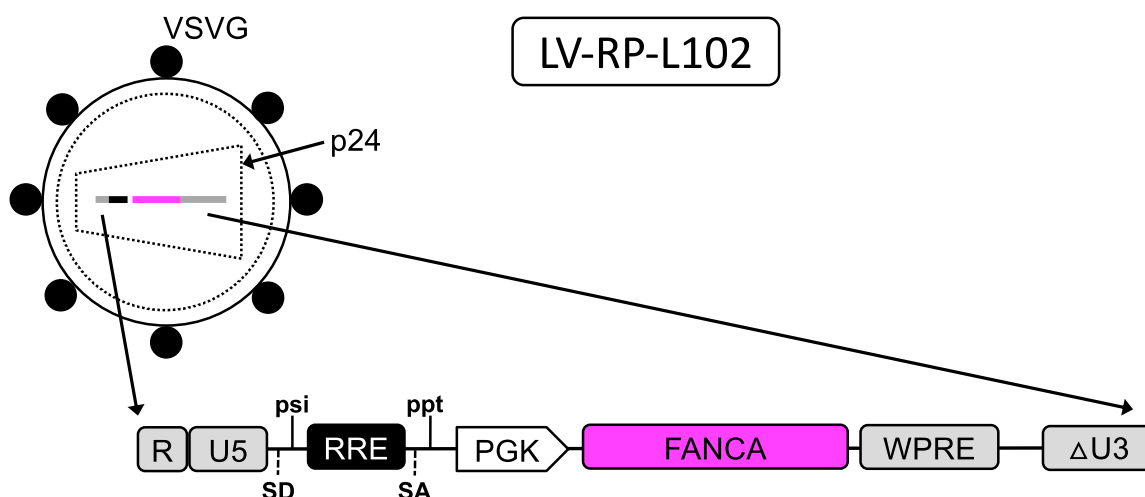


Table of features:

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
psi	packaging signal	231	368	138
RRE	protein binding	855	1059	205
cPPTCTS	enhancer	1,573	1,690	118
PGK	Eukaryotic promoter	1,731	2,246	516
FANCA	Transgene	2,273	6,643	4,371
WPRE	Regulatory	6,720	7,375	656
3' LTR (Delta-U3)	LTR	7,511	7,690	180

TRADENAME

None as of yet

SPONSOR

Rocket Pharmaceuticals, Inc.

CODE DESIGNATION

RP-L102

UNII

D82H29W72K

WHO NUMBER

11715

SCS