

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (FG-30)

SIMOLADAGENE AUTOTEMCEL

PRONUNCIATION

sim" oh lad' a jeen aw" toe tem' sel

THERAPEUTIC CLAIM

Autologous CD34+ enriched cell fraction that contains CD34+ Hematopoietic Stem and Progenitor Cells (HSPCs) genetically modified *ex vivo* using a lentiviral vector encoding for the human *ADA* cDNA sequence

DESCRIPTION

OTL-101 is a cell based drug product consisting of cells that have been genetically modified *ex vivo*. It includes autologous CD34+ hematopoietic stem cells (HSCs) from severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID) subjects transduced *ex vivo* with an EF1 α S lentiviral vector (LV) that encodes for the human adenosine deaminase (ADA) complementary deoxyribonucleic acid (cDNA) (EFS-ADA LV) [Figure 1]. The cDNA is codon optimized for human use, in a way that is designed to improve the level of transcription and translation of the ADA transgene.

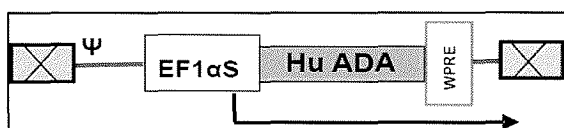


Figure 1: The EFS-ADA Lentiviral vector

EF1 α S = elongation factor 1 α shortened

huADA cDNA = human ADAcDNA

WPRE = woodchuck hepatitis post-transcriptional regulatory element

TRADEMARK

None as yet

SPONSOR

Orchard Therapeutics North America

CODE DESIGNATION

OTL-101

UNII

MOD3RG31RS

WHO NUMBER

11042

SCS