

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (MN-134) AZERCABTAGENE ZAPRELEUCEL

PRONUNCIATION ay" zer kab' ta jeen zep" re loo' sel

THERAPEUTIC CLAIM Antineoplastic

PRODUCT DESCRIPTION

PBCAR0191 is an allogeneic anti-human-CD19 CAR T cell product derived from healthy donor T cells that are genetically edited to (a) remove the expression of the endogenous T cell receptor (TCR) and (b) insert an anti-CD19 CAR gene, thereby providing the ability to specifically target, bind to, and kill CD19+ cells.

Adult allogeneic CD4+ and CD8+ enriched T cells transduced with an anti-CD19 chimeric antigen receptor (CAR) that is site-specific integrated into the cellular genome. Adult allogeneic CD4+ and CD8+ enriched T cells, gene edited through the electroporation of homing endonuclease mRNA, which is translated into endonuclease protein that introduces a double stranded DNA break in exon 1 of the endogenous T cell receptor alpha constant (TRAC) locus. Cells are subsequently transduced with a recombinant nonreplicating adeno-associated virus type 6 (rAAV6) vector, encoding an anti-CD19 CAR transgene consisting of a murine CD19-specific scFv (clone FMC63), a CD8 hinge/transmembrane domain, a novel 6 (N6) co-stimulatory domain and CD3 zeta signaling domain, under the control of the synthetic promoter JeT and the SV40 poly(A) signal. CAR transgene integration is achieved via homology directed recombination at the endonuclease target site (exon 1 of TRAC). Depletion of CD3+ cells is performed on the transduced cells to remove cells with functional endogenous T cell receptor (TCR). The substance is a mixture of non-effector (naive, stem cell memory, central memory) (CCR7+) and effector memory and effector (CCR7-) CD4+ and CD8+ T cells with generally $\geq 99\%$ T cell receptor knockout, and $\geq 40\%$ anti-CD19 CAR knock-in. Activation of the cells is accompanied in vitro by release of IFN γ , IL-2, IL-6, and TNF α in an antigen-dependent manner (Source: INN Recommended list #85)

TRADEMARK None

SPONSOR Imugene Limited

CODE DESIGNATION PBCAR0191

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WHO NUMBER 11438

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