STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (MN-210)	CEMACABTAGENE ANSEGEDLEUCEL
PRONUNCIATION	sem'' a kab‘ ta jeen an se'' jed loo’ sel
THERAPEUTIC CLAIM	Antineoplastic; cell-based gene therapy

CHEMICAL NAMES

Anti-cluster of differentiation (CD)19, T cell receptor alpha constant (TRAC)/CD52-knockout (KO) allogeneic chimeric antigen receptor (CAR) T cell therapy.

Allogeneic T lymphocytes obtained from peripheral blood mononuclear cells by leukapheresis of healthy volunteer donors, transduced with a self-inactivating, non-replicating lentiviral vector encoding a chimeric antigen receptor (CAR) targeting human CD19. The cells are also genetically modified using transcription activator-like (TAL) effector nucleases that are transiently delivered into the cell as mRNAs via electroporation and code for two pairs: one pair is designed for disruption of the T-cell receptor alpha constant (TRAC) and the other for disruption of the CD52 locus. The expressed transgene comprises a mouse kappa light chain leader sequence, an anti-CD19 single chain variable fragment (scFv) derived from clone 4G7, a CD8α hinge and transmembrane domain, and a 4-1BB (CD137) co-stimulatory and CD3ζ (TCRζ) intracellular signalling domain, under control of the human elongation factor 1 alpha (EF1α) short (EFS) promoter. The construct is flanked by 5' and 3' long terminal repeats (LTRs) and contains a ψ packaging signal, a truncated *gag*, a Rev response element (RRE), a central polypurine tract (cPPT) sequence and a mutated woodchuck post-transcriptional regulatory element (WPRE). The vector is pseudotyped with vesicular stomatitis virus(VSV) glycoprotein G. The leukapheresis material is activated by CD3 and CD28 agonists to stimulate T lymphocyte growth in media containing serum and interleukin 2 (IL-2), transduced with the lentiviral vector, genetically modified, and then culture expanded. At the end of expansion, TCR- cells are enriched by negative selection with residual TCR+ cells depleted using magnetic bead purification. The T lymphocytes (≥90%) are positive for the transgene (≥30% CAR positive) and are ≥50% CD52- and ≤3% TCRαβ+. The cells respond to CD19 expressing target cells by releasing IFN gamma, and demonstrate cytotoxicity against cells expressing CD19.

TRADEMARK	None as yet
SPONSOR	Allogene Therapeutics
CODE DESIGNATION	ALLO-501A
UNII	W3X22HS7XD
WHO NUMBER	12556

SCS