

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (BC-84)

TISAGENLECLEUCEL

PRONUNCIATION

tis'' a jen'' lek loo' sel

THERAPEUTIC CLAIM

Cellular immunotherapy for cancer treatment

CHEMICAL DESCRIPTION

CTL019 is a live, T cell therapy product for use in patients diagnosed with CD19-positive B cell malignancies. The product is produced from starting material of autologous leukocytes collected from the patient by leukapheresis. The product is both enriched for T cells and activated using CD3 and CD28 monoclonal antibodies covalently linked to magnetic beads (artificial antigen presenting cells). T cells are then modified by lentiviral mediated introduction of the transgene encoding a recombinant chimeric antigen receptor (CAR) with specificity for human CD19 antigen (a specific marker for B lymphocytes at nearly all stages of maturation after the hematopoietic stem cell). Modified T cells are expanded in culture *ex vivo*; beads are removed; the product concentrated and re-suspended in optimized storage/infusion medium. Upon infusion, the autologous redirected T cells (CTL019) target and kill CD19 bearing tumor cells. Engagement of the target cell stimulates proliferation that results in expansion of CTL019 1000 -10,000 fold while eradicating CD19 cells including normal B cells and precursors. Long term persistence of CTL019 results in chronic tumor surveillance.

STRUCTURAL FORMULA

Gene modification of T cells is mediated by stable insertion via transduction with a self-inactivating, replication- deficient Lentiviral (LV) vector containing the CTL019 transgene under the control of the EF-1 alpha promoter. The drug product is a mixture of transgene positive and negative T cells that are delivered to the patient on the basis of percent transgene positive T cells.

TRADEMARK

None as yet

SPONSOR

Novartis

CODE DESIGNATIONS

CTL019; CART19

UNII

Q6C9WHR03O

WHO NUMBER

10557

SCS

NOTE:

The adoption of ***tisagenlecleucel*** (N17/36) replaces the adoption of ***tisagenlecleucel-T*** (N14/30). The adoption statement (N14/30) is hereby rescinded.