

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (GH-22) ORVACABTAGENE AUTOLEUCEL

PRONUNCIATION or" va kab' ta jeen aw" toe loo' sel

THERAPEUTIC CLAIM Treatment of multiple myeloma

DESCRIPTION

JCARH125 consists of human culture expanded genetically modified autologous T cells for cell-based gene therapy. CD4+ CD8+ T Cells are derived from isolated blood of the patient and are transduced with non-replicative self-inactivating (SIN) lentiviral vector encoding an MSK54 anti-BCMA single chain variable fragment (scFv), IgG4-CH2-CH3 hinge region, CD28/CD137 (4-1BB), CD3zeta chimeric antigen receptor (CAR) under the transcriptional control of a hybrid promoter consisting of the human translation elongation factor 1 alpha (EF1alpha) promoter and the R region plus part of the U5 region from the human T-cell leukemia virus (HTLV-1R). Cells exhibit anti-tumoral activity in patients with multiple myeloma.

TRADEMARK None as yet

SPONSOR Juno Therapeutics Inc., A Celgene Company

CODE DESIGNATION JCARH125

WHO NUMBER 11274

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