

# STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (HI-221) ZEVORCABTAGENE AUTOLEUCEL

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

THERAPEUTIC CLAIM Treatment of relapsed and/or refractory multiple myeloma

## DESCRIPTION

CT053 CAR-BCMA T cells are living T cells. The cell viability is higher than 70%, the percentage of CAR-BCMA expressing T cells is higher than 10%, and the total T cell percentage is higher than 70%. The cell suspension mainly contains T cells (including CAR-positive T cells and CAR-negative T cells), NK cells, and low number of other non-T cells. The percentage of T cells is higher than 70%. The percentage of NK cells or other non-T cells is normally lower than 10%.

The CT053 CAR-BCMA T cells are identified using transduction efficiency assay. CT053 CAR-BCMA T cells are incubated with the biotin-labeled BCMA recombinant protein which can be specially recognized by the scFv on CAR-BCMA T cell. Followed by streptavidin PE staining, CAR-BCMA positive cells can be determined using flow cytometry.

The mechanism of action is understood as the anti-BCMA scFv binding to BCMA positive target cells which physically brings the CAR T cells to the BCMA positive tumor cells. The interaction of the CAR and BCMA results in the formation of immune synapses, similar to the natural T cell activation pathways. Formation of immune synapses triggers a cascade of T cell signaling that leads to T cell activation, expansion and differentiation. Upon activation, T cells produce cytokines for autocrine and paracrine signaling. The T cells may also produce perforin and granzymes to initiate direct cytolytic tumor cell killing.

The T cells will be transduced by HB0783 lentiviral vector. The full nucleotide sequence of HB0783 recombinant *car-bcma* lentiviral vector is listed as below.

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GGGTCTCTCT GGTTAGACCA GATCTGAGCC TGGGAGCTCT CTGGCTAACT 50
AGGGAACCCA CTGCTTAAGC CTCAATAAAG CTTGCCTTGA GTGCTTCAAG 100
TAGTGTGTGC CCGTCTGTTG TGTGACTCTG GTAAGTAGAG ATCCCTCAGA 150
CCCTTTTAGT CAGTGTGGAA AATCTCTAGC AGTGGCGCCC GAACAGGGAC 200
TTGAAAGCGA AAGGGAAACC AGAGGAGCTC TCTCGACGCA GGACTCGGCT 250
TGCTGAAGCG CGCACGGCAA GAGGCGAGGG GCGGCGACTG GTGAGTACGC 300
CAAAAATTTT GACTAGCGGA GGCTAGAAGG AGAGAGATGG GTGCGAGAGC 350
GTCAGTATTA AGCGGGGGAG AATTAGATCG CGATGGGAAA AAATTCGGTT 400
AAGGCCAGGG GGAAAGAAAA AATATAAAT AAAACATATA GTATGGGCAA 450
GCAGGGAGCT AGAACGATTC GCAGTTAATC CTGGCCTGTT AGAAACATCA 500
GAAGGCTGTA GACAAATACT GGGACAGCTA CAACCATCCC TTCAGACAGG 550
ATCAGAAGAA CTTAGATCAT TATATAATAC AGTAGCAACC CTCTATTGTG 600
TGCATCAAAG GATAGAGATA AAAGACACCA AGGAAGCTTT AGACAAGATA 650
GAGGAAGAGC AAAACAAAAG TAAGACCACC GCACAGCAAG CGGCCGCTGA 700
TCTTCAGACC TGGAGGAGGA GATATGAGGG ACAATTGGAG AAGTGAATTA 750
TATAAATATA AAGTAGTAAA AATTGAACCA TTAGGAGTAG CACCCACCAA 800
GGCAAAGAGA AGAGTGGTGC AGAGAGAAAA AAGAGCAGTG GGAATAGGAG 850
CTTTGTTCCCT TGGGTTCTTG GGAGCAGCAG GAAGCACTAT GGGCGCAGCG 900
TCAATGACGC TGACGGTACA GGCCAGACAA TTATTGTCTG GTATAGTGCA 950
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TTCAGACCCA	CCTCCCAACC	CCGAGGGGAC	CCGACAGGCC	CGAAGGAATA	1500
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TTAATGCCTT	TGTATCATGC	TATTGCTTCC	CGTATGGCTT	TCATTTTCTC	3600
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TCGGGGAAGC	TGACGTCCTT	TCCATGGCTG	CTCGCCTGTG	TTGCCACCTG	3900
GATTCTGCGC	GGGACGTCCT	TCTGCTACGT	CCCTTCGGCC	CTCAATCCAG	3950
CGGACCTTCC	TTCCCGCGGC	CTGCTGCCGG	CTCTGCGGCC	TCTTCCGCGT	4000
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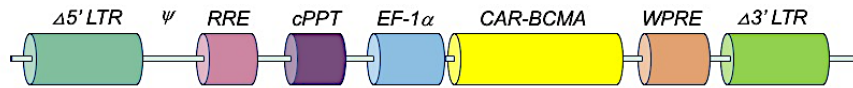
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TGTAGATCTT AGCCACTTTT TAAAAGAAAA GGGGGGACTG GAAGGGCTAA 4150
TTCAC TCCCA ACGAAGACAA GATCTGCTTT TTGCTTGTAC TGGGTCTCTC 4200
TGGTTAGACC AGATCTGAGC CTGGGAGCTC TCTGGCTAAC TAGGGAACCC 4250
ACTGCTTAAG CCTCAATAAA GCTTGCCTTG AGTGCTTCAA GTAGTGTGTG 4300
CCC GTCTGTT GTGTGACTCT GGTA ACTAGA GATCCCTCAG ACCCTTTTAG 4350
TCAGTGTGGA AAATCTCTAG CA 4372

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## STRUCTURAL FORMULA

The schematic map of entire nucleic acid of lentiviral vector is listed as below.



**Figure 3.2.S.1.2-1 Diagram of HB0783 recombinant *car-bcma* lentiviral vector**



**Figure 3.2.S.1.2-2 Diagram of *car-bcma***

TRADEMARK	None as of yet
SPONSOR	CARsgen Therapeutics Corporation
CODE DESIGNATIONS	CT053
UNII	GMV2GFP3G2
WHO NUMBER	11704

SCS