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anti-BCMA autologous CAR-T cells (CT053) receives US FDA orphan drug designation

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Under the Orphan Drug Act, the CT053 anti-BCMA product would be eligible for certain benefits including seven years of market exclusivity in the United States following marketing approval by the FDA.

CARsgen Therapeutics Inc., a clinical-stage biopharmaceutical company, on 31 August 2019, announced the United States Food and Drug Administration (FDA) has granted orphan drug designation to its investigational CAR-T cell therapy fully human anti-BCMA (B Cell Maturation Antigen) autologous chimeric antigen receptor (CAR) T Cells (ct053) for the treatment of multiple myeloma.

"FDA orphan designation is an important regulatory milestone in the continued development and commercialization of CT053 anti-BCMA CAR-T cells," said Dr Zonghai Li, Founder, CEO and CSO of CARsgen. "CT053 has demonstrated outstanding potency in an exploratory phase 1 clinical study in China. A total of 19 of 24 patients with relapsed and refractory multiple myeloma showed a complete response. And importantly, no event of grade 3 or higher cytokine release syndrome (CRS) was observed." The CT053 anti-BCMA CAR-T program has received Investigational New Drug (IND) clearance from the US FDA.

Orphan drug designation is granted by the FDA Office of Orphan Products Development to pharmaceutical products which are intended for the treatment of rare diseases that affect fewer than 200,000 people in the U.S. Under the Orphan Drug Act, the CT053 anti-BCMA product would be eligible for certain benefits including seven years of market exclusivity in the United States following marketing approval by the FDA.