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USFDA grants orphan drug designation to CARsgen Therapeutics

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CT041 is a humanized anti-claudin18.2 autologous CAR T-cell product

CARsgen Therapeutics Co., Ltd., a clinical-stage biopharmaceutical company based in China, has recently announced that the United States (US) Food and Drug Administration (FDA) has granted orphan drug designation to one of CARsgen's first-in-class drug candidates, CT041, for the treatment of gastric and gastroesophageal junction adenocarcinoma. CT041 is a humanized anti-claudin18.2 autologous chimeric antigen receptor (CAR) T-cell product and is targeted to treat patients with claudin18.2-positive tumors.

CT041 is the first claudin18.2-targeted CAR T-cell therapy that has received Investigational New Drug (IND) clearance by the US FDA and the first to receive IND clearance by the National Medical Products Administration (NMPA) in China. The initiation of an open-label, multicenter, Phase 1b clinical trial (NCT04404595) to evaluate the safety and efficacy of autologous CT041 cell therapy in patients with advanced gastric, gastroesophageal, or pancreatic adenocarcinoma is currently underway.

Orphan drug designation is granted by the FDA Office of Orphan Products Development to investigational treatments that are intended for the treatment of rare diseases affecting fewer than 200,000 people in the US. Under the Orphan Drug Act, the CT041 anti-claudin18.2 product would be eligible for certain benefits including FDA support for clinical studies, special fee exemptions and reductions, and seven years of market exclusivity in the United States following marketing approval by the FDA.