CARsgen Announces Investigational CAR-T Therapy CT053 Granted RMAT Designation by the U.S. FDA for R/R Multiple Myeloma

**Shanghai, October 28, 2019**  CARsgen Therapeutics Co. Ltd., a clinical-stage biopharmaceutical company today announced that the United States Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to its investigational CT053 CAR-T cell therapy. CT053 is a fully human anti-BCMA (B Cell Maturation Antigen) autologous chimeric antigen receptor (CAR) T Cell therapy for the treatment of relapsed and/or refractory multiple myeloma (rrMM).

RMAT designation was based on clinical data from an ongoing CT053 phase 1 study in heavily pre-treated multiple myeloma patients in China. Updated data from CT053 will be presented at the 61th annual meeting of the American Society of Hematology in Orlando on December 9.

“RMAT eligibility is an important regulatory milestone for CARsgen in the continued development and commercialization of CT053 anti-BCMA CAR T cell therapy,” said Zonghai Li, M.D., Ph.D., the chief executive officer of CARsgen. “The RMAT designation indicates that CT053 has demonstrated potential to address unmet medical needs for patients with rrMM. The designation is a
remarkable achievement towards expediting the product development and review of our planned biologics license application (BLA) and will be invaluable to bringing this cutting-edge advance to patients as quickly as possible. RMAT as well as the PRIority MEdicines (PRIME) eligibility received from the European Medicines Agency (EMA) empower us to collaborate closely with the U.S. FDA and EMA to rapidly advance the CT053 development program toward global regulatory approvals.” The CT053 anti-BCMA CAR-T program has also received Investigational New Drug (IND) clearance and Orphan Drug designation from the U.S. FDA and authorization of its Clinical Trial Application (CTA) from Health Canada.

Established under the 21st Century Cures Act, RMAT designation is a dedicated program designed to expedite the drug development and review processes for promising regenerative medicines and advanced therapies, including CAR T cell therapies. The designation includes all the benefits of the FDA's Fast Track and Breakthrough Therapy designations, providing the benefits of intensive FDA guidance on efficient drug development, including the ability for early interactions with FDA senior management to discuss surrogate or intermediate endpoints, potential ways to support accelerated approval and satisfy post-approval requirements, potential priority review of the BLA and other opportunities to expedite development and review. Between December 13, 2016 and September 30, 2019, the FDA received and assessed a total of 115 requests for eligibility. Of these, only 44 have been granted RMAT designation.
About CARsgen Therapeutics, Inc.

CARsgen Therapeutics is a clinical-stage immune-oncology company committed to the development and commercialization of CAR-T therapeutics for unmet medical need. The company has collaborated with top hospitals in China to launch several other First-in-Human studies such as anti-GPC3 CAR-T cell therapy for hepatocellular carcinoma and squamous lung cancer, anti-EGFR/EGFRvIII CAR-T cell therapy for glioblastoma multiforme and anti-Claudin18.2 CAR-T cell therapy for gastric and pancreatic cancer.

For more information, please visit: www.carsgen.com

SOURCE CARsgen Therapeutics Co. Ltd.

Related Links

http://www.carsgen.com

U.S. Expanded Access Policy