CARsgen Therapeutics Holdings Limited
科濟藥業控股有限公司
(Incorporated in the Cayman Islands with limited liability)
(Stock Code: 2171)

VOLUNTARY ANNOUNCEMENT
UPDATED RESEARCH RESULTS ON ZEVORCABTAGENE AUTOLEUCEL (CT053) PRESENTED AT THE 7TH ANNUAL CAR-TCR SUMMIT

This announcement is made by CARsgen Therapeutics Holdings Limited (the "**Company**", together with its subsidiaries and consolidated affiliated entities, the "**Group**" or "**CARsgen**") on a voluntary basis to inform the shareholders and potential investors of the Company about the latest business update of the Group.

The board of directors of the Company (the "**Board**") announces that at the 7th Annual CAR-TCR Summit, in Boston, Massachusetts, the Company gave an oral presentation on the North America Phase 2 clinical trial of zevorcabtagene autoleucel ("zevor-cel", R&D code: CT053), an autologous CAR T-cell product candidate against BCMA. This multi-center, open-label, Phase 1b/2 study (NCT03915184) is being conducted to evaluate the safety and efficacy in North American patients with relapsed and/or refractory multiple myeloma (R/R MM).

As of August 31, 2022, 17 patients with R/R MM received zevor-cel infusion in the Phase 2 portion of the clinical trial and have been followed for a median of 113 days (range: 9 to 373). Of these 17 patients, five patients (29.4%) had extramedullary disease (EMD; ≥1 plasmacytoma) and nine patients (52.9%) had high-risk cytogenetic features. Patients were heavily pretreated with a median of six prior lines of therapy (range: 4 to 17). All patients were refractory to their last line of therapy. Prior to zevor-cel infusion, patients received a lymphodepletion regimen of fludarabine (30mg/m^2 for three consecutive days) and cyclophosphamide (500mg/m^2 for two consecutive days).

Efficacy

In the 11 evaluable patients who had at least eight weeks follow-up, including four patients with EMD, the objective response rate was 100% (very good partial response, complete response, or stringent complete response) and responses deepened for subjects with longer follow-up. Since all responses are ongoing, the median progression-free survival, median overall survival and median duration of response have not been reached, and the complete response/stringent complete response rate is not yet mature. All patients with available MRD results at week 4 were MRD negative by next-generation of sequencing.

Safety

No death occurred and no patient experienced Grade 3 or higher cytokine release syndrome. Cytokine release syndrome was observed in 10 of 17 patients (59%), all reported as Grade 1 or 2. One transient Grade 3 immune effector cell-associated neurotoxicity syndrome event was reported and the patient fully recovered; no neurological toxicity with parkinsonian features was observed. For the management of post-infusion symptoms, five of 17 patients (29%) received tocilizumab and one patient (5.9%) received corticosteroids. Notably, three patients have received outpatient zevor-cel treatment in this study, and the two patients were admitted into the hospital for symptom management for 1 or 2 days.

Conclusion

Zevor-cel at a dose of 1.8×10^8 CAR T cells was well tolerated in the initial 17 Phase 2 patients with R/R MM, with promising efficacy and MRD negativity. Outpatient treatment is currently undergoing further exploration.

ABOUT ZEVOR-CEL

Zevor-cel (CT053) is a fully human, autologous BCMA CAR T-cell product candidate for the treatment of R/R MM. CARsgen is conducting a Phase 1b/2 clinical trial (LUMMICAR STUDY 2) in North America to evaluate the safety and efficacy of zevor-cel for R/R MM. The Company also plans to conduct additional clinical trials to develop zevor-cel as an earlier line of treatment for multiple myeloma.

Zevor-cel received Regenerative Medicine Advanced Therapy (RMAT) and Orphan Drug designations from the U.S. Food and Drug Administration (FDA) in 2019, as well as the PRIority MEdicines (PRIME) and Orphan Medicinal Product designations from the European Medicines Agency (EMA) in 2019 and 2020, respectively. Zevor-cel also received Breakthrough Therapy designation from the NMPA in 2020.

The Company believes that zevor-cel is well positioned to potentially reshape the treatment paradigm for multiple myeloma and become a foundational treatment for multiple myeloma patients.

ABOUT THE COMPANY

CARsgen is a biopharmaceutical company with operations in China and the U.S. mainly focused on innovative CAR T-cell therapies for the treatment of hematologic malignancies and solid tumors. The Company has built an integrated cell therapy platform with in-house capabilities that span target discovery, antibody development, clinical trials, and commercial-scale manufacturing. CARsgen has internally developed novel technologies and a product pipeline with global rights to address major challenges of CAR T-cell therapies, such as improving the safety profile, enhancing the efficacy in treating solid tumors, and reducing treatment costs. Our vision is to become a global biopharmaceutical leader that brings innovative and differentiated cell therapies to cancer patients worldwide and makes cancer curable.

DEFINITIONS AND GLOSSARY OF TECHNICAL TERMS

"BCMA"	B-cell maturation antigen, a protein that is highly expressed in a number of hematologic malignancies
"CAR"	chimeric antigen receptor
"CAR T"	chimeric antigen receptor T cell
"EMA"	European Medicines Agency
"EMD"	extramedullary disease
"FDA" or "U.S. FDA"	U.S. Food and Drug Administration
"IIT" or "investigator-initiated trial"	clinical trial sponsored and conducted by independent investigators
"MM" or "R/R MM"	multiple myeloma, a type of cancer that forms in the white blood cells; cancer that relapses or does not respond to treatment is called relapsed and/or refractory multiple myeloma
"MRD"	minimal residual disease
"NMPA"	National Medical Products Administration (國家藥品監督管理局), the successor of the China Food and Drug Administration (國家食品藥品監督管理總局), or the CFDA, the State Food and Drug Administration (國家食品藥品監督管理局), or the SFDA and the State Drug Administration (國家藥品監督管理局), or the SDA

"Phase Ib"	a phase of clinical trials that primarily assesses safety, tolerability, and pharmacokinetics/pharmacodynamics at multiple ascending dose levels prior to commencement of a Phase II or Phase III clinical trials
"Phase II clinical trial"	a study in which a drug is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the drug for specific targeted disease, and to determine dosage tolerance and optimal dosage
"pivotal trial"	the final trial or study to demonstrate clinical efficacy and safety evidence required before submission for drug marketing approval
"regenerative medicine advanced therapy" or "RMAT"	a special status granted by the FDA to regenerative medicine therapies, including cell therapies, intended to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs for such disease or condition
"solid tumors"	an abnormal mass of tissue that usually does not contain cysts or liquid areas
"U.S."	the United States of America, its territories, its dependencies and all areas subject to its jurisdiction

Cautionary Statement required by Rule 18A.05 of the Rules Governing the Listing of Securities on The Stock Exchange of Hong Kong Limited: The Company cannot guarantee that it will be able to develop, or ultimately market, zevor-cel successfully. Shareholders and potential investors of the Company are advised to exercise caution when dealing in the shares of the Company.

By order of the Board
CARsgen Therapeutics Holdings Limited
Dr. Zonghai LI
Chairman

Hong Kong, September 21, 2022

As at the date of this announcement, the board of directors of the Company comprises Dr. Zonghai LI, Dr. Huamao WANG and Dr. Hua JIANG as executive Directors; Mr. Bingsen GUO, Mr. Huaqing GUO and Mr. Ronggang XIE as non-executive Directors; Dr. Chunhai FAN, Dr. Guangmei YAN and Mr. Tak Young SO as the independent non-executive Directors.

In the case of inconsistency, the English text of this announcement shall prevail over the Chinese text.