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 THE NATIONAL MEDICAL PRODUCTS ADMINISTRATION ACCEPTED THE NEW DRUG APPLICATION FOR ZEVORCABTAGENE AUTOLEUCEL FOR THE TREATMENT OF RELAPSED AND/OR REFRACTORY MULTIPLE MYELOMA

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**") is pleased to announce that the National Medical Products Administration (NMPA) of China has accepted the New Drug Application (NDA) for zevorcabtagene autoleucel ("zevor-cel", R&D code: CT053), a fully human, autologous BCMA CAR T-cell therapy for the treatment of relapsed and/or refractory multiple myeloma (R/R MM).

The acceptance of the NDA is based on data from an open-label, single arm Phase I/II clinical trial (LUMMICAR STUDY 1 [Protocol number: CT053-MM-01]) in China. Study results showed that zevor-cel has excellent safety and efficacy profiles. Zevor-cel also represents a promising treatment option for patients with high-risk disease.

Multiple myeloma is a fatal blood cancer in which plasma cells found in bone marrow grow out of control and create abnormal proteins that can damage vital organs, including heart and kidneys.[1] According to the World Health Organization, there were more than 21,000 new cases and nearly 16,200 deaths caused by multiple myeloma estimated in China in 2020.[2] With patient survival averaging longer than five years, there were an estimated 113,000 prevalent patients with multiple myeloma including those with newly diagnosed and refractory/relapsed disease in China during the same period. Frost & Sullivan have forecasted that throughout the 2020s, this prevalence will continue to increase 8-10% each year.[3] Although patients may achieve remission with traditional therapies, most of them experience repeated disease progression.[4] Patients who relapse after traditional therapies, including protease inhibitors, immunomodulatory agents and/or anti-CD38 monoclonal antibodies, have poorer prognoses and few treatment options.[5-6] Therefore, these patients have a substantial clinical unmet need for an efficacious, safe and convenient treatment.

ABOUT ZEVOR-CEL

Zevor-cel (CT053) is a fully human, autologous BCMA CAR T-cell product candidate for the treatment of R/R MM. The NDA for zevor-cel based on the data from Phase I/II clinical trial (LUMMICAR STUDY 1) in China has been accepted by NMPA. 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. 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, lead 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
"FDA" or "U.S. FDA"	U.S. Food and Drug Administration
"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

"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
"RMAT"	regenerative medicine advanced therapy, 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

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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, October 18, 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.