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 ZEVOR-CEL AT 2023 ASH ANNUAL MEETING

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 2023 American Society of Hematology ("**ASH**") Annual Meeting, the Company presented one poster with study results for zevorcabtagene autoleucel ("**zevor-cel**", R&D code: CT053, an autologous CAR T-cell therapy candidate against BCMA), which include the 3-year follow-up on efficacy and safety results from the Phase I portion of Phase I/II registrational study in China (LUMMICAR-1, NCT03975907). Details are listed below:

Poster #4845: Three-Year Follow-up on Efficacy and Safety Results from Phase I Lummicar Study 1 of Zevorcabtagene Autoleucel in Chinese Patients with Relapsed or Refractory Multiple Myeloma

Zevor-cel is a fully human autologous chimeric antigen receptor (CAR) T-cell therapy product with a B-cell maturation antigen (BCMA) which is being developed for the treatment of patients with relapsed or refractory multiple myeloma (R/R MM).

The LUMMICAR STUDY 1 trial is a multi-center, open-label Phase I/II clinical trial ongoing in China. The New Drug Application (NDA) in China for zevor-cel is based on the Phase I/II data from LUMMICAR STUDY 1 and is currently under review.

Herein, the Company presented the updated results with 3 years of follow-up after the last patient was infused with zevor-cel in the Phase I portion of the study. Responses were assessed by investigator per the International Myeloma Working Group (IMWG) 2016 criteria.

As of July 17, 2023, 14 participants with R/R MM, who had received at least 3 prior regimens including a proteasome inhibitor and an immunomodulatory drug (IMiD) with a median of 6 prior regimens (range: 3-11), received zevor-cel infusion. A single infusion of zevor-cel was administered 1-2 days after the completion of lymphodepletion. Three participants received 1.0×10^8 CAR+ T cells, and 11 participants received 1.5×10^8 CAR+ T cells. The median age of the cohort was 54 years (range: 34-62 years); 50.0% (7/14) of the participants had high-risk cytogenetic abnormalities, 14.3% (2/14) had extramedullary disease (EMD), and 14.3% (2/14) of the participants had Stage III disease based on International Staging System (ISS).

Safety

Overall, the safety profile of zevor-cel was manageable. There were no ≥ Grade 3 cytokine release syndrome (CRS) events. There were no immune effector cell-associated neurotoxicity syndrome (ICANS) events of any grade. Three treatment-related Grade 3 infections were observed. Three patients experienced serious adverse events (SAE) including 2 patients who had treatment-related SAEs which were pulmonary infection and tumor lysis syndrome. There were overall 2 deaths on the study; neither was related to zevor-cel.

Efficacy

As of July 17, 2023, the median follow-up duration was 37.7 months (range:14.8-44.2 months). The overall response rate (ORR) was 100% (14/14) with 78.6% (11/14) patients achieving a complete response (CR) or a stringent complete response (sCR); minimal residual disease (MRD) negativity was attained in all patients achieving either a CR or sCR. The median duration of response (mDOR) was 24.1 months in all patients and 26.0 months in those achieving CR or sCR. The median progression-free survival (mPFS) was 25.0 months. A total of 7 (50%) patients were in remission lasting longer than 24 months. The median overall survival (OS) was not reached, and 92.9% (13/14) of patients were still alive at month 36.

Conclusion

At 3-year follow up of Phase I portion of LUMMICAR-1 study in heavily pre-treated R/R MM population, zevor-cel demonstrated an encouraging safety profile with deep and durable responses consistent with the initial results.

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 New Drug Application (NDA) for zevor-cel is based on the Phase I/II data from LUMMICAR STUDY 1 in China and is currently under review. CARsgen is conducting a Phase 1b/2 LUMMICAR STUDY 2 clinical trial in North America to evaluate the safety and efficacy of zevor-cel for R/R MM.

Zevor-cel received Regenerative Medicine Advanced Therapy (RMAT) and Orphan Drug designations from the U.S. FDA in 2019, as well as 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.

ABOUT THE COMPANY

CARsgen is a biopharmaceutical company with operations in China and the U.S. and is focused on innovative CAR T-cell therapies for the treatment of hematologic malignancies and solid tumors. CARsgen has established a comprehensive CAR T-cell research and development platform, encompassing target discovery, innovative CAR T-cell development, clinical trials, and commercial-scale production. 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. CARsgen's 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 plasma 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 trial
"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 a specific targeted disease, and to determine dosage tolerance and optimal dosage
"confirmatory trial" or "pivotal trial"	the controlled trial or study intended to demonstrate the required clinical efficacy and safety evidence before submission for drug marketing approval

"PRIME"	PRIority MEdicine. A scheme launched by the EMA to offer early and proactive support to medicine developers to optimize the generation of robust data on a medicine's benefits and risks, and to accelerate the assessment of the applications of medicines that target an unmet medical need with advantages over existing treatments
"regenerative medicine advanced therapy" or "RMAT"	a special status granted by the FDA to regenerative medicine therapies, including cell therapies, that are intended to treat a serious or life-threatening disease or condition, and for which preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs for such a disease or condition
"United States" or "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.

Cautionary-Language Regarding Forward-Looking Statements

All statements in this announcement that are not historical fact or that do not relate to present facts or current conditions are forward-looking statements. Such forward-looking statements express the Group's current views, projections, beliefs and expectations with respect to future events as of the date of this announcement. Such forward-looking statements are based on a number of assumptions and factors beyond the Group's control. As a result, they are subject to significant risks and uncertainties, and actual events or results may differ materially from these forward-looking statements and the forward-looking events discussed in this announcement might not occur. Such risks and uncertainties include, but are not limited to, those detailed under the heading "Principal Risks and Uncertainties" in our most recent annual report and interim report and other announcements and reports made available on our corporate website, **https://www.carsgen.com**. No representation or warranty is given as to the achievement or reasonableness of, and no reliance should be placed on, any projections, targets, estimates or forecasts contained in this announcement.

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

Hong Kong, December 12, 2023

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. Guangmei YAN, Dr. Huabing LI and Ms. Xiangke ZHAO as the independent non-executive Directors.

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