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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)

INSIDE INFORMATION ANNOUNCEMENT THE NMPA APPROVES THE BCMA CAR-T THERAPY ZEVORCABTAGENE AUTOLEUCEL FOR THE TREATMENT OF ADULT PATIENTS WITH RELAPSED OR REFRACTORY MULTIPLE MYELOMA WHO HAVE PROGRESSED AFTER AT LEAST 3 PRIOR LINES OF THERAPY (INCLUDING A PROTEASOME INHIBITOR AND IMMUNOMODULATORY AGENT)

This announcement is made by CARsgen Therapeutics Holdings Limited (the "**Company**", together with its subsidiaries and consolidated affiliated entities, the "**Group**" or "**CARsgen**") pursuant to Rule 13.09(2)(a) of the Rules Governing the Listing of Securities on The Stock Exchange of Hong Kong Limited (the "**Listing Rules**") and the inside information provision (as defined in the Listing Rules) under Part XIVA of the Securities and Futures Ordinance (Cap. 571 of the Laws of Hong Kong).

The board of directors of the Company (the "**Board**") is pleased to announce that the National Medical Products Administration ("**NMPA**") of China has approved the New Drug Application ("**NDA**") for zevorcabtagene autoleucel (R&D code: CT053, an autologous CAR-T product candidate against BCMA), for the treatment of adult patients with relapsed or refractory multiple myeloma who have progressed after at least 3 prior lines of therapy (including a proteasome inhibitor and immunomodulatory agent).

Zevorcabtagene autoleucel is an autologous BCMA-targeted CAR T-cell product generated by transducing T cells with a lentivirus encoding a CAR comprising a fully human BCMA-specific single chain variable fragment ("**scFv**"), the human CD8α hinge domain, CD8α transmembrane domain, 4-1 BB co-stimulatory domain and CD3ζ activation domain. The proprietary novel fully-human scFv has high binding affinity and stability.

The approval of zevorcabtagene autoleucel is based on an open-label, single arm, multi-center Phase II clinical trial (LUMMICAR STUDY 1, NCT03975907) conducted in China. The trial results were released at the 2022 Annual Meeting of the American Society of Hematology ("**ASH**"), and zevorcabtagene autoleucel demonstrated encouraging efficacy and a favorable safety profile.

Multiple myeloma is an incurable malignant plasma cell disorder that accounts for approximately 10% of all hematological cancers.[1] With China's ageing population coupled with an increase in life expectancy, the number of patients with multiple myeloma is expected to expand. Frost & Sullivan forecasts that the prevalence of multiple myeloma in China in 2023 is approximately 153,000 per annum, and the number of new cases would be 23,200 per annum. It is estimated that the prevalence of multiple myeloma in China is expected to grow to 266,300 by 2030.[2]

ABOUT ZEVORCABTAGENE AUTOLEUCEL

Zevorcabtagene autoleucel is a fully human, autologous BCMA CAR T-cell product for the treatment of R/R MM. As informed by the NMPA on March 1, 2024, zevorcabtagene autoleucel was approved on February 23, 2024 for the treatment of adult patients with relapsed or refractory multiple myeloma who have progressed after at least 3 prior lines of therapy (including a proteasome inhibitor and immunomodulatory agent). CARsgen is conducting a separate Phase 1b/2 LUMMICAR STUDY 2 clinical trial in North America to evaluate the safety and efficacy of zevorcabtagene autoleucel in R/R MM.

Zevorcabtagene autoleucel 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. Zevorcabtagene autoleucel 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
"CD3"	a protein complex and T cell co-receptor that is involved in activating both the cytotoxic T cell and T helper cells
"CD8"	a transmembrane glycoprotein that serves as a co-receptor for the T-cell receptor (TCR) and plays a role in T cell signaling and aiding with cytotoxic T cell-antigen interactions

"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
"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
"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
"4-1 BB"	a costimulatory molecule functioning to stimulate T cell proliferation, dendritic cell maturation, and promotion of B cell antibody secretion

Reference list

1. Kyle RA, Rajkumar SV. Multiple myeloma. N Engl J Med. 2004;351:1860-1873.

2. Frost and Sullivan. Cellular Immunotherapy Market. Independent Market Research version March, 2021. Data on file.

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 zevorcabtagene autoleucel 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, March 1, 2024

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.