CStone Pharmaceuticals

基石藥業

(Incorporated in the Cayman Islands with limited liability)

(**Stock Code: 2616**)

VOLUNTARY ANNOUNCEMENT
CSTONE ANNOUNCED THE IND APPROVAL OF CS5001, A POTENTIAL GLOBAL BEST-IN-CLASS ROR1-TARGETING ADC BY THE U.S. FOOD AND DRUG ADMINISTRATION

CStone Pharmaceuticals (the "**Company**" or "**CStone**") is pleased to announce that the investigational new drug ("**IND**") application of CS5001, a potential global best-in-class antibody-drug conjugate ("**ADC**") targeting receptor tyrosine kinase-like orphan receptor 1 ("**ROR1**") has received a STUDY MAY PROCEED ("**SMP**") letter from the U.S. Food and Drug Administration ("**FDA**"). CS5001 will commence in the clinic as one of the three most advanced ROR1 ADCs globally, marking another important milestone for CStone's Pipeline 2.0 strategy.

ROR1 is an oncofetal protein with low or no expression in adult tissues but high expression in a variety of cancers including various forms of leukemia and non-Hodgkin lymphoma, breast, lung, and ovarian cancers, making it an ideal ADC target. CS5001 is an ADC targeting ROR1 with multiple differentiated features including proprietary site-specific conjugation, tumor-selective cleavable linker and pro-drug technology. Results from pre-clinical studies showed that CS5001 exhibited potent and selective cytotoxicity to a variety of ROR1-expressing cancer cell lines and demonstrated remarkable in vivo antitumor activity in both hematological and solid tumor xenograft models.

Dr. Archie Tse, Chief Scientific Officer of CStone, said, "We are glad that the IND application of CS5001 received the SMP letter from the FDA in 2021. The preclinical pharmacology data were encouraging and demonstrated CS5001's therapeutic potential in multiple hematological and solid malignancies. There are only three ROR1 ADCs including CS5001 in clinical development. The upcoming first-in-human Phase I study aims to evaluate the safety, tolerability, pharmacokinetics and preliminary anti-tumor activity of CS5001 in advanced B cell lymphomas and solid tumors. We will make every effort to advance this clinical trial of CS5001, meanwhile we have already submitted the CTN (clinical trial notification) application in Australia and plan to submit the IND application in China soon."

About CS5001（ROR1 ADC）

In October 2020, CStone signed a licensing agreement with LegoChem Biosciences, Inc. ("**LCB**") for the development and commercialization of CS5001 which was originally generated by collaboration of LCB and ABL Bio, both South Korea-based leading biotech companies. Under the agreement, CStone obtains the exclusive global right to lead development and commercialization of CS5001 outside the Republic of Korea.

CS5001 is now a clinical-stage ADC targeting ROR1. CS5001 has uniquely designed and uses LCB's proprietary tumor-cleavable linker and pyrrolobenzodiazepine ("**PBD**") prodrug playload. Only after reaching the tumor, the linker and PBD prodrug are cleaved to release the PBD toxin, resulting in lethal DNA cross-links in cancer cells. The use of the linker plus PBD prodrug effectively helps addressing the toxicity problem associated with traditional PBD payloads, leading to a better safety profile. Additionally, CS5001 utilizes site-specific conjugation for a precise drug antibody ratio ("**DAR**") of 2 which enables homogeneous production and large-scale manufacturing.

About CStone

CStone is a biopharmaceutical company focused on researching, developing and commercializing innovative immuno-oncology and precision medicines to address the unmet medical needs of cancer patients in China and worldwide. Established in 2015, CStone has assembled a world-class management team with extensive experience in innovative drug development, clinical research, and commercialization. The Company has built an oncology-focused pipeline of 15 drug candidates with a strategic emphasis on immuno-oncology combination therapies. Currently, CStone has received five drug approvals in Greater China, including three in Mainland China, one in Hong Kong, and one in Taiwan. CStone's vision is to become globally recognized as a world-renowned biopharmaceutical company by bringing innovative oncology therapies to cancer patients worldwide.

For more information about CStone, please visit: www.cstonepharma.com.

By Order of the Board
CStone Pharmaceuticals
Dr. Frank Ningjun Jiang
Chairman

Suzhou, the People's Republic of China, January 3, 2022

As at the date of this announcement, the Board of Directors of the Company comprises Dr. Frank Ningjun Jiang as Chairman and executive Director, Dr. Wei Li, Mr. Kenneth Walton Hitchner III, Mr. Yanling Cao, Mr. Xianghong Lin and Mr. Edward Hu as non-executive Directors, and Dr. Paul Herbert Chew, Mr. Ting Yuk Anthony Wu and Mr. Hongbin Sun as independent non-executive Directors.