CStone Pharmaceuticals

基石藥業

(Incorporated in the Cayman Islands with limited liability)

(**Stock Code: 2616**)

VOLUNTARY ANNOUNCEMENT
CSTONE ANNOUNCED FIRST PATIENT ENROLLMENT IN THE US IN THE PHASE 1 CLINICAL TRIAL OF CS5001, A POTENTIAL GLOBAL BEST-IN-CLASS ROR1-TARGETING ADC

CStone Pharmaceuticals (the "**Company**" or "**CStone**") is pleased to announce that the first patient has been enrolled in the US in the Phase 1 clinical trial for CS5001. This is a remarkable milestone for CStone's Pipeline 2.0 strategy.

Key Highlights

- Commencement of the first-in-human clinical trial of CS5001 marks another key milestone for CStone's Pipeline 2.0.

- Global development of CS5001 is conducted in the form of a multi-regional clinical trial, with sites initiated in the US and Australia, and an investigational new drug ("**IND**") application accepted by the National Medical Products Administration ("**NMPA**") in China.

- As one of the most advanced receptor tyrosine kinase-like orphan receptor 1 ("**ROR1**") antibody-drug conjugates ("**ADCs**") in clinical development, CS5001 has demonstrated therapeutic potential in multiple hematological and solid malignancies.

CS5001 is a potential global best-in-class ADC, targeting ROR1. As one of the three most advanced ROR1 ADCs worldwide, CS5001 has been approved for the initiation of a multi-regional clinical trial in the US and Australia. The NMPA has accepted the IND application of CS 5001 in China. This first-in-human Phase 1 study aims to evaluate the safety, tolerability, pharmacokinetics, and preliminary anti-tumor activity of CS5001 in advanced B cell lymphomas and solid tumors.

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. 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. The preclinical data were presented as a late-breaking abstract at the 33rd AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics 2021.

Dr. Archie Tse, Chief Scientific Officer of CStone, said: "We are very glad to have the first patient enrolled in the first-in-human study of CS5001. This potentially best-in-class ROR1 ADC contains a number of differentiated features which may translate into a wider therapeutic window against a variety of cancer types, including a fully human antibody backbone, proprietary site-specific conjugation, and tumor-cleavable linker and prodrug technology. Results from the preclinical studies of CS5001 already showed its therapeutic potential in ROR1-expressing hematological and solid malignancies. We will swiftly execute the global development program of CS5001, starting with this Phase 1 study to characterize its safety and preliminary efficacy in the treatment of advanced B-cell lymphoma and selected solid tumors."

About CS5001 (ROR1 ADC)

CS5001 is a clinical-stage ADC targeting ROR1. CS5001 was uniquely designed and used LCB's proprietary tumor-cleavable linker and pyrrolobenzodiazepine ("**PBD**") prodrug payload. Only after reaching the tumor, the linker and 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) which enables homogeneous production and large-scale manufacturing.

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.

About CStone

CStone is a biopharmaceutical company focused on research, development and commercialization of 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 seven NDA approvals for four drugs. 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, March 31, 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.