CStone Pharmaceuticals

基石藥業

(Incorporated in the Cayman Islands with limited liability)

(**Stock Code: 2616**)

VOLUNTARY ANNOUNCEMENT
CSTONE ANNOUNCED RESULTS FROM THE NAVIGATOR CHINA BRIDGING STUDY OF AYVAKIT® (AVAPRITINIB) PUBLISHED IN THE ONCOLOGIST

CStone Pharmaceuticals (the "**Company**" or "**CStone**") is pleased to announce that results from the NAVIGATOR China Bridging Study of AYVAKIT® (avapritinib) have been published in *The Oncologist*. The results showed that AYVAKIT was generally well-tolerated, had marked anti-tumor activity in Chinese patients with gastrointestinal stromal tumor ("**GIST**") harboring a PDGFRA D842V mutation, and highlighted efficacy data as a fourth- or later-line treatment.

AYVAKIT® is a potent, selective and orally available inhibitor of KIT and PDGFRA mutant kinases, discovered by CStone's partner Blueprint Medicines. CStone has an exclusive collaboration and license agreement with Blueprint Medicines for the development and commercialization of AYVAKIT in Mainland China, including Hong Kong, Macau and Taiwan.

The study is an open-label, multicenter phase 1/2 clinical trial designed to evaluate the safety, pharmacokinetics and anti-tumor activity of AYVAKIT® in Chinese patients with unresectable or metastatic GIST. Tumor response was assessed using Response Evaluation Criteria in Solid Tumors (RECIST) version 1.1, and all responses were confirmed.

Efficacy:

- In 28 patients with GIST harboring PDGFRA D842V mutations, the Independent Radiology Review Committee ("**IRRC**")- and investigator-assessed objective response rates ("**ORRs**") were 75% and 79%, respectively. The clinical benefit rates ("**CBRs**") were both 86%. The median duration of response ("**DOR**") and progression-free survival (PFS) were not reached. The overall survival ("**OS**") data were immature, and the 12-month overall survival rate was 92%.

- IRRC- and investigator-assessed ORRs in 23 patients with GIST in the fourth- or later-line setting were 22% and 35%, respectively. The CBRs were both 57%. The IRRC-assessed median DOR was not reached, and the median PFS was 5.6 months. The median investigator-assessed DOR was

9.4 months, and the PFS was 5.6 months. The OS data were immature, and the 12-month OS rate was 61%.

Safety:

- AYVAKIT® had a generally well-tolerated safety profile in Chinese patients with GIST. No new safety signal was observed.

Professor Shen Lin, Principal Investigator of the NAVIGATOR China Bridging Study, corresponding author of the published paper, and vice president of Peking University Cancer Hospital, said, "The results from the NAVIGATOR China Bridging Study published in *The Oncologist* further demonstrated that AYVAKIT® had impressive response rates in Chinese patients with advanced GIST harboring a PDGFRA exon 18 mutation and had anti-tumor activity in GIST patients receiving fourth-line or later-line treatment. The results show that AYVAKIT®, as a precision medicine, brings important benefits to GIST patients, even in later-line settings. We are optimistic that AYVAKIT® will benefit more Chinese patients in the future."

Dr. Jason Yang, Chief Executive Officer of CStone, said, "We are delighted that the updated data from the phase 1/2 bridging study of AYVAKIT® in Chinese patients have been published in *The Oncologist*. The results showed that AYVAKIT® was generally well-tolerated in Chinese patients, and the data further demonstrated the drug had outstanding anti-tumor activity in Chinese patients with GIST harboring a PDGFRA D842V mutation and notable response rates in Chinese GIST patients receiving fourth-line or later-line treatment. CStone is committed to the development of innovative oncology medicines. Moving forward, we aim to continue bringing more breakthrough therapies to patients in China and worldwide."

AYVAKIT® has been approved in Mainland China, Taiwan, China and Hong Kong, China. CStone is pursuing a broad commercialization program by working with healthcare professionals, industry associations and diagnostics companies to potentially transform the landscape for precision medicine. In addition, AYVAKIT® has been listed in more than 80 commercial insurance and government-led insurance programs in Greater China, further improving the drug's accessibility and affordability.

About AYVAKIT® (avapritinib)

AYVAKIT® (avapritinib) is a kinase inhibitor approved by the National Medical Products Administration (NMPA) of China for the treatment of adults with unresectable or metastatic GIST harboring the PDGFRA exon 18 mutation, including PDGFRA D842V mutations. AYVAKIT was approved by the Department of Health (DOH), Hong Kong, China, and Taiwan Food and Drug Administration (TFDA) for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA D842V mutation.

The U.S. Food and Drug Administration ("**FDA**") has approved AYVAKIT® for the treatment of two indications: adults with advanced systemic mastocytosis (Advanced SM), including aggressive SM (ASM), SM with an associated hematological neoplasm (SM-AHN) and mast cell leukemia (MCL), and adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations. This medicine is approved by the European Commission under the brand name AYVAKYT® for the treatment of adults with ASM, SM-AHN or MCL, after at least one systemic therapy, and adults with unresectable or metastatic GIST harboring the PDGFRA D842V mutation.

AYVAKIT/AYVAKYT is not approved for the treatment of any other indication in the U.S., Europe or Greater China. The FDA granted breakthrough therapy designation to AYVAKIT® for the treatment of moderate to severe indolent SM.

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 nine NDA approvals for its four drugs. Multiple late-stage drug candidates are now under pivotal clinical trials or registration. 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.

Trademarks

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Forward Looking Statement

There is no assurance that any forward-looking statements regarding the business development of the Group in this announcement or any of the matters set out herein are attainable, will actually occur or will be realised or are complete or accurate. The financial and other data relating to the Group as disclosed in this announcement has also not been audited or reviewed by its auditors. Shareholders and/or potential investors of the Company are advised to exercise caution when dealing in the securities of the Company and not to place any excessive reliance on the information disclosed herein. Any shareholder or potential investor who is in doubt is advised to seek advice from professional advisors.

By Order of the Board
CStone Pharmaceuticals
Dr. Wei Li
Chairman

Suzhou, the People's Republic of China, December 28, 2022

As at the date of this announcement, the board of directors of the Company comprises Dr. Wei Li as Chairman and non-executive director, Dr. Jianxin Yang as executive director, 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.