CStone Pharmaceuticals

基石藥業

(Incorporated in the Cayman Islands with limited liability)

(**Stock Code: 2616**)

VOLUNTARY ANNOUNCEMENT
CSTONE ANNOUNCED UPDATED RESULTS FOR AYVAKIT® (AVAPRITINIB) IN CHINESE PATIENTS WITH ADVANCED GIST AT ESMO WORLD CONGRESS ON GASTROINTESTINAL CANCER 2021

CStone Pharmaceuticals (the "**Company**" or "**CStone**") is pleased to announce results from a phase I/II clinical study of AYVAKIT® (avapritinib) in Chinese patients with advanced gastrointestinal ("**GI**") stromal tumor ("**GIST**"), via a short oral report at the virtual European Society for Medical Oncology World Congress on Gastrointestinal Cancer ("**ESMO GI**") 2021. The results bring additional clinical evidence for the treatment of patients with GIST that could potentially improve clinical practice.

Key Highlights

- AYVAKIT® has demonstrated significant anti-tumor activity in Chinese patients with PDGFRA D842V mutant GIST, and has also shown potential for the treatment of fourth-line GIST
- AYVAKIT® had a generally well-tolerated safety profile in Chinese patients with GIST, consistent with results observed in prior studies globally

Discovered by CStone's partner Blueprint Medicines Corporation (NASDAQ: BPMC) ("**Blueprint Medicines**"), AYVAKIT® is a potent, selective and orally available inhibitor of KIT and PDGFRA mutant kinases. CStone has an exclusive collaboration and license agreement with Blueprint Medicines for the development and commercialization of AYVAKIT® and certain other drug candidates in Mainland China, Hong Kong, Macau and Taiwan. Blueprint Medicines retains development and commercial rights for AYVAKIT® in the rest of the world.

The results are based on an open-label, multi-center phase I/II clinical study, evaluating the safety, pharmacokinetics and anti-tumor activity of AYVAKIT® in Chinese patients with unresectable or metastatic GIST. As of a data cutoff date of July 31, 2020, safety was evaluated in 60 patients who received 200 or 300 mg once-daily dosing of AYVAKIT®. Efficacy was assessed in 20 evaluable patients with PDGFRA D842V mutant GIST. Clinical activity was determined by the Independent

Radiological Review Committee ("**IRRC**"), according to modified Response Evaluation Criteria in Solid Tumors (mRECIST) version 1.1.

a. The updated results further confirm the significant anti-tumor activity of AYVAKIT® for the treatment of patients with advanced PDGFRA D842V mutant GIST, who have a poor remission rate and prognosis using existing tyrosine kinase inhibitors. The objective response rate ("**ORR**") was 70%, the clinical benefit rate ("**CBR**") was 80% and the median progression-free survival ("**PFS**") has not been reached.

b. AYVAKIT® showed anti-tumor activity in Chinese patients with GIST in the fourth-line or later treatment setting. The ORR was 17%, the CBR was 52% and the median PFS was 5.6 months, supporting the potential of AYVAKIT® for patients with GIST in later lines of therapy.

c. The study further demonstrated the generally well-tolerated safety profile of AYVAKIT® for the treatment of Chinese patients with GIST, consistent with results observed in prior studies globally. The most common treatment-emergent adverse events were anemia, increased blood bilirubin, decreased white blood cell count, increased blood creatine phosphokinase, increased aspartate aminotransferase, face edema, eyelid edema, decreased neutrophil count and hair color changes.

Professor Lin Shen, Principal Investigator of AYVAKIT® in China, and Vice President of Peking University Cancer Hospital, said, "Due to the extremely limited benefits from existing therapies, patients with PDGFRA D842V mutant GIST have high medical needs. The updated data presented at ESMO GI further prove the anti-tumor activity of AYVAKIT® in Chinese patients with advanced PDGFRA exon 18-mutant GIST, and these results also support the potential of AYVAKIT® for patients with fourth-line and above GIST. AYVAKIT® has shown a well-tolerated safety profile, and brings an important additional treatment option to patients."

Dr. Jason Yang, Chief Medical Officer of CStone, said, "We are glad to present updated results from the phase I/II bridging study of AYVAKIT® in Chinese patients with GIST at the ESMO GI 2021. The data show that AYVAKIT® was well-tolerated in Chinese patients and further highlight the drug's anti-tumor activity in patients with GIST harboring PDGFRA exon 18 mutations, including PDGFRA D842V mutant GIST. AYVAKIT® also showed potential in the fourth-line or later treatment setting for Chinese GIST patients. We expect AYVAKIT® to benefit patients with GIST."

About AYVAKIT® (avapritinib)

AYVAKIT (avapritinib) is a kinase inhibitor approved by the China National Medical Products Administration ("**NMPA**") for the treatment of adults with unresectable or metastatic GIST harboring the PDGFRA exon 18 mutation, including PDGFRA D842V mutations.

The U.S. Food and Drug Administration ("**FDA**") has approved AYVAKIT™ for the treatment of two indications: adults with advanced systemic mastocytosis ("**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 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, or for any indication in any other jurisdiction by any other health authority.

Blueprint Medicines is developing AYVAKIT® globally for the treatment of advanced and non-advanced SM. The FDA granted breakthrough therapy designation to AYVAKIT® for the treatment of advanced SM, including the subtypes of ASM, SM-AHN, and MCL, and for the treatment of moderate to severe indolent SM.

About GIST

GIST is a sarcoma, or tumor of bone or connective tissue, of the GI tract. Tumors arise from cells in the wall of the GI tract and occur most often in the stomach or small intestine. Most patients are diagnosed between the ages of 50 to 80, and diagnosis is typically triggered by GI bleeding, incidental findings during surgery or imaging and, in rare cases, tumor rupture or GI obstruction. About 5 to 6 percent of primary GIST cases are caused by a PDGFRA D842V mutation, the most common PDGFRA exon 18 mutation.

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 three drug approvals in Greater China, including two in mainland China 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, July 8, 2021

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. Qun Zhao, Mr. Yanling Cao, Mr. Xianghong Lin and Dr. Lian Yong Chen as non-executive Directors, and Dr. Paul Herbert Chew, Mr. Ting Yuk Anthony Wu and Mr. Hongbin Sun as independent non-executive Directors.