CStone Pharmaceuticals

基石藥業

(Incorporated in the Cayman Islands with limited liability)

(**Stock Code: 2616**)

VOLUNTARY ANNOUNCEMENT

CSTONE ANNOUNCED NEW DRUG APPROVAL OF PRECISION THERAPY AYVAKIT® (AVAPRITINIB) IN HONG KONG, CHINA FOR THE TREATMENT OF PDGFRA D842V MUTANT GASTROINTESTINAL STROMAL TUMORS

CStone Pharmaceuticals (the "**Company**" or "**CStone**") is pleased to announce that the new drug application ("**NDA**") of first-in-class precision therapy AYVAKIT® (avapritinib) has been approved in Hong Kong, China for the treatment of adult patients with unresectable or metastatic gastrointestinal ("**GI**") stromal tumor ("**GIST**") harboring a PDGFRA D842V mutation. The drug is the first precision therapy approved in Hong Kong, China for the treatment of patients with PDGFRA D842V mutant GIST. 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.

Key Highlight

- AYVAKIT® is the first precision therapy approved in Hong Kong, China for the treatment of patients with PDGFRA D842V mutant GIST.
- This is CStone's fifth NDA approval in Greater China this year.

Dr. Frank Jiang, Chairman and CEO of CStone, said, "AYVAKIT® is CStone's first product approval in Hong Kong, China. Earlier this year, AYVAKIT® was also approved in Mainland China and Taiwan. We are very glad to provide this innovative treatment to more GIST patients whose tumors harbor the PDGFRA D842V mutation. CStone is committed to bringing forward effective, innovative therapies to patients around the world. In the future, we will strive to accelerate the development of novel therapies to fulfill the unmet

medical needs of more cancer patients."

The Hong Kong Department of Health (DOH) has approved AYVAKIT based on data from the NAVIGATOR study, an open-label, dose-escalation/dose-expansion phase I study designed to evaluate the safety and efficacy of AYVAKIT® in patients with unresectable or metastatic GIST. In December 2020, the European Journal of Cancer (EJC) published updated data from the NAVIGATOR study enrolling PDGFRA D842V mutant GIST patients. In 38 patients with PDGFRA D842V mutant GIST who received a starting dose of 300 mg or 400 mg once daily, the overall response rate ("**ORR**") was 95% (36/38 patients). In 28 of these patients who had a starting dose of 300 mg once daily, the ORR was 96% (27/28 patients). The disease control rate (DCR) of all dose groups was 100%. The median duration of response (DOR) of all dose groups was 27.6 months. 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. The data showed that AYVAKIT® demonstrated robust, durable, and deep clinical activity, with a generally well-tolerated safety profile.

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 AYVAKIT® (avapritinib)

AYVAKIT® (avapritinib) is a kinase inhibitor approved by the Department of Health, Hong Kong, China, and Taiwan Food and Drug Administration under the brand name AYVAKIT® for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA D842V mutation. The National Medical Products Administration (NMPA) of China has approved AYVAKIT® 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 ("**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 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 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, December 28, 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. 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.