CStone Pharmaceuticals

基石藥業

(Incorporated in the Cayman Islands with limited liability)

(**Stock Code: 2616**)

VOLUNTARY ANNOUNCEMENT
CSTONE ANNOUNCED THE ACCEPTANCE OF NEW DRUG APPLICATION IN HONG KONG FOR AVAPRITINIB FOR THE TREATMENT OF ADULT PATIENTS WITH UNRESECTABLE OR METASTATIC PDGFRA D842V MUTANT GASTROINTESTINAL STROMAL TUMOR

CStone Pharmaceuticals (the "**Company**" or "**CStone**") is pleased to announce the acceptance of the Company's new drug application ("**NDA**") in Hong Kong for avapritinib, an investigational first-in-class precision therapy, for the treatment of adult patients with unresectable or metastatic gastrointestinal stromal tumor ("**GIST**") harboring a PDGFRA D842V mutation. Discovered by CStone's partner Blueprint Medicines Corporation (NASDAQ: BPMC) ("**Blueprint Medicines**"), avapritinib is a potent, highly selective and orally available inhibitor of KIT and PDGFRA.

Dr. Jason Yang, Chief Medical Officer of CStone, commented, "We are pleased to see that the application NDA for avapritinib has been accepted in Hong Kong, which marks another important milestone for this product to potentially reach more patients in China. Patients with GIST harboring PDGFRA D842V mutations continue to have significant unmet medical needs, due to limited effective treatment options. Recent data from patients with PDGFRA D842V mutant GIST who were treated with avapritinib, which were presented at the 2020 Chinese Society of Clinical Oncology ("**CSCO**") Annual Meeting, showed that the target lesions were reduced in all patients, and avapritinib has demonstrated a well-tolerated safety profile as well, most treatment-related adverse events were Grade 1-2. We look forward to potentially bringing avapritinib to GIST patients soon, so they may benefit from this precision therapy approach."

The data presented at the 2020 CSCO Annual Meeting were from an open-label, multicenter phase I/II bridging study designed to evaluate the safety, pharmacokinetics and anti-tumor activity of avapritinib in Chinese patients with unresectable or metastatic GIST. As of a data cutoff date of March 31, 2020, 8 patients with PDGFRA D842V mutant GIST were evaluable for response by investigator assessment using Response Evaluation Criteria in Solid Tumors ("**RECIST**") version 1.1, and 50 patients were included in the safety evaluation.

CStone has an exclusive collaboration and license agreement with Blueprint Medicines for the development and commercialization of avapritinib and certain other drug candidates in Mainland China, Hong Kong, Macau and Taiwan. Blueprint Medicines retains development and commercial rights for avapritinib in the rest of the world.

About GIST

GIST is a sarcoma, or tumor of bone or connective tissue, of the gastrointestinal ("**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 Avapritinib

Avapritinib is a kinase inhibitor approved by the China National Medical Products Administration ("**NMPA**") under the brand name AYVAKIT® for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations. The Taiwan Food and Drug Administration has approved AYVAKIT® for the treatment of adults with unresectable or metastatic gastrointestinal stromal tumor harboring a PDGFRA D842V mutations.

The U.S. Food and Drug Administration ("**FDA**") has approved AYVAKIT™ for the treatment of adults with unresectable or 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.

Avapritinib is not approved for the treatment of any other indication in Mainland China by the NMPA, in Taiwan by TFDA, in the U.S. by the FDA or in Europe by the European Commission, or for any indication in any other jurisdiction by any other health authority.

Blueprint Medicines is developing avapritinib globally for the treatment of advanced and indolent systemic mastocytosis ("**SM**"). The FDA granted breakthrough therapy designation to avapritinib for the treatment of advanced SM, including the subtypes of aggressive SM, SM with an associated hematologic neoplasm and mast cell leukemia, and for the treatment of moderate to severe indolent SM.

About CStone

CStone is a biopharmaceutical company focused on 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 14 drug candidates with a strategic emphasis on immuno-oncology combination therapies. CStone has received three NDA approvals, including two in Mainland China and one in Taiwan, and multiple late-stage candidates are in pivotal trials or registrational stages. 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, People's Republic of China, May 12, 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.