CStone Pharmaceuticals

基石藥業

(Incorporated in the Cayman Islands with limited liability)

(**Stock Code: 2616**)

VOLUNTARY ANNOUNCEMENT

CSTONE'S PARTNER BLUEPRINT MEDICINES RECEIVED POSITIVE CHMP OPINION FOR AVAPRITINIB FOR THE TREATMENT OF ADULTS WITH UNRESECTABLE OR METASTATIC PDGFRA D842V MUTANT GASTROINTESTINAL STROMAL TUMORS

The partner of CStone Pharmaceuticals (the "**Company**" or "**CStone**"), Blueprint Medicines Corporation (NASDAQ: BPMC) ("**Blueprint Medicines**"), announced on July 24, 2020 that the European Medicines Agency's Committee for Medicinal Products for Human Use ("**CHMP**") has adopted a positive opinion, recommending conditional marketing authorization for avapritinib as a monotherapy for the treatment of adult patients with unresectable or metastatic gastrointestinal stromal tumors ("**GIST**") harbouring platelet-derived growth factor receptor A ("**PDGFRA**") D842V mutation.

The CHMP opinion will now be reviewed by the European Commission, which has the authority to grant marketing authorization for medicinal products in the European Union ("**EU**"). A final decision on the marketing authorization application for avapritinib is anticipated by the end of September 2020. If approved by the European Commission, avapritinib would be the first treatment in the EU indicated for patients with PDGFRA D842V mutant GIST and would be commercialized under the brand name AYVAKYT®.

The CHMP based its opinion on efficacy results from the Phase I NAVIGATOR trial as well as combined safety results from the NAVIGATOR and Phase III VOYAGER trials. Treatment with avapritinib showed deep and durable clinical responses and was well-tolerated in patients with PDGFRA D842V mutant GIST. Data in this patient population were published in *The Lancet Oncology* on June 29, 2020.

About avapritinib

avapritinib is a kinase inhibitor approved by the Food and Drug Administration ("**FDA**") of the United States ("**U.S.**") 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.

avapritinib is not approved for the treatment of any other indication, including systemic mastocytosis, in the U.S. by the U.S. FDA or for any indication in any other jurisdiction by any other health authority.

Blueprint Medicines is developing avapritinib globally for the treatment of advanced, smoldering and indolent systemic mastocytosis. The U.S. FDA granted a breakthrough therapy designation to avapritinib for the treatment of advanced systemic mastocytosis, including the subtypes of aggressive systemic mastocytosis, systemic mastocytosis with an associated hematologic neoplasm and mast cell leukemia.

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 gastrointestinal ("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% of primary GIST cases are caused by a PDGFRA D842V mutation, the most common PDGFRA exon 18 mutation. Prior to the U.S. FDA approval of avapritinib, there were no highly effective treatments for PDGFRA D842V mutant GIST. Published data have shown poor outcomes in patients with PDGFRA D842V mutant GIST treated with imatinib and other approved therapies, including a median overall survival of 15 months, a median progression-free survival of 3 months and an overall response rate of 0%.

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 at the end of 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, five late-stage candidates are at pivotal trials. With an experienced team, a rich pipeline, a robust clinical development-driven business model and substantial funding, CStone's vision is to become globally recognized as a leading Chinese 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, August 5, 2020

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. Guobin Zhang 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.