The forward-looking statements made in this announcement relate only to the events or information as of the date on which the statements are made in this announcement. Except as required by law, we undertake no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise, after the date on which the statements are made or to reflect the occurrence of unanticipated events. You should read this announcement completely and with the understanding that our actual future results or performance may be materially different from what we expect. In this announcement, statements of, or references to, our intentions or those of any of our directors and/or our Company are made as of the date of this announcement. Any of these intentions may alter in light of future development.

CStone Pharmaceuticals

基石藥業

(Incorporated in the Cayman Islands with limited liability)

(**Stock Code: 2616**)

VOLUNTARY ANNOUNCEMENT

CSTONE ANNOUNCES ACCEPTANCE OF ITS FIRST NEW DRUG APPLICATION IN MAINLAND CHINA BY THE NATIONAL MEDICAL PRODUCTS ADMINISTRATION FOR THE FIRST-IN-CLASS PRECISION THERAPY AVAPRITNIB IN TWO GASTROINTESTINAL STROMAL TUMOR INDICATIONS

CStone Pharmaceuticals (the "**Company**" or "**CStone**") announced that the China National Medical Products Administration ("**NMPA**") has accepted the New Drug Application ("**NDA**") of the precision therapy avapritinib for two indications: one for the treatment of adults with unresectable or metastatic gastrointestinal stromal tumor ("**GIST**") harboring a platelet-derived growth factor receptor alpha ("**PDGFRA**") exon 18 mutation, including PDGFRA D842V mutations, and the other for the treatment of adults with unresectable or metastatic fourth-line GIST. Developed by CStone's partner, Blueprint Medicines Corporation (NASDAQ: BPMC) ("**Blueprint Medicines**"), avapritinib is an investigational, orally available, potent and selective inhibitor of KIT and PDGFRA mutant kinases. This is the first time CStone has submitted an NDA to the NMPA and marks another milestone in the Company's transition towards commercialization.

The key updates include:

- The NMPA has accepted the NDA for two indications for avapritinib, a first-in-class precision therapy, in gastrointestinal stromal tumor (GIST), marking a key milestone in CStone's transition toward commercialization;

- In just three months after avapritinib was approved by the U.S. Food and Drug Administration ("**U.S. FDA**"), CStone has submitted NDAs for avapritinib in Taiwan and mainland China, with the goal of making this new drug accessible to patients in Greater China soon;
- Results from the NAVIGATOR study have shown an overall response rate ("**ORR**") of 86% in patients with PDGFRA exon 18 mutant GIST and an ORR of 22% in fourth-line GIST;
- Preliminary results from the bridging study conducted by CStone in China demonstrate safety and pharmacokinetic profiles consistent with those previously reported for the global NAVIGATOR study.

With an annual incidence rate of 1-1.5 per 100,000, there are approximately 14,000 to 21,000 new cases of GIST in China every year, and approximately 90% of these cases are associated with dysregulated cell growth due to mutations in KIT or PDGFRA tyrosine kinases. In January 2020, avapritinib was approved by the U.S FDA for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations, and became the first and only precision medicine approved for the treatment of GIST harboring a PDGFRA exon 18 mutation in the U.S.

Dr. Frank Ningjun Jiang, chairman, executive director and chief executive officer of CStone, commented: "In just three months after avapritinib was approved by the U.S. FDA, CStone has submitted NDAs for this drug candidate in Taiwan and mainland China, which we hope will make this first-in-class precision therapy candidate accessible to patients with advanced GIST in Greater China soon. As CStone continues to accelerate its transition towards commercialization, we plan to submit several NDAs in China across multiple indications for our lead assets in the next few months."

Dr. Lin Shen, M.D., Professor and Director of Department of Gastrointestinal Oncology, Vice President of Peking University Cancer Hospital and Institute, and the principal investigator for the bridging study of avapritinib in GIST in China, commented: "Avapritinib has demonstrated outstanding antitumor activity and a well-tolerated safety profile in advanced PDGFRA exon 18 mutant GIST and fourth-line GIST. Due to the very limited benefits from approved treatment options in these two groups of GIST patients, there is an urgent unmet clinical need for new therapies. As a physician, I hope avapritinib will be available in our clinical practice for the treatment of advanced GIST soon."

Results from the Phase I NAVIGATOR study of avapritinib in PDGFRA exon 18 mutant GIST and fourth-line GIST were presented at the Connective Tissue Oncology Society Annual Meeting in November 2019. As of the data cut-off date of November 16, 2018:

- 43 patients with PDGFRA exon 18 mutant GIST and 111 patients with fourth-line GIST were treated at a starting dose of 300 or 400 mg once daily and evaluable for response assessments;
- In patients with PDGFRA Exon 18 mutant GIST, the ORR was 86% with one response pending confirmation, and the median duration of response ("**DOR**") was not reached;
- In patients with fourth-line GIST, the ORR was 22% with one response pending confirmation, and the median DOR was 10.2 months.

The Phase I/II bridging study conducted by CStone in patients with advanced GIST in China has produced encouraging preliminary results demonstrating avapritinib was well-tolerated, and safety and pharmacokinetic profiles were consistent with those previously reported for the global NAVIGATOR study.

CStone's chief medical officer, Dr. Jason Yang, commented: "The current treatment approach for GIST in China is mainly based on sequential tyrosine kinase inhibitors ("**TKIs**"), but the approved TKIs only offer limited efficacy in patients with PDGFRA D842V mutations. Moreover, Chinese patients with fourth-line GIST face challenges on multiple fronts, including drug-resistant mutations and a lack of effective treatment options. I am pleased that the bridging study in China has yielded results consistent with those from the global NAVIGATOR study and I hope patients with advanced GIST who are in urgent need for new treatment options will benefit from this precision therapy in the near future."

CStone Pharmaceuticals and Blueprint Medicines have entered into an exclusive collaboration and license agreement 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 these licensed products in the rest of the world.

About Avapritinib

Avapritinib is an investigational, selective and potent inhibitor of KIT and PDGFRA mutant kinases. It is a type 1 inhibitor that works by directly binding to the active kinase conformation from which mutant KIT and PDGFRA signal. Avapritinib has demonstrated inhibition of a broad range of KIT and PDGFRA mutations associated with GIST, including potent clinical activity against activation loop mutations that are associated with resistance to currently approved therapies in Greater China.

Blueprint Medicines is pursuing a broad clinical development program for avapritinib across multiple lines of GIST treatment, as well as for advanced, smoldering and indolent systemic mastocytosis.

Avapritinib is a kinase inhibitor approved by the U.S. FDA 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 has not been approved for the treatment of any other indication in the U.S. or for the treatment of any indication by the Taiwan Food and Drug Administration in Taiwan, by the NMPA in mainland China or by any other health authority in any other jurisdiction.

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 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, April 23, 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.