CStone Pharmaceuticals

基石藥業

(Incorporated in the Cayman Islands with limited liability)

(**Stock Code: 2616**)

VOLUNTARY ANNOUNCEMENT

CSTONE SUBMITS NEW DRUG APPLICATION FOR THE TARGETED THERAPY AVAPRITINIB IN TAIWAN FOR THE TREATMENT OF ADULTS WITH ADVANCED PDGFRA EXON 18 MUTANT GASTROINTESTINAL STROMAL TUMOR

CStone Pharmaceuticals (the "**Company**" or "**CStone**") announced today that the Company has submitted a New Drug Application ("**NDA**") to the Taiwan Food and Drug Administration ("**TFDA**") for avapritinib, a precision therapy being developed for the treatment of gastrointestinal stromal tumor ("**GIST**"), and had received priority review designation from the TFDA on March 9, 2020. This drug candidate is for the indication of adult patients with unresectable or metastatic GIST harboring a platelet-derived growth factor receptor alpha (PDGFRA) exon 18 mutation, including PDGFRA D842V mutations. Discovered by CStone's partner, Blueprint Medicines Corporation (NASDAQ: BPMC) ("**Blueprint Medicines**"), avapritinib is the second drug candidate for which CStone has submitted an NDA.

The key updates include:

- Data submitted to the TFDA show an overall response rate ("**ORR**") of 84% with most adverse events reported as Grade 1 or 2 for avapritinib in patients with advanced GIST harboring a PDGFRA exon 18 mutation, including PDGFRA D842V mutations;
- In January 2020, avapritinib received approval from the U.S. Food and Drug Administration ("**U.S. FDA**") and became the first and only marketed precision therapy for the treatment of unresectable or metastatic PDGFRA exon 18 mutant GIST.

Dr. Frank Ningjun Jiang, chairman, executive director and chief executive officer of CStone, commented: "Within one year, CStone has submitted NDAs for two first-in-class targeted-therapies, demonstrating our commitment to addressing urgent clinical needs and the rapid transformation of the Company towards commercialization. GIST is a rare tumor type and PDGFRA D842V mutant GIST is resistant to currently approved therapies in the Greater China area. In addition to Taiwan, we plan to submit an avapritinib NDA for the same indication in mainland China in the first half of this year with the goal of benefitting more patients suffering from this devastating condition."

CStone's chief medical officer, Dr. Jason Yang, commented: "Avapritinib is an investigational, orally available, potent and selective inhibitor of KIT and PDGFRA. In January this year, avapritinib received an approval from the U.S. FDA for the treatment of adults with unresectable or metastatic PDGFRA exon 18 mutant GIST and became the first and only marketed therapy in the U.S. for this indication. Clinical data submitted to the TFDA show unprecedented antitumor activity of avapritinib in advanced GIST patients with a PDGFRA exon 18 mutation, including PDGFRA D842V mutations, with an ORR of 84% and most AEs reported as Grade 1 or 2. In addition, the median duration of response ("**DOR**") was not reached and 61% of these patients had a DOR of more than six months."

GIST is the most common mesenchymal tumor of the gastrointestinal tract, and it is most prevalent in patients aged 50 to 80. Approximately 90% of all GIST cases are associated with dysregulated cell growth due to mutations in KIT and PDGFRA tyrosine kinases. As GIST patients do not respond well to chemotherapy and radiotherapy, current treatment for advanced GIST is primarily based on sequential treatment with tyrosine kinase inhibitors ("**TKI**"). However, GIST patients harboring PDGFRA D842V mutations are not sensitive to existing approved TKIs in the Greater China area, with studies showing an ORR of 0%, a median progression-free survival of three to five months, and a median overall survival of about 15 months. Except D842V, the approved therapies have shown limited effects against other rare PDGFRA exon 18 mutations.

CStone 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 the Greater China area.

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 indications in the U.S. or for the treatment of any indication by the TFDA in Taiwan, by the National Medical Products Administration 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, March 27, 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.