CStone Pharmaceuticals
基石藥業
(Incorporated in the Cayman Islands with limited liability)
(Stock Code: 2616)

VOLUNTARY ANNOUNCEMENT
CSTONE ANNOUNCED PRECISION MEDICINE AVAPRITINIB (AYVAKIT®) RECOMMENDED IN CHINA'S FIRST "CHINESE GUIDELINES FOR THE DIAGNOSIS AND TREATMENT OF SYSTEMIC MASTOCYTOSIS IN ADULT PATIENTS"

CStone Pharmaceuticals (the "**Company**" or "**CStone**") is pleased to announce that avapritinib (AYVAKIT®) has been included as a recommended drug for the treatment of advanced systemic mastocytosis ("**SM**") in "Chinese Guidelines for the Diagnosis and Treatment of Systemic Mastocytosis in Adult Patients" (the "**Guidelines**") that has recently been released as the first SM guidelines in China.

Avapritinib (AYVAKIT) is a potent, selective and orally available inhibitor of KIT and PDGFRA mutant kinases, discovered by CStone's partner Blueprint Medicines Corporation (NASDAQ: BPMC) ("**Blueprint Medicines**"). CStone has an exclusive collaboration and license agreement with Blueprint Medicines for the development and commercialization of avapritinib in Mainland China, Hong Kong, Macau and Taiwan.

The Guidelines was a coordinated effort led by the Laboratory Diagnosis Group of the Chinese Society of Hematology, Chinese Medical Association and the Chinese Mastocytosis Research Network, and was based on extensive consultation with experts in China. The Guidelines provide a clear definition of SM, as well as the standardized diagnostic procedures, diagnostic and differential diagnostic criteria, prognostic and risk stratification methods, treatment regimens, and the criteria for patient response assessment in SM. The publication of the Guidelines will further standardize the clinical diagnosis and treatment of SM by hematologists in China, accelerate the innovation in the field of SM, and benefit patients with SM in China.

Professor Suning Chen, Chief Physician of the Department of Hematology, The First Affiliated Hospital of Soochow University, said, "for a long time, the diagnosis and treatment of SM has not received adequate attention from domestic hematologists, and patients often miss the crucial window of opportunity for early diagnosis and treatment. The publication of the Guidelines marks a significant step towards standardizing the diagnosis and treatment of this disease in China."

Dr. Josh Zhou, Greater China General Manager and Head of Commercial of CStone Pharmaceuticals, said, "advanced SM is a rare disease, for which almost all the cases are driven by KIT D816V mutations. Avapritinib (AYVAKIT), as a potent and highly selective inhibitor of the KIT D816V mutation, targets the primary disease-causing gene that can lead to uncontrolled proliferation and activation of mast cells. The inclusion of avapritinib (AYVAKIT) in the Guidelines as a recommended treatment indicates a key advancement in clinical practice. At CStone, our goal is to bring the first-in-class or best-in-class drugs to those patients with unmet medical needs. We will continue to improve the accessibility and affordability of these life-changing medicines for more patients."

SM is a rare disease driven by the KIT D816V mutation in about 95 percent of the cases. SM is classified into two subtypes, indolent SM, which is the most common type of SM accounting for the vast majority of SM cases, and advanced SM, which is a group of high-risk SM forms that include aggressive SM ("**ASM**"), SM with an associated hematologic neoplasm ("**SM-AHN**"), and mast cell leukemia ("**MCL**"). In addition to symptoms of mast cell activation, advanced SM is also associated with organ damage and poor survival prognosis resulting from mast cell infiltration.

Avapritinib (AYVAKIT) has been approved by the U.S. Food and Drug Administration ("**FDA**") for the treatment of adult patients with advanced SM, including ASM, SM-AHN, and MCL. The FDA granted avapritinib (AYVAKIT) Orphan Drug Designation (ODD) and Breakthrough Therapy Designation (BTD) for the treatment of advanced SM. This medicine has also been approved by the European Commission under the brand name AYVAKYT for the treatment of adults with ASM, SM-AHN or MCL, after at least one systemic therapy, and adults with unresectable or metastatic GIST harboring the PDGFRA D842V mutation.

About avapritinib (AYVAKIT)

Avapritinib (AYVAKIT) is a kinase inhibitor approved by the National Medical Products Administration (NMPA) of China for the treatment of adults with unresectable or metastatic GIST harboring the PDGFRA exon 18 mutation, including PDGFRA D842V mutations. Avapritinib was approved by the Department of Health (DOH), Hong Kong, China, and Taiwan Food and Drug Administration (TFDA) for the treatment of adults with unresectable or metastatic GIST harboring a PDGFRA D842V mutation.

The FDA has approved avapritinib for the treatment of two indications: adults with advanced SM, including ASM, SM-AHN and 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 ASM, SM-AHN or MCL, after at least one systemic therapy, and 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. The FDA granted breakthrough therapy designation to AYVAKIT for the treatment of moderate to severe indolent SM.

About CStone

CStone is a biopharmaceutical company focused on research, development, and commercialization of 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 ten NDA approvals for its four drugs. Multiple late-stage drug candidates are now under pivotal clinical trials or registration. 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.

Trademarks

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Forward Looking Statement

There is no assurance that any forward-looking statements regarding the business development of the Group in this announcement or any of the matters set out herein are attainable, will actually occur or will be realised or are complete or accurate. The financial and other data relating to the Group as disclosed in this announcement has also not been audited or reviewed by its auditors. Shareholders and/or potential investors of the Company are advised to exercise caution when dealing in the securities of the Company and not to place any excessive reliance on the information disclosed herein. Any shareholder or potential investor who is in doubt is advised to seek advice from professional advisors.

By Order of the Board
CStone Pharmaceuticals
Dr. Wei Li
Chairman

Suzhou, the People's Republic of China, February 14, 2023

As at the date of this announcement, the board of directors of the Company comprises Dr. Wei Li as Chairman and non-executive director, Dr. Jianxin Yang as executive director, Mr. Kenneth Walton Hitchner III, 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.