CStone Pharmaceuticals

基石藥業

(Incorporated in the Cayman Islands with limited liability)

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VOLUNTARY ANNOUNCEMENT
CSTONE ANNOUNCED POSITIVE REGISTRATIONAL STUDY OF THE FIRST-IN-CLASS DRUG IVOSIDENIB IN CHINESE PATIENTS WITH RELAPSED OR REFRACTORY ACUTE MYELOID LEUKEMIA, AND THE NEW DRUG APPLICATION HAS BEEN ACCEPTED BY THE NMPA OF CHINA AND CONSIDERED FOR PRIORITY REVIEW

CStone Pharmaceuticals (the "**Company**" or "**CStone**") is pleased to announce that the China registrational study CS3010-101 of TIBSOVO® (ivosidenib tablets) has met the pre-specified endpoints in Chinese patients with relapsed or refractory acute myeloid leukemia ("**R/R AML**") with a susceptible isocitrate dehydrogenase 1 ("**IDH1**") mutation. The results demonstrated efficacy and manageable safety of TIBSOVO® (ivosidenib tablets), which were consistent with previously reported data from the global study population. Detailed data of this registrational study will be reported at an international academic conference in the near future.

Meanwhile, CStone announced that the National Medical Products Administration ("**NMPA**") of China has accepted the new drug application ("**NDA**") of TIBSOVO® (ivosidenib tablets) in adult patients with R/R AML who have a susceptible IDH1 mutation and this NDA has been considered for priority review.

Key Highlights

- Ivosidenib (TIBSOVO®, the brand name in the U.S.) is the first IDH1 inhibitor in China that has demonstrated its efficacy and manageable safety in patients with R/R AML
- The NMPA of China has accepted the NDA of TIBSOVO® (ivosidenib tablets) for the treatment of adults with R/R AML with a susceptible IDH1 mutation and this NDA has been considered for priority review

Professor Wang Jianxiang from the Institute of Hematology & Blood Diseases Hospital, Chinese

Academy of Medical Sciences, the principal investigator of the CS3010-101 study, said, "In the field of AML treatment, we are facing urgent clinical treatment needs, especially for R/R AML patients with a susceptible IDH1 mutation, currently there is no targeted therapy available in China market. Congratulations on the success of the registrational study of TIBSOVO® (ivosidenib tablets) in Chinese patients with R/R AML and we look forward to its early approval and launch in China, which will bring new hope for AML patients."

Dr. Jason Yang, Chief Medical Officer of CStone, said, "We are glad that TIBSOVO® (ivosidenib tablets) demonstrated efficacy and manageable safety in Chinese patients with R/R AML. TIBSOVO® (ivosidenib tablets) is our fourth product with an NDA, after GAVRETO®, AYVAKIT®, and sugemalimab, bringing the total number of our NDAs or supplemental NDAs ("**sNDAs**") to seven. We will work closely with the NMPA of China, aiming to make this innovative drug available to Chinese patients as soon as possible."

In 2020, TIBSOVO® (ivosidenib tablets) was selected in the list of the third batch of Overseas New Drugs Urgently Needed In Clinical Settings by the Center for Drug Evaluation, NMPA of China, and granted for fast-track designation. As a potent and highly selective first-in-class oral IDH1 inhibitor, TIBSOVO® (ivosidenib tablets) was also recommended by the 2020 edition of the CSCO Guidelines for Diagnosis and Treatment of Hematological Malignancies due to its proven clinical advantages.

Currently, TIBSOVO® (ivosidenib tablets) is the only targeted therapy approved in the U.S. by the U.S. Food and Drug Administration ("**FDA**") for the treatment of AML patients with a susceptible IDH1 mutation. TIBSOVO® (ivosidenib tablets) is also approved in the U.S. as monotherapy for the treatment of adults with susceptible IDH1-mutant R/R AML and for adults with newly diagnosed susceptible IDH1-mutant AML who are not less than 75 years old or who have comorbidities that preclude the use of intensive induction chemotherapy.

TIBSOVO® (ivosidenib tablets) is commercialized in the U.S. by Servier Pharmaceuticals.

About AML

AML is the most common leukemia in adults. It is a rapidly progressing disease with a high incidence in the elderly. In the U.S., there are about 20,000 new cases each year, and the five-year survival rate is about 29%. The incidence of AML in China is 1.4/100,000 in men and 1.2/100,000 in women, and about 6-10% of patients with AML carry IDH1 mutations. Somatic IDH1 mutation results in the accumulation of oncometabolite 2-HG which impairs differentiation of hematopoietic stem cells into mature blood cells, contributing to oncogenesis. Along with the aging of the population, the incidence of AML in China has been increasing over the years, and elderly patients and R/R AML patients have a poor prognosis.

About CS3010-101 China Bridging Study

CS3010-101 is an ongoing phase I, multi-center, single-arm study in China, which aims to evaluate the pharmacokinetic ("**PK**"), pharmacodynamics ("**PD**"), safety, and clinical efficacy of orally administrated ivosidenib in Chinese adults with R/R AML with a susceptible IDH1 mutation. Being the bridging study of the global pivotal AG120-C-001 study, the study provides data on R/R AML patients in China.

About TIBSOVO® (ivosidenib tablets)

Servier is a global independent pharmaceutical group governed by a foundation. In the field of oncology, Servier conducts activities in research, development, and commercialization of therapeutic solutions in different diseases including AML. Servier and CStone entered into a collaboration and

license agreement for the development and commercialization of TIBSOVO® (ivosidenib tablets) in some territories including mainland China.

TIBSOVO® (ivosidenib tablets) is currently approved in the U.S. since 2018 as monotherapy for the treatment of adults with susceptible IDH1-mutant R/R AML. In 2019, the indication was extended by the FDA to include the treatment of newly diagnosed susceptible IDH1-mutant AML adult patients who are not less than 75 years old or who have comorbidities that preclude the use of intensive induction chemotherapy.

The FDA has granted Breakthrough Therapy Designation for TIBSOVO® (ivosidenib tablets) in combination with azacytidine for this supplemental indication and Breakthrough Therapy Designation for TIBSOVO® (ivosidenib tablets) for the treatment of adult patients with relapsed and refractory myelodysplastic syndrome ("**MDS**") with a susceptible IDH1 mutation.

About CStone

CStone is a biopharmaceutical company focused on researching, 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, CStone has received three drug approvals in Greater China, including two in mainland China and one in Taiwan. CStone's vision is to become globally recognized as a world-renowned biopharmaceutical company by bringing innovative oncology therapies to cancer patients worldwide.

This TIBSOVO®(ivosidenib tablets) NDA is the third NDA acceptance of CStone in 2021, after GAVRETO® (pralsetinib) in patients with metastatic rearranged during transfection ("**RET**")-mutant medullary thyroid cancer and RET fusion-positive thyroid cancer, and AYVAKIT® (avapritinib) in patients with PDGFRA D842V-mutant gastrointestinal stromal tumor in Hong Kong, China. GAVRETO® and AYVAKIT® were approved by the NMPA of China and commercially launched in mainland China. AYVAKIT® was also approved for launch by the Taiwan Food and Drug Administration for adults with unresectable or metastatic gastrointestinal stromal tumor ("**GIST**") who have a PDGFRA D842V mutation.

For more information about CStone, please visit: www.cstonepharma.com.

By Order of the Board
CStone Pharmaceuticals
Dr. Frank Ningjun Jiang
Chairman

Suzhou, the People's Republic of China, August 3, 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 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.