CStone Pharmaceuticals

基石藥業

(Incorporated in the Cayman Islands with limited liability)

(**Stock Code: 2616**)

VOLUNTARY ANNOUNCEMENT

NMPA HAS ACCEPTED ITS NEW DRUG APPLICATION WITH PRIORITY REVIEW DESIGNATION FOR PRALSETINIB FOR THE TREATMENT OF PATIENTS WITH RET FUSION-POSITIVE NSCLC

CStone Pharmaceuticals (the "**Company**" or "**CStone**") announced that National Medical Products Administration ("**NMPA**") of China has accepted the Company's New Drug Application ("**NDA**") for pralsetinib for the treatment of RET fusion-positive non-small cell lung cancer ("**NSCLC**") patients previously treated with platinum-based chemotherapy. Developed by CStone's partner, Blueprint Medicines Corporation (NASDAQ: BPMC) ("**Blueprint Medicines**"), pralsetinib is an investigational, oral, highly potent and selective drug targeting oncogenic RET alterations, including predicted resistance mutations. Pralsetinib showed deep and durable anti-tumor activity with a well-tolerated safety profile in the Chinese cohort of patients with RET fusion-positive NSCLC previously treated with platinum-based chemotherapy. These results were consistent with previously reported data in the global patient population of the ARROW study.

A principal investigator of the ARROW study, Professor Wu Yilong from the Guangdong Provincial People's Hospital, said, "In the field of lung cancer precision medicine, following targets such as EGFR, ALK, ROS1 and NTRK, the research and development of a selective treatment that targets RET-driven NSCLC is another huge breakthrough. At present, there is no approved selective RET inhibitor in China, representing a huge unmet medical need for RET fusion-positive NSCLC patients. The global trial of pralsetinib as well as the Chinese cohort of patients with NSCLC yielded exciting results, and we are looking forward to potentially making this agent available to benefit this group of patients."

Dr. Frank Ningjun Jiang, executive director, chairman and chief executive officer of CStone, noted, "We are pleased to see that the NMPA has accepted the company's NDA for pralsetinib with the priority review designation for the treatment of RET fusion-positive NSCLC patients previously treated with platinum-based chemotherapy. This is the third NDA submitted by CStone globally and the second in mainland China in 2020, which reflects that CStone Pharmaceuticals is accelerating its commercialization strategy transformation. We are looking forward to domestic marketing of the agent pending regulatory review and bringing new hope to RET fusion-positive NSCLC patients in China."

Dr. Jason Yang, the chief medical officer of CStone, commented, "We are glad to see that pralsetinib showed rapid and durable anti-tumor activity and was well-tolerated in clinical trials. From the time that we entered into our collaboration with Blueprint Medicines, which enabled us to develop and commercialize pralsetinib in Greater China, to successfully submitting the NDA in China, it has only taken two years. We will continue to make every effort to advance other research and development of pralsetinib in China, more broadly assessing the efficacy of this agent in patients with treatment-naïve RET fusion-positive NSCLC, as well as patients with thyroid cancer and other solid tumors driven by RET alterations, so as to meet the urgent medical needs of these groups of Chinese cancer patients at the earliest possible date."

CStone has an exclusive collaboration and license agreement with Blueprint Medicines for the development and commercialization of pralsetinib, as a single agent or a combination therapy, in greater China ("**Greater China**"), which encompasses mainland of the People's Republic of China, Hong Kong Special Administrative Region, Macau Special Administrative Region and Taiwan. Outside Greater China, Blueprint Medicines is co-developing pralsetinib with Roche and Genentech, a member of the Roche Group, globally. Roche will co-commercialize pralsetinib with Blueprint Medicines in the United States ("**U.S.**") and has exclusive commercialization rights for pralsetinib outside of the U.S. and Greater China.

About the ARROW study

The ARROW study is a global clinical study designed to assess the safety, tolerability, and efficacy of pralsetinib in patients with RET fusion-positive NSCLC, RET-mutant medullary thyroid cancer, or other advanced solid tumors with RET alterations. According to previous announcements released by CStone, Chinese sites completed dosing of the first RET fusion-positive NSCLC patient previously treated with platinum-based chemotherapy in August 2019 and completed the last patient enrollment in December of the same year. CStone plans to present additional ARROW study data for pralsetinib from patients in the Chinese cohort at an upcoming academic conference.

About pralsetinib

Pralsetinib is an investigational, once-daily oral precision therapy specifically designed for highly potent and selective targeting of oncogenic RET alterations. Blueprint Medicines is developing pralsetinib for the treatment of patients with RET-altered NSCLC, thyroid cancer and other solid tumors. The U.S. Food and Drug Administration ("**FDA**") has granted a "Breakthrough Therapy Designation" recognition to pralsetinib for the treatment of RET-fusion positive NSCLC that has progressed following platinum-based chemotherapy, and RET-mutant medullary thyroid cancer that requires systemic treatment and for which there are no acceptable alternative treatments. In May 2020, Blueprint Medicines announced that the U.S. and European Union marketing applications for pralsetinib for the treatment of locally advanced or metastatic RET fusion-

positive NSCLC were accepted by the U.S. FDA and validated by the European Medicines Agency, respectively.

Pralsetinib was designed by Blueprint Medicines' research team, leveraging its proprietary compound library. In pre-clinical studies, pralsetinib consistently demonstrated sub-nanomolar potency against the most common RET fusions, activating mutations and predicted resistance mutations. In these studies, pralsetinib demonstrated markedly improved selectivity for RET compared to pharmacologically relevant kinases, including approximately 80-fold improved potency for RET compared with VEFGR2. By suppressing primary and secondary mutants, pralsetinib has the potential to overcome and prevent the emergence of clinical resistance. This approach may enable durable clinical responses across a diverse range of RET alterations, with a favorable safety profile.

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, September 7, 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.