CStone Pharmaceuticals
基石藥業

(Incorporated in the Cayman Islands with limited liability)

(**Stock Code: 2616**)

VOLUNTARY ANNOUNCEMENT

CSTONE SUCCESSFULLY HOSTED THE FIRST U.S. R&D DAY IN NEW YORK

CStone Pharmaceuticals (the "**Company**" or "**CStone**") announces that it successfully hosted its 2020 U.S. R&D Day in New York City, the United States on January 21, 2020. CStone's management team highlighted major milestones the Company had achieved in research, clinical development, business development and commercialization strategies since its establishment. The management team also provided updates on the progress of the Company's pipeline. Leading academics and key opinion leaders ("**KOL**") from China and the United States provided insights on the latest trends in the transformation of cancer treatments in China.

Targeting five indications, CStone plans to submit three NDAs in 2020 for CS1001, an anti-PD-L1 monoclonal antibody; pralsetinib, a RET inhibitor; and AYVAKIT™ (avapritinib), a KIT and PDGFRA inhibitor, in Mainland China and Taiwan. The Company plans to release the top-line data from seven ongoing clinical trials of these three drug candidates. CStone will focus on developing and commercializing innovative immuno-oncology therapies and precision medicines to address the unmet needs in cancer treatment in China and globally, and drive its "Pipeline 2.0" strategy to accelerate its transition towards a commercial-stage biopharmaceutical company with a unique and efficient R&D platform. Moreover, the Company plans to submit NDAs for approximately ten indications for four products in the next two to three years.

Dr. Frank Ningjun Jiang, chairman, executive director and chief executive officer of CStone, commented: "China's biopharmaceutical industry is undergoing significant transformation, and CStone is the witness and promoter of such gratifying achievements. With a strategy focused on large cancer indications in China, unmet patient needs and combination therapies, we are currently conducting more than 30 clinical trials, including 13 registrational studies and 14 combination trials. We are very pleased that Ms. Shirley Zhao has joined us as

General Manager for Greater China and Head of Commercial Operations at this critical juncture in CStone's transition from clinical-stage to commercialization. In the next two to three years, Ms. Shirley Zhao will lead the establishment of CStone's commercialization capabilities and the launch of multiple innovative products in the Greater China region. Leveraging our unique and highly efficient early-stage clinical development platform, we will continue our aim to strengthen our pipeline and drive biopharmaceutical innovations in China."

The key updates of the Company include:

- Among CStone's 15 cancer drug candidates, five candidates are in pivotal late-stage clinical development, with a total of 13 on-going registrational trials.

- The Company has implemented its pipeline strategy for early-stage candidates and combination therapy. With an established clinical development platform as its growth engine, CStone is at an inflection point to transition from a research and development ("**R&D**") focused organization into a commercial-stage biopharmaceutical company.

- CStone expects to achieve up to 13 milestones in 2020, including:

    - A New Drug Application ("**NDA**") approval in Taiwan for TIBSOVO® (ivosidenib) for the treatment of IDH1 mutant relapsed or refractory acute myeloid leukemia ("**R/R AML**"), which may become CStone's first commercialized product in the Greater China region.

    - A total of five NDA submissions in Mainland China and Taiwan for CS1001, an anti-PD-L1 monoclonal antibody; pralsetinib, a RET inhibitor; and AYVAKIT™ (avapritinib), a KIT and PDGFRA inhibitor. CStone plans to release top-line data from the seven ongoing clinical trials of these three drug candidates.

    - In the next two to three years, the Company expects to launch four innovative products, targeting multiple indications. CStone will leverage its unique and highly efficient R&D platform to bolster its pipeline and sustain the competitiveness of its portfolio.

Event Highlights

(a) CStone's Achievements:

- Establishment of a strong clinical development engine: CStone has established a clinical development-driven business model and built a world-class clinical team with significant experience in translational medicine and clinical development. In addition, our Scientific Advisory Board comprised of four internationally renowned immuno-oncologists has substantially strengthened the Company's R&D capabilities in immunotherapies and precision medicines, further enhancing and optimizing CStone's R&D strategies and product pipeline. In 2020, the Company is currently conducting more than 30 clinical trials, including 13 registrational studies and 14 combination trials.

- Strong late-stage clinical pipeline: CStone has built a risk-balanced and highly competitive immunotherapy-focused pipeline. Among the Company's 15 cancer drug candidates, five are in pivotal

late-stage clinical development, two among which have received NDA approvals from the Food and Drug Administration of the United States ("**US FDA**").

Among these trials, the clinical studies of CS1001, an anti-PD-L1 monoclonal antibody, have achieved promising clinical results with exceeding one thousand patients treated with CS1001. In 2019, CStone released encouraging data from the trials of CS1001 in esophageal carcinoma and a number of other large cancer indications in China at major medical conferences, including the American Society of Clinical Oncology ("**ASCO**"), the Chinese Society of Clinical Oncology ("**CSCO**") and the American Society of Hematology.

The two in-licensed products of CStone's pipeline candidates that have received NDA approvals from the US FDA are AYVAKIT™ (avapritinib), developed by our partner company, (NASDAQ: BPMC) ("**Blueprint Medicines**"), and TIBSOVO® (ivosidenib), developed by Agios Pharmaceuticals, Inc.. Moreover, Blueprint Medicine has initiated a rolling NDA submission to the US FDA for pralsetinib. All three candidate drugs are being investigated in multiple registrational trials in China.

In December 2019, the global Phase III VOYAGER trial of avapritinib in third-line gastrointestinal stromal tumors ("**GIST**"), and the global Phase I/II ARROW study of pralsetinib in second- and later-line non-small cell lung cancer ("**NSCLC**") have both completed their targeted enrollments of Chinese patients ahead of schedule.

- Completion the strategic layout of early product line: Currently, the Company has completed the strategic layout of the early-stage product line and combination therapies. At present, multiple early-stage pipeline products are progressing smoothly, and the Company has made significant progress with two of its backbone immunotherapy assets, CS1002, an anti-CTLA-4 monoclonal antibody and CS1003, an anti-PD-1 monoclonal antibody, in their respective monotherapies in Phase I studies. In particular, CS1003 has already entered a global Phase III registrational trial. The Company has formulated a comprehensive layout in the development of combination therapies and recently initiated multiple combination trials and will continue to advance these combination studies in 2020.

- Broadening and deepening of early-stage product pipeline to create "CStone 2.0": Leveraging an innovation ecosystem, CStone has built a unique and highly efficient R&D platform and further strengthened its "Pipeline 2.0". The Company will continue to develop first-in-class and best-in-class drugs and therapies to maintain its lead in innovations.

  In 2019, CStone advanced two drug candidates in its pipeline into clinical development, and commenced more than ten preclinical research initiatives.

(b) Major Milestones Expected in 2020:

- One New Drug Application approval: TIBSOVO®, for the treatment of IDH1 mutant R/R AML, is expected to receive an NDA approval in Taiwan, which may become CStone's first commercialized product in the Greater China region.

- Five NDA submissions: CStone plans to submit NDAs targeting five indications in Mainland China and Taiwan for our self-developed CS1001 (an anti-PD-L1 monoclonal antibody) and pralsetinib, and avapritinib, the two partner products with Blueprint Medicines.

- Seven clinical trial data releases: CStone plans to release top-line data from seven clinical trials of CS1001 (an anti-PD-L1 monoclonal antibody), pralsetinib, and avapritinib, six of which are registrational studies.

(c) Our Development Strategies for 2020:

- CStone will integrate resources to focus on the successful delivery of the 13 milestones.

- CStone will build a commercial organization in the Greater China region, equipped with core commercialization capabilities to enable the Company's successful transition to the commercial-stage.

- CStone will continue to seek strategic collaborations with more key international players to drive the future growth of the Company.

- Leveraging its internal R&D, CStone will transition into the "Pipeline 2.0" and drive the sustained growth of the Company. In the next three to five years, CStone will add first-in-class and best-in-class multi-specific monoclonal antibodies and molecular backbone assets to its pipeline. The Company plans to further explore tumor microenvironment modulators, cancer vaccines, new pathway inhibitors, and innovative combination approaches that could potentially bolster the effect of anti-PD-(L)1 antibodies.

(d) New Product Launches:

- In the next two to three years, CStone plans to launch four of its drug candidates, including CS1001 (an anti-PD-L1 monoclonal antibody), avapritinib, pralsetinib and ivosidenib, in the Greater China region, covering a total of approximately ten cancer indications.

(e) Key Messages from KOL Speakers:

- Dr. Paul Bunn, former president of ASCO and distinguished professor at University of Colorado Cancer Center, commented that latest clinical data of pralsetinib, the RET inhibitor, were encouraging. The trial has already completed the patient target enrolment in RET-fusion NSCLC, which included patients who have received or not received platinum-based chemotherapy. Dr. Paul Bunn expects this precision medicine will benefit cancer patients in the future.

- Dr. Eytan Stein, professor at the Memorial Sloan Kettering Cancer Center, commented that compared to traditional standard chemotherapies, ivosidenib could bring significant clinical benefits to AML patients and its high-selectivity IDH1 inhibitor could broaden clinical benefits to cancer patients with an IDH1-mutation.

- Dr. Yilong Wu, president of the Chinese Thoracic Oncology Group, former president of CSCO, tenured professor of Guangdong Provincial People's Hospital and Guangdong Lung Cancer Research Institute, emphasized that CStone has initiated three registrational lung-cancer-related trials and the CStone will play an important role in the lung cancer field in China.

- CS1001 (an anti-PD-L1 monoclonal antibody) is currently being investigated in China in the Phase III GEMSTONE-301 study in patients with locally advanced or unresectable NSCLC, and it could potentially benefit a wider range of patients. Considered the clinical practices in China, the trial also included patients who had received either concomitant or sequential radiochemotherapy. In addition, CS1001 (an anti-PD-L1 monoclonal antibody) is also being investigated in China in a Phase III registrational trial in first-line Stage IV NSCLC.

- The study of pralsetinib in RET-fusion NSCLC has demonstrated promising clinical utility. Due to the persistent high incident rate of lung cancers and the lack of any effective treatment for RET-mutant cancers, pralsetinib has the potential of effectively addressing the current treatment gap in RET-fusion NSCLC in China.

- Dr. Jian Li, professor at Beijing Cancer Hospital and Chairman-elect of the CSCO Committee on GIST, acknowledged avapritinib's therapeutic promise in the treatment of GIST in China. Avapritinib is a first-in-class precision medicine approved by the US FDA for the treatment of GIST, and there is currently no effective treatment for PDGFRA D842V mutant GIST in China. Clinical data available of avapritinib in PDGFRA exon 18 mutant GIST (including D842V mutations) have demonstrated compelling antitumor activity and a more tolerable safety profile. The future delivery in China of this product could significantly benefit GIST patients who are in need of effective treatment options.

- Dr. Weiping Zou, professor at University of Michigan, director of the Center of Excellence for Cancer Immunology and Immunotherapy, and chair of the American Association for Cancer Research's Cancer Immunology Working Group, commented that CStone has made significant progress in the past three years, and he will likely to further collaborate with the Company in selecting Class I drug targets for future development and he is supportive to and believe "Pipeline 2.0" of CStone will bring more value and interest to itself and cancer patients.

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, January 24, 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.