CStone Pharmaceuticals

基石藥業

(Incorporated in the Cayman Islands with limited liability)

(**Stock Code: 2616**)

VOLUNTARY ANNOUNCEMENT

CSTONE RECEIVED APPROVAL TO INITIATE CLINICAL DEVELOPMENT IN CHINA OF CS1001 AND BLU-554 (CS3008) FOR HCC

CStone Pharmaceuticals (the "**Company**" or "**CStone**") announces that it has received approval to initiate clinical development in China of CS1001 in combination with BLU-554 (CS3008) in patients with locally advanced or metastatic hepatocellular carcinoma ("**HCC**"). The trial is a multi-center, open-label, and multi-dose Phase Ib/II study that aims to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamics and anti-tumor efficacy of the combination in advanced HCC.

In 2015, there were approximately 326,000 deaths caused by liver cancer in China, making it the second leading cause of cancer death. HCC accounts for approximately 85 to 90% of all liver cancer cases and is the sixth most common cancer worldwide, with approximately half of the new cases and deaths every year occurring in China. Currently, advanced HCC represents a significant unmet need for both patients and physicians, with limited approved therapies.

CS1001 is a proprietary anti-PD-L1 monoclonal antibody developed by CStone, and one of the Company's three backbone immuno-oncology products. Currently, CS1001 is being examined for the treatment of lung cancer, gastric cancers and other advanced malignancies. In a Phase Ia clinical study, CS1001 was well-tolerated and demonstrated anti-tumor activity with partial responses observed in a number of tumor types.

BLU-554 is a potent and highly selective inhibitor of fibroblast growth factor receptor 4 ("**FGFR4**") discovered by CStone's partner, Blueprint Medicines Corporation (NASDAQ: BPMC) ("**Blueprint**"). In previously presented data from an ongoing Phase I trial for the treatment of advanced HCC patients with

aberrant fibroblast growth factor 19 ("**FGF19**")-FGFR4 pathway activation, BLU-554 monotherapy was generally well-tolerated and demonstrated encouraging anti-tumor activity. The U.S. Food and Drug Administration (the "**FDA**") has granted orphan drug designation to BLU-554 for the treatment of HCC.

In June 2018, CStone entered into an exclusive collaboration and license agreement with Blueprint to develop and commercialize three therapeutic candidates, including BLU-554, in mainland China, Hong Kong, Macau and Taiwan. Blueprint retains development and commercial rights to the three licensed therapeutic candidates in the rest of the world.

Dr. Frank Ningjun Jiang, chairman, executive director and chief executive officer of CStone, commented: "One of CStone's missions is to develop novel therapies to address important unmet needs created by highly prevalent and difficult-to-treat cancers in China. Combination therapy and precision medicine are the core strategies of our pipeline. Through our partnership with Blueprint, we have already initiated a China clinical program with BLU-554 as a monotherapy for HCC earlier this year, which is part of an ongoing global study. We expect the combination of BLU-554 with CS1001 can offer an important additional treatment option for this challenging disease."

Dr. Ngai Chiu Archie Tse, chief translation medical officer of CStone, commented: "Emerging clinical data have shown encouraging results in HCC by combining immunotherapies with targeted therapies that are active as single agents. The combination of CS1001 and BLU-554 represents a great example of such an approach and a potential first-line treatment strategy for advanced HCC with FGF19-FGFR4 activation. We will rapidly advance the clinical development of this program to further explore these two drug candidates with high potential in combination."

About BLU-554

BLU-554 is an orally available, potent and irreversible inhibitor of FGFR4. BLU-554 was specifically designed by Blueprint to inhibit FGFR4 with exquisite selectivity, thereby sparing the paralogs FGFR1, FGFR2 and FGFR3. Blueprint is developing BLU-554, an investigational medicine, for the treatment of patients with HCC caused by abnormal FGF19-FGFR4 signalling. Blueprint estimates that these patients constitute approximately 30% of patients with HCC. The FDA has granted orphan drug designation to BLU-554.

About CS1001

CS1001 is an investigational monoclonal antibody directed against PD-L1 being developed by CStone. Authorized by the U.S.-based company, Ligand Pharmaceuticals Inc. (NASDAQ: LGND), CS1001 is developed by the OMT transgenic animal platform, which can generate fully human antibodies in one step. As a fully human, full-length anti-PD-L1 monoclonal antibody, CS1001 mirrors natural G-type immune globulin 4 (IgG4) human antibody, which can reduce the risk of immunogenicity and potential toxicities in patients, potentially representing a unique advantage over similar drugs.

CS1001 has completed a Phase 1 dose-escalation study in China, in which CS1001 showed good tolerability and produced sustained clinical benefits during the Phase 1a stage of the study.

CS1001 is being investigated in a number of ongoing clinical trials, including one Phase 1 bridging study in the U.S. In China, its clinical program includes one multi-arm Phase 1b study, two pivotal Phase 2 studies, and three Phase 3 studies for several tumor types.

About CStone

CStone is a biopharmaceutical company focused on developing and commercializing innovative immuno-oncology and precision medicine 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 or near 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, June 5, 2019

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.