**CStone Pharmaceuticals**

**基石藥業**

*(Incorporated in the Cayman Islands with limited liability)*

(**Stock Code: 2616**)

## VOLUNTARY ANNOUNCEMENT

# CSTONE ANNOUNCES FIRST PATIENT DOSED IN CHINA FOR GLOBAL PHASE I CLINICAL TRIAL OF FGFR4 INHIBITOR BLU-554 (CS3008)

CStone Pharmaceuticals (the "**Company**" or "**CStone**") today announces that the first patient has been dosed in China for the global Phase I clinical study of BLU-554 (CS3008), a highly potent and selective inhibitor of fibroblast growth factor receptor 4 (FGFR4) discovered by the Company's partner, Blueprint Medicines Corporation (NASDAQ: BPMC) ("**Blueprint**"). The study is part of Blueprint's ongoing global Phase I clinical trial in patients with advanced hepatocellular carcinoma (HCC), and is designed to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamics and preliminary antitumor activity of BLU-554 in advanced HCC patients.

Liver cancer is one of the most common malignant tumors in China, of which HCC accounts for approximately 85 to 90% of all cases. China is burdened with 50% of worldwide liver cancer incidence and mortality, with approximately 370,000 new diagnoses each year. In 2015, there were approximately 326,000 deaths caused by liver cancer, making it China's second leading cause of cancer death. The majority of liver cancer patients are not diagnosed until the advanced stages of the disease and are therefore not eligible for surgery. Currently, treatment options for advanced HCC are extremely limited, presenting a significant unmet need for new therapies.

Based on preclinical studies, an estimated 30% of all HCC patients have aberrantly activated FGFR4 signaling, which is believed to be the driver for HCC in these patients. BLU-554 was specifically designed to inhibit FGFR4 with exquisite selectivity and treat patients with HCC caused by abnormal FGF19-FGFR4 signaling.

In June 2018, the Company 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 licensed therapeutic candidates in the rest of the world.

Dr. Frank Ningjun Jiang, chairman, executive director and chief executive officer of CStone, commented: "Precision therapy is an important part of CStone's pipeline strategy. Through global collaborations, CStone has brought to China a number of targeted-therapy candidates including BLU-554. We will continue to deepen our exploration of BLU-554's potential as a mono therapy and in combination with PD-L1, with the aim of providing HCC patients with more effective treatments."

Dr. Jianxin Yang, CStone's chief medical officer, commented: "The U.S. Food and Drug Administration has already granted orphan drug designation to BLU-554 for the treatment of HCC. Previously presented data from the ongoing global Phase I trial showed that BLU-554 was generally well-tolerated and has the potential to provide clinical benefit to patients with advanced HCC caused by aberrant FGF19-FGFR4 pathway activation. The study now underway in China is part of this ongoing global Phase I trial that we hope will produce positive results in the clinic."

**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 and preventing potential adverse effects. Blueprint is developing BLU-554, an investigational medicine, for the treatment of patients with HCC caused by abnormal FGF19-FGFR4 signaling. Blueprint estimates that these patients constitute approximately 30% of patients with HCC.

**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 with a strategic emphasis on immuno-oncology combination therapies. Currently, 4 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, May 20, 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.*