CANbridge Pharmaceuticals Inc.
北海康成製藥有限公司

(Incorporated in the Cayman Islands with limited liability)

(Stock Code: 1228)

VOLUNTARY ANNOUNCEMENT
UPDATE ON COLLABORATIONS WITH THE UMASS CHAN MEDICAL SCHOOL AND WITH LOGICBIO® THERAPEUTICS

This announcement is made by CANbridge Pharmaceuticals Inc. (the "**Company**", together with its subsidiaries, the "**Group**") on a voluntary basis to inform the shareholders and potential investors of the Company about the latest business updates of the Group.

The board of directors of the Company (the "**Board**") is pleased to inform the shareholders and potential investors of the Company that the Group has published the attached press release, which announced that it has exercised its option to secure the exclusive global rights to develop, manufacture and commercialize a novel second-generation gene therapy to treat spinal muscular atrophy (SMA) from UMass Chan Medical School.

The Company further announced that the Group has completed the full technology transfer of gene therapy products being developed for the treatment of Fabry and Pompe diseases from LogicBio® Therapeutics, Inc. ("**LogicBio**"). As part of such technology transfer, the Group has an exclusive worldwide license to the products in the Fabry and Pompe gene therapy programs that use the adeno-associated virus (AAV) sL65 liver targeting capsid. The Group has also obtained non-exclusive worldwide rights to the LogicBio proprietary manufacturing process for the Fabry and Pompe gene therapies. As part of the Group's agreement with LogicBio, the option rights to sL65-based therapies for two additional indications, as well as to LB-001, an investigational treatment for methylmalonic acidemia, in Greater China, which were granted to the Group under the original strategic collaboration and licensing agreement between the Group and LogicBio, have been removed.

The Group cannot guarantee that the aforesaid second-generation gene therapy to treat SMA and gene therapy products being developed for the treatment of Fabry and Pompe diseases will ultimately be successfully developed and marketed. Shareholders and potential investors of the Company are advised to exercise caution when dealing in the shares of the Company.

By order of the Board
CANbridge Pharmaceuticals Inc.
Dr. James Qun Xue
Chairman

Hong Kong, January 4, 2023

As at the date of this announcement, the board of directors of the Company comprises Dr. James Qun Xue as executive director; Dr. Kan Chen, Dr. Derek Paul Di Rocco and Mr. Edward Hu as non-executive directors; and Mr. James Arthur Geraghty, Dr. Richard James Gregory, Mr. Peng Kuan Chan and Dr. Lan Hu as independent non-executive directors.

CANbridge Consolidates Gene Therapy Portfolio

- Secures Exclusive Global Rights to Potentially Best-in-Class Spinal Muscular Atrophy Gene Therapy from UMass Chan Medical School

- Completes Technology Transfer of Two Gene Therapies for Lysomal Storage Diseases

Beijing, China; Burlington, Mass., January 4, 2023 – **CANbridge Pharmaceuticals Inc.** (1228.HK), a global biopharmaceutical company, with a foundation in China, committed to the research, development and commercialization of transformative therapies to treat rare diseases and oncology, announced that it has exercised its option to secure the exclusive global rights to develop, manufacture and commercialize a novel second-generation gene therapy to treat spinal muscular atrophy (SMA) from UMass Chan Medical School.

Animal data presented in 2022 at the American Society of Gene and Cell Therapy (ASGCT), the European Society of Gene and Cell Therapy (ESGCT) and the World Muscle Congress, showed that the gene therapy outperformed the benchmark therapy along multiple key endpoints in a mouse model of SMA and exhibited much less liver toxicity when administered intravenously. The benchmark therapy uses a vector similar to that used in the only gene therapy approved for SMA. CANbridge sponsored the research and is continuing to evaluate the gene therapy to determine potential additional advantages over the current standard-of-care.

In addition, the company announced that it has completed the full technology transfer of gene therapy products being developed for the treatment of Fabry and Pompe diseases from LogicBio® Therapeutics. Under an amended agreement, CANbridge retains an exclusive worldwide license to the products in the Fabry and Pompe gene therapy programs that use the AAV sL65 liver targeting capsid. CANbridge also obtained non-exclusive worldwide rights to the LogicBio proprietary manufacturing process for Fabry and Pompe gene therapies. In addition, the option rights to sL65-based therapies for two additional indications, as well as to LB-001, an investigational treatment for methylmalonic acidemia, in Greater China, which were granted under the original agreement between CANbridge and LogicBio, are removed from the amended agreement.

“The gene therapy we developed with UMass Chan, and to which we now have global rights in the field, holds promise as a potential best in class therapeutic for SMA,” said James Xue, Ph.D., Founder, Chairman and CEO of CANbridge Pharmaceuticals Inc. “As we also add two additional gene therapy programs, for Fabry and Pompe diseases, to our pipeline at the recently opened CANbridge Next-Generation Innovation and Process Development Facility, in Burlington, Mass, we look forward to developing potential best-in-class gene therapies for these three rare diseases which currently have limited treatment options.”

"The novel *hSMN1* AAV gene therapy vector, consisting of the endogenous SMN1 promoter and codon-optimized *hSMN1*, has a remarkably improved potency and safety profile as compared to the benchmark vector, holding great promise for further clinical development," said Guangping Gao, PhD, the *Penelope Booth Rockwell Professor in Biomedical Research*, professor of microbiology & physiological systems, director of the Horae Gene Therapy Center and co-director of the Li Weibo Institute for Rare Diseases Research. "We feel confident in CANbridge's ability to develop this gene therapy for spinal muscular atrophy, which could help more patients and families suffering from this devastating disease."

About the Second-Generation Gene Therapy

The novel second-generation gene therapy (scAAV9-SMN1p-co-hSMN1) is a self-complementary AAV9 gene therapy expressing a codon-optimized human *SMN1* gene under the control of an endogenous promoter. When compared to a benchmark gene therapy in murine model study of spinal muscular atrophy (SMA) via intravenous administration, the second-generation therapy resulted in a longer lifespan, better restoration of motor function and more complete neuromuscular junction innervation, without the liver toxicity seen with the benchmark vector. The benchmark vector, which is similar to that used in the approved SMA gene therapy, expresses a human SMN1 transgene under a cytomegalovirus enhancer/chicken β-actin promoter for ubiquitous expression in all cell types, whereas the second-generation vector utilizes the endogenous SMN1 promoter to control gene expression in different tissues. Data were presented in 2022 at the American Society of Gene and Cell Therapy, the European Society of Gene and Cell Therapy and The World Muscle Society Congress.

The gene therapy is being developed by CANbridge for the treatment of spinal muscular atrophy. CANbridge holds the exclusive global development, manufacture and commercialization rights.

About Spinal Muscular Atrophy

Spinal muscular atrophy (SMA) is rare genetic disease caused by the lack of a functional motor survival motor neuron 1 (SMN1) gene, which codes for a protein essential to motor neuron survival. The result is a rapid and irreversible loss of motor neurons, resulting in debilitating motor function loss and, in most cases, death. The most common type, SMA1, onsets between birth and six months of age and accounts for 60 percent of the SMA cases, according to Cure SMA. If untreated, SMA1 leads to feeding and ventilation support or death by age two. SMA2 onsets between 6 and 24 months. Patients may be able to sit up by themselves but will be unable to walk. 30 percent die by the age of 25 (Cure SMA). SMA3 and SMA4 are rarer, later onset types of the disease that occur from childhood to early adulthood, and also result in motor function loss and death. SMA affects about 1 out of 6,000 to 10,000 newborns worldwide, according to spinalmuscularatrophy.net.

Approved SMA treatment options are limited. Even with recent approvals, there is still a large unmet medical need.

About CANbridge Pharmaceuticals Inc.

CANbridge Pharmaceuticals Inc. (HKEX:1228) global biopharmaceutical company, with a foundation in China, committed to the research, development and commercialization of transformative therapies for rare disease and rare oncology. CANbridge has a differentiated drug portfolio, with three approved drugs and a pipeline of 10 assets, targeting prevalent rare disease and rare oncology indications that have unmet needs and significant market potential. These include Hunter syndrome and other lysosomal storage disorders, complement-mediated disorders, hemophilia A, metabolic disorders, rare cholestatic liver diseases and neuromuscular diseases, as well as glioblastoma multiforme. The CANbridge Next-Generation Innovation and Process Development Facility is developing novel, potentially curative, gene therapies for rare genetic diseases, including Pompe disease, Fabry disease, spinal muscular atrophy (SMA) and other neuromuscular conditions, and collaborates with world-leading researchers and biotech companies. Animal data from the SMA gene therapy was presented in 2022 at the American Society for Gene and Cell Therapy (ASGCT), the European Society for Gene and Cell Therapy (ESGCT) and the World Muscle Congress. CANbridge global partners include: Apogenix, GC Pharma, Mirum, Wuxi Biologics, Privus, the UMass Chan Medical School, the University of Washington School of Medicine and Scriptr Global.

For more on CANbridge Pharmaceuticals Inc., please go to: www.canbridgepharma.com.

Forward-Looking Statements

The forward-looking statements made in this article relate only to the events or information as of the date on which the statements are made in this article. Except as required by law, we undertake no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise, after the data on which the statements are made or to reflect the occurrence of unanticipated events. You should read this article completely and with the understanding that our actual future results or performance may be materially different from what we expect. In this article, statements of, or references to, our intentions or those of any of our Directors or our Company are made as of the date of this article. Any of these intentions may alter in light of future development.

# # #