Abbisko Cayman Limited
和譽開曼有限責任公司

(Incorporated in the Cayman Islands with limited liability)

(Stock Code: 2256)

VOLUNTARY ANNOUNCEMENT

Abbisko Cayman Limited (the "**Company**", together with its subsidiaries, the "**Group**") hereby informs the shareholders and potential investors of the Company of the attached press release which states that the preliminary Phase Ib results of the CSF-1R inhibitor ABSK021 for advanced tenosynovial giant cell tumor of Abbisko Therapeutics Co., Ltd., a subsidiary of the Company, will be published at the 2022 Connective Tissue Oncology Society annual meeting.

This is a voluntary announcement made by the Company. The Group cannot guarantee that ABSK021 will ultimately be successfully 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
Abbisko Cayman Limited
Dr. Xu Yao-Chang
Chairman

Shanghai, November 15, 2022

As at the date of this announcement, the board of directors of the Company comprises Dr. Xu Yao-Chang, Dr. Yu Hongping and Dr. Chen Zhui as executive directors; Dr. Xia Gavin Guoyao and Ms. Tang Yanmin as non-executive directors; and Dr. Sun Piaoyang, Mr. Sun Hongbin and Mr. Wang Lei as independent non-executive directors.

Preliminary Phase Ib results of ABSK021 for advanced TGCT will be published at the 2022 CTOS annual meeting

15 November 2022, Shanghai – Abbisko Therapeutics Co., Ltd. ("**Abbisko Therapeutics**" hereafter) today announced that preliminary Phase Ib results of its CSF-1R inhibitor ABSK021 for advanced tenosynovial giant cell tumor ("**TGCT**") will be published at the 2022 Connective Tissue Oncology Society ("**CTOS**") annual meeting to be held from November 16 to November 19, 2022 in Vancouver, Canada. The data are in relation to the excellent antitumor efficacy and the safety profile of ABSK021 in the treatment of patients with advanced TGCT and will be published under the title of "*PRELIMINARY PHASE IB RESULTS OF ABSK021 FOR ADVANCED TENOSYNOVIAL GIANT CELL TUMOR: SIGNIFICANT ANTITUMOR ACTIVITY AND FAVOURABLE SAFETY PROFILE*" in a poster presentation with the poster number of "*P 164*".

About ABSK021

ABSK021 is a novel, orally available, highly selective, and highly potent small molecule inhibitor of colony-stimulating factor 1 receptor ("**CSF-1R**"), independently discovered and developed by Abbisko Therapeutics. It is also the first highly selective CSF-1R inhibitor discovered by a Chinese company that has advanced into clinical trial. A number of studies have shown that blocking the colony-stimulating factor 1 ("**CSF-1**")/CSF-1R signaling pathway could effectively modulate and change macrophage functions, and potentially treat many macrophage-dependent human diseases.

Abbisko Therapeutics has completed a Phase Ia dose escalation study for ABSK021 in the U.S., and has a Phase Ib multi-cohort expansion trial ongoing in both the U.S. and China. In July 2022, ABSK021 was granted the breakthrough therapy designation by the Center for Drug Evaluation ("**CDE**") of the National Medical Products Administration of the People's Republic of China ("**NMPA**") for the treatment of TGCT that is not amenable to surgery. In October 2022, ABSK021 was further approved by the CDE of NMPA for a randomized, double-blind, placebo-controlled and multicenter Phase III clinical study in patients with TGCT.

In addition to TGCT, Abbisko Therapeutics is actively exploring the potential of ABSK021 in treating many types of solid tumors in clinic, and has collaborated with Sperogenix (Shanghai) MedTech Co., Ltd. in exploring its potential for treating amyotrophic lateral sclerosis and other central nervous system diseases. As at the date of this press release, no highly selective CSF-1R inhibitor has been approved in China.

About Phase I trial of ABSK021 (NCT04192344)

NCT04192344 is an open-label, first-in-human Phase I study currently ongoing in China and the U.S. for ABSK021.

The trial includes the following two parts, (i) a Phase Ia dose escalation study on patients with advanced solid tumor; and (ii) a Phase Ib dose expansion study to further evaluate the preliminary antitumor efficacy among selected tumor types, including TGCT, and 50mg QD was selected as the current recommended dose for expansion ("**RDE**") for TGCT patients enrolled in Phase Ib trial. For TGCT cohort, the primary endpoint is the objective response rate ("**ORR**") under RECIST 1.1, and the secondary endpoints include overall tolerability, improvement of motion range and patient report outcome of pain and stiffness.

Safety and efficacy data of 27 patients enrolled in the Phase Ib clinical trial have been updated up to September 2022.

Results

1) Conclusion

- ABSK021 has demonstrated significant antitumor efficacy with a preliminary ORR of 68.0% (17/25, 95% CI: 46.50%-85.05%), including one complete response and 16 partial responses confirmed by independent review committee ("**IRC**") within six months.

- The safety profile of ABSK021 was favorable with no apparent hepatotoxicity. The mean treatment duration was 6.8 months, and 85.2% of patients are still on treatment.

- Range of motion, stiffness and pain preliminarily indicated a trend of alleviation after the treatment of ABSK021 in patients with TGCT.

- The changes of PD biomarkers indicated significant CSF-1R inhibition.

- This is the first result of an oral CSF-1R inhibitor with encouraging preliminary efficacy and safety results in Chinese patients with advanced TGCT, which supports the further evaluation of ABSK021 in a Phase III study.

2) Safety

- As of September 2022, the overall median treatment duration was 205 days (n=27, range: 69-280). 26 patients have been on treatment for no less than 3 months. One patient has been on treatment for less than 3 months due to the impact of COVID-19.

- Most treatment-emergent adverse events ("**TEAEs**") were grade 1 or 2 events. No hair color changes or serious liver injury cases were reported.

- CPK and transaminase elevations were asymptomatic and quickly recovered after drug interruptions.

3) Efficacy

- Tumor regression was observed in almost all evaluated patients.

- The ORR was 68.0% (17/25, 95% CI: 46.50%-85.05%), including one complete response and 16 partial responses within six months. The median duration of response was not reached.

- Flexion range of knee at week 13 was assessed in nine patients with an average improvement of 27 degrees (range: 5-58) from baseline along with a similar trend of stiffness and pain alleviation.

- Treatment with ABSK021 led to significant PD changes in TGCT patients, such as an increase in plasma CSF-1 level and a reduction of non-classical monocytes with treatment, showing the inhibitory effect of ABSK021 on the CSF-1R signal pathway.

About TGCT

TGCT is a locally aggressive neoplasm which usually affects synovial joints, mucous sacs, and tendon membranes, resulting in swelling, pain, stiffness, and decreased activity of the affected joints which seriously affect the patient's quality of life[1]. According to the 2013 World Health Organization classification, TGCTs were classified as localized TGCT and diffuse TGCT. Diffuse TGCT encompasses formerly known nodular tenosynovitis and pigmented villonodular synovitis (PVNS). Overexpression of CSF-1 occurs in most TGCTs. Surgical resection is the standard treatment for TGCT. However, not all patients are suitable for surgical treatment. It is difficult to remove tumors of diffuse TGCT patients by surgery, which may possibly lead to severe joint damage, total synovectomy, joint replacement, or even amputation, and the risk of surgical complications can be high. It has been reported that more than 50% of patients with diffuse TGCT will undergo recurrence after surgical resection[2]. For TGCT patients not amenable to surgery, there is currently no approved drug available in China.

About Abbisko Therapeutics

Founded in April 2016, Abbisko Therapeutics Co., Ltd., a subsidiary of Abbisko Cayman Limited (Stock Code: 2256.HK), is an oncology focused biopharmaceutical company founded in Zhangjiang, Shanghai, dedicated to discovering and developing innovative medicines to treat unmet medical needs in China and around the world. The company was established by a group of seasoned drug hunters with rich R&D and managerial expertise from top multinational pharmaceutical companies. Since its founding, Abbisko Therapeutics has built up an extensive pipeline of 15 innovative small molecule programs primarily focused on precision oncology and immuno-oncology, including seven clinical stage assets and eight pre-clinical stage assets. As of today, Abbisko Therapeutics has received 15 IND or clinical trial approvals in four countries and regions.

Please visit www.abbisko.com for more information.

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 date 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.

1. Vaynrub A, Healey JH, Tap W, Vaynrub M. Pexidartinib in the Management of Advanced Tenosynovial Giant Cell Tumor: Focus on Patient Selection and Special Considerations. Onco Targets Ther. 2022;15:53-66.

2. Verspoor FG, van der Geest IC, Vegt E, Veth RP, van der Graaf WT, Schreuder HW. Pigmented villonodular synovitis: current concepts about diagnosis and management. Future Oncol. 2013;9(10):1515-1531.