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SymBio Pharmaceuticals Limited
Fuminori Yoshida
Representative Director
President and Chief Executive Officer
(Securities Code: 4582)

SymBio Granted Patent in Japan for Use of Intravenous Brincidofovir for Treatment of Adenovirus Infection and Infectious Disease

TOKYO, Japan, January 19, 2024 -- SymBio Pharmaceuticals Limited (Headquarters: Tokyo, Japan, "SymBio" or the "Company") is conducting a Phase 2a clinical trial for the use of intravenous brincidofovir (IV BCV) for the treatment of adenovirus (AdV) viremia and disseminated AdV disease in severely immunocompromised patients, including patients who have had hematopoietic stem cell transplantation. SymBio has previously announced that the Company obtained data from this study showing that IV BCV is effective against AdV infection at a certain dosage and administration. SymBio today announced that a use patent that the Company applied for based on the results of this study has been granted by the Japan Patent Office.

The patent has been granted under the accelerated examination system in Japan, and SymBio will submit patent applications for the same invention in Europe, the U.S., and countries in other regions. This is the first use patent that SymBio has been granted. Going forward, as the Company proceeds with non-clinical and clinical studies, SymBio aims to increase the business value of BCV by clarifying BCV's efficacy in multiple therapeutic areas and actively building our patent portfolio.

The outline of the patent is as follows.
Target drug: BCV liquid formulation
Target Indication: Infectious diseases caused by ADV infection.
Dosage and administration:

- A fixed dose of liquid BCV is administered intravenously at regular intervals according to the patient's body weight.
- Administration is continued for a predetermined period of time and terminated according to certain discontinuation criteria.

Statement from Fuminori Yoshida, President and CEO: "We are very pleased to announce that we have been granted a use patent based on Phase 2 data demonstrating the antiviral efficacy of IV BCV as a treatment for AdV infection. As SymBio continues to develop BCV for use in therapeutic areas with high unmet medical needs, we will strive to enhance the value of the BCV business by strategically filing patent applications based on the results of our non-clinical and clinical trials."

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(Note)

About the anti-viral drug Brincidofovir

Brincidofovir (BCV) has a new mechanism of action as a lipid conjugate of cidofovir (CDV). CDV is an antiviral drug already approved and marketed in the United States and the European Union, but unapproved in Japan. BCV is expected to be an effective treatment against a wide spectrum of dsDNA virus infections (cytomegalovirus, adenovirus, Epstein-Barr virus, herpes virus, BK virus, papillomavirus and smallpox virus including monkeypox, etc.), with superior features such as high activity antiviral effect in comparison with CDV and other antiviral drugs.

Due to the breakthrough nature of the BCV molecule, in which a specific length of lipid chain is attached to the CDV, BCV is converted into a molecule that acts directly within the cell, thereby dramatically increasing the efficiency of cellular uptake and showing a high antiviral activity.

In September 2019, SymBio entered into a license agreement with Chimerix for the exclusive worldwide rights to develop, market, and manufacture BCV for all human diseases except orthopoxviruses (such as smallpox and monkeypox).

The tablets and oral suspension (oral formulation) were approved on June 4, 2021, for the treatment of smallpox in adults and pediatric patients, including neonates.

In addition to its high antiviral activity, BCV is also expected to have anti-tumor effects. We are currently conducting collaborative studies with the National Cancer Centre Singapore, the University of California, San Francisco, and other institutions to confirm its anti-cancer activity and to identify synergistic effects when combined with its antiviral activity.

Clinical trials and important R&D collaborations with prominent research institutions include:

- Initiated a Phase II clinical trial in patients with adenovirus infection after hematopoietic stem cell transplantation (March 2021) and received Fast Track designation from the FDA (April 2021). Proof of Concept (POC) of antiviral efficacy established based on data up to cohort 3 (May 2023).
- Initiated a non-clinical trial at the University of California, San Francisco Neurosurgery Brain Tumor Center to evaluate the anti-tumor effect of BCV on refractory brain tumors (September 2021).
- In recent years, large numbers of studies have demonstrated that EBV is a risk factor for MS. SymBio entered into CRADA with the NINDS in March 2023 to establish a new antiviral treatment method for MS, and has been conducting collaborative research to develop a clinical trial.
- CRADA with the National Institute of Allergy and Infectious Diseases (NIAID), affiliated with the NIH, to evaluate the efficacy of BCV for EB virus-associated lymphoproliferative diseases (April 2023).

- Research on the involvement of infection by reactivation of latent viruses in various neurological severity diseases of the brain, including Alzheimer's disease, has been ongoing for the past several years, and a simple three-dimensional mimicry of human neural stem cell cultures and brain tissue established by Tufts University in the United States, the A Sponsored Research Agreement was signed (December 2022) to examine the effect of BCV on HSV infection using a herpes simplex virus (HSV) infection/reactivation model established by Tufts University in the U.S., which uses human neural stem cells cultured to mimic brain tissue in three dimensions.

About SymBio Pharmaceuticals Limited

SymBio Pharmaceuticals Limited was established in March 2005 by Fuminori Yoshida who previously served concurrently as Corporate VP of Amgen Inc. and founding President of Amgen Japan. In May 2016, the Company incorporated its wholly-owned subsidiary in the U.S., SymBio Pharma USA, Inc. (Headquarters: Durham, North Carolina, representative: Stephane Berthier). The Company's underlying corporate mission is to "deliver hope to patients in need" as it aspires to be a leading global specialty biopharmaceutical company dedicated to addressing underserved medical needs.