April 26, 2021
SymBio Pharmaceuticals Limited
Fuminori Yoshida
Representative Director
President and Chief Executive Officer
(Securities Code: 4582)

U.S. Food and Drug Administration granted fast track designation to antiviral agent brincidofovir IV for the treatment of adenovirus infection in pediatric patients

TOKYO, Japan, April 26, 2021 -- SymBio Pharmaceuticals Limited (Headquarters: Tokyo, "SymBio") today announced that it has received a fast track designation from the U.S. Food and Drug Administration (FDA) for brincidofovir intravenous injection (“BCV IV”), an antiviral agent, for the treatment of adenovirus infections ("AdV infection") in pediatric patients.

FDA's fast track designation is a process intended to expedite the review of new drugs that are expected to be effective in treating serious or potentially life-threatening diseases and diseases with high unmet medical needs, with the goal of accelerating the process from development to review. Receiving this designation provides more opportunities for consultation with FDA including clinical trial consultations, and if clinical trials show efficacy and safety results, accelerated approval through priority reviews may be obtained.

SymBio will conduct the multinational Phase 2 clinical trial (Phase 2a) in the U.S. and the U.K. for disseminated AdV infection and AdV infection in immunocompromised patients, an area with high unmet medical need as there currently exists no effective treatment. This study will confirm the appropriate dosage and administration for pediatric patients. The Company plans to conduct a global study to be the next phase for approval applications.

BCV IV's broad antiviral activity against various double-stranded DNA viruses (Note) may be effective in preventing and treating various viral infections in immunocompromised conditions, such as after hematopoietic stem cell transplantation. Based on data on the efficacy and safety gained through the aforementioned study, we aim to expand the target area for multiviral infections after hematopoietic stem cell transplantation (Note) and to maximize the benefit of BCV IV to patients by exploring the possibility of expanding into organ transplantation, including kidney transplantation.

Statement from Mr. Fuminori Yoshida, President and Chief Executive Officer of SymBio: “AdV infection in immunocompromised conditions such as those occurring after hematopoietic stem cell transplantation is an underserved therapeutic area with urgent need for a new treatment.

We will proceed with a multinational clinical study for pediatric patients with AdV infection, an area with extremely high unmet medical need. We expect that the FDA's fast track designation will accelerate the review and our employees will work together to develop BCV IV so that it can be delivered to patients as soon as possible."

The Company does not expect the information presented herein to have any material impact on its financial outlook for the fiscal year ending December 2021.

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*Hematopoietic stem cell transplantation and infection
Hematopoietic stem cell transplantation is a treatment for many hematologic cancers, such as leukemia and malignant lymphoma that cannot be cured by anti-cancer drug treatment or radiation therapy alone. It is used to transplant the hematopoietic stem cells that are donated or stored in advance. Prior to hematopoietic stem cell transplantation, high doses of chemotherapy and total body irradiation can kill cancer cells, and at the same time the patient's own immunity is lost, so as not to reject new stem cells from the donor. Next, hematopoietic stem cell transplantation is performed by infusion. It takes about 2 to 4 weeks for transplanted hematopoietic stem cells to produce blood in the patient's bone marrow. Immune recovery after transplantation takes a long time, but the immune system is severely compromised, especially immediately after transplantation, making the patient susceptible to a variety of infections. If prophylaxis can be given, complications could be potentially reduced, leading to better prognosis. Various infectious diseases after hematopoietic stem cell transplantation are areas for which no effective therapy has been established, and in the medical field, the treatment method which combines effectiveness and safety has been desired for many years.

** Double-stranded DNA (dsDNA) viruses
Double-stranded DNA (dsDNA) viruses includes Herpesviridae (e.g., CMV, HHV-6), adenoviridae (AdVs), polyomaviridae (e.g., BK virus and JC virus), papillomaviridae (HPVs), and poxviridae.

About Anti-viral Drug Brincidofovir
Brincidofovir (BCV) is a lipid-conjugate of cidofovir (an antiviral drug already approved and marketed in the U.S. and Europe, but unapproved in Japan; "CDV"). In addition to its antiviral effect, BCV was intended to be designed to have improved safety profile as compared to CDV. Therefore, BCV is expected to be an effective treatment for a wide range of DNA viruses (e.g., herpesviruses such as cytomegalovirus (CMV), adenovirus (AdV), BK virus, papillomavirus, etc.).

Moreover, BCV is an easy-to-use and novel highly active antimultiviral agent that can reduce the risk of nephrotoxicity, which is a serious side effect of CDV.

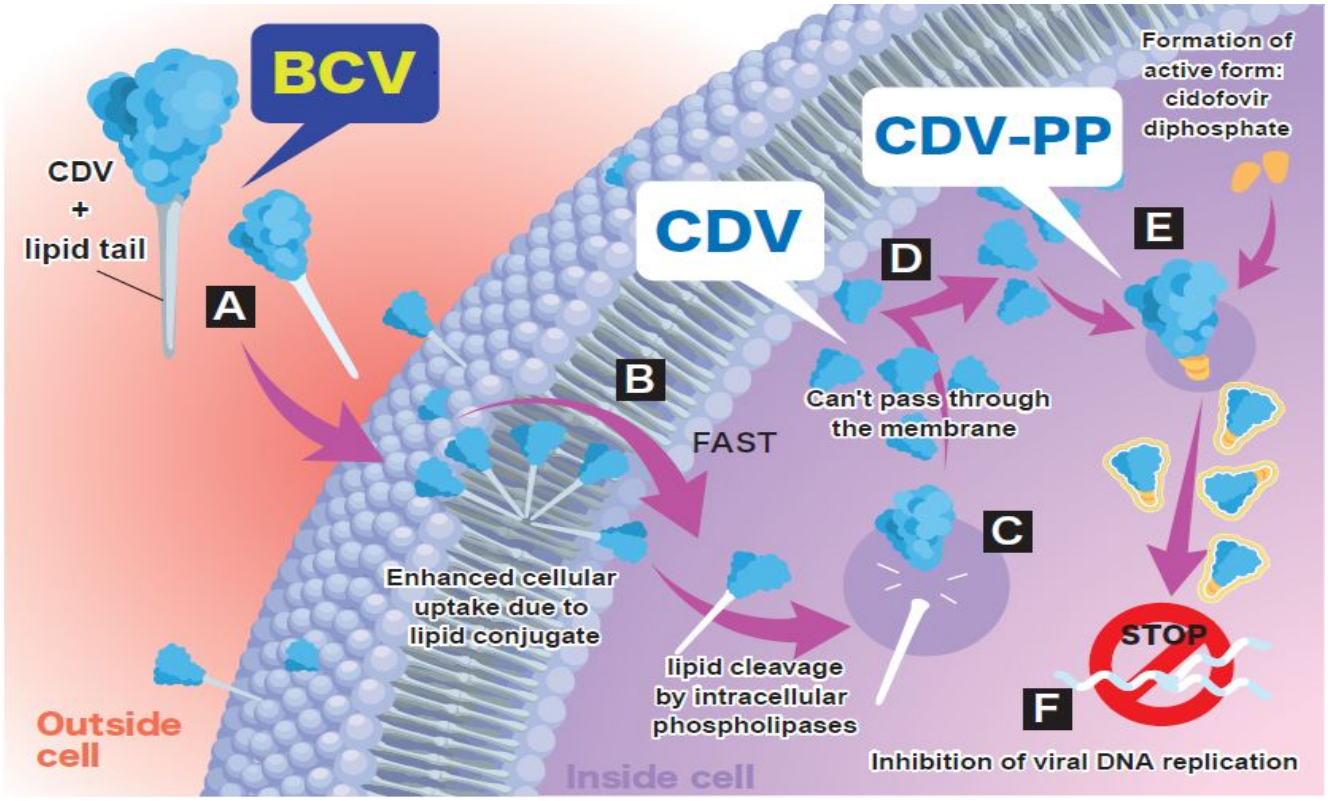
SymBio entered into an exclusive global license agreement with Chimerix Inc. (Headquarters: Durham, NC, "Chimerix") for brincidofovir (BCV) on September 30, 2019. Under the terms of the agreement, Chimerix grants SymBio exclusive worldwide rights to develop, manufacture, and commercialize BCV in all human indications, excluding the prevention and treatment of orthopox infections (which includes smallpox and monkeypox).

SymBio aims to further globalize business, establish an integrated system for the supply of high-quality pharmaceutical products, and grow as a specialty pharmaceutical company through the exclusive global license for BCV. To develop global operations, the Company is considering partnerships that will utilize regional characteristics of the target diseases, and striving to maximize the business value of the products and to deliver to patients who need our products as soon as possible. The Company aims to serve the patients not only in Japan but also in Europe and the U.S. market where the market size for organ transplantation is large, and Asia, including the Chinese market.

For further information, please see the SymBio's press release dated October 1, 2019, https://www.symbiopharma.com/news/20191001.pdf

Innovativeness of Intravenous Brincidofovir (BCV)

Intravenous brincidofovir (BCV) is a lipid conjugate of cidofovir (CDV) with hexadecyloxypropyl (HDP), showing a rapid incorporation to the plasma membrane with enhanced uptake relative to CDV . Once inside target cells, the lipid chain is cleaved by action of intracellular phospholipases releasing CDV, which is then converted to the active form, CDV diphosphate. As a result of increased concentrations of CDV diphosphate into the cells, the antiviral activity of BCV is dramatically improved compared with CDV. Furthermore, BCV can greatly reduce the risk of nephrotoxicity associated with CDV because HDP conjugation greatly reduces plasma exposure to CDV and accumulation of CDV in renal tubular epithelial cells through the transporter (OAT-1).



About Chimerix

Chimerix is a development-stage biopharmaceutical company dedicated to accelerating the advancement of innovative medicines that make a meaningful impact in the lives of patients living with cancer and other serious diseases. Its three most advanced clinical-stage development programs are BCV, ONC201 and DSTAT. BCV is an antiviral drug candidate developed as a potential medical countermeasure for smallpox and is currently under review for regulatory approval in the United States. ONC201 is currently in a registrational clinical program for recurrent H3 K27M-mutant glioma and a confirmatory response rate assessment is expected later this year. DSTAT is in development as a potential first-line therapy in acute myeloid leukemia and as a potential treatment for acute lung injury in hospitalized COVID-19 patients. For further information, please visit the Chimerix website, www.chimerix.com.

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., called SymBio Pharma USA, Inc. (Headquarters: Menlo Park, California, President: Mr. Fuminori Yoshida). 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.