January 18, 2022
SymBio Pharmaceuticals Limited
Fuminori Yoshida
Representative Director
President and Chief Executive Officer
(Securities Code: 4582)

SymBio submits clinical trial application to MHRA for Phase 2 clinical trial of anti-viral drug brincidofovir IV for treatment of pediatric adenovirus infection

TOKYO, Japan, January 18, 2022 -- SymBio Pharmaceuticals Limited (Headquarters: Tokyo, "SymBio" or the "Company") today announced that it has submitted a clinical trial application (CTA) to the Medicines and Healthcare products Regulatory Agency (MHRA) to commence a Phase 2 clinical trial (the "Study") of an intravenous formulation of anti-viral drug brincidofovir ("BCV IV") for treatment of pediatric adenovirus ("AdV") infections in the United Kingdom. The Company initiated the multinational Phase 2 clinical trial in the United States in March 2021.
The Study will evaluate the safety, tolerability, and efficacy of BCV IV for treatment of AdV infection in pediatric patients with disseminated AdV infection or who are immunocompromised[1], for whom there is currently no effective treatment, and will determine the appropriate dosage and administration for the next clinical trial.

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 an urgent need for a new treatment. Having initiated the multinational Phase 2 clinical trial in the United States, we are now starting the trial in the United Kingdom. By initiating the trial in the United Kingdom, we will advance the investigation of BCV IV for treatment of AdV infection and accelerate development for the next phase of multinational trials."

Given brincidofovir's broad antiviral activity in vitro against various dsDNA viruses[2], BCV IV is expected to demonstrate efficacy in the treatment and prevention of viral infections following hematopoietic stem cell transplantation and organ transplantation. The Company is prioritizing development for disseminated AdV infection in pediatric patients, which can be fatal and for which there is high medical need worldwide due to lack of effective treatment. Based on the efficacy and safety findings obtained in this Study, the Company will explore the possibility of expanding the target indications of BCV IV to viral infections occurring after not only hematopoietic stem cell transplantation but also kidney or other organ transplantation, aiming to maximize the benefits to patients and the business value of BCV IV.

The Company does not expect the information presented herein to have any material impact on its financial outlook for the fiscal year ending December 2022.

[Contact]
Investor Relations
Tel: +81 (0)3 5472 1125

(Note 1) Hematopoietic stem cell transplantation and infection
Hematopoietic stem cell transplantation is a treatment for certain hematologic cancers, such as leukemia and malignant lymphoma, which cannot be treated by anti-cancer drug treatment or radiation therapy alone. Prior to hematopoietic stem cell transplantation, high doses of chemotherapy and total body irradiation are used to target cancer cells and at the same time weaken the patient's own immune system in order to reduce the risk of rejection of 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. During the long period of immune-system recovery after transplantation, the patient is susceptible to a variety of infections. An effective prophylaxis against viral infections could potentially reduce infections, leading to better prognosis. Various infections after hematopoietic stem cell transplantation are areas for which no effective therapy has been established, and healthcare providers have long-sought an effective and safe treatment.

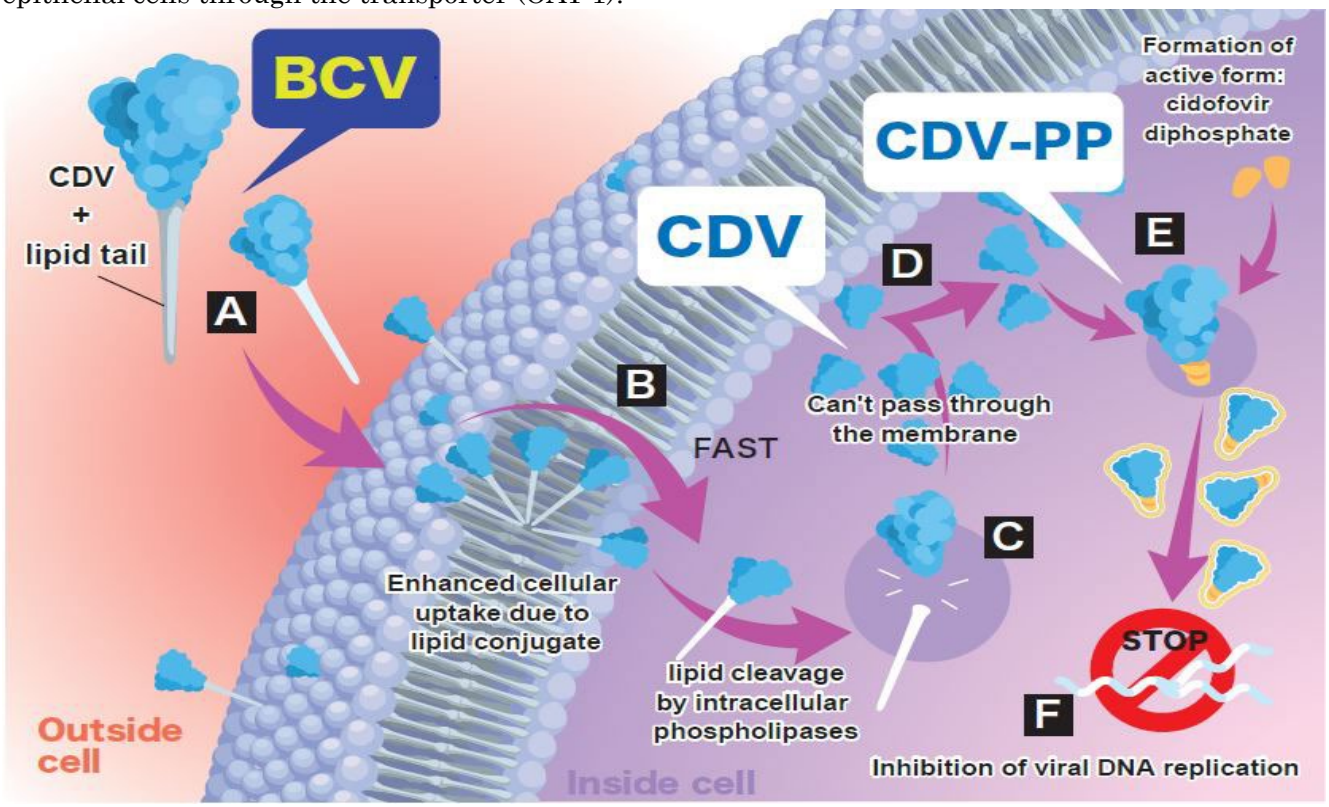
(Note 2) Double-stranded DNA (dsDNA) viruses
Double-stranded DNA (dsDNA) viruses includes Herpesviridae (e.g., CMV, HHV-6), adenoviridae (AdVs), polyomaviridae (e.g., BK virus and JC virus), papillomaviridae (HPVs), and poxviridae.

About anti-viral drug Brincidofovir
SyB V-1901 (Generic Name: Brincidofovir, BCV) is 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. As BCV exhibits not only higher anti-viral activity, but also a superior safety profile in comparison with CDV, BCV is expected to be an effective treatment against a wide spectrum of infectious diseases caused by DNA viruses, including herpes viruses (including CMV), adenovirus, BK virus, papilloma virus, and smallpox virus. Moreover, BCV is an easy-to-use and novel highly active antimultiviral agent with reduced 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 orthopoxvirus 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 Brincidofovir (BCV)

Brincidofovir (BCV) is a lipid conjugate of cidofovir (CDV) with hexadecyloxypropyl (HDP), showing a rapid incorporation to the plasma membrane with efficient cellular uptake due to the lipid conjugate. 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 enhanced uptake 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. In June 2021, the U. S. Food and Drug Administration granted approval of TEMBEXA® for the treatment of smallpox as a medical countermeasure. Chimerix has two other advanced clinical-stage development programs, ONC201 and dociparstat sodium (DSTAT). ONC201 is currently in a registrational clinical program for recurrent H3 K27M-mutant glioma and blinded independent central review is expected later in 2021. DSTAT is in development as a potential first-line therapy in acute myeloid leukemia. 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, SymBio incorporated its wholly-owned subsidiary in the U.S., SymBio Pharma USA, Inc. (Headquarters: Durham, North Carolina, President: Fuminori Yoshida). SymBio'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 address underserved medical needs.