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

SymBio Submits IND Application for Phase 2 Clinical Trial of Anti-viral Drug Brincidofovir IV for Treatment of Pediatric Adenovirus Infection

TOKYO, Japan, March 11, 2021 -- SymBio Pharmaceuticals Limited (Headquarters: Tokyo, "SymBio") today announced that it has submitted an Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA) 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 infections ("AdV infection").

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 including patients who have had post hematopoietic stem cell transplantation*, an area with high unmet medical need as there currently exists no effective treatment. At this time, the Company has filed an IND application with the U.S. FDA. The Company will leverage BCV's efficacy and safety data gained through the clinical trials to potentially expand target indications to multi-viral infections occurring after hematopoietic stem cell transplantation or viral infections after kidney or other organ transplantation, aiming to expand the market and maximize the business value of BCV IV.

Since acquiring the exclusive non-orthopox virus rights to BCV from Chimerix in September 2019, the Company has actively exchanged ideas with experts in the area of infectious diseases, both in and outside Japan, to formulate the global development plan for BCV. Given the extensive in vitro antiviral effects of BCV against various dsDNA viruses**, we expect BCV IV to demonstrate efficacy in the treatment and prevention of various viral infections occurring after hematopoietic stem cell transplantation. The Company will prioritize early development for disseminated AdV infection which can be fatal without effective treatment and for which there is extremely high medical need worldwide due to lack of effective treatment. SymBio anticipates the IV formulation of BCV will build on the clinical development already performed with the oral formulation and may improve the efficacy and safety observed against various infectious diseases such as AdV and cytomegalovirus (CMV).

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, and gradually expand target indications to other viral infectious diseases. With the initiation of this study, SymBio begins its transformation into a global business."

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.

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

*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 "blank therapeutic 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

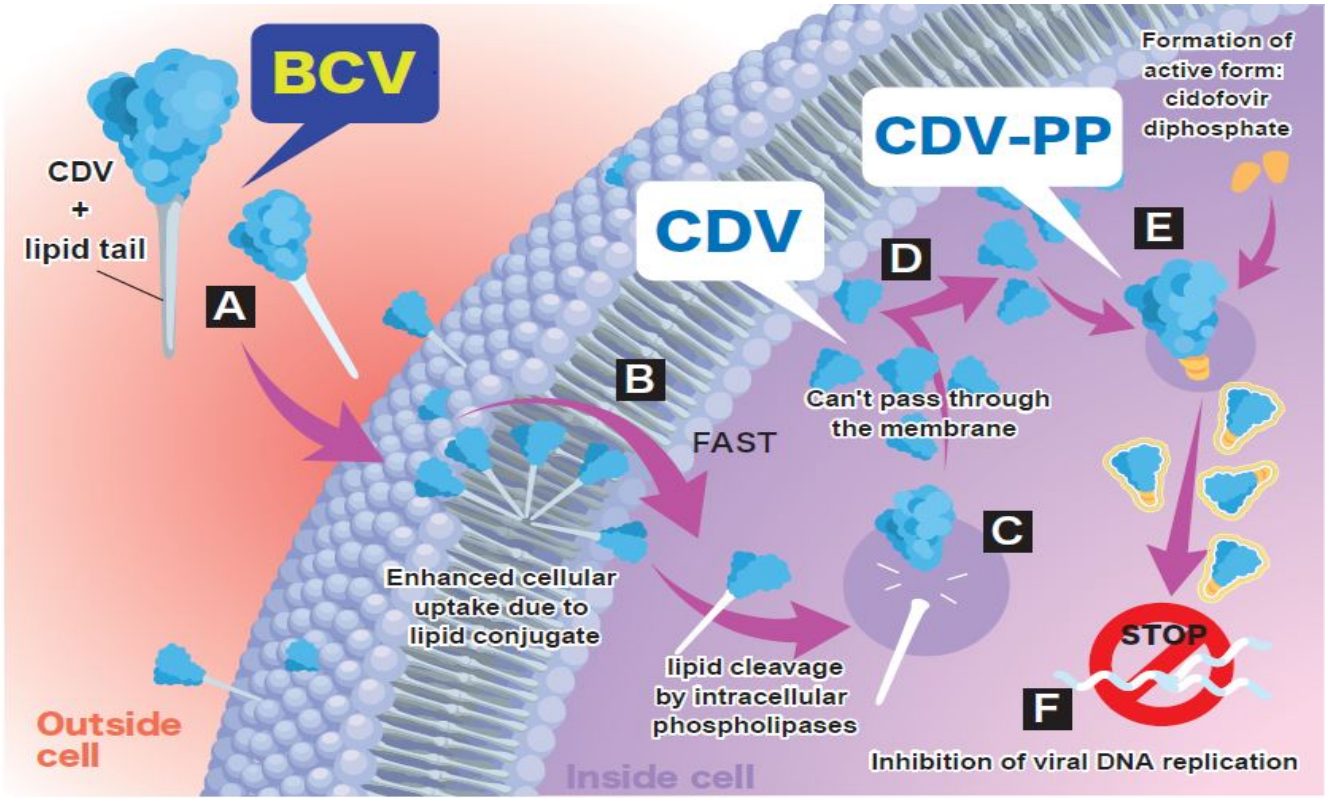
Brincidofovir (BCV) is a lipid-conjugate of cidofovir (an antiviral drug already approved and marketed in the U.S. and Europe, but unapproved in Japan; "CDV"). In addition to its antiviral effect, BCV was intended to be designed to have improved safety profile as compared to CDV. Therefore, BCV is expected 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 the 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. 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.