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SymBio Pharmaceuticals Limited
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Representative Director
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(Securities Code: 4582)

SymBio submits clinical trial notification to PMDA for Phase 2 clinical trial of anti-viral drug brincidofovir IV for patients with BK virus infection after renal transplantation

TOKYO, Japan, June 14, 2022 -- SymBio Pharmaceuticals Limited (Headquarters: Tokyo, "the Company") today announced that it has submitted a clinical trial notification (CTN) to the Pharmaceuticals and Medical Devices Agency (PMDA) to commence an international phase 2 clinical trial (the "Study") of an intravenous formulation of its anti-viral drug brincidofovir ("BCV IV") for patients with BK virus infection after renal transplantation in Japan.

The Study will evaluate the safety, tolerability, and efficacy of BCV IV in patients with BK virus infection after renal transplantation[1], for whom there is currently no effective treatment, and will determine the appropriate dosage and administration for the subsequent clinical trial.

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 BK virus infection in patients after renal transplantation, which can be fatal due to decreased renal function and loss of the transplanted kidney (graft loss), and for which there is a high level of unmet medical need worldwide due to lack of effective treatment. For this international clinical trial, the Company is preparing clinical trials for BK virus infection after renal transplantation in Australia and other regions as well as in Japan.

The Company does not expect the information presented herein to have any material impact on its financial outlook for the fiscal year ended December 31, 2022.

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(Note 1) Renal transplantation and infection
Renal transplantation is a procedure in which a new kidney is surgically transplanted to restore kidney function for patients suffer from decreased renal function. However, the immune system of the transplant recipient may recognize the transplanted organ as a foreign body and attempt to destroy it (organ rejection). Fever, malaise, irritation of the peritoneum, and pain in the wound may cause damage to the transplanted kidney and may destroy the transplanted organ within approximately a week. Immunosuppressants should be used before surgery to reduce organ rejection and protect the transplanted kidney.
Immune recovery after transplantation takes a long time. However, because immunity is severely compromised, especially immediately after transplantation, the transplant recipient becomes susceptible to various infectious diseases against which it is important to take effective measures early. The prognosis of transplanted kidneys with BK virus nephropathy is poor, and about half are said to move toward loss of the transplanted kidney (graft loss).
There is no established effective therapy for the various infections after renal transplantation, 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 the Anti-viral Drug Brincidofovir

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 dsDNA viruses such as herpesviruses such as cytomegalovirus (CMV), adenovirus (AdV), Epstein-Barr virus (EBV) and BK virus, papillomavirus. 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). 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.

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, 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.