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SymBio Pharmaceuticals Limited
Fuminori Yoshida
Representative Director
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(Securities Code: 4582)

SymBio enters into Material Transfer Agreement with Penn State College of Medicine for a non-clinical study to evaluate the efficacy of brincidofovir in a polyomavirus model

TOKYO, Japan, November 24, 2022 -- SymBio Pharmaceuticals Limited (Headquarters: Tokyo, "SymBio" or the "Company") today announced that the Company entered into Material Transfer Agreement (MTA) with Penn State College of Medicine whereby SymBio will provide intravenous formulation of the anti-viral drug brincidofovir*1 ("BCV") for use in a non-clinical study ("the Study") to evaluate the efficacy of BCV in a mouse model of polyomavirus infection*2.

Among double-stranded DNA viruses*3, polyomaviruses are known to cause serious diseases through their infection. As existing antiviral drugs show little efficacy, the development of an effective treatment is eagerly awaited. BCV has broad-spectrum antiviral activity against double-stranded DNA (dsDNA) viruses including polyomaviruses. The Study will evaluate the potential use of BCV in a polyomavirus infection model uniquely established at Penn State College of Medicine.
SymBio is currently conducting a global Phase II clinical trial in patients with adenovirus infection in immunocompromised conditions including hematopoietic stem cell transplantation and BK virus infection after renal transplantation.

Statement from Prof. Aron Lukacher (Professor and Chair, Department of Microbiology and Immunology, Penn State College of Medicine), who leads the Study: "We are hopeful that the findings from this Study will provide evidence for the development of new treatments for polyomavirus infection, for which there is still no fundamental cure."

Statement from Mr. Fuminori Yoshida, President and CEO of SymBio: "No drug has ever been approved for serious diseases caused by the polyomavirus, and effective drugs are highly sought after. We will evaluate the potential of BCV, which is arguably the only known anti-viral drug with anti-polyomavirus activity, and pursue new treatment methods that can be offered in the therapeutic area where no effective treatment has been established."

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

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(Note1) 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 characteristics profile in comparison with CDV and other antiviral drugs, 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), polyomaviruses and papillomavirus. Moreover, BCV is expected to be a highly active antimultiviral agent that can reduce the risk of nephrotoxicity or myelosuppression, which are serious side effects of other antiviral drugs including 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).

The Company aims to expand its business to become a global specialty pharmaceutical company by 2030. To maximize business value, the Company is currently conducting a global Phase II clinical trial mainly in the U.S. for patients with adenovirus infection in immunocompromised conditions including after hematopoietic stem cell transplantation, and initiated a global Phase II clinical trial mainly in Australia, Japan, and South Korea for patients with BK virus nephropathy after kidney transplantation in June 2022.

In addition to its high antiviral activity, BCV is also expected to have anti-tumor effects, and we are currently conducting joint research with the National Cancer Center of Singapore, the University of California, San Francisco, and Brown University in the U.S. to confirm BCV's anti-cancer activity and synergistic effects when combined with its antiviral activity. Furthermore, the Company has initiated a study to evaluate the potential antiviral activity of BCV against EBV in collaboration with the National Institute of Neurological Disorders and Stroke (NINDS) of the National Institutes of Health (NIH) in the U.S.

In September 2022, Chimerix announced the completion of the transfer of its BCV rights to Emergent BioSolutions Inc. (Headquarters: Maryland, U.S.A.). SymBio's exclusive worldwide license for developing, manufacturing, and marketing BCV for all indications, except for orthopoxvirus diseases including smallpox and monkeypox, is not affected.

(Note 2) Polyomavirus infection model

Normally, polyomavirus infections such as BK virus and JC viruses are asymptomatic and persist mainly in the genitourinary system, central nervous system, and hematopoietic cells. However, when the body's immune system is compromised for some reason, these viruses are reactivated and manifest as severe infections in the respective tissues. There are virtually no antiviral drugs against polyomaviruses, and there are only a few preclinical models of these viruses that infect only humans. The model established in Prof. Lukacher's lab represents polyomavirus infection in mice using murine polyomavirus which exhibits a genome structure and mode of infection similar to that of the above viruses. This test system allows us to examine the involvement of immune mechanisms as well as infection in the kidney and central nervous system.

https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7108847/#B24

(Note 3) Double-stranded DNA (dsDNA) viruses

Cytomegalovirus (CMV), adenovirus (AdV), human herpes virus type 6 (HHV-6), herpes simplex virus - type 1 or 2 (HSV-1/2), varicella zoster virus (VZV), BK virus (BKV), JC virus human papillomavirus (HPV), , smallpox virus, and others, included in Herpesviridae, Adenoviridae, Polyomaviridae, Papillomaviridae, and Poxviridae.

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: Carolyn Yanavich).

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.