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

SymBio Announces Positive Preliminary Data from Ongoing Phase 2a Study of Intravenous Brincidofovir in Immunocompromised Patients With Adenovirus Infection in an Oral Presentation at the 65th American Society of Hematology (ASH) Annual Meeting and Exposition

- Adenovirus viremia clearance from plasma was demonstrated in 100% of patients treated with intravenous brincidofovir at 0.4 mg/kg* twice weekly.
- In 90% of those patients, viral clearance was achieved in within 4 weeks of treatment.
- No serious treatment-related adverse events (TRAEs) including gastrointestinal and hepatic toxicities were observed, and all TRAEs were reversible and resolved after the completion of the treatment.

TOKYO, Japan, November XX, 2023 -- SymBio Pharmaceuticals Limited (Headquarters: Tokyo, "SymBio" or the "Company") today announced that preliminary data from Phase 2a study of intravenous brincidofovir (IV BCV) in immunocompromised patients with adenovirus (AdV) infection has been accepted as an oral presentation at the 65th American Society of Hematology (ASH) Annual Meeting and Exposition, December 9-12, 2023, in San Diego, California.

Brincidofovir (BCV) is broad spectrum antiviral agent that has demonstrated the greatest in vitro potency and broad-spectrum activity against opportunistic double-stranded DNA viruses, including adenoviruses. Oral BCV has been approved by the FDA as a countermeasure against smallpox with evidence of gastrointestinal toxicity. The intravenous preparation devoid of this toxicity to date is being developed by SymBio company for multiple indications. AdV infections occurs in approximately 30% of pediatric recipients of hematopoietic cell transplant (HCT) and 6% of adult HCT recipients. Disseminated AdV disease is associated with an approximate mortality rate of 26%, especially when it is associated with pneumonia.

In a dose ascending Phase 2a study, a total of 27 immunocompromise patients with AdV viremia, including recipients of allogeneic hematopoietic cell transplant, were treated in 3 cohorts with IV BCV twice weekly. Treatment with IV BCV at 0.4 mg/kg* demonstrated potent antiviral activity with AdV viremia clearance in 100% of 10 patients treated. Among 90% of those patients, AdV viremia clearance was achieved within 4 weeks of treatment. IV BCV was highly effective in AdV viremia clearance in a dose-dependent manner. Among all 27 patients treated with IV BCV twice weekly across all cohorts, TRAEs

were observed in 7 patients and serious TRAEs including gastrointestinal and hepatic toxicities described with the oral BCV formulation were not observed. All TRAEs were reversible and resolved after the completion of the treatment. Adverse event-related discontinuations were observed in 6 of 27 patients across cohorts, including one of 10 patients treated with 0.4 mg/kg IV BCV.

In view of promising results and in the absence of any other approved treatments for AdV infection, results support the need for further exploration and additional clinical trials of IV BCV as a treatment for AdV infections.

"*We are excited to share the preliminary data from our Phase 2a study of brincidofovir for the treatment of adenovirus infections, which continue to support the transformational potential of brincidofovir. Based on preliminary data from this study, SymBio will be exploring to initiate a phase 3 study to support the registration of brincidofovir in a highly unmet medical need. We are very pleased with the enthusiasm we are seeing from leading transplant centers around the world"* said Fuminori Yoshida, CEO, SymBio Pharmaceutical Limited

"We are pleased to have the opportunity to present this encouraging interim data from this ongoing trial as we work to bring a much needed treatment to these patients with limited treatment options," Says Nkechi Azie MD, the global chief medical officer of SymBio. "We are proud of our continued clinical progress as we remain committed to bringing this innovative, broad spectrum potent antiviral to market as a potential option for patients with difficult to treat life threatening viral infections.

Details of the presentation at ASH are as follows:

Title: Preliminary Results of a Phase 2a Clinical Trial to Evaluate Safety, Tolerability and Antiviral Activity of Intravenous Brincidofovir in Immunocompromised Patients with Adenovirus Infection (Abstract #112)

Session Name: 721. Allogeneic Transplantation: Conditioning Regimens, Engraftment and Acute Toxicities: Improving Outcomes by Reducing Transplant-Related Complications

Session Date: Saturday, December 9, 2023

Session Time: 9:30 AM - 11:00 AM

Presentation Time: 10:15 AM

Room: San Diego Convention Center, Room 11

*: or 20 mg for patients weighing ≥50 kg

[Contact]

Investor Relations

Tel: +81 (0)3 5472 1125

(Note)

About the anti-viral drug Brincidofovir

Brincidofovir (BCV) has a new mechanism of action as 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. BCV is expected to be an effective treatment against a wide spectrum of dsDNA virus infections (cytomegalovirus, adenovirus, Epstein-Barr virus, herpes virus, BK virus, papillomavirus and smallpox virus including monkeypox, etc.), with superior features such as high activity antiviral effect in comparison with CDV and other antiviral drugs.

Due to the breakthrough nature of the BCV molecule, in which a specific length of lipid chain is attached to the CDV, BCV is converted into a molecule that acts directly within the cell, thereby dramatically increasing the efficiency of cellular uptake and showing a high antiviral activity.

In September 2019, SymBio entered into a license agreement with Chimerix for the exclusive worldwide rights to develop, market, and manufacture BCV for all diseases except orthopoxviruses (such as smallpox and monkeypox).

The tablets and oral suspension (oral formulation) were approved on June 4, 2021, for the treatment of smallpox in adults and pediatric patients, including neonates.

In addition to its high antiviral activity, BCV is also expected to have anti-tumor effects. We are currently conducting collaborative studies with the National Cancer Centre Singapore, the University of California, San Francisco, and other institutions to confirm its anti-cancer activity and to identify synergistic effects when combined with its antiviral activity.

Clinical trials and important R&D collaborations with prominent research institutions include:

- Initiated a Phase II clinical trial in patients with adenovirus infection after hematopoietic stem cell transplantation (March 2021) and received Fast Track designation from the FDA (April 2021). Proof of Concept (POC) of antiviral efficacy established based on data up to cohort 3 (May 2023).
- Initiated a non-clinical trial at the University of California, San Francisco Neurosurgery Brain Tumor Center to evaluate the anti-tumor effect of BCV on refractory brain tumors (September 2021).
- In recent years, large numbers of studies have demonstrated that EBV is a risk factor for MS. SymBio entered into CRADA with the NINDS in March 2023 to establish a new antiviral treatment method for MS, and has been conducting collaborative research to develop a clinical trial.
- CRADA with the National Institute of Allergy and Infectious Diseases (NIAID), affiliated with the NIH, to evaluate the efficacy of BCV for EB virus-associated lymphoproliferative diseases (April 2023).
- Research on the involvement of infection by reactivation of latent viruses in various neurological severity diseases of the brain, including Alzheimer's disease, has been ongoing for the past several years, and a simple three-dimensional mimicry of human neural stem cell cultures and brain tissue established by Tufts University in the United States, the A Sponsored Research Agreement was signed (December 2022) to examine the effect of BCV on HSV infection using a herpes simplex virus (HSV)

infection/reactivation model established by Tufts University in the U.S., which uses human neural stem cells cultured to mimic brain tissue in three dimensions.

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: Durham, North Carolina, representative: Stephane Berthier). 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.