SymBio Pharmaceuticals Limited

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

IV Brincidofovir for Treatment of Adenovirus Infection after HSCT Receives Orphan Drug Designation from Japan's Ministry of Health, Labour and Welfare

Tokyo, Japan, September 30, 2025 - SymBio Pharmaceuticals Limited (hereinafter "SymBio" or the "Company") today announced that intravenous brincidofovir (IV BCV) was designated as an orphan drug by Japan's Ministry of Health, Labour and Welfare (MHLW) on September 29, 2025, for the intended indication of adenovirus infection in recipients of organ transplant (including hematopoietic stem cell transplantation).

This infection is a rare disease that can affect people of any age, from children to adults, and can be fatal. As there are no established prevention or treatment options and the medical need is high, the development of an effective therapy is urgently needed.

IV BCV demonstrated strong antiviral activity and established proof of concept (POC) in the Company's Phase 2 clinical trial conducted in the United States. Based on these results, we have filed a clinical trial application with the European Medicines Agency as a first step toward initiating a global Phase 3 clinical trial in patients with this disease, and we are preparing to begin clinical trials in Japan, the United States, and the United Kingdom.*

With this orphan drug designation, IV BCV becomes eligible for a range of support measures, including R&D grants and tax credits, reduced fees for regulatory consultation and marketing-authorization review, preferential pricing following approval, and an extension of the re-examination period of up to 10 years. These measures are expected to accelerate clinical development and improve post-approval commercial viability.

Fuminori Yoshida, President and CEO, commented: “Following the receipt of orphan designation in Europe, this orphan drug designation in Japan is an important step toward delivering treatment options globally to address this serious unmet medical need. By steadily advancing the international joint study, we will strive to benefit patients around the world as quickly as possible.”

The Company does not expect the information presented herein to have any material impact on its financial outlook for the fiscal year ending December 2025.

* For further details, please see the Company’s press release dated June 30, 2025

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(Note)

About Intravenous Brincidofovir (IV BCV)

IV BCV is a lipid conjugate of cidofovir (CDV), which is an antiviral drug approved and marketed in the United States but not yet approved in Japan. With a novel mechanism of action, BCV is expected to be an effective therapeutic agent against a broad range of double-stranded DNA (dsDNA) virus infections, including herpesviruses such as cytomegalovirus (CMV) and Epstein-Barr virus (EBV), adenovirus, BK virus, papillomavirus, and orthopoxviruses such as mpox and smallpox. BCV is not associated with the nephrotoxicity or myelosuppression that are significant side effects of other antiviral drugs, including CDV. Furthermore, IV BCV has shown a significantly improved gastrointestinal (GI) toxicity profile compared to the oral formulation.

BCV's Business Strategy Based on Three Therapeutic Pillars

BCV's Business Strategy Based on Three Therapeutic PillarsSince acquiring the global license to BCV in September 2019, SymBio has been conducting collaborative research with world-class research institutions to unlock BCV's potential in three therapeutic areas. Currently, our management resources and development efforts are centered on three therapeutic pillars: (1) viral infections postHSCT, (2) hematologic and solid tumors, and (3) neurodegenerative diseases. By expanding our business globally, we aim to maximize the business value of BCV. For the first pillar, we are preparing to initiate a global Phase 3 clinical trial for adenovirus infection post-HSCT, with a target start date by the end of the year. For the second pillar, in the area of hematologic cancers, we have initiated a Phase 1b/2 clinical trial for NK/T-cell lymphoma, are currently enrolling patients, and aim to submit for regulatory approval in 2028. Furthermore, within solid tumors, we are considering the initiation of clinical trials for indications such as brain tumors and head and neck cancer. In the field of neurodegenerative diseases, we plan to advance clinical trials targeting multiple sclerosis (MS) and progressive multifocal leukoencephalopathy (PML) in the future.