September 17, 2025
SymBio Pharmaceuticals Limited
Fuminori Yoshida
Representative Director
President and Chief Executive Officer
(Securities Code: 4582)

SymBio Secures EMA Approval of the Pediatric Investigation Plan (PIP) for IV Brincidofovir, a Requirement for Initiating the Global Phase 3 Study for Adenovirus Infection following HSCT

Tokyo, Japan, September 17, 2025 - SymBio Pharmaceuticals Limited (hereinafter "SymBio") today announced that the European Medicines Agency (EMA) has approved its Pediatric Investigation Plan (PIP) for intravenous brincidofovir (IV BCV) which includes the global Phase 3 study for adenovirus infection following hematopoietic stem cell transplantation.

Statement from Nkechi Azie, MD, MBA, FIDSA, Chief Medical Officer of SymBio:
"This PIP approval is critically important as we prepare to initiate a global Phase 3 study for adenovirus infection following hematopoietic stem cell transplantation, a devastating condition with no approved therapies. This infection is particularly common and fatal in pediatric patients."

This approval confirms that the Company's PIP aligns with EMA requirements, thereby establishing the foundation for a future Marketing Authorization Application in Europe. IV BCV has already received Orphan Drug Designation (ODD) from the EMA for this indication. The PIP approval allows for a two-year extension to the standard 10-year market exclusivity period granted by the ODD, resulting in a total of 12 years of market exclusivity.

With the approved PIP now in place, SymBio will advance preparations for the global Phase 3 study, in collaboration with regulators, investigators, and the patient community. A Clinical Trial Application (CTA) for the global Phase 3 study was submitted to the EMA on June 27, 2025.

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.

Notes

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.

About Adenovirus Infection Post-Hematopoietic Stem Cell Transplantation

Adenovirus infection that occurs in immunocompromised patients, such as after a hematopoietic stem cell transplant (HSCT), is a life-threatening complication, particularly for children. The global number of patients was estimated to be approximately 2,700 in 2022 and is projected to increase to over 3,500 by 2030. Furthermore, adenovirus infection is observed in approximately 30% of pediatric patients and 6% of adult patients who have undergone HSCT, with high mortality rates reported. With no approved therapies for adenovirus infection post-HSCT, the development of an effective treatment is highly anticipated to address this significant unmet medical need.

About the Global Phase 3 Trial for IV BCV

The global Phase 3 trial for adenovirus infection post-HSCT will enroll 180 patients at approximately 80 sites across four regions—Europe, the US, Japan, and the UK—and will evaluate the efficacy and safety in both pediatric and adult patients. A CTA was already submitted to the EMA on June 27, 2025. Similar to the Phase 2 trial, the study will enroll patients with adenovirus viremia (the presence of the virus in the blood). SymBio aims to first submit a Marketing Authorization Application in Europe in the second half of 2028, followed by submissions in other regions. For details on the trial design, please refer to our press release dated June 30, 2025.

About the Pediatric Investigation Plan (PIP)

A PIP is a development plan mandated by the EMA to ensure that medicines developed for adults are also appropriately studied for use in children. The PIP includes details such as clinical trials, implementation timelines, and the formulation to be used. Approval of a PIP is a prerequisite for submitting a Marketing Authorization Application to the EMA. Obtaining PIP approval extends the market exclusivity period in Europe by two years, in addition to the 10-year exclusivity from an Orphan Drug Designation, for a total of 12 years.

BCV's Business Strategy Based on Three Therapeutic Pillars

Since 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 post-HSCT, (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.