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SymBio Confirms Brincidofovir (BCV) Efficacy Against Malignant Brain Tumors and Identification of Biomarker Genes (Predictive Factors of Efficacy)

TOKYO, Japan, May 12, 2025 – SymBio Pharmaceuticals Limited (Headquarters: Tokyo, hereinafter "SymBio") has confirmed the anti-tumor effect of BCV in glioblastoma in a pre-clinical study conducted in collaboration with the Brain Tumor Center at UC San Francisco. The results of the study, which has been ongoing since 2021, were presented at the annual meeting of the American Association for Cancer Research[3] held in Chicago, USA, on April 28, 2025. In addition, the results of research on genes that serve as biomarkers to predict the efficacy of BCV in malignant brain tumors and its effectiveness were presented.

Highlights of the presentation included:

- **Confirmation of efficacy against brain tumors:** BCV monotherapy showed dose-dependent efficacy from low concentrations, and the anti-tumor effect of BCV was also confirmed in tumor cells resistant to the current standard therapy temozolomide
- **Biomarker identification:** Two genetic candidate biomarkers that can predict the effectiveness of BCV treatment have been identified, making it possible to predict the therapeutic effect of BCV on a patient-by-patient basis and leading to the expectation of improved outcomes.
- **Confirmation of anti-tumor effect in a mouse model of intracerebroventricular transplantation:** BCV administration inhibited tumor growth and significantly prolonged survival

BCV has the potential to be an effective treatment option for patients who are resistant to the standard therapy, temozolomide. In addition, the identification of genes that serve as candidate biomarkers for predicting the efficacy of BCV is expected to enable pre-selection of patients, improve the BCV treatment outcomes, accelerate clinical development timelines, and improve the cost-effectiveness of therapy. We will continue to confirm the usefulness of the biomarker through clinical trials, with the aim of developing a first-in-class treatment.

The results of this research are a major first step in the clinical development of the second pillar of the BCV business, oncology, especially solid tumors.

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Note

1. Glioma

These are brain tumors that occur in the glia cells of the brain. Of these tumors, the most aggressive, grade 4 glioblastoma, which progresses very rapidly and has a poor prognosis, has a short median survival time of 2 to 18 months after diagnosis, with a 5-year survival rate of less than 5%.

Cancers with a 5-year survival rate of 50% or less are called intractable cancers. Glioblastoma, one such cancer, affects approximately 2,000 people annually in Japan. In Europe and the United States, it is estimated that approximately 24,000 new cases are diagnosed each year.

The current standard of care is primarily temozolomide (Temodar), which is used in combination with radiation therapy. Bevacizumab (Avastin), an angiogenesis inhibitor, is also used. However, patients with mutations in the *MGMT* promoter gene are resistant to standard therapy temozolomide, rendering it ineffective, and there is currently no effective treatment.

2. BCV's development strategy for brain tumors,

SymBio confirmed the anti-tumor effect of BCV on glioblastoma cells that were resistant to the standard therapy, temozolomide. This suggests that BCV could be a highly effective treatment for glioblastoma, regardless of temozolomide resistance. Furthermore, the previously confirmed the high penetration of BCV into the brain coupled with the recent identification of candidate biomarker genes makes the development of a more selective and effective treatment for glioblastoma a reality.

SymBio has three main therapeutic areas of BCV development: post-transplant viral infections, oncology, and neurodegenerative diseases. In oncology, the second pillar of our business, we are currently conducting clinical trials for malignant lymphoma. With the promising results now obtained for glioblastoma, we will aggressively expand our efforts into treatment of other solid tumors.

3. AACR: The American Association for Cancer Research

Presentation Abstract: https://www.abstractsonline.com/pp8/#!/20273/presentation/7551