July 3, 2025

U.S. FDA Orphan Drug Designation Granted to BP2202 for the Treatment of Multiple Myeloma

Tokyo, Japan – July 3, 2025 – BrightPath Biotherapeutics ("BrightPath", TSE Growth 4594), a clinical-stage biopharmaceutical company focused on developing novel cancer immunotherapeutics, today announced that its iPS cell-derived BCMA CAR-NKT cell therapy candidate, BP2202 (the "Drug"), has been granted Orphan Drug Designation (ODD) by the U.S. Food and Drug Administration (FDA) for the treatment of multiple myeloma, as of July 2, 2025 (U.S. EST).

Multiple myeloma is a hematologic malignancy characterized by the abnormal proliferation of plasma cells in the bone marrow, causing bone lesions, anemia, and renal dysfunction. Despite advances in treatment, the disease remains incurable for many patients, experiencing relapse or becoming refractory to existing therapies[1)]. It is estimated that approximately 32,000 new cases of multiple myeloma are diagnosed annually in the U.S.[2)] and over 180,000 cases are reported worldwide[2)]. Currently, more than 100,000 patients in the U.S. are receiving treatment across various lines of therapy[3)]. Although classified as a rare disease, the global market for multiple myeloma therapeutics is projected to reach approximately USD 34.5 billion by 2030[4)].

"We are planning to initiate a clinical trial of BP2202 in the U.S. The ODD granted by the FDA underscores the significant unmet medical need for off-the-shelf, allogeneic CAR-T cell therapies that are readily available when needed for patients with multiple myeloma, " commented Kenichi Nagai, president and CEO of BrightPath. "BP2202 leverages our strategy of using iPSC-derived invariant natural killer T (iNKT) cells, which have the unique ability to engage the patient's endogenous immune system and enhance overall anti-tumor immunity. The designation marks a key milestone in our efforts to validate this approach in upcoming clinical trial in the U.S. Granted as part of our ongoing communications with the FDA in preparation for the clinical trial,, this designation enables ongoing engagement with the FDA through annual reports and other regulatory communications, which we believe will facilitate a smoother development process moving forward. We remain fully committed to delivering this innovative therapy to patients as quickly as possible."

The FDA's ODD program provide multiple benefits for drug development in the U.S., including tax credits for clinical trial costs, exemption from certain application fees, and seven years of market exclusivity following regulatory approval.

This designation is not expected to have any impact on BrightPath's financial results for the fiscal year ending March 31, 2026.

About BrightPath Biotherapeutics

BrightPath is a clinical stage biopharmaceutical company focused on the development of novel cancer therapies to transform cancer treatment for refractory or progressive cancers that cannot be treated with conventional standard therapies. BrightPath is actively involved in developing cell therapies, currently in clinical trials, and immunomodulatory antibodies.

1) Rajkumar, S. V., & Kumar, S. (2020). Multiple myeloma current treatment algorithms. Blood Cancer J, 10 (9), 94.
2) World Health Organization. Globocan 2024 : Multiple Myeloma
3) An Epidemiology Model for Estimating the Numbers of US Patients With Multiple Myeloma by Line of Therapy and Treatment Exposure. Value Health.2022 Dec;25(12) :1977–1985
4) DataM Intelligence. Jan 2025

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