

Sumitomo Pharma America Announces Enzomenib (DSP-5336) Receives FDA Breakthrough Therapy Designation for Treatment of R/R KMT2A-Rearranged Acute Leukemia

- Enzomenib is being evaluated as monotherapy in the registrational Phase 2 Horizen-1 study in relapsed/refractory (R/R) acute leukemia with KMT2A rearrangement or NPM1 mutation
- Enzomenib is also being evaluated in combination with standard of care regimens for patients with newly diagnosed and R/R acute myeloid leukemia in the ongoing Phase 1/2 DSP-5336-101 clinical trial

MARLBOROUGH, Mass.--(BUSINESS WIRE)-- [Sumitomo Pharma America, Inc.](#) (SMPA) today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation to enzomenib (DSP-5336) for the treatment of patients with relapsed/refractory acute leukemia with KMT2A rearrangement (R/R KMT2Ar). Enzomenib is an investigational, oral, small molecule inhibitor of the menin and lysine (K)-specific methyltransferase 2A (KMT2A) protein interaction, a key interaction in acute leukemia and implicated in tumor cell proliferation and growth.

FDA Breakthrough Therapy designation is designed to expedite the development and review of investigational therapies that are intended to treat a serious or life-threatening condition and clinical trial evidence, including unpublished data, indicate the therapy may demonstrate substantial improvement over available therapy on one or more clinically significant endpoints. The designation provides opportunities for enhanced interaction and guidance from the FDA throughout the development program.

“Patients with R/R KMT2A-rearranged acute leukemia continue to face poor outcomes,” said Tsutomu Nakagawa, President and CEO of Sumitomo Pharma America (SMPA). “FDA’s decision to grant Breakthrough Therapy designation underscores enzomenib’s potential and enables expedited FDA review that may potentially provide the opportunity to bring this investigational treatment to patients more quickly. We look forward to continued engagement with the FDA to advance clinical research of enzomenib with the goal of bringing it to patients as efficiently as possible.”

The FDA previously granted Fast Track designation for enzomenib for the treatment of R/R acute myeloid leukemia with KMT2Ar or NPM1m in June 2024. The safety and efficacy of enzomenib is currently being clinically evaluated in a registrational study in patients with R/R KMT2Ar acute leukemia and in patients with R/R NPM1m AML ([NCT04988555](#)), the Horizen-1 study.

R/R KMT2Ar acute leukemia is an aggressive, fast-growing blood cancer that has returned (relapsed) or not responded (refractory) to standard chemotherapy treatments. KMT2Ar acute leukemia is characterized by a rapid onset, aggressive progression, and significantly worse prognosis compared to non-KMT2Ar acute leukemias. Even with contemporary

chemotherapeutic treatments and hematopoietic stem cell transplantations (HSCT), patients with KMT2Ar acute leukemia typically experience poor outcomes and limited responses to these therapies.¹

About Enzomenib (DSP-5336)

Enzomenib is an investigational, oral, small molecule inhibitor of the menin and lysine (K)-specific methyltransferase 2A (KMT2A) protein interaction, a key interaction in acute leukemia and other tumor cell proliferation and growth. Menin is a scaffold nuclear protein that plays key roles in gene expression and protein interactions involved in many biological pathways, including cell growth, cell cycle, genomic stability, and hematopoiesis.^{2,3} In preclinical studies, enzomenib has shown selective growth inhibition in human acute leukemia cell lines with KMT2A rearrangements or NPM1 mutations.^{3,4} Enzomenib reduced the expression of the leukemia-associated genes HOXA9 and MEIS1 and increased the expression of the differentiation gene CD11b in human acute leukemia cell lines with KMT2A rearrangements or NPM1 mutation.^{5,6} The safety and efficacy of enzomenib is currently being clinically evaluated in a Phase 1/2 dose-escalation/dose-expansion study in patients with relapsed or refractory acute leukemia (NCT04988555) and the registrational Phase 2 Horizon-1 R/R mono AML/ALL (KMT2Ar + NPM1m) study. The FDA granted orphan drug designation for enzomenib for the indication of acute myeloid leukemia in June 2022 and the indication of acute lymphoblastic leukemia in July 2026. The FDA granted Fast Track designation for enzomenib for the indication of relapsed or refractory acute myeloid leukemia with KMT2Ar or NPM1m in June 2024. Japan's Ministry of Health, Labour and Welfare (MHLW) granted orphan drug designation for enzomenib for the indication of relapsed or refractory acute myeloid leukemia with KMT2Ar or NPM1m in September 2024.

About Sumitomo Pharma

Sumitomo Pharma Co., Ltd. is a global pharmaceutical company based in Japan with key operations in the U.S. (Sumitomo Pharma America, Inc.) focused on developing and delivering innovative pharmaceutical and regenerative medicines to meet patient needs across oncology, urology, women's health, rare diseases, and CNS. With products in the U.S., Canada, and Europe, and a diverse pipeline of early- to late-stage assets, we aim to accelerate discovery,

¹ Recent Developments and Evolving Therapeutic Strategies in KMT2A-Rearranged Acute Leukemia
<https://pubmed.ncbi.nlm.nih.gov/39428967/>

² Cierpicki T, Grembecka J. Challenges and opportunities in targeting the menin-MLL interaction. *Future Med Chem.* 2014;6(4):447-462. doi:10.4155/fmc.13.214

³ Matkar S, Thiel A, Hua X. Menin: a scaffold protein that controls gene expression and cell signaling. *Trends Biochem Sci.* 2013;38(8):394-402. doi:10.1016/j.tibs.2013.05.005

⁴ Kühn MW, Song E, Feng Z, et al. Targeting Chromatin Regulators Inhibits Leukemogenic Gene Expression in NPM1 Mutant Leukemia. *Cancer Discov.* 2016;6(10):1166-1181. doi:10.1158/2159-8290.CD-16-0237

⁵ Eguchi K, Shimizu T, Kato D, et al. Preclinical Evaluation of a Novel Orally Bioavailable Menin-MLL Interaction Inhibitor, DSP-5336, for the Treatment of Acute Leukemia Patients with MLL-Rearrangement or NPM1 Mutation. *Blood* 2021; 138 (Supplement 1): 3339. Available at: <https://doi.org/10.1182/blood-2021-152050>

⁶ Daver N, Zeidner JF, Yuda J, et al. Phase 1/2 First-in-Human Study of the Menin-MLL Inhibitor DSP-5336 in Patients with Relapsed or Refractory Acute Leukemia. *Blood* 2023; 142 (Supplement 1): 2911. Available at: <https://doi.org/10.1182/blood-2023-179252>



research, and development to bring novel therapies to patients sooner. For more information on SMPA, visit our website <https://www.us.sumitomo-pharma.com> or follow us on [LinkedIn](#).

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