Sumitomo Pharma

Press Release

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Sumitomo Pharma Co., Ltd.

Sumitomo Pharma Presents Latest Clinical Data on Investigational Anti-Cancer Agent Enzomenib (DSP-5336) at the 2025 Annual Meeting of the Japanese Society of Hematology (JSH)

Sumitomo Pharma Co., Ltd. (Head Office: Osaka, Japan; President and CEO: Toru Kimura) announced the presentation of new clinical data on its investigational anti-cancer agent enzomenib (generic name, development code: DSP-5336), a menin-KMT2A protein interaction inhibitor targeting relapsed or refractory acute leukemia, at the 2025 Annual Meeting of the Japanese Society of Hematology (JSH), held in Kobe from October 10 to 12.

Preliminary clinical data from the Phase 1/2 study of enzomenib were previously presented at the 2024 Annual Meeting of the American Society of Hematology (ASH), held in San Diego, USA, December 7 to 10. At JSH2025, newly extracted and analyzed data on the Japanese population were presented alongside the previously disclosed data from the overall population.
The safety analysis set of the study included 84 patients with acute leukemia, of whom 94% (79/84) had acute myeloid leukemia (AML). The study enrolled a diverse patient population, with 47.6% (40/84) being non-Caucasian, including 22 Japanese patients (96% (21/22) of whom had AML).
Enzomenib was administered continuously in 28-day cycles at doses ranging from 40 mg to 300 mg twice daily. Enzomenib was well tolerated in both the overall and Japanese populations, with a low incidence of treatment-related adverse events and no dose-limiting toxicities (DLTs). Differentiation syndrome was reported in 10.7% of the overall population and 13.6% of the Japanese population, but no cases led to death or treatment discontinuation.

Preliminary efficacy data from the dose optimization cohort (200 mg and 300 mg twice daily) were also presented. This included patients with KMT2A rearrangements or nucleophosmin 1 (NPM1) mutations who had received at least one dose of enzomenib and had not previously been treated with menin inhibitors. Among these, the overall population (n=40) showed an objective response rate (ORR) of 62.5% (25/40) and a complete remission plus complete remission with partial hematologic recovery (CR+CRh) rate of 37.5% (15/40). In the Japanese population (n=12), ORR was 75.0% (9/12) and CR+CRh was 41.7% (5/12).

These results suggest that enzomenib demonstrates a favorable safety profile and robust clinical activity in Japanese patients with relapsed or refractory acute leukemia, similar to the overall population. The data support the potential of enzomenib as a key therapeutic option for patients with KMT2A rearrangements or NPM1 mutations.

Leukemia is a type of hematologic malignancy characterized by uncontrolled proliferation of blood

cells, typically white blood cells, in the bone marrow. In acute leukemia, symptoms appear suddenly due to rapid cell growth, necessitating prompt treatment. Approximately 30% of AML patients have NPM1 mutations, and 5–10% have KMT2A rearrangements.

At JSH2025, Sumitomo Pharma also presented clinical data from a Phase 1/2 study of nuvisertib (generic name, development code: TP-3654), a PIM1 kinase inhibitor targeting relapsed or refractory myelofibrosis. These data, previously presented at the 2025 European Hematology Association (EHA) Annual Meeting in Milan, Italy, from June 12 to 15, indicated good tolerability and clinical benefits, including improvements in cytokine profiles and spleen volume correlated with symptom response.

Myelofibrosis is a rare hematologic malignancy characterized by abnormal regulation of the JAK signaling pathway, leading to fibrotic tissue accumulation in the bone marrow and impaired blood cell production. It is a serious and rare disease, with an annual incidence of 0.7 per 100,000 people worldwide.

Sumitomo Pharma remains committed to improving treatment outcomes for patients with limited therapeutic options through the continued development of enzomenib and nuvisertib.

Sumitomo Pharma has issued the following press releases related to this matter.
“Sumitomo Pharma Announces that DSP-5336 Has Received FDA Fast Track Designation for the Treatment of Relapsed or Refractory Acute Myeloid Leukemia”:
https://www.sumitomo-pharma.com/news/assets/pdf/ene20240716.pdf
“Sumitomo Pharma America Presents New Data on Nuvisertib and Enzomenib at the 2024 American Society of Hematology Annual Meeting”:
https://www.sumitomo-pharma.com/news/assets/pdf/ene20241210.pdf
“Sumitomo Pharma America Announces that Nuvisertib (TP3654) Has Received FDA Fast Track Designation for the Treatment of Myelofibrosis”:
https://www.sumitomo-pharma.com/news/assets/pdf/ene20250613.pdf

(Reference)
enzomenib (DSP-5336)
Enzomenib (DSP-5336) is a small molecule inhibitor against the binding of menin and lysine methyltransferase 2A (KMT2A) protein. Acute myeloid leukemia with KMT2A rearrangements or nucleophosmin 1 (NPM1) mutations rely on the menin-KMT2A interaction for upregulation of genes instrumental to leukemogenesis. Enzomenib has been shown to have anti-cancer activity through downregulation of the genes by inhibition of menin-KMT2A interaction in pre-clinical studies. The U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation for enzomenib for the indication of acute myeloid leukemia in June 2022 and granted Fast Track Designation for the treatment of relapsed or refractory acute myeloid leukemia with KMT2A rearrangement or NPM1

mutation in June 2024. Furthermore, the Ministry of Health, Labour and Welfare in Japan granted Orphan Drug Designation for enzomenib for the indication of relapsed or refractory acute myeloid leukemia with KMT2A rearrangement or NPM1 mutation in September 2024.

nuvisertib (TP-3654)

Nuvisertib (TP-3654) inhibits the inflammatory signaling pathways through inhibition of PIM1 (proviral integration site for Moloney murine leukemia virus 1) kinases. PIM1 kinases are frequently overexpressed in various hematologic malignancies and solid tumors, allowing cancer cells to evade apoptosis and promoting tumor growth. Nuvisertib was granted Orphan Drug Designation by the FDA for the indication of myelofibrosis in May 2022, and received Fast Track Designation for the same indication in June 2025. In addition, the Ministry of Health, Labour and Welfare in Japan granted Orphan Drug Designation for nuvisertib for the indication of myelofibrosis in November 2024. Furthermore, in July 2025, nuvisertib was granted Orphan Drug Designation by the European Medicines Agency (EMA) for the indication of myelofibrosis.

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