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Daiichi-Sankyo

Press Release

Daiichi Sankyo's VANFLYTA® Receives Approval in Japan for the Treatment of Relapsed/Refractory *FLT3*-ITD AML

- VANFLYTA® (quizartinib) is a FLT3 inhibitor MHLW-approved based on a survival benefit compared to salvage chemotherapy in adult patients with relapsed/refractory *FLT3*-ITD AML
- Patients with *FLT3*-ITD AML face a very aggressive disease with poor prognosis and VANFLYTA offers an important treatment option that specifically targets *FLT3*-ITD, a driver mutation in AML
- VANFLYTA is the first of seven new molecular entities that Daiichi Sankyo is committed to delivering from its oncology pipeline by 2025

Tokyo, Munich and Basking Ridge, NJ – (June 18, 2019) – Daiichi Sankyo Company, Limited (hereafter, Daiichi Sankyo) announced today that the Ministry of Health, Labor and Welfare (MHLW) of Japan has approved VANFLYTA® (quizartinib), an oral FLT3 inhibitor, for the treatment of adult patients with relapsed/refractory *FLT3*-ITD acute myeloid leukemia (AML), as detected by an MHLW-approved test.

Approval of VANFLYTA in Japan is based on the results from the global pivotal phase 3 QuANTUM-R study and a phase 2 study of VANFLYTA in Japan in patients with relapsed/refractory *FLT3*-ITD AML. Results from QuANTUM-R, which was the first randomized phase 3 study to show that a FLT3 inhibitor prolonged overall survival as an oral, single agent compared to chemotherapy in patients with relapsed/ refractory *FLT3*-ITD AML, were recently published in *The Lancet Oncology*.[1]

"With the approval of VANFLYTA, patients with relapsed/refractory *FLT3*-ITD AML in Japan will now have access to this important new treatment option that specifically targets the underlying driver of disease, and has a proven survival benefit compared to chemotherapy," said Wataru Takasaki, PhD, Corporate Officer, Head of Oncology Function and Head of R&D Division in Japan, Daiichi Sankyo. "We are proud that VANFLYTA is the first of seven new molecular entities we are committed to delivering by 2025 with the goal of transforming science into innovative treatments for patients with cancer."

Results of the global, phase 3 QuANTUM-R study demonstrated a statistically significant improvement in overall survival when comparing VANFLYTA to salvage chemotherapy. The hazard ratio for VANFLYTA was 0.76 [95% CI: 0.58, 0.98], and the median overall survival was 6.2 months [95% CI: 5.3, 7.2] in patients receiving VANFLYTA compared to 4.7 months [4.0, 5.5] salvage chemotherapy. The most common treatment-related adverse drug reactions in those receiving VANFLYTA were nausea (33.2%, 80/241 patients), electrocardiogram QT prolonged (24.9%, 60/241 patients), anemia (24.9%,

60/241 patients), and thrombocytopenia (21.2%, 51/241 patients) in the Japanese labeling. The open-label, single-arm phase 2 study evaluating VANFLYTA in Japanese patients with relapsed/ refractory FLT3-ITD AML met its primary endpoint of achieving a pre-determined composite complete remission rate at interim analysis, triggering an early stop of the study due to efficacy. The efficacy and safety profile of VANFLYTA observed in the phase 2 study in Japan appears consistent with that of QuANTUM-R.

About *FLT3*-ITD AML

AML is an aggressive blood and bone marrow cancer that causes uncontrolled growth and accumulation of malignant white blood cells that fail to function normally and interfere with the production of normal blood cells.[2] AML is the most common adult leukemia in Japan,[3] with approximately 5,500 new cases diagnosed each year. The five-year survival rate of AML reported from 2005 to 2011 was approximately 26 percent, which was the lowest of all leukemias.[2]

FLT3 gene mutations are one of the most common genetic abnormalities in AML.[4] *FLT3*-ITD is the most common FLT3 mutation, affecting approximately one in four patients with AML.[5] *FLT3*-ITD is a driver mutation that presents with high leukemic burden and has poor prognosis and a significant impact on disease management for patients with AML.[6]

Patients with *FLT3*-ITD AML have a worse overall prognosis, including an increased incidence of relapse, an increased risk of death following relapse, and a higher likelihood of relapse following hematopoietic stem cell transplantation, as compared to those without this mutation.[7, 8]

About VANFLYTA

VANFLTYA, an oral FLT3 inhibitor, is the lead product in the AML Franchise of Daiichi Sankyo. It has been granted Breakthrough Therapy designation for the treatment of adult patients with relapsed/ refractory *FLT3*-ITD AML by the FDA; Fast Track designation for the treatment of relapsed/refractory AML by the FDA; and, also has been granted Orphan Drug designation by both the FDA and the European Commission (EC) for the treatment of AML and by the Japan MHLW for the treatment of *FLT3*-mutated AML.

A broad and comprehensive development program is underway including phase 3 development in combination with standard chemotherapy in newly diagnosed *FLT3*-ITD AML (QuANTUM-First) in the U.S., EU and Japan; phase 1/2 development for pediatric and young adult relapsed/refractory *FLT3*-ITD AML in North America and the EU; and phase 1 development in combination with an investigational MDM2 inhibitor, milademetan, for relapsed/refractory *FLT3*-ITD AML and newly-diagnosed *FLT3*-ITD AML unfit for intensive chemotherapy in the U.S.

Milademetan is an investigational agent that has not been approved for any indication in any country. Safety and efficacy have not been established. VANFLYTA currently is only approved for use in Japan.

About Daiichi Sankyo

Daiichi Sankyo Group is dedicated to the creation and supply of innovative pharmaceutical products to address diversified, unmet medical needs of patients in both mature and emerging markets. With over 100 years of scientific expertise and a presence in more than 20 countries, Daiichi Sankyo and its 15,000 employees around the world draw upon a rich legacy of innovation and a robust pipeline of promising new medicines to help people. In addition to a strong portfolio of medicines for hypertension and thrombotic disorders, under the Group's 2025 Vision to become a "Global Pharma Innovator with Competitive Advantage in Oncology." Daiichi Sankyo research and development is primarily focused on bringing forth novel therapies in oncology, including immuno-oncology, with additional focus on new horizon areas, such as pain management, neurodegenerative diseases, heart and kidney diseases, and other rare diseases. For more information, please visit: www.daiichisankyo.com.

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