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Daiichi-Sankyo

Press Release

Daiichi Sankyo Launches FLT3 Inhibitor VANFLYTA® in Japan for the Treatment of Patients with Relapsed/Refractory *FLT3*-ITD AML

- Patients in Japan with relapsed/refractory *FLT3*-ITD AML who face a very aggressive disease with poor prognosis now have access to VANFLYTA® (quizartinib)
- Approval of VANFLYTA in Japan is based on survival benefit compared to salvage chemotherapy in adult patients with relapsed/refractory *FLT3*-ITD AML
- VANFLYTA is the first of seven new molecular entities that Daiichi Sankyo has committed to delivering from its oncology pipeline by 2025

Tokyo, Japan - (October 10, 2019) – Daiichi Sankyo Company, Limited (hereafter, Daiichi Sankyo) announced today the launch of VANFLYTA® (quizartinib), an oral FLT3 inhibitor, in Japan for the treatment of adult patients with relapsed/refractory *FLT3*-ITD acute myeloid leukemia (AML).

Marketing approval of VANFLYTA by Japan's Ministry of Health, Labor and Welfare (MHLW) in June 2019 was based on the results of the global pivotal phase 3 QuANTUM-R study and a phase 2 study of VANFLYTA in Japanese patients with relapsed/refractory *FLT3*-ITD AML. Results of QuANTUM-R were published in *The Lancet Oncology*.[1] Results from the phase 2 study in Japan patients were recently published in the *International Journal of Hematology*.[2]

"We are proud to launch VANFLYTA in Japan making it available to both physicians and patients as an important new therapeutic option with a survival benefit over salvage chemotherapy for the treatment of patients with relapsed/refractory *FLT3*-ITD AML," said Takashi Ikegami, PhD, Vice President, Head of Specialty Marketing in Japan, Daiichi Sankyo. "Now patients have access to a treatment that targets *FLT3*-ITD, a driver mutation in AML linked to poor prognosis and aggressive disease that results in increased relapsed rate and reduced overall survival for patients compared to those without this mutation."

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.[3] AML is the most common adult leukemia in Japan,[4] 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.[3]

FLT3 gene mutations are one of the most common genetic abnormalities in AML.[5] *FLT3*-ITD is the most common FLT3 mutation, affecting approximately one in four patients with AML.[6] *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.[7] 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.[8,9]

About QuANTUM-R and Phase 2 Japan Study

In the QuANTUM-R study, a statistically significant improvement in overall survival was demonstrated 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 VANFLYTA

VANFLTYA, an oral FLT3 inhibitor, is the lead product in the AML Franchise of Daiichi Sankyo. VANFLYTA currently is only approved for use in Japan for the treatment of adult patients with relapsed/refractory *FLT3*-ITD AML, as detected by an MHLW-approved test.

Ongoing studies include QuANTUM-First, a pivotal phase 3 study evaluating VANFLYTA in combination with standard chemotherapy in newly diagnosed *FLT3*-ITD AML in the U.S., Europe and Japan; and phase 1/2 development for pediatric and young adult relapsed/refractory *FLT3*-ITD AML in North America and Europe; and phase 1 development in combination with milademetan, an investigational MDM2 inhibitor, 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.

About Daiichi Sankyo

Daiichi Sankyo Group is dedicated to the creation and supply of innovative pharmaceutical therapies to improve standards of care and address diversified, unmet medical needs of people globally by leveraging our world-class science and technology. With more than 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 cardiovascular diseases, under the Group's 2025 Vision to become a "Global Pharma Innovator with Competitive Advantage in Oncology," Daiichi Sankyo is primarily focused on providing novel therapies in oncology, as well as other research areas centered around rare diseases and immune disorders. For more information, please visit: www.daiichisankyo.com.

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