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Press Release

Daiichi Sankyo Submits Application in Japan for FLT3 Inhibitor Quizartinib for Treatment of Patients with Relapsed/Refractory *FLT3*-ITD AML

- Submission based on results of pivotal phase 3 QuANTUM-R study and open-label phase 2 study in Japan of quizartinib in patients with relapsed/refractory *FLT3*-ITD AML
- Quizartinib is the first FLT3 inhibitor to demonstrate a survival benefit in a randomized phase 3 study in patients with relapsed/refractory *FLT3*-ITD AML
- Significant unmet medical need exists in Japan for AML with limited targeted treatment options for patients with relapsed/refractory *FLT3*-ITD AML, a very aggressive form of the disease associated with poor prognosis
- Application follows recent Orphan Drug designation for quizartinib from the Japan MHLW for *FLT3*-mutated AML
- Submissions in U.S. and EU based on the results of the QuANTUM-R study of quizartinib remain on track for second half of fiscal year 2018

Tokyo, Munich and Basking Ridge, NJ – (October 17, 2018) – Daiichi Sankyo Company, Limited (hereafter, Daiichi Sankyo) announced that it has submitted a New Drug Application (NDA) to Japan's Ministry of Health, Labor and Welfare (MHLW) for marketing approval of quizartinib for the treatment of adult patients with relapsed/refractory *FLT3*-ITD acute myeloid leukemia (AML). The submission to Japan MHLW is based on the results of the pivotal randomized phase 3 QuANTUM-R study in the U.S., EU and Asia excluding Japan, and an open-label phase 2 study of quizartinib in Japan in patients with relapsed/refractory *FLT3*-ITD AML.

"Quizartinib has been designed as a specific inhibitor of FLT3 with high affinity for *FLT3*-ITD, a driver mutation in AML that is linked to poor prognosis and is associated with aggressive disease that results in increased relapse rate and reduced overall survival for patients compared to those without this mutation," said Kouichi Akahane, PhD, MBA, Executive Officer, Head of Oncology Function, R&D Division, Daiichi Sankyo. "We look forward to working closely with the Japan Health Authority on our application for quizartinib in order to bring this important potential new targeted treatment option to patients with relapsed/refractory *FLT3*-ITD AML in Japan."

Quizartinib is the first FLT3 inhibitor to prolong overall survival as an oral, single agent compared to chemotherapy in patients with relapsed/refractory *FLT3*-ITD AML. This was demonstrated in a randomized phase 3 trial (QuANTUM-R) and topline results of QuANTUM-R were presented during the plenary program at the 23rd Congress of the European Hematology Association in June 2018.

The open-label, single arm phase 2 study evaluating quizartinib in Japanese patients with relapsed/refractory *FLT3*-ITD AML met its primary endpoint of achieving a predetermined composite complete remission rate at interim analysis, triggering an early stop of the study due to efficacy. The quizartinib efficacy and safety profile observed in the phase 2 study in Japan appears consistent with that of QuANTUM-R. These data were presented at the 80th Annual Meeting of the Japanese Society of Hematology (JSH) in October 2018.

In the QuANTUM-R study, the median treatment duration with quizartinib was 4 cyles of 28 days versus 1 cycle in the salvage chemotherapy arm. Incidence of treatment-emergent adverse events was comparable between patients who received single agent quizartinib and those who received salvage chemotherapy. The most common adverse events (>30 percent, any Grade) in patients treated with quizartinib included nausea, thrombocytopenia, fatigue, musculoskeletal pain, pyrexia, anemia, neutropenia, febrile neutropenia, vomiting and hypokalemia, and the most common Grade ≥ 3 adverse events (>20 percent) were thrombocytopenia, anemia, neutropenia and febrile neutropenia. The safety profile observed in QuANTUM-R appears consistent with that observed at similar doses in the quizartinib clinical development program.

About *FLT3*-ITD Acute Myeloid Leukemia

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.[1] *FLT3* gene mutations are one of the most common genetic abnormalities in AML.[2] *FLT3*-ITD is the most common *FLT3* mutation, affecting approximately one in four patients with AML.[3,4,5,6] *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.[4,7]

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 Quizartinib

Quizartinib, the lead investigational agent in the AML Franchise of the Daiichi Sankyo Cancer Enterprise, is an oral selective FLT3 inhibitor currently in phase 3 development for relapsed/refractory *FLT3*-ITD AML (QuANTUM-R) in the U.S. and EU; phase 3 development for newly-diagnosed *FLT3*-ITD AML (QuANTUM-First) in the U.S., EU and Japan; and, phase 2 development for relapsed/refractory *FLT3*-ITD AML in Japan.

Quizartinib has been granted Breakthrough Therapy designation for the treatment of adult patients with relapsed/refractory *FLT3*-ITD AML, and Fast Track designation for the treatment of relapsed/refractory AML by the U.S. Food and Drug Administration (FDA). Quizartinib 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 Ministry of Health, Labour and Welfare (MHLW) for the treatment of *FLT3*-mutated AML.

Quizartinib 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 Cancer Enterprise

The mission of Daiichi Sankyo Cancer Enterprise is to leverage our world-class, innovative science and push beyond traditional thinking to create meaningful treatments for patients with cancer. We are dedicated to transforming science into value for patients, and this sense of obligation informs everything we do. Anchored by three pillars including our investigational Antibody Drug Conjugate Franchise, Acute Myeloid Leukemia Franchise and Breakthrough Science, we aim to deliver seven distinct new molecular entities over eight years during 2018 to 2025. Our powerful research engines include two laboratories for biologic/immuno-oncology and small molecules in Japan, and Plexxikon Inc., our small molecule structure-guided R&D center in Berkeley, CA. Compounds in pivotal stage development include: [fam-] trastuzumab deruxtecan, an antibody drug conjugate (ADC) for HER2 expressing breast, gastric and other cancers; quizartinib, an oral selective FLT3 inhibitor, for newly-diagnosed and relapsed/refractory *FLT3*-ITD acute myeloid leukemia (AML); and pexidartinib, an oral CSF1R inhibitor, for tenosynovial giant cell tumor (TGCT). For more information, please visit: www.DSCancerEnterprise.com.

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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