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Daiichi-Sankyo

Press Release

FDA Grants Breakthrough Therapy Designation to Daiichi Sankyo's FLT3 Inhibitor Quizartinib for Relapsed/Refractory *FLT3*-ITD AML

- Quizartinib has received FDA Breakthrough Therapy designation in patients with relapsed/refractory *FLT3*-ITD AML, a very aggressive form of the disease associated with poor prognosis
- Significant unmet medical need exists in relapsed/refractory AML, as available treatment options are limited and there are no approved targeted therapies for patients with relapsed/refractory *FLT3*-ITD AML
- Third Breakthrough Therapy designation granted by FDA for a compound in the oncology pipeline of Daiichi Sankyo, reinforcing the company's commitment to transforming science into value for patients with cancer

Tokyo, Munich and Basking Ridge, NJ – (August 1, 2018) – Daiichi Sankyo Company, Limited (hereafter, Daiichi Sankyo) announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation to quizartinib, an investigational FLT3 inhibitor, for the treatment of adult patients with relapsed/refractory *FLT3*-ITD acute myeloid leukemia (AML).

"There have been limited advances over the past several decades for the treatment of relapsed/refractory *FLT3*-ITD AML, a very aggressive form of the disease associated with poor prognosis. Quizartinib is the first FLT3 inhibitor to significantly improve overall survival as an oral, single agent compared to chemotherapy in patients with relapsed/refractory AML with *FLT3*-ITD, an underlying driver of this subtype of AML," said Arnaud Lesegretain, Vice President, Oncology Research and Development and Head, AML Franchise, Daiichi Sankyo. "We are excited that quizartinib has received Breakthrough Therapy designation and we look forward to working closely with the FDA to bring this potential new treatment option to patients as quickly as possible."

Breakthrough Therapy designation is designed to expedite the development and regulatory review of medicines that may demonstrate substantial benefit over currently approved treatments, in order to more quickly bring new treatment options to patients with serious diseases. Significant unmet medical need exists in relapsed/refractory AML, as available treatment options are limited and there are no approved targeted therapies for patients with relapsed/refractory *FLT3*-ITD AML.

The designation was granted based on the results of the pivotal phase 3 QuANTUM-R study of quizartinib, which were presented during the plenary program at the 23rd Congress of the European Hematology Association in June 2018. QuANTUM-R is the first randomized phase 3 study to show that a FLT3 inhibitor,

quizartinib, prolongs overall survival as an oral, single agent compared to chemotherapy in patients with relapsed/refractory *FLT3*-ITD AML.

The safety profile observed in QuANTUM-R appears consistent with that observed at similar doses in the quizartinib clinical development program. Incidence of treatment-emergent adverse events was comparable between patients who received single agent quizartinib (n=241) and those who received salvage chemotherapy (n=94). 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.

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 (QuANTUM-R) and newly-diagnosed (QuANTUM-First) *FLT3*-ITD AML in the U.S., EU and Japan, and phase 2 development for relapsed/refractory *FLT3*-ITD AML in Japan.

In addition to Breakthrough Therapy designation, quizartinib has been granted Fast Track designation by the FDA for the treatment of relapsed/refractory AML. Quizartinib also has been granted Orphan Drug designation by both the FDA and the European Medicines Agency (EMA) for the treatment of 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 *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] In the U.S. this year, it is estimated that there will be more than 19,000 new diagnoses of AML and more than 10,000 deaths from AML.[2] The five-year survival rate of AML reported from 2005 to 2011 was approximately 26 percent, which was the lowest of all leukemias.[1]

FLT3 gene mutations are one of the most common genetic abnormalities in AML.[3] *FLT3*-ITD is the most common *FLT3* mutation, affecting approximately one in four patients with AML.[4,5,6,7] 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 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: DS-8201, 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. Daiichi Sankyo, Inc., headquartered in Basking Ridge, New Jersey, is a member of the Daiichi Sankyo Group. For more information on Daiichi Sankyo, Inc., please visit: www.dsi.com.

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