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# Press Release

# Daiichi Sankyo Announces Single Agent Quizartinib Significantly Prolongs Overall Survival Compared with Chemotherapy in Patients with Relapsed/Refractory AML with *FLT3*-ITD Mutations (QuANTUM-R Study)

- Quizartinib is the first FLT3 inhibitor to demonstrate improved overall survival compared with chemotherapy in patients with relapsed/refractory acute myeloid leukemia (AML) with *FLT3*-ITD mutations, a very aggressive form of the disease associated with poor prognosis
- There is high unmet medical need in relapsed/refractory AML as available treatment options are limited; currently, there are no approved targeted therapies for patients with relapsed/refractory *FLT3*-ITD-mutated AML
- Results of the global, randomized, phase 3 study, QuANTUM-R, will form the basis of worldwide regulatory submissions for quizartinib, the lead investigational agent in the AML Franchise of Daiichi Sankyo
- QuANTUM-R results support an ongoing comprehensive development program for quizartinib including the QuANTUM-First trial and multiple combination studies

**Tokyo, Munich, and Basking Ridge, NJ – (May 8, 2018)** – Daiichi Sankyo Company, Limited (hereafter, Daiichi Sankyo) announces that the pivotal QuANTUM-R phase 3 study of single agent quizartinib met its primary endpoint of significantly prolonging overall survival compared to salvage chemotherapy in patients with relapsed/refractory acute myeloid leukemia (AML) with *FLT3*-ITD mutations after first-line treatment with or without hematopoietic stem cell transplantation (HSCT). Safety appears consistent with that observed at similar doses in the quizartinib program.

"Single agent quizartinib is the first FLT3 inhibitor to show a significant improvement in overall survival compared to cytotoxic chemotherapy in a randomized phase 3 study of patients with relapsed/refractory AML with *FLT3*-ITD mutations, a very aggressive form of the disease with limited treatment options," said Antoine Yver, MD, MSc, Executive Vice President and Global Head, Oncology Research and Development, Daiichi Sankyo. "We sincerely thank all of the investigators and patients who participated in this study and will share the results of the QuANTUM-R study at an upcoming medical meeting. We look forward to working with regulatory authorities worldwide to potentially bring quizartinib to patients as quickly as possible."

QuANTUM-R is a pivotal, global, phase 3, open-label randomized study that enrolled 367 patients with *FLT3*-ITD-mutated AML who were refractory to or in relapse (with duration of remission of six months or less) following standard first-line AML therapy with or without HSCT. Patients were randomized in a 2:1 ratio to receive either single agent oral quizartinib or salvage chemotherapy. The primary objective of the

study was to determine whether single agent quizartinib prolonged overall survival compared to salvage chemotherapy.

Daiichi Sankyo intends to initiate regulatory submissions worldwide for quizartinib on the basis of the QuANTUM-R results. The topline results of QuANTUM-R will be presented at an upcoming scientific conference.

**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) AML with *FLT3*-ITD mutations globally, and phase 2 development for relapsed/refractory AML with *FLT3*-ITD mutations in Japan.

Quizartinib has been granted Fast Track designation by the U.S. Food and Drug Administration (FDA) for the treatment of relapsed/refractory AML. Quizartinib also has been granted Orphan Drug designation by the FDA and European Medicines Agency (EMA) for the treatment of AML. Quizartinib is an investigational agent that is not approved for any indication in any country. Safety and efficacy have not been established.

**About Acute Myeloid Leukemia with *FLT3*-ITD Mutations**

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] 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.[2] The *FLT3*-ITD mutation is the most common *FLT3* mutation, affecting approximately one in four patients with AML.[3,4,5,6] Patients with *FLT3*-ITD-mutated 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 HSCT as compared to those without this mutation.[7,8]

**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 acute myeloid leukemia (AML) with *FLT3*-ITD mutations; and pexidartinib, an oral CSF-1R 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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