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Daiichi-Sankyo

Press Release

Daiichi Sankyo Announces First Patient Enrolled in Phase 3 QuANTUM-First Trial Investigating Quizartinib in Newly-Diagnosed FLT3-ITD+ Acute Myeloid Leukemia

- No approved targeted therapy exists for FLT3-ITD+ acute myeloid leukemia (AML), an aggressive and difficult-to-treat form of AML affecting approximately 30 percent of AML patients
- QuANTUM-First study to examine safety and efficacy of quizartinib as a first-line treatment in combination with induction and consolidation chemotherapy and as maintenance monotherapy in newly-diagnosed patients with FLT3-ITD+ AML
- Comprehensive clinical development program with second phase 3 study of quizartinib highlights Daiichi Sankyo commitment to AML
- Another phase 3 study, QuANTUM-R, currently enrolling patients with FLT3-ITD+ relapsed/ refractory AML

Tokyo, Japan, Parsippany, NJ, and Munich, Germany – (October 11, 2016) – Daiichi Sankyo Company, Limited (hereafter, Daiichi Sankyo) today announced that the first patient has been enrolled in the global phase 3 QuANTUM-First study evaluating the oral FLT3-ITD inhibitor quizartinib in patients with newly-diagnosed FLT3-ITD-positive (+) acute myeloid leukemia (AML).

QuANTUM-First is a randomized, double-blind, placebo-controlled study evaluating quizartinib in combination with induction and consolidation chemotherapy and as maintenance monotherapy in patients with newly-diagnosed FLT3-ITD+ AML. The primary endpoint of the study is event-free survival. Secondary endpoints include overall survival, complete remission rate, composite complete remission rate and the percentage of subjects achieving a complete remission with no evidence of minimal residual disease.

Approximately 30 percent of patients with AML have a genetic mutation called FLT3-ITD, which is associated with more aggressive disease, resulting in increased relapse rate and reduced overall survival compared to those without this mutation.[1] FLT3-ITD mutations are more common than FLT3-TKD mutations, which occur in approximately 10 percent of AML patients.[1] There is controversy as to whether FLT3-TKD mutations carry as poor a prognosis as FLT3-ITD mutations.[1] Currently, there are no approved targeted treatments for FLT3-ITD+ AML, with little change in the treatment of AML for the past 30 years.[2]

“It is well established that patients with FLT3-ITD mutated AML have an overall worse prognosis compared to those without this specific mutation,” said Harry Erba, MD, PhD, Chair of the QuANTUM-First Steering Committee and Professor of Medicine and Director of the Hematologic Malignancy Program at the University of Alabama at Birmingham. “In this study we are evaluating whether adding

quizartinib to standard first-line chemotherapy will help delay or prevent relapse, which in turn may impact overall survival in patients with FLT3-ITD+ AML."

"Given the high unmet need in FLT3-ITD+ AML, we are moving forward with a comprehensive clinical development program investigating the role of quizartinib in multiple lines of treatment including induction and consolidation chemotherapy, maintenance therapy and salvage therapy," said Antoine Yver, MD, MSc, Executive Vice President and Global Head, Oncology Research and Development, Daiichi Sankyo. "Additionally, we also are looking to combine quizartinib with other investigational agents in our pipeline such as our MDM2 and BRD4 inhibitors where science suggests combining different mechanisms of action may help improve outcomes."

QuANTUM-First is expected to enroll more than 500 patients between 18 and 75 years of age in the Americas, Europe and Asia-Pacific. More information about the study is available at ClinicalTrials.gov or www.QuantumFirstStudy.com.

About Acute Myeloid Leukemia (AML)

Acute myeloid leukemia (AML) is the most common type of acute leukemia accounting for about 33 percent of all new cases of leukemia.[3] An aggressive blood and bone marrow cancer, AML 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] The five-year survival rate of AML is approximately 26 percent, which is the lowest of all leukemias.[3]

About Quizartinib

Quizartinib is an investigational oral small molecule that potently and selectively inhibits FLT3-ITD (FMS-like tyrosine kinase-3-internal tandem duplication), which is a growth driver of abnormal cells that contribute to the development of AML.[4] Quizartinib has been granted Orphan Drug Designation by the U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) for the treatment of AML. Quizartinib also has been granted Fast Track Designation by the FDA for the treatment of relapsed/refractory AML. Quizartinib has not been approved by any regulatory authority for uses under investigation.

In addition to QuANTUM-First, a global, randomized, open-label, phase 3 study called QuANTUM-R is evaluating quizartinib as monotherapy in patients with FLT3-ITD+ AML who are refractory to or have relapsed after first-line treatment with or without hematopoietic stem cell transplant (HSCT). The primary objective of QuANTUM-R is to determine whether quizartinib prolongs overall survival compared to salvage chemotherapy, and the secondary objective is to determine event-free survival. The trial is expected to enroll approximately 363 patients age 18 or older in North America, Europe and Asia-Pacific. More information about QuANTUM-R is available at www.QuantumRStudy.com or ClinicalTrials.gov.

About Daiichi Sankyo Cancer Enterprise

The vision of Daiichi Sankyo Cancer Enterprise is to push beyond traditional thinking to align world-class science to create innovative treatments for patients with cancer. The oncology pipeline of Daiichi Sankyo continues to grow and currently includes more than 20 small molecules and monoclonal antibodies with novel targets in both solid and hematological cancers. Compounds in phase 3 development include: quizartinib, an oral FLT3-ITD inhibitor, for newly-diagnosed and relapsed/ refractory FLT3-ITD+ acute myeloid leukemia (AML); pexidartinib, an oral CSF-1R inhibitor, for tenosynovial giant cell tumor (TGCT), also known as pigmented villonodular synovitis (PVNS) and giant cell tumor of the tendon sheath (GCT-TS), which also is being investigated in combination with anti-PD1 immunotherapy, pembrolizumab, in a range of solid tumors; and tivantinib, an oral MET inhibitor, for second-line treatment in patients with MET-high hepatocellular carcinoma in partnership with ArQule, Inc.

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 16,000 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 Parsippany, New Jersey, is a member of the Daiichi Sankyo Group. To learn more about Daiichi Sankyo, Inc., please visit: www.dsi.com

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