Passion for Innovation.
Compassion for Patients.™

Daiichi-Sankyo

Press Release

Not Intended for UK Media Use

Daiichi Sankyo to Present Late-Breaking Phase 3 Data for Single Agent Quizartinib in Patients with Relapsed/Refractory AML with *FLT3*-ITD Mutations at Plenary Session at EHA 2018

- Data from QuANTUM-R study demonstrating single agent quizartinib significantly prolongs overall survival compared to salvage chemotherapy in patients with relapsed/refractory acute myeloid leukemia (AML) with *FLT3*-ITD mutations to be unveiled
- Quizartinib is the first FLT3 inhibitor to show a survival benefit in patients with relapsed/refractory *FLT3*-ITD-mutated AML, a very aggressive form of the disease associated with poor prognosis
- Safety profile observed in QuANTUM-R appears to be consistent with that observed at similar doses in the quizartinib program
- Preclinical data evaluating quizartinib, the lead investigational agent in the AML Franchise of Daiichi Sankyo, in combination with investigational MDM2 inhibitor milademetan (DS-3032) will be disclosed

Basking Ridge, NJ, and Munich – (June 6, 2018) – Daiichi Sankyo Company, Limited (hereafter, Daiichi Sankyo) announced today that topline results from the pivotal QuANTUM-R phase 3 study of single agent quizartinib will be presented for the first time at the 23rd Congress of the European Hematology Association (EHA) from June 14-17 in Stockholm. The late-breaking oral presentation will be featured in the plenary program at EHA on Saturday, June 16 from 1:00 to 2:30 PM CEST.

"We look forward to sharing the results from the QuANTUM-R study of single agent quizartinib, which demonstrate the potential of quizartinib to redefine the treatment of patients with relapsed/refractory AML with *FLT3*-ITD mutations," said Antoine Yver, MD, MSc, Executive Vice President and Global Head, Oncology Research and Development, Daiichi Sankyo. "We also will be sharing new preclinical data that provide additional insight into the potential role of quizartinib as a combination therapy with milademetan, our investigational MDM2 inhibitor."

The QuANTUM-R presentation will include results demonstrating quizartinib significantly prolongs 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. This presentation at EHA follows the announcement of positive topline results from QuANTUM-R in May 2018. Data from QuANTUM-R will be submitted to health authorities for approval consideration.

Additional data from the comprehensive development program for quizartinib also will be disclosed at EHA, including a post hoc exploratory analysis of two phase 2 studies evaluating single agent quizartinib

in patients with relapsed/refractory AML with *FLT3*-ITD mutations and prior exposure to first generation FLT3 inhibitors, preclinical research exploring the combination of quizartinib and milademetan (DS-3032), an investigational MDM2 inhibitor, in *FLT3*-ITD-mutated AML, and pharmacokinetic analyses of quizartinib.

The following data from the pipeline of Daiichi Sankyo Cancer Enterprise will be presented at EHA:

- **Quizartinib Significantly Prolongs Overall Survival in Patients with *FLT3*-Internal Tandem Duplication-Mutated Relapsed/Refractory AML in the Phase 3, Randomized, Controlled QuANTUM-R Trial** (Abstract LB2600; Plenary Session 1; Saturday, June 16 at 1:15 – 1:30 PM CEST)
- **Post Hoc Exploratory Analysis of Two Phase 2 Trials of Quizartinib Monotherapy in Patients with *FLT3*-ITD-Mutated Relapsed/Refractory AML and Prior FLT3 Tyrosine Kinase Inhibitor Treatment** (Abstract PF240; Poster Presentation; Friday, June 15 at 5:30 – 7:00 PM CEST)
- **Combination of FLT3 Inhibitor Quizartinib and MDM2 Inhibitor Milademetan Results in Greater Preclinical Anti-Leukemic Activity in *FLT3*-ITD Mutant/P53 Wild-Type Acute Myeloid Leukemia Models** (Abstract PF219; Poster Presentation; Friday, June 15 at 5:30 – 7:00 PM CEST)
- **A Drug-Drug Interaction Study to Assess the Effect of Acid Reducing Agent, Lansoprazole, on Quizartinib Pharmacokinetics** (Abstract PB1725; Publication Only)
- **Effect of a High-Fat and High-Calorie Meal on the Pharmacokinetics (PK) of Quizartinib (Q) and Its Active Metabolite** (Abstract PB1724; Publication Only)

In addition, Daiichi Sankyo will host a symposium, "Taking a Personalised Approach to AML Treatment: FLT3 Inhibition" on Thursday, June 14 from 8:00 to 10:00 AM CEST in Stockholmsmässan, Room A6.

About the QuANTUM-R Study

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 hematopoietic stem cell transplantation. 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.

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.

About Milademetan (DS-3032)

Milademetan is an investigational, oral selective MDM2 inhibitor currently in phase 1 clinical development for hematologic malignancies including AML, acute lymphocytic leukemia (ALL), chronic lymphocytic leukemia (CLL) in blast phase and myelodysplastic syndrome (MDS) in the U.S. and for solid tumors and lymphomas in the U.S. and Japan.

Quizartinib and milademetan are investigational agents that have not been approved for any indication in any country. Safety and efficacy of these investigational agents 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 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.

Contact

Jennifer Brennan
Daiichi Sankyo, Inc.
jbrennan2@dsi.com
+1 908 992 6631 (office)
+1 201 709 9309 (mobile)

References

1. Leukemia & Lymphoma Society. Facts 2015-2016. 2016.
2. Small D. Am Soc Hematol Educ Program. 2006;178-84.
3. Schneider F, et al. Ann Hematol. 2012;91:9-18.
4. Santos FPS, et al. Cancer. 2011;117(10):2145-2155.
5. Kainz B, et al. Hematol J. 2002;3:283-289.
6. Kottaridis PD, et al. Blood. 2001;98(6):1752-1759.
7. Wagner K, et al. Haematol. 2011;96(5):681-686.
8. Brunet S, et al. J Clin Onc. 2012;30(7):735-741.