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# Press Release

## Japan MHLW Grants Orphan Drug Designation to Axicabtagene Ciloleucel for Treatment of Certain Types of B-Cell Lymphoma

**Tokyo and Basking Ridge, NJ - (October 3, 2018) -** Daiichi Sankyo Company, Limited (hereafter, Daiichi Sankyo) today announced that axicabtagene ciloleucel (formerly KTE-C19) has been granted Orphan Drug designation by the Japan Ministry of Health, Labour and Welfare (MHLW) for the treatment of diffuse large B-cell lymphoma (DLBCL), primary mediastinal (thymus) large B-cell lymphoma (PMBCL), high-grade B-cell lymphoma (HGBL) and transformed follicular lymphoma (TFL), which are aggressive forms of non-Hodgkin lymphoma (NHL).

"Receiving Orphan Drug designation is an important step in expediting the development of axicabtagene ciloleucel in Japan and underscores the unmet needs of patients with these aggressive forms of relapsed or refractory B-cell lymphomas," said Kouichi Akahane, PhD, MBA, Executive Officer, Head of Oncology Function, R&D Division, Daiichi Sankyo. "This designation represents the third Orphan Drug designation granted for an investigational therapy in our oncology pipeline, demonstrating our commitment to transforming innovative science into value for patients. We look forward to working closely with the Japan Health Authority to bring this important cell therapy to patients in Japan as soon as possible."

The Japan MHLW Orphan Drug designation system is designed to promote research activities and support the development of orphan drugs for serious, difficult-to-treat diseases that affect fewer than 50,000 patients in Japan, and for which significant unmet medical need exists. An investigational therapy can qualify for Orphan Drug designation if there is no approved alternative treatment option or if there is high efficacy or safety compared to existing treatment options expected. Therapies receiving Orphan Drug designation qualify for several measures intended to support development including, but not limited to, guidance and subsidies for research and development activities, priority consultation for clinical development and priority review of applications.

Axicabtagene ciloleucel is a chimeric antigen receptor T cell (CAR T) therapy directed against CD19 (a cell membrane protein), which harnesses a patient's own immune system to fight certain types of B-cell lymphoma. In January 2017, Daiichi Sankyo received exclusive development, manufacturing and commercialization rights for axicabtagene ciloleucel in Japan from California-based Kite Pharma, Inc., a Gilead company. Based on the results of a Phase 1/2 study (ZUMA-1), axicabtagene ciloleucel has been approved in the U.S. and Europe. A Phase 2 study similar to the ZUMA-1 study is currently being planned in Japan.

**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. 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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