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Japan MHLW grants orphan designation to Axicabtagene Ciloleucel

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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.

Daiichi Sankyo Company, Limited has 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.