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Press Release

Daiichi Sankyo Initiates Phase 2 Trial to Evaluate Pexidartinib in Japanese Patients with TGCT

Tokyo, Japan – (April 9, 2021) – Daiichi Sankyo Company, Limited (hereafter, Daiichi Sankyo) today announced that the first patient was dosed in a phase 2 study to evaluate the safety and efficacy of pexidartinib, a CSF-1R inhibitor, in Japanese patients with symptomatic tenosynovial giant cell tumor (TGCT) associated with severe morbidity or functional limitation and not amenable to improvement with surgery.

TGCT, also referred to as pigmented villonodular synovitis (PVNS) or giant cell tumor of the tendon sheath (GCT-TS), is a rare, typically non-malignant tumor that can be locally aggressive. TGCT affects the synovium-lined joints, bursae and tendon sheaths, resulting in reduced mobility in the affected joint or limb.[1,2,3] For most patients, the current standard of care for TGCT is surgery; however, in more severe cases, the tumor may be difficult to remove and/or may not improve with surgery.[4] Multiple surgeries can lead to significant joint damage, debilitating functional impairments, and reduced quality of life, and amputation may be considered.[4,5]

"We are committed to continuing to explore the clinical benefit of pexidartinib in patients with TGCT, a disease which can be associated with severe morbidity, functional limitations and may not be amenable to improvement with surgery," said Wataru Takasaki, PhD, Executive Officer, Head of R&D Division in Japan, Daiichi Sankyo. "This study will further serve to inform our development path forward in Japan and provide additional research insights to better understand this debilitating disease and the role pexidartinib may potentially play in Japanese patients with TGCT who are in need of alternative treatment options."

About the Study

This phase 2, multicenter, two-part, open-label, single-arm study will evaluate the safety and efficacy of pexidartinib in Japanese patients with symptomatic TGCT associated with severe morbidity or functional limitation and not amenable to improvement with surgery.

Approximately 18 patients will be enrolled into the two-part study. In the first part, pexidartinib 800 mg/day (400 mg twice a day on an empty stomach) will be administered to evaluate the tolerability and pharmacokinetics of pexidartinib to determine the initiation of the second part of the study. In the

second part, pexidartinib 800 mg/day (400 mg twice a day on an empty stomach) will be administered and efficacy, safety, and pharmacokinetics of pexidartinib will be evaluated.

The primary trial endpoints are dose-limiting toxicity, an analysis of pharmacokinetics and objective response rate (ORR), assessed by RECIST Version 1.1. Key secondary endpoints include ORR by tumor volume score, range of motion, patient reported outcomes, and treatment-emergent adverse events.

The study will enroll patients at sites in Japan. For more information visit ClinicalTrials.gov (NCT04703322) and the Japan Registry of Clinical Trials (JRCT ID: jRCT2041200074).

About TGCT (PVNS/GCT-TS)

TGCT, also referred to as pigmented villonodular synovitis (PVNS) or giant cell tumor of the tendon sheath (GCT-TS), is a rare, typically non-malignant tumor that can be locally aggressive.

The current standard of care for TGCT is surgical resection. However, in patients with diffuse-type TGCT, the tumor may wrap around bone, tendons, ligaments and other parts of the joint. In these cases, the tumor may be difficult to remove and/or may not be amenable to improvement with surgery.[4] Multiple surgeries for more severe cases can lead to significant joint damage, debilitating functional impairments and reduced quality of life, and amputation may be considered.[4,5]

Recurrence rates for localized TGCT are estimated to be up to 15 percent following complete resection. Diffuse TGCT recurrence rates are estimated to be about 20 percent to 50 percent following complete resection.[1,6] TGCT affects all age groups; the diffuse type on average occurs most often in people below the age of 40, and the localized type typically occurs in people between 30 and 50 years old.[6]

About Pexidartinib

Pexidartinib is an oral small molecule that inhibits CSF-1R (colony stimulating factor-1 receptor), which is a primary growth driver of abnormal cells in the synovium that cause TGCT. Pexidartinib also inhibits KIT and *FLT3*-ITD. Pexidartinib was discovered by Plexxikon Inc., the small molecule structure-guided R&D center of Daiichi Sankyo.

Pexidartinib is currently being studied in two studies as a potential treatment for patients with TGCT in Japan, Taiwan and China. It is not approved for use in any country outside of the U.S.

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 our DXd antibody drug conjugate (ADC) technology, our powerful research engines include biologics, medicinal chemistry, modality and other research laboratories in Japan, and Plexxikon Inc., our small molecule structure-guided R&D center in Berkeley, CA. For more information, please visit: www.DSCancerEnterprise.com.

About Daiichi Sankyo

Daiichi Sankyo Group is dedicated to the creation and supply of innovative pharmaceutical therapies to improve standards of care and address diversified, unmet medical needs of people globally by leveraging our world-class science and technology. With more than 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 cardiovascular diseases, under the Group's 2025 Vision to become a "Global Pharma Innovator with Competitive Advantage in Oncology," Daiichi Sankyo is primarily focused on providing novel therapies in oncology, as well as other research areas centered around rare diseases and immune disorders. For more information, please visit: www.daiichisankyo.com.

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