

Press Release

Daiichi Sankyo Provides Update on CHMP Review of Pexidartinib, a CSF1R Inhibitor for the Treatment of Patients with TGCT

Tokyo and Munich – (June 26, 2020) – Daiichi Sankyo Company, Limited (hereafter, Daiichi Sankyo) today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a negative opinion on the Marketing Authorization Application (MAA) for pexidartinib for the treatment of a subset of adult patients with severe tenosynovial giant cell tumor (TGCT). The European marketing authorization application was based on results of the pivotal phase 3 ENLIVEN study, which were published in *The Lancet* on June 19, 2019.¹

“We will evaluate feedback received from the Committee in order to determine the appropriate next steps for pexidartinib in the EU. Despite this setback, we continue to believe in the potential of pexidartinib for people with TGCT, who often face debilitating symptoms and currently have no approved systemic treatment option,” said Antoine Yver, MD, MSc, Executive Vice President and Global Head, Oncology Research and Development, Daiichi Sankyo.

About Pexidartinib

Pexidartinib is an oral small molecule that inhibits CSF1R (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.

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. TGCT affects the synovium-lined joints, bursae and tendon sheaths, resulting in reduced mobility in the affected joint or limb.^{2,3,4} Given TGCT is rare, it's not known exactly how many people are diagnosed with the condition each year.

The current standard of care for TGCT is surgical resection.^{2,5} However, in patients with a recurrent, difficult-to-treat, or diffuse form of 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. 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.^{5,6,7}

Recurrence rates for localized TGCT are estimated to be up to 15 percent following complete resection.^{8,9} Diffuse TGCT recurrence rates are estimated to be about 20 percent to 50 percent following complete resection.^{4,9 10} 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.²

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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