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Press Release

ENLIVEN Phase 3 Study of Pexidartinib in Tenosynovial Giant Cell Tumor (TGCT) Will Continue to Completion Following Enrollment Discontinuation

Tokyo, Japan and Parsippany, NJ – (October 21, 2016) – Daiichi Sankyo Company, Limited (hereafter, Daiichi Sankyo) announced today that it will discontinue additional enrollment of the phase 3 ENLIVEN study of the investigational oral CSF-1R inhibitor pexidartinib (PLX3397) in tenosynovial giant cell tumor; however, the study will proceed with currently enrolled patients under a revised protocol.

Following review of two recently reported cases of non-fatal, serious liver toxicity, the ENLIVEN data monitoring committee (DMC) recommended that further enrollment into the study be suspended. At the time of enrollment suspension 121 patients had been randomized, five patients short of the 126 planned for full enrollment. The DMC also recommended measures to address these safety concerns while maintaining the blinded nature of the study. As a result, ENLIVEN will continue in order to evaluate its efficacy and safety endpoints.

All regulatory authorities involved in the ENLIVEN study have been notified. All patients currently enrolled in ENLIVEN are being informed about this updated safety information and will be offered the opportunity to re-consent for continued participation in the study.

"Ensuring patient safety is our first obligation and we sincerely thank all of the investigators and patients participating in this study," said Antoine Yver, MD, MSc, Executive Vice President and Global Head, Oncology Research and Development, Daiichi Sankyo. "Upon completion of the study with currently enrolled patients, Daiichi Sankyo will conduct and report a thorough evaluation of the results and consider any and all appropriate next steps."

ENLIVEN is an ongoing global, multi-center, pivotal two-part phase 3 study evaluating pexidartinib in patients with symptomatic tenosynovial giant cell tumor (TGCT) for whom surgical removal of the tumor would be associated with potentially worsening functional limitation or severe morbidity. The first part of the study, which is the double-blind phase, is designed to evaluate the efficacy and safety of pexidartinib versus placebo. The second part of the study is a longer-term open-label study of pexidartinib.

About TGCT

Tenosynovial giant cell tumor (TGCT) – also known as pigmented villonodular synovitis (PVNS) or giant cell tumor of the tendon sheath (GCT-TS) – is a rare, usually non-cancerous tumor that affects the synovium-lined joints, bursae, and tendon sheaths, resulting in swelling, pain, stiffness and reduced mobility in the affected joint or limb.[1] It is estimated that TGCT has an annual incidence of 11 cases per million.[2] Patients are

commonly diagnosed in their 20s to 50s, and depending on the type of TGCT, women can be up to twice as likely to develop a tumor as men.[3,4]

Primary treatment of TGCT includes surgery to remove the tumor, but in patients with a diffuse form where it can wrap around bone, tendons, ligaments and other parts of the joint, the tumor is more difficult to remove and may require multiple surgeries or joint replacement, eventually advancing to the point where surgery is no longer an option and amputation may be considered. It is estimated that the rate of recurrence can be about 15 to 45 percent.[5]

About Pexidartinib

Pexidartinib is an investigational novel, oral small molecule that potently and selectively inhibits CSF-1R (colony stimulating factor-1 receptor), which is a primary growth driver of abnormal cells in the synovium that cause TGCT.

Pexidartinib has been granted Breakthrough Therapy Designation by the U.S. Food and Drug Administration (FDA) for the treatment of TGCT. Pexidartinib has also been granted Orphan Drug Designation by the FDA for the treatment of PVNS/GCT-TS and received Orphan Designation from the European Commission for the treatment of TGCT. Pexidartinib has not been approved by any regulatory authority for any use.

Pexidartinib is being evaluated in several additional potential clinical indications, including glioblastoma, ovarian, breast, colorectal, pancreatic and prostate cancer, malignant peripheral nerve sheath tumor, and pediatric cancers. It is also being investigated in combination with anti-PD-1 immunotherapy, pembrolizumab, for advanced melanoma or other solid tumors.

About Daiichi Sankyo Cancer Enterprise

The vision of Daiichi Sankyo Cancer Enterprise is to push beyond traditional thinking to align world-class science to create innovative treatments for patients with cancer. The oncology pipeline of Daiichi Sankyo continues to grow and currently includes more than 20 small molecules, monoclonal antibodies and antibody drug conjugates with novel targets in both solid and hematological cancers. Compounds in development include: quizartinib, an oral FLT3-ITD inhibitor, for newly-diagnosed and relapsed/refractory FLT3-ITD+ acute myeloid leukemia (AML); pexidartinib, an oral CSF-1R inhibitor, for tenosynovial giant cell tumor (TGCT), also known as pigmented villonodular synovitis (PVNS) and giant cell tumor of the tendon sheath (GCT-TS), which also is being investigated in combination with anti-PD1 immunotherapy, pembrolizumab, in a range of solid tumors; tivantinib, an oral MET inhibitor, for second-line treatment of patients with MET-high hepatocellular carcinoma in partnership with ArQule, Inc.; and DS-8201a, a HER2 targeting antibody drug conjugate, for HER2-expressing breast or gastric cancer or other HER2-expressing solid tumors.

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 16,000 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 Parsippany, 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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