

## Press Release

# FDA Grants Breakthrough Therapy Designation to Daiichi Sankyo's DS-8201 for HER2-Positive Metastatic Breast Cancer

- Breakthrough Therapy designation received for the treatment of patients with HER2-positive, locally advanced or metastatic breast cancer who have been treated with trastuzumab and pertuzumab and have disease progression after ado-trastuzumab emtansine (T-DM1)
- HER2-positive metastatic breast cancer often advances to the point where no currently approved HER2-targeted treatments continue to control the disease
- Designation based on preliminary clinical evidence highlights that DS-8201 has the potential to offer substantial clinical benefit to patients with a high unmet medical need

**Tokyo, Japan, Basking Ridge, NJ, and Munich, Germany – (August 29, 2017)** – Daiichi Sankyo Company, Limited (hereafter, Daiichi Sankyo) today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation to DS-8201, an investigational HER2-targeting antibody drug conjugate (ADC), for the treatment of patients with HER2-positive, locally advanced or metastatic breast cancer who have been treated with trastuzumab and pertuzumab and have disease progression after ado-trastuzumab emtansine (T-DM1).

Breakthrough Therapy designation is designed to expedite the development and review of medicines that may demonstrate substantial benefit over currently available treatments in order to ensure that patients with serious diseases have access to new treatments as soon as possible. Currently, there is no FDA-approved therapy for patients with HER2-positive metastatic breast cancer with disease progression following treatment with other HER2-targeting agents trastuzumab, pertuzumab and T-DM1.

“The Breakthrough Therapy designation for DS-8201 in HER2-positive metastatic breast cancer acknowledges the unmet medical need these patients face when currently approved treatments no longer control their disease,” said Antoine Yver, MD, MSc, Executive Vice President and Global Head, Oncology Research and Development, Daiichi Sankyo. “We remain committed to rapidly progressing the development of DS-8201 and look forward to working closely with the FDA to potentially bring this new treatment option to patients with metastatic breast cancer as quickly as possible.”

The Breakthrough Therapy designation was granted based on the results of the ongoing phase 1 study assessing the safety, tolerability and preliminary efficacy of DS-8201. In the phase 1 study, no dose limiting toxicities were observed, and the maximum tolerated dose was not reached. Preliminary results of DS-8201 from a subgroup analysis of HER2-expressing metastatic breast cancer pre-treated with trastuzumab, pertuzumab and T-DM1 were recently presented at the 2017 American Society of Clinical Oncology (ASCO) annual meeting.<sup>1</sup>

## **About DS-8201**

DS-8201 is the lead product in the ADC Franchise of the Daiichi Sankyo Cancer Enterprise. ADCs are a type of targeted cancer medicine that deliver cytotoxic chemotherapy (“payload”) to cancer cells via a linker attached to a monoclonal antibody that binds to a specific target expressed on cancer cells. Using Daiichi Sankyo’s proprietary ADC technology, DS-8201 is a smart chemotherapy comprised of a humanized HER2 antibody attached to a novel topoisomerase I inhibitor (DXd) payload by a tetrapeptide linker. It is designed to deliver enhanced cell destruction upon release inside the cell and reduce systemic exposure to the cytotoxic payload (or chemotherapy) compared to the way chemotherapy is commonly delivered.

In addition to Breakthrough Therapy designation, the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to DS-8201 for the treatment of HER2-positive unresectable and/or metastatic breast cancer in patients who have progressed after prior treatment with HER2-targeted therapies including T-DM1. DS-8201 is currently in phase 1 clinical development for HER2-positive advanced or metastatic breast cancer and gastric cancer, HER2 low-expressing breast cancer and other HER2-expressing solid tumors. DS-8201 is an investigational agent that has not been approved for any indication in any country. Safety and efficacy have not been established, and there is no guarantee DS-8201 will become commercially available.

## **About HER2-Positive Breast Cancer**

About one in five patients with breast cancer overexpress HER2, a tyrosine kinase receptor growth-promoting protein found on the surface of some cancer cells, which is associated with aggressive disease.<sup>2</sup> Many tumors advance to the point where no currently approved HER2-targeting treatment continues to control the disease. Furthermore, there is no standard of care for HER2-positive tumors following treatment with trastuzumab, pertuzumab and T-DM1.<sup>3</sup>

## **About Daiichi Sankyo Cancer Enterprise**

The vision of Daiichi Sankyo Cancer Enterprise is to leverage our world-class, innovative science and push beyond traditional thinking in order 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 Antibody Drug Conjugate (ADC) and Acute Myeloid Leukemia (AML) Franchises, our cancer pipeline includes more than 20 small molecules, monoclonal antibodies and ADCs stemming from our powerful research engines: our 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 development include: quizartinib, an oral FLT3 inhibitor, for newly-diagnosed and relapsed or refractory AML with FLT3-ITD mutations; DS-8201, an ADC for HER2-expressing breast and gastric cancer, and other HER2-expressing solid tumors; and pexidartinib, an oral CSF-1R inhibitor, for tenosynovial giant cell tumor (TGCT), which is also being explored in a range of

solid tumors in combination with the anti-PD1 immunotherapy pembrolizumab. For more information, please visit: [www.DSCancerEnterprise.com](http://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](http://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](http://www.dsi.com).

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### **References**

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2. American Cancer Society. Breast Cancer Overview. 2016.
3. NCCN Guidelines. Breast Cancer. Version 2.2017.