Sumitomo Dainippon Pharma

News Release

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Sumitomo Dainippon Pharma Co., Ltd.

Sumitomo Dainippon Pharma Receives Approval in Japan for RETHIO® for Conditioning Treatment Prior to Autologous Hematopoietic Stem Cell Transplantation

Sumitomo Dainippon Pharma Co., Ltd. (Head Office: Osaka, Japan; Representative Director, President and CEO: Hiroshi Nomura hereinafter called "Sumitomo Dainippon Pharma") announced today that it received approval in Japan for "RETHIO® for Intravenous Infusion 100 mg" (generic name: thiotepa, hereinafter called "RETHIO") in Japan on March 26, for conditioning treatment prior to autologous hematopoietic stem cell transplantation (auto-HSCT) for pediatric malignant solid tumors. Sumitomo Dainippon Pharma will launch the product after NHI drug price listing.

Thiotepa is an antitumor alkylating agent that belongs to the ethyleneimine group and inhibits DNA synthesis. In Japan, Sumitomo Chemical Co., Ltd. launched this agent as Tespamin injection in 1958, which was then taken over by Sumitomo Pharmaceuticals Co., Ltd. (current Sumitomo Dainippon Pharma) in 1984. However, following the discontinued provision of its active pharmaceutical ingredients in 2006, Sumitomo Dainippon Pharma discontinued manufacturing of Tespamin in 2009, and its marketing authorization was withdrawn in 2010. This agent has not been available since then in Japan.

After the discontinuation of its marketing in Japan, many requests for its use for this indication were made by academic societies and patient advocacy groups concerned, as thiotepa was approved for the indication in Europe in 2010. In response, the Evaluation Committee on Unapproved or Off-labeled Drugs with High Medical Needs of the Ministry of Health, Labour and Welfare of Japan (MHLW) determined that the medical need for thiotepa was high. Accordingly, the MHLW invited pharmaceutical companies to develop the agent for the indication, to which Sumitomo Dainippon Pharma replied in September 2013 and initiated a Phase 1 study in Japan from November 2016 as a pharmacokinetic study, the results of which were included in the data attached to the application in July 2018. Meanwhile, Sumitomo Dainippon Pharma is also preparing an application for approval of RETHIO® for indication of conditioning treatment prior to auto-HSCT for malignant lymphoma.

Sumitomo Dainippon Pharma expects that the approval would provide a new therapeutic option for patients in areas with high unmet medical needs, such as conditioning treatment prior to auto-HSCT for pediatric malignant solid tumors, contributing to the treatment of patients with such symptoms.

<Reference information>

About autologous hematopoietic stem cell transplantation (auto-HSCT)

Autologous hematopoietic stem cell transplantation is a therapy that aims to reconstruct hematopoietic capacity via intravenous transfusion of normal hematopoietic stem cells of the patient himself/herself after eradicating intractable cancers by conditioning myeloablative treatment

prior to transplantation using maximum levels of anti-cancer drugs or radiation. Unlike allogeneic hematopoietic stem cell transplantation that uses donor's hematopoietic stem cells, auto-HSCT is free from concerns about immunoreactions by transplanted hematopoietic stem cell-derived immune cells against host. As such, this conditioning treatment prior to auto-HSCT aims to eradicate tumor cells as much as possible through high-dose chemotherapy using anticancer drugs at doses that exceed the maximum tolerated level for bone marrow. According to the Japanese Data Center for Hematopoietic Cell Transplantation, the number of cases of hematopoietic stem cell transplantation in Japan totaled 93,902 between 1986 and 2016, among which 33,527 cases were autologous hematopoietic stem cell transplantation.

About pediatric malignant solid tumors

According to the Practical guidelines for pediatric cancer 2016, approximately 2,500 new cases of pediatric cancer occur annually in Japan. According to the Japanese Society of Pediatric Hematology/Oncology, 904 cases of solid tumors excluding hematopoietic organ tumors, such as leukemia, are registered in 2015. Compared to solid tumors in adults, pediatric malignant solid tumors are shown to be relatively chemosensitive. As a result, better outcome of high-dose chemotherapy combined with hematopoietic stem cell transplantation is expected, and transplantation is now being performed as a part of daily clinical practice. According to the Japanese Data Center for Hematopoietic Cell Transplantation, the number of hematopoietic stem cell transplantations for pediatric solid tumor, including pediatric malignant solid tumor, patients totaled 3,276 between 1991 and 2016, among which 3,058 cases were auto-HSCT.

About the Evaluation Committee on Unapproved or Off-labeled Drugs with High Medical Needs

The Evaluation Committee on Unapproved or Off-label Drugs with High Medical Needs is a committee established to promote the development of unapproved or off-label drugs by pharmaceutical companies that are approved for use in Europe and the United States, etc., but not approved in Japan. It is organized under the Ministry of Health, Labour and Welfare of Japan and consists of academic experts in medical and pharmaceutical fields.

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