Sumitomo Dainippon Pharma

News Release

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Sumitomo Dainippon Pharma Co., Ltd.

Sumitomo Dainippon Pharma to Launch 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 "Sumitomo Dainippon Pharma") announced today the launch of RETHIO® 100 mg for I.V. infusion (generic name: thiotepa) for conditioning treatment prior to autologous hematopoietic stem cell transplantation (auto-HSCT) scheduled on May 28, 2019, following its NHI drug price listing today.

RETHIO® is approved for conditioning treatment prior to auto-HSCT for pediatric malignant solid tumors. Its active ingredient, thiotepa, is an antitumor alkylating agent that belongs to the ethyleneimine group and inhibits DNA synthesis. By its capacity to cross the blood-brain barrier, it is shown to be able to rapidly penetrate into the central nervous system.

In 2012, the Evaluation Committee on Unapproved or Off-labeled Drugs with High Medical Needs of the Ministry of Health, Labour and Welfare (MHLW) of Japan determined that the medical need for thiotepa was high. Subsequently, Sumitomo Dainippon Pharma replied to develop the agent for the indication in September 2013, initiated a Phase 1 study in Japan from November 2016 as a pharmacokinetic study, and received marketing approval for the drug in March 2019.

By promoting its proper use, Sumitomo Dainippon Pharma will contribute to improved outcome for patients requiring conditioning treatment prior to auto-HSCT for pediatric malignant solid tumors, a therapeutic area with high unmet medical needs.

[note] RETHIO® has been placed in the NHI drug price list for the indication for conditioning treatment prior to auto-HSCT for pediatric malignant solid tumors but that Sumitomo Dainippon Pharma applied for a partial change in the marketing approval previously acquired in Japan for the additional indication for conditioning treatment prior to auto-HSCT for malignant lymphoma.

<Reference information>

[About thiotepa]

In 1958, Sumitomo Chemical Co., Ltd. launched thiotepa as Tesupamin® injection in Japan. However, as the supply of its active pharmaceutical ingredients was subsequently discontinued and then its marketing authorization was withdrawn in 2010, however, the agent has not been available since then in Japan. As thiotepa was approved for conditioning treatment prior to HSCT in Europe in 2010, many requests for its use for this indication were made by academic societies and other parties concerned in Japan.

[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.

[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. The Japanese Society of Pediatric Hematology/Oncology reported that 904 cases of solid tumors excluding hematopoietic organ tumors, such as leukemia, were registered in 2015. Compared to solid tumors in adults, pediatric malignant solid tumors are shown to be relatively chemosensitive. As a result, better outcomes of high-dose chemotherapy combined with hematopoietic stem cell transportation are expected, and transplantation is now being performed as 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 tumors, including pediatric malignant solid tumors, totaled 3,276 patients 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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