NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

March 28, 2025

Submission of New Drug Application for NS-401 (Tagraxofusp) in Japan

KYOTO, Japan, March 28, 2025 – Nippon Shinyaku Co., Ltd. (Headquarters: Kyoto, Japan, President: Toru Nakai) announced today that it submitted a New Drug Application (NDA) to the Ministry of Health, Labour & Welfare (MHLW) for the manufacturing and marketing approval of NS-401 (tagraxofusp) for the treatment of Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN) in Japan. Nippon Shinyaku acquired a license for NS-401 from Menarini Group (Headquarters: Florence, CEO: Elcin Barker Ergun) on March 18, 2021.

BPDCN is a rare aggressive hematologic malignancy that has features of both leukemia and lymphoma, with characteristic skin lesions, lymph node involvement, and frequent spread to the bone marrow, with poor prognosis. In the absence of an approved treatment option, BPDCN treatment is based on intensive multiagent leukemia or lymphoma chemotherapy regimens in Japan. However, the median overall survival of patients with BPDCN continues to be short (less than 12 months) and new therapeutic agents are desired.[1-2)]

Tagraxofusp is a first-in-class CD123 targeted therapy that induces apoptosis of BPDCN cells by inhibiting protein synthesis by specifically targeting CD123 expressing BPDCN cells. Based on the results of Phase I/II trials conducted overseas in untreated and relapsed/refractory BPDCN patients, tagraxofusp has been approved by the U.S. Food and Drug Administration for the treatment of BPDCN in adults and in pediatric patients 2 years and older in December 2018, and by the European Medicines Agency for adult patients with newly diagnosed BPDCN in January 2021. Tagraxofusp has already been approved in over 40 countries worldwide.

The filing of NS-401 is based on the results of the domestic Phase I/II trial in BPDCN patients, which demonstrated efficacy and safety comparable to the overseas Phase I/II trials. NS-401 was designated as an orphan drug for BPDCN by MHLW on August 23, 2023.

We believe that by delivering NS-401 to patients as soon as possible, we can contribute to the treatment of BPDCN in Japan.

References

1. Pagano L et al, Blastic plasmacytoid dendritic cell neoplasm with leukemic presentation: an Italian multicenter study. Haematologica. 2013; 98: 239-246.
2. Lourdes Martín-Martín et al, Classification and clinical behavior of blastic plasmacytoid dendritic cell neoplasms according to their maturation-associated immunophenotypic profile. Oncotarget. 2015; 6: 19204–19216.

About CD123

A cell surface protein that is a receptor for interleukin-3 (IL-3), a substance found in the body, and is involved in the growth and differentiation of hematopoietic cells. In normal hematopoietic cells, CD123 expression is either absent or minimal, while CD123 is overexpressed in most BPDCN cells.

About Nippon Shinyaku Co., Ltd.

Based on Nippon Shinyaku's business philosophy, "Helping people lead healthier, happier lives," we aim to be an organization trusted by the community through creating unique medicines that will bring hope to patients and families suffering from illness. Please visit our website (https://www.nippon-shinyaku.co.jp/english/) for products or detailed information.

About Menarini Group

The Menarini Group is a leading international pharmaceutical and diagnostics company, with a turnover of over $4.7 billion and over 17,000 employees. Menarini is focused on therapeutic areas with high unmet needs with products for cardiology, oncology, pneumology, gastroenterology, infectious diseases, diabetology, inflammation, and analgesia. With 18 production sites and 9 Research and Development centers, Menarini's products are available in 140 countries worldwide. For further information, please visit www.menarini.com.

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