NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

August 28, 2023

Orphan Drug Designation granted to NS-401

Kyoto, Japan, August 28, 2023 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced that orphan drug designation to NS-401 (tagraxofusp) was granted by the Ministry of Health, Labour and Welfare (MHLW) for an expected indication of blastic plasmacytoid dendritic cell neoplasm (BPDCN) as of August 23. Nippon Shinyaku acquired a license of NS-401 from Menarini Group (HQs: Florence, CEO: Elcin Barker Ergun) on March 16, 2021.

An orphan drug is defined as the medicine to treat a disease less than 50,000 patients in Japan, with particularly high medical needs. Orphan Drug Designation can be expected to shorten the period required for regulatory approval in Japan by several months.

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 cells by inhibiting protein synthesis by specifically targeting CD123 expressing cancer cell. 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, and by the European Medicines Agency for adult patients with newly diagnosed BPDCN. At present, Nippon Shinyaku is conducting phase I/II study of NS-401 for patients with BPDCN in Japan.

Nippon Shinyaku dedicates to continue its best efforts in order to deliver the product to patients suffering from BPDCN in Japan as soon as possible.

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

CD123 is a receptor for interleukin-3 (IL-3), a biological substance, and a cell surface protein involved in the proliferation and differentiation of hematopoietic cells. CD123 is overexpressed in most cells of BPDCN, although normal hematopoietic cells have little or no expression of CD123.

About Nippon Shinyaku

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 $4.4 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.

Contact

Corporate Communications Dept., Nippon Shinyaku Co., Ltd.
FAX: +81-75-321-9128