NEWS RELEASE

NIPPON SHINYAKU CO., LTD.

May 30, 2022

Orphan Drug Designation granted to NS-87

KYOTO, Japan, May 30, 2022 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced that orphan drug designation to NS-87 (daunorubicin and cytarabine liposome injection) was granted by the Ministry of Health, Labour and Welfare (MHLW) for an expected indication of acute myeloid leukemia (AML). Nippon Shinyaku acquired a license of NS-87 from Jazz Pharmaceuticals plc (Headquarters: Dublin, Ireland, Chairman and CEO: Bruce C. Cozadd) on March 30, 2017.

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 by several months in Japan.

AML is a disease of clonal proliferation of immature myeloid cells, and results from genetic abnormalities in myeloblasts which are immature blood cells. The uncontrollable growth of leukemia cells in the bone marrow prevents the production of normal blood which results to various symptoms such as infections and bleeding.[1] Although multiple AML drugs have been launched in recent years and the outcomes for some patient groups have improved, many patients still do not have long-term survival. Novel therapeutic agents with greater efficacy are needed.[2]

NS-87 is approved in the U.S. (under the name of Vyxeos®*) by the U.S. Food and Drug Administration (FDA) for the treatment of newly-diagnosed therapy-related acute myeloid leukemia (t-AML) or AML with myelodysplasia-related changes (AML-MRC) in adults and pediatric patients one year and older.[3] It is also approved as Vyxeos® Liposomal by the European Medicines Agency (EMA) for the treatment of adults with newly diagnosed, t-AML or AML-MRC.[4]

In a Phase 3 study of adult patients with newly diagnosed high-risk/secondary AML, NS-87 demonstrated a statistically significant improvement in overall survival compared to the current conventional treatment group of 7+3 cytarabine and daunorubicin chemotherapy.[5]

At present, Nippon Shinyaku is currently conducting phase 1/2 study of NS-87 for patients with high-risk/ secondary AML in Japan.

Nippon Shinyaku is determined to continue our best efforts in order to deliver the product to patients suffering from high-risk/secondary AML in Japan as soon as possible.

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* A mark of the Jazz Pharmaceuticals Group

About NS-87 (daunorubicin and cytarabine liposome injection)

NS-87 is a liposomal formulation of a fixed combination of cytarabine and daunorubicin at a synergistic 5:1 molar ratio. The 5:1 molar ratio has been shown to maximise synergistic antitumour activity in AML. After the drug is taken up into leukemic cells in the bone-marrow, cytarabine and daunorubicin will be released to exert their antitumor effects.

About Nippon Shinyaku Co., Ltd.

Our mission is to help people lead healthier and happier lives. Through creating unique medicines that will bring hope to patients and families struggling with illness, we aim to be an organization trusted by the community. Please visit our website (https://www.nippon-shinyaku.co.jp/english/) for products or detailed information.

About Jazz Pharmaceuticals plc

Jazz Pharmaceuticals plc (NASDAQ: JAZZ) is a global biopharmaceutical company whose purpose is to innovate to help improve the lives of patients and their families. We are dedicated to developing potentially life-changing medicines for people with serious diseases – often with limited or no therapeutic options. We have a diverse portfolio of marketed medicines and novel product candidates, from early- to late-stage development, in neuroscience and oncology. Within these therapeutic areas, we are identifying new options for patients by actively exploring small molecules and biologics, and through innovative delivery technologies and cannabinoid science. Jazz is headquartered in Dublin, Ireland and has employees around the globe, serving patients in nearly 75 countries. For more information, please visit https://www.jazzpharma.com/ and follow @JazzPharma on Twitter.

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[1] Japanese Society of Hematology. Practical Guidelines for Hematological Malignancies, 2018 (revised version), Kanehara & Co., Ltd. (Japanese only)

[2] Gurnari C et al. Deciphering the Therapeutic Resistance in Acute Myeloid Leukemia. Int J Mol Sci. 2020; 21(22):8505.

[3] U.S. Food and Drug Administration. Vyxeos Prescribing Information. https://www.accessdata.fda.gov/drugsatfda_docs/label/2021/209401s006lbl.pdf. Published 2017. Updated March 2021. Accessed May, 2022.

[4] European Medicines Agency. Vyxeos Summary of Product Characteristics. https://www.ema.europa.eu/en/documents/product-information/vyxeos-liposomal-epar-product-information_en.pdf. Published August 2018. Accessed May, 2022.

[5] Lancet JE et al. CPX-351 (cytarabine and daunorubicin) Liposome for Injection versus Conventional Cytarabine Plus Daunorubicin in Older Patients with Newly Diagnosed Secondary Acute Myeloid Leukemia. J Clin Oncol. 2018; 36(26):2684-2692.

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