

April 18, 2025

FDA Grants Orphan Drug Designation to NS-229 for the Treatment of Eosinophilic Granulomatosis with Polyangiitis

Kyoto, Japan, April 18, 2025 - Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters, Kyoto; President, Toru Nakai) announced today that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to NS-229 which is being developed for the treatment of Eosinophilic Granulomatosis with Polyangiitis (EGPA).

The Orphan Drug Designation in the US supports the development and evaluation of new medicines that affect less than 200,000 people in the US. The Orphan Drug Designation provides NS-229 with seven years of exclusive marketing rights. NS-229 has already been granted as Orphan Drug Designation in January 2024 by the European commission.

EGPA is an intractable and rare autoimmune disease of etiology not fully understood.

Symptoms of bronchial asthma and allergic rhinitis generally precede the disease, followed by marked eosinophilia, with a development of eosinophilic inflammation, granulomatous lesions, and necrotizing vasculitis in various organs such as sinuses, peripheral nerves, skin, lungs, and kidneys. These in turn cause the symptoms and comorbidities such as fever, weight loss, arthritis, rhinosinusitis, peripheral neuropathy (e.g., numbness), purpura, pneumonia, and glomerulonephritis¹.

NS-229 is a selective JAK1 inhibitor developed in-house. NS-229 suppresses excessive activation of immune cells such as T cells, B cells, and eosinophils, leading to potential reduction of tissue damages, and expected to suppress various symptoms of EGPA.

A global Phase II study of NS-229 is being conducted in North America, Europe and Japan by Nippon Shinyaku and NS Pharma, Inc. (Headquarters: New Jersey, USA, President: Yukiteru Sugiyama).

Nippon Shinyaku is committed to develop potential medicines for the treatment of intractable and rare diseases, aiming to offer a useful therapy to improve the quality of life for people with EGPA.

References

1. Emmi, G., Bettiol, A., Gelain, E. et al. Evidence-Based Guideline for the diagnosis and management of eosinophilic granulomatosis with polyangiitis. *Nat Rev Rheumatol* 2023;19(6):378-393. doi:10.1038/s41584-023-00958-w

Contact

Corporate Communications Dept., Nippon Shinyaku Co., Ltd.

e_mail_kouhou@po.nippon-shinyaku.co.jp