



Chugai Receives Orphan Drug Designation for Satralizumab in Neuromyelitis Optica and Neuromyelitis Optica Spectrum Disorder from the Ministry of Health, Labour and Welfare

- Orphan drug designation was granted based on the results from two global phase III clinical studies (SakuraStar Study and SAKuraSky Study) which showed efficacy and safety either in monotherapy or add-on therapy to baseline treatment.

TOKYO, Sep 13, 2019 -- [Chugai Pharmaceutical Co., Ltd.](#) (TOKYO: 4519) announced that satralizumab (development code: SA237), a pH-dependent binding humanized IL-6 receptor monoclonal antibody under development received orphan drug designation by the Ministry of Health, Labour and Welfare (MHLW) for neuromyelitis optica (NMO) and neuromyelitis optica spectrum disorder (NMOSD). This designation is based on two global phase III clinical studies (SakuraStar Study and SAKuraSky Study), which showed efficacy and safety either in monotherapy or add-on therapy to baseline treatment.

“NMOSD including NMO, one of the intractable diseases designated by MHLW, are autoimmune diseases with limited treatment options and high unmet medical needs. With our proprietary antibody recycling technologies, satralizumab is designed to effectively inhibit IL-6 signal transduction that is deeply related to the pathology of the diseases,” said Dr. Yasushi Ito, Chugai’s Executive Vice President, Co-Head of Project & Lifecycle Management Unit. “We are working on the regulatory filings for satralizumab with confirmed effectiveness both in monotherapy and add-on to baseline treatment to deliver it to patients as soon as possible.”

<Reference>

- SAKuraSky study

Chugai Presents Results from Phase III Study of Satralizumab in NMOSD at ECTRIMS 2018 (October 15, 2018)

https://www.chugai-pharm.co.jp/english/news/detail/20181015120001_561.html

- SAKuraStar study

Chugai Presents Results from Second Positive Global Phase III Clinical Study of Satralizumab in NMOSD at ECTRIMS 2019 (Sep 12, 2019)

https://www.chugai-pharm.co.jp/english/news/detail/20190912140300_638.html

About satralizumab

Satralizumab, created by Chugai, is a pH-dependent binding humanized IL-6 receptor monoclonal antibody, applying its recycling antibody technology. The drug is expected to suppress relapse of NMOSD by inhibiting IL-6 transduction which is deeply related to the pathology. In two global phase III clinical studies in NMOSD patients, the primary endpoint was achieved with satralizumab either as an add-on

therapy to baseline treatment (NCT02028884) or as monotherapy (NCT02073279). Satralizumab is designated as an orphan drug in the U.S. and Europe. In addition, it has been granted Breakthrough Therapy Designation by the U.S. Food and Drug Administration in December 2018.

About neuromyelitis optica spectrum disorder (NMOSD)

NMOSD is a rare, lifelong, and debilitating autoimmune disease of the central nervous system (CNS) characterized by inflammatory lesions in the optic nerves and spinal cord. Patients with NMOSD frequently experience a relapsing disease course with repeated attacks leading to accumulating neurological damage and disability. Symptoms may include visual impairment, motor disability, and loss of quality of life. In some cases, attacks of NMOSD result in death. NMOSD is commonly associated with pathogenic antibodies (AQP4-IgG) that target and damage a specific central nervous cell type, called astrocytes resulting in inflammatory lesions of the optic nerve(s), spinal cord and brain. AQP4-IgG are detectable in the blood serum of around two-thirds of NMOSD patients¹⁻⁴. The inflammatory cytokine IL-6 is now emerging as an important factor in NMOSD pathogenesis⁵⁻⁸.

Diagnostic criteria introduced in 2006 for NMO were characterized by inflammation of the optic nerve (optic neuritis) and the spinal cord (myelitis). These were revised in 2007 with the definition of NMOSD, proposed for diseases with either optic neuritis or myelitis. In 2015, the definition of NMOSD further revised to include a broader spectrum of disease. The diagnostic term NMOSD is now widely used⁹.

About orphan drugs

Based on Pharmaceuticals and Medical Devices Law, orphan drugs are designated by the Minister of Health, Labour and Welfare and granted priority review. The designation criteria are as follows: The number of patients who may use the drug is less than 50,000 in Japan; The drug is indicated for the treatment of serious diseases and there is a significant medical value such as no alternative appropriate drug or treatment, or high efficacy or safety expected compared to existing products; there is a theoretical rationale for using the product for the targeted disease and the development plan is reasonable.

Sources

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