ForPatients
by Roche

Neuromyelitis optica spectrum disorder (NMOSD)

A study to find out how safe and effective satralizumab is in Chinese people with neuromyelitis optica spectrum disorder when used alone or with their existing treatment

Trial Status	Trial Runs In	Trial Identifier
Active, not recruiting	1 Country	YN42131 CTR20220584

The information is taken directly from public registry websites such as ClinicalTrials.gov, EuClinicalTrials.eu, ISRCTN.com, etc., and has not been edited.

Official Title:

A multicenter, single-arm, open-label, monotherapy or addition-to-baseline treatment, post-authorization study to observe the efficacy, safety, pharmacodynamics, and pharmacokinetics of satralizumab in Chinese participants with aquaporin-4 (AQP4) antibody positive neuromyelitis optica spectrum disorder (NMOSD)

Trial Summary:

The main purpose of this study was to see how well satralizumab worked, how safe it was, and how the body processed it when used alone or added to existing treatment in Chinese people with AQP4 antibody-positive NMOSD. This study helped researchers learn more about how satralizumab works in Chinese people with a rare condition called AQP4 antibody-positive NMOSD.

The study showed that satralizumab reduced relapses Chinese participants with AQP4 antibody-positive NMOSD.

The results also showed that satralizumab was well tolerated in most participants, with no serious unwanted effects related to the treatment.

F. Hoffmann-La Roche Ltd., Switzerland	**IV**
Sponsor	Phase

YN42131 CTR20220584
Trial Identifiers

Eligibility Criteria:

Gender	**Age**	**Healthy Volunteers**

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Males and females	12 to 74 years	No

Inclusion Criteria:

- Chinese patients located in China, who must be diagnosed as having AQP4 antibody-positive NMOSD as defined by the International Panel for Neuromyelitis Optica Diagnosis (Wingerchuk 2015).
    - At least 1 core clinical characteristic
    - Documented history of positive AQP4-IgG test with confirmation by an established assay for anti-AQP4 antibodies at screening
    - Exclusion of alternative diagnoses
      Core clinical characteristics:
    - Optic neuritis
    - Area postrema syndrome: episode of otherwise unexplained hiccups or nausea and vomiting
    - Acute brainstem syndrome
    - Symptomatic narcolepsy or acute diencephalic clinical syndrome with NMOSD-typical diencephalic magnetic resonance imaging (MRI) lesions
    - Symptomatic cerebral syndrome with NMOSD-typical brain lesions
- Clinical evidence of at least one documented relapse (including first attack) in the last 12 months prior to screening
- EDSS score from 0 to 6.5 inclusive at screening
- Age 12 to 74 years, inclusive at the time of informed consent
- For patients who are treated with baseline treatment, one of the following baseline treatments must be at stable dose as a monotherapy for 8 weeks prior to baseline:
    - AZA
    - MMF
    - OCS
- For patients aged 12 to 17 years, either of the following baseline treatments for relapse prevention can be allowed:
    - AZA + OCS
    - MMF +OCS
- Ability and willingness to provide written informed consent and to comply with the requirements of the protocol
- For women of childbearing potential: agreement to remain abstinent (refrain from heterosexual intercourse) or use adequate contraception during the treatment period and for 3 months after the final dose of satralizumab.

A woman is considered to be of childbearing potential if she is postmenarchal, has not reached a postmenopausal state (# 12 continuous months of amenorrhea with no identified cause other than menopause), and is not permanently infertile as a result of surgery (i.e., removal of ovaries, fallopian tubes, and/or uterus) or another cause as determined by the investigator (e.g., Müllerian agenesis). Per this definition, a woman with a tubal ligation is considered to be of childbearing potential. The definition of childbearing potential may be adapted for alignment with local guidelines or regulations.

The following are examples of adequate contraceptive methods: bilateral tubal ligation; male sterilization; hormonal contraceptives; hormone-releasing intrauterine devices; copper intrauterine devices; male or female condom with or without spermicide; and cap, diaphragm, or sponge with spermicide.

The reliability of sexual abstinence should be evaluated in relation to the duration of the clinical trial and the preferred and usual lifestyle of the patient. Periodic abstinence (e.g., calendar, ovulation, symptothermal, or postovulation methods) and withdrawal are not adequate methods of contraception.

Exclusion Criteria:

- Pregnancy or breastfeeding, or intention of becoming pregnant during the study or within 3 months after the final dose of satralizumab.
- Women of childbearing potential must have a negative serum pregnancy test result at screening and negative urine pregnancy test prior to initiation of study drug
- Participants who have any surgical procedure (except for minor surgeries defined as procedures requiring only local anesthesia or conscious sedation and are done on an ambulatory/outpatient basis [e.g., toenail surgery, mole surgical excision, tooth extraction]) within 4 weeks prior to baseline
- Participants who are planning to have surgical procedure (except minor surgeries) during the study
- Evidence of other demyelinating disease or progressive multifocal leukoencephalopathy (PML)
- Evidence of serious uncontrolled concomitant diseases that may preclude patient participation, such as: other nervous system disease, cardiovascular disease, hematologic/hematopoiesis disease, respiratory disease, muscular disease, endocrine disease, renal/urologic disease, digestive system disease, congenital or acquired immunodeficiency
- Known active infection (excluding fungal infections of nail beds or caries dentium) within 4 weeks prior to baseline
- Positive screening test for hepatitis B (HBV) (defined as either of the following):
    - Positive hepatitis B surface antigen [HBsAg]
    - Positive total hepatitis B core antibody [total HBcAb] confirmed by a positive viral DNA
- Positive screening test for hepatitis C (HCV) (defined as positive HCV antibody and detectable HCV RNA)
- Participants with positive HCV antibody and undetectable HCV RNA 12 weeks after HCV treatment completion are eligible to participate in the study.
- History of drug or alcohol abuse within 1 year prior to baseline
- History of diverticulitis or concurrent severe gastrointestinal (GI) disorders (such as symptomatic diverticulosis) that, in the investigator’s opinion, may lead to increased risk of complications such as lower GI perforation
- Evidence of latent or active TB (excluding patients receiving chemoprophylaxis for latent TB infection). Refer to Appendix 2 for details on TB screening and treatment.
    - If a patient is positive for latent TB, then the patient must be treated with appropriate anti mycobacterial therapy for at least 4 weeks prior to initiating study treatment administration.
- Receipt of any live or live attenuated vaccine within 6 weeks prior to baseline
- History of malignancy within the last 5 years, including solid tumors, hematologic malignancies, and in situ carcinoma (except basal cell and squamous cell carcinomas of the skin, or in situ carcinoma of the cervix uteri that have been completely excised and cured)
- History of severe allergic reaction to a biologic agent (e.g., shock, anaphylactic reactions)
- Active suicidal ideation within 6 months prior to screening, or history of suicide attempt within 3 years prior to screening
- Any serious medical condition or abnormality in clinical laboratory tests that, in the investigator's judgment, precludes the patient's safe participation in and completion of the study
- Treatment with alemtuzumab, cyclophosphamide, IL-6 inhibitor therapy (e.g., tocilizumab), total body irradiation, stem cell therapy, or bone marrow transplantation at any time
- Treatment with anti-CD19 or CD20 depleting therapy within 6 months prior to baseline, or CD19 cell count below the lower limit of normal (LLN) of age-specific reference range at screening.

- Treatment with eculizumab, belimumab, natalizumab, teriflunomide, fumaric acid esters (such as dimethyl fumarate), interferons, glatiramer acetate within 6 months prior to baseline
- Use of sphingosine 1-phosphate (S1P) receptor modulators (fingolimod, siponimod or ozanimod) within 6 months prior to baseline
- Treatment with methotrexate within 8 weeks prior to baseline
- Any previous treatment with anti-CD4, cladribine, or mitoxantrone within 2 years prior to baseline
- Treatment with any investigational agent within 3 months prior to baseline
- Chronic IV immunoglobulin treatment (IV Ig), plasma exchange therapy (PLEX) within 4 weeks prior to baseline.
- Laboratory exclusion criteria include the following laboratory abnormalities at screening. If retest is conducted, the last value of retest before randomization must meet study criteria:
    - White blood cells < $3.0 \times 10^3/\mu L$
    - Absolute neutrophil count < $2.0 \times 10^3/\mu L$
    - Absolute lymphocyte count < $0.5 \times 10^3/\mu L$
    - Platelet count < 10 × 10#/µL
    - Aspartate aminotransferase or alanine aminotransferase > 1.5 times the upper limit of normal