ForPatients
*by Roche*

Granulomatosis With Polyangiitis

# A Phase IIa Study of Intravenous Rituximab in Pediatric Participants With Severe Granulomatosis With Polyangiitis (Wegener's) or Microscopic Polyangiitis

| Trial Status | Trial Runs In | Trial Identifier |
|---|---|---|
| Completed | 8 Countries | NCT01750697 2012-002062-13 WA25615 |

*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 Phase IIA, International, Multicenter, Open-label, Uncontrolled Study to Evaluate The Safety And Pharmacokinetics of 4 × 375 mg/m2 Intravenous Rituximab in Pediatric Patients With Severe Granulomatosis With Polyangiitis (Wegener's) or Microscopic Polyangiitis

## *Trial Summary:*

This Phase IIa international multicenter, open-label, uncontrolled study will evaluate the safety and pharmacokinetics of rituximab (MabThera/Rituxan) in pediatric participants with severe granulomatosis with polyangiitis (GPA) or microscopic polyangiitis (MPA). Participants will receive rituximab 375 milligrams per square meter (mg/m^2) intravenously (IV) on Days 1, 8, 15 and 22.

**Hoffmann-La Roche**
Sponsor

**Phase 2**
Phase

**NCT01750697 2012-002062-13 WA25615**
Trial Identifiers

## *Eligibility Criteria:*

| Gender | Age | Healthy Volunteers |
|---|---|---|
| All | #2 Years & # 17 Years | No |

## *Inclusion Criteria:*

- Diagnosis of GPA (EULAR/PRINTO/PRES 2008, Ankara criteria for childhood Wegener's granulomatosis) or diagnosis of MPA (according to the Chapel Hill Consensus Conference)

- Newly diagnosed participants or participants with relapsing disease according to the following definition:

The recurrence or new onset of potentially organ- or life-threatening disease (i.e. one or more major Birmingham Vasculitis Activity Score for Wegener's Granulomatosis [BVAS/WG] items or disease severe enough to require treatment with cyclophosphamide)

- For participants of reproductive potential (males and females), use of reliable means of contraception throughout the study participation
- For all eligible participants mandatory prophylactic treatment for Pneumocystis jirovecii infection

## *Exclusion Criteria:*

- Diagnosis of Churg-Strauss syndrome, as defined by the Chapel Hill Consensus Conference
- Limited disease that would not normally be treated with cyclophosphamide
- Severe disease requiring mechanical ventilation due to alveolar hemorrhage
- Requirement for plasmapheresis or dialysis at screening
- Incomplete recovery from recent surgery or less than (<) 12 weeks since surgery prior to baseline or planned within 24 weeks of baseline
- Lack of peripheral venous access
- Pregnancy or breast-feeding
- Evidence of other significant uncontrolled concomitant disease, or of disorder or condition that, in the investigator's opinion, would preclude or interfere with participation of participant
- Primary or secondary immunodeficiency (history of or currently active), including known history of human immunodeficiency virus (HIV) infection
- Evidence of active tuberculosis (participants receiving chemoprophylaxis for latent tuberculosis infection are eligible for the study)
- Known active infection of any kind (excluding fungal infections of nail beds), or any major episode of infection requiring hospitalization or treatment with IV anti-infective agents within 4 weeks of baseline or completion of oral anti-infective agents within 2 weeks prior to baseline. Entry into this study may be reconsidered once the infection has fully resolved
- History of deep space/tissue infection within 24 weeks prior to baseline
- History of serious recurrent or chronic infection
- History of cancer (except for basal cell and squamous cell carcinoma of the skin that have been excised and cured)
- Currently active alcohol or drug abuse or history of alcohol or drug abuse
- History of severe allergic or anaphylactic reaction to a biologic agent or known hypersensitivity to any component of rituximab or to murine proteins
- Treatment with rituximab or other biologic B cell-targeted therapy (e.g., anti- Cluster of Differentiation [CD] 19, anti-CD20, anti-CD22, or anti-B-lymphocyte stimulator [BLys]/B-cell activating factor [BAFF]) within 6 months prior to baseline visit
- Previous treatment with an anti-alpha 4 integrin antibody or co-stimulation modulator
- Previous treatment with other cell-depleting therapies, including, but not limited to, investigational agents (e.g., alemtuzumab, anti-CD4, anti-CD5, anti-CD3, and anti-CD11a)
- Receipt of oral or IV cyclophosphamide within the previous 4 months prior to the baseline visit
- Receipt of infliximab within 3 months, adalimumab within 2 months or etanercept within 1 month prior to the baseline visit
- Treatment with any investigational agent within 28 days of baseline or 5 half-lives of the investigational drug (whichever is longer)
- Receipt of any live attenuated vaccine within 28 days prior to baseline
- Intolerance or contraindications to IV glucocorticoids

- Positive serum human chorionic gonadotropin measured at screening or a positive pregnancy test prior to the first rituximab infusion for participants of childbearing potential
- Positive tests for hepatitis B surface antigen (HBsAg), hepatitis B core antibody (HBcAb), hepatitis B virus (HBV), or hepatitis C serology
- Level of Immunoglobulin (Ig) M below lower limit of normal of age-specific reference range
- Level of IgG below 5.65 milligram per milliliter
- Absolute neutrophil count < 1.5 × 10^3 per microliter and platelet count < 130 × 10^3 per microliter
- Estimated Glomerular Filtration Rate < 15 milliliter per minute per 1.73 m^2
- Alanine aminotransferase or aspartate aminotransferase levels greater than 2.5 times the upper limit of normal (for age and sex) that cannot be attributed to underlying granulomatosis with polyangiitis (GPA) or microscopic polyangiitis (MPA)