# A double-blind, randomized, multicenter, placebo-controlled, parallel-group study to evaluate the efficacy and safety of 0.5 mg fingolimod administered orally once daily versus placebo in patients with chronic inflammatory demyelinating polyradiculoneuropathy (CIDP)

Published: 19-07-2012
Last updated: 26-04-2024

The study is designed to evaluate the efficacy and safety of fingolimod in the treatment of CIDP compared with placebo. Data from this study will be used to support the registration of fingolimod in the indication of CIDP.

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|---|---|
| **Ethical review** | Approved WMO |
| **Status** | Recruitment stopped |
| **Health condition type** | Demyelinating disorders |
| **Study type** | Interventional |

## Summary

### ID

NL-OMON41319

**Source**
ToetsingOnline

**Brief title**
NVS CFTY720I2201

### Condition

- Demyelinating disorders

**Synonym**
CIDP

**Research involving**
Human

## Sponsors and support

**Primary sponsor:** Novartis
**Source(s) of monetary or material Support:** Industrie

## Intervention

**Keyword:** CIDP, Fingolimod, Orally, Placebo

## Outcome measures

### Primary outcome

The primary objective of this study is to evaluate the effect of fingolimod 0.5 mg daily compared with placebo on delaying disability progression, in patients with CIDP, measured by the time to the first confirmed worsening on the adjusted Inflammatory Neuropathy Cause and Treatment (INCAT) Disability Scale. A confirmed worsening is defined as an increase by 1 point or more on the adjusted INCAT Disability Scale from the value at Baseline.

### Secondary outcome

Key Secondary Objectives

* To evaluate safety and tolerability of fingolimod compared with placebo in patients with CIDP as measured by adverse events (AE) and serious adverse events (SAEs), hematology and biochemistry laboratory tests, vital signs, electrocardiogram (ECG), and pulmonary function tests
* To assess the change in grip strength from Baseline to Month 6/End-of-Treatment (whichever occurs first) in CIDP patients on fingolimod as compared with those on placebo
* To assess the change in Rasch-Built Linearly Weighted Overall Disability

Scale (R-ODS) from Baseline to Month 6/End-of-Treatment (whichever occurs

first) in CIDP patients on fingolimod as compared with those on placebo

# Study description

### Background summary

Fingolimod is an oral once-a-day study drug that has been approved in over 40 countries for the treatment of relapsing MS but has not been approved yet for the treatment of patients with CIDP. Therefore, Fingolimod is not available for doctors to prescribe for CIDP; it is an investigational drug. Fingolimod acts on certain types of white blood cells (lymphocytes) responsible for immune reactions. It makes a proportion of these cells move away from areas of inflammation (a concentration of white blood cells around tissue injury) and redirects them towards lymph nodes and other places in the body where they rest. These cells are believed to play an important role in the inflammation process associated with MS and CIDP.
The main purpose of this study is to determine if Fingolimod works for the treatment of CIDP and if it is safe for CIDP patients. Information from this study may be used to support the registration of Fingolimod for use in treating CIDP if it proves to be effective.

### Study objective

The study is designed to evaluate the efficacy and safety of fingolimod in the treatment of CIDP compared with placebo. Data from this study will be used to support the registration of fingolimod in the indication of CIDP.

### Study design

This study is a double-blind, randomized, multicenter, placebocontrolled, parallel-group study in patients with a confirmed diagnosis of CIDP and treated with IVIg, corticosteroids, or both therapies prior to study entry. Patients meeting the eligibility criteria will be randomly assigned in a ratio of 1:1 to receive oral fingolimod (0.5 mg/day) or matching placebo.
The study will consist of 3 periods: a Screening Period (lasting for up to 45 days), a Double-blind Treatment Period (variable duration based on pre-specified rules, but not exceeding 4 years and 6 months after study initiation), and a Follow-up Period after discontinuation of study drug treatment. Patients who complete the study will have an option to enter an extension study.
The duration of this study is flexible, based on pre-specified rules. Under the current assumption the trial duration is anticipated to be two years from the

study initiation but should not exceed approximately 4 years and 6 months.

### Intervention

* fingolimod 0,5 mg (1 capsule a day);
* placebo (1 capsule a day)

### Study burden and risks

The additional assessments/test related to this medical scientific research study are:
* Electrocardiogram/holter
* Opthalmologic exam and OCT
* Pulmonary function tests
* Dermatology test
* Completion of Questionnaires (R-ODS, C-SSRS, SF-36 , TSQM)
* Laboratory evaluations
* Pregnancy test
Some procedures as Completion of Questionnaires and Laboratory evaluations are part of routine care but may be conducted more often for this study.

The more commonly occurring side effects are listed below.
Very common:
* Infection from flu virus with symptoms such as tiredness, chills, sore throat, joint or muscles aching, fever
* Headache, Diarrhea, Back pain, Cough
* Rise of liver function test results (eg, alanine transaminase)

## Contacts

**Public**
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**Scientific**
Novartis

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CH

# Trial sites

## Listed location countries

Netherlands

# Eligibility criteria

**Age**
Adults (18-64 years)
Elderly (65 years and older)

## Inclusion criteria

Inclusion Criteria
1. Written informed consent must be obtained before any assessment is performed.
2.The diagnosis of CIDP will use the definition of the European Federation of Neurological Societies/Peripheral Nerve Society (EFNS/PNS) Task Force First Revision. Patients must either have a clinical diagnosis of CIPD fulfilling the clinical inclusion criteria for typical CIPD or one of the following forms of atypical CIDP:
* pure motor, or
* asymmetrical MADSAM (Lewis-Sumner syndrome), or IgA or IgG (not IgM) MGUS paraprotein associated.
All patients must also fulfill the clinical exclusion criteria and the definate electrodiagnostic criteria of the EFNS/PNS Task Force First Revisiopn (Van den Bergh et al 2010 and Erratum 2011)
3. Disability defined by an INCAT Disability Scale score of 1-9 or, if INCAT score is 0, a documented history of disability sufficient to require treatment within the past 2 years following reduction or interruption of CIDP treatment
4. Receiving IVIg treatment (minimal dose equivalent to 0.4 g/kg every 4 weeks for a minimum of 12 weeks) or corticosteroids (minimal dose equivalent to prednisone 10 mg/day) treatment prior to the screening visit.
5. History of documented clinically meaningful deterioration confirmed by clinical examination, during therapy or upon interruption or reduction of therapy within 18 months prior to Screening.
6. Stable CIDP symptoms without significant change in treatment regimen for the 6 weeks before randomization.
7. male or female, aged 18 years or older at Screening

## Exclusion criteria

Exclusion Criteria
1. other chronic demyelinating neuropathies, including: * Distal Acquired Demyelinating Symmetric Neuropathy (DADS) * Multifocal Motor Neuropathy (MMN) * pure sensory CIDP * hematopoietic malignancy except for MGUS IgG or IgA.
2. conditions in which the pathogenesis of the neuropathy may be different from CIDP such as: Lyme disease, POEMS syndrome, osteosclerotic myeloma, Castleman*s disease
3. treatment with: * plasma exchange within 2 months of randomization * immunosuppressive/chemotherapeutic medications: * azathioprine, cyclophosphamide, cyclosporine, mycophenolate, etanercept, methotrexate, tacrolimus or other immunosuppressive drugs within 6 months of randomization or 5 half - lives (whichever is later) * Rituximab in the 2 years prior to randomization: patients that have received rituximab between 1 and 2 years prior to randomization should have B-cell (CD19/CD20) testing performed and if values are within normal range, patients are eligible to participate * other cytotoxic immunosuppressive medications with substained effects (including mitoxantrone, alemtuzumab, cladribine) at any time * hematopoietic stem cell transplantation at any time
4. a CIDP relapse or significant worsening of symptoms within 2 months of randomization.

# Study design

## Design

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| Study phase: | 2 |
| Study type: | Interventional |
| Intervention model: | Parallel |
| Allocation: | Randomized controlled trial |
| Masking: | Double blinded (masking used) |
| Control: | Placebo |
| Primary purpose: | Treatment |

## Recruitment

NL

| | |
|---|---|
| Recruitment status: | Recruitment stopped |
| Start date (anticipated): | 11-06-2013 |
| Enrollment: | 9 |
| Type: | Actual |

### Medical products/devices used

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|---|---|
| Product type: | Medicine |
| Brand name: | Fingolimod |
| Generic name: | - |
| Registration: | Yes - NL outside intended use |

## Ethics review

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|---|---|
| Approved WMO Date: | 19-07-2012 |
| Application type: | First submission |
| Review commission: | METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht) |
| Approved WMO Date: | 30-01-2013 |
| Application type: | First submission |
| Review commission: | METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht) |
| Approved WMO Date: | 23-08-2013 |
| Application type: | Amendment |
| Review commission: | METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht) |
| Approved WMO Date: | 19-09-2013 |
| Application type: | Amendment |
| Review commission: | METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht) |
| Approved WMO Date: | 08-11-2013 |
| Application type: | Amendment |
| Review commission: | METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht) |
| Approved WMO Date: | 10-12-2013 |
| Application type: | Amendment |

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| Review commission: | METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht) |
| Approved WMO Date: | 12-09-2014 |
| Application type: | Amendment |
| Review commission: | METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht) |
| Approved WMO Date: | 15-09-2014 |
| Application type: | Amendment |
| Review commission: | METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht) |
| Approved WMO Date: | 22-05-2015 |
| Application type: | Amendment |
| Review commission: | METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht) |
| Approved WMO Date: | 16-06-2015 |
| Application type: | Amendment |
| Review commission: | METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht) |
| Approved WMO Date: | 26-06-2015 |
| Application type: | Amendment |
| Review commission: | METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht) |

# Study registrations

## Followed up by the following (possibly more current) registration

No registrations found.

## Other (possibly less up-to-date) registrations in this register

No registrations found.

## In other registers

| Register | ID |
|---|---|
| EudraCT | EUCTR2011-005280-24-NL |
| ClinicalTrials.gov | NCT01625182 |
| CCMO | NL41107.068.12 |