An Open-Label, Multi-Center Clinical Trial of Eculizumab in Adult Patients with Atypical Hemolytic-Uremic Syndrome

Published: 05-11-2010
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Assess the efficacy of eculizumab in adult patients with aHUS to control TMA as characterized by thrombocytopenia, hemolysis and renal impairment.

Ethical review	Approved WMO
Status	Will not start
Health condition type	Haemolyses and related conditions
Study type	Interventional

Summary

ID

NL-OMON34263

Source
ToetsingOnline

Brief title
C10-004

Condition

- Haemolyses and related conditions
- Immune disorders NEC
- Nephropathies

Synonym
atypical hemolytic-uremic syndrome

Research involving
Human

Sponsors and support

Primary sponsor: Alexion Pharmaceuticals
Source(s) of monetary or material Support: Alexion Pharmaceutical Inc.

Intervention

Keyword: Atypical Hemolytic-Uremic Syndrome, Eculizumab

Outcome measures

Primary outcome

The proportion of patients with aHUS with complete TMA response.

Secondary outcome

Safety and efficacy.

Study description

Background summary

Atypical hemolytic uremic syndrome (aHUS) is a very rare disease, with an estimated incidence of only approximately 3 per million in children less than 18 years of age (1). The prevalence in adults is believed to be even lower. Most patients afflicted with aHUS develop the disease before 10 years of age, but initial presentations may also occur in patients between 20-40 years of age (2). All patients with aHUS exhibit
evidence of uncontrolled complement activation with resulting pro-thrombotic and pro-inflammatory perturbations. About 50-60% of aHUS cases have known aberrations in complement regulatory proteinseither mutations or antibodies (3). The development of thrombotic microangiopathy (TMA) in patients with aHUS represents a pro-coagulant state and is presumed due to uncontrolled terminal complement
activation that occurs secondary to predominantly proximal alternative complement pathway activation (4;5). Current treatment for patients with aHUS is considered inadequate. Eculizumab is a terminal complement inhibitor and its mechanism of action suggests possible utility in the treatment of this disease.

Study objective

Assess the efficacy of eculizumab in adult patients with aHUS to control TMA as characterized by thrombocytopenia, hemolysis and renal impairment.

Study design

This is an open-label, non-randomized, single-arm multi-center clinical trial

of eculizumab in patients *18 years with aHUS.

Intervention

Eculizumab 600 mg, 900 mg or 1200 mg will be administered intravenously.

Study burden and risks

See section E9.

Contacts

Public
Alexion Pharma International Sàrl

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

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

Inclusion criteria

1. Patient must be willing and able to give written informed consent.
2. Patient*s age * 18 years.
3. Patients exhibit thrombocytopenia, hemolysis and elevated Serum Creatinine

Exclusion criteria

1. Chronic dialysis
2. Prior eculizumab use or hypersensitivity to eculizumab

Study design

Design

Study phase:	2
Study type:	Interventional
Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Will not start
Enrollment:	8
Type:	Anticipated

Medical products/devices used

Product type:	Medicine
Brand name:	SOLIRIS®
Generic name:	Eculizumab
Registration:	Yes - NL outside intended use

Ethics review

Approved WMO

Date:	05-11-2010
Application type:	First submission
Review commission:	METC Leids Universitair Medisch Centrum (Leiden)
Approved WMO Date:	04-03-2011
Application type:	First submission
Review commission:	METC Leids Universitair Medisch Centrum (Leiden)
Approved WMO Date:	28-06-2011
Application type:	Amendment
Review commission:	METC Leids Universitair Medisch Centrum (Leiden)
Approved WMO Date:	22-10-2013
Application type:	Amendment
Review commission:	METC Leids Universitair Medisch Centrum (Leiden)

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	EUCTR2010-020326-18-NL
ClinicalTrials.gov	NCT01194973
CCMO	NL33539.058.10