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

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

Ethical review	Approved WMO
Status	Pending
Health condition type	Haemolyses and related conditions
Study type	Interventional

Summary

ID

NL-OMON34260

Source
ToetsingOnline

Brief title
C10-003

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 Pharmaceuticals

Intervention

Keyword: Atypical Hemolytic Uremic Syndrome, Eculizumab, Pediatric patients

Outcome measures

Primary outcome

Efficacy and safety.

Secondary outcome

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 proteins-either 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 and safety of eculizumab in pediatric 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 pediatric patients (1 month up to 18 years) with body weight

of >= 5kg) with aHUS.

Intervention

Eculizumab. Fixed dosing is based on body weight cohorts. Adjustment of dose to accommodated patient growth is possible.

Study burden and risks

See section E9.

Contacts

Public
Alexion Pharmaceuticals

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Children (2-11 years)

Inclusion criteria

1. Patient*s parent/legal guardian must be willing and able to give written informed consent and the patient must be willing to give written informed assent [if applicable as determined by the central Institutional Review Boards/Independent Ethics Committees (IRB/IEC)].
2. Pediatric patients with aHUS. Patients may be newly diagnosed, or with previously diagnosed disease, or post-kidney transplant with the disease.
3. Patients from 1 month up to 18 years of age and body weight >= 5 kg.
4. Patients exhibit Thrombocytopenia, hemolysis and elevated Serum Creatinine.

Exclusion criteria

1. Plasma Therapy for > 5 weeks prior to enrollment.
2. Chronic dialysis (defined as dialysis on a regular basis as renal replacement therapy for end stage renal disease).
3. Prior eculizumab use or hypersensitivity to eculizumab, to murine proteins or to one of the excipients.

Study design

Design

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

Recruitment

NL

Recruitment status:	Pending
Start date (anticipated):	20-10-2010
Enrollment:	2
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:	21-12-2010
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	07-07-2011
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	22-10-2013
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)

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	EUCTR2010-020310-28-NL
ClinicalTrials.gov	NCT01193348
CCMO	NL33553.091.10