National observational study to monitor the new guideline concerning treatment of atypical hemolytic uremic syndrome

Published: 15-06-2016
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Monitoring and evaluation of the Dutch guideline for treatment of aHUS in children and adults during two years.

Ethical review	Approved WMO
Status	Recruiting
Health condition type	Immune system disorders congenital
Study type	Observational invasive

Summary

ID

NL-OMON44007

Source
ToetsingOnline

Brief title
CUREiHUS

Condition

- Immune system disorders congenital
- Nephropathies

Synonym
acute kidney failure, complement mediated hemolytic uremic syndrome

Research involving
Human

Sponsors and support

Primary sponsor: Radboud Universitair Medisch Centrum
Source(s) of monetary or material Support: ZonMw,Betaalbaar Beter Radboudumc;Alliantie met VGZ zorgverzekeraar

Intervention

Keyword: Atypical hemolytic uremic syndrome, Complement system, Eculizumab, Personalized therapy

Outcome measures

Primary outcome

During four years the new guideline will be monitored and evaluation will take place after these two years. With the new guideline we aim to demonstrate that dosing regimens can be safely adapted or even discontinued, and hereby optimizing the treatment of aHUS patients. According the new guideline, patients will be treated with eculizumab during three months (six-nine gifts) and after this period therapy will be adjusted or discontinued. Clinical and laboratorial data of the patients will be evaluated by the national Working Group aHUS-eculizumab. Data are assembled in an online webbased database, Castor.

Secondary outcome

Two add-on studies are submitted with this research protocol.

1. To gather more inside in the pharmacokinetics and dynamics of eculizumab.

2. To test the psychometric properties of the questionnaire: medication-related patient-reported experience measure (PREM) that is aimed to better understand patient experiences with (expensive) medications.

Study description

Background summary

The hemolytic uremic syndrome (HUS) is a rare, but severe thrombotic

microangiopathy (TMA), that is characterized by the trias hemolytic anemia, thrombocytopenia, and acute renal failure. Atypical HUS (aHUS), an ultra orphan disease, is seen in 5-10% of all HUS cases (estimated prevalence 1-9/1.000.000 cases per year in the Netherlands), occurs at any age and has a very poor outcome: mortality in the acute phase of the disease is 2-10% and up to 50% of patients will develop end stage renal disease. Since end 2012, there is a new drug, named eculizumab, available for the treatment of aHUS. Eculizumab is a monoclonal antibody against C5 and subsequently inhibits formation of the terminal complement complex . With the use of Eculizumab, the outcome perspectives have drastically improved for patients with aHUS. However, this drug is very expensive and may cost up to ¤500.000 per adult patient per year when following the dosing regimen of the European Medicines Agency. Recently a new guideline concerning therapy in aHUS patients and hereby addressing also the adjustment and/or discontinuation of eculizumab in aHUS is implemented in the Netherlands. This enables the physician to adapt the treatment and hereby the possibility of individualized and personalized therapy.

Study objective

Monitoring and evaluation of the Dutch guideline for treatment of aHUS in children and adults during two years.

Study design

Multicentre, prospective, observational, cohort study.

Study burden and risks

This observational cohort study, for monitoring and evaluation of effectiveness of the new guideline for treatment of aHUS, does not include risks for the participants. For all add-on studies (invasive venapuncture and/or questionnaire) informed consent is gathered separately. Atypical HUS is a very rare disease with up to 10-15 new aHUS patients each year of which 3-5 children. In this study, the possibility of personalized therapy is investigated which makes a substantial difference for the treatment with eculizumab of these same patients in the future.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Children (2-11 years)
Elderly (65 years and older)

Inclusion criteria

1. Patients of all ages, suspected of or diagnosed with aHUS
2. Treated conform the new Dutch guideline for aHUS.
3. Subject and/or his parents is able and willing to sign the Informed Consent before screening evaluations.

Exclusion criteria

1, Subject and/or his parents is not able or willing to sign the Informed Consent before start of the study.
2. Patients with other etiological forms of HUS than aHUS

Study design

Design

Study type: Observational invasive

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	10-08-2016
Enrollment:	50
Type:	Actual

Ethics review

Approved WMO Date:	15-06-2016
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	04-07-2016
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	26-09-2016
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

ID: 22544
Source: Nationaal Trial Register
Title:

In other registers

Register	ID
CCMO	NL52817.091.15
OMON	NL-OMON22544