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

No registrations found.

Ethical review	Positive opinion
Status	Pending
Health condition type	-
Study type	Observational non invasive

Summary

ID

NL-OMON22544

Source
Nationaal Trial Register

Brief title
CUREiHUS

Health condition

atypische hemolytisch uremisch syndroom, atypical hemolytic uremic syndrome, complement system, eculizumab, restrictive treatment regimen, orphan drugs, weesgeneesmiddel

Sponsors and support

Primary sponsor: Radboud university medical center
Source(s) of monetary or material Support: ZonMw, Zorgverzekeraars Nederland, Goed Gebruik Geneesmiddelen (project number 836031008)

Intervention

Outcome measures

Primary outcome

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

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 measures (M-PREM) and the expectations measure that are aimed to better understand patient expectations, experiences and satisfaction with medications.

Study description

Background summary

Atypical hemolytic uremic syndrome (aHUS) is a rare, but severe form of thrombotic

microangiopathy, and is considered the consequence of complement dysregulation. Atypical HUS has

a poor outcome with mortality up to 10% and over 50% of patients developing end stage renal

disease. Since the end of 2012, these outcomes have greatly improved with the introduction of

eculizumab.

The European Medicines Agency has approved eculizumab for the treatment of aHUS

patients. The guideline advocates lifelong treatment. However, there is no hard evidence to support

this advice. Historically, a substantial number of aHUS patients were weaned of plasma therapy,

often without disease recurrence. Moreover, the long-term consequences of eculizumab treatment

are unknown.

Recently, a new guideline concerning therapy in aHUS patients and hereby addressing

therapy adjustment and/or discontinuation in aHUS patients is implemented in the

Netherlands. In

this national, multicenter, observational study we will include all aHUS, both pediatric and adults,

patients who are treated conform this new guideline. During four years this guideline will be

monitored and evaluated.

Study objective

-

Study design

Observational study for two years

Intervention

None

Contacts

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Eligibility criteria

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

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 non invasive
Intervention model:	Other
Allocation:	Non controlled trial

Control: N/A , unknown

Recruitment

NL

Recruitment status:	Pending
Start date (anticipated):	01-08-2016
Enrollment:	50
Type:	Anticipated

IPD sharing statement

Plan to share IPD: Undecided

Ethics review

Positive opinion

Date:	08-07-2016
Application type:	First submission

Study registrations

Followed up by the following (possibly more current) registration

ID: 44007
Bron: ToetsingOnline
Titel:

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

No registrations found.

In other registers

Register	ID
NTR-new	NL5833
NTR-old	NTR5988
CCMO	NL52817.091.15
OMON	NL-OMON44007

Study results