Congenital hemolytic anemia: causes, symptoms and consequences

No registrations found.

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

Summary

ID

NL-OMON22083

Source
NTR

Brief title
ZEbRA-study

Health condition

congenital hemolytic anemia, anemia, hereditary hemolytic anemia, sickle cell disease, thalassemia, pyruvate kinase deficiency, G6PD, spherocytosis.
(Congenitale hemolytische anemie, anemie, sikkelcel ziekte, thalassemie, PKD, G6PD, sferocytose)

Sponsors and support

Primary sponsor: University Medical Center, Utrecht
Source(s) of monetary or material Support: University Medical Center, Utrecht

Intervention

Outcome measures

Primary outcome

To create insight in current disease burden by creating a descriptive cohort of patients, diagnosed with rare congenital hemolytic anemia.

Points of interest are:

- Prevalence and incidence of disease

- Quality of life

- Prevalence and incidence of iron overload

- Prevalence and incidence of comorbidities and related silent organ damage

- Prevalence and incidence of splenectomy and complications

Secondary outcome

To further analyze the pathophysiology of congenital hemolytic anemia: to perform a case control study comparing patient parameters and healthy control parameters.

Parameters of interest are:

1. The tolerability of low hemoglobin levels in rare congenital hemolytic anemia patients.

2. Patterns in laboratory parameters: pro-inflammatory profile, Red blood cell characteristics, microparticle analysis, and markers of coagulation activation.

3. RNA seq parameters for peripheral blood mononuclear cell transcriptome mapping using blood sample analysis and then compare and relate outcome to other results of the study.

Study description

Background summary

Rationale: Rare congenital hemolytic anemias share a common clinical picture and common pathophysiologic pathways such as iron overload, severe anemia and hemolysis. These patients develop comparable organ damage to patients with more common and more studied congenital hemoglobinopathies such as thalassemia and sickle cell disease. Treatment nowadays is mainly supportive. Research is necessary in order to find the best monitoring- and treatment regimens.
Objective: To create insight in current disease burden by creating a descriptive cohort of patients, diagnosed with rare congenital hemolytic anemia. To further analyze the pathophysiology of congenital hemolytic anemia by performing a case control study comparing patient parameters and healthy control parameters.
Study design: longitudinal observational descriptive cohort study and case-control study

Study population: All patients diagnosed with rare congenital hemolytic anemia. The majority

of this patient group will be composed of patients with hereditary red blood cell membranopathies and red blood cell enzyme disorders.

Main study parameters/endpoints:

Prevalence and incidence of disease

Quality of life

Prevalence and incidence of iron overload

Prevalence and incidence of comorbidities and related silent organ damage

Prevalence and incidence of splenectomy and complications

The study consist of medical chart review, yearly two short quality of live questionnaires, a 6 minute walking test and one additional venipuncture.

Study objective

Observational study. We aim to analyze the clinical consequences of congenital hemolytic anemia in order to treat and monitor patients optimally. Secondary, we aim to gain understanding in the pathophysiology of congenital hemolytic anemia

Study design

Enrollment, 1 year after enrollment, 2 years after enrollment

Intervention

not applicable

Contacts

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Eligibility criteria

Inclusion criteria

Adult patients who meet the criteria of non-immune mediated hemolytic anemia in whom acquired causes have been excluded in the diagnostic track. Such patients can be subdivided into 4 main categories:

1. red cell membrane disorders, e.g. hereditary spherocytosis

2. disorders of hemoglobin, e.g. thalassemia

3. metabolic disorders, e.g. pyruvate kinase deficiency

4. hemolytic anemia e.c.i.

Exclusion criteria

Inability to give informed consent

Study design

Design

Study type:	Observational non invasive
Intervention model:	Other
Allocation:	Non controlled trial
Masking:	Open (masking not used)

Control:	N/A , unknown

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	01-09-2015
Enrollment:	100
Type:	Anticipated

Ethics review

Positive opinion

Date:	30-07-2015
Application type:	First submission

Study registrations

Followed up by the following (possibly more current) registration

ID: 42799
Bron: ToetsingOnline
Titel:

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

No registrations found.

In other registers

Register	ID
NTR-new	NL5189
NTR-old	NTR5337
CCMO	NL53609.041.15
OMON	NL-OMON42799

Study results