Congenital hemolytic anemia: causes, symptoms and consequences

Gepubliceerd: 30-07-2015 Laatst bijgewerkt: 15-05-2024

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

Ethische beoordeling	Positief advies
Status	Werving gestart
Type aandoening	-
Onderzoekstype	Observationeel onderzoek, zonder invasieve metingen

Samenvatting

ID

NL-OMON22083

Bron
NTR

Verkorte titel
ZEbRA-study

Aandoening

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)

Ondersteuning

Primaire sponsor: University Medical Center, Utrecht
Overige ondersteuning: University Medical Center, Utrecht

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

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

Toelichting onderzoek

Achtergrond van het onderzoek

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.

Doel van het onderzoek

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

Onderzoeksopzet

Enrollment, 1 year after enrollment, 2 years after enrollment

Onderzoeksproduct en/of interventie

not applicable

Contactpersonen

Publiek

Heidelberglaan 100

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The Netherlands

Wetenschappelijk

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Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

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.

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

Inability to give informed consent

Onderzoeksopzet

Opzet

Type:	Observationeel onderzoek, zonder invasieve metingen
Onderzoeksmodel:	Anders
Toewijzing:	N.v.t. / één studie arm
Blindering:	Open / niet geblindeerd
Controle:	N.v.t. / onbekend

Deelname

Nederland

Status:	Werving gestart
(Verwachte) startdatum:	01-09-2015
Aantal proefpersonen:	100
Type:	Verwachte startdatum

Ethische beoordeling

Positief advies

Datum:	30-07-2015
Soort:	Eerste indiening

Registraties

Opgevolgd door onderstaande (mogelijk meer actuele) registratie

ID: 42799
Bron: ToetsingOnline
Titel:

Andere (mogelijk minder actuele) registraties in dit register

Geen registraties gevonden.

In overige registers

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

Resultaten