Screening of patients with Crigler-Najjar

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Screening of patients with the inherited liver disease Crigler-Najjar.

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Hepatobiliary disorders congenital
Study type	Observational invasive

Summary

ID

NL-OMON41149

Source
ToetsingOnline

Brief title
Screening of patients with Crigler-Najjar

Condition

- Hepatobiliary disorders congenital

Synonym
hereditary unconjugated hyperbilirubinemia/ hereditary jaundice

Research involving
Human

Sponsors and support

Primary sponsor: Academisch Medisch Centrum
Source(s) of monetary or material Support: ZonMW

Intervention

Keyword: Bilirubin, Crigler-Najjar, Liver pathology, Neutralizing antibodies

Outcome measures

Primary outcome

Serum:

- AAV8 antibodies

- Serum bilirubin (conjugated and unconjugated)

- Liver trasnaminases (AST and ALT)

- Serology Hepatitis A, B and C

Quality of life (questionnaire SF-36)

Extent of phototherapy (duration/intensity)

Ultrasound: liver, spleen and vena portae

Fibroscan: liver

Secondary outcome

non applicable

Study description

Background summary

Crigler-Najjar syndrome is arare, recessive inherited disorder caused by deficiency of uridine diphosphoglucuronosyl transferase (UGT1A1). UGT1A1 is an enzyme which catalyzes the glucuronidation of unconjugated bilirubin, an essential step in the excretion into bile of this neurotoxic compound. Patients with Crigler-Najjar suffer from severe unconjugated hyperbilirubinemia and are at risk of bilirubin encephalopathy. The standard treatment for Crigler-Najjar is phototherapy during early childhood and when later in life serum bilirubin levels start to increase above 400µmol/L a liver transplantation is performed. The extensive phototherapy and the side effects of life-long immune suppression warrant the development of liver directed gene therapy. Crigler-Najjar syndrome seems to be an attractive disorder for the development of liver directed gene therapy, because no liver damage is present. Before a phase I/II clinical trial with gene therapy in the treatment of Crigler-Najjar is started, a careful screening of patients with Crigler-Najjar syndrome should be performed to

determine which patients could be taken in consideration to participate in the up coming clinical trial. The screen will consist of serology on the presence of Hepatitis B and C and AAV8 antibodies, fibroscan, ultrasound of the abdomen and serum livertests.

Study objective

Screening of patients with the inherited liver disease Crigler-Najjar.

Study design

A visit to the outpatient clinic bloodsamples, questionaires, ultrasound and fibroscan will be performed.

Study burden and risks

The burden consists of only one visit to the hospital and one venapunction. The risk for the patients is very low.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

Patients with the inherited liver disorder Crigler-Najjar
With a known mutation
Able to understand and give fully informed written consent
Age between 18-75 year

Exclusion criteria

Crigler-Najjar patients who underwent a livertransplantation
Patients with jaundice/icterus caused by a different pathology other than Crigler-Najjar disease
Patients which are not able to understand and give fully informed written consent

Study design

Design

Study type: Observational invasive

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

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	28-01-2015
Enrollment:	30
Type:	Actual

Ethics review

Approved WMO Date:	24-12-2014
Application type:	First submission
Review commission:	METC Amsterdam UMC

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
CCMO	NL49630.018.14