Novel diagnostic strategies in children with sickle cell disease: detecting pulmonary hypertension; a pilot study

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1. To establish the prevalence of PH in our population of children with SCD by standardized echocardiography; 2. To investigate the predictive value of alveolar and bronchial FeNO for PH in SCD patients. 3. To establish (genetic) risk factors for PH…

Ethical review	Approved WMO
Status	Recruiting
Health condition type	Haemoglobinopathies
Study type	Observational non invasive

Summary

ID

NL-OMON31852

Source
ToetsingOnline

Brief title
Pulmonary hypertension and sickle cell disease

Condition

- Haemoglobinopathies

Synonym
HbSC), HbSS or compound heterozygous sickle cell disease (HbSbetathal, sickle cell disease

Research involving
Human

Sponsors and support

Primary sponsor: Erasmus MC, Universitair Medisch Centrum Rotterdam
Source(s) of monetary or material Support: Ministerie van OC&W

Intervention

Keyword: Nitric oxide (NO), Pulmonary hypertension, Sickle cell disease, Spirometry

Outcome measures

Primary outcome

Correlations between echocardiographic results (tricuspidalis Regrurgitant Jet Velocity Value; m/s) and FeNO at different expiratory flow velocities will be tested, in order to evaluate the predictive value of exhaled NO in diagnosis of PH.

Secondary outcome

Evaluation of possible (genetic) risk factors for pulmonatry hypertension in SCD

Study description

Background summary

As life expectancy improves in patients with hemoglobinopathies, including sickle cell disease (SCD), new chronic complications are emerging such as pulmonary hypertension. Pulmonary hypertension (PH) is a serious complication of SCD and is strongly associated with early mortality. Studies in adults report prevalences of 30-34%. Recently, similar percentages have been reported in children with SCD. Diagnostic methods to detect PH include: heart catheterisation and echocardiography. As nitric oxide (NO) depletion due to the chronic hemolysis in SCD is an important factor in the pathogenesis of PH in SCD, we hypothesized that measurement of exhaled NO (fraction of exhaled nitric oxide or FeNO), may play a complementary role in identifying patients at risk for PH. Sullivan et al. recently reported lower FeNO levels in children with SCD, but did not correlate this finding with PH.

Study objective

1. To establish the prevalence of PH in our population of children with SCD by standardized echocardiography;
2. To investigate the predictive value of alveolar and bronchial FeNO for PH in

SCD patients.
3. To establish (genetic) risk factors for PH in SCD (severity of hemolysis, level of fetal hemoglobin, level of methemoglobin, DNA analysis).

Study design

Observational pilot study (n = 20), if results prove the study feasible, a large prospective study will be initiated in our total patient cohort (n= 150), with an interim analysis after at least 50 patients. Spirometry and measurements of exhaled NO will be performed in our study group as has been extensively applicated in children in earlier studies (M.W.H. Pijnenburg; Protocol ID/ number: MEC212.505/2002/92, MEC203.288/2001/164, MEC194.549/2000/180).

Study burden and risks

In addition to the current annual diagnostic work-up (blood analysis, echocardiography, ophthalmological evaluation, trancranial doppler evaluation), spirometry and measurement of FeNO (duration of procedure: 30 minutes, non-invasive, neglible risk for patient) will take place during a regular follow-up visit to the outpatient clinic.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)

Inclusion criteria

Sickle cell patient, 12 to 18 years of age.

Exclusion criteria

Acutely or critically ill patients, recent bloodtransfusion (< 3 months prior), not able to understand Dutch adequately.

Study design

Design

Study type: Observational non invasive

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

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	17-03-2009
Enrollment:	20
Type:	Actual

Ethics review

Approved WMO

Date:	25-11-2008

Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)

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	NL23409.078.08