

N-AcetylCysteine in the treatment of Sickle Cell Disease

Submission date 23/08/2007	Recruitment status No longer recruiting	☒ Prospectively registered
		☐ Protocol
Registration date 23/08/2007	Overall study status Completed	☐ Statistical analysis plan
		☐ Results
Last Edited 08/09/2008	Condition category Haematological Disorders	☐ Individual participant data
		☐ Record updated in last year

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

Contact name
Dr B.J. Biemond

Contact details
Academic Medical Centre (AMC)
Department of Clinical Chemistry
P.O. Box 22660
Amsterdam
Netherlands
1100 DD
+31 (0)20 566 7391
b.j.biemond@amc.uva.nl

Additional identifiers

Protocol serial number
NTR1013

Study information

Scientific Title

Acronym

NAC in SCD

Study objectives

We hypothesise that treatment of sickle cell patients with N-acetylcysteine (NAC) results in reduced red cell phosphatidylserine (PS) exposure, reduced endothelial activation, increased nitric oxide (NO) availability, reduced coagulation activation and reduced inflammation detectable with specific laboratory testing, as well as a reduction of irreversibly sickled cells (ISC's) and Heinz Body formation.

Ethics approval required

Old ethics approval format

Ethics approval(s)

Ethics approval received from the local medical ethics committee

Primary study design

Interventional

Study design

Randomised, active controlled, parallel group trial

Study type(s)

Treatment

Health condition(s) or problem(s) studied

Sickle cell disease

Interventions

N-acetylcysteine 1200 mg or 2400 mg a day.

Intervention Type

Drug

Phase

Not Specified

Drug/device/biological/vaccine name(s)

N-acetylcysteine

Primary outcome(s)

Primary end-points are the effects of NAC on the laboratory markers (haemoglobin, red blood cell counts, reticulocyte counts, leukocyte counts and differentiation, platelet counts, erythrocyte sedimentation rate, a blood smear will be analysed microscopically for the number of ISC per field, as well as the number of Heinz bodies, intra-erythrocytic reduced glutathione [GSH] and oxidised glutathione [GSSG] levels, NO availability, SRBC phosphatidylserine [PS] exposure, annexin V, creatinine, blood-urea nitrogen [BUN], electrolytes, transaminase levels, albumin levels, lactate dehydrogenase [LDH], indirect bilirubin levels, free haemoglobin levels,

high sensitive C-reactive protein [hsCRP], vascular cell adhesion molecule-1 [sVCAM-1], endothelin [ET-1], interleukin-8 [IL-8], pro-thrombin fragments [F1.2], D-dimer levels, protein S [free and total] and C activity, Von Willebrand factor antigen [vWF-Ag] activity).

Key secondary outcome(s)

Tolerability of study medication (in this phase admittedly in a non-controlled fashion) at every visit by history taking and by scoring of a NAC for SCD check-list.

Completion date

31/12/2008

Eligibility

Key inclusion criteria

1. High performance liquid chromatography confirmed diagnosis of sickle cell anaemia (HbSS), sickle-haemoglobin C disease (HbSC) or sickle cell trait disease (HbSa) genotype
2. Aged 18 to 65 years
3. Written informed consent

Participant type(s)

Patient

Healthy volunteers allowed

No

Age group

Adult

Lower age limit

18 Years

Sex

Not Specified

Key exclusion criteria

1. Blood transfusion in the preceding four months
2. Pregnancy or the desire to get pregnant in the following seven months
3. Concomitant use of hydroxyurea, vitamin K antagonists or other oral anticoagulants, or contraindications for NAC
4. Impaired renal function of more than 60% (as assessed by the Cockcroft-Gault equation)
5. Known gastric or duodenal ulcer
6. Concomitant use of anti-hypertensives, sildenafil or nitrates

Date of first enrolment

01/10/2007

Date of final enrolment

31/12/2008

Locations

Countries of recruitment

Netherlands

Study participating centre

Academic Medical Centre (AMC)

Amsterdam

Netherlands

1100 DD

Sponsor information

Organisation

CURAMA Study Group (The Netherlands)

Funder(s)

Funder type

Research organisation

Funder Name

CURAMA Study Group (The Netherlands)

Results and Publications

Individual participant data (IPD) sharing plan**IPD sharing plan summary**

Not provided at time of registration