Sickle Cell Outcome REsearch

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To determine the natural history of sickle cell disease and to identify modifying factors, including: (epi)genetic, biological, pathophysiological, social/ demographic, psychological and therapeutic determinants, which contribute to morbidity and...

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

Summary

ID

NL-OMON55241

Source
ToetsingOnline

Brief title
SCORE

Condition

- Haemoglobinopathies
- Blood and lymphatic system disorders congenital

Synonym
Sickel cell Anemia, 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: Benign hematology, Cohort study, Outcome, Sickle cell disease

Outcome measures

Primary outcome

1. To describe the natural course of disease e.g. symptoms and complications, growth, psychosocial and neurocognitive development, socioeconomic and demographic characteristics of children and adults with SCD;

2. To identify (molecular) genetic and epigenetic, biological, demographic and psychological and therapeutic determinants for morbidity and mortality in children and adults with SCD;

3. To investigate the long-term effects of current and future therapies on SCD symptoms and complications, preservation of organ function, growth, psychosocial and neurocognitive development in children and adults with SCD.

4. To evaluate and improve aspects of care of children and adults with SCD by measurement of patient-reported outcomes (PROs) and patient reported experiences (PREs) and analyses of associations with health care outcome.

Secondary outcome

Not applicable.

Study description

Background summary

Sickle cell disease is an autosomal recessive, multisystem disorder, characterized by ongoing hemolytic anemia, painful ischemic episodes of vaso-occlusion, and progressive organ failure. Sickle cell disease is the most common monogenetic disease, with more than 20 million individuals affected worldwide. Estimates suggest that 300.000 infants are born annually with sickle cell disease and that this number may rise to 400.000 by 2050. Prevalence of sickle cell disease and sickle cell carriership is prominent throughout large areas of sub-Saharan Africa, Middle East, and India. However, migration from

these malaria-endemic regions to North America, Western Europe and Australia has concomitantly led to widespread distribution of the HbS allele. In the Netherlands, roughly 2000 individuals are affected, of which 1000 are children. Most sickle cell disease patients are originally from the Republic of Surinam, Asia or Africa, with a minority of Afro- Caribbean or Middle Eastern descent.

Comprehensive Care programs in the Western world have greatly extended life expectancy in sickle cell disease with almost all infants surviving into adulthood. Nevertheless, life expectancy of sickel cell disease patients is still 20 to 30 years shorter than average. Premature death is most commonly caused by chronic end-organ dysfunction in combination with infection or other comorbidities. In addition, both pediatric and adult sickle cell disease patients experience a significant decline in all domains of health-related quality of life (HRQoL) due to disease-related symptoms and complications when compared to controls. Currently, therapeutic options for sickle cell disease are still limited.Despite several prior sickle cell disease cohort studies, many unexplored areas remain. More optimal prediction of sickle cell disease severity will lead to more precise management and treatment and development of novel therapeutic options. It is therefore increasingly important to document initial patient characteristics, symptoms and complications, mortality, and treatment outcome as well as patient-reported outcomes (PROs), longitudinally and systematically in registries. In this manner, best treatment options for each individual at every time point can be identified.

Study objective

To determine the natural history of sickle cell disease and to identify modifying factors, including: (epi)genetic, biological, pathophysiological, social/ demographic, psychological and therapeutic determinants, which contribute to morbidity and mortality of the disease.

Study design

Long term retrospective and prospective observational cohort study of sickle cell disease patients in the Netherlands. Clinical data will be combined with outcome measures from the patients* perspective, both patient reported outcome measures and patient reported experience measures. Data will be collected retrospectively and prospectively at participants' half yearly regular clinic visits as part of standard care. The burden associated with participation is therefore limited to the collection of biological specimens (DNA, plasma and urine) at set time points (every other year) for decentral biobanking according to standard national protocol.

Study burden and risks

Not applicable.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Inclusion criteria

- A diagnosis of SCD of any genotype;
- Written patient (and in case of a patient <16 years of age, parental) informed consent.

Exclusion criteria

- Any medical or social reason, which obstructs or inhibits study participation according to treating physician
- Intrekking van (ouderlijke) toestemming

Study design

Design

Study type: Observational invasive

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

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	07-10-2019
Enrollment:	1000
Type:	Actual

Ethics review

Approved WMO Date:	13-09-2019
Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	05-12-2019
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	31-08-2021
Application type:	Amendment
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
Other	NL trialregister, nummer NL7873
CCMO	NL70024.078.19