Correcting mutations in vitro using CRISPR-Cas9; towards autologous stem cell transplantation in sickle cell disease and X-linked severe combined immunodeficiency

Published: 04-04-2016
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Preparation for in vivo correction of SCD and X-SCID causing mutations by CRISPR-Cas9 by in vitro studies in cell lines

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Red blood cell disorders
Study type	Observational invasive

Summary

ID

NL-OMON43429

Source
ToetsingOnline

Brief title
Correcting SCD and X-SCID causing mutations in vitro using CRISPR-Cas9

Condition

- Red blood cell disorders
- Immune system disorders congenital

Synonym
n.v.t.

Research involving
Human

Sponsors and support

Primary sponsor: Academisch Medisch Centrum
Source(s) of monetary or material Support: Ministerie van OC&W

Intervention

Keyword: CRISPR-CAS9, in vitro, sickle cell disease, X-linked severe combined immunodeficiency

Outcome measures

Primary outcome

Percentage of cells in which the SCD or X-SCID causing mutations are corrected

without detectable mutations in other genes

Secondary outcome

n.a.

Study description

Background summary

Sickle cell disease (SCD) and X-linked severe combined immunodeficiency (X-SCID) are both Mendelian, life threatening diseases that can only be cured by an allogeneic hematopoietic stem cell transplantation (HSCT). Gene editing would enable patients to receive an autologous instead of an allogeneic transplantation, with a concomitant reduction in morbidity and mortality.

Study objective

Preparation for in vivo correction of SCD and X-SCID causing mutations by CRISPR-Cas9 by in vitro studies in cell lines

Study design

Observational study with invasive measurements

Study burden and risks

The risk and burden associated with a single skin biopsy are negligible,

especially when taken during surgery using the surgical incision that already needs to be made for clinical care reasons. Gene editing would enable patients to receive an autologous instead of an allogeneic HSCT in the future, which may yield a group benefit.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Children (2-11 years)
Elderly (65 years and older)

Inclusion criteria

1. Presence of either SCD or X-SCID
2. For children: availability of an existing cell line or a planned surgical intervention for patient care reasons

3. Able to provide written permission

Exclusion criteria

none

Study design

Design

Study type: Observational invasive

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

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	04-05-2016
Enrollment:	6
Type:	Actual

Ethics review

Approved WMO Date:	04-04-2016
Application type:	First submission
Review commission:	METC Amsterdam UMC
Approved WMO Date:	09-12-2016
Application type:	Amendment
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	NL56205.018.16