From fibroblasts to stem cells to motorneurons in amyotrophic lateral sclerosis and related motor neuron diseases

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The two objectives of our study are:(i) to develop a disease model using stem cells, with which further research into the underlying disease mechanisms of ALS and future therapies can be done(ii) genetic modification of stem cells to correct or…

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

Summary

ID

NL-OMON37675

Source
ToetsingOnline

Brief title
Stem cells in amyotrophic lateral sclerosis

Condition

- Neuromuscular disorders

Synonym
Amyotrophic Lateral Sclerosis (ALS), motor neuron disease

Research involving
Human

Sponsors and support

Primary sponsor: Universitair Medisch Centrum Utrecht

Source(s) of monetary or material Support: Vici toekenning Prof. dr. L.H. van den Berg,ZONMw

Intervention

Keyword: ALS, amyotrophic lateral sclerosis, iPS, stem cell

Outcome measures

Primary outcome

A better understanding of the pathogenesis of ALS is essential for the development of future treatment options for patients suffering from ALS. By creating iPS-cells from fibroblasts we will develop cell lines which will be used for disease modeling, with which further research can be done into the pathogenesis of ALS.

Secondary outcome

nvt

Study description

Background summary

ALS is an adult-onset, disabling and fatal disease characterized by progressive degeneration of motor neurons in brain and spinal cord. No cure is available for ALS and the median survival is 3 years. There is no definitive diagnostic test available for ALS and other conditions can mimic ALS clinically. The diagnostic delay is often more than one year and misdiagnosis is also common. In approximately 10% of patients ALS occurs in families. Familial ALS is clinically and pathologically indistinguishable from sporadic ALS. The pathogenesis of ALS is unknown but there is convincing evidence that several molecular pathways play a role.

Study objective

The two objectives of our study are:
(i) to develop a disease model using stem cells, with which further research into the underlying disease mechanisms of ALS and future therapies can be done

(ii) genetic modification of stem cells to correct or induce mutations

Study design

We aim to use fibroblasts acquired from patients and healthy controls for reprogramming into stem cells and further development into a disease model. Through collaboration with Prof. dr. N. Geijsen we can use a special technique to reprogram fibroblasts into pluripotent stem cells (iPS-cells). Using these iPS-cells we will differentiate these into motor neurons and develop a disease model for ALS.
Further analysis will be performed regarding morphology, genetic background and molecular phenotype of different cell lines. Also we will genetically modify stem cells to repair or induce mutations. This study shall lead to a better understanding of the pathogenesis of ALS and possibly to future therapies.

Study burden and risks

Risks in participating in this study are the risks associated with undergoing a skin biopsy. This is a safe and frequently performed exercise with infrequent complications. The procedure is not time consuming.

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

For healthy controls:
- Without neurological disease or a disorder similar to ALS
- Age > 18 years;For patients:
- Diagnosis of ALS or other ALS-mimic disorder
- Age > 18 years

Exclusion criteria

For healthy controls:
- Neurological disease or a disorder similar to ALS
- Age < 18 years;For patients:
- Diagnosis unknown
- Age < 18 years

Study design

Design

Study type:	Observational invasive
Intervention model:	Other
Allocation:	Non-randomized controlled trial
Masking:	Open (masking not used)
Control:	Active
Primary purpose:	Basic science

Recruitment

NL
Recruitment status: Recruitment stopped

Start date (anticipated):	03-10-2012
Enrollment:	200
Type:	Actual

Ethics review

Approved WMO Date:	18-09-2012
Application type:	First submission
Review commission:	METC NedMec

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	NL39918.041.12