Enroll-HD: A Prospective Registry Study in a Global Huntington*s Disease Cohort

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Objective 1: To improve the understanding of the dynamic phenotypic spectrum and the disease mechanisms of HD by:a. collecting natural history data covering the cognitive, behavioral and motor domains permitting estimates of rates of progression in...

Ethical review	Approved WMO
Status	Recruiting
Health condition type	Movement disorders (incl parkinsonism)
Study type	Observational invasive

Summary

ID

NL-OMON55913

Source
ToetsingOnline

Brief title
Enroll-HD

Condition

- Movement disorders (incl parkinsonism)

Synonym
Huntington's disease

Research involving
Human

Sponsors and support

Primary sponsor: CHDI Foundation, Inc.
Source(s) of monetary or material Support: CHDI Foundation;Inc.;New York;VS

Intervention

Keyword: Enroll-HD, Huntington's Disease, Longitudinal, Worldwide Observational Study

Outcome measures

Primary outcome

There are no fixed outcomes within Enroll-HD. This observational study is continuing and provides a platform for communication and exchange of knowledge and ideas about the disease and will serve as a basis for research. The data obtained will answer as much as possible research questions (most of them still to be established within the course of the study).

Secondary outcome

not applicable.

Study description

Background summary

Huntington*s disease (HD) is an autosomal dominant neurodegenerative disorder that results from an unstable expansion of the trinucleotide repeat CAG in the HD gene IT-15. HD has a prevalence of 5-10 per 100.000 in the general population. The clinical features of HD usually emerge in adulthood (mean age of onset is 40 years) with movement disorders, cognitive dysfunction and psychiatric symptoms. The course of HD is relentless, leading to functional disability and death over a period of 10-20 years. With genetic testing (following genetic counseling) it is possible to predict that a person will develop HD a long time before clinical symptoms and signs develop. To date, there is no treatment that has been shown to alter the progression of the disease. Benefical effects have been reported when applied in model systems of HD but the predictive value of these results for patients are yet unknown. As HD is a rare disease, extensive cooperation is essential to be able to include the number of participants required for conclusion well powered studies.

Study objective

Objective 1: To improve the understanding of the dynamic phenotypic spectrum

and the disease mechanisms of HD by:
a. collecting natural history data covering the cognitive, behavioral and motor domains permitting estimates of rates of progression in
HD and allowing insights into the neurobiology of HD,
b. collecting data and biologic samples to identify genetic
and environmental factors influencing and/or modifying the HD
phenotype and disease progression, and
c. promoting interrogatory studies that may provide clues to the pathogenesis of HD.

Objective 2: To promote the development of evidence-based guidelines to inform clinical decision making and improve health outcomes for the participant/family unit by:
a. assisting in the identification of beneficial interventions (clinical, pharmaco-therapeutic, non-pharmacologic),
b. facilitating the dissemination and implementation of
currently
proposed best clinical practices,
c. providing a platform for conducting outcome research, and
d. promoting exploratory data analysis projects that may
identify processes to further improve the healthcare of affected individuals and their families.

Objective 3: To provide a platform to support the design and conduct of clinical trials by:
a. providing a resource to identify, develop and qualify
novel assessment tools, clinical endpoints and biomarkers,
b. collecting longitudinal data to inform disease modeling
studies, and
c. facilitating the identification of potential trial
participants informing the selection of potential trial participants using data
to
estimate and quantify slopes/rates of disease
progression (providing *run-in* data).

To achieve these objectives suitably de-identified and coded clinical information and biological samples collected from study participants will be made available to investigators for research purposes in accordance with procedures adopted by the steering committee.

Study design

Enroll-HD is an observational, prospective, multi-national, multi-centre study without experimental treatment.

Study burden and risks

Since Enroll-HD is an observational study, participants do not undergo specific risks by participating. Their burden is limited to a minimum as the evaluations occur within or immediately after the ambulant care of the Neurology department on a regular basis. Participants will receive no immediate benefit from participation in this study. The only potential benefit is a better understanding of HD and the possibility that the information obtained in this study lead to potential treatments and to plan future research studies of experimental drugs aimed at slowing disease progression or postponing the onset of HD.

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

Individuals eligible to participate in Enroll-HD are classified into two major

categories:
1. Carriers: This group comprises the primary study population and consists of individuals who carry the HD gene expansion mutation.
2. Controls: This group comprises the comparator study population and consists of individuals who do not carry the HD expansion mutation.

Exclusion criteria

1. Individuals who do not meet inclusion criteria,
2. Individuals with choreic movement disorders in the context of a negative test for the HD gene mutation.
3. For Community Controls: those individuals with a major central nervous system disorder will be excluded (e.g. stroke, Parkinson disease, Multiple Sclerosis, etc.).

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:	Recruiting
Start date (anticipated):	07-10-2014
Enrollment:	800
Type:	Actual

Ethics review

Approved WMO

Date:	14-07-2014
Application type:	First submission
Review commission:	METC Leiden-Den Haag-Delft (Leiden)
	metc-ldd@lumc.nl

Approved WMO

Date:	17-11-2014
Application type:	Amendment
Review commission:	METC Leiden-Den Haag-Delft (Leiden)
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Approved WMO

Date:	13-04-2016
Application type:	Amendment
Review commission:	METC Leiden-Den Haag-Delft (Leiden)
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Approved WMO

Date:	04-01-2021
Application type:	Amendment
Review commission:	METC Leiden-Den Haag-Delft (Leiden)
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Approved WMO

Date:	16-01-2023
Application type:	Amendment
Review commission:	METC Leiden-Den Haag-Delft (Leiden)
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Approved WMO

Date:	12-10-2023
Application type:	Amendment
Review commission:	METC Leiden-Den Haag-Delft (Leiden)
	metc-ldd@lumc.nl

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
ClinicalTrials.gov	NCT01574053
CCMO	NL45724.058.13