



EHDN

our joint path to clinical research

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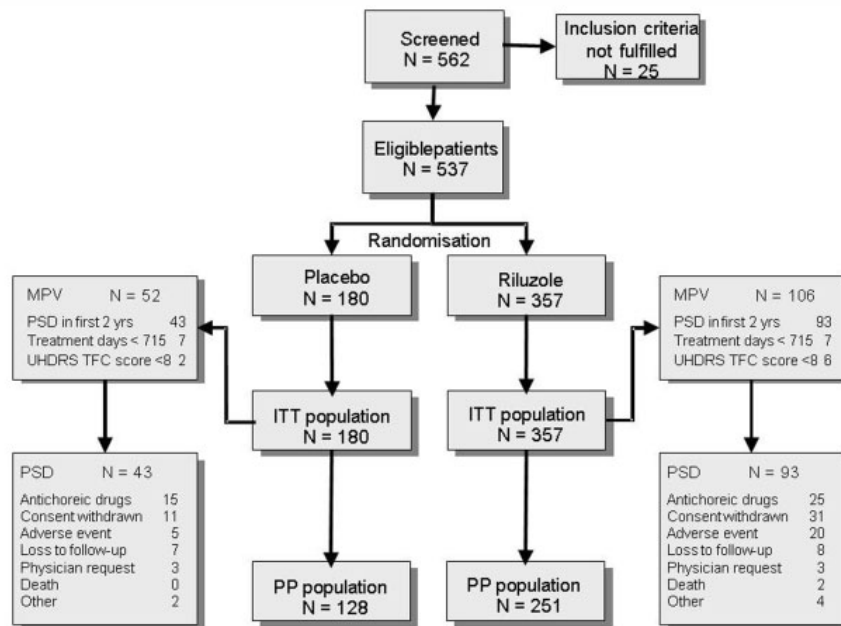
EHDN: together for progress

- **The beginnings**
- **Organisation**
- **Activities**
- **Conclusion**



Riluzole in Huntington's Disease: A 3-Year, Randomized Controlled Study

G. Bernhard Landwehrmeyer, MD, PhD,¹ Bruno Dubois, MD,² Justo Garcia de Yébenes, MD,³ Berry Kremer, MD, PhD,⁴ Wilhelm Gaus, PhD,⁵ Peter H. Kraus, MD,⁶ Horst Przuntek, MD,⁶ Michel Dib, MD,² Adam Doble, PhD,⁷ Wilhelm Fischer, PhD,⁸ Albert C. Ludolph, MD, PhD,¹ and the European Huntington's Disease Initiative Study Group



Organisation

- Founded 2004
- Network
- Collaboration
 - HD researchers
 - HD clinicians
 - HD affected



EHDN



Advancing Research, Conducting Trials, Improving Care

- A not-for-profit research organisation
- Committed to
 - advancing knowledge of HD
 - supporting the development of better therapeutic interventions
 - improving the quality of life of families affected by HD
- more than 2,000 members in 50 countries

- Multicentre (160), multilingual (13)
- Prospective observational study
- Participants (around 14000 up to 14 years)
- Legacy database

EHDN: together for progress

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executive committee



Advancing Research, Conducting Trials, Improving Care

Nine: elected by the
EHDN membership

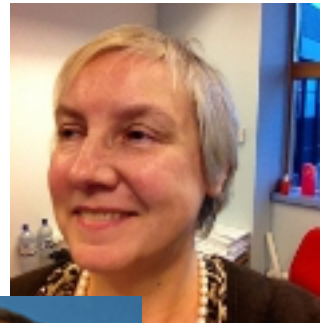
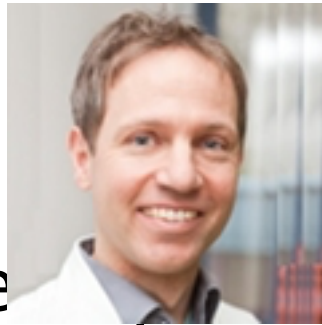
executive committee



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Nine: electe
EHDN membershi



executive committee



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A collage of ten portrait photographs of the Executive Committee members, arranged in a non-uniform, overlapping grid. The members include a woman with brown hair, a man with short brown hair, a man with blonde hair, a man with a beard and orange tie, a man with white hair and glasses, a woman with brown hair, a man with glasses and a blue shirt, a man with short dark hair, a man with glasses and a light blue shirt, and a woman with short dark hair.

One: Appointed by EHA

Scientific and bioethic advisory committee responsibilities

- Elected
- Advise members of the EHDN network
- recommendations to members EC



Central coordination



Language areas coordinators



Enroll-HD team



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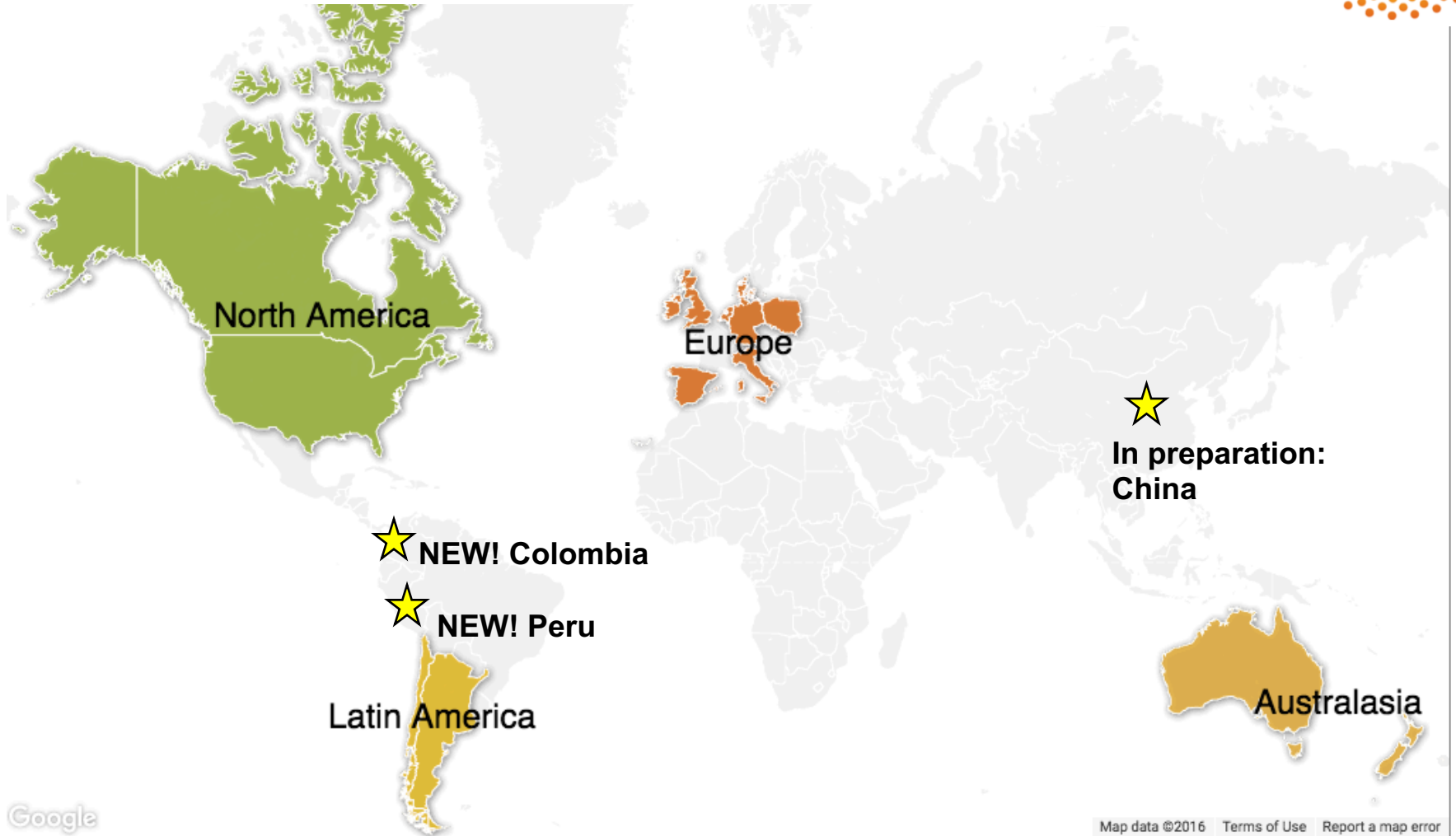




EHDN Activities

Clinical research
Science Think tank
Training
Networking

A Global Study



Enroll-HD



- A world wide clinical research platform and observational study for Huntington's disease
- has three main goals
 - **better understand HD**
 - **improve the design of clinical trials**
 - **improve clinical care for HD patients**

Enroll HD: Study Status

- Study sites: 147 active in 15 countries
 - North America
 - Latin America
 - Oceania
 - Europe
- Participants: 13,742 active participants (1.11.2017)



Support of Clinical trials

- Endorsement
 - EC, based on analysis of SBAC, CTTF,
 - Jointly with HSG
- Study preparation
 - Site evaluation
- Clinical site certification
- Study logistics
 - Contact with sites
 - Collaboration with clinical research organisation

Studies endorsed and supported

- Pridopidine (TEVA)
 - Pride HD
 - Open Pride
- Laquinimod (TEVA)
 - Legato
- PF-02545920 (PDE-10 Inhibitor) (Pfizer)
 - Amaryllis
 - Open Amaryllis
- Deep brain stimulation
- Antisense oligonucleotides
 - IONIS
 - WAVE



EHDN Activities

Clinical research
Science Think tank
Training
Networking

EHDN scientific think tank

- Collect ideas
- Develop an interactive database
- Generate actions
- Follow-up





The HD Science Think Tank

An EHDN initiative to advance HD research

THINK TANK PURPOSE

[Learn more](#)



WHO ARE WE?

[Learn more](#)



CONTACT

[Learn more](#)



A worldwide observational study for
Huntington's Disease families

Think Tank Purpose

The HD Science Think Tank is an EHDN initiative to advance HD research. Based on the 2017 Scientific Strategic Plan the HD Science Think Tank intends to complement other EHDN initiatives such as the [Working Groups](#). The HD Science Think Tank engages with the HD research community in three different ways:

Working group:



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- **Phenotype**

- Motor phenotype
- Cognitive phenotype
- Behavioural phenotype
- Functional ability
- Quality of life
- **Health economics**

- **Pathophysiology**

- Genetic modifiers
- Biological modifiers

- **Therapy**

- Physiotherapy
- **Invasive therapies**

Task forces

- **Driving**
- **Assessment of dysphagia**
- Incidental findings in genetic studies
- Microbiome in HD



EHDN Activities

Clinical research
Science Think tank
Training
Networking



🕒 2 Hours

Motor Rater Training 2017

Learn how to conduct and score the UHDRS Motor Exam. Enroll-HD Motor Raters must complete certification on an annual basis.



🕒 2 Hours

Motor Rater Training 2016

Learn how to conduct and score the UHDRS Motor Exam. Enroll-HD Motor Raters must complete certification on an annual basis.



🕒 2 Hours

Cognitive Rater Training

Provides raters with guidance on the instructions for administering and scoring the Enroll-HD Cognitive Assessment Battery.



🕒 2 Hours

Behavioural Rater Training

PBA-s Teaching Video and the PBA-s Webinar Training materials, along with an introduction to the psychopathology of HD and



🕒 2 Hours

Functional Rater Training

Provides raters with training materials on how to administer and score the UHDRS Functional Scale.



🕒 2 Hours

HDCC Rater Training

Provides an introduction on how to administer and rate the onset of signs and symptoms that may or may not be related to HD using the HD Clinical Characteristics



🕒 2 Hours

Good Clinical Practice

The Barnett International Good Clinical Practices (GCP) training module provides teaching and assessment of GCP aimed at global clinical research

Training



Advancing Research, Conducting Trials, Improving Care



Enroll-HD Clinical Training

Enroll-HD is designed to accelerate the discovery and development of new therapeutics for Huntington's disease. This portal hosts training materials from various sources that are relevant to HD clinical trials and studies.

[REQUEST AN ACCOUNT →](#)

Education & Training

- **Joint training activities**
 - MDS-ES, ERN-RND, EHDN
- **Fellowship Exchange Programme**
 - Joint with MDS-ES

Plenary meeting



[HD PATIENTS AND FAMILIES](#)[HD CLINICIANS AND RESEARCHERS](#)[CLINICAL TRIAL SPONSORS](#)

Welcome to the European Huntington's Disease Network

Our Mission

The EHDN is an independent nonprofit network dedicated to advancing research, conducting clinical trials and improving care for people affected by Huntington's disease. The network is comprised of clinicians, researchers and people affected by HD, working together to accomplish our mission.

[LEARN MORE ABOUT EHDN](#)

Newsletter



EHDN *News*

March 2017 · Issue 30

EUROPEAN HUNTINGTON'S DISEASE NETWORK



EHDN Activities

Clinical research
Science Think tank
Training
Networking

CHDI: aim



CHDI Foundation
Collaboration
developing therapies

substantially improve lives
of individuals with HD

European Huntington Association

- Close collaboration
- Strong support for clinical trials and research
- Voice at the core
- Support of local activities



Other HD networks



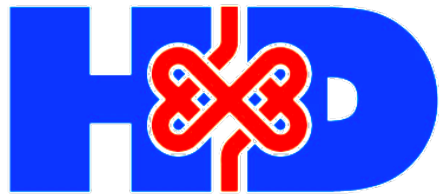
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seeking treatments that make a difference

- Mutual participation at meetings
- Joint trial evaluation
- Joint programs
- Regular leadership calls

Other HD networks



中国亨廷顿病协作网

Chinese Huntington's Disease Network

- Sharing of investigation protocols
- Collaborative work



中国亨廷顿病协作网

Chinese Huntington's Disease Network

**CHDN
clinical
centres
2016**



中国亨廷顿病协作网2016成都会议
Chinese HD Network 2016 Chengdu Conference



ERN objectives



Facilitate improvements in
access to diagnosis
treatment
provision of healthcare

Focal points for
medical training
research
information dissemination
evaluation.

EUROPEAN ACADEMY OF NEUROLOGY AMSTERDAM JUNE 24 - 27, 2017



**THIRD CONGRESS OF THE
EUROPEAN ACADEMY OF NEUROLOGY!**



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NETWORK

Advancing Research, Conducting Trials, Improving Care

Background

Huntington's disease (HD) is a rare, dominantly inherited fatal neurodegenerative disease with worldwide prevalence caused by a single known gene mutation (*HTT* CAG-repeat expansion mutation). The European Huntington's Disease Network (EHDN) was founded as a collaborative platform for health care professionals, researchers, people affected by HD and their relatives.

About EHDN

EHDN has > 2,000 members in 50 countries and ~ 160 study sites throughout Europe.

Organisation:

- Elected **Executive Committee**, supported by
- Elected **Scientific and Bioethics Advisory Committee**
- **Clinical Trial Task Force (CTTF-HD)**
- **Central Coordination** (operations, science management, administration, IT)
- **Language Area Coordinators** supporting their local study sites
- **Working Groups**



Our Mission

Advance knowledge of Huntington's disease, support the development of better therapeutic interventions and improve the quality of life of people affected by HD.

Our Strategy

Advancing Research

- Collaboration of researchers, doctors, HD families
- Establish large cohort of HD participants with clinical data and biosample collection available for research¹
- Seed funding scheme to promote novel scientific ideas; grant manager to support grant applications
- Working groups: promote collaboration on key research questions in HD

Facilitating Clinical Trials

- CTTF-HD
 - Provide advice and support to industry partners.
 - Assist in feasibility assessment of trial sites.
- Large patient databases (REGISTRY and Enroll-HD)
 - facilitate trial recruitment
 - data-mining to assist with trial design
- Site qualification and training
- Operational support by EHDN staff with in-depth HD knowledge and long-standing relationships with sites

Improving Clinical Care

- Standardisation of care, sharing of best practice.
- Information platform for patients and their relatives

Our Accomplishments

Research

- Longitudinal observational study (**REGISTRY**) with > 50 publications^{1,2}

REGISTRY (May 2017)	N
Countries	20
Sites	157
REGISTRY HD participants*	13,250
REGISTRY Controls	1,500
Biosamples (# individuals)	10,000

* > 87% 2 consecutive visits; ~50% with 5 visits over 5 years

Since 2013, transfer from REGISTRY to Enroll-HD, the first global HD observational study.



Clinical Trials

- Successful partnerships with industry in clinical trials^{3,4,5}
- Online rater training and certification
- Development of assessment tools
- Rating scales available in all network languages

Care

- Standard of Care guidelines⁶
- Contribution to international guidelines for predictive testing

References and contact

For general enquiries please contact: info@euro-hd.net

¹ GeM-HD, Cell, 2015; ² Orth et al, JNNP, 2011; ³ de Yebenes JG et al, Lancet Neurol., 2011; ⁴ Ferreira JJ et al, Mov. Disord., 2015;

⁵ Group HlotHS, EHDN, JAMA Neurol., 2013; ⁶ Neurodeg. Dis. Man., 2012, Vol 2, No 1



**MDS Berlin
2016**

EHDN in all of Europe





Public relations



EHDN: together for progress

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- **Organisation**
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Join!



Advancing Research, Conducting Trials, Improving Care

[ABOUT HD](#)

[ABOUT EHDN](#)

[NEWS & EVENTS](#)



HD PATIENTS AND FAMILIES

HD CLINICIANS AND RESEARCHERS

CLINICAL TRIAL SPONSORS

About EHDN

The EHDN is a nonprofit research network committed to advancing research, facilitating the conduct of clinical trials, and improving clinical care in HD. Through the EHDN a platform has been created such that basic scientists, clinicians, patients and families can collaborate on academic and industry studies to fulfil its mission. The EHDN is supported by and collaborates closely with CHDI Foundation, Inc.

As a network of stakeholders in HD, the EHDN offers its members the opportunity to take a lead and propose, conduct and publish studies. Anyone who has an interest in HD, including those affected directly by the disease, may join the EHDN and, in so doing, make a personal contribution to HD events and research.

[Want to join the EHDN?](#)

Join!

[ABOUT HD](#)[ABOUT EHDN](#)[NEWS & EVENTS](#)

disease, may join the EHDN and, in so doing, make a
to HD events and research.

Want to join the EHDN?

ADVANCING RESEARCH

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Want to join the EHDN?

CONDUCTING TRIALS



Merci pour votre attention