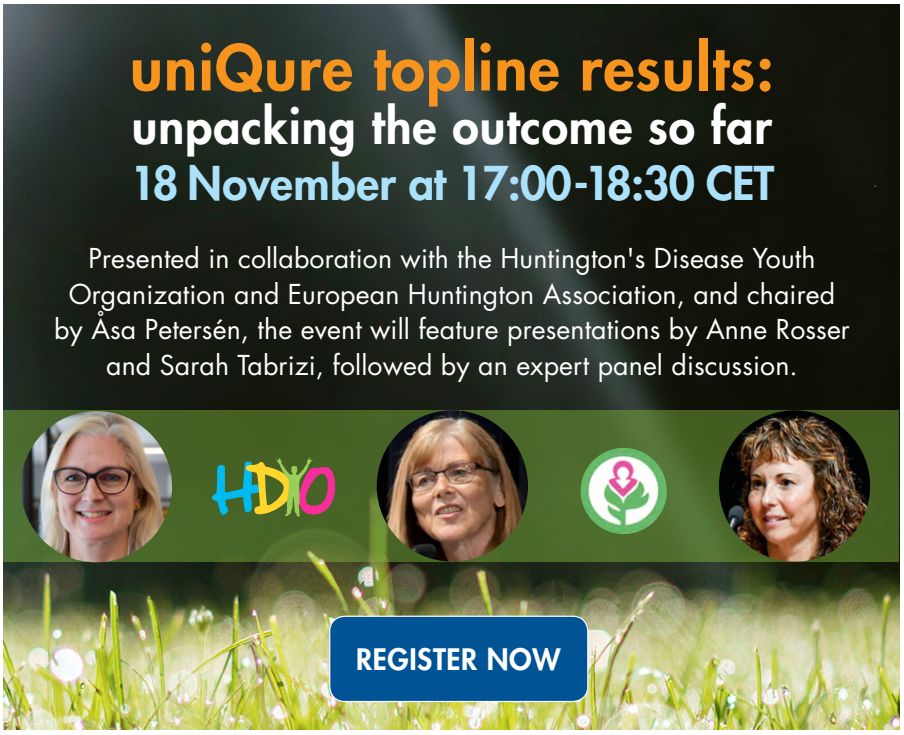
November 2025 · Issue 56

EHDN *News*

EUROPEAN **HUNTINGTON'S DISEASE** NETWORK



Welcome...

Catherine Deeprose

The HD community shares a renewed sense of excitement, purpose and vigour following uniQure's announcement on 24 September that their AMT-130 gene therapy may slow progression of the disease.

AMT-130, one of several huntingtin-lowering approaches currently in development, is delivered directly to the striatum in the brain through a surgical procedure. Topline results for uniQure's Phase 1/2 study showed that the high dose of AMT-130 met the study's primary endpoint by demonstrating statistically significant slowing of disease progression (as assessed by the composite Unified Huntington's Disease Rating Scale) at 36 months compared to a propensity score-matched external comparator (Enroll-HD natural history data). The high dose also demonstrated statistically significant slowing of disease progression as measured by Total Functional Capacity, a key secondary endpoint, at 36 months compared to the propensity score-matched external comparator.

EHDN Chairs Patrick Weydt and Åsa Petersén provided a full statement on uniQure's press release, which you can read here: ehdn.org/statement-from-ehdn-chairs-on-uniqures-topline-results/. They explained, 'While these results are positive, it is important to remember that these are preliminary, topline results from an early-stage clinical trial in a small number of highly selected

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Imprint:
Editorial Board of EHDN *News*:
Catherine Deeprose, Editor
Gabriele Stautner Artifox Communication Design (Ulm, Germany),

patients… we look forward to the release of further analyses and the peer-reviewed publication of the findings'.

Although uniQure's announcement marks a significant step forward, it represents just the beginning of a longer journey – one that EHDN is well positioned to support.

EHDN Chair Emeritus, Anne Rosser, shared, 'The Advanced Therapies Working Group brings together a diverse group of contributors, including neurologists, neurosurgeons and other healthcare workers, scientists, and other interested parties from academia, healthcare providers and industry to address the technical, regulatory and ethical issues of delivering substances and cells to the brain for therapeutic purposes.'

Anne Rosser and colleagues at the Advanced Therapies Working Group meeting in Venice, September 2025

You can find out about EHDN's Advanced Therapies Working Group at ehdn.org/advanced-therapies-wg/ and read an interview with Anne Rosser at ehdn.org/wp-content/uploads/2024/11/EHDN-NL-53-final.pdf. Further information about all EHDN-endorsed studies can be found in this issue in Update: Clinical Trials and online at: ehdn.org/previous-ongoing-research/.

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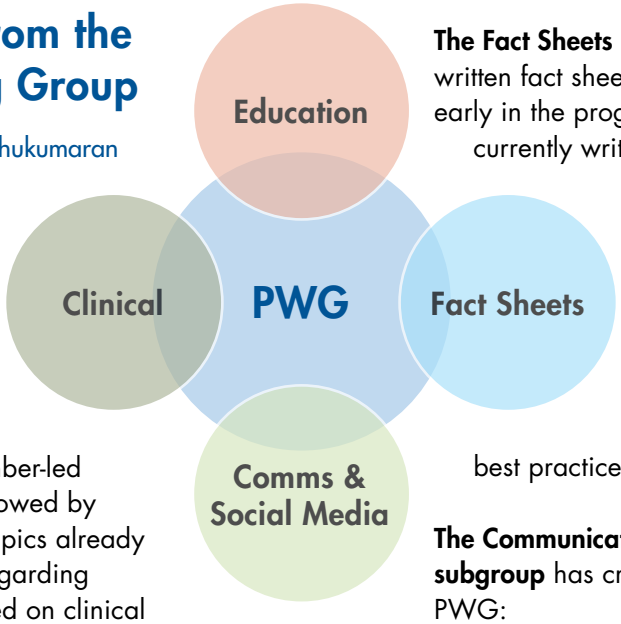
Recent Developments from the Physiotherapy Working Group

Camilla Ekwall, Magdalena Filip and Muthukumaran Thangaramanujam



The Physiotherapy Working Group (PWG) comprises approximately 50 members from across Europe, Australia, and the US, with representation from both clinicians and researchers. The group holds regular online meetings featuring member-led presentations on topics of interest, followed by discussions and Q&As. Some of the topics already discussed include ethical reasoning regarding people with HD and case studies based on clinical gait analysis. In October, we had a wonderful lecture, *Beyond Symptoms: How Illness Narratives Shape Physiotherapy Approaches in HD,* followed by thoughtful discussion.

PWG currently has four different subgroups, each focusing on a specific area: Education, Fact Sheets, Communication and Social Media, and Clinical Practice.

The Education subgroup focuses on disseminating knowledge about physiotherapy in HD. The group has applied to the EHDN Strategic Fund for a project to support the development of a toolkit for physiotherapists implementing evidence-based practice.

The Fact Sheets subgroup has already written fact sheets on physiotherapy early in the progression of HD. We are currently writing fact sheets for both clinicians and family members about physiotherapy for individuals with more advanced HD. The fact sheets are based on our previous clinical guidelines, best practice and expert consensus.

The Communication and Social Media subgroup has created a new logo for the PWG:

EHDN PHYSIOTHERAPY WORKING GROUP

The Clinical Practice subgroup is working to present clinical examples of how physiotherapists can work with individuals across the full trajectory of HD.

At EHDN & Enroll-HD 2024 in Strasbourg, a new Lead Facilitator and two new Co-Lead Facilitators were elected:
Camilla Ekwall (Sweden), Magdalena Filip (Poland) and Muthukumaran Thangaramanujam (Ireland).

Lead Facilitator:
Camilla Ekwall, Lecturer,
Regd. Physiotherapist,
Specialist in Physiotherapy in Neurology
Uppsala, Sweden

Muthukumaran Thangaramanujam
Regd. Physiotherapist / Adjunct Assistant Professor (Psychiatry)
TCD School of Medicine, Trinity College Dublin,
Dublin, Ireland

Co-lead Facilitors:
Magdalena Filip, Lecturer,
Regd. Physiotherapist,
Physical Therapy Division,
University of Physical Education in Kraków, Poland

We are welcoming new members, so if you are interested in joining our PWG, please contact Camilla Ekwall (camilla.ekwall@uu.se).

A Personal Reflection on the EHDN and MDS-ES Fellowship Programme 2024

Ayotomiwa Fagbemi (Consultant Neurologist, Nigeria),
Reza Kiani (University of Leicester and Leicestershire Partnership NHS Trust, UK)

Since its conception about 35 years ago, the Leicestershire Partnership NHS Trust HD service has grown over time to become one of the most well-developed HD neuropsychiatric centres in the UK. It comprises four different components:

- An inpatient unit (Mill Lodge) providing admission beds nationally
- A community multidisciplinary team (MDT) providing holistic care
- An advisory service covering a neighbouring county
- A research clinic

Ayotomiwa Fagbemi

Reza Kiani

In 2024, we were thrilled to learn that Ayotomiwa (based in Nigeria) had been awarded an EHDN and International Parkinson's and Movement Disorder Society European Section (MDS-ES) Fellowship to undertake a placement at the Leicestershire HD Service for a period of six weeks.

The Nigerian Context

In Nigeria, access to HD testing and diagnosis is extremely limited, and the associated cost is unaffordable for many. Medication availability is also restricted; for example, tetrabenazine is largely unavailable in the country. Despite these hurdles, Nigerian healthcare professionals strive to provide optimal care and improve services for the HD community.

Overview of Placement

Week 1:

Ayotomiwa's initial experiences included visiting people with HD in care homes and participating in MDT meetings, which served to provide a better understanding of

the multifaceted clinical presentation of HD and the necessity of holistic symptom management. Participation in ward rounds at Mill Lodge further enhanced insight into clinical care.

Week 2-3:
The subsequent weeks involved several more residential and nursing home visits, as well as home visits, which provided invaluable experiences in community-based practice. Further opportunities included 1) attending the Clinical Genetics department to gain experience of genetic counselling, capacity assessment and the associated legal/ethical frameworks, and 2) shadowing the community matron delivering a session on HD awareness training, aiming at broadening the knowledge of psychosocial impacts of HD, associated risks and improving quality of life.

Week 4:
Another excellent opportunity was provided by the Department of Genetics at the University of Leicester. This involved visiting the cell culture labs and fruit-fly labs, and observing the cutting-edge equipment (including flow cytometry and electronic microscopes) used in world-class research.

Week 5–6:
The final weeks involved further attendance at the MDT clinic to increase understanding of the patient journey, which included being assessed by various team members during a single appointment. Active participation in case formulation and management plans was encouraged. Attending the Enroll-HD clinic provided an extremely valuable introduction to global observational research.

Non-Clinical Experiences

Throughout the fellowship, emphasis was also put on the role of other organisations and the importance of social rehabilitation and social prescribing. This included becoming familiar with the roles of Social Services, housing associations, benefit agencies, health commissioners, and charity organisations (e.g., Huntington's Disease Association, Huntington's Disease Youth Organization).

Furthermore, it was ensured that a balance was maintained between clinical/research activities and leisure time. The latter included embarking on nature walks, playing table tennis, and exploring local places and restaurants.

Post-Fellowship Impact

Upon returning home to Nigeria, the insights gained during the placement were shared in a hospital grand round presentation to help establish an HD support group, along with MDT input for HD patients in South-west Nigeria. A long-term objective is to develop an HD registry to determine the service prevalence and characteristics, as well as the broader impact of HD in the local area.

We are profoundly grateful to all the staff at EHDN and MDS for their support in sponsoring this extremely rewarding fellowship, which ultimately will pave the way for further improvement in HD service provision.

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Get in Touch with the Think Tank!

EHDN's Think Tank brings together EHDN members and staff who are closely involved in supporting scientific research – including members of the Executive Committee, Central Coordination and the working groups – and it engages with the HD research community in three ways:

- Researchers may contact the Think Tank for help in identifying potential collaborators or funding opportunities, or to discuss scientific ideas
- The Think Tank welcomes suggestions of research topics, and has provided a contact form on its website via which these can be submitted
- The Think Tank may occasionally propose specific research topics that could be addressed by a dedicated task force working for a defined period of time

For more information about the Think Tank, please contact Kinga.Kolodziej@ehdn.org

Update: Enroll-HD – Seventh Periodic Dataset Now Available!

Olivia Handley, Enroll-HD Global Platform Director

On 3 September 2025, Enroll-HD announced the seventh release of the Enroll-HD periodic dataset (PDS7), curated from the Enroll-HD study database. PDS7 includes data from 30,511 Enroll-HD study participants and 112,992 study visits (Figure 1). This makes it one of the world's largest cohort datasets available to researchers.

Figure 1. Enroll-HD sample size by PDS release. Source: PDS7 Overview.

PDS7 includes approximately 1,000 participants with available RNA data, following the collection of the first sample under this initiative in February 2024. Additionally, PDS7 contains data from nearly 1,000 participants who have had 10 or more Enroll-HD study visits. As with previous releases, historic data from Registry (15,501 visits) and additional clinical data from other sources (1,044 visits) are also included.

In terms of demographic characteristics, the majority of participants (19,354; 63%) are from Europe, followed by 9,553 (31%) from North America, 936 (3%) from Australasia (comprising Australia and New Zealand), and 668 (2%) from Latin America. The cohort is predominantly female (16,989; 56%), and the average (mean) participant age at baseline is 47.5 years. The majority ethnic group is Caucasian (28,170; 92%), and over half of the participants (16,191; 53%) have obtained tertiary-level qualifications or higher.

As with PDS6, PDS7 includes imputed Huntington's Disease Integrated Staging System (HD-ISS) variables, including imputed HD-ISS stage for eligible participants and visits (Figure 2). Further information about the HD-ISS is provided in Figure 3. The majority of persons with HD were HD-ISS Stage 3 at baseline (12,920; 61%).

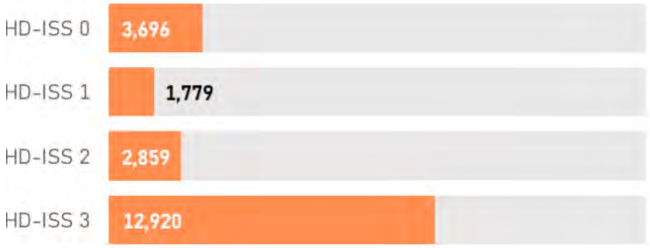

Figure 2. HD-ISS imputed stage at baseline Enroll-HD visit. Source: PDS7 Overview.

Regular releases of the Enroll-HD PDS provide HD researchers access to valuable participant data. The availability of high-quality, longitudinal data from an extremely large cohort provides a unique opportunity for HD researchers, enabling the interrogation of a wide range of scientific questions. There are over 170 publications to date which have leveraged the Enroll-HD data, biosamples, and/or infrastructure; the full list can be viewed here.

Enroll-HD datasets have undoubtedly contributed to our greater understanding of HD. None of this would be possible without the continued dedication and commitment of HD families and the Enroll-HD study sites, coupled with the highly motivated Enroll-HD platform team. PDS7 represents a huge achievement and opportunity for the HD community, and one that we will continue to build on with future releases.

If you are interested in finding out more about PDS7 or the range of other HD datasets and sample collections made available via the Enroll-HD platform, please visit the Enroll-HD website.

The Huntington's Disease Integrated Staging System (Figure 3) was published by Sarah Tabrizi (University College London) and colleagues in 2022.

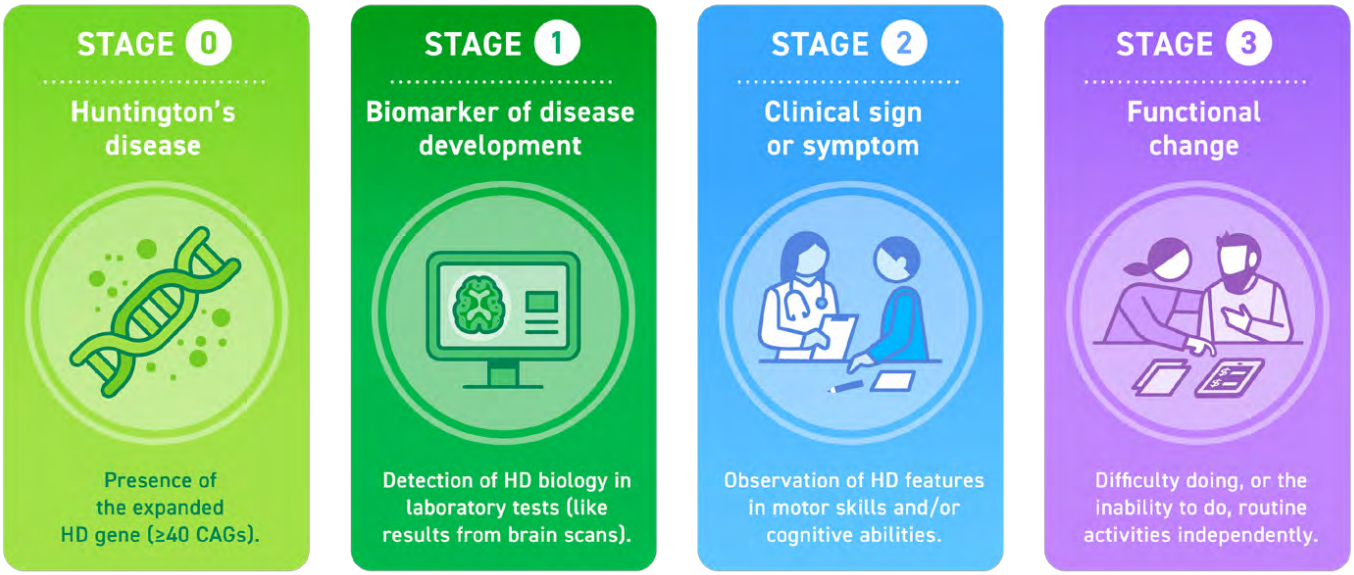

Figure 3. HD Integrated Staging System (HD-ISS) overview. The HD-ISS is a staging system developed for clinical research, dividing the progression of HD into four distinct stages. It provides a common standard and shared language for the scientific community, allowing researchers to study each phase of HD more precisely, and to compare research findings more readily.

> *'PDS7 represents a huge achievement and opportunity for the HD community, and one that we will continue to build on with future releases.'*

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William Gray, Anne Rosser and Patrick Weydt were among the eager delegates at the HD Clinical Research Congress

Huntington's Disease Clinical Research Congress 2025

The inaugural HD Clinical Research Congress was held 12–13 October in Nashville, Tennessee, and proved to be a tremendous success. Organised by the Huntington Study Group (HSG) and CHDI Foundation, the event attracted delegates primarily from North America, South America and Australasia who came from Enroll-HD sites in these regions, as well as industry representatives. Day 1 offered delegates comprehensive updates on ongoing HD clinical trials, emerging clinical developments, and advances in biomarker research. Highlights from Day 2 included an in-depth consideration of translational issues in HD research and an inspiring session dedicated to young people and HD. There was an ancillary meeting on 11 October, the HD Community Research Day, featuring sessions designed to demystify clinical research for participants and families.

Update: Clinical Trials

Jenny Townhill, EHDN Clinical Trial Director; Director, Enroll-HD External Partnerships

Key updates since the last newsletter are provided below for EHDN-endorsed trials; please refer to **Table 1** for a summary of all ongoing endorsed research. An expanded description of EHDN-endorsed trials and studies (completed and in progress) and details of the EHDN endorsement process are published on the EHDN website. Please note that only clinical trials with EHDN endorsement are covered here.

uniQure reported topline 3-year data on 24 September for the European and US gene therapy trials of AMT-130. Recruitment has started for an additional cohort of up to six participants, in the US only, with lower striatal volume than previous cohorts, to receive high-dose AMT-130. All participants in the trial will be followed up for 5 years after treatment.

There are two EHDN-endorsed trials currently recruiting, as summarised below.

Alnylam Pharmaceuticals: This trial of ALN-HTT02 involves a single intrathecal dose of a huntingtin-lowering siRNA. HD-ISS Stage 2 and early Stage 3 participants are currently being enrolled in Germany, the UK and Canada.

Vico Therapeutics: The open-label phase 1/2 trial of VO659 is enrolling participants with spinocerebellar ataxia types 1 and 3 (SCA1 and SCA3), and HD. SCA1, SCA3 and HD are all caused by CAG repeat expansions; VO659 binds to these expansions in the RNA transcripts of the affected genes, reducing the levels of the mutant proteins relevant to each of the diseases. Different dose levels of intrathecal VO659 are being tested, with SCA1 and SCA3 participants enrolled into the first two dosing cohorts, and HD participants with a Unified Huntington's Disease Rating Scale© (UHDRS) Total Functional Capacity Score between ≥11 and ≤13 and a UHDRS Diagnostic Confidence Level of 4 also enrolled into subsequent cohorts. The duration of participation ranges from around 10 to 13 months, depending on cohort, with cohorts 1–4 receiving multiple doses of V0659 and cohort 5 receiving a single dose.

For additional details about these trials and information on recruiting sites, see Table 1 and the direct links to clinicaltrials.gov.

Table 1: Current EHDN-Endorsed Trials and Studies

Registration ID	Sponsor	Trial name	Phase	Investigational Product	Mode of Action	Delivery	Treatment Goal	Target Enrolment	Location(s)	Status
NCT06585449	Alnylam Pharmaceuticals	ALN-HTT02-001	1	ALN-HTT02	Htt lowering; siRNA	Intrathecal	Disease modification	54	Canada, Germany, UK	Recruiting
NCT05686551	Hoffmann-La Roche	GENERATION HD2	2	Tominersen	Htt lowering; ASO	Intrathecal	Disease modification	300	Argentina, Australia, Austria, Canada, Denmark, France, Germany, Italy, New Zealand, Poland, Portugal, Spain, Switzerland, UK, USA	Active, not recruiting
NCT05358717	PTC Therapeutics	PIVOT-HD	2	PTC518	Htt lowering; mRNA splicing modifier	Oral	Disease modification	162	Australia, Austria, Canada, France, Germany, Italy, Netherlands, New Zealand, Spain, UK, USA	Active, not recruiting
NCT05243017	uUniQure	HD GeneTRX2	1b/2	AMT-130	Htt lowering; miRNA AAV delivered gene therapy	Surgical, intrastriatal	Disease modification	15	Poland, UK	Active, not recruiting

Note. AAV = Adeno-associated virus; ASO = antisense oligonucleotide; Htt = huntingtin; mRNA = messenger ribonucleic acid; miRNA = micro ribonucleic acid; siRNA = small interfering ribonucleic acid

HDClarity

Update: HDClarity

Gail Owen, Principal Research Associate, University College London, UK

Recruitment Status

After record recruitment in 2024, HDClarity sites have exceeded expectations for 2025 and have already equalled the total number of successful cerebral spinal fluid (CSF) collections from last year. The essential longitudinal sample collection continues to grow, with approximately 30% of participants now having completed at least two annual visits and over 100 individuals providing three or more samples. The record currently stands at six CSF collections thanks to our incredible participants!

What Is Cerebral Spinal Fluid and How Is It Collected?

CSF is a clear fluid that surrounds the brain and spinal cord, providing important information about the brain and nervous system that is impossible to obtain in any other way. CSF is collected by a procedure known as a lumbar puncture or spinal tap. During the procedure, the participant sits upright or lies on their side. The study doctor then inserts a very fine needle between the vertebrae in the lower back to reach the space where the CSF is located. Typically, a local anaesthetic is injected first to numb the area. A syringe is then used to remove the CSF. In HDClarity, CSF and blood are collected while the participant is fasted, as this is a well-known method to reduce potential variability in the samples.

Opeyemi Kinyomi, a neurologist based at University College London, has performed numerous lumbar punctures as part of the HDClarity study. She explains that while the procedure may feel slightly uncomfortable, it is rarely described as painful. The brief, sharp sting of the local anaesthetic is typically the most noticeable sensation. Many participants have shared that they were surprised by how well they tolerated the procedure, often describing it as 'uncomfortable rather than painful'. The CSF sampling itself usually takes only a few minutes and is followed by a well-earned meal after the six-hour fast. This consistent feedback from participants highlights the care taken to ensure the experience is as smooth and reassuring as possible.

Sample Distribution

HDClarity CSF and blood samples are available to investigators for further research and analysis to help understand HD processes, identify new biomarkers, and develop treatments for HD and other neurological conditions. For further information on requesting HDClarity samples for research, visit enroll-hd.org/for-researchers/access-data-biosamples/.

Further information

The current HDClarity protocol and lab manual are available at www.hdclarity.net. The UCL Central Coordination team is always happy to answer any questions (hdclarity-cc@enroll-hd.org).

Update: Projects and Education

Fionnuala Margreiter, Grants & Collaborations Manager
Juliana Bronzova, Science Director

Fellowship Programme

The fellowship programme is a six-week programme organised by EHDN together with the MDS-ES to allow healthcare professionals from all over the world to attend an established HD multidisciplinary clinic in Europe and learn about HD care.

Launch of 2026 Fellowship Programme

The 2026 EHDN and MDS-ES Fellowship Programme is now open for applications. Six clinical fellowship grants of €2,500 plus travel expenses (up to €1,500) will be awarded, allowing applicants to visit centres with HD expertise in Europe.

Eligibility: Applicants must be under 40 years old or have less than five years since their final professional qualification, be Board-Certified or in the final year of training for Board Certification in Neurology, Psychiatry, Psychology, Physiotherapy, or Clinical Genetics, or another healthcare professional, and be fluent in English or the local language of their preferred host country. Previous awardees are not eligible to reapply.

Six-week observer placements focus on clinical practice within multidisciplinary clinics, with priority given to applicants from underserved HD regions. Applications should clearly justify the need, either in relation to overall HD care or to specific aspects of HD care.

Applications must be submitted online ehdn.org/hd-clinicians-researchers/fellowship-programme/ by **Monday, 15 December 2025, 17.00 CET**. Results will be announced in March 2026.

2025 Programme Progress

Six fellows were awarded placements in 2025, and their six-week placements are still ongoing in the UK, Spain, and France. Many thanks to the host clinics for placements already hosted or soon to be hosted in Leicester, UK (Maria Dale); Liverpool, UK (Rhys Davies and Sundus Alusi); Cruces, Bilbao, Spain (Tamara Fernández); Burgos, Spain (Esther Cubo); Bordeaux, France (Marie Pierrer Baudier and Cyril Goizet); Cambridge, UK (Roger Barker and Katie Andresen).

Fionnuala Margreiter

Juliana Bronzova

Programme Impact

Reporting from the last cycle of the Fellowship programme, which took place after the Fellowship Impact Project, was very positive and indicated a high level of satisfaction in all areas, including structure and organisation of the fellowship programme, changed perspective on providing clinical care, support received, and so on.

EHDN/MDS HD Joint Online HD Course Series (In Spanish)

EHDN and the International Parkinson's and Movement Disorders Society, Pan American group, recently co-organised an accredited online course series titled *Multidisciplinary Management of Huntington's Disease*. The course, which was held in Spanish over two Friday afternoons in August 2025, saw an impressive 608 registrations, primarily from South American countries. Enroll-HD Regional Director for Latin America, Iberian Peninsula, and Italy, Claudia Perandones, served as the Course Director on behalf of EHDN. The event featured several renowned international speakers and received positive feedback from participants.

Following the successful completion of the course, discussions are now underway to translate the course material into English for broader accessibility. The course lectures will also be available online for free on-demand access.

Education Project

EHDN is currently undertaking an education project to review and ensure easy accessibility of courses, podcasts, lectures, guidelines, and other materials already available on various channels through the work of Working Groups, Task Forces and EHDN. Contacts are also being made with other organisations to ensure that information is shared and communicated to the HD community.

Grant opportunities and updates are regularly shared on the EHDN website. An overview of individual and collaborative grant opportunities can be found at ehdn.org/hd-clinicians-researchers/grant-manager/, as well as some information on the grants and collaboration service.

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José Lucas

Michael Taylor

Update: Lesley Jones Seed Funds

EHDN has recently awarded seed funding for two exciting new projects.

José Lucas (Spanish National Research Council, Madrid) was awarded funding for a project titled *CSF Thiamine Deficiency in HD as Predictive Biomarker of Onset of Functional Impairment.* Thiamine (vitamin B1) is essential for organs with high metabolic demand, like the brain. The Lucas group previously detected alterations in brain thiamine metabolism in HD participants, leading to decreased levels of a thiamine derivative (TMP) in CSF. Their recent research indicated that these decreases preceded motor symptoms and are also associated with early neuropsychological signs. The current project aims to confirm these interesting findings with a higher number of CSF samples from HDClarity.

Michael Taylor (University of Cardiff, UK) was awarded funding for a project titled *Analysing the Neurodevelopmental Hypothesis for HD Using Optogenetics*. It is established that mutant Huntingtin (mHTT) causes nerve degeneration in HD. However, there is evidence that mHTT affects the development of these nerves prior to this. The team aims to discover when mHTT acts on developing nerves to affect subsequent neurodegeneration in an HD model, and whether this can be prevented by manipulating specific gene expression. This work will advance understanding of the potential of early-life therapeutics in HD.

Flaviano Giorgini

The Lesley Jones Seed Fund programme is intended to support pilot studies that will eventually kickstart larger projects. The next deadline for applications is 1 March 2026.

Kinga Kolodziej

More information about the programme and how to apply can be found here or you can contact Flaviano Giorgini (flaviano.giorgini@ehdn.org) or Kinga Kolodziej (kinga.kolodziej@ehdn.org) for further information.

Anja Kletsch (Germany)

Isaura Vanoppen (Belgium/Netherlands)

Update: Staff News

We are thrilled to welcome Anja Kletsch (German Lanco team) and Isaura Vanoppen (Belgium/Netherlands Lanco team) to EHDN!

A full list of EHDN staff can be found at ehdn.org/about-ehdn/ehdn-structure/. For our latest career opportunities, please see: ehdn.org/about-ehdn/careers/.

The Rome EU Monitoring and EHDN Language Area Coordination Meeting involved a team-building exercise, which was enjoyed by all!

EU Monitoring and EHDN Language Area Coordination Meeting

Catherine Deeprose

The latest EU Monitoring and EHDN Language Area Coordination Meeting took place in Rome, Italy, 17–18 September 2025. This face-to-face meeting was led by Nicci Robinson and Hasina Hussain (taking over meeting coordination from Ruth Fullam). We discussed the latest updates on Enroll-HD, clinical trials, working groups, and the various projects in which Lancos are engaged. The meeting also featured a stimulating presentation on the psychiatric aspects of HD by EHDN Deputy Chair Åsa Petersén.

European Huntington Association Conference 2025

European Huntington Association Team

The European Huntington Association (EHA) conference was a powerful and moving event marked by warmth, empathy, and a profound sense of solidarity. Over several impactful days, the HD community came together – sharing not only knowledge and insights, but also moments of joy, laughter, reflection, and heartfelt emotion.

This year's conference welcomed many first-time attendees, with a particularly strong presence of young people. Their energy, engagement, and passion for advocacy infused the event with a fresh sense of momentum and optimism for the future.

The programme was thoughtfully curated to meet the broad needs of the HD community. It balanced accessible information combined with shared lived experiences. These personal testimonies resonated deeply with participants – bringing the realities of life with HD closer, strengthening understanding, and leaving a lasting emotional mark.

EHDN staff Kinga Kolodziej and Naomi Ueda

Throughout the conference, the dedication of the EHA and the unwavering commitment of the wider HD community were both evident and deeply appreciated. The conference once again underlined the importance of working together to build a more supportive and hopeful future for all those affected by HD.

Dina De Sousa,
European Huntington Association
Board Member

Real-Life Experiences for Better Care

Filipa Julio, European Huntington Association Project Manager

The *Huntington Academy* is a multinational, multilingual initiative designed to address the significant gaps in care provision for families impacted by HD. The initiative originated from an Erasmus+ Small-Scale Partnership, led by the European Huntington Association in collaboration with the Ligue Huntington Francophone Belge (Belgium), the Bulgarian Huntington Association (Bulgaria), and the Asociación de Corea de Huntington Española (Spain). Its mission is to empower the HD community through knowledge, skills, and collaborative learning, fostering improved care, advocacy, and quality of life.

The innovative and comprehensive e-learning platform provides accessible and high-quality educational resources for both formal (healthcare professionals) and informal (family members and friends) caregivers of individuals affected by HD. The contents are available in four languages – Bulgarian, English, French and Spanish. Next steps include expanding both the content and reach of this valuable resource.

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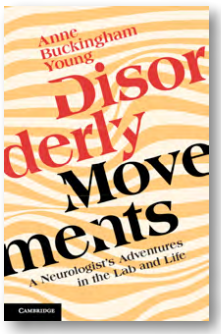
European Huntington Association's TrialFinder

Dina De Sousa, European Huntington Association Board Member

Our TrialFinder was launched in 2019 as a resource to help HD families learn more about studies and clinical trials across Europe, presented in clear and accessible language: hdtrialfinder.net/en/home/. The resource is regularly updated to reflect the constantly evolving HD research landscape.

EHDN BOOKSHELF



We're currently reading *Disorderly Movements: A Neurologist's Adventures in the Lab and Life,* Anne Young's inspiring memoir published earlier this year. Young recounts her remarkable career in the field of movement disorders, including her collaboration with Nancy Wexler and the Huntington's Disease Collaborative Research Group in the search for the huntingtin gene. With candour and depth, she explores themes of loss, mental health, and the delicate balance between personal and professional life – reminding us of the power of resilience and perseverance.

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EHDN Working Group and Task Force Virtual Forum

On 17 October, 18 EHDN working groups and task forces convened via Zoom for an online forum attended by over 70 participants. Anne Rosser opened the meeting by welcoming attendees and highlighting the fundamental role of working groups and task forces within EHDN, as well as the importance of discussion and collaboration.

Kinga Kolodziej and Flaviano Giorgini provided an overview of both existing and newly established groups, along with the various Lanco, financial, and publicity support mechanisms available to them.

The afternoon's programme was structured into three thematic clusters, featuring short presentations followed by brief Q&A sessions. The success of the forum was evident throughout, reflected in the thoughtful questions raised and the initiation of several promising collaborations.

For more information about EHDN working groups/ task forces, and the support available, please contact Kinga.Kolodziej@ehdn.org.

Guided by Purpose: Jamie Levey Reflects on Two Decades of EHDN

As Chief Operating Officer since 2005, Jamie Levey has been a cornerstone of the European Huntington's Disease Network from the beginning. Her dedication, energy and leadership have been instrumental in shaping the organisation we are incredibly proud to be part of today. In 2014, Jamie shared her personal reflections as a member of an HD family with *EHDN News* readers. Now, in 2025, she looks back on two decades with EHDN and considers the future of HD research.

Tell us about your early career and influences

When my mother started receiving clinical care for HD, I was in my early 20s and studying abroad at the Universidad de Sevilla. Many people hadn't heard about HD at this time, and the causative gene was yet to be discovered. To learn as much as I could about the disease, I attended events hosted by Nancy Wexler, who was a professor at Columbia University, and continued to follow her work and that of the Hereditary Disease Foundation [HDF], founded by her father, Milton Wexler.

As we started out in our adult lives, my siblings and I knew about the risk of inheriting HD, but our main concern was caring for our mother rather than ourselves. Over the next ten years or so, we began to think about getting married and starting our own families. At 29 years old, I decided to undertake the HD biomarker test. The results were negative, and confirmed again as negative following the discovery of the gene in 1993, which, of course, made direct genetic testing possible.

By this stage, I was working in finance with biotech companies and keeping in touch with Ethan Signer and Allan Tobin, who were advisors at HDF. During my maternity leave several years later, I began thinking seriously about my career direction. I came to the realisation that I wanted to dedicate my professional life to HD, utilising my background in finance and consultancy in life sciences.

How did you get involved with EHDN?

In the early 2000s, I spoke with Ethan and Allan, who were now advisors for the newly established High Q Foundation – later to become CHDI – led by Robi Blumenstein. I met with Robi, and around the same time, I was moving from New York to Paris. Robi suggested that I meet with Bernhard Landwehrmeyer, who was leading a small CHDI-funded initiative called the European Huntington's Disease Network, with a view to taking up the operational and business aspects. I immediately recognised Bernhard's scientific vision and saw how our skills would complement each other.

I joined EHDN in 2005, when Bernhard had already established connections with countries including Germany, Italy, Spain, France, the UK, Portugal, and the Netherlands. We were a small team and had just started Registry. We continued to grow the network and expand the study, which in 2014 officially transitioned into the global Enroll-HD study. Meanwhile, I moved back from Paris to New York. My brother and sister were both in the advanced stages of HD, and I wanted to be closer to my family.

What other activities were developed at this time?

Under the direction of the EHDN Executive Committee, we strengthened Central Coordination – the operational

hub of EHDN, where many of my own efforts were, and still are, focused. We also developed Language Coordination further by increasing and managing the resources needed to operate Registry (followed by Enroll-HD) and to implement EHDN's scientific strategy. We continued to solidify our administrative relationship with Ulm University's legal and finance departments, and worked to expand the core of the network.

The working groups generated outstanding contributions – most notably the Biomarker Working Group, which evolved into the TRACK-HD study led by Sarah Tabrizi at University College London. Working groups remain one of EHDN's most valued and dynamic components. We also implemented the EHDN Seed Fund programme to support early-stage pilot research and the Fellowship programme, conducted in collaboration with the Movement Disorders Society, to train young HD professionals from underserved regions, as well as online educational course series in English and Spanish. These initiatives continue to thrive today.

Bernhard was the Chair of EHDN from 2004 to 2014. We recognised the need to bring in new people, resulting in Bernhard becoming Chair and Founder Emeritus, and **Jean-Marc Burgunder** being voted in as Chair. I then worked with Jean-Marc and Anne Rosser as the Co-Chair. Together, we introduced minor amendments to the Constitution, which were voted on at plenary meetings. We established that the Scientific and Bioethics Advisory Committee would be appointed as a slate to ensure the requisite expertise to review seed fund applications and clinical trial protocols for

Jamie's daughter and brother in Central Park, New York, August 2022

Jamie and family celebrating her daughter's graduation, June 2025

Jamie and EHDN colleagues in Rome, September 2025

> *'Enroll-HD 2.0 will help us map the early disease trajectory with unprecedented clarity – and this is critical for drug development efforts.'*
>
> ***Jamie Levey***

endorsement, making recommendations to the Executive Committee.

What are the links between EHDN and CHDI?

EHDN is a research project based at Ulm University and is funded by CHDI. CHDI is a 501(c)(3) organisation in the USA. Our missions are clearly and closely aligned, and as such, we have CHDI representation on the EHDN Executive Committee via Robi and, more recently, Emily Gantman.

A key part of my role is to bridge the gap between EHDN and CHDI, as well as among their respective committees and groups. Although we are separate entities, we are closely linked, sharing a common vision and working together extremely well.

What is your involvement in Enroll-HD?

From 2012, as the global aspirations for Enroll-HD became a reality, I took on a broader director role on behalf of CHDI. I partnered with Tim McLean, who was already well-entrenched in clinical operations for both studies. Together with CHDI and EHDN colleagues, we developed what is now known as the Enroll-HD Platform.

Fast forward to the current day, and I am very excited about Enroll-HD 2.0. I believe the protocol amendment is long overdue – we know that you are born with the genetic mutation, that HD is a lifetime disease, and its effects begin long before clinical symptoms appear. Research in recent years has confirmed what many of us in HD families already suspected about the impact of early disease-related changes. Enroll-HD 2.0 will help us map the early disease trajectory with unprecedented clarity – and this is critical for drug development efforts.

What are your views on the future of HD drug development?

Like many in the HD community, I was thrilled by uniQure's topline results in September, and the recognition of the value of Enroll-HD through the use of these data as a comparator. This potential disease-modifying therapy for HD may mark the beginning of a new era. Of course, there is a lot of work ahead, and it won't all be rosy. Clinical development is fraught with setbacks, as we have seen with tominersen. Yet every trial – positive or negative – advances our understanding. This is a particularly hopeful and pivotal time for the HD community.

Jamie and Patrick Weydt in New York, preparing for a busy day of meetings

Data from Enroll-HD 2.0 will undoubtedly be a powerful tool in future HD drug development, particularly by informing discussions with regulatory authorities. We also anticipate further advances in utilising Enroll-HD data for comparative purposes in innovative, flexible approaches to drug development. Ultimately, all this work also aims to improve clinical care. Our HD community is fantastic in advancing, collaborating and sharing knowledge across the globe. I am very proud to be a part of it.

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What will the next EHDN plenary meeting offer?

We plan to build on the success of the EHDN and Enroll-HD 2024 meeting in Strasbourg and the integration of a clinical development programme. The Programme Committee is chaired by Hoa Nguyen and Nayana Lahiri from EHDN's Executive Committee, and Franziska Steck and Franziska Bernsdorff from Central Coordination lead the Organising Committee. We are also supported by Jerry Turner and his team at CHDI.

Going forward, we plan to have a collaboration between CHDI and EHDN for biannual plenary meetings – such as Strasbourg 2024 – and on alternate years, a collaboration between CHDI and the Huntington Study Group, as seen in Nashville 2025. These meetings provide an excellent complement to CHDI's annual HD Therapeutics Conference, which is typically held in Palm Springs and dedicated to preclinical and translational research.

Looking ahead to Krakow 2026, the meeting will retain familiar features, including the EHDN Business Meeting and a strong emphasis on clinical trials. Presentations will highlight ongoing and upcoming studies, as well as cutting-edge scientific developments as we launch Enroll-HD 2.0.

Dates for Your Diary

- **1 November 2025:** Applications for the EHDN Lesley Jones Seed Fund programme close at 11.59 CET.
- **1–2 November 2025:** Huntington's Disease Community Conference and AGM 2025 takes place in Crewe, UK.
- **15–19 November 2025:** SfN Neuroscience 2025 takes place in San Diego, USA.
- **15 December 2025:** Applications for the EHDN/MDS (ES) Fellowship programme close at 17.00 CET.
- **31 January 2026:** Closing date for Beyond the Frame within the Journey – Documenting life with movement disorders photography competition.
- **29–31 March 2026:** 6th RNA Metabolism in Neurological Disease Conference will take place in San Diego, USA.
- **22–24 October 2026:** **EHDN Clinical Research Congress** takes place in Krakow, Poland.
- **Ongoing:** Hidden world of HD photographic research project funded by the Edith Cowan University in Perth.

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