July 2026 · Issue 58

EHDN *News*

EUROPEAN **HUNTINGTON'S DISEASE** NETWORK

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EHDN CLINICAL RESEARCH CONGRESS

KRAKOW, POLAND
22 TO 24 OCTOBER 2026

REGISTRATION:
1 JUNE TO 31 JULY 2026

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Welcome!

Catherine Deeprose

Our 58th issue arrives at an exciting time for the network: **EHDN Elections 2026 are now open**, and we encourage all regular members to cast their vote before 19 October 2026. With an exceptional field of candidates standing for the Executive Committee – and a Scientific and Bioethics Advisory Committee slate to approve – this is your chance to have a say in the future of the network.

Results will be announced at the **EHDN Clinical Research Congress** in Kraków, Poland, 22–24 October 2026 – and if you haven't yet registered, we hope to see you there. The preliminary programme features keynote lectures from Alexandra Durr (Paris Brain Institute, France) and Nobel Prize winner Aaron Ciechanover, plenary sessions spanning the treatment pipeline from biomarkers to access and affordability, a dedicated session on clinical trial progress and regulatory perspectives, plus dedicated sessions for the wider HD community and networking opportunities.

This issue also marks a major milestone – the award of the 100th Lesley Jones Seed Fund, representing over €3 million in research funding since the programme's inception – and features an interview with Charlotte Spearing, whose remarkable work is changing the conversation about HD, one post and one connection at a time.

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From Small Seeds, Big Science Grows: The EHDN Seed Fund

Flaviano Giorgini, Science Director and Kinga Kolodziej, Science Project Manager

EHDN is celebrating a significant milestone with the award of the **100th Seed Fund**. The Lesley Jones Seed Fund was designed to help support and fast-track pilot studies in HD research that are required for larger funding applications at other organisations. A key aspect of seed funding is supporting projects that explore novel ideas with the potential to move HD research in new directions. Funds may be requested to support a broad range of activities – from fundamental pre-clinical studies to clinical work, including biomarker identification and pilot studies to support clinical trials. To date, **524 applications** have been submitted and **103 seed funds have been awarded**, representing **>€3M** in research funding.

Notably, researchers from **13 European countries** have been supported by seed funds, highlighting the programme's broad reach and impact across Europe. Seed funding has fostered novel collaborations across Europe and beyond, and supported HD researchers across all career stages. Importantly, early-stage research supported by seed funding has secured funding for several larger studies and contributed to numerous scientific publications. Recent developments have seen the maximum budget per project increase to **€75,000** and new deadlines for submissions (**1 March** and **1 October** each year).

To help celebrate this important milestone, we are officially launching the **Seed Fund Impact Survey**. This will allow us to better understand the programme's reach and impact since its inception. If you are a past or current recipient of seed funding, we would greatly appreciate your input on the added value seed funding has provided. We welcome any insights you can share, including follow-on grant funding and research publications generated, as well as how your research may have impacted the broader community through outreach activities and by informing public policy and clinical practice/guidelines. We aim to identify the most successful aspects of the programme, as well as areas that could be further refined and enhanced to better support the HD community. In addition, we will feature successful seed fund projects that have generated significant impact as case studies in future issues of EHDN *News* and on our website.

Click to access the survey.

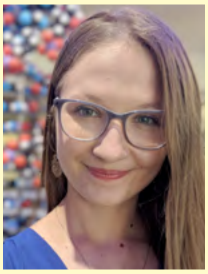
• • •

Roger Barker

Lesley Jones Seed Fund: Latest Award

EHDN has recently awarded seed funding to Roger Barker and team at the University of Cambridge, UK, and Johan Jakobsson and team in Lund, Sweden, for an exciting new project. The project will involve detailed analyses of human postmortem brains to test whether transposable elements (DNA sequences that can change position within a genome) that are usually silent become activated in HD, leading to expansion of the CAG abnormality and driving inflammation.

Flaviano Giorgini



Kinga Kolodziej

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 October 2026.

More information about the programme and how to apply is available here or you can contact Flaviano Giorgini (flaviano.giorgini@ehdn.org) or Kinga Kołodziej (kinga.kolodziej@ehdn.org) forfurther information.

EHDN Elections 2026

Biennial elections for the EHDN Executive Committee (EC) and Scientific and Bioethics Advisory Committee (SBAC) are now open, running from 1 June to 19 October 2026, in accordance with the EHDN Constitution. Results will be announced at the EHDN Clinical Research Congress in Kraków, Poland.

Executive Committee

The EC governs EHDN, directs and oversees its activities, and sets the network's strategic direction, as outlined in Article 4 of the Constitution. Election presents an important opportunity for members to contribute to the future direction of the network. Four positions will be filled in 2026, with votes cast for individual candidates.

Candidates standing for re-election:

Åsa Petersén

(current EC Deputy Chair)
Professor in Medical Research, Medical Faculty, Lund University, Sweden
Senior Consultant in Psychiatry, Region Skåne, Sweden

'I think the EHDN is a very important organization bringing together healthcare professionals, scientists, families and other stakeholders in order to advance clinical care and research in HD. The mission of the EHDN is aligned with my own and I would therefore very much like to continue my engagement in the network as deputy-chair. For future development, I envision that the EHDN can play an even more important role in defining standards of care, providing education and influencing developments in research and clinical trials.'

Edward Wild

Professor of Neurology, University College London (UCL), UK
Associate Director, UCL HD Centre, UK
Consultant Neurologist, National Hospital for Neurology & Neurosurgery, UK

'My focus on the committee has been, and will continue to be, ensuring that the Network funds and endorses the most promising science and rigorous clinical trials that represent the best use of the limited human and financial resources available to us as a community. Supporting newcomers to the field and the invaluable efforts of the working groups, and facilitating hopeful and accurate communication will remain a focus for me too. I hope that my continued presence on the Executive Committee will bring helpful continuity as we welcome new members in 2026.'

New candidates:

Mayke Oosterloo

Assistant Professor, Maastricht University, Netherlands (NL)
Chair, Expertise Center HD Maastricht University Medical Center (MUMC), NL
Movement Disorders Neurologist, MUMC, NL

'Looking ahead, I believe the future development of EHDN should include expanding educational initiatives such as webinars and podcasts for both clinicians and patients, to improve knowledge dissemination and engagement. In addition to the existing clinical fellowship, I see strong value in establishing a scientific fellowship to further support the development of researchers within the network. Finally, I believe that strengthening collaboration between EHDN working groups, facilitating funding opportunities for research, supporting (small) clinical trials, and contributing to the development of guidelines should remain priorities for EHDN.'

Niall Pender

Professor of Psychology/ Head of Department of Health Psychology, Royal College of Surgeons in Ireland
Clinical Neuropsychologist, Blackrock Health Hermitage Clinic, Dublin, Ireland

'I am particularly committed to strengthening the integration of research, clinical care, education, and advocacy, while supporting the next generation of HD researchers and clinicians. I am committed to broadening engagement and strengthening international partnerships in HD research. I would welcome the opportunity to bring energy, experience, and strategic leadership to support the next phase of EHDN's growth and impact.'

Niels Henning Skotte

Associate Professor, Department of Drug Design and Pharmacology, University of Copenhagen, Denmark

'My vision is for EHDN to become the global reference for HD biomarker development, where fluid biomarkers move from discovery to regulatory-qualified tools that shape how trials are designed and how therapies are approved. Building on the harmonized pre-analytics established through HDClarity, this requires standardized assays anchored in longitudinal cohorts, and biomarker endpoints validated not only against clinical scales but against what matters to patients and families. Delivering on this ambition depends on transparent collaboration across academia, clinical practice, and industry, and on the active inclusion of early-career researchers who will carry this work forward.'

Chiara Zuccato

Associate Professor of Pharmacology, University of Milan, Italy

'I would propose the creation of an HD Young Scientists Network within the EHDN community to connect early-career researchers working on HD across Europe. Activities could include an annual virtual meeting for young investigators, and a dedicated session during the EHDN official meeting. The goal would be to strengthen the next generation of HD researchers by establishing a structured international community of young scientists, sustained through regular activities. In addition, I would promote a dedicated session at the EHDN conference where young scientists meet the HD families. I envisage EHDN continuing to drive the translation of basic research into clinical trials, while supporting a network of young HD researchers actively engaged with the patient community.'

Scientific and Bioethics Advisory Committee

Established under Article 5 of the constitution, the SBAC meets regularly to review research projects submitted to EHDN requiring access to data and biosamples, applications to the Lesley Jones Seed Fund, and clinical trial protocols submitted for EHDN endorsement. Seven committee positions will be available in 2026 and will be voted on as a slate.

Constitution Amendment

A fifth constitutional amendment is proposed concerning Section 5.3.2: that the SBAC shall consist of a minimum of 12 members to ensure broad representation of the research and clinical interests of EHDN.

Who can vote?

All regular EHDN members are eligible to vote. Regular and associate membership is free – if you aren't already a member, find out more and apply here.

How do I vote?

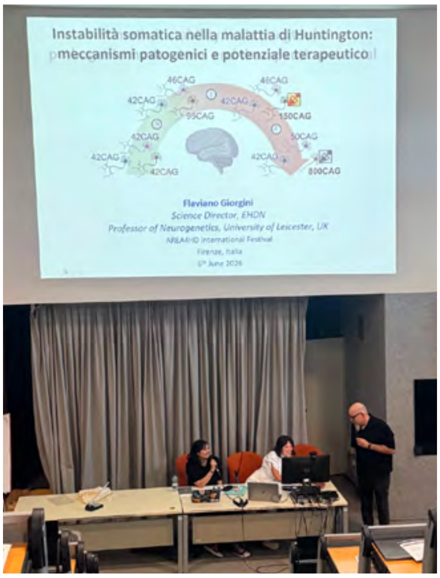
Most existing regular members will have already received a direct email from the Conventex/Genolive voting platform containing a unique voting link. If you have not received this (or require it to be sent again), please visit the voting platform directly to request online access and a link will be sent to you.

Questions?

For technical queries or any other questions, contact info@ehdn.org.

Hold Me Tight (dance)

Cara & Lyn – A Twisted Fantasy (graphic novel)



The Visible Gene: Art and Research in Huntington's Disease (scientific conference)

AREA4HD International Festival

Selene Capodarca, Co-Founder, That Disorder - Global Human Community

The first AREA4HD International Festival took place in Florence from 1 to 6 June 2026. Organised by That Disorder – Global Human Community ETS, the festival welcomed participants from across the globe.

Over six days, the programme brought together photography, documentary cinema, dance performance, live music, graphic novel, and seminars on neuroaesthetics, creative languages in rare disease communication, and cinema as a human rights tool.

Built around the three pillars of art, science, and advocacy, AREA4HD served as a platform for the wider HD and rare disease world. That Disorder presented Borderless (photography), Hold Me Tight (dance), Anticipation (documentary), Cara & Lyn – *A Twisted Fantasy* (graphic novel), and Carolyn (contemporary art). Further contributions included two photographic exhibitions about the Venezuelan HD families from Factor-H and Edith Cowan University's *Hidden World of Huntington's Disease* project.

AREA4HD
INTERNATIONAL FESTIVAL
Art | Research | Education | Awareness
Florence, May 31 - June 6, 2026

> *'The festival beautifully demonstrated how art, science, and lived experience can come together to educate, understanding, challenge stigma, and inspire change. Every conversation, presentation, exhibition, and performance reinforced the importance of keeping the voices and experiences of people and families affected by HD at the centre of our work.'*
>
> ***Cassie Caratti,***
> ***researcher with lived experience of HD.***

The scientific conference *The Visibile Gene: Art and Research in Huntington's Disease* combined sessions on art and empowerment with research and clinical presentations, bringing together a genuinely multidisciplinary audience of researchers, clinicians, families, and advocates. An energetic celebration of dance, live music, film and contemporary art provided a fitting close to the event.

The festival attracted significant interest from broadcast and news media. That Disorder intends to build on this success in future festivals – if you are interested in collaborating, please contact us!

EHDN Lancos Celebrate HD Awareness Month

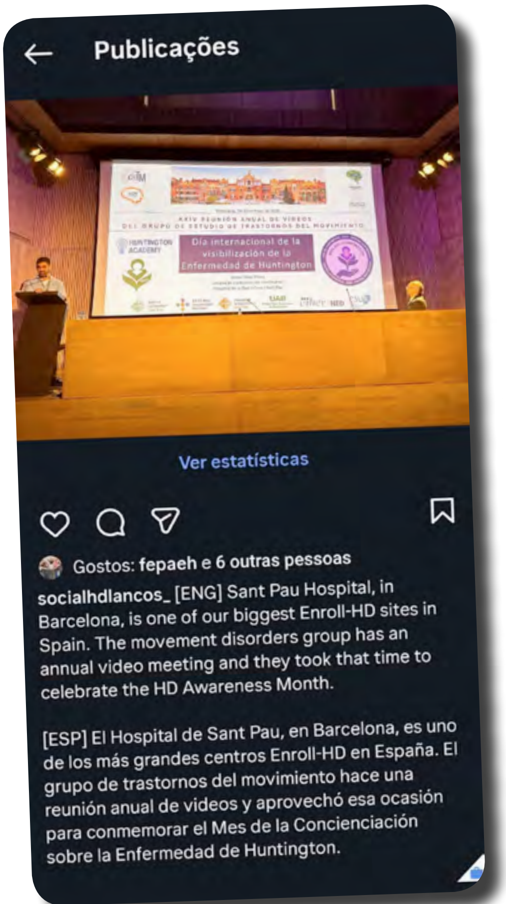
EHDN Lancos celebrated HD Awareness Month throughout May by sharing photos from their regional sites and associations across social media. Follow them to stay connected!

Instagram:
@socialhdlancos

BlueSky:
@socialhdlancos.bsky.social

Facebook:
@Social HD Lancos

TikTok:
@social.hd.lancos



Gostos: **fepaeh** e **6 outras pessoas**

socialhdlancos_ [ENG] Sant Pau Hospital, in Barcelona, is one of our biggest Enroll-HD sites in Spain. The movement disorders group has an annual video meeting and they took that time to celebrate the HD Awareness Month.

[ESP] El Hospital de Sant Pau, en Barcelona, es uno de los más grandes centros Enroll-HD en España. El grupo de trastornos del movimiento hace una reunión anual de videos y aprovechó esa ocasión para conmemorar el Mes de la Concienciación sobre la Enfermedad de Huntington.

Gostos: **jennaheilman** e **4 outras pessoas**

socialhdlancos_ In the spirit of HD Awareness Month, the Leicester team showed up to their team meeting in beautiful blue to help raise awareness of Huntington's Disease!

Some staff members are also taking part in HDWalk26, where they'll walk 202 miles throughout May, one mile for every ENROLL-HD participant enrolled at their site. They're currently sitting in 2nd place on the leaderboard. Go Leicester!

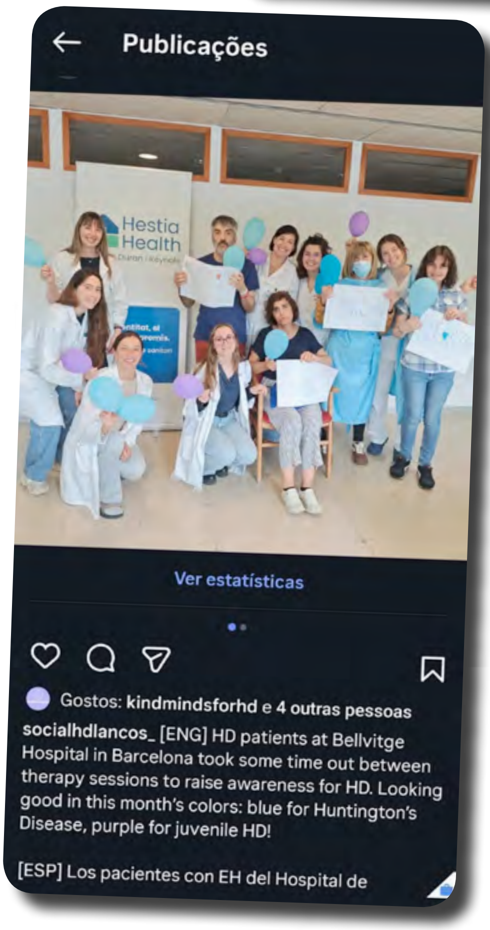
#hdawarenessmonth
#huntingtonsdisease

Gostos: **hdyofeed** e **5 outras pessoas**

socialhdlancos_ The clinical genetics & research team at Leeds, one of our Enroll-HD sites, is looking sharp wearing blue for HD and purple for juvenile HD.

#huntingtonsdiseaseawarenessmonth
#huntingtonsdisease

22 de maio



Gostos: **kindmindsforhd** e **4 outras pessoas**

socialhdlancos_ [ENG] HD patients at Bellvitge Hospital in Barcelona took some time out between therapy sessions to raise awareness for HD. Looking good in this month's colors: blue for Huntington's Disease, purple for juvenile HD!

[ESP] Los pacientes con EH del Hospital de

Catherine Deeprose

Sarah Tabrizi Awarded CBE in King's Birthday Honours

Congratulations to Sarah Tabrizi, Director of the UCL Huntington's Disease Centre, UK, on being appointed a Commander of the Order of the British Empire (CBE) in the King's Birthday Honours for her outstanding services to people with HD. Sarah commented: 'This award reflects many years of collaborative effort with an extraordinary community of researchers, clinicians and, above all, individuals and families affected by Huntington's disease. I am profoundly grateful to the patients and families who have worked alongside me and contributed to research over many years – their commitment makes progress possible, and their resilience and tremendous courage inspires everything I do.'

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Postcard from Palm Springs 2026

The Postcards are a series of video roundups aimed at non-scientists that convey the major themes, scientific breakthroughs, and clinical trial updates from conference series that CHDI organises: the Huntington's Disease Therapeutics Conference held (most usually) in Palm Springs, California in February, and the Huntington's Disease Clinical Research Conference in collaboration alternately with the Huntington Study Group and the European Huntington's Disease Network in the US and Europe in September/October.

The latest Postcard is now online, featuring highlights from the 21st Annual CHDI Huntington's Disease Therapeutics Conference held in February.

Publication Spotlight

'Coping with Huntington's: A Path to Better Mental Wellbeing' was published on 1 July 2026 by Sarah Gunn (University of Leicester, UK). The book offers practical mental health support and self-care strategies for people with HD and their families, grounded in a therapeutic approach. EHDN Chair Patrick Weydt described it as 'a very brave and personal book and an important resource. Moving and inspiring, it will be very useful for our community of people affected by or dealing with Huntington's.'

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Are You Sitting Comfortably? New Clinical Tips

EHDN's Occupational Therapy Working Group (WG) has published updated and expanded Occupational Therapy Clinical Tips for Huntington's disease – Seating and Lying. The evidence base primarily draws on UK documentation and guidance; however, the principles of postural management in HD apply globally. Seating and lying are highly problematic for people with HD, with consequences including reduced participation, deprivation of liberty, and injury for all parties, including families and caregivers. Alongside the updated tips, the WG will shortly submit a position paper to highlight these difficulties, which they hope professionals and the community can use worldwide.

The Occupational Therapy WG is also working to update its Community Mobility Tips, which include driving. They will bring these to the Occupational Therapy WG meeting at the EHDN Clinical Research Congress (12:00–1.30 pm on Thursday, 22 October*) and welcome collaboration.

To get involved in any of this work, please reach out to the Occupational Therapy WG Lead Facilitators (Alex Fisher, Manon van Kampen and Marieke Clemens) or EHDN Lanco Madalena Esteves.

**Please note that the programme is preliminary and subject to change. See ehdn.org/ehdn2026 for the latest updates.*

HEATED Task Force: News and Developments

Hugh Rickards (Honorary Professor of Neuropsychiatry, University of Birmingham) and Jenna Heilman (Executive Director, HDYO)

The HEATED (Huntington's Equal Access to Effective Drugs) Task Force (TF) brings together a group of people dedicated to identifying and overcoming the barriers that prevent people with HD from accessing effective drugs globally.

Over the past 10 years, we have seen an explosion of high-quality research aimed at developing effective treatments for HD. However, in a field dominated by molecular biologists, the issue of drug access has been relatively neglected. The view of the HEATED TF is that the true marker of success in drug development is the widespread availability of effective treatments – not just their existence.

We are pleased to announce that the HEATED TF will be holding its first plenary session at the upcoming EHDN Clinical Research Congress. Speakers include Josie Godfrey, a recognised expert in drug access. Josie has previously worked on other neurodegenerative diseases, such as Duchenne Muscular Dystrophy, where she is a driving force behind Project Hercules, a collaborative global initiative to overcome barriers to drug access for this condition. Josie will present work she is leading in HD to identify barriers to access and existing research gaps. In addition, Ignacio Muñoz-Sanjuan (Factor-H Founder and President) will present on the global landscape of drug access in HD. As many of you will already know, Nacho is an effective advocate for medicines and services for people with HD living in Latin American countries, including Venezuela.

The HEATED TF will also host a meeting at the congress (12:00–1.30 pm on Thursday, 22 October*), bringing stakeholders together to develop durable networks and make key decisions about what we need to achieve and how to achieve it. We warmly invite people with HD, families, advocacy groups, industry partners, researchers, clinicians, and all other interested individuals to attend.

If you would like to get involved or have any questions, please get in touch with Lead Facilitator Hugh Rickards, Co-lead Facilitator Jenna Heilman or EHDN Lanco Tomas Bernard.

Please note that the programme is preliminary and subject to change. See ehdn.org/ehdn2026 for the latest updates.

Researchers, clinicians, and individuals impacted by HD who want to contribute to HD research are welcome to join EHDN's working groups. Find out more: ehdn.org/about-ehdn/ehdn-working-groups/.

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EU Monitoring and EHDN Language Area Coordination Meeting

Catherine Deeprose

The latest EU Monitoring and EHDN Language Area Coordination Meeting took place in Brussels, Belgium, 26–28 May 2026. Presentations included updates on core EHDN and Enroll-HD activities as well as a dedicated session on regulatory and clinical science by Pavlina Stefanova-Konstantinova (EHDN). Further highlights included a poignant yet uplifting guest talk by Charlotte Spearing (see interview) and a scientific presentation by Flaviano Giorgini (EHDN) sharing insights into the HTT1a transcript in HD.

The latest EU Monitoring and EHDN Language Area Coordination Meeting in Brussels, Belgium, 26–28 May 2026

Update: Clinical Trials

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

Key updates since the last newsletters 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), along with details of the EHDN endorsement process, is published on the EHDN website.

Cardiff University is sponsoring an investigator-led feasibility trial of sertraline, Developing EVIdence for AntidepreSsant ChoicE to Treat Depression in Huntington's Disease (DEVISE-HD). There is uncertainty around the efficacy of antidepressants in treating depression in HD and the impact of antidepressants on HD progression. This double-blind randomised controlled trial (RCT) is designed primarily to assess the feasibility of conducting a full-scale RCT of the efficacy of antidepressants in HD. It will assess some of the current clinical and methodological uncertainties around the effect of antidepressants on disease progression in HD, including assessment of different measures of depression, HD clinical rating scales, and inflammatory biomarkers.

The trial plans to enrol approximately 40 participants with mild/moderate symptoms of depression, across three sites in the UK. Participants will be randomised to sertraline or placebo for 6 months. Depression and other HD symptoms will be measured at baseline and at 6 months; in addition, blood and cerebrospinal fluid will be analysed to assess whether treatment with sertraline affects inflammatory markers.

PTC Therapeutics reported 24-month interim results from the ongoing PIVOT-LTE study of votoplam in April 2026. This long-term extension study enrolled individuals who had previously participated in the 12-month phase 2 PIVOT-HD study. Participants who had received placebo in PIVOT-HD were randomised to 5 mg or 10 mg of votoplam daily; participants who had already received votoplam continued with their previously assigned dose.

The data suggest dose-dependent slowing of disease progression, as measured by the composite Unified Huntington's Disease Rating Scale (cUHDRS) at 24 months in Huntington's Disease Integrated Staging System (HD-ISS) Stage 2 participants compared with a matched natural history cohort from Enroll-HD. No treatment-related increases in neurofilament light (NfL) were observed at 24 months, and mean NfL levels in HD-ISS Stage 2 participants were reported as lower than baseline levels. PIVOT-HD, now led by Novartis, is planned to continue until mid-2027, with votoplam also being tested in INVEST-HD.

Novartis Pharmaceuticals: The first participant has been treated in the phase 3 INVEST-HD trial. There is continued progress in the global expansion of the study investigating the safety and efficacy of votoplam. The study is now open at sites across the US, Canada, and the UK. Additional sites are expected to open by the end of June in Australia, Argentina, China, Israel, Japan, South Korea, Switzerland, and Taiwan, which will broaden access to INVEST-HD trial participation. Additional information can be found here.

Skyhawk Therapeutics announced interim 12-month results from the phase 1/2 trial of SKY-0515 on 1 June 2026. Utilising propensity score-weighted analyses – which use Enroll-HD and TRACK-HD natural history datasets – positive mean changes from baseline Composite Unified Huntington's Disease Rating Scale (cUHDRS) scores were demonstrated at three, six, nine and twelve months for patients receiving SKY-0515. Recruitment is ongoing for the EHDN-endorsed FALCON-HD trial (see previous update in the March 2026 issue).

In April 2026, **uniQure** reported encouraging interactions with the UK Medicines and Healthcare products Regulatory Agency and plans to submit a Marketing Authorisation Application based on the data from the 3-year analysis from the ongoing European and US studies of AMT-130 in the third quarter of 2026. In the US, discussions continued with the Food and Drug Administration and on 17 June, uniQure announced that, during a recent Type B meeting, it was communicated that the 3-year analysis from the phase 1/2 study would be acceptable as the primary basis of a Biologics License Application (BLA) for the accelerated approval of AMT-130, intended to be submitted in the third quarter of 2026. In addition, the FDA seeks to align with uniQure on the design of the required confirmatory study prior to the BLA submission, including consideration of concurrent control on standard-of-care therapy instead of a sham procedure. uniQure is also exploring potential regulatory pathways for registration of AMT-130 in other regions and expects to provide a further update in the second half of 2026.

EHDN Endorsement

EHDN endorsement is a recognised marker of quality for HD clinical studies, demonstrating that a study has met rigorous scientific and ethical standards through independent expert review. Importantly, the process is designed not only to evaluate studies, but also to strengthen them through constructive feedback and specialist guidance.

Applications are reviewed by the independent Scientific and Bioethical Advisory Committee (SBAC), which assesses the study protocol, scientific rationale, safety considerations, feasibility, and the overall demands placed on participants, clinicians, and research sites. Based on these evaluations and recommendations, the EHDN Executive Committee (EC) makes the final endorsement decision. Applicants may also receive detailed feedback to help clarify outstanding questions and refine aspects of the study design.

For the HD community, EHDN endorsement offers reassurance that a study is scientifically robust, ethically appropriate, and developed with participant welfare as a key priority. This recognition can support informed decision-making by potential participants and clinicians considering involvement in clinical research. For sponsors, endorsement may also strengthen discussions with regulators and ethics committees, while increasing visibility through EHDN communication channels, including the website and newsletter.

The process begins with the submission of a study protocol and relevant supporting documentation, including the investigator brochure, with reviews typically completed within six weeks. Companies or researchers seeking further information can contact Jenny Townhill or submit an application to endorsement@ehdn.org.

Table 1: Current EHDN Endorsed Trials

Registration ID (CT.gov)	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	66	Canada, Germany, UK	Recruiting
NA	Cardiff University	DEVISE-HD	4	Sertraline	Selective serotonin reuptake inhibitor	Oral	Feasibility	40	UK	Recruiting
NCT05686551	Hofmann-La Roche	GENERATION HD2	2/OLE*	Tominersen	HTT lowering; ASO	Intrathecal	Disease modification	301 (actual)	Argentina, Australia, Austria, Canada, Denmark, France, Germany, Italy, New Zealand, Poland, Portugal, Spain, Switzerland, UK, USA	Active, not recruiting
NCT07246941	Hoffmann-La Roche	POINT-HD	1	RO7764733	mHTT lowering, ASO (SNP directed)	Intrathecal	Disease modification	40	Argentina, Australasia, Europe	Recruiting
NCT07326709	Novartis Pharmaceuticals	INVEST-HD	3	Votoplam	HTT lowering, splicing modifier	Oral	Disease modification	770	Global	Recruiting
NCT07378644	Skyhawk Therapeutics	FALCON-HD	2/3	SKY-0515	HTT & PMS1 lowering, splicing modifier	Oral	Disease modification	400	Argentina, Brazil, Canada, Chile, Colombia, Georgia, Germany, Mexico, Peru, Poland, Spain, UK	Recruiting
NCT05243017	UniQure Biopharma B.V.	HD GeneTRX2	1b/2	AMT-130	HTT lowering; miRNA AAV delivered gene therapy	Surgical, intrastriatal	Disease modification	15	Poland, UK	Active, not recruiting
NCT05822908	Vico Therapeutics	VO659-CT01	1/2, open-label	VO659	HTT lowering; allele-preferential ASO	Intrathecal	Disease modification	68 (SCA1, SCA3 and HD)	Denmark, France, Israel, Germany, Netherlands, UK	Recruiting

Note. AAV = Adeno-associated virus; ASO = antisense oligonucleotide; HD = Huntington's disease; HTT = huntingtin; mHTT = mutant huntingtin; PMS1 = postmeiotic segregation increased 1; SCA1 = spinocerebellar ataxia type 1; SCA3 = spinocerebellar ataxia type 3; SiRNA = small interfering ribonucleic acid; SNP = single nucleotide polymorphism

Behind the Data: How Enroll-HD Maintains Data Quality

Olivia Handley, Enroll-HD Global Platform Director

Enroll-HD maintains a trained and experienced network of approximately 160 HD clinical sites across the world, with a well-characterised study population of more than 35,000 ever-enrolled[1] participants, offering comprehensive support to HD study sponsors through scientific consultation, guidance, and platform support coordination. This is facilitated through three dedicated Enroll-HD governance committees: the Clinical Trial Committee for therapeutic trials, the Scientific Oversight Committee for non-therapeutic trials, and the Scientific Review Committee for data and biosamples requests.

As the number of clinical trials for HD therapeutics continues to grow, so too does the demand for Enroll-HD data to support such initiatives. Pharmaceutical companies such as Roche, Novartis, uniQure, PTC Therapeutics and Skyhawk utilise external comparator datasets and trajectory modelling for patient populations, while linked Enroll-HD datasets support pre- and post-trial analyses for partners including Roche, uniQure and Prilenia. At the site level, a Study Site Data Use Agreement – currently executed at more than 100 sites – permits use of a site's own Enroll-HD data for research and provides access to site-specific cohort characteristics reports to help identify potentially eligible participants for trials and studies. Enroll-HD also supports nested studies, such as HDClarity, which utilise Enroll-HD participants and their existing data as an integral part of the study design. Together, these examples illustrate the platform's growing value in advancing global HD research efforts.

> *'As the number of clinical trials for HD therapeutics continues to grow, so too does the demand for Enroll-HD data to support such initiatives.'*
>
> ***Olivia Handley***

Ensuring that the data underpinning these initiatives are of the highest quality requires careful coordination across a wide and dedicated team. Enroll-HD sites receive training during initiation visits and are required to complete periodic retraining thereafter – including annual Unified Huntington's Disease Rating Scale motor certification – while maintaining up-to-date Good Clinical Practice certification.

Enroll-HD participant data are entered into the electronic data capture (EDC) system, with sites encouraged to enter data directly wherever possible. Each case report form (CRF) used within the EDC to collate data incorporates a series of edit checks designed to promote completeness and accuracy at the point of entry.

Once all CRFs for a visit have been submitted, the data enter a queue for remote data review, which operates on a monthly cycle. Data are exported and subjected to hundreds of programmed quality control checks looking for completeness and plausibility. The quality control checks look for possible issues both within individual CRFs and by comparing data across CRFs, and also examine the data longitudinally to identify data entry errors. Findings are communicated to individual sites by the Enroll-HD data monitoring team and resolved before the data progress to the next stage: onsite monitoring.

Onsite monitoring requires at least one Enroll-HD monitor (in Europe, these are EHDN Lancos) to visit a site and review source records against the study data. This process follows a risk-based data monitoring (RBDM) approach, focusing on the highest-risk data

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[1] *Defined as participants who have signed an informed consent form and started at least one visit.*

points and processes rather than requiring exhaustive verification of source data. RBDM enables early identification of underperforming or non-compliant sites, allowing corrective and preventative action to be taken before data are prepared for use in downstream datasets. A staggering 384 monitoring visits were completed by a dedicated global team of 33 monitors in 2025 alone. Maintaining this high volume of visits for the Enroll-HD study has only been made possible by long-lasting collaborative relationships among Enroll-HD sites, monitors, and the study management team.

Ahead of any dataset distribution, a final series of quality checks is applied to the data to confirm that the data meet the threshold for minimising risk of identification, that informed consent has been obtained, that the source data has been verified, and that additional quality checks specific to the dataset are in place.

This approach is increasingly aligned with standards expected by regulators: the Food and Drug Administration and European Medicines Agency recognise RBDM as best practice. This is particularly important in the context of Enroll-HD, where data are shared with multiple partners for modelling, comparator analyses, and nested studies. Ensuring high data quality is central to the platform's role in supporting the global effort to develop effective treatments for HD.

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HDClarity

Update: HDClarity

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

HDClarity contributes to one of the primary aims of the Enroll-HD platform by providing data and cerebrospinal fluid (CSF), plasma, and serum samples that researchers can use to identify and evaluate biomarkers for HD. A biomarker is a measurable indicator of disease progression that helps researchers better understand how HD affects the brain. Improved biomarkers for HD may help accelerate the development of therapeutics that will benefit the HD community.

To streamline access to data and samples, datasets from studies such as HDClarity are periodically released from participant data collected at study visits worldwide, which is then coded to protect participant identities. The periodic datasets, and corresponding biosamples, are made available to researchers from academia, government institutions, nonprofit organisations, and public and private companies worldwide via the Enroll-HD platform.

The first HDClarity dataset was created in 2018 and included cross-sectional data from just 121 participants located across four countries. Two further datasets were released in 2021 and 2023, and the study was redesigned for longitudinal data and sample collection, providing invaluable repeated observations from the same participants. As a result, the fourth HDClarity dataset, which has just been released, includes data from 999 participants at 38 sites across nine countries. It comprises 1,440 sets of visits, each including one or more successful CSF collections, which represents an increase of over 70% compared to the last dataset in 2023. The HDClarity team is very excited about the potential of this dataset to support new and ongoing HD research, and we are indebted to our participating sites and HD families.

For further information about requesting HDClarity samples for research, please see enroll-hd.org/for-researchers/access-data-biosamples. The current HDClarity protocol and lab manual are available at hdclarity.net, and the UCL Central Coordination team is always happy to answer any questions about the study (hdclarity-cc@enroll-hd.org).

Update: Fellowship, Education and Funding

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

Fionnuala Margreiter

Juliana Bronzova

2026 Fellowship Programme

(written with Asun Martinez)

Six applicants were selected for the 2026 Fellowship Programme, together with an additional placement carried over from the 2025 programme. All placements are now organised in host clinics in Spain (Burgos, Madrid, Barcelona and Bilbao), Portugal (Lisbon), France (Paris) and the UK (Birmingham). Many thanks to all the clinics and local language coordinators who have agreed to host fellows and support this valuable programme.

Clinics interested in hosting a fellow for future programmes are invited to register their interest by email to fellowship@ehdn.org and the Fellowship Team will follow up with you directly.

The first placement started in June 2026. This year, most of the fellows will be able to combine their fellowship placement with the EHDN Clinical Research Congress in October.

We are delighted to hold the second alumni face-to-face meeting in Kraków during our lunchtime EHDN Fellowship workshop, chaired by Juliana Bronzova, on Friday 23 October. A fellowship poster for the poster session during this congress has also been prepared in collaboration with current and past fellows.

Meet the 2026 Fellows

Name	Photo	Home country	Profession	Host clinic
Yasser Mecheri		Algeria	Neurologist	Paris, France
Elizabeth Castro		Venezuela	Neurologist	Barcelona, Spain
Andrea Zamora		Uruguay	Neurologist	Madrid, Spain
Gustavo Barbosa		Brazil	Neurologist	Lisbon, Portugal
Amos Salami		Nigeria	Neurologist	Birmingham, UK
Natalia González Rojas		Argentina	Neurologist	Bilbao, Spain
Carolina Hernández		Chile	Neuro-psychologist	Burgos, Spain

Fionnuala Margreiter

2027 Fellowship Programme – Applications Opening 1 September

Gain hands-on experience in multidisciplinary HD care at leading European centres through the six-week EHDN and Movement Disorder Society – European Section (MDS-ES) Fellowship Programme. Assistance grants are available for fellows. Applications for 2027 will open on 1 September 2026. Learn more at ehdn.org/hd-clinicians-researchers/fellowship-programme.

Communication and Education Group News

EHDN was delighted to participate in the recent European Academy of Neurology (EAN) Congress in Geneva from 27–30 June 2026 as an exhibition partner. This was an excellent opportunity to showcase EHDN's activities and to promote the upcoming EHDN Clinical Research Congress 2026. Collaborative discussions were also held with various organisations, including the MDS-ES, the European Reference Network for Rare Neurological Diseases (ERN-RND), and the European Academy of Neurology. EHDN and the EAN continue to work closely on the co-promotion of activities such as the regular EAN congresses and EHDN's Clinical Research Congress 2026.

Factor-H

EHDN is pleased to continue supporting the work of Factor-H through the provision of online Spanish-language course material developed during the courses organised by EHDN and MDS Latin America. In addition, a Spanish-speaking genetic counselling expert will be supported to take part in a dedicated workshop at Factor-H's upcoming third Latin American HD Conference in Medellín, Colombia, this September.

Grants

Grant opportunities and updates are regularly shared on the EHDN website. An overview of individual and collaborative grant opportunities is available at ehdn.org/hd-clinicians-researchers/grant-manager/.

Get in Touch with the Think Tank!

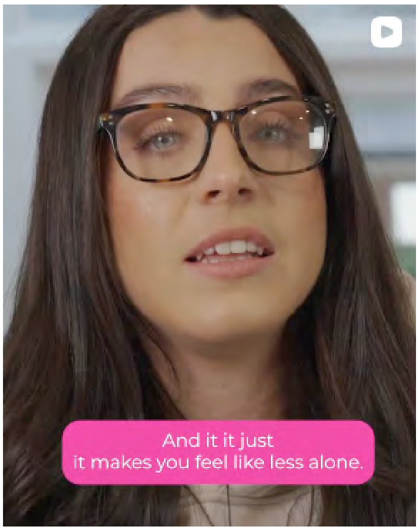
EHDN's Think Tank brings together EHDN members and staff who are closely involved in supporting scientific research – including members of the Executive Committee, Central Coordination and the working groups – and it engages with the HD research community in three ways:

- 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

Catherine Deeprose

July 2026 · Issue 58



Less alone

Huntington's Disease Awareness Day, Malta, 2026

All photos kindly provided by Charlotte Spearing

Changing the Conversation: An Interview with Charlotte Spearing

Charlotte Spearing is a UK-based HD advocate, caregiver, and ambassador whose work spans social media, charities, and national and international advocacy and support organisations. Appointed to EHDN's Executive Committee as its first young adult representative in 2024, she has made it her mission to change the conversation about HD – one post, one presentation, one connection at a time.

How did you get involved in advocacy?

As a member of an HD family, I decided to do a fundraiser for the Huntington's Disease Association at the age of 18. I Googled what you could do when you were 18 that you couldn't do when you were 17 – and it said you could go skydiving. So I did a skydive! After checking with my family that they were happy for me to talk about it, I shared information online about why I was fundraising. I found that people were really receptive to learning more about HD. In sharing my own story, I found an online community with lots of opportunities for involvement with different organisations, and I found all of this work personally very rewarding.

Charlotte and her mother

@thehdhun

Tell us about 'the HD hun'

This is my advocacy and fundraising Instagram account, set up in 2022. I'd been inspired by other people posting about their experiences in HD families, and realised that in relating to these, I felt much less alone. I decided that if sharing my and my family's journey helps even one person, then it would be worthwhile.

Although I'm generally quite an optimistic person, I pride myself on providing an honest perspective and not sugar-coating things. My mum is really passionate about openly sharing our experiences of life with HD and helping people understand the realities of the disease. My nan never spoke of HD – even with us – so this openness that we have is hugely different. I find writing very cathartic and often share just my own thoughts. Often, when I've shared these thoughts online, people reach out to say they didn't realise anyone else felt the same way.

TikTok came about by accident! After I got my gene-positive test result, I made a video that I thought nobody would watch. It was basically me describing my HD story, that I'd been tested, and that I wanted to live every day and make the most of life. And that went viral – it's been viewed by about half a million people! Lots of people started messaging me to ask more about my experiences and to ask questions. My TikTok and Instagram accounts have continued to grow over the years.

What other advocacy work are you involved in?

I became an ambassador for the Huntington's Disease Youth Organization when the programme first launched in 2021 – now there are ambassadors from over 30 countries, which is just mind-blowing! We started as a small group of people in the community who wanted to share their stories and use advocacy to support other people with HD. The ambassador role comes with opportunities to give presentations, share information on social media, and connect with other people impacted by HD. For example, just last week, I got a message about someone thinking about going through testing. As an ambassador, I was asked if I would like to connect with them directly and share my own experiences, because not everybody feels comfortable messaging on social media.

My work with the Huntington's Disease Association is quite similar. I also sit on HD-CAB, which is a partnership between the Huntington's Disease Youth Organization, the European Huntington Association, and the International Huntington Association that allows us to be selected for various advisory boards, such as those for pharmaceutical companies.

HD Gratitude Day, 2026

> *'You lose so much control when you are impacted by HD, and for me, advocacy gives that control back.'*
>
> ***Charlotte Spearing***

Taking part in HD-YAS, 2025

I am also part of HD Youth Voice, which is a Huntington's Disease Association project funded by the National Lottery. Here, we work on various projects for young people impacted by HD across the UK. For example, we've developed a teacher guide, which provides information for people working in schools about HD. We've also developed an employment guide and initiatives to help support people's mental health. There are currently 12 of us, and to be involved, you have to apply and be appointed. Sadly, this is my last year because I'm getting too old. And of course, I sit on EHDN's Executive Committee as their young adult representative, which is exciting!

What is your driving force?

I want to empower the HD community to talk about HD. This doesn't have to mean talking at conferences or sharing your life story with thousands of people online – it could just be sharing something about HD with a friend.

> *'I want to empower the HD community to talk about HD'*
>
> ***Charlotte Spearing***

I know I'm going to have the symptoms of the disease, and I've seen it play out multiple times. My great-nan had HD, and I visited her as a toddler in a nursing home. My nan had HD, and I cared for her throughout our time together. Now my mum has it, and I understand what the future looks like for me.

I think it's really important to grab life by the proverbial horns and make the most of every day. At the same time, I'm doing everything in my power to improve things for the future, not only just for me but for other people as well. I like the idea of leaving a legacy, and I don't want my suffering or my family's suffering to be in vain. You lose so much control when you are impacted by HD, and for me, advocacy gives that control back. It gives me a reason to keep going, and I know positive things will come of it. If I have kids one day, I hope they will see a world in which HD looks completely different.


Running the London Marathon, 2025

Instagram: ***instagram.com/thehdhun/***
TikTok: ***tiktok.com/@thehdhun***

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Dates for Your Diary

- **1 June – 19 October 2026:** Voting period for the EHDN Elections 2026.
- **1 September 2026:** Applications open for the EHDN and Movement Disorder Society – European Section (MDS-ES) Fellowship Programme 2027.
- **2–4 September 2026:** Third Latin American Huntington's Disease Conference takes place in Medellín, Colombia, organised by Factor-H in partnership with the University of Antioquia and the Fundación de Antioquia.
- **1 October 2026:** Applications close for the EHDN Lesley Jones Seed Fund programme.

- **22–24 October 2026:** EHDN Clinical Research Congress takes place in Kraków, Poland. Registration is open from 1 June to 31 July.
- **12–14 March 2027:** Huntington's Disease Youth Organization's International Young Adults Congress takes place in Barcelona, Spain.

Would you like to share an upcoming event with our readers?
Please email the details to newsletter@ehdn.org

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