



Photo: EHDN

A snapshot of the Enroll-HD 10-year celebrations

## Updates, Insights and Perspectives on the Drive for Positive Change

Catherine Deeprose

It's been a busy few months in the HD field, as reflected in our July edition of EHDN News. In addition to featuring important updates from our regular contributors, we share insights and perspectives on clinical research developments, the latest LanCo meeting, a new study looking at the benefits of music and drumming, and the Multidisciplinary Working Group's recently published position statement. We are also delighted to include an interview with Jenna Heilman, Executive Director at the Huntington's Disease Youth Organization.

### BREAKING NEWS:

Preliminary data from PTC Therapeutics and uniQure on their huntingtin-lowering trials are encouraging!

We look forward to further updates.

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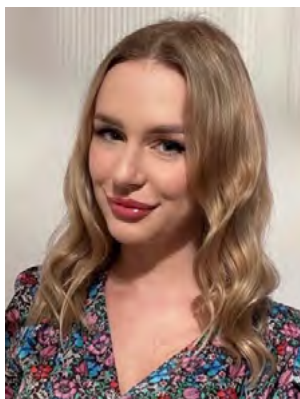
## A Postcard from the Paris LanCo Meeting

Marta Łaciak, LanCo for Poland

**LanCo Meetings have already become an indispensable part of the Enroll-HD study and a critical part of our work. After a very successful two-day meeting in Barcelona, the EHDN team and part of the CHDI team once again came together to discuss important issues and Enroll-HD. This time, the meeting took place in Paris, the capital of France, and although it was a bit shorter than usual, we had enough time to exchange insights and experiences and also integrate with people who had recently joined the team or whom we had only met online so far.**

### Paris LanCo Meeting

Most of us arrived on 11 May to go to the LanCo dinner. This is always a great opportunity to catch up after a long time of not seeing each other face to face, and to talk and relax after travelling. While it was only the second LanCo dinner that I have had the pleasure to attend, I will remember it for the rest of my life. Frankie (Franziska Bernsdorff – Event Manager) surprised us by taking us on an amazing culinary and also a literal



journey through the most beautiful places in Paris, which we viewed from a bus with audio guidance.

The next day, the LanCo meeting consisted of 11 thematic sessions. Each lasted between 15 to 60 minutes, depending on the complexity of the topic, led by experts from the EHDN and Enroll-HD – all of whom are well known to us.

Among the topics discussed, we heard about brain banking in early HD, a review of platform studies, feedback from the HDYO International Young Adults Congress in Glasgow and of course, Enroll-HD focussed topics such as study and monitoring updates, data security, new standard operating procedures and newly adopted requirements. As usual, the audience did not disappoint and asked the presenters a lot of detailed questions during the Q&A sessions. These questions asked often turned into lively discussions – which I personally believe is the most important part of each LanCo meeting. This exchange of experiences is incredibly valuable in facilitating our work. It is also an important opportunity to tighten bonds between us and create new friendships. Many interesting discussions continued during breaks and even remotely when we returned home.

As usual, the meeting passed very quickly, and after it, there was sadness that we won't see each other for another four months. Fortunately, the time between meetings passes as quickly as the meeting itself and so we look forward to seeing each other again and learning more about the world of science and of course, Enroll-HD. Every meeting is inspiring and I'm always full of hope that we will hear even more about breakthroughs in HD research.



Photos: EHDN

**The Paris LanCo meeting was a great opportunity to meet each other in person**



## An Exciting New Initiative: HD-DRUM

Claudia Metzler-Baddeley,  
Cardiff University, UK

**HD-DRUM is based on research suggesting that music and percussion may help with movement and concentration problems after brain injury or disease. After 2 months of Bongo drumming, we observed changes in the brain connections of people with HD. World-renowned drummer Trey Gray shares his experience with HD and drumming here in an interview with the HD Youth Organisation <https://youtu.be/15ZHHk2 Rhs>.**

We are inviting Enroll-HD volunteers to help trial a movement training app at Cardiff University (funded by Health and Care Research Wales). In HD-DRUM, people listen to audio recordings of musician Jimi (pictured) who explains how to drum along on two virtual drums on a tablet (a blue triangle and a red circle, also pictured). This is done first without and then with background music. As people progress through the programme, these patterns get gradually longer and faster. Participants practise until they reach a level of competency meaning they unlock the next and more difficult level.

We plan to randomise 50 people with HD into one of two groups: the HD-DRUM group or a control group. We will lend people in the HD-DRUM group a tablet with the app and ask them to use the app at home for 10 minutes, 5 days per week over 2 months. People in the control group will simply continue their normal activities over this time – but can try the app out when finished in the study. We will also



HD-DRUM



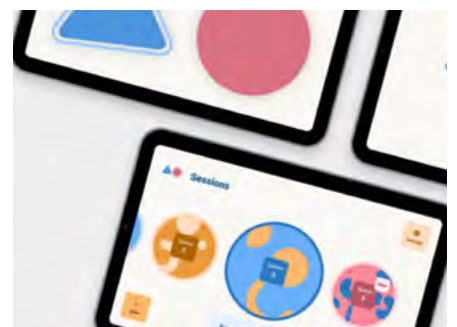
**Claudia Metzler-Baddeley explains the project**

ask participants to come to the Cardiff University Brain Research Imaging Centre for some mental function and motor assessments and brain imaging before and after the 2 months. We are also looking for healthy volunteers to test the app and undergo all the same tests.

We hope to find out whether people enjoy the HD-DRUM training and find it beneficial. We also would like to find out if there are any changes in mental and movement functions and the brain after 2 months of HD-DRUM training.



**Jimi explains how to drum along**



**HD-DRUM offers progressively difficult levels**

**If you are interested in taking part and would like more information, please email Claudia Metzler-Baddeley and Vasilis Ioakeimidis at [hd-drum@cardiff.ac.uk](mailto:hd-drum@cardiff.ac.uk)**

## Publication Spotlight: A Position Statement on Multidisciplinary Treatment and Care

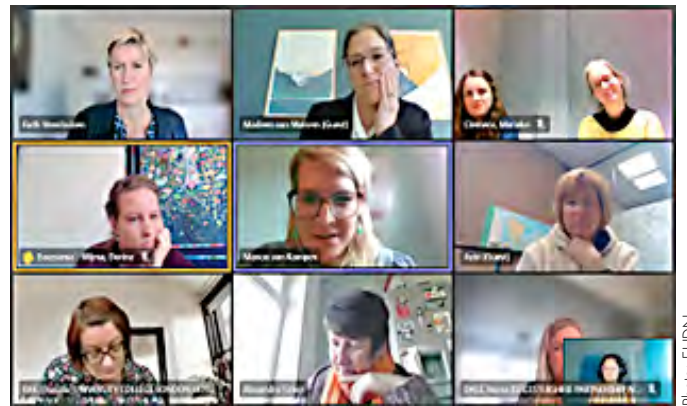
Recently, the EHDN Multidisciplinary Working Group (MDT WG) published the paper *'What we don't need to prove but need to do in multidisciplinary treatment and care in Huntington's disease: a position paper'* in the peer-reviewed, open access journal [Orphanet Journal of Rare Diseases](#). Authors Alex Fisher, Astri Arnesen, Travis Cruickshank and Ruth Veenhuizen explain why this work is so important.

Whilst the development of disease-modifying treatments continues, we (the reformed MDT WG) believe that the provision of optimal care for people living with HD should also be reflected in the EHDN's mission.

The MDT WG brings together the array of professionals needed in HD care. Importantly, the group is also supported by those with the lived and living experience of HD. In early 2023, our position statement was published in the Orphanet Journal of Rare Diseases. The intention was to summarise the current MDT knowledge base and critically, our collective wisdom on what has been done, what has not been done, and most importantly, what needs to be done right now both practically and politically.

The main focus was a need to communicate several pragmatic imperatives. First and foremost, the WG wanted the paper to be an immediate 'go-to' regardless of the reader's knowledge. Beyond this, it needed to break down commonly held myths. Martha Nance called this 'therapeutic nihilism' in 2012 and the WG targeted the 'we can't do that because there's no evidence' mentality to show that the MDT rehabilitation approach in HD has demonstrated benefits both collectively and in isolation.

The statement is the first to recognise the distinctly 'different' way in which HD specialists practice. This interdisciplinary practice and the stretching beyond the scope of a single discipline is emphasised, as it reflects the complexity of the illness and the inherent flexibility required – without which, as the paper highlights, individuals and families may be left in precarious states.



*The Multidisciplinary Working Group represents a unique collaboration of experts*

Whilst the WG feel that the specialist knowledge that can be found within an MDT should be at the heart of global HD community care, and professionals within it facilitated through funded structures to develop practice guidelines which value their contribution, they also recognise the disparity and inequity of resources world-wide, and that a different way of supporting the community and those working within it is also needed. Many of the WG 'practice' within differently resourced systems and models but in the main, working within state- or insurance-funded systems is most typical.

A key question surrounds what actually happens when access to specific resources or particular professionals just isn't possible. Does the 'optimum' care model meet reality? With this in mind, the WG is now exploring ways of tailoring the interdisciplinary nature of HD MDT practice to the patient and family journey at an individual and local level, wherever that might be. Meaningful and accessible resources that offer HD health literacy and that are community-facing are critical. The position statement has received a warm welcome from advocacy associations and many are hosting it on their various websites.

***If you are interested in being part of this work, please get in touch.***

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## Update: Clinical Trial Developments

Jenny Townhill and Tim McLean, Central Coordination

Key updates for EHDN-endorsed trials and studies presented at [HDIC](#) are provided below; please refer to [Table 1](#) for a summary of the main study information. An expanded description of all EHDN-endorsed trials and studies (completed and in progress) as well as details of the EHDN endorsement process is available on the EHDN website: [ehdn.org/ehdn-news-update-clinical-trials](https://ehdn.org/ehdn-news-update-clinical-trials)



**Novartis:** the VIBRANT-HD phase IIb trial of branaplam, a small molecule mRNA splicing modulator, was suspended in August 2022, due to signs and symptoms of peripheral neuropathy in participants who received branaplam. Development of branaplam for HD was then discontinued in December 2022 following a further review of longer-term data safety which found that there were signs of nerve damage in around 85% of participants. Results of an interim analysis were presented, showing ~50% of these participants still had at least one sign/symptom at 25 weeks post-last dose. Neurofilament light (NFL) levels also increased in blood and cerebral spinal fluid (CSF) with duration of treatment. Participants continue follow-up safety monitoring visits and have 1–2 visits remaining. Final data will be shared towards the end of 2023 after the study completes.



**Prilenia:** Preliminary top-line results from the PROOF-HD phase III trial of pridopidine, an orally administered small molecule selective 51R agonist, were presented. Although pridopidine was well-tolerated, with no serious side effects, the primary endpoint (Total Functional Capacity Score), change from baseline to 65 weeks, and key secondary endpoint (composite Unified Huntington's Disease Rating Scale; cUHDRS) did not reach statistical significance. However, when participants taking neuroleptics and chorea medications were excluded from the primary analysis, nominally significant benefits were seen on some assessments, including the cUHDRS and Stroop Word Reading, across several time points. Further detailed analyses of the data will be presented when available.

Participants in the PROOF-HD Open-Label Extension and Expanded Access programmes will continue to have access to pridopidine, and Prilenia remains committed to developing pridopidine as a treatment for HD and amyotrophic lateral sclerosis.



**Roche:** the GENERATION-HD2 trial of tominersen (ASO) is now open to recruitment in multiple countries, with a total of 75 sites in 15 countries planned to participate. Findings from the GENERATION-HD1 trial have informed the design of GENERATION-HD2, which will include participants at an earlier disease stage and investigate lower and less frequent doses of tominersen. The primary outcomes are safety and biomarker data, and efficacy trends will be explored. Further data from the GENERATION-HD1 and GEN-EXTEND trials were presented, which provide support for the dosing regimens selected for GENERATION-HD2. Transient increases in NFL that occurred in GENERATION-HD1 were not observed in a GEN-EXTEND participant group who received tominersen every 16 weeks, whilst lowering of mutant huntingtin in CSF was still achieved.



**UniQure:** Updates were reported for the ongoing gene therapy surgical trials of AMT-130: HD-GeneTRX1 (US, sham-controlled) and HD-GeneTRX2 (Europe,



open-label). For HD-GeneTRX1, 28 surgical procedures have been completed in 26 participants [16 received study drug and 10 were sham-operated (2 of these have cross-over subsequently received study drug – ‘cross-over’ group)], with remaining cross-over surgeries planned for the second half of 2023. An additional planned cohort will address the objective of shortening the surgical procedure time.

For HD-GeneTRX2, 10 procedures have been completed and 5 participants are still to be enrolled in the high-dose group.

All enrollment is expected to be complete by 2H2023.

AMT-130 is generally well-tolerated, and trends in CSF mHTT for the low-dose group are supportive of target engagement, i.e. reduction of mHTT (~54% at 12 months).

**Table 1: Current EHDN Endorsed Trials and Studies**

Registration ID (CT.gov)	Sponsor	Trial name	Phase	Investigational Product	Mechanism of Action	Delivery	Target Enrollment	Location(s)	Status
NCT05541627	BrainVectis, a subsidiary of Asklepios BioPharmaceutical, Inc. (AskBio)	ASK-HD-01-CS-101	I/II	AB-1001	Cholesterol metabolism dysfunction (AAV gene therapy)	Surgical, striatal	18	France	Recruiting
NCT05686551	F. Hoffmann-La Roche	GENERATION HD2	II	tominersen	ASO	Intrathecal	360	North America, Europe, Oceania	Recruiting
NCT05111249	Novartis	VIBRANT-HD	IIb, OLE	branaplam	mRNA splicing modifier	Oral	75	Belgium, Canada, Germany, Hungary, Italy, Spain, UK, USA	Terminated; participant follow-up ongoing
NCT04556656	Prilenia Therapeutics	PROOF-HD	III, OLE	pridopidine	Sigma-1 receptor agonist	Oral	480	Canada, Europe, USA	Complete, OLE ongoing
NCT05358717	PTC Therapeutics	PIVOT-HD	II	PTC518	Small molecule mRNA splicing modifier	Oral	162	Australia, France, Germany, Netherlands, UK, USA	Recruiting
NCT05107128	Sage Therapeutics	DIMENSION	II	SAGE-718	NMDA receptor modulator	Oral	178	Australia, Canada, UK, USA	Recruiting
NCT05358821	Sage Therapeutics	SURVEYOR	II	SAGE-718	NMDA receptor modulator	Oral	80	Canada, USA	Recruiting
NCT05655520	Sage Therapeutics	PURVIEW	III, OLE	SAGE-718	NMDA receptor modulator	Oral	300	Australia, Canada, UK, USA	Recruiting
NCT05475483	SOM Biotech	SOMCT03	IIb	SOM3355 (bevantolol hydrochloride)	VMAT2 inhibition	Oral	129	France, Germany, Italy, Poland, Spain, Switzerland, UK	Recruiting
NCT04120493	UniQure	HD GeneTRX2	Ib/II	rAAV5-miHTT	miRNA AAV gene therapy	Surgical, striatal	15	Germany, Poland, UK	Recruiting
NCT05032196	Wave Life Sciences	SELECT-HD	Ib/IIa	WVE-003	Allele-selective ASO	Intrathecal	36	Australia, Canada, France, Germany, Italy, Poland, Spain, Switzerland, UK	Recruiting

**Note.** AAV = Adeno-associated virus; ASO = antisense oligonucleotide; mRNA = messenger ribonucleic acid; NMDA = N-methyl-D-aspartate; OLE = open-label extension; VMAT2 = vesicular monoamine transporter 2

## Clinical Trials Perspective: Therapeutic Development Highlights from the Annual CHDI Huntington's Disease Therapeutics Conference

The 18<sup>th</sup> Annual CHDI Huntington's Disease Therapeutics Conference was held 24–27 April in Dubrovnik, Croatia, and brought together scientists from academia and industry, companies developing therapies for HD and non-profit organisations to discuss the latest developments in HD research.

More than 150 scientific posters were presented, and speaker sessions covered a wide range of topics including advances in understanding the DNA and structure of mutant huntingtin, genetic modifiers, potential new candidates for therapies and the development of biomarkers. A summary of the conference can be found at <https://en.hdbuzz.net/343> and presentations will be available on the CHDI website soon.

## New Therapeutic Approaches in Development

**Alchemab Therapeutics:** Alchemab's platform combines computational discovery and laboratory sequencing techniques, using samples from millions of individuals, to identify protective antibodies associated with increased resilience to neurodegenerative disease. They have identified HTT (huntingtin) antibodies that are convergent across 'at-risk' resilient patients with frontotemporal dementia, Alzheimer's disease and Parkinson's disease, but not in 'progressors' from these groups.

Their lead antibody, ATLX-1095, binds to multiple forms of mHTT and has been shown to be highly effective in reducing HTT in vitro. It is currently undergoing proof-of-concept testing in R6/2 mouse models.

**Alnylam Pharmaceuticals:** Alnylam announced that they have a new C16-siRNA conjugate in development for HD that combines a small molecule, C16, which binds to neuronal receptors, with a siRNA drug. This allows the drug to cross the blood-brain barrier (BBB) and deliver the siRNA drug to brain cells. They presented early data showing a large reduction of HTT protein in the cortex of non-human primates (NHP) with effects lasting at least 3 months.

**Arvinas Operations:** PROteolysis Targeting Chimera (PROTAC) small molecules exploit the ubiquitin-proteasome pathway, one of the body's protein degradation systems. PROTACs work by binding an E3-ubiquitin ligase and a target protein together, thereby instructing cells to dispose of the target protein. Orally administered PROTACs have been shown to cross the BBB of NHPs and are distributed in deep brain structures. Arvinas identified a novel ligand that can be used in PROTACs that selectively target mHTT. They have data from in-vitro models showing good binding of the ligand with mHTT, a potent reduction in both soluble and aggregated mHTT and sparing of wild-type huntingtin (wtHTT). This ligand is now being tested in mouse models.

**LifeEdit Therapeutics:** LifeEdit have a gene editing platform and are exploring how CRISPR DNA editing can be used to edit the huntingtin gene. They have developed new RNA-guided nucleases which are delivered via an adeno-associated virus (AAV-5), and are allele-selective, allowing separate targeting of specific single nucleotide polymorphisms (SNPs) that are only associated with the expanded gene. They shared new preclinical data testing different versions of their compound LEG-SNP-AAV in YAC128 and BACHD transgenic mouse models and have demonstrated dose-response reductions of mHTT of >50% in striatal mHTT protein, with effects that last at least 12 weeks.

**VectorY Therapeutics:** Vectorised Transformative antibodies (VecTabs) are being developed by VectorY. Using in vitro models, they identified VecTab candidates that selectively reduce soluble and aggregated mHTT, then developed VTx-003 which uses their new technique called VecTron. VTx-003 comprises two parts – a transgene that instructs cells to make the vectorised antibody and an AAV vector, into which the transgene is packaged for delivery to the cells. This approach exploits the proteolytic pathway, with the antibody binding to mHTT protein and labelling it for degradation. VTx-003 has been tested using in vivo models, including mice and pigs, and reduced mHTT aggregates with no serious side effects. R6/2 mice also showed some improvement in motor function.

**Vico Therapeutics:** Vico's VO659 drug, an allele-preferential ASO for the treatment of polyglutamine diseases, is currently being tested in a phase I/IIa basket trial for HD, SCA1 and SCA3. VO659 targets expanded CAG repeats in the mutant mRNA transcript, and both inhibits mRNA translation and induces exon-skipping. In

proof-of-concept HD mouse studies, VO659 significantly reduced mHTT protein and improved phenotype, including motor performance. Tests in HD patient fibroblast cell models have also shown an allele-preferential reduction in mHTT with the most marked reduction in fibroblasts from JHD patients (who have longer CAG repeats).

**Voyager Therapeutics:** newly engineered AAV capsids (TRACER-AAVs) in development by Voyager can cross the BBB using intravenous delivery, unlike conventional

AAVs, and can therefore be used to deliver gene therapies to the brain. Testing of vectorised siRNA capsids in several species, including NHPs, has shown improved penetrance and biodistribution in the brain compared to conventional AAVs. This technology could be used to deliver multiple therapies for HD simultaneously, for example, an HTT lowering drug and a drug that targets MSH3 (the DNA mismatch repair protein which is involved in somatic instability of the mHTT gene which is a genetic modifier of onset and severity of HD).

• • •



## Update: Enroll-HD

Olivia Handley, Enroll-HD Global Platform Manager

Ten years ago, on 25 July 2012 in Tennessee, the first participant entered Enroll-HD. Since reaching the 10-year milestone last summer, the Enroll-HD team has been celebrating with sites and lay associations across regions, reflecting on the remarkable achievements that we, as a community, have made over the last decade.



Photo: Gabriele Stauner - artifax.com

hang up in their clinical or research space. The data viz is an illustration of every participating site in the Enroll-HD study. It has been organised such that each site is named with a visual representation of which region they are from (coloured star), how long they have been in the study (coloured bar), and how many participants have ever been recruited (grey dot). The display is centered around the Enroll-HD rosette which represents the community Enroll-HD has been able to build together.

We have also been fortunate to attend many family and lay association meetings in Spain, Scotland, Belgium, Norway, Denmark, Germany, Spain, Poland and Italy. The [Huntington's Disease Youth Organization](#) (HDYO) inaugural in-person Congress was held in Glasgow in March this year. The event was attended by approximately 350 young adults (18–40 years old) as well as healthcare professionals, researchers and pharmaceutical companies. HDYO kindly invited the Enroll-HD team to speak to the community about the 10-year milestone and also our achievements and hopes for the future. In June, the Enroll-HD team also attended the [Huntington's Disease Society of America's](#) Annual Convention and welcomed this opportunity to celebrate together and reflect on the impact of Enroll-HD.

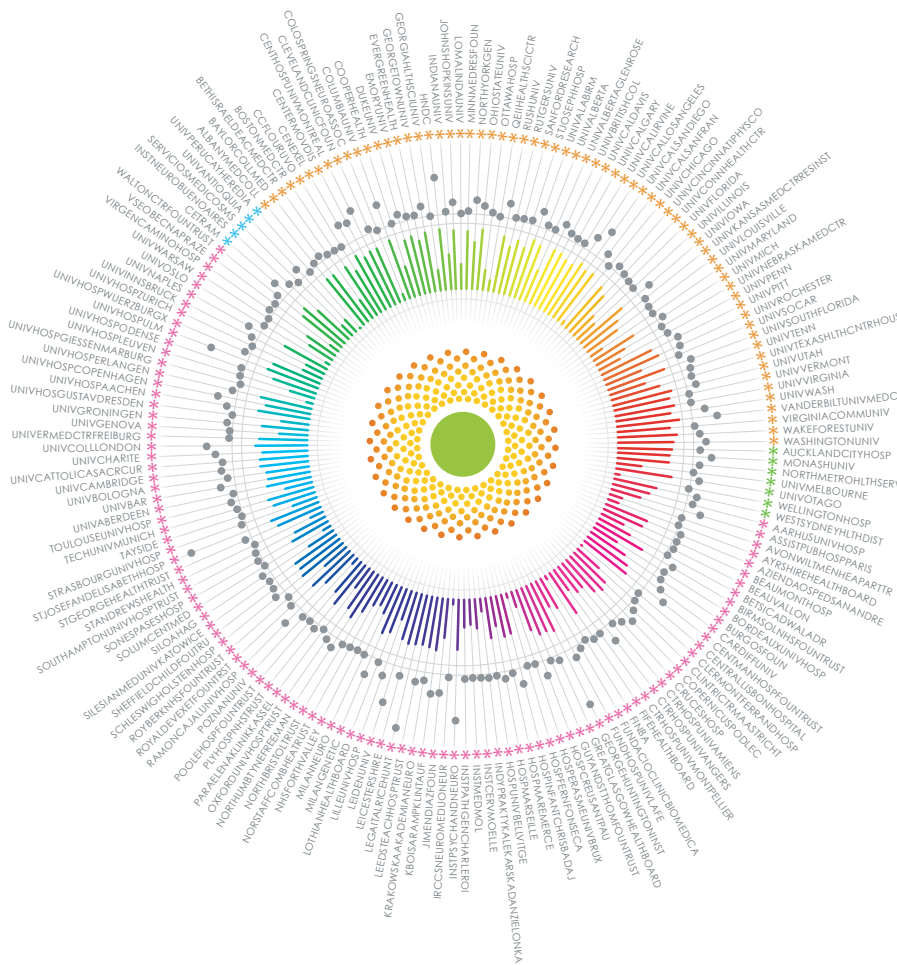
Photo: EHDN



Members of the Enroll-HD team have completed an impressive 74 10-year celebration events at Enroll-HD sites, with a further 19 events being made possible through

remote meetings. So far, the team has visited Colombia, US, France, Poland, Italy, Spain, Portugal, UK, Denmark, Belgium, Germany, Norway, Switzerland, Argentina, and Chile. As a token of our gratitude, each Enroll-HD site has been given an Enroll-HD 'data viz' to





ENROLL-HD STUDY SITES: 2012-2022

REGION; RECRUITMENT; TENURE

DATA SOURCE: ENROLL-HD | DATA CUT: JULY 25, 2022

Enroll-HD

The major aim of having these celebrations is to allow the Enroll-HD team to show their gratitude and recognition of the contribution and ongoing support received from site staff, the HD community, and also the lay associations. It has been hugely positive to share together the many successes borne out of the platform. Specific highlights include support for clinical trials, with around half of all participants in current or recently completed HD clinical trials also participating in Enroll-HD. The rich, high-quality data and biosamples have led to exciting and significant scientific breakthroughs such as identifying several genetic modifiers of HD and the development of a new classification system for disease staging in research. Through encouraging regular clinic visits, standardising assessment tools, training health care professionals, and creating and fostering a culture of excellence, Enroll-HD has been able to work towards improving clinical care in HD. Enroll-HD's achievements simply would not be where they are today without the dedication, hard work, and commitment of all those involved.

The Enroll-HD team will continue to organise and attend 10-year celebration events during the remainder of 2023. The Enroll-HD community is one that we have grown together, and we look forward to continuing to grow.



# HDClarity

## Update: HDClarity

Seema Maru, Study Coordinator, Gail Owen, University College London

The aim of HDClarity is to generate high-quality CSF collection and plasma samples (from blood) to evaluate biomarkers and pathways to enable development of novel treatments for HD.

All HDClarity participants must be part of the Enroll-HD study and HDClarity is aiming to recruit 2,500 participants worldwide. The latest version of the protocol includes a juvenile cohort and an incomplete penetrance group:

- |   |   |
|---|---|
| <ul style="list-style-type: none"> <li>• Control</li> <li>• Early Premanifest</li> <li>• Late Premanifest</li> <li>• Incomplete Penetrance</li> </ul> | } <b>18-75 years</b> – controls, early/late premanifest HD and incomplete penetrance HD |
| <ul style="list-style-type: none"> <li>• Early Manifest</li> <li>• Moderate Manifest</li> <li>• Late Manifest</li> </ul>                              | } <b>21-75 years</b> – early/moderate/advanced manifest HD                              |
| <ul style="list-style-type: none"> <li>• Juvenile Manifest</li> </ul>   | } <b>≥11 years</b> – juvenile HD  |

HDClarity consists of two annual visits (Screening and Sampling) within a 30-day window for a duration of four years in total. Some participants may be able to attend an Optional Sampling Visit 4–8 weeks later.

**Screening visit:** Confirm eligibility, participation on the Enroll-HD study and obtain written consent.

Safety assessments: Safety Bloods, Neurological and Physical examination.

**Sampling Visit:** UHDRS Motor, Neurological and Physical examination.

Sample collection: 20 ml CSF collection by lumbar puncture and 50 ml blood collection by venepuncture.  
Safety Phone call 1-3 days later.

**Optional Repeat Sampling:**  
Repeat of Sampling Visit procedures.

Only required for the first year of enrolment.

## Current Site Status

HDClarity is active at over 30 sites in 8 countries located across Australasia, Europe and North America (Figure 1). We are pleased to announce that since January 2023, we have opened new sites in Canada (Ottawa Hospital and North York General Hospital) and New Zealand (University of Otago).

We are currently working on opening new sites in Australia, Canada, Columbia, the Czech Republic, France, Germany, Italy, the Netherlands, New Zealand, Norway, Portugal, Switzerland and the USA.

North American HDClarity Sites



European HDClarity Sites



Australasian HDClarity Sites



Figure 1. HDClarity Clinical Sites

## Total Recruitment

HDClarity sites have successfully completed

- 824 Screening Visits
- 725 Sampling Visits and
- 111 Optional Repeat Sampling Visits

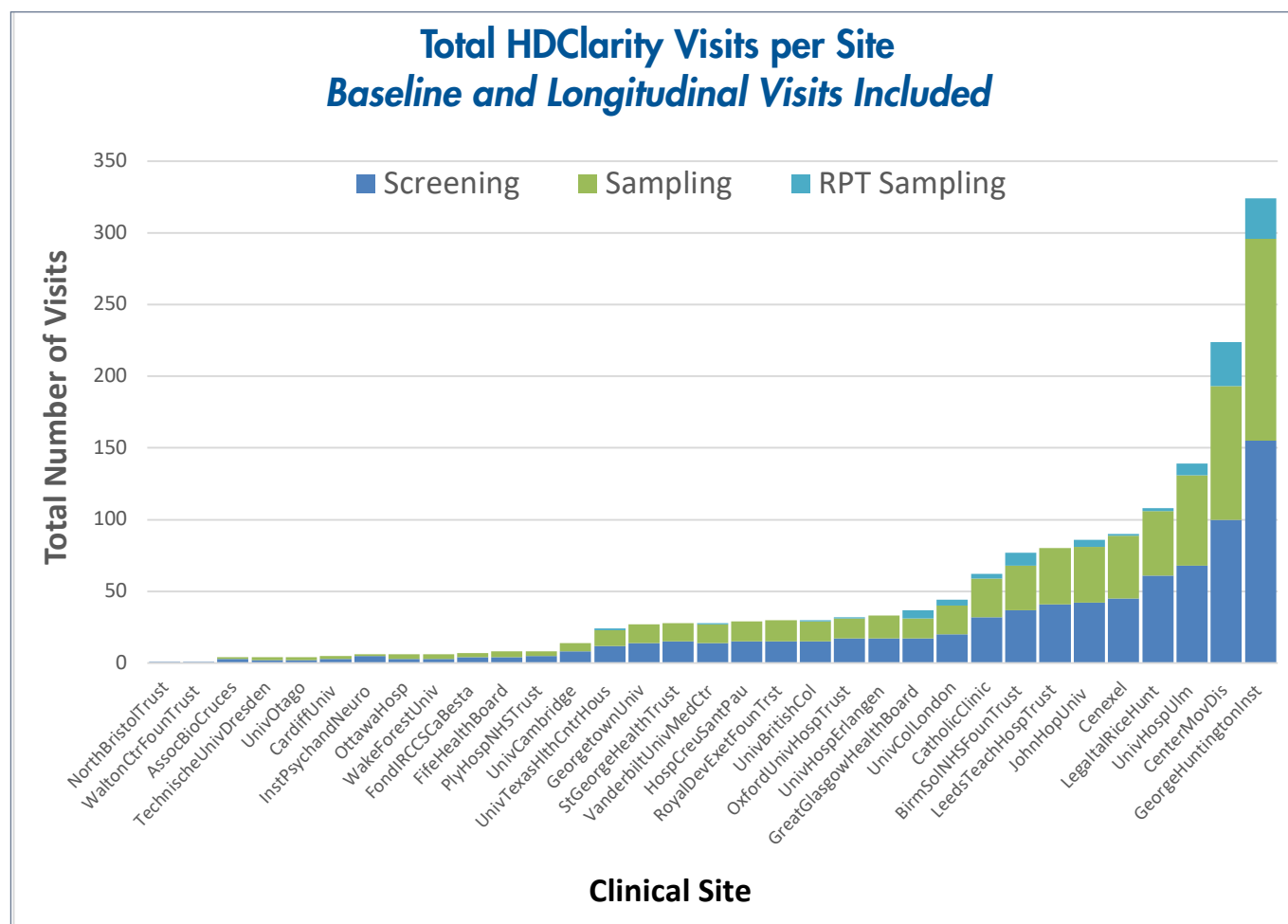


Figure 2. Total HDClarity Recruitment (May 2023)

## Research

To date, over 800 lumbar punctures across 6 participant categories have been conducted, collecting over 12 litres of CSF in total. Any qualified researcher may request CSF, plasma samples, serum samples and data from HDClarity and samples have already been distributed for use in over 25 research projects. This includes biomarker and protein analysis for the development of new treatments in HD.

## Further Information

Detailed information on HDClarity can be located on a variety of platforms including the National Institutes of Health clinical trials website and Twitter.

## Interested in Participating?



[enroll-hd.org](https://enroll-hd.org)



[clinicaltrials.gov](https://clinicaltrials.gov)



[hdclarity.net](https://hdclarity.net)



[@HDClarityStudy](https://twitter.com/HDClarityStudy)

The protocol and further information are available at [www.hdclarity.net](https://www.hdclarity.net) and the study team, led by Professor Edward Wild, are always happy to answer any questions. For more information and details on participating in HDClarity, please email [hdclarity-cc@enroll-hd.org](mailto:hdclarity-cc@enroll-hd.org).



## Update: Funding Opportunities

Fionnuala Margreiter,  
Grants & Collaborations Manager



Photo: Gabriele Stalner - artifox.com

- The [Horizon Europe](#) two-stage collaborative funding call on biomarkers ('validation of fluid-derived biomarkers for the prediction and prevention of brain disorders') first deadline is 1 September 2023. Find out more [here](#).
- The [European Molecular Biology Organization](#) is offering funding for workshops up to €43 000. The next deadline is 1 August 2023 (two deadlines per year).

**Are you interested in funding for research networking? Great opportunity for making contacts with a view to future collaboration. Check out the COST Networking Grants – join an existing action or set up your own networking project.**

COST is a networking programme that promotes research collaboration and knowledge exchange among scientists across Europe. It supports research networks called COST Actions, enabling researchers to collaborate on specific topics through meetings, workshops, and conferences. COST emphasises inclusiveness and interdisciplinary collaboration, integrating researchers from different career stages and countries. It is funded by the EU's Horizon 2020 programme and operates through national research funding organisations. Overall, COST facilitates scientific advancement and innovation in Europe.

For more details: <https://www.cost.eu/cost-actions/what-are-cost-actions/>

The EHDN and MDS-European Section HD Clinical Fellowship Programme received 45 applications from across the globe. Six fellowships were granted including an additional one this year for the Ukrainian fellow sponsored by donations gathered during registration for the EHDN Plenary meeting held in Bologna last September.

*'We were delighted to see such an interest in the HD clinical fellowship programme this year after the break due to COVID-19. The ongoing collaboration with the Movement Disorders Society is instrumental in helping to share the information worldwide with healthcare professionals interested in movement disorders and specifically HD.'*

Fionnuala Margreiter

## The following fellowships were offered in 2023:

- **Mariana Andriievskia**, (Ukraine, to be hosted in UK)
- **Arcel De Luca**, (Venezuela, to be hosted in Spain)
- **Mehri Salari**, (Iran, to be hosted in UK)
- **Victoria Zubiri**, (Argentina, to be hosted in Spain)
- **Ninel Arakelyan**, (Armenia, to be hosted in UK)
- **Elison Sarapura Castro**, (Peru, to be hosted in Spain)
- **David González Ruffino Biagio** (Venezuela, to be hosted in Spain)

**Find out about current research funding programmes and keep up to date with opportunities on the [EHDN website](#) and follow me on Twitter [@EHDN GRANTM](#)**



## Get in touch with the Think Tank!

The EHDN's HD Science 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 Yury Seliverstov: [yury.seliverstov@euro-hd.net](mailto:yury.seliverstov@euro-hd.net)**

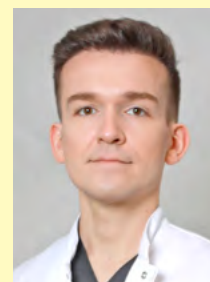


Photo provided by Yury Seliverstov

## Update: New Lesley Jones Seed Fund Awarded

The EHDN seed fund programme has been renamed in honour of Professor Lesley Jones, who was deeply involved in developing the programme and the review of applications.



Photo provided by Stefan Kochanek

**From left: Manuel Seefelder, Stefan Kochanek, Fabrice Klein**

Stefan Kochanek at Ulm University (Germany) has recently been awarded funding to identify new proteins interacting with HTT and its partner protein known as huntingtin-associated protein 40 (HAP40). A novel computational model will be generated to predict these interactions and the data will be experimentally verified in the laboratory. At the same time, the researchers will use biochemical methods to identify novel protein interactors of HTT and HAP40. The ultimate goal of this project is to support the discovery of new HD biomarkers and the identification of new therapeutic targets.



Photo provided by C. Capper-Loup

**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 November 2023.**

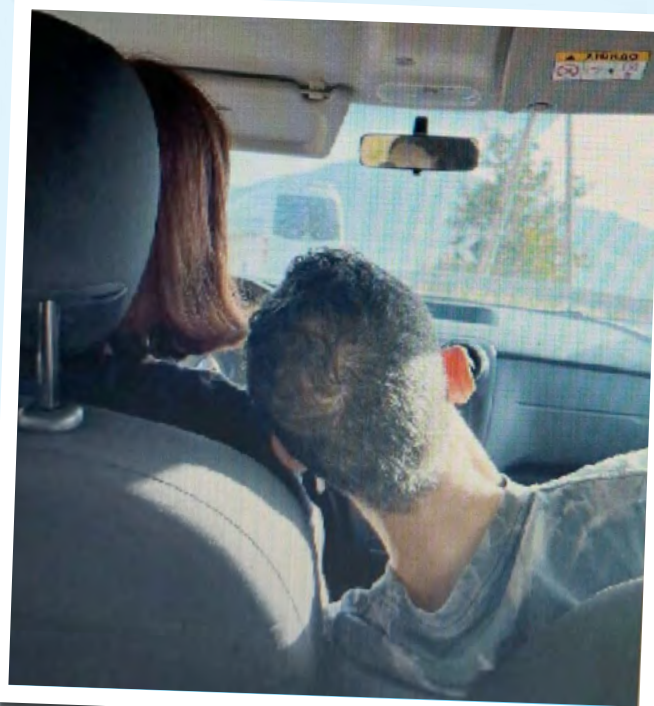
**More information about the programme and how to apply can be found [here](#) or you can contact Christine Capper-Loup ([Christine.Capper-Loup@siloh.ch](mailto:Christine.Capper-Loup@siloh.ch)) or Kristina Bečanović ([kristina.becanovic@euro-hd.net](mailto:kristina.becanovic@euro-hd.net)) for further information.**



Photo provided by K. Bečanović

## Send us your photos!

This edition's photo was sent by Antonio Fontana of the Associazione Italiana Huntington. The group has collected PhotoVoices as part of a project on HD and storytelling, and shared this great example of optimism and 'seeing the glass half full'.



***'I'm Christian, I'm 49 and diagnosed with Huntington's disease 11 years ago. Since then, together with my family, I've been trying to live life to the fullest, adapting to new situations and trying to grasp the positive side.***

***For example, I loved driving very much, but now that I can't do it anymore and that my wife drives for me, I can enjoy the pleasure of drinking a few more glasses of wine at the restaurant, without taking risks behind the wheel.'***

### Please get involved in sharing your photos!

Whether you're affected by HD personally, or you're a carer, clinician or scientist working in the field, we'd like to publish your images in the newsletter. If you have a photo that provides an insight into your daily life that you think might interest or inspire other EHDN members – or make them think differently about the disease – please send it to us along with a few words explaining who you are and what the image shows: [newsletter@euro-hd.net](mailto:newsletter@euro-hd.net)





Photos provided by Jenna Heilman

*HDYO is a truly international organisation**Demonstrating the Gratitude Day Logo*

## Bringing About Positive Change for Young People: An Interview with Jenna Heilman

Jenna Heilman is the Executive Director at the Huntington's Disease Youth Organization (HDYO). Working with Project Coordinator and Founder Matt Ellison, Jenna has brought a whirlwind of exciting activity and new ideas to our HD community. She took a brief pause from her busy schedule to tell us about their work bringing about positive change for young people affected by HD across the globe.

### *Could you start by telling us about your background and how you got involved in HD?*

Before HDYO, I was the Executive Director at Head for the Cure, an organisation supporting brain tumour and brain cancer research in the USA. Like many people during the pandemic, I needed to take a break from what I had been doing and of course, working in rare diseases can be really challenging for lots of reasons. Then HDYO put out the advertisement for an Executive Director – and it seemed like just too good an opportunity to miss. I was really drawn to the international focus, and the opportunity to learn more and connect with the team. At this time, I didn't have a direct, personal connection with HD but later on, I found out that on my husband's side of

the family, we had family members both with HD and at risk of HD.

### *What does an Executive Director do on a day-to-day basis?*

My main mission is to propel the mission of HDYO – supporting, educating and empowering young people aged 35 years and under impacted by HD. It is also to support the structure around these young people – their families, medical community, professionals, friends, and anybody who wants to learn more. While we are a really small organisation, we accomplish a lot, so I am extremely hands-on with the day-to-day operations, often meeting partners like folks at EHDN and other associations across the globe to either share the perspectives of young people, or figure out how we can collectively make a bigger impact. Such partnerships are key to fostering solutions. I also work with pharmaceutical companies and people in industry to share perspectives about the latest research news and help bridge the gap between pharmaceutical companies, communities and individuals. Our HD community is incredibly well equipped to be involved in the science and I think it's important for pharmaceutical companies to continue to invest in this community and build trust and respect. For me, it's really important to share the perspectives of young people and families in all of this.

### *How have recent clinical trial outcomes impacted your work?*

In recent years, some trials haven't panned out as hoped. At HDYO, we focus on the useful information



that emerged – a trial never really fails, it just hasn't met its goal, and there are always takeaways from that. Empowering people through education has been really important and the resiliency of the HD community, especially young people, has continued to amaze me, along with their thirst for knowledge and eagerness to engage in the next steps. Whether at risk, having tested positive, or having loved ones directly affected by HD, our community remains hopeful for an effective treatment and welcomes the opportunity to get involved in clinical trials. As an observational study, Enroll-HD is a good way to begin and allows individuals to share their voices and engage with the research process.

### How does HDYO progress education?

We have an amazing [Ambassador](#) programme to help advance education and resources. Now, we have more



than 60 ambassadors across the globe and our role is to help them develop as leaders. We provide training every other month and host a very active WhatsApp chat where individuals support

each other and provide content that's most impactful for the age group. This is all part of our strategy for developing and managing programmes to give young people affected by HD a voice that can be heard.

Our [International Young Adults Congress](#) held in Glasgow earlier this year was the first international event of its kind. It was incredibly empowering to meet in a community, in person, especially after COVID-19. We tried to create an event that was a bit different to the others and for the content to be specifically driven by the community. Our ambassadors were key in identifying the important topics and sessions that proved critical to the success of the congress. We also worked with our pharmaceutical partners to help break down the science to ensure that it was accessible to the community and could make clear the importance of getting involved in research.



Ask the experts at the first International Young Adults Congress in Glasgow



An attentive audience at the International Young Adults Congress

What we saw and what we heard was simply overwhelming – to be in a room with more than 300

*‘Empowering people through education has been really important and the resiliency of the HD community, especially young people, has continued to amaze me...’*

people where HD didn't need to be explained but you could just be yourself was amazing. You didn't have to be an outward advocate for the whole community... you could just share your story and be understood. That was incredibly

powerful for all of us. The feedback we received showed that participants valued the safety of the event, and by that, I mean they felt safe approaching people and at the same time, being able to share their own stories. You can watch the recorded sessions [here](#).

I'm thrilled that we were able to offer 100 scholarships from HDYO and several other associations sent members, families and young people through their own pro-

grammes as well. We will continue to strive to target the communities that don't have access to care and support, and to keep growing each and every year to ensure that we truly offer international representation and opportunity.

**You Can Make A Difference by Sharing Your Experiences!**



HDYO Survey Series



Scan the QR to find out more!

## What plans do you have for the future with HDYO?

The programme for ambassadors and the next congress will be our key focus but we are also working on our educational programmes and associated components. To this end, we recently launched our first survey, with the aim of better understanding our community. To begin, we are looking at how people search for resources and support. This is an ethically approved, international survey open to anyone aged 18 or older and impacted by HD. After that, we will look at different social and research-based stigmas around HD, the milestones of life and those associated with HD, and the support that is currently available.

We are currently in the process of launching HD STRIVE, which stands for Supporting and Training International Voices to Excel. This is an online, membership platform for people working in a wide range of various roles to support young people impacted by HD. We also hope to start offering continuing



**Jenna Heilman with Charles Sabine and Matt Ellison**

*‘We will continue to strive to target the communities that don’t have access to care and support, and to keep growing each and every year to ensure that we truly offer international representation and opportunity.’*

education opportunities in which we connect to the international community while also encouraging people to start supporting young people if they don’t already have programmes in place. At our congress, we spoke with professionals to hear about what would be most beneficial to them and we’re working on those nuts and bolts pieces right now.

Finally, I’d like to mention ‘[Huntington’s Disease Heroes: Inspiring Stories of Resilience from the HD Community](#)’. This is a collection of stories from more than 20 advocates across the UK and much further afield. We have a chapter explaining the need for HDYO and how we’ve evolved our services to address young people impacted by HD. It’s a fantastic learning resource for everyone, and for scientists who don’t have the opportunity to go to meetings and conferences, it is a great way to hear snippets of people’s powerful stories. It’s available on Amazon and as we’re the charity partner, 50% of the proceeds come back to HDYO.

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## Dates for your diary

- The [European Academy of Neurology Congress](#) takes place in Budapest, 1–4 July 2023. Representatives from the EHDN will be there – come see our stand at the Neurohood exhibition section.
- The International Congress of Parkinson’s Disease and Movement Disorders takes place in Copenhagen, Denmark, 27–31 August 2023. [Registration](#) is still open.

- The 11<sup>th</sup> IBRO World Congress of Neuroscience takes place 9–13 September 2023 in Granada, Spain. Regular [registration](#) is still open.

- The European Huntington’s Association Congress takes place in Blankenberge, Belgium 19–22 October 2023.



Registration is now open and further details are available [here](#).

**Would you like to share an upcoming event with our readers?**  
Please email the details to [newsletter@euro-hd.net](mailto:newsletter@euro-hd.net)



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