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EHDN News

EUROPEAN HUNTINGTON'S DISEASE NETWORK

Photo: EHDN

Anne Rosser and Mariana Andriievska

Harnessing Challenge to Create Positive Change

Catherine Deeprose

In this inspiring issue of EHDN News, we hear how Mariana Andriievska, a neurologist in Ukraine, embraced the opportunities provided by the EHDN and MDS-ES Joint Fellowship Programme to take key skills back to her home country and share these with colleagues to improve HD care. We review Erin Paterson's recent anthology, *Huntington's Disease Heroes: Inspiring Stories of Resilience*, and contributor Ashley Clarke also shares her personal reflections on the power of writing when facing challenges. We update on the thriving activities of the EHDN, including current studies and funding opportunities, and share exciting news about EHDN & Enroll-HD Strasbourg 2024 from the programme chairs, Åsa Petersén and Sandrine Humbert.

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Building Strength Through Knowledge and Action

Mariana Andriievska,
Catherine Deeprose

My interest in movement disorders has grown during my neurological practice. Unfortunately, the diagnosis and management of HD are poorly developed in Ukraine, and we do not have dedicated HD clinics. I undertook the EHDN and MDS-ES Joint Fellowship Programme in 2023 to help me develop critical skills in clinical care and management of rare moment disorders, particularly HD, in Ukraine.

Mariana Andriievska, Neurologist, PhD student and Assistant in the Department of Nervous Diseases at the Vinnytsia National Pirogov Memorial Medical University, Ukraine

My fellowship was supervised by Anne Rosser at the University of Cardiff, where I was able to observe individuals with HD and learn about the manifestations of symptoms across different stages of the disease with their families and in their homes.

My clinical practice in Ukraine has changed dramatically as a result of the fellowship. I am now confident in neurological examination and the use of clinical assessments in HD. My experiences in Cardiff mean that I can now confidently describe and recognise the psychiatric manifestation of HD. In addition, I gathered vital skills in genetic testing and genetic counselling, and in developing symptomatic therapies for patients. Finally, I developed critical insights into the importance of a multidisciplinary approach in HD and the necessity of research.

During my fellowship, I recorded short videos and posted them on my Instagram page to increase awareness of HD. When I returned to Ukraine, many individuals contacted me asking for further information about HD diagnosis, having watched my videos.

I am now planning to develop a database of individuals with HD in Ukraine, and for my department at Vinnytsia National Pirogov Memorial Medical University to join Enroll-HD. I also hope to create courses for colleagues to learn more about the diagnosis and management of HD and to refine the use of clinical scales and assessments among neurologists and psychiatrists in our region. I am also starting to consult with HD-affected families about the possibilities of predictive testing.

Ukrainian doctors deal with horrific human losses every day. We have all suffered and experienced huge losses in this war, and it has made me reconsider my values and priorities. We are doing the impossible and fighting every day to defeat Russia. All support from other countries, including my fellowship, helps bring us one step closer to victory. By enriching ourselves through knowledge and action, Ukrainians are united. Thanks to the fellowship, I am able to improve the quality of HD care in Ukraine and motivate other doctors to do the same. We remain hopeful. I am very grateful for the fellowship and, particularly, Anne Rosser for her guidance and support.

Mariana at the laboratory

Huntington's Disease Heroes: Inspiring Stories of Resilience from the HD Community

Catherine Deeprose

This inspiring anthology of 25 highly personal stories, written by those impacted by HD in some way, was coordinated and edited by Erin Paterson and published with the key message – 'you are not alone'. In addition to suggestions for different uses of the text (for example, by support groups, families, and professionals working in the field), a helpful topic guide points readers to chapters covering a diverse range of themes, and each chapter is accompanied by suggestions for reflection and discussion. The charity partner for the book is HDYO, meaning that 50% of the profits go to supporting their vital work. HD advocate and co-author Ashley Clarke talks here about her involvement in the book and her immeasurable dedication to HD advocacy.

Co-author and blogger Ashley Clarke

You talk about how we can't expect the general public to understand HD but seem to have done an amazing job of doing exactly that! How have you managed it?

I have spent several years perfecting my skills when talking to the general public. I can remember being out with Dad shopping or doing general daily tasks, and people would stare. On days that Dad's HD wasn't great, he would cause an episode in public (I hate saying that), and this would result in a lot of people staring and passing comments. Back then, I was quick to respond, with a temper that I inherited from my dad – I felt defensive! As I've grown, matured, and educated myself, I realise that we can't hold it against people when they don't know about a rare disease. I don't know about many other diseases out there, and if I encountered someone, I would like for it to be explained to me.

I prepared a speech in my head so that when I encountered a situation, negative or positive, I could respond in a way that was educating the person rather than attacking them because they simply didn't know something. As much as I would defend my dad to the ends of the earth, he did look drunk, and sadly, we live in a world where people think the worst.

The work that I do and the opportunities I have been provided still to this day blow my mind. I sit and look in the mirror, pack my overnight bag for another trip, or look back at photos and videos, wondering how I ever got through that public speaking event or how I will get through the next one. But I do. I am driven by the community; we have an amazing HD community around the world! I have great support from friends and family, and honestly, as nervous as I get, it's also exciting.'

Northern Ireland is really quite small, and HD is very rare. Do you think there are specific challenges for HD-affected families in the region?

There are so many advantages and disadvantages to Northern Ireland being so small! Sadly, we don't have an active government (yet again), making it very difficult to make policy changes and improvements to statutory services. From a funding perspective, it's a

small region, and you are raising money for a very rare disease many have not heard about, but since Brexit, the demand on existing funding streams is incredibly high and difficult in terms of long-term sustainability for a charity which means it will have a knock-on effect on service users.

I think the biggest advantage of being from a small country is the sense of community. Growing up, I knew no one affected by HD, and now I have so many friends not only here in Northern Ireland but around the world. Here in Northern Ireland, it's easier to bring the families together. It builds a sense of friendship and community. It raises the support we have here and makes us a family. HDANI's slogan is 'it's a family thing' because we are one big family, trying to get through this together.

How did you get involved in writing the chapter?

I believe Erin [Paterson] reached out to me on Instagram. People always told me I should write a book; my life has been like something off a TV show, and it seemed like a fun opportunity. We talked about others who might get involved and which charity would be a good one to connect with. I suggested HDYO because they support people around the world.

Have people reached out to you having read it?

Yes – it's been amazing! People have messaged me on social media, saying how much reading my chapter and the book as a whole has helped. People have stopped me in my local area or at events I have attended and told me how much they enjoyed my chapter and how it touched their hearts. It's one of those 'pinch-me' moments. I never thought I'd see the day I would be asked to sign a book that I am a co-author of! It happened at two events, and I seriously need to perfect my signature!

Erin Paterson coordinated and edited the anthology of 25 highly personal stories; foreword by Charles Sabine

> *'I put this book together because I wanted to give people in the HD community the opportunity to share stories about the real-life impacts of living with the disease in their families. It is important for us to be able to express the hardships we have been through at the same time as providing hope to others. By reading the stories in the book it helps us understand that there are so many different viewpoints within the HD community and we are not alone.'*
>
> ***Erin Paterson,***
> ***Writer and HD Advocate***
> ***(and also author of All Good Things: A Memoir About Genetic Testing, Infertility and One Woman's Relentless Search for Happiness)***

When people share their stories with me through private messages, my heart breaks for some of them, but I am also so proud of them for reaching out. I have always wanted to help people; that's why I share my story. After the book was launched, a local news team did a story on me. A staff member in a local school reached out to me on Instagram – she had a student and HD had recently been found in their family. I was able to direct them to the support that pupil will need. It's times like this that make the late nights, TikToks, blog posts, emails, meetings, and everything else I do all worthwhile.

Was it challenging to talk about these issues? Would you recommend writing to others?

I've been writing my blog since 2016, and it has always helped me to share with others what it was like growing up caring for my dad with HD. I get comments and private messages from people sharing their stories with me, and it makes me feel less alone. Back when I first found out about HD, I didn't know anyone else my age going through anything similar or even affected by HD. That's totally different now – social media is such a powerful thing; it allows us to connect with people around the world.

I personally find writing helps. I would recommend it, whether it is a blog you never publish, a diary, or a letter to yourself that you throw away. Anything, get it down on paper, out of your head. Sometimes I read

Ashley Clarke – living life to the full

things back and think, 'Wow, did I actually feel that way?', 'say that?', and so on. When you're in the middle of something terrible, it's hard to think about it rationally. Writing it down always helped me to process it. What have you got to lose, really?

What's next?

I never planned any of the opportunities that came my way, but I am incredibly grateful for them. 2023 was an exciting year, with some of my biggest opportunities, but it was also the hardest year for me after losing Daddy. I found it empowering to speak out, share my story, and help members of the general public, community, and research/ science community.

I have some exciting news about a new book titled 'Livable Lives'. This combines the author's HD story with stories from around the globe, including mine, to illustrate how an incurable family disease impacts relationships, major life decisions, the genetic testing decision, and more. Conversations with the HD community revealed the importance of pursuing what one HD-positive woman calls her 'livable life' – a life lived with intention and meaning – before symptoms appear. The book brings together a wide variety of experiences to tell a collective HD story that can raise awareness and educate others about this disease. I have truly enjoyed working with Christy Dearien. She has portrayed my story beautifully in the book, and I can't wait for you all to read it. If you want to find out more, visit her socials: Facebook www.facebook.com/ChristyDearien, Instagram @christydearien

I'm not sure what 2024 will bring, but I'm excited about it and welcome all new opportunities! Dad left a legacy, and I want to keep it going. I want to continue to help the HD community as much as I can.
https://www.imnotdrunklifestyleblog.co.uk/
https://www.instagram.com/kyra_ashley93/
https://www.tiktok.com/@kyraashleyclarke

'I was very touched by the courage and candour with which the contributors of this inspiring book reflect on the impact of HD on their personal lives. For physicians, researchers, and therapists (for whom the main aim is to treat the symptoms of HD), these personal accounts offer a window into the greater impact on individuals, far beyond the overt neuro-psychiatric symptoms that we see clinically. It is incredibly encouraging to read how this diverse and resilient community often finds very positive ways to confront the inherent challenges of HD.'

Patrick Weydt,
EHDN Executive Committee Deputy Chair

• • •

Significant Funding Boost to Support Young People Affected by HD

Vicki Rutland, Head of Communication and Marketing, Huntington's Disease Association

The Huntington's Disease Association Youth Engagement Service (HDYES) recently announced the receipt of £418,360 in funding from The National Lottery Community Fund in the UK. This financial support will enable HDYES to continue its crucial work with young people and young adults living in families affected by HD. The project will enable more people to fulfil their potential by working to address issues at the earliest possible stage. Find out more: https://www.hda.org.uk/information-and-support/getting-help/children-and-young-people/

Local Language Coordinator Meeting in Cologne

Tania Velletri, Local Language Coordinator for Italy

In 2023, Language Coordinator (Lanco) meetings were held in three major European cities: Barcelona (January), Paris (May), and most recently, Cologne (20–21 September). During these meetings, Lancos and EHDN staff discuss topics related to Enroll-HD and share experiences and ideas. Every Lanco meeting is carefully planned to provide unique opportunities to establish and consolidate relationships, develop collaborations, and connect with the EHDN management.

Music & Brain

On the first day, we had the chance to participate in a special event hosted at DZNE (German Center for Neurodegenerative Diseases) in Bonn: Music & Brain. After lectures by Patrick Weydt and Bernhard Landwehrmeyer, Nora Guthrie (daughter of Woody Guthrie, who died of HD in 1967) shared personal insights about her experiences and work in HD. We were also treated to a concert of Woody Guthrie songs translated into German by Wenzel, a German singer-songwriter.

Presentations and Updates

Over these two days, we attended 17 presentations. We heard about European and global updates for Enroll-HD, the latest news in clinical trials, genetic testing and counselling, plasma collection, and RNA collection. Site certification and participant recruitment were discussed, and our Lanco colleague, Marta Laciak, presented on the work that she has been doing in Poland. All the presentations were followed by Q&A sessions, meaning that we could share queries, suggestions, and ideas for future developments.

A Personal Perspective

Lanco meetings remind me that I am part of the global family that is the HD community, with families, researchers and scientists all collaborating with the same shared aim. It is amazing that we all can contribute in our own way, and the opportunity to listen to others and discuss ideas helps me appreciate what we have learned and what we can do better. When I return home, I am refuelled with positive energy that I take into my work. I look forward to meeting everyone again in 2024!

Update: Clinical Trial Developments

Jenny Townhill and Tim McLean, Central Coordination

Key updates since the last newsletter are provided below for EHDN-endorsed trials and studies; please refer to Table 1 for a summary of all ongoing research. An expanded description of all EHDN-endorsed trials and studies (completed and in progress), as well as details of the EHDN endorsement process, is published on the EHDN website.

Photos: Gabriele Stautner · artifox.com

On 21 June 2023, UniQure and PTC Therapeutics issued positive results summarising interim data analyses on their respective huntingtin (HTT) lowering programmes:

UniQure US Phase I/II trial of AMT-130 Gene Therapy (HD-Gene TRX-1)

Interim analysis of up to 24-month data on the 26 participants enrolled in the US study showed that AMT-130 continues to be generally well tolerated with a manageable safety profile.

A comparison was also made between AMT-130 treated participants and a natural history cohort (31 participants from the TRACK-HD study with similar characteristics to the trial population) and showed potential clinical and functional benefits. Data were also shared on neurofilament light chain (NfL) and mutant huntingtin protein (mHTT) levels in cerebral spinal fluid (CSF). The surgery produced an expected initial increase in NfL in CSF, which then decreased in all participants. For further details on the analysis, see the press release.

PTC Therapeutics, PIVOT-HD Phase II Study of PTC518 RNA Splicing Modifier

Encouraging interim 12-week data from 24 participants exposed to PTC518 (total participants was 33 including those receiving placebo) showed that PTC518 was well-tolerated, with a favourable safety profile. Dose-dependent lowering of HTT levels was demonstrated, and the intended exposure was confirmed in CSF. There was no spike in CSF NfL, as detailed in the press release.

Table 1: Current EHDN Endorsed Trials and Studies

Registration ID (CT.gov/ ISRCTN)	Sponsor	Trial name	Phase	Investigational Product	Mode of Action	Delivery	Treatment Goal	Target Enrolment	Location(s)	Status
NCT05541627	BrainVectis, subsidiary of AskBio	ASK-HD-01-CS-101	I/II	AB-1001	Restoration of cholesterol metabolism dysfunction (AAV gene therapy)	Surgical, striatal	Disease modification	18	France	Active, not recruiting
NCT05686551	Roche	GENERATION HD2	II	Tominersen	htt lowering ASO	Intrathecal	Disease modification	360	North America, Europe, Oceania	Recruiting
NCT05111249	Novartis	VIBRANT-HD	IIb, OLE	Branaplam	mRNA splicing modifier	Oral	Disease modification	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	Disease modification	480	Canada, Europe, USA	Active, not recruiting OLE ongoing
NCT05358717	PTC Therapeutics	PIVOT-HD	II	PTC518	mRNA splicing modifier	Oral	Disease modification	162	Australia, France, Germany, Netherlands, UK, USA	Recruiting
NCT05107128	Sage Therapeutics	DIMENSION	II	SAGE-718	NMDA receptor modulator	Oral	Symptomatic	178	Australia, Canada, UK, USA	Recruiting
NCT05358821	Sage Therapeutics	SURVEYOR	II	SAGE-718	NMDA receptor modulator	Oral	Symptomatic	80	Canada, USA	Recruiting
NCT05655520	Sage Therapeutics	PURVIEW	III, OLE	SAGE-718	NMDA receptor modulator	Oral	Symptomatic	300	Australia, Canada, UK, USA	Recruiting
NCT05475483	SOM Biotech	SOMCT03	IIb	SOM3355 (bevantolol hydrochloride)	VMAT2 inhibitor	Oral	Symptomatic	129	France, Germany, Italy, Poland, Spain, Switzerland, UK	Recruiting
NCT04120493	UniQure	HD GeneTRX2	Ib/II	rAAV5-miHTT	miRNA AAV gene therapy	Surgical, striatal	Disease modification	15	Germany, Poland, UK	Recruiting
NCT05032196	Wave Life Sciences	SELECT-HD	Ib/IIa	WVE-003	Allele-selective htt lowering ASO	Intrathecal	Disease modification	36	Australia, Canada, France, Germany, Italy, Poland,	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

Update: Enroll-HD

Olivia Handley, Enroll-HD Global Platform Manager

In July this year, it was announced that the Huntington Study Group's (HSG) clinical research organisation (CRO) – HSG Clinical Research Inc. – would now provide CRO services for the Enroll-HD study.

The Huntington Study Group

Ira Shoulson and colleagues at the University of Rochester, USA, founded the HSG in 1993. The primary aim was to establish a strong network of researchers, clinicians, and family members to work together with external partners, including the EHDN, to conduct research and develop treatments for HD. Over the last 30 years, the HSG has successfully run approximately 40 clinical studies and trials in HD, accumulating invaluable experience in delivering high-quality clinical research in HD.

Enroll-HD, REGISTRY and COHORT: A brief history

Several years before Enroll-HD existed, there were two large-scale, prospective, multi-centre observational studies in HD – EHDN's REGISTRY study and HSG's COHORT study (Cooperative Huntington's Observational Research Trial). Both studies recruited individuals affected by HD and who were part of an HD family to attend regular study visits. REGISTRY was Europe-based, whilst COHORT involved study sites located in the US, Canada and Australia. Both studies were funded by CHDI. By 2012, both studies had evolved into Enroll-HD, allowing for a single study to be run across global regions.

Enroll-HD and the Huntington Study Group

With its proven track record of conducting more clinical trials in HD than any other CRO, the Enroll-HD team is excited to have the HSG undertake on-site monitoring visits. The HSG is working specifically with Enroll-HD sites located in North America. During on-site monitoring visits, the HSG team reviews the conduct of the study with site investigators and coordinators to ensure the high-quality collection of Enroll-HD data and biosamples is maintained. This is the same on-site monitoring activity that is undertaken by the EHDN team for Enroll-HD sites based in Europe.

Photo: Gabriele Stautner · artifox.com

Outlook

Enroll-HD continues to reach new milestones. It celebrated its 30,000th participant recruited from the Westmead Hospital, Sydney, Australia, on 17 July 2023. The demand for the platform's resources remains high, which necessitates a greater emphasis on ensuring our infrastructure can meet its goals. The addition of the HSG will undoubtedly support us in our effort to provide a global research platform available to the HD community.

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

Photo provided by Yury Seliverstov

For more information about the Think Tank, please contact Yury Seliverstov: yury.seliverstov@euro-hd.net

HDClarity

Update: HDClarity

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

We are delighted to announce that there have now been over 1,000 CSF samples collected as part of HDClarity[1]. This amazing milestone was reached thanks to the dedication and support of the HDClarity sites and participants.

The youngest participant was just 21 years old at the time of enrolment, which was the minimum age permitted until a recent protocol amendment extended the study to include juvenile participants. Our most senior participant is currently 74 years old and is still taking part. Many participants have returned for a 2nd or 3rd annual lumbar puncture to provide extremely valuable longitudinal samples. The current record is five longitudinal sample donations, but many HDClarity sites are now using the new longitudinal protocol, and we hope that this number will continue to grow!

The newest HDClarity sites in Canada (Ottawa Hospital and North York General Hospital) and New Zealand (University of Otago) are now recruiting, and we are continuing work on opening the first sites in countries such as Australia, France, the Netherlands, Norway, Portugal and Switzerland. The UK and Germany continue to contribute the most samples overall; however, regulatory research approvals differ between regions, making it easier to open more study sites in some countries. Working with study site investigators and their teams to obtain local regulatory approval and extend HDClarity participation to more HD families continues to be a primary aim.

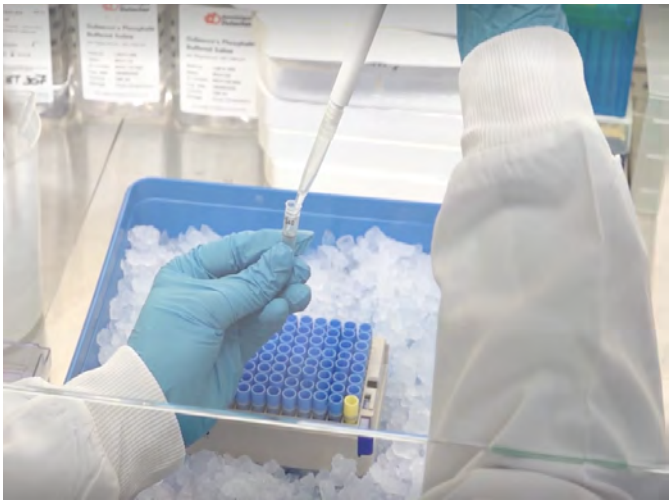
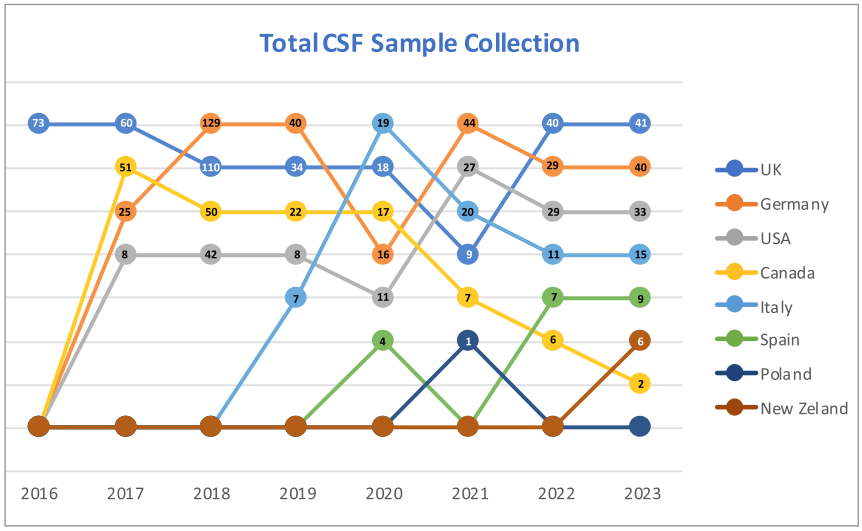


Photo provided by Gail Owen

Further information, including the current protocol, is available at www.hdclarity.net, and the central coordination team are always happy to answer any questions (hdclarity-cc@enroll-hd.org). Information about the study can also be located on a variety of platforms, as shown.

Researchers wishing to request samples should visit https://www.enroll-hd.org/for-researchers/biosamples.

[1] including samples collected as part of the University College London HD-CSF study with support from CHDI.



Interested in Participating?

Enroll-HD enroll-hd.org

U.S. National Library of Medicine ClinicalTrials.gov clinicaltrials.gov

HDClarity hdclarity.net

@HDClarityStudy

Scan the code for more site information:

Update: Funding Opportunities

Fionnuala Margreiter, Grants & Collaborations Manager

Photo: Gabriele Stautner · artfox.com

EHDN/MDS HD course in Spanish

EHDN and MDS have been successfully collaborating over several years in running the fellowship programme and, more recently, in the organisation of HD online courses. Following a successful online HD course series in Europe, an online HD course was recently organised with the MDS Pan American group. The course was planned to ensure that it was clinically interesting and relevant for HD professionals and communities in Latin America, delivered in Spanish, and held over four Friday afternoons in July 2023.

There were almost 800 registrations, representing over 35 countries (with Mexico, Argentina and Peru having the highest numbers of attendees). The course was well received and ended with a lively Q&A session. An evaluation report is now being prepared, and ongoing access to the course lectures is available to all registered attendees.

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EHDN/MDS fellowship programme

Seven fellowship places were granted this year (six placements and one additional placement for Ukraine). Fellowship placement visits are currently taking place in the UK and Spain, and initial feedback has been very positive.

A specific project to measure and assess the overall impact of the fellowship programme is currently taking place, with data being gathered from past fellows and also host clinics with the assistance of the EHDN and Lanco teams. The findings will be used to improve the programme in future years and disseminated in the coming months. Processes for the application, evaluation and management of the applications are also being reviewed.

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Upcoming funding opportunities

European Molecular Biology Organization: Two funding deadlines for workshops are announced each year: https://www.embo.org/funding/funding-for-conferences-and-training/workshops/
Marie Skłodowska-Curie Actions (MSCA): European research fellowships are offered as part of the Horizon Europe framework programme. The goal of MSCA is to support excellent research training, mobility and career development for researchers at all career stages: https://marie-sklodowska-curie-actions.ec.europa.eu/

For regular updates about funding opportunities, please see the EHDN website and the Twitter(X) account @EHDN_GRANTM.

Update: New Lesley Jones Seed Fund Awarded

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

Photo provided by Eric Reits

Eric Reits

Eric Reits at Amsterdam University Medical Centers has been awarded funding for a project to be conducted in collaboration with Monique Mulder (Leiden University Medical Center) and Sabine Schipper-Krom (Amsterdam University Medical Centers). This novel work aims to reduce the degradation of mutant huntingtin protein fragments as a potential therapeutic strategy for HD.

Photo provided by Niels Skotte

Niels Henning Skotte

Niels Henning Skotte at the University of Copenhagen has been awarded funding to progress our understanding of the complex interplay between different brain cells, define new targets for therapeutic intervention, and discover novel biomarkers for monitoring disease progression using a mouse model in the laboratory.

Photo provided by C. Capper-Loup

Christine 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 March 2024.

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

Photo provided by K. Bečanović

Kristina Bečanović

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Pridopidine: All May Not Be Lost

Catherine Deeprose

The EHDN hosted a virtual meeting on the latest Proof-HD Phase 3 study analyses on 9 October via Zoom. Prilenia was represented by Michael Hayden (CEO), and Jeff Long (University of Iowa) discussed the specifics of the most recent analyses. Earlier this year, it was announced that the study did not meet the primary endpoint of benefits for pridopidine compared to placebo (Unified Huntington Disease Rating Scale-Total Functional Capacity). We heard that recent analyses excluding participants on antidopaminergics (as pre-specified in the Phase 3 study) and performed posthoc on the data from the previous studies, may point to benefits for pridopidine for this specific subset of participants. More work to confirm and explain this possibility is required.

Photos provided by Alex Fisher

Send us your photos!

Here is a snapshot of the experiences of Alex Fisher, who spent three weeks in Venezuela with the combined might of the teams of Habitat Luz and Factor-H, who provide humanitarian assistance and care to HD families in St Luis, St Francisco and Barranquitas. Alex will be writing a more detailed piece for our next newsletter, but in the meantime, check out https://factor-h.org/factor-h-september-2023-trip-to-the-maracaibo-hd-communities

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

Photo: Gabriele Stautner · artifox.com

Åsa Petersén

Photo provided by Sandrine Humbert

Sandrine Humbert

EHDN & Enroll-HD Strasbourg 2024: An Interview with Åsa Petersén and Sandrine Humbert

EHDN & Enroll-HD 2024 will be a collaborative event combining the biennial EHDN Plenary Meeting and the Enroll-HD Congress over three days (12–14 September 2024) in Strasbourg, France. We spoke with programme chairs Åsa Petersén (Lund University, Sweden) and Sandrine Humbert (Paris Brain Institute, France) to find out more about the exciting plans afoot.

When did planning start?

Åsa: We started to plan actively in January 2023. As chairs of the programme of the meeting, Sandrine and I first needed to think about the programme committee, which was formed in April. Since then, we've had regular meetings with the programme committee, which comprises scientists and clinicians from different parts of Europe and much further afield. We also have a representative from the HD advocacy community.

Sandrine: This is going to be the first joint meeting between the EHDN and Enroll-HD, so the format will be slightly different from previous years in that one whole day (Saturday) will be dedicated specifically to Enroll-HD. As such, we are expecting a record turnout in 2024!

Åsa: We see this as a unique opportunity to bring our HD community together in Europe – clinicians, healthcare professionals, scientists and families, who together, as a community, represent many different countries and perspectives.

Exciting! What can you share now about the programme?

Åsa: An important topic, and one that everyone is interested in, will be the update of ongoing and planned clinical trials. We will ensure that perspectives are shared by HD families, researchers and clinicians. Our main keynote speakers are leaders in the HD field, and we will also have sessions for more junior researchers to present their research, including poster presentations. Research themes will focus on genetic modifiers, somatic expansion, developmental and ageing aspects, and metabolic aspects of HD and related disorders. When it comes to clinical aspects, we will cover both the global impact of HD and this year, we'll focus on cognitive symptoms and rehabilitation as well as palliative care.

Sandrine: While the topics for 2024 are [not all] new, and we will have new speakers bringing fresh perspectives alongside our well-known speakers, the key ethos of interaction between researchers, clinicians and family members will remain. We can say now that our keynote speakers will include Jean-Louis Mandel (University of

Strasbourg) – one of the first researchers to identify a disease-causing triplet repeat expansion in a gene. Sarah Tabrizi (University College London) is a leading HD clinician-scientist who will be speaking, and Harry Orr (University of Minnesota), who (together with Huda Zoghbi) cloned the gene for spinocerebellar ataxia 1 (SCA1, another triplet repeat disorder), will be joining us.

What social events can we look forward to?

Sandrine: There is lots to look forward to! In addition to a special event by HDBuzz, we will have several opportunities for networking and interaction.

We should mention as well that Strasbourg is a truly beautiful city, and well worth visiting. It's going to be great!

Photo: Bruno Klomfar

Åsa: Yes, we are really looking forward to it. We have been overwhelmed by the hugely positive responses from all the speakers and chairs we have spoken to, and of course, the dedication and enthusiasm of the EHDN staff, who are working incredibly hard to put everything in place for the meeting to be a tremendous success.

The full programme and calls for registration and abstract submission will be announced in early 2024. If you have any questions about the congress, please email strasbourg2024@euro-hd.net

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Dates for your diary

- 2–4 November 2023: The Huntington Study Group Annual Meeting takes place in Phoenix, AZ. Look out for EHDN staff there!
- 4 November 2023: The Huntington's Disease Association's Community Conference and AGM takes place online (and registration is free).
- 11–15 November 2023: The Society for Neuroscience Annual Meeting will take place in Washington, DC.
- 26–29 February 2024: CHDI's 19th Annual HD Therapeutics Conference returns to Palms Springs, CA. Read about the 2023 meeting here.
- 16–19 May 2024: The MENA Congress for Rare Diseases will take place in Abu Dhabi, UAE.
- 12–14 September 2024: EHDN & Enroll-HD 2024 takes place in Strasbourg, France. Further details (including programme, registration and abstract submission) will be announced in early 2024. For details about past meetings, click here.

Would you like to share an upcoming event with our readers?
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