Invitation to online EHA webinar September 7, 6-8 pm

Topic: Capacity building and mobilization of the HD community in regulatory advocacy

Registration link:
https://us02web.zoom.us/meeting/register/luwXDPweSseOfwS2ImzbDg

Agenda:

1) Welcome and round of presentations
2) Introduction and background
3) Presentation of survey
4) Presentation of campaign materials
5) Discussion

Background:

EHA is deeply concerned because we experience a lack of understanding of the disease burden and unmet needs in HD among regulators in Europe.

We suspect there may be some underlying discrimination behind the lack of access to care and the unwillingness to use accelerated pathways to facilitate expedited access to therapies.

We see that other companies are approaching regulators in other parts of the world to apply for market approval based on limited data. This is the case for Skyhawk in Australia (Skyhawk Announces Australia's Therapeutic Goods Administration Has Determined SKY-0515 for Huntington's Disease Meets Eligibility Criteria for Registration via the Provisional Approval Pathway)

Uniqure will apply with the US FDA as well as in the UK in Q3 this year. (Press Releases | Investors & Media | uniQure)

Cellavita is applying for their stemcell therapy in Brazil in Q4 this year.

But in EU at the EMA there is no opening to consider any of these or other HD therapies (like Pridopidine/Ferrer, Votoplam/Novartis or SOM3355/SomBiotech). This is the status for HD, despite other applications for other diseases being granted access by the EMA, although there is limited data and consequently uncertainty about the results. But EMA consider the unmet needs and urgency in these diseases to be very high. But isn't it very high also for HD??

The conservative attitude towards HD is in our opinion unacceptable. The big question is what can we do about it?

For EHA this is one of our focus areas and we want to take action to try to change the opinion within the regulatory authorities.

As many of you know, we hosted a workshop in November 2025 about Health Technology Assessment (HTA) (basically value evaluations – cost/benefit analysis) with 12 member associations present. I have attached the minutes for your reference as we see this webinar as a follow-up from that in-person meeting. The EMA and the HTA procedures are closely linked.

In Prague we discussed many things that might be valuable as next steps and we have put together a plan for what EHA can do in collaboration with our members.

What are we currently doing or going to do this year?

- Collecting video testimonies about disease burden and implications - Done
- Do a multinational survey towards family members and health care professionals, about disease burden, unmet needs, symptom ranking and treatment preferences - Survey template finalized and translated to 14 languages
- Conduct focus group interviews based on survey questions with both international and national groups Can we do this during EHDN?
- Identify and involve HD experts to co-author a position paper with conclusions from the data collected We need to identify professionals who are willing to support the initiative and be included as writers of a white paper
- Recruit and run a group of advocates with lived experience in HD – run trainings in regulatory procedures and prepare for involvement in future meetings with EMA as well as HTA.

Looking forward to the webinar and to get your input and thoughts on how we can do this capacity building as best as possible.