

Stronger together!

From September 2016 to December 2017

2017 has been an active year. The status and renewed strategy for Enroll-HD has changed the aim of the project from general recruitment to more specifically enhance participation from people who are presymptomatic, in prodromal or early stages of HD.

The funding of Stronger together is the main driver behind all the activities described in this report. Without both the funding and the network of EHA this could not happen. The synergy between Stronger together and the EHA structure and human resources is very fruitful.

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Project aims:

- Increase speed on recruitment to Enroll-HD
- Increase speed in recruitment to clinical trials
- Improve retention in Enroll-HD
- Improve retention in clinical trials
- Increase patient advocacy engagement in the planning process of clinical trials
- Increase patient advocacy engagement in regulatory approval processes both in EU and national bodies

The main aims are still relevant except that focus in Enroll-HD recruitment has been revised to more presymptomatic, prodromal and early phase participants aligned with CHDI policy.

I have chosen to structure this report on the basis of the 5 strategies outlined in the original project action plan.

Strategy 1: Networking to increase cooperation and learning from each other throughout Europe.

Actions: Visit associations and clinics. Participate in meetings organized by the associations. Since the project started we have presented our work at conferences and family meetings in Poland, Slovenia, Russia, Malta, France, Norway, Sweden, Denmark, Finland, Italy, Portugal, Scotland, England,

We have visited clinics in Ireland, Slovenia, Germany, the Netherlands, Poland and France.

We experience enhanced interest for European collaboration, particularly from the HD associations in Germany, France, England and Scotland.

“You have brought me out of my sadness. Now I don’t feel alone anymore.”

Bulgarian woman, age 91, attending the EHA conference

Much more active relations and collaboration with the HD associations in Russia, Malta, Hungary and Romania.

The travel and increased contact with the associations and clinics gives us the opportunity to strengthen the network and know more about where/who we can refer to in different matters. We see that more people experience the importance of collaboration on a European level and that it actually inspires more to be active both locally and internationally.

In Malta the Italian partner of EHA, LIHR, has reached out and arranged meetings with both family members and professionals. There has been established a collaboration with Caritas in Malta to improve the health services for HD affected. At the first meeting arranged in October 2017, 18 family members turned up and this was the first time Maltese HD families gathered to talk about their situation.

In Russia we have attended meetings with family members and professionals in Moscow, St. Petersburg and Nizny Novgorod. The talk in St.Petersburg was videotaped and is published on the webpage of Orphan People Centre. The centre is registered as the HD association in Russia and work mostly with HD families.

The HD group in Russia is growing. They are now close to 400 people connected by social media. The stigma is still very hard to break and people are reluctant to come forward. But things are happening. Orphan people have two young women working as volunteers. One is early symptomatic and the other gene-negative. They are both doing a fantastic job as ambassadors and give HD a face. One of them is recruited as HD-COPE team member. Another young woman has established a webpage with information about HD in Russian.

All the increased contact across Europe has resulted in much more exchange of resources and contacts.

The EHA conference was organized in Sofia Bulgaria in September 2017. 250 participants from 26 countries participated in workshops, plenary sessions and activities. By the help of sponsors we were able to support 46 participants from Bulgaria, Russia, Hungary and Romania with most of the conference costs. On the feedback survey people gave very positive score. Many expressed that they learn from each other and that they are inspired by being part of the community. From the survey we got this comment: *"I have learned so many new facts about HD, and I meet so many people from other countries and when we talked about HD you can feel, .. we are not alone we are a very big HD Family!!"* The situation for HD affected in Bulgaria is very difficult. One statement from a Bulgarian lady, age 91, says so much about the importance of reaching out: *"You have brought me out of my sadness. Now I don't feel alone anymore."*

During the first year in the project we have visited clinics in Ireland, Slovenia, Germany, the Netherlands, Poland, Austria and France. This gives us valuable insight in strengths and expertise and enable us to connect people and learn from each other. It also gives us good insight in what challenges and success criteria the institutions experience. In Germany,

Poland and France we visited hospitals/Enroll-HD sites. In Ireland, Slovenia, the The Netherlands and Austria we visited care facilities.

This year we will focus on new “territories” Bosnia, Kroatia, Slovakia, Greece, Iceland and the Canary islands to locate contacts, visit and participate in meetings and enhance people to establish or reenergize HD associations.

Strategy 2: Motivate the HD community for involvement and participation in research

Strategy 3: Provide education in Enroll-HD for professionals and lay communities

I have chosen to report on strategy 2 and 3 together as I see the objectives and activities to overlap considerably.

Activities:

Participation in meetings arranged by the HD associations in Norway, Sweden, France, Poland, Portugal, Russia, Slovenia, Italy, Finland. The meetings had both family members and professionals attending. In the meetings we presented EHA and EHND and how we see the importance of the Enroll-HD study in a big picture as a register and as a data base/tool for research. We regard it of equal importance that professionals and family members understand the significance of the Enroll-HD study and clinical trials and the need for everyone to support and encourage participation when possible. We experience a lot of interest from people but also hurdles in access to clinics and long waiting time (Norway, Sweden, Ireland, parts of Spain). Some countries like Poland, Norway, Russia express that it is difficult to prioritize premanifest participants in clinic as the neurology departments experience a high pressure on capacity and therefore tend to focus on more advanced symptomatic patients.

On the webpage we have published several articles and videos about Enroll-HD and clinical trials and the importance of taking part. Also in the videos launched on Rare Disease Day in 2017 and again in 2018 this was the message.

The use of videos is an important part of the communication strategy. We experience that recordings of people expressing their thoughts and points of view, reaches much further than written text alone. Since September 2017 the webeditor has increased hours pr month from 20 to 40. Our activity in Facebook is reaching a lot of people. The definitely most popular post on Facebook was the news about the Ionis Trial. We made a short video explaining the key message. This post reached almost 12.000 people and the video was downloaded by more than 4000. We see that it is first and foremost through Facebook that we reach the widest audience, very fast. The last post about HD-COPE has reached over 4000 and the video has been seen by more than 1600.

The last year a lot of effort has been done in order to have HD-COPE formally established. Due to the funding of Stronger together we were able to go to Canada to have a face-to-face meeting with HSC and HDSA to work out an action plan for HD-COPE. Recruitment of 10 European HD-COPE team members was done via our network and we are very happy with the

group being heterogeneous both geographically (9 countries) and in relation to HD family experience.

EHA has made several articles and two videos about HD-COPE and these are very well received by the community. It clearly seems to raise awareness of the importance of community engagement in HD research. We regard HD-COPE to be the single most important “effect” of Stronger together in the sense that all the time needed to organize and make it happen would not have been possible in the same timeline without the funding of Stronger together. Neither could the recruitment of team member have been so effective without all the contact we have had with the European HD community the last year and a half.

Strategy 4: Increase participation in Enroll-HD and clinical trials

Actions:

We have had one meeting with the key management of EHDN to discuss how family engagement can contribute to increased involvement in Enroll-HD. After these discussions we decided to approach Poland, Denmark and the Netherlands.

In Poland we have had two meetings with the ENDH Lancos and the national association. We also met with 6 family members to discuss with them how we can stimulate more young, presymptomatic people to participate in the study. As a follow-up the national association has arranged one meeting in collaboration with the EHDN Lancos and informed about the importance of the study and encouraged people to participate.

In Denmark the HD association stated that recruitment to Enroll-HD is good and people express overall satisfaction with their participation. For this reason, it was not seen as necessary to prioritize improved participation.

In the Netherlands the HD association expressed that their capacity is overloaded and found it impossible to engage in an effort to improve the engagement in Enroll-HD in the general HD community.

Due to the change in focus for Enroll-HD we regard Norway to be a good place to renew effort for recruitment. There has been a quite passive recruitment policy from the sites while waiting for the transition from Registry. We had meetings with the HD association, EHDN Lanco and Registry PI in 2016 and decided to wait with a renewed effort to enhance participation until Enroll-HD was implemented. By the mid of 2018 Enroll-HD should be up and running in Norway and this will be a very good time to launch an offensive in terms of information and encourage people to participate.

In our communication with HD affected we have learned that participation in studies like Enroll-HD has several psychological implications. People express that it can be stressful and painful to have their motor and cognitive functionality being measured. It's easy to feel that you have deteriorated. Some clinics and staff are very good in taking care of these psychological issues and provide good counselling and support. Others are not aware of the needs. We see a need for improved communication and dialogue between the staff and

participants. EHA has developed a tool to get feedback from participants. We want to use the feedback to learn more about peoples experience and in communication with sites about potential improvements. We regard this to be an important action in order to improve retention and have people follow up their commitment over time. As a pilot we will introduce a feedback survey to participants from two or three Enroll-HD sites in spring 2018.

The last year we have had several meetings with pharma companies in order to give input to their planning of clinical trials. In September we met with TEVA to discuss their plans for a Phase III trial on Pridopidine.

Roche Pharmaceuticals has had several discussions with both EHA and a meeting with HD-COPE. Both parties have expressed that the dialogue is fruitful and good. For the first time we experience a continuous collaboration between a pharma company and the family representatives from the very early stages of planning the trial. This is a huge accomplishment and will influence the quality and retention in the trial.

We have also had several meetings with Uniqure and Wave to discuss their plans of clinical trials and potential collaboration with EHA and national associations. We see a great potential for involving the associations in providing information to the general HD community and support trial participation.

Strategy 5: Impact authorities and policy making

Actions:

The establishment of HD-COPE has been the most important action taken to give ourselves a tool to improve contact with the European regulators and policy makers. An EMA representative had a presentation at the first HD-COPE training seminar in London in February 2018. The meeting was a very good two-way process where we learned about EMA and they learned about the HD community.

EHA has several family members registered in the EMA database as resource persons who can be contacted about HD. I have also applied to become a member of the Committee of Orphan Medicinal Products (COMP 17/P) in EMA. The decision will be taken by the European Commission in May 2018. The COMP committee is one of six decision making bodies in EMA. If I am accepted as member I will be in a unique position to advocate for the needs of HD families.

The last year EHA has established collaboration with the non-profit organization Active Citizenship. A main aim for the organization is better patient rights. We have participated in two “hearings” in the EU Parliament and advocated for the need for better care and improved competence in HD.

Eurordis – a non-profit patient organization for rare diseases in Europe arranges several seminars and training for patient advocates. We have had participants at seminars in Romania and England. We will also participate at their summerschool and winterschool first half of 2018. Participation in the meetings of Eurordis gives us valuable insight in other rare

diseases and strengthen our connection and collaboration with them. Eurordis has a very strong position in the EU system.

Our collaboration with EFNA as a pan-European patient organization continues. EFNA is also present in several EU meetings and we regard them as a very important collaborator in our effort to raise policymakers understanding of the situation for HD affected.

In addition to being present and make our voice heard in EMA and EU meetings we will continue to recruit advocates. The experience we have so far in HD-COPE is so good and we see a potential to have more people involved in other areas beside HD-COPE. EHA will seek to recruit around 10 more people and arrange training for them aligned with what we did for HD-COPE. The group will be invited to speak in different forums.

Conclusion:

It has been an active and successful first year and a half. The network is strengthened, more people are engaged in the European collaboration, more people understand the importance of research and the need for contribution from HD affected.

We are living in exciting times with so much promising research being brought to clinical trials. We want to thank the sponsor and CHDI for the generous support that enables us to take actively part in the efforts and hopefully make effective medical treatment reality!