

Stronger together!

January to June 2018

First half of 2018 has been a continuation of focus and activities from 2017. 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

I have chosen to structure the 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.

Visit associations and clinics and participate in meetings organized by the associations: The first half of 2018 we have participated in meetings in Gothenburg/Sweden, Gran Canary/Spain, Turin/northern Italy, Trondheim/Norway and Athens/Greece.

We have visited clinics in Las Palmas in Gran Canary Island, Groningen and Gau in the Netherlands and the University of Turin in Italy, where they do stemcell research among other HD related studies.

We see efforts being made in Italy to unite HD associations and institutions working with HD in a national network. At the meeting in Turin in May, for the first time people from the University of Milan and Turin met and discussed how they can collaborate. We think these efforts are inspired by Stronger together.

Coordinated by EHA, family representatives in Ireland, UK, the Netherlands, Germany, Italy and Czech Republic

have been actively involved in a EU call. This project aims to investigate factors that influence HD families quality of life and subsequently provide ehealth services adapted to their needs. The application is coordinated by University staff from institutions inside and outside EHDN, but the initiative came from EHA and we have been actively involved throughout the entire process.

EHA regards EHDN to be a vital resource in the European HD community. The collaboration between professional and lay entities is good, but can still be improved. We have made a plan together with members of the Executive Committee in EDHN and made several proposals to how the collaboration can be developed further both on an overall and national level.

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:

We have attended the Swedish National HD conference in April and the first national meeting in Italy in Turin in May. We worked together with the neurologist and HD association in Gran Canary island in order to have the hospital approve the new Enroll-HD contract with CHDI. Unfortunately, we did not succeed and Gran Canary is not taking part in the study.

We want to learn more about how people experience being part of Enroll-HD and have developed a survey where participants can give feedback. The survey has been tested as a pilot in Rome. In the nearest future it will be launched and used at some clinics in Germany. The responses can be used in dialogue with the sites to reinforce good practise and also potentially identify things that needs to be improved.

HD-COPE is an important key in improving knowledge and understanding for HD research. All the European HD-COPE team members are active in their national and local HD communities/associations. Roche, Wave and Unique in particular, have shown great interest in learning from the HD-COPE team members. In September the European members of HD-COPE team will meet with Wave in Vienna for an Advisory Board.

Education and raising awareness: Facebook posts and videos seems to be the channels that reach out to a large number of people. During the last 6 months we have made and launched two videos about HD-COPE and promote family members as a huge resource in the collaboration needed to develop treatment for HD. We have made a video about the Roche/Ionis trial and one about Waves project. They are all very popular. The one about Wave was published early June and has been seen by close to 19.000 people. In July we published a short video about Enroll and the importance of the study for use in research. This post has been seen by more than 1.800. The overall policy of the videos is to make them simple and present human faces. A key message is that research, drug development and advocacy is done by people – we can all do our share.

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

Actions:

Poland: National meeting in Warsaw in March for professionals and family members. EHA was present and talked about the importance of Enroll-HD from a family perspective.

Italy: Meeting in Turin with professionals and family members from all over Italy. EHA talked about the importance of collaboration and contribution from the lay community.

Sweden: National meeting in Gothenburg in April for professionals and family members. EHA talked about HD-COPE and the value of our lay expertise for research.

Norway: Regional meeting in Trondheim in June. EHA talked about the clinical trials in the pipeline for the nearest future and the importance of Enroll-HD. Norway is still not activated for Enroll and recruitment mobilization is still on hold here. We've had meetings with the new PI Lasse Pihlstrøm and the Norwegian HDA and there is high motivation to get started as soon as possible.

Collaboration between EHDN Lancos and HD associations: We have discussed with the Executive Committee in EHDN how the collaboration between the lancos and the associations can be more focused on Enroll and recruitment. EHA will send out a survey to both lancos and associations to get a better picture of how the collaboration is understood by both parties and how it can be developed further.

Strategy 5: Impact authorities and policy making

Actions:

Approval of our application for being accepted as a partner for European Medicine Agency has been delayed due to the office moving from London to Amsterdam. But we have received confirmation that we will be accepted as one of EMA's 35 partner organizations. To be accepted as a partner adds great credibility in future dialogue with both EMA and others. EHA will also be invited to an annual meeting with EMA and the other partners.

In December we applied to become a member of the Committee of Orphan Medicinal Products in EMA. The European Commission announced their decision in June and unfortunately EHA was not elected for this position.

EHA's position in Eurordis (European organization for Rare Diseases) has been strengthened. In March I attended a one week winter school organized by Eurordis for patient advocates.

We have also focused on the European Reference Network (ERN) and been active in the patient advocacy group. ERN is the main strategy for the EU to improve access to expertise and equal health services for all European citizens affected by a Rare Disease. We regard this network to be a vital tool in improving health service for HD affected in the future.

The collaboration with Active Citizenship has been continued and in April we were present in a meeting in the EU parliament about patient rights and patients voice in health related policy making.

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.

Conclusion:

First half of 2018 has been a good continuation of last year. We experience more awareness of EHA both external and internal. We also feel greater understanding and interest in cross border collaboration. More people know and understand more about the ongoing research in HD, but there is still a lot of work to be done.