

STRONGER TOGETHER – WHAT HAS BEEN ACHIEVED?

STATUS AND PROSPECTS

Stronger together has been a 3-year project starting off September 2016 and finalizes end of December 2019. The project has had 5 main objectives:

- increase the 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 project has enabled the European HD community to be better connected and have a common focus on the main objectives.

All 7 EHA board members have been actively involved in the project as well as a group of volunteers from our member associations.

Stronger together has focused on informing the HD community about the importance of all steps in clinical research to develop treatment for the disease. We have also done a lot to underline the importance of contributions from the community to take part yourself or support the ones that take an active role.

One important communication channel has been speaking at meetings arranged by the associations, visiting HD clinics and care homes and co-arrange meetings/HD weeks etc.

We have also published a lot of articles, posts on Facebook and made several videos to inform the community and encourage to be proactive.

The establishment of HD-COPE has been an important part of the capacity building. The European HD-COPE team members are all active in their respective countries. They write articles and talk in meetings to inform about the ongoing research and tell about the importance of input from the community. It is also very valuable to the European community to have a strong collaboration with the US, Canada and the other partners in HD-COPE.

There has been established family/patient representative groups in DOMINO-HD, HEALTHE-RND and CARE-HD, all EU funded projects aiming to improve the situation for HD affected families. In addition, we have one representative in the project group in the IMI project IDEA-FAST.

At the end of the final year of Stronger together, we are able to launch a European version of HD trial finder. We start with the German speaking countries and will very soon follow up with the English-speaking countries. Many more European countries will be included one by one within the end of 2020.

We have an ongoing project together with the Dutch and Flemish part of Belgium on Enroll-HD. The objective is to recruit more presymptomatic and prodromal stage participants. The main strategy is to involve the associations in a collaboration together with the Lanco and the clinics. The group is defined as an EHDN task

force, and the project has just started. We think the experience from this project can be very useful for other countries and clinics.

As a part of the aim to build increased capacity and influence in patient advocacy, EHA has been active in the European umbrella organizations in neurological disease (EFNA) and rare disease (Eurordis). 8-10 of our members have attended advocacy training arranged by EFNA during the last couple of years.

EFNA and Eurordis enjoys high credibility on the European stage and are very important stakeholders in the ongoing work to form the future health and research strategy within brain diseases and rare diseases. Our voice is appreciated, and we experience that we are able to some extent to influence the agenda and the advices given by both EFNA and Eurordis.

EHA is also very active in the patient representative groups in European Reference Networks for Rare Diseases.

As there most probably will be one or more drugs approved within a few years, we have decided to take action on how we can make sure that once approved, the drugs will be available for all patients in Europe. We have connected the leaders of the HD associations in a common effort to approach both national and European politicians and decision makers. Being a strong partner in the European neurological and rare disease communities gives us a stronger voice as our objectives are very much aligned with other disease groups.

What has Stronger together achieved since September 2016?

The network of HD affected in Europe has increased and strengthened. We have a few common goals:

- a) involve in research
- b) lobby for building an infrastructure to provide access to HD expertise and treatment
- c) lobby for future access to drugs.

For the first time 16 associations have coordinated their efforts and had one common message towards EU politicians and bureaucrats.

Enroll-HD is recruiting well, and the proportion of presymptomatic/prodromal phase participants is increasing. It's not possible to measure to what extent Stronger together has influenced this, but we will argue that it has had some importance.

Recruitment speed for Generation HD hit all previous records. The HD community is better mobilized than ever before. We need to keep up this sense of mobilization as new trials will require many participants.

Number of active patient advocates has increased considerably, and they are connected to each other. To be part of a coordinated network empowers each one and makes them more confident and stronger.

Even though it is hard to measure, we will claim that the number of individuals aware of the ongoing research in HD has increased considerably. The HD community has better knowledge and higher understanding of all the steps involved in clinical research. The community appreciate the importance of participation in research and communicate more about it in both meetings and on social media etc.

More individuals than ever are involved as patient representatives in ongoing trials and projects. These individuals take a great responsibility in informing about the ongoing work.

What are the next steps?

The European trialfinder will be a good tool to give everyone access to updated information about where the trials are executed and where they need participants. It will be used by both the associations and within their network, as well as of clinicians and researchers. The trialfinder needs to be continuously updated and extended to cover most European countries.

Keep up informing and mobilize the community. In order to maintain the level of knowledge and have people mobilized, they need to continuously inform and communicate about the progress and potential setbacks. In particular if there are setbacks, we need to inform, and also encourage to not give up. Setbacks is a part of the effort and must be expected. It will also be a huge challenge to maintain mobilization and the understanding of continuously need for volunteers to new trials when the first treatments are approved. The understanding that the first treatment is not the final answer needs to be understood in order to maintain motivation to continue to support new research and trials.

Continue to increase the proportion of presymptomatic and prodromal participants in Enroll-HD. There will most probably be a high demand for trial participants from this group in the nearest future.

Continue to lobby for access to treatment. This work has just begun, and we need to have the associations and communities all over Europe involved in communicating with decision makers and regulators about how to secure access to treatment for all patients.

This is of course not a complete list of what's needed but are crucial points on our agenda for future focus. We would love to discuss potential further collaboration with CHDI on how EHA can proceed engaging the HD community in the continued effort still needed.

Søgne November 13, 2019

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