News from the Hereditary Disease Foundation

The Magic of New Beginnings
We're excited about the beginning of a New Year and the promise 2019 holds for dramatic advances in HD research. Great things are happening…brilliant minds are at work. With your support, we will continue to push until we push HD out of the normal lifespan. Momentum begins with a moment. This is our magical moment…together we will make it happen for our families and for the future.

Manipulating HD
Anne Ast is one of the brilliant minds at work. Her exciting HDF-funded research is being conducted at the Max-Delbrück Center for Molecular Medicine in Germany.

Anne Ast

Every protein needs a specific architecture in order to execute its duties within the cell. In HD, the disease-causing huntingin protein has lost its original folding instruction and forms stable protein clumps. These clumps operate as templates and pass on their corrupted folding pattern to other normally folded huntingin molecules. It is suspected that this process could be a driving force in HD development and progression. So, Anne wants to understand the connection between templated protein misfolding and toxicity and find a way to manipulate this process as a therapeutic approach.

Breaking Down Barriers
For some of us who are not scientists, the language of research can be challenging. But the goal is the same in any language – banishing HD. Piere Rodriguez of Stanford University has received an HDF grant for his exceptional project titled: "Dissection of the function and structural communication within the Huntingtin protein and its interaction with the human TRiC chaperonin." It's all about breaking barriers.

Piere Rodriguez

We know that the risk of developing Huntington's disease is tightly linked with the length of a specific region within the Huntingin protein (Htt). Mutation can make this region longer than normal, i.e. more than 36 amino acids – producing a mutant Htt with characteristics that induce neuronal decay. The underlying mechanism behind this length-dependent toxicity remains largely unexplored. This is primarily because current biochemical/structural methods face two major technical barriers for the study of Htt: 1) Htt's high aggregation propensity, which hinders the ability to perform experiments under biological conditions, and 2) Htt's remarkable conformational heterogeneity, which has hindered structural studies of Htt with high resolution. To circumvent these technical barriers, Piere is using a novel single-molecule approach to obtain insights about the molecular mechanism that render Htt toxic, and to obtain information about Htt not accessible through traditional methods. This information is relevant for the identification of novel therapeutic targets and the development of new drugs against mutant Htt toxicity.

Funding Great Science
Each year HDF awards grants to innovative scientists who are changing the lives of families affected by Huntington's disease. Stay tuned for updates and details about other HDF-funded research projects that are underway in the US and around the world. Brilliant minds…breakthrough research…it's happening!

Read about HDF-funded research projects. Funded Research Projects.

How to Apply for a Grant or Fellowship
The Hereditary Disease Foundation is currently accepting Letters of Intent for Grants and Postdoctoral Fellowships. The Letters of Intent are due by February 1, 2019. To apply go to: Funding the Future

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