

## Excerpts from HD Blog - At Risk for Huntington's Disease

by Gene Veritas: <http://www.curehd.blogspot.ie/2015/04/engaging-scared-population-of.html>

**April 07, 2015 - Engaging a 'scared population' of Huntington's disease families by respecting their journeys**



The number of clinical trials for Huntington's disease treatments has increased exponentially, pushing up the demand for volunteers. Now advocates ask a pressing question: how to inspire more affected families and individuals to participate? I have addressed this theme with increased frequency in recent years, [...] (see blog). As a carrier of the devastating HD mutation who saw his mother succumb to the disease, I feel in my gut the urgency to involve other members of the community.

*"If no patients or gene-positive people show up for trial participation there will be no novel treatments, ever!"* Daniel P. van Kammen, M.D., Ph.D., [...] chief medical officer for CHDI Foundation, Inc., the nonprofit virtual biotech focused exclusively on developing HD treatments. [...] *"The notion that if you build it they will show up, just does not work!"* Dr. van Kammen continued. *"In general only 5% of people diagnostically eligible for drug development study participation, do so. This is fine for a large population with Alzheimer's. Not for the HD community either at risk or diagnosed. So people have to come forward."* [...]

### People deciding at their own pace

Over my nearly two decades of advocacy - my mother was diagnosed in 1995 - I have learned that it's important to respect the unique journey of each individual touched by HD. Without that respect, we cannot begin to engage what Dr. van Kammen described as a "scared population." I often wish that more individuals from the untested at-risk pool - the vast majority of those people don't get tested - would also test and/or participate in programs such as Enroll-HD, which doesn't require that people learn their genetic status.

[...] When I meet people new to HD, I am aware that I can offer ample advice based on experience. However, I stick to the basics, allowing them to ask questions and share their stories and fears at their own pace. I remind myself that testing for HD is often an extremely trying process, with implications for the extended family. This personal decision requires time and reflection. [...]

### Enjoying the present, planning the future

Twenty years into the cause and ever closer to disease onset, I need to focus on my health, enjoying life, and my family[...]. As our daughter approaches college age and my wife and I initiate conversations about retirement plans, we are also focusing on shoring up the family finances. At 55, I also face the normal challenges of aging. Over the past eight months I have struggled with a nagging elbow pain that has prevented me from swimming, my preferred exercise and excellent for cardiovascular and brain health. Luckily, with my doctor's okay, I am back swimming, although the pain continues. Because of pain elsewhere in the body, I have spent many an hour at physical and occupational therapy sessions. I want to remain as limber as possible to facilitate coping with HD symptoms. I also continue to exercise my mind. [...] *"Writing for survival is my motto."*

### Affirming the good in our lives

[...] I am grateful for remaining asymptomatic today, for the many people bravely struggling to come to terms with HD, and for the great respect the members of the HD community have for each other as we live our unique moments together. I'll be even more grateful when more people enroll in clinical trials. Yes, we may be a "scared population." But we may also be a "sacred population," one dedicated to a deeper purpose, helping to conquer this awful disease.

### May 20, 2015 - The search for Huntington's disease treatments is indeed 'rocket science' - and we can all help build the rocket

For people facing HD and other devastating, untreatable conditions, the powerful wish for a cure can conjure up the image of an elated scientist bursting from a laboratory and declaring "Eureka!"

However, it is unlikely a treatment for HD will emerge in this way[...]

From Isaac Newton to Charles Darwin to Stephen Hawking, we have oversimplified the process of discovery, prominent physicist Dr. Mlodinow explains in the May 16, 2015 edition of The New York Times.

Rather than the eureka moments popularized in books and the media like the apple falling on Newton's head these scientists' discoveries involved years of hard work and questioning of assumptions, including their own. Thus, Dr. Mlodinow reminds us that breakthroughs result from the cumulative build-up of many moments of discovery by scientists past and present. He thus underscores a crucial point for the HD community: finding treatments will necessarily involve a collective effort by scientists and volunteers in research studies and clinical trials[...]. We in the HD community must all play our part in the quest for treatments. [...]

### A road paved with cooperation

Enthusiasm is essential, but it must be tempered with the recognition that scientists need time - and money - to test hypotheses. It took some two decades to discover the huntingtin gene. At the time of this breakthrough in 1993, people in the HD community celebrated. Rightfully so, hope for treatments increased significantly.

Since then, hundreds of researchers from around the globe have published thousands of scientific papers on HD. Along the way they have identified hundreds of potential HD drug targets (biological pathways).

From the 1970s until today, thousands of individuals from HD-affected families have participated in research studies and, more recently, a growing number of clinical trials.

While many of us are disappointed that successful treatments have not emerged, we must recognise that the enormous amount of scientific work regarding HD should contribute - perhaps in ways no one yet knows - to future progress. The road to treatments is paved with cooperation, and with the recognition that multiple drugs may be needed to manage this complex genetic disorder. (Thus, scientists don't say "cure" when referring to HD.)

### Something larger than ourselves

Our society worships individual "heroes." However, in the fight to defeat HD, each participant contributes with his or her talents and resources: financial donations, scientific expertise, caregiving, and daily dedication to the cause. In this long-term commitment, we strive for the well-being of those beyond ourselves: the children who have yet to develop symptoms, the future generations of HD families, and other disease communities such as Alzheimer's, Parkinson's, and many conditions even rarer than HD. [...]

**Yes, defeating HD is rocket science. When, collectively, we have completed that rocket, we can all ride it together.**