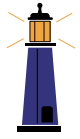


# PHAROS SPOTLIGHT



This newsletter is especially for PHAROS participants and their families.

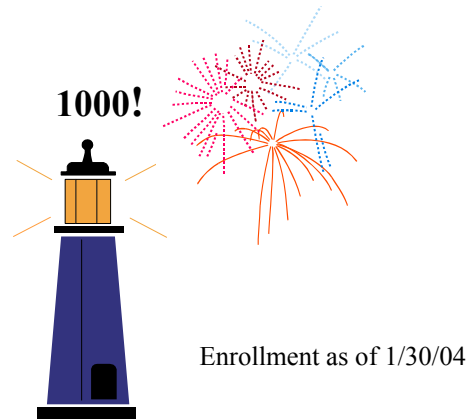
Volume 1 No. 1

2004

## *PHAROS...A Roadmap to the Future for HD*

*Dear PHAROS Participants and Families,*

*As we enter a new year, we often look back to see where we have been and how far we have come. This year is particularly exciting as we met our goal, set in 1999, to enroll 1000 participants (we actually enrolled 1001) in the first study to carefully monitor people at risk for developing Huntington's disease (HD). We would like to extend our gratitude to all of you participating in the **Prospective Huntington At Risk Observational Study (PHAROS)**. Without you, this type of research would not be possible. Your dedication to the PHAROS project is an inspiration to us and increases our resolve to make a difference in fighting HD.*



*The bi-annual PHAROS Spotlight Newsletter that you are reading is a new publication that we are proud to provide to you in return for your participation in this unprecedented research project. We hope you will find this newsletter helpful as we bring you news about the ongoing study and feature articles about other activities in HD. Now that we have enrolled the entire cohort, we anticipate that analyses will be done shortly on the group's baseline data and published within the next year. Please feel free to contact your site coordinator throughout the year if you have questions or concerns. We look forward to a year of new discoveries.*

*Sincerely,*

*The PHAROS Research Editorial Team*

*Susan Bernstein, Jody Goldstein, Carolyn Gray, Vicki Hunt, Elise Kayson, Letty Laskowski, Ania Mikos, Carol Moskowitz, Aileen Shinaman, Marguerite Wieler*

## What is the History of PHAROS?

Ideas about the PHAROS study started in the mid 90's when researchers realized that before we could test experimental drugs in healthy individuals who carry the HD gene, we needed to address important issues involving all persons at risk for HD, whether or not they have inherited the abnormal HD gene. As you recall from the consent you signed, your participation in this study will help us to answer the questions about the first signs of the disease and determine those tests that are most effective at detecting the disease in its earliest phases.

The study began in May 1999, headed by Drs. Ira Shoulson (Principal Investigator) and Anne Young (Co-Principal Investigator) and was funded for 3 years through the support of the advocacy organizations HDSA, HDF, and the High Q Foundation. In September 2002 the National Institutes of Health (NIH) funded this study for at least an additional 5 years of follow up.

## What's

As the study progressed and new information emerged, necessary changes were made to the project, including the implementation of surveys about study confidentiality and your personal perceptions about life events, medical history and your habits. The life event surveys will help us understand the choices made by people who are at risk for a disease. We suspect there are other factors that may also contribute to the onset of HD. The environment may be one of those factors, which is why we are asking about your medical history and your life habits (e.g. smoking, eating, medications, etc.). Answers to these questions may not only benefit you but will also be helpful in the study of other diseases as well.

As we move forward there may be other new discoveries that will necessitate changes to the study and your involvement. Your dedication to the study and flexibility with those changes are key to making PHAROS successful. You are a special group of people, and your participation in the study is critical in helping us to make a difference in HD.

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## Meet the Researchers

We would like to introduce to you, two very important people who had the vision to make this study happen: **Dr. Ira Shoulson and Dr. Anne Young.**

Dr. Ira Shoulson started out in internal medicine, and then began a research fellowship at the National Institute of Mental Health (NIH). Though not a neurologist at the time, Dr. Shoulson became interested in Huntington's disease through his work with Dr. Tom Chase and a group of Huntington's disease research participants. In 1973, during his time at the NIH, Dr. Shoulson conducted his first HD study. The study looked at the effects of lithium and valproate in treating HD. He would later go back to the University of Rochester to complete a specialty in neurology. Throughout his career, Dr. Shoulson has been a member of numerous neurological associations and advisory boards, including the Huntington's Chorea Research Group for the World Congress of Neurology, the Scientific Advisory Council of the Huntington Society of Canada and the National Institute of Neurological Disorders and Stroke (NINDS) Advisory Council. Currently, Dr. Shoulson serves as the Louis C. Lasagna Professor of Experimental Therapeutics and as Professor of Neurology, Pharmacology & Physiology, and Medicine at the University of Rochester School of Medicine. He also continues his practice as a neurologist at Strong Memorial Hospital. Dr. Shoulson resides in Rochester, NY with his wife and two children.



Dr. Anne Young has always been fascinated with biology. Growing up in a household where science was often discussed, she decided to pursue scientific research in college. At the suggestion of a family friend, she decided to look into a joint MD/PhD program when applying for graduate school. Dr. Young chose Johns Hopkins and pursued a course of study of her own design, which included a PhD in pharmacology. Her research dissertation led to the publishing of numerous papers and invitations to speak at national meetings. Dr. Young went on to train in neurology and would become a professor at the University of Michigan. While at Michigan, Dr. Young became interested in movement disorders and was attracted to

researching HD after working with a family. From 1981-1991 she was involved in her first HD study, PET (positron emission tomography) in HD. Several years later she accepted the appointment as Professor and Chair in the Department of Neurology at Massachusetts General Hospital, a position she holds today. Dr. Young resides in Boston, MA and has two daughters.

In 1981, Dr. Shoulson and Dr. Young began working together, along with Dr. Nancy Wexler and a team of researchers from around the world, on a study known as the U.S. Venezuela HD Project. It would be the first of many trips to numerous small villages on Lake Maracaibo, where a large concentration of inter-related persons with HD was identified. Yearly, the researchers collected information from these families and were eventually able to isolate the gene that causes HD. In 1993 Dr. Shoulson and Dr. John (Jack) Penney, Anne Young's husband, established the Huntington Study Group, a non-profit organization searching to find treatments for HD. The idea of PHAROS and its counterpart study PREDICT (Neurobiological Predictors of HD) stemmed from a desire to observe healthy people who were at risk of developing HD.

**We asked the two researchers what their primary goals were for Huntington's disease:**

**Dr. Shoulson replied, "To develop a treatment that slows the progression or postpones the onset of HD."**

**Dr. Young expressed a similar sentiment. Her primary goal is "to find a cure for Huntington's disease."**

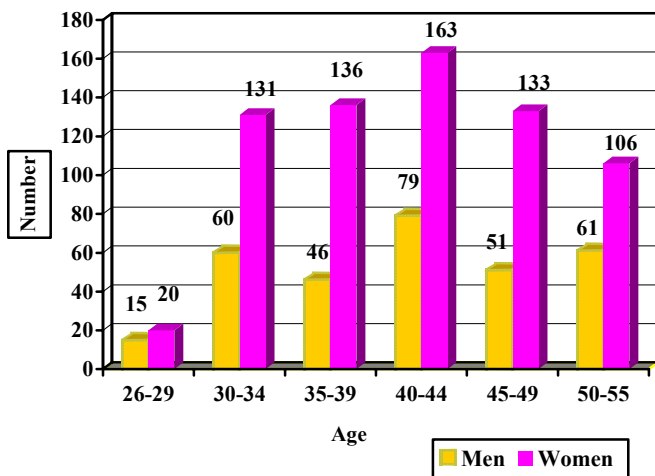
## Who is Participating in PHAROS?

Of the 1001 participants in PHAROS 69% are women and 31% are men with a mean age of 42 years. The ethnicity is predominantly Caucasian. As you can see, we have many more women than men. The reasons for this disparity are not clear, but we are grateful for the 312 men as well as the 689 women who have agreed to participate.

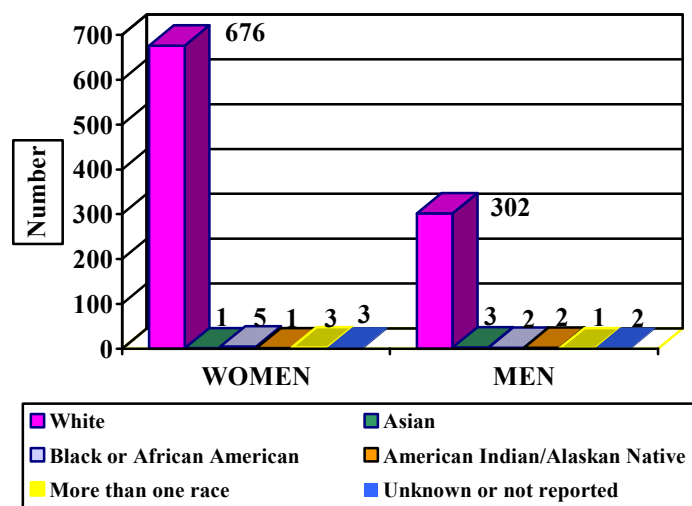
*“I guess they think I’m terrific because I am a man. Not many men have had the guts to join PHAROS. So, you see, in a way, I’m really an exceptional guy.”- Anonymous -*

As you can see from the graphs below we have exceeded our enrollment goal by 1 participant (1001)!

Age and Gender Distribution of Participants  
N=1001



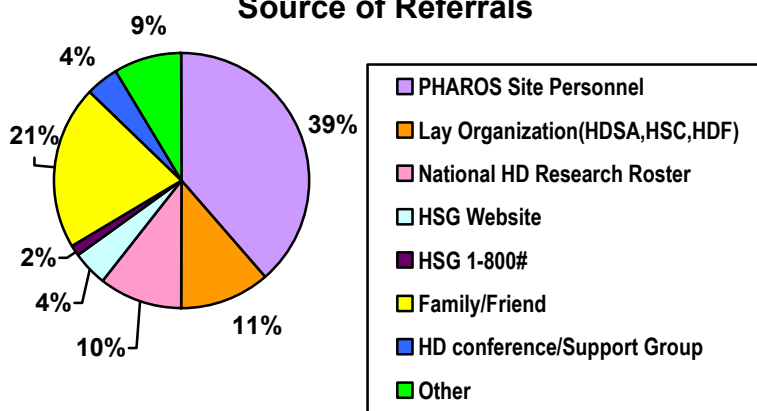
Race Distribution of Participants



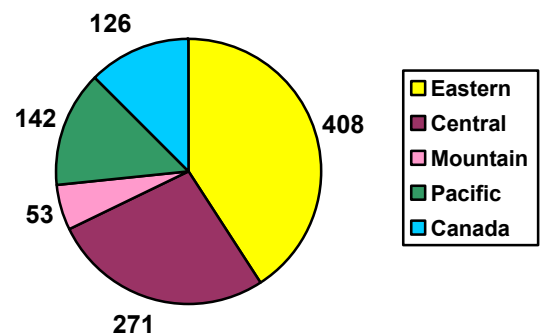
## Where Did You Find Out About PHAROS?

It is interesting to note that 39% of our participants came directly from the participating sites. That means many of our investigators and coordinators already had a relationship with you and your family before PHAROS. A family member or friend referred 21% of participants. Thank you to all who passed along the word about PHAROS.

Source of Referrals

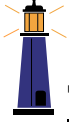


Number of Participants by Region

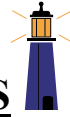


## **Benefits of Participation**

We've heard from some of our participants about why they like being involved in PHAROS and thought we'd share some of their comments with you. We would be interested to know why *you* have volunteered for PHAROS and continue to stay in this observational study. Please contact the coordinator at your site if you would like to contribute any thoughts to the newsletter. We will continue to select comments and print them anonymously in future Newsletters.



### **TOP 5 REASONS TO BE IN PHAROS**



#### **◆ I AM CONNECTED ◆**

“The primary reason for me is to stay connected to people who are expert HD clinicians. I feel comfortable knowing that they will be there ‘if’. I have the inside track on what is new. When an announcement hits the papers promising ‘exciting new developments’ in HD research, I no longer get on that emotional rollercoaster of high hopes followed by a crash of disappointment. I pick up the phone and call the study coordinator and ask what does this really mean for my dad or for me now and in the future.”

#### **◆ IT FEELS GOOD ◆**

“Although I get jumpy, anxious, and nervous before each visit, it also makes me feel good. I’m really grateful to be able to do the visits. I know my participation is appreciated. They thank me all the time. I am respected for my volunteering.”

#### **◆ IT’S MY BIG SECRET ◆**

“Confidentiality is a big reason for my staying in PHAROS. I don’t worry about having someone find out. The study staff is very serious about keeping my identity from anyone else. I have learned that my family history should not be so openly shared. It never occurred to me before participating in PHAROS that this was such a big issue. Here’s another benefit. I finally have long term care insurance, just in case. Everyone should. I know all this but always put it off. Do it for yourself and your family.”

#### **◆ WHO ELSE BUT ME? I’M THE RESPONSIBLE TYPE ◆**

“It doesn’t seem such a big deal to participate in PHAROS because years ago when it first started my grandmother put our family in the Indiana Roster. My mother did a drug study. She came in here every couple of months and took medication that could have been nothing but a placebo. She did it for us kids. So, why wait and worry? I am doing this for me, my brothers and sisters, and of course all our kids. I am not the kind of person to just wait and see if I get HD, I’m doing something about it. At this point, I guess it’s a family tradition.”

#### **◆ WE ARE PIONEERS ◆**

“In a sense, there has been no other time in history when people at risk can make such a major difference. This is a once-in-a-lifetime opportunity to make a unique contribution. It is a gift of knowledge to researchers, who, after all, are working toward OUR DREAM! Being part of PHAROS means that I am working toward a better future for my family and yours.”

## Topics of Interest



### **Did you know?**

PHAROS was a Greek island with a large lighthouse, supposedly the first ever built (283-246 B.C.), near Alexandria, Egypt. It guided sailors through the Aegean Sea passage. This structure became famous, first among mariners and later to people in general. In time, the Greek word “Pharos” became not only the name of the lighthouse, but also a common noun for all lighthouses. We chose the term PHAROS for our study and the lighthouse for our logo as the guiding light for HD research.

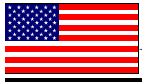
### **What has your study team published recently?**

- Huntington Study Group (E Kayson, presenter) Safety and Feasibility of the Prospective Huntington at Risk Observational Study (PHAROS): A Preliminary Report. *Neurology* 2002 58 (suppl 3):A334-335, abstract #S46.005.
- Huntington Study Group (MA Shinaman, presenter) Willingness to consent for future genetic testing by individuals at risk for or with Huntington’s disease: A preliminary report *Mov Disord* 2001; 16:7.
- Huntington Study Group (E Kayson, presenter). Preliminary report of the safety and feasibility of a controlled observational study of adults at risk for Huntington’s disease (PHAROS). *Proceedings of the 19th International Meeting of the World Federation of Neurology Research Group on Huntington’s Disease*, August 25-28, 2001, Copenhagen, Denmark. Publication of the Panum Institute, p. 30, 2001.

These and other abstracts from HD researchers can be found on the Huntington Study Group website: [www.huntington-study-group.org](http://www.huntington-study-group.org)



## Upcoming Events



### U.S. Events

- The 19th Annual Huntington's Disease Society of America (HDSA) Convention will be held from **June 11-13, 2004** at The Adams Mark in St. Louis, Missouri. Information and registration for this event will be available shortly. If you have any questions, please contact HDSA at [hdsainfo@hdsa.org](mailto:hdsainfo@hdsa.org) or toll free at **1-800-345-4372**.

For more information on national or local events see [www.hdsa.org](http://www.hdsa.org)



### Canadian Events

- The Huntington Society of Canada (HSC) will hold its annual conference in Winnipeg from **October 28-30, 2004** at the Holiday Inn Airport West in Winnipeg, Manitoba. For more information call the HSC toll free in Canada **1-800-998-7398** or email [info@hsc-ca.org](mailto:info@hsc-ca.org)

For more information on national or local events visit: [www.hsc-ca.org/english/events.htm](http://www.hsc-ca.org/english/events.htm)



### World Events

- World Congress on Huntington's Disease (WCHD) will hold its bi-annual conference in Manchester, England. It will take place **September 10-13, 2005** at the Crowne Plaza Midland Hotel.

For more information see the WCHD website at: <http://www.hda.org.uk/congress/index.html>

## For More Information



"Huntington Study Group"

[www.huntington-study-group.org](http://www.huntington-study-group.org) or contact the HSG toll free at **1-800-487-7671**

"Huntington's Disease Society of America"

[www.hdsa.org](http://www.hdsa.org) or contact HDSA toll free at **1-(800) 345-HDSA (1-800-345-4372)**

"Huntington Society of Canada"

[www.hsc-ca.org](http://www.hsc-ca.org) or contact HSC at **1-800-998-7398**

"Huntington Project"

[www.HuntingtonProject.org](http://www.HuntingtonProject.org)

"Hereditary Disease Foundation"

[www.hdfoundation.org](http://www.hdfoundation.org)

"International Huntington Association"

[www.huntington-assoc.com](http://www.huntington-assoc.com)

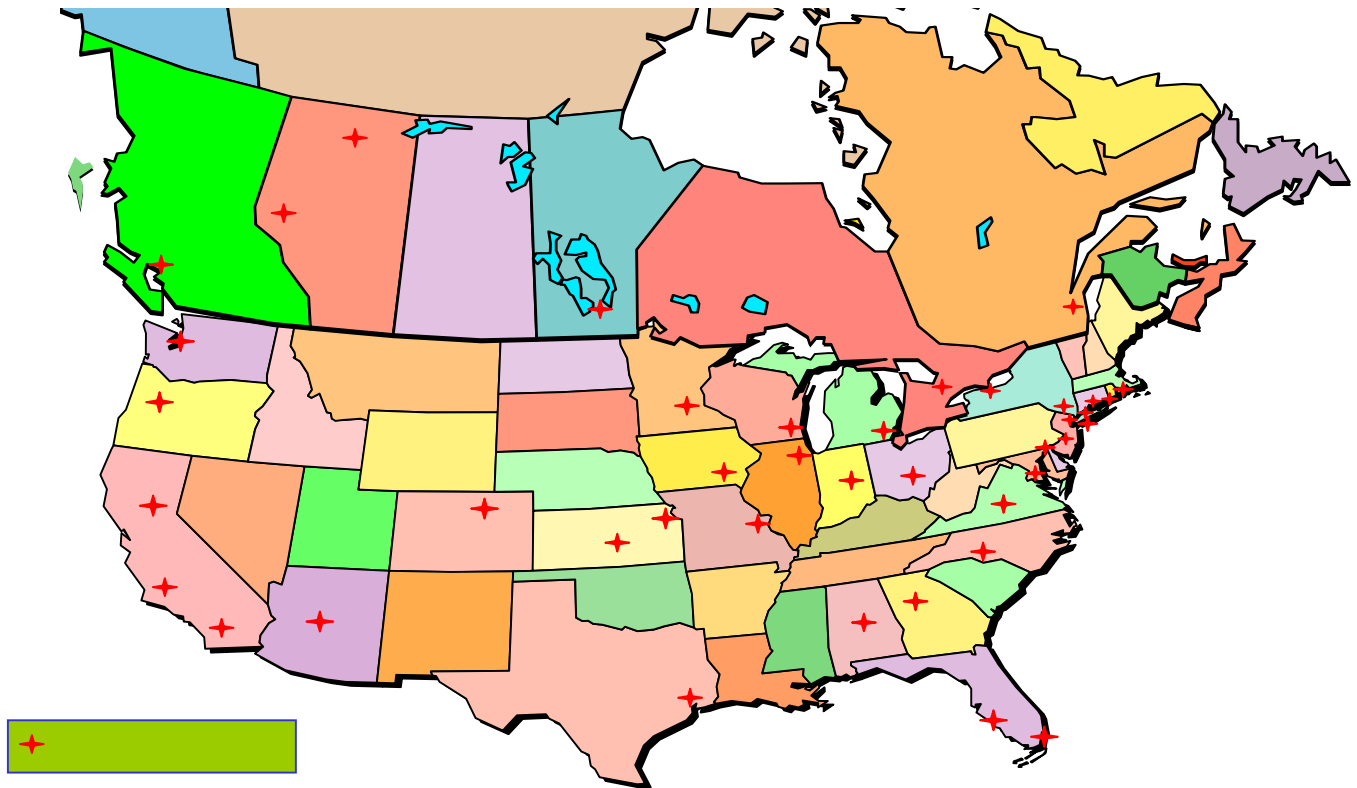
## Upcoming Issues

- **What's New in PHAROS?**
- **Topics of Interest**
- **HD Research Update**
- **New Publications**
- **WCHD 2005**

### Editorial Board

Susan Bernstein  
Jody Goldstein  
Carolyn Gray  
Vicki Hunt  
Elise Kayson  
Letty Laskowski  
Ania Mikos  
Carol Moskowitz  
Aileen Shinaman  
Marguerite Wieler

## PHAROS Participating Sites



University Of Alabama At Birmingham, AL  
 Mayo Clinic Scottsdale, AZ  
 University of California Davis, CA  
 Colorado Neurological Institute, CO  
 UCLA Medical Center, CA  
 University of California, San Diego, CA  
 Institute for Neurodegenerative Disorders, CT  
 University of Connecticut, CT  
 University of Miami, FL  
 University of South Florida, FL  
 Emory University School of Medicine, GA  
 University of Iowa, IA  
 Rush-Presbyterian-St. Luke's Medical Center, IL  
 Indiana University School of Medicine, IN  
 Hereditary Neurological Disease Centre, KS  
 University of Kansas Medical Center, KS  
 Boston University, MA  
 Massachusetts General Hospital, MA  
 Johns Hopkins University, MD  
 University of Maryland School of Medicine, MD  
 University of Michigan, MI  
 University of Minnesota, MN

Washington University, MO  
 Wake Forest University School of Medicine, NC  
 UMDNJ, NJ  
 North Shore University Hospital, NY  
 Albany Medical College, NY  
 Columbia University Medical Center, NY  
 University of Rochester, NY  
 University of Pennsylvania, PA  
 Ohio State University, OH  
 Oregon Health & Science University, OR  
 Brown University, RI  
 Baylor College of Medicine, TX  
 University of Virginia, VA  
 U of Washington/ VA Puget Sound Health Care System, WA  
 Medical College of Wisconsin, WI

### Canada

Hotel-Dieu Hospital-CHUM, QC  
 The Centre for Addiction and Mental Health, ON  
 University of Alberta, AB  
 University of British Columbia, BC  
 University of Calgary, AB  
 Winnipeg Clinic, MB