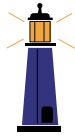


# PHAROS SPOTLIGHT



This newsletter is especially for PHAROS participants and their families.

Volume 2

January 2005

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

*Dear PHAROS Participants and Families,*

*Welcome to our second edition of the PHAROS Spotlight newsletter. We would like to start by thanking you all for taking the time to fill out your PHAROS Surveys at each visit! With enrollment completed the focus of our attention in the next few years will be on maintaining the well being of our PHAROS participants and your interest in the PHAROS study. We are excited to announce that we have begun to review the data you provided at your first visit. We plan to provide you with information about the progress of the study and any findings that are of interest to you and your families.*

*In order to accomplish these goals and ensure the continued success of PHAROS we must continue working together. Through this combined effort from HSG Researchers and PHAROS Participants we hope to maintain your PHAROS participation and learn more about ideas you have to improve your PHAROS experience. Please feel free to contact your site coordinator and investigator if you have any questions or concerns. We look forward to finding what the Roadmap to the future for HD will reveal!*

*Sincerely,*

*Ira Shoulson, MD  
Principal Investigator*

*Anne B Young, MD, PhD  
Co-Principal Investigator*

### **Newsletter Articles**

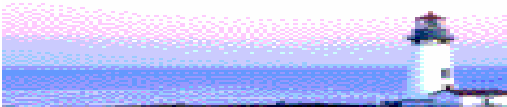
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## What's

As the needs of our PHAROS participants have changed over time we have had to be very aware of the impact of these changes. As a result, we have adjusted our study to ensure the safety and well being of our PHAROS participants. Participants have informed us via survey comments that they would like more contact with the sites and, most importantly, feedback about the progress of the study. “[You should consider a] possible follow-up call (2 days) after the first initial visit – it might help delayed anxiety,” wrote one participant. Another commented, “Since appointments are made so far in advance, a reminder call or note a few days before the scheduled appointment would be beneficial.”

Partly in response to some of the suggestions we have received and the needs of our participants, the PHAROS Steering Committee recently amended the study and the consent form to ensure greater clarity in the procedures for discussing clinical concerns with participants and to enhance communication with our participants. With the participants’ permission, these new changes require the addition of a telephone contact between visits, distribution of PHAROS updates via mail and a request for the participant to name a back up contact person (when all efforts to contact you have failed). We have also supplied the sites with PHAROS Appointment Reminder cards (*see below*) to assist with scheduling and to provide easy access of site contact information. Additionally, we have made the PHAROS Spotlight newsletter available online (<http://www.huntington-study-group.org/PHAROSNewsletterVolumes.htm>)

### Example of New Appointment Reminder Cards

<b>PHAROS</b>	
<b>Sun Mon Tue Wed Thur Fri Sat</b>	
<b>Date</b> _____ <b>at</b> _____ <b>AM</b>	<b>Name of Site Investigator</b>
_____ <b>PM</b>	<b>Name of Site Coordinator</b>
	<b>1-800-487-7671</b>

*Front of card*

*Back of card*

## Meet the Researchers

We would like to introduce to you a group of key people who oversee this study: the PHAROS Steering Committee. Members of the Steering Committee (SC) are chosen by the Principal Investigator and Co-Principal Investigator. The Steering Committee is comprised of several Huntington Study Group (HSG) investigators that may or may not be directly involved in the evaluation of subjects for PHAROS, including: a chief biostatistician (who is in charge of analyzing the data), the director of the project and data coordinating center and a project manager for the study. Responsibilities of the Steering Committee include selecting site investigators to conduct the study, overseeing the well being of the study participants, assisting in implementing the study, and analyzing and reporting the study results. Most importantly, the Steering Committee strives to adhere to the Huntington Study Group policies governing confidentiality of research subjects, conflict-of-interest and publication.

The following individuals are members of the PHAROS Steering Committee:

**Ira Shoulson, MD (Principal Investigator)**, *University of Rochester, Rochester, NY*; **Anne Young, MD, PhD (Co-Principal Investigator)**, *Massachusetts General Hospital, Boston, MA*; **Steve Hersch, MD, PhD**, *Massachusetts General Hospital*; **Elise Kayson, MS, RNC** (Project Manager), *University of Rochester*; **Karl Kieburtz, MD** (Director of Project and Data Management Center), *University of Rochester*; **Karen Marder, MD**, *Columbia University Medical Center, New York, NY*; **David Oakes, PhD** (Chief Biostatistician), *University of Rochester*; **Jane Paulsen, PhD**, *University of Iowa, Iowa City, IA*; **Kimberly Quaid, PhD**, *Indiana University School of Medicine, Indianapolis, IN*; **Aileen Shinaman, JD** (HSG Executive Director), *University of Rochester*; **Eric Siemers, MD**, *Lilly Corporate Center, Indianapolis, IN*; **Caroline Tanner, MD**, *The Parkinson's Institute, Sunnyvale, CA*; **Hongwei Zhao, PhD** (Biostatistician), *University of Rochester*.

This issue we will feature three members of the PHAROS SC. Karen Marder, MD, Kim Quaid, PhD, and Caroline Tanner, MD have been instrumental in developing the participant surveys.



**Caroline Tanner, MD**

*The Parkinson's Institute, Sunnyvale, CA*

I started work in Huntington's disease research as a resident in neurology, in 1979, when I investigated the effects of lecithin as a treatment for Huntington's disease. My first introduction to Huntington's disease was as a medical student working in my mentor's (Harold Klawans, MD) movement disorder's clinic. My goal is to find a way to prevent or to cure the disease. While the path to that goal is not an easy one, I believe

that through the combined efforts of scientists, persons with Huntington's disease and their family members, we will conquer this.

**Karen Marder, MD**

*Columbia University Medical Center, New York, NY*

I have been involved in HD research since 1987 when, as a fellow in behavioral neurology with Richard Mayeux MD, I assisted in a pre-symptomatic testing program directed by Nancy Wexler PhD. I was immediately hooked. In 1991, Carol Moskowitz RNC asked me if I would help her start a center for HD families.



Subsequently I began participating in the US Venezuela Collaborative research project in 1993. Both experiences have been tremendously rewarding. My primary interest has been to use my neuroepidemiology background in an effort to characterize the natural history of people with, or at risk for HD so that we can more accurately plan therapeutic interventions.



**Kimberly Quaid, PhD**

*Indiana University School of Medicine, Indianapolis, IN*

I became involved in Huntington's research in 1983 while I was finishing my Ph.D. in Psychology and Public Health at The Johns Hopkins University. This was just after the first linked markers to the HD gene were found. I became the coordinator of one of the first programs offering HD testing to persons at risk at Johns Hopkins. In 1990, I was recruited by Michael Conneally, PhD. to set up a testing program in Indianapolis, Indiana. I continued my work offering testing and became more interested in the ethical aspects of providing genetic testing. My ultimate goal for HD research is to find a cure for HD, or at least a treatment that can delay the onset of the disease by several decades.



## Topics of Interest

### **Why are these surveys so long?**

A little over a year and a half ago we added new questionnaires looking at Life Events and Environmental issues. These questionnaires asked a range of questions about your personal values, life events and dietary history. The study coordinators developed inventive ways to make completion of the surveys as easy as possible - even giving out cookies and milk while participants fill out the surveys. Interestingly, we have had a tremendous amount of mostly positive feedback to help guide us as we continue to learn. The suggestions we received from you have inspired the creation of this newsletter and the opportunity for you to complete the questionnaires before your study visits.

“Why are these surveys so long?” is a remark frequently made in the comments section of the PHAROS questionnaires. These extensive survey questionnaire packets were developed under the direction of Karen Marder, MD, Kimberly Quaid, PhD, and Caroline Tanner, MD. The goal of these surveys is to try and identify a variety of potential factors that might help determine if any correlations to HD onset emerge, to develop better clinical care practices, future research studies and legal policy. Each questionnaire was selected for a specific reason. Confidentiality, and the consequences if breaches of confidentiality occur, is important to determine if disclosure of gene status (intentional or inadvertent) would affect your ability to obtain health insurance or obtain and keep your job and familial relationships. Little is known about your life experiences, your family relationships, your personal attitudes and beliefs and how being at risk impacts the decisions you make. We do not know if or how environmental exposures affect onset of HD. In PHAROS, the researchers are looking for potential environmental modifiers (such as diet, caffeine, medications, nutritional supplements, head injury, alcohol and tobacco use) that might influence the onset of HD.

It is very important to formally ask about the ethical and social challenges faced by our PHAROS participants. In future newsletter issues we will discuss some of the preliminary findings of the surveys. We are very grateful that you have agreed to spend the additional time required at each visit to complete the questionnaires. This information will help us better understand what it is like to live at risk for HD.



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

Huntington Study Group/PHAROS Investigators (Elise P. Kayson, University of Rochester; Rochester, NY). The Welfare of Research Participants in the Prospective Huntington at Risk Observational Study (PHAROS). *Mov Dis* 19:9, 2004.

Huntington Study Group/PHAROS Investigators (Elise P. Kayson, University of Rochester; Rochester, NY). Features and Well Being of the Prospective Huntington At Risk Observational Study (PHAROS) Cohort. *HDF Summer Symposia*, August 2004 Cambridge, MA.

Huntington Study Group/PHAROS Investigators (Aileen Shinaman University of Rochester; Rochester, NY). Willingness to Consent for Future Use of Biological Specimens in the Prospective Huntington At Risk Observational Study (PHAROS). *HDF Summer Symposia*, August 2004 Cambridge, MA.

Huntington Study Group/PHAROS Investigators (Elise P. Kayson, University of Rochester; Rochester, NY). Depression and Suicidality at Baseline in the Prospective Huntington At Risk Observational Study (PHAROS). *Mov Dis* 2004; 19(9):1128.

Huntington Study Group/PHAROS Investigators (Kevin M. Biglan, MD, Johns Hopkins School of Medicine, Baltimore, MD). Baseline Characteristics of the Prospective Huntington At Risk Observational Study (PHAROS) Cohort. *Annals of Neurology* 2004; 65(suppl 8).

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)



**HDSA**

## **HDSA Annual Convention**

The Huntington's Disease Society of America (HDSA) held its nineteenth annual convention for patient families and allied health care professionals at the Adams Mark Hotel in St. Louis Missouri from June 11-13, 2004. There were more than 500 HD family members in attendance. The convention featured research, care and advocacy forums as well as workshops geared for the various stages of HD. The theme of the convention was "Reaching for Excellence in Care and Cure."

The atmosphere was warm and friendly yet very informative. Presenters included some of our own PHAROS study personnel: Peter Como, PhD, from the University of Rochester (Rochester, NY) who presented "HDSA/Center Initiatives" and Martha Nance, MD, from the University of Minnesota (Minneapolis, MN) who presented "Clinical Research Update: New Developments from the Huntington Project, PHAROS and PREDICT-HD and Updates on CoQ10, Creatine, Riluzole and Tetrabenazine," as well as conducting a workshop, "Understanding Behavioral Features and Managing Cognitive Symptoms."

The true essence of the HDSA convention can be summed up by using the name of one of the workshops entitled, "Sharing and Caring."

The HDSA 20<sup>th</sup> Annual Convention will be held at the Atlanta Hilton in Atlanta Georgia from June 24-26, 2005. For more information please check the HDSA national web site at [www.hdsa.org](http://www.hdsa.org) after January 1, 2005.

## Who is Participating in PHAROS?

We reviewed our enrollment information and found that 70% of our participants are married, average  $14.9 \pm 2.6$  years of education and 96% are employed predominately in a professional capacity.

Recruitment of 1001 PHAROS research participants was achieved in 4.5 years.

### Occupation During Most of Career

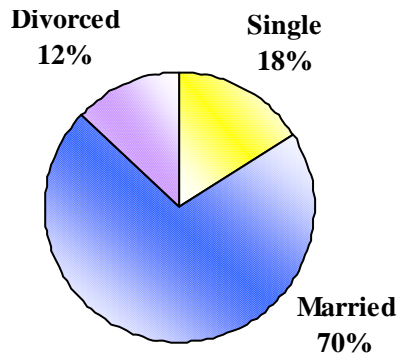
(N=1001 participants)

	N	(%)
<b>Professional</b>	<b>455</b>	<b>(46)</b>
<b>Managerial</b>	<b>357</b>	<b>(36)</b>
<b>Service Worker</b>	<b>81</b>	<b>(8.0)</b>
<b>Craftsman</b>	<b>52</b>	<b>(5.0)</b>
<b>Not in Labor Force</b>	<b>43</b>	<b>(4.0)</b>
<b>Laborer</b>	<b>13</b>	<b>(1.0)</b>

### Marital Status

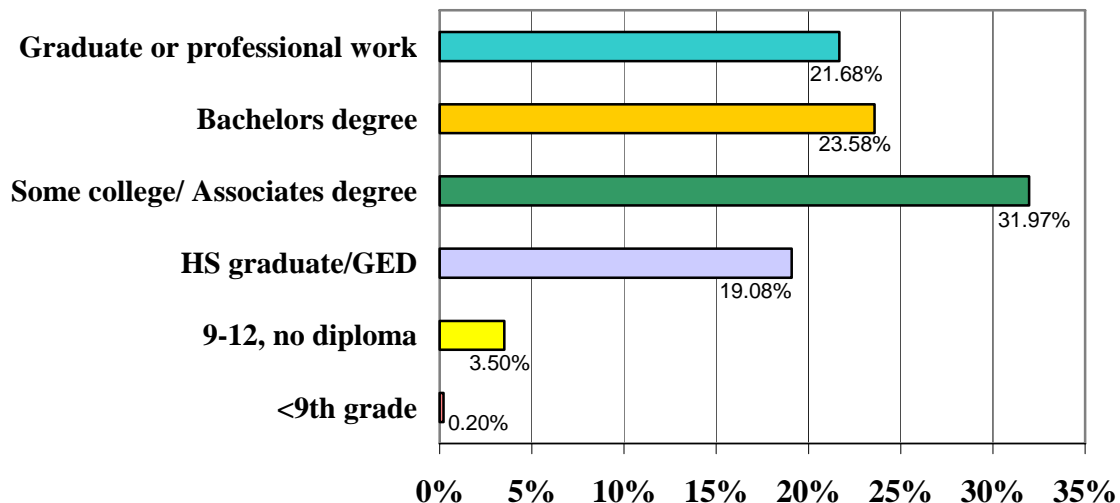
N=1001

PHAROS participants are largely married with a divorce rate paralleling U.S. census reports.



### Highest Education Level Achieved

(N=1001 Participants)



## Thoughts on Participant Retention

What is the PHAROS participant looking for during a study visit? What makes one PHAROS participant return time and time again and another not? Current publications on the topic of recruitment and retention do not address studies for HD research. Information from PHAROS will help define more effective and sensitive recruitment and retention methods for future studies.

Survey responses were recently reviewed to get a better idea of how participants viewed their study visits and to find out what we can do to improve visits. That is, we tried to understand the visit through the eyes of the participant.

There were many comments about time constraints. “I appreciate that the researchers are considerate of the time and get me in and out rather quickly,” said one participant. Another remarked “Consider looking at the coordination of the actual visit day so it’s organized and I can get in and out without a lot of delay.”

Numerous reasons for continued participation were cited. Some return because they have developed a relationship with the site staff, “I feel I have come to know the investigators, I was happy to find they were willing to give me as much time as I needed to talk about any issues I had.” Others keep coming back because being in the study is important to them and their children’s future, “Being involved has kept me better informed and more positive about my risk for HD.” One person who decided to stop participation indicated, “I completed 5 visits and felt this was all I felt comfortable contributing.”

After PHAROS survey responses were reviewed, the PHAROS site investigators and coordinators were polled to identify successful scheduling techniques and practices. The site coordinators’ scheduling practices and techniques were impressive. First priority is given to scheduling a PHAROS visit when it is most convenient to the participant. Important consideration is given to the participant’s work schedule, providing parking tokens, calling or mailing reminders, and preserving confidentiality. The common goal is to “work it out.” Mary Lou Klimek, site coordinator from the University of Calgary, adds, “We just keep juggling our schedule to accommodate people’s needs. It’s because my participants are so great that I keep trying to work it out.” Other coordinators added similar comments indicating that their respect for the PHAROS participants is a major motivator.

As an HSG community of PHAROS participants and researchers we will need to continue to work closely together to meet the objectives of this study over the next several years.



## Upcoming Events



### U.S. Events

- The 20<sup>th</sup> Annual Huntington's Disease Society of America (HDSA) Convention will be held June 24-26, 2005 in Atlanta, GA. 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 dates and venue of the 2005 Huntington Society of Canada (HSC) annual conference have not been published yet. 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. The Congress is a joint meeting of the World Federation of Neurology Research Group on Huntington's Disease and the International Huntington Association (IHA).

For more information see the WCHD website at: <http://www.hsc-ca.org/english/congress/index2.htm>

## 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**

"PHAROS Spotlight" newsletter online

<http://www.huntington-study-group.org/PHAROSNewsletterVolumes.htm>

"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"

<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)

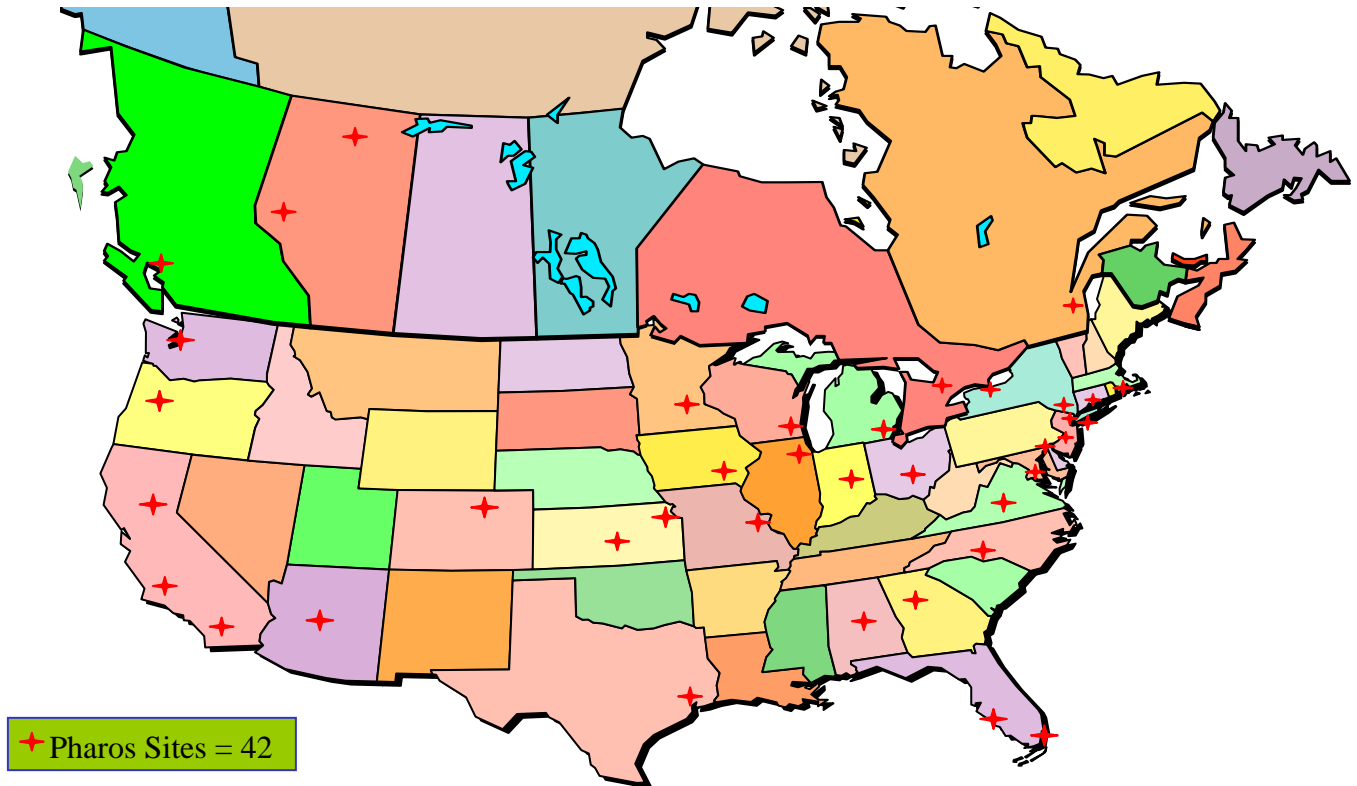
## Upcoming Issues

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

### Editorial Board

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 Letty Laskowski (University of Rochester)  
 Carol Moskowitz (Columbia University Medical Center)  
 Aileen Shinaman (University of Rochester)  
 Greg Suter (Hereditary Neurological Disease Centre)

## 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  
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 University of Virginia, VA  
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 Medical College of Wisconsin, WI

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