At the Huntington Study Group Plenary Session, Dr. Ira Shoulson recognized several individuals who have made significant contributions to the HD Community, by bridging the gap between the scientists and the lay population. He made a special point of recognizing the contributions of Marsha Miller for her work. We are fortunate that Marsha has become a regular contributor and manages the research section of this website, in addition to her years of service through the HD Lighthouse.

Dr. Shoulson also cited the work of Dr. LaVonne Goodman, and her invaluable website, HDDrugworks.org. We are fortunate that Lavonne also contributes her insights to the HDSA website, (including the following piece on the HSG meeting).

I hear from HD Community members regularly about how grateful they are that Marsha is keeping them aware of what is going on in the research world – in a manner they can understand and follow. It's my great pleasure and honor to work with both Marsha and LaVonne, and I applaud the HSG for recognizing the important contributions they both make. And as anyone who knows them, it's important to understand that their contributions go way beyond these reports, and their websites. My thanks to Jean Miller as well, who contributed to the following report on HSG. Jean keeps those of us at HDSA and the entire HD Community aware and on our toes on a constant basis.

Fred Taubman
Director of Marketing Development and Communications, HDSA

Highlights from the 2008 Huntington Study Group Meetings and Symposium

LaVonne Goodman M.D. and Jean Miller

December 4, 2008

The Huntington Study Group (HSG) held its annual meeting in St. Petersburg, Florida November 12-14 2008 for clinical investigators and invited guests. These meetings culminated in the 2nd annual HUNTINGTON DISEASE CLINICAL RESEARCH SYMPOSIUM. The HSG meetings and the Symposium are unique research events because the single focus is clinical research, or that which is limited to human study.

The HSG Symposium is not only unique, it is -- by design -- a remarkably inclusive event. HSG leader Ira Shoulson purposely planned the symposium to be a place where researchers and patient families sit side-by-side to learn from presentations and each other. As in the previous year, Huntington family representatives were included all the way from the beginning planning stages, to participation of more than 100 families in this year's final event.

Symposium Highlights

Keynote Speaker Charles Sabine: Mr. Sabine, an NBC correspondent set the stage for the Symposium with a powerful and energizing address. By alternating his personal news clips from war torn countries with photographs of his Huntington-stricken family he wove a visual tapestry of suffering, intermingled with images of heroic care for the afflicted, and lastly of human hope that spurs action to overcome obstacles which may at first seem impossible.

Citing recent U.S. election results, Mr. Sabine stressed the important role that hope in the larger community can play, both to start the work and to maintain the momentum needed to overcome obstacles: By analogy, if it is possible to see a black man as president of the United States, it can be possible to organize effectively, and to find treatments for Huntington's. He also stressed that there can be hope in the Huntington family community only when research information is shared by all -- in a language that can be understood by all.

Keynote Speaker Lewis Maltby, JD: Mr. Matlby, President of the National Workrights Institute highlighted the implications for Huntington's Disease families and clinicians alike on the Genetic Information Nondiscrimination (GINA) Act of 2008 which goes into effect May 21, 2009 (for health insurance protection), and November 21, 2009 for employment protection). Thirty-four individual state Genetic Discrimination laws exist that will have precedence over the GINA Act if they provide greater protection. GINA will supercede those state laws that offer lesser protection. He cautioned that even with the added protection provided in the GINA Act, an inherent disclosure still exists. What information people willingly provide is still considered 'fair game'.

He further stressed that GINA covers medical insurance and employment discrimination only for those without symptoms before diagnosis of Huntington's or other genetic disease. It does not cover these issues after diagnosis is made. Nor does it apply to long-term, life or disability insurance.

Lifestyle Activity and Age of Onset in Huntington's: Researchers from Australia reported on a study of age of symptom onset in 157 patients. They found that age of onset occurred earlier than anticipated by CAG counts in individuals who had more passive, or less active (leisure and work) lifestyles. They further found that lifestyle activity during adolescent years had greatest impact. This study may show that -- as in mouse studies - increased activity levels appear to benefit people; or it could also mean that that other (unidentified) factors cause earlier onset and decreased activity level.

Dimebon Phase 2 Update: This 3-month study was conducted by HSG investigators in 81 HD patients with mild to moderate HD in order to examine the safety of Dimebon, an experimental drug that has shown encouraging signs of improving cognition in patients with Alzheimer disease. Dimebon treatment compared with placebo was found to be safe and well tolerated in the HD patients and was associated with a 1-point improvement in the Mini Mental Status Examination (MMSE) scale, but not in other measures of intellectual functioning.

More studies in HD are being planned. Medivation Inc. and Pfizer Inc., the pharmaceutical companies sponsoring this Dimebon trial, have indicated their intention to include HD in their ongoing clinical development of this experimental drug.

FDA Keynote Speaker Russell Katz: Dr. Russell Katz, Director of Neurology Division at the FDA gave the closing address. His presence at this meeting is very significant and shows that the FDA is working closely with researchers and drug developers to move effective drugs as promptly as possible to the Huntington's community. His message stressed 2 separate but related points.

Drug Approval in Manifest HD: A new drug must show benefit at levels that "make a difference" to patients. This means that a new drug must show that the life of the patient is improved in a meaningful way, and that improvement in test measures -- by themselves -- will not be sufficient. This also means that -- at present -- drug approvals will be considered only for HD indications when change can be measured.

Drug Approval in Pre-Manifest HD: Because at present, there is no accepted tool for measuring disease progression before the disease begins, biomarkers must be used for study in premanifest HD individuals. Biomarkers are tests (in blood, spinal fluid or brain x-rays etc) that tracks consistantly with disease progression. At present such a marker, or combination of markers have not been validated, or proved that they can track with disease progression.

This means that the FDA will not accept use of biomarkers in premanifest HD until they are proved to be valid in symptomatic HD drug trials. He wisely encouraged that all Huntington clinical trials have mechanisms in place for saving samples that could be used for later biomarker validation study.

Other Event Highlights

In addition to symposium speakers, there were a variety of educational sessions, working groups, clinical trial discussions during the earlier meetings, and a poster session at the symposium presenting new research on many different topics. An educational session on prescribing Xenazine (tetrabenazine) has been reviewed separately. On a general level, there was evidence of impressive collaborative efforts across the international Huntington research and community organizations.

Working Groups: Much effort has focused on defining methods and scales that can be used to measure Huntington symptom response and discussion for anticipated clinical trials.

Poster Presentations: Twenty-four posters provided reports on new unpublished research.

Expert Preferences for Symptom Treatment: The authors surveyed expert on their preferred treatments for depression, anxiety, irritability, agitation, insomnia, apathy,

chorea and rigidity. It is hoped that the findings can serve as a step towards creation of expert consensus statements to formulate appropriate care standards for Huntington's patients.

Effects of Assistive Devices on Gait Stability: The authors found that use of a 4-wheeled walker resulted in fewer stumbles and greater maneuverability than a standard walker. They also found that a variety of canes were not helpful.

FuRST-pHD and IRT: There were several reports from the Functional Rating Scale Task Force for pre-Huntington Disease (FuRST-pHD). This is a new multinational work group -- supported by CHDI -- to develop tools for a new functional rating scale that can be used for clinical trials in pre-Huntington and very early symptomatic Huntington individuals. Item response theory (IRT) a type of sophisticated data analysis procedure being used to study PREDICT-HD. The goal is to define measures more sensitive to change than the UHDRS.

Neuroimaging: There was one symposium presentation and 4 posters regarding use of various neuroimaging techniques as biomarkers to measure disease progression. It is likely that no single technique is sensitive enough to be used alone.

Clinical Trial Reports: Clinical trial working sessions were limited to investigators only, but Dr. Ira Shoulson has told us that in addition DIMOND (Dimebon) results from the Domino (minocycline) clinical trials will soon be communicated. Trial participants will receive information prior to more broad communication to the entire community.

Atomoxetine: This drug, also known as Strattera was tested in 20 individuals. According to the poster presentation there were only small self-reported improvements in attention and psychiatric ratings in those that used the drug, but similar benefit occurred (to a lesser extent) in the placebo treated group. Unfortunately there were frequent adverse effects that included dizziness, trouble sleeping and loss of appetite in the drug treated group. Though this drug may benefit individuals, this is not a benign drug for off-label use in Huntington's, and if used should be followed carefully.

HSG Clinical Trials In (or soon to be) Process: Investigator meetings occurred on trials presently -- or soon to be -- enrolling participants: Those presently enrolling: Coenzyme Q-10 (2CARE), and ACR-16 (HART) trials, and those in planning stages: Creatine (CREST-E) and Coenzyme Q-10 for premanifest HD (PreQuel).

Editors' Comments: Events from this meeting included the announcement of the availability of Xenazine, the first drug approved drug for Huntington's, and the planning for new and better clinical trials for both neuroprotection and symptom treatment. Attendees came away from these meetings with optimism for more and better things to come in 2009, and a continued push toward greater collaboration in efforts here and abroad.

This HSG research meeting comes on the heels of the European Huntington Disease

Network (EHDN) annual meetings in early September 2008. Upcoming Huntington meetings in 2009 include CHDI's annual meeting in April (to be held in Europe), and the World Congress Meeting in Vancouver, Canada in September, and -- back to full circle -- the 3rd Annual Huntington's Disease Research Symposium November 2009 (location to be announced).

Isn't it really good news that so much work is happening in Huntington's that we need several different meetings to present it all?