“Team HD” gathers at HDSA

By Sean Thompson
HIND-Sight Editor

If the over 1,000 attendees at the 2011 Huntington's Disease Society of America National Convention constituted a team of HD advocates, caregivers, fundraisers and family members, then Bryan Viau was their coach.

Giving the convention’s keynote address on June 25 in Bloomington, Minn., the HDSA Minnesota Chapter President energized the crowd much like a coach leading his team into the big game. Everyone has a role to play in “Team HD,” as Viau calls it, and it is up to each team member to choose where they fit in.

“By being here, you have already empowered yourself,” Viau said. “You have a chance to spread the word and be an advocate. You can make a difference every day.”

There are a number of things people can do to get involved and be a part of Team HD, Viau said. People can get involved in research as a participant. They can educate their children about HD. And they can honor the memory of their loved ones by moving on and enjoying life to the fullest, said Viau, who lost his wife Debbie to HD this spring.

“It could be said that Debbie beat HD by the way she lived her life,” Viau said, referring to her work as an HD advocate and her courage to face HD head on.

If the convention is any indication, Team HD is growing in size each year. The record attendance was bolstered by several scholarships and grants made available by HDSA state chapters and other sources, including the UI HDSA COE. About one-quarter of attendees received financial assistance to cover part or all of the cost of attending.

The UI HDSA COE had 14 staff members in attendance to talk with attendees about the PREDICT-HD study. Several staff members also presented informational sessions or led support groups.

Convention attendees took part in sessions covering a wide range of topics, including eating healthy for the HD family, juvenile Huntington disease and explaining HD to the community at large.

During a session on potential new therapies for HD, Martin Goulet, Ph.D., from drug company Alnylam and Bill Kaemmerer, Ph.D., from medical technologies company Medtronic, discussed “silencing” Huntington proteins. Researchers would do this by delivering a drug containing silencing RNA (siRNA) directly to the brain through a small pump that would be surgically implanted underneath the skin. The drug would be pumped through a catheter that would run under the skin all the way to the brain. The therapy is still in the initial phases of development and would need to be tested for safety and effectiveness in humans. But it has shown to reduce Huntington protein in animals.

The convention also included a Team Hope walk, a silent auction and talent show presented by the HDSA’s National Youth Alliance, and culminated with the awards dinner and dancing.

Given the popularity of the convention in recent years, attendance is only likely to climb once again next year when it is held at one of the top vacation destinations in the U.S.: Las Vegas.

Stephen Cross and Nancy Downing contributed to this article.
Leserman receives national HDSA award

As the national awards were being announced June 25 at the 2011 HDSA National Convention, UI HDSA COE Coordinator and Social Worker Anne Leserman watched and listened, while thinking ahead to the candle-lighting ceremony she was going to take part in after the awards announcements.

Then, unexpectedly, she heard her own name announced as the winner of the 2011 HDSA Patient and Family Service Award winner.

“I was thrilled and totally surprised to win this award,” Leserman said.

In announcing the award, HDSA Board Chairman Don Barr mentioned the many roles Leserman fills at the UI HDSA COE, including support group leader, clinic coordinator, educator and advocate.

The HDSA Iowa Chapter nominated Leserman for the award. In its nominating letter, chapter leadership referred to Leserman as the face of the UI HDSA COE, someone who goes above and beyond her job requirements.

“Anne is a very compassionate, empathetic and understanding social worker who has put her heart and soul into serving those suffering from the effects of Huntington disease,” said HDSA Iowa Chapter Board Member Carrie Tibben of Audubon.

UI HDSA COE Co-director Jane Paulsen said Leserman is not only the go-to person for HD-related questions in Iowa, but also a leader nationally among social workers in the HD community.

“Anne’s amazing level of caring and compassion for our patients and all families affected by Huntington disease are what make her an outstanding social worker,” Paulsen said.

HDSA Iowa Chapter Board Member Tammy Harmon said she was grateful Leserman was making a difference for her and so many other HD families.

“Anne was a tremendous source of strength, knowledge and assistance for me and my family over many years,” Harmon said. “She personally holds a very special place in my heart and always will.”

As the coordinator and social worker for the UI HDSA COE since 2003, Leserman has been a fixture in the lives of Iowa’s HD families. She says those families deserve much of the credit for making her the HD social worker she is today.

“I thank the HDSA Iowa Chapter members for their kind words about me,” Leserman said. “I value the relationships I have developed with the Iowa families, and appreciate all the things they have taught me about HD.”

From the editor

It was great to see folks from Iowa and around the country at the national convention in June! And convenient to have it so close. I’m always up for a road trip!

As is always the case, feel free to contact me with feedback at sean-thompson@uiowa.edu or (319) 384-4094. Also, if you want to write something for HIND-Sight, please contact me with your ideas!

Sean Thompson, HIND-Sight editor
Study transition underway

Following an announcement in June, the COHORT study is transitioning to become a new global HD study. Study funders CHDI Foundation, Inc., announced the decision to conclude COHORT. The study is being combined with its European counterpart REGISTRY into a worldwide study called Enroll-HD. A short interruption between studies was deemed necessary in order to concentrate on startup activities for Enroll-HD, said UI HDSA COE COHORT Coordinator Michelle Harreld.

“The transition between the two studies was not totally seamless,” Harreld said, “but progress continues toward making sure Enroll-HD is up and running by the end of the year.” Study designers are describing Enroll-HD as the next phase of COHORT. The overall goal of Enroll-HD is to accelerate the development of treatments for HD by compiling data and biological samples collected from participants at annual visits.

Researchers say the information collected could lead to a better understanding of the natural history of HD. Study designers also want to make this data available for any HD researcher worldwide to use in designing their own studies or testing hypotheses. Sub studies that could be part of Enroll-HD will also be facilitated.

Additionally, researchers say Enroll-HD can serve to speed up recruitment of participants for clinical trials of HD treatments in the coming years. They say Enroll-HD participants could be notified of a clinical trial they are eligible for based on the qualifications required by the trial.

By combining COHORT and REGISTRY, Enroll-HD will build on the many successes of the two studies. The proposed plan is to roll the previously collected COHORT data into the new database, Harreld said. The database of information will be much larger than the COHORT database, given that Enroll-HD is a global study.

The goal is to have as many COHORT sites as possible transitioned to Enroll-HD by the end of 2011, Harreld said. COHORT participants will be contacted with further information when the UI HDSA COE is prepared to transition to Enroll-HD. Most participants will notice little difference between COHORT and Enroll-HD study visits.

“Thank you for all your continued commitment to HD research,” Harreld said. “We really appreciate your patience and understanding through this transition and hope to see you back soon for your first Enroll-HD visit.”

Assistance sought compiling services for HD directory

The UI HDSA COE is attempting to compile a list of helpful services for HD families across Iowa, and we need your help! The categories of services we’d like to feature in the directory include: doctors and other health care providers, nursing homes, in-home services, local mental health providers, transportation and legal services. But you don’t need to limit yourself to these categories. We’re looking for any services you’ve found to be helpful in your day-to-day lives as families living with HD.

Please send entries with as much contact information for the service as you have to anne-leserman@uiowa.edu. Thank you for your help!
Drug makers reviving pharmaceutical industry


The pharmaceutical industry is showing signs it is coming back to life.

Credit a revamped research approach by the industry, which, after years of focusing on me-too drugs for ills that were already well treated, is pouring firepower into diseases that aren’t.

Companies have won marketing approval so far this year for 20 innovative drugs that work differently or better than existing drugs, or tackle ailments lacking good treatments, according to the Food and Drug Administration. “New molecular entities,” the FDA calls them. There were just 21 such approvals all last year.

Recently approved are the first therapy shown to extend life for people with advanced melanoma, the deadly skin cancer; the first new treatment for lupus in over 50 years; and two drugs for hepatitis C that are far more effective than current care.

“We’re seeing a lot of innovation, much more than in recent memory,” said Janet Woodcock, director of the FDA’s drug division, calling today’s laboratory output a “turning point” in drug development.

“If you’re a patient with a terrible disease, a serious cancer or something like that, I think you ought to take heart from what we are seeing,” Woodcock said.

That would include Sharon Belvin, who was diagnosed at age 22 with melanoma that had spread, but who, in a 2005 clinical trial, received the melanoma drug that has just been approved. Belvin is now a 29-year-old mother of two with no sign of the disease.

“It is not just the risky, nimble biotechs that are developing these novel agents,” said Belvin’s doctor, Jedd Wolchok of Memorial Sloan-Kettering Cancer Center in New York. “It is the large pharma companies that are making substantial commitments to a field that was considered very speculative until recently.”

Results can’t come soon enough for these companies, as they are losing patent protection on many big sellers. Companies are looking at a cumulative loss of more than $100 billion in revenue through 2015, by some estimates, as these drugs face competition from low-priced generics.

More than 20 innovative drugs with the potential for annual sales of $1 billion or more each have strong odds of winning FDA approval over the next three years, according to a Credit Suisse analyst, Catherine Arnold.

Says Pfizer’s Mikael Dolsten: “We’re coming back to a period where companies are starting to grow and have a reasonable flow in their pipelines again.”