

# Highlights from the US Hereditary Angioedema Association's (HAEA) 2009 National Patient Conference

Nearly 500 members of the HAE community streamed into beautiful Orlando, Florida to attend the 2009 National Patient Conference, and celebrate the HAEA's 10<sup>th</sup> Anniversary. Below are highlights from the conference:

## **Opening Remarks by HAEA President Anthony Castaldo**

Tony opened up the conference by emphasizing that the availability of C1 inhibitor concentrate—the worldwide gold standard HAE treatment—provides an opportunity for every HAE patient to lead a normal life. He went on to say, “HAE-related suffering, disability, and death from throat swelling will become a relic of the past if we continue to unite as a patient community. “

Tony also said, “The HAE Association is steadfast in demanding that physicians and insurance companies recognize that HAE patients have a fundamental right to choose a treatment program that best fits their individual needs. We now have a variety of products from which to choose, and we are optimistic that additional HAE treatments are going to be approved. The HAEA's Patient Services group is dedicated to continuing and expanding its efforts to provide each and every HAE friend and their physicians with all of the information necessary to establish a treatment program to control and hopefully eliminate HAE attacks.”

In closing his remarks, Tony reiterated that the HAEA's philosophy on our 10<sup>th</sup> Anniversary is identical to that articulated on our 1<sup>st</sup>: The HAEA is a patient services and research organization run by patients. He went on to say, “Our day-to-day interactions with HAE friends provides a chance for us to demonstrate the HAEA's “culture” of caring and kindness, and absolute devotion to doing whatever humanly possible to help every HAE friend lead a normal life. Tony then invited everyone to enjoy the weekend and feel the love!

## **The Conference Keynote Presentation "*HAE – A Quickly Changing Landscape*," was delivered by Dr. Bruce Zuraw, Professor of Medicine in Residence at the University of California, San Diego School of Medicine.**

The Conference Keynote Presentation "*HAE – A Quickly Changing Landscape*," was delivered by Dr. Bruce Zuraw, Professor of Medicine in Residence at the University of California, San Diego School of Medicine; and Chairman of the HAE Association's Medical Advisory Board

Professor Zuraw provided conference attendees with a fascinating history of HAE from its first mention in the medical literature by Dr. William Osler in 1888 to today where five different companies are heavily involved in either producing or researching HAE treatments. The presentation also provided patients with an excellent overview of what

is happening in our bodies when we have an HAE attack and some preliminary findings from two studies conducted by Professor Zuraw and the HAEA: 1. the safety and effectiveness of home infusion with C1 inhibitor concentrate and 2. HAE patient views on the safety and effectiveness of anabolic steroids (731 patients participated). In a nutshell, Professor Zuraw noted that the studies provide compelling data showing that (1) home infusion with C1 inhibitor is safe, effective, and leads to a better quality of life, and (2) anabolic steroids are effective, but many patients are looking to discontinue the therapy because androgen side effects outweigh the drug's benefits, and non-steroidal therapies are now available.

Professor Zuraw established an important goal for the future—ensuring that HAE becomes an incidental issue instead of the dominant issue in the lives of affected patients. This will be accomplished, according to the Professor, by an HAE Association led effort to improve diagnosis and patient access to knowledgeable physicians. Professor Zuraw ended his talk by discussing the importance of the HAE Association's Scientific Registry. The Professor said that the quickest path to curing HAE is getting each and every HAE friend to join and continue participating in the HAEA Registry.

### **Representatives from each of the five drug companies provided updates on the status of their US clinical programs.**

**ViroPharma** ViroPharma's C1 inhibitor (Cinryze) was approved last year by the FDA as an attack prevention therapy. ViroPharma has put together an infrastructure, Cinryze Solutions, to make their product available to every HAE friend that could benefit from the medicine. The HAE Association is ready to (1) provide you with a direct referral into the Cinryze Solutions program, and (2) act as your advocate and confidant and help you work with your doctor, the specialty pharmacy who will ship your medicine, drug company representatives, and insurance providers. Please contact the HAEA's Patient Services group for more information-- Donna Davis [donna-davis@haea.org](mailto:donna-davis@haea.org) or Michelle Williamson [michellewilliamson@haea.org](mailto:michellewilliamson@haea.org).

**CSL Behring (Berinert brand of C1 inhibitor)** has received FDA approval for their C1 inhibitor infusion for treating acute HAE attacks (attacks that have begun) on October 12, 2009. CSL has put together an infrastructure, B.E.N., to make their product available to every HAE friend that could benefit from the medicine. The HAE Association is ready to (1) provide you with a direct referral into the B.E.N. program, and (2) act as your advocate and confidant and help you work with your doctor, the specialty pharmacy who will ship your medicine, drug company representatives, and insurance providers. Please contact the HAEA's Patient Services group for more information-- Donna Davis [donna-davis@haea.org](mailto:donna-davis@haea.org) or Michelle Williamson [michellewilliamson@haea.org](mailto:michellewilliamson@haea.org) .

**Dyax (Ecallantide)** Ecallantide is a recombinant product that inhibits kallikrein—an enzyme that plays a key role in the biochemical processes that lead to HAE-related

swelling. The company filed for FDA licensure in September 2008 and anticipates a response in early December 2009.

**Shire (Firazyr)** Firazyr is an acute attack therapy that is administered by a subcutaneous injection (under the skin injection similar to how diabetics take insulin). The product has already been approved in Europe, and Shire is currently conducting a clinical trial in the US. If you would like more information about participating in this clinical trial, please contact the HAEA's Patient Services group for more information-- Donna Davis [donna-davis@haea.org](mailto:donna-davis@haea.org) or Michelle Williamson [michellewilliamson@haea.org](mailto:michellewilliamson@haea.org) .

**Pharming NV (recombinant C1 inhibitor)** cited successful clinical trial results for their product, Rhucin-a recombinant human C1 inhibitor protein that is derived from the milk of genetically altered rabbits. Clinical trials have shown that Rhucin is a safe and effective treatment for acute HAE attacks. The company is still in the process of applying for a US license for their product.

**HAE International** Henrik Boysen, from Denmark, is an HAE patient as well as the newly appointed Executive Director of HAE International – an association that serves HAE organizations around the world. HAE President Anthony Castaldo serves as the President of HAEI. During lunch on Saturday, Henrik filled us in on initiatives of the new HAEI which include an updated website that, among other things, will provide patients who travel abroad with information on hospitals and physicians who treat HAE in the country to be visited.

**US HAE Association Scientific Registry**—Vice President Janet Long presented a brief overview of the US HAEA's Scientific Registry. Dr. Zuraw and the HAEA, working together, designed a state-of-the-art registry that will produce high quality "scientific grade" data. The Registry was launched on August 12, 2009 – to promote new research in HAE, to study HAE genetics, and to increase understanding of the disease – ultimately finding a cure! Conference attendees were given the opportunity to join the Scientific Registry right at the Hotel – by the end of the weekend, 153 new members had signed up, had their blood drawn and submitted DNA samples!

[If you are interested in participating in this great project, please go to [www.haea.org](http://www.haea.org) and simply click on the Scientific Registry icon.]

**Physician Question and Answer Panel**—US HAEA Medical Advisor Professor Bruce Zuraw and HAE specialists- Dr. Henry Li from Wheaton, MD, Dr. Marc Riedl from UCLA and Dr. Leonard Bielory from Springfield, NJ fielded a wide variety of questions from the patient community. There is not enough space to post the detailed questions and answers in this summary. However, the HAE Association is happy to field any and all questions from each and every member of our community, and get definitive answers from world class HAE scientists/physicians. HAE friends can contact their Patient Services Representative or submit a question through our website at this link:

<http://www.haea.org/contactus.php>

**The US HAE Association 2009-2010** –Sunday’s patients-only session presented on-going programs and plans that will benefit all HAE patients. The programs discussed included:

- Celebrating all of the accomplishments of the US HAE on its 10 year anniversary.
- Expanding the HAEA's Patient Services- we are growing to ensure we are well positioned to serve as the primary problem solver for HAE friends who are interested in exploring new therapies, or have any HAE-related issues or needs.
- Rolling out and recruiting patients for our HAE Scientific Registry.
- Implementing a robust research infrastructure that includes offering medical research grants aimed at bettering the lives of HAE patients.
- Conducting regional meetings, workshops, and lectures for patients and physicians interested in learning more about HAE.
- Maintaining our 24/7 toll free number –to help patients deal with HAE issues that arise.
- Continuously upgrading our web page to ensure it remains the most authoritative source on the web for HAE-related information.

Conference attendees were separated into groups by region to meet one another as well as touch base with their respective Patient Services Representative. The Sunday session closed with a video featuring our HAE children attendees! This conference was our first to provide a separate program for our HAE children and youth – art, games, a carnival time and a separate movie night while the grownups were entertained by the amazing psychic Robert Channing.

Your US HAE Association continues to be your authoritative source for HAE information and resources. We continue to send out newsletters with updates on the status of all HAE medicines – approved and awaiting approval. The Newsletter will also keep you informed on Association news and events. The best way to be certain that you are always up-to-date on new HAE treatments and everything the US HAEA is doing on your behalf is to make sure that you and all of your affected family members have signed up as HAE Association members [http://www.haea.org/hae\\_form.php](http://www.haea.org/hae_form.php)

Remember its free! So, please, if you have not already done so, fill out the membership form! And keep in mind, we go to great lengths to ensure your personal information is secure and protected. Access to information is strictly limited and tightly controlled. We have never and will never disclose any personally identifiable information to an outside party.

We were glad to see all of our HAE friends who helped us celebrate 10 incredible years of the US HAEA. We look forward to seeing everyone at our next Conference!

*Tony Castaldo, President US HAEA*

*Janet Long, Vice President*

*Donna K. Davis, Director of Patient Services and Clinical Programs*