haea
US Hereditary Angioedema Association

US HAEA TODAY

Fall 2014 Newsletter

A Message from the President

Dear HAEA friends,

After years of planning and effort, we are happy to announce that the US HAEA Angioedema Center at the University of California at San Diego is now officially open and seeing patients. The article below provides more information about our comprehensive care/research facility along with details regarding how to go about setting up an appointment at the Angioedema Center.

Also, please join me in congratulating two of our Angioedema Center physicians -Dr. Bruce Zuraw and Dr. Sandra Christiansen- on their successful climb of Mt. Kilimanjaro, which was dedicated to raising funds for HAE patient care.

The HAEA continues to work with the medical/scientific community and the pharmaceutical industry to encourage new generations of HAE therapy. We are excited that Ruconest -- the fifth new HAE treatment approved since late 2008 -- will soon be available! Ruconest is a recombinant C1 Inhibitor acute attack treatment delivered by intravenous injection.

In This Issue

Our patient community has always recognized that clinical trial participation is the path to improved HAE therapies for ourselves and our children. We encourage HAE friends to call the HAEA's Clinical Trial Coordinator John Williamson at (972) 984-0621 to discuss trial opportunities in your area.

The HAEA is committed to helping our patient community gain access to and reimbursement for HAE therapies. We encourage anyone who is dealing with an insurance denial or overly restrictive limits on treatment to contact the HAEA's Nurse Reimbursement Manager Nikia Davis at (609) 287-9540. Nikia has the experience and skills to help HAEA Friends navigate the complex and often frustrating process of dealing with insurers.

Please enjoy our Autumn 2014 newsletter and do not hesitate to contact the HAEA for any and all of your HAE-related needs.

Warm regards,
Tony Castaldo
President, US HAEA

Capitol Hill Day 2014

The US HAEA Scientific Registry Report - Research for a Cure

Your US HAEA Patient Services Team

US HAEA Angioedema Center at UCSD Is Open

The US HAEA Angioedema Center Officially Opens its Doors!

As you know, the US HAEA Angioedema Center at UC San Diego has been at the heart of the HAE Association's vision - lifelong health for all HAE patients.

Designed to provide a positive experience that features exceptional care by expert HAE physician/ scientists in a friendly and supportive environment, the Center works toward a better future for those who suffer from all types of angioedema.

The Center features:

- comprehensive care for angioedema patients, setting the standard for patient care in the US and around the world
- clinical trials and investigator-initiated research as well as groundbreaking basic and translational research
- educational opportunities to US and international physicians, nurses and other healthcare professionals

Kili Care Climb Success!

HAE patients have had a long and sometimes tough "climb" in our ongoing efforts to receive an accurate diagnosis and to gain access to effective and innovative HAE therapies.

HAE expert physician/scientists Dr. Bruce Zuraw and Dr. Sandra Christiansen have stood by our patient community from our first steps as an Association. They serve on the HAEA Medical Advisory Board were the key drivers in establishing ***(1) the US HAEA Scientific Registry and (2) the US HAEA Angioedema Center at the University of California at San Diego.***

Drs. Zuraw and Christiansen and Mr. Caesar Sweitzer-- a long-time friend and financial advisor to the HAEA Board of Directors -- took on Mt. Kilimanjaro this past August. Mt. Kilimanjaro, at 19,340 feet, is the tallest freestanding mountain in the world!

They made the ascent of Kili for 2 reasons: ***1) to raise HAE awareness and 2) to collect donations for the US HAEA Patient Benevolence Fund, which helps HAE patients gain access to HAE expert medical care.***

We are grateful to the Kili Care Climb team who pushed themselves to their physical limits, ever mindful of the physical limits that HAE can bring - climbing to reach the peak of Kilimanjaro and to help every HAE patient reach the peak of lifelong health.

Ruconest - New FDA-approved HAE Therapy

Salix Pharmaceuticals, Ltd. and Pharming Group NV announced on July 17, 2014 that the Food and Drug Administration (FDA) has approved RUCONEST® (C1 Esterase Inhibitor [Recombinant]) for the treatment of acute angioedema attacks in adult and adolescent patients with Hereditary Angioedema

(HAE).

The US HAEA has always advocated to make as many HAE therapies as possible available to our patient community. Ruconest provides the HAE community with another FDA-approved option.

The press release announcing the approval of Ruconest is available to read at haea.org and the table of angioedema types at http://www.haea.org/patients/angioedema-table/ has been updated to include this important new treatment option.

You can also call your Patient Support Team member to discuss Ruconest.

Clinical Trial Participation-The Path to Better HAE Medicines

Clinical trials show us what works (and what doesn't) in medicine and health care. They are the best way to learn what works best in treating diseases like HAE. Clinical trials are designed to answer 2 important questions:

- **Does the new treatment work in humans?** If it does, doctors are also looking for how well it works. Is it better than what's now being used now to treat HAE? Can we find something with fewer side effects? Or does it work in some people who aren't helped by current treatments? In other words, is it a step forward?
- **Is the new treatment safe?** No medicine is entirely without risk, but do the benefits of the new treatment outweigh the possible risks?

Some of us in our HAE community might say, "we already have medicines!" But not all HAE therapies work for everyone. And for some, one therapy may work better in one type of swelling than in another.
New HAE clinical trials help to refine current therapies, investigate brand new therapies, and, ultimately, find a cure.

There are HAE clinical trials going on right now! If you want to help move forward new HAE therapies, please contact Patient ServicesTeam member John Williamson at john@haea.org

Thanks to everyone who participates in a clinical trial, we will *all* continue to enjoy a better future living with Hereditary Angioedema.

What's new at HAEA.org ?

Have you noticed? The HAEA web site has changed its look!
We are always working to make it easier to find all of the latest information you may need on: HAE (disease symptoms, triggers, etc.), HAE Therapies, Therapy Access & Reimbursement, Tools for Living with HAE, etc.

Be sure to take a special look at the newest additions to the web site.

Research, Advocacy, Compassion, Empowerment

Not Just a Treatment...

A CURE.

You *(yes, you!)* can help us find a cure for HAE.

Learn how »

Living With Hereditary Angioedema

A genetic, inheritable disease, HAE can affect all aspects of your life.

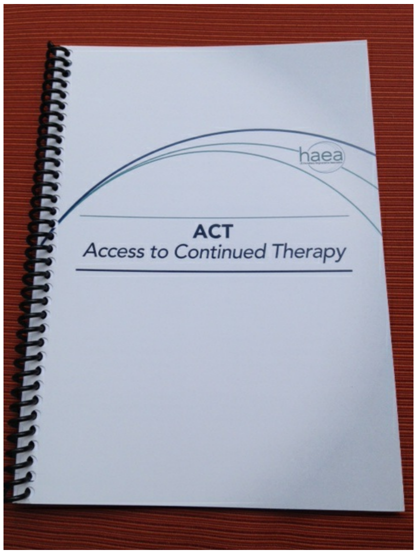
Take time to read through the authoritative

Angioedema Center at UCSD

Designed to provide a positive experience that features exceptional care by expert HAE physician/ scientists in a friendly and

- Become an HAEA member!
- 2015 National Patient Summit
- May 16 is HAE DAY
- Patient Stories

Access to Continued Therapy (ACT) Log



The Access to Continued Therapy (ACT) Log

The HAEA has designed a simple and easy to use personal log for you to use to record your HAE attack information. The 2013 Recommendations (p.463) for the management of HAE published by the US Medical Advisory Board strongly encourages HAE patients keep this type of record.

You can find the ACT in the new Patient Toolbox link or just click here to register: http://www.haea.org/resources/introducing-the-access-support-record-asr/

A soft-bound ACT booklet will be mailed directly to you, once you register. This booklet provides a handy, durable, and concise record of the most pertinent information about your HAE.

A new digital electronic version of ACT (an app for your iPad and iPhone, etc.) will be available soon as well.

Remember, this simple record can be a powerful tool in advocating for your access to HAE therapy.

Do you need help with access and reimbursement?

Do you need help with access and reimbursement ?
Don't forget that the US HAEA Nurse Reimbursement Manager is here to help.

Nikia Davis, RN BSN CCM has joined the HAEA Patient Services Team as our Nurse Reimbursement Manager.

Nikia has extensive reimbursement and product access casework experience conducting patient utilization/medical care access casework in the hospital setting. Nikia brings expertise in professional reimbursement guidance for our HAE patient community (insurance denials/appeals, prior authorizations, and insurance benefits investigations).

If you have any access or reimbursement questions, please contact Nikia at nikiadavis@haea.org

Capitol Hill Day 2014

The HAEA's 2014 Capitol Hill Day held in July proved to be another great success.

HAEA friends from throughout the country met in Washington and visited about 50 congressional offices. One of the "asks" was to have HAE included once again on the coveted list of just 22 rare diseases eligible for government funding for research. Once again, our Hill efforts have brought results - HAE is on the list again!

Our thanks to the HAEA friends who braved a typically hot summer day in Washington for their effective HAE advocacy efforts.

Sen. Jim Costa (CA)

FALL - US HAEA Scientific Registry Report - Research for a Cure

Coming soon from your Scientific Registry:

3rd Quarter Reports and Attack Event Reports!

We want to extend a special welcome to our newest members!

And thanks again to all those who have returned their blood sample donation Kits!

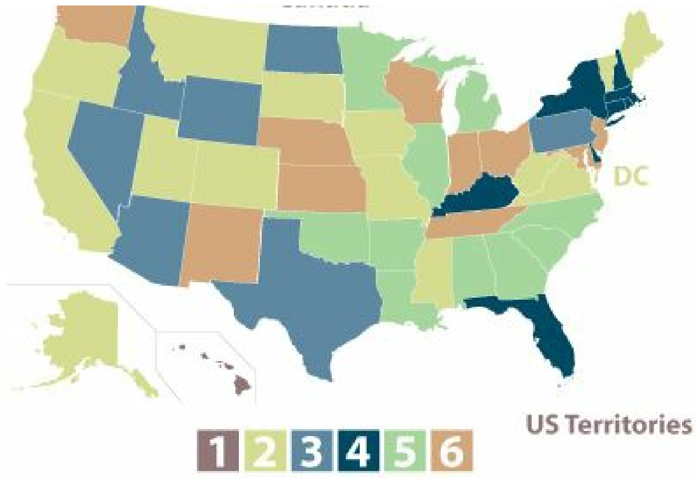
We appreciate everyone's time and effort in making the Scientific Registry a *success!*

You are making a difference!

To contact the HAEA Scientific Registry staff with any questions you may have, please email scientificregistry@haea.org or call the HAEA toll free number at 866-798-5598.

To learn more about the US HAEA Scientific Registry, please visit **http://www.haea.org/get-involved/us-haea-scientific-registry/**

Your Patient Services Team by Region



Your US HAEA Patient Services Team

Find your region and your Team Member at
http://www.haea.org/contact/patient-services-representatives/

The US HAE Association publishes its newsletters to ensure everyone in our HAE family is informed about important clinical developments and all HAE Association activities.
We hope you have enjoyed this issue!

The United States Hereditary Angioedema Association
Seven Waterfront Plaza
500 Ala Moana Blvd., Suite 400
Honolulu, HI   96813

Toll free phone: (866) 798-5598