Leading the Way in Patient Empowerment

US Hereditary Angioedema Association
What is Hereditary Angioedema (HAE) or C1 Inhibitor Deficiency?

- Very rare and potentially life-threatening genetic condition that occurs in about 1 in 50,000 people

- Characterized by episodes of edema (or swelling) in various body parts - including the hands, feet, face and airway

- May include bouts of excruciating abdominal pain, nausea and vomiting caused by swelling in the intestinal wall

Airway swelling is particularly dangerous because it can lead to death by asphyxiation
Who is the US HAE Association (HAEA)?

- Non-profit patient advocacy organization dedicated to serving persons with all types of angioedema
- Founded and staffed by HAE patients and patient caregivers
- Provides HAE patients and their families with a support network and a wide range of services (including individualized patient support)
- Works to increase HAE awareness within medical community and general public
- Committed to advancing and conducting clinical research designed to improve the lives of HAE patients and, ultimately, find a CURE
Areas of Strategic Focus

- Driving Continuous Improvement in HAE Care (with an emphasis on access and reimbursement for HAE therapies)
- Patient Advocacy, Empowerment and Engagement
- Research
Driving Continuous Improvement in Care

Patient Care, Access, Reimbursement and Payer Support
Helping patients understand the path to therapy

• HAEA Nurse Reimbursement Case Manager
  Navigate insurance hurdles, including denials, appeals, quantity limits and insurance selection

HAEA Nurse Case Manager
Difficult cases, physician referrals, Center referrals

• HAEA Patient Advocates
  Understand HAE and empathize with patient daily journey
  Empower patients to become their own best health advocate
Advocacy, Empowerment and Engagement

Patient member tools for advocating for optimal care

- US HAEA Medical Advisory Board Peer-Reviewed Journal Publication: 2013 Recommendations on the Management of HAE Due to C1 Inhibitor Deficiency

- 2015 Patient Declaration of Rights
  Concise outline of optimal approach to HAE management based on 2013 Recommendations

Patient disseminated to local ER, local treating physician, caregivers, family, anyone playing a role in their care
Additional patient engagement opportunities

HAEA Café - online community for HAEA’s patients, families and staff

Café members can:
• chat live with their HAEA Patient Advocate
• participate in webinars on issues such as HAE therapies, Clinical Trials, and advocating for children with HAE
• participate in virtual support groups

Additional resources to foster community engagement:
Facebook closed group, Facebook business page, Twitter and Instagram
Quarterly Newsletter
Web site that provides authoritative and unbiased disease information
US HAEA Angioedema Center at the University of California, San Diego

- Established in 2014 – 1st comprehensive angioedema center in the US
- Comprehensive care for angioedema patients
- Clinical trials and ground-breaking HAE research
- Medical consultations on angioedema cases
- Educational opportunities for physicians, nurses and other healthcare professionals

www.angioedemacenter.com
Research Publications

In conjunction with the Center, MAB and patient contributed data


In-House Research

US HAEA Scientific Registry

Institutional Review Board (IRB) approved in 2009

Two sides: Biorepository (DNA samples)  
Data collection (online questionnaires)

Goals of the Registry:

- Drive novel research in angioedema, including HAE genetics
- Increase disease knowledge
- Ultimately, to find a CURE
Clinical Trials

HAEA energized the patient community to expedite pivotal trials - resulting in patient access to five FDA-approved HAE therapies

- Continuing clinical trial recruitment
- Assistance navigating through process of blind trials
- Helping drive further research by demonstrating an invested patient community
Research - Educational Resources

- Continuing Medical Education (CME) online course on HAE for all healthcare professionals
  2016 - added module on Management of HAE in the ER
  [www.haeedu.com](http://www.haeedu.com)

- Webinars for patients on clinical trials, Scientific Registry, etc.

- Physician and clinical trial referrals from a robust database of knowledgeable HAE physician/researchers

- HAEA Patient Advocates - clinical trial participant identification and distribution of clinical trial information
Research - Legislative Support

Public Policy Advocacy Program

- Capitol Hill Day – 22 patients and family
  Legislative/Regulatory Outreach

- FDA Meeting, DOD Peer Reviewed
  Research Program, Patient Focused
  Drug Development Meeting

- Congressional Letters for NIH support
  of HAE Research
Research – Financial Support

HAE IN-MOTION® 5K

Opportunity for patients to hold a fun, fundraising event in their own community
HAEA provides full support - all logistics
Funds raised for research

Empowers patient community to raise disease awareness among local physicians and the general public

hae day :-)  
- Recognized in the US by a Congressional Resolution in 2012  
- Patients (around the world) participate in HAE awareness events and fundraisers  
- Held annually on May 16

Patient Donations
Looking ahead to 2017

National Patient Summit – 2017
Power in numbers – a united voice

- Expert Physician Panel
- Research updates
- On-site Research

HAE in MOTION 5k
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- Questions?

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