

US HEREDITARY ANGIOEDEMA ASSOCIATION

2019



A N N U A L R E P O R T



PRESIDENT'S MESSAGE

Twenty years ago, a brave group of patients and caregivers united by the unwavering belief that patients suffering from hereditary angioedema deserved a better quality of life established the US HAE Association (HAEA). Since then, we have worked tirelessly to transform this noble belief into tangible results.

Today, we celebrate what the HAEA community has achieved to dramatically improve the lives of HAE patients everywhere. We now boast seven safe and effective medications for the treatment and prevention of hereditary angioedema attacks, which paves the way for many to live a life unhindered by HAE.

As fellow patients and caregivers, our team is proud to lead an amazingly vibrant, 7,000+ strong, HAEA community. Our successes as an organization are a direct reflection of your commitment to achieve a healthier and fuller life. As part of our 20th Anniversary celebration I want to highlight some of our most notable accomplishments:

- o An unprecedented level outreach and problem solving personal communication with our community through patient assistance, physician referrals, webinars, HAE-in Motion events and in person meet gatherings,
- o Record-breaking attendance of 1,000 HAEA friends at our 2019 HAEA National Patient Summit - Imagine the Possibilities,
- o An exciting and productive, first-ever Global Youth Advocacy Program,
- o The release of Nico's Lunchbox, a children's book specifically created to help kids learn to cope and manage HAE,
- o Significant increases in Pam King Scholarship recipients,
- o Our first HAEA Social Media Internship Program affording 13 high school and college students practical experience to succeed in the job market

We are particularly delighted by the great successes of HAEA's Youth Leadership Council in 2019. Through creativity and innovation, these young leaders engaged members through webcasts, podcasts and social media advocacy campaigns, and participation in Capitol Hill Day. Their assertive and committed voices are an inspiration to all of us.

Thanks to our younger members, we know that the future is bright for our HAE community. We are excited about the opportunities that lie ahead and reinvigorated by the amazing support we receive from our patients, caregivers, physician/scientist community, and sponsors.

Warmest regards,



Anthony J. Castaldo
US HAEA President and CEO



ABOUT THE US HAEA

Founded in 2000 and staffed by HAE patients and HAE patient caregivers, the US Hereditary Angioedema Association (HAEA) is a 501 (c)(3) non-profit patient advocacy organization dedicated to serving individuals with Hereditary Angioedema. The US HAEA has grown to a vibrant, goal-advancing organization with a membership of over 7,000.

OUR MISSION

To lead a nationwide advocacy movement to increase HAE awareness and education, empowering patient access to a suitable therapy, and fostering groundbreaking research that includes searching for a cure.

OUR VALUES

Providing the HAE community with authoritative and unbiased information. Offering highly personalized services with kindness and compassion.

OUR ROADMAP TO SUCCESS: THE HAEA PILLARS



HEALTH

The HAEA Health team is committed to helping everyone with HAE attain wellness, health, and happiness. Our goal is to ensure that everyone in our community has the information and support needed to forge a path that starts with an accurate diagnosis and continues through obtaining a therapy that results in wellness.

Because the first step to a healthy future starts with an accurate diagnosis, we developed an extensive database of physicians who can evaluate, diagnose, and treat people suffering from chronic swelling symptoms. Our HAEA Health Team uses the database to find and then refer people to the closest knowledgeable physician.

A tailor-made, personalized treatment plan that fits each individual's specific needs is the next step on the path to wellness. Our Health team is

always available to answer questions and provide support as you implement your treatment plan.

Our HAEA Health Team is also available to help you with any issues insurance-related issues encountered in gaining access to and reimbursement for the HAE medicine(s) you have been prescribed.

The path to wellness would be incomplete without considering the emotional and mental health needs of our community. We offer an array of virtual support groups, where patients can seek understanding, advice and comfort. These safe spaces help our members share their stories while providing valuable support and problem-solving strategies.

HAEA by the Numbers

6,773

HAEA members

557

New HAEA Members

758

Reimbursements & Benefits Assessment Support

1,607

Physician Referrals and Follow-ups

5,121

Patient Outreach

4,494

Patient/Caregivers Survey Responses

2,834

Physician Outreach

375

Support Group Participants

1,715

Responses to Inquiries from Patients and Caregivers



ADVOCACY



The HAEA is proud to lead a nationwide patient advocacy movement to protect the rights of HAE patients. Our advocacy efforts focus on the HAE Youth community, legislative and regulatory matters that might affect those with HAE, and activities designed to maintain and expand our access to modern HAE medicines.

We put considerable emphasis on our HAE Youth because they are the advocates of the future and it is vitally important to offer opportunities for them to further develop communications and leadership skills. In 2019, we built a diverse and dynamic Youth Advocacy Program and invested in tools that included Action Advocacy Workshops, webinars and created a Youth Advocacy Toolkit.

CAPITOL HILL DAY 2019

More than 90 HAEA volunteers including patients, caregivers, and staff descended on Capitol Hill and visited 87 congressional offices. During each visit, members of our community urged legislators to ensure any future health care legislation maintains the current ban on lifetime maximums and continues protecting individuals with pre-existing conditions. Additionally, the HAEA advocates also called for a solution to a regulatory loophole that allows insurance companies to deny payments from charities that provide co-pay and premium assistance.



UNITED FOR CHARITABLE ASSISTANCE

The concern over the regulatory loophole cited above combined with other governmental actions that pose a challenge to charitable assistance, prompted the HAEA to form and lead a coalition of rare and chronic disease organizations that are working hard to ensure patients and families living with chronic condition can continue receiving charitable assistance that enables access to critically needed therapies.

On July 10th, HAEA CEO Tony Castaldo, Senior Executive VP Michelle Cuevas, and HAEA Patient Advocate John Williamson were on hand in Washington, DC to officiate the United for Charitable Assistance Coalition in honoring Chronic Disease Day. The event helps bring awareness on behalf of patients with chronic illnesses while promoting healthy practices and offering support to patients.



As part of the Chronic Disease Day festivities, Mr. Castaldo hosted a congressional luncheon and introduced rare disease champion South Dakota Senator Kevin Cramer, who spoke about the critical role of charitable assistance for patients with rare and chronic diseases."

HEALTH ECONOMIC RESEARCH ON THE VALUE OF HAE THERAPIES

The HAEA unveiled the results of groundbreaking research that quantifies the value of HAE medicines from a health economic perspective during a poster session at the annual meeting of the Academy of Asthma, Allergy and Immunology.

The poster titled: A Comprehensive Approach to Assessing the Value of Prophylactic Therapy for the Rare Disease Hereditary Angioedema Using Real-World Patient Data, includes data provided by members of the US HAE Association who completed an anonymous online survey designed to capture the HAE patient experience with respect to: education, employment, attack frequency, treatments, comorbidities, caregiver economic costs, and actual billed costs for attack-related hospitalizations, physician office visits, and emergency room admissions.

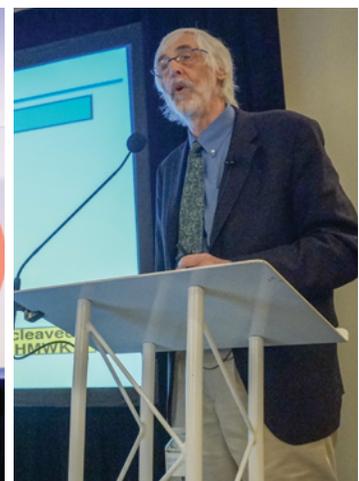
The study highlighted the tremendous value of modern HAE medicines in terms of improving patients' quality of life by curbing the number of ER visits, hospital stays, missed workdays, and loss of social and educational opportunities.

This innovative work plays an important part in our advocacy efforts for continued access and insurance coverage for HAE medicines.

US PAYER ADVISORY BOARD SYMPOSIUM

In May, the HAEA organized a two-day educational symposium for health insurers that took place in Chicago, Illinois. The symposium is part of HAEA's ongoing advocacy efforts to ensure that every patient gets access to, and reimbursement for, HAE medicines. The reach of this event proved significant the participating insurance companies account for over 254 million insured lives in the United States. During the Symposium, we provided attendees with a comprehensive overview of HAE while emphasizing the horrifying symptoms and psychological burden experienced by HAE patients when the condition is inadequately treated.

The initial part of the meeting focused on educating insurers about the indications and uses for each of the FDA approved HAE medicines. During the sessions that followed, we provided a data centered analysis to show the value of HAE therapies using the results of the previously mentioned Health Economic Research that showed how modern HAE medicines yield (1) dramatic increases in patient quality of life, and tangible economic benefit by (1) reducing the need for intensive medical treatment such as ER visits and hospitalizations, and (2) allowing patients to get off disability, pursue educational opportunities, and maintain employment. Afterwards, attendees shared valuable insight on the insurance industry processes for approving or denying access to therapies.







ENGAGEMENT

The HAEA Engagement team is dedicated to providing our community with information and events that promote a healthy and happy life unhampered by HAE. The team also leads a powerful multifaceted Youth Program that includes training for future HAEA advocates.

2019 HAEA NATIONAL PATIENT SUMMIT - IMAGINE THE POSSIBILITIES

Over 1,000 physicians, patients, caregivers, medical professionals, scientists, and HAEA friends traveled to Atlanta, Georgia for the 20th Anniversary National Patient Summit – Imagine the Possibilities. The event featured content designed to keep the community up to date on available and emerging therapies, trends in access to and reimbursement of HAE medicines, the latest scientific advances, and the status of clinical trials.

The event's keynote speaker, former U.S. Representative Henry Waxman, shared his personal and professional experiences with the power of advocacy and engagement following his years of service in Congress working to pass the Orphan Drug Act.

The Summit served as the venue for a Global Youth Advocacy Leadership Program with over 171 participants (ages 12-25) from 20 countries. The program included opportunities for our HAE Youth to learn about HAE advocacy from some of the world's leading HAE advocates and translate that knowledge in action plans for making positive and lasting changes within their home countries. As part of the program's media training component, our future HAE advocates participated in a storytelling workshop, and made on camera presentations. Participants earned an HAEA/HAEi Advocacy certification after the completion of the program and learned valuable skills in advocacy, storytelling, and engagement.



2 0 1 9 H A E A
NATIONAL PATIENT SUMMIT



Notably, the Summit also featured an inaugural Professional-Scientific Program geared toward providing medical professionals with the latest thinking regarding HAE clinical care and research. Topics addressed during the program included:

- o HAE in Children and Pregnant/Lactating Women
- o Novel Therapies: Shift Toward Greater Use of Prophylaxis?
- o A Clinical Approach to Patients with HAE with Normal C1-Inhibitor, and
- o A panel on HAE Registries

HAEA YOUTH LEADERSHIP COUNCIL

The HAEA's longstanding commitment to Investing in our HAEA youth's leadership skills was on display throughout 2019. The Youth Leadership Council (YLC) is a peer-to-peer support group for those diagnosed with HAE that cultivates HAE youth leaders ages 12-25 and fosters member engagement.

Throughout the year, YLC members demonstrated innovation and creativity by designing special youth targeted events and delivering them on a variety of platforms. YLC members held monthly meetings to engage youth in program development ideas, social media strategies, and idea generation. Their formidable accomplishments include:

YOUTH EDITION WEBINARS

- o All About the Brady Club
- o HAE and Anxiety
- o What to expect at the 2019 Summit
- o Youth Advocacy Month

HAEA YOUTH INSTAGRAM PAGE

- o Over 540 followers in 8 months

YLC BEYOND HAE PODCAST

- o Youth-produced, monthly episodes to share unique stories about living with HAE
- o Over 520 podcast plays since November (from 19 countries and the US)

#BEYONDHAE YOUTH ADVOCACY MONTH CAMPAIGN

- o 75 youth participated by sharing their #BeyondHAE images on social media
- o Generated over 28,480 social media engagements

HAEA SOCIAL MEDIA INTERNSHIP PROGRAM

- o Participation of 13 high school and college interns for 5 months for each session

CINCINNATI REDS PARADE AND ADVOCACY TRAINING

- o Members of the YLC and their families participated in a three-day packed agenda, that included awareness visits to local fire stations and the University of Cincinnati Hospital.

CAPITOL HILL DAY 2019

- o Participation of 33 youth under the age of 26 as HAE advocates before Congress

BEYONDHAE CAMPAIGN FOR YOUTH ADVOCACY MONTH

To celebrate Youth Advocacy Month, the HAEA launched an interactive social media campaign named #BeyondHAE. Over 150 of our HAEA youth participated in the event, sharing their pictures and stories on social media doing using the #BeyondHAE sticker and hashtag.

BRADY CLUB

The Brady Club is an online, safe space just for children, ages 4-12 diagnosed with HAE and their siblings. The Brady Club allows us to engage with kids, answer their questions, give them a place to share their experiences, so they learn to better understand and cope with HAE.

In addition to the online interactive space, in 2019 we launched the Brady Club app, available for Android and iPhones, making it more accessible for kids. We also introduced a brand-new activity book that Brady Club members receive on a quarterly basis by mail. Moreover, we developed Brady Club merchandise, including very popular t-shirts, for in-person events.



“ Parker is so happy! He’s got a really bad virus and was worried he was going to swell. This has been very hard on him. He’s missed like 25 days of school and couldn’t play on his basketball team this season. I’m crying right now, thank you so much for your kindness and making him smile.

- Parker’s Mom ”



The Brady Club was also featured during our HAEA National Patient Summit! Our youngest HAEA members formed friendships with other kids, allowing them to share their experiences and forge support systems to manage HAE as they grow together.

During the year we introduced something very special for our Brady Club members. Kids under eight who experience their first swelling receive a HAEA “First Attack Support Box” to help them cope with the new symptoms.

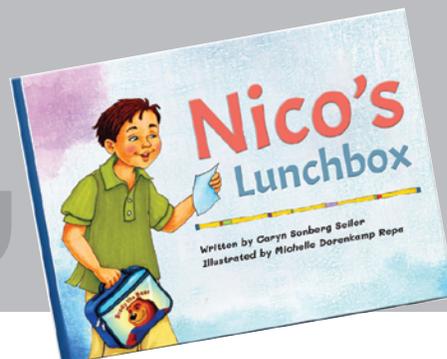
As a result of these innovations, last year we doubled our Brady Club membership.

NICO’S LUNCHBOX

The HAEA teamed up with children’s author Caryn Sonberg Seiler to develop a three-book series to help kids learn about HAE in a relatable and interesting way.

In October 2019, we released Nico’s Lunchbox, the first of this three-book series. Available through haea.org and Amazon.com, Nico’s Lunchbox has become a valuable resource for HAE families with small children. More importantly, Nico has become a relatable friend for kids seeking understanding and comfort. To date over 500 books have been distributed through HAEA programs and online orders.

“ While I was reading it, I cried because I truly empathize with Nico. Anyone with HAE has felt all alone with this disease and just wished someone else would understand.





FAMILIES4HAE

In 2019 we invited community members and their networks to support the HAEA Youth Programs through the #Families4HAE Campaign. Community members enthusiastically donated to the next generation of HAE advocates. The campaign generated over 150 donations. These funds will enable us to expand our HAEA Youth Advocacy offerings which give kids and young adults the tools they need to turn their stories into action.

MEET & GREET PROGRAM

Last year, we launched a series of nationwide Meet & Greet events so we could connect with HAEA friends in their hometown communities, better understand their needs, and let everyone know about our programs and support services. In 2019, we hosted 11 Meet & Greet events. Stay tuned for Meet & Greet events in 2020!

PAM KING HAEA SCHOLARSHIP PROGRAM

Named in honor of former HAEA Chief Operating Officer Pam King, this scholarship program offers financial support for HAE patients seeking to improve their lives through academic achievement. In 2019, we simplified the application process and streamlined the administration's program in order to facilitate access. In 2019, 74 college students received scholarships to help them achieve their lifelong educational goals while celebrating the Pam King legacy.

85

Scholarships to

57

students awarded
in 2019

\$2,500

Annual Award
per Recipient

50%

Increase In
Applicant
Submissions
Over 2018



HAE DAY :-)

On May 16th, we joined the HAE community from around the world to commemorate hae day :-)

As part of the hae day :-) awareness efforts, we launched new HAE ER Tool Kits to assist first responders, medical personnel and emergency facilities in diagnosing and responding to HAE attacks. The HAE ER Tool Kit explains HAE, provides an overview of current HAE therapies, and printed HAE resources that include:

- o Patient Guide to HAE – a pocket guide to better understand the disease state, symptoms, attack triggers, diagnosis, treatment options, and more.
- o HAE Poster – a perfect resource to bring to your local urgent care
- o ER HAE Infographic – easy to read HAE disease facts that you can share with your peers
- o US HAEA Angioedema Center Card – details the many ways the Center can partner with your own HAE physician
- o HAEA CME Card – 3 free online courses available to your HAE healthcare provider

We also celebrated our first Virtual Walk/Run, where HAE patients and family members registered an amazing 6,285,500 steps to raise awareness for HAEA.

HAE IN-MOTION® 5K

In 2019 the HAEA hosted 7 HAE IN-MOTION® 5K run/walk events nationwide. These unique events offer HAE members an opportunity to raise awareness on HAE while raising funds for important life-saving research.

SOCIAL MEDIA OUTREACH

The HAEA Engagement team successfully managed a strong, dynamic, and robust online presence to connect with our members. In 2019, we were thrilled to work with our first-ever interns, who were tasked with building an HAEA Instagram page to attract and improve communications with our younger HAE members. The 5-month program enabled us to gain valuable insights on online youth preferences and interests so we may better communicate with them. We are grateful for the knowledge gained from the creative interns we hosted, while providing them with practical work experience and training that expand their opportunities to prosper.

50%

Increase in Total Number of Engagements on Twitter, Facebook, Instagram, and LinkedIn.

42%

Increase in Total Facebook Impressions

4,255

Followers of HAEA Fan page

13

HAEA Social Media Interns

EDUCATIONAL WEBINARS

Following the successful launch of our webinar program in 2018, we began 2019 with an aggressive agenda of topics aimed at reaching more members and better targeting their needs. We were able to offer 17 webinars reaching almost 800 people, including many topics geared towards young patients.

2019 HAEA WEBINAR SERIES

- o All About the Brady Club
- o HAE Health & Advocacy in 2019
- o Discover First-of-their-Kind HAE Treatment Options
- o Understanding Anxiety and HAE
- o Women and HAE
- o Advocating for Yourself
- o HAE, Wellness and You
- o Managing HAE in your Family
- o All About the 2019 Patient Summit Youth Program
- o Navigating School with HAE
- o 2020 Open Enrollment
- o Community Members Share their Experience with the Only C1-INH Subcutaneous Therapy
- o Advocacy Month #BeyondHAE
- o 2019 Capitol Hill Day
- o The Importance of Having the Right On-Demand Treatment"
- o HAE, the Holidays and You
- o The Importance of Clinical Trials – Leading the Way to HAE Treatments

ADVANCES IN

RESEARCH

The US HAEA is more committed than ever to the fundamental mission that has always guided us -- helping every HAE patient lead a life that is unhindered by HAE. We are proud to collaborate with medical and pharmaceutical industries as part of our continued efforts to (1) promote clinical research aimed at upgrading HAE care, and (2) support scientific inquiries into the causes of HAE in a quest to find new therapeutic targets.

VALIDATION OF POTENTIALLY GROUNDBREAKING BLOOD TEST DISCOVERED BY PHYSICIAN/SCIENTISTS AT THE US HAEA ANGIOEDEMA CENTER AT UNIVERSITY OF CALIFORNIA AT SAN DIEGO (UCSD)

The US HAEA Angioedema Center at UCSD (Angioedema Center) is the only medical facility in the United States solely dedicated to expert HAE clinical care, professional medical education, and a unique combination of basic and translational research. In 2019, the HAEA obtained the rights to begin validation testing on a potential breakthrough blood test—discovered by Angioedema Center physician/scientists—designed to determine the biological cause of swelling in cases where the diagnosis is not clear. This blood test could help clarify the cause of swelling and help optimize treatment plans for HAEA members with the HAE normal C1 inhibitor diagnosis.

HAE CLINICAL TRIALS

For twenty years, the HAEA has actively helped recruit patients for clinical trials to help advanced scientific research on the safety and effectiveness of new medications. As a result, HAEA provided 69 qualified referrals to active clinical trial sites.



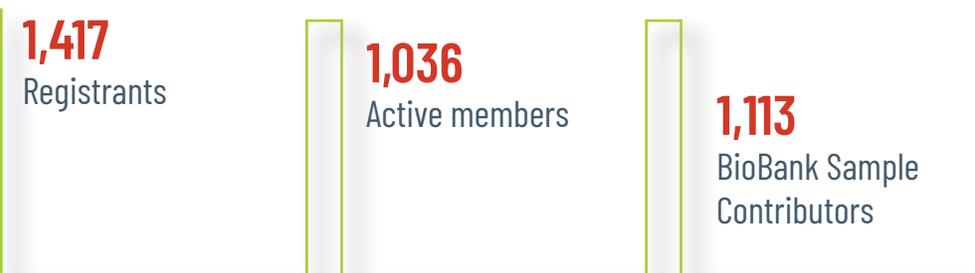
WORKING WITH PHARMA TO BRING NEW MEDICINES TO HAE PATIENTS

The HAEA's decade long efforts to unite patients, physicians, and pharmaceutical companies in a concerted effort to test HAE therapies have paid off with our community benefitting from 7 FDA-approved medicines. We remain committed to maintaining an environment that encourages and supports new and improved therapies. In 2019, the HAEA reached out to inform 2,298 HAEA friends about clinical trials being conducted in their area of the country.

ADVANCED HAE SCIENTIFIC REGISTRY

The HAEA Advance HAE Scientific Registry is a confidential database that allows researchers access to uniform, long-term, and accurate information on HAE directly from patients. This is the only registry in the US that collects data directly from patients and their family members to support HAE research. Launched over 10 years ago, Advance HAE continues to play a key role in helping our physician/scientists better understand HAE genetics and biochemistry with an eye towards identifying more effective treatments.

Last year, we surpassed our goals for obtaining patient information and blood samples. We collected 186 new members and collected blood for eventual DNA sequencing and another sophisticated testing. Meanwhile, we made improvements to the Advance HAE mobile app, (now available for iPhones and Androids), to ensure easy reporting for our patients and faster, more accurate data collection. Every submission takes us one step closer in our search to solve the remaining mysteries of HAE.



OUR 2018 CORPORATE SPONSORS

HAEA PRESIDENT'S CIRCLE



HAEA LEADERS



HAEA CONTRIBUTOR



US HAEA MEDICAL ADVISORY BOARD

The US HAEA Medical Advisory Board is composed of HAE experts from across the country who advise the Association on everything from medical research to peer-reviewed publications.



Bruce Zuraw, MD
MAB Chairman
Professor of Medicine
Director and US HAEA Endowed Chair
US HAEA Angioedema Center at UCSD
San Diego, CA



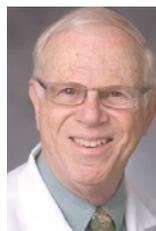
Aleena Banerji, MD
Assistant Professor
Massachusetts General Hospital
Boston, MA



Mark Davis-Lorton, MD
Director
Clinical Immunology
Winthrop-University Hospital
Mineola, NY



Jonathan Bernstein, MD
Professor
Clinical Medicine
University of Cincinnati
College of Medicine
Cincinnati, OH



Michael Frank, MD
Professor of Pediatrics,
Medicine and Immunology
Duke University Medical Center
Durham, NC



Paula Busse, MD
Associate Professor
Clinical Immunology
Mount Sinai Hospital
New York, NY



Henry Li, MD, PhD
President
Institute for Asthma and Allergy
Chevy Chase, MD



Sandra Christiansen, MD
Professor of Medicine
Director of Translational Research
US HAEA Angioedema
Center at UCSD
San Diego, CA



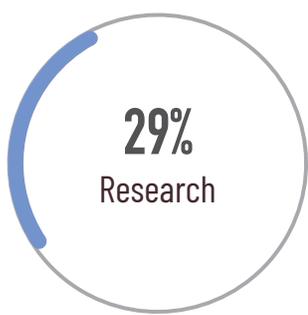
William Lumry, MD
Clinical Professor Internal
Medicine University of
Texas Southwestern Medical
School Dallas, TX



Timothy Craig, DO
Professor of Medicine and
Pediatrics
Penn State University
Hershey, PA



Marc Riedl, MD
Professor of Medicine
Clinical Director
US HAEA Angioedema
Center at UCSD
San Diego, CA



FINANCIAL

The distribution of expenditures shown to the left reflect the US HAEA's commitment to focusing on high impact programs, activities, and research aimed at improving the lives of people with HAE.

100 percent of every dollar donated by an individual and/or non-corporate source goes to the following three patient support programs: the Pam King HAEA Scholarship Fund, the HAEA Compassion Fund that provides travel assistance for patients seeking care from an expert HAE physician, and/or groundbreaking research including our HAEA Scientific Registry.

Corporate donations are earmarked to fund our many wide-ranging programmatic activities as well as administrative expenses.

The HAEA maintains rigorous and disciplined internal financial controls. A senior-level Director, who is independent from day to day operations and reports to the Board of Directors, carefully reviews all expenditures to ensure strict compliance with the HAEA's policies and procedures.

US HAEA BOARD OF DIRECTORS

Karen Baird

Henrik Balle Boysen

Anthony J. Castaldo

Tricia Mullins

Dr. Eric Phillips

Larry Salus

Christopher Whalen (In Memoriam)

YOUR HAEA TEAM

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Larry Salus, Financial Director

Christine Selva, Executive Project Manager

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Janet Long, Director of Research

Lois Perry, Director of Health

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Sally Urbaniak, Health Advocate

Troyce Venturella, Health Advocate

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Sherry Swanson, Patient Advocate, Market Research & HAE IN-MOTION®

Ianice Viel, Social Media Manager, Digital Platforms & Engagement

John Williamson, Patient Advocate, Advocacy & Clinical Trials

INFORMATION TECHNOLOGY

Frank Palaia, Information Technology Analyst

Joyce Wilmot, Information Technology Analyst

Cube Whidden, Information Technology Analyst

HAEA ADMINISTRATIVE SUPPORT

Michele Agostinho, Accounting Assistant

Nicole Jewell, Administrative Support