



2014

HAEi Annual Report



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Executive Director's Annual Report Message



Dear HAEi Friends,

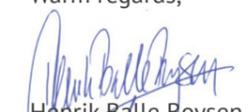
On behalf of the HAEi Executive Committee (members listed below), I am pleased to present our 2014 Annual Report.

- Mr. Anthony J. Castaldo, President United States of America
- Mr. Michal Rutkowski, Vice President, Poland
- Ms. Alejandra Menendez, Secretary, Argentina
- Ms. Fiona Wardman, Treasurer, Australia
- Ms. Sarah Smith Foltz, Spain
- Mr. Jørn Schultz-Boysen, Denmark
- Mr. Peter Hermeling, Germany
- Ms. Rachel Annals, United Kingdom
- Ms. Beverley Yamamoto, Japan
- Ms. Natasha Jovanovska, Macedonia

2014 was an exciting year for HAEi as we continued our worldwide advocacy efforts to improve diagnosis and patient access to suitable HAE treatments. Our 2nd HAE Global Conference in Washington D.C. stood out as a special event, that enabled patients and care givers from 35 countries to network, share experience and create new friendships. Also, in 2014 we issued 6 newsletters and dramatically increased our activity on various social media platforms.

I hope that you find the information on these pages both interesting and useful. Please do not hesitate to contact me should you have any questions or comments. We look forward to building upon our 2014 accomplishments and to making even greater strides in our quest to improve the quality of life for HAE patients throughout the world by further uniting the efforts of patients, care givers, physician/scientists, and industry.

Warm regards,


Henrik Balle Boysen
Executive Director

A Word from the President



Dear HAEi Friends,

2014 proved to be yet another busy and productive 12 months for HAEi. Throughout the year, we maintained our customary focus on (1) helping to find, organize, and empower patients with interest in forming new HAEi member organizations, and (2) supporting our existing groups in their ongoing efforts to raise awareness, increase diagnosis, and improve patient access to and reimbursement for life saving therapies.

Noteworthy 2014 accomplishments—to name only a few—included

- expanding HAEi membership,
- sponsoring and organizing the successful 2nd HAE Global Conference,
- achieving significant growth in **hae day :-)** activities, and
- launching the HAEi Global Access Program.

Our 2015 activities will center around

- conducting advocacy training workshops in various regions of the world;
- continued emphasis on patient identification & early diagnosis;
- assisting member organization advocacy for access to life saving medicines;
- publishing a global state of HAE management report;
- offering research grants through the HAEi Research Program;
- further growth in **hae day :-)** activities, and
- implementing the Global Access Program.

We look ahead with great excitement and a steadfast commitment to helping patients create new HAE groups and providing member organizations with programs, and services directed at achieving early diagnosis and broad access to HAE treatments.

Warm regards,


Anthony J. Castaldo
President

Reaching out to the World

Now represented in 40 countries

HAEi started out in 2004 with just a handful of countries, but has grown into a truly global umbrella organization dedicated to raising awareness of C1 inhibitor deficiencies around the world.

The HAEi member organizations are independent associations (or groups) working for the benefit of patients in their respective countries. The 40 countries that comprise HAEi include:

- Africa: Kenya
- Asia: China, India, Japan, Malaysia, Russia, United Arab Emirates
- Australia: Australia, New Zealand
- Europe: Austria, Belarus, Belgium, Bulgaria, Croatia, Czech Republic, Denmark, Finland, France, Germany, Hungary, Ireland, Israel, Italy, Macedonia, Norway, Poland, Portugal, Romania, Slovenia, Spain, Sweden, Switzerland, The Netherlands, Ukraine, United Kingdom
- North America: Canada, Mexico, USA
- South America: Argentina, Brazil

Current information on HAEi's presence around the globe can be found at www.haei.org. The website listing for each country includes contact data for the member organizations, information on care centers, hospitals, physicians, and trial centers, as well as a list of available medication in the specific country.

HAEi will continue focusing on building new organizations and strengthening existing relationships with patient groups and associations throughout the world.



HAE Global Conference 2014 – a great success



Feedback from attendees—450 delegates representing over 30 countries—confirmed that the HAE Global Conference in Washington D.C., USA 15-18 May 2014 was a great success.

With a focus on 'Setting New Standards', the conference provided HAE advocates from all over the globe with an opportunity to assess the state of HAE care in their countries and discuss strategies for making improvements with colleagues from other countries. The active participation of the delegates led to productive interactions between patients, the scientific/medical community, and pharmaceutical companies.

The conference was designed to serve as an incubator for developing creative collaborations and innovative approaches to improving HAE care throughout the world.

The overall aim was to inspire delegates to translate the knowledge and motivation gained during the conference into systematic action to improve diagnosis and access to life saving HAE therapies.

The conference began with two tracks - a scientific program for physician/researchers and a patient track. The rest of the conference consisted of sessions with all attendees participating.

The welcome reception, conference dinner, and guided bus city tour around Washington D.C. provided attendees with ample opportunities to meet, greet, and exchange thoughts and ideas. Participants also had the opportunity to interact with representatives from the conference sponsors Shire, ViroPharma, CSL Behring, Dyax Corp., BioCryst Pharmaceuticals and Salix Pharmaceuticals.

The Scientific Program - co-chaired by Prof. Bruce Zuraw, USA and Prof. Konrad Bork, Germany - focused on "Understanding Angioedema: How Do We Differentiate Different Forms?" and "Treating Angioedema Due to C1 Inhibitor Deficiency." Speakers in this session were:

- Prof. Jonathan Bernstein (University of Cincinnati, USA),
- Dr. Andrea Zanichelli (University Hospital Luigi Sacco Milan, Italy),
- Prof. Christian Drouet (University Hospital Grenoble, France),
- Dr. Alejandro Malbran (British Hospital Buenos Aires, Argentina),
- Dr. Camila Lopes Veronez (Federal University Sao Paulo, Brazil),
- Dr. Hilary Longhurst (Barts Health NHS Trust, United Kingdom),
- Assoc. Prof. Marc Riedl (US HAEA Angioedema Center San Diego, USA),
- Dr. Yung Chyung (Dyax Corp., USA), •!Prof. Timothy Craig (Penn State University, USA), and
- Dr. Inmaculada Martinez-Saguer (Hämophilie-Zentrum Rhein Main, Germany).

Among the speakers in the patients' program were Ms. Sarah Smith-Foltz (Spain) and Ms. Alejandra Menendez (Argentina), both from the HAEi Executive Committee. Their topic was "Ten Things Every Patient and Caregiver Should Know About HAE".

During the general sessions representatives of seven national organizations provided valuable insights into the present state of HAE diagnosis and treatment in their respective corners of the globe.

Other speakers in this session were:

- Prof. Bruce Zuraw (University of California San Diego, USA),
- Prof. Michael Frank (Duke University, USA),
- Prof. Henriette Farkas (Semmelweis University, Hungary),
- Assoc. Prof. Aleena Banerji (Harvard Medical School, USA),
- Suzanne Wait, Phd. (SHW Health Ltd., United Kingdom),
- Frank J. Sasinowski, Board Member, National Organization for Rare Diseases (USA),
- Melissa Hogan, Patient Advocate, Blogger, Saving Case & Friends Website (USA), and
- HAEi President Anthony J. Castaldo and Executive Director Henrik Balle Boysen, who presented a global assessment survey of HAE management.

During the last conference session, the audience had 90 minutes to ask HAE world class HAE physician/scientists questions about any HAE-related topic. This highly successful segment produced important information about the HAE diagnosis and treatment as well as tangible suggestions for solving real world HAE-related problems.

Before adjourning the 2014 HAE Global Conference, HAEi President Anthony Castaldo thanked the patients, caregivers, and medical professionals for attending and also thanked the pharmaceutical companies for their generous support. Mr. Castaldo summed things up with a call to action that challenged each attendee to use some of the insights and ideas learned during the conference to "Set New Standards" and improve the lives of HAE patients in their respective countries.



HAEi Research Program

A primary aim of HAEi is to make a significant contribution to efforts that will ultimately result in improving the lives of the global HAE community. To implement this goal, HAEi's Executive Committee created a grant program to support research that seeks better treatments and/or a cure.

The grant program encourages the development of new information that contributes to the understanding of the basic etiology and pathogenesis of HAE. Preference is given to research projects that provided a role for junior researchers, and could be carried out in the period of time and with the resources provided by the grant. Winning applicants can expect to receive \$50,000 USD per year for two years.

HAEi received 16 grant proposals, which were reviewed by a panel of independent expert physicians/scientists. The HAEi Executive Committee had no involvement, nor influence over the independent panel's deliberations and decision.

After thorough evaluation, the panel chose these submissions for funding :

- **"Regulation of B1 bradykinin receptors in HAE"**, Prof. Bruce Zuraw, US HAEA Angioedema Center at UCSD, San Diego, California, USA
- **"One cut too many: Factor XII mutations that cause hereditary angioedema enhance activation by plasmin"**, Assistant Professor Coen Maas, University Medical Center Utrecht, Department of Clinical Chemistry and Hematology, Utrecht, The Netherlands.



HAEi Communications

In accordance with its basic operating procedures, HAEi disseminates information to its national member organizations and routinely communicates with medical professionals, pharmaceutical companies, and the medical/scientific research community.

Throughout the year, HAEi provided the HAE community with frequent contact through Newsletters, Facebook pages, LinkedIn page, Twitter, YouTube channel, HAEi website, hae day :-) campaign website, and patient stories.

recounted lives with HAE. We expect to publish more patient stories throughout 2015.

Newsletter

The number of HAEi newsletters rose again from 2013 to 2014, bringing us to a total of six (March, May, July, August, October, and December).

Facebook

The HAEi group page on Facebook grew to 1,300 members who hail from all corners of the world. Also, there is a HAE Awareness Day group on Facebook, followed by some 14,000 people.

LinkedIn

In 2013 HAEi introduced a company page on LinkedIn, a popular business social media platform.

Twitter

HAEi opened a Twitter account in 2014, and we now have about 200 followers.

YouTube channel

A few years ago HAEi introduced a YouTube channel with a handful of short videos. They have been watched roughly 2,000 times.

HAEi website

In 2014, we gave the HAEi website a thorough makeover. The new website will be launched on hae day :-) 2015.

The hae day :-) website is also being redone with a new version being planned for launch before 1 April 2015.

Patient stories

In 2014, HAEi introduced the first of a series of HAE patient stories from around the globe. Ann Price from the United Kingdom, Rashad Matraji from the United Arab Emirates, and Cindy Hughes from Australia



Global Awareness Day 2014

The world has been celebrating HAE awareness day on 16 May since 2012 - and the number of events steadily increases every year.

On hae day :-) HAE advocates hold events that engage the general public and the medical community while raising funds to support programs designed to raise awareness of the need for (1) earlier and more accurate diagnosis, and (2) access to life saving medicines.

The 2014 hae day :-) was once again a great success due to the participation and support of the steadily growing number of HAEi members throughout the world.

In order to make participation in the 2014 global hae day :-) as easy as possible, HAEi once again put together a collection of useful information and documents to guide organizers through the key stages of an awareness campaign. Each country has different resources and levels of need, so HAEi designed a toolkit that allowed participants to pick and choose items appropriate for their specific use.

The 2014 hae day :-) toolkit included an overview of aims, objectives, and key messages; suggestions regarding engagement with the media; a pre-prepared Frequently Asked Questions; templates for letters to the advocacy group's members, physicians, and other rare disease or community groups; and tips on how to best use social media



Improved Access to Medication

It is the mission of HAEi to promote and facilitate effective HAE diagnosis and management throughout the world. Access to medication varies greatly from country to country – and there is still very much to be done. Here are some of the breakthroughs from 2014:

Argentina: After a long struggle HAE has finally been included in the list of chronic diseases to be covered by the Argentine refund system.

Brazil: In January Berinert was approved for the treatment of HAE and in August the product was launched. In October the Brazilian Health Surveillance Agency (ANVISA) approved self-administration of Firazyr.

Canada: The Ministry of Health Services in British Columbia is considering Firazyr for PharmaCare coverage. Under the PharmaCare program, the ministry seeks to provide coverage for drugs that support the health and well-being of British Columbians and provide value for money. Before a drug can be included in the PharmaCare formulary, it undergoes a review that takes place in three stages: Health Canada review, Common Drug Review, and Ministry of Health Services Drug Review. In British Columbia the ministry conducts its own review before making its coverage decision. The ministry has implemented a process that allows patients, caregivers and patient advocacy groups to submit input on specific drug reviews. The deadline for input was in November 2014 and HAE Canada is now waiting for the ministry's decision.

Israel: Ruconest has been approved and added on the Israel Health basket with no extra costs.

Mexico: The Mexican Board of General Health included Icatibant in the basic scheme and catalogue of medicines in the group "Endocrinology and Metabolism".

Poland: It is now possible to obtain reimbursement for Berinert for treatment of severe attacks of HAE as well as in pre-procedure prevention of acute episodes (short term prophylaxis). It is available for patients free of charge in open pharmacies on prescription.

Russia: For a couple of years HAE has been one of 24 rare diseases officially included on the list with obligations by the government to treat patients. Presently Firazyr and Danazol are available in Russia - and Ruconest is ready for registration, while Berinert is registered but not available on the market yet.

USA: The US HAEA Angioedema Center at UCSD adds to the list of clinics throughout the world that specialize in swelling disorders.

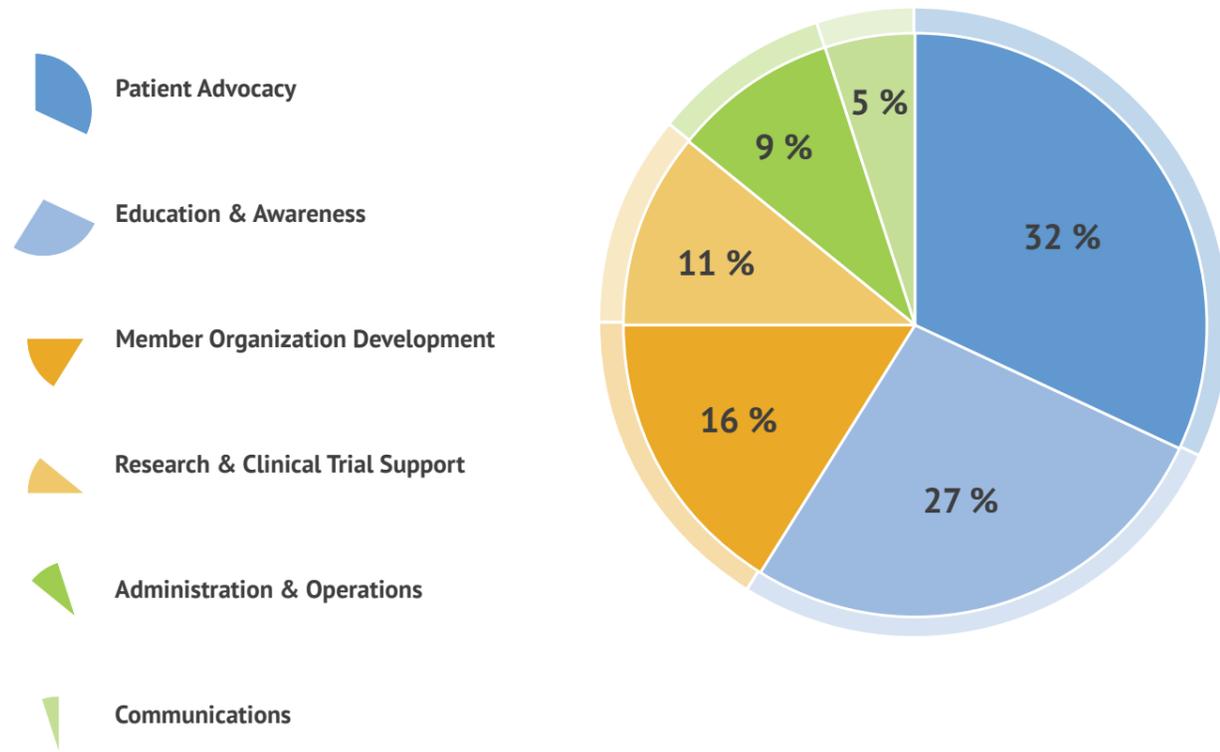
Opened in late 2014, the Center offers comprehensive care compassionately provided by academic physicians who possess a unique depth of angioedema knowledge and expertise.

The Center also has an ambitious research program that includes the previously mentioned examination of the B1 bradykinin receptor's role in HAE. The Center is also launching a large-scale project to study HAE with normal C1 inhibitor. This research will involve careful clinical characterization of patients suspected of this condition, collection of biologic samples, and laboratory analysis aimed at understanding the molecular and genetic basis of the disease.



Fiscal Year 2014

HAEi Expenses by Key Activity Category



Financial Internal Controls

During 2014, the HAEi Treasurer – who was elected by and reports to the Executive Committee – makes payments from the HAEi bank account after receiving documentation sufficient to support proposed expenditures. The HAEi President, who had a longstanding career in auditing, periodically (and on a random basis) reviews invoices and expense reports to ensure strict compliance with the contract between HAEi and the Executive Director.

Looking towards 2015 and 2016

Throughout 2015 and 2016 (assuming continued success in fundraising efforts) HAEi will be focusing on activities designed to expand and accelerate the pace and effectiveness of global HAE advocacy efforts:

Patient Identification & Diagnosis

HAEi will work with the member organizations to create an outreach program for attracting new patients and potential patients who can then be encouraged to get a diagnosis. This effort would include webinar workshops, in person meetings, and toolkits that can be used as reference guides.

National Access Support

HAEi is putting together a program to help member organizations identify and work with local experts who can provide guidance on the best way to approach health ministries and regulatory authorities. HAEi will then assist the members as they put together campaigns to communicate (1) the need for modern life saving therapies, and (2) the remarkable impact these medicines have on saving lives and reducing the burden of illness.

Global State of HAE Management Report

HAEi will conduct a survey and prepare a 'Global State of HAE Management' report that will help the organization better assess the treatment and diagnosis landscape throughout the world. The document will be produced in 2015 and will be translated into a variety of languages.

hae day :-)

Our global awareness day will continue to be an important platform for raising awareness of HAE. Also in 2015 HAEi is investing in a new website for hae day :-) as well as updated toolkits for member organizations.

Regional Workshops

HAEi will organize workshops in areas where there is limited or no access to medicines. The objective of the workshops will be to bring together patients, physician/researchers, and industry to establish working relationships and action plans. We plan to hold meetings in the following regions: The Balkans, Latin America, the Gulf Region, and Eastern Europe.

Global Member Organization Advocacy Training

HAEi is initiating a pilot program to identify, train, and motivate key individuals in the member organizations. These advocates will provide their local HAE community with a variety of services such as physician referrals and clinical trial recruitment.

HAEi Research Grant Program

HAEi expects to issue a Request for Proposals for HAEi Research Grants in the second half of 2015. Similar to the 2014 program, HAEi will provide 50,000 USD per year for two years to one or more research projects selected by an independent panel of scientists. Two projects were selected for funding in 2014 and HAEi envisions the same for 2015. The 2014 recipients will not be allowed to submit grants for the 2015 program. Once again, the HAEi President, Anthony J. Castaldo, will administer the program.

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Looking towards 2015 and 2016 (cont.)



HAEi Global Access Program

Together with Clinigen Group, HAEi has created a unique Global Access Program (HAEi GAP).

The HAEi GAP model includes

1. a Named Patient Program (with full payment/reimbursement),
2. a Compassionate Program (with free medication to certain parts of the world), and
3. an Early Access Program (where access to medication will be given in conjunction with a phase III clinical trial, until the medication is commercially available).

It is the first time that a patient group has initiated this type of medication distribution program. HAEi hopes to be able to update you on the progress of this exciting program in 2015.





HAEi

HAEi is a global non-profit umbrella organization dedicated to working with its network of national HAE member organizations to raise awareness of HAE.



You are not alone

Corporate Information

HAEi is officially registered as a non-profit/charity organization in the Canton of Vaud in Switzerland.

The registered address is:

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Avenue de Montchoisi 33
1006 Lausanne
Switzerland

Corporate Registration Number:
CHE-160.474.141

Bank Connection:
UBS Nyon, Switzerland

EUR Account:
IBAN: CH06 0022 8228 1117 3360 T
SWIFT/BIC: UBSWCHZH80A

USD Account:
IBAN: CH54 0022 8228 1117 3361 Z
SWIFT/BIC: UBSWCHZH80A

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