



HAEi Newsletter



IN THIS NEWSLETTER

**NEWS FROM OUR
NATIONAL MEMBER
ORGANIZATIONS**

**NEWS FROM OUR
NATIONAL PATIENT
GROUPS**

**GLOBAL ADVOCACY
WORK**

NEW PAPERS ON HAE

**NEWS FROM
THE INDUSTRY**

**HAEi AROUND
THE GLOBE**

A Message from the President

Dear HAE Friends,

One of HAEi's fundamental missions is to support the formation of new HAE patient groups in any corner of the globe where there is interest. I met several HAEi friends at the HAE Global Conference in Washington D.C. who were motivated to begin organizing a patient group in their country. To say the least, we encourage and are ready to wholeheartedly support - at every step along the way - anyone looking to form a group. The key question then is what needs to be done to start a new patient organization?

The good news is that over the years, HAEi National Member Organizations have established a path to success that can be duplicated by any group working to get organized. There are three key steps in the process of starting a patient group: Collaboration; Initial and Formal Organizational Meetings; and Legal Recognition.

Collaboration: The momentum for starting a patient organization begins with motivated patients, however, joining forces with (1) compassionate and action oriented physicians, and (2) pharmaceutical companies can bring vital expertise and resources to the organizing efforts.

Formal Organizational Meeting: Patients can then work with physicians and pharmaceutical companies to organize a meeting where all the stakeholders can participate. At that meeting, patients can designate a leadership group that will (1) establish the organizations vision and goals, and (2) work towards creating a legally recognized entity.

Legal Recognition: Obtaining registration as either a non profit or charitable organization is optimal and makes it easier to receive financial support that will fund the new group's activities. We recognize, however, that attaining legal recognition is not possible in some countries. Whether or not legal recognition can be achieved, it is still important that patients establish an organization dedicated to helping people in their country suffering from HAE.

Keep in mind that HAEi will enthusiastically help anyone with an interest to begin to apply the steps outlined above.

I wish all HAEi friends my warmest regards,

Anthony J. Castaldo
President, HAEi



HAE NEWS FROM AROUND THE GLOBE

From our National Member Organizations

Australia (www.haeaustralasia.org.au)

ASCIA Conference: HAE Australasia was represented at the ASCIA Conference held in Melbourne early September 2014. The conference program featured presentations by speakers from the USA, UK and Australasia on a range of topics pertinent to allergy and other immune diseases. It also included the ASCIA 2014 Allergy and Immunology Update for nurses, dietitians and other health professionals and the ASCIA 2014 Immunopathology Update. At the conference HAE Australasia hosted an exhibition booth in order to spread the word about HAE and HAE Australasia. HAE Australasia reports that this is the fourth ASCIA Conference the group has attended – and that all the medical professionals, unlike in previous years, had heard of HAE and the Patient Group.

2015 Patient Meeting: The next annual patient meeting will take place 16 May 2015 in Sydney.

Brazil (www.abranghe.org.br)

The WAO International Scientific Conference 2014 (WISC 2014) and the Annual Congress of the Brazilian Association of Allergy and Immunology (ASBAI) will be held in Rio de Janeiro 6-9 December 2014. The overall theme of the two events is 'Advancing the borders of allergy: From treatment to prevention by targeting the environment, infections and the susceptible patient'.

Denmark, Norway and Sweden (www.haescaan.org)

HAE Scandinavia has hosted three information meetings in Denmark: In Aarhus on 17 October, in Odense on 18 October, and in Copenhagen on 19 October 2014. The agenda for all meetings included information on the new Scandinavian organization, a treatment status for Denmark, and news on upcoming medication. The Copenhagen event also included the annual general meeting of the new organization.

The Swedish information meetings will be held on 15 November 2014 in Stockholm followed by Malmö on 16 November 2014.

In Norway the information meetings will take place 29 November 2014 in Bodø and 30 November 2014 in Oslo.

France (www.amsao.fr)

The next French patients meeting is scheduled for 14 March 2015 at Cochin Hospital in Paris.

Germany (www.angiooedem.de)

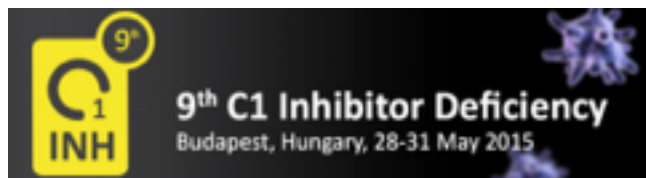
National meeting: The national HAE meeting in Germany took place 2-5 October 2014 in Brühl near Cologne. The program included the film project 'You are not alone' as well as presentations by Prof. Bork, Dr. Kreuz, Dr. Martinez-Saguer, and Dr. Aygören-Pürsün. HAEi's Executive Director also hosted a workshop for the HAE Youngsters, focusing on living a flexible life and pursuing the life's potential despite of HAE.



Bradykinin Symposium: A Bradykinin Symposium hosted by the Charité and icare was held in Berlin on 15-16 October 2014. International experts were invited to discuss the role of bradykinin in present and in the future. The symposium was complemented by the Charité/UNEV/icare/GA²LEN Angioedema School on 16-17 October 2014.

Hungary (www.haenet.hu)

If you want to take part in the 9th C1-Inhibitor Deficiency Workshop in Budapest you should get the dates 28-31 May 2015 in your calendar. The conference focuses on bradykinin-mediated angioedemas, and particularly on the types resulting from C1-inhibitor deficiency. The topics of this four-day long event cover a wide range of subjects. These are, among others, the latest achievements in the diagnostics of the disease. They also include the exploration of its hereditary, pathogenetic, and clinical background; as well as the management and follow up of the patients.



The main organizers of the 2015 workshop are the C1-inhibitor Deficiency Working Group, the Hungarian Angioedema Center, and the Hungarian Society for HAE Patients.

Much more information on the conference can be found at www.haenet2015.hu.

Macedonia (www.haemacedonia.mk)

Recently HAE Macedonia has embarked on an educational caravan in Macedonia with the purpose of raising awareness of HAE among the general public - but also in order to identify potential patients, help them enter the HAE network and provide them with guidance. Another purpose of the caravan is to intensively teach MD's on how to recognize the disease and to refer patients to adequate facilities to have them tested.

The caravan began on 18 October in Shtip and until the end of November it will be covering seven cities throughout Macedonia. The lectures are given by Prof. Vesna Grivcheva-Panovska, PhD, and they have been accredited by the Medical Chamber of Macedonia.

At this point there are 17 HAE patients in Macedonia, two of whom are children. Thus far, *no* treatment has been available to these patients.

Mexico (www.facebook.com/AEH.Mexico?fref=ts)

Through Asociación Mexicana de Angioedema Hereditario we have received the very good information that the Mexican Board of General Health had included Icatibant in the basic scheme and catalogue of medicines in the group 'Endocrinology and Metabolism'.

**Poland** (www.hae.org.pl)

The first national conference on angioedema took place in Warsaw on 18-19 October 2014. About 100 people participated, among them patients, physicians, representatives from pharmaceutical companies, as well as guests from the US and Germany.

Spain (www.angioedema-aedaf.org)

The 3rd workshop for Spanish HAE patients was held in Alicante on 18 September 2014. Also this time it was sponsored by Shire.

Switzerland (www.hae-vereinigung.ch)

The annual meeting of HAE Switzerland will be held on 7 November 2014 at Restaurant Arlecchino in Olten.

The Netherlands (www.hae-qc.nl)

7-13 November 2004 The Netherlands will host the week of the chronically ill. Further information can be found at www.chronischziek.nl.

**United Kingdom** (www.haeuk.org)

Website coming up: The big update of the HAE UK website is on its way. If you have suggestions for additions please e-mail rachel.annals@haeuk.org.

National Patient Day: This years' patient day will be held on 6 December 2014 at Addenbrookes Hospital in Cambridge. Speakers include Dr. Hillary Longhurst, Dr. Bill Egner and Specialist Immunology Nurses John Dempster and Christine Symons. The meeting is open for all HAE patients and their immediate family members (over 16 years of age). Register online at www.haeuk.org/patient-day.

Patient Meeting in North West England: Patients in the North West of England will be having their own Primary Immuno-deficiency and HAE patient day at the Haydock Park Racecourse in November 2014. This meeting is being hosted by the North West Immunology Centres for patients who attend their clinics. Check the HAE UK website for details.

United States of America (www.haea.org)

ACAAI Annual Scientific Meeting: The 71st ACAAI Annual Scientific Meeting will take place in Atlanta, Georgia 6-10 November 2014. The meeting title is 'Faces and Facets of Allergy & Immunology', selected by the Annual Program Committee to represent the faces of the many types of patients that Allergists/Immunologists see in their offices and the facets of treatment they offer.

National Patient Summit: US HAEA is planning the next National Patient Summit for the fall of 2015. The upcoming summit promises to offer even more opportunities to take part in important research, talk to reimbursement specialists, hear the latest on HAE research and, of course, meet US HAEA friends familiar and new.

As a warm-up to the 2015 Summit you might want to have a look at the opening remarks from the 2013 event - you will find it here: [2013 Summit Opening Remarks](#).



Special Blood: The documentary 'Special Blood' by Natalie Metzger is one of 25 films selected for the Cinecause Women's Initiative. Cinecause is the owner of the Hollywood Film Festival, and the initiative is to help the selected films get the rest of their funding as well as distribution.

Have a look at the film project at www.specialblood.com or at the Facebook page at www.facebook.com/rareandpotentiallyfatal.

AAAAI Annual Meeting: Houston, Texas will be hosting the 2015 AAAAI Annual Meeting 20-24 February 2015. Thousands of allergist/immunologists, health and healthcare professionals will get together for five days with hundreds of educational offerings on a variety of topics such as allergic disease, asthma, immunotherapy, food allergy, skin disease, practice management, new technologies, and health care reform.



Climbing the mountain: As mentioned in the previous newsletter Prof. Zuraw, Prof. Christiansen and US HAEA financial advisor, Mr. Caesar Sweitzer have climbed Mount Kilimanjaro. Here is an extract of what Prof. Zuraw wrote about the experience earlier this month:

It was a unique and wonderful experience to be on Kilimanjaro. We felt that the climb partially fit the metaphor of patients coping with HAE. The uncertainty, challenge, hope and ultimately the step-by-step journey to success were all there. We recognize, however, how much more difficult the challenge of living with HAE really is. Our hope is that the money and awareness raised by this effort as well as the continuing efforts of so many people will help reduce the magnitude of this challenge. Working together we met and exceeded our ambitious goal of raising 50,000 USD to support the US HAEA Patient Benevolence Fund. This money will provide essential support for HAE patients in need of expert medical care. Each of us contributed to the success of this effort by coming together as a community. Let us continue to work together to raise the standard of care, improve the quality of life for affected individuals, and hasten the day when we can talk of a cure for HAE.



Fundraising: The 14 year old high school student Michael Ardito managed to raise awareness as well as money for research to find a cure for HAE. Along with close family friend Luke Tanner Michael took a plunge into the cold waters of the Boston Harbor and on 20 September 2014 they swam across the harbor as participants in the Boston Sharkfest Swim. At <https://fundrazr.com/campaigns/dqfd9> you can see that this fundraising campaign has raised more than 8,800 USD – or 177 percent over the budget. Way to go, Michael.

From our National Patient Groups

Belarus (www.hereditary-angioedema.org)

Mr. Viktor Lebedz, former member of the HAEi Executive Committee, is presently working on setting up a HAE workshop in Minsk, Belarus.

Mr. Lebedz is also the person behind the very special Belarusian HAE advocate, Nadia, presented earlier this month. Have a look

at Nadia at www.haedoll.org - and read more about her in the next newsletter.

Kenya

We are happy to announce our newest National Patient Group, formed in Kenya on the African continent. The contact person is Ms. Patricia Karani in Nairobi (frepashe@yahoo.com).

Global Advocacy Work

Recent activities

HAEi is invited to participate in a variety of international meetings. This is a brief overview of some of the activities in the recent months:

Prof. Marco Cicardi has established a group that will apply for a grant available from the "EU framework Program for Research and Innovation." In July Dr. Cicardi invited HAEi to be a member of this group and participate in a meeting in **Brussels, Belgium**. Since HAEi no longer is registered in a EU country, Dr. Cicardi has suggested and approved that HAE Scandinavia will be listed as one of the project members. The inclusion of HAE Scandinavia is solely for practical matters and in order to be eligible for EU funding, should this very exciting project be approved.

Early September HAEi visited **Sydney, Australia** in order to work with Fiona Wardman, the new treasurer of the organization. Among many topics the meeting included a review of the Swiss rules and banking regulations to ensure HAEi compliance, an overview of the online bank account at UBS, a discussion of HAEi's financial internal controls, and the review by HAEi's Swiss accountant.

HAEi has had a longstanding interest in establishing a program that would allow manufacturers to either donate or otherwise provide access to HAE medicines in countries where these products are currently not registered. In September HAEi met with a **Birmingham, United Kingdom** based company that has the expertise to help the organization design and implement a legally sound and regulatory compliant global program to broaden the availability of HAE medicines. This meeting will help HAEi determine if it is possible to come up with an

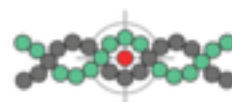
innovative approach to widen access to these rare disease medicines.

Late September HAEi was invited to speak at a physician meeting in **Ulm, Germany**. Dr. Jens Greve was hosting the meeting to further raise awareness of HAE in – primarily – southern Germany.

In October HAEi was invited to participate in and conduct a workshop for the German HAE Youngsters (boys living with HAE) at the annual meeting of the German patient association in **Cologne, Germany**. Also, we were invited to attend the Polish national HAE meeting in **Warsaw, Poland** as well as a NIH/US HAEA sponsored HAE scientific meeting, taking place in **Bethesda, Maryland, USA**.

Future activities

Mid November HAEi has been invited by Prof. Konrad Bork to speak about the Burden of Illness and the Patient Perspective during the annual German Dermatological Conference in **Mainz, Germany**.



جمعية الأمراض الجينية الوراثية
UAE Genetic Diseases Association

HAEi, together with the UAE Genetic Disease Association, will conduct a workshop for Gulf Region physicians in **Dubai, UAE** in mid December 2014.

In the first part of January 2015 HAEi will participate in the first patient/physician meeting in **Tokyo, Japan** since HAE Japan became a recognized NGO.



NEW PAPERS ON HAE

Here are summaries of some of the HAE-related scientific papers published since our last newsletter:

Tissue factor expression on the surface of monocytes from a patient with HAE – by K. Iwamoto, Hiroshima University, Japan, et al.:

Environmental factors, minor trauma and oral contraceptives have been reported to induce angioedema attack, but the trigger may often be uncertain. Activated factor XII controlled by C1-INH facilitates bradykinin generation and also regulates coagulation cascade, but the relationship between edema formation and coagulation is still unclear. (*J Dermatol.*, Sep 2014)

HAE: Children Should be Considered for Training in Self-Administration – by O. Abdel-Karim et al., Odense University Hospital, Denmark:

Studies have shown that home therapy for angioedema reduces disease severity, leads to faster relief of symptoms, and improves quality of life. Most studies have been conducted in adults. We report a 13-year-old boy who quickly learned self-administration, which resulted in reduced frequency and severity of attacks. (...) children should be considered for self-administration training and (...) the process does not have to be long or complicated. (*Pediatr Dermatol.*, Sep 2014)

Paediatric HAE: A survey of UK service provision and patient experience – by N. Read et al., Brighton and Sussex Medical School, United Kingdom:

For affected children in the UK, there are relatively few data regarding disease prevalence, service organization and the humanistic burden of the disease. To improve knowledge in these areas, we surveyed major providers of care for children with HAE. A questionnaire was sent to major pediatric centres, to determine patient numbers, symptoms, diagnostic difficulties, management and available services. To our knowledge this study represents the first dedicated survey of pediatric HAE services in the UK and provides useful information to inform the optimization of services. (*Clin Exp Immunol.*, Aug 2014)

C1-esteraseinhibitor treatment: preclinical safety aspects on the potential prothrombotic risk – by D. Schürmann et al., CSL Behring GmbH, Germany:

The data suggest that treatment of healthy rabbits with high doses of C1-INH could potentially inhibit coagulation and thrombus formation rather than induce a prothrombotic risk. (*Thromb Haemost.*, Aug 2014)

Activation of the ficolin-lectin pathway during attacks of HAE – by D. Csuka, Semmelweis University, Hungary, et al.:

There is a marked heterogeneity of the pathomechanism and development of HAE attacks in different patients. Our results suggest that the activation of the ficolin-LP may deplete the innately low level of C1-INH and thus, it may contribute to the uncontrolled activation of plasma cascade systems, and thereby to edema formation. (*J Allergy Clin Immunol.*, Jul 2014)

An evidence based therapeutic approach to hereditary and acquired angioedema – by K. Bork, Johannes Gutenberg University, Germany:

The number of management options for HAE-C1-INH and similar conditions has increased considerably within the last few years, thus helping to alleviate the burden of these rare diseases. (*Curr Opin Allergy Clin Immunol.*, Aug 2014)

HAE in Greece: The First Results of the Greek Hereditary Angioedema Registry – F. Psarros et al., Naval Hospital of Athens, Greece:

Until recently, there has been a significant degree of underdiagnosis of HAE in Greece. Very low compliance with the provisions of the applicable international guidelines and consensus positions, with adverse consequences on the patients' quality of life, was also observed. A systematic recording of HAE cases was undertaken following a physician awareness campaign and confirmation of diagnosis. 116 patients from 41 non-related families were recorded. The average delay in diagnosis was 16.5 years and the incidence of death in the families of patients was 1 for every 2 families. The use of newer therapeutic agents seems to fall significantly short of the existing needs. (*Int Arch Allergy Immunol.*, Sep 2014)



News from the Industry



The U.S. Patent and Trademark Office (USPTO) has issued two new patents related to DX-2930, an investigational fully human monoclonal antibody inhibitor of active plasma kallikrein from **Dyax**

Corp. DX-2930 is developed as a subcutaneous injection for prevention of HAE attacks.

One patent contains claims covering the specific sequence of DX-2930, while the other contains claims covering monoclonal antibodies that bind to the active form of human plasma kallikrein and do not bind human pre-kallikrein. Both patents are expected to provide coverage for DX-2930 until at least 2032.

“These newly issued patents contain broad composition of matter claims which provide important long-term exclusivity for DX-2930, especially its binding properties to human plasma kallikrein, which we believe is a critical target in the treatment for HAE”, said Gustav Christensen, President and CEO of Dyax. “Collectively, they further validate Dyax’s discovery capabilities and confer a unique and robust intellectual property estate surrounding DX-2930, enhancing its commercial potential.”



Pharming Group NV has developed the recombinant C1 inhibitor product Ruconest. This product is marketed in Europe and some other countries through their partner **Swedish Orphan Biovitrum (Sobi)** - and Sobi in September 2014 opened the company’s new North American office in Massachusetts.

“North America is an increasingly important region for Sobi, and building our team in the Boston area supports us in our efforts to better address the needs of patients and healthcare providers throughout U.S.A. and Canada”, says Geoffrey McDonough, Sobi’s President & CEO. “We are delighted to partner and collaborate with the Massachusetts Life Sciences Center as we build and grow in the years to come.”



Dyax Corp. has highlighted recent progress and upcoming milestones in its Licensing and Funded Research Portfolio (LFRP), including, most recently, licensee Eli Lilly and Company’s announcement of the fourth positive Phase 3 study outcome for CYRAMZA (ramucirumab). Product candidates in Dyax’s LFRP are eligible for royalties on the first ten years of commercial sales

from each antibody program, as well as certain milestone payments. The LFRP currently has 10 product candidates in clinical trials and multiple preclinical programs.

“Our proprietary phage display technology, through LFRP licenses and collaborations, has helped give us the financial flexibility to expand our HAE portfolio and focus on additional opportunities in the plasma kallikrein pathway”, said Gustav Christensen, President and CEO of Dyax.

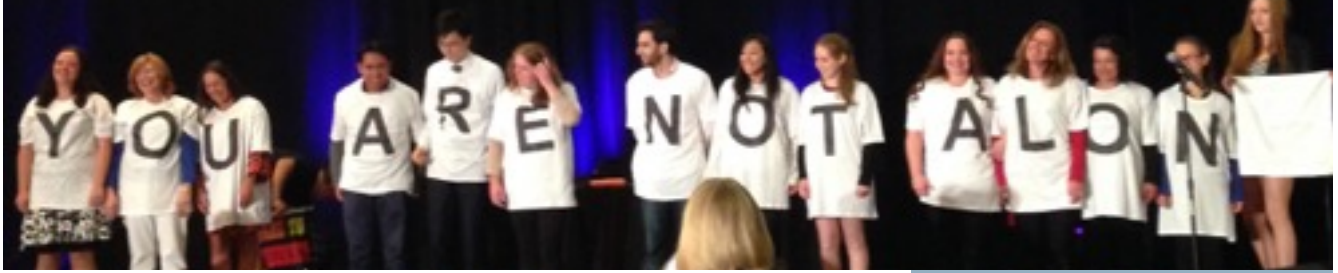


Pharming Group NV and **Swedish Orphan Biovitrum (Sobi)** has announced the amendment and extension of their Ruconest distribution agreement. Pharming will focus on direct commercialization in Austria, Germany and the Netherlands, while Sobi will extend its Ruconest sales territory with the addition of Azerbaijan, Belarus, Georgia, Kazakhstan, Russia, Serbia and Ukraine.

Pharming has recently started the hiring of a small European team of experienced HAE commercialization and medical affairs specialists to take-over the direct commercialization activities from Sobi for Ruconest in Austria, Germany and Netherlands.

To ensure a seamless handover, in particular to guarantee the continuous availability of Ruconest to patients and the drug safety monitoring and reporting of Ruconest, Sobi will continue to deliver Ruconest as before and will continue the drug safety monitoring and reporting during the remaining months of 2014.

Sijmen de Vries, Pharming CEO commented, “This step forward into direct commercialization of Ruconest became possible as a result of our recently improved balance sheet and opens up new opportunities for Pharming to not only grow revenues, but also, as those revenues build, to put in place the right size of specialist commercial infrastructure which could, over time, be leveraged through the marketing of other products. This move is expected to, almost immediately, have a positive impact on our financial performance as we will be able to improve the margin from sales in the EU territories with every vial of Ruconest sold in Austria, Germany and Netherlands. In addition, the extension of the Sobi territory will enable Sobi to continue growing Ruconest revenues further.



HAEi around the globe

HAEi is a global network organization dedicated to raising awareness of C1 inhibitor deficiencies around the world.

Our **National Member Organizations (NMO)** are independent associations working for the benefit of patients in the specific country. Currently we have NMO's in 27 countries:

- **Asia:** Japan
- **Australia:** Australia, New Zealand
- **Europe:** Austria, Belgium, Czech Republic, Denmark, Finland, France, Germany, Hungary, Israel, Italy, Macedonia, Norway, Poland, Portugal, Spain, Sweden, Switzerland, The Netherlands, United Kingdom
- **North America:** Canada, Mexico, United States of America
- **South America:** Argentina, Brazil

Our **National Patient Groups (NPG)** are HAE patient representatives in countries where no formal association has yet been founded or where the process of

founding an association is starting up. Presently we have NPG's in 11 countries:

- **Africa:** Kenya
- **Asia:** China, Malaysia, United Arab Emirates
- **Europe:** Belarus, Bulgaria, Croatia, Ireland, Romania, Slovenia, Ukraine

You will find much more information on the HAEi representations around the globe at www.haei.org. For instance, under each of the countries there is contact data for the national organization/group, information on care centers, hospitals, physicians, trial centers, and pharmaceutical companies as well as a list of available medication in the specific country.

The information on www.haei.org is being updated as soon as we receive fresh data from the NMO's or NPG's.

Your feedback is very welcome

Please let us know what you believe should be included in future newsletters. You can do that by providing feedback to Executive Director [Henrik Balle Boysen](mailto:h.ballsen@haei.org) or Communications Manager [Steen Bjerre](mailto:s.bjerre@haei.org). In addition, we invite you to

submit articles on any topics that you believe would be of interest to other readers. We look forward to your comments and working with you on future newsletters.

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

NEWSLETTER STAFF

Mr. Steen Bjerre
Communications Manager
E: s.bjerre@haei.org
P: +45 22 20 46 01

Mr. Henrik Balle Boysen
Executive Director
E: h.boysen@haei.org
P: +45 31 591 591

Subscription

If you would like to subscribe to our Newsletter please send an email to info@haei.org or register directly on our website www.haei.org.

HAEi Newsletter
HAEi - Hereditary Angioedema
International Association



hae
day:-)

MANY FACES
ONE FAMILY

