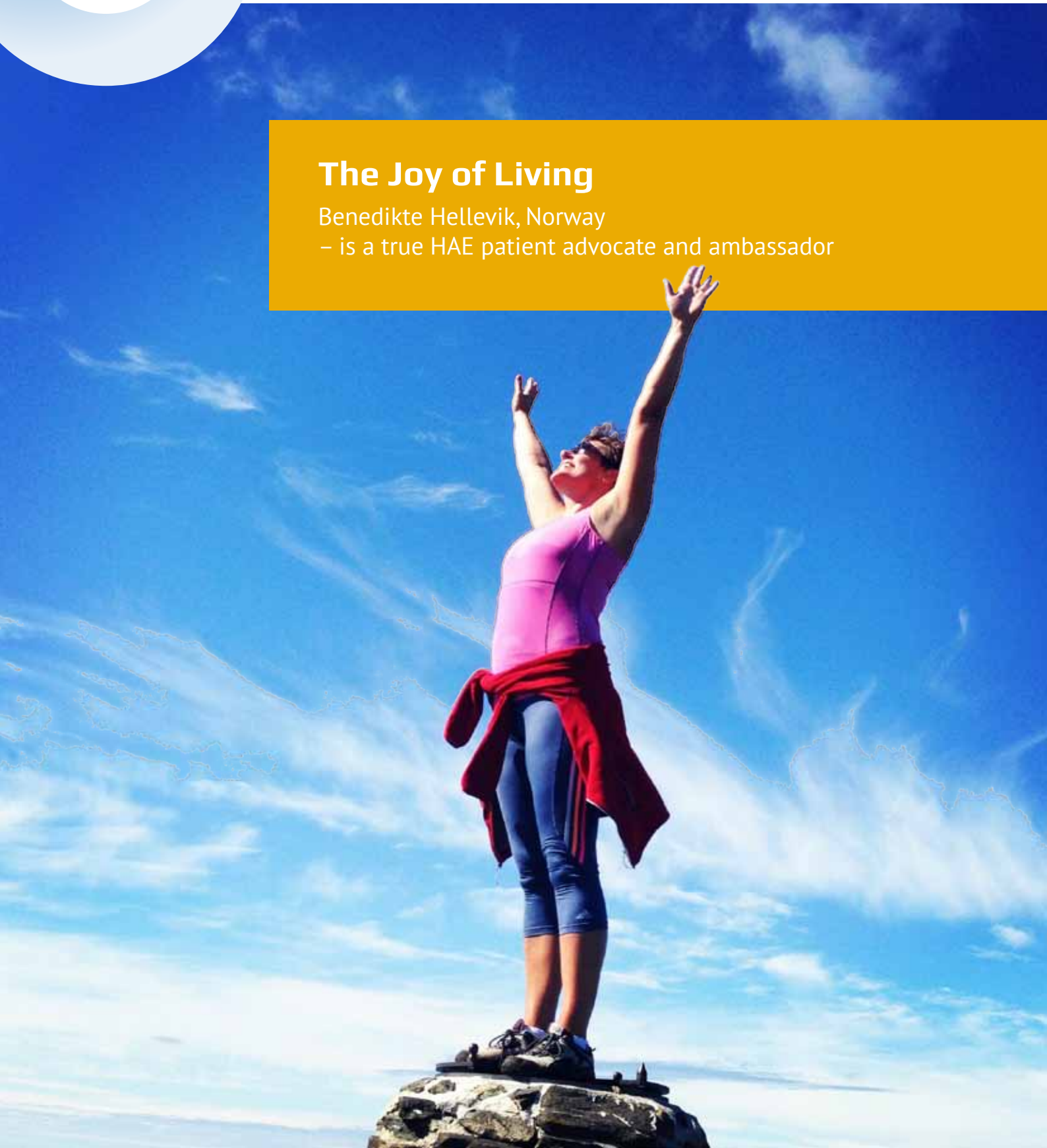
Issue 7 · November 2015

HAEi Newsletter



The Joy of Living

Benedikte Hellevik, Norway
– is a true HAE patient advocate and ambassador

Tabel of Content

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

HAEi Newsletter · Issue 7 · November 2015

Front page photo of Benedikte Hellevik, HAE patient in Norway – read her story on page 4

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HAEi is registered as a non-profit/charitable organization in Switzerland

A Message from the President

Dear HAEi Friends,

Our 7th HAEi newsletter published in 2015 features the captivating story of Benedikte Hellevik from Norway. This remarkable HAEi Friend exemplifies indomitable human spirit, humor, and unflappable dedication to a cause – all in the face of heartbreaking tragedy. Benedikte's emotional and loving tribute to the beauty of life strikes at the very chord of our humanity and challenges us to embrace family and friends while striving to be better at everything we do.

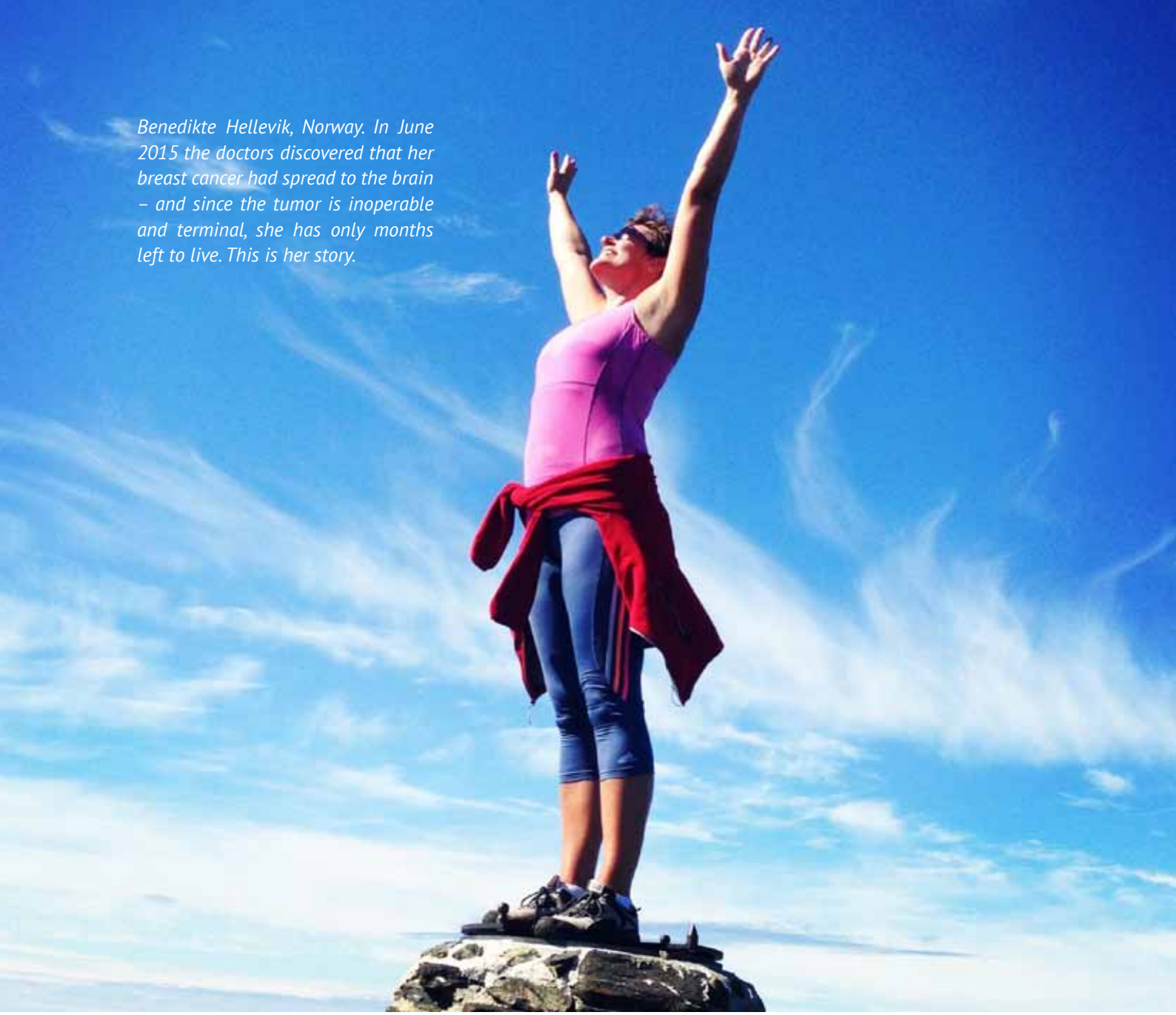
Family testing is an important issue that does not receive sufficient attention. Because HAE is an inheritable, unpredictable, and potentially fatal disease, HAEi believes it is essential that every member of a family with a parent who has the HAE diagnosis must be tested. The article on family testing in this newsletter provides links to documents HAEi developed to educate patients and physicians on this important and potentially life saving topic.

Planning is well underway for the third HAE Global Conference (19 – 22 May 2016 in Madrid, Spain), which will focus on the theme – Creative Advocacy for Expanding Access to Therapy. In this newsletter HAEi announces the El Camino Walk—an exciting **hae day :-)** awareness raising prelude to the Global Conference that will take place 14 to 18 May 2016 on the historic pilgrimage route El Camino de Santiago. HAEi Friends, relatives, caregivers, doctors, nurses, and industry representatives are invited to sign up for this fun walk, which symbolizes HAE patients' progress in improving their quality of life despite their HAE.

Finally, HAEi has published its 2015 State of Management of HAE in Europe as a follow up to the similarly titled document issued over three years ago. Unfortunately, the new report concludes that basic access to life saving therapies still eludes patients in many European countries and more needs to be done to help HAE patients in Europe. The report contains a unique perspective on the personal and family burden of illness and provides a "Call to Action" with advocacy principles that can be used in countries where access to modern HAE medicines is limited. The document is a must read for all of us in the global HAE advocacy movement and can be downloaded from the link provided in this newsletter.

I wish all HAEi friends happiness and good health,

Anthony J. Castaldo
President, HAEi



Benedikte Hellevik, Norway. In June 2015 the doctors discovered that her breast cancer had spread to the brain – and since the tumor is inoperable and terminal, she has only months left to live. This is her story.

The Joy of Living

Benedikte Hellevik has only months left to live. So how on earth can she talk about the joy of living? Well, for her it's more important than ever. Life isn't over yet, and she is going to enjoy every second of it.

For quite some time I've thought about how to keep the spirit going, living with HAE, I mean. I have HAE II and so has my sister, my father and my 17 year old son Tobias. All of a sudden it got pressing for me to tell my HAE story, since I got seriously ill in the summer of 2014. It turned out that I had breast cancer, and I had to have my right breast removed along with all my lymph nodes in my right arm. I went through chemotherapy and radiation, and was done with my treatment in February 2015. Little by little I started to work again and had a new job in May - but by the end of June the doctors discovered that my cancer had spread to the brain. As the tumor is inoperable and terminal I have only months left to live.

I can't stop wondering how on earth you can choose the title 'The Joy of Living' for your story then.

Well, for me it's more important than ever, isn't it? Life isn't over yet, and I´m going to enjoy every last second of it. I'm full of different medications, my face is like a blowfish, but still nothing like an HAE-attack to the face, you know. Now, the situation is what it is and the truth of it is that I have never felt more blessed than today. I would like to get back to that an a little while.

Okay. Let's talk about HAE in you family. You grew up knowing what your condition was?

Yes, in that respect I was lucky as both my family and I always knew what this was all about. My father always wanted my sister and me to believe we could do anything we wanted - in spite of having HAE. So I never knew anything else, other than to try doing what I wanted, even if my hand or foot was a bit swollen. This was the way HAE mostly showed itself in my case. For my sister it was worse as she had much more painful swellings to the stomach. I played soccer, and I knew that after each summer break my feet would swell after my first soccer practice. But I also knew that after the next practice they would be fine.

I'm not saying this way of doing it is for everyone. However, if there is one thing I'm sure of it is that HAE patients have to fight harder, be better, be stronger and more engaged than others. And this can be hard, because we often know a bit more about HAE than most general physicians.

Ahh, doctors....

Yes, we love them and we hate them, don't we? They are the bearers of both the good and the bad news. Yes, you have HAE. Yes, there is medication. You might have to inject it into your bloodstream yourself. It may cost you money - is your insurance good?

In the Scandinavian countries we are very lucky as we have good reimbursements. However, now that I am in fact dying - of cancer, not of HAE - I all of a sudden realize a few things: HAE patients in China, Russia, Ukraine, and a number of other countries are in my situation. They can also die tomorrow, in a few months, next year. This is a horrible insight, honestly. And it makes me want to fight harder for our fellow HAE friends, my extended HAE family. We must never forget how lucky we are, and never stop fighting for all the others who doesn't have the benefits that we know in for instance the Scandinavian countries.

I know that HAEi is doing a lot worldwide but we as patients must never stop supporting and helping as we best can. Our knowledge is very valuable.

How do you cope with the situation you are facing?

Well, in all this happening to me humor is my number one weapon. Let me assure you - it's not even good humor. I sometimes lose my sense of hearing, and my sister who was driving me around - they won't let my drive anymore, how fun is that?! - and I said to her that it was sometimes nice to not hear everything. It made us laugh quite hard. Stupid humor releases tension in a difficult time and it feels liberating. It keeps us from crying. Believe me, I've cried my share too, but I'd rather laugh.

I remember a few years ago, I met a Dutch brother and sister with HAE at a conference, and we had a marvelous time discussing how HAE Olympics would look like. These were some of the exercises I can remember we thought could be suitable: 100 m with a swollen foot; Longest vomit; Javelin with swollen hands. There might have been a few drinks involved in this, but I never think anyone of us ever laughed so hard. My point is that laughter ignites my spirit - and engaging in HAE makes me want to fight for those less fortunate.

Being together with other HAE patients is like being with family to me. I never feel more understood than when I'm with other people with HAE. One of the saddest things about this is that I'm leaving them. I hate that! But I love the way we take interest in each other, share our stories, laugh at that joke that when our lips swell "we have natural fillers". We must never stop having fun with this serious disease. We are not getting rid of it anyway, are we now?

Continues on next page

In you opinion, is it possible to make family members that are not HAE patients see this condition from a somewhat more positive side - to laugh along with you?

Well, we as HAE patients might worry much about it after all and there is no doubt that the ones in our nearest family often worry more than we do ourselves. Naturally, you'll have to be sure to assure them when you are fine.

How can HAE patients create more understanding of the disease?

We are all HAE warriors in our own way, and we must never ever be afraid of telling health professionals or others what HAE is. It's the only way to create understanding and acceptance of this strange and rare thing. It's the only way. So go to your heart, find the strength, lean on your friends and family, and fellow HAE friends and fight. Fight to be whatever and whoever you want to be. And remember to laugh in the face of HAE sometimes, tell yourself that HAE cannot define who you are. Let your spirit do that.

So, back to why you feel blessed?

Indeed. I have all my HAE family that understand my situation, I have lots of love in my life, I try to spend and enjoy every day to the fullest - I just have to be open to take it all in.

And you spend some of the little time you have left being a HAE warrior?

We all should, you know. Be warriors for HAE, and back up any patient, doctor or family member who needs it.

To all the HAE patients, I would really like you to think about this: If you woke up tomorrow without fear of anything at all, what would you do? You have a choice.

PS: You can also watch a short video with Benedikte Hellevik on the HAEi Youtube channel:

https://youtu.be/1U9qrtRfveQ

Patients' stories

On the HAEi website at www.haei.org HAE patients from Australia, Belarus, Brazil, Denmark, Hungary, Norway, Russia, the United Arab Emirates, and the United Kingdom tell their touching and motivating stories. We invite you to read about the HAE lives of a diverse group of fellow patients that include a university lecturer, a truck driver, a retired nurse, an operations manager, and a cattle farmer.

HAEi GAP GLOBAL ACCESS PROGRAM

HAEi Global Access Program

Helping to change the lives of patients with hereditary angioedema (HAE)

HAE is a rare, potentially life threatening, disease that affects an average of 1 in 30,000 people no matter where they live. Modern HAE medication is not available in every country. HAEi (the international umbrella organization for the world's HAE patient groups) understands the frustration and desperation felt by patients living without modern HAE medicines. We are committed to helping the global HAE patient community obtain access to life saving therapies, that is why we created the HAEi Global Access Program (GAP). This program provides an opportunity and a mechanism for physicians to access modern HAE medication for their patients.

HAEi GAP Project Manager, Deborah Corcoran, would be delighted to provide more details to anyone interested in the program. Deborah can be contacted at

d.corcoran@haei.org

Formal inquiries regarding the HAEi GAP program, should be directed to our distribution partner, Clinigen/ Idis, customer services via:

customer.services@clinigengroup.com

+ 44 1283 494340 (UK)

2016 HAE Global Conference in Madrid

The 2016 HAE Global Conference will take place 19 – 22 May 2016 in Madrid, Spain. The theme for international gathering is "Creative Advocacy for Expanding Access to Therapy."

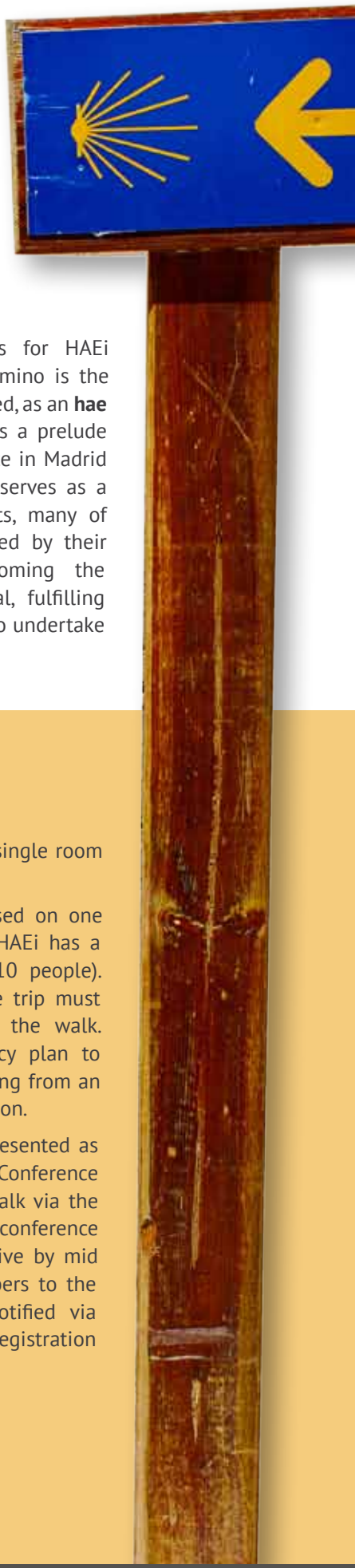
Attendees at the 2016 HAE Global Conference can expect a wide variety of important information and learning opportunities that include HAE fundamentals, the most recent clinical advances and consensus treatment recommendations, and advocacy strategies/techniques for gaining or broadening access to HAE medicines. There will be a separate track for young patients that will enable peer group interaction and sharing of insights on how to cope with HAE. Health care professionals will also be an integral part of the conference. HAEi will offer an educational and networking session for nurses specializing in HAE; and HAE physician/researchers from throughout the globe will gather to present abstracts and discuss future research opportunities.

Registration for the conference

HAEi is delighted to be able to offer patients and their care givers (spouses) and approximate family the opportunity to apply for a travel grant in conjunction with the 2016 HAE Global Conference. All details about registration will be available on the registration website, which is expected to be launched in the first half of December. All subscribers to the HAEi newsletter will receive an email with a link and instructions as soon as registration is opened. In order to be eligible for a travel grant, you will have to a) have the diagnosis hereditary angioedema, b) be a member of the "HAEi Family" (by subscribing to this newsletter you are a member), and c) be a member of your national HAE organization. Unfortunately HAEi is not able to offer travel grants to healthcare professionals (physicians, nurses and others).

Join the HAEi Camino Walk

HAEi is excited to announce a very special hae day :-) event that will serve as a prelude to the 2016 HAE Global Conference. HAEi is providing an opportunity for patients, relatives, caregivers, doctors, nurses, and industry to walk the pilgrimage route El Camino de Santiago (The Way of St. James) to the shrine of the apostle Santiago (St. James) in the Cathedral of Santiago de Compostela in the northwestern part of Spain.



Over three days (15 to 17 May 2016), participants will walk a segment of the Camino measuring 20 km (day 1), 15 km (day 2) , and 13 km (day 3), allowing everyone interested to take part. And there will be ample time to see things, go places, rest, and just be together.

Among many other things the Camino is a trail, a hike, a pilgrimage, a cultural tour, a historical journey. Some walk the Camino for spiritual reasons – others for culture, history or sport. Whatever the purpose, it will be an enriching, rewarding experience.

One of the major reasons for HAEi arranging a walk on the Camino is the publicity that can be generated, as an **hae day :-)** event in itself and as a prelude to the HAE Global Conference in Madrid 19-22 May 2016. The walk serves as a statement that HAE patients, many of them frequently incapacitated by their attacks, are slowly overcoming the obstacles to leading normal, fulfilling lives and can now feel free to undertake a journey of this nature.

More about the Camino

The Camino is named after Santo Iago (St. James), one of the Apostles. It comprises a large number of pilgrim routes leading to Santiago de Compostela. Among them is Camino Francés (the French Way) – a 780 km path starting in France.

The Camino has great significance to HAE patients: Dr. Carmen Marcos from Spain was one of the first to report on several HAE Type III families in Galicia. Dr. Sven Cichon from Switzerland has traced the Factor XII mutation back to the 11th century in Central Europe. It is very likely that ancestors with the mutation traveled the Camino and brought HAE to Galacia.

The price per person for taking part in the HAEi Camino Walk is 200 EUR. This price includes: Bus from and back to Madrid; bus during all three days of walking; guide for the whole trip (including the walks); four nights at hotels (in shared double rooms) including breakfast; travel insurance. Not included: Lunches and dinners.

Please note: If you prefer a single room the price is 295 EUR.

The price per person is based on one bus with 55 people – and HAEi has a maximum of two buses (110 people). Everyone signing up for the trip must be ready to participate in the walk. There will be a contingency plan to accommodate anyone suffering from an HAE attack, injury or exhaustion.

The HAEi Camino Walk is presented as part of the 2016 HAE Global Conference - and you sign up for the walk via the conference website. The conference website is expected to be live by mid December 2015. All subscribers to the HAEi newsletter will be notified via email with a link to the registration website.

HAEi Recommends Testing for HAE in All Family Members

There is clear evidence supporting the requirement that every family must be tested when a person is diagnosed with HAE. Approximatey half of all HAE patients suffer from at least one attack of swelling in their throat during their lifetime. What's more, recently published data shows that the risk of death from suffocation due to throat swelling is considerably higher in undiagnosed HAE patients.

HAEi has developed important family testing educational materials for patients and physicians regarding family testing. You can download these documents from the the HAEi website or directly from the links:

Supporting Family Testing: **Talking to Your Family About HAE**

http://haei.org/wp-content/uploads/2015/07/SFT_Patient_Part-1-Talking-to-your-family-about-HAE-0715.pdf

Supporting Family Testing: **The Test and Your Results**

http://haei.org/wp-content/uploads/2015/07/SFT_Patient_Part-2-0715.pdf

Supporting Family Testing: **Talking to Your Patients about Testing for HAE**

http://haei.org/wp-content/uploads/2015/07/SFT_Physician-0715.pdf

Ask the Doctors

Earlier this year the US HAE Association implemented a process for answering patient's questions about HAE. Physician/Scientists at the US HAEA Angioedema Center at the University of California San Diego field questions and the answers are posted on the Facebook pages for Angioedema Center Facebook Page and the US HAEA.

Below, Dr. Sandra Christiansen, Dr. Marc Riedl, and Dr. Bruce Zuraw answer a recently asked question.

If a child has tested negative (ages 5-10) should they be retested? If so, how much after the first testing?

Dr. Christiansen: It is critical to have children tested for HAE if there is an affected parent. Families can then establish an effective treatment plan to 'be prepared'. For type I HAE this would involve checking a C4 level and C1 inhibitor protein level while for type II HAE it would involve checking a C4 level and C1 inhibitor functional level. It is important that samples be handled properly in particular for the C1 inhibitor functional testing; the chromogenic assay is the most accurate, but is subject to falsely low values if the sample is not processed correctly. These studies should be reliable after the age of one. If done correctly there is actually no need for a repeat but patients and physicians may want to check 'one more time' just to be sure. At this juncture for the HAE with normal C1 inhibitor I would recommend checking for the factor XII mutation if that is positive for the parent. We do not have other testing which provides a marker for this type of HAE at present. There are many encouraging developments however so 'stay tuned'.

Dr. Riedl: I agree that laboratory testing for C1INH deficiency is generally quite reliable after one year of age. So testing done in this specific case should be accurate. There are certain situations where one might consider repeat testing after normal results if a child is showing symptoms strongly suggestive of HAE, particularly if there is a family history of C1INH deficiency. There are also some specific nuances to C1INH functional testing. One of the assays used may rarely misclassify function as 'low' or 'normal' – essentially the test is wrong a very small percentage of the time. So for families with Type II HAE (normal C1INH levels but low C1INH function) or when the overall lab result profile (C4, C1INH level, C1INH function) is conflicting, a repeat round of testing may be important. However, these circumstances arise due to issues with the laboratory tests themselves, not due to changes in the child's C1INH protein levels or function. Those C1INH levels are pretty much set and stable by age 1. If retesting is performed, it's generally advisable to wait a month between tests.

Dr. Zuraw: I'd like to stress two points. First, that a normal C4 level does not exclude the possibility of HAE type I or II. There can be so much variation in normal C4 levels that one occasionally does see normal C4 between attacks. Second, the C1 inhibitor testing required can be tricky for type II HAE where the protein level is not helpful and the 'usual' test can occasionally give a falsely normal result. So, the full answer really depends on what testing had been done previously. There are theoretical reasons to believe that there could be SERPING1 mutations that cause HAE (that is the C1 inhibitor fails to inactivate plasma kallikrein or factor XII) but do not result in abnormalities in C1 inhibitor levels or function (because it does inactivate C1). While such a mutation has not yet been found, the converse (a mutation that results in abnormal C1 inhibitor function but does not result in angioedema because the inhibitor does inactivate plasma kallikrein and factor XII normally) has in fact been found. This possibility has led to some suggestions that we change how C1 inhibitor function is measured, using an assay for inhibition of the contact system rather than the complement system.

HAE News from Around the Globe

Brazil **www.abranghe.org.br**

In June 2015 Sanofi Aventis – the laboratory that produces the drug Ladogal (Danazol) in Brazil – informed the Ministry of Health that it would stop manufacturing the drug in the doses of 100 mg and 200 mg, this due to change in the manufacturing site.

This has caused great concern, since Brazilian HAE patients use Danazol continuously, as prophylaxis for crisis. Danazol is provided free of charge by the government to patients. However, it is already missing in almost all public sectors in Brazil.

HAE Brazil has asked the competent authorities to deal with it as a priority in order to avoid total shortage of the drug on the market and health risks to patients of HAE. HAE Brazil is monitoring the situation.

Canada **www.haecanada.org**

The most recent patient event took place in Calgary, Alberta 17 October 2015. Dr. Tom Bowen was one of the physician speakers for this event that was undertaken in partnership with NRBDO Alberta. Events are also being planned for Winnipeg and Victoria in 2016.

Switzerland **www.hae-vereinigung.ch**

The 14th meeting for the members of the Swiss HAE organization took place on 6 November 2015 in Olten.

Macedonia **www.haemacedonia.mk**

Access to medication

The first access to HAE medication happened in October 2015 but with no clear regulation about how patients access it; named patient or needs basis. There are only two hospitals in Macedonia that treat HAE, one for children and one for adults. Ruconest and Berinert are available, with Berinert mostly being for children. The two hospitals are in Skopje, so patients have to travel there for treatment. There is currently a clinical trial for Ruconest and this helps with access at the moment.

Working together I

The countries near Macedonia have similar difficulties in not having access to medicines. HAE Macedonia has applied for a grant to have a regional meeting with patients as there is a physician in each of the countries, aware of patients and wanting to do something. In Albania and Slovenia as well as in Serbia, Montenegro, Croatia, and Kosovo there is great interest in doing a patient meeting.

Working together II

HAE Macedonia is working with the National Alliance of Rare Diseases to build its reputation and seek access to medications. Most patients can only access therapy via clinical trial, private donations or individual fundraising.

Patient registry

The hospitals and health regulatory body are positive towards a patient registry, but there will be challenges around how to collect substantial and accurate data. HAE Macedonia hope to build a list to give to the Minister of Health.

Germany **www.angiooedem.de**

Sponsoring sports teams

CSL Behring recently supported the Adendorfer EC ladies ice hockey club with an donation. Using this donation, the team has been equipped with two brand new sets of jerseys featuring the logo of HAE-Vereinigung e.V., that is the German HAE association. HAE Germany is convinced that this is a great support of the HAE awareness efforts.


Lokales

Selbsthilfegruppe als Sponsor

Vereinigung für seltene Erkrankheit spendiert Eishockey-Damen neue Trikotsätze

HAE Germany is also supporting a youngsters handball team in Eschweiler with new jerseys, featuring the organization's logo. This team has an especially good reason for helping in the awareness work since one of the team members is suffering from HAE.

Angioedema meeting

HAE Germany held the 20th meeting of the German Society for Angioedema (Deutschen Gesellschaft für Angioödeme) in Mainz on 18 November 2015.

Three-country-meeting

On 28 November 2015 HAE Germany will be arranging a three-country-meeting, this time in cooperation with Uniklinik München.

Colombia **www.facebook.com/pages/Asociación-Colombiana-de-Angioedema-Hereditario/119669588091359**

One more South American country has been added to the HAEi world map as Colombia has joined the family. The HAE contact in Colombia is Jackeline Sus Moreno who can be contacted at:

jacksus2@hotmail.com.

If you want to take a closer look at the HAE organization in Colombia, please visit the national Facebook group at

www.facebook.com/pages/Asociación-Colombiana-de-Angioedema-Hereditario/119669588091359.

Poland **www.hae.org.pl**

Courses for patients

Over 2014-2015 HAE Poland has organized 9 self-administration courses for subcutaneous and intraveneous injection in six cities with around 150 attendees. The courses aimed to train patients to self-administer so they don't have to waste time going to and being at the hospital. Over the next two years, HAE Poland aims to create 12 regional centers with key expertise – and from November-December 2015 self-administration courses will be starting in the 12 cities.

Information book

In the first quarter of 2016 HAE Poland plans to publish a book with important information for patients, doctors and caregivers as awareness and knowledge is the major issue.

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Denmark, Norway and Sweden **www.haescan.org**

The first Scandinavian HAE conference since the foundation of HAE Scandinavia took place on 6-8 November 2015 in Nyborg, Denmark, and gathered close to 100 people from Denmark, Norway, and Sweden.

Friday evening began with an exciting talk on "A path to genetic treatment of HAE" by Professor Jacob Giehm Mikkelsen, Aarhus University, Denmark – and Saturday's program began on the same quality level, since the first man on the podium was Professor Marcus Magerl from Charité Berlin, Germany. He spoke under the headline "All you need to know about HAE", after which Anthony J. Castaldo, President of HAEi, reported on "HAE Around the World". The morning also featured an introduction to "HAE calendar app" by Professor Anette Bygum and Marie-Louise Krogh, both from Odense University in Denmark.

Only fitting for a Scandinavian conference there were briefings on the situation in the three member countries – by Anette Bygum (Denmark), HAE Scandinavia board member Trine Dahl Johansen (Norway), and Associate Professor Patrik Nordenfelt (Sweden). Moreover, there were room for an update from HAE Scandinavia as well as strong, personal patient stories from Sweden's Nathalie Eriksson and Norwegian Benedikte Hellevik.

The conference, which was conducted with support from CSL Behring and Shire, ended Sunday with the General Assembly of HAE Scandinavia.

Clockwise: HAE Scandinavia ready for the general assembly – HAEi Executive Director Henrik Balle Boysen and HAEi President Anthony J. Castaldo – HAEi President Anthony J. Castaldo and Professor Markus Magerl, Charité University Hospital, Berlin – The venue for the first HAE Scandinavia Conference was spectacular.

Slovakia

From Milos Jesenak (Center for HAE in Martin, Slovakia www.unm.sk) and Katarina Hrubiskova (Center for HAE in Bratislava, Slovakia) HAEi has received this information:

Slovakia is a country in eastern central Europe with a population of over 5 million people. The exact data about the prevalence of HAE are missing, however, effective therapies are available (human plasma derived C1-INH concentrate, recombinant rabbit C1-INH, icatibant). According to the general prevalence, there should be at least 100-300 patients with HAE in Slovakia. Recently, the national study about the number of HAE patients was performed through the questionnaire-based survey in Out-patient clinics for allergy and clinical immunology. Based on the recieved questionnaires, there are all together 87 living patients with HAE (aged 35.93 ys.; 43% males) from 39 families. 15 patients already have died due to suffocation. The most prevalent form was HAE type I (87%). The diagnostic delay between the first symptoms and diagnosis estimation was 8.60 ys. In the majority of the patients (68%) a combination of various clinical symptoms (skin, gastrointestinal, laryngeal, genital) was observed. However, 17% patients suffered from isolated skin symptoms, 6% from gastrointestinal and 3% from laryngeal angioedemas. Five patients are still asymptomatic. Regarding the prophylaxis, 42% were without prophylactic treatment, 47% took attenuated androgens, 8% tranexamic acid, and 3% either pd-C1-INH or rh-C1-INH. Acute attacks were treated in 30% with danazol, in 26% with icatibant, in 22% with pd-C1-INH and in 22% with rh-C1-INH. The survey is still ongoing.

The creation of a national HAE registry as well as a Slovak HAE patients' organisation is planned.

Peru www.facebook.com/ AngioedemaHereditarioPeru

There is now a formal national HAE organization in Peru with Suzet Lam Torres as President and Rosario Lam as Vice President. There is no website yet but you'll find the most recent information on HAE in Peru at www.haei.org. You can also follow HAE Peru at www.facebook.com/AngioedemaHereditarioPeru.

USA www.haea.org

2015 is the "Year of the Youth" for the US HAEA. The recent Patient Summit in Denver had a strong focus on youth and the Medical Advisory Board is in the process of publishing an expert opinion guideline for managing children with HAE. In late October, 980 patients responded to a 17 question HAEA designed survey that collected data on variety of key HAE topics. The data is currently being analyzed and will be submitted to a medical journal for publication.

Argentina www.aehargentina.org

By now HAE Argentina knows 420 patients, which is around 40 percent of the expected total patient population. The organization has continued with self-infusion workshops, including two for young people. In Buenos Aires a center for diagnosis of HAE. An iPhone app for patients has been developed. HAE Argentina is arranging regional workshops, typically wih 15-20 patients participating.

The organization is working to build a formal registry in the country, however there seems to be challenges due to animosity amongst the physicians.

Spain www.angioedema-aedaf.org

Website

The website of HAE Spain is currently being updated and it is expected to be the first one hosted under the HAEi umbrella.

Medication

Berinert, Cinryze, and Firazyr are available in Spain, while there is no price agreement for Ruconest. Stanozolol (attenuated androgen) is no longer being marketed in Spain.

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Australia **www.haeaustralasia.org.au**

Guidelines

HAE Australia has raised its profile with the government as well as the members and is working with Rare Voices Australia on a communiqué for the government to pass guidelines on how someone with a rare disease is treated and gets access to medicine.

Reimbursement

The decision on reimbursement for C1 (Cinryze/ Berinert) is expected the month. Currently only Firazyr is reimbursed in Australia – and it has recently been passed for reimbursement in New Zealand.

New patients

For **hae day :-)** 2015 HAE Australasia worked with a communications company and secured TV, magazine and newspaper coverage around the day. A couple of new patients were found through this.

Quality of Life Survey

Patients attending the 2015 HAE Australasia Patient meeting held in Sydney were invited to participate in a quality of life survey conducted by Professor Connie Katelaris. Her findings indicate that although the situation for those living with HAE has improved, the survey reveals the continuing burden of living with this condition. Twenty two participants at the annual conference in May 2015 gave consent and completed a questionnaire. The survey looked at family history, attack triggers, and numbers of days lost from work. Patients current medication use and the use of on demand treatments is also discussed. To read the full summary of this important survey:

http://www.haeaustralasia.org.au/sites/default/files/public/documents/HAE%20survey%20report%202015.docx

Meet Up in Brisbane

The meetup for the patients in and around the Brisbane area – another great opportunity to get together to have a chat over lunch – took place 17 October 2015.

Meet Up in Western Australia

29 November 2015 Western Australia HAE patients and carers are invited to attend a Meet Up in East Fremantle. This is an opportunity to meet with others affected by HAE, share stories and hear and learn more about HAE. The guest speakers are A/Prof Richard Loh (HAE in pregnancy and children), Health & Wellbeing Coach Kathy Stratford (stress management and meditation techniques), and Dr. Dominic Mallon (general information on HAE). Morning tea, lunch and afternoon tea will be provided free of charge.

Japan **www.haej.org**

The Japanese Ministry of Health Labor & Welfare has announced that BioCryst's BCX7353 is one of six products designated under the ministry's new 'Sakigake' fast track review system. The Sakigake Designation System promotes R&D in Japan, aiming at early market availability for innovative pharmaceutical products. This designation provides for additional interactions with the regulatory agency in Japan from early development through filing, prioritized development and review, and introduction of the product as soon as possible to address a serious unmet medical need.

Patients meetings

HAE Japan – currently with 370 diagnosed patients registrered – has held three patient meetings over the last year: One with doctors, one very fruitful study meeting with patients and doctors, and one meeting around hae day :-) in May – and the organization is planning a Christmas party on 6 December 2015 in Tokyo.

Access to medication

In Japan, HAE patients can only access one treatment, Berinert, in designated university hospitals. Doctors insist on CT scans before giving medicine even if the patients are in agony – much more awareness is needed.

Raising awareness

HAE Japan expect to be putting together a patient survey sometime during November. At the same time the organization is working on an update of the website – and making contact with hemophilia patient groups in Japan.

United Kingdom **www.haeuk.org**

Over a hundred members of HAE UK attended the Annual Patient Day 7 November 2015.

The meeting was held in Birmingham and the speakers were mostly from the Birmingham Heartlands Immunology Centre, which has a large cohort of HAE patients. This year's theme was "HAE through the Lifespan".

The first speaker was Dr Scott Hackett, paedriatic immunologist, who gave a very comprehensive presentation on diagnosing HAE in infants and treatment of children from babyhood to adolescence. The next speaker, possibly slightly in the wrong order, was Dr Sarah Goddard speaking on conception and pregnancy followed by a patient story from Izzy Richards who was at the meeting with baby Benjamin (two months old) as evidence of a successful pregnancy.

Dr Hilary Longhurst then gave a detailed and interesting presentation on managing the transition from paediatric services to adult, which can be a difficult time for teenagers because in the UK this move happens at age 16 which is just when they are in the middle of examinations as well. Dr Richard Baretto led the participants through the management of HAE in older patients and showed some very good slides on quality of life. The morning was finished off by Dr Aarn Huissoon who changed the theme with a presentation on drug trials; what possibilities there are in the various company portfolios and the importance of taking part in clinical trials. Nicola Bowen spoke excellently on the patient experience of being involved in trials.

After lunch there was a short presentation by Laura Szutowicz, the new CEO for HAE UK, outlining some events and plans for next year. One of the most exciting items is that Ford have kindly allowed one of their lorries to have panels advertising HAE UK. This was organized by Ray Frappell who works for Ford at Dagenham and it will not only be driving around the UK but it will be going over to the European mainland too. Laura Szutowicz also wants to establish a separate wing of HAE UK specifically for younger patients who will be tasked with designing their own literature and so on.

The afternoon sessions consisted of break out groups with topics similar to the morning and extra HAE and Employment Issues, Women's Issues and Preparing your Child for School. A question and answer session concluded the day.

HAE UK has had a great deal of good feedback from the day and many suggestions to make next year even better – and has already started planning it.

Laura Szutowicz sends her thanks to all the speakers, to the Medical Advisory Panel – Dr Longhurst, Dr Gompels, Dr Crouch, John Dempster, Christine Symons and Fran Ashworth – the HAE UK Trustees John Price, Ann Price, Ed Price and Ann Harding. She also thanks everyone who helped to make the day a success, especially Rachel Annals "without whom I would have been completely at sea!"

The final excitement for the Patient Day was the launch of the HAE UK A&E cards. These are a credit card sized cards that will be presented any time the patient has to attend an emergency unit. They give brief details of the patient, HAE type and detail appropriate medication. Hopefully, these will prevent some of the lack of knowledge and slow treatment that many of the members have faced in the past.

Let us know what happens in your country

Please send all relevant information about activities in your country to our Communications Manager Mr. Steen Bjerre at

s.bjerre@haei.org

The State of Management in Europe

HAEi's recently published report "2015 State of Management of HAE in Europe" concludes that more must be done to help HAE patients in Europe. The report notes that basic access to life saving therapies still eludes patients in many European countries because of deficiencies in disease recognition along with barriers to treatment approval and reimbursement.

HAEi believes that broader access to modern therapies can reduce the pain, suffering, and disability associated with this serious disorder, and dramatically increase quality of life. The report confirms that there is still much to do to ensure that all HAE patients in Europe have access to modern HAE therapy, so that they can control their symptoms, feel safe, and fulfill their life's potential at school, work, and in their relationships.

The report includes a call to action that asks for:

- Governments, health authorities and health professionals to recognize that HAE is a serious disabling, potentially life-threatening and chronic condition that must receive timely, accurate diagnosis and effective treatment.
- Professional education that enables health professionals to recognize HAE symptoms, diagnose the condition, and appropriately treat attacks, and understand the importance of specialist referral and ongoing care.
- Public and patient education that increases HAE awareness, and encourages patients to;
  - Seek information on currently available treatment options
  - Form a partnership with their HAE treating physician
- A treatment plan that meets each patient's individual needs considers home treatment as a viable option and follows the HAE Modern Management Principles.
- Co-operation among stakeholders – patients, scientists, specialist doctors and industry – to continue research, including clinical trials, to improve treatment and ultimately to find a cure for HAE.
- Co-operation among national governments, regulatory authorities and industry to ensure continuing and where appropriate, improved access to available treatments.

Please feel free to download the 2015 version of the report here:

http://haei.org/wp-content/uploads/2015/11/HAEi_EU_SoM_Report.pdf

Social Media

HAEi is growing steadily on the social media. At the moment there are 1,474 subscribers to this newsletter – and here a some other quite interesting numbers:

HAEi Facebook group –1,597 members
http://www.facebook.com/groups/172320032283

HAE Awareness Day Facebook page – more than 13,000 followers
http://www.facebook.com/haeday

HAEi LinkedIn profile – 141 followers
https://www.linkedin.com/company/3363107?trk=tyah&trkInfo=clickedVertical%3Acompany%2CclickedEntityId%3A3363107%2Cidx%3A1-1-1%2CtarId%3A1447156956819%2Ctas%3Ahaei

HAEi Twitter account – 309 followers
https://twitter.com/haeday

HAEi YouTube channel – 29 subscribers
http://www.youtube.com/user/haedaychannel

HAEi Executive Committee Meeting and Workshop

The HAEi Executive Committee met for the 2015 Meeting and Workshop in Frankfurt, Germany on 30 October – 1 November. It was an intense and successful weekend where the strategic direction of HAEi was discussed and agreed upon. The Executive Committee members are all committed to bring HAEi further and to establish ways of helping patients with HAE no matter where they live.

Vice President Michal Rutkowski, Poland – EC member Sarah Smith-Foltz, Spain – Project Manager Deborah Corcoran, United Kingdom – Secretary Alejandra Menendez, Argentina – EC member Beverley Yamamoto, Japan – EC proxy Natasha Angjeleska, Macedonia – EC member Peter Hermeling, Germany – Treasurer Fiona Wardman, Australia – EC member Jørn Schultz-Boysen, Denmark – President Anthony J. Castaldo, USA – Communications Manager Steen Bjerre, Denmark – Executive Director Henrik Balle Boysen, Denmark

Global Advocacy Work

Recent activities

30 October-1 November The HAEi Executive Committee met in ***Frankfurt, Germany***

6-8 November HAEi participated in and spoke at the first HAE Scandinavian conference that took place in ***Nyborg, Denmark***.

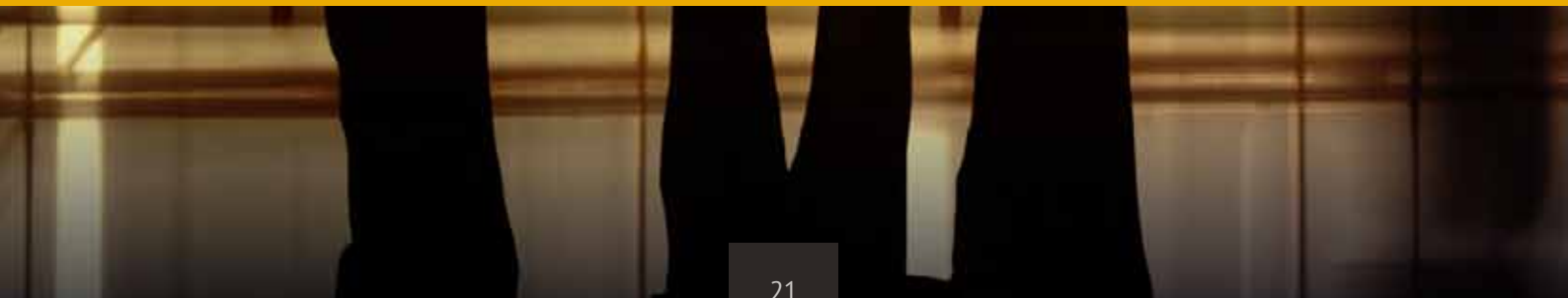
13-15 November HAEi participated in and spoke at the 2015 Shire Global HAE Forum in ***Madrid, Spain***.

Future activities

27-30 November HAEi will participate in and speak at an HAE educational meeting for physicians, nurses, and patients in ***Iceland***.

11-13 December HAEi is planning on participating in the Middle East Asthma, Allergy and Clinical Immunology Conference in ***Abu Dhabi, United Arab Emirates.***

14-15 January HAEi will participate in the Plasma User Group (PLUS) Consensus Meeting in ***Estoril, Portuga***l.



Clinical Trials

According to the International Clinical Trials Registry Platform under World Health Organization (WHO) and clinicaltrials.gov under the U.S. National Institutes of Health the following trials are recruiting at the moment:

First-in-Human Study to Evaluate the Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of BCX7353 in Healthy Volunteers

Recruiting in United Kingdom.

https://clinicaltrials.gov/ct2/show/study/NCT02448264

Safety of Ruconest in 2-13 Year Old HAE Patients

Recruiting in Germany, Israel, Italy, Macedonia, Poland, and Romania.

https://clinicaltrials.gov/ct2/show/NCT01359969

Firazyr® Patient Registry Protocol (Icatibant Outcome Survey - IOS).

Recruiting in Austria, Brazil, Denmark, France, Germany, Greece, Ireland, Israel, Italy, Spain, Sweden, and United Kingdom.

https://clinicaltrials.gov/ct2/show/NCT01034969

12-Week Safety and Efficacy Study of BCX4161 as an Oral Prophylaxis Against HAE Attacks OPuS-2

Recruiting in Belgium, Canada, France, Germany, Hungary, Italy, United Kingdom, and USA.

http://clinicaltrials.gov/show/NCT02303626

Safety and Efficacy Study of Cinryze for Prevention of Angioedema Attacks in Children Ages 6-11 with HAE

Recruiting in Germany, Mexico, Romania, United Kingdom, and USA.

http://clinicaltrials.gov/show/NCT02052141

A European Post-Authorisation Observational Study Of Patients With HAE

Recruiting in Belgium, France, Germany, Italy, Spain, and United Kingdom.

http://clinicaltrials.gov/show/NCT01541423

C1 Inhibitor Registry in the Treatment of HAE Attacks

Recruiting in the Netherlands.

http://clinicaltrials.gov/show/NCT01397864

A Pharmacokinetic, Tolerability and Safety Study of Icatibant in Children and Adolescents With HAE

Recruiting in Argentina, Australia, Austria, Canada, Colombia, Germany, Hungary, Israel, Italy, Spain, and USA.

http://clinicaltrials.gov/show/NCT01386658

Study to Assess the Tolerability and Safety of Ecallantide in Children and Adolescents With HAE

Recruiting in USA.

http://clinicaltrials.gov/show/NCT01832896

A Phase 2 HAE Prophylaxis Study With Recombinant Human C1 Inhibitor

Recruiting in Canada, Czech Republic, Macedonia, the Netherlands, Romania, and United States.

https://clinicaltrials.gov/ct2/show/NCT02247739

Screening Protocol for Genetic Diseases of Mast Cell Homeostasis and Activation

Recruiting in United States.

https://clinicaltrials.gov/ct2/show/NCT00852943?term=hereditary+angioedema&rank=62

Pathogenesis of Physical Induced Urticarial Syndromes

Recruiting in United States.

https://clinicaltrials.gov/ct2/show/NCT00887939?term=hereditary+angioedema&rank=63

An Open-Label Study of Icatibant in Japanese Subjects with Acute Attacks of HAE

Recruiting in the Japan.

http://www.shiretrials.com/sitecore/content/studies/clinicaltrialsen/2015/05/14/06/44/shp-fir-301?sc_lang=en

Pilot Study of the Safety and Efficacy of Oxandrolone in the Prevention and Treatment of Malnutrition in Infants.

Recruting in USA.

https://clinicaltrials.gov/ct2/show/NCT01048632?term=hereditary+angioedema&recr=Recruiting&rank=11

These trials are not yet recruiting but are expected to do so soon:

Study to Evaluate the Clinical Efficacy and Safety of Subcutaneously Administered C1 Esterase Inhibitor for the Prevention of Angioedema Attacks in Adolescents and Adults With HAE.

https://clinicaltrials.gov/ct2/show/NCT02584959?term=hereditary+angioedema&recr=Not+yet+recruiting&rank=1

HAE, Neurobiology and Psychopathology

Will be recruiting in Italy.

https://clinicaltrials.gov/ct2/show/NCT02159430

Efficacy and Safety Study of DX-2930 to Prevent Acute Angioedema Attacks in Patients With Type I and Type II HAE.

https://clinicaltrials.gov/ct2/show/NCT02586805?term=hereditary+angioedema&recr=Not+yet+recruiting&rank=3

HAE Papers

Here are summaries of some of the recently published HAE related scientific papers:

HAE in Swedish Adults: Report From the National Cohort – by P. Nordenfelt et al., Department of Internal medicine, Pulmonary medicine and allergology, Jönkoping, Sweden:

A total of 146 patients were identified; 110 adults and 36 children with HAE type I or II, giving a minimal HAE prevalence of 1.54/100,000. Adult females reported 19 attacks in the previous year vs. 9 for males, and they reported 10 days of sick leave vs. 4 days for males. For all treated acute attacks, plasma-derived C1-inhibitor concentrate (pdC1INH) had a good effect. For maintenance treatment, 43% used attenuated androgens and 8% used pdC1INH, which reduced their attack rate by more than 50%. *(Acta Derm Venereol., November 2015)*

Safety and efficacy of C1 esterase inhibitor for acute attacks in children with HAE – by W. Lumry, AARA Research Center, Dallas, Texas, USA, et al.:

Human plasma-derived nanofiltered C1 esterase inhibitor (C1 INH-nf) is used to treat acute HAE attacks, but data regarding use in children are sparse. Patients 2 to <12 years of age, body weight ⩾10 kg, with a diagnosis of HAE type I or II, were recruited for a multicenter open-label trial. Nine children were treated: 3 (10-25 kg) received 500 U; 3 (>25 kg) received 1000 U; and 3 (>25 kg) received 1500 U. Median time to unequivocal symptom relief was 0.5h. Treatment of a single attack with C1 INH-nf doses of 500 U (in patients 10-25 kg), 1000 U, and 1500 U (in patients >25 kg) were well tolerated. Doses of C1 INH-nf <1000 U may be appropriate in some pediatric patients. *(Pediatr Allergy Immunol., November 2015)*

The Levels of the Lectin Pathway Serine Protease MASP-1 and Its Complex Formation with C1 Inhibitor Are Linked to the Severity of HAE – by C.B. Hansen, University Hospital of Copenhagen, Denmark, et al.:

The levels of the lectin complement pathway serine proteases MASP-1 and MASP-1/C1-INH complexes are reduced in HAE patients compared with controls. Both MASP-1 and MASP-1/C1-INH complexes are related to the degree of complement C4 consumption, as well as the severity of disease. These results suggest that MASP-1 may exert a previously unrecognized role in the pathophysiology of HAE. *(J Immunol., October 2015)*

Phase II study results of a replacement therapy for HAE with subcutaneous C1-inhibitor concentrate – by B.L. Zuraw, University of California, San Diego, USA, et al.:

Long-term prophylaxis with twice-weekly intravenous injections of plasma-derived C1-inhibitor (pdC1-INH) has been established as an effective treatment. Subcutaneous (SC) administration of pdC1-INH has not been studied in patients with HAE. This open-label, dose-ranging, crossover study (COMPACT Phase II) was conducted in 18 patients with type I or II HAE who received two of twice-weekly 1500, 3000, or 6000 IU SC doses of highly concentrated volume-reduced CSL830 for 4 weeks each. After SC administration, a dose-dependent increase in trough functional C1-INH activity was observed. C1-INH and C4 levels both increased. The two highest dose groups achieved constant C1-INH activity levels above 40% values, a threshold that was assumed to provide clinical protection against attacks. Compared with intravenous injection, pdC1-INH SC injection with CSL830 showed a lower peak-to-trough ratio and more consistent exposures. All doses were well tolerated. *(Allergy, October 2015)*

Report on the First Survey of Iranian Patients with HAE – by S. Shahinpour et al., Tehran University of Medical Sciences, Iran:

Among 63 patients who were clinically suspicious for angioedema (23 males and 40 females), 8 cases were diagnosed with HAE – 3 with HAE type 1 and 5 with HAE type 2. The mean ages of HAE type 1 and type 2 patients were 25.6 ± 13.5 and 22.4 ± 12.32 years. The mean age of onset in HAE type 1 group was 8 ± 5 years and in HAE type 2 group was 18.8 ± 11.84 years. The mean diagnosis delay was 17.6 years in HAE type 1 patients and 2.6 years in HAE type 2. The most common clinical manifestation was facial swelling presented in all HAE patients followed by swelling of extremities which was present in 7 patients. *(Iran J Immunol., September 2015*)

HAE Due to C1 Inhibitor Deficiency in Serbia: Two Novel Mutations and Evidence of Genotype-Phenotype Association – by S. Andrejević, Clinical Center of Serbia, Belgrade, Serbia, et al.:

C1-INH-HAE was diagnosed on the basis of clinical and laboratory criteria in 40 patients from 27 families. Disease-causing mutations in SERPING1 were identified in all patients. In C1-INH-HAE type I, we identified 19 different mutations, including 6 missense mutations, 6 nonsense mutations, 2 small deletions, 1 small insertion, 2 splicing defects and 2 large deletions. Two of the mutations are reported here for the first time. All C1-INH-HAE type II patients from three families harbored the same substitution. *(PLoS One, November 2015*)

News from the Industry

28 Oct, 2015

Pharming Group N.V. has presented its unaudited financial report for the first nine months ended 30 September 2015. CEO Sijmen De Vries comments (in extract):

"During the third quarter, the number of prescriptions for our lead product Ruconest® increased by 29%, which indicates more patients are using Ruconest® to deal with their HAE attacks. We expect to see the effect of this increase in prescriptions continuing through the fourth quarter. The acquisition of our US partner Salix by Valeant Pharmaceuticals International in the first half of 2015 has led to a revision in the way Ruconest® is being marketed in the US. Valeant is concentrating sales effort on larger HAE clinics, which deal with significant numbers of patients with acute HAE attacks, the indication for which Ruconest® is approved.

In July, we went live with the "HAEi GAP" (the Hereditary Angioedema International Patient Organization's Global Access Program) in collaboration with Clinigen Group PLC, to provide patients with access to Ruconest® in countries where the drug is not yet commercial available. On behalf of their patients, physicians may request Ruconest® through an ethically and regulation-compliant "Named Patient Program" mechanism. The first requests under this program have already been received by Clinigen.

We have stepped up our research and development activities. We have two ongoing studies: A randomised, double-blind placebo controlled Phase II study for Ruconest® in prophylaxis of HAE, and a Phase II pediatric study for treatment of HAE in young children (2-13 years of age), progressing during the quarter. These studies are expected to finish during the first half of 2016.

After the quarter end, we received notification from the U.S. Food and Drug Administration (FDA) that they have granted Ruconest® extended data exclusivity (from 7 years to 12 years), which means that no bio-similar version of Ruconest® can be approved in the US before July 2026. This should enable us to develop our current pipeline of products to full commercialisation before revenues from Ruconest® come under generic competition."

(Source: Pharming)

2 Nov, 2015

Shire will acquire **Dyax Corp.**, the publicly traded biotechnology company primarily focused on the development of plasma kallikrein (pKal) inhibitors for the treatment of HAE.

Dyax has already successfully developed and commercialized Kalbitor, which is approved for HAE acute treatment in patients 12 years of age and older, and represented an early innovation in HAE treatment. Dyax's most advanced clinical program is DX-2930, a Phase 3-ready, fully humanized monoclonal antibody targeting pKal with proof-of-concept Phase 1B efficacy data. DX-2930 has received Fast Track, Breakthrough Therapy, and Orphan Drug designations by the FDA and has also received Orphan Drug status in the EU. It is expected to enter Phase 3 clinical trials by year-end 2015.

Shire CEO Flemming Ornskov, M.D., commented: "This highly complementary transaction aligns with and accelerates our strategy to build a global leading biotechnology company focused on rare diseases and specialty conditions. It adds to our portfolio of best-in-class therapies addressing unmet needs in our core therapeutic areas, expanding and extending our leadership position in HAE. We have closely followed DX-2930's progress in the evolving HAE landscape for some time, and we admire the work of the Dyax team in moving this next-generation therapy forward. Through compelling proof of concept clinical data, this potentially transformative therapy has been shown to be both highly efficacious and convenient, two key product attributes desired by both physicians and patients."

Dr. Ornskov continued, "DX-2930 is a strategic fit within our HAE domain expertise, and we are well-positioned to advance the development, registration, and commercialization of DX-2930 for the benefit of HAE patients. This transaction also offers other potential upside opportunities, including Dyax's early-stage pipeline. Following the close of this transaction, we look forward to welcoming Dyax employees, who will bring to Shire substantial clinical and commercial expertise in HAE. Dyax is to be commended for the world class organization they have built focused on HAE."

Dyax President and CEO Gustav A. Christensen said: "We believe this transaction will deliver substantial value to our shareholders and highlights our shared commitment to bringing innovative medicines to patients who suffer from the devastating effects of HAE. Our approved product, Kalbitor, was an important first step to bringing a range of HAE medicines to patients. Shire's expertise and proven rare disease patient identification and management capabilities make it the ideal partner to efficiently bring DX-2930 to HAE patients worldwide. I'm proud of the company that our team has built, and I'm confident that Dyax's important mission and focus on improving the lives of patients will continue as part of the Shire family."

(Source: Shire and Dyax)

5
Nov, 2015

At the presentation of the BioCryst Pharmaceuticals, Inc. financial results for the third quarter of 2015 Jon P. Stonehouse, President & CEO, said: "The completion of enrollment of our OPuS-2 trial of avoralstat and the successful outcome of the Phase 1 healthy volunteer study of BCX7353 have positioned both programs to reach important value creating events in 2016. We expect to initiate the APeX-1 (Angioedema ProphylaXis) proof of concept trial of BCX7353 in HAE patients and to report OPuS-2 results by early 2016. These oral kallikrein inhibitors have the potential to revolutionize HAE treatment, providing patients the ability to lead normal lives."

(Source: BioCryst)

9
Nov, 2015

Dyax Corp. presented two oral presentations describing clinical data from its DX-2930 Phase 1b study at the American College of Allergy, Asthma, and Immunology (ACAAI) Annual Meeting. DX-2930 is an investigational fully human monoclonal antibody inhibitor of plasma kallikrein (pKal) being developed for the prevention of HAE attacks.

Burt Adelman, M.D., Executive Vice President of Research and Development and Chief Medical Officer at Dyax, said: "Collectively, these results support further clinical investigation of DX-2930 as a prophylactic treatment for HAE. We remain on track to enroll the first patient in our Phase 3 clinical trial for DX-2930 for HAE prophylaxis by year-end 2015."

Final Results of a Phase 1b Study demonstrated that DX-2930 was well tolerated at all dose levels. There were no deaths or subject discontinuations due to an adverse event. There were no serious adverse events in subjects treated with DX-2930 and no evidence of dose-limiting toxicity. There was no safety signal in treatment-emergent adverse events, clinical laboratory results, vital signs, or electrocardiograms. Subcutaneous injection was well tolerated.

Post-hoc analyses of the Phase 1b study of DX-2930 in patients with HAE were conducted to determine if the clinical response to DX-2930 was influenced by factors such as historical attack rate, functional C1-INH levels, or the amount of cleaved (2-chain) high-molecular-weight kininogen (HMWK). C1-INH function in HAE patients ranged from 0 to 45% of normal. Neither historical nor observed attack frequency was related to baseline C1-INH levels, and baseline levels of 2-chain HMWK also did not correlate with historical attack rates. As expected, levels of 2-chain HMWK at baseline were inversely correlated with levels of functional C1-INH. It was observed that treatment with 300 or 400 mg DX-2930 reduced levels of 2-chain HMWK in a dose- and time-dependent manner, and these reductions were not influenced by historical attack rate or baseline C1-INH function. All subjects in the 300 and 400 mg dose groups with high baseline attack rates were attack-free during the Day 8 to 50 assessment period.

(Source: Dyax)

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.

HAEi Worldwide

Currently you will find HAE member organizations in 49 countries:

North America (2): Canada, United States of America

Central America (3): Costa Rica, Mexico, Puerto Rico

South America (8): Argentina, Brazil, Chile, Colombia, Ecuador, Peru, Uruguay, Venezuela

Europe (25): Austria, Belarus, Belgium, Bulgaria, Croatia, Czech Republic, Denmark, Finland, France, Germany, Hungary, Ireland, Italy, Macedonia, Norway, Poland, Portugal, Romania, Slovenia, Spain, Sweden, Switzerland, The Netherlands, Ukraine, United Kingdom

Middle East (3): Israel, Turkey, United Arab Emirates

Africa (1): Kenya

Central Asia (1): Russia

South Asia (1): India

East & Southeast Asia (3): China, Japan, Malaysia

Australia/Oceania (2): Australia, New Zealand

You will find much more information on the HAE representations around the globe at www.haei.org. On our World Map you will find contact information for our member organizations as well as care centers, hospitals, physicians, available medication, and clinical trials.

The information on www.haei.org is being updated as soon as we receive fresh data from the national member organization.

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 or Communications Manager Steen Bjerre. 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.

Corporate Information

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

HAEi
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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