



Issue 8 · December 2015

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# HAEi Newsletter



2016

## Happy Holidays

With this last newsletter of the year, we wish you a joyous holiday season and sincere best wishes for a healthy new year

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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 8 · December 2015**

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## A Message from the President

### Dear HAEi Friends,

Welcome to the 8th and final HAEi newsletter for 2015. This is a very special time of the year that brings us closer to loved ones and friends as we celebrate holidays and look forward to a happy and healthy new year. On behalf of the HAEi Executive Committee, I wish you a joyous holiday season and sincere best wishes for a healthy new year.

Gift giving is a special holiday season tradition that we all enjoy. During the next several weeks, I kindly ask you to pledge a very important gift for the greater good of your family and the HAE community – a few hours of your time.

The HAEi member organization in your country sponsors meetings and has a variety of projects and tasks that need help from volunteers. We are blessed in that effective medicines are currently available and even better therapies are on the horizon.

Companies are taking the risk to test new medicines because it is widely recognized that HAE patients are united and part of a well-organized international community that continues to grow in numbers and fiercely advocates for improving patient quality of life.

Your gift of time and involvement in your country's HAE group will contribute to the very things that distinguish us among rare disease patients organizations – consistent growth, visibility, and effectiveness of HAE advocacy activities.

Finally, we cannot forget the cold, hard fact that we have much to accomplish in 2016. Our assessment of the international HAE landscape reveals that patients in many parts of the world remain undiagnosed or misdiagnosed and have little or no access to modern life saving HAE medicines.

Surprisingly, less than optimal situations for HAE patients exist in all regions of the world. For example, HAEi's recently published 2015 *State of Management of HAE in Europe* concludes 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."

Significant progress can be made in solving these problems. HAEi looks forward to helping our member organizations expand their program offerings while maintaining aggressive patient advocacy activities.

Warm holiday regards to all,

Anthony J. Castaldo  
 President, HAEi





## Helping to change the lives of those with HAE

This year HAEi launched the first HAE medication access program, called HAEi Global Access Program (GAP). Below, HAEi Executive Director Henrik Balle Boysen and GAP Project Manager Deborah Corcoran discuss this exciting new initiative.

### Where did the idea for HAEi GAP come from?

**Henrik:** There are challenges for all patients living with rare diseases and access to medicine is one of the greatest. Despite the advances in recent years, there are still so many countries around the world with no access to modern HAE medications at all. Our advocacy work is helping to change this, but we wanted to be able to deliver a global solution for HAE patients now. Well – yesterday, really.

About 18 months ago I saw a presentation that described how it is possible to supply medicine to a country where that medicine isn't specifically licensed, and that there were different ethical and compliant ways you can do this. At that time this was only done in cooperation with pharmaceutical companies. We knew this was something we had to look into for HAE – and immediately pursued a way for a patient organization to coordinate such efforts.

### It sounds like HAEi GAP has been thought about for a long time. Tell us about progress so far.

**Henrik:** Behind the scenes there have indeed been a lot of thoughts and plenty of discussion over the years. We have always thought that being able to help with the supply of medication would be a natural and noble thing for us at HAEi.

To deliver HAEi GAP we have partnered with Clinigen Group who are experts in the logistics and ethics of compliant supply of medicines to countries where they do not currently have a license. It will be the team from Clinigen Customer Services that manages requests and, when all the boxes are ticked, supply medication. We're delighted that Pharming Group is the first pharmaceutical company on board and so the first medication available through HAEi GAP is Ruconest®. Ruconest® is the only recombinant C1-INH replacement therapy for the treatment of HAE and it is licensed by both the European Medicines Agency and FDA, that is the US Food and Drug Administration. With these partners on board, we were very excited to officially launch HAEi GAP on **hae day** :-)) this year.

### Could you recap what HAEi GAP is?

**Henrik:** HAEi GAP provides an opportunity and mechanism for physicians in countries where limited or no modern HAE medications are available, to access modern HAE medication for their HAE patients. Currently Ruconest® medication is available through a 'Named Patient Program' in countries where it is not commercially available. A named patient program is where a doctor orders the medication and the cost of the medication is paid for by the government, hospital or the patients medical insurance.

### To establish any global mechanism for access to medicine is a great achievement. What has the response been like so far?

**Henrik:** The response has been very positive. We're delighted to share that Clinigen Customer Services has already had requests for information about Ruconest® as part of HAEi GAP from physicians in many countries around the world. At the same time we have engaged with Deborah Corcoran as our project manager on this exciting project, and hope that we – all together – can push this program forward to the benefit of patients around the world.

### That sounds a great start, where do you want to go from here?

**Debs:** We want to make sure every HAE patient and physician knows about the potential access mechanism that HAEi GAP provides. Where HAEi GAP might be able to help, we hope that we will hear from HAE patients and physicians in those countries. Our ultimate aim is that through HAEi GAP there will be patients able to access modern HAE medication that they don't currently have access to and wouldn't be able to access in any other way.

### So, how can the HAE community help?

**Debs:** If you think that HAEi GAP could help you, please talk to your doctor about the program. Unfortunately, Clinigen Customer Services can only respond to requests from physicians. You can find information to share with your physician in a number of places. And don't forget that HAEi wants to support you and your HAE physician every step of the way with HAEi GAP, so please keep in touch and also let your physician know we are here to help in any way we can.

### What do you hope the future holds for HAEi GAP?

**Henrik:** We will continue to explore opportunities for HAEi GAP with other pharmaceutical companies and hope that we will be able to offer additional HAE medications through HAEi GAP soon.

**Debs:** At the moment, we can only offer medication through a 'Named Patient' basis. In the future we would also like to include Programs where medication can be provided free of charge via donation or accessed as part of a phase III clinical trial.

We will have more articles on HAEi GAP in future editions of this newsletter and on the HAEi website, bringing you the latest updates as they happen.

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#### HAEi website

🌐 haei.org/hae/global\_access\_program

#### HAEi flyer

Download the HAEi information flyer at

📄 <http://haei.org/wp-content/uploads/2015/12/GAP-flyer-A5-1115-FINAL.pdf>





## HAE GLOBAL CONFERENCE MADRID 19-22 MAY 2016

## Registration for the third HAE Global Conference is now open

**HAEi cordially invites you to attend the 2016 HAE Global Conference which will take place in Madrid, Spain 19-22 May 2016.**

Following the great success of the 2012 HAE Global Conference in Copenhagen and the 2014 HAE Global Conference in Washington DC, HAEi looks forward to welcoming you to this third HAE Global Conference. The conference brings together patients, care givers, health care professionals, and also invites industry representatives, to learn more about HAE, share experiences and knowledge in a friendly atmosphere conducted and driven by the patient community through HAEi.

The theme for the 2016 HAE Global Conference is *"Creative Advocacy for Expanding Access to Therapy,"* where the aim continuously is to find ways to improve time to diagnosis, to secure life saving therapies and get funding for these – allowing HAE friends around the world to lead a safer life and fulfill their life's potential.

### Registration for the conference is now open

Registration will take place on a first come, first serve basis – and since HAEi again offers some extremely attractive registration rates and conditions, please do

not wait too long registering for this exciting conference, expected to gather 550-600 people in Madrid.

### Travel grants – IMPORTANT information (please read carefully)

For the 2016 HAE Global Conference HAEi is delighted to offer travel grants for patients and their care givers (relatives). If you are applying for a travel grant, please do so no later than Sunday 3 January 2016 at 16:00 (4:00pm) Central European Time.

In order to be eligible for a travel grant you must a) live in one of the eligible countries (see list below) and be part of the HAE member organization in your country, and b) have the diagnosis of HAE confirmed (or be a near relative to a patient), and c) be a member of the HAEi Family (you are a member if you are receiving the newsletters from HAEi.) If you do not receive these newsletters, please make sure to sign up with HAEi on [www.haei.org](http://www.haei.org) before registering to the conference. The above will be validated before the travel grants can be approved.

In order to apply for a travel grant you need to have a valid passport at the time of registration (this passport must be valid at least until December 2016). You will also need to confirm and prove that you have a valid VISA (if applicable) to Spain for the conference period. Your VISA needs to be in place no later than 3 January 2016. Finally, you will also have to confirm that you have a valid credit card for your trip to Madrid. There is a limited amount available for travel grants, and HAEi therefore has pre-allocated the following numbers (see the brackets) for the eligible countries:

- **North America:** Canada (8), United States of America (40)
- **Central America:** Costa Rica (2), Mexico (20), Puerto Rico (8)
- **South America:** Argentina (8), Brazil (30), Chile (2), Colombia (4), Ecuador (2), Peru (8), Uruguay (2), Venezuela (2)
- **Europe:** Austria (8), Belarus (2), Belgium (8), Bulgaria (2), Croatia (2), Czech Republic (8), Denmark (8), Finland (8), France (14), Germany (20), Greece (8), Hungary (8), Iceland (2), Ireland (2), Italy (14), Macedonia (8), Norway (8), Poland (8), Portugal (8), Romania (2), Slovenia (2), Sweden (8), Switzerland (8), The Netherlands (8), Ukraine (4), United Kingdom (14)
- **Middle East:** Israel (8), Turkey (8), Gulf Region (incl. Saudi Arabia, Kuwait, Oman, Qatar, Bahrain and United Arab Emirates) (8) Africa: Kenya (2)
- **Central Asia:** Russia (4)
- **South Asia:** India (2)
- **East & Southeast Asia:** China (6), Japan (20), Malaysia (2)
- **Australia/Oceania:** Australia (8), New Zealand (8)

Please observe that HAEi will not support travel grants to members from the country hosting the conference. This means that there will be no travel grants for Spanish members registering for the conference.

Once you have registered you will hear from HAEi again between 6 and 8 January 2016. It is VERY IMPORTANT that you ensure that you have daily access to your email during this period. Please also make sure to check your spam folder/junk mail folder for emails from Marine Travel.

**If you are approved** for a travel grant you will receive a suggested travel itinerary from HAEi's travel agency, Marine Travel. You will have to confirm this itinerary no later than 24 hours after it was sent from the travel agency. Otherwise your travel grant will be withdrawn and given to someone else.

**If you are NOT approved** for a travel grant you will be given the option to cancel your registration for the conference – and have your registration fee refunded (please note that the credit card transaction fee will not be refunded). Alternatively, you are, of course, welcome to find other ways of funding your return travel to Madrid and keep your registration for the conference.

Should HAEi have travel grants left after the first round of allocations is complete, these will then be distributed on a first come, first serve basis.

Flight tickets for accepted travel grants must be booked via HAEi and be handled by the HAEi travel agency. If delegates book their own flights, they will not be reimbursed.

Go to the registration website:

<http://www.trippus.net/hae2016-website>

**Please observe:** Registration for the HAEi Camino Walk 2016 (14 – 18 May) will take place in the registration process for the 2016 HAE Global Conference.

Please visit the registration website frequently for the latest information on sessions, exhibitor information and more. HAEi is looking forward to seeing as many of you as possible in May in Madrid.

### REGISTRATION IS NOW OPEN

Go to the registration website at

<http://www.trippus.net/hae2016-website>



## Join the HAEi Camino Walk

**For the 2016 version of the global awareness day hae day :-) HAEi bring together HAE patients, relatives, caregivers, doctors, nurses, and industry for a mutual experience: An HAEi walk on 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) the participants will walk three fairly short legs of the Camino (~20, ~15 and ~13 km), allowing everyone interested to take part. And there will be ample time to see things, go places, rest, and just be together.

The aim of **hae day :-)** on 16 May every year is to raise awareness of HAE among the general public and medical community in order to create an environment in which there is better care, earlier and more accurate diagnosis and knowledge that HAE patients can lead a healthy life.

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 to walk 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. It will serve 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.

### Why the Camino?

The Camino is the name of 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. That is the one HAEi has chosen, or at least a small part of it.

The Camino is named after Santo Iago (St. James), one of the Apostles. According to legend, his remains were buried under what is now the cathedral of Santiago de Compostela. For more than a thousand years millions of people have walked the Camino to visit the disciple.

From an HAE point of view the Camino is also very appropriate: Dr. Carmen Marcos from Vigo in Spain was one of the first to report on several HAE Type III families in Galicia and Dr. Sven Cichon, Switzerland has traced the Factor XII mutation back to the 11th century in Central Europe – and it may very well be that it arrived in Galicia via the Camino.



### Price

The price per person for taking part in the HAEi Camino Walk is 200 EUR. This includes: Bus from/back to Madrid; bus transfer during all 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. Also, you should note that you will have to make arrangements for your hotel stay in Madrid for the night of 18 to 19 May 2016.

### Group size

The price per person is based on one bus with 55 people – and there is a maximum of two buses (= 110 people). Everyone signing up for the trip must be ready to participate in the walk. However, if someone is unable to finish all days due to HAE attack, injury or exhaustion they are obviously free to use the accompanying bus but participants should only come along in order to do at least part of the walk.

### Sign-up

The HAEi Camino Walk is presented as part of the 2016 HAE Global Conference – and you sign up for the walk via this website:

[www.trippus.net/hae2016-website](http://www.trippus.net/hae2016-website)

### Your own Camino

Should anyone wish to do a longer distance, completing a typical Camino stage every day (most are 20-30 km), they would have to make their own arrangements. The same goes for anyone who cannot take part in the whole event but would like to be there for one or two days.

However, HAEi (Steen Bjerre ✉ [s.bjerre@haei.org](mailto:s.bjerre@haei.org)) and AEDAF (Sarah Smith ✉ [s.smith.foltz@haei.org](mailto:s.smith.foltz@haei.org)) would be happy to provide advice and suggestions. They would also coordinate a meeting time and place for these people to join the main group for the walk on **hae day :-)** 16 May or for the descent into Santiago 17 May 2016 – or both.



## 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 provided 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](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](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](http://haei.org/wp-content/uploads/2015/07/SFT_Physician-0715.pdf)

## International HAE nurses' survey

The HAE International Nursing Organization (HAE INO) has put together an on-line survey as they want to evaluate the role of the nurse in HAE care. With this survey they want to find out:

- What nurses actually do in their work with patients and families affected by HAE
- What preparation, training and support they get for this work
- How they work with their colleagues – doctors and other professionals
- How they would like their work to develop

To achieve this, HAE INO hope to have input from HAE nurses around the world before 15 February 2016.

Karin Andritschke from the HZRM Hemophilia Centre Rhein Main in Germany is heading the survey. She says:

“All individual responses will be kept confidential within HAE INO nurses working group. The responses will be analysed by the HAE nurses working group and the results will be discussed with our contact physicians and presented at professional conferences.”

The survey is only intended for nurses working with HAE patients. If you are not a nurse, you are very welcome to forward the link to the survey to a HAE nurse you know.

You will find the survey here:

[www.surveymonkey.com/r/9ZDMVD](http://www.surveymonkey.com/r/9ZDMVD)



## Ask the Doctors

In early 2015 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 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.

*What is the difference between food allergies and food triggers for HAE?*

**Dr. Christiansen:** The simple answer to the question is that a true food allergy is the result of the immune system recognizing the structure of food as an allergen and making allergic antibodies (IgE) to that food. With exposure to what should be a harmless material (food) the sensitized individual will unleash a firestorm of activation of the cells that contain histamine (mast cells) by the network of specific IgE molecules. The release of histamine during these reactions will cause a myriad of symptoms including itching, hives, at times respiratory distress, gastrointestinal symptoms, possible collapse and even death. The second part of the question is more difficult. Attacks of swelling do not relate to histamine but rather the generation of bradykinin. Often the attacks are unpredictable—by nature we always want a reason for something that happens. As the one thing we are always doing is eating it can certainly coincide with an attack. The issue is whether it is truly causal or coincidental. Our reader's question highlights a concern by many patients that there is a link between their diet and swelling. We actually do not have any data that can answer the question. I have had patients tell me that alcohol can be a trigger and others express concern about a variety of foods. I am left with simply telling patients that it is OK to avoid something if it seems to make the swelling worse. I have been teaching allergy fellows for over 30 years. One of my favorite bits of wisdom to pass on is 'listen to the patient and they will tell you the answer'. I think that we have finally started to 'listen' about this dietary issue.

**Dr. Zuraw:** I agree that many patients bring this question up, and therefore it's important to us to obtain solid information about it. The UC San Diego Angioedema Center along with the US HAEA Medical Advisory Board is preparing to conduct a study to see if there are any patterns that link dietary practices to HAE severity. We will be using an anonymous one-time online diet questionnaire to collect this information. While most of us are skeptical that there is an actual link between diet and HAE severity, I feel that it's important not to dismiss the concern and potentially miss this connection if it really exists. Should the diet questionnaire suggest such a link, we would then set up a more rigorous follow-up study that would track the relationship looking forwards (that is, prospectively).



**Dr. Riedl:** The concept of specific foods as triggers for HAE attacks is an interesting area that really hasn't been studied. I certainly hear from some patients that they strongly suspect certain foods are symptom triggers so much so that they've eliminated these foods from their diet. Unfortunately, to date, we don't have any study data to confirm this is a general or predictable trigger in HAE. We also don't have any known biologic mechanism by which this might occur and therefore no available lab tests to look for this trigger factor in HAE. So this is another area ripe for study. Environmental (in this case food) exposure studies are notoriously difficult to design and conduct in a rigorous fashion, but surveys such as the one being planned at the Angioedema Center could point us in the right direction. Food allergy, on the other hand, is generally a histamine-mediated condition caused by food-specific IgE (allergic antibody) produced by the immune system. Because we better understand the mechanism of these reactions, we have fairly reliable means of identifying this allergic antibody through blood or skin testing. This highlights the importance of distinguishing between the pathways of allergic reactions (IgE-mast cell-histamine) and HAE reactions (C1INH-kallikrein-bradykinin) and recognizing these different types of clinical reactions require different treatments.

**Dr. Zuraw:** I agree, but I'd also emphasize that it's important to recognize that there may be additional mechanisms at play here. It would be very easy to show that IgE-mediated reactions to foods are not a significant cause of HAE attacks. By taking a very broad and open-minded approach such as the one we proposed for the study, we run the risk of finding relationships that turn out to be incorrect. That's why I believe that positive findings can only be a starting point, and must be followed by controlled prospective studies.

## HAE News from Around the Globe



United Kingdom [www.haeuk.org](http://www.haeuk.org)

### Status from the CEO

After the excitement of Patient Day, the next event in the calendar of the HAE UK CEO Laura Szutowicz was the UKPIN (UK Primary Immunology Network) meeting. Held in Belfast this year, it was hosted by Dr David Edgar who is chairman of UK PIN and although it is primarily an immunology meeting there was quite a considerable emphasis on HAE this year, with several posters that were on HAE related topics. Also, there was a very interesting presentation from Dr Ania Manson of Addenbrookes Hospital in Cambridge on gene therapy and HAE. But according to Laura Szutowicz the highlight of the meeting was the Shire symposium at which HAE UK member Faye Davenport had been asked to give a presentation. Ruby, her daughter, was diagnosed aged six and is now nine. Faye was diagnosed a little over a year ago. She spoke brilliantly on the impact the diagnosis has made on their lives, not just of her and is extraordinary and many of the clinicians were very moved by her presentation. As one of them said: 'We know about the condition, but we very rarely think of the impact on the person.'

Laura Szutowicz am now in to her sixth month of running HAE UK and she tells us that she has learnt so much and made so many new friends in those six months:

- I have many plans for the future of the organisation, some very long term but others that will be set in place early in 2016. So life promises to continue to be exciting! One of the things I am really looking forward to in 2016 is the Global Conference in Madrid, when I hope to meet and put faces to many of the people that at the moment are names. My first six months with HAE UK would not have been possible without the tremendous help and encouragement I have received from many people. Notable Ann and John Price, who gave me a very thorough grounding in how to run the charity. The other Trustees Ann Harding and Ed Price; our Medical Advisory Panel of Dr Hilary Longhurst, Dr Mark Gompels, Dr Tim Crouch, the Specialist nurses Christine Symons,

John Dempster and Fran Ashworth. Great thanks to Furkhanda Haxton who runs our Facebook page and was wonderfully busy and helpful at our Patient Day; and Rachel Annals, who as well as being our Executive Officer and keeping all the things like the website up and running, is a tremendous source of knowledge and expertise for me to call on. Only remains for me to wish everyone the 'Compliments of the Season' and a very Happy New Year. Forward to 2016!

### Women with HAE

Are you a woman who has HAE? Are you over 18? Then HAE UK and Teesside University invites you to take part in a unique and first of its kind study into HAE. The study aims to gather your experience of attacks and how they have affected your life, your family, your professional life and social life etc., and your experience of living with the condition and how this has affected your family/ family life, your professional life, relationships etc. Furthermore, it is the aim to gather information on your experience of the healthcare system and medication in relation to HAE. Read more about the study at [www.haeuk.org/news-events](http://www.haeuk.org/news-events).



Peru [www.facebook.com/AngioedemaHereditarioPeru](https://www.facebook.com/AngioedemaHereditarioPeru)

Presently there are nine people diagnosed with HAE patients in Peru. There are two care centers, both in Lima. Also, there are a few physicians treating HAE patients. Available HAE medication in Peru is Danazol and tranexamic acid, where there is no access to Berinert, Firazyf, or Ruconest at the moment.



Turkey <https://www.facebook.com/groups/hereditoranjyoodem>



### Meeting in Germany

Representatives from HAE Turkey attended the three-country-meeting by invitation of Lucia Schauf, chairman of HAE Germany. Ersan Sevinç, International Communication Manager of HAE Turkey, writes: "During three days of accommodation in Germany, Mrs Lucia and all the staff of HAE Germany were pretty warm and enlightening. Attending a developed association's meeting gave us a chance of seeing the way how it works properly and it also guides us to how we can help our patients. Furthermore, one of the most significant parts of having been at the meeting was to get familiar with the latest research and development in HAE. It is very important for us to share with our patients what research has been done and what are the newest solutions. This experience helped us very much."

### Travelling School Project

Even though it has been just almost a year since the organization was established, the Travelling School Project of HAE Turkey proceeds steadily. It is the hope of the organization to build an intense awareness of HAE in hospitals and among doctors through the Travelling School Project.



Canada [www.haecanada.org](http://www.haecanada.org)

### HAE Radio

HAE Canada is proud to present the release of the first full episode of the organization's podcast HAE Radio at [www.haecanada.org/hae-radio](http://www.haecanada.org/hae-radio). In the episode HAE Canada posed the question, "What is HAE Canada?" to some of the members and board members. HAE Radio is a collaboration between HAE Canada and HAEi and it is now available on iTunes, Soundcloud and Stitcher. Episode 2 is scheduled to be released on 29 January 2016.

### Upcoming Patient Events

HAE Canada will be holding patient events in various Canadian cities throughout 2016. Information about events in Winnipeg and Victoria will be available on the organizations' website early in the new year.

### Membership Kits

Membership kits have been distributed to all of the HAE Canada members across nation. The kits include information about HAE, the organization and other helpful information.

### Current Membership

HAE Canada has reached 329 members. This represents a 60 per cent increase since 1 July 2014. Membership is free and is open to Canadians with HAE, their family members, friends and health care providers who treat those with HAE. Please visit [www.HAECanada.org](http://www.HAECanada.org) for more information on how to join.

### Volunteer Development Program

Work has been completed on HAE Canada's Volunteer Development Program. The program focuses on creating positive and fulfilling volunteer experiences. Those who have already offered to assist will be contacted early in the new year to confirm their willingness to assist and to schedule their training session.



Japan [www.haej.org](http://www.haej.org)

HAE Japan organized a Christmas party in Osaka on 6 December 2015. In the words of HAE Japan chairman Beverley Yamamoto: "It included a wonderful talk by Dr Ohsawa, delicious cake and plenty of meaningful conversation. It has been a great year for HAEJ."



**Australia** [www.haeaustralasia.org.au](http://www.haeaustralasia.org.au)

#### Meet Up

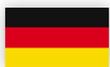
The beautiful view of the Swan River provided the perfect backdrop for the Western Australia HAE meet up held at the East Fremantle Yacht Club on 28 November 2015. As patients and carers came together to meet with others living in this part of Australia, friendships were formed and attendees were educated by the guest speakers.

A/Professor Richard Loh discussed HAE in children with some interesting information of a higher rate of coeliac disease in children with HAE and reiterating the importance of every child with HAE wearing a MedicAlert bracelet at all times. Next up Clinical immunologist Dominic Mallon addressed the group, discussing HAE in adults and pregnancy. He spoke of the hospitals in Western Australia where patients should attend and explained the importance of patients carrying and referring to their HAE ASCIA action plan. The participants also heard from health & wellbeing coach Kathy Stratford who discussed the importance of trying to reduce stress and shared some meditation techniques that patients can learn themselves to use in their times of need.

#### Treasuring the Good Days

Take 5 magazine recently published the story of HAE patient Tania Bogoyovich and her family. You can read their inspirational story here:

[www.haeaustralasia.org.au/treasuring-good-days](http://www.haeaustralasia.org.au/treasuring-good-days)



**Germany** [www.angioedem.de](http://www.angioedem.de)

On 28 November 2015 HAE Germany held a three-country-meeting, this time in cooperation with Uniklinik München. Participants were more than 100 members of the patient organizations in Austria, Germany Switzerland and many renowned experts from German university hospitals. Among them were Prof. Bork who spoke not only about the different forms of angioedema, but also told about the beginnings of his angioedema consultation back in 1974. Among the other speakers were Dr. Martinez-Saquer and Dr. Greve, Dr. Bas and Dr. Ayygören-Pürsün.



**Israel** [www.edema.co.il](http://www.edema.co.il)

Presently there are about 200 registered HAE patients in Israel of a population of approximately

8 million in total. This is possibly due to lack of awareness and diagnoses by primary health care providers.

Up until 2009 the common treatment of the Israeli patients was based on fresh/frozen stored human plasma, anti fibrinolytics (tranexamic acid; Hexacapon), and attenuated androgens (Danzol). Today the available treatments included in the National Health Insurance are human plasma C1 INH (Breinert; Cinryze) and BK antagonist (Firazyf icatibant).

The treatment of Israeli patients is divided between these HAE care centers: Bnei-Zion Hospital in Haifa with an HAE center headed by Dr. Elias Tobey, Sourasky Med. Center in Tel-Aviv with an HAE center headed by Prof. Shmuel Kivity, Sheba Med. Center Tel-Hashomer in Ramat-Gan with an HAE center headed by Dr. Avner Reshef, Rabin Hospital in Petach Tikva with an HAE center headed by Dr. Yael Graif, and Ha'emak Med. Center in Afula with an HAE center headed by Dr. Menachem Rotem.

Each of these centers provide a 24/7 support for patients, families and medical staff, whenever and wherever needed, by outstanding dedicated nurses and doctors.

The Israeli HAE patients' association was formed seven years ago. The goals set by the association include: Centralize all HAE information, patient data and knowledge throughout the country, assist and support patients in any possible way in their negotiations with the authorities, increase awareness and education to HAE within hospitals and primary care clinics throughout the country, and finally to organize education, meetings and training to patients and their families.



**Poland** [www.hae.org.pl](http://www.hae.org.pl)

As planned during the national HAE conference in October, HAE Poland has launched a series of regional patients meetings with self administration courses at each meeting. The aim is to let patients get the knowledge of self infusing (iv and subcut) and to establish or create a foundation for regional HAE centers.

The meetings and courses are organised in 12 hospitals in 12 regions of Poland where there are physicians. Apart from the main HAE center in Krakow HAE Poland hopes to cover the country through regional centers in Warsaw, Poznan, Wroclaw, Bydgoszcz, Olsztyn, Szczecin, Gdansk, Bialystok, Lublin, Rzeszow, Lodz, and Katowice - all being cities with at least one physician with some knowledge about HAE.

21 November 2015 the first meeting took place in Warsaw (15 patients and care givers), followed by a meeting in Poznan 12 December 2015 (28 patients and care givers). Upcoming meetings are in Krakow 16 January,

Wroclaw in February, and Bydgoszcz in March 2016. The HAE Poland partners for these events are CSL Behring, Shire and Sobi.



**Spain** [www.angioedema-aedaf.org](http://www.angioedema-aedaf.org)

#### Website

The Spanish organization (AEDAF) plans to launch its new website between mid- and late December 2015. This is expected to be the first member organization website hosted under the HAEi umbrella. It will be a responsive site adapted to mobile platforms and will have a link to the current AEDAF website, which will remain as a database and source of more detailed country-related information.

#### Patient Workshop

AEDAF held a regional workshop for HAE patients and their families in Pamplona (Navarra) on 26 November 2015. This was the eighth of a series of workshops that began in 2014: Antequera (Malaga), Valladolid, Alicante and Bilbao in 2014, and Talavera de la Reina (Toledo), Oviedo, Santa Cruz de Tenerife (Canary Islands) and Pamplona in 2015. These meetings are held at a regional level, targeting members and non-members of AEDAF in areas outside Madrid and at a distance from the hospitals considered as reference centers that treat HAE, to provide updated information on the State of Management of HAE and the latest association news, followed by a roundtable discussion on patient concerns and needs and the situation in their regions. The next workshop - sponsored by Shire - is expected to be held in Tarragona in January 2016.

#### 2016 Global Conference and the HAEi Camino Walk

AEDAF will be actively involved in the planning leading up to the 2016 HAE Global Conference in Madrid (19-22 May 2016) and to the HAEi-AEDAF sponsored HAEi Camino Walk (14-18 May 2016).



**Iceland** <http://haei.org/location/hae-in-iceland>

Late November 2015 Iceland was country no. 50 to join the HAEi family. The HAE contacts in Iceland are Reynir Thor Eggertsson [✉ reynir.thor.eggertsson@mk.is](mailto:reynir.thor.eggertsson@mk.is) and Ingibjörg Omarsdóttir [✉ ingacastaldo@simnet.is](mailto:ingacastaldo@simnet.is). There was an inspiring interest from physicians, nurses, patients and their care givers to participate in the first gathering ever in Iceland. Prof. Björn Ludvíksson and his nurse Hildur Sveinsdóttir have been a great resource for HAE patients in Iceland - and with the involvement of Reynir and Ingibjörg the future is looking very promising for patients in this beautiful country as well.



HAEi Executive Director Henrik Balle Boysen (middle) with HAE patient group leaders in Iceland, Reynir Thor Eggertsson (left) and Ingibjörg Omarsdóttir (right).



**Greece** [www.facebook.com/groups/HAE.GREECE](http://www.facebook.com/groups/HAE.GREECE)

While we are at new countries: Please help us in welcoming Greece as HAEi member no. 51.

The Greek contact person is Stavroula Labiri [✉ haegroup.greece@gmail.com](mailto:haegroup.greece@gmail.com) and you can follow the new group via this Facebook link:

[www.facebook.com/groups/HAE.GREECE](http://www.facebook.com/groups/HAE.GREECE)



## Viktar's story

On the HAEi website at [www.haei.org](http://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.

One of the most recent stories is from Viktor Lebedz, Belarus. He thinks that children's disorders give parents additional impulses to go ahead and find a way to protect the new generation from what the older ones have had to go through. This is his story:

### *Just a few years ago I was alone*

I have been suffering from and trying to understand HAE for over 25 years of my life. During this period I have had many sudden swellings, unnecessary treatments and even surgery. The first attack I remember was a swelling on my feet. I was a four-year-old kid and it was painful for me to walk so I crawled from one room to another. As a child I liked active games, but almost every accidental hit to my forehead caused an acute facial edema with my eyes closing and lips inflating to unbelievable size. Often my hands were swollen, too.

***At that point in your life you weren't diagnosed – in fact no one in your family were. How did you and your family react to attacks from a disease you did not know what was?***

Naturally the repeating cases of an unknown allergy – combined with the absence of effective medicine – made both my family and me more cautious. However, the pleasure of playing together with friends was much more valuable, so the attacks would continue. During my school years I frequently suffered from stomach pain, usually with vomiting and weakness.

These were the symptoms of abdominal edema but at that time it was diagnosed as gastritis and in one case unfortunately also as appendicitis. My father Josef had similar symptoms and laryngeal attacks, too. The hereditary form of our illness was evident to everybody, but no one knew what was wrong with us and no one could stop the swellings.

***Your laryngeal edemas started in puberty.***

Yes, during those years I had one to two per year. They limited me when it came to traveling – I couldn't very well be far away from a medical center. Well-known to the physicians at the intensive therapy, I was usually intubated whenever I went to the hospital with a throat attack.

***How did the attacks affect your ability to study?***

Due to the episodes of swellings and stomach pain, I missed quite a number of my classes at school and later at the university as well. Fortunately I was able to catch up with the lessons I missed, so the disease hasn't affected my performance seriously. In addition, I was lucky enough not to miss my exams. The attacks usually appeared right after them.

In 2002 my son Nikita was born. Knowing that my attacks had appeared already in infancy and not seeing the same symptoms on Nikita, we – that is my wife Luda, the whole family and I – hoped that the child hadn't inherited his father's rare and dangerous condition. Well, that lasted until 2009.

***But before then something you characterize as "a happy coincidence" happened.***

Yes, at the eve of the New Year 2005 – at the age of 25 – I suffered a particularly bad laryngeal attack. Afterwards I couldn't see for a few days and I hardly spoke and walked for a couple of months. An involuntary shaking in my hands still reminds me of that episode. It was during this hospitalization that I met Dr. Valentina Zhemoitiak – a pediatrician and medical scientist, who got to know about me by what was indeed a happy coincidence. She identified my symptoms, diagnosed HAE and ultimately changed my life by prescribing steroids, the only treatment option at the time – and that helped me.

***And what about other members of your family?***

My father was diagnosed right after me – and a few years later my younger sister Olia. She had neither visible swellings, nor periodic abdominal pain till the age of 26 when her first attacks appeared. Her disorder showed it could be hidden for years.



***And Nikita?***

Well, one morning – he was six years at the time – Nikita woke up with one of his hands swollen. I remember he asked me why his arm didn't move, why it had occurred, why it happened to him, if it would happen in the future, how to stop it and so on. It was not easy to answer these questions and properly explain what was going on, trying not to frighten the child. A week later Nikita's HAE diagnosis was confirmed.

***It is only fair to say that Nikita's diagnosis was a turning point for you, isn't it?***

Indeed. It is true that children's disorders give parents additional impulses to go ahead and find a way to protect the new generation from what the older ones have had to go through. At that time I began researching HAE on the Internet.

Soon I learned of others who had HAE and realized I was not all alone with this condition. I came across the HAEi website and it provided me with so much information. Shortly after this, I created [www.hereditary-angioedema.org](http://www.hereditary-angioedema.org), a HAE-related website in Russian that gives Russian-speaking people of the former USSR a chance to learn about the



symptoms and treatment, share their personal stories and receive support. Sharing words of encouragement is especially important for patients who live in places where treatment for acute attacks is not available.

In 2010 we established the organization “Save Immunity” in Belarus. It unites patients with Primary Immunodeficiency as well as with HAE. Together we set up a connection between patients and their relatives, medical centers, pharmaceutical companies and the government.

The year after I met Henrik Boysen, the Executive Director of HAEi. It is only fair to say that it was a meeting that transformed my life as I became deeply involved in the international HAE activities. From 2012 to 2014 I was member of the HAEi Executive Committee and I met lots of great people who are raising awareness of HAE throughout the world.

***So Belarus joined the international HAE community.***

We did – and eagerly so. Soon we installed special relationships with the HAE organization in our neighboring country Poland and its President, Michal Rutkowski. For me Poland is a very good example for many others national patients’ communities of how to improve things.

My HAE friends from Belarus, Poland, the Czech Republic, Norway, Denmark, France, the United States, Hungary, the United Arab Emirates, Ukraine, Russia and many more now are my HAE family. Understanding of this is priceless when you realize that just a few years ago I was all alone with an unknown hereditary disorder.

***How did the United States get into the picture for you – and why?***

It so happened, that in 2014 my family and I got a chance to move to the US. The availability of effective

HAE medication was our major reason for moving, really. Now, we live in Fort Wayne, Indiana and my son, who just recently entered his adolescence period with all possible dangerous HAE consequences, finally has access to intravenously delivered plasma derived C1-inhibitor. That really is invaluable.

The move to the United States gave me the possibility to join the US HAEA community that has provided great activities and an incredible patients’ support. With the assistance of HAEA President Anthony Castaldo, Patient Services Team Member Sherry Porter, and Nurse Reimbursement Manager Nikia Davis we found an HAE physician as well as the appropriate health insurance plan and the people from US HAEA have made our entry to the community very smooth.

***But you are still involved in HAE Belarus?***

For sure I am. Thanks to modern means of communication it is easy to be in touch despite geographic location and time difference. Together with other patients, physicians and volunteers from Belarus we have developed a set of activities to improve HAE diagnosis – that really is the major problem – and support HAE patients.

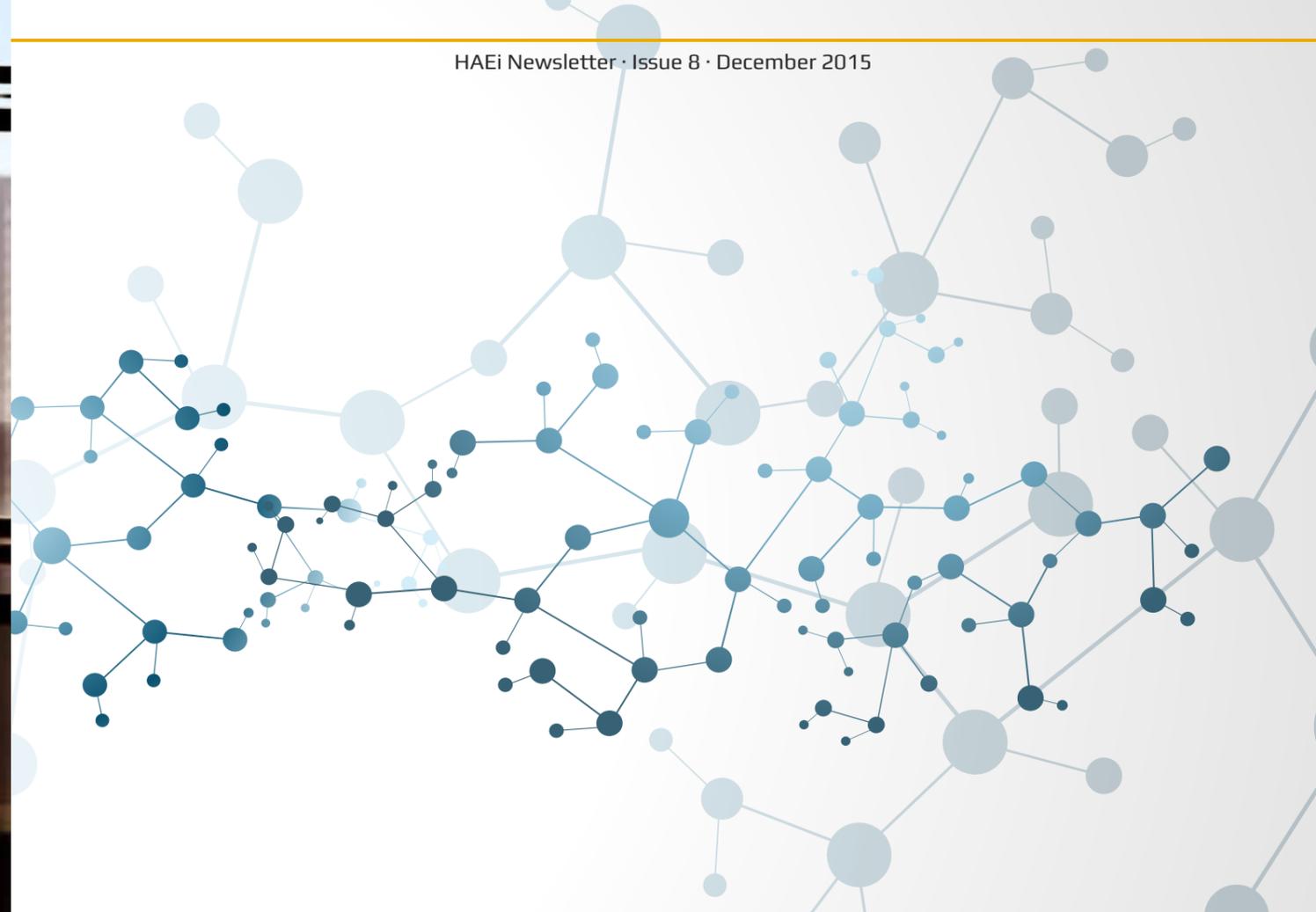
One of our actions in HAE Belarus is a story about an exclusive HAE patient – the beautiful handmade doll Nadia who suffers from HAE and is looking forward to effective medication available in Belarus. If you want to meet Nadia you should go to [www.haedoll.org](http://www.haedoll.org).

As one of the most recent efforts HAE Belarus arranged the first HAE scientific medical workshop in Minsk, Belarus. It took place in April 2015 and was among many others joined by the renowned American HAE scientist, Prof. Bruce Zuraw who gave a wonderful lecture to Belarusian allergologists and immunologists about HAE, its symptoms, modern diagnosis and treatment. I want to believe that all these and future steps and efforts will gradually improve the situation with HAE in my native country.

***And how is the HAE situation in your family in Belarus now?***

Well, at the moment the youngest generation with HAE in my family is my lovely one-year-old niece Julia. Recently her blood was tested in the lab and it has been confirmed that she has the same gene mutation as mine. So far she has no symptoms, but we have to be prepared for the illness that could wake up.

I really wish that there was some sort of “miracle” that could stop HAE attacks. It would provide HAE patients and their families with a sense of protection. Until we get there I will do my best to raise awareness of HAE.



## Global Advocacy Work

### Recent activities

**27-30 November** HAEi participated in a HAE educational meeting in Reykjavik, Iceland, coordinated between Prof. Björn Ludviksson, Nurse Hildur Sveinsdottir and HAEi. There was significant interest in the meeting from physicians, nurses, and patients. During the meeting, HAEi met with patients who have formed a member organization in **Iceland**.

**11-13 December** HAEi participated in the Middle East Asthma, Allergy and Clinical Immunology Conference in **Abu Dhabi, United Arab Emirates**. Here it was important to spread the word about the HAEi Global Access Program. Also HAEi met with Regional Representative Rashad Matraji to discuss the format and dates for the 2nd Gulf Region Workshop, which will take place in March 2016 in **Doha, Qatar**.

### Future activities

**14-15 January** HAEi will take part in the Plasma User Group (PLUS) Consensus Meeting in **Estoril, Portugal**.

HAEi is currently also planning a meeting with the patient organization in **Turkey** to discuss various activities, including the current state of management in the country.

## HAE Papers

Here are summaries of recently published HAE related scientific papers:

### **Pediatric HAE: Onset, Diagnostic Delay, and Disease Severity – by SC Christiansen, D Davis, AJ Castaldo and B Zuraw, USA:**

HAE typically presents in childhood. Large gaps remain in our understanding of the natural history of HAE during childhood. We examined age of onset, delay in diagnosis, androgen exposure, and their influence on ultimate disease severity in a large cohort of patients with HAE. Median age of first swelling was 11 years with a median age at diagnosis of 19 years. Our observations highlight the importance of early HAE diagnosis and suggest the necessity of a disease management plan once the diagnosis has been made. (*Clin Pediatr (Phila)*, November 2015)

### **Icatibant for Multiple Hereditary Angioedema Attacks across the Controlled and Open-Label Extension Phases of FAST-3 – by W.A. Lumry, AARA Research Center, Dallas, Texas, USA, et al.:**

In randomized, controlled, double-blind, multicenter phase 3 studies, one icatibant injection was efficacious and generally well tolerated in patients with a single HAE attack. Here, the efficacy and safety of icatibant for multiple HAE attacks was evaluated across the controlled and open-label extension phases of the For Angioedema Subcutaneous Treatment (FAST)-3 study. Icatibant was efficacious and generally well tolerated across multiple HAE attacks, including laryngeal attacks. (*Int Arch Allergy Immunol.*, November 2015)



## Clinical Trials

According to the International Clinical Trials Registry Platform under World Health Organization (WHO) and [clinicaltrials.gov](http://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 Czech Republic, 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 United States.

<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, Israel, Italy, Mexico, Romania, United Kingdom, and United States.

<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>

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

Recruiting in United States.

<http://clinicaltrials.gov/show/NCT01832896>

### A Phase 2 HAE Prophylaxis Study With Recombinant Human C1 Inhibitor.

Recruiting in Canada, Czech Republic, Italy, Macedonia, 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](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.

Recruiting in United States.

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

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

Recruiting in United States.

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

*These trials are not yet recruiting but is expected to be 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>



## News from the Industry

### PHARMING

7  
Dec, 2015

The European Medicines Agency (EMA) has recently renewed the marketing authorization for Ruconest for an unlimited period. Ruconest, the first and only recombinant (non-blood-derived) C1-esterase inhibitor replacement therapy, was first approved by the EMA in June 2010 for the treatment of acute attacks of HAE. Such initial marketing authorisation is normally issued initially for five years, and reviewed for extension after these first five years.

The recommendation of the Committee for Medicinal Products for Human Use (CHMP) for renewal of the marketing authorization is based on the positive patient benefit-risk profile for Ruconest for the treatment of HAE attacks.

In addition, Pharming reports that Rucovita, the Company's patient support programme, is now available to eligible HAE sufferers in Austria, Germany and the Netherlands. Rucovita aims to provide patients with timely on-demand therapy at their home or other specified location to assist them in dealing with their HAE disease and optimize their HAE treatment. The on-demand, on-location care services are provided in Germany and Austria by homecare organisation Atlantis Healthcare Deutschland GmbH and in the Netherlands by Dutch homecare organisation Eurocept B.V. through their country-wide networks of community-based specialised nurses. Eligible HAE patients can enrol in Rucovita through their physician.

Sijmen de Vries, the CEO of **Pharming Group N.V.**, commented: "We welcome the positive conclusions reached by the EMA in extending the marketing authorization for Ruconest. We are also very excited that we have been able to organize and initiate Rucovita. This round-the clock, seven days a week "on-demand, on-location" care programme is accessible for every eligible HAE patient in Austria, Germany and Netherlands. We know that by investing in offering HAE patients such care to treat acute attacks with Ruconest, we will be able to help them improve their quality of life and limit the impact of HAE on their lives by treating an attack as early as possible."

(Source: Pharming)

### CSL Behring

10  
Dec, 2015

**CSL Behring** is opening operations in Russia in order to provide more patients with greater access to treatment. This is particularly significant in Russia where the healthcare system has some unmet needs for state-of-the-art biotherapies and blood plasma products.

CSL Behring CEO and Managing Director Paul Perreault, said the new office enables CSL Behring to partner more closely with the Russian Federation, healthcare providers, patient groups and the scientific community. He noted CSL Behring will be closer to patients, listen to them more carefully, and better understand their medical needs. The company currently has seven products registered in Russia.

"It is this process of listening and engaging that enhances our ability to deliver new and innovative medicines that make such a huge difference in people's lives," Perreault said. He added that it will now be easier to launch products in Russia and offer new therapy options to doctors and their patients.

In addition, CSL Behring is investigating opportunities to contribute to the development of the Russian pharmaceutical industry, and identify the best ways to partner with the Russian government. As an example, because the amount of human plasma that is currently collected in Russia is insufficient to meet the growing demand for protein-based medicines, Perreault said it may be possible to transfer CSL Behring's plasma collection technology to Russia, and initiate toll manufacturing in that country.

(Source: CSL Behring)

### Shire

15  
Dec, 2015

In 2016, the **Shire** plc pipeline will include more than 10 programs in Phase 3 trials and compounds already under regulatory review. Shire's planned Phase 3 programs for 2016 include SHP-616 (Cinryze) for subcutaneous delivery for HAE prophylaxis, SHP-616 (Cinryze) for HAE prophylaxis in Japan, and Firazyr for HAE in Japan.

Pending the approval by Dyax shareholders and the close of the proposed acquisition by Shire of Dyax Corp., Shire's Phase 3 pipeline would also include DX-2930. A long-acting injectable monoclonal antibody for HAE prophylaxis, DX-2930 has the potential to lower rates of HAE attacks and significantly improve patient convenience based on clinical trial data reported to date. DX-2930 received Fast Track, Breakthrough Therapy and Orphan Drug designations by the FDA and Orphan Drug status in the EU.

(Source: Shire)

## survey

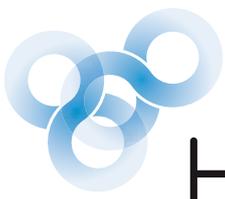
### Newsletter readers' survey

In order to make this newsletter still better HAEi would very much like to know your answer to a few questions. It will take you maximum two minutes but never the less be very valuable for the further development of the newsletter.

Please check the survey at

<https://www.surveymonkey.com/r/YPMMBS3>





# 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

## HAEi Worldwide

Currently you will find HAE member organizations in 51 countries:

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

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

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

**Europe (27):** Austria, Belarus, Belgium, Bulgaria, Croatia, Czech Republic, Denmark, Finland, France, Germany, Greece, Hungary, Iceland, 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](http://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](http://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:

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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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