

# Global perspectives:

HAEI MAGAZINE · ISSUE 1/2018



**The 2018 HAE Global Conference  
welcomes you to Vienna, Austria**



### Dear HAEi Friends,

The time is fast approaching when our HAEi patient and caregiver community will be heading to Vienna, Austria for the 2018 HAE Global Conference. As in the past, we will also welcome health care providers, scientists who study HAE, and representatives from the pharmaceutical industry. The program this year promises to be the best ever as we gather to meet the challenge put forth in the theme for our time in Vienna, "Take Control of HAE." HAEi friends can be assured that plenty of time will be allocated for sharing experiences and insights with participants from throughout the world. Of course, the conference will not only be about business – we have planned some fun and interesting activities!

In Vienna, we will convene a General Assembly to elect five Executive Committee (EC) members. I would like to personally thank the entire HAEi EC, especially those whose initial term has been fulfilled. Each of these EC members – who have filed for reelection – have served HAEi with distinction and have made significant contributions to improving the lives of HAE patients. Our EC is comprised of truly remarkable HAE advocates whose tireless work not only yields extraordinary results in their home countries but provides HAEi with the steady, experienced, proven, and mature leadership essential to the success of a global organization. Never ones to be satisfied with the status quo, however, this EC always pushes for more efficient and creative ways to expand the services and level of engagement provided to our member organizations.

A heartfelt thanks to EC members looking to be reelected for their selfless, dedicated, and highly effective service! In addition, I wish best of luck to all of the candidates who are participating in the forthcoming election!

As you will glean by reading the pharmaceutical company press releases that appear in the last section of this magazine, clinical research for new HAE therapies continues at an astonishingly high level. HAEi friends can thank themselves for this fortuitous situation! Over the years we have created the elements that attract industry interest in developing therapies – an organized, global group of motivated patients willing to participate in clinical trials and physicians with the experience and capability to fulfill research protocols.

While we are encouraged by the prospects for new medicines, we cannot forget and must focus on the plight of patients in countries that have either limited or no access to modern HAE therapy. My dear friends, the solution to this difficult situation is not easy, but as you will see and hear in Vienna, is very possible through forming a steadfast and committed advocacy organization! HAEi member organizations that have successfully taken control of HAE will tell their stories in Vienna, and we will make sure that this information is captured and available for viewing on the HAEi website.

I look forward to personally welcoming each and every HAEi friend at the 2018 HAE Global Conference!

Warm regards,

Anthony J. Castaldo  
President, HAEi

#### Cover photo

The Spanish Riding School  
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HAEi is registered as a non-profit organization in Switzerland



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

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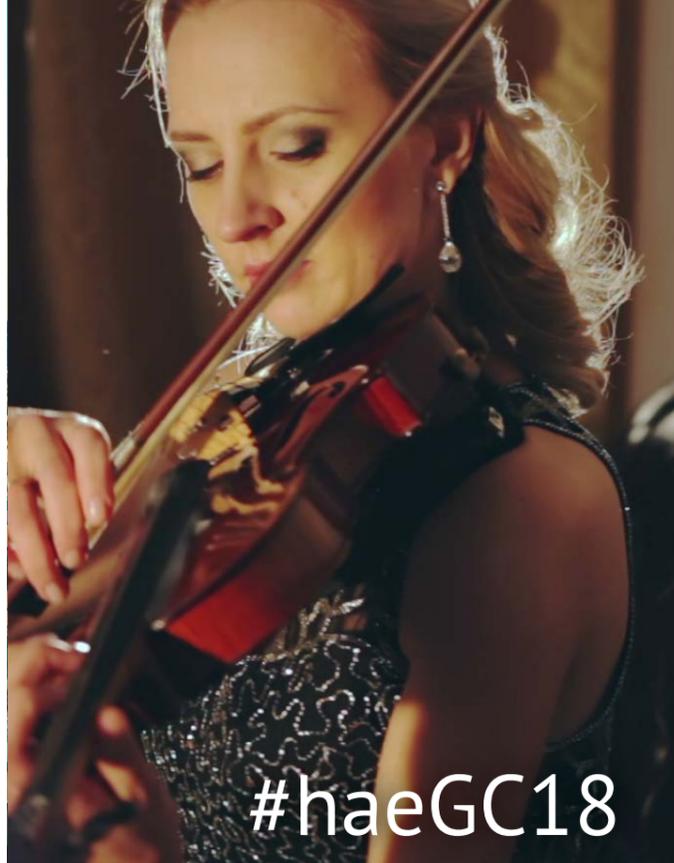
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#haeGC18




**HAE  
GLOBAL  
CONFERENCE  
VIENNA  
17-20 MAY  
2018**



## 2018 HAE Global Conference

Following the great success of the 2012 HAE Global Conference in Copenhagen, the 2014 HAE Global Conference in Washington DC, and the 2016 HAE Global Conference in Madrid, HAEi looks forward to welcoming the participants to the 2018 HAE Global Conference in Vienna, Austria, 17-20 May 2018.

“This our fourth HAE Global Conference will once more bring together patients, care givers, health care professionals, and industry representatives to learn more about HAE, share experiences and knowledge in a friendly atmosphere conducted and driven by the patient community through HAEi”, says HAEi President Anthony J. Castaldo.

The theme for the 2018 HAE Global Conference is “Take Control of HAE”, where the aim continuously is to find ways to improve time to diagnosis, to secure lifesaving therapies and get funding for these – allowing HAE friends around the world to lead a safer life and fulfill their life’s potential.

“At this point we have more than 600 people signed up for the conference. As registration is open until 1 May 2018 (or until we reach our limit), we expect to gather some 650 people in Vienna”, says HAEi Executive Director Henrik Balle Boysen. “Like the previous years registration takes place on a first come, first serve basis.”

HAEi recommends that anyone interested in the HAE Global Conference visit the registration website at [www.trippus.net/hae-global-conf-2018](http://www.trippus.net/hae-global-conf-2018) frequently for the latest information on sessions, exhibitor information and more.

**REGISTRATION IS STILL OPEN**

Go to the registration website at [www.trippus.net/hae-global-conf-2018](http://www.trippus.net/hae-global-conf-2018)

Photos: ©WienTourismus/Peter Rigaud, Lois Lammerhuber, Robert Osmark

# News from the Regional Patient Advocates

The Regional Patient Advocates (RPAs) continue their work supporting organizations in their region, and HAEi is extremely thankful for their hard work. To help them in their roles HAEi organized an intensive training weekend in Frankfurt, Germany in February 2018. Four of the RPAs were able to attend and learn more about HAEi resources as well as enhancing their skills to help them support member organizations in their region even more. There is so much going on in regions that it is only possible to update you on the activities of a select number of countries in this magazine. More information about the RPAs is available on [www.haei.org](http://www.haei.org).



**Patricia Karani**  
Sub-Saharan Africa



## IN GENERAL

In many countries, there is no awareness of HAE, and it is very difficult for patients to be diagnosed and treated. I have been quite busy establishing contacts in a number of countries and will continue to reach out to individuals around Africa, who I hope will raise awareness of HAE and who I will also be able to support with future initiatives and projects.



## KENYA

I am pleased to say there have been many exciting updates in Kenya. Firstly, I was invited to meet with the then Cabinet Secretary for the Ministry of Health, Dr. Cleopha Mailu, alongside other patients with rare diseases. Coming from a genetics background, Dr. Mailu was very supportive of our ideas and agreed to be the guest of honor at our Rare Disease Day 2018 event. This was a great success with over 80 people in attendance. We are also lucky that Dr. Kibet, who is the Chairman of the Kenya Haemophilia Association, has agreed to use his knowledge and experience to

help get HAE Kenya officially recognized. I hope will help us raise awareness and improve the treatment of patients across the country. We also held the First African Doctors HAE Seminar in Kenya in January 2018. The event was a great success with 28 doctors and specialists in attendance.

I am delighted to share an interview I conducted recently for a national TV program, where I talked about HAE. You can see this on the new HAE Kenya twitter page: [www.twitter.com/HAEKenya](http://www.twitter.com/HAEKenya).



## BENIN

I have been in communication with a doctor in Benin who is interested in helping us establish a patient support group for HAE patients in the country. He is currently reaching out to other doctors who could be interested in supporting our cause. After we have established these contacts, we hope to take the next steps to create a patient support group, which would be hugely significant for HAE patients in Benin.



**Natasa Angeleska**  
South East Europe



## IN GENERAL

I have been busy with all the countries in my region, and there have been many exciting developments so far this year. The RPAs and HAEi have been working with the member organizations to translate the HAEi Emergency Card into a number of languages including Macedonian, Turkish, Bulgarian, and Romanian and these will soon be available via [www.haei.org](http://www.haei.org). I have also been supporting patient registration for the 2018 HAE Global Conference in Vienna in May, and I hope that we will have representatives from a number of the countries in my region for the first time.



## MACEDONIA

Following meetings with the Ministry of Health, I was invited to present to the Rare Disease Committee to support the need for increased quantities of HAE treatments for patients in Macedonia. Dr. Vesna Grivceva Panovska, who is now a member of the committee, was a great help and also provided information to the committee to support this need. The meeting was a huge success, and we managed to secure the approval of around four times the available quantity of HAE medication in Macedonia in 2018 compared to 2017. Although there have been some issues with supply of this medication, this is still fantastic news which we hope will improve the lives of many HAE patients in Macedonia. HAE Macedonia, as a member of the National Alliance for Rare Diseases in Macedonia, organized the celebrations for Rare Disease day at the end of February. As part of the celebrations, there were official speeches, and the 'Special Blood' documentary was shown for the guests.



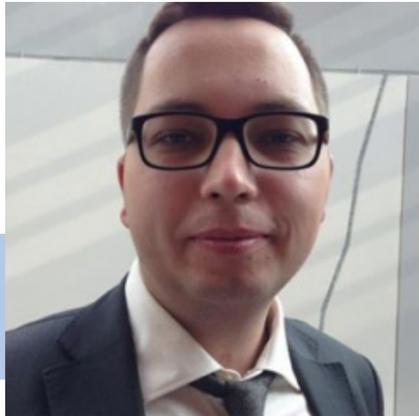
## SLOVENIA

In Slovenia, there have been some barriers preventing a patient organization specifically for HAE being registered. As a result, the patient representative Teja Iskra is in contact with rare diseases societies and is hoping to have HAE officially supported by one of these organizations. Teja Iskra was also invited to speak at the rare disease society's 4th National Conference on Rare Disease Day 2018 in February), which is a great step in raising awareness of HAE.



## BULGARIA

Bulgaria held a screening of the 'Special Blood' documentary for Rare Disease Day as part of their public awareness activities. A workshop is planned for the end of March, where medical specialists from all over the country will come to learn about HAE and how to properly diagnose and treat patients. Dr. Maria Staevska is one of the doctors presenting at this meeting, and we hope it will help to raise awareness about HAE in emergency departments across Bulgaria. We will also be delivering special brochures about the emergency treatment of HAE attacks to all emergency departments in the hospitals in the country.



**Michal Rutkowski**  
Central and Eastern Europe



### IN GENERAL

I have been busy in the last few months as the HAE community in my region is expanding. There are many motivated patients, caregivers, and physicians working to improve the lives of HAE patients in their countries. Despite some obstacles, the work continues, and we have had some great successes.

### BELARUS

We are very excited to announce the successful registration of the HAE patients association in Belarus, called the Belarusian National Public Organization for HAE Patients' Care. This has taken several years to do and means there is now a registered association for HAE patients and their relatives. Volha Puhach, an HAE patient and caregiver, was elected as the very first President, and we will be working closely with her to try to gain access to treatments for HAE patients in the country. I feel very positive about this and hope it will only be a matter of time before treatment is available. I have also been busy helping to organize the HAE session at the 5th International Conference of Immunology Diseases that will take place on 19-21 April 2018.



### KAZAKHSTAN

Kazakhstan is one of the newest countries to join the HAEi family in my region, and the biggest goal for us is to create a registered HAE patients' organization. Yekaterina Tatarenko, a patient, and Sergey Morozov, a caregiver, have been working hard to raise awareness of HAE, especially amongst healthcare professionals. They have been very successful and gained large amounts of support, so they are now in a position to begin the process of establishing a national HAE patients' organization – this is fantastic news.



### CZECH REPUBLIC

In the Czech Republic, there is a very good system in place to provide access to treatments for HAE patients, which has been established with the help and commitment of many dedicated physicians. The Association of Patients with Immunodeficiency HAE/AE is the registered patient organization, run by Mrs. Iveta Maselli and her son. We continue to work with the HAE family in the Czech Republic to raise awareness of HAE and reach out to patients.



**Javier Santana**  
Latin America



### IN GENERAL

There have been many exciting developments in the Latin American region, and I am immensely proud that HAEi can help in so many ways. I am pleased to say that HAEi is now hosting the HAE national association's websites of Peru, Ecuador, and Uruguay and we also hope to add Costa Rica to this list soon. Hosting information under [www.haei.org](http://www.haei.org) reinforces the credibility of the associations, and this link to the international organization has been beneficial in many situations.

### CHILE

In Chile, HAE is now officially covered by the Ricarte Soto Law, which means the government subsidizes the cost of medicines for patients with few resources and no health insurance. This is a groundbreaking achievement for the HAE patient community after years of advocating for access to healthcare. HAEi is proud to have assisted and supported HAE Chile in this process, especially with HAEi President Anthony J. Castaldo's video endorsement at the annual conference of HAE Chile.



### COSTA RICA

HAE Costa Rica recently completed the application to have the organization officially registered in the country. They also marked rare disease day with an event for physicians and shared a press release, fact sheet and infographics with participants. Along with a number of other stakeholders, HAE Costa Rica is part of a group working to explore national initiatives for rare diseases. HAE Costa Rica is very proud to be the lead in ensuring that patient voices are present and heard during these discussions.



### PANAMA

Through the wonderful efforts of Dr. Melcina Pino, a number of HAE patients who are interested in organizing a Panama HAE association have been identified. Dr. Pino would hope to continue to be a medical advisor to the association and is supporting the efforts of completing the documents to have the new organization officially recognized.

## Roles of the Regional Patient Advocates

- Supporting the member organizations already in place
- Assisting in setting up new groups in countries with no existing organization



**Rashad Matraji**  
Gulf Region and Middle East



**IN GENERAL**

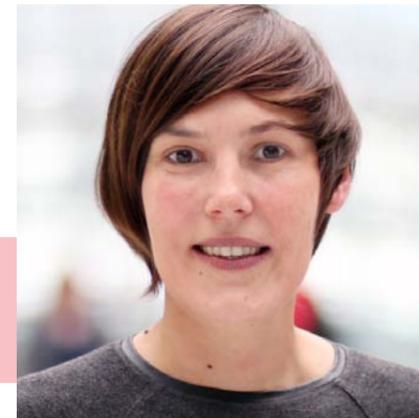
It has been a very exciting time for me as I appeared on *The Quest*, an adventure TV show in Dubai. It was a great opportunity to tell my story and raise awareness of HAE. After the appearance, my story was reported in *The National*, one of the most well-known publications in UAE.

 **UNITED ARAB EMIRATES**

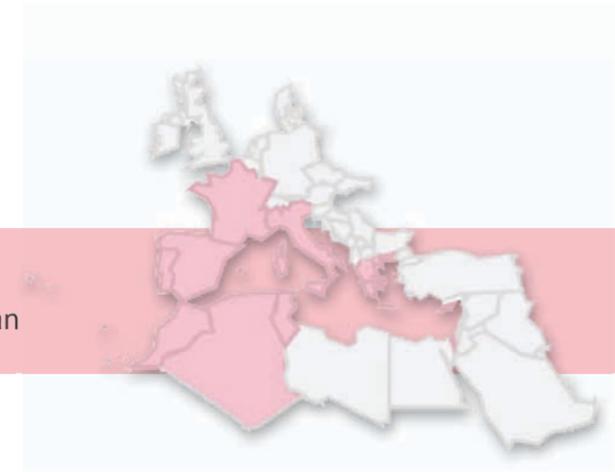
Following the release of the story in *The National*, a number of HAE patients contacted me, and I have been able to put them into contact with HAE physicians. We are also organizing a meeting for doctors to increase awareness of HAE amongst healthcare professionals in the country, which will be led by Dr. Ravi.

 **LEBANON**

We are talking to other patient societies and hope we will be able to increase awareness of HAE with these organizations so that we can work together to improve the lives of HAE patients in Lebanon. I also hope that we can organize a meeting later in the year to provide a chance for doctors interested in HAE to meet and share ideas.



**Maria Ferron**  
Mediterranean



**IN GENERAL**

There have been many activities across the region, and I am pleased to say that a lot of progress is being made by the patient organizations. We are striving to translate the HAE Emergency Card into many of the languages from this region, and these should be available soon for patients to use. Throughout the region, I have also been supporting member organizations in their work to raise awareness of HAE and improve HAE patients' quality of life.

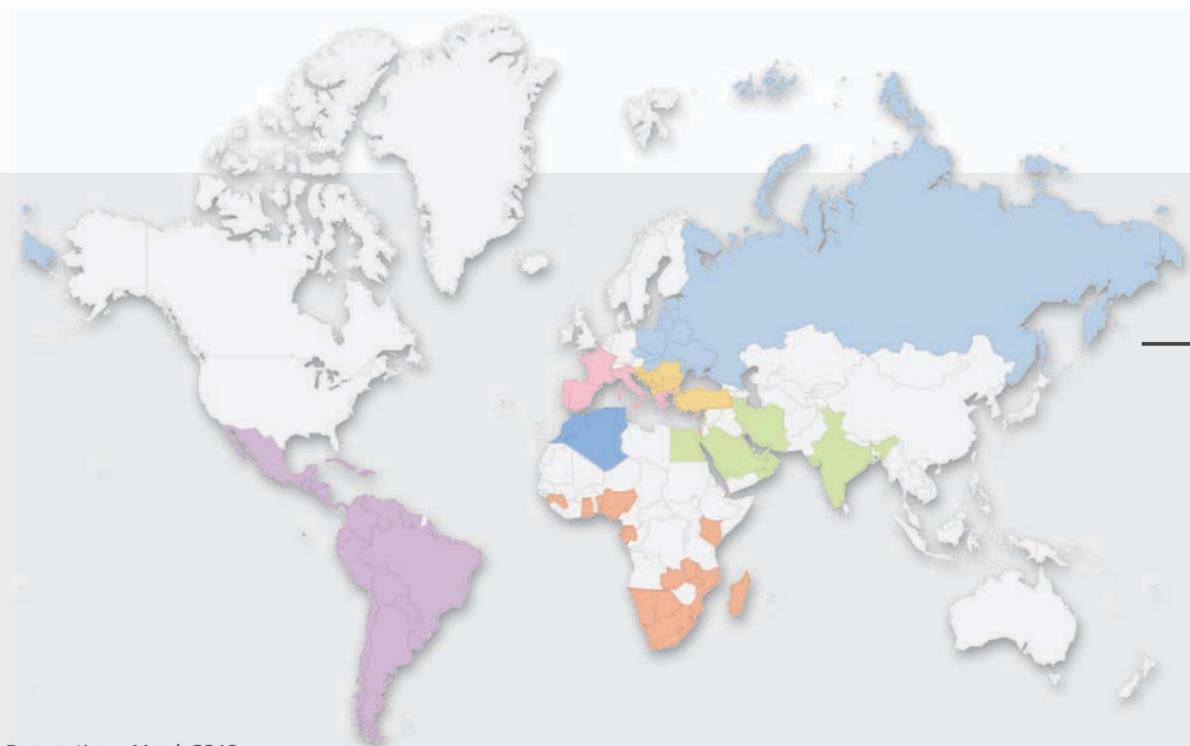
 **MOROCCO**

The Alliance of Rare Diseases in Morocco (AMRM) was created in 2017 and 24 February this year they organized the first National Rare Disease Day in Morocco. The theme for the day was 'For a better awareness of rare diseases in Morocco' which is an integral part of the work in Morocco, where many rare diseases are still not well known. The meeting was attended by health professionals, patients, and the general public and discussed many aspects of rare diseases, including diagnosis, medical training, and

health policy. We are also very happy to report that a dedicated national HAE association is in the process of being established in Morocco. However, an office/steering committee has been already set up including ten people, half physicians and half patients which we hope will provide support for patients and HAEi initiatives.

 **SPAIN**

The Spanish HAE association AEDAF held their 20th General Assembly on 10 March 2018 in Hospital Universitario La Paz in Madrid. The meeting was a great success and brought together patients from across the country. Attendees were able to hear and learn from physicians and patients had the opportunity to discuss important developments for the organization itself and to exchange experiences. As part of the meeting, HAEi's IT project manager and I were able to demonstrate and answer questions about HAEi Connect, for AEDAF to consider whether to utilize this HAEi resource in the future.



*The Regional Patient Advocates*

- **Michal Rutkowski**; Central and Eastern Europe
- **Maria Ferron Smith**; Mediterranean
- **Natasa Angjeleska**; South East Europe
- **Patricia Karani**; Sub-Sahara Africa
- **Javier Santana**; Latin America
- **Rashad Matraji**; Gulf Region and Middle East
- **Maria Ferron og Rashad Matraji**



## Potentially 28,000 HAE patients in China

By Fiona Wardman, HAEi Executive Committee member and President of HAE Australasia

China has a population of approximately 1.4 billion, which means there are potentially 28,000 patients with HAE in this huge country. Based on these numbers there is only a small percentage of patients already diagnosed and being treated by knowledgeable physicians.

At this stage, Chinese HAE patients currently only have access to androgens and tranexamic acid for prophylaxis, and fresh frozen plasma for emergencies, and with such a high number of patients and no access to modern treatments there are unnecessary procedures and sadly deaths.

As in other countries, China also suffers from the lack of awareness within the community and within the medical profession, and therefore HAE is misdiagnosed and under-diagnosed as is so common (still) around the world.

With all of this in mind, there is some good news. China has some dedicated physicians who are extremely passionate and knowledgeable, and there are a handful of committed patients willing to become a united voice and work in the HAE China organization. After the 12th Annual ICORD Conference and 6th China Rare Disease Summit in Beijing last year there has also been an undertaking by the government to recognize rare diseases and make treatments a priority. Hopefully, these changes will be made sooner rather than later.

Over the past few months, HAEi and CORD (Chinese Organisation for Rare Diseases) have been working together to host an HAE China Workshop, which was held on 11 March 2018 in Beijing. The workshop was attended by 100 patients and carers from within Beijing and from other cities within China, Chinese physicians, international speakers, CORD, pharmaceutical company representatives as well as representatives from HAEi.

We all gathered in the Grand Ballroom at the DoubleTree by Hilton in Beijing for an early start. The day was packed full of speakers with lots of valuable information, which included:

- 'What every patient needs to know about HAE' by Dr. Henry Li (USA),
- 'Current situation of HAE in China' by Dr. Yuxiang Zhi (China),
- 'Current situation and the chance of Chinese rare disease organization in China' by Kevin Huang (CORD),
- 'History of HAE treatments and what is on the horizon' by Prof Konrad Bork (Germany),
- 'Development of HAE treatment in China' by Dr. Yingyang Xu (China),
- 'The foundation and history of HAEi' by Executive Director Henrik Balle Boysen (HAEi),
- 'Disease Burden of Hereditary Angioedema patients in China' by Dr. Shuang Liu (China)



We also heard from three Chinese patients: Ms. Nan Zhang spoke about 'The History of HAE in China, and the thinking on the development of HAE in China', and two other patients spoke about their horrific experiences with laryngeal attacks and losing family members because of HAE.

There was also a panel discussion on starting a patient organization, and a physician Q & A. There was good involvement by the patients and carers in the panel discussions with plenty of questions asked about starting an organization, how to get involved, and medical type questions on HAE.

After the workshop, many of the patients and carers stayed behind and networked with each other.

Access to some social media in China is not allowed. However, they already established a great patient network via a WeChat group. Here patients and carers can discuss issues and support each other. Dr. Zhi who makes herself available to all patients across China monitors this group – she is a true superstar.

The workshop was a great success, and HAEi would like to thank everyone who attended and helped organize the event. Also, thank you to everyone who attended the dinner that HAEi hosted on the night before the workshop so everyone could come together and meet each other beforehand.



We wish the patients, carers, and physicians in China the best of luck with changing the landscape for HAE patients in their country, we know they will be successful, and HAEi will continue to support HAE China with their endeavors.





## Two decades with AEDAF in Spain

Asociación Española de Angioedema Familiar por Deficiencia del Inhibidor de C1 – or in short AEDAF – held its 20th Annual Meeting and General Assembly in Madrid Saturday 10 March 2018. Dr. Teresa Caballero Molina opened the meeting, taking place at Hospital Universitario La Paz. After the 2017 Activities Report presented by the AEDAF President Sarah Smith Foltz, the 2017 Financial Report was submitted and approved. Then followed the election process for secretary of AEDAF with Maria Ferron being elected.

One of the international guests at the meeting was HAEi Project Manager Ole Frølich who gave an insight into HAEi Connect, a free online membership database for HAEi's member organizations. Ole Frølich characterized HAEi Connect as a valuable tool for HAEi member organizations to build, maintain and grow their member database as well as handle and communicate with members. After the presentation in English and Spanish, the General Assembly decided to adopt HAEi Connect with implementation as soon as possible.

Following a coffee break, the Annual Meeting opened with a tribute to the professional career of Dr. Margarita López Trascasa. The Dr. Caballero Molina spoke about international collaboration, and Dr. Alberto Lopez Lera gave an insight into HAE research projects, while Dr. M. Concepción Lopez Serrano talked about the history of collaboration and founding of AEDAF. Finally, Dr. Margarita López Trascasa gave a presentation under the headline "A career dedicated to Hereditary Angioedema".

Sarah Smith Foltz presented two suggestions for a new AEDAF logo, and the General Assembly decided on the one described as "the molecular logo".

Lunch was followed by an overview of the current situation of HAE in Spain and a debate on topics raised by the participants. Furthermore, HAEi President Anthony J. Castaldo congratulated AEDAF on the first 20 years of existence.



*The newly elected AEDAF Secretary Maria Ferron – who is also HAEi Regional Patient Advocate for the Mediterranean region – with HAEi President Anthony J. Castaldo and HAEi Project Manager Ole Frølich.*

## 2017 revision and update of the international WAO/EAACI guideline for the management of HAE

An international expert panel has reviewed the existing evidence and developed 20 recommendations that were discussed, finalized and consented during the guideline consensus conference in 2016 in Vienna, Austria. The final version of this update and revision of the guideline incorporates the contributions of a board of expert reviewers and the endorsing societies.

The goal of the guideline update and revision is to provide clinicians and their patients with guidance that will assist them in making rational decisions in the management of HAE with deficient C1-inhibitor (type 1) and HAE with dysfunctional C1-inhibitor (type 2).

These are the recommendations:

**#1:** We recommend that all patients suspected to have HAE 1/2 are assessed for blood levels of C1-INH function, C1-INH protein, and C4. If any of the levels are abnormally low, the tests should be repeated to confirm the diagnosis of HAE 1/2. (≥90 agreement)

**#2:** We recommend that all attacks are considered for on-demand treatment. We recommend that any attack affecting or potentially affecting the upper airway is treated. (100% agreement)

**#3:** We recommend that attacks are treated as early as possible. (100% agreement)

**#4:** We recommend that HAE attacks are treated with either C1-INH, ecallantide, or icatibant (18/20). (90% agreement)

**#5:** We recommend that intubation or surgical airway intervention is considered early in progressive upper airway edema. (100% agreement)

**#6:** We recommend that all patients have sufficient medication for on-demand treatment of two attacks and carry on-demand medication at all times. (100% agreement)

**#7:** We recommend short-term prophylaxis before procedures that can induce an attack. (100% agreement)

**#8:** We recommend prophylaxis be considered for patients who face events in life that are associated with increased disease activity. (≥90 agreement)

**#9:** We recommend that patients are evaluated for long-term prophylaxis at every visit. Disease burden and patient preference should be taken into consideration. (100% agreement)

**#10:** We recommend use of C1-inhibitor for first-line long-term prophylaxis. (50-75% agreement (majority vote))

**#11:** We suggest to use androgens as second-line long-term prophylaxis. (50-75% agreement (majority vote))

**#12:** We suggest adaptation of long-term prophylaxis in terms of dosage and/or treatment interval as needed to minimize burden of disease. (100% agreement)

**#13:** We recommend testing children from HAE-affected families be carried out as soon as possible and all offspring of an affected parent be tested. (100% agreement)

**#14:** We recommend C1-INH be used for treatment of HAE attacks in children under the age of 12. (>90% agreement)

**#15:** We recommend C1-INH as the preferred therapy for HAE attacks during pregnancy and lactation. (100% agreement)

**#16:** We recommend that all patients have an action plan. (100% agreement)

**#17:** We suggest that HAE-specific comprehensive, integrated care is available for all patients. (100% agreement)

**#18:** We recommend that all patients who are provided with on-demand treatment licensed for self-administration should be taught to self-administer. (100% agreement)

**#19:** We recommend that all patients with HAE should be educated about possible triggers, which may induce HAE attacks. (100% agreement)

**#20:** We recommend that family members of individuals with HAE should be screened for the condition based on:

- autosomal dominant inheritance
- delayed diagnosis leads to morbidity and decreased quality of life without appropriate therapy
- risk of the first angioedema event being fatal due to airway involvement without appropriate therapy (100% agreement)



Authors of the 2017 revision and update of the international WAO/EAACI guideline for the management of HAE are M. Maurer, M. Magerl, I. Ansotegui, E. Aygören-Pürsün, S. Betschel, K. Bork, T. Bowen, H. Balle Boysen, H. Farkas, A. S. Grumach, M. Hide, C. Katelaris, R. Lockey, H. Longhurst, W. R. Lumry, I. Martinez-Saguer, D. Moldovan, A. Nast, R. Pawankar, P. Potter, M. Riedl, B. Ritchie, L. Rosenwasser, M. Sánchez-Borges, Y. Zhi, B. Zuraw, and T. Craig.

See the full text here: <https://onlinelibrary.wiley.com/doi/full/10.1111/all.13384>.



## HAEi Jakobsweg Walk 2018

After the successful HAEi/AEDAF Camino Walk on the Camino de Santiago in northwestern Spain in May 2016 and May 2017, HAEi is organizing yet another walk.

In order to raise awareness, HAEi brings together HAE friends – patients, relatives, caregivers, doctors, nurses, and industry – for the HAEi Jakobsweg Walk 2018. Just as for the previous walks this is a mutual experience in honor of the global **hae day :-)** and this time the walk will take place on part of the pilgrimage route Jakobsweg Wien (The Way of St. James) through Vienna, Austria.

“Camino de Santiago, Way of St. James, and Jakobsweg are names in different languages for the same thing: A huge network of pilgrims’ ways leading to the shrine of the apostle St. James the Great in the cathedral of Santiago de Compostela in Galicia in northwestern Spain, where tradition has it that the remains of the saint are buried. Many follow its routes as a form of spiritual path or retreat for their spiritual growth. Traditionally, as with most pilgrimages, the Way of St. James began at one’s home and ended at the pilgrimage site, often following highly travelled routes through Europe. One of these traditional routes goes right through Vienna – and that is the one we will be walking for the HAEi Jakobsweg Walk 2018”, says the HAEi Communications Manager Steen Bjerre.

Pilgrimage to Santiago de Compostela began around the year 950 but the ancient roads through Vienna are far older as Roman roads were already present when the pilgrimage began. One of these Roman roads connected the garrison cities Aquincum (Budapest), Brigetio (Komaron) and Carnuntum (German Altenburg) with Vindobona (Vienna). The Way of St. James, which starts in Budapest, also takes this route. Indeed, the Romans knew how to build very durable roads.

The HAEi Jakobsweg Walk takes place 16 and 17 May 2018 and the program consists of four consecutive stages. It is, however, entirely up to the participants if they want to take part in just one, more or all of the stages.

At the moment there are 143 participants from 21 countries for the HAEi Jakobsweg Walk: United States, Brazil, Argentina, Denmark, Mexico, Australia, Canada, Italy, Spain, Venezuela, United Kingdom, Chile, France, New Zealand, Norway, Peru, South Africa, Austria, and Switzerland.

“However, there is room for more participants. If you know of anyone going to the HAE Global Conference who would like to do the HAEi Jakobsweg Walk but haven’t signed up when registering for the conference please have them send an e-mail to [s.bjerre@haei.org](mailto:s.bjerre@haei.org)”, says Steen Bjerre.

The event is also open for people who would like to join the group of pilgrims but are not going to the conference.

They can register directly at <https://goo.gl/FDR7uQ>.



# HAEi Global Walk 2018

As quite a number of people have told HAEi that they would really have liked to take part in the HAEi Jakobsweg Walk 2018 but are for one or the other reason unable, HAEi will once more be arranging the HAEi Global Walk. It works in this way:

You can participate wherever you are. All you need to do is

1. Walk any distance you like wherever you like – on your own or together with others,
2. Go to the campaign website at [www.haeday.org](http://www.haeday.org), and
3. Enter your walk.

HAEi will then add your steps to those of all the people doing the HAEi Jakobsweg Walk.

The HAEi Global Walk 2018 campaign is open from 1 April to 31 May – and you are free to enter as many walks as you like. A small walk alone around your block, a weekend stroll with friends, an event with many participants or indeed your preparations for the HAEi Jakobsweg Walk – it's all up to you. Every step counts.

“At [www.haeday.org](http://www.haeday.org) you will be able to see how many steps have been taken so far, who has entered their walks, and where in the world the walkers are located. Please visit – and please note that even though you do the HAEi Jakobsweg Walk you are very welcome to use the campaign page. It could be a great way to keep track of your training before we meet in Vienna”, says the HAEi Communications Manager Steen Bjerre.

# Get your website hosted at haei.org

A growing number of national HAE organizations have their own websites with their own individual hosting solution. However, some of them would like to change hosting or altogether change the look and content of their websites. And others would like to just have a website at all.

“In order to accommodate any such national HAE organization we have established a system under the HAEi website allowing us to host national websites as well as provide them with templates for an individualized website – naturally all in their native language”, says HAEi Executive Director, Henrik Balle Boysen.

At this point national websites have been launched for these 16 countries:

- **Australia:** [www.haeaustralasia.org.au](http://www.haeaustralasia.org.au)
- **Ecuador:** [http://haei.org/ae\\_h\\_ecuador/](http://haei.org/ae_h_ecuador/)
- **Greece:** <http://haei.org/greece/>
- **Hungary:** <http://haei.org/hungary/>
- **Iceland:** <http://haei.org/iceland/>
- **Kenya:** <http://haei.org/haekenya/>
- **Macedonia:** <http://haei.org/haemacedonia/>
- **New Zealand:** [www.haeaustralasia.org.au](http://www.haeaustralasia.org.au)
- **Peru:** <http://haei.org/peru/>
- **Poland:** <http://haei.org/pl/>
- **Romania:** <http://haei.org/romania/>
- **Serbia:** <http://haei.org/rs/>
- **South Africa:** <http://haei.org/southafrica/>
- **Spain:** [www.angioedema-aedaf.org](http://www.angioedema-aedaf.org)
- **Turkey:** <http://haei.org/turkey/>
- **Uruguay:** <http://haei.org/uruguay/>

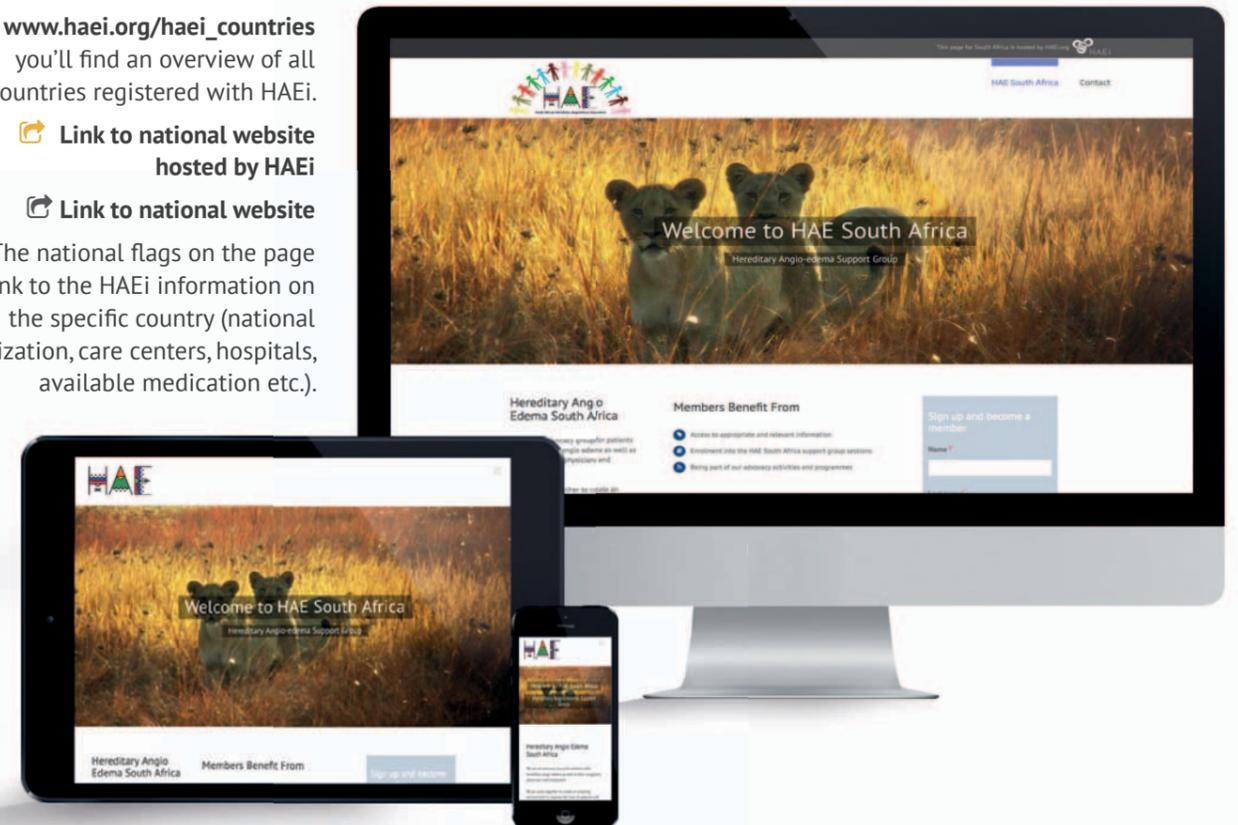
“We are preparing one or two at the moment and many more are welcome to join us during 2018”, says Henrik Balle Boysen.

At [www.haei.org/haei\\_countries](http://www.haei.org/haei_countries) you'll find an overview of all 63 countries registered with HAEi.

[Link to national website hosted by HAEi](#)

[Link to national website](#)

The national flags on the page link to the HAEi information on the specific country (national organization, care centers, hospitals, available medication etc.).





**URUGUAY**  
haei.org/uruguay/

**Oscar Sanchez – the man behind the logo for HAE Uruguay – talks about the logo idea:**

Of course, the main idea is to show that despite suffering from a genetic disease, which can lead to very bad times, the sun will always come out and you should never lose hope. For this, I found two elements that I wanted to use:

(1) Something that would identify us as country = colors and sun taken from our flag

(2) A minimalist part of a DNA – and obviously not a normal one, but a very rare one.

Leaving aside the symmetry and perfection, I would like to emphasize that sometimes imperfections or (in this case, the sun) rare pathologies such as ours can also illuminate the path and make us appreciate the simplicity of everyday situations.

**ITALY**  
www.angioedemaereditario.org

It is with great pleasure that HAE Italy (A.A.E.E.) presents the Italian HAE Patient Registry, now operative after being approved by the Executive Committee and by all reference centers in January 2018. It took time to achieve this goal to which physicians, experts, technicians and patients worked together to implement and improve knowledge and research.

Data availability and sharing of knowledge related to rare diseases are paramount in the correct definition of the patient condition with all the implications on treatment and quality of life. Data gathering and elaboration is a fragmented and very time-consuming process.

The registry model used until 2017 was based on paper diaries updated year by year. This required great coordination and collection efforts, and put limitations to the freshness and quality of data gathered on such a long time span, which needed at the end of each year to be manually collected and uploaded in a digital database.

The approach to solve this problem was directed towards the implementation of an advanced registry management model exploiting cloud-based technology, organization protocols and procedures, enabled by user friendly ICT solutions, with an interoperable database registry and document flow management. Last but not least, ethic/privacy rules needed to be natively embedded in the design of the solution.

The project was made possible by the collaboration of A.A.E.E. for financing, the reference center for angioedema at Ospedale Sacco-Milan for clinical and study protocol, and the company Cloud-R srl for the solution design and implementation.

The first presentation of the concept took place in September 2016 at the international conference “Treating Patients With C1 Inhibitor Hereditary Angioedema - Third Hawk Consensus Conference” in Gargnano, Italy. After two months the first prototype was presented in Sofia, Bulgaria to the representatives of 12 countries from Europe, North America and South

**UNITED ARAB EMIRATES**  
haei.org/location/hae-in-united-arab-emirates

“One man’s battle to improve quality of life for sufferers of swelling disease”. That was the title of an article brought by The National, a private English-language daily newspaper published in Abu Dhabi, United Arab Emirates.



The article tells about Rashad Matraji, HAEi Regional Patient Advocate and HAE representative in United Arab Emirates and quotes him:

“I inherited the condition from my father and grandfather before him. No one knew what I had. Doctors would diagnose randomly, but no one could find a cure.” (...) “I even had my appendix unnecessarily removed a few years ago. But I always knew there must be a treatment and I vowed to find it.”

You can find the article at <https://www.thenational.ae/uae/one-man-s-battle-to-improve-quality-of-life-for-sufferers-of-swelling-disease-1.698092>

**ECUADOR**  
haei.org/aeh\_ecuador

Ecuador is the fifth South American country to have a website – and the third to host it under the wings of HAEi. Have a look at the new Ecuadorian website at [http://haei.org/aeh\\_ecuador](http://haei.org/aeh_ecuador).

America. They all agreed on the validity of the new approach and to move forward. Further presentations were carried out in Atlanta, USA at the American Academy of Asthma & Immunology Congress in March 2017 and in Budapest at the 10th C1 Inhibitor Deficiency workshop in May 2017, in both cases with the publication of scientific publication posters.

The registry architecture has different layers, with visibility defined by the role of the user, always keeping the privacy of patients strictly enforced. Only the patient and his doctor/reference center can see personal identification data. All data transfers are encrypted, and the cloud-based infrastructure is selected for its capacity of being always at the latest technology level, ensuring the highest security protection possible.

An important advancement in engaging patients in the registry has been the electronic diary that makes possible to patients, already enrolled in the registry, to enter attack data themselves. The diary can be accessed via app or web on both smartphone and computer.

This new procedure will make it possible to eliminate the paper form of collection of information on the attacks, permitting registration in real time and in only two minutes. Patients are required to fill in an attack form and tick the options considering edema site, edema gravity, treatment, dose of medicine, self infusion, care giving assistance, beginning attack time, final attack time, admission to hospital, and absence from school/work.

Once the data have been entered, they will be validated by the physician of the reference center, thus guaranteeing also the control by the specialist. In this way the data in the registry will always be updated and secure.

We really hope for a better future for all patients and that this revolutionary instrument can help all people involved in HAE to work and live better and better.



The 19th HAE Switzerland Patient Meeting will take place on 9 June 2018 at the Swiss Museum of Transport in Lucerne. The agenda includes these topics:

- “Correct handling of health insurance → Avoiding pitfalls!” (Prof. Dr. med. Walter A. Wuillemin, HAE-Zentrum Luzern, Luzerner Kantonsspital)
- “Leading an independent life by means of home therapy” (Prof. Dr. med. Walter A. Wuillemin, HAE-Zentrum Luzern, Luzerner Kantonsspital, and President Helene Saam, HAE Switzerland)
- “Presentation of the service of HTHC/High Tech Home Care AG, Rotkreuz → Assistance in learning self-injection” (Dr. Claudia Hartmann, Managing Director, and Caroline Christinger, Nurse)
- “New HAE prophylaxis using tablets or subcutaneous drugs” (Dr. med. Urs Steiner, Immunology, UniversitätsSpital Zürich)
- “Structure of the Swiss HAE register → Join us!” (Dr. med. Urs Steiner, Immunology, UniversitätsSpital Zürich)
- “HAE in everyday life for children, families and learners” (Dr. med. Christina Weber, HAE-Zentrum Zürich, UniversitätsSpital Zürich)



The App HAE Diary Switzerland is available for free download at both Apple Store and Google Play Store. With the app you can record the course of your illness and its treatment via a simple questionnaire with checkboxes. As a digital diary, this app can help you document your attacks as well as your HAE prophylaxis. Naturally, the app does not replace the advice of your doctor. More information about the app – developed by Shire especially for patients with HAE – can be found at [www.hae-vereinigung.ch](http://www.hae-vereinigung.ch).



**Paige Gunderson, HAE Canada youth member, has sent us this:**

These are the highlights HAE Canada has been involved with, in amazing 2018 thus far.

Some of our provinces held celebrations for Rare Disease Day on 28 February 2018. Canada has been participating in this special day since 2008. Globally, it is the 11th International Rare Disease Day with hundreds of patient organizations from countries and regions around the world participating in various local events. This year's theme was “Research”. This benefits not only my family, but also many other people dealing with HAE and other rare diseases. As we can all appreciate, research is essential to further understand how to improve medications, treatments and above all Quality of Life. In honor of Rare Disease Day, a video was posted on the HAE Canada website of Ken Howlett, HAE Canada's Vice President, describing his life with HAE.



To further mark Rare Disease Day, Ken, along with his wife, Linda Howlett, HAE Canada's Secretary, attended Question Period at Ontario's Legislative Assembly in support of the Canadian Organization for Rare Diseases (CORD)'s presentation to Members of the Provincial Parliament (MPPs).

One big thing we are all excited about is the up and coming 2018 HAE Global Conference, hosted this year in Vienna, Austria 17-20 May 2018. At least 27

participants from all over Canada are gearing up to go and represent our wonderful country at the conference this year. We will be sporting red and white HAE Canada shirts, so keep your eyes open for us.

While on a personal holiday in California, USA in February, one of our Regional Directors, Bob Simon, had the opportunity to tour the US HAEA Angioedema Center at UC San Diego. While on tour, he learned what the center has to offer, including their care for patients with angioedema, state-of-the-art diagnostic tests and techniques, therapeutic modalities, patient support programs, and research. Pictured with Bob is Patient Representative Ms. Michelle Martinez.

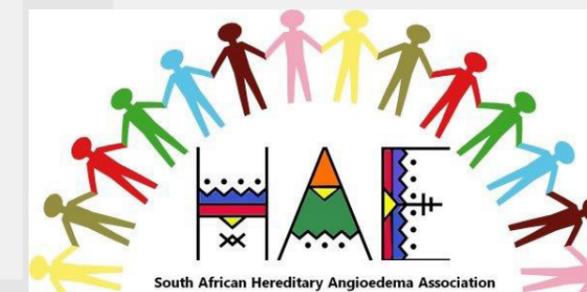


For the last 3 ½ years a member of CHAEN, Dr. Amin Kanani and HAE Canada's President Jacquie Badiou were involved in advocating the British Columbia (BC) Provincial Government for approval of funding for patients in BC to gain access to Firazyr (icatibant injection). A positive recommendation was recently obtained to provide coverage, which is fantastic news!

Check out our website, our Facebook page, Twitter & Instagram accounts to follow along on our journey through this year's achievements and progress. Our official Twitter account was launched in February: @HAE\_Canada. We are also aiming to re-launch our newly updated website for HAE Awareness Day on 16 May. Stay tuned for more of our social media updates.



For the Rare Disease Day in Bulgaria HAEA Bulgaria organized a screening of Natalie Metzger's movie “Special Blood”. Patients, caregivers and doctors saw the heartbreaking story of the patients with HAE and an exciting discussion full of tears of compassion and hope was held after the movie. The screening was organized with the special help of Baja Tours Ltd., Universiada JSC and Novimed Ltd.



Things are really going places in South Africa. The first doctor and patient HAE meeting was held in Cape Town at the Groote Schuur Hospital on 25 October 2017 gathering patients from various regions of South Africa. Shortly after that HAE South Africa introduced a logo. And over the next few months a website will be in place under the HAEi umbrella.



For Rare Disease Day, Dr. Dušanka Paskaš Marković and Jovana Cvetković, Vice President of HAE Serbia, met on Serbian TV to discuss HAE. You can find the program at <https://youtu.be/m3CZAzsjG-A>.



News from around the Globe



**Michelle Cuevas, the US HAEA Director of Communications, writes:**

Members of the HAEA patient community, families, friends and our company partners will come together to run, walk or cheer, but most importantly, have fun, all for the cause near and dear to our hearts – raising awareness and funding for programs that benefit patients who suffer from HAE.

The HAE IN-MOTION® 5K events are the largest fundraising effort of the US HAEA. Proceeds raised from these events go to support three programs:

- HAEA Scholarship Program – provides financial support for HAE patients who are entering or attending college and seeking to improve their lives through academic achievement
- HAEA Compassion Fund – offers financial assistance for patients in need who must travel to see an HAE medical specialist at the US HAEA Angioedema Center at UCSD
- HAE Research – supports expert researchers in their efforts to solve the remaining scientific mysteries of HAE through the US HAEA Angioedema Center at UCSD

These events offer a good opportunity for you to meet patients and doctors in your area, while enjoying a fun outing. Register today at <http://events.haea.org/> and make a difference in the lives of HAE patients. For more information, to set up a virtual team, or if you would like to help us fundraise within your local community, contact Christine at [cselva@haea.org](mailto:cselva@haea.org).

*To raise awareness and funding for programs that benefit patients who suffer from HAE members of the HAEA patient community come together to run, walk or cheer while having have fun*

- Saturday 18 April 2018: Winding Trails, Inc., Farmington, CT
- Sunday 22 April 2018: Hopkins Park & Shelter, DeKalb, IL
- Saturday 2 June 2018: Holmes Lake Park, Lincoln, NE
- Saturday 4 August 2018: Danada Forest Preserve, Wheaton, IL
- Saturday 8 September 2018: Blue Lake Park, Fairview, OR
- Saturday 22 September 2018: Nomahegan Park, Cranford, NJ
- Saturday 6 October 2018: Roger Williams Park - Carousel Village, Providence, RI
- Saturday 7 November 2018: Brushy Creek Lake Park, Cedar Park, TX

Check <http://events.haea.org> for additional events to be added

The HAEA is now active in more social media platforms, helping us be better connected to our HAE community and their needs. We're excited to join you in your everyday engagement through the multiple digital platforms, and look forward to sharing more experiences together. Follow us on Instagram and Twitter and let's keep the conversation going.

- Twitter: @us\_haea
- Tony Castaldo on Twitter: @tonyecastaldo
- Instagram: @us\_haea
- Instagram: @haeinmotion5k



The HAEA Café has a fresh new look. Join TODAY at [www.haeacafe.org](http://www.haeacafe.org) to enjoy access to HAE exclusive events, news and webinars. You will be able to enjoy new features in this great patient resource to help you stay connected with the HAE community.

Check out the exquisite new menu and click on our four pillars to learn more about our specialty interest areas. Chat live with other members and HAEA patient advocates. Take a look at our Bulletin Boards for events and activities near you. Discover the latest breakthroughs through our HAEA Treatment Education Series. Share your stories and upload your event pictures. Participate in our virtual support groups for patients and family members. Join the new HAEA Café today.



We've added a brand new page to our website. The [haea.org](http://haea.org) events page is a tool to keep the HAEA community updated on events and activities taking place year-round. It's also designed to help you host and promote your own HAE awareness event, with fun ideas on building new relationships locally and educating your own community on HAE.

The HAEA joined thousands of allergist/immunologists, medical specialists, allied health and related healthcare professionals around the world for the AAAAI annual meeting and World Allergy Congress in Orlando, Florida. Tony Castaldo, Janet Long, Saira Shaikh, Anna Chenoweth, Sally Urbaniak, and Troyce Venturella exchanged ideas and information on the topic of HAE with leading allergist/immunologists and other attendees on the national and international level. This annual conference presents an important opportunity for the HAEA to raise awareness among specialty physicians about the challenges of diagnosing HAE.



PERU

haei.org/peru



28 February Rare Disease Day was celebrated around the globe – naturally also by HAE patients.

In Peru the President of the national HAE organization Suzet Lam Torres (left) and patients representing other rare conditions participated in an event of the Health Department of Peru.



ROMANIA

haei.org/romania



Yet another country has chosen to have its website under the HAEi umbrella, this time Romania. Have a look at the new website at <http://haei.org/romania>.

Since December 2017 Swelling Beautifully managed to organize these regional meetings:

- 9 December 2017: Regional Patients' Meeting in Łódź (Central Poland) with 14 patients, five healthcare professionals, and four caregivers;
- 13 January 2018: Regional Patients' Meeting in Warszawa (Central Poland) with 26 patients, two healthcare professionals, and two caregivers;
- 24 February 2018: Regional Patients' Meeting in Lublin (Middle East Poland) with 15 patients, two healthcare professionals, and three pharmaceutical representatives;



POLAND

www.haei.org/pl

From the HAE Poland President Michal Rutkowski:

It has been a quite busy and productive time for the Polish HAE Patients' Association Pięknie Puchnę (English translation 'Swelling Beautifully') as we have attended, organized, and prepared numerous events and materials for patients, caregivers, and healthcare professionals.

We have been continuing with the organization of Regional Patients Workshops in different regions of Poland, which gives the opportunity to meet existing and new patients and provide them with the latest information about the disease, treatment options, tools helping them to manage the disorder, and increase the awareness in general. One of the main points on the agenda of the meetings is the self-administration course that allows the patient to get the ability of intravenous and subcutaneous injections. The workshops are open to every single HAE patient, regardless if he or she is a member of our organization or not, caregivers, healthcare professionals, and pharmaceuticals' representatives. Only by working together and understanding the patients' needs and expectations we can provide the best possible long-term service to our patients and eventually increasing their life quality.



*One of the main points on Regional Patients Workshops is the self-administration course that allows the patient to get the ability of intravenous and subcutaneous injections.*

24 March 2018: Regional Patients' Meeting in Katowice (Upper Silesia Region in South Western Poland) with 15-20 participants, including several new HAE patients.

Swelling Beautifully, as the member of National Rare Diseases Organization, has participated in the Rare Disease Day organized in Warszawa on 28 February 2018. The three-hour long meeting gathered 12 patients' organizations representing different therapeutic areas, many patients, and caregivers, healthcare professionals, pharmaceutical representatives, as well as the Vice-Minister of Health. Due to the presence of lots of TV stations, brief information regarding this celebration meeting was spread around the country. Eventually, it occurs that rare diseases are not quite that rare, and given that around 2,5 million Poles suffer from a rare disease, makes them quite common though.

With the 2018 edition of the Regional HAE Patients Meetings, we have started to distribute tools and materials for the patients. The new Patients' Diary has been created in late 2017, with plenty of useful information both for patients and healthcare professionals, especially general practitioners.

Since December 2017 we have already distributed approximately 240 copies of the diary, and due to a huge demand for it, the new delivery is expected to arrive late March. This Patients' Diary, which is also a calendar, is not only a valuable source of information for the patient and his physician, but also a document that registers individual attacks and used medications, and might be an important confirmation during the National Health Fund's control of the issuing of prescriptions by the individual health care professionals.



The other significantly important tool has been distributed since the beginning of 2018, and it is Patients' Emergency Card, which was created by HAEi to unify the layout of Patients' Emergency Cards globally. The material and the size of it (the same as a credit card) makes it very convenient to carry, and most of the patients keep it in the wallet. We have already distributed over 150 copies of it and keep going with the delivery, as there is a high demand for it. The size, material, and mostly the content make it a very useful source of information an immediate help in the emergency situations our patients may experience.

Swelling Beautifully is proud to inform you about the launch of a new project, which is the most challenging and difficult one we have ever started. It is a documentary about living with HAE from the perspective of Polish patients, the burden of the disease, access to medication and effective diagnosis. The aim is not only to increase the awareness of the disease itself, but also to create a tool, that can be used by the patients and pharmaceuticals in order to convince the Health Ministry (the 'payer') to extend the reimbursement indications and to approve the reimbursement for the new therapies coming to the market and used mostly in the long-term prophylaxis. Our motivation to make this kind of project was the US HAEA 'Special blood' film that beautifully and truly shows what the HAE patients have been going through, but also gives hope for a better future. We hope that with our documentary we will deliver another motivational and inspirational tool.

**SPAIN**  
www.angioedema-aedaf.org

**From Sarah Smith, the President of HAE Spain (AEDAF):**

AEDAF held its 20th General Assembly and Annual Meeting on Saturday 10 March 2018 at La Paz University Hospital in Madrid. During our General Assembly, we voted in favor of joining HAEi Connect and will soon be using the system. We also decided to change the AEDAF logo for a more modern, distinctive one which is suggestive of the molecular basis of HAE but which can also be seen as symbolizing people connected through AEDAF. We also had elections for a new secretary during the General Assembly; Maria Ferron, the HAEi Regional Patient Advocate for the Mediterranean region, will now also be serving as secretary of AEDAF.



We were pleased to welcome Anthony J. Castaldo, President of HAEi, to our meeting, as well as Project Manager Ole Frølich, who gave a very good explanation of the HAEi Connect system to our members, and Communications Manager Steen Bjerre of HAEi, who will prepare an article on the event.



The high point of the meeting was a tribute to Dr. Margarita Lopez Trascasa in recognition of her long career in the Immunology Laboratory Department of La Paz University Hospital in Madrid and her excellent work in the study and research of HAE to help improve the quality of life of HAE patients. Our sincere thanks again for all she has done for us!

Several members of AEDAF will be walking a couple of stages of the Austrian Jakobsweg in the area around Salzburg during the days leading up to the HAEi Jakobsweg Walk 2018 and the 2018 HAE Global Conference in Vienna, Austria. We will then join the Jakobsweg Walk and of course attend the Global Conference, where AEDAF will be represented by more than 15 people between patients, caregivers, and healthcare professionals.

**MACEDONIA**  
haei.org/haemacedonia

The Rare Disease Day in Macedonia was marked on 28 February with a press conference, which was attended by head decision-makers at the healthcare institutions and for the first time in Macedonia the Rare Disease Day was attended by the Minister of Health. He addressed the audience by stressing that one of the top priorities of the Ministry of Health is continued advancing of the treatments for rare disease patients, pledging that the Ministry will continue to work in that direction.



The minister also informed the audience that there is an on-going upgrade of the electronic healthcare system aimed at a electronic register of rare disease patients and explained that it will be connected to the clinics

and chemists at the clinics where patients are treated. That would ensure recording of the available amounts of medicines as well as monitoring of the issuance of the medicines to patients, thus avoiding the risk to run out of medicines.

After the press conference, there was screening of Natalie Metzger's "Special Blood" – a film about HAE, and the words of Lora Moore ring out: "This disease is not taking anybody else without a fight."

Furthermore, HAEi Regional Patient Advocate Natasa Anjeleska was invited to speak on TV 24 station about the Rare Disease Day and explain about the specifics of HAE.

**СТЕ ЧУЛЕ ЛИ ЗА ХЕРЕДИТАРЕН АНГИОЕДЕМ (ХАЕ)**

**ШТО Е ХАЕ?** - Мутација во генот кој го контролира создавањето на еден крвен протеин наречен Ц1 инхибитор. Како резултат на генетскиот дефект, се создава неправилен или нефункционален Ц1 инхибитор. Тоа влијае врз капиларите и поттикнува ослободување на течност од нив кон околиното ткиво, при што се појавуваат отоци.

**30%** СМРТНОСТ КАКО РЕЗУЛТАТ НА ОВНЕ НАПАДИ  
Травење на отоците: **2-5 дена**, но исто така е можно да се јават и повторливи епизоди на отоци.

**50 000** Преваленца?

**20%** од ново дијагностицираните ХАЕ пациенти немаат семејна историја бидејќи нивниот генетски дефект се појавува спонтанно при зачнувањето.

**СИМПТОМИ НА ХАЕ:**  
Едеми (отоци) на:  
Лице, очи, уста, јазик, грло (дишни патишта)  
Скелет  
Раце  
Нозе

**ВНИМАНИЕ!**  
Отокот на грло (дишните патишта) може да предизвика смрт со гушење. Потребна е итна медицинска грижа и администрирање на лек веднаш штом дојде до појава на симптоми. Отоците на stomак може да предизвикаат огромна болка, лошење, повраќање и дијареја. ХАЕ нападите НЕ се предизвикани од алергиски реакции. Поради тоа антихистаминиците, кортикостероидите и епинефринот не се ефикасни ни соодветен третман за ХАЕ.

**И двата пола може да се погодени од ХАЕ**

**Дете на родител со ХАЕ има 50% изгледи да ја наследат болеста**

**ШТО МОЖЕ ДА ПРЕДИЗВИКА ХАЕ**  
Стрес, траума од повреди, стоматолошки зафати и операции, настинка и грип, лекови за крвен притисок – АСЕ, инхибитори.

**КАЈ ЖЕНИТЕ**  
Менструација, таблети за контрацепција врз основа на естроген и терапија за замена на хормони, хормонски промени коишто настануваат во бременоста.

**ТРЕТМАН:**  
Се уште не постои лек за ХАЕ, но третманите коишто постојат – а голем дел од нив може да се само-администрираат – им овозможуваат на пациентите контрола врз ХАЕ нападите. Тековните истражувања упатуваат на тоа дека има подобри третмани за ХАЕ и на ХАЕ пациентите им претстои подобра иднина.

www.haemacedonia.mk  
e-mail: haemacedonia@gmail.com  
Контакт: 070788420

HAE Macedonia is embarking on yet another exciting journey: 24 March 2018 the HAE Caravan began again with a series of lectures on HAE for the general practitioners and ER staff. Encompassing several cities in Macedonia the purpose of the lectures is to raise awareness among the medical population on the disease and possibly find HAE patients who might be struggling without proper diagnosis. The lectures are being held by Professor Vesna Grivcheva Panovska, Ph.D. and they are accredited by the Medical Chamber of Macedonia. Posters and brochures are distributed in hospitals and ERs throughout Macedonia.

**COSTA RICA**  
haei.org/location/hae-in-costa-rica

HAE Costa Rica has been very busy and recently completed the application to have the organization officially registered in the country. More patients continue to be identified in the quest to find the 500 that statistically are expected to be found in the population.

HAE Costa Rica co-hosted an event leading up to rare disease day with the country's top geneticist from the National Children's Hospital giving a presentation on rare diseases. At the event, it was announced that a regional LATAM expert physician network for those working with rare diseases would be launched. Please send any HAE physician names/emails to aehcostarica@gmail.com so we can see that they are included in this network being sponsored by Pfizer.

Rare disease day found Costa Rican HAE patients and family members gathered in Plaza de la Cultura where infographics were handed out, and multiple media interviews were given.

Along with a number of other stakeholders, HAE Costa Rica is part of a group working to explore founding a national federation of rare diseases. HAE Costa Rica is very proud to work towards ensuring that patient voices are present and heard during these discussions.

In mid-March, a 16-year-old HAE patient progressed towards near asphyxiation while hospital staff waited hours to apply plasma that had been ordered thawed hours prior to administering. Subsequently, HAE Costa Rica is currently working on establishing hospital protocols along with dental 'best practice' protocols to meet the goal that 'No Costa Rican HAE patient loses their life related to an HAE complication.'



#### From the HAE Australasia President Fiona Wardman:

HAE Australasia has been busy over the last few months with quite a few projects; one of them is organizing 'Meet Ups' and 'HAE Healthy Minds Workshops' around Australia and New Zealand. These events are a great opportunity for patients and carers to socialize with others, who know exactly what they are going through, network, gain support, and learn about HAE. In the past few months we have met up with patients in Brisbane and Adelaide, and we have a family day being held 24 March in Perth. Other events coming up will be in Auckland, New Zealand and Mackay (Far North Queensland), Australia – information can be found on our website.

For Rare Disease Day this year we engaged a communications company to run an awareness campaign. The campaign included newspapers – online and print, radio interview and TV interviews. This was a huge success as it generated 61 media stories and provided an estimated 761,766 opportunities to see HAE Australasia's key messages. Social media activity generated an additional 2,021,754 opportunities to help create awareness.

It's an exciting time for patients with HAE with so many new treatments coming our way. Our organization wants to ensure that all patients in Australia and New Zealand have access to clinical trials and subsidized treatments. HAE Australasia through all its projects are continually encouraging patients to complete the membership form either via our website or contacting us directly, so they are accounted for. If there are any patients, carers or family members in Australia and New Zealand who are not yet receiving information directly from HAE Australasia, please register with us via this link [www.haeaustralasia.org.au/stay-in-touch/become-a-member](http://www.haeaustralasia.org.au/stay-in-touch/become-a-member) or email us directly [info@haeuaustralasia.org.au](mailto:info@haeuaustralasia.org.au).

To help support our patients, carers, and supporters we will be moving our membership database to a more secure, flexible and user-friendly platform using HAEi Connect. More information will be sent out shortly.

We are excited to have 19 patients and carers from Australia and New Zealand (combined) attending the 2018 Global Conference in Vienna in May. This is our biggest number of attendees yet, and everyone is really looking forward to it.

Our 2019 Patient and Carers Conference is currently being organized; we will have speakers and guests from overseas as well as from our region. It's going to be bigger and better than ever before. More information will be sent out to our membership very soon.

We are also working on more projects like community grant applications and fundraising, creating awareness and education.



**Youth Ambassador:** Two years ago now HAE UK appointed Alex Graham to be the Youth Ambassador and she has done great work since then to encourage younger members of HAE UK. Her presentation at the Patient Day 2016 was inspirational showing exactly how fantastic the up-and-coming generations are. HAE UK is are delighted that Jack Cope has agreed to take on the dual role with Alex. His first 'official' engagement will be attending the 2018 HAE Global Conference in Vienna, Austria where he and Alex will be on the 'Youth Track'.

**Calling all dentists:** Do you attend a dental practice that understands HAE and is happy to treat you? Please send the practice name and address to [laura.szutowicz@haeuk.org](mailto:laura.szutowicz@haeuk.org) so she can contact them to ask if they are prepared to be included on the HAE UK register of HAE friendly dentists. This is a question HAE UK is frequently asked by patients so the organization is keen to be able to recommend dentists to you to help you feel confident with being treated.

**HAE video:** Would you like to help produce an inspirational video about HAE? HAE UK is producing a short video about some of the achievements of the members, which is following on from the great presentation by Alex Graham, the Youth Ambassador, at the patient day in 2016.

The project aims to inspire and encourage others and show that, with the right medication and care plan in place, you can follow and achieve your dreams.

HAE UK hopes to produce this short video in time for hae day :- ) 16 May 2018, so it can be widely shared to not only show personal stories and achievements but also to help to raise awareness of HAE in general. If you have a story, a quote, some inspirational news to share, or any questions, please get in touch with Rachel Annals at [rachelannals@haeuk.org](mailto:rachelannals@haeuk.org).



The 2017 version of the HAE Austria patient meeting took place in Linz with the participation of more than 20 HAE patients.

The HAE Austria President Adelheid Huemer gave an overview of the development over the last decade – from two members in 2006 to more than 50 today and from only Berinert for emergency treatment in 2006 to now Firazyr, Cynrize, Ruconest, and Berinert.

Mrs. Huemer also spoke about the intensive cooperation with the larger organization HAE Germany as well as the very good collaboration with the pharmaceutical companies.

Vice President Christian Müllner informed about interesting visits to the pharmaceutical companies in Vienna, while Board Member Clemens Schöffl presented a map of Austria with the location of HAE patients. Furthermore, Dr. Wiednig spoke about "HAE in children – new possibilities for diagnosis".

# HAEi Connect

## – online membership database free for all member organizations



HAEi is proud to present a new initiative for the member organizations: HAEi Connect – a cloud-based member database for national organizations to manage their members.

“In our close collaboration with the national member organizations, we have learned that management of members is handled in many different ways – ranging from simple Word files and small local databases to Excel spreadsheets and more complex systems. In order to make management of the member database a lot easier for the national organizations under the HAEi umbrella we now offer a cloud-based platform for member management”, says HAEi Executive Director Henrik Balle Boysen.

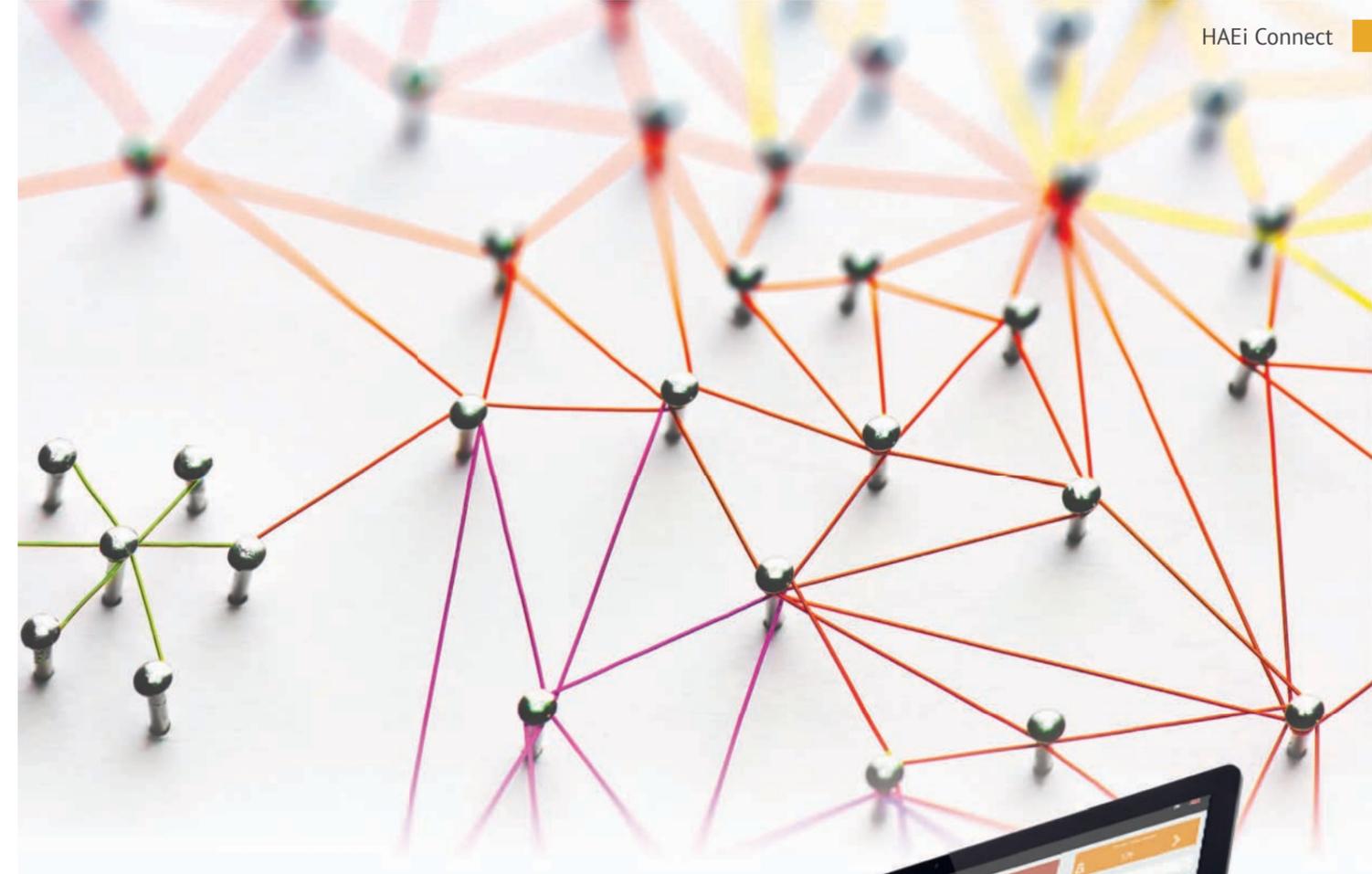
HAEi Connect – a user-friendly interface with instant member e-mail communication – is a platform created and maintained by HAEi. Project Manager Ole Frølich explains:

“HAEi Connect will secure a uniform and secure member management worldwide. In addition to this, we will further develop the system based on ideas and requests from the member organizations”.

Among the key features of HAEi Connect are:

- Secure management of member information
- EU GDPR compliant system
- User-friendly interface
- The interface in the language(s) of the national member organization
- Dashboard with member data status
- Access to news from HAEi, download area and webinars
- Advanced function to search groups/segments of the members based on for instance age, gender, HAE type, member type, and type of treatment
- Send instant e-mails to members – single or searched segments
- Send profile update e-mail reminders to members – single or segments
- Customize e-mails and profile page identity with the national member organization name and logo
- Customize welcome e-mail and profile reminder e-mail
- Fields in the directory can be suited to national needs

“We have a very strong focus on securing the data of the members when entered into HAEi Connect as we want both the national organizations and every single member to feel safe using the tool. Many precautions have been made to secure the data as well as the server. Just to mention a few: All data traffic is handled in encrypted SSL protocol, the domain [www.haei-connect.org](http://www.haei-connect.org) is privately registered, and admin access is a two-factor login with username, password and text code. Furthermore, HAEi Connect is EU GDPR compliant with staff training, consent, privacy and all needed documents for non-disclosure agreement and code of conduct”, says Ole Frølich.



Currently, HAEi Connect is used by HAE Scandinavia, HAE Kenya, HAE Australasia, HAE Mexico, and HAE Spain (AEDAF), while HAE Poland, HAE Chile, and HAE Argentina have applied.

Please visit [www.haei.org/connect](http://www.haei.org/connect) if you want to know more about how to have your national member organization move its database to HAEi Connect.



## The HAEi Global Access Program helps changing lives of HAE patients

Since announcing the appointment of Inceptua Medicines Access as the new HAEi Global Access Program (GAP) distribution partner last year, HAEi has been responding to a number of enquiries from those in countries where RUCONEST® is not commercially available.

“HAEi is committed to helping the global HAE patient community obtain access to life saving modern therapies, which is why we created the HAEi GAP. 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 patients. This is the first known program of this type initiated through a patient group”, says Project Manager Deborah Corcoran.

HAEi GAP currently provides access to RUCONEST® to patients in all countries where it is not yet commercially available, via a ‘Named Patient Program’ mechanism. 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 patient’s medical insurance.

“If you are an HAE patient and think that HAEi GAP could help you, please talk to your doctor about the program. More information about the HAEi GAP and RUCONEST® can be found on the HAEi website, including a flyer that you can share with your doctor to explain how to request access to treatment. Any formal

request for medication to the HAEi GAP must come from a physician, as Inceptua Medicines Access are not able to respond to enquiries from patients”, Deborah Corcoran explains.

If you are a physician and you think HAEi GAP and RUCONEST® could help your HAE patients, please contact HAEiGAP@inceptua.com.

HAEi is here to help patients and physicians in any way the organization can, so if you do have any questions please contact the HAEi GAP team Deborah Corcoran and Nevena Tsarovska at gap@haei.org. HAEi and the team is waiting to hear from you.

See more about the HAEi GAP at:  
[http://haei.org/hae/global\\_access\\_program](http://haei.org/hae/global_access_program)



## Join the community for HAEi youngsters

As you may recall the HAE Global Conference 2016 in Madrid, Spain contained a full track for HAE youngsters – and in August 2017 HAEi arranged the first ever HAEi Youngsters' Summer Camp, which took place in Frankfurt, Germany.

Based on connections first made or strengthened during these two events a group of energetic HAEi youngsters have decided to found the HAEi Youngsters Community.

"With presently 63 member organizations around the world, we are proud to add the HAEi Youngsters Community to our constantly growing global family", says HAEi President Anthony J. Castaldo.

Connected via social media, the HAEi Youngsters Community is an open community for everyone to join, allowing the participants to share, create new friendships and strengthen old ones, discover life-changing experiences by learning about different

cultures, countries and traditions, and make the community something bigger than the disease. As they initiators put it: "Laughing and sharing each other's daily lives, we understand the feelings and struggles of friends, patients and caregivers."

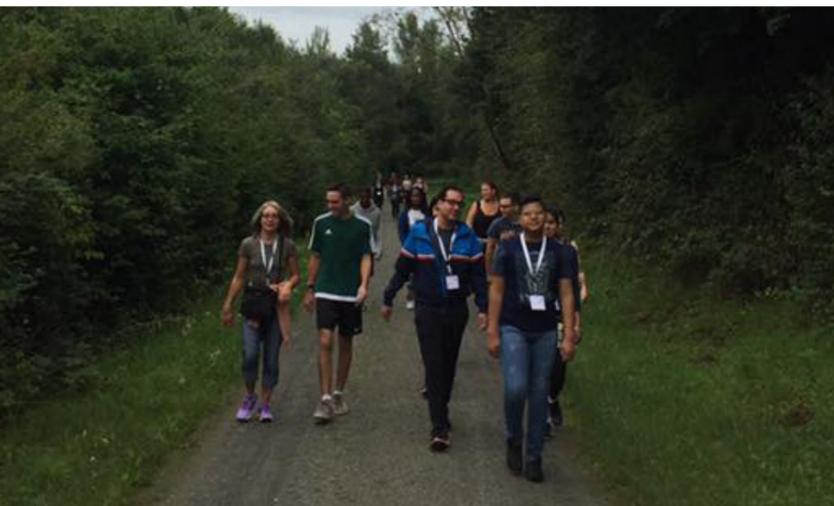
With members from 23 countries, the goal of the HAEi Youngsters Community is to work together for a better quality of life.

The HAEi Youngsters Community has designed a logo based on the overall idea "Many different faces, one big family". It symbolizes the community as a place of understanding and a forum for facing similar challenges.



If you want to know more about the #HAEi Youngsters Community feel free to visit

[www.facebook.com/groups/470967886612519](http://www.facebook.com/groups/470967886612519)



## Ask the Doctors

Physician/Scientists specialized in HAE answer patients' questions on the Facebook pages for the US HAEA Angioedema Center at the University of California San Diego as well as the US HAEA. Here is one of the most recent questions – and the reply from Dr. Sandra Christiansen, Dr. Marc Riedl, and Dr. Bruce Zuraw.

*“My doctor wants me to go on the Ruconest, which is a recombinant C1 inhibitor. I recently read a social media post where someone stated that Ruconest caused blood clots. I did see, however, that this person used a port to take the medicine. Another patient posted that Ruconest causes allergic reactions because the C1 inhibitor medicine is derived from the milk of rabbits. Can you provide some expert advice?”*

**Dr. Christiansen:** This is a very timely question for multiple reasons. As many of you are aware there have been recent manufacturing problems at Shire with Cinryze and resulting shortages. CSL Behring has also announced that they will not be able to meet the demand for plasma-derived C1 inhibitor (pdC1INH), with their available stocks. Patients are rightfully anxious about running out of medication. Recombinant C1 inhibitor (rC1INH) is identical to the plasma-derived C1 inhibitor protein. It is expressed and purified from the milk of transgenic rabbits. Intravenous rC1INH has been shown to be safe and effective for the treatment of acute attacks of angioedema in a pivotal phase III study. A Phase II study led by Dr. Riedl demonstrating efficacy for prophylaxis of attacks has also been published this last year. Fortunately for patients, there is no shortage of supply of rC1INH and the manufacturer, Pharming, is dedicated to facilitating availability to patients. As is the case for pdC1INH there have been no significant safety concerns. The questioner raises the issue of rabbit allergy. A warning about rabbit allergy is included in the package insert in the US, which stems from a single case of anaphylaxis during the trials in an individual

with rabbit allergy – which had not been disclosed to the investigators. With the manufacturing process, there are very low levels of host-related impurities (HRIs) in the final product and such that the risk is very low of a reaction even in rabbit allergic patients. A large study in a rabbit allergic cohort has not been done, however. In the event that rC1INH is the best option for a patient with a history of rabbit allergy, everything has a risk-benefit ratio. I would consider skin testing with rabbit and the rC1INH to check prior to use. If the testing for rC1INH is negative and the rabbit positive to err on the side of caution I would then give a test dose of rC1INH under observation in the office to see if it is safe to use.

**Dr. Riedl:** In order to address the question about allergic reactions to the recombinant human C1INH (rhC1INH/Ruconest), let's quickly review the process by which it's manufactured: rhC1INH is produced in rabbits that have been designed to generate the human C1INH protein in their milk. The rabbit milk goes through extensive complex processing to purify the human C1INH protein and remove all other materials. At the end of the process, the medication contains less than 0.002% rabbit-related

proteins. So it is an incredibly pure human C1INH medication. To my knowledge, there has been only one confirmed case of an allergic reaction to rhC1INH due to rabbit allergy. This occurred during the early clinical studies with the drug when a young woman treated in the study did not mention to study physicians that she had previously had allergic reactions to rabbits. On receiving the medication, she developed symptoms of a serious allergic reaction but was treated and recovered without complication. Since that time additional studies have carefully monitored for this potential allergy issue, but no additional allergic reactions related to rabbit allergy have been observed. For a while, the European regulatory agency required that all patients have rabbit allergy testing performed prior to using rhC1INH, but this requirement was removed in 2016 due to the fact no additional issues with such allergic reactions had been seen. The FDA has never required such testing though of course warns of the possible risk in the medication label. Since FDA-approval, an additional study investigating this issue showed that four of five individuals with positive allergy tests to rabbit in fact tolerated rhC1INH treatment without any allergy issues. The fifth individual was the aforementioned woman who had an allergic reaction in the study; she also had the strongest rabbit allergy test of the group. All told, it appears that rabbit allergy issues related to rhC1INH are exceedingly rare. If someone has a history of allergic reactions to rabbit exposure, then it would be important for them to speak with their physician about rabbit allergy test (blood or skin test) prior to using rhC1INH. But for the vast majority of people, this is not going to be a significant risk or concern, since rabbit allergy is quite uncommon.

**Dr. Christiansen:** As to the thrombosis (clotting) issue there is no more concern than has been raised for the pdC1INH. The rC1INH is identical to the pdC1INH with the exception of a small difference in the carbohydrate decoration on the plasma human-derived protein. It does have a shorter half-life than the pdC1INH, however, the higher dose (50 IU/kg) appears to compensate. There is really no published data linking rC1INH to thrombosis and no cases during the trials with the drug. There is much more data on pdC1INH, all of which is reassuring and can be extrapolated for the rC1INH. In a study by Henriette Farkas of 144 patients over 29 years the incidence of thromboembolism was actually less than for individuals not treated by pdC1INH. Paula Busse has also published results from an international registry on 135 subjects with 3196 infusions of pdC1INH and found no evidence to suggest that pdC1INH was an



*From a HAEi visit to the US HAEA Angioedema Center at the University of California San Diego in 2016.*

independent causative risk factor for thromboembolism. Concern about potential thrombogenesis stems from a report of off-label use in 13 neonates without HAE who developed thrombus at up to 500 IU/kg in an attempt to prevent capillary leak during cardiac bypass for severe congenital heart defects. There have been five reports of thrombosis on Cinryze in the open-label study, but all patients had underlying risk factors. Studies in animals have actually reported that C1INH may be antithrombogenic rather than prothrombogenic. All this being said there are still concerns for patients with an indwelling port which is a risk factor for thrombosis.

**Dr. Zuraw:** I can only echo what you've already said. C1INH inhibits two clotting factors, activated factor XII and activated factor XI. In C1INH deficient patients, the clotting times are shorter, possibly indicating an enhanced tendency to clot – although the relationship between these lab measurements and the clinical risk of clotting is far from simple or clear. Replacing C1INH activity with either pdC1INH or rC1INH should improve the inhibition of these clotting factors and thus reduce the risk of thrombosis. My own sense is that it is the other risk factors, especially indwelling ports, that increase the risk of clotting and that both plasma-derived and recombinant C1INH concentrates do not contribute to this risk.



## 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 should be recruiting at this moment:

### Safety of Ruconest in 2-13 Year Old HAE Patients

– recruiting in Czech Republic, Germany, Hungary, Israel, Italy, Macedonia, Poland, Romania, and Slovakia.

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

– recruiting in USA.

### A Long Term Safety Study of BCX7353 in HAE (APeX-S)

– recruiting in Austria, Denmark, Germany, Macedonia, and United Kingdom.

### C1 Inhibitor Registry in the Treatment of HAE Attacks

– recruiting in Bulgaria, Czech Republic, France, Germany, Hungary, Italy, Norway, Poland, Slovakia, and Sweden.

### Biomarker for HAE Disease Type 1 (BioHAE)

– recruiting in Germany.

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

– recruiting in Austria, Brazil, Czech Republic, Denmark, France, Germany, Greece, Israel, Italy, Spain, Sweden, and United Kingdom.

### Determination of Specific Biomarkers of Acute Attack of Angioedema Within Pediatric Population (BRADYKID)

– recruiting in France.

### The Role of the Coagulation Pathways in Recurrent Angioedema (Angiocoag)

– recruiting in France.

### Study of Clinical, Biological Characteristics and Quality of Life of Patients With Hereditary or Acquired Non Drug-induced Bradykinin-mediated Angioedema, Monitored in Besançon's Partner Site Reference Center for Studies of Kinin-mediated Angioedema (CREAK) (QUALANGIO)

– recruiting in France.

### Long Term Safety Study of BCX7353 in HAE

– recruiting in Australia, Austria, German, Hungary, Macedonia, and Switzerland.

### A study in patients to assess the effectiveness, safety and patient tolerability of different doses of BCX7353 to treat acute HAE attacks compared to placebo

– recruiting in Austria, Denmark, Germany, Hungary, Israel, Macedonia, and Switzerland.

### BCX7353 for the prevention of HAE attacks

– recruiting in Australia, Austria, Canada, Denmark, Germany, Hungary, Macedonia, Spain, Switzerland, and United Kingdom.

### A placebo controlled trial of of three doses of BCX7353 to evaluate the safety and efficacy in the prevention of attacks in patients with HAE

– recruiting in Canada, Germany, Hungary, and United Kingdom.

### A Phase 3, Multicenter, Randomized, Single-Blind, Dose-Ranging, Crossover Study to Evaluate the Safety and Efficacy of Intravenous Administration of CINRYZE® (C1 Esterase Inhibitor [Human]) for the Prevention of Angioedema Attacks in Children 6 to 11 Years of Age With HAE

– recruiting in Argentina, Germany, Italy, Mexico, Romania, United Kingdom, and USA.

### Pathophysiological study for autoimmune dysregulation of HAE

– recruiting in Japan.

### Pharmacokinetics and Safety of Human Pasteurised C1-Inhibitor Concentrate (Berinert/CE1145) in Subjects with Congenital C1-INH Deficiency

– recruiting in Italy.

### A randomized, placebo-controlled, double-blind Phase III study of the efficacy and safety of recombinant human C1 inhibitor for the treatment of acute attacks in patients with HAE

– recruiting in Italy.

Read more about these and other clinical trials at <https://clinicaltrials.gov> and [www.who.int/ictcp/en](http://www.who.int/ictcp/en).

## Global Advocacy Work

### Recent events

- 8 - 10 January:** HAEi had meetings in **Copenhagen, Denmark** with Marine Travel for the travel grant program as well as with ConferenceCare for logistics around the 2018 HAE Global Conference.
- 10 - 12 January:** HAEi participated in the annual Plasma Users Group (PLUS) stakeholder meeting in **Dublin, Ireland**.
- 19 - 20 January:** HAEi had its Executive Committee meeting and workshop in **Tokyo, Japan**. BioCryst, CSL Behring, Shire and Pharming took part in order to discuss strategic approaches for development of and access to medicines.
- 24 - 25 January:** HAEi met with the Portuguese member organization in **Lisbon, Portugal**. The focus was to get an update on the current situation in Portugal and to evaluate how HAEi can help HAE Portugal to achieve a better and sustainable solution for Portuguese patients.
- 16 - 18 February:** HAEi had its Regional Patient Advocate workshop in **Frankfurt, Germany**.
  - 2 - 4 March:** HAEi participated in the annual American Academy of Allergy, Asthma and Immunology (AAAAI) in **Orlando, USA**.
  - 8 - 10 March:** HAEi conducted and hosted the 2018 HAEi China Patient and Physician Workshop in **Beijing, China**.
  - 8 - 10 March:** HAEi participated in the 20th anniversary of HAE Spain (AEDAF) in **Madrid, Spain**.
  - 20 - 22 March:** HAEi was in **San Diego, USA** to work on youngsters programs and concepts with the US HAEA Youth Representative Lisa Facciolla as well as on HAEi strategies for 2018. A visit to the US HAEA Angioedema Center at UCSD was included in the intense program.

### Upcoming events

- 3 - 5 April:** HAEi will have meetings in **Frankfurt, Germany** to finalize the program for 2018 HAE Global Conference and work on additional content for this big event.
- 12 - 14 April:** HAEi will participate in the HAE Global Registry Foundation's Registry Meeting in **Bucharest, Romania**.
- 1 - 2 May:** HAEi will meet with the financial auditors Ofico in **Lausanne, Switzerland** to finalize the 2017 fiscal year.
- 14 - 20 May:** HAEi will be in **Vienna, Austria** for the 2018 HAE Global Conference and the HAEi Jakobsweg Walk.
- 27 - 30 May:** HAEi will attend the 2018 European Academy of Allergy and Clinical Immunology (EAACI) meeting taking place in **Munich, Germany**.



## Medical Papers

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

### **HAE education in otolaryngology residencies: survey of program directors – by V.A. Patel, The Pennsylvania State University, USA, et al.:**

This study is the first to offer insight into C1-INH-HAE education and management principles in otolaryngology training programs. Surveyed program directors believe residents need a strong knowledge base in the management of C1-INH-HAE but less than half feel their trainees acquire the necessary exposure to this emergent disease process. Future research efforts in this area should aim to determine optimal educational activities as well as how to best incorporate this into otolaryngology residency curricula. (*Int Forum Allergy Rhinol.*, March 2018)

### **Specialist Advice Support for Management of Severe HAE Attacks: A Multicenter Cluster-Randomized Controlled Trial – by N. Javaud, Université Paris, France, et al.:**

We included 100 patients in a SOS-hereditary angioedema (SOS-HAE) call center group where emergency physicians were trained to advise or prescribe specific treatments and 100 in a control group. During two years, there were 2,368 HAE attacks among 169 patients. The mean number of hospital admissions per patient in the period was significantly greater in the usual-practice group. A national dedicated call center for management of severe HAE attacks is associated with a decrease in hospital admissions and may be cost-effective if facilities and staff are available to deliver the intervention alongside existing services. (*Ann Emerg Med.*, March 2018)

### **Evaluation of infectious and non-infectious complications in patients with primary immunodeficiency – by S. Bazregari, Hormozgan University of Medical Sciences, Iran, et al.:**

Primary immunodeficiency disease patients are at risk of multiple infectious and non-infectious problems. Timely diagnosis of Primary immunodeficiency diseases not only improves their outcome and quality of life, but

also helps prevent these troubling complications. (*Cent Eur J Immunol.*, December 2017)

### **HAE with normal C1 inhibitor: Four types and counting – by B.L. Zuraw, University of California San Diego, USA:**

HAE has been described as an experiment in nature that has taught us much of what we know about the plasma contact system. The clear phenotype of HAE, as well as the easily measurable response to treatment, make HAE an excellent model to test experimental hypotheses. Nature's experiments continue to teach us, and we might now see HAE contribute to our understanding of vascular biology. (*J Allergy Clin Immunol.*, March 2018)

### **Health-Related Quality of Life with Subcutaneous C1-Inhibitor for Prevention of Attacks of HAE – by W.R. Lumry, Allergy and Asthma Research Associates Research Center, Texas, USA, et al.:**

In patients with frequent HAE attacks, a treatment strategy of routine prevention with self-administered twice weekly C1-INH (subcutaneous) had a greater impact on improving multiple HAE-related health-related quality of life impairments, most notably anxiety and work productivity, compared with on-demand treatment alone (placebo prophylaxis). (*J Allergy Clin Immunol Pract.*, January 2018)

### **New Treatments for HAE – by N.M. Johnson NM1 and M.A. Phillips, Virginia Tech Carilion School of Medicine, USA:**

The past decade has been marked by the development of new treatment options for the management of HAE. Many international and national consensus guidelines exist, but none recommend specific therapies, citing a lack of head-to-head trials. The decision to use prophylactic therapy requires weighing the patient's disease burden with the benefits and risks of therapy.

For those patients who do require preventative therapy, Cinryze (C1 esterase inhibitor) appears to be equally as effective as synthetic androgens, and neither is recommended above the other. Berinert (C1 esterase inhibitor), Ruconest (conestat alfa), Firazyr (icatibant), and Kalbitor (ecallantide) appear to be equally efficacious, and the decision of which particular agent to use should depend on patient factors, including setting of administration and responsiveness to a particular agent during previous acute episodes. (*Skin Therapy Lett.*, January 2018)

### **HAE with a mutation in the plasminogen gene – by K. Bork, Johannes Gutenberg University, Germany, et al.:**

HAE with normal C1-INH (HAEnCI) may be linked to specific mutations in the coagulation factor 12 (FXII) gene (HAE-FXII) or functional mutations in other genes that are still unknown. We sought to identify and characterize a hitherto unknown type of HAE with normal C1-INH and without mutation in the F12 gene. HAE with a mutation in the plasminogen gene is a novel type of HAE. It is associated with a high risk of tongue swellings. (*Allergy.*, February 2018)

More papers can be found at for instance the National Center for Biotechnology Information, U.S. National Library of Medicine at [www.ncbi.nlm.nih.gov/pubmed](http://www.ncbi.nlm.nih.gov/pubmed).

# News from the Industry

6 January 2018

**Adverum Biotechnologies, Inc.**, a clinical-stage gene therapy company targeting unmet medical needs in serious rare and ocular diseases, reviews recent progress and provides an outlook for 2018:

“In 2017, our newly-assembled team achieved our stated goal of transforming Adverum into a clinical-stage company,” said Amber Salzman, Ph.D., president and CEO of Adverum Biotechnologies. “We are accelerating the development of our pipeline of gene therapies and plan to submit an investigational new drug application with the FDA in the second half of 2018, for ADV-053 in HAE. With our platform of industry-leading technology and experienced leadership team, we are well positioned to advance our pipeline of novel gene therapies in 2018.”

(Source: Adverum)

**ADVERUM**  
BIOTECHNOLOGIES

6 January 2018

**KalVista Pharmaceuticals, Inc.** will be initiating a Phase 1 trial for KVD 900, the second candidate in the oral HAE portfolio. The trial commenced in December 2017, in line with KalVista’s previously stated 2017 objectives. Says Andrew Crockett, CEO of KalVista:

“2018 will be an exciting year for our HAE portfolio, with our second oral plasma kallikrein inhibitor candidate in a Phase 1 clinical trial and an anticipated regulatory filing for a third candidate before year-end.”

KVD900 is the second clinical candidate from a portfolio of oral plasma kallikrein inhibitors for potential treatment of HAE. KalVista’s strategy is to develop and evaluate multiple oral molecules in pursuit of a best-in-class therapy for HAE patients. This portfolio approach may also lead to the development of multiple molecules to address an unmet need in both prophylactic and on-demand market segments. The Phase 1 trial of KVD900 is actively screening healthy volunteers to evaluate the safety, tolerability, and exposure of the drug candidate and a plasma-based assay will be used to assess the pharmacodynamic effect of KVD900. KalVista expects to provide an update on the status and progress of the HAE portfolio, including KVD900, in mid-2018, with a goal to advance at least one additional candidate to the clinic before the end of 2018.

(Source: KalVista)

**KalVista**  
PHARMACEUTICALS

## 11 January 2018

**Verseon** has presented preclinical results in its HAE program at the 2018 Biotech Showcase in San Francisco, USA. The data show that Verseon's plasma kallikrein inhibitors are well-suited as oral treatments for this rare genetic disease.

To treat HAE, Verseon is developing oral small-molecule inhibitors of plasma kallikrein, a serine protease central to the HAE pathway. The Company's class of drug candidates spans multiple different chemotypes that show excellent in vitro potency and are selective against other related serine proteases.

In contrast to currently marketed therapeutics, which rely on intravenous or subcutaneous injections, Verseon's drug candidates demonstrate pharmacokinetic exposure suitable for convenient oral dosing. Dr. David Kita, Verseon's Vice President of R&D, also presented promising preclinical results that show strong reduction of swelling after oral administration in a well-established HAE efficacy model.

"We are very encouraged by these results," said Dr. Kita. "We started the HAE program only recently, and have already demonstrated that our oral plasma kallikrein inhibitors have great potential for the effective management of HAE attacks. The next milestone this year will be the nomination of our first development candidate for advancement into clinical trials."

(Source: Verseon)



## 19 January 2018

The U.S. Food and Drug Administration (FDA) has accepted for review **Pharming Group N.V.**'s supplemental Biologics License Application (sBLA) for Ruconest [Recombinant Human C1 Esterase Inhibitor/ conestat alfa] for routine prophylaxis to prevent attacks in adult and adolescent patients with HAE. The FDA has indicated that the sBLA is sufficiently complete to permit a substantive review and has set an action date of 21 September 2018.

Ruconest is currently approved for the treatment of acute attacks in adult and adolescent patients with HAE. If approved for this new indication, Ruconest would become the first C1 inhibitor therapy that would be approved for both acute treatment and prophylaxis of HAE attacks.

(Source: Pharming)



## 22 January 2018

**BioCryst Pharmaceuticals, Inc.** and **Idera Pharmaceuticals, Inc.** have signed a definitive merger agreement to form a new enterprise focused on the development and commercialization of medicines to serve more patients suffering from rare diseases. The combined company will be renamed upon closing and will be led by Vincent Milano, CEO of Idera, who will also serve as a member of the Board. BioCryst Chairman, Robert Ingram, will be Chairman of the Board of the combined company and BioCryst CEO Jon P. Stonehouse will serve as a member of the Board of Directors.

"Both of our companies have aspired to become successful providers of therapeutics for patients suffering from rare life-threatening diseases. Both share a culture that puts patients first and keeps their interests at the very core of what we do, and how we do it," stated Vincent Milano, Idera's CEO. "By merging our unique talents, experiences, and assets, we instantly strengthen our ability to become a significant force for patients suffering from a broad range of rare diseases. We will also gain operational synergies and strengthen our financial position."

"The new company will have a robust late-stage pipeline with two Phase 3 candidates and two Phase 2 candidates, with several important catalysts for these programs anticipated in 2018. We will also have a deep early-stage pipeline that will continue to expand via our combined drug discovery capabilities and clinical expertise. We are extremely excited about our combined rare disease portfolio. We believe we will be well positioned to bring that portfolio to market with our proven commercial leadership team and business development opportunities."

"Bringing these two companies together accelerates the strategic initiatives of both organizations and immediately forms a substantial and differentiated biotech company serving patients in the rare disease community," stated Jon P. Stonehouse, BioCryst's President and CEO. "Combining our respective pipelines, infrastructures and financial resources should enable the new company to grow faster, deliver for patients more rapidly and ultimately create sustainable shareholder value well beyond what either would achieve separately."

The combination of two companies is expected to capitalize on the collective skills sets, internal expertise and combined assets to create a comprehensive, sustainable rare disease-focused biotechnology leader highlighted by among other things:

- A robust development pipeline, including 4 late stage programs that provide near-term commercial and partnering opportunities:
  - BCX7353 – Phase 3 program for the prophylactic treatment of HAE in a capsule formulation with FDA orphan drug designation
  - BCX7353 – Phase 2 program for the acute treatment of HAE in a liquid formulation
- Proven leadership across commercial, development, scientific and clinical functions, providing a combination of management and scientific talent that marries the necessary ingredients for a successful, sustainable biotech company.
- Synergistic discovery engines highlighted by two distinct research technologies and expertise which expands the number of rare disease therapeutic targets and candidates.
- Financial strength with approximately \$243 million net cash balance (unaudited proforma cash balance as of December 31, 2017), with opportunities to add further non-dilutive capital to fund internal clinical development efforts, commercial launch efforts, and continued business development activities.

(Source: BioCryst)



## 24 January 2018

The U.S. Food and Drug Administration (FDA) has granted approval for the technology transfer of Cinryze drug product manufacturing process to the **Shire** manufacturing site in Vienna, Austria.

“With the FDA’s approval of the tech transfer, we are pleased to strengthen our supply reliability for adult and adolescent HAE patients who count on Cinryze to help prevent attacks,” said Matt Walker, Head of Technical Operations.

Shire will begin commercial manufacturing of Cinryze drug product in Vienna in the first quarter of 2018. Cinryze will also continue to be produced by a third-party supplier, providing an additional supply source to meet patient demand.

Cinryze is one of the leading therapies approved for the treatment of HAE in adolescent and adult patients. It can help reduce how often attacks occur, how severe they are, and how long they last. Shire’s OnePath program offers patients help with their access to the therapy and product support needs.

(Source: Shire)



## 16 February 2018

The U.S. Food and Drug Administration (FDA) has accepted the Cinryze (C1 esterase inhibitor [human]) supplemental Biologics License Application (sBLA) to expand the currently approved indication to include children aged six years and older with HAE. The filing has received Priority Review designation from the FDA, which means Cinryze has an accelerated review target of eight months, instead of the standard of 12 months. The FDA is expected to provide a decision on the expanded indication of Cinryze by June 20, 2018, based on the Prescription Drug User Fee Act V action date.

Cinryze has been approved in the U.S. since October 2008 for routine prophylaxis against attacks in adolescents and adults living with HAE.

“Adults and adolescents living with HAE have used Cinryze to help reduce the frequency and severity of attacks for nearly a decade,” said Jennifer Schranz, Global Development Lead, HAE, **Shire**. “Shire committed to studying the safety and efficacy of our HAE therapies in children aged six years and older because we understand the importance of this work to families in the HAE community. We look forward to working closely with the FDA in the coming months on this important review.”

This sBLA for Cinryze is supported by data from two open-label studies (LEVP 2006-1 and LEVP 2006-4) and two pediatric clinical studies (0624-203 and 0624-301). The pediatric studies used in this filing are the only clinical trials investigating a prophylactic therapy in the HAE pediatric population.

The FDA grants Priority Review designation to drugs that have the potential to provide significant improvements in the safety or effectiveness for the treatment, diagnosis or prevention of a serious disease.

(Source: Shire)



## 20 February 2018

The U.S. Food and Drug Administration (FDA) has accepted the Biologics License Application (BLA) and granted priority review for Lanadelumab (SHP643), an investigational treatment being evaluated for the prevention of angioedema attacks in patients 12 years and older with HAE.

“Every day, patients living with HAE struggle to manage their disease – not knowing when their next attack will occur,” said Andreas Busch, Ph.D., Executive Vice President, Head of Research and Development at **Shire**. “Lanadelumab if approved will be the first monoclonal antibody for HAE, a serious and potentially life threatening disease. Lanadelumab provides a new mechanism of action inhibiting plasma kallikrein for the prevention of HAE attacks. Lanadelumab will offer patients a new option to help control this disease with the potential to change the treatment paradigm. The FDA’s decision underscores Shire’s serial innovation in HAE and commitment to improving treatment options for patients.”

The BLA for Shire’s investigational HAE treatment is supported by data from four clinical trials, including HELP™, the pivotal Phase 3 efficacy and safety study, along with interim data from its extension study. HELP is the largest prevention study in HAE conducted to date, and enrolled a total of 125 patients aged 12 years and over with type I/II HAE. The HELP study demonstrated that subcutaneous administration of 300 mg Lanadelumab once every two weeks resulted in an 87% reduction in the mean frequency of HAE attacks. In addition, an exploratory endpoint, which will require further confirmatory studies, showed that during the steady state stage of the trial (day 70-182) a 91% attack reduction was achieved with 8 out of 10 patients reaching an attack free state. In this study, no treatment-related serious adverse events or deaths were reported. The most common adverse event was injection site pain (29.3% placebo vs. 42.9% combined Lanadelumab arms).

“Physicians as well as patients in the HAE community are excited to see Lanadelumab moving forward for FDA review because there is now the real possibility of having a new way to prevent HAE attacks,” said Aleena Banerji, M.D., Massachusetts General Hospital, Boston,

MA, and clinical trial investigator. “As an investigator, I am appreciative of the HAE patients who participated in the clinical trial to help advance science in a way that may transform the treatment of HAE.”

The FDA grants Priority Review designation to drugs that have the potential to provide significant improvements in the safety or effectiveness for the treatment, diagnosis or prevention of a serious disease. Drugs with Priority Review designation have an accelerated review target of eight months, instead of the standard of 12 months. The FDA is expected to provide a decision on Lanadelumab by 26 August 2018, based on the Prescription Drug User Fee Act V action date.

(Source: Shire)



## 27 February 2018

The European Medicines Agency (EMA) has granted an accelerated assessment for Lanadelumab (SHP643), an investigational treatment being evaluated for the prevention of angioedema attacks in patients 12 years and older with HAE.

“The EMA decision, coupled with the U.S. FDA’s recent Priority Review designation for Lanadelumab, reinforces **Shire’s** dedication to advancing new treatment options for patients suffering from HAE,” said Andreas Busch, Ph.D., Executive Vice President, Head of Research and Development at Shire. “As the first investigational monoclonal antibody being studied in HAE, Lanadelumab utilizes a novel mechanism of action inhibiting plasma kallikrein for the prevention of HAE attacks. We look forward to further progressing Lanadelumab through the regulatory review process, as we strive to bring new and innovative solutions to the patients who need them most.”

Shire is on track to submit its EU Marketing Authorization Application (MAA) in the coming weeks. Accelerated assessments by the CHMP of a marketing authorization filed under the centralized European procedure, reduces the number of evaluation days required, from 210 to 150. The EMA will grant, upon request, accelerated assessment of an EU MAA if they deem the product to be of major interest for public health and therapeutic innovation.

“I’m excited to see Lanadelumab receive an accelerated assessment in Europe because we are one step closer to potentially having a new option to help prevent HAE attacks,” said Marcus Maurer, Prof. Dr. Med., Department of Dermatology and Allergy, Charité – Universitätsmedizin Berlin, Germany, and clinical trial investigator. “As an investigator, I want to express my gratitude to the HAE patients and their families who participated in the clinical trial and the dedicated study site personnel, who have helped to advance science in a way that may transform the treatment of HAE.”

(Source: Shire)



## 28 February 2018

**BioCryst Pharmaceuticals, Inc.** has dosed the first patient in APeX-S, a long-term safety trial evaluating two dosage strengths of BCX7353 administered orally once-daily as a preventive treatment in patients with HAE.

“Initiation of the APeX-S trial is an important milestone to support filing and approval of BCX7353, and furthers our core strategy of bringing a once-daily, oral prophylactic treatment to HAE patients,” said Jon Stonehouse, CEO. “Long-term safety results from this trial will supplement efficacy and safety data from the APeX-2 Phase 3 trial, which is also expected to commence in the first quarter of 2018. The ability to run these trials concurrently is beneficial, as it may allow us to more rapidly get this important medicine into the hands of patients who are seeking a better quality of life by eliminating their current injection-based treatment programs.”

APeX-S is an open label two-arm trial to evaluate the safety of two dose levels of BCX7353 (110 mg once daily and 150 mg once daily) over 48 weeks in patients with Type I and II HAE. The trial will enroll approximately 160 patients. Initially, APeX-S will enroll patients who have participated in a previous clinical trial of BCX7353, and in time will be expanded to include patients who have not previously received the drug. Endpoints of the trial include long-term safety, durability of response and quality of life measures.

“The launch of this trial has brought within arm’s reach the fulfillment of the long-standing need of HAE patients for a convenient, easy-to-administer, oral remedy, developed on purpose for the prophylaxis of angioedema attacks,” said Henriette Farkas, MD, PhD, DSc and Principal Investigator of the APeX-S trial.

(Source: BioCryst)



## 28 February 2018

At the announcement of the **BioCryst Pharmaceuticals, Inc.** financial results for 2017 President & CEO Jon P. Stonehouse said:

“Our team made significant progress in 2017 and we are off to a strong start in 2018. We are keenly focused on continuing that momentum by advancing our pipeline, adding additional programs and driving our BCX7353 oral prophylactic program toward approval and launch. We are on track to report top-line results from the APeX-2 pivotal trial of BCX7353.”

Mr. Stonehouse continued, “In January, we announced our proposed merger with Idera Pharmaceuticals, Inc. that we believe will build greater and more sustainable value for the benefit of stockholders as well as patients with rare diseases beyond what we could achieve alone. The BioCryst Board determined this combination was compelling from both a strategic and financial perspective following a careful evaluation of a range of strategies to enhance long-term stockholder value. The transaction will create a leading rare disease company with a robust pipeline including two promising Phase 3 programs and combines synergistic discovery engines that will not only expand the number of rare diseases we can target but create meaningful opportunities for differentiation in the market through joint small molecule and oligo treatments. Importantly, joining with Idera will also enable us to achieve cost synergies and increase our financial strength and flexibility.”

(Source: BioCryst)



## 7 March 2018

From the **Adverum Biotechnologies, Inc.** financial results for the fourth quarter ended 31 December 2017 and corporate update:

“We enter 2018 with significant momentum following a year of critical execution to transform Adverum into a clinical-stage company,” said Amber Salzman, Ph.D., President and CEO. “We plan to submit an Investigational New Drug Application with the FDA in the second half of 2018 for ADVM-053 in HAE. We begin this exciting year of clinical development and regulatory progress in a strong position, funded to execute our three lead programs through the end of 2019 with preliminary clinical data for at least two of these programs.”

(Source: Adverum)



## 7 March 2018

At the presentation of the **Pharming Group N.V.** preliminary (unaudited) financial report for the full year ended 31 December 2017 CEO Sijmen de Vries said:

“The remarkable growth reported in 2017 was a direct result of our strategic decisions to reacquire the commercial rights to Ruconest in North America and implement direct marketing in the major Western European markets. We successfully established the commercial infrastructure to support our existing patients and expand the patient population benefiting from Ruconest. As a result, we delivered 547% growth in revenues from product sales in one year and reported our first year of operating profitability. We also continued to invest in our long-term growth through the expansion of the improved delivery methods for Ruconest. We are confident that with our increasing patient reach and advancing pipeline, we will be able to continue to deliver significant value to our stakeholders.

The HAE market is dynamic and product choice has increased and will continue to increase as new products enter the market for prophylaxis. Ruconest has a unique competitive advantage in that it remains the only product with the potential to be approved for both prophylaxis and treatment of attacks of HAE in the same dosage form. In order to increase the convenience of Ruconest for patients, we are also developing new forms of Ruconest with new routes of administration, including subcutaneous and intramuscular injection.

During the year we have also taken next steps in the initiation of clinical development for additional indications for Ruconest, including support for as-yet undisclosed Investigator Sponsored Studies.

As a result of taking direct control of key EU and US markets, we now operate with an appropriate commercial presence in both Western Europe and the USA and can focus fully on delivering on our commitment to become a net earnings-generating company during 2018.

(Source: Pharming)



## 15 March 2018

In a keynote address on patients' access to medicines at the eyeforpharma Barcelona summit, **CSL Behring** CEO and Managing Director Paul Perreault told fellow biotech industry leaders that access to life-saving medicines remains a global challenge that cannot be ignored.

“Our role in ensuring patients have access to these medicines is an imperative, not an afterthought. As an industry, we must be a reliable and engaged partner in understanding and overcoming barriers to access,” said Perreault.

Global barriers to patient access include regulatory delays of approving new therapies, reimbursement challenges, and supply disruptions. In addition, Perreault emphasized the importance of promoting early diagnosis and treatment of conditions to give patients the best opportunity to effectively manage their illnesses.

“Patients have taught us that our industry can deliver innovative medicines to the market and prove their value, but without timely and accurate diagnoses, we've failed,” he said.

Perreault noted it may take years, multiple visits to different doctors, and several misdiagnoses before a person with a rare disease is accurately diagnosed. He encouraged industry to engage patients and patient advocacy groups that are most familiar with specific conditions in order to promote better awareness and education.

CSL Behring closely collaborates with patient groups such as HAEi.

Perreault shared lessons learned throughout his career to ensure patient access to life-saving therapies:

- To start conversations about unmet needs and the value of medicines early, and to listen closely.
- To put “boots on the ground” in different countries to

help navigate local laws and regulations.

- To strengthen Patient Focus at all levels of the organization, in all functions.

“Although CSL Behring has grown significantly, I never want us to lose connection with the people who rely on our medicines,” said Perreault. “I tell our people that they should work every day like a patient's life depends on it because it usually does. I can't think of a better motivator.”

(Source: CSL Behring)

## CSL Behring

## 16 March 2018

**BioCryst Pharmaceuticals, Inc.** announces the dosing of the first patient into APeX-2, a Phase 3 clinical trial evaluating two dosage strengths of BCX7353 administered orally once-daily (QD) as a preventive treatment to reduce the frequency of attacks in patients with HAE.

“The ability to safely prevent attacks of angioedema with an oral medicine has been a dream for many HAE patients. Today marks a significant milestone toward the realization of that dream,” said Bruce Zuraw, MD and Principal Investigator of the APeX-2 trial. “I am excited to be able to participate in this important trial, which we believe will lead to an effective oral prophylactic treatment offering HAE patients the opportunity to lead a normal life.”

APeX-2 is a randomized, double-blind, placebo-controlled, three-arm trial testing two doses of BCX7353 (110 mg and 150 mg) for prevention of angioedema attacks. The trial is expected to enroll approximately 100 patients with Type I and II HAE in the United States, Canada, and Europe. The primary efficacy endpoint of APeX-2 is the rate of angioedema attacks over 24 weeks of study drug administration.

“Beginning dosing in the APeX-2 pivotal trial of BCX7353 brings us one step closer to providing a once-daily, oral prophylactic treatment to HAE patients. Our entire organization is encouraged and excited by this important milestone, largely because both physicians and patients have told us they are waiting for a convenient and efficacious HAE treatment that allows patients to lead a more normal life,” said Jon Stonehouse, CEO of BioCryst. “We look forward to reporting top-line results from the APeX-2 Phase 3 trial in the first half of 2019.”

(Source: BioCryst)



## 16 March 2018

Imagine not knowing when you might experience unpredictable, severe and painful swelling without warning in different parts of your body, including the abdomen, face, and larynx. This is what it's like for patients with HAE who often go undiagnosed well into their adult years, "imprisoned" by their rare disorder.

Life Sciences Pennsylvania presented its 2018 Patient Impact Award to **CSL Behring** for developing HAEGARDA®, C1 Esterase Inhibitor Subcutaneous (Human), which has been shown to reduce HAE attacks by 95% versus placebo (median), and is administered subcutaneously rather than intravenously.

Most patients can learn to administer their own injections subcutaneously at a time and place that's convenient for them. The therapy can improve the lives of patients who often describe the impact of HAE on their lives as being like a "roller coaster" because of its unpredictability.

In accepting the award, CSL Behring's Executive Vice President and Chief Commercial Officer, Bill Campbell said the company and its employees are proud to receive the 2018 Patient Impact Award as another affirmation that the 20,000 employees of CSL, "always put patients first."

"HAEGARDA," Campbell continued, "is the most recent example of CSL Behring's commitment to delivering on its promise to bring innovative new medicines to patients with rare and serious diseases. We begin by listening to patients and healthcare providers to better understand the types of medicines they need and want."

HAEGARDA was granted Orphan Drug Exclusivity for seven years by the U.S. Food & Drug Administration.

(Source: CSL Behring)

**CSL Behring**

## 16 March 2018

**KalVista Pharmaceuticals, Inc.** reports operational and financial results for the fiscal third quarter ended 31 January 2018.

“We are pleased to have the second candidate from our oral HAE portfolio in a Phase 1 trial as we continue to pursue a best-in-class therapy,” said Andrew Crockett, CEO of KalVista.

KalVista has announced the initiation of a Phase 1 trial for KVD900, the second clinical candidate in the HAE portfolio. KalVista also intends to bring at least one additional HAE drug candidate to the clinic before the end of 2018.

(Source: KalVista)





# HAEi around the world

Currently there are HAE member organizations in 63 countries. You will find much more information on the HAE representations around the globe at [www.haei.org](http://www.haei.org) as the world map will provide you with contact information for the 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 HAEi receives fresh data from the national member organizations.

