

Global perspectives:

HAEI MAGAZINE · ISSUE 4/2017



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



Dear HAEi Friends,

On behalf of the HAEi Executive Committee, I wish all HAEi friends and their families a joyous holiday season and sincere best wishes for a healthy New Year!

As you read through the pages of this final 2017 edition of the HAEi magazine, I am sure you will get a sense of what we have seen building throughout the year – a global HAE advocacy movement.

2017 has been a year of exciting growth and progress for HAEi's 61 member organizations. Throughout the year we have witnessed remarkable progress as our HAEi groups increase their membership and expand activities to address the fundamental advocacy issues – awareness, diagnosis, and access to modern HAE therapies. The year is also notable for the emergence of our HAEi Youth as a force for global HAE advocacy. In August, a group of energetic youngsters got together at the HAEi Summer Camp in Frankfurt, Germany to laugh, share experiences with daily life, and understand the feelings and struggles that come with having HAE. The group now represents 23 countries and intends to actively participate in the global fight for a better quality of life. The wisdom and positive influence of these youngsters is exhibited by the logo they have designed for the group, which represents "Many different faces, one big family".

HAEi has always focused on helping our member organizations manage their operations, and we are excited to offer an automated solution. HAEi Connect is a data security compliant user-friendly database tool that will simplify membership management and enhance the ability to disseminate information, conduct surveys, and recruit for clinical trials. The HAEi Regional Patient Advocates – who continue to support existing member organizations and actively work with patients and physicians looking to create a HAE group – will be responsible for rolling out HAEi Connect.

Looking to the New Year, we are extremely excited to once again bring the HAEi community together at the 2018 HAE Global Conference that will take place in Vienna, Austria, 17-20 May. This gathering of HAE patients, caregivers, healthcare professionals, and industry representatives will be the largest international HAE meeting ever held. The focus of the conference is: "Take Control of Your HAE." Attendees will hear about the latest HAE research and therapies, and have ample opportunities to share ideas on strategies for (1) building a strong advocacy organization, (2) improving time to diagnosis, and (3) gaining access to modern lifesaving therapies. In addition, based on our experience from past HAE Global Conferences, we can predict that everyone will have a lot of fun!

Warmest Holiday Regards,

Anthony J. Castaldo
President, HAEi

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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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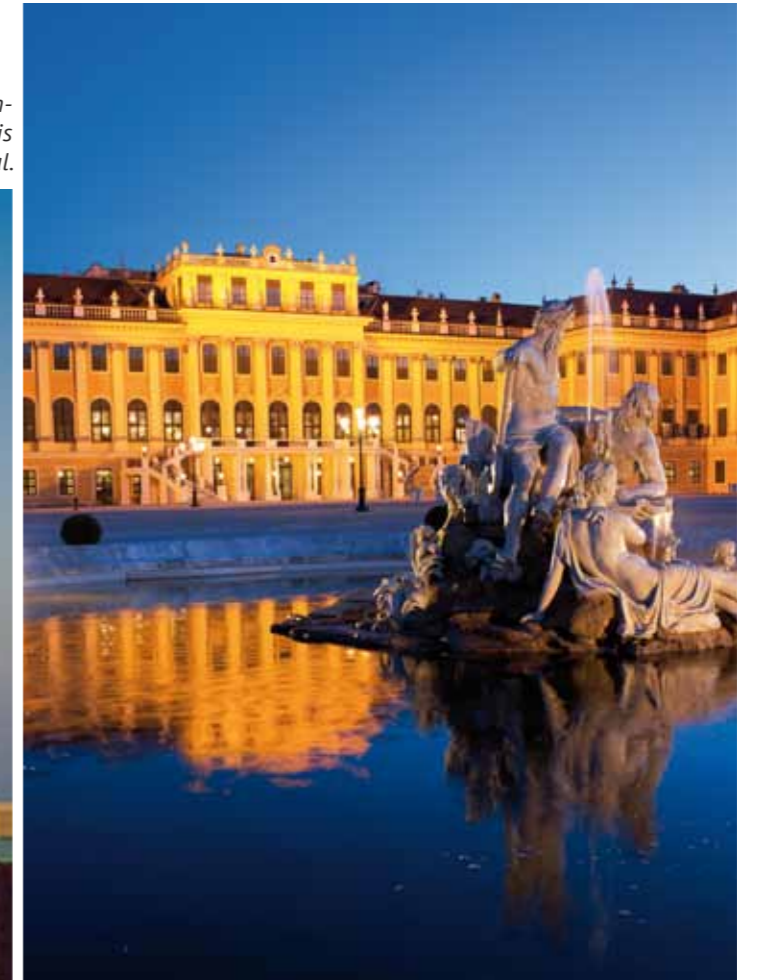
58 HAEi AROUND THE WORLD



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#haeGC18



HAE Global Conference 2018

17-20 May 2018 the global HAE community will again unite for the HAE Global Conference – this time in Vienna, Austria.

“Once again, we welcome HAE patients, caregivers, healthcare professionals, and industry representatives to the largest international gathering of its kind solely with a focus on HAE”, says HAEi President, Anthony J. Castaldo.

The theme for the 2018 HAE Global Conference is “Take Control of Your 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.

“Registration will open very soon, and in several stages – and since we again offer some extremely attractive registration rates and conditions, anyone interested in attending should already now contact their national member organization and let them know about their interest in this exciting conference, where we expect to gather some 650-700 people,” says HAEi Executive Director, Henrik Balle Boysen.

A robust program will focus on a number of important areas:

- Patient identification and diagnosis – training (including a toolkit handout) that reinforces the importance of testing for every member of a family affected by HAE, including relatives
- Education regarding currently available medicines as well as therapies undergoing clinical trials
- Advocacy training on strategies and techniques for (1) building a solid local HAE organization, (2) persuading health ministries and/or insurers to provide access to and reimbursement for HAE medicines
- Training for raising HAE awareness by strategic use of social media, holding local events, and obtaining local press coverage
- Youth Advocacy – there will be a separate track that enables young patients to interact, share their stories, get together with expert physicians to learn more about HAE medicines, and learn about advocacy from the HAEi Regional Patient Advocates.

HAEi is working hard to finalize the program for the fourth HAE Global Conference, but can already now promise some very nice surprises including traditional Austrian culture and other elements to further enhance the possibility of networking with fellow HAE friends.

“We have changed the format of some sessions, to make them even more attractive and engaging”, says Henrik Balle Boysen.

Similar to previous conferences, HAEi is offering travel grants to as many patients and caregivers (close relatives) as possible. However, based on the experience from the last two global conferences, HAEi will introduce a new policy for everybody receiving a travel grant.

“As always, we try to be as flexible as possible with travel dates, but we expect that everybody who has received a travel grant – or who participates at any of the reduced conference rates – will be at the venue and actively participate in the conference sessions on Friday as well as Saturday. Arrival will be on 17 May 2018 and departure on 20 May 2018, but for those who want to come earlier and/or stay longer, we will offer the option of booking extra room nights at our conference rate”, says Henrik Balle Boysen:

“When checking in at the Hilton Vienna, the hotel will block an amount on the participant’s credit card. At checkout, this amount will be released again once

the conference sessions on both 18 and 19 May have been attended. Consequently, it is very important to remember to scan the personal conference badge, both when entering a session Friday and Saturday, and when leaving the session. Saturday late afternoon or early evening we will then look at the attendance for each of the participants. Should someone fail to show up to the sessions on Friday and Saturday, he or she will be charged the full amount. However, since we expect everybody to attend the sessions the likelihood of anyone being charged is very small.”

Also, HAEi is in the final stages of launching the 2018 HAE Global Conference registration website, where patients and care givers (relatives) are able to request travel grants (similar to previous conferences). All travel grant receivers will be approved by their national member organization prior to receiving an itinerary from the HAEi conference travel agency Marine Travel.

Read all about the registration process and the travel grant application at the 2018 HAE Global Conference registration website that will be officially launched just after New Year. Make sure to stay tuned via the HAEi website – and don’t forget to sign up for the HAE Global Conference newsletter at <http://haei.org/haegc18>.

News from the Regional Patient Advocates

The HAEi Regional Patient Advocates (RPAs) have been working hard on a number of initiatives and we are delighted to bring you some updates from the last few months. As the number of RPAs have grown, we are not able to feature updates from every country or even every region in each magazine. You can find out more about all of the RPAs on the HAEi website and check which countries each RPA supports via the interactive map on www.haei.org.

Updates from the recent regional workshops in South Eastern Europe as well as Central and Eastern Europe are included separately in this magazine.



Patricia Karani
Sub-Saharan Africa



IN GENERAL

Since joining HAEi as RPA in July 2017, I have been establishing contact with patients and healthcare professionals across the region and encouraging some fantastic progress. In October, the first HAE patient and doctor meeting was held in South Africa, which you can read about in this magazine. I have also been busy organizing the first HAE Doctors Seminar for Kenya, which is scheduled to take place in January 2018.



NAMIBIA

A patient recently contacted me seeking assistance in Namibia. The patient and two children have a diagnosis of HAE and I am now working with this patient to establish contact with doctors and medical officers in Namibia, with the intention to set up a patient group in the country. This could be a fantastic step to bring together people with HAE in Namibia.



SWAZILAND

I am in contact with a doctor who has a real interest in helping to improve care for HAE patients in Swaziland. Together with the doctor I am contacting the Ministry of Health in the country to discuss what can be done for patients with HAE and how HAE can be more quickly and accurately diagnosed. Working with the Ministry of Health could help improve education about HAE in this country and I am excited to see what happens in the future.

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

I am continuing to have conversations with doctors across the region, discussing how to improve access to treatment and care for HAE patients.



LEBANON

I have been working with The Lebanese Society, to see if there is the potential to include HAE on the agenda for one of their monthly scientific meetings. The positive news is that the Society has agreed and HAE will be on the agenda in March 2018. This provides the opportunity to introduce HAEi to the Society, and also to present some HAE cases. All of the Lebanese Society's members should be at the meeting, offering a great opportunity to make new contracts and see support for advocacy for future activities.



IRAN

The HAE national registry in Iran has been working hard to provide education and support for patients in their country. Physicians from the registry are visiting patients and educating them about HAE, as well as helping those who aren't based in Tehran to coordinate their treatment with their local doctor or hospital. They

have also established an advice line where patients can ask questions about any concerns they have relating to their disease. In the future, they are planning to hold an HAE-meeting, which will be a great opportunity for patients to meet and communicate further.



QATAR

I hope to have some good news for HAE patients in Qatar soon. There is ongoing work to make more treatments available, and I hope a specific treatment for HAE should be registered before the end of this year.



Javier Santana
Latin America



IN GENERAL

There has been a lot of activity in Latin America over the last few months. I have been supporting the member organizations in their work to ensure that governments and health institutions across the region are considering HAE. Helping bring HAE to the attention of these institutions is an incredibly important step in improving access to treatment.



ECUADOR

In Ecuador, there is a township where many of the residents have HAE. Unfortunately, this township is far from a major city and it can be difficult for people to travel to see doctors. Edison Galarraga from the national member organization is working with Dr. Keguisamos, a doctor who has a great interest in HAE. They are investigating ways to support these patients and to try to fund a study on HAE in this community, which could help to improve the care they receive in the future.

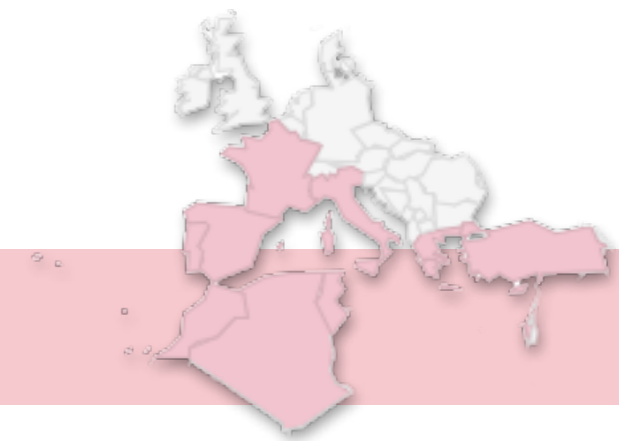


COSTA RICA

I am in contact with Lisa Layera from AEH Costa Rica, the HAE member organization. She has identified two doctors who are motivated to help educate and raise awareness about HAE in their country; Dr. Sergio Castro, a Dental Pathologist and Professor has plans to write up the case of a Costa Rican patient who had dental work done and due to undiagnosed HAE received a tracheotomy as a life-saving measure; and Dr. Espinoza Mora, a professor at the University of who is a willing investigator for clinical trials related to HAE research.



Maria Ferron
Mediterranean



IN GENERAL

I have been busy working with patients and healthcare professionals in my region to raise awareness of HAE and improve the care available for patients. I am also sharing and collating feedback on the HAEi Emergency Card and I hope to have different examples available on the HAEi website in the future.



ALGERIA

There is currently no patient organization in Algeria, but this may change with the hard work from individuals in the country. I have been supporting an individual with a family link to HAE, and an HAE patient to contact other patients and healthcare professionals in the region about establishing a patient organization. They need to identify a minimum of 15 HAE patients to be able to register a group – so if you are reading this and are from Algeria please do contact me at m.ferron@haei.org.



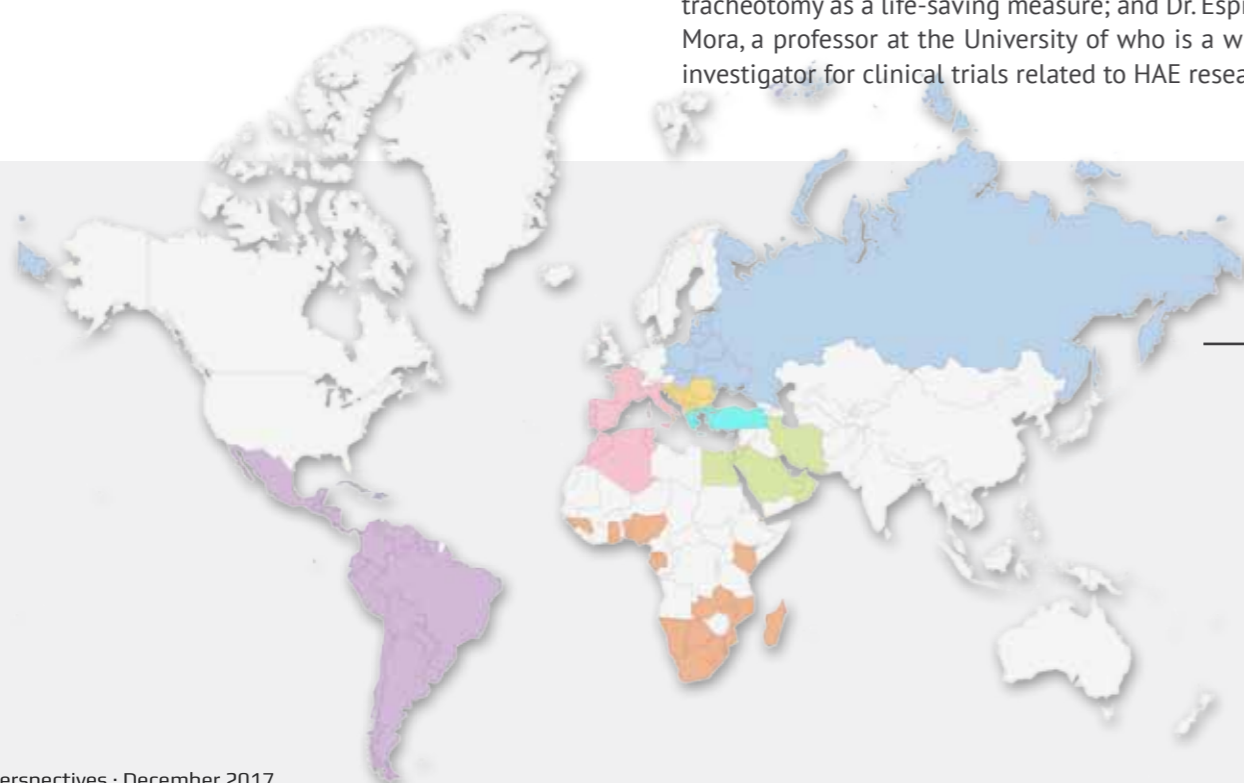
MOROCCO

I have been providing assistance and answering questions from a number of new patients in Morocco regarding HAE. I am continuing to support patients and healthcare professionals in the country, helping them to get in touch with each other and providing a much needed support network.



SPAIN

I have been working with Dr. Teresa Caballero to support her during the recent shortage of Cinryze in Spain and we are working to ensure that patients are able to access the treatment they need throughout this time. Dr. Caballero is also hoping to improve access to more treatment options in Spain and we will keep you updated on her progress.



The Regional Patient Advocates

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



HAEi South Eastern Europe Workshop 2017

HAE Macedonia and the Regional Patient Advocate for South Eastern Europe Natasa Angjeleska, hosted the second regional meeting for HAE patients, caregivers and physicians in Skopje 29-30 September 2017. This is a report from Mrs. Angjeleska:

Participants met each other again, or for the first time, shared the challenges and successes in each of the 10 countries they represent – and listened to presentations from renowned researchers and physicians in the presence of Professors Marco Cicardi and Henriette Farkas.

The HAEi South Eastern Europe Workshop 2017 took place at the Marriot Hotel in Skopje, and offered pleasant working and cheerful atmosphere with intertwined learning and exchange of experiences among participants. At the opening of the workshop, the HAEi president Anthony J. Castaldo expressed his excitement to come to Macedonia again and hear about the progress of patients, patient groups, and patient organizations in their pursue for improved treatment and care in the countries of the region. The president of HAE Macedonia, Natasha Jovanovska Popovska, who is also a member of the HAEi Executive Committee, welcomed our guests and shared her motivational

moments for looking into the better future for all HAE patients. She presented about the story of an eight year old boy who ran 5 km marathon after receiving his medicine for an abdominal attack. Journalists declared him a hero of this marathon and the story was shared in the media, which confirmed that “having the illness is not a burden for HAE patients, but lack of medicine is”. Natasha said that we should all run our marathons and not let obstacles force us back from our battle for quality life.

The first day of the workshop was concluded by a presentation by myself in my capacity of HAEi Regional Patient Advocate for the South Eastern European region. I compared the data that we had at the beginning of my work in April 2016, after the first Balkan meeting was held in June of that year, with the accomplishments until now. Working in a more or less compact region like SEE, creates a unique opportunity to meet across borders that divide our countries and



start building friendships, establish communication, use social media, share challenges, learn from each others' mistakes and successes, compare health care systems, and start thinking and working in a more organized and systematic way with the assistance that HAEi offers on a global level: Participating in meetings, offering resources for advocacy, hosting web pages for member organizations, networking through Facebook and other social media groups, as well as informing each other through HAE Connect. Although being an advocate requires to abandon the personal safety zone, working in the field dedicating yourself to community and less self-time is uplifting in a way that you have the opportunity to listen, educate all the time, ask for assistance, participate, and use all available resources. I took the opportunity to thank everybody for the hard work and cooperation in the past 15 months, and expressed readiness to continue with the good work already initiated.

The Second day of the workshop started with the lecture by Professor Cicardi titled “The HAE Treatment Landscape – Current Options, Future Prospects”. Using pictures and graphic schemas, his presentations explained the

reasons and physiological aspects for the clinical signs of HAE, explained about the mechanisms for treatment of HAE, and data from the newest clinical trials. The lecture initiated great interest and was followed by a series of questions by participants. Afterwards our host physician from Macedonia, Professor Vesna Grivceva Panovska, presented the developments of diagnostics and treatment for HAE in Macedonia. She compared data from the very beginning of the first identified patients in 1980, and the cooperation established with experts from abroad, such as Professors Farkas, Cicardi and Molina, in order to keep in line with the newest protocols and treatments for patients in Macedonia.

Professor Farkas had an excellent review in her presentation “Hereditary angioedema with C1 inhibitor deficiency: clinical and therapeutic features in pediatric patients – how do kids differ from adults?”. That was one of the rare occasions an audience could follow the comparison in diagnostics, manifestation of the illness as well as differences in treatment options for children and adults. The lecture included graphic presentations, videos and pictures, and the participants were excited to hear about some of the data presented.



This session was followed by another set of questions that continued during the lunch break. Afterwards the pediatrician doctor from Macedonia, Professor Katarina Stavric, presented case study results from the two years of available home treatment for children patients in Macedonia. She was among the first to recognize that home treatment with C1 inhibitor for children is improving their life, firstly because they are not traumatized by very frequent and long stays in hospitals, accompanied by psychological stress and unnecessary administrative workload of the hospital staff.

The afternoon sessions were dedicated to country presentations. The presenters consisted of a patient representative and a physician from each of the participating countries and they gave valuable information and data about the number of diagnosed patients, available treatments, and activities undertaken for raising public awareness and education about HAE. We were happy to have patient representatives from Albania for the first time at the meeting, as well as to follow the presentation of the president of a new HAE organization in Bulgaria. We had the privilege to hear from the country representatives listed here in alphabetical order:



- **Albania** – Patient Silvi Bastri and Doctor Mehmet Hoxha
- **Bosnia and Hercegovina** – Doctor Mensuda Hasanhodzic
- **Bulgaria** – Danail Dimov from HAE Bulgaria and Doctor Maria Staevska
- **Croatia** – Patient Ivana Markovic and Doctor Ljerka Kardadzalpic
- **Macedonia** – Verce Jovanovska Jankovska from HAE Macedonia
- **Montenegro** – Patient Sanja Cejovic
- **Romania** – Patient Ana Maria Baltatescu and Doctor Noemi Bara
- **Serbia** – Ivana Golubovic from HAE Serbia
- **Slovenia** – Patient Teja Iskra and Doctor Matija Rijavec
- **Turkey** – Ersan Sevinc from HAE Turkey

The day continued with session about the “Importance of patient advocacy” lead by the President of HAEi Anthony J. Castaldo and the HAEi Executive Director Henrik Balle Boysen. Mr. Castaldo referred to the importance of patient advocacy worldwide in order to raise awareness and contribute to the general awareness of HAE as well as educating officials with the assistance of physicians and researchers about the importance of access to timely diagnosis, modern treatment options and improved quality of life. Afterwards Mr. Balle Boysen delivered his presentation about the global HAEi network of patient groups and patient organizations under one umbrella giving them equal access to available basic information about the illness and available treatments, data from clinical trials, documents, contacts, the HAEi global magazine, free hosting for web pages, the HAEi Global Access Program, as well as the latest initiative HAE Connect. This part of the workshop also announced the fourth HAE Global Conference scheduled in May 2018 in Vienna, Austria. All member countries of the HAEi global network are welcome to apply for participation.

Before the end of this interesting workshop, the vice-president of HAE Macedonia Verce Jovanovska Jankovska and I held a short presentation in which we addressed the importance of “Advocacy with officials”. This last presentation was not aimed to give a recipe for the topic covered, but selected activities and actions aimed at advocacy with officials were listed with practical examples of what worked in Macedonia. The presentation also covered the importance of building alliances and networking with individuals, groups and organizations outside the HAE family that might be useful for advocacy with officials.

In total we had 58 participants from 10 countries in the region and guest lecturers from Italy and Hungary, the Regional Patient Advocate for the Mediterranean countries Maria Ferron, the HAEi President and Executive Director as well as one representative from SOBI and one from Macedonia Lek, two out of five pharmaceutical companies that supported the meeting. The meeting was perceived and evaluated as a much-appreciated opportunity for sharing and exchanging ideas, knowledge and extended communication by all.



HAEi Central and Eastern Europe Workshop 2017

7-8 October 2017 the combined 4th HAE National Conference and the 2nd HAEi Central and Eastern Europe Workshop took place at the Westin Warsaw Hotel in Warsaw, Poland. This is a report from the HAEi Regional Patient Advocate Michal Rutkowski:

The organizers – HAEi and the Polish Association of Angioedema Patients' Aid (with a Polish name that in English translates to 'Swelling Beautifully') – put lots of efforts in bringing the meeting to the next level and provide to all the attendees an exceptional program with the latest news from the HAE world. The event was organized under the auspices of the Jagiellonian University, the third oldest university in Europe, and Professor Tomasz Grodzicki, MD, PhD, Vice-Chancellor of the Jagiellonian University, was the honorary chairman of the organizing committee.

The two days of conference gathered over 140 participants from 12 countries, particularly those from Central and Eastern Europe: Belarus, the Czech Republic, Hungary, Kazakhstan, Poland, Russia, Slovakia and Ukraine.

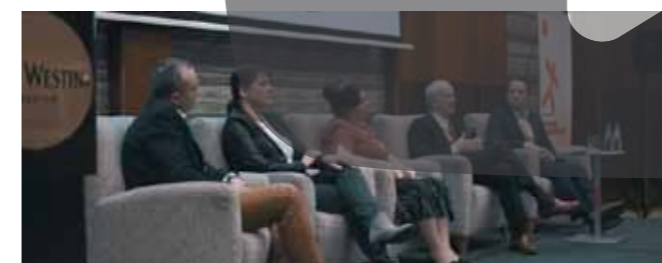
The most recent edition of this undoubtedly important meeting attracted the world-class scientists and physicians Professor Henriette Farkas from the Hungarian HAE Center at Semmelweis University in Budapest, Professor Marco Cicardi from the University Hospital L. Sacco in Milan, Professor Markus Magerl from the Charité University in Berlin and Professor Marc Riedl from the US HAEA Angioedema Center at the University of California San Diego. Additional presenters included Professor Krystyna Obtulowicz and in Stobiecki, MD from the National HAE Center at Jagiellonian University in Krakow, Anthony J. Castaldo and Henrik Balle Boysen, respectively the President and the Executive Director of HAEi, and myself as Vice President of HAEi and Regional Patient Advocate for Central and Eastern Europe.

The program was full of updated information regarding improvements within HAE, current and future treatment options, recommended therapies, children prophylaxis, and the latest WAO HAE World Guidelines. If this was not enough, the HAE patients' community supported participating physicians by organizing a closed breakout session carried out by Professor Cicardi.

However, the most powerful session was a short country presentation, where each CEE country shared the current situation of HAE patients, areas to be improved, and obstacles to be overcome. Mutual empowerment, advocacy and support it key to any success, so it is in the global HAE family. Volha Puhach from Belarus, Camelia Isaac from the Czech Republic, Arianna Kitzinger from Hungary, Sergey Morozov from Kazakhstan, Denis Sosinskiy from Russia, Michaela Bednarova from Slovakia, Olena Mykal from Ukraine and myself on behalf of the Polish HAE association were the patients' leaders and CEE representatives that introduced the outstanding presentations with many positives and challenges. Most of all the presentations included much inspiration and subsequent motivation for providing our community a better quality of life.

- **Countries in total:** 12 (Belarus, the Czech Republic, Denmark, Germany, Hungary, Italy, Kazakhstan, Poland, Russia, Slovakia, Ukraine and USA)
- **Attendance:** HAE patient 72 %, Health Care Professionals 22 %, Other 6 %
- **Country presentations:** 8
- **World-class scientists and physicians:** 4
- **Evaluation:** 100 % of the patients rated the conference as extremely useful – and 100 % of the patients stated that they would attend the conference in the future

Please have a look at the official video from the HAE National Conference & HAEi CEE Workshop 2017 at <https://youtu.be/7Yx3Pv8YCP0>





First HAE doctors and patients meeting in South Africa

The first doctor and patient HAE meeting was held in Cape Town at the Groote Schuur Hospital on the 25 October 2017 gathering patients from various regions of South Africa. There were 27 patients and four doctors present. HAEi funded the meeting financially and The Allergy Foundation of South Africa under the leadership of Dr. Mike Levin arranged for the venue and organized the program for the day.

President Anthony J. Castaldo, Executive Director Henrik Balle Boysen, and Patricia Karani who is the HAEi Regional Patient Advocate for Sub-Sahara Africa represented HAEi. Mrs. Karani says:

“We held two successful meetings, one with the doctors and the next with the patients. The doctors’ meeting highlighted doctors who are enthusiastic and willing to provide support for the HAE patients in the country. During the patient meeting a number of participants found the courage to share their heartbreaking stories on misdiagnosis and wrong surgical procedures where some even got their body organs removed due to misdiagnosis. Many patients shared their happiness to finally meet and interact with other HAE patients in South Africa and all could afford a smile. They were also glad to meet very knowledgeable and experienced doctors who have been treating other HAE patients and have even been involved in clinical trials in the region.”

The HAEi team assured that they would help support the doctors and the patients and provided various tools for the organization namely; HAEi Connect to keep a state of the art membership database in the national organization, HAEi GAP (Global Access Programme) which can be enrolled to help patients get access to medication, and the HAEi free website hosting for the national organization. Patricia Karani continues:

“At the end of the meeting, the patients decided to form a committee for the new HAE South Africa patient group with Adrienne de Jongh as the National Patient Representative. Adrienne has started a HAE South Africa Whatsapp group for more flexible interactions between doctors and patients within the country.”

South Africa has a wealthy resource of doctors who will assist the newly formed support group grow and raise awareness. Mrs. Karani explains:

“Dr. Mike Levin is very key for patients in South Africa because of his willingness to provide them with awareness campaign tools which would help the organization to grow. Professor Paul Potter – a now retired physician – also amazed us with his the broad knowledge and work, which he has conducted on HAE patients in South Africa. Dr. Siphon Duncan who had come all the way from Hlabisa Hospital in Kwa Zulu Natal Province gave a good presentation on HAE as well as shared with us about the HAE patients he handles in his region. In addition, Dr. Jonny Peter who took over Professor Potter’s practice is also an enthusiastic doctor who is well versed with all of the professor’s work. South African patients are surely in good hands with these doctors who are more than willing to be part of the HAEi family.”

Tamsin van Vlaanderen from the committee for the new HAE South Africa patient group adds:

“A group of patients had been gathered by Professor Potter about 25 years ago at Groote Schuur and many of these people have formed the basis of our patient group. We are aware that there are yet undiagnosed patients in some rural areas and that will form part of our initial focus. At the moment the only treatments available here are Danogen and freeze-dried plasma. Anything else has to be imported on a patient named basis, which is both prohibitively expensive and takes too long for emergency treatment.”

For many HAE patients this was the first time meeting other sufferers and there was a great feeling of a new supportive community in the making.





HAE Scandinavia Conference 2017

The second weekend in November 2017 the beautiful conference venue Bergendal – right at the rim of Edsviken bay in Sollentuna just 20 minutes north of Stockholm, Sweden served as the inspirational setting for the HAE Scandinavia Conference 2017.

The conference opened with two international keynotes. First Professor Markus Magerl from Europe's largest university clinic Charité in Berlin, Germany gave a historical insight into the development of HAE since the 1880ies – and not least elaborated on the possible future treatment options for HAE patients. Professor Magerl said that there has been a huge interest in development of new medication over the last decade and that much will come over the next few years. We will see further development of well-known products as well as new solutions, maybe a C1-inhibitor from plants (iBio) or a gene block (Ionis Pharmaceuticals). Professor Magerl expects that a further focus on prophylaxis will change the treatment paradigms, as it will pave the way for better and more individualized treatment.

HAEi President Anthony J. Castaldo, who spoke about the importance of patient advocacy, delivered the second international keynote. Among many other things

he referred to a HAEi survey covering 37 countries and around 10,000 patients – and proving that access to modern medication leads to happier and healthier lives. He stressed that if HAE patients are looking to live normal lives advocacy can lead them there.

The Danish Professor Annette Bygum from Odense University Hospital delivered a third keynote presentation. She told about the experiences of the Danish hospital from treating HAE patients for around 20 years – and emphasized the importance of patients taking part in clinical studies. In her words: "Without clinical studies there will be no development of new medicines".

The very full program also included two sessions with updates from HAE Scandinavia. Among other things the HAE Scandinavia President Henrik Balle Boysen told the around 125 conference participants about the new Scandinavian treatment recommendations

that the organization is presently working on together with the Medical Advisory Panel. The first version of the recommendations is expected to be ready for publication in the middle of 2018.

As for an online survey on patient/relative behavior – funded by CSL Behring – Henrik Balle Boysen said that it has involved more than 150 patients and relatives and he referred to some of the findings regarding the patients:

- 30 percent had to wait for more than 10 years before diagnosis,
- 40 percent had 25 or more attacks during the last 12 months,
- 51 percent treat their attacks right away while 39 percent treats within one to four hours,
- 84 percent have been instructed in self-medication,
- 47 percent have been treated wrongly during an acute attack,
- 39 percent have been told that the medication is expensive and shouldn't be taken unless you really need it,
- 27 percent say that HAE has had an influence on their possibilities/choices regarding work.

Henrik Balle Boysen also updated the participants on the new HAE Scandinavia member database HAEi Connect, developed and sponsored by HAEi. Furthermore, he spoke about recently published information material, experiences from the HAEi Youngers' Summer Camp 2017, and expectations regarding the HAE Global Conference 2018.

The conference program also included updates on the HAE situation in Denmark (Annette Bygum), Norway (Dr. Olav Rogde Gramstad from Oslo University Hospital), and Sweden (Dr. Annika Wahlin from Umeå University Hospital).

The 2017 Scandinavian Conference closed with a Q&A session with all the presenting doctors taking part – and it was followed by the General Assembly only for members of HAE Scandinavia.



Scandinavian
Conference

2017 · Stockholm · HAE Scandinavia



There now also is a community for HAE 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, taking 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 61 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 walk 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



HAEi JAKOBSWEG WALK 2018

Join the HAEi Jakobsweg Walk 2018

In order to raise awareness, HAEi brings together HAE friends – patients, relatives, caregivers, doctors, nurses, and industry – for the HAEi Jakobsweg Walk 2018.

“After the successful HAEi/AEDAF Camino Walk on the Camino de Santiago in Spain in May 2016 and May 2017, we are organizing yet another walk. We aim to bring 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 to commemorate the global hae day :-) and this time we will be walking part of the Jakobsweg – that is German for “The Way of St. James” – pilgrimage route through Vienna, Austria”, says HAEi Communications Manager Steen Bjerre.

While in 2016 and 2017 all participants walked all stages this time the idea is to accumulate walkers:

“We will start out with a pretty limited number of pilgrims in the morning, add some at the beginning of stage 2 and then even more – no doubt the majority – at the beginning of stage 3. And should anyone feel like more they can sign up for the second days of walking. The price for participation is the same regardless if you walk one, more or all stages”, says Steen Bjerre.

The HAEi Jakobsweg Walk 2018 program consists of four consecutive stages. It is entirely up to the individual walker is he or she wants to take part in just one, more or all of the stages:

16 May 2018

Stage 1 (Schwechat to Basilika Kaisermühlen)

12+ km = approx. 2 hours 30 minutes

Stage 2 (Basilika Kaisermühlen to Stephansdom)

5 km = approx. 1 hour

Stage 3 (Stephansdom to Schloss Schönbrunn)

6+ km = approx. 1 hour 30 minutes

17 May 2018

Stage 4 (Schloss Schönbrunn to Jakobskirche Purkersdorf)

12+ km = approx. 2 hours 30 minutes

Are you ready for one, two, three or even four stages of awareness walking on the Jakobsweg through Vienna before the HAE Global Conference 2018? Then don't hesitate to sign up – see more at www.haei.org.

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 11 countries:

- **Australia:** www.haeaustralasia.org.au
- **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
- **Peru:** <http://haei.org/peru/>
- **Poland:** <http://haei.org/pl/>
- **Serbia:** <http://haei.org/rs/>
- **Spain:** www.angioedema-aedaf.org
- **Turkey:** <http://haei.org/turkey/>

"We are preparing one or two at the moment and hopefully many more will join us within the next year", says Henrik Balle Boysen.

At www.haei.org/haei_countries you'll find an overview of all 61 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.).



News from around the Globe



CHILE www.angioedemahereditario.cl

Leading up to the elections in November 2017, the HAEi point of contact in Chile Lorena Merino continued her proactive efforts to secure meetings with the individual Chilean presidential campaigns. She has been able to meet with a number of the candidates' campaign work teams to discuss HAE, the lack of access to therapies, the high costs of medications, and the daily struggle patients experience in Chile once they have to seek emergency care at the hospitals. It is Mrs. Merino's mission to educate and obtain a commitment from the candidates to pursue the improvement of care for HAE patients.

The annual meeting of HAE Chile was held 25 November 2017. Among the participants were patients and their families, immunology physician experts, and the Sub Secretary of the Health Department of Chile, Mr. Roganterio Cristian Herrero. Via video HAEi President Anthony J. Castaldo talked about the support that HAEi is giving to Lorena Merino and HAE Chile and the importance that the patients with HAE in Chile can be included in the law "Ricarte Soto" to protect them.

*From the HAE Chile annual meeting:
Carlos Quintana, President of HAE Chile and Alliance of Chile Patients;
Dr. Paula Bustos, Immunologist;
Lorena Merino, HAE Chile; Dr. Marta Villaverde; Dr. Daniel Vázquez; and Roganterio Cristian Herrera, Sub Secretary of Health.*

BULGARIA www.haebg.org

HAEi would like to welcome the formal national HAE organization in Bulgaria, Hereditary Angioedema Association Bulgaria (HAEA Bulgaria), to the global HAE family. See more at <http://haei.org/location/hae-in-bulgaria>.



From Laura Szutowicz, CEO HAE UK:

Since the last International newsletter much of HAE UK time has been taken up with planning and then holding the two Patients Days. But before those we had a "huddle" in the glorious sunshine and wonderful surroundings of the Regent's Park Café. As well as some old friends we welcomed some new members to HAE UK and had a jolly time. Before that, during the day, Rachel and I walked the streets (seven miles in total!) to visit Accident and Emergency departments in several London hospitals to leave information about HAE and to raise awareness of how it should be treated in the A&E. One of the targets of the new Rare Diseases Strategy is that A&E departments will be tasked with ensuring that "alert" cards have been requested and assessed to ensure appropriate treatment is given.

The Scottish Patient Day was held at the end of September 2017 in the elegant surroundings of the Grand Central Hotel in Glasgow. Some 50 patients and family members (and a dog!) attended to hear presentations from Dr. Scott Hackett on how HAE varies from birth to maturity; John Dempster on maintaining a healthy lifestyle; and Hazel Millar on learning from the expert patient. We also had some excellent patient stories from Graham, Furkhanda and Dana and lots of lively discussion. Pippa Adams presented her psychology Masters dissertation "The Well Being of patients with Hereditary Angioedema". HAE UK are proud to have contributed some sponsorship to Pippa for this work, which has been presented at two International Psychology meetings and will be presented at the UKPIN this month.



It is lovely to be in Scotland and meet such an enthusiastic group of members and their nurses. A real privilege and we look forward to next year, which is already in the planning.

It seemed no time at all before we set out for Sheffield for the National Patient Day in the middle of November. Despite Rachel Annals organizing everything so well, I suffer many sleepless nights before these events and this time really alarmed my husband by sitting up bolt upright at 3 am one morning shouting "I must order more lettuce!". I still have not got to understand that one!



This meeting was a chance to launch our GP Information Pack, which we encouraged patients to take to their general practitioner. The idea behind these packs is that the average family doctor will never see an HAE patient, unless there is one registered in their practice. However, raising awareness can only help to improve the treatment of existing patients and may just speed a diagnosis of another.

Ray, one of our members, works for Ford and they have kindly covered one of their lorries and a car transporter with HAE UK logos. They will mostly be working in Europe so please let me know at laura.szutowicz@haeuk.org if you see one anywhere. The more strange the place, the better :)

We have started having an evening reception and get together the night before "the day"; this is the second year and it was great how many people came to that and to get to know one another in a social environment. We are fortunate to have so many loyal and enthusiastic members! And we all got to bed in reasonable time for the next day.

We were fortunate to have as our first speaker Dr. Ravi Sargur, who has been carrying out research into bradykinin. His excellent presentation "Why do I swell" was a really clear and concise explanation of what goes on during an attack and how the various medications act in different parts of the pathway. Many people remarked that they had never had HAE explained so well before. Dr. Sargur was followed by specialist dietician Victoria Gallivan with an excellent presentation on "Eating for Health". She put great emphasis on maintaining a balanced diet and a good selection of food groups. This was again very well received by attendees, particularly her advice on how to correctly assess if any one food is a trigger for attacks, which does seem to happen for some people.

Jack Cope told us all about the HAEi Youngsters' Summer Camp and several of the younger members of the audience enquired about when was the next one. Jack is a great addition to HAE UK.



Faye Davenport relating her and Ruby's stories of diagnosis and treatment and how it affected the whole family topped off this section of the morning. Faye is an excellent speaker and her sometimes sad, sometimes hilarious account showed what a very strong family they are. After coffee Dr. Mel York stood in at short notice for Dr. Bill Egner who had a family bereavement. Dr. York gave an excellent presentation on "New Treatments and New Uses for Old Treatments" and again was an excellent speaker.



Next was Becky with her patient story, her eventual successful treatment has enabled her to train as a midwife, which she obviously adores. This is the rewarding side of hearing the patient stories, how people overcome their condition to carry on with their dreams.

We were very privileged to have great international input at our Day. Michal Rutkowski, President of HAE Poland, Regional Patient Advocate for Eastern Europe and Vice President of HAEi gave an excellent insight into "Treatment in Eastern Europe" and the differences from treatment here in the UK. Despite our grumbles about the NHS and treatment we are really fortunate to have so many dedicated clinicians, expert in HAE and access to many modern treatments. And as Dr. York pointed out: more on the way.

John Dempster gave an excellent presentation on the importance of good planning and then we broke for lunch and the all-important opportunity to network. Our first speaker after lunch gave no one a chance of a quick sleep: We were honored to have Anthony J. Castaldo, President and founder member of both US HAEA and HAEi. In his former life he was Associate Inspector



General at the United States Federal Bank, so a great example of what well-managed HAE patients can achieve. His "retirement" project of his dual Presidencies sees him tirelessly jetting all over the world, helping to set up new country and regional groups and advocating for improved treatment everywhere. He was an inspirational speaker and gave us all such a sense of belonging to one big forward thinking and moving family. How can we not succeed?

Anna gave us her story of how she eventually got a diagnosis and, now well managed, she is in the throes of a psychology degree. She also won my personal award for "best dressed lady" – I need to get some advice! Our final speaker was Pippa Adams, MSc. HAE UK gave a small financial grant to Pippa to support her dissertation for her Masters degree (which she got! Hooray!). She has produced a superb piece of work on Psychological Wellbeing of patients with HAE, which has been presented at two European conferences and at the UK PIN meeting in Brighton in December. We hope to be able to use this work further for use with our telephone counseling line in due course. Pippa was the last of our presentations, we then had "break out" groups hosted by Dr. Patrick Yong, Fran Ashworth, John Dempster, Paul Carroll, Christine Symons, Pippa Adams and a final Q&A session with the above plus Anthony J. Castaldo and Michal Rutkowski.

It was difficult to believe it was all over for another year! My great thanks to, in no particular order, our fantastic speakers, our ever-helpful medical advisory panel, our Trustees, our international colleagues. Particular thanks to Rachel who organizes the venue and other logistics so well and to Furkhanda who mans the registration,



We have many plans for the coming year, but until the next HAEi magazine I wish everyone a Happy and Peaceful Christmas and New Year.



KAZAKHSTAN

<http://haei.org/location/hae-in-kazakhstan>

A patient group has been formed in Kazakhstan with Mr. Sergey Morozov in Almaty as the national HAE representative. Please find contact information at <http://haei.org/location/hae-in-kazakhstan>.

News from around the Globe



VENEZUELA

www.facebook.com/Angioedema-Hereditario-De-Venezuela-1475867039316898

Early November 2017 the XXVth Venezuelan Congress of Asthma, Allergy and Immunology was held at La Trinidad Medical Teaching Center in Caracas, Venezuela. The conference gathered the most prestigious professionals of this branch of medicine in Venezuela – and Fundación AngioEdema Hereditario de Venezuela (AEHVE) participated in the event with very encouraging results for HAE patients. In the block corresponding to the disease, the following topics were presented by a qualified group of Immunologists:

- Hereditary Angioedema: Multi-etiological Disorder – Doctor Ana Antelo
- From the Clinic to the Diagnosis in Angioedema Hereditary – Doctor Diana Briceño
- Current Events and Advances in the Treatment of Hereditary Angioedema - Doctor Morella Bouchard, President of the Venezuelan Society of Asthma, Allergy and Immunology
- Intervention of Fundación de Angioedema Hereditario – Mary Bocaranda, AEHVE

The presentation by Mary Bocaranda began with an affirmation of Doctor Alejandro Malbrán, with the purpose of raising awareness about the HAE to the attending physicians: "We have the challenge of getting professionals to be more attentive to this disease". The participants were informed about the most relevant aspects of the foundation and the purpose that they have every day of disseminating more information about the HAE and the progress made worldwide. Emphasis was placed on the critical situation in order to obtain medicines for the treatment of this dangerous disease, which are practically nil in Venezuela.



IRAN

<http://haei.org/location/hae-in-iran>

We welcome the formation of a patient group in Iran. The Iranian HAE point of contact is Ms. Mehernoosh Eslampanah in Teheran, please see <http://haei.org/location/hae-in-iran>.



URUGUAY

www.facebook.com/Angioedema-Hereditario-Uruguay-261467430693742

The HAE patient group in Uruguay is working to find the necessary funds in order to be able to establish the formal organization HAE Uruguay (AUAEH).



Mrs. Bocaranda pointed out that AEHVE is a member of HAEi and that hae day :-)) is celebrated worldwide on 16 May every year. Furthermore, she expressed that the objectives in the short term consist of:

- To capture all HAE patients throughout the Venezuelan territory and not to leave any person submerged in ignorance of this rare disease
- That these patients have a short-term diagnosis and try to have the availability of treatments through government agencies and that they are available in all cities, towns, etc. in the country

Achievements were immediately seen as a meeting was planned with the President of Social Security of Venezuela through a work team of the High Cost Committee, which is responsible for the supply of high-cost medicines.



BELARUS

www.hereditary-angioedema.org

A little while ago the Ministry of Justice officially registered the Belarusian National Public Organization HAE Patients Care organizing Belarusian HAE patients and their relatives.



TURKEY

<http://haei.org/turkey/>

From the International Communications Manager Ersan Sevinc:

The last few months have been very busy for HAE Turkey. The beginning of fall brought us to Skopje, Macedonia in order to gather with our beloved Macedonian friends and other Balkan countries. Everyone's purpose was clear as always: Learn and share more! In the meeting, as each country did, HAE Turkey shared the improvements since the first HAEi South Eastern Europe Workshop, focusing on the fact that we have found more and more patients with our new projects in comparison to the last years.

We have finally come to terms in our negotiations with the Ministry of Health. As a result of our perpetual visits to the Ministry of Health and the Social Security Institution, which most of the population and our patients use for insurance, from now on they accept HAE Turkey as the official patient organization to refer to and ask about HAE. This important step means

a lot to us and now we are waiting for a date for a roundtable discussion on HAE issues with the ministry and physicians of the ministry. If we can succeed in getting home therapy approval from the officials, then the biggest problem that we all faced in Turkey will be history.

We have heard that a rare diseases association is in the making in Turkey. Now we strongly hope to contact with other rare disease and patient organizations to bond and get stronger together.

The biggest step that we have taken during this year is the XXIV National Allergy and Clinical Immunology Congress. It is organized by the Turkish National Allergy and Clinical Immunology Association, which most of our HAE physicians are affiliated with. Even though it was full of meetings primarily for physicians, the President of the association Professor Bülent E. Şekerel let us attend and open the HAE Turkey booth in the meeting place. Our booth was open to every physician attending and as the estimated number of attendants was around 650 physicians this congress was very significant for us as it was a great chance to build strong connections



with physicians from all around Turkey. More than 200 physicians visited our booth and more than 50 percent were specialized immunologists who already knew HAE or were eager to learn and help our patients and us. In addition to expanding our connections and contacts during the congress, we met with very well-known physicians who were really kind and ready to help HAE Turkey in terms of promoting us in the social media and include HAE and HAE Turkey in their speeches. It is equally important to emphasize that four of the meetings were specialized regarding HAE issues. These HAE sessions helped us to reach more physicians and as a result of that we succeed in organizing an exclusive meeting with HAE experts and other physicians. During our meeting, our well-known physicians Professors Suna Büyüköztürk, Gül Karakaya, Okan Gülbahar, Mustafa Güleç and Nihal Mete Gökmen were with us to help. We would like to thank them all for their endless efforts for HAE and HAE Turkey.

While the National Allergy and Clinical Immunology Congress was going to start 18 November 2017, I flew to Berlin in order to be with HAE Germany to celebrate the 20th anniversary of the organization. HAE Germany is a great example in terms of quality of life for patients with HAE because in Germany none of the HAE patients have any trouble in getting treatment. Lucia Schauf is not only a successful President but also a quite affectionate and charitable person with her approach to HAE and all the patients her country. The first step of the friendship between HAE Germany and HAE Turkey was taken in Munich, Germany in 2015 with an invitation from Lucia. She invited me to show the standards that we must achieve as HAE patients. With the things we learned from Germany, we shaped our attitude and started to work harder to get what we deserve. We deeply



appreciate their business and wish them many more anniversaries. During the meeting in Berlin, milestones and the steps of association beginning in 1997 were shared by Professor Bork who had also great impact not only on HAE in Germany but all around the world with his remarkable publications. Professor Marcus Magerl and Dr. Martinez Saguel informed us regarding how HAE was originally called Quincke and now what we know about HAE. After attending the HAE Germany meeting Professor Magerl flew to Antalya, Turkey for the Turkish National Allergy and Clinical Immunology Congress and here he gave a speech about "Handling Hereditary Angioedema; prevention and treatment".

Since the establishment of our association in 2015, we have learned a lot and applied our knowledge as much as we can. Today, we are still learning and expanding our borders, we are focusing on our patients and therapies to prevail in this world where they do not know or understand us. In the light of these significant events, we have prepared and started to work on new projects, which focus on improvement of the current situation and developing a new approach to our work.



COSTA RICA

www.facebook.com/aehcostarica

From Lisa Layera, AEH Costa Rica:

Gaining access to modern HAE medicines is at the center of the work of Costa Rica's National HAE Association where currently, the "best and only" treatments available are plasma and androgens.

After multiple denials for C1 esterase inhibitor therapy for HAE patients from the Central Committee of Pharmacotherapy, we are submitting a formal appeal to the Constitutional Court.

This appeal is called a "Recurso de Amparo" and it will be made in the name of the youngest diagnosed HAE patient, Tayra Corrales Gonzales who has just turned 10. "Amparo" means "protection" in Spanish; we believe that Costa Rican HAE patients are being denied their constitutional and institutional rights when the prescription for C1 esterase inhibitor by the treating doctor is rejected. If there is any known precedent of similar initiatives or successful efforts that have yielded positive outcomes we would certainly appreciate hearing about them. We welcome anyone willing to help us in this effort and can be reached at aehcostarica@gmail.com.

We are incredibly grateful to the physicians around the world who have created a body of work that provides the proof required by the Court to rule in favor of our patient – a ruling that would require the national system to supply modern HAE treatment. What will the Court be examining? Evidence-based medicine. What will the Court need to determine about the merits of the medicine?

- efficacy and safety
- high therapeutic value
- low-risk
- clear outcomes
- well-designed controlled trials
- international standards

Due to the work of physicians in tandem with HAE associations, patients with HAE are lucky to have WAO Guidelines and an International Consensus for the management of HAE in both adult and pediatric populations. In 2012, an International Working Group which included HAE expert physicians from around



the world, stated unequivocally that, "Treatment for HAE has been revolutionized in the last 10 years by three new drugs developed for the treatment for acute attacks." Proving that new treatments are safer, more efficient and have irrefutably better outcomes for the patient is neither easy, cheap nor fast. We thank those who have dedicated their time and intellectual capacity to ensuring that HAE as a disease is not invisible. New evidence coming out that the use of C1 esterase inhibitor prophylactically reduces the cost and frequency of HAE in very real numbers is incredibly valuable.

While patients with other diseases have attempted to get approval for medicines through this legal route, the situation of HAE patients stands in stark contrast as the treatment being prescribed by their physicians is neither risky, deadly nor of marginal benefit. The new medicines are indeed, revolutionary.

HAE patients can and do lead productive lives while on modern treatment, and can also expect a normal lifespan. Success in this effort to grant due to constitutional and institutional rights to HAE patients can pave the way for dozens of other diagnosed Costa Rican HAE patients in the future. It will also increase awareness about HAE at the national level; helping to identify the hundreds of undiagnosed HAE patients we know statistically do exist in Costa Rica.

News from around the Globe



LEBANON

<http://haei.org/location/hae-in-lebanon>

The global HAE family has been joined by Lebanon as there is now a patient representative in Beirut: Mr. Elie Hayek. Please see further information at <http://haei.org/location/hae-in-lebanon>.



SWITZERLAND

www.hae-vereinigung.ch

10 November 2017 the 16th General Meeting of HAE Switzerland took place. The President Helene Saam welcomed 21 members. The longtime former President, Paula Hunkeler, who resigned last year, was elected the first honorary member of HAE Switzerland. All other agenda items were also decided unanimously.



On the TV program "Praxis Gesundheit" Professor, Dr. med., Dr. phil. Walter A. Wuillemin, Chief Physician Hematology at the Luzerner Kantonsspital, and President Helene Saam from HAE Switzerland spoke on "HAE – life-limiting and often late diagnosed". The program was broadcasted on several regional Swiss TV stations in November 2017 and can be viewed at www.hae-vereinigung.ch/news/fernseh-interview-ueber-hae-2.

The 19th HAE Switzerland Patient Meeting will take place on 9 June 2018 at the Swiss Museum of Transport in Lucerne. Registrations will be sent in March 2018.



POLAND

www.haei.org/pl

December is a quite busy month for HAE Poland as the organization will participate in the HAE session of the 9th Allergy and Clinical Immunology Symposium in Krakow, organize a regional patients' workshop in the Department of Clinical Immunology, Rheumatology and Allergy at the University Hospital in Lodz, and participate and partner in the 12th International Symposium for Evidence-Based Health Care "HTA - Evidence and value in decision-making paradigm" in Krakow. Furthermore, HAE Poland will take part in the III EUROPLAN national conference "European project aimed at fostering the adoption and implementation of integrated national plan for rare diseases" taking place in Warsaw.



EGYPT

<http://haei.org/location/hae-in-egypt>

Also Egypt is now on the global HAE map. In Cairo you will find Mr. Walid Hassan Abed El Rahman as the national HAE representative for Egypt – please see <http://haei.org/location/hae-in-egypt>.



AUSTRALASIA
www.haeaustralasia.org.au

From HAE Australasia CEO Fiona Wardman:

HAE Australasia took part in ASCIA 2017 in Auckland where we caught up with clinical immunologists and nurses from around Australia and New Zealand. It's great to see so much interest in HAE, and in what we do as an advocacy organization.

We also had an information desk at the Macarthur Rare Disease Symposium where we sponsored morning tea for the participants. The event was worthwhile for everyone as doctors and nurses were able to learn more about HAE from experts from Australia and overseas.

Our website has been upgraded and is now hosted by HAEi. This has made a big difference in us being able to update information easily, quickly and efficiently. We now have an online membership form for Australian and New Zealand patients to join our database.

We held a successful Meet Up in Tamworth recently. We met three separate families for the first time, and we chatted and offered support and information over lunch. Tamworth is a (largeish) country town, and as it turns out some of the people knew each other from school, work and social activities but had no idea about the HAE connection.

16 December 2017 HAE Australasia had a Meet Up in Brisbane and the next event – a Family Meet Up in Perth – is scheduled for 24 March 2018. Information on activities and events in Adelaide, Auckland and regional areas and a few other locations will be coming soon. Keep checking for updates on www.haeaustralasia.org.au and our Facebook pages.

HAE Australasia is now six years old and we've had a growth spurt! Behind the scenes, we have been working hard towards the organization's goals which is to continue to support patients and carers around Australia and New Zealand, locate others that are yet to be diagnosed or needing access to treatments, provide HAE education where needed and raise awareness of HAE in the medical and general community. To carry out these activities, time is spent sourcing essential funding and donations. These things take time, and up until recently, volunteers carried out HAE Australasia's activities wherever their time could be spared.

News from around the Globe

We are proud to let you know that HAE Australasia now has two part-time employees: Louise Ridout as Chief Finance Officer and myself as Chief Executive Officer, and we will now dedicate 24 hours a week (combined) to work in the organization. I will be responsible for the day to day running of the organization, establishing funding partnerships, advocating, organizing and facilitating events, creating awareness, educating and supporting patients, and Louise will be responsible for financial, compliance and governance tasks, and securing grants.

Anne Wilkinson is Chair of the Board, with Olivia Worthington, Evelien De Bruin and Dr. Connie Katelaris remaining as Board Members along with Louise and myself.

HAE Australasia has partnered with the MedicAlert® Foundation to make sure that support and protection is available to more Australians who are living with HAE. For a limited time they're offering our member's the opportunity to apply for a free MedicAlert ID and one year membership.

As Australia's most trusted and recognized provider of the lifesaving 24/7 Emergency Response Service and medical jewelry, MedicAlert Foundation is passionate about doing their part to help keep the community protected in an emergency. If you or a loved one is living with HAE and wants to find out if they qualify please find out more at www.medicalert.org.au/benevolent-program-hae.

HAE Australasia would like to wish everyone a merry Christmas and a safe, happy and swell free start to 2018!



ALBANIA
<http://haei.org/location/hae-in-albania>

Yet another European country has joined the HAE family, this time Albania. The national HAE representative is Ms. Silvana Bastri. Please see the contact information for Albania at <http://haei.org/location/hae-in-albania/>



USA
www.haea.org

Medicine shortage: The US HAEA recently conducted a survey among HAE patients using the Cinryze therapy. The survey revealed that over 50% of respondents ran out of medicine during the recent shortage. Over half of the patients who ran out of medication experienced five or more attacks and many patients experienced HAE attacks that resulted in an emergency room visit and in some cases emergency intubation.

The US HAEA continues to monitor the medicine shortage issues and is engaged in ongoing discussions with the FDA, therapy manufacturers, and the Medical Advisory Board to help mitigate patient challenges. Keep in mind that HAEA's Health and Patient Advocates are here to help. If you need assistance, don't hesitate to reach out to your advocate.

Youth webinar: The "StandUpStrong4HAE – Youth Edition" webinar will explain HAE in easy to understand terms for young people, while comparing the old school vs. new school ways of thinking about HAE. Guest Speaker is Dr. Johnston – and the date and time is 18 January 2018 at 7:30PM EST, 4:30 PM PST. You can join from PC, Mac, Linux, iOS or Android at <https://haea.zoom.us/j/689442657>. From a smart phone one-tap US: +16699006833,689442657# or +16465588656,689442657#. Or Telephone: Dial US: +1 669 900 6833 or +1 646 558 8656 Meeting ID: 689 442 657.

Two surveys: Looking forward to 2018, the US HAEA asks that patients provide feedback so the organization can better serve the patient community by expanding the existing programs and developing new and exciting initiatives. There are two surveys on the future strategy:

- 2017 National Patient Summit Survey: the US HAEA is already making plans for the 2019 National Patient Summit and hopes to make it the best one yet. If you attended this year's National Patient Summit in Minnesota, the US HAEA would like to know about your experience: https://docs.google.com/forms/d/e/1FAIpQLSe_SVlvkKhUX9DtQYMxZVlyl1T1zr88slsBGHEaZSqugnepNA/viewform
- Patient Interest Survey – As the community continues to grow, the US HAEA wants to make sure that the organization is in tune with what the patients want and need. This survey will help the US HAEA personalize services for patients and their family: <https://www.haea.org/ci/form/survey>

Open for kids: The US HAEA Angioedema Center at UCSD is thrilled to announce that the Center can now see children with HAE, ages 8 and up. The Center's expert physicians specialize in the care of patients with all types of angioedema. To learn more about how to arrange a visit for your child, please contact a member of the HAEA Health Team – Heidi Polek at heidipolek@haea.org or Troyce Venturella at troyce@haea.org.



MACEDONIA
www.haei.org/haemacedonia

An Expert academy for HAE was held in Skopje, Macedonia 10 November 2017, initiated and organized by Professor Vesna Grivcheva-Panovska, MD. The event was accredited by the Macedonian Medical Chamber and took place at the premises of the Medical Faculty in Skopje. Professor Kalina Grivcheva-Stardelova, chair of the Medical Chamber of Macedonia, welcomed the attendees and after that Professor Maria Teresa Caballero Molina talked about Quality of Life with HAE, while Professor Marco Cicardi introduced the

audience to established and upcoming therapies for HAE with C1 inhibitor deficiency. Professor Timothy J. Craig discussed evolution in HAE Care in the USA, while Professor Anastasios E. Germetis presented a precise medicine prototype in HAE. Patient Management & Healthcare Issues were described to the participants by Avner Reshef MD and Professor Grivcheva-Panovska talked about her experience in treating HAE patients in South Eastern Europe. Doctors, specialists and pharma representatives as well as members of HAE Macedonia attended the meeting.



CANADA

www.haecanada.org

From Social Communications Coordinator Jessica Howlett:

At HAE Canada we have evolved so much over the past few months. All of our board members have been extremely busy forging relationships with Health Organizations and Medical Groups throughout Canada. In September, HAE Canada President Jacquie Badiou, Directors Richard Badiou and Bob Simon, and HAE Canada member Judy Simon represented Canada at the 2017 US National Patient Summit in Minnesota, USA. Vice-President Ken Howlett and Regional Director Anne Rowe were no less busy, attending the Association des Patients Immunodéficients du Québec (APIQ) - Journée Éducative AOH in Montreal, Quebec. In October, Bob attended the Saskatchewan Society of Medical Laboratory Technologists (SSMLT) Conference in Regina, SK., along with Physician Dr. Andrea Fong, where they presented an overview of HAE, the importance of efficient access to HAE treatments, and a patient perspective of HAE. Later that month, Anne, Ken and Linda Howlett (Regional Director) attended the Canadian Society of Allergy and Clinical Immunology (CSACI) Annual Scientific Meeting in Toronto.

In November, we held our HAE Canada 2017 AGM and Patient Event in Toronto. This was a wonderful opportunity for our HAE Canada family to come together, interact and learn more about our rare disease. Also in November, Jacquie, Richard, Ken and Linda attended the Canadian Organization for Rare Diseases (CORD) conference. The following weekend they were guests for an elegant evening at Canadian Immunodeficiencies Patient Organization's 20th Anniversary Gala and Excellence Awards. Lastly they participated in the Network of Rare Blood Disorder Organizations (NRBDO) Comprehensive Care Symposium.

Canada's network of physician HAE specialists (CHAEN) continues to foster the development of HAE learning and international collaboration. In 2017 CHAEN implemented an elective program for residents registered in a Canadian Clinical Immunology and Allergy Program for a three week observer elective at the US HAEA Angioedema Center at the University of California, San Diego. Dr. Lisa Fu of the University of Toronto was selected from the applicants and attended earlier this year.



Our HAE Canada Survey concluded in September. This survey is going to be an excellent tool in allowing us to better serve the needs of our HAE Canada family members by ensuring we are abreast of the needs of our membership, enabling us at HAE Canada to provide ongoing support and advocacy now and in the future. We are grateful for the positive response and participation we received from our HAE Canada members.

At the end of the summer I was appointed Social Communication Coordinator and I have been working hard to increase our online presence through social media platforms like Facebook, Instagram, and Twitter. For the first time in HAE Canada history, our Annual AGM and Patient Education Day was broadcast live throughout the day via Facebook and will also be available later on the HAE Canada website. At the event, we also unveiled our Navigating the ER Tool Kit, a project spearheaded by HAE Canada board member Linda Howlett in collaboration with the Woodstock General Hospital. We have also made some changes to our HAE Canada website, in an effort to provide better and more current information for a more user-friendly experience.

HAE Canada founding member and long time Regional Director Atlantic, Anne Rowe has decided to step down from her Director's position with HAE Canada. During her time with HAE Canada, Anne has been instrumental in connecting all our HAE Family across Canada, as well as fostering HAE education and awareness in Canada. Without her influence we would not be the organization we are today. In her absence, we are pleased to welcome new HAE Canada Regional Director Atlantic, Kari Feere and look forward to all the skills Kari brings to our team.

At HAE Canada we know that our organization will only continue to improve and grow, and we look forward to everything the future holds for us and our HAE Canada Family.



SLOVAKIA

www.haei.sk

From President Michaela Bednářová, HAE Slovakia:

In October we were a part of the beautiful international HAE conference in Warsaw, Poland. It was a special experience for us as we had the possibility to learn more about the disease and inform other Slovak patients about our existence. It is important to make new friendships and help each other. We would like to thank the organizer Michal Rutkowski for the possibility to join to global HAE family.



BRAZIL

www.abranghe.org.br

By November 2017 the HAE organization in Brazil had registered 1,413 patients (994 female and 419 male).

Among the organization's many activities during 2017 has been participation in rare disease events in Brazil as well as in international gatherings, for instance the C1-INH Deficiency Workshop in Budapest, Hungary in May and the HAEi Youngsters' Summer Camp in Frankfurt, Germany in August. Furthermore, the organization has among other things attended a patient workshop organized by Shire, meetings for health professionals as well as medical conferences. Patient meetings were held in Salvador, Rio de Janeiro, Vitória, Fortaleza, Santo André, and Bom Despacho in May, while further patient meetings took place in Sao Paolo and Bom Despacho in November.



At the 2017 Brazilian congress of allergy the organization had an information stand and almost 70 doctors were registered to receive information. By now HAE Brazil has registered 508 doctors, allergists/immunologists, dermatologists, gynecologists, pediatricians, dentists, and other specialties forming a network of care for HAE patients.

Among the means of communication of the Brazilian HAE organization are a website (www.abranghe.org.br), a blog (www.abranghe.blogspot.com.br), and two Facebook pages (www.facebook.com/AEHBrasil and www.facebook.com/groups/1648041425415509).

News from around the Globe



PERU

www.haei.org/peru

My name is Suzet Lam and I am the President of HAE Peru. I am a midwife, I am married, and I have a daughter. Two of my three sisters also have HAE. I have had HAE since I was two years old, but I lived without knowing so until I was 31. I was diagnosed through the Dutch Red Cross. At that time, no laboratory in Peru took the C1 esterase inhibitor test. Nowadays, only one private lab takes it.

There is no available treatment for HAE acute attacks like Berinert or Firazyr. We only have preventive treatments like Danazol and tranexamic acid. I took Danazol for 15 years which has caused several side effects on my health, among them interstitial lung disease (ILD). The most common symptom of ILD is dyspnea or shortness of breath, which prevents me from doing things that I love like dancing or going out for a walk.

A couple of years ago, I had an esophageal edema, it was a very, very hard time for my family and me. Thank God, I survived even though there is no treatment in my country. However, every time I have an acute crisis, there is not much I can do but to rest waiting for the crisis to go away. This is the sad picture of how people with HAE and other rare diseases live in Peru.

The Peruvian government approved the Rare Diseases Act in 2011, which recognizes only 399 out of the more than 7,000 rare diseases in the world. Fortunately, HAE is one of them. For this act to be implemented, the Peruvian congress must approve some guidelines. However, it's been more than six years, and nothing has been done. Meanwhile, hundreds of rare disease patients see their rights to access an affordable health care violated.

In 2014, with the support of my family and friends, I decided to create HAE Peru. Through the organization I have met fellow Peruvians who also live with HAE. We share our experiences and we refer new patients to care centers. Nowadays, thanks to the HAE members and Oscar Calderon M.D, we continue our mission of educating ourselves and raising awareness within the medical community, so more people living with HAE have a faster diagnostic.



FINLAND

www.haefinland.org

On 1 December 2017 HAE Finland organized a seminar for Finnish doctors and medical personnel in order to celebrate the anniversary of the organization.



UKRAINE

<http://haei.org/location/hae-in-ukraine>

From Olena Mykal, President of the Ukrainian Patients Association for HAE:

In 2017 HAE patients established an association in Ukraine. The major goal of the association is to get access to the medications and diagnostics as well as spread awareness about HAE among doctors and general public in Ukraine. On September 23, the association had its first general meeting. The patients elected the members of the Executive Committee and voted for the signing of the memorandums on cooperation with the Ukrainian NGO "Rare immune diseases" and HAE Poland. Dr. Zabrodska, who is a leading doctor on HAE in Ukraine, gave a presentation about the disease, and instructed how to behave during the severe attacks. She also distributed a brochure on how to perform a tracheotomy in case of laryngeal edema and no access to medication, which is a case in Ukraine.

In cooperation with the Centre of Upper Airway Diseases of the Institute of Otolaryngology of Ukraine our association has issued a patient card for HAE patients with explanation of the disease and instructions for the doctors on what to do in case of severe attacks. Now each patient member of the association has such card.

Presently, the Ukrainian HAE Association has 25 members, 21 of them patients.



ISRAEL

www.edema.co.il

A new angioedema center is opening at the Barzilai University Hospital in Ashkelon. The hospital is located 40 miles south of Tel-Aviv and will be affiliated to the Immunology Division and the Immunology/Complement laboratory, headed by Prof. Eli Magen. The director of the Center is Dr. Avner Reshef MD while two other Allergy/Immunology specialists and residents

of Internal Medicine will be involved in consultation and treatment of HAE patients. The nursing staff will be led by an experienced HAE nurse, Ms. Iris Leibovich Nassi, chairwoman of the International HAE nursing organization. The new center welcomes patients from all regions of Israel, hoping to extend its services to the Palestinian Authority population as well.

News from around the Globe



SPAIN

www.angioedema-aedaf.org

From AEDAF President Sarah Smith Foltz:

AEDAF held its 13th regional patient workshop in Madrid on 30 November 2017. Even though the attendance was not as large as expected based on the number of people who had confirmed, there were still more than 50 people and everyone seemed to think the workshop was interesting, informative and useful. Along with patients and relatives/caregivers, quite a few physicians (mostly residents) from Madrid's major public hospitals joined us. Sarah Smith and

Dr. Concepción Lopez Serrano, Vice President of AEDAF, Dr. M. Luisa Baeza and Dr. Teresa Caballero of Gregorio Marañón and La Paz University Hospitals, respectively, and myself presented the activities of AEDAF and HAEi, an update of HAE and current treatment options, the situation of HAE in the region of Madrid, and a review of

national and international scientific research activities and study groups.

AEDAF will be celebrating its 20th anniversary in 2018. The 20th General Assembly and Annual Meeting will take place on 10 March 2018 in La Paz University Hospital in Madrid. We hope to have a good turnout to commemorate this important event – and we are pleased that Project Manager Ole Frølich and Communications Manager Steen Bjerre of HAEi will be joining us as well.

From Spain, AEDAF would like to wish everyone in the international HAE Community a very Happy Holiday and a Healthy New Year!



GERMANY

www.angiooedem.de

20 years is a perfect reason to meet and celebrate and that was just what members and friends of HAE Germany did 18 November 2017. Among the many speakers on the packed agenda were Professor Magerl from Charité Berlin, Dr. Martinez Sager from HZRM Mörfelden-Walldorf, Professor Bork from Uniklinik Mainz), Professor Schäfer from Uniklinik Marburg), Dr. Greve from University Hospital Ulm, and Dr. Aygören-Pürsün from the University Hospital of Frankfurt). Please see the next issue of this magazine for thorough coverage of the event.



HAEi Connect is ready to help the global member organizations

Over the last months HAEi has been working hard on completing HAEi Connect – a member database, communication management tool, and much more that HAEi is offering free of charge to its member organizations.

“HAEi Connect is now ready for our member organizations worldwide. We are finalizing the last corrections from the beta phase in Scandinavia, Australia, and New Zealand, but we are now starting to enroll HAEi Connect globally”, says HAEi Executive Director Henrik Balle Boysen:

“With HAEi Connect our member organizations will have an easy way to stay in touch with their members in their countries. Having a solid and secure member database is vital for our member organizations – it can and will help them grow their organization. HAEi Connect allows our member organizations to target the information that is sent out to the members, making sure it is relevant for the individual, especially when it's about clinical trials, market surveys, and relevant events. With HAEi Connect, our member organizations will also be able to create and send news to their members directly from Connect.”

HAEi Connect complies with the EU-GDPR General Data Protection Regulation, ensuring the protection of member information, and is built with the highest possible data security both on storage and login functionality.

Further information on HAEi Connect can be obtained via the HAEi Regional Patient Advocates or by reaching out to HAEi directly at info@haei.org.





Pharming and HAEi partners with Inceptua for Global Access Program

End September 2017 Pharming Group N.V in association with HAEi announced the appointment of Inceptua Medicines Access as their new distribution partner for the “HAEi Global Access Program” (HAEi GAP) enabling patients in all countries where Pharming’s product Ruconest is not commercially available to gain access to the drug through an ethical and regulatory compliant mechanism. It is the only known program of this type, which has been initiated through a patient group.

The program is the only Global Access Program in HAE. There is currently no cure for HAE, but certain treatments exist to prevent and ease attacks. These treatments are not licensed or available in all countries worldwide, leaving patients unable to prepare for and treat attacks.

Ruconest is the first treatment to be made available through the HAEi GAP program in countries where it is not commercially available. Ruconest is a recombinant human C1- inhibitor, approved by the European Medicines Agency (EMA) and US Food and Drug Administration (FDA) for the treatment of acute attacks of HAE.

Physicians wishing to request Ruconest for their patients through the HAEi GAP program should contact HAEiGAP@inceptua.com or call +44 20 3910 7670. Please note that direct patient inquiries cannot be handled.

“HAEi exists to support patients with HAE gain access to HAE therapies to improve their quality of life and potentially be life-saving in the case of a laryngeal attack,” commented Henrik Balle Boysen, HAEi Executive Director. “Through this partnership with Pharming Group N.V. and Inceptua Medicines Access, physicians, who may otherwise have no access to modern HAE therapies, have the opportunity to request Ruconest for their patients, effective immediately.”

“We are delighted to announce, in association with HAEi, that Inceptua Medicines Access are our new trusted distribution partner, offering a very tailored solution, providing navigation support to physicians in an ethical and regulatory compliant way to gain access to this urgently-required treatment for their patients,” said Paul Janssen, Pharming Group N.V., Vice-President Commercial Operations Europe and ROW.

Mark Corbett, Executive Vice-President, Inceptua Medicines Access commented: “We are providing a transformational approach to medicines access, we will strive to provide unrivaled service in navigating access to Ruconest for the treatment of patients in need. We are delighted to work with both HAEi and Pharming in delivering what is the only known Medicines Access Program instigated by a patient organization. We will navigate access to this medicine with agility, quality, and regulatory compliance, ultimately to help the treatment of patients in need.”





Clinical Trials

According to clinicaltrials.gov under the U.S. National Institutes of Health the following trials should be recruiting at this moment – please see more at <https://clinicaltrials.gov>:

Safety of Ruconest in 2-13 Year Old HAE Patients

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

Study of BCX7353 as a Treatment for Attacks of HAE

– recruiting in Austria, Denmark, France, Germany, Hungary, Israel, Macedonia, Romania, Switzerland, and the United Kingdom

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

– recruiting in the United States

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

– 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 the United Kingdom

Determination of Specific Biomarkers of Acute Attack of Angioedema Within Pediatric Population

– recruiting in France

The Role of the Coagulation Pathways in Recurrent Angioedema

– 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)

– recruiting in France

According to the International Clinical Trials Registry Platform under World Health Organization (WHO) the following trial is also recruiting at this moment – please see more at <http://apps.who.int/trialsearch>

Pathophysiological study for autoimmune dysregulation of HAE

– recruiting in Japan

Global Advocacy Work

Recent events

- 29 – 30 September:** HAEi participated in and conducted the 2017 HAEi South Eastern Europe Workshop in **Skopje, Macedonia**.
- 7 – 8 October:** HAEi participated in and conducted the 2017 HAEi Central and Eastern Europe Workshop in **Warsaw, Poland**.
- 13 – 16 October:** HAEi met with the Philippine Society for Orphan Diseases (PSOD) and a HAE patient family in **Manila, Philippines** as HAEi is helping the physician and patients identify more HAE families with an eye towards an inaugural HAE Philippines meeting in late 2018.
- 24 – 26 October:** HAEi attended the first meeting of a newly formed South Africa patient group in **Cape Town, South Africa**.
- 10 – 12 November:** HAEi participated in the 2017 Scandinavian Conference by HAE Scandinavia in **Stockholm, Sweden**.
- 17 – 19 November:** HAEi participated in and presented at the 20th anniversary of HAE Germany in **Berlin, Germany**.
- 17 – 19 November:** HAEi participated in and presented at the HAE UK Patient Day in **Sheffield, United Kingdom**.
- 22 – 23 November:** HAEi participated in Professor Konrad Bork's annual German meeting on HAE in **Mainz, Germany**.
- 27 – 30 November:** HAEi met with the patient group and leading physician team in **Bangkok, Thailand** to pursue new options to activate the HAEi Global Access Program.
- 8 – 9 December:** HAEi had a strategic meeting for the Gulf Region in **Dubai, United Arab Emirates**. Part of the objective was to get a better understanding of the current limitations in the region – and see how the HAEi Global Access Program and other HAEi tools can best be used in the region.

Upcoming events

- 10 – 12 January:** HAEi will participate in the annual Plasma Users Group (PLUS) stakeholder meeting in **Dublin, Ireland**.
- 19 – 20 January:** HAEi Executive Committee meeting and workshop in **Tokyo, Japan**.
- 16 – 18 February:** HAEi will host a Regional Patient Advocate Workshop in **Frankfurt, Germany**.
- 2 – 4 March:** HAEi will participate in the annual American Academy of Allergy, Asthma and Immunology (AAAAI) in **Orlando, USA**.
- 8 – 10 March:** HAEi will conduct and host the 2018 HAEi China Patient and Physician Workshop in **Beijing, China**.
- 8 – 10 March:** HAEi will participate in the 20th anniversary of HAE Spain (AEDAF) in **Madrid, Spain**.



Medical Papers

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

Outcomes of long-term treatments of type I HAE in a Turkish family – by G. Akoglu, Ataturk Training and Research Hospital, Turkey et al.:

Patients with C1-INH HAE may be misdiagnosed as having familial Mediterranean fever in regions where the disorder is endemic. Medical history, suspicion of HAE and laboratory evaluations of patients and their family members lead the correct diagnoses of HAE. Danazol and C1 replacement treatments provide significant reduction in HAE attacks. (*An Bras Dermatol., Sep-Oct 2017*)

Genetic Determinants of C1 Inhibitor Deficiency Angioedema Age of Onset – by P. Gianni et al., University of Thessaly, Greece:

Functional alterations in genes of proteins involved in bradykinin metabolism and function affect the clinical phenotype and possibly contribute to the pathogenesis of C1-INH-HAE. Given that an earlier onset of symptoms is inversely correlated with the subsequent course of the disease and, eventually, the need for long-term prophylaxis, these polymorphisms may be helpful prognostic biomarkers of disease severity. (*Int Arch Allergy Immunol., 2017*)

HAE with Normal C1 Inhibitor and F12 Mutations in 42 Brazilian Families – by C.L. Veronez, Federal University of São Paulo, Brazil, et al.:

A large number of patients with FXII-HAE, and, as the first such study conducted in a South American population, it highlighted significant differences between this and other study populations. The high number of symptomatic males and patients with estrogen-independent FXII-HAE found here suggests that male sex and the absence of a hormonal influence should not discourage clinicians from searching for F12 mutations in cases of HAE with normal C1-INH. (*J Allergy Clin Immunol Pract., Nov 2017*)

Endocan: A Novel Marker of Endothelial Dysfunction in C1-Inhibitor-Deficient HAE – by M. Demirturk et al., Istanbul University, Turkey:

The increased endocan and Vascular cell adhesion molecule levels may reflect an endothelial activation even in attack-free periods in HAE patients. (*Int Arch Allergy Immunol., 2017*)

Health-related quality of life in Danish children with HAE – by A. Aabom, Odense University Hospital, Denmark:

All Danish children ages 2-18 years with C1-INH-HAE were invited to complete questionnaires regarding health-related quality of life. The children assessed on average had a normal health-related quality of life and better than those with other common skin disorders. However, according to our findings, health care providers should be especially attentive to health-related quality of life when children with C1-INH-HAE become symptomatic. (*Allergy Asthma Proc., Nov 2017*)

Efficacy of recombinant human C1 esterase inhibitor for the treatment of severe HAE attacks – by H.H. Li et al.:

RhC1-INH was efficacious in resolving severe HAE attacks, including oropharyngeal-laryngeal attacks. The rhC1-INH rescue treatment rapidly improved symptoms for patients who received placebo and who experienced worsening or sustained symptoms. (*Allergy Asthma Proc., Nov 2017*)

Treatment of HAE due to C1 inhibitor deficiency in Argentina – by E. Malbran, Asma e Inmunología Clínica, Buenos Aires, Argentina, et al.:

Two hundred and twenty five patients registered in the Argentina HAE Patient Association (AHAEPA) were randomly selected and invited to participate in a web based questionnaire on accessibility to icatibant and pdC1-INH, self-treatment, delay to treatment, and coverage. Argentina's C1-INH-HAE patients had a sustained improvement in their access to medication. Efforts should continue to further improve accessibility and optimal management of HAE acute attacks to all patients in the country. (*Medicina (B Aires), 2017*)

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

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, Aug 2017*)

The relationship between anxiety and quality of life in children with HAE – by A. Kessel, Technion Faculty of Medicine, Israel, et al.:

C1-INH-HAE children have higher anxiety trait and state, which correlate with reduced health-related quality of life domains. (*Pediatr Allergy Immunol., Nov 2017*)

Treatment Effect and Safety of Icatibant in Pediatric Patients with HAE – by H. Farkas, Semmelweis University, Hungary, et al.:

The objective of this phase 3 study was to evaluate the efficacy/safety of a single subcutaneous dose of icatibant (0.4 mg/kg; maximum 30 mg) in pediatric patients with C1-INH-HAE. Symptom relief was rapid, and a single icatibant injection in pediatric patients with C1-INH-HAE was well tolerated. (*J Allergy Clin Immunol Pract., Nov-Dec 2017*)

Diminished capacity of opsonization and immune complex solubilization, and detection of anti-C1q antibodies in sera from patients with HAE – by D. Honda et al., Juntendo University Faculty of Medicine, Japan:

Sera from patients with HAE exhibit anti-C1q antibodies, with a lower capacity for opsonization and immune complex solubilization. This may be associated with immunological abnormalities and should be investigated further to facilitate accurate diagnosis of HAE. (*Allergol Int., Oct 2017*)

Complement factor C4 activation in patients with HAE – by A. Aabom, Odense University Hospital, Denmark, et al.:

The C4c/C4 ratio seems to be a better diagnostic measure than total antigenic C4 alone. Our findings underline that screening with total antigenic C4 implies a risk of overlooking C1-INH-HAE patients. (*Clin Biochem., Oct 2017*)

Angioedema in the emergency department: a practical guide to differential diagnosis and management – by J.A. Bernstein, University of Cincinnati, USA, et al.:

Emergency department physicians must be aware of the different pathophysiologic pathways that lead to angioedema in order to efficiently and effectively manage these potentially fatal conditions. (*Int J Emerg Med., Dec 2017*)

News from the Industry

28 September 2017

Verseon Corporation has announced the launch of a drug program developing oral treatments for HAE. In their interim report, the Company presents data from a well-established HAE disease model, which demonstrates the efficacy of their plasma kallikrein inhibitors in reducing swelling.

An oral therapeutic would be a life-changing development for HAE patients who currently rely on treatments administered via intravenous or subcutaneous injections. Verseon is building on expertise established in its diabetic macular edema program to develop small-molecule plasma kallikrein inhibitors suitable for oral dosing. Plasma kallikrein is an established target central to the HAE disease pathway, which is used in existing HAE treatments, including Shire's injectable HAE drug Kalbitor.

Verseon has developed a range of plasma kallikrein inhibitors using its computationally driven drug discovery platform. Many of these inhibitors have demonstrated good oral pharmacokinetic exposure, which is critical for convenient once-a-day oral dosing. In the interim report, Verseon presents data on a lead candidate that reduces swelling with efficacy comparable to a positive control in a carrageenan-induced paw edema model, a standard preclinical model for HAE.

Dr. David Kita, Vice President of R&D at Verseon, commented, "We are excited about the promising preclinical results for our HAE candidates. We are continuing to optimize our drug candidates for oral bioavailability, efficacy, and safety to develop an effective and convenient treatment, which has the potential to change the standard of care for HAE patients."

Verseon is a technology-based pharmaceutical company that employs its proprietary, computational drug discovery platform to develop novel therapeutics that are unlikely to be found using conventional methods. The Company is applying its platform to a growing drug pipeline and currently has four active drug programs in the areas of HAE, anticoagulation, diabetic macular edema, and oncology.

(Source: Verseon)



2 October 2017

Pharming Group N.V. announces positive data from a clinical trial with the use of Ruconest [Recombinant Human C1 Esterase Inhibitor/ conestat alfa] for the treatment of HAE attacks in children.

The open-label, single arm, Phase II clinical trial was designed in agreement with the European Medicines Agency (EMA) as part of a Paediatric Investigation Plan (PIP) to assess the pharmacokinetic, safety and efficacy profiles of Ruconest at a dose of 50 IU/kg in paediatric HAE patients ages 2-13 years in support of a paediatric indication for treatment of HAE attacks.

A total of 20 children with HAE were treated for 73 HAE attacks at a dose of 50 IU/kg (up to a maximum of 4200 IU). The study reported clinically meaningful relief of symptoms assessed using a visual analogue scale (VAS) completed by the patient (assisted by their parent). The median time to onset of relief was 60 minutes (95% confidence interval: 60-63), and the median time to minimal symptoms was 122 minutes (95% confidence interval: 120-126). Only 3/73 (4%) attacks were treated with a second dose of Ruconest.

Ruconest was generally safe and well-tolerated in the study. No patients withdrew from the study due to adverse events. There were no related serious adverse events, hypersensitivity reactions, or neutralizing antibodies detected.

The detailed efficacy and safety results will be further analyzed and submitted for presentation at an upcoming major medical meeting.

Prof. Bruno Giannetti, Pharming's Chief Operations Officer, commented:

"The results of this study provide important safety and efficacy data on the use of Ruconest in children and show good clinical concordance with results from adolescents and adults with HAE. Children with HAE have limited therapeutic options, and we will work expeditiously with regulatory authorities to expand choices for these patients."

(Source: Pharming)



26 October 2017

The European Commission (EC) has approved a label extension granting a new indication for Firazyr (icatibant injection), broadening its use to adolescents and children aged 2 years and older, with HAE caused by C1-esterase-inhibitor (C1-INH) deficiency. Firazyr has been approved in the European Union (EU) since 2008 for symptomatic treatment of acute attacks of HAE in adults with C1-INH deficiency.

“As a long-term partner to the HAE community, we understand the unique burden this disease places on children living with HAE and their caregivers,” said Jennifer Schranz, Global Development Lead, HAE, **Shire plc**. “This approval in Europe demonstrates our unwavering commitment to helping patients and represents a significant advance for paediatric patients, who now have a subcutaneous treatment option for acute HAE attacks.”

The use of Firazyr in paediatric patients was studied in an open label, non-randomised single-arm study, involving 32 paediatric patients with HAE. The efficacy population consisted of 11 children and 11 adolescents with attacks. The primary efficacy endpoint was time to onset of symptom relief (TOSR) based on the investigator-assessed composite post-treatment symptom score, defined as earliest time post-treatment when 20% or more improvement in the composite symptom score was achieved, without worsening of any single component score.

Overall, median TOSR was 1.0 hour, with no differences between children and adolescents. More than 70% of patients experienced symptom relief at 1.1 hours, and more than 90% by 2 hours post-treatment.

The majority of paediatric patients who were treated with subcutaneous Firazyr experienced injection site reactions such as erythema, swelling, burning sensation, skin pain and itching/pruritus; these were found to be mild to moderate in severity and consistent with reactions that have been reported in adults.

The study showed Firazyr was well tolerated and demonstrated rapid resolution of symptoms during an HAE attack through a single injection. The study that led to this approval is the first and only trial investigating a subcutaneous therapy in the HAE paediatric population.

“Due to the unpredictable and debilitating nature of HAE attacks, children living with the condition can benefit from having a new treatment option that can provide symptomatic relief of acute HAE attacks with a subcutaneous injection,” said Henrik Balle Boysen, Executive Director of HAEi. “Clinical work to bring treatment options to younger patients is vitally important and greatly appreciated by the global HAE community.”

Firazyr will be available for use in paediatric patients in Europe beginning in Q4.

(Source: Shire)



27 October 2017

Pharming Group N.V. presents its (unaudited) financial report for the first nine months and the third quarter ended 30 September 2017.

Operational highlights during the third quarter

- On September 11, following the conclusion of the End-of-Phase 2 interactions with the US Food and Drug Administration (FDA), Pharming announced that it will submit a supplemental Biologics License Application (sBLA) to the FDA for review in Q4 of this year for prophylaxis of angioedema attacks in adolescent and adult patients with hereditary angioedema (HAE) as an expanded indication for Ruconest [Recombinant Human C1 Esterase Inhibitor/ conestat alfa]
- On September 26, the Company, in association with HAEi announced the appointment of Inceptua Medicines Access as their new distribution partner for the “HAEi Global Access Program” (HAEi GAP) enabling patients in all countries where Pharming’s product Ruconest is not commercially available to gain access to the drug through an ethical and regulatory-compliant mechanism
- Positive results were obtained from a Phase II clinical trial investigating the use of Ruconest for the treatment of HAE attacks in children.

CEO Sijmen de Vries commented: “We are on the right track with our strategy for Ruconest in all markets. We now see real growth in terms of both volume and value for Ruconest. In addition, we continue to make good progress with our pipeline research and development programs.

Towards the end of the quarter we were informed of acute shortages of HAE medication as a result of manufacturing issues for certain competitor HAE products, mainly in the US. To help resolve this situation for patients, we immediately offered instant access to our patient care programme, Ruconest Solutions, including its free starter medication and bridging support for those patients in acute need of alternative medication to treat their HAE attacks. We have therefore been supplying considerable amounts of Ruconest free-of-charge to cover treatment of attacks for the period during which those patients are being cleared for Ruconest reimbursement. Patients are at the very centre of Pharming’s business and we are doing our best to ensure that HAE patients get effective treatment. As a result of this situation, we have accelerated planned increases in capacity across our supply chain. We do not believe this situation has had any real effect on our results for the third quarter, but it is likely to have a positive effect on the company’s performance in the fourth quarter.”

(Source: Pharming)



27 October 2017

Shire plc announces unaudited results for the three months ended September 30, 2017. Flemming Ornskov, M.D., M.P.H., Shire CEO, commented:

“We delivered strong growth this quarter despite a Cinryze supply shortage. We experienced a product shortage of Cinryze during the quarter due to a manufacturing interruption at a third-party

manufacturer. The issue has been addressed and production of Cinryze has resumed. Product was shipped to customers in early October. To enhance reliability of supply, we plan to start in-house production of Cinryze by Q1 2018, subject to FDA approval, as sustainable and unconstrained Cinryze supply is a top priority.”

(Source: Shire)



31 October 2017

CSL Behring presents data indicating that, at the approved dose of 60 IU/kg, HAEGARDA® reduced the median number of HAE attacks per month by 98 percent in subjects who had frequent attacks, from a 16-week placebo period to a 16-week treatment period. Additionally, the breakthrough attack rate – extrapolated to one year – was reduced from approximately 70 attacks per year for subjects on placebo to approximately six attacks per year for the same subjects when on HAEGARDA. These findings were presented in an oral abstract session at the 2017 American College of Allergy, Asthma & Immunology (ACAAI) Annual Scientific Meeting.

“Approximately 30 percent of HAE patients experience weekly attacks, and the majority of those attacks can be debilitating to daily living,” said Timothy Craig, D.O., Professor of Medicine and Pediatrics, Pennsylvania State University Medical School, Hershey, Pennsylvania. “These data demonstrate the ability of HAEGARDA to effectively prevent HAE attacks in patients who have severe and frequent HAE attacks.”

The presented subgroup analysis was based on data from the Phase III COMPACT trial. The subgroup analysis evaluated 21 severely impacted patients of the 43 study subjects who received 60 IU/kg of subcutaneous C1-esterase inhibitor (C1-INH) and a corresponding placebo over a 16-week treatment period each. High attack frequency was defined as experiencing at least one HAE attack per week (on average, greater than or equal to four attacks per month) while on placebo.

“These results reinforce the efficacy we’ve seen across a broader patient population, and add to our body of knowledge by showing that HAEGARDA is also highly effective in reducing the number of attacks in patients severely affected by HAE,” said Debra Bensen-Kennedy, MD, Vice President for Medical Affairs, North America, CSL Behring. “HAE is an extremely debilitating disease, and this continued research and the development of HAEGARDA reflects our commitment to improving patients’ lives.”

HAEGARDA is a self-administered, plasma-derived concentrate of C1-INH injected twice weekly subcutaneously. HAEGARDA targets the root cause of HAE by replacing deficient or dysfunctional C1-INH protein, restoring C1-INH levels above 40 percent, which is proposed to reduce the risk of HAE attacks. Subcutaneous administration of C1-INH builds and maintains steady-state functional C1-INH levels within three to four doses of HAEGARDA.

The U.S. Food and Drug Administration (FDA) approved HAEGARDA on 22 June 2017 for routine prophylaxis to prevent HAE attacks in adolescent and adult patients.

(Source: CSL Behring)

CSL Behring

10 November 2017

Amber Salzman, Ph.D., President and CEO of **Adverum Biotechnologies Inc.**, in the company’s financial results for the third quarter of 2017:

“We continue to make progress advancing our gene therapy programs to reach our goal of transforming Adverum into a clinical-stage company by the end of this year. Looking ahead, we plan to file an Investigational New Drug application for HAE with the FDA in the second half of 2018. We are well positioned and well capitalized to accelerate the development of our pipeline of novel gene therapies in 2018.”

ADVM-053 is the Adverum gene therapy product candidate designed for the treatment of patients with HAE. ADVM-053 is an AAV based gene transfer vector that aims to provide persistent levels of human C1-esterase inhibitor (C1-INH) to potentially correct the deficiency state that can lead to life threatening attacks.

(Source: Adverum)

ADVERUM
BIOTECHNOLOGIES

27 November 2017

Pharming Group N.V. has submitted a supplemental Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for Ruconest [Recombinant Human C1 Esterase Inhibitor/ conestat alfa] for routine prophylaxis to prevent attacks in adult and adolescent patients with HAE.

The submission includes data from two completed trials of Ruconest for the prophylaxis of HAE attacks: a randomized, double-blind, placebo-controlled trial and an open-label study. The two studies enrolled a total of 56 patients and showed consistent efficacy and safety results.

Dr. Bruno Giannetti, MD PhD, COO of Pharming, commented: “HAE patients in the US are currently facing a shortage of plasma-derived C1 inhibitor used to prevent attacks. We understand that this supply disruption has had serious consequences for them, including additional stress, disease-related complications, and hospitalizations. We look forward to working with FDA and potentially providing these patients an alternative and plasma free option for HAE prophylaxis.”

(Source: Pharming)

 **Pharming**

14 December 2017

KalVista Pharmaceuticals, Inc. today reported operational and financial results for the fiscal second quarter ended October 31, 2017.

The initial focus is on inhibitors of plasma kallikrein, which is an important component of the body’s inflammatory response and which, in excess, can lead to increased vascular permeability, edema and inflammation. KalVista has developed a proprietary portfolio of novel, small molecule plasma kallikrein inhibitors initially targeting HAE. The company has created a structurally diverse portfolio of oral plasma kallikrein inhibitors from which it plans to select multiple drug candidates to advance into clinical trials for HAE. KalVista’s most advanced program, an intravitreally administered plasma kallikrein inhibitor known as KVD001, has successfully completed its first-in-human study in patients with DME and is being prepared for Phase 2 studies in 2017. Andrew Crockett, CEO of KalVista, comments:

“We submitted the regulatory filing to enter the clinic with our second oral plasma kallikrein inhibitor candidate for potential treatment of HAE, KVD900, and there will be at least one additional HAE portfolio candidate entering the clinic in 2018.”

(Source: KalVista)


KalVista
PHARMACEUTICALS



HAEi around the world

Currently there are HAE member organizations in 61 countries. You will find much more information on the HAE representations around the globe at 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 is being updated as soon as HAEi receives fresh data from the national member organizations.

