


Global perspectives:

HAEI MAGAZINE · ISSUE 3/2019

 78 Member countries



**HAE
GLOBAL
CONFERENCE
FRANKFURT
14-17 MAY
2020**

HIGHLIGHTS FROM THE 2019
US HAEA national patient summit

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EMERGENCY CARDS
now in 28 languages

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**Global Perspectives
Issue 3/2019
October 2019**

Cover photo

The Sheraton Frankfurt Airport Hotel and Conference Center is located directly in Terminal 1 of Frankfurt Airport - and it is the venue for the 2020 HAE Global Conference.

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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



DEAR HAE INTERNATIONAL FRIENDS,

It is with great sadness and a deep sense of loss that we note the passing of Professor Marco Cicardi from Milan, Italy – a truly extraordinary leader in the global HAE community and founder of HAE International. Beloved by all, Professor Cicardi was nothing short of a true giant in HAE circles. A world-class scientist and compassionate physician, he spent his professional career unlocking HAE's genetic and biological causes and taking care of HAE patients. We mourn while struggling to deal with the untimely death of this amazingly kind and talented man. We will always be grateful for his life's work and magnificent legacy of improving the quality of life for HAE patients throughout the world.

While we cannot replace Professor Cicardi, we can continue to pursue his objective of making life better for HAE patients and their families. Consistent with this idea, HAE International is developing and field testing an exciting survey instrument that member organizations can use to systematically and independently collect information on patient quality of life and the cost of HAE care in their respective country. Our proprietary survey tool is being designed to produce a robust data set and slide deck that quantifies the impact of untreated or under-treated HAE and will therefore show the value of modern HAE therapies. This real-world, independently collected data will arm patient advocates with a powerful and authoritative voice that Health Ministries and other decision-makers cannot deny or ignore as they deliberate access to therapy. We will provide more information on this

exciting program as we finalize the field-testing phase.

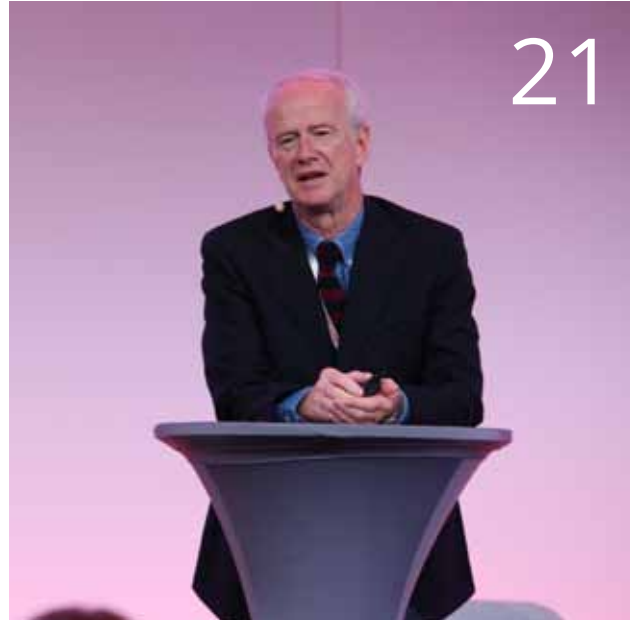
Finally, excitement is building for the 5th HAE Global Conference that will take place in Frankfurt, Germany on 14-17 May 2020. Well over 1,000 people are expected to attend this largest-ever international gathering of patients, caregivers, healthcare professionals, and the pharmaceutical industry. Our Regional Patient Advocates are very much looking forward to conducting meetings with conference attendees from their regions. The Regional Patient Advocates will be asking our member organizations for new ideas, perspectives, and feedback on HAE programs and activities. Similar to prior global conferences, the 2020 conference will offer specific "tracks" for patients and caregivers, physicians/scientists, and HAE Youngsters. As suggested by the theme for the 2020 conference – Creating the Path for Better Health – we are looking to create an environment where all attendees can interact and share insights, strategies, and techniques for either broadening or winning access to modern HAE therapies.

I wish all HAE International friends happiness and good health.

Warm regards,

Anthony J. Castaldo
President & CEO, HAE International

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NEWS FROM THE HAEI REGIONAL PATIENT ADVOCATES

Many countries around the world are benefiting immensely by the work that each of the HAE International Regional Patient Advocates is doing. Each edition of the *Global Perspectives* magazine showcases the great work, and this one is no different.

Regional Patient Advocates took part in the Youngsters Advocacy Workshop in Atlanta. The workshop was a great event, each youngster inspired all the advocates, and we all enjoyed working with them and helping with their projects. The Regional Patient Advocates also presented to the youngsters during the workshop on different aspects of advocacy and awareness.

Some of the work completed during the last few months is the translation of many emergency cards into local languages, organizing and attending regional meetings and workshops, establishing contact with patients and physicians, and the creation of new patients groups.

Fiona Wardman

Chief Regional Patient Advocate



NATASA ANGJELESKA
SOUTHEASTERN EUROPE



I am facilitating discussions with patients from **Albania** and **Croatia** on the State of Management survey. As for **Bosnia & Herzegovina**, there are some developments with regard to the translation of the emergency card, the HAE patient group being part of a partnership with the Rare Disease Alliance to improve patient numbers and educate healthcare professionals in emergency departments on HAE. In **Bulgaria**, a seminar has been held for patients to learn about self-administration while in **Montenegro** the President of HAE Montenegro has translated the emergency card into the Montenegrin language. Furthermore, in **Serbia**, the patient organization is creating an emergency card that will have physician approval. An article about HAE has been published in a media in **Slovenia** in which Teja Iskra from HAE Slovenia discussed her experience with HAE, and professor Mitja Kosnik from the Golnik Clinic explained about the disease genetics and treatment options. In **North Macedonia**, the HAE Macedonia team took part in a charity party with more than 200 attendees organized to raise funds intended for the organization.



MARIA FERRON
MEDITERRANEAN & MAGHREB COUNTRIES



Earlier this year the full Maghreb region – encompassing all the North African countries – was added to my domain. In **Tunisia**, patients are being encouraged to visit their doctors to have an official HAE diagnosis; following this, a patient group has been created. Discussions with physicians in **Libya** have taken place, along with the patient lead who is in contact with other patients in the country. In **Mauritania**, contact has been made with a doctor who is interested in HAE and work will begin on creating awareness amongst the doctor's colleagues. A social media awareness campaign is taking place in **Algeria** to locate more HAE patients – and Algeria will host an HAE International regional patient and physician workshop during the first quarter of 2020.



PATRICIA KARANI
SUB-SAHARA AFRICA



I am assisting with information and resources for an upcoming meeting in **Benin** with a paediatric doctor. Correspondence with RADD (Rare Diseases and Disabilities Africa Foundation) has begun as we aim to start working with doctors to raise awareness and create a steering committee for HAE. Discussions are continuing with a doctor in **Senegal** who is linking me with patients, and working on ways to develop HAE awareness between healthcare professionals and colleagues, and the start of a steering committee. In **Rwanda**, ongoing discussions are taking place with The Allergy Society to incorporate HAE as a topic of discussion amongst the members. Doctors in **Sudan** are willing to be points of contact, and a meeting is being scheduled to discuss how awareness of HAE can be raised. Following the introduction of the HAEi Global Access Program in **South Africa**, the first patients in the country are now receiving modern treatment.



FERNANDA DE OLIVEIRA MARTINS
SOUTH AMERICA & MEXICO



Mexico and **Colombia** are in the process of setting up a website through HAE International along with HAEi Connect. I am supporting a patient in **Ecuador**. Very recently, we have added **Paraguay** as HAE International country no. 77 – and we are working on setting up both a website through HAE International and HAEi Connect. In **Brazil**, I am assisting with the translation of the next Brady Club Activity Book as well as working on using HAEi Connect for the Brazilian member database.



MICHAL RUTKOWSKI
CENTRAL EAST EUROPE,
MIDDLE EAST & GULF REGION



Armenia, Georgia and **Lithuania** shown great interest in being part of HAE International and I expect them to join our family soon. The patients organizations in **Belarus, Hungary** and **Ukraine** are in the start-up process with HAEi Connect. A HAE patient survey in **Poland, Armenia, Belarus, Estonia, Georgia, Hungary, Kazakhstan, Lithuania, the Netherlands, Russia, Slovakia** and **Ukraine** is collecting data for the upcoming Central Eastern Europe Regional Workshop in October 2019. A workshop is being organised for the Gulf & Middle East patients and physicians and it is expected to take place 9-11 January 2020 in Beirut, **Lebanon**. The HAE International emergency card has been translated into Arabic, Armenian, Belarusian, Dutch, Georgian, Hungarian, Kazakh, Polish, Russian, Slovakian, and Ukrainian – and soon Iranian and Lithuanian will follow.



JAVIER SANTANA
CARIBBEAN AND CENTRAL AMERICA



Meetings and discussions have taken place with pharma representatives to discuss the modern therapy situations in **Puerto Rico**, the **Dominican Republic**, **Costa Rica**, **Panama**, and **Guatemala**. The HAE patient group in Panama is continuing the efforts to advocate for the government to consider granting access to HAE treatments. HAE materials and resources have been provided in Spanish to the patient group in Guatemala – the group is working towards being a registered organization and becoming a member of the Association of Rare Diseases in Guatemala. In **El Salvador**, the website for the national group is now live, and the group wishes to use this for educational campaigns. A nurse in El Salvador is creating awareness of HAE by visiting allergy clinics and providing them with resources.



FIONA WARDMAN
ASIA PACIFIC



A very successful first of its kind patient and doctor meeting was held in **Hong Kong** in August; a patient group was formed, and HAE Hong Kong is now busy working on a website, using HAEi Connect and planning the next get together. In **China**, I have met with the patient leads from HAE China which was a good opportunity to put some plans in place for a dedicated website under the HAE International umbrella along with the use of HAEi Connect member database. I am working with HAE Japan President Beverley Yamamoto on the agenda and presentations for the patient meeting in October in Tokyo, **Japan**. A Facebook page has been set up for the national organization in **India**. Finally, the agenda and speakers for the upcoming patient and physician meeting in November in Seoul, **South Korea** has been finalized.





2020 HAE GLOBAL CONFERENCE – “CREATING THE PATH FOR BETTER HEALTH”

The 5th HAE Global Conference, taking place in Frankfurt, Germany 14-17 May 2020, will be the biggest ever global gathering of patients, caregivers, healthcare professionals, and the pharmaceutical industry.

“We expect around 1,000 participants in Frankfurt. This number is based on the travel grant application process that was concluded on 30 September 2019 and for which we saw an overwhelming interest”, says Henrik Balle Boysen, the HAE International Executive Vice President and COO.

Registration for the 2020 HAE Global Conference is now open. Everyone who received a travel grant have now been notified – and must then register before 3 November 2019. From that date, their travel grant will become invalid.

“People without travel grants can register at any time. We do, however, recommend that you don’t wait too long as registration will be on a first come first serve basis”, says Henrik Balle Boysen.

HAE International is investigating the possibility of simultaneous translation during the conference. As simultaneous translation is coming along with a high additional cost HAE International will this time evaluate whispering translation if around 50 people share the same language.

“Please observe that we cannot guarantee translations at this point – and registrants cannot expect their preferred language to be added until HAE International has officially announced this. The main conference language will remain English”, says Henrik Balle Boysen.

HAE International is currently working on the conference program but can reveal that this conference – like the previous ones – will offer a Patient Track, a Scientific Track, and a Youngsters Track.

Read more about the tracks on the conference website at haegc20.haei.org where you will also be able to follow the program for the conference as it develops around the conference theme “Creating the Path for Better Health”.



HAEi JAKOBUSWEG WALK 2020



READY FOR WALKING THE JAKOBUSWEG?

Following the successful HAEi/AEDAF Camino Walks on the legendary *Camino de Santiago* in Spain in 2016, 2017 and 2018 as well as the walk on the *Jakobsweg* in Vienna, Austria in 2018, HAE International is planning yet another mutual walking experience.

“This time we will be walking in Frankfurt am Main,, Germany as this is where the 2020 HAE Global Conference takes place”, says HAE International Director of Communications Steen Bjerre:

“Once again the purpose is to raise awareness through bringing together HAE friends – patients, relatives, caregivers, doctors, nurses, and industry – for a mutual experience in honor of the **hae day :-)** global awareness day.”

While the three walks in Spain have taken place on the *Camino de Santiago*, the event in Vienna followed the pilgrimage route *Jakobsweg* (The Way of St. James) through the Austrian capital. Steen Bjerre says:

“*Camino de Santiago*, Way of St. James, *Jakobsweg*, and *Jakobusweg* are names in different languages for the same thing: A huge network of pilgrims’ ways leading to the shrine of the apostle St. James the Great in the cathedral of Santiago de Compostela in northwestern Spain, where tradition has it that the remains of the saint are buried. Many follow its routes as a form of spiritual path or retreat for their spiritual growth. Traditionally, as with most pilgrimages, the Way of St. James began at one’s home and ended at the pilgrimage site, often following highly travelled routes through Europe. One of these traditional routes is found in Frankfurt – and that is the one we will be walking for the HAEi *Jakobusweg* Walk 2020.”



The walk is to place Wednesday 13 and Thursday 14 May 2020 – in other words just before the 2020 HAE Global Conference. The program consists of three stages. It is, however, entirely up to the participants if they want to take part in one or more of them.

“You can choose either a short walk or a long distance: The short HAEi Jakobusweg Walk takes place Thursday morning with the Mutter vom Guten Rat Church as starting point and goes via St. Leonhard’s Church to the Frankfurt Cathedral. That is a distance of 4.9 km”, says Steen Bjerre.

The long HAEi Jakobusweg Walk starts Wednesday morning and consists of a 5.2 km walk from the Mutter vom Guten Rat Church to St. Mauritius’ Church followed by a 4.2 km walk from St. Mauritius’ Church to St. Justinus’ Church - the oldest building in Frankfurt and indeed one of the oldest in Germany built between 830 and 850.

“Furthermore, on Thursday morning the participants doing the long walk will join the pilgrims doing the short one – in other words once more starting at the Mutter vom Guten Rat Church but now going via St. Leonhard’s Church to the Frankfurt Cathedral. The total distance of the long walk is 14.3 km”, Steen Bjerre says.

The fee for participation in the HAEi Jakobusweg Walk 2020 is 15 EUR per person for the short distance, 25 EUR per person for the long distance. The fee includes a HAEi Jakobusweg Walk T-shirt as well as train from Frankfurt Airport to the starting point. All other transportation, snacks, drinks and meals are not included.



SIGN UP!

If you want to take part in the HAEi Jakobusweg Walk 2020, you just add the event when you go through the signup procedure for the 2020 HAE Global Conference.

Please see <https://haegc20.haei.org>

Highlights from **THE 2019 US HAEA** **NATIONAL PATIENT SUMMIT**

By Ianice Viel, Patient Advocate/Social Media Manager, HAEA



Over 1,000 HAE patients, caretakers, medical professionals, scientists and HAEA friends participated in the 2019 HAEA National Patient Summit under the heading “Imagine the Possibilities”. The conference took place in Atlanta, Georgia from 24 to 28 July, and marked the 20th Anniversary National Patient Summit for the US HAEA chapter. The five-day event included parallel programs for all ages including a Youth Leadership Program, the kids’ Brady Club, the US HAEA Patient Program, a special jointly sponsored HAEA/HAE International Global Youth Advocacy Workshop and the Summit’s first Professional-Scientific Program.

The 20th Anniversary Summit tragically came following the loss of two HAE advocates: former HAEA Chief Operating Officer Pam King and founding Board member Chris Whalen. In addition to recognizing their years of service and legacy at the Summit, the US HAEA honored their memory by dedicating two programs in their name: the Pam King HAEA Scholarship Program and the Chris Whalen HAEA Compassion Fund.

2019 HAE GLOBAL YOUTH ADVOCACY WORKSHOP

This innovative program provided the HAE youth with the basic skills required to advocate for themselves and their HAE community. The workshop provided an intense two-day immersive learning experience that includes lectures, hands-on activities, and fun group interactions. Participants enjoyed learning

from case studies and interacting with leading HAE advocates from around the world including Henrik Balle Boysen from Denmark, Natasa Angjeleska from North Macedonia, Michal Rutkowski from Poland, Fiona Wardman from Australia, John Williamson from the United States of America, Fernanda de Oliveira Martins from Brazil plus Rikke Sørensen and Ole Frølich from Denmark.



PROFESSIONAL-SCIENTIFIC PROGRAM

The US HAEA first Professional-Scientific Program provided physicians, scientists, and other health care professionals with an opportunity to hear from leading experts on the latest thinking regarding HAE diagnosis, classification, and treatment. The meeting also featured specialized panels covering four key topics: HAE in Children and Pregnant/Lactating Women; Novel Therapies: Shift Toward Greater Use of Prophylaxis; A Clinical Approach to Patients with HAE with Normal C1-Inhibitor; and the HAE Registries.



PATIENT PROGRAM

The US HAEA full-day patient program provided valuable information for patients on current, and future treatments for HAE, as well as discussions regarding the important role patients play as HAE advocates. Keynote speaker, former Representative Henry Waxman, who is the father of the US Orphan Drug Act, discussed the continuing importance of that legislation and shared his perspective on the power of patient advocacy and engagement in the legislative process. Dane Christiansen, Executive Vice President of the Health and Medicine Counsel of Washington, offered participants with an overview of the US HAEA legislative advocacy legacy as well as the next steps in this effort.



BRADY CLUB

The youngest members of the US HAEA family participated in a fun-filled day as part of the Brady Club. This unique program offers kids an opportunity to learn more about HAE in an interactive environment. Club members elated Summit goes with a performance at the General Session and celebrated a successful day by having an Imagine Your Bear Workshop Party.

YOUTH LEADERSHIP PROGRAM

HAE youth and their siblings received training on how to become a successful storyteller and use this skill as a tool for advocacy. Activities included a visit to the Center for Civil and Human Rights, a hands-on workshop hosted by storytelling experts, and a series of media-making training on storytelling through filmmaking, photography, visual arts, and improvisation.

HAE IN-MOTION

The Summit concluded with an HAE In-Motion 5k walk/run at Piedmont Park in Atlanta. The event helped raise awareness on HAE and create a greater sense of community and camaraderie among summit participants.





UNDERSTAND IT
Understanding a problem is the first step to finding a solution.





2019 GLOBAL HAE YOUTH ADVOCACY WORKSHOP IN ATLANTA

UNDERSTAND IT, PLAN IT AND DO IT

A few days before the HAEA National Patient Summit in July, around 70 members of the HAE International Youngster's Community travelled to Atlanta in the United States of America to participate in the 2019 Global HAE Youth Advocacy Workshop. Here they would not only dive into the world of advocacy but also have the opportunity to reunite with old friends and make new ones.

After a long journey for many of the attendees, the youngsters were delighted to be greeted by a dinner full of typical Southern United States foods. After reigniting friendships over dinner, they had the opportunity to play games and meet members of the community before the workshop started in full the following day.

Day one kicked off with an introduction from Lisa Facciolla, the Workshop and HAEA Youth Programs Leader. She was excited for what was in store over the next couple of days for the HAE International Youngster's Community and was thrilled to provide them with a taste of what to expect. Her enthusiasm spread through the audience and by the end of the introduction the room was buzzing with excitement and anticipation for what was to come.

It was then the turn of HAE International and HAEA President/CEO Anthony J. Castaldo and HAE International Executive Vice President/COO Henrik Balle Boysen to give a special message to the youngsters. They talked through the history of HAE International to show the youngsters what was possible when a group of motivated individuals came together with a common goal, highlighting the impact advocacy can have when 'no' is not accepted as an answer.

The following workshop was split into three sections over the two days: "Understand It", "Plan It" and "Do It" – three vital stages to any advocacy campaign and this format helped the youngsters appreciate and follow the processes involved. First, it was important to explain the concept of advocacy and help the youngsters understand the role it had played in their lives up to this point as well as being the reason they were all in the room together.

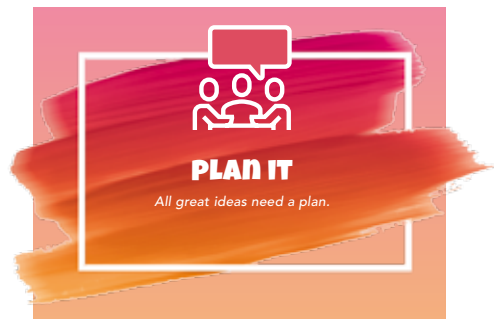
The attendees were then divided into groups of mixed age and nationalities to plan an advocacy campaign and deliver a presentation about their project to the rest of the group at the end of the workshop. Each group was led by a facilitator, including members of the HAE International Board, the Regional Patient Advocate network and the HAEA Patient Advocates, who shared their advocacy experience with the youngsters to help them understand how to apply what they were learning to real-world situations.

As they moved into the "Plan It" section of the workshop, the HAE International Youngster's Community got to hear a real-world advocacy case study from one of the Regional Patient Advocate facilitators and learn about some of the challenges regularly faced by the people working to improve the lives of HAE patients. It gave them an understanding of the obstacles they could face and the determination that members had shown to get the community to where it is today. Inspired by what they had heard, they moved back into their groups to continue with their projects for the rest of day one.

The start of day two began with the "Do It" section and it kicked off with further presentations from the facilitators to show the range of advocacy activities that Youngsters could involve themselves in. The participants also heard from two more HAE International staff members Communication Design and Graphics Manager Rikke Sørensen and Enterprise Technology Manager Ole Frölich about different ways to create and present content.

After putting the finishing touches on their presentations, it was time to see how the youngsters had used their learnings from the workshop as they presented their advocacy campaigns to the rest of the workshop attendees.

From the moment the presentations started, everyone in the room could feel the workshop had been a success. As the groups took turns to present their eye-catching campaigns it was clear the youngsters had taken on board the expert advice that had been shared with them over the last two days. No group had the same





idea; each one was unique and reflected the individual personalities in the groups. There was a wide variety of projects: ranging from a fun-packed disease awareness and fundraising HAE carnival; to campaigns to help people with the mental burden of living with the condition; to specific HAE training courses for dentists. It was special for everyone present to see that number of young people speaking passionately about advocacy and working together to overcome common obstacles and it was clear the HAE International Youngster's Community had learnt a lot.

After the presentations, to wrap up the workshop and celebrate what had been achieved, the youngsters and facilitators headed to SunTrust Park, home of the baseball team the Atlanta Braves, for a tour of the stadium and to learn about the history of Major League Baseball's longest-running franchise.

The day after the workshop the youngsters joined other HAEA youngsters to participate in the Young Leaders Program part of the HAEA Summit. They attended the lively Summit opening before visiting The National Center for Civil and Human Rights to learn how advocacy had positively changed the world for so many people. Afterwards, there was a workshop with the wonderful creatives from Re:Imagine/ATL, that gave the HAE International Youngster's Community the

chance to sample different and bold ways to tell their story, such as theatrical improvisation and portrait photography.

The members of the HAE International Youngster's Community who attended the workshop learnt an incredible amount; 100 percent of those who responded to the feedback survey said the workshop improved their understanding of advocacy either a lot or a little. As well as advocacy, they hopefully also they learnt about themselves and the wider HAE community. Special thanks must be given to the HAE International and HAEA team who made it all possible, including Nevena Tsutsumanova, HAE International Operations Manager and Youngster's Community Secretariat, who helped to plan the workshop but was unable to attend in person.

HAE International want to provide the Youngster's Community with the tools to develop into the next generation of patient advocates who will work to create an easier life for all HAE patients. This is achieved by providing them with the correct knowledge and empowering them to have the confidence to showcase all their wonderful individual talents.

HAEI YOUNGSTERS' COMMUNITY

You can follow all the Youngsters' Community's activities via their blog at haei.org/youngsters.

If you're a young person living with HAE and are interested in joining the HAEi Youngsters' Community or want to share your own inspiring story get in contact using Facebook, Instagram or email.



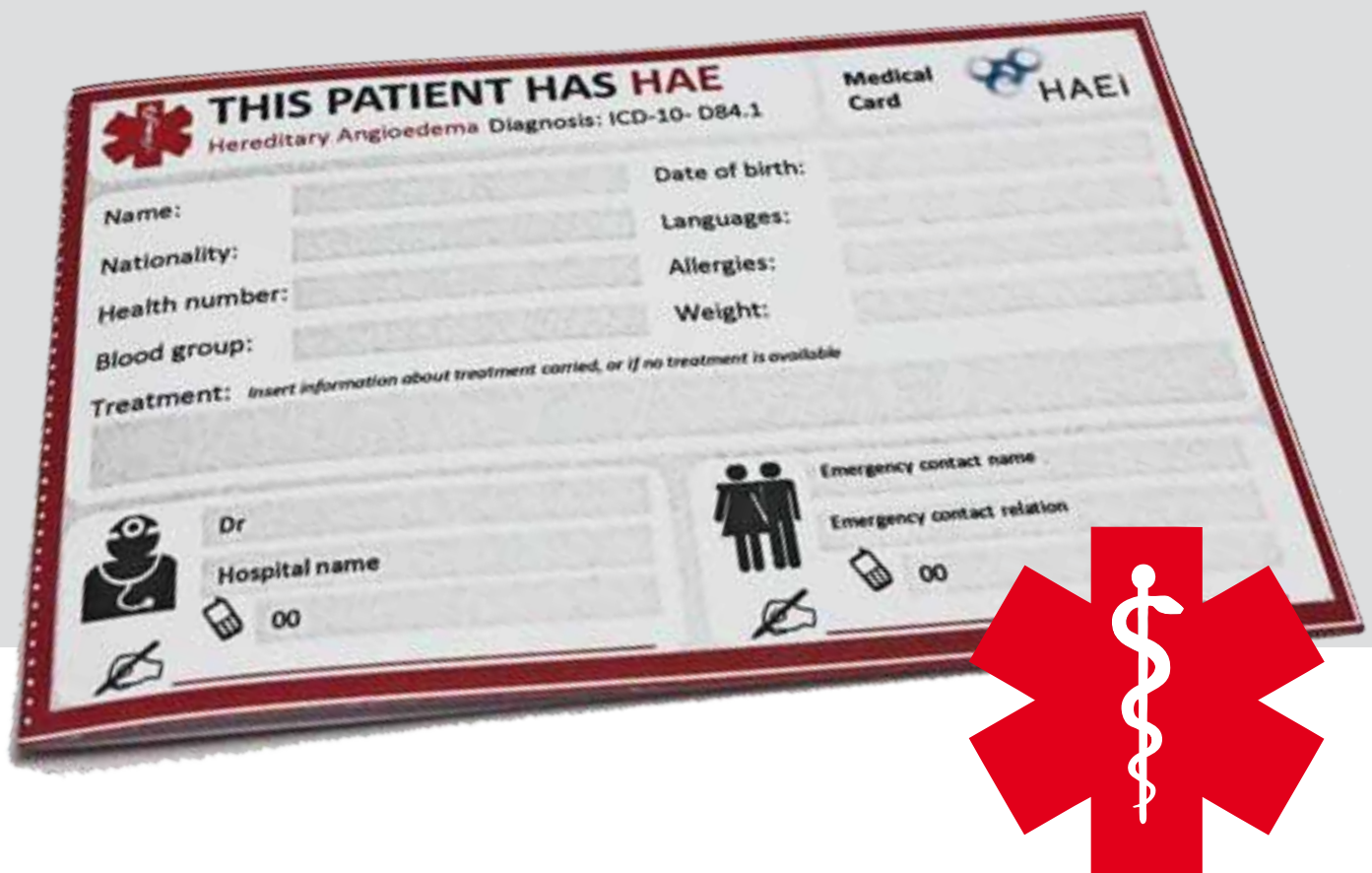
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EMERGENCY CARD NOW IN 28 LANGUAGES

The HAE International Regional Patient Advocates have developed a template Emergency Card with clear and simple information about HAE and treatment required during an attack. It also contains space for patients to add personal information such as emergency contact details and their specialist treatment center.

At this point, the Emergency Card has been translated into 28 languages and is being used by HAE advocacy organizations around the world.

“Each card is designed to be printed at home, cut out and folded in half to create a format that can fit within a wallet or purse. It is designed to be used by HAE patients from all countries and can be adapted by any HAE International member organization. If you are a member organization and have designed your own emergency card that could be displayed on the HAEI website, or if you would like help from us in adapting a card, please contact me”, says Chief Specialist Projects and Research Deborah Corcoran:

“If you are not a patient or part of an HAE International member organization and would like to use one of the cards, please contact the Regional Patient Advocate for your area and inform him or her that you plan to use the card.”

Currently, the Emergency Card exists in Albanian, Arabic, Armenian, Belarusian, Bosnian, Bulgarian, Chinese, Croatian, Dutch, English, French, Georgian, Greek, Hebrew, Hungarian, Italian, Kazakh, Macedonian, Montenegrin, Polish, Portuguese, Romanian, Russian, Slovakian, Slovenian, Spanish, Turkish, and Ukrainian.

Deborah Corcoran:
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Regional Patient Advocates:
<https://haei.org/about-haei/meet-the-rpas>



MARCO CICARDI IN MEMORIAM

August 2019 saw the untimely passing of Professor Marco Cicardi – an innovator, a true giant and in the medical and scientific HAE circles the driving force behind setting up HAE International.

“All of us, as patients and caregivers, have directly benefited from Professor Cicardi’s lifelong dedication to the HAE community. We bid farewell with a deep sense of loss, but will never forget this brilliant, dedicated, and incredibly kind visionary. We honor the memory of a special man who spent his life conducting research and providing clinical care that has dramatically improved HAE patient quality of life”, says the HAE International President & CEO Anthony J. Castaldo.

Born in 1950, Marco Cicardi completed his medical degree in his home country Italy and then trained as a fellow at Milan University with Professor Agostoni. In the 1960’s he went to the USA to join Dr. Virginia Donaldson in the Children’s Hospital University of Cincinnati as a research fellow and later on he worked at the Boston Children’s Harvard University Medical Center with Drs. Fred Rosen and Alvin Davis.

Professor Cicardi showed a specific interest in Complement System diseases with angioedema due to C1 inhibitor deficiency as his primary field of research. Among his research achievements are the definition of the molecular mechanisms leading to the disease and the role of the contact system activation and bradykinin formation. Over the last five decades Professor Cicardi contributed immensely to clinical trials that led to the development of new therapeutics for HAE; identification and validation of disease biomarkers;

investigating the mechanisms leading to acquired C1 inhibitor deficiency and idiopathic systemic capillary leak syndrome (Clarkson Disease).

With his radiating and amiable personality, Marco Cicardi was an incredible leader of world experts. He organized professional meetings to provide international treatment guidelines for HAE and more recently pioneered the classification of new kinds of angioedema with normal C1-INH. Furthermore, he motivated and was the driving force behind the establishment of a global registry for HAE.

Until his sudden death Marco Cicardi was a Professor of Internal Medicine and Head of the complement and research laboratory at the University of Milan, Chairman of the department of biomedical and clinical sciences at Luigi Sacco and ASST Fatebenefratelli medical centers, President of the Medical Advisory Board of the Italian Association for Angioedema; Co-Chairman of the Medical Panel of HAE International as well as Chairman of the recently established HAE Global Registry.

Professor Cicardi contributed more than 150 publications in high esteemed peer-reviewed journals, was regarded as an international leader in the field of angioedema and followed more than 600 patients with this disease.

40 Years of C1-INH

– highlighting CSL Behring's promise to HAE patients

By Faraz Kermani, Sr. Manager European Commercial Communications, CSL Behring

A strong focus on patients has been at the core of CSL Behring's work since its establishment over 100 years ago. We strive each day to deliver on our promise to patients, with a view to making their lives, and that of family and friends who support them, more normal and more fulfilled. This maxim is applied to all the disease areas in which we work, including HAE.

To raise the level of disease awareness about HAE, CSL Behring had the pleasure of hosting a large number of patient representatives and key opinion leaders at its symposium to mark 40 years of C1-inhibitor (C1-INH) therapy, a key therapy option to the treatment of HAE. The event took place at CSL Behring's facilities in Marburg, Germany, with speakers and delegates outlining and discussing the evolution of therapy in the field of HAE and the value that C1-INH has brought to lives of patients.

Professor Konrad Bork, who has conducted some of the most valuable research in this disease area to date, set the stage. He detailed the history of the past 40 years of C1-INH, highlighting his work on the first prophylactic use of C1-INH in HAE. He outlined significant developments in the story of HAE, from its first description as a swelling disorder, by Quincke in 1882, to the results presented from the COMPACT OLE study, which demonstrated the long-term preventative effects of subcutaneous C1-INH with over two years of therapy in 2019.

We were also extremely pleased to welcome President & CEO Anthony J. Castaldo and Executive Vice President & COO Henrik Balle Boysen from HAE International. Henrik detailed the gradual increase in access to C1-INH across the globe and highlighted CSL Behring's ongoing commitment to our HAE patients. This promise is reflected in the establishment of a new production facility for the therapy at our Marburg site. Tony spoke

of his personal experiences with HAE and the impact it has had on his family, particularly his daughter. He praised the continued investment by pharmaceutical companies, including CSL Behring, in research and development in the area of HAE. He noted that patients understood fully the complexity of bringing C1-INH to market and were therefore fully appreciated of the efforts undertaken by the therapy's manufacturers.

CSL Behring's Dirk Hoheisel, General Manager Germany, Austria and Emerging Europe noted that the HAE community was a small but highly committed and energetic group of stakeholders who have established a global support network that is very good. He praised the work undertaken by patient groups in the HAE space in raising disease awareness and stressed that collaboration amongst patients, industry and healthcare professionals must continue to blossom if further improvements to the lives of HAE sufferers are to be realized effectively.

Professor Paul Keith from McMaster University in Hamilton, Canada, highlighted the prevalence of HAE in Canada, pointing out that it seemed to affect women more than men. The use of an antigen assay in detecting levels of C4 in the blood, which could be an indicator of HAE, was the first major step on the diagnostic route, he explained.

The Mayor of the city of Marburg, Thomas Spies, agreed that pharmaceutical companies had made great strides in addressing rare diseases, highlighting the role that CSL Behring has in the city. He added that Marburg as a city would play its part in boosting the production of C1-INH to treat even more people suffering from HAE. He also lauded the work of patient representatives, without whom knowledge of the condition would remain relatively unknown.



The German federal government was keen to address the issue of rare diseases in general, said Michel Meister, Parliamentary Secretary of State for Education and Research. He explained that the German federal government was working hard to increase funding in this area. He further told that the government was aware of the significant scientific challenges posed to companies working in this field.

CSL Behring's General Manager for Europe, Lutz Bonacker, highlighted the complexities of working with plasma, meanwhile underscoring the company's commitment to its HAE patients. He stressed that it was vital to anticipate patients' needs, as human plasma is a finite substance, with limited availability. He furthermore explained how CSL Behring ensures reliable supply by having one of the largest and most efficient networks of plasma collection centers worldwide combined with an excellent manufacturing network.



At the symposium, a suitable tribute was made to a giant in HAE research and treatment, Professor Marco Cicardi, whose passing in August was a blow to the HAE community. CSL Behring's Senior Director Georg Henkel noted that his work was critical to improving patient lives. He also praised Professor Cicardi for committing himself to work in the HAE field at a time when it was neglected and severely underfunded.



The symposium served to highlight the ongoing collaboration between patients, manufacturers and healthcare professionals over the past 40 years. CSL Behring knows this collaboration is essential if new solutions are to be found to improve the lives of HAE sufferers further and feels honored to be working towards achieving this important goal.



FUNDRAISING IN NORTH MACEDONIA

On 30 August 2019 an annual charity party with more than 200 attendees was organized in Skopje, North Macedonia. The party was hosted by Ms. Nora Buklevska and Mr. Petar Kajeovski – two enthusiasts who strive to bring about positive changes and constructive mindset in mundane environment. This year the funds raised were intended for HAE Macedonia.



The team of HAE Macedonia was honored to attend the event, and the President of the organization, Ms. Natasha Jovanovska Popovska gave a short speech focusing on how patients tackle the challenges of having a rare disease in a country where up until 2015 no medications were available. She also spoke about how the fight for access to medications changed HAE patient advocates in North Macedonia for the better, provided them with new skills and how the quality of their lives improved. The team of HAE Macedonia also distributed brochures, mingled and clarified all the questions about this rare disease that attendees had, in an open, cheerful, jubilant and pleasant atmosphere.

These types of parties are not commonplace in North Macedonia so they are all the more important as they set an example of social responsibility not only in terms of dealing with rare diseases but dealing with many burning issues in general.





HAEi Global Access Program

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

https://haei.org/resources/global_access_program



READY TO HOST YOUR WEBSITE



Still more national HAE organizations across the globe are letting HAE International host their websites. The reasons for this are pretty obvious if you ask Enterprise Technology Manager Ole Frølich:

“We have established an advanced system under the HAE International website allowing us to host national websites as well as provide them with templates for an individualized website – naturally all in their native language. And not to forget: This service is for free for all our member organizations. At this point, there are four website templates to choose from – and in the backend, there are powerful editing tools for the website administrator. Furthermore, the HAE International solution supports the organization’s own domain name just as it supports https secure protocol.”

Presently, HAE International is in contact with quite a few national organizations and hopefully we will see the launch of their websites within the next couple of months.

To this date 23 countries have chosen to have HAE International host their website:

- **Australia:** <https://haeaustralasia.org.au>
- **Belarus:** <https://by.haei.org>
- **Brazil:** <https://www.abranghe.org.br>
- **Costa Rica:** <https://costarica.haei.org>
- **Denmark:** <https://haescan.org>
- **Ecuador:** <https://aehecuador.haei.org>
- **El Salvador:** <https://elsalvador.haei.org>
- **Greece:** <https://greece.haei.org>
- **Hungary:** <https://hungary.haei.org>
- **Iceland:** <https://iceland.haei.org>
- **Kenya:** <https://haekenya.haei.org>
- **New Zealand:** <https://haeaustralasia.org.au>
- **North Macedonia:** <https://haemacedonia.haei.org>
- **Norway:** <https://haescan.org>
- **Peru:** <https://peru.haei.org>
- **Poland:** <https://pl.haei.org>
- **Romania:** <https://romania.haei.org>
- **Serbia:** <https://rs.haei.org>
- **South Africa:** <https://southafrica.haei.org>
- **Spain:** <https://angioedema-aedaf.haei.org>
- **Sweden:** <https://haescan.org>
- **Turkey:** <https://turkey.haei.org>
- **Uruguay:** <https://uruguay.haei.org>

According to Ole Frølich, another 10 national websites are in the pipeline, including countries like Bulgaria, Czech Republic, India, and Ireland.

NEWS FROM MEMBER ORGANIZATIONS AROUND THE GLOBE



HONG KONG

4 August 2019, the very first HAE patient meeting – with 44 patients participating – took place in Hong Kong. The meeting led to the founding of HAE Hong Kong and thus the addition of member number 76 to the HAE International family. HAE Hong Kong already has a Facebook group – you can find it at www.facebook.com/haehongkong.



More good news from Hong Kong: Now there is also an HAE knowledgeable hospital on the HAE International map – please see <https://haei.org/location/hospital-hong-kong>. Furthermore, an HAE knowledgeable physician has been added to the map – more information can be found at <https://haei.org/location/physician-hong-kong>.



SWITZERLAND

From President Helene Saam

20th HAE Patient Meeting: On 18 May 2019, the 20th Patient Meeting of HAE Switzerland took place in Bern. Approximately 50 interested HAE patients, relatives, doctors, representatives of the pharmaceutical companies and guests participated at the Novotel Hotel. HAE experts filled the morning with exciting lectures and our guest speaker, the HAE International Executive Vice President & COO Henrik Balle Boysen gave us an insight into the worldwide HAE work. It was very gratifying to hear from him that the future looks good for HAE sufferers. And indeed, a lot of research and various drug studies are running. Our Patient



LEBANON

There is a new HAE point of contact in Lebanon – please see <https://haei.org/hae-member-countries/lebanon>.

Meeting rounded off with a very interesting tour through the production facility of CSL Behring Bern in the afternoon. A big thank you to the two pharma companies for sponsoring our meeting.



Takhzyro in Switzerland: The pharmaceutical company Takeda Pharma AG has on 6 June 2019 received approval for the new drug Takhzyro from SwissMedic and thus this drug is available from September 2019 in Switzerland. Takhzyro can be used in patients over the age of 12 years to prevent HAE attacks. This medicine contains the active substance lanadelumab and is administered subcutaneously as a prophylaxis. Further information on the new drug can be found at www.hae-vereinigung.ch.

Preview next dates: On 8 November 2019, we have the 18th Swiss HAE General Meeting and on 20 June 2020 it is time for the HAE Patient Meeting in Zurich with HAE expert lectures as well as a guided tour of the TV studios of SRFF.

HAE Care Center information: The HAE International map has been updated with fresh information on HAE Care Centers in Switzerland – please see <https://haei.org/hae-member-countries/switzerland>.



RUSSIA

From Chairman Elena Bezbozhnaya

On 19-20 June 2019, HAE Russia held the International Scientific and Practical Conference “Strategy and tactics for treating patients with HAE. Life without fear”.

Organized for the third time, this year the conference received the status of a major international event. The conference gathered highly experienced doctors and members of patient organizations from Belarus, Hungary, Kazakhstan, and Poland as well as patients diagnosed with HAE from 40 regions of the Russian Federation. The best specialists of metropolitan and regional medical centers: allergists, immunologists, genetics, heads of pediatric services, clinical psychologists, representatives of pharmacological companies, and lawyers interacted and shared their experience and knowledge. The conference focused on the routing of patients with HAE, access to essential medicines, correct diagnosis and treatment, disease control and prevention in Russia and abroad.

I opened the Conference, welcomed the participants of the event and turned to the HAE issue in Russia. I noted that many regions fail to supply HAE patients with preferential medicine, and most patients derive no health improvement without contemporary drugs or being provided in insufficient quantities: *“We recognize a difficult situation for patients. People are often seeking legally prescribed drugs for themselves. Officials and many doctors seem to “forget” that the disease is life-threatening, and serious irreversible consequences of inaction shall not be neglected or ignored. We look forward to the federal patient support program. We also promote the idea of creating a genetic screening program for children whose families have relatives with hereditary angioedema. HAE is a hereditary disease, so many families have several patients diagnosed with HAE. The sooner the disease is diagnosed and treated, the better the prognosis for a child, the better the outcome.”*

I then invited the doctor of medical sciences, professor, corresponding member of the Russian Academy of Sciences, Director of the Medical Genetic Scientific Center, Head of the Department of Molecular and Cellular Genetics, Pirogov Russian National Research Medical University, chief freelance specialist in medical genetics of the Ministry of Health of the Russian Federation Sergey Kutsev. Mr Kutsev congratulated the participants with opening of the conference and addressed the meeting of the Committee on Health



Protection of the State Duma of the Russian Federation on rare (orphan) diseases, which, in particular, covered preferential drug provision of patients from the list of 24 nosologies to be transferred from the regional level to the federal one to significantly simplify the process of obtaining pathogenetic drugs for patients with HAE.

The second day of the conference started with the modular block “Routing program as a possible mechanism for HAE patient and doctor interaction” and ended with an open discussion for all participants who got the chance to ask questions to doctors, lawyers and the management of the Society.

Within two days of the conference, young participants – patients with HAE – enjoyed a special program. The psychologist Yuliya Faykova taught children to overcome anxiety states that can provoke edema, using psychological games and guidance.

Summing up the event, the Vice-President of HAE International Michal Rutkowski appreciated the high level of organization of the forum: *“That was a highly interesting and informative conference complemented with focused thematic reports for patients, doctors, scientists, and lawyers. We discussed rather important and sensitive problems and focused on comprehensive solution strategies. We shared our own experience and opinions. The successful experience of one country can be relevant to other countries. Together we become stronger.”*

Dmitry Rogachev National Medical Research Center Of Pediatric Hematology, Oncology and Immunology in Moscow, Russia has been added to the HAE world map – have a look at <https://haei.org/location/care-center-moscow-russia>.



EL SALVADOR

El Salvador has three HAE knowledgeable physicians – please see the HAE International map at <https://haei.org/hae-member-countries/el-salvador>

HAE El Salvador now has a website under the HAEi wings - please see <https://elsalvador.haei.org>.



BELARUS

Two HAE care centers have been added to Belarus on the HAE International map. Scroll down and find them on <https://haei.org/hae-member-countries/belarus>.

Furthermore, Iryna Ausianik is the new President of HAE Belarus.



NETHERLANDS

From President Jan van Leeuwen

The Association for Angio Edema Netherlands is an association with about one hundred and fifty members. We deliberately do not use the name “hereditary” because we represent the hereditary as the acquired angioedema. There are good facilities in the Netherlands; we can always go to university hospitals because the information is stored in the European knowledge center AMC-UVA Amsterdam. In addition, other universities also have specialized knowledge. The biggest bottleneck is with family doctors and smaller hospitals, where little or nothing is known about our condition. We are busy bringing knowledge to those hospitals. Together with the society of family doctors, we have developed a brochure for members to request from the association and hand over to the doctor. It explains how he or she can find all the information on the Internet. Also, with the AMC-UVA Amsterdam, we have developed a special app for the youngsters. They can then chat with fellow sufferers, request information and advice from a nurse or specialist. We also have a special app for all patients where they can keep a diary, download and upload photos, and send everything via e-mail to their specialist to ask for advice. They can also keep their medication list there with use and dosage.



INDIA

HAE India now has a Facebook page – have a look at <https://www.facebook.com/HAEindia>.

The HAE International map has been updated with eight HAE knowledgeable physicians in India - please have a look at <https://haei.org/hae-member-countries/india>.



NORTH MACEDONIA

Two HAE knowledgeable physicians in Skopje, North Macedonia have been added to the HAE International map – please see <https://haei.org/hae-member-countries/north-macedonia>.



FRANCE

A good handful of HAE Care Centers in France have been added to the HAE International map – please see <https://haei.org/hae-member-countries/france>.



DENMARK, NORWAY & SWEDEN

HAE Scandinavia now also has an Instagram account – please have a look at <https://instagram.com/haescandinavia>.

One more Norwegian hospital has been added to the HAE International map – please see <https://haei.org/location/hospital-trondheim-norway>.

Leading HAE experts from Scandinavia will be speaking at the third HAE Scandinavia conference taking place in Oslo, Norway 8-10 November 2019. Among the speakers are:

- Christian Jervelund from Copenhagen Economics – speaking on health and socioeconomic topics underlining the importance of access to the right treatment,
- Dr Linn Landrø from Oslo University Hospital, Norway – speaking on how to prepare children for prophylactic treatment,
- Dr Robert Brudevold from Ålesund Hospital, Norway – focusing on how the daily life of HAE patients has changed as still more have gained access to

- prophylactic treatment,
- Dr Anette Bygum from Odense University Hospital, Denmark – among other topics on the role of the HAE patient in the society and the new possibilities arising,
- The German HAE expert Markus Magerl from the university hospital Charité in Berlin – speaking on the exciting development within new HAE treatments,
- HAE patient Nanna Maria Boysen from Denmark – talking about the work of HAE youngsters globally in order to create a network crossing borders.

Furthermore, the Danish motivational speaker Mark Anthony will inspire the participants and Scandinavian nurses will provide advice and give a mini course in self-administration.

Norrlands universitetssjukhus Umeå in Sweden has been added to the HAE International map as an HAE Care Center – please see <https://haei.org/location/care-center-umea-sweden>.



BRAZIL

24 August 2019 the second Takeda Expert Meeting in HAE took place in Vitória. During the event, doctors interested in HAE from all over Brazil and HAE specialists from several reference centers discussed topics related to the disease. This expert meeting aimed to raise awareness and train doctors on HAE.



The HAE International map has been updated with fresh information on 17 HAE Care Centers, two hospitals and a large number of HAE knowledgeable physicians in Brazil - please see <https://haei.org/hae-member-countries/brazil>.

At the Brazilian Allergy Congress end September 2019, ABRANGHE presented the book “Doctor, I have HAE”.





CYPRUS

The HAE International map has been updated with three HAE knowledgeable physicians in Cyprus – please see <https://haei.org/hae-member-countries/cyprus>.



UKRAINE

One more HAE knowledgeable hospital has been added to the HAE International map, this time in Lviv, Ukraine – please see <https://haei.org/location/hospital-lviv-ukraine>.



KAZAKHSTAN

An HAE knowledgeable physician in Astana, Kazakhstan has been added to the HAE International map. Further information can be found at <https://haei.org/location/physician-astana-kazakhstan>.



LIBYA

The first HAE knowledgeable physician in Tripoli, Libya has been added to the HAE International map – please see <https://haei.org/location/physician-tripoli-libya>.

If you are interested in following HAE Libya you should have a look at the organization's Facebook page at <https://www.facebook.com/HAE-Libya-106027060798335>

HAE Libya also has a Facebook group – please see <https://www.facebook.com/groups/504807226972545>



MONTENEGRO

An HAE knowledgeable hospital in Montenegro has been added to the HAE International map – have a look at <https://haei.org/location/hospital-podgorica-montenegro>.



SLOVAKIA

One more Slovakian HAE Care Center has been added to the HAE International map – please see <https://haei.org/location/care-center-kosice-slovakia>



FINLAND

Four HAE Care Centers have been added to the HAE International map – and there is a new President: Tiina Kortesmäki. See more at <https://haei.org/hae-member-countries/finland>.



CZECH REPUBLIC

The HAE International world map has been updated with four HAE knowledgeable hospitals in the Czech Republic – have a look at <https://haei.org/hae-member-countries/czech-republic>.



SPAIN

From President Sarah Smith

In Memoriam Professor Marco Cicardi: On Sunday 15 September, AEDAF attended the “Hereditary Angioedema” Lunch Seminar of the 17th European Meeting on Complement in Human Disease, held in Madrid 14-17 September, to which Professor Marco Cicardi had been invited to speak on “New treatments for HAE”. Because of Prof. Cicardi’s unexpected death in August, the session was reorganized and dedicated to his memory. Margarita Lopez Trascasa, who had originally invited Prof. Cicardi to speak at the seminar, paid tribute to his career and his contributions to HAE in her presentation “In memoriam Marco Cicardi”. Teresa Caballero presented “Current therapeutic approaches to hereditary angioedema” and Christian Drouet talked about the “C1 inhibitor gene: impact of a high number of variants on HAE and its 3D-structure”. All three expressed their deep sorrow at Marco’s passing and at the loss of a good friend and exceptional colleague, emphasizing the lasting legacy he has left as a brilliant physician and scientist and his dedication to patients.

first European Group in HAE. This early initiative led to the first C1 Inhibitor Deficiency Workshop in Hungary, which took place in Visegrad in May 1999. In a meeting almost without precedent, patient representatives were invited to that workshop, to come together with physicians, researchers and industry representatives in a joint effort to advocate for advances in HAE diagnosis, treatment and research. Then, in the wake of a poorly designed C1 inhibitor concentrate trial and in another unprecedented move, Marco Cicardi summoned several patient representatives to the XIX International Complement Workshop in Palermo, Italy in September 2002, where he impressed on us the need to establish a legally recognized international patient organization. Under Marco’s guidance, HAE International was legally founded in 2004. The rest is history.

We will always be grateful for everything Marco Cicardi did for the HAE cause and we will sorely miss him. And I for one will never forget that smile. We will carry on.... but it won’t be the same without him.

Patient Workshop In Zaragoza: AEDAF will be holding its 16th regional patient workshop on Tuesday 29 October 2019 in Zaragoza, in Hotel Vincci Zaragoza Zentro, C/ del Coso 86, 50001 Zaragoza. We hope to have patients and medical professionals attending from around north central Spain, in particular the regions of Aragon, Rioja and Navarra.

Zaragoza (and the region of Aragon) is now represented on the Spanish Study Group on Bradykinin-Induced Angioedema (SGBA, or its initials in Spanish GEAB) by Dr. Lucia Ferrer at the Hospital Clinico Universitario “Lozano Blesa”. She is a welcome addition to the network of HAE physicians and care centers in Spain.

Another new member of GEAB is Dr. Teresa Macías at Hospital Galdakao y Usansolo of Galdakao (Vizcaya).

22nd General Assembly And Annual Meeting: AEDAF will be holding its 22nd General Assembly and Annual Meeting on 18 April 2020.



A Complement conference was a fitting venue for recalling all of Marco’s achievements, as the beginnings of the global HAE movement can really be traced back to another Complement conference, specifically the XVII International Complement Workshop held in Rhodes in October 1998. There Prof. Cicardi met with several experts working in the field of HAE, including Margarita Lopez Trascasa of Spain, to propose the creation of the



CANADA

From President Jacquie Badiou and Daphne Dumbrille from the HAE Canada office.

Summers are always too short and 2019 was no exception. July was particularly eventful for two HAE Canada Youth members, Paige and Makayla, who were fortunate to attend HAEi's Youngsters Summer Camp in Atlanta. Their experience is best heard from their perspectives:



Paige: This July I attended a Youth Advocacy Workshop held in Atlanta, Georgia. This was my second Youngster's Camp and of course it was nice to see everyone again and get to meet all the new faces. It's amazing to see how our little community is growing at a rapid rate. We had the most newcomers at this year's conference, which was fantastic. We were split into groups and got to choose a topic that we as a group thought was important to us. My group chose a brochure for hospital staff to have a more improved Emergency Department experience. Collectively we put together a presentation about our idea to share with the rest of the Youngsters. Everyone's ideas were very inspiring to listen to. I have

aged out this year so hopefully other Canadian youth will apply to attend next time round. Many of us keep in touch since becoming good friends through this outstanding family.



Makayla: I was not feeling the best when I got to Atlanta, but I was so excited to see everyone I tried my best to push through. I love being able to see all of the familiar faces and getting to meet new people my age. I am an introverted person so talking to a lot of people makes me nervous. That being said, sitting in a room full of people my own age that are also patients and caregivers, not just adults all older than me, is calming in a way. Not needing to explain yourself to everyone, knowing they all understand; it is indescribable. I am more than grateful I was chosen to be able to attend this workshop. Being able to go to these events brings the community together more and for youth like me they make the world a little less lonely knowing there are people out there going through the same things as I do. I hope that I get to attend the next conference at

the HAE Global Conference next year to see everyone again. Thank you HAEi and HAE Canada for this opportunity.

Thank you, Paige and Makayla, for representing Canada so well at the fantastic HAEi Youngster's Camp; HAE Canada is very proud of our youth members.

HAE Canada is pleased to announce the completion of the patient submission for the Common Drug Review (CDR) for the Canadian Agency for Drugs and Technology and Health (CADTH) in support of the new treatment, Takhzyro. The submission, which HAE Canada is hoping will help ensure this new treatment becomes available to all Canadians, is now currently under CADTH review. You can find a copy of the "Patient Group Input Submission" at www.cadth.ca/lanadelumab. Our appreciation and gratitude continues to all who contributed to this valuable and important project.

Over the summer, HAE Canada was happy to assist the Canadian HAE Network (CHAEN), an organization of physicians who treat HAE patients, by distributing their Quality of Life survey to our membership. Our Regional Directors enjoyed speaking with their members about the importance of the survey and were pleased at how many members participated in the survey. We would like to thank everyone who took the time to fill out the survey; their invaluable assistance was appreciated. We are excited to share the results once they are made available to us.

In August, the HAE Canada team was honored to attend the 40 Year Anniversary Celebration of C1 Inhibitor Therapy in Marburg, Germany, where they presented our Real World Data posters. HAE Canada is very appreciative of all of the HAE specialists, both globally and in Canada, who gave informative presentations on a wide variety of issues. We would like to extend a special thank you to HAEA's President Tony Castaldo for his very moving talk on the patient perspective. The HAE Canada team enjoyed the tour of the CSL Behring facility where they witnessed the facility staff's pride, knowing they are improving the lives of HAE patients across the globe. HAE Canada wishes to thank the CSL

Behring global staff who organized the plant tour, as well as the Canadian CSL Behring team who treated the HAE Canada team so very well.

While in Ottawa in September, Jacquie Badiou, HAE Canada President, Anne Rowe, Board member and a participant in the patient/physician group since 2003, along with Daphne Dumbrille from the HAE Canada office in Ottawa attended a meeting at the Canadian Blood Services (CBS)'s Ottawa headquarters to discuss the upcoming subcutaneous long-term prophylactics, Haegarda and Takhzyro. Also while in Ottawa, the HAE Canada team was honored to attend the CSL Behring Boardroom dedication in memory of Joseph Andolfatto, who in his life showed compassion and support to Canadian HAE patients. Joseph is missed by all who were lucky to know him.

It was an indeed a busy summer for HAE Canada, and we are excited to continue to support and represent Canada's HAE patients into the lovely fall season. HAE Canada is happy to announce that starting this November, HAE Canada will be holding Patient Events in Ottawa, Calgary and Halifax. Once the details are finalized, updates will be found at www.haecanada.net/category/blog/events.



SLOVENIA

The HAE International map has another big national update – this time seven HAE knowledgeable hospitals in Slovenia. Have a look at <https://haei.org/hae-member-countries/slovenia>.



ARGENTINA

Please have a look at Argentina on the HAEi map - more than 15 HAE knowledgeable physicians have been added: <https://haei.org/hae-member-countries/argentina>.



COSTA RICA

Costa Rica has also opened a website under the HAEi umbrella - please visit <https://costarica.haei.org>.

An HAE knowledgeable hospital has been added to the map of Costa Rica - please see <https://haei.org/location/hospital-san-jose-costa-rica>.



PARAGUAY

Mid September 2019 HAE International could welcome Paraguay as the 77th member country of the global HAE organization. National contact is Nathalia Portillo – see more at <https://haei.org/location/hae-paraguay>.



TUNISIA

HAE International welcomes country no. 78 to the global HAE family: Tunisia. Please see <https://haei.org/location/hae-tunisia>.



AUSTRALIA AND NEW ZEALAND

From CEO Fiona Wardman

HAE Australasia has met with the Pharmaceutical Benefits Advisory Committee (PBAC) to provide consumer comments on the funding of one of the new prophylaxis therapies. The presentation to the committee and comments were well received by the panel; however, unfortunately, the government at this time rejected the results of the submission. The panel have recognized that there is a need for this therapy and would like to make it available to as many patients as possible. More data is needed on things such as quality of life before the PBAC can reconsider the therapy.

HAE Australasia have also met with the National Blood Authority (NBA) to discuss the status of the submissions for other therapies.

We had the opportunity to present to Healthcare Professionals during the HAE Takeda Forum in Sydney on 'What matters to patients'. The feedback from the HCPs was that the presentation was very well received.

HAE Australasia is working with Professor Connie Katelaris on gaining ethics approval for the HAEi Global Registry.

More patients have been added to the Australasian member database in the past few months.

The HAE Australasia Board held their Annual General Meeting; all board members have been re-elected. A date will be set for the next strategy meeting to work on plans, projects and events for 2020.



USA

From Ianice Viel, Patient Advocate and Social Media Manager



Nico's Lunchbox Book: The US HAEA Engagement team continues to work hard to make sure our youngest patients receive support in a way that keeps them engaged and entertained. This year, the US HAEA chapter teamed up with children's author Caryn Sonberg Seiler to develop a three-book series to help kids learn about HAE in a relatable way. The first book, scheduled to be released this fall, is an introduction to dealing with the condition

for kids with HAE or their siblings. The book offers quick definitions for tougher words, giving kids a valuable tool to better understand HAE.



October Youth Advocacy Month #BeyondHAE: We are excited to celebrate the second annual HAE Youth Advocacy month. In celebration, the US HAEA launched #BeyondHAE, a social media awareness campaign where young advocates share pictures and testimonials of what their lives are like beyond HAE. The campaign aims to highlight the tenacity and courage of our youth advocate while raising awareness and motivating new youth advocates.



HAE In-Motion Fall 2019 Events: This year, the US HAEA has celebrated four exciting HAE In-Motion events in the cities of Wheaton, Atlanta, Memphis, and Fairfax. These events allow patients, caregivers, friends, families, and the HAE community to come together, share stories, and support one another. We will wrap up the excitement-filled 2019 HAE In-Motion series with two incredible upcoming events in the cities of Portland and Providence. These fun family events help raise awareness within local communities, and generate funds for our HAEA patient support programs: the Pam King HAEA Scholarship Program, the Chris Whalen Compassion Fund, and the HAE Research for a Cure.



PERU

One more HAE Care Center has been added to the map of Peru: Hospital Nacional Guillermo Almenara. Please see <https://haei.org/location/care-center-lima-peru>.



UNITED KINGDOM

From CEO Laura Szutowicz and Executive Officer Rachel Annals

HAE UK has had a busy summer with some great events thanks to our amazing members.



Huge thanks to Ann Harding, one of our Trustees, for organizing the Family Fun Day at the Railway Hotel, West Horndon. June and Peter Cole kindly came along to help, and Rachel and Laura were kept firmly under control by Miss Darcy Annals. This was a perfect venue with plenty of car parking and a safe enclosed garden where we were able to set up the bouncy castles and other activities, garden games, crafts and also included a very charming pony who cheerfully gave rides to all the children.

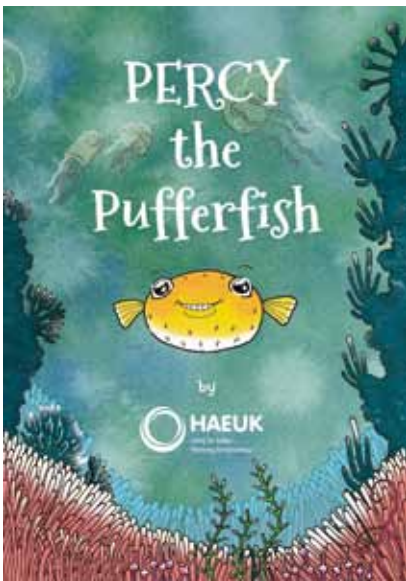


We were a little worried by the weather initially as rain seemed threatened but actually we were lucky to have a glorious afternoon. The pub garden was packed, the face-painting lady was unable to take a tea break and the bouncy castles saw great use. Declan, Ann's partner, was the super-chef in charge of the barbecue and spent all day producing delicious food, we had some fabulous prizes donated for the raffle and a great time was had by all. It was lovely to see how quickly the children got along with each other even though they had not met, and the 'regulars' in the pub all quickly got involved, very generously buying raffle tickets (they were all after the box at a West Ham football match) and lots of people enquiring who we were and about HAE, so as well as a fun day we hopefully were able to raise some awareness of the condition.

Our next great event was the Young Advocates meeting at Drayton Manor, with 14 attendees. Alex, Jack and Rhiannon had been to the training meeting in Atlanta, USA and they shared their knowledge with other members at the first of our Youngsters only meetings. Our three youngsters had a fantastic time in Atlanta working with other HAE youngsters from across the world, making new friends and supporting one another. They returned with lots of ideas on how to advocate which they will use to help develop the growing youngsters team in the UK.



As well as learning about advocacy they brainstormed more projects that they can work on through the coming year to raise awareness. They then were able to spend the time in the Theme park, which was kindly sponsored by some of our amazing fundraisers.



At the HAE UK patient day in November 2018, the youngsters who were participating in the event decided to create a story, which they titled 'Percy the Pufferfish'. The organization recently commissioned an illustrator to help turn the story into a book, and it has now been printed. The book is a simple story about HAE aimed at younger patients, and it will be available at our two Patient Days.

We have two more events coming up soon: On 2 November 2019 members will be meeting in Glasgow, Scotland for the 4th Scottish patient day – and 23 November 2019 we will be holding the 9th annual patient event, this time in Manchester. As always, we are having a little reception the night before so that people can get a chance to meet up with old and new friends. More details, including registration, are available on www.haeuk.org.

We are very excited that Lanadelumab was passed by the National Institute for Health and Care Excellence so that, assuming there are no substantiated objections, it will soon be available to be prescribed under NHS England guidelines. Effectively, this will place it in the same area as C1-INH prophylaxis, so for patients having two or more clinically significant attacks per week. Rachel and Laura were involved all the way through the process and it was pleasing to have the NHS England team very complimentary about the evidence we provided, much of which was taken from our patient surveys, interviews and discussions at the Patient Days.



HAE UK has a new logo to reflect our continuous working together with patients, medical professionals and others to grow our organization for the benefit of HAE patients. We will gradually change our literature and so on as we run out.



EGYPT

Ahmad Lofty is the new National contact in Egypt – please see haei.org/hae-member-countries/egypt.



SOUTH AFRICA

From President Adrienne de Jongh

We are in the process of registering a nonprofit company so that we can in future access funding and consolidate the organization. We have increased our register of patients to 90.

In July Hana Faulds attended the youth camp in Atlanta and learnt lots about advocacy, which we intend to incorporate in future plans. Here is Hana's report:

The HAE Youth Summit took place in July this year, with more than 100 HAE youth members from around the world. The Summit was run over five days, with a full itinerary involving unique, educational and fun activities for each day. Most activities were workshop based with the aim of equipping youth with essential advocacy tools.



The workshops involved teambuilding activities where members were tasked to create a unique advocacy project and challenged to think outside the box when it comes to raising awareness for HAE. This encouraged younger generations to take control of their HAE by spreading awareness and contributing to the global



HAE community. Additionally, we had courses, lead by professionals in their field, on advocacy resources and creative ways to spread awareness effectively: this involved photography and film classes, art, storytelling, social media tools and much more.

In between educational workshops, we had time to explore Atlanta, with a private tour of the famous Braves baseball stadium, a visit to the National Center for Civil and Human Rights, ending off with a scenic 5km run through Piedmont Park to raise awareness with HAEA.

This year was the first time HAE South Africa had a representative attending the youth summit. We, as a South African community, are extremely grateful for the opportunity to learn from our global HAE family and connect our youth globally. We would like to thank HAEA and HAE International, as well as everyone involved, for paving the way forward for all HAE patients. We look forward to growing our HAE South Africa youth community.

STILL MORE COUNTRIES USING HAEi CONNECT



In 2018 HAE International presented HAEi Connect – a cloud-based member database for national organizations to manage their members. Due to popular demand, this free-of-charge offer was updated earlier this year with a number of highly useful features. Enterprise Technology Manager Ole Frølich explains:

“First and foremost, HAEi Connect includes an easy to use editor for creating emails to members as well as a template for saving and re-using emails. Another addition to the system is the possibility of contacting members via text message. Other updated features are four new sections in the HAEi Connect dashboard to initiate contact to members as well as right-to-left language support.”

To date, HAEi Connect has been implemented in Australia, Bulgaria, Denmark, Hong Kong, India, Israel, Kenya, Mexico, New Zealand, North Macedonia, Norway, Peru, Poland, Slovenia, South Africa, Spain, Sweden, Turkey, and the United Kingdom.

“Furthermore, we are talking to more than 13 national organizations so it is my hope that we will soon be able to add countries like Belarus, Brazil, Greece, Hungary, Japan, Romania, Ukraine, and the United States of America”, says Ole Frølich.



NEWS FROM THE INDUSTRY

13 August 2019

BioCryst Pharmaceuticals, Inc. has announced the financial results for the second quarter ended 30 June 2019 and provided a corporate update.

“We have just returned from the 2019 HAEA National Patient Summit with more than 1,000 attendees from the U.S. and around the world. Patients’ excitement about BCX7353 was resounding as they told us an oral option with our safety and efficacy profile could change their lives and the lives of their family members with HAE. Our customers want our product, and we cannot wait to deliver it to them next year,” said Jon Stonehouse, president and CEO of BioCryst.

“We are on-track to submit an NDA to the FDA in the fourth quarter, followed by regulatory submissions in Europe and Japan in the first quarter of 2020. We are also preparing for the commercial launch of BCX7353 in the U.S. later in 2020”, Stonehouse added.

(Source: BioCryst)



13 August 2019

At the presentation of the financial results for the quarter ended 30 June 2019, Kenneth T. Mills, President and CEO of the clinical-stage biotechnology company **REGENXBIO Inc.**, said:

“We recently announced the expansion of our pipeline using NAV Vectors to deliver therapeutic antibodies, beginning with novel treatments for HAE. This approach has enormous potential for patients who lack treatments or who are currently underserved by existing therapies. We currently have an internal pipeline of novel gene therapy candidates across large, chronic diseases and rare, monogenic diseases, which positions us to potentially develop meaningful treatments for a broad spectrum of patients with significant unmet needs.”

(Source: REGENXBIO)



10 September 2019

The clinical-stage company **Pharvaris B.V.** which is focused on the discovery and development of novel oral B2-receptor antagonists for the treatment of HAE, has completed a 66 million USD Series B financing. In addition, the company announces its entry into clinical development with the initiation of a Phase 1 study of its lead compound, PHA121, in healthy volunteers.

“This financing and our outstanding syndicate of investors positions Pharvaris as a clinical leader for the development of oral treatments for patients with HAE,” said Berndt Modig, CEO and co-founder of Pharvaris. “Our experienced team is capitalizing on its deep knowledge of drug development and HAE as we progress in the clinic with PHA121, a new chemical entity targeting the same mechanism as icatibant, a leading therapy for the treatment of HAE attacks. The demands for less invasive routes of drug administration, more convenient dosing regimens, and additional treatment options support the development of an oral therapy to improve the quality of life of patients with HAE.”

The proceeds from the Series B financing will enable Pharvaris to expedite the clinical development of PHA121. The first subjects have been dosed in a Phase 1, randomized, double-blind, placebo-controlled, single-ascending-dose study to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of PHA121 in healthy subjects. A multiple-ascending-dose study is anticipated to start in early 2020. In preclinical studies, PHA121 demonstrates oral bioavailability, selective antagonism of the B2 receptor, and potent and rapid activity in bradykinin-mediated disease models.

Jochen Knolle, Ph.D., Chief Scientific Officer and co-founder of Pharvaris, added, “Patients with HAE are eager for effective oral therapies. The development of a novel, oral B2-receptor antagonist could represent a new standard of care for the treatment and prevention of HAE. This first-in-human Phase 1 study is expected to yield important safety and tolerability data. In addition, we expect to demonstrate pharmacodynamics for blocking bradykinin signaling, which will inform the design of subsequent clinical trials.”

Dr Knolle is an inventor of icatibant and, as Chief Scientific Officer and Head of R&D at Jerini AG, was instrumental in the development and first approval of icatibant for treatment of HAE.

(Source: Pharvaris)

PHARVARIS

10 September 2019

Many people who have rare diseases yearn for a diagnosis – to give their symptoms a name and have a path forward. Too often with rare diseases, medical experts have no treatment to offer. So it was until 1979 for patients who have HAE, an inherited condition that causes dangerous swelling attacks primarily due to missing or low levels of the plasma protein C1 esterase inhibitor (C1-INH). This year, **CSL Behring** celebrates the 40th anniversary of a milestone for HAE patients: That the absent plasma protein could be replaced through C1-INH therapy, reducing the likelihood of attacks.

(Source: CSL Behring)

CSL Behring

18 September 2019

The National Institute for Health and Clinical Excellence (NICE), the health technology appraisal body in England and Wales, has issued its Final Appraisal Determination (FAD) recommending Takhzyro (lanadelumab) subcutaneous injection as an option for preventing recurrent attacks of HAE in patients aged 12 years and older only if: (1) they are eligible for preventive C1-esterase inhibitor (C1-INH) treatment in line with NHS England's commissioning policy, (2) the lowest dosing frequency of lanadelumab is used in line with the summary of product characteristics, and (3) the company provides lanadelumab according to the commercial arrangement.

Laura Szutowicz, CEO of HAE UK said, "The recommendation from NICE means that people living with HAE across England and Wales have another medication that gives them the chance to lead a full and active life. We hope the NHS and Healthcare Professionals will provide swift access to patients who could benefit from this new treatment."

Lanadelumab is the first preventative treatment for Type I/II HAE, self-administered by patients at home as a single subcutaneous injection every two to four weeks. In clinical studies, monthly attack rates were reduced by 87 percent relative to placebo, and patients experienced improvements including less fear and shame about unpredictable attacks, less impairment in their ability to work, socialise, and perform other physical activities and reduced fatigue during the day and a better night's sleep.

Dr Sinisa Savic, Consultant in Clinical Immunology and Allergy, said, "the replacement of frequent intravenous injections with at-home subcutaneous administration every two to four weeks, and the chance of being attack free, means that lanadelumab has the potential to transform care for some patients. It represents a real step-change in the treatment and clinical management of patients who experience recurrent HAE attacks."

Key evidence behind the NICE recommendation for Takhzyro was data from the HELP-03 (Hereditary Angioedema Long-term Prophylaxis) Study. In this 26-week study, with 125 people with HAE, Takhzyro reduced the mean number of monthly HAE attacks by 87 percent compared with placebo when administered at 300 mg every two weeks and 73 percent compared

with placebo when administered at 300 mg every four weeks. A prespecified, exploratory analysis showed that over the entire 26-week study, 44 percent of patients taking Takhzyro 300 mg every two weeks were attack-free vs 2 percent of patients taking placebo. A post-hoc sensitivity analysis showed that 77 percent of the patients receiving Takhzyro 300 mg every two weeks were attack-free during a steady-state vs 3 percent of patients on placebo. The HELP Study is the largest randomised, controlled clinical prevention study conducted to date in this rare disease.

The FAD is part of the final guidance to the NHS in England and Wales expected to be published in October 2019. Pending any appeals from key stakeholders, based on this positive recommendation, the NHS should make Takhzyro available in England within three months of this date.

"Ensuring people living with rare diseases, such as HAE, have the best care and access to innovative treatments is of the utmost importance to us and this recommendation by NICE represents a huge milestone," said Jon Neal, Managing Director, UK and Ireland, **Takeda UK Ltd.** "Takeda is really proud to be able to bring this novel medication to those living with this extremely debilitating condition."

Takhzyro is approved in the United States of America (August 2018), Canada (September 2018), the European Union (November 2018), Australia (January 2019), and Switzerland (June 2019) and additional regulatory submissions are ongoing worldwide.

(Source: Takeda UK Ltd.)



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

Risk factors for diagnostic delay in Chinese patients with HAE – by *S. Liu, Peking Union Medical College Hospital, China et al.:*

An Internet-based survey was sent to 129 patients with type 1 and 2 HAE shows that the median diagnostic delay was 11.04 years. A younger age of onset and earlier decade of onset were predictors of a diagnostic delay of >6 years. Seventy-five percent of the patients reported receiving more than one previous misdiagnosis. The patients with a previous misdiagnosis had longer diagnostic delays compared with patients without a misdiagnosis. (*Allergy Asthma Proc.*, September 2019)

Subcutaneous C1 inhibitor for prevention of attacks of HAE: additional outcomes and subgroup analysis of a placebo-controlled randomized study – by *H.H. Li, Institute for Asthma and Allergy, Chevy Chase, the United States of America, et al.:*

C1-esterase inhibitor (subcutaneous) prophylaxis demonstrated a preventive treatment effect with evidence of benefit within 2 weeks. A consistent treatment effect at recommended C1-esterase inhibitor (subcutaneous) dosing was evident in all subgroups of patients with type I/II HAE and by various measures of disease and treatment burden. (*Allergy Asthma Clin Immunol.*, August 2019)

Recombinant human C1 esterase inhibitor as short-term prophylaxis in patients with HAE – by *A. Valeriewa, Medical University of Sofia, Bulgaria, et al.:*

Limited data are available on recombinant human C1 esterase inhibitor as short-term prophylaxis. A case series of 51 patients (70 procedures) indicated that recombinant human C1 esterase inhibitor short-term prophylaxis administered within several hours before a medical/dental procedure was efficacious and well tolerated. (*J Allergy Clin Immunol Pract.*, August 2019)

Driving towards Precision Medicine for angioedema without wheals – *A.E. Germenis, University of Thessaly, Greece, and M. Cicardi, Università Degli Studi di Milano, Italy:*

Evidence accumulated over the last two decades indicates that recurrent angioedema without wheals constitutes a diverse family of disorders with a much higher complexity than was previously regarded. During the last two years, novel variants of three genes other than SERPING1 and F12 have been identified in association with HAE. Functional studies of at least one of these variants imply the existence of a new disease endotype in which the altered bradykinin metabolism and function does not play a central role. Therefore, using conventional approaches, it seems that the complexity of this disease cannot be sufficiently elucidated and any attempt to interrelate its many diverse aspects seems unrealistic. Similar to other rare and chronic diseases, a Precision Medicine approach, discovering the right target and giving “the right drug, for the right patient, at the right time, every time” seems the optimal future practice. There is a need for a switch of angioedema research into high-throughput approaches. (*J Autoimmun.*, August 2019)

HAE Due to C1-Inhibitor Deficiency in Pediatric Patients in Croatia - First National Study, Diagnostic And Prophylactic Challenges – by *L. Karadža-Lapić, Šibenik General Hospital, Šibenik, Croatia, et al.:*

Patients were recruited during a four-year period at five hospitals in Croatia. Complement testing was performed in patients with a positive family history. This pilot study revealed nine pediatric patients positive for C1-INH-HAE type I, aged 1-16 years, four of them asymptomatic. Before the age of one year, C1-INH levels may be lower than in adults; it is advisable to confirm C1-INH-HAE after the age of one year. Plasma-derived C1-INH is recommended as acute and short-term prophylactic treatment. Recombinant C1-INH and icatibant are licensed for the acute treatment of pediatric patients. In Croatia, HAE is still underdiagnosed in the pediatric population. (*Acta Clin Croat.*, March 2019)

Elderly versus younger patients with HAE type I/II: patient characteristics and safety analysis from the Icatibant Outcome Survey – by A.

Bygum, Odense University Hospital, Denmark, et al.:

We conducted descriptive analyses in younger (age < 65 years) versus elderly patients (age ≥ 65 years). Elderly patients with C1-INH-HAE were significantly older at diagnosis and had greater delay in diagnosis than younger patients. Elderly patients contributed to approximately 10 percent of the icatibant-treated attacks. We found similar adverse events rates (overall and possibly/probably related) in icatibant-treated elderly versus younger patients, despite the fact that elderly patients had significantly more comorbidities and were receiving a greater number of concomitant medications. (*Clin Transl Allergy*, July 2019)

Lanadelumab to treat HAE – by B. Wedi, Hannover Medical School, Germany:

There is a clear need for a non-plasma-derived, safe, effective and convenient prophylaxis of HAE attacks to reduce patients' daily burden of disease and disability. The percentage of patients who were attack-free for the last 16 weeks of a controlled study was 77 percent in the group receiving 300 mg lanadelumab every two weeks, compared with 3 percent with placebo. The most common side effects were mild injection-site reactions. Lanadelumab has the potential to change the approach from on-demand treatment to prophylaxis in HAE. (*Drugs Today (Barc)*, July 2019)

Successful Long-Term Prophylactic Treatment With Subcutaneous C1 Esterase Inhibitor in a Patient With HAE – by J. Hahn, University Medical Center Ulm, Germany, et al.:

Self-administered SC prophylactic use of C1-INH over a period of 16 months seems to be a well tolerated and efficient. The patient's quality of life improved, and by learning self-application, the patient gained independence. (*J Pharm Pract*, June 2019)

Safety of recombinant human C1 esterase inhibitor for HAE attacks during pregnancy –

by D. Moldovan, MediQuest Clinical Research, Romania, et al.:

Limited clinical data are available on HAE treatments during pregnancy. A case series of 14 pregnant women demonstrated that treatment with recombinant human C1 inhibitor was generally safe and well tolerated. (*J Allergy Clin Immunol Pract*, June 2019)

HAE emergency management of attacks by a call center – by N. Javaud, Université Paris, France, et al.:

Use of emergency departments and hospitalizations are reduced by the use of a coordinated national call center in HAE after therapeutic education program that promoted self-administration of specific treatment and use of call to call center. (*Eur J Intern Med*, September 2019)

HAE-Associated Acute Pancreatitis in C1-Inhibitor Deficient and Normal C1-Inhibitor Patients: Case Reports and Literature Review

– by C.L. Veronez, Universidade Federal de São Paulo, Brazil, et al.:

Pancreatitis was efficiently treated in two patients using Icatibant, with pain relief within hours. When conservatively treated, pancreatitis pain took longer time to resolve. Although rare, severe abdominal HAE attacks could cause pancreatitis; HAE-specific treatments may be efficient for HAE-associated pancreatitis. HAE should be considered as a differential diagnosis of acute idiopathic pancreatitis. (*Front Med (Lausanne)*, April 2019)

Assessment on HAE burden of illness in Brazil: A patient perspective – by A. Abdon Barbosa,

Faculdade de Medicina do ABC, et al.:

Patients with HAE report understanding how severe their diagnosis represent, but they did not ponder how important their commitment to treatment may decrease the constant fear brought by the disease in its possible swelling crisis. Family data supported this conclusion. (*Allergy Asthma Proc.*, May 2019)

Recombinant human C1 esterase inhibitor treatment for HAE attacks in children – by A.

Reshef, Barzilai University Hospital, Israel, et al.:

Recombinant human C1-INH was efficacious, safe, and well tolerated in children. Data support use of the same dosing regimen for HAE attacks in children (50 IU/kg; up to 4200 IU, followed by an additional dose, if needed) as is currently recommended for adolescents and adults. (*Pediatr Allergy Immunol.*, August 2019)

A randomized trial of human C1 inhibitor prophylaxis in children with HAE – by E. Aygören-Pürsün,

University Hospital Frankfurt, Germany, et al.:

C1-INH prophylaxis was effective, safe, and well tolerated in children aged 6-11 years experiencing recurrent angioedema attacks. A post hoc analysis indicated a meaningful improvement in health-related quality of life with C1-INH. (*Pediatr Allergy Immunol.*, August 2019)

New monoclonal/bi-specific antibodies: Reshaping transfusion medicine beyond replacement – by H. Goubran,

University of Saskatchewan, Canada, et al.:

Since the first successful transfusion in 1818, Transfusion Medicine has evolved significantly. The advent of plasma fractionation and availability of recombinant products allowed for precision replacement therapy to treat many hematological conditions, such as HAE. A deeper understanding of the pathophysiology underlying those conditions along with the development of safer monoclonal and bispecific antibodies is now offering safe and effective alternatives to the simple conventional approach of replacing a missing plasma protein. Many biologicals are already in wide clinical use in areas such as rheumatology, gastroenterology, and medical oncology. The introduction of novel therapeutic antibodies within the realm of Transfusion Medicine will likely reshape the field and challenge the role of local blood establishments as the gatekeepers of such therapies. (*Transfus Apher Sci.*, April 2019)

Clinical profile and quality of life of Puerto Ricans with hereditary angioedema – by Y.M.

Arce-Ayala, University of Puerto Rico School of Medicine, Puerto Rico, et al.:

Puerto Rican Hispanics showed a similar epidemiologic and clinical profile to previous studies, however; higher frequency of attacks was prominent. There was a substantial and noteworthy decrease in quality of life in HAE patients and an increase risk for depression, particularly among women. (*Allergy Asthma Proc.*, March 2019)



CLINICAL TRIALS

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

A clinical trial to assess 2 different doses of BCX7353 compared to placebo as an oral treatment for the prevention of attacks in people with HAE

– recruiting in Austria, Belgium, Bulgaria, Canada, Czech Republic, Denmark, Germany, Hungary, Ireland, the Netherlands, North Macedonia, and the United States of America

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

– recruiting in Australia, Austria, Denmark, France, Germany, Hong Kong, Hungary, Israel, Italy, Korea, New Zealand, North Macedonia, Poland, Serbia, Slovakia, South Africa, Spain, Switzerland, the United Kingdom, and the United States of America

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

– recruiting in Argentina, Germany, Italy, Mexico, Romania, the United Kingdom, and the United States of America

A Phase 3, randomized, double-blind, placebo-controlled, parallel-group study to evaluate the efficacy and safety of two dose levels of BCX7353 as an oral treatment for the prevention of attacks in subjects with HAE

– recruiting in Japan

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

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

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

– recruiting in Italy

A Study to Investigate CSL312 in Subjects With HAE

– recruiting in Australia, Canada, Germany, Israel, and the United States of America

Biomarker for HAE Disease Type 1 (BioHAE)

– recruiting in Egypt, Georgia, Germany, and India

BCX7353 for the prevention of HAE attacks

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

C1 Inhibitor Registry in the Treatment of HAE Attacks

– recruiting in Bulgaria, Croatia, Czech Republic, France, Germany, Hungary, Italy, North Macedonia, Norway, Poland, Slovakia, Slovenia, and Sweden

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

– recruiting in France



Epidemiological Analysis for HAE Disease (EHA)

– recruiting in Germany, Poland, Turkey, and the United Kingdom

Firazyr General Drug Use-Results Survey (Japan)

– recruiting in Japan

Firazyr Patient Registry (Icatibant Outcome Survey - IOS)

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

Global Registry to Gather Data on Natural History of Patients With HAE Type I and II (HGR)

– recruiting in Italy

Pathophysiological study for autoimmune dysregulation of HAE

– recruiting in Japan

Patient Registry to Evaluate the Real-world Safety of Ruconest

– recruiting in the United States of America

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

– recruiting in Italy

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

– recruiting in the United States of America

Study to Evaluate the Real-world Effectiveness of Lanadelumab in Participants With HAE (EMPOWER)

– recruiting in Canada, Puerto Rico, and the United States of America

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

– recruiting in France

A Safety, Pharmacokinetics (PK), and Pharmacodynamics (PD) Study of Lanadelumab to Prevent HAE Attacks in Pediatric Participants of 2 Years to Less Than (<) 12 Years of Age (SPRING)

– list of countries recruiting has not yet been disclosed

A Study to Assess the Clinical Efficacy of IONIS-PKK-LRx in Participants With HAE

– list of countries recruiting has not yet been disclosed

Read more about these and other clinical trials at <https://clinicaltrials.gov> and <http://apps.who.int/trialsearch>



HAEi AROUND THE GLOBE

Currently there are HAE member organizations in 78 countries. You will find a great deal of vital information on the HAE representations around the globe at haei.org – and the world map will provide you with contact information for the member organizations as well as care centers, hospitals, physicians, and available medication. The information on haei.org is being updated as soon as HAE International receives fresh data from the national member organizations.

