


Global perspectives:

HAEI MAGAZINE · ISSUE 2/2023

 98 Member countries



HAEI
REGIONAL
CONFERENCE
EMEA
MUNICH
1-3 SEP 2023

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Global Perspectives
Issue 2/2023
October 2023

Cover photo

Participants from 61 countries came together in Munich, Germany, for the **2023 HAEi Regional Conference EMEA** – read more on page 18

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HAEi is registered as a non-profit organization in the USA.

HAEi is a global non-profit network of member organizations dedicated to raising awareness of hereditary angioedema (HAE) and improving the lives of people with HAE.

DEAR HAEi FRIENDS AND SUPPORTERS,

Welcome to the second issue of Global Perspectives, HAE International's window into the world of Hereditary Angioedema that includes the latest developments, inspiring stories, and impactful initiatives that define our global HAE community. This edition highlights the community's commitment to making a difference in the lives of those affected by HAE.

First and foremost, we invite you to relive the magic of the **2023 HAEi Regional Conference EMEA**, which took place from 1 to 3 September in Munich, Germany. This inspiring conference brought together 650 of our dearest HAEi friends, united in the quest to "Take Action!" Together we shared knowledge, built connections, and charted a path towards a brighter future for the HAE community. The **2023 HAEi Regional Conference EMEA** was not just a gathering—it was a vivid testament to the commitment, resilience, and shared vision of our global HAE community. The EMEA region, with its diverse cultures, languages, and healthcare frameworks, presented unique challenges. Yet, what stood out was the collective spirit, the shared stories of courage, and the unwavering determination to shape a better tomorrow for everyone impacted by HAE.

In conjunction with the **2023 HAEi Regional Conference EMEA**, HAEi and our US HAEA Member Organization jointly sponsored a scientific symposium dedicated to helping people in our global community who are diagnosed with "HAE with normal C1-INH." We brought together 31 esteemed scientists and researchers from across the globe, who will publish a trailblazing publication that includes the latest recommendations on diagnosing and treating HAE with normal C1-INH. We'll bring you more on this important initiative in a future issue.

Following the successful conference in Munich, the entire team at HAEi are working hard on the preparations for the next big event. We look forward to welcoming HAEi friends from the Americas (North, Central, and South) to the **2024 HAEi Regional Conference Americas**, which will take place on 15 – 17 March 2024 in Panama City, Panama.



We are also delighted to report on the 2023 US HAEA National Summit held in July. The Summit gathered a record-breaking 1,200 attendees including patients, caregivers, healthcare professionals, and industry representatives.

Our Member Organizations and Regional Patient Advocates play an essential role in our global mission, and we are delighted to bring you the latest updates from their tireless efforts. Additionally, we offer news from the future advocates, the HAEi Youngsters Community, and a special update on HAEi LEAP, our educational program that teaches young people new skills to help them develop as individuals and HAE advocates. We are currently recruiting for the 'Class of 2024'.

As always, we remain committed to keeping you informed about innovative clinical trials that are testing the next generation of HAE treatments and the most recent published HAE research.

The global HAE movement is thriving because of your unwavering support and commitment.

Your dedication and enthusiasm continue to drive our shared purpose and fuel the sense of unity that paves the path toward a brighter future for the global HAE community.

Happy reading, and let's keep taking action together!

Warm regards,

A handwritten signature in blue ink that reads "HENRIK". The signature is stylized with a long horizontal line extending from the end.

Henrik Balle Boysen
President, HAE International (HAEi)

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NEWS FROM HAEi'S REGIONAL PATIENT ADVOCATES

HAEi Regional Patient Advocates – get your message out there!

Being a patient advocate is like riding a rollercoaster... you can go up slowly, only to go down so fast just a few seconds later with no prior notice. The action changes like a kaleidoscope and is always coupled with many emotions. It can be fun, but at the same time, it can be challenging and difficult.

Therefore, HAEi has 11 Regional Patient Advocates (RPAs) around the world. They have experience in organizing events either online or in person. They can provide advice and support and are always available to you. RPAs can help make connections with doctors and others with HAE in your area.

We recently experienced the power of RPAs at the **2023 HAEi Regional Conference EMEA**, where we gathered the HAE community from Europe, Middle East, and Africa. Our RPAs were working very hard to make the EMEA conference successful and to have as many attendees as possible. Thank you!

Those RPAs not involved in the EMEA conference focused on their regions' challenges and advocacy activities. But, soon, some of them will be working on the 2024 HAEi Regional Conference Americas. It is amazing to see how hard work pays off and how the HAEi global community grows; it wouldn't be possible without the HAEi decentralized approach.

Please take a moment to review our RPAs update section and learn more about the fantastic job done by our on-the-ground forces, Regional Patient Advocates. I encourage all our Member Organization leads to contact their RPA- if you haven't already- and get your message out there!

Michal Rutkowski

Director, Regional Patient Advocate Program



FERNANDA DE OLIVEIRA MARTINS SOUTH AMERICA AND MEXICO

I am working with many countries in my region to support them in implementing some of HAEi's wonderful tools and services. Chile has recently launched a "web2go" website. This solution is a quick process, free, and helps Member Organizations be more visible, increasing HAE awareness. You can see their website here: <https://aehchile.haei.org/>

We expect that more patients, caregivers, and physicians can now find the Chilean HAE Member Organization more easily.

HAEi has run the Baseline Burden of Illness survey in Mexico. I have provided support throughout the process, including joining the presentation of the results to support with translations. The results are really helpful to have a better understanding of the impact of HAE in Mexican patients and an overview of the treatments in use in the country. The results can now be used to increase awareness of the disease and to show the importance of getting treatment. HAE Mexico plans to share the results at the Latin American Society for Immunodeficiencies (LASID) conference for healthcare professionals.

The HAE community in the region is very excited about the upcoming **2024 HAEi Regional Conference Americas** in Panama next year. Over the past weeks, I have supported the Member Organization leaders with questions regarding the information for the travel grant process. Some Member Organizations, such as Bolivia, are new and have never participated in a regional or global HAE event. I'm delighted to be able to help and look forward to seeing representatives from Bolivia and all the countries in my region in Panama. We hope for a great meeting!

I continue to share important information via our region's Instagram: https://www.instagram.com/haei_sudamerica_y_mexico/

It increases awareness of the disease and helps in disseminating the activities from the countries in the region. Follow us and stay up-to-date with the activities in our region.





JAVIER SANTANA
CENTRAL AMERICA AND CARIBBEAN

In Central America and the Caribbean, every day, there are more doctors interested in learning about Hereditary Angioedema (HAE) and how to help patients in their respective countries.

New doctors in El Salvador and Costa Rica have been in communication with HAEi and me to find a way to promote events aimed at educating more about HAE.

In communications with health officials of the Government of Costa Rica, we were informed about their plan to purchase and acquire medications for patients with HAE in Costa Rica. There would be two medications, Berinert and icatibant. This news has doctors and HAE patients in Chile on alert to initiate their requests.

For the first time, patient groups from the Dominican Republic and Cuba met to find a way to unite efforts and support for patients with HAE in both countries. Both Cuba and the Dominican Republic have clinically diagnosed HAE patients. Although it is not always available in Cuba, doctors use Berinert in cases of emergency, such as attacks on the face and throat. Other conventional therapies are used to treat attacks.

In Panama, the group of HAE patients and Dr. Olga Melcina, the group's medical advisor, continue to meet with government officials to seek approval of new treatments in their country and that they are available for patients with low economic resources. Although Panama has one HAE medication, it is not accessible to everyone.

In 2024, the first HAEi regional conference of the Americas will be held in Panama. All groups of patients with HAE from the countries of Latin America, Central America and the Caribbean, the United States, and Canada will participate. The patients in the region and their leaders are excited for the moment when they can share experiences with other patients from nearby countries, meet other group leaders, hear about new HAEi strategies and tools, and learn what medical specialists from around the world have to say about the issue of HAE and the progress achieved. It will be a special and unique event in the region. For more information about it, look at <https://americas.haei.org/>



PATRICIA KARANI
SUB SAHARA AFRICA

The **2023 HAEi Regional Conference EMEA** drew the attention of 9 doctors from different countries in the Sub Sahara Africa. The doctors got a chance to meet and discuss the challenges affecting them in the region and develop mitigation plans to ensure that they do not misdiagnose HAE patients or lose them to follow-up.

During the conference scientific track poster session, Dr. Anne Barasa, a Clinical Immunologist from Kenya, showcased a case study on HAE and bowel obstruction.

During the patient and caregiver track, we had the pleasure of hearing about the sometimes challenging journey to diagnosis from a patient from Mozambique and how HAEi has been able to give her courage and know that she is not alone and that there is hope for a brighter future ahead of her with correct therapy.

With the increase in doctor and patient awareness, the region has seen an increase in correctly diagnosed patients.

Burundi: I had the opportunity to meet with a doctor from Burundi at the African Society for Immunodeficiencies (ASID) conference held in Morocco, and she was very positive about working with me to find out if there are any patients in Burundi. We have agreed to have a call with our HAE Doctor in Kenya so that they can chat about what to look out for. I also shared our HAEi emergency room poster with her so that she can use it to raise more awareness amongst the healthcare professionals in her country.

Comoros: I met an HAE patient and her family at the **2023 HAEi Regional Conference EMEA** in Germany. She led me to contact her family back in Comoros because she said it would be great if we reached out and tried to help them. I contacted the family, and we are having more dialogue on what HAE is and how we can best identify it. I am also seeking to discuss HAE with their doctor and see how we can assist them.

Senegal: A discussion we had with Dr. Indou from Senegal during the conference led us to know there are around 10 patients who have been diagnosed with HAE. These patients are currently being cared for by the dermatology department in a hospital in Dakar. Hopefully, we can plan to have a first patient meeting in Dakar soon so that these patients can also meet with HAEi representatives and know they are not alone.





MARIA FERRON
THE MEDITERRANEAN, NORTH AFRICA
AND THE BRITISH ISLES

The main focus during these last 3 months has been the organization of the **2023 HAEi Regional Conference EMEA**. After hard work and the great support provided by our HAEi Conference Operations team, it was an enormous pleasure to meet up in Munich with the patients, caregivers, and physicians from all the countries that I look after and have a focus on the region's HAE patients. You will find a lot more information about the EMEA Conference in this magazine, and I hope this will be an inspiration to Take Action!

In June, Fiona Wardman (at the time HAEi's Chief RPA), Patricia Karani (HAEi RPA for Sub Sahara Africa), and I had the pleasure of being invited to talk about HAE & HAEi during the 8th Congress organized by the African Society for Immunodeficiencies (ASID) held at Mohammed VI University of Sciences and Health, Casablanca. We presented to all the participating healthcare professionals. We gave an overview of the current situation of patients with HAE in those countries and introduced ourselves as a point of contact should they know about any HAE cases or want to increase their knowledge of HAE. We also distributed the Emergency Department poster developed by HAEi in Arabic, French, and English to be displayed in their hospitals to create awareness locally.

The day after the Congress in Casablanca, Fiona and I went to Cairo, Egypt, to attend the HAE Patient Workshop organized by HAEi. I took the opportunity to invite to this workshop some of the North African neighboring countries that may experience problems with their visa applications to attend the Regional Conference EMEA. We were honored to have 35 participants from Egypt, Jordan, and Libya, and 3 from Tunisia participated in the meeting virtually. During the workshop, we had the opportunity to hear from patients and physicians about the current situation in their countries. It was a great opportunity to catch up with Libya's Member Organization and physician lead. At the end of the meeting, an open and friendly atmosphere helped attendees to talk about their

personal experiences. This helps people better cope with the disease and avoid the feeling of loneliness that many patients face... it was a really great experience!

As is common during the summer months in the region I support, I received several requests from patients about where to go and who to ask for in case of an HAE emergency while traveling, the accessibility and availability of modern treatments in the country they are visiting, and any other helpful information they can use during their trips. In my responses to these requests, I always suggest downloading the HAE Companion app, which provides an easy way to access the HAEi emergency card in many languages and a list of ACARE Centers and HAE-knowledgeable hospitals and physicians.

This type of request proves the great network HAEi has created worldwide and how HAE patients are well-connected to live their lives without fear and make their dreams come true.

One of the youngsters from the Spanish association, AEDAF, joined the HAEi LEAP educational program. The youngster, Naiara, has presented to AEDAF the project on Social Media developed during the course, and the association has delegated their social media channels to her. AEDAF is really looking forward to applying for another youngster to join the LEAP 2024 program to support its local advocacy activities and prepare them to be the future leaders of the association.

We have a new country using HAEi Connect. The local association in Portugal, ADAH, is now using this tool to securely handle all their members' personal details and communicate with them.

Finally, I am happy to announce that Libya is in its last steps to obtain access and reimbursement to the first HAE modern treatment; distribution of the product is expected by the end of the year if everything goes as planned.



JØRN SCHULTZ-BOYSEN
NORDICS, GERMANY, AUSTRIA,
SWITZERLAND, AND ISRAEL

Over the past months, the main focus has been the **2023 HAEi Regional Conference EMEA** in Munich, Germany, 1-3 September 2023. You can read a lot more about the conference and the impact it has made amongst patients, caregivers, healthcare professionals, and representatives from the pharmaceutical companies in this edition of Global Perspectives.

Participants from all over the region were in Munich – Finland, Norway, Sweden, Denmark, Israel, Austria, Switzerland, and Germany – and in all three tracks – patient/caregivers, youngsters, and the scientific track. All countries had prepared a short presentation and an important message to share with everyone at the conference. These messages were presented and read out in the patient/caregiver track with the statements from all the other countries. This was a powerful moment with all Member Organizations represented.

Apart from the many inspirational presentations at the conference, it was great to feel the connection between participants, and the networking during breaks was massive. It was time to meet new and “old” friends and catch up with the latest from the pharma companies supporting the conference. At the same time, seeing so many youngsters participate in their particular track was wonderful. It's great to see so many young people who are the future for HAE.

At the conference, it was a pleasure to announce that the Pharmaco and Socioeconomic study has now been launched in Germany and Switzerland for all patients and caregivers to respond to. It will soon also be open in Austria. We hope that as many of you as possible will take your time to answer the survey. The HAE organizations in Germany and Switzerland will contact you about this (and soon Austria as well) – and those in Munich received a one-pager describing the study.

Our friends from Switzerland have produced an excellent short film explaining HAE in simple terms. It is explained in non-medical language and covers what triggers the disease, how the processes in the body work, and what

can be done about it. The movie is spoken in Swiss-German and is subtitled in English, German, French, and Italian – see the film here: bit.ly/hae-simply-explained

In Scandinavia, a youngster completed the HAEi LEAP 2023 program, and the project being worked on together with the local Member Organization is developing an app for patients and caregivers. The app will sum up the content provided to the community in the Scandinavian countries and focus on not only the illness but also the tools available to all to make daily life with HAE easier. It will be available in the languages spoken in the Nordic countries, which offers great potential for other Member Organizations to use the app's content.

Also, I am very happy to say there is interest in HAEi hosted websites from two countries in the region – Finland and Austria. Hopefully, we will soon get started on these two websites.

As always, a shout-out to the two apps offered by HAEi. **HAE TrackR**, the app that enables people with HAE to take control of their HAE by registering acute and prophylactic treatments, and HAE Companion, the pocket-size travel partner app. Both are highly recommended and available in Apple's App Store and Google Play.

HAE TrackR is a great tool now available in 36 languages – several spoken in “my” region (Danish, Finnish, French, German, Norwegian, and Swedish). The **HAE TrackR** app is easy to use and it will help the patient and physician in their dialogue on current and future treatment options.

The HAE Companion app deserves another mention. This app will help you find local HAE treatment centers, directions to find them, and Emergency Cards available in more than 80 countries.

But nothing beats meeting in person. A big thank you to all who traveled to Munich to participate in the **2023 HAEi Regional Conference EMEA**. It was wonderful to meet you all and to experience this special conference with all of you. I can't wait to see you next time again!



MICHAL RUTKOWSKI
CENTRAL EASTERN EUROPE,
BENELUX, AND MIDDLE EAST

Hello HAEi Friends!

I truly hope you had an enjoyable and relaxing summertime spent with your loved ones.

I am happy to catch up with you again and to share what happened over June, July, and August. It has been a very busy time dominated by the **2023 HAEi Regional Conference EMEA**.

Having this opportunity, I would like to express my gratitude to all patients, caregivers, and expert physicians from Central Eastern Europe, Benelux, and the Middle East who participated at the HAEi Regional Conference for our HAE community from Europe, the Middle East, and Africa. It is always so important to get together, share experiences, get to know the latest updates on the condition and treatment availability and interact with HAE stakeholders. And, most of all, to be among those who understand us very well.

The conference in Munich gathered HAE people from the following countries in the regions I look after (in alphabetical order): Armenia, Belgium, the Czech Republic, Egypt, Estonia, Georgia, Hungary, Iran, Kazakhstan, Latvia, Lebanon, Lithuania, the Netherlands, Oman, Poland, Saudi Arabia, Slovakia, Ukraine, and the United Arab Emirates. Unfortunately, visa issues prevented friends from remaining countries joining us in Germany.

Although my main focus was preparations for the HAEi Regional Conference EMEA, before the conference in Munich, many other amazing things happened.

In mid-June, HAEi organized and attended the HAE Patient Workshop in Cairo, Egypt, dedicated to patients, caregivers, and local doctors. 35 people from Egypt, Jordan, and Libya participated in person, and 3 from Tunisia participated virtually. We had an opportunity to interact with HAE Expert physicians, Dr. Elham Hossny, Professor of Pediatrics at Children's Hospital, Ain Shams University and Secretary General of Egyptian Society of Pediatric Allergy and Immunology, and Dr. Wala Mohamed Shoman from Alexandria University Children's Hospital. Also, there was a session on the global perspective of HAE presented by HAEi Executive Vice President Global Advocacy Fiona Wardman, and a session on HAEi tools and resources presented by HAEi Advocacy Facilitator Middle East and North Africa (MENA) Mohamed Osman. There is so much to do in Egypt, as among the total population of 110 million people, there are only just over 40 HAE patients diagnosed. Therefore, HAEi discussed with Dr. Elham Hossny, the leading local HAE expert, to have a pilot project of the Heat Map Survey in Egypt. The Heat Map Survey is the methodology for countries where there are few, if any, people diagnosed with HAE. The objective is to identify pockets of people who may have the condition. I enjoyed attending a virtual meeting with Dr. Elham Hossny and HAEi Chief Scientific Officer Deborah Corcoran. Fingers crossed, we will have this survey running in Egypt shortly.





In late June/early July, I helped to organize the first-ever HAE Poland Youngsters Summer Workshop. This event was part of Dominika Blacharska's HAEi LEAP project; Dominika is a HAEi Youngsters Advisory Group member and an HAE Poland Regional Patient Advocate. The project was to empower and motivate young people with HAE to start advocating for themselves and other HAE patients. There were 43 attendees at the 3-day meeting, including 29 youngsters, their chaperones, expert physicians, a nurse, a psychologist, and a famous Polish celebrity!

Also, in July, I was privileged to attend the 2023 US Hereditary Angioedema Association Summit in Orlando, Florida. It was an extraordinary experience for me to see 1,200 HAE friends in one room and to see the work that the HAEA team has done - great motivation and inspiration for further advocacy activities in my regions.

Furthermore, I was invited to attend the HAE Junior Czech Republic Summer Camp. However, due to the

date conflicting with two other advocacy events, I couldn't participate in person in this important meeting. Therefore, I recorded a video for the children and adolescents who attended the 3rd edition of the Summer Camp.

During June, July, and August, I had numerous different virtual meetings with:

- HAEi Member Organizations from Algeria, Georgia, the Netherlands, Oman, and Qatar
- Industry partners from BioCryst, BioMarin, CSL Behring, KalVista, Pharvaris, and Takeda
- HAEi Global Advocacy Team and Operations Team
- HAEi Advocacy Facilitator MENA.

As always, I constantly work on implementing HAEi resources and projects in the regions. I am happy to share with you that HAE Georgia has launched its website, hosted by HAEi.



LIM YONG HAO
EASTERN ASIA

I began my official Regional Patient Advocate (RPA) journey in June of this year, taking on the responsibility of supporting the countries and Member Organizations in the Eastern Asia region, including China, Taiwan, Hong Kong, Singapore, Malaysia, Indonesia, and Sri Lanka, from Fiona. It was a daunting transition, and I often found myself questioning whether I could fill the proverbial “big shoes” Fiona left behind. Fiona provided invaluable guidance, and I was deeply grateful for the unwavering support of my fellow RPAs and Michal, who has since assumed the Director, Regional Patient Advocate Program role.

In the first three months of my tenure as an RPA for Eastern Asia, I focused on two primary objectives.

First, I dedicated myself to understanding the landscape of HAE in the region. Through extensive conversations with Member Organizations, patients, physicians, pharmaceutical companies, and online research, I gained a deeper understanding of the specific norms, policies, and systems that would influence public awareness initiatives and access to treatments, as well as the unique challenges faced by Member Organizations in their respective countries. For example, in Hong Kong, I discovered that elderly patients were less inclined to embrace digital solutions, posing a challenge in persuading them to use **HAE TrackR**. In Singapore, I compiled valuable information from two hospitals; one primarily managed adult HAE patients, while the other focused on pediatric cases. This information included treatment availability, clinical management guidelines, hospital-level initiatives, and plans.

With a clearer picture of the regional landscape, I became better equipped to contextualize HAEi's efforts, as well as those from other RPAs and Member Organizations, and to distill those relevant or adaptable initiatives to my region. Within the region, I facilitated knowledge sharing among countries and Member Organizations, providing them with a reference point and a source of inspiration. For instance, Indonesia

expressed interest in using social media for public awareness, and I shared China's impressive social media campaigns as a valuable reference. Moreover, I was able to leverage opportunities as they arose. For instance, during a patient meeting in Malaysia, I raised the issue of updating the indication of lanadelumab for younger children in the region, especially in China, which prompted discussions with Takeda representatives. In the future, I aspire to deepen my understanding of the landscape and strive to become the “patient advocate thought leader” for the region, as described by Henrik.

My second objective was to foster the growth of Member Organizations in countries where progress has been limited or where no Member Organizations had been established. In countries with more established Member Organizations, I provided informational support to complement their existing plans. For example, in Hong Kong, HAE HK recently celebrated its fourth anniversary and successfully registered as an organization, enabling them to seek funding from pharmaceutical companies for expanded activities. I offered them a sample sponsorship proposal to streamline their preparation process. In China, I provided HAEi social media resources and offered Mandarin translations to support their social media campaigns.

The most significant highlight of this outreach was the successful execution of the first patient meeting in Kuala Lumpur, Malaysia. The event brought together physicians, patients, caregivers, and pharmaceutical companies, generating tremendous enthusiasm for improving the lives of HAE patients in Malaysia. From this meeting, HAE Malaysia was born, and Mr. Nazim, whose brother is an HAE patient, volunteered to become the National Contact for Malaysia. We also translated **HAE TrackR** and the Emergency Card into Bahasa Melayu, the common language used in Malaysia, Brunei, and Indonesia, making these resources more accessible. While modern HAE treatments are not yet available in Malaysia, the incident of a patient experiencing rapid

relief after receiving icatibant during an attack at the meeting underscored the importance of bringing modern treatments to the country.

In other countries, such as Sri Lanka and Indonesia (and even Brunei, which is currently outside of HAEi support), the strategy focused on gathering leads from patients and physicians outside the countries, pharmaceutical companies, and other sources to identify potential patients. While I obtained numerous leads, some showed promise, while others were less fruitful. If there are any potential leads to patients and physicians in these countries, please do not hesitate to share them with me.

Unexpectedly, over the past few months, both Fiona and I received numerous invitations from pharmaceutical

companies and other organizations to speak at various platforms. This development, in hindsight, may not have been entirely surprising, given the outstanding work HAEi has accomplished, contributing to its strong reputation. Fiona is scheduled to speak at the APAAACI Conference in October, hosted by the Asia Pacific Association of Allergy, Asthma, and Clinical Immunology, and at an internal workshop for Takeda in Singapore. In December, I will have the privilege of speaking at the World Allergy Congress in Bangkok. Additionally, Fiona and I will participate in a panel discussion for the ISMPP Asia Pacific Education Committee Webinar (International Society for Medical Publication Professionals) in October. With this lineup of activities for the remainder of the year, we aim to elevate awareness of HAE among medical professionals to new heights.



FIONA WARDMAN
ASIA PACIFIC AND SOUTH AFRICA

The countries I look after in the Asia Pacific region have reduced due to HAEi's newest RPA, Yong Hao Lim, taking them over. Yong Hao now looks after Eastern Asia - the countries included in his region are Singapore, Indonesia, China, Taiwan, Malaysia, and Sri Lanka. You can read about the updates in these countries in Yong Hao's write-up.

HAEi, HAE Bangladesh, and doctors from Hong Kong and India held an educational webinar for doctors in Bangladesh on 16 September to share information on HAE, diagnosis, and treatment options. We hope to locate more patients in Bangladesh through the broader community of doctors.

HAE Korea will soon have **HAE TrackR**, an emergency card, ED posters, and the Understanding HAE guide translated into Korean. We look forward to Korean patients and families using these great resources.

Due to the unfortunate situation in Afghanistan, HAE patients are struggling with attacks with no medication access and a lack of knowledge of HAE in the country. We are looking at ways to improve the current status.

Congratulations to HAE India, whose committee has successfully registered the organization. This exciting development will open doors to advocacy, running programs, and projects for Indian patients and their families. India now has multiple pharmaceutical companies with HAE therapies in the country, and they are keen to assist patients with a better quality of life.

HAE South Africa had 20 patients and family members attend our recent **2023 HAEi Regional Conference EMEA** in Munich. It was wonderful to see the engagement and meet those who attended. There was also an opportunity for the leaders of HAE South Africa to meet with Daniella from Mozambique, who told her personal story during the Saturday session at the conference.



NATASA ANGJELESKA
SOUTH EASTERN EUROPE

This summer was not only for taking time to rest and vacation but also for finalizing preparations and facilitating Q&A regarding the **2023 HAEi Regional Conference EMEA** with Member Organization leads, individual patients/caregivers, physicians, and pharma representatives. Although I was busy sharing demands for invitation letters for some participants or sharing links for the agenda, accommodation, transport, etc., it was my pleasure to collect information from all Member Organizations and patient groups in the SEE region with messages from patient leads. These messages were shared with all the participants at the EMEA Conference. In addition, I also proposed a speaker representing N.Macedonia, Verche Jovanovska Jankovska, for the EMEA conference session for patient stories.

Understanding HAE was translated, approved, and launched in the Turkish language. It is also prepared as a print version that HAE Turkey will use in future meetings and activities.

I shared Social Media post text for translation purposes to HAE Greece so that they can use them and post them on HAE Greece social media channels.

I was able to assist with several individual patient requests. In this regard, I facilitated communication and provided information about some HAE resources (Emergency Card and HAE Companion app) to a caregiver from Germany who contacted me as his daughter would travel to Italy for summer vacation. I received a request to share information about hospital and Emergency Room sites from a patient who lived in Turkey and is now transitioning to live in London. I provided him with input from the HAEi website and suggested downloading the HAE Companion. I facilitated and assisted with information about HAE resources (HAE Companion, website, knowledgeable physicians, and hospitals) for a Romanian patient who will study in Holland. I've included my fellow RPA colleagues (Jørn and Maria) for the respective regions in all communications mentioned.

I communicated with several pharma representatives regarding the request for their support to conduct a SEE meeting in Skopje with Member Organization leads and physicians.

We've all been ready and eager to participate in the **2023 HAEi Regional Conference EMEA**, in Munich. The opportunity to meet face-to-face with so many patients, caregivers, physicians, and industry representatives is always emotional, motivational, and inspirational. It gives us more energy to "keep rolling" and work to identify new patients, support those we already know, create meaningful partnerships, and think about what actions can make our future brighter.

So Take Action and join us in all the initiatives we implement for improving the quality of life for all HAE patients, and take advantage of many of the existing HAEi resources for Member Organizations. Start with downloading HAE Companion, which allows you to access helpful information while traveling or in general, or **HAE TrackR**, which enables you to track your attacks and treatment easily and carefully manages your data so you can decide to share information about your condition that you can share with your physician.



HAEi Advocacy Academy

Your first step to successful advocacy

HAEi Advocacy Academy is a free online learning platform, designed to support people with HAE in everyday life and help anyone who is interested in becoming a successful advocate.

If you are just beginning to think about advocacy and have never worked with it before, or you have already worked with advocacy for a long time, we offer a range of courses that will help you. In our courses, you will have the chance to hear from HAE advocates and individuals who are making a difference for people with HAE every day.

New courses are added regularly and are written in manageable 'bite-size chunks'. This allows you learn new things in your own time and at your own speed and finish a course in a timely manner.

Features of HAEi Advocacy Academy:

- ✓ Is a free, online training platform with a range of courses, advocacy training, and supporting tools
- ✓ Can be accessed in your own time, and courses can be completed in bites
- ✓ Comes in manageable 'bite-size chunks' that will allow you to start and finish a course in a timely manner
- ✓ Has Member Organization's exclusive access courses providing information and training about HAE, advocacy, running an advocacy organization, and HAEi resources
- ✓ Has an open access area available to everybody interested in knowing more about HAE or becoming an advocate



**HAEi
REGIONAL
CONFERENCE
EMEA
MUNICH
1-3 SEP 2023**

EMEA – Let’s Take Action!

Across 1-3 September, the 2023 HAEi Regional Conference EMEA motivated an audience of around 650 HAE patients, caregivers, physicians/scientists, and industry representatives from 61 countries to Take Action in the knowledge that every single person in the room can make a difference. Here, we present highlights from the second of HAEi’s three Regional Conferences.

In this special feature section, we take you through what happened at the **2023 HAEi Regional Conference EMEA** in pictures, summaries of talks and words of our speakers and participants. The first conference specifically for this region included three tracks, with the opportunity to hear from global experts and advocates, share experiences across borders, and spend time with friends old and new.

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Conference Welcome

Stepping onto the stage, HAEi Chief Executive Officer and Chairman of the Board **Anthony J. Castaldo** and HAEi President and Member of the Board **Henrik Balle Boysen** welcomed “650 of our best friends from this amazing region.”

Tony reiterated the importance of being together again, and Henrik reminded the audience that the HAEi team is dedicated to caring for everyone in the global HAE community. HAEi wakes with a heartfelt motivation to provide Member Organizations (MO) with the tools and assistance they need to gain access and reimbursement for life-saving and life-giving HAE medicines.

Tony invited the audience to remember the late, great Professor Marco Cicardi, whose pioneering work and vision led to the formation of HAEi. Tony also invoked the ‘Spirit of Budapest’ as he paid tribute to the early efforts to foster a community of physicians, scientists, patients, and caregivers. Ten years after that first

meeting in Romania, HAEi has grown to 96 affiliated MOs across the globe.

Henrik outlined that the conference theme “**Take Action**” encapsulates the can-do attitude of the whole HAE community, something that the HAEi leadership, as fellow patients and caregivers, understands only too well.

Henrik gave an update on HAEi’s five critical areas of focus:

- Bringing HAEi closer to the countries and regions through a decentralized operational footprint
- Offering a suite of highly relevant technology-driven tools, apps and services
- Supporting the advocates of the future through a dedicated program for young people with HAE
- Delivering in-person events to bring together the community and HAE advocacy leaders
- Producing real-world data and research to demonstrate the value of HAE medicines

The two leaders turned their attention to the decentralized structure of HAEi and the mantra of **“Think global, act local”**. According to Tony, key to this are the accomplished Regional Patient Advocates, who were chosen because of their remarkable talent for advocacy and familiarity with the geographies they serve. Tony stressed that the RPAs are ‘always listening’ to understand the unique needs of a region like EMEA.

Sharing highlights from the upcoming program, Tony described the ACARE initiative, which accredits the best HAE treatment centers, further reinforcing the global and local link. Henrik then drew attention to the wide range of programs and services, each designed to allow every member organization to punch above their weight, achieving more for people with HAE.

Moving from tools to research, Tony explained the importance of data in helping MOs get access to and reimbursement for medicines. Tony recounted an early encounter with US policymakers: “We would go to Congress and tell them our often very moving stories. We’d make these grizzled politicians shed a tear, but nothing happened. We recognized that people react emotionally, but to truly act, they need data.”

HAEi supports and develops the data needed to give MOs a seat at the table when decisions are made about treatment. This research ranges from demonstrating the value of effective medicine to people with HAE through to baseline information on number of patients, number of attacks, and any geographical clustering. Tony noted these studies: “Put HAE at the front of the line because everyone in this room knows what it’s like to suffer from HAE attacks.”

Henrik took the opportunity to thank the pharmaceutical company sponsors, who manufacture great medicines and offer the promise of more through clinical trials; their generous support made this conference possible. Henrik thanked diamond sponsors: BioCryst and Takeda; gold sponsors: CSL Behring and Pharming; silver supporters: Astria, BioMarin, Intellia Therapeutics, Ionis, and KalVista Pharmaceuticals. The audience showed their appreciation with warm applause.

No Patient Left Behind

Taking two stairs at a time, **Professor Marcus Maurer’s** energy radiated from the stage. Welcoming the conference to his native Bavaria, Prof. Maurer said that the title of his presentation, No Patient Left Behind, was no mere slogan but a global rallying cry. He also said it is a universal message. Whether you are in a country with modern medicine or one currently without, Prof. Maurer made clear that this rallying cry is for you.

Prof. Maurer showed the audience a road, a patient journey with HAE. He told the audience that the road of HAE should be short, but it’s not. Too often, it is a long, winding, rough road for people, from diagnosis to treatment to complete control.

Prof. Maurer turned to the global guidelines on HAE management. His favorite recommendation was that the goal of HAE treatment is total control of the disease and to live a normal life. Marcus asked the audience: “Does that sound good? Then how do we get there?” His answer was to make sure that no one has attacks, that no one lacks control, and that no one needs to step back from living their best life.

Prof. Maurer accepted that the way to the destination can be “riddled with hurdles that you need to overcome.” He promised that through the work of HAEi and its partnership with GA2LEN (Global Allergy and Asthma Excellence Network) to create ACARE centers of leadership, the obstacles can be removed.

“The first way to overcome obstacles to complete control is providing knowledge to people living with HAE, especially youngsters, the next generation of advocacy,” Prof. Maurer told the assembled HAE community.

Prof. Maurer gave a history of ACARE. From the initial partnership with GA2LEN to the current situation with 88 centers across 35 countries. Prof. Maurer called on attendees to support ACARE’s efforts. He said: “The goal is to have at least one ACARE in every country. Thirteen new members have joined this year. Ask yourself or your clinician, why don’t you become an ACARE and do better?”

The first hurdle on the HAE journey was diagnosis. There are patients with many relations with HAE, and many who are the first in their family to have the condition; both experience delays in diagnosis. He outlined ACARE programs to educate physicians: “There’s a wonderful webinar series that is freely available to all interested, and almost 1,000 physicians have already done so, helping them to diagnose HAE from a picture of a swelling.” He also mentioned online Interact Masterclasses, bringing face-to-face preceptorships (structured guidance and support) and reaching more physicians with education on early recognition and treatment of HAE.

To educate, we must speak the same language. The fact that HAE is not just one disease can make things very difficult. As Prof. Maurer put it: “HAE comes in many types and flavors.” A vast global project called DANCE is underway to harmonize how physicians talk about angioedema and all its different subtypes and manifestations. The work of 91 experts, DANCE, is being finalized right now.

“All these projects can help target delayed diagnosis, but what of misdiagnosis?” Prof. Maurer asked. To address misdiagnosis, ACARE offers several “simple, super tools.” The first is titled: ‘10 Questions That Save Lives’. Next is the tricky topic of genetic testing; **Project Imagine makes free genetic testing available for anyone with suspected HAE anywhere on the planet.**

Next, Prof. Maurer focused on treatment with strong words: “We now believe that androgens are dangerous in the long run, with side effects.” He accepted that for now, they are still used in Europe, the Middle East, and Africa. In order to change this, he sought data. “The project SHAERPA wants to hear from you if you ever had androgens in your treatment,” Prof. Maurer said.

Prof. Maurer said: “You can see the goal, but it’s not quite working. We lack the fine-tuning, the monitoring.” Using **HAE TrackR** will help you take control, and it helps clinicians see patient-reported outcome measures. “Not physician guessed, but patient-reported,” quipped Prof. Maurer. Tell your physician that you want a way to give your assessment. An angioedema control test (ACT) score is a tool that is available in 80 languages, so ask your physician, because no one knows your HAE as well as you. Prof. Maurer told the audience: **“You have the information to make your physician a better treater.”**

We have the data, and we have improvements, but as Prof. Maurer said, we must optimize. “We shouldn’t be happy with good. We want perfect.” Here, according to Prof. Maurer, the guideline is one of our most powerful weapons. It shows people what can be done and what the right decisions can achieve. Prof. Maurer said that the global HAE guideline is up for review, and called on physicians, patients, and caregivers in the room to sign up to be part of the next update.

Another major research project is the launch of a global angioedema registry, CARE. A big database shows us the situation with access, care, and how HAE compares to other types of angioedema. Prof. Maurer implored the audience to say yes if they were asked to donate their data. “We are building a global brain” Prof. Maurer said.

Prof. Maurer’s final project was MENTALIST. This, he told the audience, asks physicians, industry, and policymakers to share their burning questions for future HAE research so that the answers are there to enable the right decisions to be made.

In closing, Prof. Maurer returned to No Patient Left Behind. He called on all present to make it: “easy and fast,” concluding: “This is a message of hope and of vision. We will go down that road together, and we will overcome these hurdles.”

With that stirring message and to loud applause, Prof. Maurer left the stage. The audience then split into the three tracks of youngsters, scientific, and patient/caregivers for the rest of the evening.



No patient left behind!

“This is a message of hope and of vision. We will go down that road together, and we will overcome these hurdles.”

– Prof. Marcus Maurer



HAEi Patient Resources: Take Action!

To conclude the first evening of the HAEi EMEA Regional Conference, another double act was welcomed to the stage, this time **Fiona Wardman**, HAEi's Executive Vice President Global Advocacy and Chief Diversity Officer, and **Jørn Schultz-Boysen**, Executive Vice President Global Operations and Chief Compliance Officer.

Welcoming everyone back from the movement break, Fiona introduced the session. She and Jørn will present some services and programs to help MOs, patients, and caregivers improve their quality of life and win access and reimbursement for life-saving and life-giving HAE medicines.

Jørn told the audience about the travel partner, HAE Companion. Although it started as a tool to make travel easier, it also helps with emergency department visits, explained Jørn, as it can store emergency cards in 80 languages and put every ACARE center on earth at your fingertips, even guiding you straight to the nearest to you. Jørn urged everyone present to download HAE Companion. It's free, available via Google Play and the App Store, and accessible in 84 countries.

HAE TrackR was the second resource shared. Based on an easy-to-use electronic diary, **HAE TrackR** helps people take control of their HAE by recording treatments, attacks, and disease burden and giving them sole ownership of all the data they enter. Jørn remarked that **Prof. Maurer had already mentioned the importance of using a diary app to personalize treatment for every individual on their terms.** Currently available in 34 languages, **HAE TrackR** can be accessed from any internet-enabled device. Later in the meeting, Jørn told the audience there had been 62 downloads of **HAE TrackR** in the audience and encouraged everyone to get it and see the great benefit.

Fiona took on the next tech, HAEi Connect, addressing all the MOs who deal with members' personal data. With security at its heart, HAEi Connect complies with the EU General Data Protection Regulation (GDPR), encrypting data and using two-factor authentication. She told the audience that, far more than just a data repository, HAEi Connect is a powerful tool to communicate with members.

Next up was the emergency card, which Fiona recommended to the audience as a quick and effective

way to let your healthcare professional know you have a diagnosis of HAE on arrival at a hospital or care center. Containing clear and straightforward information about HAE and treatment required during an attack in a wide range of languages, "it could be a lifesaver," Fiona concluded. Complementing this are the emergency posters, which can be hung in hospitals and emergency rooms to educate non-specialist physicians about HAE.

Finally, the Understanding HAE patient guide was in the spotlight. Fiona informed the audience that this stemmed from MO requests and was developed with the strong support of the US Hereditary Angioedema Association (US HAEA) to describe all things HAE and HAEi comprehensively. Sections include traveling and triggers, diagnosis and treatment, HAE and children, pregnancy, and more.

Rounding things off for the evening, attendees were given their first chance to sample the fine Bavarian hospitality alluded to by Prof. Maurer a short while earlier.



"It could be a lifesaver."

– Fiona Wardman





Welcome to day two

HAEi's new Coordinator, Global Advocacy and ACARE, **Rachel Annals**, welcomed all patients and caregivers to the second day of the conference and outlined a packed day, including:

- Updates from Regional Patient Advocates
- The path to success in the EMEA region
- Patient stories from HAE members in the region
- Presentations from HAE physicians on what patients need to know and the latest developments in science and therapies
- Two sessions where HAE expert panels answer questions from Member Organizations and conference participants

Rachel reminded everyone of the importance of meeting old and new friends at the conference, as it can be powerful to share experiences. She then opened the first session of the day, inviting Fiona Wardman on stage to lead the EMEA Regional Patient Advisors' update.

HAEi EMEA Regional Update

Fiona started the session by getting the audience energized for the day ahead. She then provided some statistics to set the scene of HAE across the EMEA region before inviting each of the Regional Patient Advisors to give an update on their region.

Some of the facts Fiona shared were:

- There are 8 billion people in the world
- Between 7-8,000 different rare diseases
- On average, one in 26.6 people has a rare disease
- There are more than 100 countries and 2.2 billion people living in the EMEA region
- There are approximately 74,000 people with HAE in EMEA

Fiona then told the audience that according to the World Economic Forum, the top three challenges for rare diseases are lack of priority, limited capacity and capability for diagnostics, and limited accessibility to treatments. Challenges that many people in this room know only too well, she said.

Following her introductory remarks, Fiona invited the five EMEA RPAs onto the stage: Michal Rutkowski, Patricia Karani, Natasa Angjeleska, Maria Ferron, and Jørn Schultz-Boysen.

Benelux, Central Eastern Europe, and Middle East

Michal Rutkowski explained that Benelux covers Belgium, the Netherlands, and Luxembourg. All three are in the European Union (EU), easing access and reimbursement for modern treatments. Identifying patients and healthcare professionals in Luxembourg has been a priority. In Central Eastern Europe, a vast and diverse region, some countries are in the EU. The situation is more challenging outside the EU, with perhaps one modern treatment or none at all. Access to clinical trials can also be a problem. **This means strong local Member Organizations are vital, and this is a crucial priority for Michal.** Finally, Michal described the general situation across the Middle East. Although there are 12,300 potential HAE patients, only 10% have been diagnosed. Across the region, there is a great deal of difference between countries. In the six countries of the Gulf Cooperation Council (GCC), medical insurance means reimbursement of modern therapies is possible for citizens, whereas non-citizens (non-nationals of GCC countries) can face problems. The situation is more challenging in countries outside the GCC, such as Egypt, Lebanon, Syria, Jordan, Iran, Iraq and Yemen. Michal is heavily involved in advocacy work in these countries.

Nordics, Germany, Austria, Switzerland, and Israel

Jørn Schultz-Boysen told the audience that his region stretched from Greenland in the far north to Israel in the south, as well as taking on the German-speaking countries of Germany, Austria, and Switzerland and the Nordic countries of Finland, Sweden, Norway, Denmark, Iceland and the Faroes. Jørn described his region as fortunate, being well served with treatment options. **For Jørn, the key word across the region is 'maintain.' He works hard with MOs to ensure that access is preserved.** Increasing medicine prices in some countries lead policymakers to consider restrictions, so continuing advocacy work is vital. Additionally, Jørn remarked that maintaining the support and commitment of patients and caregivers to their local organization is vital. He ended with a message for all those present to support their local HAE organization, participate in meetings, complete surveys, and encourage young people to join activities.

South Eastern Europe

Fiona then turned to **Natasa Angjeleska**. Natasa said her region of 147 million people showed many of the different challenges facing HAE advocacy. She made clear that for the countries in the EU, there is reason for optimism around access and reimbursement, but other

What are the main issues in your region?

Michal raised communication difficulties as a challenge. Even within Belgium, the Flemish and French-speaking parts made setting up a single, strong advocacy organization more difficult. In the Middle East, around 90% of patients are undiagnosed. Finally, Michal mentioned the challenge of attracting young people into advocacy.

Natasa described the complicated administrative structure of countries in her region, such as Bosnia and Herzegovina, where politics adds challenges. However, the priority across her region is to get access to preventive treatment and ensure all twelve countries have a Member Organization.

Maria agreed with the issues raised. Additionally, finding people prepared to take on patient advocacy is

an issue due to the work involved. Spreading the load is crucial. **She encouraged people to get involved with their local organizations. Even a little extra help will be hugely appreciated.**

For **Jørn**, we must not allow what we have to be lost. For him, it's a right for patients to be treated and have the quality of life we know they can have. Staying united as a Member Organization is so important.

Patricia spoke of stigmatization. With a disease that doctors do not know well, there is still the potential to be labeled as 'cursed.' This can lead to patients being lost or not wanting a diagnosis. She fights this stigma by making people aware of the global community. People can then see it's not just them as individuals; they are not alone and are accepted.

countries in the region fared less well. She highlighted that some countries, such as Bosnia and Herzegovina, Kosovo, and Albania, have no medicines access; patients rely on donations or clinical trials. **It is here that the regional approach comes into its own, she said, as countries can assist and support each other.** There are also reasons for positivity. In North Macedonia, the first confirmed diagnosis has occurred, which is core to Natasa’s focus on ensuring patients are identified and not left alone and in pain. She agreed with Professor Maurer’s rallying cry of No Patient Left Behind. She never takes no for an answer, preferring to see each no as an opportunity to fight harder.

Mediterranean, North Africa, and British Isles

Next up was **Maria Ferron** and her region, which straddles Europe and Africa. Even within EU countries, Maria mentioned frustrations, such as needing individual areas within Italy and Spain to approve medication reimbursement. There are some reimbursed medicines in Algeria and Libya, but in Morocco, reimbursement has yet to be accepted. **Her ongoing work is heavily focused on supporting new Member Organizations and working to locate more patients.**

Sub Sahara Africa

Patricia Karani spoke next. Her region is significantly populated, with around 1.2 billion people and 40,000 potential HAE patients. Unfortunately, she said, in most of Africa, Governments focus on common diseases like malaria, so money is not set aside for rare diseases. She focused on positive steps. **Patients are being trained to speak up and become advocates.** More healthcare professionals are being trained to identify and diagnose HAE, which has been a five-year journey. Some modern treatment has been acquired. Patricia made clear her focus is to raise awareness amongst healthcare professionals to give patients hope of a diagnosis. She said, “It’s one step at a time.”

Fiona added to Patricia’s presentation with an update from South Africa. Fiona has been assisting the Member Organization to locate more patients. The tools and resources HAEi offers are critical to this, allowing the MO to grow by securing funding.

Fiona then invited all RPAs to take part in a panel discussion.

What is the number one priority for your region?

Fiona described the situation in South Africa and the need for funded prophylactic treatment so patients do not need to wait for an attack. Locating more patients, especially those living outside big urban settings, and getting them the right doctor for a diagnosis. She highlighted that: **“Action is the key that solves all of your problems.”**

Maria made clear her immediate priority is to support the creation of a Member Organization in Tunisia and to aid in the identification of patients in Mauritania.

Natasa wanted to emphasize the need for family testing. Patients understandably don’t want children to inherit the disease, but this means they are not tested. The earlier you get a diagnosis and access to medicine

that could save your life, the better. She also prioritized educating family doctors as this can have a significant impact on the speed of diagnosis.

Michal highlighted the need for strong Member Organizations in all countries, which HAEi is only too happy to help and support.

Jørn returned to the theme of building, and to do this, the priority is to grow the next generation of advocacy and medical professionals—that way, the successes to date will be maintained in the future.

Patricia closed this question by telling the audience that her priority in the region was increasing knowledge among healthcare professionals.

How important is the patient voice in your region?

Fiona introduced this question, commenting that **an educated patient is an empowered patient.**

The group agreed that industry needs to hear the patient voice, to help them directly develop and meet the needs of people.

The theme of telling your story to the media was returned to as a potent way for the patient voice to be heard.

Jørn spoke about the uniqueness of each patient experience and that; **“No one knows HAE better than we do as patients and caregivers; we should be the ones that speak and have the privilege to be heard.”**

Patricia told the audience that she was scared of speaking about her HAE, but when she started, it gave her strength, and she began to accept that it was OK to have HAE.

How can/will advocacy impact the countries in your region?

Moving onto the next question, **Patricia** stressed the importance of advocacy in educating healthcare professionals.

Jørn felt that although advocacy is hard work, it has an enormous impact. Maintaining access will be entirely a result of the advocacy work across his region.

Natasa spoke powerfully about how advocacy was ‘fighting for the right to live’ and that sharing our stories can motivate and empower others to do the same. Educating yourself about treatments and research will give you the ability to speak with those who make decisions on your behalf.

The power of media advocacy was raised. One African patient telling his story on radio and TV led to increased access to treatments in a short period of time. **Maria** encouraged everyone to share their experience.

Fiona spoke of how receptive HAEi is to anyone with ideas about advocacy in their region or country. **“Speak to your RPA,” she told the audience. ‘They can share knowledge of where others may have done something similar or help you in any way.’**

How important is data in advocating for HAE?

On the final question, **Fiona** remarked that data is the summary of many individual stories, making it so powerful.

Jørn commented that much work is being done in his region to generate data, which is important to create stronger arguments when we speak to policymakers.

Patricia recalled meeting a health minister who asked how many people with HAE there were, but at the time, the only people in the country were the four members

of her family. The minister was dismissive of such small numbers, but the actual figure will be much higher than that, which makes data so important.

The group also agreed that data is how patients can better manage their condition; get a quicker diagnosis; find an effective treatment and get reimbursement.

The effectiveness of having your own data was also mentioned. The panel highly recommended HAE TrackR to collate your own data.



Only 7 % of rare diseases have treatments

“This demonstrates how lucky HAE patients are to be part of that small percentage that does have treatment.”

– Fiona Wardman, HAEi



Fiona then turned to the audience with a question: What percentage of rare diseases have treatments? The options were: 67%, 47%, 27% or 7%. There wasn't a clear consensus from the audience, but the answer was 7%, which Fiona said demonstrates how lucky HAE patients are to be part of that small percentage that does have treatment.

Before turning to messages of support from across all the Member Organizations within HAEi, Fiona stressed with a Dr. Zeus quote, **“We are the ones we've been waiting for. You have the power to Take Action and improve things for yourself and for the HAE community where you live.”**

And with that, the panel shared the Member Organization's messages. You can read all these by following the QR code to see the slides. Fiona thanked all the Member Organizations for their fantastic work and the RPAs for their efforts in support.

In conclusion, Fiona told the delegates that we have made much progress in raising awareness and access to modern treatment in the past few years. **Even though many challenges remain, there is good reason for hope and optimism.**

Read the Member Organizations' messages – scan the QR-code or visit haei.org/emea-mo-messages





Take Action!





Raising HAE Awareness in EMEA

Jørn and Michal returned to the stage to discuss awareness raising. The first question posed was to Michal: Why is raising awareness important? Michal said it's the basis of success, increasing enthusiasm and support, stimulating action, and generating local knowledge.

So, 'where do you start with raising awareness?' asked Jørn. 'Talk to as many people as possible,' said Michal. He also encouraged people to look around them in the community; look at who is speaking and consider what they are saying. You can use social media as a really powerful tool, but use it to impact, not impress, people. He said to use it to show people who you are and what you stand for. Make as many connections as possible, both on and off social media.



"Well done and thank you, it has been an amazing experience, I learned a lot, made new friends and am inspired to help others with HAE and those that do not know they have HAE. The conference was excellent. I appreciate the opportunity to have been able to attend."

– Patient/Caregiver Track participant

"Great space to gather together patients, caregivers, physicians and industry and share best practices and updates."

– Industry Representative



Michal continued that awareness raising can also be turbo-charged by the tools and resources HAEi offers:

- Use **emergency cards** and **emergency posters** to spread awareness. They are in a vast range of languages
- Make use of **resources for schools** to educate when children start their studies
- Create or update a **website for your community** using HAEi's free templates and web hosting. Jørn showed new websites from HAE Scandinavia, HAE South Africa, HAE Turkey, HAE Kuwait and HAE Georgia
- **Share information materials.** Your Member Organization will have hard copies and electronic versions available. Another example was an information film produced by HAE Switzerland
- **Attend meetings.** These can be local, national, regional and global events, casual catch-ups, or formal leadership sessions. Jørn made clear that everyone can benefit from finding out the most up-to-date information, as knowledge is power
- **Encourage young people to become involved.** The HAEi Youngsters Community is over 100 young people who share personal experiences and meet others. HAEi LEAP helps equip the advocates of tomorrow with skills and experience to raise awareness and advocate for HAE; it's also great fun
- **Take courses on HAEi Advocacy Academy** to learn more and brush up on skills like press release writing
- **Get involved with hae day :-).** Everything you do helps bring us all together as one big family. The **hae day :-)** website has all you need to get ready for 16 May
- **Contact government officials** to raise awareness. Writing and meeting local politicians regularly will keep HAE front of mind for people
- **Offer to tell your story to the media.** Talking in public can be daunting, but it becomes easier once you've done it the first time, and the benefits are huge

Jørn encouraged people to be persistent when taking action and celebrate all successes, no matter how small. He commented, "Persistence will get you there, but consistency will keep you there."

Finally, both speakers reiterated that they and their fellow RPAs have lots of experience raising awareness and are only too willing to help. Just ask!



The Path to Success in EMEA – Take Action!

Fiona and **Tony** led the audience through the path to success and how best to Take Action.

Tony explained what Take Action meant in practice for him. It means participating in patient-driven research. It means being involved with your Member Organization. But for the HAEi team, according to Tony, it means waking up every day thinking about how to help the HAE community.

“For us,” Tony said: “HAE is personal. We know what it’s like to suffer. We feel what you feel. And we take this emotion into our day-to-day work.”

Starting with research, Tony described the highly successful **Heat Map** survey as perfect for new Member Organizations or areas with little diagnosis. He described the situation in the Philippines, where doctors told him there were “no HAE patients.” After a Heat Map Survey, 28 pockets of swelling disorder symptoms were uncovered, and a previously unknown group of 14 HAE patients was found. Once pockets are identified, Member Organizations can go there and educate people so that diagnoses will follow. Similar surveys are now planned in Rwanda and Bangladesh.

Just one person’s actions can make a difference. Tony described the situation in Macedonia, where patients didn’t take no for an answer and worked hard to make things happen.

The **State of Management of HAE** is another fantastic resource, Tony told the audience. It provides an individual analysis of country circumstances to establish the needs of people with HAE. Tony remarked: “As we all know when this rare condition is not adequately treated, it qualifies as a catastrophic unmet medical need.” These studies are now due to be updated, so look out for your opportunity to be involved.

Tony then explained the **Baseline Burden of Illness** research. These are for countries where access to therapy is inadequate. “Testimonials will bring a tear to the eye, but only combined with the data that these studies produce will you get decisions made.” This research shows the true burden of living with HAE, such as the number of attacks experienced, days of work and school missed, and the impact on healthcare systems through emergency room visits and deaths. Research of this kind has recently taken place in India and Mexico, with a study ongoing in South Africa and plans for Colombia.

HAEi conducts a **Pharmacoeconomic, Socioeconomic, and Quality of Life Assessment** study. This is focused on countries where reimbursement may be lacking. Already complete for the Nordics, Australia, New Zealand, and the UK, with research just starting in Germany and Switzerland, these show governments that there is great value in these medicines because they reduce hospitalizations, emergency room visits and allow people to work and study more.

Finally, Tony said there are **Shared Decision-Making Tools**. These capture your preferences and assessment of HAE so that better decisions are made about your treatment when the data is shared with your doctor. This is a pilot in the United States right now but will go global in the near future.

“Our research will make a difference to your ability to Take Action,” Tony concluded his remarks before handing over to Fiona.

Fiona told the audience the path to success is: “Simply put, one of community involvement. It is critical. What goes on in your HAE community directly affects you.” She shared another powerful observation: “Luck is what happens when preparation meets opportunity.” She illustrated this by demonstrating her journey of co-founding HAE Australasia almost 13 years ago. She admits she had no idea what she was doing. But a combination of determination, commitment, patience, and the tools available from HAEi made it much easier to create a productive group.

“When people put their hand up and take action on advocacy, it’s up to all of us to support them,” she said. It can be scary and fun, but it never stops. And if it does stop, then things will quickly slip backward.

Referencing the pioneering work and spirit of Dr. William Osler (who was the first to describe the illness that became known as HAE), Fiona asked the audience to think if they are happy with the current situation for HAE, and, if things are good, not just to assume others will do the work to maintain this. Everybody needs to take part, doing what they can. Your fresh ideas and opinions are welcome. Just let your local patient group know you’d like to support them and what you can do (whether it’s an amount of time or a skill like accountancy or social media).

Fiona asked people to imagine their medication or care being taken away. Having something now is not a long-term guarantee. She concluded the session by saying: **“Don’t be a spectator; become an active participant in making the world what you would like it to be. Always believe that you can make a difference because the smallest things you do can lead to the biggest change.”**

Patient Voices – Advocating for Change

Introduced to the stage by Michal Rutkowski, four brave and committed people with HAE took turns to tell their own stories in their own words.

Marijk Beekman-Kortekaas – Netherlands



“I’ve always looked at what I can do instead of what I can’t do.”

Speaking first, Marijk revealed she waited almost 30 years to get a diagnosis despite living in a rich country with an excellent healthcare system. One clue to this delay is that she is the only person in her family with HAE. The lack of a multidisciplinary approach also contributed, as many different specialists didn’t communicate with each other, meaning no one could agree that it was a single disease. In those 30 years, Marijk nearly died three times from throat attacks and underwent three unnecessary surgeries. She lost count of the hospital admissions, with her symptoms starting when she was just a baby. Over the years, she has been misdiagnosed many times, with illnesses including allergies, asthma, reflux disease, and Crohn’s disease, leading to a vast number of medicines which, of course, didn’t work.

Growing up, Marijk remembers missing out on life, never making birthdays, holidays, or parties. When puberty hit, her attack frequency and severity increased, eventually reaching one or two attacks a week. Hand and foot attacks after sports stopped her from playing tennis or running. Finishing her education was difficult, and work was impossible due to the constant need for sick leave. Despite all this, Marijk maintains a positive outlook; “I’ve always looked at what I can do instead of what I can’t do.”

Finally, her gastroenterologist conducted a colonoscopy during a swelling and told her he knew what was wrong with her.

Embarking on a quest to educate herself on her ‘new’ condition, Marijk got a referral to a leading HAE specialist in the Netherlands. Treatment was available but required hours of discussion in hospital. At this point, Marijk admits it was time for self-management, but she was scared.

Marijk recalls: “My specialist was so empowering. He trained me to administer for myself, and as a test, he let me put a needle in his vein. That was such a powerful moment. Putting a needle in the vein of your doctor, I knew I could handle living with the disease.”

Despite attacks and trying lots of treatment options, she is now getting periods of relief every month. To help others, she decided to become President of the Member Organization in the Netherlands. She was nervous about traveling to an HAEi meeting in Frankfurt to get ideas and connections, but her Regional Patient Advocate welcomed and empowered her. She left the meeting a different person. She concluded by saying: “Together we can move mountains.”



With new knowledge from the conference, Daniella is excited to go home and work with her doctors.

The next to speak was Daniella from Mozambique. She, like Marijk, is the first patient in her family to have been diagnosed with HAE. She described Mozambique as a developing country on the south and eastern coast of Africa, which means diagnosis of HAE is “a little bit complicated.”

Daniella describes her first attack at the age of four. “Out of nowhere,” she says: “my lips started swelling so much that my mouth closed. Everybody looked at me like I was a piece of art in a museum before being rushed to hospital.”

Afterward, Daniella tells of many years of similar attacks. Every time she went to the hospital, they had no answers. At 14, a tongue swelling started to cut off her breathing. In what she and her family describe as a “miracle,” she started breathing again and got better. After a trip to South Africa for tests yielded no results, the doctors gave her the prognosis that she wouldn’t live long.

Things started to change for Daniella when she left Mozambique to study in India. After a severe attack put her in hospital in a medically induced coma, she met Dr Pratik Agarwal, who had some information about HAE. Blood samples were taken and sent away to Singapore for testing. A couple of months later, Daniella finally had a diagnosis. Daniella recalls: “I was so scared. I was alone in a country getting a very rare disease diagnosis. I stopped going to the hospital for a while, but after another attack, my doctor finally convinced me to get treatment.” Daniella found relief with stanazolol, but it caused side effects. During attacks, FFP and tranexamic acid were used.

Her attacks continued, but Daniella was encouraged that her Indian doctors were willing to learn, but she was nervous about returning to Mozambique after her studies. She worried: ‘How am I going to explain to doctors this is not an allergy or some sort of curse, but a real disease?’ Her nervousness was helped when she contacted HAEi and met Patricia Karani (the RPA for Sub Sahara Africa). Patricia supported and connected her with a doctor in Mozambique who had information about the disease.

Although treatment options in Mozambique remain very limited, Daniella continues to live her life. Earlier this year, she traveled to a remote province of her country to work with the communities there, living for a whole year without treatment. She credits a vegetarian diet for reducing her symptoms.

Daniella remarked that being at the HAEi Regional Conference EMEA had been great for her, with the chance to question experts about treatment. Armed with new knowledge, she is excited to go back home and work with her doctors.

Daniella expressed a hope that in the future there will be a Member Organization in Mozambique, and is motivated by a memory of spending time in hospital with a young guy who had the same symptoms as her: “He used a handkerchief to hide his face, which was the same thing I had done for years and years because I was ashamed. My biggest dream is to be able to find him and tell him that I have an answer for this disease.” For that reason, Daniella is committed to bringing testing for HAE to Mozambique.

Meryem Jennane – Morocco



She found the HAEi website and sent an email. Someone responded, and her life changed.

Meryem from Morocco recalled her mother suffering several painful and debilitating episodes of illness. She was the only one in her family to suffer. The only name they gave her mystery condition was “periodical illness.”

Meryem herself first experienced symptoms at the end of stressful high school examinations. Her hand swelled, which she thought was all the writing during the tests. But, from that moment, she had what she described as many “crises.”

Meryem recounts how her HAE, which she now knows is HAE type III (HAE with normal C1 inhibitor), is highly sensitive to stress. Her stressful job in finance led to a scary throat attack. Thankfully, Meryem’s parents were staying with her at the time and could get her to hospital. Lying in hospital, Meryem felt frightened. She told herself this cannot continue. She increased her research. Eventually, she found the HAEi website and sent an email. Someone responded, and her life changed.

Meryem was put in touch with the Moroccan organization. Plucking up the courage to join a meeting organized to coincide with the national day of hereditary angioedema, she went along and, for the first time, didn’t feel alone and could get her questions answered. At this point, she says she could take matters into her own hands.

Despite challenges with care and medication in Morocco, Meryem describes doctors who are really careful to help her. She is working with her Member Organization and sees signs of progress. She concluded her speech by telling everyone present: “We don’t lose hope because hope brings life.”

Verche Jovanovska Jankovska – North Macedonia



We all get used to the everyday fights of people with HAE, but to others, they can be an inspiration.

Verche started with a story. A few years back, her then nine-year-old son wanted to run in a 5km race. On the morning of the event, he had an HAE attack, but after treatment, he insisted on still participating. Then, a journalist contacted Verche. To Verche, the story seemed mundane, but the journalist insisted it was very newsworthy. The resulting media coverage turned her son into a hero of the event and helped increase awareness of HAE.

Verche told the story to illustrate how we all get used to the everyday fights of people with HAE, but to others, they can be an inspiration.

Thinking back further, Verche described her diagnosis. Despite a first attack at the age of four, diagnosis took another four years. Her twin sister was hospitalized for

weeks with a facial attack. Luckily, a knowledgeable doctor was on hand, and he called every member of our family. In just a few weeks, a diagnosis of HAE was confirmed. At the time, treatment was unknown.

One of the everyday fights Verche mentioned was not to let HAE define her. She said: “We had dreams to lift our single mother from the verge of poverty. I continued my education and got a pharmacy degree. I now work in quality control for the biggest pharmaceutical company in the region. Mine is the final signature that means a drug can go out. Colleagues told her: ‘If you deal with this (HAE), you can deal with everything.’”

Verche’s mother remembers that her mother died at 33 years old. She was undiagnosed, but when she died, she was swollen. Speaking more about her family, Verche praised her sister, who helped found the Macedonian HAE Member Organization in 2009, along with physicians and Verche. Since then, she described their success as: “We really moved mountains.”

Verche also praised her husband, who took on the role of caregiver willingly and has gone from being: ‘The person who was afraid of needles to the best medical guy in the family, administering therapy as if it is nothing and then going back to bed.’

Verche and her husband hope their son is the last to have HAE. Diagnosed at just one-year-old, he struggled with very frequent attacks. Expensive medicines were bought, but that wasn’t sustainable. Verche started writing, asking for support. She said, “The only person who ever answered was Henrik Balle Boysen. He brought us to Copenhagen for the first global conference in 2012. We were welcomed with ribbons that said, ‘Together we are strong’. We started to cry; it was the first time we felt we belonged somewhere.”

That started what Verche described as the ‘Macedonian miracle.’ Henrik personally came to help in advocating with officials for treatment. A vast range of public awareness-raising activities were undertaken, from riding bikes to running marathons. Verche recalls being on TV so often she was recognized by a bartender.

Verche told the audience not to wait patiently for someone else to solve your problems: “When you think your life is not good, remember you are one of the 7% that Fiona mentioned earlier. We have treatments and

we have options. If you feel stigmatized, remember we all of us felt that way at some point. We have left the dark ages of just 10-15 years ago.”

At the conclusion of these emotional and powerful presentations, all speakers were given a long and warm round of applause from all present.



“I had some really fantastic opportunities to connect with other patient leaders, patients, and pharma companies.”

– Patient/Caregiver Track participant

“I want to thank everyone at HAEi for being there for us and for giving us the opportunity to share our stories!”

– Patient/Caregiver Track participant





Introduction to HAE: 10 Things Patients Need to Know

The iconic **Professor Bruce Zuraw** opened his presentation with cosmology. Prof. Zuraw likened his awe at seeing an image of a black hole in his lifetime with the advances made in HAE. He told the audience that he had been working in HAE for more than 40 years and didn't think so much progress could be made so fast.

He introduced his talk as giving the 10 things patients need to know; to help them be better advocates and help themselves, their families, and the entire HAE community.

Why me?

Prof. Zuraw made clear this is something that everybody asks at some point. The answer, he said, started at the genes. With HAE, you have a mutation. If you have C1-inhibitor deficiency, one gene has mutated, so C1-inhibitor either doesn't work or can't get out of the cell. That's what causes most types of HAE.

We now know seven genes that may be mutated but don't know the mechanism. With more work, this will become clearer for all.

Am I in danger?

Unfortunately, he told the audience, yes, and we must deal with that. Prof. Zuraw illustrated that airway and intestinal swelling looked remarkably similar, and the mechanism is the same: fluid leakage from blood vessels into the tissue.

Prof. Zuraw cautioned: "Anybody, no matter your history, could have a laryngeal attack, and we must be prepared to treat that."

Prof. Zuraw referenced the work of HAEi and its CEO, Tony Castaldo, in demonstrating that there is a lot of illness with HAE, and people continue to die from laryngeal attacks. The study by Tony and colleagues showed that 100% of patients felt that HAE prevented them from advancing in life. 50% missed work or school. 80% said that an extremity attack caused severe dysfunction.

"We concluded that all attacks matter," Prof. Zuraw said.

What about my family?

HAE runs in families, said Prof. Zuraw. If somebody in the family inherits the gene, each of their children has a 50:50 chance of getting HAE. It affects males and females equally, though females tend to have slightly worse disease. The genetics are simple, easy to predict, and hard to escape.

The death rate for HAE was far higher in undiagnosed people. Prof. Zuraw stressed that all family members of somebody diagnosed with HAE must be screened.

"Please, go back to your families; if anybody has refused to get screened, convince them. It's important. That applies to children, too."

What causes attacks?

Prof. Zuraw showed a study by Dr. Hilary Longhurst, which showed that the level of C1-inhibitor people had directly correlated to the risk of attack. People were at very high risk at 20% of the normal level. Control was more possible at levels above 40%.

The immediate cause of attacks can be diverse. Emotional stress is a factor, so consider protecting yourself during stressful periods. Even mild trauma in an untreated patient can lead to an attack. People with HAE should not be given ACE inhibitor drugs for blood pressure as these can provoke an attack. Other medicines containing estrogens, such as contraception or hormone replacement, may worsen your disease.

Can I treat attacks?

Prof. Zuraw now turned his attention to treatment. On-demand (or rescue) treatment is there to eliminate the attack once it starts, before it can cause harm. There are helpful treatment guidelines, and Prof. Zuraw appreciated there is medication variation across the world.

One crucial point was that antihistamines, steroids, and epinephrine do not work. Prof. Zuraw cautioned: **“You have to help yourself. Doctors who don’t specialize in this don’t understand it. Unfortunately, it falls on you.”**

Prof. Zuraw concluded that the earlier you treat an attack, the faster it will resolve. You shouldn’t wait to know if they’re severe because the medicine won’t work as well if you wait that long. Treat early and treat every attack, he said.

Can I prevent attacks?

Prof. Zuraw stated, “I think it’s much better to prevent than treat if we can,” and again referenced treatment guidelines. He also made it clear that there is no formula for who should be on preventative therapy. This must be individualized based on improving quality of life.

How do I find the right doctor?

Quoting Voltaire, Prof. Zuraw explained that perfect is the enemy of good. You don’t need to find the world’s best expert; you need to find a good doctor willing to listen, learn, and treat you as a core decision-maker. Working with your doctor, you can develop and regularly update a management plan that covers which medicine, where to get it, what happens when you travel, etc.

Who can I turn to for help?

Prof. Zuraw outlined that it is vital to know that HAEi are patients just like you, who understand and work for you. It is an unbiased source of information and help. HAEi understands the need to deliver life-saving medicines to everybody in the world.

Prof. Zuraw highlighted how HAEi has worked to overcome the perspectives of others outside the community, particularly that the costs of ignoring adequate treatment are far higher than insurers and governments recognize. Effectively treating patients is a fiscally sound decision.

Is there a cure?

In the past, the idea of a cure might’ve seemed a cruel joke. However, Prof. Zuraw indicated, **“Good management of HAE leads to full control. We should see no deaths and no disruption in your life. If it leads to a normal life, I would argue while it’s not perfect, it is a functional cure”.**

Looking to the future, Prof. Zuraw outlined three possible answers to a better cure. One is gene editing to correct your DNA. The second would be gene therapy, inserting extra DNA into your cells to get you producing enough C1-inhibitor. The third would be ‘protein rescue,’ which works to get more of your existing C1-inhibitor to escape your cells.

What can I do to help?

Echoing Prof. Maurer, Prof. Zuraw mentioned registries. By participating in registries, you will ensure data is available to identify important information, such as whether people with HAE have fewer heart attacks than the rest of the population or that they have an increased risk of kidney disease.

Do you believe?

This question was for the audience. Prof. Zuraw encouraged his audience to believe. Quoting Eleanor Roosevelt, he said: “The future belongs to those who believe in the beauty of their dreams.” He felt sure that the dream of a cure would be achieved. He encouraged everyone to never settle for good enough but to keep pushing for a better future. And he hoped that he’d be able to help people get there.



“The future belongs to those who believe in the beauty of their dreams.”

– Eleanor Roosevelt

Topics raised by Member Organizations in EMEA

Members of the Scientific Committee presented on key topics of interest as identified by the EMEA region Member Organizations.



Diagnosing HAE – with and without labs – Associate Professor Jonny Peter

A/Prof. Peter outlined two main take-home themes from his talk. The first diagnosis saves lives. The second is that some basic biology can be really helpful for diagnosis.

His take-home message for the audience was: “Be an advocate for yourself. Look for clinical clues and get as familiar as you can with testing and diagnosis.”



Medical emergencies – laryngeal attacks, how do they feel and what should you do – Dr. Fotis Psarros

Dr. Psarros told the audience that laryngeal attacks are a crucial medical emergency. Common symptoms include feeling a lump in the throat, voice changes, and struggling to breathe, speak or cough.

If you suspect a laryngeal attack, please go immediately to the emergency department, Dr. Psarros stressed. It's crucial to be able to maintain a patient's airway and save a patient's life.



Accessing medications (androgens, FFP, modern therapies) – Dr. Iman Nasr

Dr. Iman Nasr outlined that modern therapies must be the goal in the long run. However, many countries don't have modern therapies.

Other therapies exist, but efficacy, risks, and side effects must be considered. Treatment options, especially long-term prophylaxis, should be individualized in collaboration with the patient.

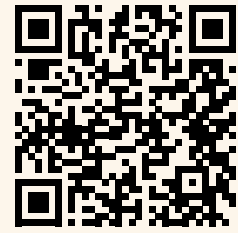
A treatment plan for patients with frequent attacks is necessary, as obtaining SDP/FFP can take time. And, understanding triggers for HAE attacks can help decide when short-term prophylaxis is needed.



“Be an advocate for yourself.
Look for clinical clues and get as
familiar as you can with testing
and diagnosis.”

– A/Prof. Jonny Peter

Read a full write-up on – scan the QR code or visit
haei.org/topics-raised-by-mos-in-emea



HAE in women, children, pregnancy, family testing – Professor Henriette Farkas

Professor Farkas told the audience that pregnancy and having a baby are wonderful, and she always encourages her HAE patients to have children. However, she stated that these patients require special care. Changes in estrogen hormone levels during pregnancy can affect HAE.

When considering children with HAE, Prof. Farkas asked everyone to remember that children are not simply small adults and discussed diagnosis, treatment, and additional support to help, for example, at school.

In conclusion, Prof. Farkas made clear that HAE patients may have a successful pregnancy and children with HAE can live happy lives.



Taking part in clinical trials – what’s in it for patients – Professor Vesna Grivcheva-Panovska

Prof. Grivcheva-Panovska told the audience that clinical trials are the backbone of medical progress. Whenever a patient takes part in a trial, they are not just adding to the greater good, but also helping themselves and their families.

Prof. Grivcheva-Panovska made clear that you should feel comfortable to ask and have all your questions answered before agreeing or not agreeing to take part. For many people the chance to contribute or give back to the HAE community is an incentive to take part.

Finally, she said: ‘Clinical trials have the potential to shape the future of the world. Please stay informed, stay engaged and together we can make a difference’.



“Clinical trials have the potential to shape the future of the world. Please stay informed, stay engaged and together we can make a difference.”

– *Prof. Vesna Grivcheva-Panovska*

The Latest Developments in HAE Science and Therapies

Taking the stage for the next to last presentation of the day, **Dr. Danny Cohn** remarked on the inspiring nature of the day and how overwhelming he had found the levels of engagement and advocacy from all the participants.

Introducing his talk, Dr. Cohn highlighted three reasons to search for new treatments: tolerability/safety issues, limited efficacy, and administration issues. Dr. Cohn made clear he understood that, for some, older treatments were all that was available. He had no wish to offend and explained these three points are the questions that drive improvement.

Recalling Prof. Maurer's talk, Dr. Cohn showed that the latest guidelines indicate five first-line treatment options, with the ultimate goal being total control of the disease and normalization of the patient's life. This means efficacy but also a lack of side effects and ease of administration. He outlined that people don't want to be constantly reminded about their condition, so infrequent administration is attractive.

Dr. Cohn told the audience that he felt that, in a way, HAE is a fortunate patient population. Of course, he acknowledged that HAE is very serious. **However, a great interest in the disease has led to new drugs.** Part of this interest, he felt, was that results come quickly. You administer a drug and soon find out if it's effective, as attacks won't appear anymore.

Showing the targets for HAE treatment, such as Factor XII, kallikrein, bradykinin, and others, Dr. Cohn then outlined some of the new types of treatment being trialed to address these targets, which were: monoclonal antibodies, small molecules, oligonucleotide antisense (ASO) therapy, gene editing and gene therapy. These potential medicines come with different modes of administration and time between doses.

Dr. Cohn discussed two potential oral options (**sebetralstat** and **deucricitibant**) for acute therapy, as existing medicines are all injectable. These can be self-administered and may be more acceptable for children. Summing up current evidence on efficacy, Dr. Cohn made clear that when a patient first experiences the signs of an attack, these drugs should be effective very quickly.

Deucricitibant is also being evaluated as an oral prophylactic medicine, and two other experimental medicines called **ATN-249** and **KV998086**. These were early in their development, said Dr. Cohn, but show the potential for more oral drugs for long-term prophylaxis.

Moving onto injectables for prophylaxis, garadacimab, donidalorsen, STAR-0215, NTLA-2002, and BMN-331 were highlighted.

Garadacimab is an inhibitor of Factor XII and a monthly injection. In trials, it led to an average reduction in monthly attacks of 87% compared to placebo. 62% of patients were completely free of HAE attacks.

Donidalorsen was described as a new way of targeting HAE, according to Dr. Cohn. It uses short strands of nucleotides to prevent the formation of a protein called prekallikrein, which is involved in angioedema attacks. Using the analogy of a recipe, Dr. Cohn said that donidalorsen acted to disrupt the chef's recipe so less of the prekallikrein is produced in the liver. In a phase-II (safety and small-scale efficacy) trial, 90% of angioedema attacks were prevented, and a second dose led to a 97% reduction in attacks. The drug was well tolerated in trials, with the latest study hoping to report results in the coming months.

STAR-0215 is a long-acting version of the currently available medicine, lanadelumab. Hopefully, this will enable extended periods between injections, even up to three or even six months.



But prolonged periods between doses is still regular medication, Dr. Cohn noted. Gene therapy and editing offer the potential for a single dose. AAV5 gene therapy (**BMN-331**) contains the DNA the body needs to produce more C1-inhibitor. There are concerns that some people may be resistant to the method of getting the DNA into the body (a harmless virus). There is also a small risk of other genetic material being added as well. Returning to his cooking analogy, Dr. Cohn likened the technique to adding more chefs into the mix, creating more of the C1 inhibitor by following the same recipe at the same time.

Moving on, Dr. Cohn explained gene editing in more detail. These potential treatments use the ‘genetic scissors’ of CRISPR technology. This permanently and

precisely targets a patient’s DNA, in effect cutting up the recipe so the body cannot make prekallikrein. In a small study of 10 patients, this treatment with NTLA-2002 resulted in a reduction in prekallikrein of 60%, which is associated with disease control in HAE. All patients were able to stop their long-term prophylaxis. As before, there is a very small possibility of causing harm, and long-term studies are needed.

Looking to the future, Dr. Cohn said: **“Beyond the goal of achieving a normal life, we can set the bar even higher; to have such effective long-term prophylaxis that you actually forget you have HAE. This is on the horizon.”**



Q&A – Panel Discussion

Dr. Danny Cohn was joined onstage by **Prof. Henriette Farkas**, one of the Scientific Committee Co-Chairs along with Dr. Cohn, and the following members of the Scientific Committee: **Dr. Isabelle Boccon-Gibod**, **Dr. Marcin Stobiecki**, **Dr. Mauro Cancian**, and **Dr. Teresa Caballero**, for the Expert Panel Q&A.

Here we present a few of the questions asked:

Can I play sports with HAE?

The group generally believed that playing sports should be possible, and when undertaken regularly, it could lead to fewer attacks. The panel discussed concerns about contact sports such as karate or rugby. Still, with the advances in on-demand treatment and prophylaxis, the panel recommended playing as much sport as possible to all patients, especially children.

I don’t want my children to have HAE; what should I do?

Speaking from experience in Spain, Dr. Caballero described the possibility of pre-implantation genetic testing to see which embryos don’t have the disease. It is not easy and is not available everywhere, but it can be successful. With the many treatments available, she feels confident that a child with HAE will have a completely different life from its parents.

The panel also highlighted that although a serious disease, HAE does not progress and never causes tissue damage. The recommendation of one panelist was for everybody to have children because of the new treatments. Treated effectively, HAE patients can live a full life.

PATIENT AND CAREGIVER TRACK



When should I start my child on preventative medicine?

The panel generally believed it should be a balance of quality of life and number of attacks. If attacks are frequent, long-term prophylaxis is important. The panel also discussed that effective treatments and data for children are still really needed.

Should short-term prophylaxis be used for cosmetic procedures such as Botox, breast implants, tattoos, piercings, etc.?

One panelist suggested being prepared when having tattoos, as there's some mechanical stress and injury that could trigger an attack. Another felt that anything injected into the lips could provoke a dangerous attack. The general advice was to make sure you have your on-demand treatment available.

Debs and Nevena were delighted to announce that HAEi LEAP would happen again in 2024!

They told the audience that young people aged 16-25 and their Member Organization should get together to discuss what's involved, what a project might entail, and how best to apply. Some examples of projects being implemented by young people include:

- A youngsters' meeting in Poland
- A youngsters' community in Australasia
- A website update in Peru
- Writing a guide for HAE caregivers in the USA
- Organizing a healthcare professional meeting in Oman
- Conducting healthcare professional research in Qatar
- Organizing a sporting/ walking event to raise awareness in Serbia

Introducing HAEi LEAP 2024

Debs Corcoran, HAEi's Chief Scientific Officer and **Nevena Tsutsumanova**, Manager, Youngsters' Community and Special Projects came to the stage to present "One More Thing", the HAEi LEAP program.

They explained that HAEi LEAP is an educational program that allows young people to learn new skills and develop as individuals and advocates. Ultimately, they told the audience, it paves the way for future leaders. The program was launched in 2023 and was amazingly successful, with 20 young people gathered in Dubai. The feedback has been excellent and has led to many exciting new projects with these young people engaged with their Member Organizations.

Our young people are the future of advocacy and will change the world. We can't wait to see what they do in HAEi LEAP 2024. Read more about LEAP 2024 on page 66 in this issue of Global Perspectives.

Closing Remarks

Henrik and Tony thanked everyone who had given so much to the incredible weekend and reminded people of the significant conference theme, **Take Action**. Everyone should return to their home countries with the motivation and knowledge that they can make a difference.

Supporters

The global HAEi family is grateful for the pharmaceutical companies that supported the 2023 HAEi Regional Conference EMEA:

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Poster Session, Friday evening

Of 26 abstracts submitted to the Scientific Committee, 19 were accepted for poster presentation, and another 5 were accepted for oral presentation, making the Scientific Track a hub for the latest data on the diagnosis and treatment of HAE.

At the meeting, 18 posters were available, and 15 were presented in a fast oral format, with each author having two minutes to communicate key information to their peers.

Read a summary of all the following posters

– scan the QR code or visit:

haei.org/emea-scientific-poster-session



1. Recognizing the Importance of Early On-Demand Treatment in the HAE Attack Journey
2. In Their Own Words – Patient Descriptions of the Earliest Recognition of HAE Attack Onset
3. Bowel Obstruction Secondary to a Small Bowel Tumour in a Hereditary Angioedema Patient: A Case Report
4. Case Study: The Very First Identified Case of HAE in Kosovo
5. Moving from Inadequate Acute Treatment and Prophylaxis to Successful Management with Lanadelumab
6. Hereditary Angioedema within a Family: Clinical Profiles, Treatment Response, and Laryngeal Edema Awareness
7. People Living with Hereditary Angioedema (HAE) Prioritize Attack-free Status as a Target for Therapeutic Efficacy
8. Real-life Experience with Lanadelumab and scpd-C1-INH Prophylaxis After Almost One Year of Follow-up in Hungarian Patients with Hereditary Angioedema due to C1-Inhibitor-Deficiency
9. Attack-Free Status Across Subgroups of Patients with Hereditary Angioedema (HAE) After 96 Weeks of Berotralstat 150 mg Treatment: Results from the Apex-S Trial
10. Early Symptom Relief Following Treatment with the Oral Bradykinin 2 Receptor Antagonist Deucricitabant Immediate-Release Capsule (PHVS416) in Patients with Hereditary Angioedema Attacks
11. Efficacy and Safety of Bradykinin B2 Receptor Antagonism with Oral Deucricitabant in Prophylaxis of Hereditary Angioedema Attacks: CHAPTER-1 Phase 2 Trial Design
12. A Retrospective Study (INTEGRATED) of Real-world Effectiveness of Lanadelumab in European Patients With HAE Type I/II
13. Is it Late-onset Hereditary Angioedema or Acquired Angioedema?
14. Real-world evidence of the effectiveness of C1-INH SC in patients with HAE in Spain and Germany
15. A Design of a Phase 3 Open-Label Study Evaluating Garadacimab for Prophylactic Treatment of Paediatric Patients (aged 2–11 years) with Hereditary Angioedema
16. A Brief Report of Immunology, Asthma and Allergy Research Institute Activities for HAE Patients in Iran
17. An Unexpected Association of a Novel MYOF Variant with Generalized Myopathy and HAE-nl-C1-INH
18. Updated safety and efficacy of NTLA-2002, a CRISPR/Cas9-based gene editing therapy targeting KLKB1, in a Phase 1 study of patients with hereditary angioedema



Co-chair Welcome, Saturday Morning

Opening the Scientific Track, the Co-Chairs of the Scientific Committee, **Professor Henriette Farkas**, **Professor Petra Staubach-Renz** and **Dr. Danny Cohn**, welcomed the more than 120 attending healthcare professionals from all across the region, from Algeria and Armenia to Ukraine and Zimbabwe.

The Young Researcher/Investigator Award was presented to **Dr. Remy Petersen** from the University of Amsterdam. Dr. Petersen discussed the award-winning research in the first oral presentation of the Scientific Track.

The audience heard plenary talks from two HAE lead experts. **Dr. Teresa Caballero**, focused on Diagnostic approaches to different types of HAE and indicated a wide range of classifications of angioedema and that diagnosis can be challenging. Dr. Caballero left the audience with a slide describing a diagnostic decision tree with a wide range of differential diagnoses and required testing at each stage.

Associate Prof. Jonny Peter focused on Diagnosing and managing HAE in low-middle-income countries. A/Prof. Peter stressed the importance of strong patient advocacy, with data showing that no on-demand or prophylaxis treatment is available without a patient support group in a country. Concluding, A/Prof. Peter said that there are considerable challenges but that the situation could be improved by; Increasing local data and advocacy, being creative in championing HAE, showing scientists the opportunities for discovery and encourage the industry to invest.

Read a full summary of their talks – scan the QR code or visit:
haei.org/emea-sci-track-welcome





Clinical Case Presentation, Voting and Discussion

The three Co-Chairs then took to the stage for the next session, the goal of which was to illustrate the hurdles physicians face and to encourage discussion.

Prof. Staubach-Renz presented a case involving a newly diagnosed patient who was pregnant. She invited participants to consider the current treatment guidelines before considering how they would approach a management strategy for the patient. Participants voted for one of a selection of potential strategies, prompting comments and discussion from the audience.

She concluded by telling her peers that:

- Pregnancy can increase, decrease, or not affect HAE disease activity. However, the increase in hormonal levels and emotional stress can cause more attacks
- HAE treatment options are limited to plasma-derived C1-inhibitor in pregnancy and lactation
- Personalized treatment and delivery plans are highly recommended and should be developed in partnership with gynecology colleagues
- Weight gain during pregnancy can require an increase in the C1-inhibitor dose.

Dr. Danny Cohn presented the case of a 46-year-old patient with HAE. A widower with three children, she was self-isolating during the COVID pandemic. During a video conference consultation, Dr. Cohn questioned the patient about an action plan and the availability of acute treatment; he was told the patient had neither. In addition, when asked about laryngeal attacks, the patient confirmed that she was having one right now during the consultation. The patient could not leave home as she cared for children and lived approximately 45 minutes from a clinic.

Asking his audience, ‘What would you do?’, he gave them options such as to go personally to the patient, make them go to the emergency room, etc.

Ultimately, Dr. Cohn resolved the potentially life-threatening situation by couriering acute medicine directly to the patient in such a way that it could be posted through the door. Dr. Cohn was then able to instruct the patient on how to self-administer during the teleconsultation.

He encouraged those present to make sure patients are aware of the dangers of their condition and have an action plan and proper treatment. He said, “We must also make sure all clinicians understand better the disease and how to act.”

Finally, in a case with sadly a less favorable outcome, **Prof. Farkas** presented a fatal laryngeal attack. The patient was a young man with a positive family history of HAE. Despite suffering from 20-24 attacks, the patient had ignored follow-ups, and compliance with therapy was poor.

She asked the assembled physicians for their views about the cause of the patient’s death; was this lack of education or patient support, for example? The questions prompted much debate and discussion with the audience. All assembled agreed that any death from an attack is one too many. Prof. Farkas then provided three key recommendations:

- Education on the course of laryngeal attack. She made clear there is “no such thing as mild laryngeal edema”
- Education on treatment strategy and that all patients should have an action plan
- Education on drugs for acute treatment. Where possible, all patients should be taught to self-administer, and drugs for the treatment of two attacks should be available at all times



“It was an amazing and very inspiring conference.”

– *Scientific Track participant*

“This opportunity will help me improve my knowledge.”

– *Scientific Track participant*



Oral Abstract Presentations

The track concluded with 5 oral abstract presentations.

(1) A Core Outcome Set for Measuring Efficacy of Acute Treatment for Hereditary Angioedema: A Global Delphi Consensus Project

The Young Researcher/Investigator Award recipient, Dr. Remy S Petersen, presented results from her recent research. The research aimed to help standardize outcome measures in clinical trials for HAE, as a literature search indicated that 72 efficacy outcome terms were used in 13 trials.

The AURORA project (Acute Treatment Outcomes in Hereditary Angioedema) sought to develop a Core Outcome Set (COS) of key measures that should be reported as a minimum in all clinical trials for acute treatment of HAE patients.

The project involved an international panel of patients, clinicians/researchers, industry representatives, and regulators. Following a series of structured discussions to find consensus, Dr. Petersen presented the final, agreed COS, which consisted of the following outcome measures:

- Change in overall symptom severity (at one predetermined time point between 15 minutes and 4 hours after treatment)
- Time to stop of progression of all symptoms
- Need for rescue medication during an entire attack
- Impairment of daily activities
- Treatment satisfaction

Dr. Petersen thanked all participants and her fellow authors and stressed further research should determine the best instruments to measure the agreed COS outcomes.

(2) Efficacy and Safety of the Oral Bradykinin B2 Receptor Antagonist Deucricitibant Immediate Release Capsule (PHVS416) in Treatment of Hereditary Angioedema Attacks: Results of Rapide-1 Phase 2 Trial

The research investigated a new oral medicine for treating HAE attacks, as existing injection-based medication could mean that treatment of attacks is delayed or doesn't occur.

Presenting the results and conclusions of the research, Prof. Markus Magerl outlined that deucricitibant had been trialed by 74 patients from 13 countries. Successfully reaching all primary and secondary trial endpoints, Prof. Magerl concluded that deucricitibant, in an immediate release capsule for on-demand treatment of attacks, demonstrated rapid onset of action, symptom relief, and resolution of HAE attacks. It substantially reduced the use of rescue medication and was well tolerated at all the dose levels tested in the study.

The authors felt that the trial results support further developing deucricitibant immediate-release capsule as a potential on-demand treatment for HAE attacks.



“Thank you for your work and contribution to the development of knowledge and skills of this disease. Life has become easier, and dreams come true!!!”

– Patient/Caregiver Track participant

(3) Endothelial Related miRs are Dysregulated in C1-INH Angioedema Patients: New Potential Biomarkers and Therapeutic Targets

Dr. Maria Bova presented new potential targets for future treatments based on a growing understanding that angioedema is a disorder of the endothelium -the tissues that line organs and cavities in the body, including blood vessels- which can be a sign of the severity of HAE. Dr. Bova and colleagues aimed to use this knowledge to evaluate whether the body producing a particular kind of short, simple genetic code, called miRNAs, indicates endothelial function in a population of C1-INH HAE patients.

In a small study of 12 patients, Dr. Maria Bova presented results which, she concluded, showed that:

- There is a specific miRNAs signature in patients with HAE
- These miRNAs represent a potential new way to monitor and treat the endothelial function in people with HAE
- One specific type, called miRNA-486, could predict angioedema severity

(4) « BESQAOH » : A National Survey for Assessment of Health Status, Quality of Life and Expectations of French HAE Patients

Dr. Isabelle Boccon-Gibod presented research into the health status, quality of life, and expectations of people with HAE living in France. 158 patients were reached with an online questionnaire that asked about their disease activity, what treatments they used, and their quality of life.

In conclusion, Dr. Boccon-Gibod made clear that:

- New treatments, especially long-term prophylactic treatments, seem to have been adopted by HAE patients and have led to a good level of satisfaction, with 85.3% of patients being satisfied with treatment
- Comparison with an earlier study from 2013 indicates that the quality of life for people with HAE in France has gone up since the availability of new HAE treatment
- Patients who completed a program of education reported improved ability to self-manage their HAE treatment and told the authors that it had improved their daily life

(5) Prevalence of Comorbidities Among Hereditary Angioedema (HAE) Patients in North Macedonia

Prof. Vesna Grivcheva-Panovska gave the final oral presentation. She focused on other conditions and age-related diseases that people with HAE may suffer from, given that these patients may be living to an increasing age due to improvements in on-demand and preventative treatment. The authors also considered the impact and interplay of multiple medicines for HAE and other conditions.

Investigating 35 HAE patients registered on the National Registry of Rare Diseases, with a control group from the general population, the authors compared deaths, cancer rates, and medical prescriptions.

The findings included:

- The HAE patient group had a higher prevalence of cardiovascular disease and a higher rate of elevated blood cholesterol
- Other frequent disorders in people with HAE were anxiety, gastrointestinal disorders, depression, and high blood pressure

Prof. Vesna Grivcheva-Panovska concluded that despite HAE patients receiving regular health checkups, there is a growing need for more awareness and proactive action to prevent cardiovascular disease, strokes, and other issues related to blood clots. It is also crucial to exercise caution regarding potential side effects and adverse reactions from using medications alongside prescribed HAE treatment.

“

“I also got a bit emotional because it was so nice to be able to talk to people with the same experience. Thank you HAEi for organizing this amazing event!”

– *Youngsters Track participant*



This is the HAEi Youngsters' Community!

Friday evening

Following the Keynote Presentation, the unique HAEi Youngsters Community split from the main patient and caregiver track, 60 young people and caregivers from 39 countries across the EMEA region joined together for networking, education, and fun!

The Youngsters' Advisory Group (YAG), comprised of 8 incredible young advocates, was critically involved in the planning and delivery of the program in the Youngsters' Track.

Speaking before the start of the session, **Nevena Tsutsumanova**, Manager, Youngster's Community and Special Projects, told us of her admiration for these young people: "I'm looking forward to this conference shining a light on the amazing work of this Advisory Group. I'm beyond grateful to work with these eight amazing individuals."

The opening discussion in the Youngsters' Track started with a question about how many youngsters were at a conference for the first time, with most present saying they were. The rest of the hour was a chance to introduce all the Youngsters' Track offers to people living with HAE and their caregivers.

Saturday morning

Opening the second session was an informal get-to-know-you session where young people could share thoughts, feelings, and experiences about their disease.

One of the YAG members, **Isabel**, told us that being at the event was a chance to connect with others. She said: **"You just walk into the room and sigh in relief because everyone knows there's nothing you have to keep hidden. Everyone knows what you're going through and has been through similar."**

Another YAG member, **Hana**, said that these conferences were amazing for her: "Youngsters are able to make friends much easier because they have something huge in common. The first conference I came to, we just made jokes about our swollen hands and looking like crazy people, but we loved it, had a huge laugh, and became friends."

Introduction to LEAP and the Successful Class of 2023

Nevena gave an update on the first full year of the HAEi LEAP program and the successful class of 2023. She encouraged everyone between 16-25 to speak with their Member Organization if they were interested in getting involved and stressed the valuable education opportunities offered to young people with HAE.

Face2Face with an HAE expert – Dr. Hilary Longhurst and Dr. Marcin Stobiecki

Next, the program moved on to a highly anticipated session: the chance to have their burning questions answered by an HAE expert.

The Youngsters' Track split into two broad age groups to accommodate a wide range of questions. For the group with **Dr. Longhurst**, there was an interesting shift to questions about the future, being an adult, having a relationship, starting a family, and treating children with HAE. We are happy that the young people felt comfortable asking all these questions.

The session with **Dr. Stobiecki** focused on understanding HAE, what is happening with your body, triggers, and how to prevent/treat your attacks.

We caught up with Dr. Hilary Longhurst after her session with young people. We asked her about the questions she commonly gets asked and how she responds. She told us: "The main questions from young people were about wanting to both work and study abroad, and travel. Ten years ago, that question would be met with disbelief. But now the answer is, of course, how can we make it happen? It's very good to see."

The whole Youngsters' Track thanked Dr. Hilary Longhurst and Dr. Marcin Stobiecki for their participation and for creating a safe space for the youngsters.

Get to Know Each Other/Networking

As part of the networking, we shared a series of motivational postcards. The young people took the idea by heart and not only exchanged cards but also left some of them behind to be digitized and made available for the global community. You can find them on youngsters.haei.org under 'community postcards.'

Find Your Strength and Hold It Up: Panel on Selfcare and Wellbeing

One of the new sessions of the Youngsters' Track was a self-care and wellbeing panel discussion with EMEA youngsters and members of the YAG. **Hana** told us, "We've been working together on this conference so hard we really feel a part of it. It feels like it's bringing community one step further."

It was the first time some presenters shared their experiences, and the session's goal was to encourage the audience to share their experiences, too. The setting was relaxed, with an open floor for discussion and 7 panelists happy to share their thoughts on the two topics.

Faye Marshall, a clinical nurse working to support people who have conditions that are managed rather than can be cured, hosted the panel. She introduced her contribution by telling us: "I am used to helping people navigate their experiences and helping people to feel heard, seen and exploring emotions in a safe way. I will be the treasure hunter for the discussion, and there's lots of treasure amongst these wonderful young people."

The level of audience engagement was extremely high, with lots of enthusiasm and questions for the panel.

One panel member described how these sessions help: "empowering young people to see that they can take control of their HAE by being more involved. We try to show them they can have control over this part of their life."

Another hoped that the impact of the whole meeting would be similar to: "the sensation I got walking into my first international conference, just having that community and knowing that there are people out there that will support you when you need help."

Sharing her perspective, **Jess** told us: **"Before we had good medication, I never saw myself leaving Australia. Suddenly, I'm in Germany, halfway around the world. It's the sense of freedom and liberation to change the way I thought my life would go."**

Summing up the value of the session and the Youngsters' Track, Faye said: "Young people have their whole life ahead of them. People with HAE should be able to embrace and celebrate their life like everybody else."

Find Your Strength and Hold It Up: Creative Workshop

The following session got the youngsters to use creative writing to process and share their experiences, connect, and recognize their resilience.

Faye took the youngsters on a quick trip through poetry as part of a beautiful 30-minute workshop. She promised everyone that by the end, they would all be poets. We asked Faye why poetry and creative writing was so important. She told us: "Creative work helps people express their feelings in a less conventional way. Poetry is a wonderful way to connect with yourself to become your own friend. There's immense therapeutic benefit in expressing yourself."

Youngsters dove into the art of words and started expressing their feelings after only 15 minutes of exercises and associations. They then spent the final 15 minutes writing a poem together. The thought of 60 people writing one piece of poetry together sounds overwhelming to many people, but they made it happen.

The hope is that the poem the youngsters created, reproduced on the next page, will become an anthem of the HAEi Youngsters' Community and a symbol of how it supports everyone.

Wrap Up the Youngsters Track

As she wrapped up the Youngsters' Track, Nevena asked for a show of hands. "How many of you will join us next time she asked?" Every hand in the room went up, indeed a positive sign.

Some participants also gave messages to those unable to attend this meeting. Faye told us she would tell young people not present: "Always be ready for possibilities."

From the YAG, the inspiring message was: **"Don't be shy to reach out. Even if you can't attend a conference, there are plenty of ways to get involved: we have online hangouts and lots of social media engagement. You don't have to do it alone. You've got this."**

In conclusion, Nevena thanked all the participants and presenters for their immense contributions. She told the young people that a similar conference is soon to take place in the Americas and that there are huge plans to build on the success of these regional conferences.

*Please note that the poem explores emotionally sensitive themes, including compassion, kindness and resilience.
You may wish to consider this before reading or sharing.*

Joy is beautiful
But nothing more than a feeling
To turn me into gold
I strive for freedom, I seem electric
I recognize it from the stars

I can see the growth of the future
To shine, be brave, and stay safe
To surprise myself with scenic adventures
To take authentic breaths
I will be wonderful

I hope that all the flowers will flourish
I hope to have new friends
I need to make connections
I hope I don't let anything stand in my way
And make my parents proud of me

I wish life was more certain
Yet, I am resilient
My dream is to prove my younger self wrong
A time to imagine
In clear perspective

The present is the one and only gift
To call upon our creative side
Allow the light and the dark to protect
The brightness of us
And make a difference

Kindness is a soft art
We are all human, yet so different
We have the privilege of sharing our stories
We are a family
We are not alone



UPDATE FROM HAEi'S EXECUTIVE VICE PRESIDENT GLOBAL ADVOCACY AND CHIEF DIVERSITY OFFICER, FIONA WARDMAN

Advocacy is an essential tool to drive change. In my new role as Executive Vice President of Global Advocacy and Chief Diversity Officer for HAEi, I aim to make a positive impact on the current HAE situation by shaping the organization's global influence, advancing its advocacy agenda on a worldwide scale with strategic leadership, and managing international advocacy efforts and initiatives.

For those of you at the recent **2023 HAEi Regional Conference EMEA**, you will have heard more about our strategy to think global and act local. My role will be to bring that to life for all of our stakeholders. There is a lot of work to do, and I am excited by the opportunity to increase awareness and access to modern therapies for people living with HAE.

What is the key to improving people's lives with HAE so they don't need to suffer for most of their lives? The solutions are:

- Targeted local and global awareness programs, education, awareness
- Locating patients in all countries around the globe
- Securing access to and reimbursement for modern therapies is the key to a better quality of life for everyone living with HAE.

In order to make those a reality, every day, I will work to improve the lives of people with HAE by:

- Ensuring that HAEi's advocacy strategy and global objectives align with the organization's mission and goals
- Key to this will be listening to the needs of Member Organizations, people with HAE, caregivers, and physicians/scientists
- Planning, developing, and implementing advocacy campaigns, guiding HAEi's external communications
- Working with the Director, Regional Patient Advocate Program, and the Regional Patient Advocates, the backbone of HAEi, to pinpoint gaps and unmet needs
- Implementing solutions to impact each country by increasing awareness of HAE to the broader

community with the goal of patients having access to reimbursed modern treatment options. Partnerships and facilitating conversations with rare disease groups, pharmaceutical companies, physicians, and health ministries are key.

- Monitoring the impact of our RPA team globally with internal and external stakeholders, international developments, and identifying opportunities to influence policy and regulation change.
- Providing leadership, guidance, support, and accountability to integrate diversity and inclusion within HAEi, to ensure those from diverse backgrounds can thrive and contribute to HAEi's success

I know that I cannot achieve all of that alone. I'm excited to work with Jørn Schultz Boysen, HAEi's Executive Vice President Global Operations and Chief Compliance Officer, on new tools and resources and implement them to assist our HAE community and Member Organizations in their advocacy efforts. I'll be supported by teams responsible for executing HAEi's efforts worldwide. And most of all, I need you.

I look forward to working with all our HAE friends and partners around the globe to put plans in place.

I can't wait to get started!



UPDATE FROM HAEi'S EXECUTIVE VICE PRESIDENT GLOBAL OPERATIONS AND CHIEF COMPLIANCE OFFICER, JØRN SCHULTZ-BOYSEN

Great to see so many people at the **2023 HAEi Regional Conference EMEA!**

For the past year (and longer), preparations have gone into the **2023 HAEi Regional Conference EMEA**, in Munich, Germany. And after such a long preparation period, it was finally showtime!

Being part of the Global Operations team, a lot of effort went into planning and executing the conference. This means everything from booking rooms and working with the conference venue and the AV team to communicating around the conference.

If you've engaged at all with HAEi before, during, or after the conference, then it's likely you have dealt with our operations team. Here's a snapshot of the team's work:

- Handling Travel Grants and registrations – as well as the online platform to capture these
- Booking flight tickets
- Preparing the three tracks (patient/caregiver, youngsters, and scientific tracks)
- Producing and packing all the materials for the conference (stage material, program, hand-outs, banners, brochures, posters, etc.)
- Developing online tools such as the conference app and links to Wordly for translations
- Post-conference survey, and of course
- Running the show with the right presentations at the right time to ensure the conference delivered the biggest impact amongst the participants.

In short, it's been a massive job, and I am proud of the team's effort to make the conference a huge success. We welcomed 650 participants; patients and caregivers, physicians/scientists, young people, and industry representatives. We aim to connect people and ensure we all have somebody we can go to with our HAE. As Professor Maurer said, "No patient is left behind"!

With one conference ending, our work begins on the next: The 2024 HAEi Regional Conference Americas is

from 15-17 March 2024. And we continue to provide a range of exciting and extremely helpful tools that HAEi can offer both local Member Organizations and patients and caregivers. You can hear lots more about these in the write-up from the **2023 HAEi Regional Conference EMEA**, but I want to call out a few here:

- Apps for iOS and Android: **HAE TrackR** – your electronic HAE diary, including a reminder functionality for prophylactic treatments. **HAE TrackR** will keep your data safe! **HAE Companion** – your pocket-size travel partner. Store the emergency card on your smartphone in the language of the country you are in. And know exactly where to find help if an HAE attack occurs.
- For Member Organizations: Free **hosted websites** service and **HAEi Connect**, the free and secure (and EU GDPR compliant) membership database from which you can also communicate with your members. No more Excel sheets on a computer that can crash!
- And **HAEi Advocacy Academy**, where our Member Organizations, patients, and caregivers can find a wealth of information and learning a lot of topics.

Also, a big shout out to all HAE youngsters (patients and caregivers) between 16 and 25: Look out for the **HAEi LEAP 2024** educational program! We invite you to apply for a place in next year's LEAP class. Speak to your Member Organization about a project that you can work on to benefit your HAE community.

You're reading Global Perspectives, but please encourage your family and friends to sign up for the magazine on our website: haei.org.

The show now moves on from Munich to Panama City. Thank you to everyone who made the recent conference so amazing, especially the supporters, for making it possible. We can already feel the impact of so many patients and caregivers, physicians, and industry representatives being together under one roof. We want you to 'Take Action' on the HAE challenges and opportunities. Here's to the next one!



**HAEi
REGIONAL
CONFERENCE
AMERICAS
PANAMA CITY
15-17 MAR 2024**

TAKE ACTION



Welcome to the 2024 HAEi Regional Conference Americas

HAEi looks forward to welcoming people from the Americas – North, Central, and South America regions – to the upcoming 2024 HAEi Regional Conference Americas, which takes place in Panama City, Panama, from 15 to 17 March 2024.

Designed to fulfill the needs of the entire HAE community, the conference features a Patient and Caregiver track, a Youngsters track (12-25 years old), and a Scientific track. Prior to the conference, the detailed programs for each track will be finalized and available at the conference website at americas.haei.org.

This conference is the third of HAEi's regional conferences and HAEi expects to welcome around 450 HAEi friends (including patients, caregivers, HAE physicians/scientists, and industry supporters) to share experiences, meet others in the region, have fun, and learn how to improve quality of life for people with HAE.

We look forward to welcoming you to the 2024 HAEi Regional Conference Americas in Panama City, Panama, from 15 to 17 March 2024.

Register now!

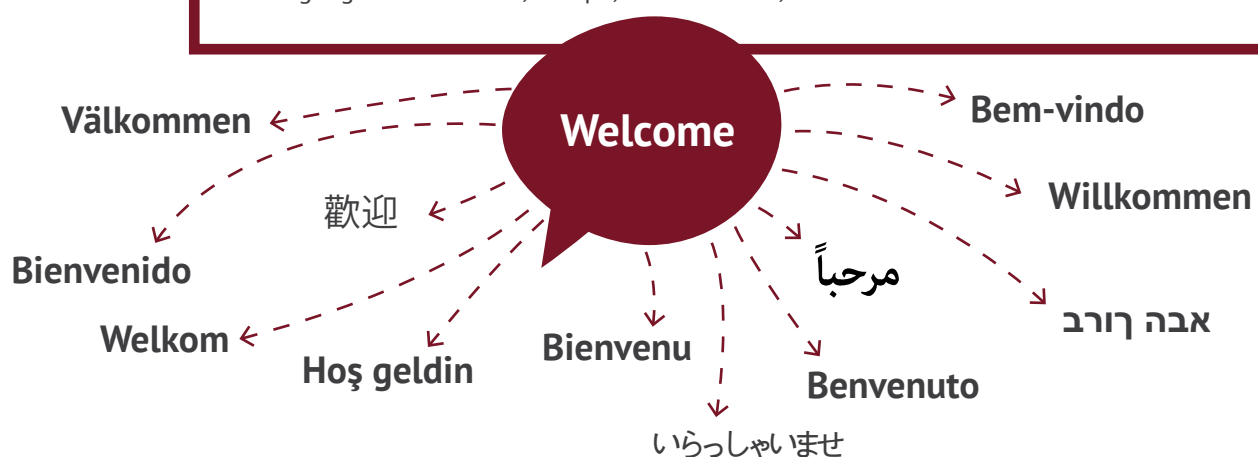
Registration for the 2024 HAEi Regional Conference Americas is on a first-come, first-serve basis. We offer extremely attractive registration rates and conditions, so we advise you to register as soon as you can!

Please register at americas.haei.org/registration

Wordly – Interpretation options: Bring your headphones!

We will offer translation during our 2024 HAEi Regional Conference Americas using our external partner Wordly and its AI-powered translations.

Wordly offers real-time simultaneous interpretation into 40+ languages and supports over a dozen speaker language options. That equates to over 1,500+ language interpretation pairs! Wordly doesn't limit you to translating from English, as it covers the most common business languages used in Asia, Europe, Latin America, and the rest of the world.





HAEi GLOBAL LEADERSHIP WORKSHOP COPENHAGEN 3-6 OCT 2024

SAVE THE DATE!

Following the success of the first ever HAEi Global Leadership Workshop in Frankfurt, Germany, in October 2022, HAEi is thrilled to announce the date for the next workshop:

2024 HAEi Global Leadership Workshop

(covering all HAEi member countries)

3-6 October 2024 in Copenhagen, Denmark

Almost 500 participants representing +75 countries participated in the **2022 HAEi Global Leadership Workshop**, which featured almost 1,000 minutes of talks, presentations, and interactive sessions across the Member Organization Lead and Scientific Programs.

HAEi looks forward to welcoming Patient Advocacy Leaders of HAEi Member Organizations, HAE Physicians/Scientists, HAEi Youngsters' Advisory Group, and industry sponsors to the upcoming **2024 HAEi Global Leadership Workshop** and recommend you save the date for this event.



2023 US HAEA National Summit - Living Beyond Boundaries

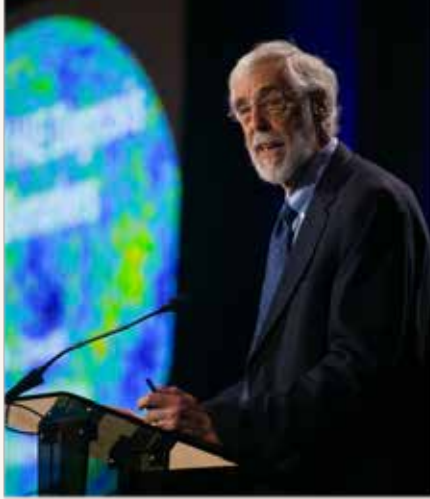
We are all familiar with the enriching conversations, valuable educational content, and unforgettable memories that unfold when a significant gathering of HAE community members takes place, and the 2023 US HAEA National Summit was no exception. For the first time in four years, the US HAEA was honored to host our HAE community in Orlando, Florida, for the 2023 Summit.

This event attracted over 1,200 HAE friends, along with caregivers, physicians, healthcare professionals, industry partners, and devoted volunteers, all congregating at the beautiful Rosen Shingle Creek Resort. Staying true to our steadfast mission of catering to the diverse and unique needs of our HAE community members, this summit featured several exciting tracks. Among them were main general sessions, a Professional-Scientific track, a Youth Health Management and Advocacy track, a Youth Leadership track, a Brady Cubs track (ages 5-7), and a Brady Bears track (ages 8-11).

The dynamic two-day general summit sessions proved to be an invaluable opportunity for members to not only connect after four long years, but also take advantage

of the comprehensive offerings of educational presentations that were intricately woven around the summit's overarching and inspiring theme, *Living Beyond Boundaries*. The General Summit commenced with a heartfelt introduction and opening remarks by Tony Castaldo, HAEA's President & CEO. He passionately urged the community to stand together with the HAEA in their ongoing efforts to champion and advocate for individuals affected by HAE.

"If we stick together and continue unleashing the magical superpowers that are sparked by an active and united HAEA community, it will be easy to continue protecting what we have already achieved while enabling future progress.



So today, here in this room, let's make a promise that we will continue to be part of the HAEA community and invoke our magical superpower to fight the good fight. Because, dear friends, we NEVER EVER want to hear anyone in the community ever say...why can't someone help me," Tony Castaldo said in his Keynote/Welcome Speech at the 2023 US HAEA National Summit.

The audience experienced a range of emotions as they witnessed a blend of heartfelt patient stories and informative industry and physician presentations, offering a sincere glimpse into the remarkable progress our HAE community has achieved together over multiple decades. The general sessions continued to explore highly informative topics like recent advancements in HAE research, treatment options, diagnosis, and discussions on grassroots advocacy. Additionally, two highly-anticipated physician Q&A sessions effectively bridged the gap between medical expertise and questions from the community. Also, of particular importance was the exploration of the future of HAE, with a focus on the treatment prospects for pediatric patients, individuals with Normal C1-Inhibitor, and members of our community transitioning to Medicare, underscoring the HAEA's commitment in ensuring that no one is left behind.

The youth health management and advocacy track started on Thursday, July 20th, with informative sessions

on how to care for your mental health and wellness while managing a chronic illness. On Friday, the young attendees participated in a workshop to better understand how they can participate in legislative advocacy to protect access to HAE medications. At the end of the advocacy workshop, youth leaders took the initiative to write over 60 letters to their legislators in Congress asking them to support the Safe Step Act, which would protect people with HAE from dangerous fail-first policies.

The youth leadership track provided ample opportunity for our youth participants to spread HAE awareness by drafting their own HAE awareness campaign and giving back by making gift boxes for *Give the Kids the World Village*. Attendees worked together in teams to create care packages for children living with chronic illnesses. Each care package included an remote controlled Race Car, a felt tie blanket, and a superhero cape. It was amazing to see everyone work together as a team to create these projects. More importantly, with the care packages created, we brought smiles to 12 kids who received a reminder that they are not alone!

As we wrapped up our experience at the end of the day, we came together for an open mic session that provided everyone with an opportunity to listen to their peer's stories and their experiences with HAE. Youth attendee, Jasmeen, said, "It felt wonderful to get up and

share my thoughts and feelings. The activity helped me to experience a genuine and meaningful connection with my fellow youth community members.”

The Brady Cubs and Bears enjoyed several exciting and engaging activities throughout their program, including a visit from Brady Bear and Dr. Raffi Tachdjian to answer questions they may have about HAE. The Brady Cubs and Bears also learned about hosting a cookie pop-up event from a fellow kid cookie and fundraising expert, Dana, who founded Cookies4Cures in 2017. After collaborating with Dana and the pastry team from the Rosen Shingle Creek Resort, the kids held a Cookies4HAE fundraiser at the summit welcome dinner and raised funds for HAE research.

The Professional-Scientific Track drew an audience of nearly 200 healthcare professionals, physicians, researchers, and scientists. Led by the HAEA Medical Advisory Board, and enriched by insights from Copenhagen Economics researcher, Nikolaj Siersbæk, the sessions were thoughtfully curated to encompass a wide range of key topics. These topics included updates on diagnosing HAE and crucial diagnostic biomarkers, emerging treatments, and the development and validation of a new HAE Quality of Life (QoL) Instrument. Interactive panel discussions within the track fostered dynamic idea exchanges, addressing significant considerations in treatment decisions, the care of women and pediatric patients, and the management of HAE with Normal C1-INH. Furthermore, the track featured an engaging poster-viewing session, showcasing a total of 28 posters that highlighted the latest advancements in HAE research.

Following two days of general sessions, the HAE IN-MOTION® event provided a chance for community members to come together, get active, and socialize following four years of separation. From leisurely walks along the hotel's beautiful nature trail, to playful lawn games and an ice cream stand, attendees had a chance to unwind and enjoy the outdoors, all while celebrating their unique HAE journey! Overall, this event was an opportunity for participants

to reconnect and share experiences that bind the community together. Over 1,000 participants registered to participate in this event. It was an inspiring display of awareness as the HAE community filled the majestic nature trail wearing their HAE IN-MOTION® T-shirts. Following the walk, the resort's hallways were bustling with HAEA community members, their faces radiating not only from the Florida July sun but also with gratitude and a newfound sense of empowerment.

The 2023 US HAEA National Summit ended with an unforgettable evening dedicated to appreciating and recognizing caregivers for their commitment to the HAE community. Dr. Jennifer Hartstein, a nationally recognized child, adolescent, and family psychologist, presented tips on how caregivers can maintain a healthy and balanced perspective and lifestyle while supporting loved ones managing a chronic illness like HAE. The evening was undoubtedly filled with heartfelt appreciation and the sharing of experiences among caregivers, creating a sense of empathy and understanding. Dr. Hartstein's expertise added a professional dimension to this dinner, offering caregivers guidance on how to navigate the unique challenges they face while caring for individuals with HAE. Following the presentation, attendees celebrated the event's conclusion with karaoke and live music. Meanwhile, the youngest members of the HAE community enjoyed a Disney-themed dinner and movie night, with special guests, Mickey and Minnie. This memorable evening served as a perfect ending to another successful HAEA National Summit!





Postcards from Munich and Beyond!

By Nevena Tsutsumanova, Manager, Youngsters' Community and Special Projects

Phew, it has been a whirlwind of activity in the Youngsters' Community.

We've just waved goodbye to our friends with the conclusion of the **2023 HAEi Regional Conference EMEA**. With 60 young people with HAE and caregivers jetting in from 39 countries, it was a great event. You can read all about the fun we had and what we learned in the conference round-up in this edition of Global Perspectives.

But it hasn't only been about the conference. There's been loads happening to strengthen our community. Our latest project is postcards. When your life demands an extra dose of encouragement, these little reminders are the HAEi Youngsters' Community offering a friendly voice saying, "You've got this!"

INTRODUCING THE COMMUNITY POSTCARDS: BE THE HAE HERO

With the HAEi Youngsters' Advisory Group, we've crafted six postcards designed to resonate with the global Youngsters' Community. Each postcard carries a message, and we are thrilled to share them with you and get you involved.

In a world brimming with heroes, we've come to understand that heroism knows no bounds. It surpasses age and size, and it's the extraordinary superpower within us that sets us apart. With a lot of enthusiasm, we

present the community hero postcards, each designed with the heartfelt intention that they will instill our members with newfound strength and determination.

You can choose your superpower – the power to uplift, inspire, and connect. Your words have the potential to transform lives. So, grab a postcard from our youngsters' webpage, download it, and write your personal message. Your words are an unlimited canvas, but you might:

- Write a message to your younger self, a chance to share wisdom from your journey
- Pen a message to your former undiagnosed self, offering comfort and hope
- Craft words of encouragement for a younger sibling, fostering their dreams
- Share inspirational quotes and motivational messages, serving as a light of positivity

Alternatively, you can spread joy and inspire your friends in the HAE community by emailing your message to youngsters@haei.org. We promise to transform these heartfelt messages into digital masterpieces and showcase them on the youngsters' webpage. Together, we can brighten every day with the power of words.

Whatever you do, join us in celebrating the beauty of HAE heroism, connection, and inspiration. Let your words be the force that brings a smile!

Still haven't joined the HAEi Youngsters' Community?

The community is free and open to youngsters aged between 12-25 who are members of their local Member Organization! Head over to our website: youngsters.haei.org, and click the "Join us" button – it is green and right at the top.

100
**JOIN THE
COMMUNITY
TODAY**
sometimes we have cookies! 🍪





Be the HAE hero!

Grab a postcard from our youngsters' webpage, download it, and write your personal message – your words have the potential to transform lives!

Choose your super power: youngsters.haei.org/community-postcards

NEW!
Youngsters'
Community
Postcards



HAEi LEAP 2024 is now open for applications!

*After an incredible success of LEAP 2023, HAEi announced the launch of LEAP 2024 at the **2023 HAEi Regional Conference EMEA**. We are very excited to let you know that LEAP 2024 is now open for applications!*

WHAT IS HAEi LEAP?

HAEi LEAP is an online educational program, developed by HAEi, that allows young people to learn new skills and develop as individuals and advocates.

Young people will also have the chance to gain experience working with their MOs to apply all those skills on a project to support advocacy, in their member organization, and their community. Paving the way for future leaders.

WHO IS LEAP FOR?

HAEi LEAP starts with a 3-day in-person seminar in mid-April 2024, followed by a 14-week program of online and virtual learning, and it's for youngsters in the global HAE community. They should be:

- Someone with HAE – a caregiver or a family member
- Aged between 16 and 25
- A member of your organization (of course!)
- And able to travel on their own

WHY CHOOSE HAEi LEAP?

LEAP offers a dynamic learning experience. Our online courses and practical exercises cover content planning, online content management (web and social), presenting, strategy, and more. As a LEAP graduate, the youngster will have the unique opportunity to put all those new skills into practice by collaborating with the HAE Member Organization real-life project that supports advocacy.

HAEi will provide a one-time monetary grant to the Member Organizations of successful LEAP graduates to support the project agreed upon with the youngster.

By joining LEAP, the youngster will become a part of a class of like-minded young individuals and organizations dedicated to advocacy and growth.

LEAP WEBSITE AND APPLICATION PROCESS ARE NOW OPEN

In the LEAP section of the HAEi Youngsters' website, you can find the important dates and the application process and draw inspiration for a project from our idea bank:

- **A key date for your diary is 12 November 2023**, when the application process closes.
- **Places are limited**, so if you want to apply, please ensure this reaches us before the deadline.
- **How to apply:** The Youngster needs to complete an individual application and the Member Organization needs to complete a separate form to support the youngster's application.

If you have any questions, you are always welcome to contact the LEAP team, Nevena Tsutsumanova and Debs Corcoran, at leap@haei.org

To learn more about LEAP and apply, visit youngsters.haei.org/leap-welcome-program



HAE Companion – Your Pocketsize Travel Partner

HAEi's app, HAE Companion, is developed to make travel and life in general easier for people with HAE and their caregivers.

- Download HAEi's digital emergency cards for the locations you are going to visit.
- Find contact information on HAE knowledgeable hospitals (including ACAREs) and physicians worldwide.



Download on the
App Store

apple.co/33Qn4ZK



GET IT ON
Google Play

bit.ly/3osxkzm

NEWS FROM HAEi COUNTRIES AROUND THE GLOBE



CZECH REPUBLIC

From HAE Junior

HAE juniors' families joined the fourth educational summer camp. The fourth edition of the HAE Junior's summer camp is successfully over! Let's take a look back at the four days we spent together with HAE children & teenagers from all corners of the Czech Republic.

A variety of educational, sporting, and experiential activities. This year's program offered learning experiences, sporting, and educational activities – including the favorite HAEro Games – as well as expert lectures and group discussions on topics that are currently relevant for HAE patients. Important updates and valuable know-how were shared, among others, by Radana Zachová, MD, from the Department of Immunology at the University Hospital in Motol, Michal Rutkowski, Vice President of HAE International, and René Brečťan, Vice President of the Czech Association for Rare Diseases.

There was a high interest in topics such as the development of HAE awareness in the Czech Republic, access to orphan drugs legislation, and patient advocacy. Parents of HAE juniors also appreciated practical tips and recommendations on traveling with HAE, healthy diet and lifestyle, and so on.

In addition, the participants were offered the latest printed leaflets, "HAE and travel", posters for health professionals, or the Czech translation of the children's story "The Rare Boy and the Talkative Little Balloon".



The 4th HAEro Games have their winners. The juniors enjoyed a rich program that tested their knowledge, as well as creative and sporting skills & talents. What they liked the best was the conversation and workshop with paramedics who visited us by ambulance and the traditional multidisciplinary HAEro Games competition, which became the highlight of the stay. As every year, the participants received their well-deserved prizes – HAEroic cups and diplomas.

Acknowledgments and tHAEnks! We want to say tHAEnk you to all the speakers, participants, and partners whose contributions enabled us to hold the educational family camp in 2023. The event was financially supported by the following partners: CSL Behring, Takeda, KalVista, Pharming, Sdružení pro plazmaferézu, Amberplasma, and other individual donors.



IRAN

From Parichehr Bahrain, HAE Iran

HAE International Day (16 May 2023) in Immunology, Asthma and Allergy Research Institute, (IAARI) Tehran University of Medical Sciences, Iran

On the Hereditary Angioedema (HAE) International Day, IAARI organized a webinar about the diagnosis and management of HAE, in which allergists, clinical immunologists, emergency medicine specialists, and biomedicine experts had lectures about HAE.

The webinar covered updates on the diagnosis, prevention, and management of HAE to raise awareness among healthcare providers all around the country.

The speakers had lectures about challenges in the diagnosis and management of HAE, a brief report on the HAE registry in Iran, and updates on HAE diagnosis and management.

The duration of the webinar was 4 hours, and about 150 physicians, pharmacists, and other healthcare providers participated.

The webinar had a continuing medical education score for general practitioners, allergists, pediatricians, internists, immunologists, pharmacists, biomedicine, and genetic experts.



Also, on May 19th, 2023, the Immune Deficiency Patients Advocacy Association (IDPA), in collaboration with IAARI, held a webinar in respect of the international hae day :-), just as they did last year. This event aimed to raise awareness among HAE patients and their families and improve their quality of life. An allergist and clinical immunologist explained the proper control measures for the disease, as well as the importance of family screening for unknown cases. At the end of the program, patients asked their questions and were provided with guidance in the session.



SERBIA

From Jovana Cvetković Lazić,
President, HAE Serbia

Empowering HAE Advocacy: 'Doctors in Purple' Project Shines Light on Early Diagnosis

In collaboration with esteemed medical professionals, HAE Serbia has recently unveiled the impactful 'Doctors in Purple' project. This initiative aims to raise awareness and enhance the understanding of hereditary angioedema (HAE) diagnosis.

We extend our heartfelt appreciation to the doctors who have joined this noble cause, marking a significant step towards improving understanding and facilitating timely treatment of this rare genetic disease.

This initiative holds profound significance for all of us at HAE Serbia, as it aligns with our commitment to illuminate the path to diagnosis and provide unwavering support to HAE patients. It brings us great joy to welcome medical experts who will join us in our mission.

Through our collaborative efforts with doctors, our association aims to amplify visibility for hereditary angioedema, heighten public awareness, and ensure that patients receive prompt diagnosis and support.

The 'Doctors in Purple' project emphasizes the critical importance of recognizing early symptoms during the uncertain and anxious period preceding a diagnosis. Through this initiative, we aspire to give a voice to all HAE patients still grappling with undefined symptoms and diagnostic challenges.

The 'Doctors in Purple' project has garnered an exceptionally positive response, outfitting participating doctors with thirty-one purple uniforms and lab coats. HAE Serbia remains committed to sustaining this initiative, hoping more doctors join this significant endeavor.

We cordially invite all doctors and medical professionals to unite with us in this project, contributing to enhancing the lives of HAE patients.





THE NETHERLANDS

*From Maria (Marijk) Beekman-Kortekaas,
President HAE the Netherlands*

We're back! For years, HAE the Netherlands has not been very active. A year ago, a new board took over, and we started to rebuild the organization.

A lot has changed since then. We became more active on social media and launched our Instagram account: [instagram.com/hae.qenl](https://www.instagram.com/hae.qenl). This is an excellent tool to stay in touch with the international HAE community and to share news and information with HAE patients in the Netherlands. Instagram is also very helpful in keeping in touch with the youngsters in our country. We are in the process of establishing a youngsters' community.

One of our goals is to bring HAE patients up to date on everything concerning HAE. That's why we're working on a new website. We're planning to launch it in the second half of September.

We're pleased to announce that on 7 October 2023, we'll be hosting a live event for HAE patients in the Netherlands. It will be a day filled with interesting presentations by HAE experts, and of course, there will be a chance to share experiences with fellow patients.

If you're an HAE patient living in the Netherlands and you want to attend this event, send an email to jongeren@hae-qe.nl

have conveyed our full support. In May, she gave us pleasant news: she was doing a school research project on HAE together with some classmates, research that was later presented and exhibited in their educational center. For this reason, the family of HAE PERU is grateful for their commitment to disseminate and raise awareness of this rare disease.

Given such a welcome initiative, HAE PERU made available to Carla and her classmates various materials that could contribute to disseminating HAE in her school, including the banner with the design provided by the HAEi.



The objective of encouraging the youngsters' participation was greatly reinforced thanks to the first HAEi LEAP 2023 Educational Program organized by HAE International, in which María Fe and Luis Eduardo had the opportunity to participate and which we will soon begin to see its fruits.



PERU

*From Carla M. Goachet Boulanger,
President, HAE Peru*

One of the goals of the 2023 proposed by the Board of Directors of HAE PERU was to encourage, motivate, and support the participation of youngsters from all over Peru in the various activities of the association, always respecting the voluntary initiative of each one of them, since we are aware that strengthening the initiatives and participation of young people will make the Association last over time and in this way continue to improve the work we have been doing.

Last year in November, we met Carla Rivas, a 14-year-old girl diagnosed with HAE. We welcomed her to the AEH PERU Family with all our love, and since then, we



Likewise, Kamila's participation as a member of the HAEi Youngsters' Advisory Group makes us feel that we are on the right track when we think of the youth of HAE PERU. We hope that Carla, Kamila, Luis Eduardo, and María Fe will be an inspiration so that more young people participate every day in creating awareness of HAE, especially in our country, that is so needed.



CANADA

*From Michelle Cooper, President,
Jacquie Badiou, Past President, and
Daphne Dumbrille, COO, HAE Canada*

A few years ago, HAE Canada's Past President, Jacquie Badiou, realized that HAEC would benefit from gaining accreditation from Imagine Canada, a national organization that helps not-for-profits become and remain effective, accountable, and productive. Since then, she, along with past volunteers from the Governance Committee, Board member Kerstyn Lane, and staff member Daphne Dumbrille, have worked closely with Heather Dow from Events and Management Plus to demonstrate to Imagine Canada that HAEC fulfills the necessary requirements for accreditation, such as excellence in Board governance and financial accountability. We are extremely proud to report that our hard work has paid off and we have officially received accreditation. Special thank yous go to Heather, Kerstyn, Jacquie, Daphne, Michelle, and our treasurer, Carmen Craciun, for working diligently to achieve this goal. HAE Canada has always remained accountable to our members; and thanks to Imagine Canada's accreditation, it's now official.

We are excited by our latest addition to our website: Patient Stories. We hope many viewers will relate to

these amazing stories highlighting how HAE patients can lead happy, fulfilling lives. Recognizing that patients with HAE often feel alone, we understand that many HAE patients have some negative feelings. We hope these stories will help alleviate these negative feelings and show that HAE patients are not alone; there are others who have similar struggles and are leading a fulfilled life. We are extremely grateful to Jordyn, Shannon, and Kim for opening their doors and hearts to share their experiences and to let others know that having a rare disease does not define who you are.

Our abstract titled "Medical resource utilization and quality of life of HAE patients based on data from the 2020 national survey" was recently accepted by the Canadian Society of Allergy and Clinical Immunology (CSACI)'s Annual Scientific meeting. Michelle, Jacquie, and Daphne are looking forward to attending CSACI's meeting in Ottawa in October and showcasing the abstract's data at the poster presentation. Thank you to Suzanne Kelly from Red Maple Trials for her work developing this abstract, along with fellow authors and members of the HAEC Advocacy Committee.

In March 2023, the Canadian federal government announced they plan to provide up to \$1.5 billion over three years to implement the Rare Disease Drug Strategy. To better understand and keep well informed of the progress the government is making on this strategy, HAEC Board members have attended webinars and conferences hosted by the Canadian Organization for Rare Disorders (CORD) that focuses on this much anticipated strategy. We will continue to advocate for implementing this strategy and participating in CORD's events to ensure our voices are heard.

Our Board is looking forward to some big projects coming up, specifically developing our next Five-Year Strategic Plan and designing our next National Report Card survey. We also plan to attend multiple national and international conferences to remain up-to-date in the current treatment and rare disease landscape. We are getting excited to attend the HAEi Regional Conference in Panama City in March 2024, and judging by the number of new HAE Canada members, it's clear there is a lot of interest in this upcoming event.

We are grateful for the work our Regional Directors are doing with the HAEC members to keep everyone engaged and well-informed. Our next Patient Information Update on November 4th will take place in Winnipeg, Manitoba, and we will be offering a virtual option for members who do not live in the Winnipeg area.



SWITZERLAND

From Ernst Greber, HAE Switzerland

HAE Film: 'HAE - Simply Explained' with added French and Italian subtitles: The Swiss HAE Association has had a film produced in which the rare disease Hereditary Angioedema (HAE) is explained in a simple manner for individuals affected by HAE and those interested in HAE. It is explained in non-medical language what triggers the disease, how the processes in the bodywork, and what can be done about it.

We have now supplemented the film with French and Italian subtitles as well.

Link to the film: 'HAE - Simply Explained' with German, English, French, and Italian subtitles: bit.ly/hae-simply-explained

This year, as well, we surprised the patients of the Swiss HAE Association with a gift for **hae day** :-). This year, it's a snap band that serves as a safety or warning band in the dark for safety purposes.



UNITED KINGDOM

From Angela Metcalfe, CEO and Rachel Annals, EO, HAE UK:

In the last few months, we have had lots of meetings with healthcare professionals and our pharmaceutical company representatives. We have also just returned from the HAEi Regional Conference in Munich and were delighted to have so many UK patients attending and participating in the patient and caregiver track and having the opportunity to meet old and new friends. We learned lots at this event and are excited to use this knowledge to help our members.

As a patient advocacy organization, we are here to support you, the patients. We are always keen to hear what you most want to learn about, whether we are doing enough to share all the latest information with you, and, of course, we are always here to help when needed. We are aware many of you have recently had home delivery problems for medication via the Sciensus company. Due to pressure from our CEO on behalf of patients, we now have a dedicated HAE contact, so if ever you have any issues with your deliveries, do get in touch with us.

We are shortly going to start revamping our HAE UK Website. We want to make it easier to find information on new medications and treatments currently being researched, new ideas and practices to support mental health and wellbeing and to have access to all the latest research and trials currently taking place. We will still have the patient stories, information for those newly diagnosed patients, travel information, and, of course, more about how we can support you and advocate for all HAE patients in the UK.

In the coming weeks, we are going to undertake a large mailout, sending a letter and poster to every single GP Practice in the UK, as well as all A&E Departments. We hope this will help raise awareness and knowledge of HAE in the medical community, as well as to possible undiagnosed patients who may see the posters in waiting areas.

We are looking at running a few virtual workshops via Zoom or Teams. Topics for these sessions could include 'Supporting my mental health and wellbeing', 'Should I take part in drug trials?', 'Getting the best support from my health care professional', 'Keeping an attack diary' etc. We hope to run the first of these towards the end of November, more details will be sent to all UK members in the coming weeks.

We will, of course, still look to hold our annual patient day if this is something you, our members, would like.

We also have lots of other exciting projects in the pipeline, including working with Staffordshire University on a project about HAE, continued engagement with pharmaceutical companies about the latest developments worldwide in treatments and medications, and, most importantly, sharing information and learning from you.

We would love to hear from you with any feedback, comments, or suggestions: support@haeuk.org.



CROATIA

From Mihaela Sogoric, HAE Croatia

The Association of Patients with Hereditary Angioedema HAE Croatia successfully organized the 3rd meeting of parents and children suffering from HAE with pediatricians in Tuheljske toplice.

The pediatricians chose very good lecture topics and, in my personal opinion, this was the most successful meeting so far.

Saturday, September 30, 2023:

- Primarius Irena Ivković Jureković, MD, PhD – Food as an attack trigger for HAE
- Renata Vrsalović, MD, PhD – Long-term and short-term prophylaxis
- Nevenka Cigrovski, MD – Remission after bone marrow transplantation in a patient with leukemia (case report)
- Iva Topalušić, MD – HAE in girls
- Barbara Kvenić, MD – HAE and comorbidities, edema of other etiology
- Med-tech. Lidija Galović – Education of children and parents about self-administration of medicines

We continued our gathering on Sunday, October 1, where the parents had a psychological workshop on “Self-care and HAE”.

Among the guests from our region, we were joined by the president of HAE Serbia, Jovana, whom I would like to thank this way.

We ended the meeting with a visit to Matija Gubac's museum “Museum of the Peasant Revolt”, thus ending our third meeting. We are looking forward to the new one next year and we hope it will be even more successful. I would like to thank especially the donors Takeda and Swixx BioPharma Croatia, without whom this meeting would have been impossible. Also, I would like to thank Tuheljske Toplice for the trust shown for the third year in a row.





USA

The US HAEA is committed to delivering engaging and informative programs, services, and activities to our expansive membership of over 8,000 individuals. These initiatives aim to unite the HAE community in pursuit of our collective and shared objective: enhancing the quality of life for those with HAE.



2023 US HAEA National Summit – Living Beyond Boundaries: After four long years apart, the HAEA hosted an unforgettable and remarkable 2023 US HAEA National Summit that unified nearly 1,200 HAE friends in Orlando, Florida. Together, we embarked on an amazing 3-day journey filled with thought-provoking youth workshops, general sessions that featured a blend of heartfelt patient stories and informative industry and physician presentations, and fun-filled activities, including an inspiring HAE IN-MOTION® awareness walk.

The Professional-Scientific Track drew an audience of nearly 200 healthcare professionals, physicians, researchers, and scientists, highlighting key updates in HAE diagnosis and research, and also featured an engaging poster-viewing session, showcasing a total of 28 posters that highlighted the latest advancements.

A genuine thank you to our united HAEA community for making this 2023 Summit a memorable success!

You can read more about the exciting details of this event on page 61-63.

HAE IN-MOTION® – Step It Up for HAE: This fall, members of the HAE community embraced our challenge to Step it Up for HAE, and raised awareness through our 2023 HAE IN-MOTION® event! Participants enthusiastically tracked their miles as they engaged in a diverse range of activities, from walking and running, to cycling and swimming. This collective effort aimed to achieve an impressive goal

of 30,000 miles, uniting the community in a fun and collaborative way that also served as an effective platform for educating others about HAE!



Additionally, the HAE IN-MOTION® event hosted at our 2023 US HAEA National Summit proved to be an opportunity for members of our community to gather together and mingle after four long years apart. From leisurely walks along the hotel's beautiful nature trail, to playful lawn games and an ice cream stand, attendees had a chance to unwind and enjoy the outdoors, all while celebrating their HAE journeys.

All funds raised from these events will support key HAEA programs including the Pam King HAEA Scholarship Program, the Chris Whalen HAEA Compassion Fund and the HAE Research Fund.

HAEA Community Blog: The HAEA Community Blog is a platform that allows people with HAE to share their unique stories on a wide variety of topics ranging from emotional health, navigating insurance challenges, and journey to diagnosis and treatment.



Featured Blog Articles:

"So, you've completed your diagnostic journey and have been diagnosed with Hereditary Angioedema (HAE). Your doctor has prescribed a treatment protocol and you discover that your healthcare insurance will not cover the cost of the medicine. Now what?"

This situation can be all too common for people with a rare disease and their caregivers, but it is not impossible to overcome. Just as you went through a diagnostic journey, the second part will be a care coverage journey.”

Read **You, Your Medications, and Your Healthcare Insurance** at haea.org/pages/bp/blog19_Anita



“When I was younger and still having several HAE attacks a month, I would have never dreamed I could study abroad in college, but I was determined not to let my HAE limit me.”

Read **HAE Didn't Hold Me Back From Seeing the World** at haea.org/pages/bp/blog21_Nathan



Advances in Research:

HAE Specific Quality of Life (QoL) Instrument: In the Fall of 2021, the HAEA initiated a research study to redefine how HAE affects overall quality of life. The research enabled us to design and validate a quality of life questionnaire that truly captures the way HAE affects the everyday life of individuals and

families in the US. This instrument will demonstrate to health insurers and others the value of life-changing improvements in health and quality of life that result from the development and availability of modern HAE treatments. A medical journal article featuring the new HAEA QoL instrument will be published in the Fall of 2023.

HAEA Sponsored Study Reveals Harmful Impact of Insurance Denials for HAE Medicines: Members of our community who face insurance company decisions to delay or initially deny coverage for a prescribed HAE medicine are forced to somehow cope with a wide range of devastating consequences. The HAEA commissioned a study to highlight the disturbing and costly effect of delays and denials as part of our continuing campaign to reduce delays and denials by demonstrating the holistic value of HAE medicines.

In our study:

- 70% of participants reported an increased frequency of attacks resulting from insurance delays,
- More than 50% missed work/school days because of increased attacks,
- 90% reported greater anxiety,
- 25% of respondents reported an increase in costly urgent care or emergency department visits,
- Almost all participants reported that insurance-related problems had a negative impact on health, family, and work/school life.

You can access the publication free of charge in The Journal of Allergy and Clinical Immunology: In Practice free of charge by visiting [sciencedirect.com/science/article/pii/S2213219823002908](https://www.sciencedirect.com/science/article/pii/S2213219823002908)

You can also access the AAAAI Press Release here: aaaai.org/About/News/News/2023/insurance

New Continuing Medical Education (CME) for Healthcare Professionals:

Educating the physician community in the latest HAE clinical care advances helps drive continued improvements in the health of the HAEA community. That is why the HAEA produced and launched a new, free CME program for Medical Professionals - Updates in Hereditary Angioedema Care From the HAEA. The program is designed to meet the educational needs of healthcare professionals involved in the diagnosis and/or management of people with HAE. This CME provides physicians, nurses, or pharmacists the opportunity to earn free continuing medical education credits, and spread awareness on HAE and current treatment guidelines. View the program on the HAEA website here: haea.org/pages/p/CME



KOSOVO

HAEi is delighted to have welcomed HAE Kosovo as country no. 97 to join the global HAE family.

The national contact is Edison Sylejmani. You can read more about HAE Kosovo on the country page on our website: haei.org/hae-member-countries/kosovo.

In our **2023 HAEi Regional Conference EMEA** Scientific Track, Dr. Rushit Ismajli presented a poster describing the first identified case of HAE in Kosovo. You can find out more on page 46.



MOZAMBIQUE

Please join us in welcoming yet another member country to the global HAE family: HAE Mozambique as member country no. 98.

The national contact is Daniella Nhaguilunguana, and you can read more about HAE Mozambique on the country page on our website: haei.org/hae-member-countries/mozambique.

Daniella shared her story as part of the Patient Voices session at the **2023 HAEi Regional Conference EMEA**. You can read about this on page 35.



AUSTRALIA & NEW ZEALAND

From Fiona Wardman, HAE Australasia



HAE Australasia has a new CEO! Lisa Foster is an inclusive and authentic leader with extensive experience across Health, Disability, Mental Health, and Community Support Sectors.

Lisa was the Chief Executive for Rare Disorders New Zealand and has a background in biomedical science. As a veteran of advocating for those with rare diseases by building cooperative relationships, Lisa's focus is achieving meaningful change for people impacted by Hereditary Angioedema in Australia and New Zealand.

MEDICAL PAPERS

In addition to a wide range of case reports and small series, we present summaries of recently published HAE-related scientific papers (data search undertaken end of August 2023). You can find the abstracts via the search function at pubmed.ncbi.nlm.nih.gov

Hereditary Angioedema: A review of the current and evolving treatment landscape

Stephen D. Betschel, et al.

Writing in the Journal of Allergy and Clinical Immunology: In Practice, the authors review current and potential future treatments for HAE. They also offer an expert perspective on managing special HAE patient populations, including women and pediatric patients.

(JACI-In Practice, August 2023)

Neurologic and Psychiatric Manifestations of Bradykinin-Mediated Angioedema: Old and New Challenges

Ilaria Mormile, et al.

Writing in the International Journal of Molecular Sciences, the authors review what is currently known about neurological symptoms of HAE, including headache, visual disturbances, and stroke-like episodes. The authors also report symptoms preceding HAE attacks, including itching, burning, pain, and pressure. In addition, the authors examine the psychological burden of HAE, such as stress, fear, and depression) and consider whether this may exacerbate or cause attacks and whether they may be regarded as symptoms in their own right.

The authors conclude that neurological symptoms of HAE are an under-examined area, with limited data on their prevalence, with more research needed to better understand and develop new treatments for these unusual symptoms.

(International Journal of Molecular Sciences, July 2023)

Biochemistry, molecular genetics, and clinical aspects of angioedema with and without C1 inhibitor deficiency

Toshiyuk Miyata and Takahiko Horiuchi.

The two authors review the latest science behind what causes HAE, both for people with and without C1 inhibitor deficiency. In particular, they examine the latest advances in understanding the role of the contact activation system, a complex cascade of proteins involved with blood clotting. Additionally, the biochemistry and molecular genetics of HAE are discussed in detail.

(Allergology International, May 2023)

Disease burden and societal costs of hereditary angioedema

Lauré M Fijen, et al.

Reporting a study amongst adult HAE patients in the Netherlands, the authors sought to establish the entire burden of HAE, including disease control, treatment satisfaction, reduction in quality of life (QOL), and societal costs. Results from the study included:

- One-third of patients in the sample did not have well-controlled disease, leading to a relatively low quality of life
- The medical consumption of Dutch patients with HAE, productivity costs, and informal care costs equaled €22,764 per year, mostly related to HAE medication and medical costs
- The impact of an HAE attack on health-related QOL (HRQOL) is significant, with the average score for HRQOL dropping from 0.720 to 0.420 during an attack

The authors conclude that additional prophylactic strategies are needed to improve disease control and that their results were comparable with those of Swedish, German, Spanish, and Danish patients surveyed in earlier studies.

(JACI-In Practice, March 2023)



Hereditary angioedema and Wilson's disease during breastfeeding

Philip O. Anderson.

The presence of HAE in people breastfeeding is the focus of this review. The possibility of increased maternal HAE attacks, likely due to increased hormonal levels, was described, along with the use of treatments while breastfeeding.

(Breastfeeding Medicine, August 2023)

Hereditary angioedema and its new treatments: An update

By David Launay, et al.

In a French language review, the authors examine the latest medicines to treat and prevent HAE attacks and their impact on quality of life.

(La Revue de Médecine Interne, July 2023)

Factor XII structure-function relationships

Alexander Shamanaev.

As Factor XII is involved in the process leading to HAE attacks, it is an important area of clinical research. This article reviews research into the structure and function of Factor XII. The authors also found that Factor XII circulates in the blood in a 'closed' form, which is resistant to activation. It is only when Factor XII interacts with other molecules that it becomes activated. The authors conclude that these findings have implications for how Factor XII contributes to HAE and the development of new treatments.

(Seminars in Thrombosis and Hemostasis, June 2023)

Are pediatricians familiar with hereditary angioedema?

Herberto José Chong-Neto, et al.

This study used an online survey to investigate levels of HAE knowledge amongst Brazilian pediatricians. All registered pediatricians in Brazil were contacted, with 2.6% responding. Of those individuals, the median number of correct answers to questions about HAE was 7 out of 12. The authors conclude that knowledge about HAE among Brazilian pediatricians is unsatisfactory and that increasing awareness may lead to improvement in diagnosis and treatment.

(World Allergy Organization Journal, June 2023)

Current challenges and future opportunities in patient-focused management of hereditary angioedema: A narrative review

Anete S Grumach, Centro Universitario Faculdade de Medicina ABC (FMABC), Brazil, et al.

This review considers the wide range of clinical guidelines currently available in HAE and the impact of including new treatment options. The authors conclude that this has changed the HAE landscape of HAE treatment, offering patients more options and the chance of shared decision-making. Better identification of patients most likely to benefit from these new treatments may aid uptake. The important contribution of patient-reported outcomes and shared decision-making in reducing the burden of HAE are also considered.

(Clinical and Translational Allergy, May 2023)

Genetic variants leading to urticaria and angioedema and associated biomarkers

Jonathan J Lyons, et al.

This review considers the known genetic (inherited and acquired) causes of angioedema (and similar conditions), highlights specific features of their clinical presentations and discusses the benefits and limitations of biomarkers to distinguish them from each other.

(JACI-In Practice, May 2023)

The multifactorial impact of receiving a hereditary angioedema diagnosis

Jason Raasch, et al.

The authors review the challenges that arise on the pathway to a confirmed diagnosis of HAE and explore the multifactorial impact of receiving an HAE diagnosis.

They conclude that it is important to continue to raise awareness of HAE because delays to diagnosis have a direct impact on patient suffering and quality of life. Since many patients will seek help from hospitals during their first swelling attack, it is vital that emergency department staff are aware of the different pathological pathways that distinguish HAE from other forms of angioedema to ensure that the most appropriate treatment is administered. As disease awareness increases, it is hoped that patients will be diagnosed earlier and that pre-authorization and insurance coverage of HAE treatments will become easier to obtain, ultimately reducing the treatment burden for these patients and their caregivers.

(World Allergy Organization Journal, June 2023)

Increased thromboinflammatory load in hereditary angioedema

Olav Rogde Gramstad, et al.

This study investigated blood clotting and inflammation (thromboinflammation) levels in HAE patients in clinical remission. It identified increased levels of thromboinflammation in people with HAE compared to healthy controls. The authors believe that even HAE patients in clinical remission are in a subclinical HAE attack state. However, biochemical remission (with no signs of abnormal laboratory results) is possible with effective prophylactic therapy. In conclusion, the authors suggest that markers of thromboinflammation could be valuable tools to guide and individualize the choice and dosing of prophylactic HAE treatment.

(Clinical and Experimental Immunology, August 2023)

A safety review of prophylaxis drugs for adolescent patients with hereditary angioedema

Henriette Farkas and Zsuzsanna Balla.

This review examines the safety data from clinical and observational trials of the currently available medicines to prevent HAE attacks. The expert opinion of the authors is that available therapeutic products have a good safety and efficiency profile and are recommended

as first-line treatments in international guidelines. The authors conclude that the choice of medicine should be made based on evaluating medicine availability and the patient's preference.

(Expert Opinion on Drug Safety, June 2023)

Consequences of insurance coverage delays and denials for patients with hereditary angioedema

Nonie S. Arora, et al.

The authors, including representatives from HAEA, looked at the impact of insurance delays and denials on patient health and well-being. The results of an online survey amongst 20 patients with HAE (type 1 and 2) indicated that 70% of participants had more HAE attacks as a result of insurance delays or denials. The authors conclude that insurance delays and denials significantly impact individuals with HAE, leading to; increased emergency department visits; missed school/work days; higher levels of anxiety and a negative impact on family life. The paper calls for improvements in the insurance authorization process for medications, as this would significantly benefit patients and their well-being.

(JACI-In Practice, April 2023)

Cost control versus optimal patient care in hereditary angioedema

Osman Dokmeci and Richard Honsinger.

In an accompanying editorial to 'Consequences of insurance coverage delays and denials for patients with hereditary angioedema,' the authors elaborate on some of the issues raised and the complexities of managing HAE and identifying patients most likely to benefit from long-term prophylaxis due to concerns about the cost of these medicines. The authors suggest that decisions about management should reside with allergy/immunology professionals, as they are best able to understand the condition. To be successful, they say, patients, professional colleagues, and payors will need further education.

(JACI-In Practice, April 2023)

Hereditary angioedema with normal C1 inhibitor: US survey of prevalence and provider practice patterns

Marc A. Riedl, et al.

In what the authors believe to be the first study of its kind, researchers analyzed data from US healthcare professionals to develop an estimate of the prevalence and current management of HAE with normal C1 inhibitor (HAE-nl-C1NH). After correcting for potential statistical issues, the results from US clinicians across 32 states estimated 1,230-1,331 patients with HAE-nl-C1NH. The results also showed that the average time to diagnosis of HAE-nl-C1NH was six years, with response to medication often being used as a tool for diagnosis. More than half (54%) of HAE-nl-C1NH received long-term preventative treatment. The authors suggest that these data will provide insights into current diagnosis and treatment of HAE-nl-C1NH in the United States, which may inform further studies on how best to manage this condition.

(JACI-In Practice, January 2023)

Expert review and consensus on the treat-to-target management of hereditary angioedema: From scientific evidence to clinical practice

Teresa Caballero, et al.

A Spanish expert consensus was sought to steer HAE management towards a treat-to-target approach and clarify some aspects of existing Spanish guidelines. A panel of 53 HAE experts produced recommendations on previously unclear aspects of HAE management with on-demand treatment and short and long-term prophylaxis based on clinical and patient-oriented goals.

(Journal of Investigational Allergology and Clinical Immunology, February 2023)

A national survey of hereditary angioedema and acquired C1 inhibitor deficiency in the United Kingdom

Patrick FK Yong, et al.

This study aimed to obtain more accurate data on the demographics of HAE and acquired C1 inhibitor deficiency in the United Kingdom, including available treatment and services. It was hoped this would assist in planning service provision, identifying areas of improvement, and leading to better care.

The data allowed an estimate of HAE type 1 and 2 prevalence in the UK of 1:59,000. The authors indicate this is close to the often-quoted prevalence of 1:50,000. However, they also caution that there appears to be a significant number of patients who are not currently engaging with a specialist HAE center, which could mean the UK prevalence may be higher. Acquired angioedema is much rarer, with a minimum prevalence of 1:734,000, while the prevalence of HAE with normal C1 inhibitor in the UK is 1:3,000,000.

Several challenges were identified in the treatment and management of patients. Only 61% of patients were able to self-administer therapy for acute attacks despite good provision of treatment. Additionally, the data indicated significant androgens and tranexamic acid usage for long-term prophylaxis, despite not being first-line agents in international guidelines.

The authors conclude with the hope that their research will inform future decision-making to ultimately provide the best care for patients with HAE.

(JACI-In Practice, August 2023)

PLAUR splicing pattern in hereditary angioedema patients' monocytes and macrophages

Lucie Ballonová, et al.

This paper sought to test the hypothesis that a gene called PLAUR plays a role in HAE severity. In conclusion, the authors suggest no evidence of a significant change in the PLAUR splicing pattern between healthy controls and HAE patients. However, this was only tested using two types of immune cells, leading the researchers to suggest that the PLAUR gene may still play a role in the disease by impacting on other cell types.

(Molecular Biology Reports, June 2023)

Evolution of guidelines for the management of hereditary angioedema due to C1 inhibitor deficiency

Branco Ferreira, et al.

Globally, HAE guidelines have evolved with the availability of new treatments and a change in the management paradigm towards an individualized, patient-centered approach where quality of life is central. Over time, guidelines have prioritized home-based treatment. The introduction of long-term prophylaxis also led to more ambitious treatment goals, from reduction in severity, duration, and frequency of

attacks to total disease control and a normal life for patients.

The authors conclude that guidelines for the management of HAE have come a long way, and that regular updating of national and international guidelines is necessary to ensure innovations in management can be incorporated into practice in a timely fashion.

(Journal of Investigational Allergology and Clinical Immunology, May 2023)

Mechanisms involved in hereditary angioedema with normal C1-inhibitor activity

Alexander Shamanaev, et al.

This study analyses the latest evidence into the causes of HAE with normal C1 inhibitor (HAE-nl-C1NH). The authors indicate that a mechanism dubbed Plasminogen-Glu may cause HAE-nl-C1NH). They report that this may have important implications for treatment, as commonly used therapeutic options to treat HAE may be less effective in patients with Plasminogen-Glu.

(Frontiers in Physiology, May 2023)

Angioedema with normal complement studies: What do we know?

Constance H Katelaris, et al.

The authors examine the process of differential diagnosis of a variety of clinical conditions; including the mechanisms which cause the disease, the impact of certain medicines, and forms of diagnostic testing.

(Journal of Allergy and Clinical Immunology, August 2023)

Concomitant medication in patients with bradykinin-mediated angioedema – there's more than ACE inhibitors

Robin Lochbaum, et al.

In this article, the authors consider a range of medications that people with HAE may also need to take and the impact this may have on their angioedema. The authors make clear that all healthcare professionals who manage HAE should weigh the risk of angioedema against the benefits of the treatment used in their patients.

(Journal der Deutschen Dermatologischen Gesellschaft, July 2023)

Hereditary angioedema (HAE) in Belgium: Results from a national survey

Marie-Line Van der Poorten, et al.

Due to a lack of data on the number of people with HAE in Belgium, and little understanding of the impact of the disease on their lives, the authors undertook the first nationwide study, which involved the eight Belgian hospitals known to treat HAE patients. On average, Belgian patients received a diagnosis of HAE seven years after their first symptoms. 60% of patients with symptoms reported being on long-term prophylactic treatment, with 56.3% receiving human plasma-derived C1-esterase inhibitor concentrate. The authors conclude that the impact of HAE is not to be underestimated and that this data will help raise awareness, leading to improved therapies and ongoing management of the condition.

(Frontiers in Allergy, May 2023)

Diagnosis and treatment procedures and health management for patients with hereditary angioedema

Min Zhou, et al.

In a Chinese-language study, the authors investigated a new rapid emergency response system to support patients with HAE who experience a sudden or severe attack. This 'one-click help' aimed to offer specialized assistance for people with HAE.

(Zhonghua Yu Fang Yi Xue Za Zhi, August 2023)

Clinical practice of hereditary angioedema in Belgium: Opportunities for optimized care

Didier Ebo, et al.

This study examined the extent to which Belgian clinical practice is aligned with the latest revised guidelines from WAO/EAACI on diagnosis and management of HAE. The authors identify five necessary action points for Belgian clinical practice in HAE to be optimized in line with the WAO/EAACI guidelines. These are:

- Work towards total disease control and normalize patients' lives by considering new and innovative long-term prophylactic therapies
- Inform HAE patients about new long-term prophylactic therapies
- Ensure the availability of on-demand therapy for all HAE patients
- Implement a more universally used assessment including multiple aspects of the disease (e.g., quality of life) in daily clinical practice
- Continue and expand the existing patient registry

(Acta Clinica Belgica, May 2023)

Management of hereditary angioedema in resource-constrained settings: A consensus statement from Indian subcontinent

Ankur Kumar Jindal, et al.

In collaboration with the HAE Society of India, the authors showcase new consensus guidelines on treating HAE in resource-constrained settings, such as India, where the only available first-line treatment option for HAE is intravenous pd-C1-INH. The guidelines were developed because it may not be possible for all patients to access the recommended therapy and at the recommended doses as suggested by the international guidelines, and it may not be feasible to follow algorithms suggested in international guidelines.

(Asia Pacific Allergy, June 2023)

Challenges in the management of hereditary angioedema in urban and rural settings

J Allen Meadows, et al.

The focus of this paper was the experiences of allergy and immunology health professionals in diagnosing and treating patients with HAE, including those living in rural settings.

The authors conclude that the challenge of diagnosing HAE and HAE with normal C1 inhibitor, and the

economic challenges of treatment, are compound for those living in rural areas. They suggest that better payment through Medicaid and Medicare could better support patients in rural settings.

(Annals of Allergy, Asthma and Immunology, June 2023)

Efficacy and safety of lanadelumab in Japanese patients with hereditary angioedema: A phase 3 multicenter, open-label study

Michihiro Hide, et al.

The safety and efficacy of lanadelumab for the prevention of HAE attacks had not previously been studied in Japanese patients. The authors conclude the results show the effectiveness of lanadelumab in preventing HAE attacks in Japanese patients with HAE type I / type II. The efficacy and safety were consistent with those reported in the global phase 3 lanadelumab studies.

(Journal of Dermatology, August 2023)

Efficacy, pharmacokinetics, and safety of subcutaneous C1-esterase inhibitor as prophylaxis in Japanese patients with hereditary angioedema: Results of a Phase 3 study

Tomoo Fukuda, et al.

This study investigated the efficacy and safety of subcutaneous (SC) C1-esterase inhibitor (C1-INH (SC)) to prevent HAE attacks in Japanese people with the condition. The authors conclude that C1-INH (SC) was efficacious and well-tolerated as a preventative medicine against HAE attacks in Japanese patients with HAE types I and II.

(Allergology International, July 2023)

The US HAEA scientific registry: Hereditary angioedema demographics, disease severity and comorbidities

Sandra C. Christiansen, et al.

This research aimed to develop a national patient-centric registry to address deficiencies in knowledge about the course of HAE and the real-world impact of therapeutic interventions. Results indicated that 36.9% of people with HAE in the study reported the death of a family member from an HAE attack (the figure was 15.4% for people with HAE with normal C1 inhibitor). There were also increases in depression, sleep disorders, kidney disease, anemia, hepatitis, and cardiovascular

problems across the enrolled patients. The authors believe the registry provides an opportunity to enhance knowledge of HAE and non-histaminergic angioedema (INHA), which was also included.

(Annals of Allergy, Asthma and Immunology, August 2023)

Recent advances in clinical research on rare intractable hereditary skin diseases in Japan

Masashi Akiyama, et al.

A research group for rare and intractable skin diseases has been established within the Japanese Ministry of Health, Labour, and Welfare. This group conducts research into eight rare skin diseases, including HAE. This review introduces and summarizes the activities of this group in Japan, including the dissemination of information to academic societies, medical professionals, patients, and the general public.

(Keio Journal of Medicine, June 2023)

A multicenter chart review of patient characteristics, treatment and outcomes in hereditary angioedema: unmet need for more effective long-term prophylaxis

Joan Mendivil, et al.

Data on the use of long-term prophylaxis and HAE attack rates were examined in this multi-national study. The findings, the authors conclude, support using long-term prophylaxis to prevent HAE attacks. However, the type of long-term prophylaxis used varied between countries, with tolerability and accessibility to specific treatments playing important roles in decision-making by clinicians.

(Allergy, Asthma and Clinical Immunology, May 2023)

Safety, efficacy, and pharmacokinetics of icatibant treatment in Japanese pediatric patients with hereditary angioedema: A phase 3, open-label study

Michihiro Hide, et al.

A Japanese research group examined the safety and efficacy of subcutaneous icatibant for treating acute HAE attacks in Japanese pediatric patients. The authors conclude that their results support the safety and efficacy of icatibant in Japanese pediatric patients.

(Journal of Dermatology, June 2023)

CU06-1004 alleviates vascular hyperpermeability in a murine model of hereditary angioedema by protecting the endothelium

Sunghye Lee, et al.

A Korean research group investigated the potential of a new therapeutic option for HAE in this laboratory study. The potential treatment, called CU06-1004, is an endothelial dysfunction blocker aiming to act on the effects of bradykinin release. The study demonstrated that the oral administration of CU06-1004 reduced the hyperpermeability in a model of HAE, indicating that this mode of action could be a potential prophylactic/therapeutic approach for HAE patients.

(European Journal of Allergy and Clinical Immunology, May 2023)

Restriction of C1-inhibitor activity in hereditary angioedema by dominant-negative effects of disease-associate SERPING1 gene variants

Laura Barrett Ryø, et al.

Most cases of HAE are caused by genetic variants in the SERPING1 gene, leading to problems with the C1-inhibitor protein. More than 500 different HAE-causing variants have been identified in the SERPING1 gene, but the disease mechanisms are largely unknown. The researchers provide a functional classification of these gene variants, suggesting that different SERPING1 variants drive disease through different and sometimes overlapping mechanisms.

(Journal of Allergy and Clinical Immunology, June 2023)

Lanadelumab for the prevention of hereditary angioedema attacks: A real-world UK audit

Anthony D. Dorr, et al.

The introduction of lanadelumab into the UK has meant that increasing numbers of people with HAE receive it as long-term prophylaxis. Still, their experience is not represented in published trials. Additionally, to qualify for funding from the National Health Service, patients must have more frequent attacks than those seen in trials of lanadelumab. In a short research letter, these UK authors outline that they have collected attack, treatment, and prophylaxis data before and after commencing lanadelumab in the first national UK lanadelumab audit. The authors report that their real-world data demonstrate that lanadelumab is effective in patients with severe HAE despite prior prophylaxis,

including those previously receiving prophylactic C1-inhibitor. The conclusion is that these data provide assurance for lanadelumab efficacy and support its use as a first-line treatment. Dosing can also be reduced while preserving efficacy, improving cost-effectiveness.

(European Journal of Allergy and Clinical Immunology, May 2023)

Long-term lanadelumab treatment improves health-related quality of life in patients with hereditary angioedema

William R. Lumry, et al.

Building on recent safety and efficacy clinical trial data on lanadelumab, this study investigated the impact of long-term lanadelumab treatment on patient-reported outcomes (PROs). The authors note that the latest management of HAE places more attention on the broader burden of the disease, leading to a focus on individual patient preferences around treatment decisions. As a result, it is important to consider understanding and reflecting patient goals in clinical studies. The authors conclude that their data indicate that long-term treatment with lanadelumab provided sustained clinically meaningful improvement in health-related quality of life for patients with HAE, which supports the use of lanadelumab to prevent attacks.

(Annals of Allergy, Asthma and Immunology, April 2023)

Berotrastat for the prophylaxis of hereditary angioedema – Real-world evidence data from the United Kingdom

Manisha Ahuja, et al.

Aiming to evaluate the real-world clinical outcomes of berotrastat amongst people with HAE in the UK, this research obtained data on treatment, side effects, attack frequency, and disease severity from 54 people with HAE. The authors indicate that their results demonstrate comparable efficacy of berotrastat compared to previous clinical trials. However, they note a higher rate of side effects than seen in clinical trials and that further studies are needed to evaluate long-term outcomes.

(European Journal of Allergy and Clinical Immunology, January 2023)

Fibrin and fibrinogen degradation products/ D-dimer ratio can be a useful marker for differentiating an acute attack of hereditary angioedema from thrombotic conditions

Daisuke Honda, et al.

The authors investigate potential laboratory tests to distinguish between HAE attacks and other blood-clotting events. Examining a ratio of two blood values: fibrinogen degradation products and D-dimer ratio, the authors conclude that this could be an effective way to determine whether someone was having an HAE attack or a blood-clotting event. The authors acknowledge that this may be challenging due to the increasing use of on-demand treatment for self-administration, meaning patients may be less likely to go to hospital where blood tests could be conducted.

(Allergology International, August 2023)

Living with hereditary angioedema in Australia: Findings from a national observational study using short message service to monitor the burden of disease

Constance H Katelaris, et al.

This study used text messages (short message service or SMS) to communicate with patients to see if this was an effective way to collect information from people with HAE about their condition, treatment, and quality of life.

The authors indicate that this was the first study of its kind to gain real-life information from people with HAE in Australia and was successful in getting valuable information from patients, showing that despite the availability of on-demand therapies, HAE remains a burden for patients and that wider access to safe and effective prophylactic therapies is needed.

(JACI-In Practice, August 2023)

Health related to quality life of patients with hereditary angioedema in Argentina. A multicenter study

Iris Medina, Centro Médico Vitae, Argentina, et al.

This Spanish language paper is focused on the quality of life of people with HAE living in Argentina. The authors found that health-related quality of life was notably reduced in Argentinean patients with HAE and concluded that multidisciplinary strategies for this complex disease are needed.

(Revista Alergia Mexico, June 2023)

Myeloid lineage cells evince distinct steady-state level of certain gene groups in dependence on hereditary angioedema severity

Lucie Ballonová.

This study looked for factors that could explain the variation in severity of hereditary angioedema, as even within individuals with the same variant of the disease-causing SERPING1 gene, there can be huge differences, ranging from asymptomatic disease to frequent life-threatening attacks. The authors conclude that the number of differently expressed genes is elevated in patients with mild disease, but this becomes even more prominent in those with a more severe disease course. The authors conclude that all these findings show that transcript levels in myeloid cells achieve different activation or depression levels in HAE-C1INH patients than in healthy controls and/or based on disease severity and could participate in determining the HAE phenotype.

(Frontiers in Genetics, July 2023)

C1 inhibitor deficiency enhances contact pathway-mediated activation of coagulation and venous thrombosis

Steven P. Grover, et al.

Using a series of laboratory-based tests, these researchers investigated the impact of C1 inhibitor deficiency on processes that lead to blood clotting, as previous research had indicated that people with HAE had a moderate but significant increased risk of deep vein thrombosis.

The authors confirm their earlier findings that people with HAE deficient in C1-inhibitor protein may be at risk of thrombotic events such as blood clots.

(Blood, May 2023)

Hereditary angioedema and thrombosis

David Gailani.

In this accompanying editorial to the above Grover paper, the author discusses the implications of the findings, concluding that while they may not be clear at this point, it seems reasonable to consider prophylactic blood-thinning medication when people with HAE are undergoing illnesses or medical procedures that might lead to HAE attacks. There is also discussion of how long-term prophylaxis for patients with thrombotic events may be difficult with traditional infusions of

C1-INH, but newer agents with long half-lives such as anti-FXII(a) or anti-PK(a) antibodies or chronic therapy with oral PKa inhibitors may be helpful in reducing thrombotic risk.

(Blood, May 2023)

Decreased adhesion to endothelium leads to elevated neutrophil granulocyte count in hereditary angioedema patients

Erika Kajdácsi, et al.

Comparing the blood of people with HAE with a group of healthy controls, these researchers are looking for other factors that play a role in the development of HAE, as elevated bradykinin cannot be the only cause. The authors conclude that their results, together with the lack of evidence of chronic inflammation in the HAE patients, suggest that the high neutrophil granulocyte (NG) counts in these patients may be caused by disturbed adhesion to endothelial cells (EC), biasing the ratio between marginated and circulating pools. In contrast, only a mild activation of circulating NGs occurs as a consequence of more active plasma serine proteases and/or from the elevated soluble E-selectin levels. It is largely unknown if this mild activation of NGs has positive or negative consequences for the C1-INH-HAE patients, but it is worth further investigation.

(Nature Scientific Reports, August 2023)

Systematic approach revealed SERPING1 splicing-affecting variants to be highly represented in the Czech national HAE cohort

Hana Grombirková.

In a genetic study of Czech people with HAE, the authors identified a significant correlation between the presence of the SERPING1 variant c.-21 T>C in the trans position to key aspects of the disease, including causal variants, and the frequency of attacks per year, disease onset, as well as clinical severity score. Overall, the authors conclude that their study provides new insights into the genetic landscape of C1-INH-HAE in the Czech population, including identifying novel variants and a better understanding of genotype-phenotype correlations. Our findings also highlight the importance of comprehensive screening strategies and functional analyses in improving the C1-INH-HAE diagnosis and management.

(Journal of Clinical Immunology, August 2023)

CLINICAL TRIALS

According to clinicaltrials.gov under the U.S. National Institutes of Health, the EU Clinical Trials Register, and the International Clinical Trials Registry Platform under the World Health Organization (WHO), the following trials should currently be recruiting. This information is correct to the end of September 2023.

A 3-Part Study to Evaluate the Pharmacokinetics, Safety, and Efficacy of Subcutaneous Ecallantide in Prepubertal Paediatric Patients Experiencing Acute Attacks of Hereditary Angioedema

– recruiting in Germany

A Gene Therapy Study of BMN 331 in Subjects with HAE

– recruiting in Spain, the United States

A long-term study to evaluate if KVD900 is safe and effective in treating attacks in patients with hereditary angioedema

– recruiting in Australia, Austria, Bulgaria, Canada, France, Germany, Greece, Hungary, Israel, Italy, Japan, the Netherlands, New Zealand, Poland, Portugal, Romania, Saudi Arabia, Slovakia, South Africa, Spain, the United Kingdom, United States

A Phase 1/2, Open-Label, Dose-Escalation Study to Determine the Safety, Tolerability, and Efficacy of BMN 331, an Adeno-Associated Virus (AAV) Vector-Mediated Gene Transfer of Human SERPING1, in Subjects with Hereditary Angioedema (HAE) due to Human C1 Esterase Inhibitor (C1-INH) Deficiency

– recruiting in Spain

A Randomized, Double-Blind, Placebo-Controlled, Phase 3, Three-way Crossover Trial to Evaluate the Efficacy and Safety of Two Dose Levels of KVD900, an Oral Plasma Kallikrein Inhibitor, for On-Demand Treatment of Angioedema Attacks in Adolescent and Adult Patients with Hereditary Angioedema Type I or II

– recruiting in United States, Japan, Australia, Bulgaria, Canada, France, Germany, Greece, Hungary, Israel, Italy, the Netherlands, New Zealand, North Macedonia, Poland, Puerto Rico, Romania, Spain, the United Kingdom, United States

A safety and pharmacokinetic study of oral berotralstat for HAE attacks in pediatric patients

– recruiting in Austria, Canada, France, Germany, Hungary, Israel, Italy, North Macedonia, Poland, Romania, Spain, the United Kingdom

A Study in Adults with HAE who Currently Receive Icatibant at Home

– recruiting in the United Kingdom

A Study of Lanadelumab (SHP643) in Chinese Participants with Hereditary Angioedema (HAE)

– recruiting in China

A Study of Lanadelumab in Teenagers and Adults with HAE

– recruiting in the United Kingdom

A Study of Lanadelumab in Teenagers and Adults with HAE in Argentina

– recruiting in Argentina

A Study of STAR-0215 in Participants with Hereditary Angioedema

– recruiting in the United States

A Study to Assess the Long-Term Safety and Efficacy of Donidalorsen in the Prophylactic Treatment of HAE

– recruiting in Belgium, Bulgaria, Canada, France, Germany, Israel, Italy, the Netherlands, Poland, Puerto Rico, Spain, Turkey, the United Kingdom, the United States



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EU Clinical Trials Register

A study to assess whether different doses of KVD824 are effective in preventing attacks of HAE Type I or Type II

– recruiting in Australia, Bulgaria, Canada, Czech Republic, France, Germany, Hungary, Italy, New Zealand, North Macedonia, Romania, the United Kingdom, the United States

A Study to Evaluate NTLA-2002 in adults with HAE

– recruiting in Australia, France, Germany, the Netherlands, New Zealand, the United Kingdom, the United States

A Study to Evaluate the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of BW-20805 in Healthy Subjects

– recruiting in Australia

A Study to Review the Treatment and Outcomes of Teenagers and Adults with Non-histaminergic Angioedema with Normal C1 Inhibitor in Canada

– recruiting in Canada

A Study with Lanadelumab in Persons with HAE in Poland

– recruiting in Poland

A Survey of Icatibant in Pediatric Participants with HAE

– recruiting in Japan

A Survey of Lanadelumab in Participants with HAE

– recruiting in Japan

An extension phase 2/3 study to test the safety of long-term administration of oral PHA-022121 for acute treatment of angioedema attacks in patients with HAE

– recruiting in Bulgaria, Canada, Czech Republic, France, Germany, Hungary, Israel, Italy, Poland, Spain, the United Kingdom, the United States

An Open-Label Extension Study of ISIS 721744 in Patients with Hereditary Angioedema

– recruiting in the Netherlands

An Open-label Extension Trial to Evaluate the Long-term Safety of KVD900, an Oral Plasma Kallikrein Inhibitor, for On-demand Treatment of Angioedema Attacks in Adolescent and Adult Patients with Hereditary Angioedema Type I or II

– recruiting in Australia, Austria, Spain, Bulgaria, France, Germany, Greece, Hungary, Israel, Italy, Japan, the Netherlands, North Macedonia, Poland, Italy, Germany, Portugal, Slovakia, South Africa, Spain, the United Kingdom, United States

An Open-label Extension Trial to Evaluate the Long-term Safety of KVD900 for On-Demand Treatment of Angioedema Attacks in Adolescent and Adult Patients with HAE

– recruiting in France, the Netherlands, the United States, Austria, Bulgaria, Germany, Greece, Hungary, Israel, Italy, Japan, North Macedonia, Poland, Slovakia, South Africa, Spain, United Kingdom

An Open-label Study to Evaluate the Long-term Safety and Efficacy of CSL312 (Garadacimab) in the Prophylactic Treatment of Hereditary Angioedema

– recruiting in Germany, Czechia, Hungary, the Netherlands, Italy

An open-label study to provide berotralstat access to subjects with type 1 and 2 hereditary angioedema who were previously enrolled in berotralstat studies

– recruiting in France, Czechia, Slovakia, Poland

An Open-Label, Long Term Safety and Efficacy Study of Donidalorsen in the Prophylactic Treatment of HAE

– recruiting in Belgium, Bulgaria, Canada, Denmark, France, Germany, Israel, Italy, the Netherlands, Poland, Spain, Turkey, the United Kingdom, the United States

Armenian NAtionwide REGistry of Systemic Autoimmune and Autoinflammatory Disease

– recruiting in Armenia

Berotrastat Treatment in Children with HAE

– recruiting in Austria, Canada, France, Germany, Israel, Italy, Poland, Romania, Spain, the United Kingdom

C1 Inhibitor Registry in the Treatment of Hereditary Angioedema (HAE) Attacks

– recruiting in Bulgaria, Croatia, Czechia, France, Germany, Hungary, Italy, North Macedonia, Norway, Poland, Slovakia, Slovenia, Sweden, Bulgaria, Croatia, Czechia, France, Germany, Hungary, Italy, North Macedonia, Norway, Poland, Slovakia, Slovenia, Sweden, the Netherlands

Characterization of Rhythmicity Profiles of Bradykinin-mediated Angioedema Attacks Using a Tracking Smartphone Application

– not yet recruiting

CLOUD-R HAE REGISTRY

– recruiting in France

CSL312_3003 Safety and Pharmacokinetic Study in Subjects 2 to 11 Years of Age with Hereditary Angioedema

– recruiting in Israel

Deployment to the Multidisciplinary Prospective Cohort (IMMINeNT)

– recruiting in France

Digital Biomarkers Study in Patients with Hereditary Angioedema in Japan: Exploratory Longitudinal Study about Trigger Factors of HAE Attacks

– recruiting in Japan

Extension Study of Oral PHA-022121 for Acute Treatment of Angioedema Attacks in Patients with HAE

– recruiting in Bulgaria, Canada, Czechia, France, Germany, Hungary, Israel, Poland, Spain, the United States

Firazyr General Drug Use-Results Survey (Japan)

– recruiting in Japan

Firazyr Patient Registry (Icatibant Outcome Survey – IOS)

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

Global Registry to Gather Data on Natural History of Patients with HAE Type I and II

– recruiting in Italy

HAE multi-national survey study

– recruiting in Argentina, Brazil, Colombia, Croatia, Denmark, Germany, Hungary, Ireland, Norway, Poland, Portugal, Romania, Sweden

Identification and Characterization of Genetic Variants in Hereditary Angioedema

– recruiting in Spain

Involvement of Monocytic B1 and B2 Receptors in Inflammation and Chronic Vascular Disease in Patients with Hereditary Bradykinetic Angioedema

– recruiting in France

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

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

– recruiting in Italy

Phase 1/2 Study to Evaluate Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of NTLA-2002 in Adults with HAE

– recruiting in France, Germany, the Netherlands, New Zealand, the United Kingdom

PK Subtrial in Adolescent Patients with HAE Type I or II Participating in the KVD900-302 Trial

– recruiting in Australia, Bulgaria, France, Germany, Greece, Israel, Japan, Netherlands, Slovakia, Spain, the United Kingdom, the United States

Safety, Tolerability, PK, PD of ADX-324 in Healthy Volunteers and HAE Patients

– recruiting in Australia

Status of Dental Care Practices in Patients with HAE

– recruiting in France

Stopping androgen treatment in patients with HAE

– **characterization of reasons and protocols and development of advice for patients and physicians**

– recruiting in Brazil, China, Germany, France, Hungary, India, South Korea, Oman, Peru, Portugal, Russia, Thailand, South Africa

The Role of the Coagulation Pathways in Recurrent Angioedema

– recruiting in France

You can check the latest status and read more about these and other clinical trials at:

- clinicaltrials.gov
- clinicaltrialsregister.eu
- trialsearch.who.int

NEWS FROM THE INDUSTRY

20 July 2023

Astria Therapeutics presented patient survey data at the 2023 US HAEA National Summit

Astria Therapeutics, who are developing STAR-0215 for the treatment of hereditary angioedema (HAE), presented information regarding patient preference for efficacy measures when starting on or switching to a new preventative therapy at the 2023 US Hereditary Angioedema Association (HAEA) National Summit in Orlando, Florida from 20-23 July 2023.

Sarah Friedhoff, Director of Patient Advocacy at Astria Therapeutics, presented the following paper at a poster session: People Living with Hereditary Angioedema (HAE) Prioritize Attack-Free Status as a Target for Therapeutic Efficacy.

(Source: Astria)



20 July 2023

KalVista Pharmaceuticals presented new HAE attack journey data at the 2023 US HAEA National Summit

KalVista Pharmaceuticals presented data on the hereditary angioedema (HAE) attack journey and a patient preference study at the 2023 US Hereditary Angioedema Association (HAEA) National Summit in Orlando, Florida, from 20-23 July 2023.

The following presentations occurred at the HAEA Summit:

- In Their Own Words - Patient Descriptions of the Earliest Recognition of HAE Attack Onset. Presenter: Dr. Michael E. Manning
- Recognizing the Importance of Early On-Demand Treatment in the HAE Attack Journey. Presenter: Dr. Michael E. Manning
- Preferences of People with Hereditary Angioedema for On-Demand Treatment: A US-Based Qualitative Study. Presenter: Dr. Paula J. Busse

Andrew Crockett, Chief Executive Officer of KalVista, commented: "The data presented at HAEA adds to the growing evidence that there remains an important unmet need for both people using only on-demand therapies as well as those on long-term prophylaxis."

By offering the first oral, on-demand medication in sebetralstat, we intend to address this gap in treatment as well as enable future physician/patient discussions about new options that may allow people with HAE to live better lives."

(Source: KalVista)



20 July 2023

Pharvaris Clinical Data presented at the 2023 U.S. HAEA National Summit

Pharvaris shared data from the RAPIDe-1 Phase 2 clinical study of PHVS416 (deucricitbant immediate-release capsules) for the on-demand treatment of HAE attacks, including data focused on time-to-symptom relief and resolution of HAE attacks, in two poster presentations at the 2023 U.S. Hereditary Angioedema Association (HAEA) National Summit in Orlando, Florida from 20-23 July 2023.

The two presentations were:

- Efficacy and safety of bradykinin B2 receptor antagonism with deucricitbant immediate-release capsule for treatment of hereditary angioedema attacks: results of RAPIDe-1 phase 2 trial. Presenter: Dr. Marc A. Riedl
- Treatment with oral bradykinin B2 receptor antagonist deucricitbant immediate-release capsule improves hereditary angioedema attack symptoms. Presenter: Dr. John Anderson

(Source: Pharvaris)



3 August 2023

BioCryst Pharmaceuticals provided a corporate update during reporting of financial results for the second quarter ended June 30, 2023

Charlie Gayer, Chief Commercial Officer of BioCryst, commented: “The significant step-up in revenue we expected and achieved in the second quarter reflects the continued strong growth in patients taking ORLADEYO, the normal seasonality in revenue that follows first quarter prescription reauthorizations, and our steady improvement in helping patients get to reimbursed therapy.

“We recently attended the HAEA summit, attended by 1,200 patients and family members. Their strong interest and enthusiasm gave us even more confidence in our expectations for sustained long-term demand for ORLADEYO in the U.S. and globally.”

Jon Stonehouse, President and Chief Executive Officer of BioCryst, said: “Our strong second quarter keeps us firmly on track to achieve no less than \$320 million in ORLADEYO revenue this year as our base of patients grows larger and larger every quarter. While ORLADEYO revenues continue to grow, we are also excited to host an R&D day in November to introduce new molecules and programs from our discovery platform that have the potential to replicate or exceed the success of ORLADEYO.”

Sales from outside the U.S. contributed 10.1 percent of global ORLADEYO net revenues in the second quarter.

ORLADEYO® (berotralstat) is an oral, once-daily treatment for the prevention of hereditary angioedema (HAE) attacks.

(Source: BioCryst)



3 August 2023

Over 70 new US patients enrolled on RUCONEST® in each quarter of 2023

As part of its reporting of the second quarter and the first half of 2023 financial results, Pharming indicated that underlying in-market demand for RUCONEST® in the U.S. continues to be strong. Pharming reported having received over 70 new patient enrollments in each quarter of 2023. The company concludes that this underpins the importance of RUCONEST® to HAE patients, including those patients on prophylaxis who should have medication on hand to treat any breakthrough attacks.

Chief Executive Officer, Sijmen de Vries, commented: “The second quarter of 2023 was strong for Pharming. After the reimbursement disruptions experienced in the HAE market in the first quarter, RUCONEST® recovered significantly in the second quarter, increasing 20% over the first quarter of 2023. Our RUCONEST® business performed well across a variety of leading revenue indicators, positioning us well for the second half of the year. RUCONEST® continues to provide a stable base for Pharming’s future growth, and we continue to foresee low single-digit growth in sales for the year.”

RUCONEST® is marketed for the treatment of acute HAE attacks.

(Source: Pharming)



3 August 2023

Progress of clinical trials of NTLA-2002 outlined during Intellia Therapeutics second quarter 2023 financial results presentation

Intellia announced that due to the substantial interest from physicians and patients to participate in the NTLA-2002 clinical program, all patients have been identified for the global Phase 2 portion of the study, with enrollment expected to be completed in the second half of 2023.

Based on the strong momentum of the program, the company plans to complete enrollment at ex-U.S. sites in the ongoing Phase 2 study. Further, following the March 2023 IND clearance, the FDA requested supplemental preclinical data related to the inclusion of female patients of child-bearing potential. Intellia expects to submit these data in advance of the planned Phase 3 trial, which will complement the clinical data collected from female patients of child-bearing potential dosed in the ongoing Phase 1/2 study.

Intellia plans to initiate the global pivotal Phase 3 study, including U.S. patients, as early as the third quarter of 2024, subject to regulatory feedback.

In June, Intellia announced additional positive interim results from the Phase 1 portion of the ongoing Phase 1/2 study of NTLA-2002. The company report that:

- Across all 10 patients, a 95% mean reduction in monthly attack rate was observed after a single dose of NTLA-2002 through the latest follow-up.
- The median duration of follow-up was 9.0 months (range of 5.6 - 14.1 months).
- At all three dose levels evaluated, NTLA-2002 has been well tolerated, and the majority of adverse events were mild in severity.
- These interim data were presented at the European Academy of Allergy and Clinical Immunology (EAACI) Hybrid Congress 2023, held in Hamburg, Germany, and virtually.

Intellia President and Chief Executive Officer John Leonard said: "For NTLA-2002, we are thrilled to see the excitement for this investigational therapy from the HAE community. In just a handful of months, we were able to identify all patients required to fully enroll in the ongoing Phase 2 study and now expect to initiate the pivotal Phase 3 program next year."

NTLA-2002 is designed to knock out the KLKB1 gene in the liver, with the potential to permanently reduce total plasma kallikrein protein and activity, a key mediator of HAE. This investigational approach aims to prevent attacks for people living with HAE by providing continuous reduction of plasma kallikrein activity following a single dose. NTLA-2002 is being evaluated in a Phase 1/2 study in adults with Type I or Type II HAE.

(Source: Intellia)



11 August 2023

Ionis Pharmaceuticals provided an update on two trials in HAE during reporting of financial results for the second quarter of 2023

Ionis Pharmaceuticals, who are developing Donidalorsen (formerly IONIS-PKK-LRx) for the prevention of HAE attacks, provided an update on two trials of the medicine, while reporting on financial results for the second quarter of 2023.

The company confirmed that they had:

- Completed enrollment of the Phase 3 OASIS-HAE study of donidalorsen in patients with hereditary angioedema; on track for data readout in H1:2024
- Reported positive topline Phase 2 open label extension data of donidalorsen in patients with hereditary angioedema treated for two years

Brett P. Monia, Ph.D., Chief Executive Officer of Ionis Pharmaceuticals, said: "Ionis is creating significant value in 2023 as we successfully execute on our strategy to bring a steady cadence of transformational medicines to the market. Our growing late-stage pipeline now includes eight medicines for 10 indications."

Donidalorsen, is an investigational ligand-conjugated antisense (LICA) medicine designed to target the prekallikrein (PKK) pathway. PKK plays an important role in the activation of inflammatory mediators associated with acute attacks of HAE. By inhibiting the production of PKK, donidalorsen could be an effective prophylactic approach to preventing HAE attacks.

(Source: Ionis)



11 August 2023

Pharvaris provided an update on clinical development of potential future medicine for HAE, as part of reporting on financial results for the second quarter of 2023

Pharvaris, a clinical-stage company developing novel, oral bradykinin-B2-receptor antagonists to treat and prevent hereditary angioedema (HAE) attacks, today reported financial results for the second quarter that ended June 30, 2023 and provided a business update.

Berndt Modig, Chief Executive Officer of Pharvaris, commented: “The completion of enrollment in our Phase 2 CHAPTER-1 prophylactic HAE study, provides momentum as Pharvaris prepares to announce our first in-patient prophylactic clinical data by the end of the year.

“Resolving the on-demand clinical hold of deucricitibant enables us to proceed with the clinical development of deucricitibant for the on-demand treatment of HAE; we intend to initiate our global Phase 3 on-demand study by the end of the year. We are focused on resolving the remaining clinical hold on deucricitibant for the long-term prophylactic treatment of HAE in the U.S. We appreciate the ongoing support of our external partners, including the investors in our most recent financing, which we believe demonstrates excitement for the Pharvaris story, confidence in our team’s ability to execute against key initiatives, and the need for oral therapies for the treatment of HAE that are efficacious, safe, and easy to administer.”

In addition to financial data, the company highlighted the following developments:

- CHAPTER-1, a global Phase 2 study of deucricitibant for the prophylactic treatment of HAE attacks, has completed the enrollment. CHAPTER-1, which is currently on hold in the U.S., was designed to enroll approximately 30 patients globally with a goal of evaluating deucricitibant as an oral prophylaxis against HAE attacks, using PHVS416 (immediate-release deucricitibant capsules) as proof of concept. The efficacy and safety of deucricitibant (10 mg and 20 mg, twice-daily) and placebo will be evaluated by comparing the number of investigator-confirmed attacks during participants’ 12-week treatment period. Data from this proof-of-concept study is expected to inform the design of an anticipated Phase 3 study utilizing PHVS719, a once-daily extended-release formulation of deucricitibant. Pharvaris anticipates announcing top-line data of CHAPTER-1 by the end of 2023.
- Following review of data from a preplanned interim analysis of the ongoing 26-week nonclinical study, the U.S. Food and Drug Administration (FDA) lifted the clinical hold on the Investigational New Drug (IND) application for deucricitibant for the on-demand treatment of HAE. The resolution of the hold has enabled Pharvaris to resume RAPIDe-2 in the U.S., an extension study of RAPIDe-1 evaluating PHVS416 for the on-demand treatment of HAE attacks.
- An End-of-Phase 2 meeting has been scheduled with the FDA, during which Pharvaris will seek feedback

and alignment on the key elements of a proposed Phase 3 clinical study for PHVS416. Globally, Pharvaris has been working on study startup activities with clinical site investigators and staff, and upon alignment with regulators, is prepared to initiate RAPIDe-3 by the end of 2023.

- The IND of deucricitibant for long-term prophylaxis remains on hold in the U.S. A 26-week nonclinical rodent toxicology study, which is intended to provide additional data to address the remaining hold in the U.S., is ongoing. Pharvaris anticipates submitting the results of this nonclinical study to the FDA by the end of 2023.
- Pharvaris presented data from clinical and non-clinical studies at the 13th C1-inhibitor Deficiency and Angioedema Workshop, the European Academy of Allergy & Clinical Immunology (EAACI) Hybrid Congress 2023, and the 2023 U.S. HAEA National Summit.

(Source: Pharvaris)

PHARVARIS

11 August 2023

Astria Therapeutics provided an update on development of STAR-0215 for HAE, as part of reporting on financial results for the second quarter of 2023

Astria Therapeutics, a biopharmaceutical company developing STAR-0215 for the treatment of hereditary angioedema (HAE) and focused on life-changing therapies for rare and niche allergic and immunological diseases, today reported financial results for the second quarter that ended June 30, 2023, and provided a corporate update.

Jill C. Milne, Ph.D., Chief Executive Officer at Astria Therapeutics, said: “We are making excellent progress with our STAR-0215 program and are proud to have received Fast Track Designation for the treatment of HAE.

“We believe that STAR-0215 has the profile of a potential first-choice preventative therapy for HAE based on its well-established mechanism, trusted modality, long half-life, and potential for infrequent dosing. We are looking forward to sharing additional data from our Phase 1a trial in Q4. We expect these results will provide additional information on dose selection for three and six-month administration,

with data on safety, tolerability, pharmacokinetics, and pharmacodynamics from a wide range of single dose levels.”

The company highlighted the following developments:

- STAR-0215 was granted Fast Track Designation by the U.S. Food and Drug Administration for the treatment of HAE.
- The ALPHA-STAR Phase 1b/2 trial of STAR-0215 in people with HAE is progressing well. Initial proof-of-concept results are expected in mid-2024. ALPHA-STAR is a global, open-label, proof-of-concept trial assessing single and multiple doses of STAR-0215 in patients with HAE types I and II. The trial is evaluating safety and tolerability, changes in HAE attack rate, pharmacokinetics, pharmacodynamics, and quality-of-life assessments. For each dose cohort, efficacy will be assessed at 3 months and 6 months after the last STAR-0215 dose administered.
- In the fourth quarter of 2023, the company expects to announce final results from three single dose cohorts in healthy subjects. Initial results from the two additional dose-ranging cohorts in healthy subjects are also expected to be shared in Q4. Overall, the data are expected to provide more information around plans for future trials, dose selection for potential three and six-month administration, safety and tolerability, and PK and PD from a wide range of single dose levels.
- A Long-Term Open-Label Trial named ALPHA-SOLAR will be open to participants from ALPHA-STAR and is expected to initiate in the fourth quarter of 2023. It will assess the long-term safety, tolerability, and efficacy of STAR-0215. Participants will be assigned to a dosing regimen based on their cohort assignment in the ALPHA-STAR trial and all are expected to receive STAR-0215 every three or six months.
- Pending proof-of-concept results from the ALPHA-STAR trial, Astria expects to progress directly to a pivotal trial.
- In June 2023, Astria presented at the European Academy of Allergy and Clinical Immunology Annual Meeting in Hamburg, Germany. Results included an overview of the design of the ALPHA-STAR clinical trial, a summary of the positive initial Phase 1a results of STAR-0215 in healthy subjects, and details about STAR-0215’s differentiated mode of plasma kallikrein binding.
- Human mechanistic modeling data were presented at the C1-Inhibitor Deficiency and Angioedema Workshop in May 2023. These data support the potential for STAR-0215 to be administered once

every three or six months for robust suppression of HAE attacks. Additionally, Principal Investigator Dr. Marcus Maurer presented the ALPHA-STAR trial, including design, dose selection rationale, and partnerships with the HAE community.

STAR-0215 is a monoclonal antibody inhibitor of plasma kallikrein in development for the treatment of HAE, with dosing every three or six months. The Phase 1b/2 ALPHA-STAR trial evaluating STAR-0215 in HAE patients is ongoing, with proof-of-concept results expected in mid-2024.

(Source: Astria)



16 August 2023

CSL provided an update on HAE clinical development as part of reporting on annual company results

CSL today reported financial results for the year that ended June 30, 2023 and provided a business update.

Dr Paul McKenzie, CSL’s Chief Executive Officer and Managing Director said, “Our strong performance in the 2023 financial year was delivered against a challenging operating environment. Our CSL Behring business rebounded strongly driven by exceptional growth in immunoglobulin sales and record plasma collections.

“We remained focused on executing on our strategy of delivering innovative medicines to best serve our patients and protect public health. This, combined with the efforts of our people is now delivering positive momentum for CSL and our patients in more than 100 countries.”

In addition to financial data, the company highlighted that its potential new once-monthly prophylactic treatment for HAE attacks, garadacimab (Anti-FXIIa), is being prepared for global regulatory submission.

(Source: CSL)

CSL Behring

18 August 2023

Eugia Pharma receives US FDA approval for generic icatibant

Aurobindo Pharma Limited has announced that its subsidiary company, Eugia Pharma Specialities Limited, has received final approval from the US Food & Drug Administration (FDA) to manufacture and market Icatibant Injection, 30mg/3mL (10mg/mL), single-dose prefilled syringe, which is bioequivalent and therapeutically equivalent to the reference listed drug Firazyr® (Icatibant Injection) by Takeda Pharmaceuticals USA Inc.

The product is to be launched in September 2023.

Icatibant Injection is indicated for the treatment of acute attacks of hereditary angioedema (HAE) in adults 18 years of age and older.

(Source: Aurobindo)



24 August 2023

ADARx Pharmaceuticals announces additional funding for clinical trials in HAE and other conditions

ADARx Pharmaceuticals, a clinical stage biotechnology company developing next generation RNA therapeutics, has announced the successful close of a \$200 million Series C financing.

The proceeds from the Series C financing will be used to further advance ADARx clinical programs including ADX-324 and ADX-038. ADX-324 represents an innovative approach for the treatment of hereditary angioedema, a rare genetic disease that causes painful, rapid, and potentially life-threatening swelling attacks.

Dr. Zhen Li, President and Chief Executive Officer of ADARx Pharmaceuticals, said: "The strong support from our new and existing investors is a testament to the significant progress we have made in advancing next generation RNA therapeutics for hepatic and extrahepatic targets.

"The early phase 1 clinical data strongly supports the potential of ADX-324 for patients suffering from

hereditary angioedema. Moreover, the clinical data validate our proprietary targeted delivery PLR™ platform and our broad SPE™ technology. With this financing, we are well-positioned to achieve our clinical milestones for multiple clinical programs and to build a leading company in RNA therapeutics."

(Source: ADARx)



25 August 2023

Astria Therapeutics to present patient survey data at HAEi EMEA Regional Conference 2023

Astria Therapeutics Inc, a biopharmaceutical company developing STAR-0215 for the treatment of hereditary angioedema (HAE) and focused on life-changing therapies for rare and niche allergic and immunological diseases, will present information regarding patient preference for efficacy measures when starting on or switching to a new preventative therapy, at the upcoming 2023 HAEi Regional EMEA Conference in Munich, Germany from September 1-3, 2023.

Remy Petersen, MD, Amsterdam University Medical Center, will present: 'People Living with Hereditary Angioedema (HAE) Prioritize Attack-Free Status as a Target for Therapeutic Efficacy' in a poster session beginning on Friday, September 1, at 7:30pm CEST.

(Source: Astria)



29 August 2023

KalVista Pharmaceuticals to Present HAE Attack Journey Data at 2023 HAEi Regional Conference EMEA

KalVista Pharmaceuticals, Inc, a clinical stage pharmaceutical company, today announced the acceptance of multiple abstracts at the 2023 HAEi Regional Conference EMEA meeting taking place in Munich, Germany from 1-3 September 2023. The presentations are:

- In Their Own Words - Patient Descriptions of the Earliest Recognition of HAE Attack Onset: Michael E. Manning, Autumn F. Burnette, Sally van Kooten, Markus Heckmann, Sherry Danese, Ledia Goga, Mar Guilarte, Anna Valerieva (presenter only). Results shared as a poster presentation and Q&A
- Recognizing the Importance of Early On-Demand Treatment in the HAE Attack Journey: Douglas H. Jones, Princess Ogbogu, Sally van Kooten, Markus Heckmann, Sherry Danese, Ledia Goga, Markus Magerl (presenter). Results shared as a poster presentation and Q&A

(Source: KalVista)



31 August 2023

Astria Therapeutics Announces Publication of STAR-0215 Preclinical Data

Astria Therapeutics, a biopharmaceutical company developing STAR-0215 for the treatment of hereditary angioedema (HAE) and focused on life-changing therapies for rare and niche allergic and immunological diseases, today announced that preclinical data supporting STAR-0215's profile as a potential long-acting therapy for hereditary angioedema (HAE) dosed once every three or six months has been published in the Journal of Pharmacology and Experimental Therapeutics.

Jill C. Milne, Chief Executive Officer at Astria Therapeutics, said: "We were very pleased by our publication in the Journal of Pharmacology and Experimental Therapeutics, further validating the exciting science behind STAR-0215. These results demonstrate that STAR-0215 shows high potency in

vitro and in vivo and long half-life, suggesting potential for sustained, durable HAE attack suppression with subcutaneous administration."

(Source: Astria)



7 September 2023

KalVista Pharmaceuticals Gives Operational Update on Potential HAE Medicine

KalVista Pharmaceuticals, Inc, a clinical stage pharmaceutical company, provided an operational update and released financial results for the first fiscal quarter ended July 31, 2023.

Andrew Crockett, Chief Executive Officer of KalVista, said: "We know from our extensive interactions with people living with hereditary angioedema (HAE) at the recent conferences how much they are anticipating having sebetralstat available to them, and the data from the KONFIDENT trial in Q4 is the next step.

"We continue to build our Commercial operation with the addition of Nicole Sweeny as Chief Commercial Officer and other key members of the team to support an NDA submission in the first half of 2024 and a rapid launch upon FDA approval."

Relevant business highlights include:

- Published "Evaluation of patient-reported outcome measures for on-demand treatment of hereditary angioedema attacks and design of KONFIDENT, a phase 3 trial of sebetralstat" in Clinical and Translational Allergy. This publication outlines the rigorous, multi-factorial approach used to design a phase 3 trial with an optimized, patient- and physician-preferred measure to assess measure efficacy, and discusses reasons for the superiority of the selected primary endpoint over other potential measures
- Presented real-world patient data on the HAE Attack Journey at the HAEi Regional Conference EMEA meeting showing there remain important unmet needs for both people using only on-demand therapies as well as those on long-term prophylaxis
- Achieved enrollment target of 114 patients in the phase 3 KONFIDENT trial. Data readout remains on

track for the fourth quarter of 2023 and, if the trial is successful, KalVista anticipates submitting an NDA to the FDA in the first half of 2024

- Presented real-world patient data at the 2023 US HAEA National Summit showing people living with HAE continue to delay treatment for attacks due to challenges from injections or infusions, even though they know early treatment means an earlier return to normal activities
- Revealed patient survey data at the 13th C1-inhibitor Deficiency & Angioedema Workshop demonstrating the burden associated with injectable on-demand treatments for HAE and that those living with HAE had a strong preference for oral medication for on-demand treatment of attacks over self-administered injectable treatments when efficacy and safety profiles were similar
- Continued to advance plans for the eventual worldwide launch of sebetralstat by hiring a General Manager, Japan. Based on interactions to date with Japanese physicians and regulatory authorities, KalVista believes sebetralstat has the potential to significantly improve HAE therapeutic options in Japan.

(Source: KalVista)



19 September 2023

BioCryst Pharmaceuticals announced that ORLADEYO has been recommended for public reimbursement in Québec, Canada

BioCryst Pharmaceuticals today announced that the Institut national d'excellence en santé et services sociaux (INESSS) has issued a positive recommendation for ORLADEY (berotralstat) to be reimbursed for the prevention of hereditary angioedema (HAE) attacks in adults and pediatric patients 12 years of age and older in Québec.

Anand Janack, vice president and general manager of BioCryst Canada, said: "We are committed to bringing ORLADEYO to as many Canadians living with HAE as possible. This announcement from INESSS, which follows the positive recommendation from CADTH earlier this year, takes us one step closer to making our oral, once-daily therapy available to Canadians who are in need of a new option to help improve control of their HAE attacks."

ORLADEYO (berotralstat) is an oral, once-daily treatment for prevention of HAE attacks.

(Source: BioCryst)



26 September 2023

Takeda receives CHMP opinion recommending approval of lanadelumab for prevention of HAE attacks in patients aged 2 years and older

Takeda today announced the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) has recommended the approval of lanadelumab for the routine prevention of Hereditary Angioedema (HAE) in patients aged 2 years and older. If approved, lanadelumab will be the first long-term prophylactic treatment available in the EU for patients under the age of six. The European Commission (EC) will consider the CHMP positive opinion and decide upon potential marketing authorization in the coming months. Lanadelumab is currently indicated for the routine prevention of recurrent attacks of HAE in patients aged 12 years and older.

Didier Relin, Head of International Regulatory at Takeda, said: "We are so pleased to achieve this positive step towards providing the first long-term prophylactic treatment option to prevent attacks in this vulnerable population. We know that HAE can be a complex, debilitating condition, and we are committed to being a champion for all individuals living with HAE."

(Source: Takeda)



6 October 2023

Ionis shares significant business and pipeline progress at Innovation Day, including on donidalorsen

Ionis Pharmaceuticals today held an investor and analyst event, Ionis Innovation Day: Discovering, Developing and Delivering Transformational Medicines. Presentations from Ionis leaders and external physicians highlight key pipeline and technology highlights.

Brett P. Monia, Ph.D., Ionis' Chief Executive Officer, said: "Our leadership in RNA-targeted medicine includes multiple modalities empowering us to advance the right medicines for the right targets. Our powerful and prolific research and development engine has discovered ground-breaking medicines for people with devastating diseases."

As part of the Innovation Day, an update on the potential HAE treatment, donidalorsen was provided:

- Donidalorsen was recently granted U.S. Orphan Drug Designation
- Ionis plans to report Phase 3 results with donidalorsen for prophylactic treatment of hereditary angioedema (HAE) in the first half of 2024. Ionis also plans to report topline data from a second Phase 3 study evaluating switching from currently approved HAE treatments in mid-2024.
- Two-year results from the Phase 2 open-label-extension trial showed that donidalorsen treatment resulted in a 96% overall sustained mean reduction from baseline in HAE attack rates. These data will be presented at a medical congress later this year.
- Raffi Tachdjian, M.D., MPH, FAACAP, FACP, Associate Clinical Professor of Medicine and Pediatrics UCLA School of Medicine; Chief, Division of Allergy and Immunology Providence St. John Medical Center will discuss the need for new prophylactic HAE treatments

as more than one-third of patients on prophylactic treatment report more than two attacks per month.

(Source: Ionis)



13 October 2023

Intellia Therapeutics Receives Priority Medicines (PRIME) Designation From the European Medicines Agency for NTLA-2002

Intellia Therapeutics, Inc today announced that the European Medicines Agency (EMA) has granted Priority Medicine (PRIME) designation to NTLA-2002 for the treatment of hereditary angioedema (HAE).

NTLA-2002 is an in-vivo CRISPR-based investigational therapy designed to prevent potentially life-threatening swelling attacks in people with HAE.

The PRIME designation was created by the EMA to provide early and proactive support to developers of promising medicines to optimize their development plans and speed up evaluation. The goal is to help patients benefit as early as possible from innovative new therapies that have demonstrated potential to significantly address unmet medical need.

Intellia President and Chief Executive Officer John Leonard M.D, said: "We are very pleased the EMA has granted PRIME designation to NTLA-2002. This designation provides valuable regulatory benefits and highlights the potential of our novel in-vivo gene editing candidate to address an unmet medical need for people living with hereditary angioedema. We look forward to continuing to work closely with the EMA and regulatory agencies around the world to bring this innovative therapy to patients as quickly as possible."

(Source: Intellia)





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Features of HAEi's HAE TrackR App

- ✓ Easy-to-use electronic diary
 - ✓ Safe and secure – all data is the sole property of the user
 - ✓ Store and share data about your HAE with your physician
 - ✓ Product and company neutral with no commercial interests
 - ✓ Endorsed by the ACARE network
 - ✓ Accessible from anywhere at any time and in many languages
- New Features added in Version 2:**
- ✓ Smart reminder functionality for prophylactic treatments
 - ✓ Improved reporting for easy sharing of data with physician
 - ✓ Option to only list preferred/used treatment(s)
 - ✓ Option to add clinical trial medication if needed
 - ✓ Upload function for photo(s) of batch/LOT number for documentation



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HAEi AROUND THE WORLD

Currently there are HAE member organizations in **98** 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 ACARE centers, hospitals, physicians, and available medication.

The information on **haei.org** is being updated as soon as HAEi receives fresh data from the national member organizations.

