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Global perspectives:

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99 Member countries

HAEi REGIONAL CONFERENCE AMERICAS PANAMA CITY 15-17 MAR 2024

Global perspectives

Global Perspectives
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April 2024

Cover photo
Participants from 26 countries came together in Panama City, Panama, for the **2024 HAEi Regional Conference Americas** – read more on page 20

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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,

We are thrilled to share the highlights of the tremendously successful **2024 HAEi Regional Conference Americas** that brought together over 650 delegates from North, Central, and South America. An intense sense of community was on display at the Panama City, Panama venue. The event came alive as people with HAE, caregivers, healthcare professionals, and industry representatives enthusiastically committed to applying the conference theme – **"Take Action!"**. Each participant left the conference empowered and equipped with the knowledge and support to make a positive impact on the health and well-being of their local HAE community. We invite you to read the feature article that provides key conference highlights.

The success and tangible impact of the **2024 HAEi Regional Conference Americas** serves as a motivating force for the HAEi team that is planning the upcoming **2024 HAEi Global Leadership Workshop** (GLW). Scheduled to take place in Copenhagen, Denmark, in October, this pivotal event stands out for several reasons. Firstly, it serves as a vital platform where key patient opinion leaders from our extensive network of Member Organizations worldwide come together to share insights, foster collaboration, and drive our collective mission. Secondly, we are thrilled to announce that the scientific sessions will be led by HAEi's partner, ACARE, and mark the debut of the **Global Angioedema Forum** (GAF). The synergy of GLW and GAF represents a pioneering step towards a meaningful convergence of patient advocacy and scientific exploration. We eagerly anticipate welcoming many friends and colleagues to Copenhagen for the trailblazing GAF and GLW events.

This Global Perspectives issue is a testament to HAEi's ongoing growth and global reach. Our 11 Regional Patient Advocates have been working tirelessly and we are excited to bring you updates on their important activities. These updates serve as a reminder of the vibrant, active, and growing HAEi network that is united in the fight for better access to and reimbursement for HAE therapies.

As we approach **hae day :-)**, we invite each of you to participate in our Activity Challenge. Every activity you engage in during April and May will be converted into steps, which helps us symbolically circle the globe multiple times, and underscores our global solidarity. Your every activity counts and contributes to this significant cause.

Finally, this issue includes valuable updates from both **ACARE** and our **HAEi Youngster's Community**, along with insights from our Member Organizations worldwide. You will also find the latest on medical papers, clinical trials, and industry news, ensuring you are well-informed about every aspect of HAE.

As we continue to grow and evolve, your involvement and support remain our greatest strength. Together, we are shaping the future of HAE care and advocacy. Thank you for your continued commitment and happy reading!

Warm regards

Anthony J. Castaldo
Chief Executive Officer and
Chairman of the Board, HAE International

Henrik Balle Boysen
President, HAE International

IN THIS ISSUE OF GLOBAL PERSPECTIVES

HAEi REGIONAL CONFERENCE
AMERICAS
PANAMA CITY
15-17 MAR 2024

NEWS FROM HAEi'S REGIONAL PATIENT ADVOCATES

HAEi Regional Patient Advocates – Global Strength Through Regional Presence!

Welcome to the first 2024 edition of Global Perspectives magazine, and I wish you all an amazing year ahead! HAEi doesn't stand still and constantly strives to improve the Quality of Life for HAE friends all over the world. Our effectiveness results from our decentralized global footprint, and our Regional Patient Advocates (RPAs) are always ready to provide support. These highly talented people focus on networking and identifying HAE patients to assist them in creating new Member Organizations that will soon be ready to intensely advocate for access to and reimbursement for life-saving HAE therapies.

This is what happened in Q1 this year. Our efforts peaked in the **2024 HAEi Regional Conference Americas** held in Panama City, Panama, from 15-17 March. 650 attendees from North, South, and Central America met up to witness mutual understanding and multidisciplinary collaboration to bring a brighter future for all HAE patients from this region. I want to say a heartfelt thank you to Fernanda de Oliveira Martins, RPA for South America and Mexico, Javier Santana, RPA for Central America and the Caribbean, and Anthony J. Castaldo, RPA for North America, for their tireless efforts to improve the situation in the Americas. I encourage all of you to read more about this exciting regional conference in this edition of the Global Perspectives magazine.

Besides the **2024 HAEi Regional Conference Americas**, all RPAs were working hard to inform our Member Organizations about another exciting event later this year. The **2024 HAEi Global Leadership Workshop** is designed for the leaders/key people of the Member Organizations and organized in parallel with the first ACARE Global Angioedema Forum 2024, a meeting dedicated to physicians and scientists.

Also, RPAs attended different HAE meetings organized by either patient organizations or physicians' societies. As a result, HAEi was present and represented in:

- San Jose, Costa Rica, in February at the local patient meeting
- Abu Dhabi, United Arab Emirates, in February at the ACARE Preceptorship and 3rd Emirates Allergy and Clinical Immunology Conference
- Kaunas, Lithuania, in February, at the local patient and physician meeting
- Milan, Italy, in March at the ITACA Conference, and
- Lisbon, Portugal, in March at the local patient meeting.

All RPAs' activities aim to increase awareness of HAE and support the wider availability of HAEi's tools and resources translated into various languages. The collection of resources expands very quickly! Check them all, and I am sure you will find something tailored to your needs.

Please enjoy news from HAEi's RPAs, and please do not hesitate to contact them if you need to know more about the regions they serve.

Michal Rutkowski
Director, Regional Patient Advocate Program

FERNANDA DE OLIVEIRA MARTINS
SOUTH AMERICA AND MEXICO

The **2024 HAEi Regional Conference Americas** in Panama was amazing. It was a powerful source of inspiration for HAE patients, caregivers, and everyone involved with HAE. The conference equipped Member Organizations (MO) with knowledge, fostered collaboration and empowered the MOs to continue their work and make a real difference in the lives of HAE patients across the Americas. Seeing the progress being made on a regional scale inspired patients to return home with renewed purpose and a clear action plan.

MOs were very glad and thankful for having, for the first time, a regional meeting with presentations in Spanish and Portuguese, in addition to English. The participants also recognized and thanked HAEi for its efforts to make Wordly ready for use, allowing people to understand all presentations and get the most out of their participation.

For the first time during this conference, representatives from all the Americas region were invited to speak on stage and share their countries' situations, experiences, and stories. Participants liked the opportunity to hear and learn more about the condition of HAE patients and the resources, such as access to modern therapies, in the different countries in the Americas region.

I want to express my gratitude to everyone who made the effort to be there, as well as the entire HAEi team, for putting this event together and making it possible!

Now, let's "TAKE ACTION".

Although we just delivered the **2024 HAEi Regional Conference Americas**, we are already looking forward to the next opportunity to be together again. The MOs recently informed the HAEi team about the number of leaders in each country willing to participate in the **2024 HAEi Global Leadership Workshop** in October.

Follow @haei_sudamerica_y_mexico on Instagram.

FIONA WARDMAN
ASIA PACIFIC AND SOUTH AFRICA

During the last few months, I have been working closely with South Africa and India. They are working towards impactful patient meetings aimed at bolstering support and awareness for HAE patients. These gatherings will serve as crucial platforms for patients, caregivers, and healthcare professionals to come together, share experiences, and discuss advancements in HAE management.

Meanwhile, in Bangladesh and Nepal, efforts are underway for me to engage with government officials in discussions about the importance of raising awareness of HAE and improving access to modern treatments. These initiatives will highlight the growing recognition of HAE as a significant health concern that requires urgent attention and action.

I have also received an invitation to participate in a physician meeting in Bangladesh, which will coincide with the meeting with the government. This opportunity will allow further collaboration and discussion on HAE management and awareness.

A key focus for the HAE community is the upcoming **2024 HAEi Global Leadership Workshop**, where advocacy leaders from around the world will convene to discuss strategies for advancing HAE awareness and care. I have been working with countries to secure travel grants to ensure that Member Organization representatives from all countries can participate, contributing to a more inclusive and impactful event.

Additionally, collaboration with Dr. Li from Hong Kong and Debs, HAEi's Chief Scientific Officer, on reporting results for a survey completed by physicians in the Asia Pacific region underscores the commitment to HAEi's impact within the community.

HAEi's tools and resources are being utilized to provide general support and guidance to HAE organizations worldwide. These resources play a vital role in empowering communities to advocate for improved access to treatments, raise awareness, and enhance the quality of life for HAE patients.

JAVIER SANTANA
CENTRAL AMERICA AND CARIBBEAN

HAEi delivered the first **2024 HAEi Regional Conference Americas** in Panama City, Panama, where hundreds of patients, caregivers, doctors, and pharmaceutical representatives met to talk about advances of HAE in the Americas region, new treatments, and empowering patients, caregivers, and their families in the search for a better quality of life. The event was a complete success, and many patients, family members, and medical specialists expressed their gratitude to HAEi for the event. The content of the presentations, the information shared by professionals, and the variety of sessions demonstrated to the patients that there is a positive future in their respective countries regarding HAE.

Good news! Costa Rica now has generic icatibant available in even more hospitals. The Costa Rican doctors tell me they can prescribe this medicine. Berinert continues to only be available in hospitals for emergency situations. However, it is a good advance that Costa Rica now has two treatments for HAE. I visited Costa Rica to meet with patients, doctors, and HAE treatment representatives there. I also had the opportunity to visit the main Pediatric Hospital, where I shared information and HAEi tools to educate doctors and other healthcare professionals about HAE.

In the Dominican Republic, the HAE treatment distributors for the Caribbean region are in talks with government officials to explore the possibility of registering specific medications for HAE. The same happens in Panama due to the **2024 HAEi Regional Conference Americas**.

In Puerto Rico, HAE patients continue to work hard so that the country's governor will finally sign the law on Hereditary Angioedema. In the past months, patients have been visiting the media to discuss the issue.

The US HAEA, in coordination with Dr. Christina Ramos from Puerto Rico and Takeda Pharmaceuticals, held a virtual conference in Spanish about lanadelumab. Patients and doctors from Puerto Rico and other Spanish-speaking countries in my region participated.

ANOTHONY J. CASTALDO
NORTH AMERICA AND SOUTH EAST ASIA

NORTH AMERICA

HAEi has frequent and productive contact with the HAE Canada (HAEC) Team, including President Michelle Cooper, Past President Jacquie Badiou, and Chief Operating Officer Daphne Dumbrille. HAEC is working to promote HAEi Advocacy Academy to their 500 members and make the HAEi Emergency Department poster available in Canada. We were pleased to visit the capital city of Ottawa and meet with HAEC's leadership along with a key HAE physician/scientist in late 2023. Discussions centered on the progress in getting new HAE medicines approved and reimbursed in Canada and how HAEi can help.

HAEi helped HAEC overturn a regulatory decision denying approval of a modern HAE prophylaxis therapy. Our role in the process involved writing a forceful letter to the Canadian drug approval authorities that included an impactful contribution from our Chief Medical Advisor, Prof. Marcus Maurer. The letter communicated our surprise at the denial and noted that Canada stands as an outlier since many other countries had already approved the medicine and were providing reimbursement.

HAEC has been granted a coveted "accreditation" from Imagine Canada, an organization that evaluates non-profit entities against a very high standard for governance and overall effectiveness. The organization is also hard at work preparing its National Report Card, which will provide stakeholders with an overview of key activities and accomplishments. Finally, HAEC's leadership and members made important contributions in various sessions at the **2024 HAEi Regional Conference Americas**.

The US HAE Association (HAEA) continues to serve its 10,516 members with personalized programs and services to improve the community's overall health and well-being. HAEi often provides advice and collaborates on research projects, demonstrating the burden HAE imposes and the transformative impact of modern HAE treatments. Recent HAEA medical journal publications include studies that show (1) dramatic quality-of-life improvements in people receiving preventive treatment and (2) the impact of insurance delays in denials in terms of increased attacks, ER and hospital visits, and feelings of anxiety. The HAEA recently submitted for publication a paper that summarizes the development of a new, comprehensive HAE Quality of Life instrument that we believe can also be validated for use in many other countries. A manuscript for a study that estimates the prevalence of HAE in the United States is currently being drafted and will be submitted to a medical journal in late May.

The HAEA is planning a special gala to honor people who receive college financial assistance from its Pam King Scholarship Fund. The gala will feature inspirational stories of success from recipients who have used the scholarship money to earn college degrees that propelled them into successful careers. Finally, in May, the HAEA will bring a large group of community members to Washington, DC, for visits with elected officials and discussions about the needs of people with HAE.

UPDATE FROM SOUTH EAST ASIA

We are very excited to report on great progress in the Southeast Asia region.

Late last year, we held meetings with both the patient organization and physicians in Thailand. The **2023 HAEi Regional Conference APAC**, which took place in Bangkok in March 2023, has dramatically increased the "in-country" awareness of HAE. Our Thailand Member Organization has definitive plans to expand outreach and enhance HAE awareness. Through dialogue with

HENRIK BALLE BOYSEN
SOUTH EAST ASIA

physicians in Thailand (primarily Dr. Chantaphakul), we have been informed that there is a large cohort of people with HAE in Thailand. Dr. Chantaphakul and his colleagues have the capability to run clinical trials, and we are therefore pursuing options to bring trials to Thailand. Furthermore, the Thai Food and Drug Administration (FDA) appears willing to accept approved HAE medicines, thereby opening a path to securing access for people with HAE in Thailand.

We plan to conduct the HAEi "Baseline Burden of Illness Survey" this year in Thailand. We will meet with the team again in May to follow up on the many activities currently underway. We also plan activities with the Allergy, Asthma, and Immunology Association of Thailand (AAIAT) to speed up the process further in Thailand.

In Vietnam, we continue to work with Dr. Dinh to find patients interested in and motivated to become the national point of contact. Dr. Dinh has the capability to run clinical trials at his clinic in Hanoi, and we are working to see if there are any options to initiate a clinical trial there. As part of HAEi-initiated programs in Vietnam, we plan to run our "Heat Map Survey" and "Baseline Burden of Illness Survey" in 2024. We will meet with the team in Hanoi later in the year to continue working on the ambitious plans for getting a vibrant patient organization up and running in Vietnam.

We are making great strides in building our Member Organization and coordinating with the physician community in the Philippines. We now have an HAE Philippines website and are working to translate our Emergency Department poster and Understanding HAE guide into Tagalog. We have regular contact with the MO lead and are working to help her use HAEi resources to expand membership. As more patients are identified, an inaugural patient meeting becomes a realistic goal for early 2025.

Three physicians, including the President of the Philippine Society of Asthma, Allergy, and Immunology (PSAAI), have taken a special interest in HAE and have used a version of our heat map methodology to identify potential patients throughout the country. These physicians are working with HAEi on a plan that will result in opening and accrediting an ACARE Center in Manila within the next two years. We continue working to raise HAE awareness in the physician community. As part of this effort, we arranged for Dr. Marc Riedl to provide an overview of how to diagnose HAE at the last PSAAI Annual Meeting.

These three Southeast Asian countries plan to participate in the upcoming **2024 HAEi Global Leadership Workshop** and the **Global Angioedema Forum 2024** in Copenhagen in October.

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

I am happy to share the great progress and big steps we have been making in the Nordics, DACH (Germany, Austria, Switzerland), and Israel. It is really rewarding to see how our combined efforts are paying off, raising awareness, and offering support to everyone with HAE and their caregivers.

We have so many different activities ongoing, all aimed at making more people aware and knowledgeable about HAE. Social media has been crucial in all these countries, helping people find information and connect with others.

An event I have mentioned previously is from HAE Scandinavia, and the lasting impact of this meeting really emphasizes how important it was. A big moment for HAE Scandinavia was their 5th Scandinavian Conference in Malmoe, Sweden, from 10-12 November 2023. Over 100 people came together, including patients, caregivers, healthcare professionals, and representatives from pharmaceutical companies. Bringing together such a diverse group for talks, discussions, and networking was incredibly valuable. It motivated and educated everyone involved and gave them confidence in dealing with HAE. I hope it will inspire others to organize similar meetings.

In another exciting development, HAE Scandinavia, working with a student from the HAEi LEAP 23 program, launched the SumsUp app.

This app, which started as a LEAP 23 project, is now live and helps patients, caregivers, and others navigate the complexities of HAE. It is a big win, showing the success of the LEAP 23 program and the power of collaboration in bringing helpful tools to our community.

Looking ahead, we are planning patient meetings across the region in 2024, promising a busy year ahead. I am especially looking forward to these gatherings and meeting members of our community.

The Swiss HAE Organization has made a short film that explains HAE in simple terms. Available in Swiss-German with subtitles in several languages, it is an excellent resource for anyone wanting to learn more about HAE. I strongly recommend watching this film: https://bit.ly/hae-simply-explained.

We are also focused on making resources easy to access. For example, Austria and Finland are interested in the free website hosting offered by HAEi, ensuring organizations have a strong online presence.

We are involved in an important study examining the costs and quality of life related to HAE treatments in Germany, Switzerland, and Austria. I urge patients and caregivers in these countries to participate in the survey: https://tinyurl.com/HAEUmfrage.

It is open until the end of May 2024, and the responses will help us understand the impact of HAE treatments in these countries.

There is so much going on in our region. The hard work of HAE organizations in creating active communities is amazing. Let's keep up the engagement, participation, and support for each other.

Finally, I wish everyone a wonderful **hae day :-)** and look forward to the happiness and togetherness it brings. Together, we are making a real difference in the lives of those affected by HAE, both now and in the future.

MARIA FERRON
MEDITERRANEAN, NORTH AFRICA, BENELUX, AND BRITISH ISLES

The year started with the incredible news that I had gained 3 new countries as part of my Regional Patient Advocate (RPA) responsibilities: the Netherlands, Belgium, and Luxemburg, also known as Benelux.

2024 looks like a very exciting year for HAE. The countries in my region closed 2023 with a large number of advocacy activities, and this year, they are very active and have great enthusiasm to continue with the great work they are doing at the local level. You can read some of the great work done locally in our section "News From Around the World," but here you have some examples:

The Spanish organization AEDAF (HAE Spain) has been working on several projects with pharmaceutical companies. They are also organizing its 26th General Assembly and Annual Meeting, which will take place on 13 April at Hospital Universitario La Paz, Madrid.

The President of HAE Netherland, Marijk Beekman-Kortekaas, has been actively working on setting up the association's 2024 plans. HAE Netherlands has submitted a project for the fifth edition of CSL Behring's European Local Empowerment for Advocacy Development (LEAD) Grants.

HAE Tunisia is running an awareness campaign in different regions of the country. The campaign they ran in the south resulted in 37 newly diagnosed patients.

In January, I had the honor to be invited to talk about HAEi and share my patient story with the BioCryst EMEA team at their internal meeting, "Shaping the Future." It was a great opportunity for all BioCryst employees to hear about the amazing work that HAEi is doing around the globe and to understand how their work impacts the lives of HAE patients.

February was an active month for local advocacy activities taking place thanks to Rare Disease Day on 29 February. Some examples are Dr Moussayer, President of the Alliance of Rare Diseases in Morocco and Vice-President of HAE Morocco, who organized a "Rare Diseases Day" in Casablanca, including a walk as part of their advocacy activities. In Ireland, the Member Organization lead Bettina Carty, took part in the "I Am Number 17" campaign to spread the word about rare diseases.

During March, I was also honored to be invited to two great meetings: the joint ITACA—A.A.E.E meeting in Milan from 14-16 March and the first face-to-face ADAH (HAE Portugal) annual meeting in Lisbon.

ITACA is the Italian Network for Hereditary and Acquired Angioedema, and A.A.E.E is HAE Italy. I attended the joint ITACA—A.A.E.E meeting with Michal Rutkowski, HAEi's Vice President and Director, RPA Program. Michal had the opportunity to share HAEi's work with the ITACA network of doctors, and we had the pleasure of hearing Stefano Del Giacco, current EAACI President, share the work that the Academy is doing.

After the ITACA meeting, I flew to Lisbon to attend the first face-to-face ADAH meeting. During the meeting, I discussed the great tools and resources that HAEi offers to Member Organizations to help associations run effectively and provided an overview of our advocacy activities. 45 Members attended the meeting, and another 20 joined online.

I am really looking forward to all the amazing activities that will take place during 2024!

LIM YONG HAO
EASTERN ASIA

The new year is a natural juncture for reflecting on the year that has just ended and thinking about the prospects for the year ahead. I was very fortunate to end 2023 on a high note with an invitation to speak on patient advocacy at the LeadHAE Summit in Bangkok. I reconnected with familiar faces, made new connections, got fresh insights, explored novel perspectives, and uncovered promising patient and physician leads.

Preparing for my presentation allowed me to dive deeper into what patient advocacy, particularly within the context of HAE, meant for me personally. For patients with HAE, the aspiration is for a "normal life" - characterized by well-managed symptoms and unhindered lifestyle choices. To achieve this, advocacy efforts must confront five pivotal pillars: ensuring unconstrained access to treatments, facilitating early diagnosis, mastering trigger management, mitigating social stigma, and reshaping public perceptions.

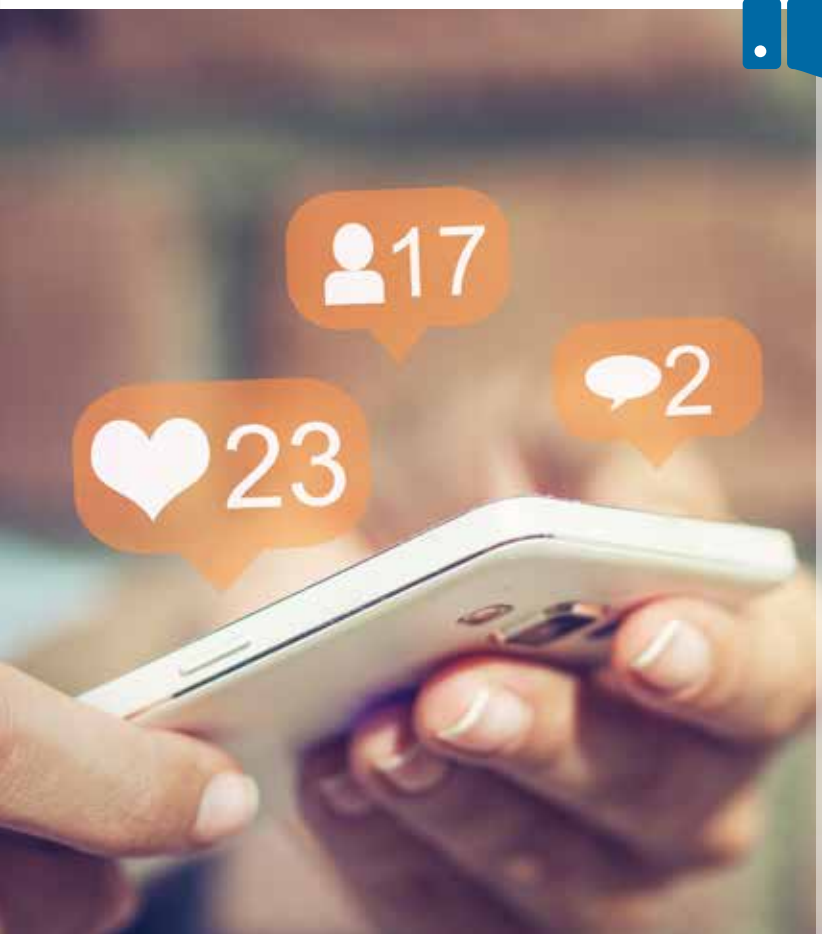
The issue of treatment access resonated deeply during these deliberations. Access to treatment extends beyond mere availability; it encompasses factors such as ease of access, affordability, and the availability of a diverse array of treatment options tailored to individual patient needs. Hong Kong (HK) has made commendable strides in this regard, offering Berinert at no cost since 2023, with icatibant to follow in April 2024. Nevertheless, the absence of modern prophylactic treatments remains conspicuous. Conversely, China and Taiwan have made notable headway, providing access to both modern on-demand and prophylactic treatments, with icatibant and lanadelumab available in both locales. Yet, in China, regional disparities in subsidies and physical availability hindered equitable access across the different provinces; in Taiwan, a highly restrictive indication for lanadelumab excludes the majority of the patients. Modern treatments are still unavailable in Singapore, Malaysia, Indonesia, and Sri Lanka, leaving patients to resort to second-line treatments like attenuated androgens and tranexamic acid. Although patients in Singapore may access Berinert in the Emergency Department and potentially obtain named access to Haegarda, lanadelumab, or icatibant, the costs are prohibitively high. Therefore, while there have been notable improvements in treatment access within the region, significant gaps remain in availability, affordability, appropriateness, and options. Supporting access to treatment remains a top priority for me in the coming year.

Dialogue with Member Organizations underscored a surprising concern: the importance of sustaining the momentum of HAE patients, particularly in regions where treatment access has improved. There is a perception that since modern treatments are now available, the advocacy job is "complete," and the respective Member Organizations can rest on their laurels. This perception poses a threat to the continuity of advocacy efforts. There are many avenues through which advocacy can continue to effect progress, ranging from facilitating early diagnosis to fostering trigger management strategies and bolstering public awareness. HAE HK is expanding beyond patient meetings to include workshops or activities geared towards trigger management, such as stress management, to benefit patients. HAE Taiwan has kickstarted 2024 with a patient meeting to maintain momentum and aims to increase attendance from their patient community. In Singapore, I'm preparing to officially register the patient group, enabling fundraising and the organization of more engaging activities to involve patients, physicians, and the public. Additionally, we're exploring the feasibility of establishing a physician workgroup for HAE, drawing inspiration from successful initiatives in Thailand.

In addition to supporting local initiatives, I have also been fostering connections with the global HAE community and enhancing access to helpful resources. From facilitating participation in international events such as the **2024 HAEi Global Leadership Workshop** to spearheading the translation and dissemination of informative materials in local languages, these endeavors underscore our commitment to an interconnected and informed community. I facilitated previously halted efforts to have HAEi Emergency Department Posters printed and distributed. I am happy to say that these posters are currently being printed and distributed across hospitals in China. Additionally, the informative HAE guides, "Understanding HAE" and "Women with HAE," are being translated into the languages spoken in the region. I anticipate these translated materials will be available soon, and I'm hopeful they will contribute significantly to raising awareness of HAE among people in the region.

Regional collaboration has been at the forefront of my thinking lately. Witnessing the success of recent events such as the Rare Disease Day celebration in Singapore, the 2nd Southeast Asia Rare Disease Summit in Bangkok, and the **2024 HAEi Regional Conference Americas** in Panama City, I am convinced that advocacy efforts can yield greater impacts when we join forces. Inspired by these examples of effective publicity, coordinated planning, and international collaboration, I actively seek ways to foster greater cohesion and collective action within the region.

If anyone has any ideas or suggestions on how we can make this happen, please do not hesitate to contact me. I am eager to explore new avenues for collaboration, and together, we can make a real difference in the lives of those affected by HAE.



FOLLOW HAEi ON SOCIAL MEDIA

Are you aware that we post HAE-related news on our social media platforms almost daily?

And that HAEi has a closed group for HAE patients and caregivers to share personal stories and initiatives and interact with other patients and caregivers around the world?

You can find us on Facebook, Instagram, LinkedIn, and X (Twitter) – see more and find links:

>> HAEI.ORG/SOME

MICHAL RUTKOWSKI
CENTRAL EASTERN EUROPE AND MIDDLE EAST

Hello HAEi Community!

I am very excited to share the general overview of my Q1 2024 RPA activities and achievements with you. As always, it was a very hectic period, including travels, multiple in-person and virtual meetings, and hard work on different resources.

I will start by saying that HAEi's tools portfolio has expanded in my region, with the implementation of the following:

1. Emergency Department Poster in Hungarian
2. Understanding HAE patient guide in Arabic, Armenian, and Hungarian. Additionally, ongoing translation takes place in Georgian, Slovak, and Ukrainian.
3. Women with HAE guide in Armenian and Polish languages and
4. Social media image posts in Hungarian, Persian, and Russian.

I held virtual meetings with HAEi Member Organizations (MO) from the Middle East Region (together with HAEi Advocacy Facilitator MENA, Mohamed Osman): Bahrain, Iraq, Kuwait, Saudi Arabia, Armenia, and with MOs from the Central Eastern Europe Region: Belarus, Czech Republic, Hungary, Georgia, Kazakhstan, Russia, Slovakia, and Ukraine. Besides common tasks to be raised during such meetings, I strongly advertised the **2024 HAEi Global Leadership Workshop** and **Global Angioedema Forum 2024** in Copenhagen, Denmark, in October this year.

Also, I held either in-person or virtual meetings with BioCryst, CSL Behring, KalVista, NewBridge, and Takeda to strengthen regional and local collaboration. Via a virtual link, I presented on "Life with HAE" at Takeda's HAE Expert Meeting Eastern Europe 2024, which took place in Tallinn, Estonia.

I was privileged to present on behalf of HAEi at the ACARE Preceptorship Abu Dhabi (United Arab Emirates) in early February. Around 80 physicians from Bahrain, Egypt, Kazakhstan, Kuwait, Qatar, Oman, Saudi Arabia, and UAE were present. I shared with the audience "The patient organization perspective on Hereditary Angioedema management." Following the ACARE meeting, I participated in the 3rd Emirates Allergy and Clinical Immunology Conference, where I had the great opportunity to network with HAE expert physicians from the region.

On 29 February, Rare Disease Day, I presented in Kaunas, Lithuania, at the seminar "Lithuanian patients with Hereditary Angioedema (HAE) due to C1 esterase inhibitor deficiency," which was organized for HAE patients and specialist physicians.

I always keep looking to connect with new patients and physicians from countries that are not yet members of HAEi. This time, I succeeded in establishing a connection with Professor Gulmira Razikova from Tashkent, Uzbekistan, the Director of the Republican Scientific and Specialized Allergy Center, and with Professor Lala Allahverdiyeva from Baku, Azerbaijan, the Head of Allergy and Clinical Immunology Department at Azerbaijan Medical University. I will work to strengthen our collaboration, make connections with local HAE patients, and have both Uzbekistan and Azerbaijan join HAEi as Member Organizations.

Please stay tuned!

NATASA ANGJELESKA
SOUTH EASTERN EUROPE

I am happy to share that almost all Member Organizations (MO) in the South Eastern Europe (SEE) region expressed their interest in participating in the **2024 HAEi Global Leadership Workshop** in Copenhagen this year and applied for travel grants. We are all excited about the opportunity to meet again in person later this year.

More and more countries started translating the very informative booklets prepared by HAEi: in addition to the Slovenian, Croatian, and Turkish languages, Understanding HAE is being translated into Montenegrin, and Women with HAE is being prepared in Slovenian, Albanian, and Montenegrin languages. I believe both booklets will be used for many awareness, education, and advocacy purposes by the representatives from MOs. Social Media posts were translated into Greek and are being used by HAE Greece.

In cooperation with Michal Rutkowski and Deborah Corcoran, we agreed to implement the *State of Management of HAE Study* for SEE. I believe the data collected will assist all countries, specifically those who advocate for access to modern therapies like Bosnia and Herzegovina, Kosovo, or improved access for Albania and Montenegro. We will also highlight the improved quality of life of HAE patients when they have access to preventive treatment through case studies from N.Macedonia, Serbia, and Greece.

In a discussion with HAE Greece representatives regarding information about HAE and disability benefits in EU countries, I explained that each country has its own regulations. Most often, disability depends on the burden and the consequences that the condition has on patients, and an expert commission usually evaluates this burden. I was able to share information from Poland received from Michal Rutkowski. I had an earlier discussion on the same topic with HAE Croatia, and I was informed that they had implemented an introductory HAE session for some of the Croatian disability commission members in order for them to be better informed about the burden of the illness.

At the beginning of the year, Dr. Mensuda Hasanhodzic advised she managed to put branded icatibant for HAE patients in Tuzla Canton, Bosnia, on the hospital list for procurement. This will be the first official access to modern on-demand therapy in Bosnia. For the past 2 years, the practice relied on donations. The doctor is working with other hospitals to copy this example and enable patients' access to the therapy.

I participated in the event marking Rare Disease Day on 29 February in Skopje, North Macedonia. I delivered a speech and also presented to different media about the challenges of life with HAE, as well as the need for on-time diagnosis, modern on-demand treatment and preventive therapies to be available to all patients.

PATRICIA KARANI
SUB SAHARA AFRICA

The year has started very positively, with great motivation from doctors and patient leads in my region. I shared the new Women and HAE booklet with the Africa Patients Support Group, which consists mainly of women who are HAE patients and patient leads from Mozambique, Namibia, Kenya, Eritrea, and Zimbabwe. This will be good awareness material for the patients and their families to read and understand more about how HAE affects women.

I am working with Fatoumia Said from HAE Comoros on advocacy activities, especially awareness among doctors. We are currently establishing a cohort of doctors who will be willing to handle HAE patients in Comoros with best practices and best management of HAE in their resource-limited set-up.

Much of my work so far this year has been with healthcare professionals. Our Sub Sahara Africa Regional Medical Advisory Panel (RMAP) has been busy, with new doctors joining the group: Dr. Leonilde Lavres from Sao Tome and Principle, Dr. Hamidou Tahirou from Niger, Dr Virgilio from Mozambique, and Dr. Patrice Tapsoba from Burkina Faso. The Sub Sahara Africa RMAP has started a genetic testing project initiated by Dr. Jonny Peters from South Africa. We discussed HAE in their countries and how we could best roll out this project in different countries.

Dr. Hamidou Tahirou from Niger is a new doctor contact that we have made this year. Dr. Tahirou is an allergy specialist and occupational physician working at the Amirou Boubacar Diallo National Hospital in Niamey, Niger. He has done extensive work on HAE in his country. He has also published literature on HAE patients entitled "Hereditary Bradykinin Angioedema: About a case described in Niger and review of the literature." He continues to raise awareness amongst healthcare providers and medical students in his country.

I assisted HAE Mozambique and HAE Comoros to form groups of interested doctors willing to learn more about HAE and assist in identifying more patients in their countries. Mozambique formed the AMAEH Medicos group, and we are still in the process of forming the Comoros doctors group. These important medical groups work well in the region as information is disseminated as well as correct information is sought and utilized to ensure best practices. I have also shared the latest HAE awareness posters in these groups.

With the assistance of Dr. Clarissa Dusenge, we formed a new doctor group in Burundi. In the group, doctors shared the challenges faced in diagnosing and managing patients, and Dr. Priya Bowry from Kenya has volunteered to assist them with ways of overcoming some of these challenges. We have planned an initial online training to discuss HAE more.

I was honored to participate as a speaker and panelist in a rare diseases and disorder discussion organized by ASNEN (Africa Special Needs Network). I gave a presentation on my difficult journey to diagnosis and proper care of HAE in Kenya. ASNEN is an organization dedicated to advocating for an inclusive world. It has a multifaceted approach to supporting individuals and families with special needs and disability in Africa. Their mission stems from the challenges that rare diseases and people with disabilities in Africa face in trying to access proper care, from diagnosis to management of their conditions. Their relevance comes in advocacy and awareness about rare diseases, support services, research and data collection, collaboration and partnerships, empowerment, and capacity building for patients and their families.

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 AMERICAS PANAMA CITY 15-17 MAR 2024

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Hola Americas – Let's Take Action

From 15-17 March, around 650 HAE patients and caregivers, physicians/scientists, and industry representatives from 26 countries met in Panama for the 2024 HAEi Regional Conference Americas. The enthusiasm and motivation to Take Action were real, and each participant left knowing they have the power to make a positive difference.

This third and final regional conference completes this cycle of HAEi events. At each meeting, we've covered topics important to the worldwide HAE community. All attendees heard these vitally important presentations at their own conference. In this special feature section, we take you to the heart of the first conference in the Americas region. In addition to the keynote speeches, news, and views, we have interviews from key personalities in Panama while signposting you to earlier in-depth articles on topics also covered in Munich (**2023 HAEi Regional Conference EMEA**) and Bangkok (**2023 HAEi Regional Conference APAC**).

Patient and Caregiver Track

Scientific Track

Youngsters Track

The Conference Opens

We were honored to receive a formal address and welcome from **Dr. Oscar González**, the Deputy Director of Public Health at the Ministry of Health in Panama (Ministerio de Salud de Panamá (MINSA)), to open the **2024 HAEi Regional Conference Americas**.

Anthony J. Castaldo (HAEi Chief Executive Officer and Chairman of the Board) and **Henrik Balle Boysen** (HAEi President) then took to the stage to welcome 650 delegates from across the Americas to the first HAEi Regional Conference Americas, covering north, central, and south America.

While explaining some of HAEi's history, Tony highlighted that some of the organization's pioneers are from this region, including Argentina and the United States. These individuals, bolstered by Henrik's skills and experience, figured out what was needed to create a highly effective global umbrella organization. They found a way to **Take Action**, which Henrik reiterated is the spirit and theme of this conference. It is the reason that HAEi is active across five areas of focus:

- Creating a decentralized operational footprint
- Developing highly relevant technology-driven tools, apps, and services
- Fostering the advocates of the future
- Convening in-person conferences and workshops
- Producing real-world data to demonstrate the value of HAE medicines

Tony previewed some of the upcoming sessions, including hearing from the ever-listening, Regional Patient Advocates (RPAs) in the Americas, with updates on the exciting progress being made in this region.

Henrik reviewed the great tools, technology, and services available to Member Organizations (MO) and patients completely free of charge. You can see a recap of these and read how Member Organizations in the Americas are using these tools to turbo-charge their advocacy on page 32.

It's not just tools and technology, Tony told the assembled delegates. Data from research is the currency to gain access to HAE medicines. To support this, HAEi conducts several forms of patient-driven research, including the "Pharmaco and socio-economic studies" series, the "Baseline burden of illness survey," and the "Heat map survey." Read more about these https://haei.org/resources/advocacy/research/

Concluding, Henrik and Tony asked everyone present to express their thanks to the pharmaceutical companies who are supporting this important conference. To loud applause, BioCryst, Takeda, CSL Behring, Pharming, Biomarin, Intellia, Ionis, KalVista and Pharvaris, were called out for their generous support.

Read highlights of the MoH speech – scan the QR code or visit **haei.org/hola-americas-lets-take-action**

"I want to help more people in other countries see better healthcare and be able to get the meds like the US."

– Patient/Caregiver Track Participant

As an HAEi event returns to the Americas for the first time in a decade (the **2014 HAEi Global Conference** was held in Washington DC, USA), Global Perspectives sat down with **Tony Castaldo**, to hear his thoughts on what has changed and what the future holds.

What changes have you seen in the 10 years from 2014-2024?

"First and foremost, continued advancements in therapeutic options for people with HAE. Several prophylactic therapies have had regulatory approval and have been game-changers in certain countries. As important, is the participation we see at the Conference. HAEi has expanded, and we have a significant contingent from Mexico, the Caribbean, and South America as well."

What are you most proud of?

"I'm most proud of our ability to take the idea of HAEi and turn it into a reality where MOs want to be part of this greater network. I'm also very proud of our RPAs. They are the backbone of HAEi, helping MOs achieve all they want to."

"HAEi becomes more relevant every year because of the tools, services, and programs it offers. They allow any MO, no matter what size, to punch above their weight, bringing them closer to achieving the fundamental goal of access to, and reimbursement of, modern HAE therapies. It is very heartening to see the uptake of these here in the Americas."

What do you think the next 10 years hold for people with HAE in the Americas?

"We are always thinking about how every country can get access to and reimbursement for modern HAE medicine. The ultimate difference to achieving this goal is a patient organization in a country."

"We are willing, ready, and able to work closely with MOs and their physician advisors to help them convince their health ministry and Government to approve and reimburse these medicines. Without appropriate treatment, HAE remains a catastrophic unmet medical need."

What are the special challenges that people with HAE in the Americas face today?

"Fundamentally, it's diagnosis. At this conference, we have a robust scientific track designed to ensure that physicians responsible for diagnosing HAE can access world-leading experts and increase their knowledge of how to diagnose HAE accurately."

"The second challenge is motivating enough people with HAE to become a collective force. We call it the power of one. We've seen this happen in so many countries worldwide. When you get that one person completely dedicated to the cause of HAE education and awareness; good things happen. That's why we're here. To motivate and educate exactly these key people in the Americas. To demonstrate that there always is hope no matter what the situation looks like. As the theme says: 'Take Action!'"








Take Action and You Will Succeed

With an enthusiastic introduction from Tony Castaldo, **Prof. Marcus Maurer**'s passion for HAE and patients was clear as he began his keynote lecture.

Prof. Maurer told the audience that bringing them a message of hope gave him great joy. He believes that "together, as a community of patients, physicians, industry, family and friends, we give people with HAE a better life, a normal life." To achieve that, we need to TAKE ACTION. He acknowledged that we can be very proud of what has been achieved. But what we're doing right now is not enough. There is still a long way to go.

He recounted a discussion with a patient on the journey from the airport in Panama. She told him that her care was not where she wanted it to be, and she feared attacks. Prof. Maurer said that this reflects what many patients in many of the Americas countries go through.

The Angioedema Centers of Reference and Excellence (ACARE), of which Prof. Maurer is a leading part, number 99 globally. In the Americas, many countries have a participating center, such as Panama, Costa Rica, Bolivia, Brazil, Argentina, Colombia, Ecuador, as well as the US and Canada.

"We have to bring this to all patients everywhere," he said. Prof. Maurer encouraged those in the audience to act by identifying physicians in their country and encouraging them to become part of ACARE. He continued, "ACARE is a bottom-up organization driven by the desire of patients to make this world a better world for them and others like them."

ACARE means you can expect the highest possible standard of care. There are 32 criteria to become an ACARE. It's not easy. Unsurprisingly, Prof. Maurer's favorite criterion is the 'Never Give Up' attitude. ACAREs don't give up. Problems don't stop them. Ways are found to overcome obstacles to help patients get the care they need. Prof. Maurer reiterated that ACARE is not just for physicians. "Patient advocates, working with physicians, make ACAREs great."

But there's more than just care; there's research. ACARE seeks to push the boundaries, to better understand the disease, to understand the unmet needs, and to understand where things are not working so that we can change them together. "Research gives you, people with HAE, the loud voice needed to convince people who have the power to make decisions about better treatments, care, and diagnostics," said Prof. Maurer before sharing some ongoing research projects.

First, a program called Sherpa which looks at switching from old androgen treatment to modern treatment

Genetic testing is available to every patient in ACARE. This identifies the underlying genetic mutation, which is the cause of the disease that affects you and your family, Prof. Maurer informed the audience.

Calling HAE a horrible disease that 'stinks,' Prof. Maurer described the importance of patient-reported outcomes being used to show the need to treat. These measure disease activity and impact and should be used extensively in clinical practice, Prof. Maurer said, which is why an ACARE project is looking at them.

Another program examines HAE in pregnancy to ensure that physicians can better guide female patients and offer the best care.

Prof. Maurer admitted to being very excited about an artificial intelligence project. This program uses just 10 questions to decide on a patient's diagnosis of HAE. It is already correct in more than 90% of cases. Prof. Maurer admitted this is better than he or anyone else can do, and this program is working to make the machine even better.

Prof. Maurer turned to the angioedema registry, CARE, which he called his favorite project, "It demonstrates that no one is alone." Whether in the Americas, Asia, Africa, or Europe, people with HAE have similar and different challenges to face with their disease. Pulling together a global database means we can learn. His call to action was to encourage your physicians to join, or if you are a physician, encourage your patients to be included in CARE.

Highlighting the many ongoing programs to educate physicians, Prof. Maurer encouraged all expert HAE patients to share their knowledge to educate all doctors about the disease. He heard patients in the audience describe waiting 12, 15, 26, and even 36 years for a diagnosis, which "should not be tolerated. Educating young and frontline physicians would make a real difference to speeding up diagnosis."

Finally, Prof. Maurer encouraged everyone to look at the HAE Guidelines. It is a compass and a guide to how patients with HAE should be treated. The ultimate emphasis is on total control, which is the ability to lead a normal life. He acknowledged that for many present, that is a dream, not reality. But it can be, he told them, "We have the drugs today on this planet. Maybe not in your country, but let's change that."

Concluding, Prof. Maurer encouraged everyone to continue working together to open closed doors. He said that he did not care about the cost. It is every HAE patient's right to have safe and effective treatment. Prof. Maurer told the delegates that the Americas is a shining example. Let's make it even more so, "I know you have the energy. TAKE ACTION, and you will succeed."

"I'm not alone and there are people worried about helping me."

"For me it was excellent in all aspects, promoting knowledge and engagement of all."

– Persons with HAE

Global Perspectives met **Prof. Marcus Maurer** to ask how things had changed since his keynote address to the **2023 Regional Conference EMEA** 6 months ago.

What has changed in your talk since we met in Munich?

"A lot has changed. The theme in Munich was 'No Patient Left Behind.' And coming from this, we now talk about TAKE ACTION. That's the conference theme and my call to action for patients and physicians. The unmet needs will only be addressed if we TAKE ACTION. We have to change it. This is true for individual patients and everyone who makes up the HAE community."

What similarities and differences do you see in how HAE is managed?

"The situation is really quite different in different countries. Speaking to delegates from South America, we are not where we want to be. I talked to patients who have no on-demand treatment, only ineffective adrenaline pens and danazol. This is a reminder that we need to work with patients in their countries, make noise, effect change, and bring all countries to the global level we want to achieve. Diagnosis is another difference. Patients still wait a long time, and often, their families are not properly screened. However, education is a similar need across the globe. We must educate and collaborate with patients and physicians to get to the levels of knowledge we need."

Are there examples of HAE care improving?

"There are a lot of examples of care improvement, which is due to the relentless action of many physicians and patients in these countries. Patients in Brazil do have -although limited- access to modern and first-line recommended treatment options as per the global guidelines. I just heard that modern on-demand treatment is becoming available in Panama, and it wasn't before. These are all very encouraging examples where raising our voices, speaking as one, and working together can really improve the situation and bring modern medication."

What role is ACARE playing?

"Well, the aim of ACARE is to bring good treatment options everywhere on this planet. We want an ACARE in each and every country. We have strong countries in the Americas but we don't have ACAREs in all countries here. To change this, we need to work with patient organizations in the countries."

And finally, what is your message to anyone outside this conference?

"Now is the time to act. Now is the time to be united. Go to websites like HAEi and ACARE that provide information on where you can get care, and add to the network of patients working together with physicians dedicated to the care of HAE. I know there are problems. We will only solve them by acting; that's what we need to focus on right now. Together, through our actions, we can change things and make them better."

Getting to Know the Americas Region

After the keynote, **Fiona Wardman**, Executive Vice President Global Advocacy and Chief Diversity Officer, and **Jørn Schultz-Boysen**, Executive Vice President Global Operations and Chief Compliance Officer, introduced the next sessions that were dedicated to understanding more about the Region.

The region focus kicked off with a special welcome from our Panama hosts before each Member Organization from the region introduced themselves.

Watch the videos or read the English transcript of the messages – scan the QR code or visit **haei.org/getting-to-know-the-americas-region/#1**

Fernanda de Oliveira Martins, South America and Mexico

"I represent 11 countries in the Americas, where 440 million people live. It is estimated that 14,700 people with HAE live in these countries. We have the opportunity and perhaps the responsibility to help, but this is not an easy task. People with HAE in South America and Mexico face many difficulties, including delays in diagnosis and difficulties in accessing treatment."

"I support the incredible job my region's patient associations do to ultimately secure a better quality of life for those living with HAE in their countries."

"There are lots of activities, meetings, and patient follow-ups. We meet with doctors, contact pharmacists, and liaise with government bodies. The results are showing. More patients are diagnosed and registered with associations. More drugs are approved and available to patients, and we move towards a better quality of life for those with HAE."

"I'm very proud to be part of this event. May the **2024 HAEi Regional Conference Americas** help us renew our purpose and inspire us to return home with a clear plan to Take Action."

Javier Santana, Central America and Caribbean

"I represent 7 countries in my region. The combined population of my region is about 59.2 million people, and we estimate that there will be 1970 patients in the region. Right now, we are not even half that number. Challenges we face include governments sometimes not accepting the disease, and doctors not knowing about the disease, and patients not following up on medical appointments as they are frustrated there are no treatments."

"Many years ago in Costa Rica, there were no treatments; the government did not want them, and the doctors didn't know about the disease. But thanks to the efforts of patients and doctors who did care about HAE, they got meetings with the Government. Today, Costa Rica already has two treatments to treat HAE."

"There is work to be done. Nothing is easy in life. Difficult things that are worked well always have a purpose and always succeed. You can count on us in HAEi to carry out the necessary work. You can count on us as RPAs. That is our job, to help you."

Tony Castaldo, North America

"In North America, comprising the United States and Canada, there are approximately 375 million people and approximately 12,500 HAE patients. The United States and Canada both have experienced, well-organized, and very effective organizations. Both organizations have led the fight, and I mean fight, and have been successful in winning access to and reimbursement for modern HAE medications."

"Securing access to all new medicines and keeping access is now the challenge. We're putting on the gloves, and we're ready to fight. We will fight fiercely for continued access for our community."

"Each organization here in the Americas can rely on the experience and reach of HAEi to help formulate and implement an action plan so you can expedite the process of accessing HAE medicines."

"We must then work to protect our access to medicines. Ultimately, it is about disease education and advocacy."

Ending the first evening of the **2024 HAEi Regional Conference Americas**, Member Organizations from across the region, shared video messages for patients and caregivers.

Watch the video or read the English transcript of the messages – scan the QR code or visit **haei.org/getting-to-know-the-americas-region/#2**

Raising HAE Awareness in the Americas

Javier and **Fernanda**, two of the RPAs for the Americas region, opened the second day. Together, they told the assembled patients and caregivers about the importance of raising awareness of HAE and shared some effective ways to do so.

Why is raising awareness important?
It increases enthusiasm and support, stimulates action, and generates local knowledge and resources. It can show government policymakers the will for change. It can improve the lives of people within the community.

Where do you start with raising awareness?
The first way described was **social media**. Although it may seem obvious, it is only powerful when used properly. Javier and Fernanda told the audience, 'Don't use it to impress people; use it to impact people.' Use social media to show who you are and what you stand for. Use it to tell compelling stories, ensuring they are spread far and wide.

The next step is to **talk to as many people as possible**. Anyone can be an ally in the fight for better care. Share factual information from others, from HAE organizations. Be honest about your life with authentic conversations.

Take the opportunity to **make connections with others**, especially patients and caregivers in your country and beyond. This can lead to knowledge exchange and greater awareness opportunities. Change happens one person, one moment at a time.

Contact officials such as government officials, meet with your members of Parliament or talk to people at health ministries. Javier and Fernanda shared a key lesson; know what matters to politicians and tailor your message to match their interests. Research interviews and speeches they've given. Keep them up to date whenever you have developments or new materials.

Use **HAEi's free website hosting** and templates to give your organization a strong online presence. These have been used effectively in the region. Javier and Fernanda shared examples from Canada, Panama and Brazil.

Use and share the **HAE Emergency Cards**. Use the **Emergency Department Posters** as an opportunity to raise awareness. These can create life-saving knowledge and awareness when people need it most.

Of course, Javier and Fernanda remarked that **attending HAE meetings** such as this one in Panama can be powerful in raising awareness, especially as a chance to network with others and share ideas.

Celebrate success; no matter how small, sharing progress keeps your campaign top of mind and demonstrates your impact. And don't forget to share photos and information on social media to inspire even more people.

Use the **media** to help you to get your message out there. The two RPAs explained they knew it could be uncomfortable to talk in front of people or a camera, but the benefits could be huge in making the case for change. **Telling your story** to a newspaper or magazine can reach so many people. Doing radio interviews keeps you away from the camera, and reaches another audience group. **HAEi Advocacy Academy** has free tools and advice on telling your story to the media, and the RPAs are always on hand to support you. HAEi offers support with the facts and information crucial to getting media interest.

The **Youngsters' Community** and **HAEi LEAP** program offers opportunities to reach critical younger audiences and build the advocates of tomorrow. One youngster from Brazil said, 'This community makes us feel like we belong to a group that accepts us, loves us, and helps us just the way we are.' Our young people are also a skilled resource; their ability with social media can be very helpful. Their unique perspective can be very appealing to media and politicians.

hae day :-), 16 May, is a huge opportunity, as an established annual global awareness day. Share your story. Organize a local event. Get involved, however you want. The HAEi provides everything you need at www.haeday.org

Javier and Fernanda concluded by reminding all the delegates of their RPA role. There are 11 RPAs active for HAEi across the world. They have experience. They have courage. They have endurance. They can help you make connections, raise awareness, and make a difference.

Empowering HAE Advocacy – A Comprehensive Toolkit

Representatives from our Americas MOs shared how they use some of the tools and services offered by HAEi and the impact they have on their own advocacy. RPAs Javier and Fernanda facilitated the session, performing double duty as translators as each MO representative spoke in their native language.

Lucas Benicio da Silva from Brazil explained how he uses the **Emergency Card**: "Thanks to the Emergency Card I presented in hospital environments, I could be quickly assessed and given the best possible care. The back of the card provides guidelines for emergency treatment, making it much easier when I'm seen by professionals who take care of my case in hospital networks. Thanks to the HAE Emergency Card. I can say it has saved my life several times."

Raquel Fuentes from El Salvador explained the impact of the **Emergency Department Poster** in her country, "When a patient in El Salvador visits most medical centers, the doctor does not know about the disease. We've placed the flyers outside of the allergy and immunology area. This helps with patients who might currently be misdiagnosed. I feel like we create more awareness. It means that the next time, the doctors will know what is and isn't going to work."

Carla María Goachet Boulanger from Peru and **Michelle Coronado** from Panama shared their experience of the **social media** resources provided by HAEi.

Carla said, "We share the resources HAEi provided as images and short reels on Facebook and Instagram. This means we do not need to invest money in creating these things. We can just re-post information or use free tools like Canva to add music or make short videos." Michelle added, "These have helped us a lot in finding patients. They see the posts and tell you that I have this. It means you do not feel alone."

Using **HAEi Hosted Websites** means you can be online quickly and easily. **Caroll Batista** from the Dominican Republic said, " They helped provide me with an email, and I was able to create the webpage. The support, continuity, and follow-up they gave me have helped me a lot, as obviously, I am not a professional in the area. I recommend it to other countries. It really helps us to find more patients and raise awareness."

Michelle Cooper from Canada recommended **HAEi Advocacy Academy** as a source of education and learning: "I've been finding out some of the things that I didn't know. There are so many programs on there. It's been great for raising my level of understanding of advocacy. We've invited all of our advocacy committee members to do it, and we really encourage our board members to do it because advocacy is such a big part of what we're doing now."

Turning to **Michelle Cuevas** from the USA, the hosts asked about her experience with **HAE TrackR**. Michelle explained, "In the US, we have to go through an insurance reauthorization process, and many insurers are now asking patients to provide information about their attacks. HAE TrackR allows you to record your attacks. You can set reminders about when your next treatment is due. It's very safe and secure, and you don't have to worry about companies getting your information."

Sandra Nieto from Mexico explained how she used data secured through **HAEi's Research Methodologies** to secure positive action on HAE from the Government and physicians, "The survey yielded very important data. We presented it at the Latin American Congress of Immunodeficiencies held in Mexico City. We showed that Mexican HAE patients were not going to the emergency room for fear of being given ineffective medicine. This created alarms among the medical society; they understood they were failing because of their ignorance of the disease. It created awareness among medical personnel across Latin America.

In our presentation to the Government, we could show that patients without treatment lost 20 days being sick in a period of 4 weeks. We are talking about an economically active population, and obviously, it is not in the government's interest for people to be sick without working because this creates higher expenses.

We convinced the Government to issue a decree that in Mexico, every year **hae day :-)** will be celebrated. Furthermore, all the patients who have HAE in Mexico are vulnerable people who have rights that must be respected. This will give rise to more initiatives within the Chamber of Deputies to achieve laws that can improve our quality of life and allow us to access all treatments free of charge."

Several MOs then spoke about the various activities they organize for **hae day :-)**. Michelle from Canada told everyone, "We encourage all of our members to participate in the HAE activity challenge and to post activities on the website. For many years, we were number one. This year, we're putting a challenge out to our members again. We really want to get closer to the top of the list again!

Last year we had politicians stand up in Parliament and announce **hae day :-)**, which was perfect. For Rare Disease Day, some local monuments and bridges have been lit up. This year for **hae day :-)** we want to light everything up purple.

Carla from Peru uses **hae day :-)** to organize lots of different types of activities: "This year, in health, we are planning to give a talk—hopefully with educational credits—on Angioedema at the Medical College of Peru with Dr. Calderón; for children, we are planning to tell many stories about families with HAE, which we can take into schools; and in sports, we are organizing a soccer championship—there will be a Peruvian Cup for the winner."

Finally, the hosts turned to Michelle from Panama to ask about **hae day :-)** in her country, "In the past, letters were sent to public institutions, hospitals, clubs, and some private banks to light up their monuments and buildings, with the purple and light blue that defines HAE. We tell them that the disease can be fatal and that medicine is not available. Almost all the institutions have supported us.

We have also shared flyers in different hospitals and offer talks about angioedema. Dr. Olga shares information in her hospital and presents photos of HAE episodes."

"Thanks to the HAE Emergency Card. I can say it has saved my life several times."

– Lucas Benicio de Silva, Brazil

HAEi Resources – A Comprehensive Toolkit

- **HAEi hosted websites**; a secure turnkey web solution for MOs
- **HAEi Connect**, a regulatory, compliant, user-friendly membership database platform
- **HAEi Event Zoo**, a state-of-the-art event management system to help MOs arrange meetings and events
- **HAEi Advocacy Academy**, an e-learning platform that helps MOs, patients, and caregivers become more effective HAE advocates
- **HAEi LEAP** and our HAEi Youngsters' Community help youngsters learn new skills, develop personally as individuals, and work on advocacy
- **Global Perspectives**, HAEi's magazine, offers a comprehensive overview of everything going on in the world of HAE
- **HAE Emergency Card** gives clear and straightforward information about HAE and the treatment required during an attack
- **Emergency Department Poster** to help educate emergency department physicians on recognizing and treating HAE attacks.
- A significant presence on **social media** platforms, sharing knowledge and raising awareness
- **Understanding HAE**, a brochure with comprehensive and easy-to-understand information that every patient needs to know about HAE
- **Women with HAE**, a brand new guide that focuses on the unique challenges faced by women with HAE
- **hae day :-)**, to support the grassroots movement with our MOs, championing and using their creativity to raise awareness of HAE
- **HAEi Global Access Programme** offers access to HAE medication in countries where modern therapies are otherwise unavailable
- **HAE Companio**n a great tool when you're traveling
- **HAEi Research Methodologies**, to help all our MOs with data specific to their country to support advocacy for modern therapies and improved quality of life
- **HAE TrackR**, an easy-to-use electronic diary -designed by patients for patients- to record treatment, attacks and give reminders
- **HAEi Virtual Centers**, a sophisticated, secure, peer-to-peer video-based platform that connects physicians and people with chronic swelling disorders who may live in remote areas or otherwise cannot travel

Further information on all the tools is freely available to all at HAEi.org under "Resources."


EMERGENCY CARDS

Take Action!

HAE in the Americas – MO Panel Discussion

Javier and Fernanda brought representatives of all the Member Organizations to the stage for the Americas MO panel discussion. For many representatives, this was their first time speaking at a conference, and we truly appreciate everyone's contributions. Here we report a summary of the questions and responses.

The first question was, how is HAE diagnosed in your country? Each country responded, and the disparity in getting an accurate diagnosis was clear. Some countries have doctors who know about HAE. Some countries have access to testing; blood tests and genetic testing. Some countries have both. Many countries have none.

Panel members were then asked, why did you become an advocate?

Nicolas Velasquez from Colombia answered: "I have a personal and professional commitment to the cause. I have lived in Colombia. I have lived in the United States. No country is perfect. But I am privileged, and I have a voice. As a father, I know that there may be one person in this country somewhere who doesn't have the same access that I had, and that is why I became an advocate."

Edison Galarraga from Ecuador said: "There are many drivers for many people, but my driver was my family. My son was diagnosed, and so I understand that it's a fight. It's a fight against the government, against institutions to be able to have access to the quality health care that is needed. You need to keep at it. The end result is what's important, and that's what moves me."

The panel then discussed advocacy and the patient voice, why it is important, and what your future plans as an advocate are. The key message on the importance of advocacy is raising awareness of HAE to get access to medicines. This is the common fight: to be able to live a better life.

Plans for the future include collecting and using data to support activity, pushing for the approval and reimbursement of medicines, maintaining access to those medicines once they are available, expanding the reach of our community, and getting more people involved in clinical trials.

"Data is important to identify who we are, and how many we are. We need to identify ourselves to show people that we are important. If you don't have data, we do not exist," was a comment from Renata Martins, Brazil, and Carla Boulanger from Peru said: "I'm a patient. It's very important to raise your voice, in your country. Because if the patients themselves don't speak up, then the education is not going to have an impact."

In a quick-fire final question, the panel members were asked for a one word answer to what is the number one priority for your country?

Patient Voices – In Our Own Words

The audience then heard from three courageous women intent on telling their personal stories to inspire and educate their peers. All three gave powerful speeches that resonated with all in the audience, who showed their appreciation with prolonged applause for every speaker.

Taira Corrales Gonzalez , Costa Rica

I am 16 years old, and I am from Costa Rica.

Today, I want to tell my story and how I have managed, along with my family, to cope with this disease.

The first person to be diagnosed in my family was my grandfather. His name was Gerardo Gonzalez Medina. He was diagnosed at the age of 60 and passed away from angioedema four years ago. Years ago, in Costa Rica, the knowledge of HAE was scarce. For this reason, I can say that my grandfather's death occurred because the doctors did not have the correct knowledge to treat him. Today, approximately 70 patients around Costa Rica have this disease. Costa Rica is still working on a law to protect us as patients of a rare disease.

My C1 and C4 tests were performed in a private medical center since the Costa Rican Social Security Fund did not perform such tests. I was the first person to receive a genetic test at the Children's Hospital. Patients with HAE only had danazol or fresh frozen plasma, which caused me a skin allergy. Costa Rica has evolved in treatment, and although there are areas for improvement, today, we have two drugs for HAE. Last Friday, I received -with much love and happiness- the first dose of icatibant to administer at home.

It is worth mentioning that the registration of the medicines took about seven years of constant effort. In the case of Costa Rica, a lawyer donated his time and his ability to write letters with his signature. It was impossible to work with the government without it. Achieving the medicine registration would not have been possible without his help.

I was diagnosed by Dr. Mario Martinez, a specialist in Allergology at Hospital Mexico. He was the one who diagnosed my whole family. My family has been misdiagnosed multiple times. My uncle and my mother had surgery, as the doctors thought it was appendicitis.

Seeing that Costa Rica has improved over time fills my soul with strength and peace. I remember I spent most Christmases in the hospital. It reminds me of the little arguments my mom had with the doctors because they did not understand what it meant to have HAE. As a family, we have been able to deal with the different situations that arise from having this disease.

My family lives in constant fear that my grandfather's story will repeat itself. This year, I decided to take action and get more involved in Costa Rica. I took charge of the HAE patient group. It is impressive to see how much impact the patient community has.

I hope that Costa Rica can organize itself further in the future. I hope not to have to go to the hospital so often. I hope to be able to spend Christmas as a family. I hope that my family can be more united to understand what it means to suffer from HAE. I hope that awareness of this disease will spread and more people around the world will know about it. Finally, I hope my story has touched your heart.

> "This year, I decided to take action and get more involved in Costa Rica. I took charge of the HAE patient group. It is impressive to see how much impact the patient community has."
>
> *– Taira Corrales Gonzalez, Costa Rica*

Anne Morin, Canada

I'm 39 years old, and I'm from Canada. I have HAE type 1. I was diagnosed at the age of 12, but started suffering with attacks at the age of 3 to 4 years old.

At the age of three years old, I remember being sick and telling my mother, why am I here? I knew that my siblings and I experienced sickness differently. I knew I was the odd one out. Now, I'd go back to my 3-year-old self and say knowledge is power, and to educate myself.

When I was younger, there were no treatments. There were no outreach groups. There was nothing for me to relate to and nobody to speak about my disease. So, I fought for years to try to have a diagnosis.

Having the advocacy group HAE Canada helped me enormously. It was one of the leading factors of my advocating for HAE in my community.

When I was younger, attacks were mostly in my face. When I became a teenager, they went to my abdomen, feet, and hands.

A year ago, I saw a fatal accident while working as a coach driver. I had to be the first responder, and it ended up causing throat swells when previously I had none. I know that mental health and stress play a big role in HAE. You have to know yourself. You have to make sure that your mental health is good.

My grandfather passed away with HAE when he was 42 years old. When I was diagnosed at 12, I was told that I wouldn't live a day past my 40s. I'm about to be 40 this year, and I cannot wait to go see the doctor and tell him that he was wrong.

Looking back on my treatments, they would often treat me with antihistamines, anti-inflammatories, and steroids. All did nothing. When I was in my 20s, I finally had the treatments that I needed, and now, in my 30s, I get the preventative treatments, which are a lot better.

Traveling was a no-go for me before. I was not able to go more than 100 kilometers away from my home. Now, I'm able to travel worldwide. It's amazing to be here. Even at the airport in Colombia, I was going to be denied boarding because I was starting to have a throat swell. I had to inform the stewardess that I had my treatments; I was able to administer them, and I would be okay. I got upgraded to first-class because they were trying to say that I was not fit to travel, but I proved them wrong. I was able to travel.

One of the things that HAE Canada helped me with is to act. Social media helped me too. I know that one of my triggers is emotional. If I'm stressed it will definitely cause triggers. I was told that rashes or bruising weren't a part or a symptom of HAE. I have realized that every time I get a bruise, within a day, I will have a swell. Now, I infuse right away.

It makes me very happy to know that more people are listening to my journey. It will help other patients to get better treatment, so don't give up. I encourage everyone to participate in research. I've participated in many clinical trials.

I used to go through attacks on an almost daily basis. Now I'm getting them once or twice a week.

I keep fighting. I fight for my medication. I fight my disease. It will always live inside me. Even with a disease, you can move on and go forward.

> "I keep fighting. I fight for my medication. I fight my disease. It will always live inside me. Even with a disease, you can move on and go forward."
>
> *– Anne Morin, Canada*

Claudia Rafael, Peru

Eight years ago, one night I was about to go to sleep. It was late, and I received a WhatsApp message.

It was my sister Vanessa. She said to me: 'Claudia, you have this?' She sent me a link, which I followed. It had a lot of information about HAE. I immediately saw myself in all the information. After a bit, I realized that I had a rare disease that had no medication in my country and no cure. I fell asleep, crying then not crying, and thinking about my daughter, who was only 4 years old.

The next morning, I had two choices. My first option was to continue in denial and ask: Why is this happening to me? The second option I had was to accept what had come into my life and that it was going to stay. I returned to the information I had on the web and contacted Dr. Oscar Calderon. He graciously helped me, not only with the diagnosis. Dr. Calderon introduced me to a small group of patients. I think we were 5 or 6 patients at that time.

After getting around some barriers, we formalized and founded HAE Peru. We knew what we wanted. We wanted to work for a better quality of life for patients, which was so lacking in Peru. And so we did. In some cases, formalizing an organization tends to be long and complex. We did it in record time. When we drew up the first work plan, it lasted six months. The second work plan was for one year. It was really comforting, not only for me but also for my colleagues, seeing how little by little we were carrying out each of the projects we had proposed.

The following year, in 2018, a medicine was registered in Peru. I asked my insurance company for coverage of this medicine. You know what happened. I was denied. But the insurance company did not count on something. I was going to review my policy. I did, and I found a little paragraph whose interpretation favored me. Also, the insurance company didn't count on the fact that Dr. Calderon had instructed me on the entire medical report that he had prepared for the insurance company, explaining and substantiating why I needed the medication in a vital way.

I left that meeting with a lot of sunshine—happy. I was sure that the answer I would get would be positive—and I got the positive answer! I believe I am the first patient in Peru to receive a drug covered by insurance in an amount higher than 80%.

I shared this process of having the medicine provided through private insurance with all the patients of the Association. Many obtained the medicine, others, unfortunately, did not have the same success.

It is here that all of us in HAE do not stop when we do not get the medicine. This is the moment when we have to persevere the most. We have learned that we have the right to health, and we have to be informed to be able to complain.

We have been working on the project of a story, told by adults for children. We are working with teachers and book editors on this, but the pandemic made funding difficult. We raised money to print 100 books and worked for 3 months to make it a reality. By the end of 2021, we got funding to make a video version and have the money for 500 books. It is now on sale, and all the proceeds will help us help even more people with HAE. The story also means we can take HAE into schools and build a truly inclusive society.

HAE Peru awakened my 17-year-old self, a law student, who today, at 44 years of age, can do a little more. We continue to work together in HAE Peru, in collaboration with Dr. Calderón and Milagros Cordoba. I am grateful for the opportunity, and I know that if there is something useful for any association, they will take it.

> "It is here that all of us in HAE do not stop when we do not get the medicine. This is the moment when we have to persevere the most."
>
> *– Claudia Rafael, Peru*

HAE: 10 Things Patients Need to Know

Taking on the challenge of following powerful, personal stories was world-renowned **Prof. Bruce Zuraw**. Introduced by Tony Castaldo as an 'accomplished physician and researcher who always keeps the voice of our community in mind,' Prof. Zuraw took to the stage.

Prof. Zuraw talks about the 10 things he felt all HAE patients need to know. He told the audience that over 40 years ago, when he started treating HAE, he couldn't have imagined the progress that would be made. That progress has to translate into better care for everyone with the disease.

Prof. Zuraw framed his 10 things around 10 questions: Why me? How will HAE affect me? What about my family? What causes attacks of HAE? Can I treat attacks? Can I prevent attacks? How do I find the right doctor? Who can I turn to for help? Can I dream about a cure? What can I do to make that dream a reality?

As an essential talk, we wanted all participants at each of our Regional Conferences to hear Prof. Zuraw. You can read more about his talks from the **2023 Regional Conference APAC** and **2023 Regional Conference EMEA**.

Read the talks – scan the QR code or visit **haei.org/hae-10-things-patients-need-to-know/**

We share a few points specific to the Americas and Prof. Zuraw's incredibly inspiring conclusion before bringing you a new interview.

How do we treat attacks?

Prof. Zuraw told the audience that while we don't all live in countries with all the modern rescue medicines, that is what we're fighting for.

If you can get one of the first-line rescue medicines (two forms of C1-inhibitor and icatibant), these are proven to be effective. Fresh frozen plasma or solvent-detergent treated plasma can be used in an emergency. It has C1-inhibitor in it, but it has certain risks.

All patients with HAE should have one of the modern rescue medicines available, ideally two doses. You don't know if you'll need a second dose or if you'll have another attack before you can get it renewed. Prof. Zuraw reiterated that all attacks are eligible for treatment, and you should treat them as soon as possible. "Antihistamines, steroids, and epinephrine are not going to work. Don't let doctors in emergency rooms try to convince you otherwise," he said.

Can I prevent attacks?

Prof. Zuraw stated that patients really want a normal life. Right now, in the United States, he said, we have four approved long-term modern prophylactic medications: two different forms of C1-inhibitor (IV and subcutaneous injection) and two different medications that target plasma kallikrein.

Androgens are second-line. If you can avoid taking androgens, that's good. If you have no other alternative, you need to use what you have.

Prof. Zuraw told the audience that treatment must be individualized, "use your critical voice, and don't let the decision be made by a doctor only, as they don't live your life."

In concluding, Prof. Zuraw told the audience that his personal dream is that all patients with HAE will be able to live an entirely normal life. He said that this future will depend on you. He asked all those present to believe and to pursue it.

Global Perspectives secured some valuable minutes with Prof. Zuraw in Panama to ask him about his presentation and the conference.

Your 10 things are based on common questions. Do you get many unexpected questions from patients?

"People ask questions that are unexpected all the time! People have their own reasons for what they ask. We don't have the answers to everything, but we always answer as best we can. Sometimes, the answers are relatively easy, and sometimes, the answer is we have the data to give an answer."

What has changed in your talk since we met in Munich?

"Each time, I think, 'What will patients understand, and what will make sense to people?' I really want people to understand, so my only changes are to try to make my answers even clearer and easier to digest while still educating. I want to make sure the messages are easy to remember."

Do you see a lot of different perspectives across the Americas?

"All patients with HAE have similar questions, and their problems are fundamentally the same. The differences are in their access to medications and sometimes their access to the health care system. The differences are more organizational and logistical, than differences between patients in different countries. People are people."

Your talk is about 10 things. If it could only be 1 thing, what message would you have for people with HAE?

"Patients need to understand their HAE sufficiently so they can effectively advocate for themselves to get really good care. In the Americas, we're very fortunate to have excellent doctors throughout the region. Patients have to figure out how their local doctor can contact these experts and ensure they're getting good care. An individual patient can't change the reimbursement system in a country. I'm confident that, over time, we will find ways to move the needle on access. But, an individual patient can take action to understand their HAE and recognize their responsibility to ensure their doctor is doing a good job; that goes a long way."

"Use your critical voice, and don't let the decision be made by a doctor only, as they don't live your life."

– Prof. Bruce Zuraw, USA

"Extraordinary experience, both for me (caregiver) and my son (patient). It was our first time and it was very healthy to meet other people with the disease and exchange experiences about it. We learned a lot and returned home refreshed."

– Patient/Caregiver Track Participant

HAE Hot Topics

HAE in women and pregnancy, Prof. Anete Grumach

Prof. Grumach spoke first. She told the audience that most studies and registries show that women are more severely affected by HAE. Studies indicate between 56-75% of cases in registries around the world are women.

The main reason for this is estrogen production, which is, of course, a big issue in pregnancy. Additionally, oral contraception containing estrogen can be a trigger for attacks. Prof. Grumach explained that estrogen can be the trigger for attacks as it can lead to decreases in C1 inhibitor production.

Prof. Grumach presented case studies of HAE patients and their experiences with pregnancy and childbirth. She showed the audience data from a study of HAE pregnancies in Turkey. It indicated that pregnancy-related complications such as pre-term labor, spontaneous abortion, and lower birth weight were similar to that seen in the general population.

In general, Prof. Grumach indicated that the frequency or severity of attacks during one pregnancy does not predict the risk of attacks in future pregnancies. She also counseled that labor and delivery rarely trigger angioedema.

In terms of treatment, Prof. Grumach made the following recommendations:

- Ensure access to acute HAE medication for use as needed
- In line with guidelines, use plasma-derived C1-inhibitor as the preferred therapy during pregnancy and lactation
- Consider the use of long-term plasma-derived C1-inhibitor as prophylactic treatment for women who need it or who will benefit from it
- There is a recommendation to use short-term prophylaxis for procedures, but normal delivery is not really a problem for the pregnant woman with HAE
- Have a plan of action or care plan which is based on shared decision-making
- Monitor HAE symptoms after birth because of potential increased risk of attacks. Remember that there will be hormone variations after the delivery and during breast-feeding

Prof. Grumach concluded her talk by telling the audience that HAE patients considering pregnancy to ensure access to treatment, be prepared, and ensure regular communication and monitoring with their doctor.

HAE in children and the importance of family testing, Dr. Francisco Alberto Contreras-Verduzco

Dr. Francisco Contreras spoke next on HAE in children and family testing.

He indicated that HAE attacks are very similar in children to that seen in adults. He said that attacks may occur at any time of life, but on average, the first attack occurs before the age of 10 with worsening symptoms during puberty. The most common attack triggers in children were mental stress and airway infection. The usually reported symptoms were colicky abdominal pain, nausea, vomiting, and diarrhea.

Dr. Contreras indicated that children's smaller airways are a cause for concern in HAE as they can lead to rapid deterioration in laryngeal attacks.

Prodromal symptoms have been reported in almost 50% of cases, which can lead to misdiagnosis, particularly as urticaria.

Dr. Contreras mentioned that prospective parents may consider pre-natal (during pregnancy) diagnosis if HAE is already known in the family. This should be conducted with appropriate counseling and careful evaluation.

Blood complement testing, and values of C1-inhibitor are usually used. In general, he told the audience, genetic testing is really not necessary to confirm the diagnosis. In newborns this testing can be done using blood from the umbilical cord. Any testing of new borns should be repeated after a year in order to confirm a diagnosis, as blood values can change.

Moving to family testing, Dr. Contreras suggested that international guidelines recommend that children be tested for HAE if they are part of an affected family, that this should be carried out as soon as possible, and that all offspring of an affected parent should be tested.

"SO grateful for this opportunity! Muchas gracias!!!"

– Patient/Caregiver Track Participant

How patients benefit from clinical trials, Dr. Huamin Henry Li

Dr. Huamin Henry Li was next up, taking the first of two clinical trial-related talks.

Dr. Li referenced HAEi's own Tony Castaldo as a clinical trial participant. He told the audience that Tony takes part in trials, 'Not only for me, but I have two daughters who suffer from HAE, and I have grandchildren. I want them to have a better life; they can have better treatment.'

So, this is one motivation, Dr. Li said. He made it clear that there are many different types of clinical trials. Some are related to treatment, but others consider other aspects of living with and caring for HAE. All of these are intended to improve the lives of people with HAE.

That said, Dr. Li conceded that the trials most people are interested in involve investigating a new treatment. This can mean that patients in these studies are able to access fascinating new treatments before they're widely available while contributing to the knowledge of the disease state and treatment development.

Additionally, Dr. Li told the audience that participants in clinical trials often get much more comprehensive care, with more regular contact with experts. Your time and travel are also commonly covered for your participation.

Dr. Li concluded by saying that participating in clinical trials is hugely important. It allows access to cutting-edge medical treatment and expert care and also contributes to medical knowledge. He told the audience that most of his patients report that participating in clinical trials has benefitted them.

Clinical trials: What can patients expect from taking part, Dr. Rosario Espinoza Mora

Finally, Dr. Rosario Espinoza Mora spoke. She told the audience she was in charge of the clinical research department at her hospital. She explained the concept of 'phases' for new drug trials. These were, she said:

- Phase 1- to evaluate the toxicity of the new molecule, which is a candidate for becoming a new treatment
- Phase 2- you evaluate effectiveness and safety but in a small group of patients
- Phase 3- you continue evaluating safety and efficacy, but in a larger group of patients. After phase 3, the data is usually considered for approval by regulatory organizations such as the US FDA.

Dr. Mora told the audience that often (but not always) participating in a clinical trial can ensure access to that medication for a long time, even after the study itself finishes. It can be a highly valuable way to access treatment for HAE patients.

She encouraged people to look for information about trials and to consider researching trials that may be relevant to them. This information can be found in newspapers, official communications, flyers, websites, and even WhatsApp, which is used in Costa Rica.

Very importantly, Dr. Mora confirmed, that participation in a clinical trial is never obligatory for patients. Even if you have a good relationship with your physician or your caregiver, they must not pressure you to take part. They can, of course, attempt to recruit or persuade you, but the final decision is always yours. This is also important for children in clinical trials. Children can be recruited into clinical trials, but only with parental consent.

When researching clinical trials, Dr. Mora asked people to consider international clinical trial registries, such as clinicaltrials.gov. This is maintained by the US National Institutes of Health and covers almost all trials worldwide. She hoped that a similar system would be established in Latin America, but this is still under construction. She also suggested that pharmaceutical companies are a good data source on available trials.

"Loved learning about what is new and happening in the HAE world and where advances have been made."

"I'm so inspired to do more and advocate for my family and others that suffer with HAE."

"Thanks for all your commitment and extraordinary effort to provide the HAE community the support we need in so many important areas. As a patient, I am truly grateful for everything you have done."

– Patient/Caregiver Track Participants

Latest Developments in HAE Science and Therapies

Tony Castaldo introduced **Prof. Marc Riedl**, a world-class physician with amazing skills in patient care, to the audience. Prof. Riedl began his talk by addressing the conference theme of 'Take Action.' He told the audience that everyone does things every day to make their lives easier. However, big challenges remain. There's no easy solution. It's the same with HAE. Governments and health systems are not easy. "We can demonstrate that HAE has come a long way," said Prof. Riedl, "The most difficult things are the ones worth doing, and we can achieve success by working together."

Moving on to diagnosis, Prof. Riedl acknowledged that the blood tests for C1-inhibitor levels and functioning aren't available everywhere. He mentioned the importance of measuring bradykinin, especially in those with HAE with normal C1, but said, "It's tough to measure bradykinin; lots can go wrong as it's very unstable."

Prof. Riedl mentioned that across many ACARE centers, up to 25% of patients with HAE had normal lab results and normal C1 inhibitors. The pipeline for diagnostics is strong, and multiple people and groups are trying to bring more reliable bradykinin measures to the clinic.

Addressing the topics of HAE treatment and management, Prof. Riedl acknowledged the massive contributions of past physicians, especially Marco Cicardi and Michael Frank. Both dedicated their lives to improving the situation of the HAE community.

Prof. Riedl mentioned the importance of international guidelines in driving science forward. Guidelines recommend that every HAE patient has a rescue or on-demand treatment to deal with an attack when it happens and that every attack should be treated as early as possible. They also have recommendations for long-term prevention or prophylaxis. Prof. Riedl stated, "Prophylaxis may not be used by every patient with HAE, but increasingly, we're seeing the benefits of just preventing swelling episodes. Why wait until you get sick?"

Moving onto the investigational therapies, Prof Riedl confirmed that the drugs he would discuss were not approved by any regulatory agency. There is a lot to discuss: "Our little condition of HAE—and I don't mean that in a bad way—it is a rare condition, yet there is this long list of medicines that we are excited about."

Prof. Riedl started his whirlwind tour of future medicines with a drug called **garadacimab**. This is a designer monoclonal antibody that targets FXII. It is a preventative medicine given subcutaneously once a month. Studies showed an 86% reduction in the mean or average attack rate per month compared to placebo. In the 6-month study, 61% of the patients on the treatment had no attacks during that time. The company will submit this data work to the FDA and other regulatory bodies. We should get a decision in the next six to 12 months.

The second drug was an RNA-based therapy called **donidalorsen**. This therapy has a very specific design; it works in the liver cells to return balance to the various proteins involved in HAE, hopefully preventing attacks. In a small phase two study, at about 4 months, we saw a 90% reduction in attacks.

The next treatment under investigation is an on-demand therapy. **Sebeltralstat** is an oral medicine that targets plasma kallikrein. Patients were asked to take a pill as soon as they knew they were having an attack. The people who got the medicine started to feel better within one to two hours. People who got a placebo took well over six hours before they reported feeling better. This data is being compiled to be submitted to the FDA and other agencies.

Another oral medicine is **deucrictibant**, which blocks that B2 receptor. This drug is interesting because it's been developed to treat attacks, and to potentially prevent attacks. As a rescue treatment, it seems to work quickly and improve people's symptoms, compared to placebo. 60% of the people who got placebo had to use their regular rescue medicine. In people taking deucrictibant at the highest dose, less than 10% needed to use a regular rescue medicine. For the prevention of attacks, patients take the pill twice a day, every day. At the highest dose, there's an 84% reduction in the monthly attack rate. We also saw a 92% reduction in the need for rescue medicine during the month people were on treatment compared to the placebo. These are smaller studies, and we need a larger study in phase three.

Prof. Riedl turned his attention to the two gene therapy programs. The first one uses **CRISPR technology**. In this, nanoparticles are infused into the person. These go into the liver, and effectively, 'molecular scissors' clip out the defective gene very specifically in the DNA. It's only been tested in 10 patients to date, but it showed a 95% reduction in attacks. The second gene therapy Prof. Riedl outlined is **AAV** to deliver gene therapy. This takes a little piece of DNA and puts it in a virus that doesn't cause human disease. This delivery service, he said, goes into the cells and unloads the DNA, so the gene and your cells integrate that and start to make whatever protein that gene codes for. This is an effort to produce more C1-inhibitor in the body by delivering gene therapy into the liver.

Other potential medicines include a monoclonal antibody called **STAR-0215** and another RNA-targeted therapy **ADV324**.

"The HAE treatment landscape is incredible and exciting," said Prof. Riedl, "But there's a lot of questions, most importantly long-term safety. We need to see the results from long-term studies." In conclusion, Prof. Riedl invoked Chat GPT to summarize the current challenges in HAE. In response, he encouraged people to seek out their experts, participate in advocacy in their local country, engage with HAEi, participate in clinical trials, and work with government legislators and health systems. "It can be scary," Prof Riedl said, "But there is power in numbers. Everything we do now makes a huge difference for tomorrow."

Global Perspective posed a few questions to Prof. Riedl before his presentation.

What are- for you- the most exciting developments in HAE?

"There are a couple of things that continue to be very exciting. We still struggle to diagnose or be sure of HAE, particularly in HAE with normal C1. Hopefully, we're moving closer to having clinical tests that we can use. There are also many clinical trials looking at managing HAE, whether treating attacks when they occur or preventing them. Hopefully, this will lead to more effective treatment options with fewer side effects. It is exciting to make progress on effectively managing HAE with the least disruption to people's lives."

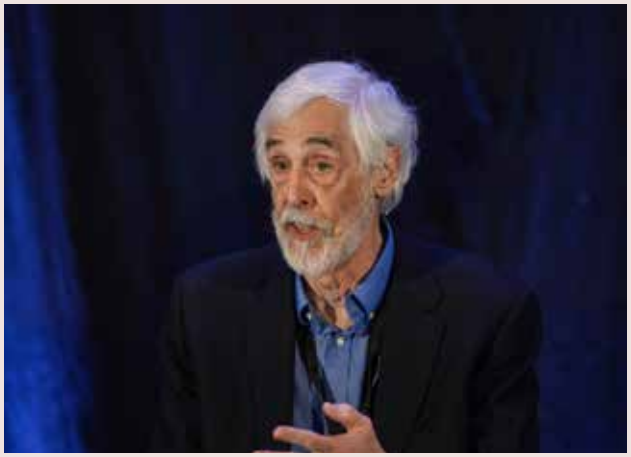
When might some of these potential new medicines become more available?

"Drug development is a slow process, which is understandably frustrating to many people. We want to be as certain as possible that these medicines work and are safe. Regulatory agencies have to make sure the data answers the efficacy and safety questions. The hope is that, at least in some parts of the world where the drugs have finished large trials, we could see possible approvals in the next one or two years. For other drugs in earlier phases, you're looking at two years and more."

What role do you think patients play in scientific progress?

"We only make progress with patient involvement. You can work with cells in the lab, but when it comes to developing treatments, you need blood donations from patients, their participation in questionnaires, and people willing to enroll in clinical trials."

"We've only made progress thanks to patients' enthusiasm and agreement to participate in these research efforts. It remains critically important that patients and families are engaged and interested in research because when that ends, progress will end. I'm fond of saying that my patients will tell me when I'm done with my work because they will say, 'I'm good. We don't need to do any more research.' And we haven't heard that yet!"



Expert Panel Q&A

Prof. Marc Riedl was joined onstage by fellow Scientific Committee Co-Chairs **Dr. Olga Barrera**, **Prof. Anete Grumach**, and Americas keynote speaker, **Prof. Bruce Zuraw**. Fellow keynote speaker and HAEi's Chief Medical Advisor, **Prof. Marcus Maurer**, facilitated the session.

We present a few of the questions asked.

I'm not sure if it is an attack or a stomach ache; how do I know to use my on-demand therapy?

The panel suggested that this is a tough one. Many things cause abdominal pain, not just HAE. Patients are experts on their own bodies, and they often know the difference between HAE and other causes of abdominal pain. The general recommendation is if you think it could be an HAE attack, go ahead and treat it. And try not to wait too long. If the rescue medicine doesn't work, and your abdominal pain continues to get worse, you have to be worried it's not HAE. Go and seek medical help.

Can a person with HAE have Botox or use hyaluronic acid?

Absolutely, responded the panel. You should be able to do anything that you want. If you want a piercing, get a piercing; if you want a tattoo, get a tattoo. It's your life; you should be able to live as you wish. But you need to know that these are all situations that can lead to an attack. So you need good protection, you need treatment, and then you can do anything that you want.

Hormones and stress affect my HAE: What can I do?

Hormone effects are well described, especially estrogen, which is part of the reason women in general have more symptomatic HAE. It's a tough one because there is a downside to lacking estrogen. We don't generally recommend depleting estrogen or blocking estrogen. It is wise to avoid oral contraceptives containing estrogen. Similarly, in menopause, avoid estrogen replacement therapy. Both will worsen HAE symptoms.

On the stress side, there's no magic wand. You'll have less stress if you have the medicine to treat the attacks. But, effective medicine is not a prescription for no stress in your life. It's not that easy. Life is stressful. You need to find the tools to help you manage your stress. Whether exercise, meditation, reading, or spending time with your family, do whatever helps you.

We don't have modern medicines in my country. Can I use older medicines like androgens?

Access to medicine is a big issue and one we all continue to work on. If you don't have access to modern medications in your country, you have to work with what you do have. Androgens can be effective. The concern is potential side effects. Discuss this with your angioedema specialist. If you decide to go on androgens, the guidelines include recommend monitoring, and always use the lowest dose that will prevent the symptoms.

What are upcoming plans to bring access and care to countries with small or no HAEi Member Organizations?

Dr. Barrera told the audience she felt this conference had opened a path for those at the meeting to reach and teach the rest of the doctors who do not know the disease and to get the appropriate drugs for our patients.

Prof. Maurer added that patients are the reason and purpose for this conference. The volume of studies, works, talks, and lectures from the important specialists in the field has been incredible and can only help everyone to make progress.

Closing Remarks

Henrik and Tony thanked everyone who had worked hard to make this weekend a success and reminded everyone that HAEi will not stop or be satisfied until every patient has sustained access to a suitable HAE therapy. We know that we have more work to do, and together we are going to Take Action and do it.

Poster Session, Friday evening

20 submitted abstracts were accepted for presentation during the Scientific Track of the **2024 HAEi Regional Conference Americas**. Fifteen of these were presented as posters and five as oral abstracts.

Each poster presenter had the opportunity to share key information in a 2-minute short talk before being at their posters to answer questions from participants as they walked the room.

To read a summary of all the posters, including interviews with some of the presenters – scan the QR code or visit: **haei.org/americas-scientific-poster-session/**

1. Registry of Members of the Association of Patients with Hereditary Angioedema of Peru
2. Early-onset Response to the Oral Bradykinin B2 Receptor Antagonist Deucrictibant Immediate-Release Capsule in Patients with Hereditary Angioedema Attacks
3. Registry of Members of the Association of Patients with Hereditary Angioedema in the Republic of Panama
4. Efficacy and Safety of the Oral Bradykinin B2 Receptor Antagonist Deucrictibant Immediate-release Capsule in Treatment of Hereditary Angioedema Attacks: RAPIDe-3 Phase 3 Trial Design
5. Anxiety Associated with Refilling On-demand Therapy for HAE Attacks Contributes to Treatment Delay and Non-treatment
6. HAE Attacks in Canadian Patients with HAE: Triggers and Treatment Based on Data from the 2020 National Survey
7. Characterizing the Negative Impact of Delayed On-demand Treatment of HAE Attacks
8. Characterization of Prodromes in Hereditary Angioedema: Findings from an Online Patient Forum
9. Characteristics of Hereditary Angioedema Attacks Among Long-term Prophylaxis Users
10. Delayed On-demand Treatment of Hereditary Angioedema Attacks: Patient Perceptions and Associated Barriers
11. Findings and Insights from a Colombian National Survey on Hereditary Angioedema due to C1-Inhibitor Deficiency
12. Treatment Patterns of Patients Requiring Redosing of an On-demand Treatment After the Return of an HAE Attack
13. Efficacy and Safety of Bradykinin B2 Receptor Antagonism with Deucrictibant Immediate-Release Capsule for Treatment of Hereditary Angioedema Attacks: Results of RAPIDe-1 Phase 2 Trial
14. Anxiety Associated with On-demand Treatment for Hereditary Angioedema (HAE) Attacks
15. Diagnosis and Treatment of Patients with Hereditary Angioedema in Cuba

Scientific Session, Saturday

Running concurrently with the Patient Track, the Scientific Track of the **2024 HAEi Regional Conference Americas** was the hub for the latest data and discussion on the diagnosis and treatment of HAE. Three physicians from across the Americas region chaired the Scientific Track: **Prof. Anete Grumach** from Brazil, **Dr. Olga Barrera** from Panama, and **Prof. Marc Riedl** from the USA, and welcomed 75 attendees to the program.

The assembled physicians had a full program of presentations and discussions on Saturday morning. The Co-Chairs presented the Young Researcher/ Investigator award to **Dr. Karla Robles-Velasco** from Ecuador. You can read about her award-winning research in the oral abstract presentations.

> "Wow. What a surprise! I just came to present our study, but I go back to my country with a prize. This honor is for the work, not only by me but for the whole team. It was a really amazing moment."
>
> – *Dr. Karla Robles-Velasco, Ecuador*

Invited Talk: Obtaining Medication for HAE in our Central American Region

Dr. Francisco Alberto Contreras-Verduzco, Mexico, told the assembled delegates that he would provide an update on the situation in the Central American region. He categorized the region as having little territory and a large population but little development, noting that only 2 countries have adequate scores according to the Human Development Index. As a result, he highlighted four main challenges to access for HAE medicines:

- Limited availability
- High costs
- Lack of awareness
- Underdeveloped health infrastructure

To learn more, Dr. Contreras-Verduzco contacted the health ministries in all 7 Central American countries: Belize, Costa Rica, El Salvador, Honduras, Guatemala, Nicaragua, and Panama. Unfortunately, he received mixed responses; some great, some a little or nothing. Speaking with colleagues in these countries, he showed that there are more than 137 patients diagnosed across the region, but prevalence data indicates that there are likely to be more than 1000 patients with HAE in the region.

On available medication, Costa Rica approved icatibant and plasma-derived C1-inhibitor in 2023. Beyond this, no other countries have approved medications recommended as first-line HAE treatment. Where possible, wealthier patients can travel abroad, commonly to Colombia and Mexico, to acquire medicine. Some patients are also able to access donated medicine. He cautioned that most patients do not have the money to obtain treatment for the rest of their lives. Without a regular supply of HAE medicine to the countries in the region, there are growing concerns that people are obtaining medicine, potentially fake, from alternative channels.

Dr. Contreras-Verduzco proposed 8 areas in which patients could be better supported to access HAE medicines:

1. Education and awareness – to reduce stigma and lead to earlier diagnosis
2. Health policy development – to promote the participation of HAE patients in the development of appropriate health policies
3. Specialized clinics or reference centers – to create centers of excellence to act as reference centers for other hospitals and to support diagnosis and treatment
4. Training of health professionals – to address a lack of knowledge about rare diseases
5. Data collection and patient registry – to understand the needs and prevalence of HAE in the region
6. Support for patient groups – to work together to improve the quality of life for patients
7. International collaboration – to partner across borders to improve research and development
8. Access to treatment – To enact medication access programs, negotiate prices, and import generics as appropriate

Dr. Contreras-Verduzco concluded that a patient registry is imperative to understand the situation better. Through collaboration with governments, healthcare professionals, patients, and other stakeholders, it is possible to address the complex challenges of improving access to medicines for rare diseases like HAE.

Invited Talk: Diagnostic approaches to the different types of HAE

Global Perspectives caught up with **Dr. Ricardo Dario Zwiener**, Argentina, ahead of his talk. We asked him to tell us a little about the talk he will give.

"The diagnostic approaches to different types of HAE are very important. It helps us give an early and accurate diagnosis of HAE, which is critical for patients and their families or caregivers."

"I always say that HAE is like a wolf in sheep's clothing because it can be confused with many allergic conditions. Without a diagnosis, a patient is always at risk of death because HAE is a life-threatening condition."

"As physicians, we have different tools to diagnose HAE. The first is taking a patient history. When we talk to the patient, we get their viewpoints and hear about things like recurrent episodes of unpredictable edema with wheals, recurrent abdominal pain, and perhaps even an affected relative. The lack of response to classical anti-allergic treatment like corticosteroids and antihistamines and increased attacks after things like oral contraception are all important clues."

"If, after the patient history, we suspect that the patient could have HAE, we ask for a lab to confirm our clinical suspicion. If that is normal but the suspicion remains high, we can seek genetic tests to search for another mutation. I am from Argentina, and in my country, we have a center to analyze blood samples and perform genetic testing. We also offer genetic counseling, especially to others in the family who may have the disease."

How could patients and caregivers help in the diagnostic process?

"Asking HAE patient associations for a physician recommendation can help get someone with the right experience. Establishing a good relationship between physician and patient is essential. Both should be able to work together as a team to get a plan of action."

What do you think still needs to improve in terms of diagnosis?

"Awareness is the first issue. It is a global problem. We all need to join forces: patients, caregivers, physicians, advocacy organizations, and the pharmaceutical industry to improve diagnosis, testing, and access to medication."

"I think a patient has a right to have an accurate diagnosis. A correct diagnosis is the key to understanding this isn't psychological, and it means access to medication. You are more protected."

Thank you. This is clearly an important topic for you.

"Everyone has a purpose in life. One of my main purposes is to help HAE patients survive, reach an early diagnosis, and have medications to control the disease. I work with HAE Argentina, the patients' association. Together, we help patients and caregivers with medication, support, and education. For me, HAE patients really are heroes."

"Diagnostic approaches to the different types of HAE" is a core subject for all physicians managing HAE. You can read the summary of Dr. Teresa Caballero's talk on this subject from our **2023 HAEi Regional Conference EMEA** – scan the QR code or visit: **haei.org/emea-sci-track-welcome**

> "I work with HAE Argentina, the patients' association. Together, we help patients and caregivers with medication, support, and education. For me, HAE patients really are heroes."
>
> – *Dr. Ricardo Dario Zwiener, Argentina*


Sleep quality assessed by QSAQ


Dr. Karla Batres-Velasco


...WHEN YOU HEAR HOOFBEATS,
THINK HORSES NOT ZEBRAS.


Clinical Case: Additional Follow up


Human Development Index.

Clinical Case Presentation, Voting and Discussion
After the oral presentations, the Scientific Track heard clinical case presentations from the Co-Chairs. This was followed by a discussion of these cases among the attending delegates.

Prof. Marc Riedl presented the case of a 23-year-old female with HAE type I, treated with lanadelumab and stable for four years. At a more recent follow-up, the patient said that abdominal symptoms had led to around 15 days off work in the last six months and that icatibant only seemed to help on occasion.

Prof. Riedl asked delegates what else they would like to know to better respond to the patient, before describing a series of diagnostic tests that showed mild anemia, some enlarged lymph nodes, and a little thickening of the wall of the intestines. The patient was referred to a gastroenterology expert who -ultimately- diagnosed chronic gastritis and celiac disease. The patient's symptoms improved with medication and dietary changes.

He counseled delegates to consider the following when distinguishing an HAE abdominal attack from other causes of abdominal pain:

- Most abdominal HAE attacks are not associated with fever, peritoneal signs, or raised white blood cell count
- Elevated blood values (such as raised neutrophil counts, hypovolemia from fluid loss, and hemoconcentration) are reported in severe attacks
- Imaging is not routinely necessary in a known HAE patient with characteristic symptoms
- Bowel wall swelling may be found later in an attack

Finally, he clarified that a clear response (or lack of response) to effective on-demand medication should be a sign that it may not be an HAE attack. C1-INH level or function can be a useful biomarker while on C1-INH replacement therapy.

Prof. Grumach presented her case of a 21-year-old patient hospitalized 5 times in 10 years due to lip swelling and abdominal pain. Prof. Grumach saw the patient for the first time in 2021 and worked systematically to establish a diagnosis. She advised that the patient was previously non-responsive to antihistamines and steroids before asking the audience what percentage of angioedema patients do not respond to antihistamines (answer: 15-20%).

The association of subcutaneous and abdominal symptoms and self-limiting and recurrent attacks helped guide a diagnosis of HAE. However, the woman was the only one in her family affected, had late onset of symptoms and normal C4 and C1-INH values. This led to a suspicion of HAE with normal C1 inhibitor. The audience was asked if they would conduct genetic testing (100% yes). Prof. Grumach confirmed that genetic testing had occurred, and the patient had a mutation of Factor XII.

Prof. Grumach concluded by telling the audience that angioedema must be evaluated as a symptom associated with several factors. She reminded delegates that mast cell-mediated angioedema is the most common type of angioedema and that there is, as yet, no ideal biomarker for recurrent angioedema.

"I gained more knowledge about angioedema by being present at talks and interacting with patients, caregivers, and medical colleagues."

"I will become an ACARE."

"I wanted to thank you for the opportunity to live those moments. I want to participate next time because this conference enriched my life."

"I will use everything provided."

– *Scientific Track Participants*

Abstract Oral Presentations

During the Scientific Track, 5 oral abstract presentations were given, allowing participants to hear new scientific data on HAE and its treatment.

Relationship Between Time to On-demand Treatment and Quality of Life During Hereditary Angioedema Attacks

Global Perspectives spoke to **Prof. Sandra Christiansen**. We asked her to summarize the key implications of the research she was presenting. She said:

"This is part of a cooperative effort with HAEA in the United States. We focused on the use of on-demand therapy in people with HAE (C1-INH); what are people doing and why? We recruited 94 individuals, including some adolescents over the age of 12, divided between people who only had on-demand therapy and those with on-demand plus long-term prophylaxis."

"We're all aware that the guidelines indicate that all attacks are worthy of treatment to reduce morbidity and prevent mortality. And, that you should treat as soon as you recognize you're having an attack. That's the message that we think we're conveying to patients."

"In the study, the average delay to treatment was close to four hours, ranging from one hour or less to over eight hours. There also appeared to be a connection between the delay in on-demand medication usage and quality of life."

"The bottom line is that people with HAE wait too long before they treat an attack. The longer we wait, the worse the consequences. As doctors, we need to do better in our message to patients: treat early and treat all. And we have an unmet need, particularly for adolescents, who in the United States only have intravenous options."

Efficacy and Safety of Bradykinin B2 Receptor Antagonism with Oral Deucritibant in Prophylaxis of Hereditary Angioedema Attacks: Results of CHAPTER-1 Phase-2 Trial

Global Perspectives asked **Prof. Marc Riedl** to summarize the implications of his research. He told us:

"This presentation is on a medicine that's actually being studied for treating attacks on-demand, and also for preventing attacks. I'm sharing some data on the preventative approach. This is a targeted oral medicine, taken on a long-term basis, designed to prevent attacks. In this phase-2 trial, we compared it to placebo and saw that it looked effective and safe. A longer, larger, phase-3 study needs to be conducted to prove that it's both safe and effective."

"We continue to look for additional effective, potentially less burdensome ways to prevent attacks and this may be one strategy to do that. Oral medicine may be easier to administer, which may be useful in getting access to medicines in places currently experiencing challenges. It's another potential step forward in the quest to really bring more effective, safer, and potentially less burdensome treatments to more people with HAE."

Long-Term Effectiveness and Safety of Lanadelumab Treatment for Hereditary Angioedema in Patients from Puerto Rico: Data from the EMPOWER Study

Dr. Rafael Zaragoza-Urdaz told the audience that the EMPOWER study was designed to evaluate the long-term effectiveness and safety of lanadelumab in the United States and Canada. He presented data on Puerto Rican HAE patients who were treated with lanadelumab for up to three years.

Dr. Zaragoza-Urdaz gave a breakdown of the clinical trial methodology and details on the patients. He showed that pre-enrollment, 'new user' study participants from Puerto Rico had 2.22 attacks per month, reducing to 0.91 per month over time. In the larger group of 'prevalent users' (patients who had received 4 or more doses of lanadelumab at enrollment), the attack rate per month was 0.36. There were few reported side effects caused by treatment.

In conclusion, Dr. Zaragoza-Urdaz said that the EMPOWER data from Puerto Rico show reduced HAE attack rates after patients start on lanadelumab, and this low attack rate was maintained for up to three years. He described the treatment-related adverse events as non-serious and that lanadelumab was well tolerated overall.

Screening Sleep Disorders in Hereditary Angioedema: A Comprehensive Cross-sectional Study

Global Perspectives asked **Dr. Karla Robles-Velasco** to tell us about her award-winning research.

"In the general population, a sleep disorder can lead to many adverse health outcomes, like cardiovascular diseases. HAE patients already have a chronic inflammatory disease that involves many immunologic pathways and has a high impact on their lives. We believe it is important to identify and treat sleep disturbances in HAE to avoid adverse health outcomes in the future."

"We found that more than half of our participants had at least one sleep disturbance, with the most common being insomnia. More than 30% of our patients also had a risk of obstructive sleep apnea."

"Patients who have a sleep disturbance have worse HAE activity and worse quality of life. Patients should talk to their doctor if they don't have control of their disease, if they are not responding to treatment, or if they have higher disease activity. It could be an underlying sleep disturbance that is having an impact on their HAE."

Understanding Patient Reasons not to Treat All Hereditary Angioedema (HAE) Attacks and Characteristics of Untreated HAE attacks: Results from a Real-world Survey

Global Perspectives asked **Prof. William Lumry** to tell us more about his research. He told us:

"This was a real-world survey based on physician-diagnosed HAE patients. Around 60% were on long-term prophylaxis, and only 75% reported being prescribed on-demand treatment, which is somewhat against the guidelines. We recommend that everybody have an on-demand therapy available, whether they're on prophylaxis or not."

"We asked patients; did you treat your most recent attack? 85% said yes. When asked how long it took from the time they had symptoms to the time they treated. The median time for how long it took from the start of symptoms to the time they treated, was immediate. The duration of an untreated attack is in line with what we would expect, around 15 to 20 hours of symptoms."

"A little bit distressing is that 15% of patients who chose not to treat had to go to the emergency room or be admitted to the hospital because of the HAE attack. Half said they didn't treat the attack because it didn't proceed. In some cases, patients reported forgetting to take their medication or being concerned about the time and inconvenience of starting an IV or having an injection. The key message for patients is to follow the guidelines and treat attacks early. Period. Don't wait. Even an attack that starts out mild may turn into something that takes you to the hospital."

> "The key message for patients is to follow the guidelines and treat attacks early. Period. Don't wait. Even an attack that starts out mild may turn into something that takes you to the hospital."
>
> *– Prof. William Lumry, USA*


Why be a part of the community?


HAE youth ac
workshop in Atl

Youngsters in the Americas: The Future is Bright

Following the keynote presentation, the unique HAEi Youngsters' Community is split from the main patient and caregiver track. 90 registered young people and caregivers from 20 countries across the Americas region joined together for networking, education, and fun!

The team in Panama consisted of **Victoria Schultz-Boysen**, **Kamila Moran Salaverry**, **Debs Corcoran** and poet and nurse, **Faye Marshall**. They were supported by a host of volunteers, including social media interns who captured the conference from every angle.

Reflecting after the conference, **Nevena Tsutsumanova**, Manager, Youngster's Community and Special Projects, told Global Perspectives: "We can safely say this is one of the biggest youngsters tracks we've had! We are grateful that so many young people made the trip and actively participated in all the activities we had, and thankful for all the hard work of the team. If you'd like to be part of this amazing community, just click the join us button on the Youngster's Community page." (https://youngsters.haei.org/)

This is the HAEi Youngsters Community, Friday evening

The opening of the Youngsters' Track was a chance to introduce all that the Youngsters' Community offers to young people living with HAE and their caregivers. To set the scene, Global Perspectives spoke with Victoria Schultz-Boysen. We asked her: *What would you say to encourage more young people to be involved in HAE advocacy?*

"Please don't be afraid to reach out, whether to us or your organizations. We need you. When young people get together like this, great things are going to happen. For me, being in the Youngsters' Community has helped me be more confident in myself. It has made me grow as a person"

> "For me, being in the Youngsters' Community has helped me be more confident in myself. It has made me grow as a person."
>
> *– Victoria Schultz-Boysen, Denmark*

Get to Know Each Other/Networking, Saturday morning

The second session started with an informal get-to-know-you session where young people could share thoughts, feelings, and experiences.

Introduction to LEAP 2024 and the Successful Class of 2023

Throughout the Youngsters' Track, the delegates were introduced to the LEAP program. Debs Corcoran told them where the idea came from before explaining the education available to participants, both online and during a special LEAP meeting in Dubai. A special panel discussion of LEAP graduates gave their personal experiences and encouraged anyone 16-25 years old to speak with their Member Organization if they were interested in getting involved. More information is available on the dedicated HAEi Youngsters Community website: youngsters.haei.org/leap-welcome-program/

Face2Face with an HAE expert – Profs. Sandra Christiansen and Markus Magerl

Next, the program gave young people the chance to have their questions answered by an HAE expert. To accommodate the full array of topics, the Youngsters' Track split into two broad age groups, each with their own expert.

Global Perspectives spoke to both HAE experts to learn about the session and the importance of asking questions. First up, we heard from **Prof. Sandra Christiansen** who led the session with our older Youngsters' Track participants. We asked: *What do young people with HAE want to know about their condition?*

"I think one of the wonderful things about organizations like HAEi is it lifts a sense of isolation and aloneness off people."

"My message to younger people is that it is happening to other people, and let's talk about what you could expect. This is a variable disease for the individual, their families, and their lifetime. But we know what could happen, which means we can be prepared. For example, for younger people, puberty is a time when it's like lighter fluid on a barbecue. They can have worsening attacks in terms of frequency and severity."

"The Q&A with the experts was excellent. It has answered several questions I always had about HAE."

"I loved it; the whole experience ... meeting new friends and see a lot of perspectives of the future with HAE."

"The best aspect was all the new information, and the way the sessions were presented."

– Youngsters Track Participants

"Close your eyes-exercise!"

> "I think one of the wonderful things about organizations like HAEi is it lifts a sense of isolation and aloneness off people."
>
> *– Prof. Sandra Christiansen, USA*

"From the standpoint of HAE management, one should hope for the best and plan for the worst. That means you need to know about your on-demand therapy and the options for preventative therapy. Beyond that, you need to be prepared for things that will happen in your life. What if you want to use contraception? You want to use the options that will not make your HAE worse. Then, mainly directed at women, you can become pregnant, but you need to have a plan. There's a lot of data that shows that people with HAE do just fine with getting pregnant, carrying to term, and giving birth. Be prepared to tell healthcare professionals about your therapy, what delivery you want, and what you are worried about afterward - let alone during the pregnancy- and you can make a plan together."

Do young people ask unexpected questions?

"Some of the questions are personal concerns. With swelling, people are worried about lasting damage. Young men ask about genital swelling. It is rare but very, very alarming, and they ask: Is this going to go away? Is it going to cause any lasting damage? These are justifiable worries, and the answer is very reassuring- it will be okay after treatment for the attack."

Next, we asked **Prof. Markus Magerl** about his session, after he'd finished chatting with the younger participants of the track:

"Young people have a completely different view from adults with HAE (and also physicians!), and they have their own anxieties and concerns about how they will live with a disease. Some of the questions might seem basic, but they are fundamental. For example, 'What internal organs can be affected?' The first step is to explain the anatomy of the organs and the abdominal cavity to explain then what happens in these organs when they are affected by an attack."

"Another question is, 'What makes me feel so strange when I have a swelling in the neck region?' The underlying question is what exactly happens when there's throat swelling. The tongue doesn't usually completely close the airway, but it produces a lump or restriction. I think this question is driven by the concern of what if this happens in the future and 'How do I imagine this disease activity inside me?'"

What would you say to young people about asking questions of doctors and experts?

"It's extremely important to ask questions! The most fruitful part of this session was the questions. The answers were tailored rather than just background. I think it's a patient's duty to ask questions: to question the recommendations and advice from their doctor. Ask 'Why is this?' to better understand and find out if the advice is good for you - rather than advice given to anybody."

Find Your Strength and Hold It Up: Creative Workshop

The Youngsters' Track was built around the idea of storytelling. Faye Marshall facilitated a session on the power of words and the impact your words can have on other people.

Faye started with a panel discussion and asked participants to tell everyone their story. Following this, Faye led the young people in a storytelling session. Underneath every chair was an envelope filled with words. Working in pairs, participants used the words as prompts to talk and tell their own story to someone else. Before the session, Faye told us that she wanted participants to go away thinking, "I'm not alone," and to understand the huge levels of compassion and support in this community.

Wrap Up the Youngsters Track

The Youngsters Track came to a close, sharing the support that continues through HAEi and its dedicated website for young people. Victoria told us that from her perspective, the Youngsters' Community is vital because: "You know you're not alone, that you have someone to rely on. It doesn't matter where you are in the world, this community will support you, and you don't have to explain anything. We want everyone out there to know that we've got your back, and we are one family."

> "By empowering people with language and words, we enable them to communicate more effectively with the people around them, because connection is paramount to everything. That's where support is born."
>
> – *Faye Marshall, UK*

Faye Marshall is the resident poet and storyteller for the Youngsters' Track. Global Perspectives sat down with her to understand more about why these sessions are so special.

What did you learn from the first workshop [held in Munich during the HAEi Regional Conference EMEA]?

"In Munich, I had the privilege of facilitating a poetry workshop to help people to recognize their strength and resilience. I learned that the community connection is incredibly strong. We talked about some pretty big things, like how HAE impacts people's lives, whether patients or caregivers. Everyone wanted to share their experience. People were so respectful and so kind and compassionate to each other. There just seems to be a culture of respect and compassion that runs through the youngsters' track. It was like it was like magic in the air."

Things are a little different this time. What have you changed for this conference?

"The focus is still on resilience and strength and advocating for yourself. In Munich, we focused on poetry. In Panama, we'll be talking about the power of stories and the power of language to create connections. The languages spoken in the room may be different, but the story you tell yourself is powerful in altering your perspective and helping you live with positivity."

You are also a nurse; is language important from a clinical perspective, too?

"Yes, absolutely. As humans, we often measure things in time. We don't often measure things in quality. It can be a privilege and honor to experience life with a condition that makes you value time in a different way."

"Part of my day job is to equip people, including, young people, with ways to express themselves as accurately as possible. For example, it's okay to have a down day. It's okay to have a sad time. But language enables us to define what that sadness or that unhappiness is. 'I'm sad', might mean, 'I'm disappointed' or 'I'm scared' or 'I'm lonely.' By empowering people with language and words, we enable them to communicate more effectively with the people around them, because connection is paramount to everything. That's where support is born."

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

I am continually inspired by the dedication and effort shown by our community, from the tireless work of individual Member Organizations to the incredible team at HAEi. The unwavering commitment to improving the lives of those affected by HAE, both locally and globally, is truly commendable.

The recent success of the **2024 HAEi Regional Conference Americas** in Panama was a testament to this dedication. I extend my heartfelt thanks to all who attended and contributed to making the conference a resounding success. Each HAEi conference is uniquely tailored to its region, providing targeted information and capturing the essence of the community. I eagerly anticipate the next series of conferences.

The **2024 HAEi Global Leadership Workshop**, scheduled for later this year, promises to surpass the achievements of 2022. We are listening to feedback from our community and designing a workshop offering more networking opportunities, information sharing, and learning experiences. It will be a valuable opportunity to hear from the incredible advocates within the HAEi network. Alongside the **ACARE Global Angioedema Forum 2024**, this event is not to be missed. See you in Copenhagen!

Reflecting on my previous update, in which I emphasized the importance of improving the lives of those with HAE, I am pleased to report that our teams are intensifying their efforts in targeted local and global awareness programs, education, and events.

HAEi remains dedicated to expanding its reach, locating more patients, and supporting new Member Organizations. This ongoing effort is yielding positive results. Thanks to collaborative efforts involving individuals, Member Organizations, and pharmaceutical companies, patients in many countries are gaining improved access to and reimbursement for modern therapies.

Our commitment to improving the quality of life for all people with HAE is unwavering. HAEi continues to improve its resources and tools, making them available in more languages and tailored to the specific needs of our patients. We constantly seek new ways to support our Member Organizations and those they serve.

Together, we are making significant strides in the fight against HAE, and I am confident that our collective efforts will continue to drive positive change for our community.

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

It feels like only moments since I last updated you in the second edition of the Global Perspectives magazine last year. Back then, I talked about the preparations for and hosting the **2023 HAEi Regional Conference EMEA** in Munich, Germany. This time, it will be another conference that took much of the team's time and effort to plan and execute, the **2024 HAEi Regional Conference Americas**, which took place in Panama City, Panama, from 15-17 March 2024.

I now echo much of what I said then: the team's effort to get such a great event planned, prepared, and executed. That goes for handling travel grants and registrations, booking flight tickets, preparing the three tracks (patient/caregiver, youngsters, and scientific), all the materials needed for the conference (stage, program, hand-outs, etc.), the conference app with personalized information per participant and the Wordly app to secure online, real-time translation of the conference, post-conference survey (we are working hard on learning from the surveys and preparing the overview), and not least running the show with the right presentations at the right time. All this helped ensure the conference was the biggest success possible with the highest impact for all participants.

It was a fantastic conference, and the early survey results indicate this, too.

The Panama conference concluded the first round of three Regional Conferences: Bangkok in March 2023 for the APAC region with more than 200 participants, Munich in September 2023 for the EMEA region with around 650 participants, and Panama for the Americas region with another 650 participants. Hosting a conference like this every six months is a massive task for the team, and a big shout-out goes to everyone who makes these conferences so successful.

Even more so, it is incredibly satisfying to gather 1500 patients/caregivers, healthcare professionals, and pharma representatives to make a real difference. The impact of the three conferences can be read in the survey feedback. The motivation every single participant took home was to continue the fight to raise awareness of HAE and improve the lives of people with HAE. And this is the mission for many more people than could participate in the conferences.

We now look ahead to the **2024 Global Leadership Workshop** (GLW), held in Copenhagen, Denmark, from 3-6 October 2024. The Workshop will be for Board Members and key people involved in the -currently- 99 Member Organization's activities. In addition, we are enhancing the Scientific program by transforming the Scientific track into an HAEi / ACARE co-sponsored event, the **Global Angioedema Forum 2024** (GAF). Industry supporters will also join us for the GLW and GAF and host exhibition spaces to share HAE-related information.

And let us not forget our current **hae day :-)** campaign. Our annual highlight brings our global community together to celebrate our network and each country's remarkable initiatives. It is heartening to see everyone's participation on the haeday.org platform - every story and picture shared celebrates our journey and contributes to our mission of raising HAE awareness.

Here and on haei.org, you can discover and learn a lot more about the resources we offer:

As a Member Organization, whether you need a hosted website, an online, GDPR-compliant, and safe membership database (HAEi Connect), or skills to help with advocacy and to run your Organization (HAEi Advocacy Academy), you will find more information on our website.

As a patient or caregiver, you can access the Understanding HAE and Women with HAE guides, learn about the youngsters' community, and perhaps dive deeper into the LEAP educational program. If you want to take control of your HAE, the **HAE TrackR** app is for you. A personalized, GDPR-compliant diary to record your treatments – and to share your data with your doctor – it is an app built for patients by patients. And finally, the HAE Companion app gives you the HAEi Emergency card on your smartphone for more than 80 countries, including directions to the nearest hospital that can help you.

For all of the above, please contact your Regional Patient Advocate to know more or get started.

In celebrating **hae day :-)** and embracing the full span of activities through April and May, we are reminded that every step we take is a stride toward a better future for everyone affected by HAE. Here is to a successful **hae day :-)** and a year filled with groundbreaking steps and transformative LEAPs!

HAEi GLOBAL LEADERSHIP WORKSHOP COPENHAGEN 3-6 OCT 2024

GAF 4-5 Oct 2024 Copenhagen
Global Angioedema Forum

REGISTER AND SECURE YOUR PLACE!

3-6 October 2024 in Copenhagen, Denmark

2024 HAEi Global Leadership Workshop

GLW is for HAEi Key Patient Opinion Leaders – our Board Members and Leaders from our Member Organizations – and Industry Representatives.

Explore **"Navigating the Future"** in inspirational and educational sessions across two days, 4 and 5 October 2024.

ACARE Global Angioedema Forum 2024

GAF replaces the previous GLW scientific track and is for HAE Physicians/Scientists and Industry Representatives.

Learn and exchange ideas on all forms of angioedema across two days, 4 and 5 October 2024.

Our Call for Abstracts is open now.

All participants join together when 'GAF meets GLW' during the afternoon 5 October.

Register for GLW and GAF now >> glw.haei.org

hae day :-)

MANY FACES ONE FAMILY

Join us be #active4HAE for **hae day :-)**

16 May is **hae day :-)**, our annual celebration of the HAE community and an important chance to raise awareness. Time is running short, but we know you will take it in your stride.

As ever, there's so much going on. The activity challenge is open for submissions and waiting for you.

Time spent on every activity, from the smallest act of self-care to the biggest adventure, counts towards our global total.

Join the many participants who are already logging their physical and well-being activities and unlocking badges on their profiles!

Get involved and inspired at haeday.org

Walking the walk **hae day :-)** inspiration from HAE Serbia

One new event will be an awareness hike in Serbia, which is down to the hard work of the Member Organization (MO) in Serbia, especially one of the 2023 LEAP students, Sofija Popović.

HAEi LEAP allows young people to learn new skills and develop as individuals and advocates. Each student gains experience working with their MOs to apply those skills on a project to support advocacy, their MO, and their community. Sofija was part of the LEAP Class of 2023 and told us that the idea of an awareness hike came from the desire to create an engaging way for the local community to increase knowledge of HAE while spending time together in nature.

Sofija credits the HAEi LEAP 2023 program for helping her plan and execute the awareness hike effectively. She said, "Participating in the HAEi LEAP program was not only incredibly enriching but also inspiring. LEAP provided a crucial foundation, covering a spectrum of essential skills ranging from time and project management to writing reports and proposals for advocacy projects and event coordination."

"As the event approaches, I feel excitement and anticipation. I am very excited to see the community come together, and I look forward to seeing the positive impact this event will have on raising awareness and support," added Sofija. "I would like to express gratitude to everyone involved in making this awareness hike possible, from everyone involved in the HAEi LEAP program to the members of HAE Serbia. Together, our community is making a difference and supporting those with HAE."

Jovana Cvetkovic, the President of HAE Serbia, told Global Perspectives she is excited about the upcoming event, "I am eager to walk together in support of individuals living with HAE and raise awareness. I hope that our event will make a significant impact on our mission." Jovana outlined that the event will allow HAE Serbia to support the global campaign, raise local awareness of HAE, and engage association members, doctors, and the public. "I want to thank HAEi and the Youngsters' Community for giving Sofija a chance to showcase her exceptional organizational and creative skills in raising awareness about HAE. I'm thrilled that this is just the beginning of our partnership, and I'm excited to continue working together to positively impact the lives of people with HAE."

You can follow HAE Serbia and keep up with the action from the hike on instagram @hae_srbija and Facebook HAE Srbija

Jovana Cvetkovic
President of HAE Serbia

Sofija Popović
2023 LEAP student

Sofija and Jovana's tips for organizing an event for hae day :-)

- Plan in advance to ensure you can get everything done in time
- Engage with the community to share skills and resources
- Involve partners and sponsors who can promote the event and provide support
- Focus on creating a supportive environment where members are motivated to participate, as this will enhance the impact of the event
- Be consistent in your efforts to raise awareness about HAE and engage with their local community

We're very excited to see the results on #activeforHAE, and we'll catch up with Sofija and Jovana in a future edition to hear more.

Whatever you do, big or small, HAEi wants to hear about it. You'll find all the details on how to submit events at haeday.org. Just check out the Get Inspired section.

ACARE ANSWERS

We asked ACARE centers from our Asia Pacific, Americas, and EMEA regions to tell us a little about their journey. This time, ACARE Answers is focused on the Americas region. The ACARE centers and experts answering the questions are from Canada, Ecuador, and Peru.

University of Alberta, Canada

Questions answered by Dr. Adil Adatia, Clinical Immunology and Allergy, Assistant Professor, University of Alberta.

What benefits have you experienced since becoming an accredited ACARE center? Since joining the ACARE family late in 2023, our center has been involved in two important HAE research initiatives: MENTALIST and IMAGINE 2.0. Additionally, we regularly participate in ACARE educational programs and conferences to ensure we provide patient care informed by the latest evidence.

How has care improved for your patients since the University of Alberta became an accredited ACARE center? For us, accreditation was the final step in an extensive process that embodies a clinic's commitment to excellence in patient care. Dr. Bruce Ritchie founded the angioedema clinic in the early 2000s. Since then, a vision materialized into a large program that has been a beacon of hope and relief for many patients suffering from angioedema, particularly HAE.

Central to our philosophy is the belief that effective care encompasses more than just managing physical symptoms. To realize this vision, we established a multidisciplinary team that included angioedema physicians, nurses, social workers with mental health expertise, and dentists. We also regularly offer patients opportunities to participate in clinical research, including trials for many modern HAE treatments available today. Joining the ACARE network provides us with a platform to continue advancing angioedema care.

Espiritu Santo University (UEES), Ecuador

Questions answered by Prof. Ivan Cherrez Ojeda, Professor of Allergy, Immunology & Pulmonary Medicine at the Department of Allergy, Immunology and Pulmonology, Espiritu Santo University.

What benefits have you experienced since becoming an accredited ACARE center? As an ACARE center, we can access the most current research findings and guidelines for managing angioedema. We have also undergone training and specialized education, enabling us to stay up-to-date in diagnosing, managing, and monitoring patients with angioedema. Being part of a network with centers worldwide, we can participate in a variety of scientific projects, present our projects, and, therefore, collaborate with the centers in the network. ACARE has equipped us with custom-designed resources and technical support to facilitate patient education and provide assistance tailored to the distinctive requirements of individuals with HAE and their families. We have also had support to increase awareness regarding angioedema. This empowerment has enabled both patients and healthcare providers to manage all facets of angioedema effectively.

How has care improved for your patients since Hospital Clinica Kennedy Policentro became an accredited ACARE center? By expanding our understanding of angioedema, we've enhanced the quality of life for our patients through optimal management practices, a feat that wouldn't have been achievable otherwise.

What has becoming an accredited ACARE center enabled you to achieve? One example is that becoming an ACARE center has been pivotal in elevating our standing within the angioedema community of physicians and patients, instilling credibility and professionalism in our institution. This credibility allowed us to undertake a ground-breaking research project focusing on the prevalence of sleep disorders among angioedema patients. Leveraging our ACARE status, we could extend invitations to the Latin American community, fostering enthusiastic collaboration. This collaboration proved invaluable, leading us to secure a prestigious prize during the **2024 HAEi Regional Conference Americas**.

Clínica SANNA el Golf, Peru

Questions answered by Dr. Oscar Manuel Enrique Calderón Llosa, Allergist and Clinical Immunologist, Clínica SANNA el Golf, Peru.

What benefits have you experienced since becoming an accredited ACARE center?

In the last 5 years, we have improved the blood testing we can offer for HAE to provide a better diagnosis for our HAE patients. As more patients are diagnosed, we have a greater opportunity to request HAE-specific treatments from the Peruvian government. As an ACARE center, our patients and physicians can participate in different studies carried out by the network, such as the IMAGINE 2.0 project. This year, we were eligible for a clinical trial with an investigational HAE therapy molecule. We are always working for the benefit of our patients.

How has care improved for your patients since SANNA Clinica el Golf became an accredited ACARE center?

Our ACARE center is a "safe place" for our diagnosed patients. It is a place where they can receive counseling, and we attend to their HAE attacks. Our patient care is standardized through our protocols, a key part of the accreditation process.

What has becoming an accredited ACARE center enabled you to achieve?

One example among many is that during the COVID-19 pandemic, in coordination with the HAE Perú board of directors, we facilitated coronavirus vaccination in our patients through an ACARE certificate from the center.

The best success story for me is that now patients arrive at the emergency room at the Golf Clinic and are recognized by the doctors and nurses in the emergency room. These healthcare professionals are the ones who provide them with the first care, generating a certain feeling of calm in patients.

Visit haei.org to for more information about how to become an ACARE center. On the website, you will also find contact details for ACARE centers and a link to an updated list of ACARE Centers across the globe.

>> HAEI.ORG

Update from the HAEi Youngsters' Community

The HAEi Youngsters' Community has a lot going on. You can read all about the education and fun we had in Panama in the special Regional Conference section, and here are a few other highlights.

INTRODUCING VICTORIA

We are very happy to introduce Victoria Schultz-Boysen, who has joined the HAEi family as Coordinator, Youngsters' Community.

You will hear more from Victoria in the coming months as Nevena Tsutsumanova, HAEi's Manager, Youngsters' Community and Special Projects, is soon heading off on maternity leave.

Victoria loves to savor all the little things in life: beautiful sunsets, flowers, and people smiling :-).

Victoria has been part of the Youngsters' Community since 2017 and is a caregiver to her brother and father, who live with HAE. She was also a part of the first LEAP program in the Class of 2023.

You can get in contact with Victoria via her email: v.schultz-boysen@haei.org

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 join us!

SOCIAL MEDIA INTERNSHIP PROGRAM

In January 2024, the HAEi Youngsters' Community launched our first Social Media Internship Program! Four wonderful interns are filling our social media channels with interesting and educational content.

The Social Media Internship Program aims to build a vibrant online community for youngsters with HAE and their caregivers.

Our interns have had the chance to interview inspiring youngsters with HAE, create informative and engaging content, learn more about social media, and actively contribute to our global mission of raising awareness about HAE.

A few of our wonderful interns joined the **2024 HAEi Regional Conference Americas** to cover the Youngsters' Track activities!

Here are some highlights from their work so far:

Follow us on Instagram @haeiyoungsters and enjoy more of our interns' work!

>> https://www.instagram.com/haeiyoungsters/

Interested in becoming a SoMe Intern?

Would being a social media intern be something you would like to do?

If yes, then you are in luck! The HAEi Youngsters' Community Social Media internship Program will open for new applications later this year! Sign up for our Youngsters' Community newsletter to be the first to know when applications are live: youngsters.haei.org/join-us

NEWS FROM HAEi COUNTRIES AROUND THE GLOBE

ARGENTINA

From HAE Argentina

AEH Argentina participation in HAEi's Conference of the Americas. From March 15th to 17th, AEH Argentina had a chance to attend HAEi's Regional Conference of the Americas. Twenty-five members of our organization, including patients, caregivers, and representatives of our Youngsters' group, traveled to Panamá to participate in the event. Carrying luggage full of hopes in anticipation of the meeting ahead, they were all ready to "Take Action", as the conference motto proposed.

As expected, from beginning to end, the conference provided a perfect setting for learning and sharing, allowing our group to experience that unique sense of togetherness, only possible when surrounded by those speaking the same common language: HAE. By bringing together diverse stakeholders: patients, physicians, and industry representatives, the meeting proved to be a unique platform for collaboration and communication, fostering mutual understanding and empowerment among all the parties included.

The panel of Patient Organizations was extremely enriching for us all; listening to other organizations' journeys instilled confidence in an even brighter future and determination to continue helping those most in need. Needless to say, the empathy, support, and encouragement among the patient community were awe-inspiring.

The Youngsters' Track provided yet another highlight of the weekend. In a joyful atmosphere, with endless networking opportunities, our young representatives left the meeting filled with enthusiasm and a wealth of knowledge and information. Inspired by the support and encouragement of many new friends, they are ready to continue their HAE road ahead, now armed with new and very useful tools.

Our group was also enhanced by the participation of one of our HAE specialists from Argentina, Dr. Ricardo Zwiener, who made presentations in the Poster Session and the Scientific Track of the event.

Retrospectively, after two amazing conference days, we couldn't help but travel back in time 20 years ago, when most of our patients lived in fear of their condition and would not even dare dream of being away from home, let alone traveling abroad. The fact that HAE patients can now embark on these new "adventures" unquestionably proves that with access to modern treatments, we have finally attained the long-awaited freedom. Patients in Argentina can now choose among three different modern prophylaxis options for HAE and three medications for on-demand usage, giving us the chance to tailor our own treatment plans according to our own specific needs.

We can't thank HAEi enough for giving our patient community a chance to share these wonderful days among friends, and we look forward to future opportunities that tremendously enrich our patients' lives. This type of event confirms, yet again, the galvanizing power of HAEi as a key pillar for the HAE global movement.

At the end of our "Panamanian dream", we are very happy to realize that long gone are those days when HAE was all about suffering. The fight for the cure continues.

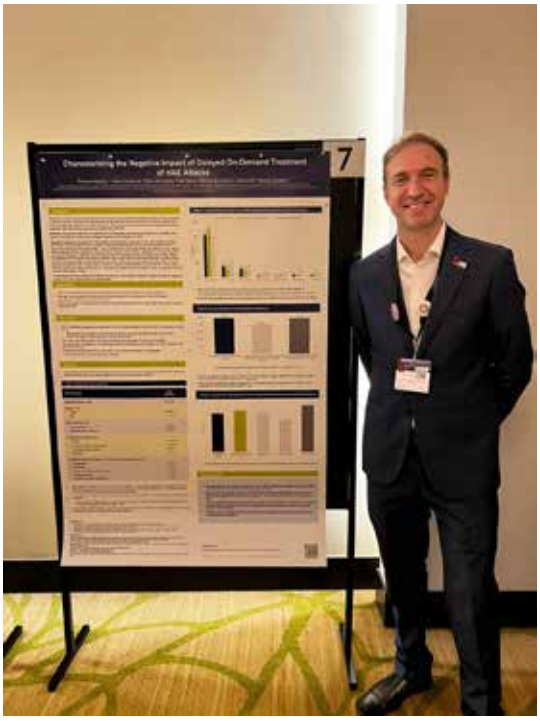
Patients' quotes from the conference:

"The conference was an incredibly revealing experience. It was our first time in an international event. So much to learn and share with this wonderful group of amazing people. Very thankful to our organization and to HAEi. We are thrilled to feel part of this community."

"There is nothing more comforting than being among other people who understand 100 % what we feel and think."

"Amazing learning and fraternity experience. Short for words."


AMERICAS
PANAMA CITY
15-17 MAR 2024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AUSTRALIA & NEW ZEALAND

From Fiona Wardman, HAE Australasia

HAE Australasia has released a series of videos highlighting the impact of access to prophylaxis medication for Hereditary Angioedema (HAE) patients. The videos compare the current access in Australia, which is not ideal but better than New Zealand, where access is not yet available.

Featuring young people with HAE and their parents, the videos depict the challenges faced before access to prophylaxis medication, including frequent attacks and emotional strain. They also showcase the positive changes brought about by treatment, such as increased freedom and improved mental health.

These videos are a call to action that shows the importance of access to treatment and the need for change.

The videos aim to raise awareness and urge policymakers to address the disparity in access to treatment for HAE patients.

The videos can be viewed via the following link: https://haeaustralasia.org.au/resources/video-resources/

BRAZIL

From HAE Brazil

Representing Brazil at the Regional Conference of the Americas - HAEi, held in March 2024 in Panama City, there were 58 participants, most of them patients. This group, on behalf of ABRANGHE, included patients and caregivers who shared their experiences, accessed important information, explored relevant topics, and gained a deeper understanding of the work carried out by the International Association.

One of the most inspiring moments occurred during the testimony of Lucas, a patient who highlighted how the Identification Card can save lives. Brilliantly, he shared his experience with an episode of glottis edema and the actions taken to reverse this situation, which is a constant concern for all HAE patients.

This and other stories highlighted the importance of unity and teamwork to publicize Hereditary Angioedema, as well as the need to join forces to improve treatment conditions for this disease. Without a doubt, the Conference was a space for very enriching exchanges and learning.

CANADA

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

The HAE Canada Board of Directors began 2024 thinking about the next five years. In January we had the first of five meetings to plan our next Five-Year Strategic Plan. We are keen to plan new projects, set goals and update our mission and vision. We will continue to work on this over the next few months, and we are excited to begin this important task.

Rare Disease Day is celebrated each February, and to honor this international event, HAE Canada Board members Kerstyn Lane and Carmen Craciun, along with staff member Daphne Dumbrille, attended the Canadian Organization for Rare Disorders (CORD) Rare Disease Day Summit in Ottawa (February 28-29). HAE Canada joined others in the rare disease space to learn about many issues, such as how Canada can speed up access to drugs and why the government needs to move faster and launch the promised National Strategy for Drugs for Rare Diseases.

On Thursday, February 29th, CORD invited HAEC, along with other patient organizations and some Members of Parliament (MPs), to attend the "Breakfast on the Hill." CORD took this opportunity to share patient stories and stress the importance of implementing the National Strategy for Drugs for Rare Diseases. Following the reception, we marched to Parliament Hill to further raise awareness for Rare Disease Day and in support of the Fight for Our Lives campaign.

Throughout the year, the Rare Disease Day organization is "raising awareness and generating change for the 300 million people worldwide living with a rare disease, their families and carers." One of their initiatives is the Light Up For Rare campaign, which encourages people to arrange for local monuments to be lit up in the Rare Disease Day colors at 7:00 pm (local time) on February 28th or 29th (on a leap year). HAE Canada's Kim Speiss (Central Regional Director) wanted her city to Light Up, so she reached out to the City of Winnipeg in Manitoba to have lights shine on the Esplanade Riel Footbridge – and they said yes. This Global Chain of Lights campaign not only illuminates the world but also spreads awareness to everyone living with a rare disease.

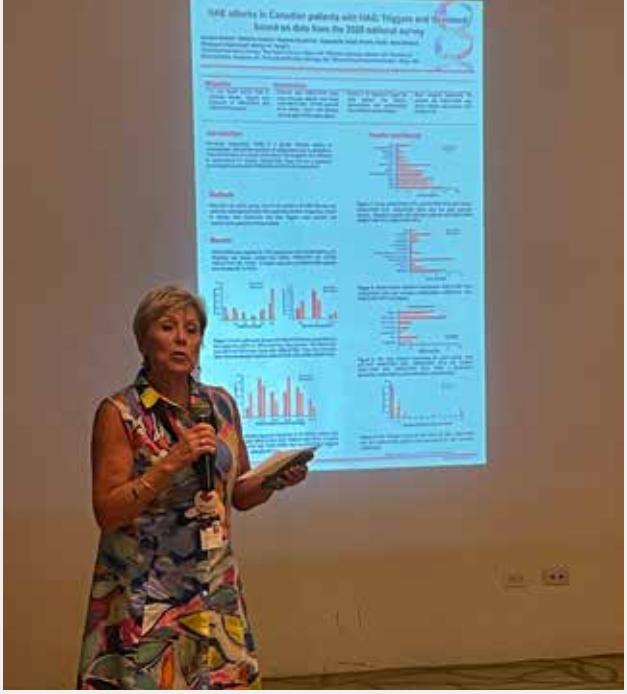
Access to new treatments can take a very long time, and the wait in Canada is especially long. According to Innovative Medicines Canada, Canadian patients can wait up to two years for access to approved new medicines through public drug plans. HAE patients in Canada are experiencing the frustration firsthand as they wait for access to berotralstat (Orladeyo). Berotralstat was approved for reimbursement back in March 2023, and price negotiations between the pan-Canadian Pharmaceutical Alliance (pCPA) and the manufacturer should have been completed 40 business days later, but patients are still waiting. HAE Canada sent letters to the pCPA and the manufacturer, urging

them to accelerate negotiations so patients can gain access to this oral treatment. We now understand negotiations were finally initiated by the pCPA in January 2024, so we are hopeful the process will move at an accelerated pace.

We had the amazing opportunity to attend the HAEi Regional Conference Americas from March 15 to 17. Patients, caregivers, leadership teams, physicians, and industry representatives met just outside Panama City to learn and connect with one another. Thanks to HAE International's travel grants, over 30 HAE Canada members joined HAEC Board and staff members in Panama. Knowing there were going to be over 600 people at the conference, we hosted an informal Meet & Greet on Friday afternoon to allow our members to get to know one another before the busy schedule began. It was amazing to spend valuable time with our fantastic members - in person!

The conference officially began on Friday evening with a keynote presentation and an official welcome from HAEi. Day Two's schedule was jam-packed with presentations from HAEi, physicians, Member Organizations, and patients. There is no need to provide full details about the conference's content since it is expertly summarized earlier in this publication, but we do need to say how we are all incredibly grateful that we were given the opportunity to experience this worthwhile event.

Canada was lucky to have two people present at the conference. Anne from Ontario thoughtfully and kindly told her patient journey. She spoke from the heart, and her presentation touched everyone in the room.



We are grateful for Anne's dedication to advocacy and openness to share her perspective. We are also proud of Michelle Cooper, HAEC President, who represented HAE Canada extremely well on two panels during the Patient and Caregiver Track, one titled "The Americas Member Organizations' Panel Discussion" and the other titled "The Path to Success in the Americas." Michelle was kept busy as she also attended the Scientific Track to present our poster titled "HAE attacks in Canadian patients with HAE: Triggers and treatment based on data from the 2020 national survey". Thank you, Michelle and Anne, for making us proud!

COSTA RICA

From Isabel González Rodriguez, Secretary, HAE Costa Rica

Our country has gradually evolved on the issue of Hereditary Angioedema. In Costa Rica, there was not the slightest knowledge of this disease. My father (Gerardo González Medina) was diagnosed with HAE

at sixty years of age by Dr. Mario Martínez, who is a specialist in allergology at the México Hospital in San José, Costa Rica.

Approximately six years ago, the tests to diagnose this disease were not performed in the CCSS (Costa Rican Social Security Fund), only in private centers that sent the C1 and C4 inhibitor tests to the United States. But now, the CCSS is carrying out these exams.

Two years ago, patients with Hereditary Angioedema only had Danazol and Fresh Frozen Plasma, which took a long time to thaw and caused allergies in some patients.

Currently, patients in Costa Rica have two drug options focused on HAE: Berinert, which is financed by the hospital, and generic icatibant, which is financed by Social Security.

The Children's Hospital in Costa Rica performed the first genetic examination on my daughter (Tayra Corrales), and we have noticed great progress in Costa Rica. Our country is advancing in the right way.

There is still much to achieve as a country, such as expanding knowledge of this disease to more doctors and specialists and ensuring more treatments for every patient with HAE.

ECUADOR

From Edison Galarraga, HAE Ecuador

For the benefit of the group of patients with Hereditary Angioedema without diagnosis in Ecuador, the 'De Las Americas' University (UDLA) based in Quito, the Capital of Ecuador, after meeting various ethical, legal, and methodological requirements and approval of resources, dated 6/march/2024, began the execution of the research project "FUNCTIONAL CHARACTERIZATION OF HEREDITARY ANGIOEDEMA TYPE I AND II, IN THE ECUADORIAN POPULATION ASSOCIATED WITH THE MUTATIONS OF THE SERPING1 GENE".

In the first phase, the university will work with several diagnosed and undiagnosed patients in several cities where the incidence of this pathology is more recurrent.

For the next stages, the University will be able to have a diagnostic protocol that will benefit patients, family members and future patients who may join HAE Ecuador, an important step to visualize this rare disease before the country's health authorities.

For the third consecutive year, the University (UDLA) reaffirms its position as the #1 institution in scientific research in Ecuador. This was confirmed by the 2024 edition of the outstanding SCImago world university ranking.

The group of HAE patients in Ecuador thanks such a prestigious University for its interest in contributing with its scientific research and resources and taking a giant step towards the reality of this rare disease in the country.

GREECE

From HAE Greece

In March 2024, I was invited to speak at a national allergiology conference for doctors working in military hospitals. I was very honored and excited to accept this invitation because this was a great chance to inform doctors and the heads of many public hospitals in Greece.

What some people might not realize is that military doctors in Greece see every man at least once in their lives since military service is mandatory for all males in Greece, and they also see all citizens in places where there are no doctors or hospitals except small islands. As you can understand, this makes a big difference in reaching people who might not have been diagnosed and have limited access to healthcare.

Dr. Psarros, who is in charge of the HAE ACARE center at the Naval Hospital of Athens, and Dr Kassiani Tzeli, who organized the conference, decided to highlight HAE to spread awareness about rare diseases.

More so, they called me so I could share my experience from the patient's perspective so doctors can understand how to better take care of HAE patients and identify symptoms in undiagnosed patients.

With Dr Psarros and Dr Tzeli's approval, we also invited Dr Argyriou, a pediatric psychiatrist, to speak on the psychological impact HAE has on patients and especially young children and how doctors should encourage people to get support if needed. Dr Argyriou has experience with HAE patients and chronic and rare diseases and was also present in the last HAEi conference in Munich.

Also, a caregiver and wife of a patient shared her story about the loss of someone with HAE due to denial of the disease and how detrimental it can be to them and their families.

During my speech, I shared my journey from my first symptoms and attacks to my diagnosis and the impact HAE has had on my life. I highlighted the importance and improvement modern therapies have made in my everyday life and how proper management of HAE can lead close to a normal life.

My take after my experience was how important it was to include patients in an event like this. A lack of the patient's perspective might make doctors not understand patients' needs and that might decrease the quality of care we receive. A better communication will lead to a better collaboration for the patients best interest.

HUNGARY

From HAE Hungary, Maros Zoltán

Dear fellow patients and interested parties,

On the occasion of Rare Disease Week and World Day, the Association of People Living with Rare and Congenital Disorders (RIROSZ), with its 61 Member Organizations, including the participation of the Hungarian HAE Patient Organization and the University of Sopron, in cooperation with the city of Sopron, organized the "rare day" for the seventeenth time this year February 2024 on Saturday the 24th. The aim of the program is to create a forum where the relevant interest groups can jointly discuss the situation and plans of people living with rare diseases. This year, the main topics were the inclusion of the NW area of the country in the "circulation" of domestic and international rare diseases, the second National Rare Disease Plan, equal opportunities, and fairness. Our organization was represented at the event by our fellow patients, Dr. Arianna Kitzinger (associate professor of the University of Sopron and member of the organizing committee), and Péter Stráhl. The Hungarian Angioedema Center of Reference and Excellence was represented by Dr. Hanga Horváth, PhD student. It was very useful that both the patients and the doctor were able to share their thoughts and knowledge with the interested people visiting our stand. Among our interested parties were other patients suffering from rare diseases, representatives of pharmaceutical companies, health consultants, and family doctors. We tried to give a comprehensive picture of what surrounds us in relation to HAE, both from the side of patients and doctors. We agreed with other patients suffering from rare diseases and the representatives of patient organizations that the most difficult task at present is the involvement of our fellow patients in the daily work and the sensitizing of other fellow patients who may need help and more information.

IREALND

From Bettina Carty, HAE Ireland

Hi All.

Well, it has been a busy few months here with HAE Ireland, and I was very proud to have been asked to take part in the "I Am Number 17" campaign to spread the word about rare diseases. It was a great way to let the public know more about HAE.

It was opened with an exhibition in The CHQ Building in Dublin on 7th February, which was attended by the Irish Minister for Health, Stephen Donnelly. There were 17 portraits unveiled on the day, one for each of the 17 patients with different diseases. Then, on 27th February, they went on public display in St. Stephen's Green Park in the centre of Dublin City for a month. It was also attended by some government ministers, including our Minister for Disability, Anne Rabbitte. Having it so public will mean thousands of people will be able to walk by and see a patient representation of HAE …

Numerous articles were printed in the media, and I was also interviewed on a popular Radio show – it was amazing to share the HAE journey from a patient's perspective.

Looking forward to what the rest of 2024 brings …

JAPAN

From HAE Japan (HAEJ)

This past year, we have placed an emphasis on having smaller meetings in regional areas. In addition to our main meetings in Tokyo and Osaka, we have also had smaller meetings in Okinawa, Hiroshima, Kyoto, and Sapporo. These smaller meetings have allowed us to meet patients who generally would not travel to Osaka or Tokyo. It also allows us to renew relationships with physicians who are a part of our medical advisory board but also make links with new treating physicians. The Okinawa meeting was timed to coincide with a major academic meeting for dermatologists, and we hosted a booth over the two days of the conference. We went to Sapporo in Hokkaido in March and had a fantastic meeting not only with patients but two physicians and one nurse attended, plus ten (10) nursing students. Our industry partners also made a huge effort with representatives from all the companies with products for HAE attending.

In February this year, as usual, we hosted the Rare Disease Day (RDD) event in Kobe. It was very well attended not only by HAE patients and family members, but also other patients with rare diseases. Again, we had a very sound turn out from industry representatives.

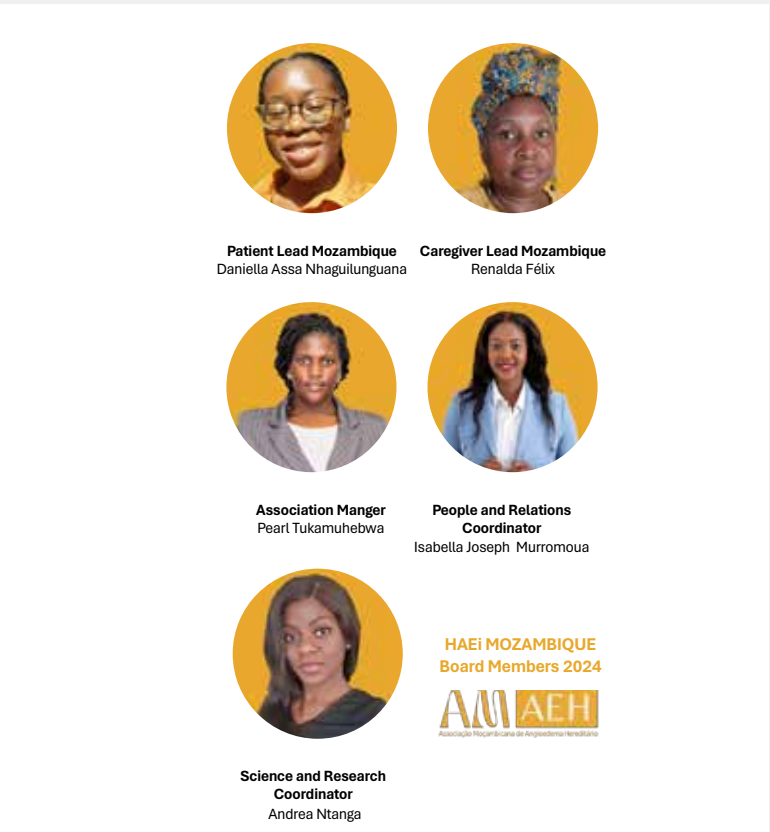
We have been developing a new web page and we hope to launch it over the next month.

Finally, we are making big plans for hae day :-), which coincides with the 10th anniversary of HAEJ as a registered Non-profit organization. In addition to a walking event in Tokyo, we are working with the Discovery Consortium (Diagnostic Consortium to Advance the Ecosystem for Hereditary Angioedema), a nationwide light-up event over three days across HAE Day. We currently have 18 light-up sites confirmed throughout Japan (castles, big wheels, towers, famous tower blocks, etc.). The lighting will be purple, and we will be sending steering committee members out to different sites to maximize media coverage.

MOZAMBIQUE

From Daniella Assa, Patient Lead, HAE Mozambique

February: During the month of February, we held our first meeting as an MO, and because some of the members are dispersed throughout Mozambique, it was held online. Our first online meet was held on the 29th of February, where we also celebrated Rare Disease Day! We had a total of eight members attend, including doctors, biologists, social advocates, and patients. We were encouraged by the motivation behind each member and content to have opened a space where we could share knowledge about the disease. Members demonstrated clear interest and desire to advocate and learn more about HAE in Mozambique. From introducing ourselves and debating on issues surrounding healthcare in Mozambique, we all had a fun time. The highlight of our meeting, however, was the participation of Patricia Karani, our regional Patient Advocate, who shared a story of resilience and tenacity and inspired us to push through this new mission with much courage and a strong belief that we can indeed make a change. Patricia gave us hope and encouraged us to continue, dedicated to advocacy. This year, we will be taking action.



March: This month, we successfully conceived the board of members for our MO. Our team is composed of a diversity of talented women with different areas of training and expertise (science and research, social work, and project management), bound by a solo passion for advocating while standing in the gap for all existing and yet-to-be-diagnosed patients in Mozambique. We have a lot of work to do ahead of us. However, we continue to be confident that with dedication, we can deliver a positive impact in Mozambique. We hope for a future where Mozambican HAE patients and caregivers have access to resources that better the quality of their lives.

THE NETHERLANDS

From Maria Beekman, President, HAE the Netherlands

It's been a busy few months for HAE the Netherlands! We spent the better part of January Brainstorming and planning activities to raise awareness and support patients and their families in 2024. One project we worked on was a national survey. The last time a survey was rolled out was many years ago. A lot has changed since that time. With this survey, we hope to identify where information is lacking, for instance, about new treatment options. We believe it's also a great way to find out how people in the Netherlands living with HAE experience living with this disease. With this kind of information, we will be able to improve our services.

We're hoping a lot of people will participate in this survey. If you're a person with HAE living in the Netherlands or you're a parent of a child diagnosed with HAE, please participate in this survey. Click on the link to go to the survey: https://bit.ly/google-form-NL.

February was the month of Rare Disease Day. Rare Disease Day or even Rare Disease Awareness Month is

not a big thing in the Netherlands. The general public is not aware of how many people are living with a rare disease, and why it's important to raise awareness.

For the first time ever, HAE the Netherlands participated in the global awareness campaign via social media. We reached quite a number of people, even though our campaign was small. On 29 February, we participated in an event hosted by the Dutch organization for rare diseases. In the afternoon, we listened to presentations about regulations, cooperation, and challenges. Afterward, we enjoyed a networking dinner. That gave us plenty of opportunities to expand our network. The evening focused on patients, patient advocates, and research. There were several presentations from researchers and patient advocates about specific research projects. We were very pleased that one of our members, who participated in an important research project, was given the opportunity to share his experiences with the audience. We're grateful that he was willing to share his story. Awareness starts with patients advocating and sharing stories. The most important thing we learned in February is that there's a lack of attention to rare diseases in the Netherlands. That's why we joined a focus group of patient advocates in all fields of rare diseases to work together in raising awareness and improving the lives of people in the Netherlands living with rare diseases.

Raising awareness of rare diseases in general will help us create awareness of HAE in particular. Together, we can accomplish great things.

In March, our focus was mainly on organizing our annual patient meeting. We plan to have the meeting on 12 October 2024 in Breukelen. For people in the Netherlands: please save the date! We can't yet share an agenda for the meeting since we're still brainstorming. We can tell you that it will be a fun day, full of presentations and opportunities to talk to fellow patients and caregivers. Follow our social media accounts to make sure you have the latest updates on the annual meeting.

NORTH MACEDONIA

From Natasha Jovanovska Popovska, President, HAE North Macedonia

The Rare Disease Day 2024 in Macedonia was marked by a panel discussion in Skopje, organized by the Macedonian National Rare Disease Alliance. The event was under the veil of discussing the current situation of people with rare diseases and their families. The need for systematic solutions was also addressed through the adoption of a Rare Disease Strategy with an action plan and precisely defined areas of action (health, labor, social aspect, and education).

The event was attended by the first lady of Macedonia, Mrs. Elizabeta Gjorgievska, who gave a warm speech but also decisively addressed and made an appeal for better conditions for people with rare diseases. Macedonian Minister of Health Ilir Demiri also attended the meeting and expressed his support for this group of patients, giving hope for better future.

The HAEi RPA Natasha Angjeleska was announced as the next president of the National Rare Disease Alliance, receiving the sincere support of most Member Organizations. Natasha addressed the event, which was greatly covered by media, by saying that strength is not in numbers but in character and integrity, elaborating that over the years, she has learned that regardless of whether you are one, part of a group, or part of the majority, you are worth as much as your character and actions. She added that, although rare disease patients are always asked about numbers, numbers don't really matter; what matters is that each patient is a valuable individual. Natasha also added that she had met great people throughout her ordeal with her child's diagnosis and mentioned that doctors and nurses felt like an umbrella to her, come rain or shine. She, however,

stressed that the greatest people she had met in her life, who felt like her second family, were rare disease patients and their families.

The Rare Disease Day was also marked by another prominent member of HAE Macedonia, Verce Jovanovska Jankovska, giving a statement for the Balkan branch of Aljazeera media group. She spoke about her son and dealing with HAE challenges from the moment he was diagnosed without proper therapy in the country, to the moment when he received preventive subcutaneous treatment, that has greatly improved the quality of his life. Several members of HAE Macedonia also attended the Rare Disease Day event.

In marking International Women's Day, 8 March, a local Television 24 chose four (4) brave women who fight challenging battles in life. Natasha Angeleska from HAE Macedonia and the HAEi RPA was chosen to be one of those heroines, speaking about the struggle with her son diagnosed with HAE. In her talk, she stressed, "We don't need celebration and flowers, but equality and breaking stereotypes. They are the factors that still create a glass ceiling in the battle for full equality of women".

PARAGUAY

Please enjoy the pictures from HAE Paraguay's commemoration of Rare Diseases' Day in Paraguay:

On February 27, 2024, in a meeting with the Minister of Health Maria Teresa Baran, together with FEPPER, Hereditary Angioedema Paraguay expressed the desire to urgently have specific medication, care protocol, diagnostic methods, reference centers, and assigned professionals for the treatment of HAE.

PERU

From Carla M. Goachet Boulanger,
Directora General, HAE Peru

We begin the year 2024 by spreading the AEH with our young people from AEHPERU;

- Mafe Elera carried out an awareness HAE talk to her schoolmates.
- Kamila Salaverry carried out a dissemination campaign in the different health centers in the city of Trujillo, carrying the Emergency Poster designed by HAE International.




In February, for rare disease month:

- Claudia Raffael and Milagros Cordova, together with the Ministry of Education and FEPER, held an awareness talk about rare diseases and Aeh for health personnel at the Breña Children's Hospital.


- Carla Goachet was invited by the Ministry of Health of Peru, representing Feper, to speak about the problems and barriers that patients face in reaching a diagnosis of HAE.


- In the city of Chimbote, we carried out an information campaign and HAE training talk by Dr. Oscar Calderón at the Eleazar Guzman Barron Regional Hospital.


- We are very happy that, for the first time, our AEHPERU members, patients, caregivers, and family members could participate in an HAEi Patient Conference. An experience that allowed us to learn about HAE, to meet and to share with the entire HAE AMERICAS community.

PORTUGAL

From HAE Portugal

On 16 March, ADAH held its first face-to-face meeting on the subject of "Zero attacks, dream or reality". The meeting was divided into three parts: the scientific part, with the presence of three specialist doctors who addressed the topics "New therapies for HAE", "The potential of new therapies in pregnancy and childhood", and "Perspectives for patients currently excluded from new therapies". This was followed by an associative part where HAEi and ADAH gave presentations on their role and contribution to improving patient access to new therapies. Finally, we had testimonies from three patients about their experiences with new therapies and from a mother of two children with HAE. The meeting was followed by a small get-together. We had a large participation both in person and online, and the feedback we received was very good. All participants really enjoyed it and found this type of meeting very important. To be repeated.

PUERTO RICO

It's Law – Another Law approved on Hereditary Angioedema.

After several years of intense work, meetings, and perseverance of patients with HAE in the country of Puerto Rico, together with the commitment of Dr. Rafael Zaragoza, author of the bill, US HAEA, HAE International (HAEi), and Puerto Rican legislators José Enrique Meléndez and Sol Higgins, the HAE legislation finally became law. We congratulate all the patients of Puerto Rico for their commitment, effort and demonstration of what it means to work as a team. We also thank the Governor of Puerto Rico, Hon. Pedro Pierluisi and the Presidents of the House of Representatives and the Senate 2020-2024 for their support and sensitivity to patients with HAE and their families in Puerto Rico.

The new approved Law establishes the following:

- The School of Medical Sciences will offer continuing education for health professionals on the identification, diagnosis and treatment of Hereditary Angioedema disease.
- It is established that every academic program within the jurisdiction of Puerto Rico that provides a degree in any of the branches of medicine, nursing, emergency medical technicians, will include topics related to the diagnosis, treatment and management of the disease of Hereditary Angioedema.
- Official medical guidelines on the treatment and diagnosis of Hereditary Angioedema and the medical equipment necessary to care for patients, which is used in other jurisdictions in the United States, will be adopted and will be distributed and applied in all emergency centers. and hospitals in Puerto Rico.
- The Department of Health and the Government of Puerto Rico must organize and celebrate activities to commemorate and raise awareness about the condition of Hereditary Angioedema.
- The School of Medical Sciences will promote and collaborate in the development of studies on the causes and treatment of patients with HAE in Puerto Rico.
- The Puerto Rico Health Insurance Administration will include the condition of Hereditary Angioedema in the Government Health Plan and the Insurance Commissioner of Puerto Rico is ordered that every health insurance organization or private insurer in Puerto Rico must include the condition of Hereditary Angioedema in its coverage.

RUSSIA

From HAE Russia

Dear colleagues, we would like to remind you that we have launched a series of psychological training courses. Training in group 2 for parents of children with HAE took place on 12/02/2023 on the Google Meet platform. Topic of the training: "How a child's diagnosis affects child-parent relationships: features and risks".

On January 28, we invited children diagnosed with HAE to the next online meeting, held as part of the "Call a Friend" project. At this meeting, analytical psychologist and art therapist Yulia Faikova told children from different regions about the benefits of laughter.

The main topic of the January meeting was laughter and its impact on the human body. The ability to laugh is an important human quality. Laughing at failures helps you overcome them and not break down. Good laughter often helps establish friendly relationships between people.

Cheerful people are less likely to get sick and irritated, are less susceptible to depression, and live longer.

On February 2, the Interregional public organization "Society of Patients with Hereditary Angioedema" (MOO "OPNAO") held a webinar titled "Drugs: Ineffectiveness and Side Effects." The event participants included patients with HAE from different regions of the country.

Opening the webinar, Elena Viktorovna, Chairman of the Board of OPNAO, explained the topic chosen for the online meeting. In Russia today, many generics of the drug icatibant (trade name Firazyr) are registered, and feedback on the effectiveness and reactions to these generics is now being collected from members of the OPNAO. In January 2024, the organization conducted an electronic survey among patients with HAE. We presented the webinar results to the participants.

Legal consultant of the Federal State Budgetary Institution "NMITs DGOI named after. Dmitry Rogachev," Candidate of Legal Sciences, Associate Professor at the Higher School of Law of the Institute of State History and Public Administration of the Russian Academy of National Economy and Public Administration under the President of the Russian Federation Ilya Ushankov commented on the results of the survey and gave a presentation "Efficiency and Safety. Procedure for reporting side effects of drugs."

The lawyer presented the mechanism of action for a patient with HAE who encountered complications when using a drug (adverse reactions)

The final part of the meeting was traditionally held in a question-and-answer format.

At the end of the meeting, Elena Viktorovna noted that Russia occupies a leading position in the provision of medicines to orphan patients, but there are also problems that need to be solved together.

Chairman of the Municipal Public Organization "OPNAO" Bezboznaya E.V., representative of the Moscow Region branch of the organization Faykova Yu.S., and clinical psychologist of the Federal State Budgetary Institution National Medical Research Center of DGOI named after. Dmitry Rogachev - Shutkova E.S., on March 1 and 2, took part in the first doctor-patient conference "Rare people and rare diseases: know, treat and build the future", which was held at the Federal Children's Rehabilitation Center "Korablik" of the Russian Children's Clinical Hospital of the Ministry of Health Russia.

At the event, issues related not only to drug therapy for patients with orphan diseases were raised, but also issues of rehabilitation, social integration, stigmatization, legal aspects, and issues of psychological assistance to patients and their relatives were discussed.

SPAIN

From Sarah Smith, President, HAE Spain (AEDAF)

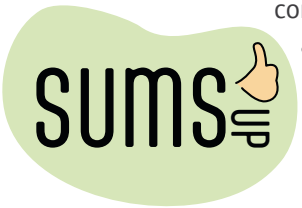
AEDAF: 26TH GENERAL ASSEMBLY & ANNUAL MEETING The 26th General Assembly and Annual Meeting of AEDAF will take place on Saturday, 13 Abril, in La Paz Hospital in Madrid. This year, we have elections for the members of the AEDAF Board and hope to get some of our younger members more actively involved in our activities and operations. We will also be presenting a summary of the online course "Expert Patient in HAE", which was launched in late 2023, and we will also be providing valuable information to our members about the 3 CSURs (Centers, Services and Units of Reference) and the ACARE centers in Spain.

COURSE FOR THE "EXPERT PATIENT IN HEREDITARY ANGIOEDEMA (HAE)" Since late 2023, the "Expert Patient in HAE" Course has been available at the following link: https://soyexpertoenalergia.es/p/angioedema-hereditario. This course is promoted by the "patient school" of the Spanish Society of Allergology and Clinical Immunology (SEAIC), with the participation of the Spanish HAE Association (AEDAF) and the collaboration of CSL Behring. This course, which is hosted on the patient training portal "I am an allergy expert" of the SEAIC, is free of charge with registration required, and at the end of the course, a certificate is automatically generated.

SCANDINAVIA: DENMARK, NORWAY, AND SWEDEN

From Jørn Schultz-Boysen, Vice President, HAE Scandinavia

We are thrilled to share some exciting news with you. Thanks to the hard work of an outstanding HAEi LEAP 23 student and the team at HAE Scandinavia, we have developed a new app that is all about supporting and connecting our HAE family. This app is a big win for us, bringing to life the fantastic work of our LEAP 23 participant and offering a valuable resource to our community.



Packed with useful information about HAE, inspiring patient stories, and more, this app is your go-to guide and reminder that you are never alone on this journey.

There is more good news! HAE Scandinavia has lined up three patient caregiver meetings in Denmark during the spring of 2024. These events are set to take place in Aarhus on 17 May, Odense on 29 May, and Copenhagen on 14 June. They are perfect opportunities for meaningful conversations, updates on HAE, and networking with both old and new friends.

And, like our friends worldwide, we are gearing up to celebrate the **hae day :-)** awareness campaign from 1 April until the end of May. We are eager to see all the engaging activities shared from across the globe.

Stay connected, and let us enjoy these moments together!

The Expert Patient in Hereditary Angioedema (HAE) course aims to raise awareness of this pathology, improve the diagnostic process, present the specific treatments available for patients, and offer resources to help patients manage their HAE in their day-to-day lives. It includes rigorous, current, and quality information with language that is generally accessible to the general public but with potential interest for clinicians.

On-demand training available anywhere. Available on the SEAIC online training platform and accessible on demand through audiovisual material created by healthcare professionals who are experts in pathology and by patients.

Multidisciplinary, accessible, current, and quality content. The content includes theoretical blocks, as well as round table discussions where knowledge and experiences are shared in first person. By taking this course, patients can become informed, expert patients with better tools and resources to manage their disease and improve their quality of life.

We hope this course will be of interest and benefit to all our Spanish-speaking friends in the HAE community!

SWITZERLAND

On Saturday, 20 April 2024, we will hold our annual patient meeting. After the welcome coffee, we will have:

- 5 HAE experts ready to answer questions and participate in a brief panel discussion
- HAE patients sharing experiences and offering valuable tips on medication use
- The board will also share many insights

After a joint lunch, we will enjoy an interesting airport tour. The day will conclude with a closing aperitif.

We want to extend a huge thank you to HAEi for expertly bringing everyone together for this incredible experience. The conference illustrates just how lucky HAE patients across the globe are to have a well-informed, engaged, and organized international organization.

TUNISIA

From HAE Tunisia (ANAOH)

These actions were carried out by ANAOH over the last four months:

- December 2023: Organization of a medical caravan to southern Tunisia (70 symptomatic patients, including 37 confirmed).
- January 27, 2024: A popularization session on angioedema during the National Stomatology Congress.
- February 2, 2024: Training on HAE for front-line doctors (northwest of Tunisia).
- February 14, 2024: The organization, in collaboration with the National Association of Young Internists, the 1st meeting on hereditary angioedema. - March 29, 2024: The Tunisian medical and associative experience was presented during the 17th national CREAK day in Grenoble.

UK

From Angela Metcalfe, CEO, HAE UK

My goodness, it has been a busy time for HAE UK over the past year or so.

I will start by saying how much hope there is now out there with a range of new drugs in clinical trials in the UK and beyond, and, of course, the much-anticipated gene therapy, which also has seen its first foray into England. The future is indeed looking very bright for new medications that will hopefully come on the market in the near future, some of which will transform patient lives and enable living virtually attack-free. Not that, as we know, being an HAE patient can stop you from doing anything if you put your mind to it!

Sadly, the wheels have turned rather more slowly with the launch of an interactive part of the UK NHS digital app program, but we are grateful indeed for the HAEi TrackR app that many patients in the UK are now using. We are also putting continued pressure on the NHS to approve an Emergency Card that would be recognized in Emergency Medicine in the UK. It is not an easy task, but we are hopeful that combining the process of getting this off the ground with many other of the 7000 rare diseases in the UK may eventually give traction.

Our Emergency Medicine programme also continues a pace, and we are shortly going to be filming some patient and clinician stories that will fulfil part of a PhD Doctorate programme that HAE UK are sponsoring. The more images and information we can get out there, the better.

We were busy too in February this year when, on the 29th, the UK marks Rare Disease Day. A reception was held at the UK Parliament at the House of Commons, where the Minister for Rare Diseases laid out his plan for the next couple of years to improve diagnosis and treatment for everyone living with a rare disease.

Also, on Rare Disease Day, we launched our new HAE UK website. We have been working hard over the past few months to update all the information and make it easier to navigate. One of our special features for the launch was a fantastic video from Dr Sorena Kiani, titled 'An overview of hereditary angioedema and how it is treated': https://www.youtube.com/watch?v=U-hHECn05Co.

We have also rewritten and updated a lot of our literature for patients, family members and clinicians alike, and will soon be sending a letter and information sheet to all Doctors in the UK and Northern Ireland. Again, the more HAE comes across a health care professional's desk, the better.

Last month, one of our trustees, June, arranged for a fantastic Rock Choir FlashMob in Canterbury, Kent. There were well over 100 Rock Choir singers, with many people stopping to watch the signing and listen to June's husband, Peter, give a brief talk about HAE: https://www.youtube.com/watch?v=hpOxmKyc804

Finally, but by no means least, we are currently organizing our 2024 Patient Day following our very successful one last year. Some 70 people – patients, carers, and clinicians – met in London, and we did a walk at lunchtime around one of London's splendid parks. We also had strong support at the event, with attendance from some of the pharmaceutical companies: they really appreciated being invited to hear firsthand about how HAE impacts patients' lives. We will share information about our 2024 patient day soon.

So, we continue to move onward and upward, raising awareness of Hereditary Angioedema, as whilst the future is looking bright for treatments, we need to continue to work hard to ensure that patients have all the right information in front of them to plan the best medical regime for every individual.

UKRAINE

From Natalia Formaniuk, President, HAE Ukraine

This year, the association started planning activities and events to spread even more information about HAE.

On February 15, a work meeting of experts on the diagnosis and therapy of hereditary angioedema was held in Lviv. Important issues related to improving treatment and prevention, holding webinars, and creating a national registry of HAE were discussed with leading experts from all regions of Ukraine. They agreed on the diagnosis of family members with HAE and new patients under the free program. The event was supported by Takeda LLC.

I am glad to share the news about the creation of an orphan center in Kyiv in a polyclinic, where patients of the Kyiv Medical Center are served. Many thanks to Eduard Krasyuk, our immunologist Tetyana Bondarenko, Olena Mykal, a member of the board of the HAE association, and the "RID" organization for organizing the admission of patients to this center.

On February 29, in Kyiv, I met with Lyudmila Zabrodska and Anastasia Bondarenko, members of the Medical Council of the HAE association, and met offline with the leaders of the public organization of Primary Immunodeficiencies "RID" at a conference dedicated to the Day of Rare Diseases. I told all the participants about the course and treatment of HAE in Ukraine. We agreed to cooperate in conducting educational events. We visited a modern plasma center and received a wonderful educational tour.

At the meeting of patients and doctors in May, we plan to continue the discussion of new ideas and hold an HAE school and educational training on self-administering drugs.

Join our HAE Ukraine page on social networks and follow updates.

USA

From US HAEA

The US HAEA Is Celebrating Its 25th Anniversary. Twenty-five years ago, a group of people who were diagnosed with an extremely rare and serious condition called HAE found each other online and decided that it was high time for bold action to address what they were all experiencing—pain, suffering, disability, and a tragically high death rate among affected family members. These HAE pioneers understood that fierce advocacy for better treatments was the only solution for avoiding frightening emergency room visits and having to rely on anabolic steroids as the only approved HAE treatment. Realizing that there is strength in numbers, these tenacious activists established the US HAE Association (HAEA) so that HAE sufferers, caregivers, and family members could band together and advocate for a better life.

Leadership of the newly formed Association went right to work, learning all they could from other successful patient groups. The early "lessons learned" informed an advocacy model consisting of three key components that are simple in concept but so effective that they continue delivering improved therapies and a better quality of life for the HAE community. The HAEA, first and foremost, strives to unite, motivate, and serve people with HAE, as well as their caregivers and families. Simultaneously, substantial effort is continuously put into identifying and nurturing relationships with compassionate specialist physicians willing to treat people with HAE and participate in clinical trials. The third component involves attracting pharmaceutical industry investment in new medicine development by making sure companies know that there is an organized HAE community, including experienced specialist physicians, who are organized and willing to enthusiastically participate in clinical research.

To celebrate the 25 years of the US HAEA, we have outlined 25 milestones accomplished by the HAEA. See what our community has accomplished, visit: https://www.haea.org/pages/p/HAEA_25th_Anniversary.

HAEA Community Blog. Since its launch in January 2022, the HAEA Community Blog has showcased the powerful stories of our fellow HAE community members. The HAEA community blog features personal narratives detailing the daily struggles of managing HAE and empowering accounts of resilience and advocacy. Through this platform, we celebrate the diversity and strength of the HAE community. The HAEA Community blog has empowered individuals with HAE, their caregivers, and other HAE advocates to navigate their HAE journey with confidence and resilience.

"My journey as a young person with HAE has taught me that people aren't judging you nearly as much as you think they are. I was afraid that people would look at me differently if they knew I had a disorder, but since becoming more open about it, I have found just the opposite to be true. Once I embraced my diagnosis, others did too, and a whole world of opportunity opened up for me." - Nathan, Individual with HAE, Article: HAE Didn't Hold Me Back from Seeing the World.

"My involvement with the HAEi LEAP program opened my eyes to the disparities in access to proper diagnoses and treatment in the international community. These experiences allowed me to see how advocating for HAE awareness and treatment can help improve access, regardless of location, and help reduce the stigma of rare disease to create a more supportive community for patients and their families and caregivers." - Jasmeen, Individual with HAE, Article: Finding my Confidence through Advocacy and Youth Leadership.

"I think it's incredibly important for any caregiver to have a voice, but when it comes to youth caregivers, and specifically siblings, it's huge. A lot of times, there's nobody closer to you in your childhood than your siblings. My family grew up incredibly close. My siblings are some of my best friends. And to see everything my brother had gone through with his journey of HAE, from a diagnosis to living alone on a college campus three hours away, is

an amazing progression. It shows what advocacy can do, what education can do, and what being part of a bigger community can do for the entire family." - Ally, Caregiver to her brother with HAE, Article: HAEA Youth Leadership Council Represents at The Rare Fair Youth Fest 2023

Explore the HAEA Community Blog and discover the heartwarming stories, invaluable insights, and empowering voices that unite us in the journey of living with HAE at https://www.haea.org/autoforms/f/210.

Engaging the US HAEA Community Through Meet and Greet Events. US HAEA Meet and Greet events unite people with HAE, their families, and caregivers. They serve as a safe space for anyone affected by HAE to ask questions and share their experiences with others who understand what they are going through. In addition, Meet and Greet attendees learn about the helpful tools and resources available to HAEA members.

In January, HAEA friends connected from the comfort of their homes through a virtual Zoom Meet and Greet. The HAEA hosted a February in-person Meet and Greet in sunny San Diego, California. Members of the HAEA who live in the surrounding area gathered to listen to an exciting presentation from Dr. Marc Reidl on developments in HAE research. We plan to bring HAEA community members together at two other in-person Meet and Greet events this spring in Cleveland, Ohio and Fairfax, Virginia.

Honoring Rare Disease Week at the RDLA Conference in Washington D.C. The US HAEA Advocacy Team and select members of the HAEA Grassroots Advocacy Network traveled to Washington D.C. to celebrate Rare Disease Day on February 29th, 2024. While on Capitol Hill, the HAEA participated in the Everylife RDLA conference where they learned about the legislative changes needed for all people affected by rare disease. This conference connected over 800 advocates for a variety of rare diseases to collaborate on mutually beneficial goals.

During Rare Disease Week, HAEA advocates spoke with congressional offices and urged lawmakers to co-sponsor bills to improve access to HAE treatments. HAEA advocates conducted more than 25 meetings with offices from 8 different states.

To learn more about the US HAEA Advocacy program, please visit our website, haea.org –> Get Involved –> Advocate: https://www.haea.org/autoforms/f/156

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 22 March 2024). You can find the abstracts via the search function at pubmed.ncbi.nlm.nih.gov

CRISPR-Cas9 In Vivo Gene Editing of KLKB1 for Hereditary Angioedema

Hilary J Longhurst, et al

The authors conclude that in this small study, a single dose of NTLA-2002 led to robust, dose-dependent, and durable reductions in total plasma kallikrein levels, and no severe adverse events were observed. In exploratory analyses, reductions in the number of angioedema attacks per month were observed at all dose levels.

(New England Journal of Medicine, 1 February 2024)

The US Hereditary Angioedema Association Scientific Registry: hereditary angioedema demographics, disease severity, and comorbidities

Sandra C Christiansen, et al

The authors developed a national patient-centric registry to improve understanding of the natural history and comorbidities of HAE and idiopathic nonhistaminergic angioedema (INHA). To date 485 HAE-C1INH and 26 HAE nl-C1NH have been enrolled. 36.9% of HAE-C1INH reported family members had died from HAE. HAE led to a significant increase in depression, sleep disorders, kidney disease, anemia and hepatitis. Co-existing cardiovascular problems were reduced in the HAE-C1INH group.

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

Increased thromboinflammatory load in hereditary angioedema

Olav Rogde Gramstad, et al

The authors investigated the thromboinflammatory load in people with HAE. They conclude that C1Inh deficiency, in HAE, is associated with an increased baseline thromboinflammatory load. These findings may reflect that HAE patients are in a subclinical attack state outside of clinically apparent oedema attacks.

(Clinical and Experimental Immunology, November 2023)

Contactless edema via plasmin

Sidney Strickland, Erin H Norris

The authors, writing a companion editorial to a paper by Dickeson et al (see below), conclude that genetic analysis has revolutionized our understanding of disease. However, in many cases, knowing the genetic mutation that causes a disease does not immediately reveal the mechanism. The article by Dickeson et al is an elegant example of how pursuing the biochemical mechanism based on a genetic mutation can provide insight into pathogenesis. The hope in general, and in this particular case, is that mechanistic understanding can lead to specific and effective therapeutic intervention in genetically defined patients.

(Blood, February 2024)

A mechanism for hereditary angioedema caused by a methionine-379-to-lysine substitution in kininogens

S Kent Dickesen, et al

Following the above editorial in Blood (see above), this article describes a mutation in the genes for a protein, that leads to HAE, and the impact this has on how the disease is caused.

(Blood, February 2024)

Hong Kong-Macau severe hives and angioedema referral pathway

Phillip H Li, et al

The authors describe a new pathway to improve knowledge, collaboration and regional exchange. The conclusion given is that the severe hives and angioedema referral pathway (SHARP) provides guidance for the management and specialist referral of patients with severe hives and angioedema in Hong Kong and Macau.

(Frontiers of Allergy, December 2023)

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

DG Ebo, et al

The authors set out to see how well Belgian clinical practice was aligned with the latest WHO/EAACI guidelines on the diagnosis and management of HAE. The authors conclude that five action points were identified and several other suggestions made to optimize Belgian clinical practice in C1-INH-HAE.

(Acta Clinica Belgica, December 2023)

COVID-19 infection and vaccination in patient with hereditary angioedema: a multicentric study

Inês Farinha, et al

The authors say that there has been concerns that SARS-CoV-2 infection may trigger HAE attacks or that COVID-19 may be worse in people with HAE. The authors conclude that, following their research amongst patients, that the severity of COVID-19 infection does not to be increased in HAE patients. They also indicate that patients with HAE can safely COVID-19 vaccination.

(European Annals of Allergy and Clinical Immunology, January 2024)

Clinical progress in hepatic targeting for novel prophylactic therapies in hereditary angioedema

Marc A Riedl, et al

This report reviews currently available data on hepatic-focused (targeted in the liver) interventions for HAE that have advanced into human trials. The paper considers: Donidalorsen, ADX-324, BMN-331 and NTLA-2002. The authors conclude that the findings from studies of these drugs, along with other ongoing studies, are highly anticipated with the expectation of expanding the array of treatment options in HAE.

(Journal of Allergy and Clinical Immunology: In Practice, April 2024)

Hereditary angioedema outcomes in US patients switched from injectable long-term prophylactic medication to oral berotralstat

Marc A Riedl, et al

The authors were investigating the potential of a new once-daily treatment for long-term prevention of HAE attacks, called berotralstat. They were looking at the safety, effectiveness and treatment satisfaction in patients who were switched from currently available injectable medication. The authors found that the transition from injection to berotralstat was generally well tolerated. Patients who switched maintained good control of their HAE symptoms and reported improved treatment satisfaction.

(Annals of Allergy, Asthma and Immunology, April 2024)

Plasma-derived C1 esterase inhibitor pharmacokinetics and safety in patients with hereditary angioedema

Inmaculada Martinez-Saguer, et al

The authors are investigating a new, stable, virus-inactivated, nanofiltrated concentrate of C1-INH, derived from human plasma. This study looked at how the medication acted in the body, and its safety profile, in people with HAE during an attack free period. The authors found that the pharmaco-kinetics (activity of drug in the human body) was consistent with those reported for other C1-INH concentrates. This new form of C1-INH was felt to have a favorable safety profile, and that further studies are needed to look into effectiveness and long-term safety.

(Journal of Allergy and Clinical Immunology Global, November 2023)

Oral FXIIa inhibitor KV998086 suppresses FXIIa and single chain FXII mediated kallikrein kinin system activation

Allen C Clermont et al

The kallikrein kinin system (KKS) in the body is an established target for the treatment and prevention of attacks in HAE. The authors found that small molecule FXIIa inhibitors, designed to target its active site, also inhibit the enzymatic activity of FXII zymogen. Combined inhibition of FXII zymogen and FXIIa may thereby suppress both the initiation and amplification of KKS activation that contribute to hereditary angioedema attacks and other FXII-mediated diseases.

(Frontiers of Pharmacology, December 2023)

Clinical evaluation of pediatric patients with hereditary angioedema

Ayşe Kırmızıtaş Aydoğdu and Gizem Ürel Demir

The authors wished to learn more about the clinical course of HAE in children. Commonly sites of edema at disease onset were the hand (32.3%), the feet (9.7%) and faces (25.7%. Abdominal attacks occurred in 32.3% of patients. The authors reported that a diagnosis is only made when the patients requests examination following recurrent angioedema. The conclude that as severe laryngeal attacks in patients without a diagnosis of HAE are fatal at a higher rate than attacks in patients with a diagnosis, awareness of symptoms and correct diagnosis are essential to proper treatment.

(Tohoku Journal of Experimental Medicine, January 2024)

Clinical characteristics and quality of life in a cohort of Polish pediatric patients with hereditary angioedema

Katarzyna Piotrowicz-Wójcik, et al

The authors describe the quality of life and management of HAE in a group of 21 Polish children. They conclude that the burden of HAE on the quality of life of children and their families has a widespread impact.

(Children (Basel), February 2024)

Patients outcomes associated with subcutaneous C1INH prophylaxis for hereditary angioedema: a retrospective analysis

William Lumry, et al

The authors conducted a real-world assessment of the impact of injectable C1 inhibitor prophylaxis of health outcomes and quality of life. They concluded that long-term prophylaxis with C1INH markedly improves to the goal of achieving total disease control and normalization of patient's lives, including fewer and less severe attacks.

(Journal of Allergy, Asthma and Clinical Immunology, December 2023)

Kallikrein inhibitors for angioedema: the progress of preclinical and early phase studies

Henriette Farkas and Zsuzsanna Balla

The authors give their expert opinion, that the targeted treatment of the dysregulated kinin-kallikrein system with specific inhibitors is promising for the prevention of angioedema attacks. They indicate that ongoing phase-III studies will provide further insight into safety and efficacy.

(Expert Opinion on Investigational Drugs, February 2024)

Hereditary angioedema with normal C1 inhibitor associated with carboxypeptidase N deficiency

Denis Vincent, et al

The authors aimed to look into the genes related to the kallikrein-kinin system in a small group of families with a deficiency of carboxypeptidase N (CPN). The authors found that gene variants related to this deficiency are related to HAE with normal C1 symptoms.

(Journal of Allergy and Clinical Immunology Global, February 2024)

Berotralstat for long-term prophylaxis of hereditary angioedema in Japan: Parts 2 and 3 of the randomized APeX-J Phase III trial

Daisuke Honda, et al

This study aimed to evaluate the safety and efficacy of a medicine for HAE, berotralstat, in Japanese people with HAE. The authors conclude that long-term prophylaxis with berotralstat raised no new safety signals and was effective at reducing attacks and improving patient-reported outcomes.

(World Allergy Organization Journal, March 2024)

The disease burden of hereditary angioedema: Insights from a survey in French-Canadians from Quebec

Jean-Nicolas Boursiquot, et al

The authors indicate that more data is needed on the clinical profile and disease burden of HAE amongst Canadians. The conclusion reached is that HAE manifests as frequent moderate-to-severe attacks and a high disease burden, with the HAE subtype affecting the care needed. The authors call for urgent efforts to increased awareness and education about HAE to treating physicians.

(Journal of Immunology Research, March 2024)

Prodromal symptoms of hereditary angioedema (HAE) attacks: A patient survey in UK and Spain

Patrick Yong and Mar Guilarte

The authors conducted a patient survey to better understand HAE prodromes and their role in diagnosis and treatment. The authors conclude that patients who experience prodromes can always or usually predict impending swelling. They also indicate that clearer communication to patients may help to encourage more patients to treat attacks early.

(Allergy, March 2024)

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 examined the ratio of fibrin and fibrinogen degradation product (FDP) to D-dimer in people suffering an acute HAE attacks, to see if it could help with accurate diagnosis. The authors conclude that is could help distinguish between an acute HAE attack and thrombotic conditions.

(Allergology International, January 2024)

Plasma microRNAs as biomarkers in hereditary angioedema

Timothy Craig, et al

The study tried to identify microRNAs which make play a role in HAE. The authors conclude that they found microRNAs that could distinguish patients with and without RNA, and that could be used to identify different types of HAE.

(Annals of Allergy, Asthma and Immunology, February 2024)

Screening for type II hereditary angioedema – the "poor man's C1-inhibitor function"

Ankur Kumar Kindal, et al

The study aimed to assess the diagnostic performance of elevated C1-INH levels in diagnosing type II HAE. The authors conclude that low C4 and elevated C1-INH levels may be considered a screening tool for type II HAE, especially in countries where C1-INH function testing is not readily available.

(Journal of Allergy and Clinical Immunology Global, October 2023)

Effect of lanadelumab on attack frequency and QoL in Japanese patients with hereditary angioedema: Report of five cases

Chika Hioki, et al

The authors examine patients taking lanadelumab to prevent HAE attacks. They found that all the Japanese patients that continued treatment showed quality of life and reduced attack frequency.

(Journal of Dermatology, January 2024)

Prophylaxis in hereditary angioedema: a United Kingdom Delphi consensus

Patrick Yong, et al

The authors sought to update the last UK consensus on treatment of HAE, from 2014. The authors conclude that UK access criteria to modern long-term prophylaxis, which is based on number of attacks is too simplistic. They also argue that patients should be seen in specialist centers and that remote monitoring was popular.

(Clinical and Experimental Immunology, March 2024)

The effect of estrogen-containing birth control pills on the constituents of bradykinin expression in plasma

Janette M Birmingham, et al

The authors investigated the link between estrogen and bradykinin, to what may exacerbate HAE with C1 inhibitor deficiency. They conclude that estrogen may make angioedema worsen by increasing the production of cleaved high-molecular weight kininogen and activated factor XII.

(Journal of Allergy and Clinical Immunology Global, February 2024)

Hereditary angioedema in older adults: Understanding the patient perspective

Alan P Baptist, et al

The authors evaluated the impact of HAE on older people by asking them about their lives. They found that older adults with HAE have specific challenges and concerns that may be unique compared to younger patients. These include worsening of attacks with age, and the effects of other conditions such as arthritis and memory loss and the menopause.

(Annals of Allergy, Asthma and Immunology, January 2024)

Berotralstat in hereditary angioedema due to C1 inhibitor deficiency: first real-world evidence from a Canadian center

Cindy Srinivasan, et al

The study looked at data investigating the effectiveness and safety of berotralstat. The authors conclude that berotralstat is an effective agent for long-term prophylaxis in HAE. Most patients experienced no adverse effects or mild, transient gastrointestinal symptoms.

(Frontiers of Immunology, January 2024)

The current situation of hereditary angioedema patients in Germany: results of an online survey

Markus Magerl, et al

This research was focused on asking patients about their current situation around disease. The results led the authors to conclude that despite the high level of satisfaction with current medication, 62% of patients expressed strong interest in oral long-term prophylaxis. It was felt that this was because the simplicity of and limited time involved in long-term prophylaxis would be beneficial to patients' quality of life.

(Frontiers of Medicine (Lausanne), January 2024)

A clinical evaluation of patients with known mutations (plasminogen and factor XII) with a focus on prophylactic treatment

Robin Lochbaum, et al

This study analyzed all patients with HAE with normal C1 being treatment at a clinic in southern Germany. The results indicated that the clinical course and symptoms of the patients treated with lanadelumab for long-term prophylaxis. The authors conclude that the therapeutic management of HAE type I and HAE type II should form the basis of treatment of HAE with normal C1, including prophylaxis.

(Journal of Dermatological Treatment, December 2024)

Patient-level indirect treatment comparison of lanadelumab versus pdC1-INH iv in hereditary angioedema patients: PATCH study

Markus Magerl, et al

The study compared, indirectly, the efficacy and safety of lanadelumab and plasma-derived C1-esterase inhibitor in patients with HAE on long-term prophylaxis. The authors conclude that there was a statistically significant reduction in HAE attacks with lanadelumab compared to plasma-derived C1-INH given intravenously.

(Frontiers of Medicine (Lausanne), January 2024)

Comorbidities in angioedema due to C1-inhibitor deficiency: An Italian survey

Andrea Zanichelli, et al

The research was conducted to investigate the incidence of comorbidities in patients with HAE using long-term prophylaxis with attenuated androgens or tranexamic acid. The authors conclude that there were more comorbidities than previously recorded in the literature, and an association between comorbidities and long term prophylaxis with attenuated androgens.

(Journal of Allergy and Clinical Immunology: In Practice, April 2024)

A phase 2 open-label extension study of prekallikrein inhibition with donidalorsen for hereditary angioedema

Remy S Petersen, et al

This study provided an interim analysis of an ongoing study investigating the use of an experimental medicine, donidalorsen, as long-term prophylaxis for HAE. The authors conclude that in this two year interim analysis, patients with HAE demonstrated no new safety signals, with donidalorsen being well-tolerated. The efficacy was durable with a 96% reduction in HAE attacks.

(Allergy, March 2024)

Efficacy outcomes in trials with prophylactic hereditary angioedema therapy: A systematic review

Remy S Petersen, et al

This study looked at the measures used to decide on the efficacy of HAE medicines in clinical trials. The authors conclude that time-normalized attack frequency was consistently chosen as the primary outcome across studies of HAE prophylaxis. They believe their research establishes a foundation for the selection of meaningful outcomes and optimal instruments in future studies assessing efficacy of HAE prophylaxis.

(Allergy, November 2023)

Once-daily oral berotralstat for long-term prophylaxis of hereditary angioedema: The open-label extension of the APeX-2 randomized trial

Sorena Kiani-Alikhan, et al

This study analysed the safety and efficacy of long-term prophylaxis with berotralstat. The authors conclude that the medicine was generally well-tolerated, provided rapid and sustained reductions in HAE attacks and improved quality of life over 96 weeks.

(Journal of Allergy and Clinical Immunology: In Practice, March 2024)

Hereditary angioedema: do patients have a specific 'digital footprint' in Danish registries?

Jakob Lillemoan Drivenes

This research considered if undiagnosed patients could be found by searching for familiar patterns in how these individuals accessed healthcare systems, in this case in Denmark. The author concluded that HAE patients had several hospital contacts due to swelling attacks prior to diagnosis. It was felt that hospitalized patients with angioedema of unknown cause should be screened routinely for HAE.

(Acta Dermatovenerologica APA, January 2024)

Hereditary or acquired? Comprehensive genetic testing assists in stratifying angioedema patients

Marija Rozevska, et al

This study examines the genetic basis of HAE, particularly unresolved cases and those with normal levels of C1-inhibitor. The authors conclude that advanced genetic sequencing techniques are important, and having an integrated diagnostic strategy can unravel the complexities of HAE, especially cases with normal C1-inhibitor.

(Journal of Clinical Medicine, November 2023)

A cross-sectional study of quality of life in patients enrolled in the Romanian hereditary angioedema registry

Valentin Nadasan, et al

This research investigated the quality of life of people with HAE from Romania. The authors found that, despite the availability of on-demand treatment for all patients, there is a need for other diagnostic and therapeutic interventions to improve the management of the disease and the quality of life for HAE patients from Romania.

(Cureus, January 2024)

Real-life experience of subcutaneous (SC) plasma derived C1-inhibitor (pdC1INH) as long-term prophylaxis (LTP) in HAE-C1INH

A Entrala, et al

This study followed a small group of patients from Spain who received long-term prophylaxis with injectable pdC1INH for at least six months. The authors conclude that using a specific protocol designed by a group of specialist Spain doctors was useful in individualizing treatment for the treated patients.

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

Complex analysis of the national hereditary angioedema cohort in Slovakia – Identification of 12 novel variants of the SERPING1 gene

Adam Markocsy, et al

This study presented the first genetic analysis of patients with HAE in Slovakia. The authors identified 12 previously un-published genetic variants of the SERPING1 gene. Their conclusion is that their findings expands the knowledge of HAE due to C1-inihibitor deficiency.

(World Allergy Organization Journal, March 2024)

Real-world reporting rates of administration-site reactions with on-demand treatment of hereditary angioedema attacks

Raffi Tachdjian, et al

This research aimed to characterize the burden of treatment associated with existing on-demand treatments for HAE. The authors conclude that their findings show that rates of side effects in the real-world are consistent with that seen in clinical trials, and suggest that patients experience substantial treatment burden when using currently approved on-demand therapies for HAE attacks.

(Allergy and Asthma Proceedings, January 2024)

What lessons are learned? Our changing practice during 30 years of hereditary angioedema treatment

Sevdenur Keskin et al

The authors reviewed the 33 patients with various degrees of documented C1-inhibitor deficiency leading to HAE. They provide some lessons learned in treating these patients over a 30 year period. These include the knowledge gap surrounding the disease, leading to delayed treatment. Additionally, they argue that C4 level may not be the best screening tool for HAE. They believe that although useful, it can be normal in patients with mild disease, so should not be relied on entirely to rule out a diagnosis of HAE. In general, a third of patients chose to use as-needed treatment only, rather than long-term prophylaxis.

(Annals of Allergy, Asthma and Immunology, February 2024)

An expert panel's review on patients with hereditary angioedema switching from attenuated androgens to oral prophylactic therapy

William Lumry, et al

This study aimed to share expert insights and patient feedback on transitioning from attenuated androgens to berotralstat as an alternative oral prophylactic therapy. The authors conlude that the emergence of new highly specific drugs for long-term prophylaxis for HAE call for recommendations and guidelines on transitioning onto these drugs from attenuated androgens.

(Allergy and Asthma Proceedings, January 2024)

Transmission patterns of C1-INH deficiency hereditary angioedema favors a wild-type male offspring: Our experience in Chandigarh, India

Sanghamitra Machhua, et al

This research looked into the risk of transmission of SERPING1 gene variants from father or mother to their offspring. The researchers conclude that a normal version of the SERPING1 gene is more commonly transmitted to male offspring, especially when the father is the carrier of the mutated gene, hence, overall, fewer males and more female offspring inherited the variant.

(Immunobiology, March 2024)

Clinical experience with berotralstat in patients with hereditary angioedema with normal C1-esterase inhibitor: A commented case series

Henry Janarek and Drew Austin Saville Mutschelknaus

This study looked at the efficacy of the oral medicine berotralstat in treating HAE with normal C1. The authors conclude that berotralstat was an effective prophylactic treatment option in most patients with HAE with normal C1. However, they caution that treatment outcomes varied considerably between patients, highlighting the importance of a personalized approach to disease management.

(Journal of Asthma and Allergy, February 2024)

Delay in diagnosis is the most important proximate reason for mortality in hereditary angioedema: our experience at Chandigarh, India

Ankur Kumar Kindal, et al

This research was to report factors associated with the death of patients with HAE. The authors reviewed the health records of 65 families, concluding that delay to diagnosis was the most important reason for death in this group.

(Clinical and Experimental Dermatology, April 2024)

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 World Health Organization (WHO) the following trials are currently or soon to be recruiting (as start of April 2024).

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

Belgium
Bulgaria
Canada
France
Germany
Israel
Italy
Netherlands
Poland
Puerto Rico
Spain
Turkey
United Kingdom
United States

https://clinicaltrials.gov/study/NCT05392114

A Study With Lanadelumab in Persons With Hereditary Angioedema (HAE) in Poland

Poland

https://clinicaltrials.gov/study/NCT05147181

A Gene Therapy Study of BMN 331 in Subjects With Hereditary Angioedema

Spain
United States

https://clinicaltrials.gov/study/NCT05121376

A Study of Lanadelumab in Teenagers and Adults With Hereditary Angioedema (HAE)

United Kingdom

https://clinicaltrials.gov/study/NCT05469789

A Study in Adults With Hereditary Angioedema (HAE) Who Currently Receive Icatibant at Home

United Kingdom

https://clinicaltrials.gov/study/NCT05489640

A Survey of Lanadelumab in Participants With Hereditary Angioedema

Japan

https://clinicaltrials.gov/study/NCT05397431

A Survey of Icatibant in Pediatric Participants With Hereditary Angioedema

Japan

https://clinicaltrials.gov/study/NCT05509569

A Study of STAR-0215 in Participants With Hereditary Angioedema

Canada
United States

https://clinicaltrials.gov/study/NCT05695248

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

Australia
Austria
Bulgaria
France
Germany
Greece
Israel
Japan
Netherlands
New Zealand
Spain
United Kingdom
United States

https://clinicaltrials.gov/study/NCT05511922

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

Israel
United States

https://clinicaltrials.gov/study/NCT05819775

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

Canada

https://clinicaltrials.gov/study/NCT05578417

Firazyr Patient Registry (Icatibant Outcome Survey - IOS)

Australia
Austria
Brazil
Czechia
Denmark
France
Germany
Greece
Israel
Italy
Spain
Sweden
United Kingdom

https://clinicaltrials.gov/study/NCT01034969

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 Hereditary Angioedema (HAE)

Australia
Austria
Bulgaria
France
Germany
Greece
Hungary
Israel
Italy
Japan
Netherlands
New Zealand
North Macedonia
Poland
Portugal
Slovakia
South Africa
Spain
United Kingdom
United States

https://clinicaltrials.gov/study/NCT05505916

Extension Study of Oral PHA-022121 for Acute Treatment of Angioedema Attacks in Patients With Hereditary Angioedema

Bulgaria
Canada
Czechia
France
Germany
Hungary
Israel
Poland
Spain
United States

https://clinicaltrials.gov/study/NCT05396105

Status of Dental Care Practices in Patients With Hereditary Angioedema

France

https://clinicaltrials.gov/study/NCT05776784

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

Australia

https://clinicaltrials.gov/study/NCT05691361

Identification and Characterization of Genetic Variants in Hereditary Angioedema

Spain

https://clinicaltrials.gov/study/NCT05833620

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

Bulgaria, Croatia

https://www.clinicaltrials.gov/study/NCT01397864

A Study of Lanadelumab (Takhzyro) and Icatibant (Firazyr) in Persons with HAE in China

China
https://www.clinicaltrials.gov/study/NCT06346899

Study of Oral Deucrictibant Soft Capsule for On-Demand Treatment of Angioedema Attacks in Adolescents and Adults with Hereditary Angioedema (RAPIDe-3)

United States

https://www.clinicaltrials.gov/study/NCT06343779

Long-Term Follow-Up (LTFU) of Subjects Treated With NTLA 2002

France
New Zealand
United Kingdom

https://www.clinicaltrials.gov/study/NCT06262399

Angioedema Biomarker Research Study

United States

https://www.clinicaltrials.gov/study/NCT06210698

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

14 December 2023

CSL's Garadacimab feceives FDA and EMA filing acceptance

CSL today announced the US Food and Drug Administration (FDA) has accepted the company's Biologics License Application (BLA) for garadacimab (CSL312) as a once-monthly prophylactic treatment for hereditary angioedema (HAE). The company also announced the European Medicines Agency (EMA) has accepted the submission for CSL's Marketing Authorization Application (MAA) for garadacimab. If approved, garadacimab would become the first treatment for HAE in the U.S. and EU to target activated Factor XII (FXIIa).

Garadacimab is a novel, first-in-class, recombinant monoclonal antibody targeting activated FXII. FXIIa is a plasma protein that initiates the kallikrein-kinin cascade of HAE attacks. By targeting FXIIa, garadacimab inhibits this cascade at the top as compared to HAE therapies that target downstream mediators.

Emmanuelle Lecomte Brisset, Pharm D, Senior Vice President and Global Head of Regulatory Affairs at CSL, said: "CSL is a company with a deep heritage in developing innovative treatments for the rare disease community, and we are extremely proud that our first homegrown recombinant monoclonal antibody is progressing our commitment to support HAE patients in need. We believe that garadacimab has the potential to become a promising therapy in the prevention of HAE attacks and we look forward to working closely with global health regulators throughout the review process."

Orphan-drug designation for garadacimab as a therapy for hereditary angioedema has been granted by both the FDA and the EMA.

(Source: CSL)

CSL Behring

18 December 2023

Ionis announces European licensing agreement with Otsuka for donidalorsen in hereditary angioedema

Ionis Pharmaceuticals Inc. today announced that it has entered into a license agreement with Otsuka, under which Otsuka obtains exclusive rights in Europe to commercialize donidalorsen, which is an investigational prophylactic treatment for hereditary angioedema (HAE).

Ionis will maintain responsibility for the non-clinical and clinical development of donidalorsen, and Otsuka will be responsible for European regulatory filings and commercialization.

Ionis plans to independently launch donidalorsen in the U.S. if approved, as part of the company's strategy to deliver a steady flow of wholly owned medicines to patients.

Brett P. Monia, PhD, Ionis' Chief Executive Officer, said: "We are excited to collaborate with Otsuka given their proven results in bringing rare disease medicines to patients in Europe. This agreement is aligned with our strategy to initially focus our commercialization efforts on the U.S. market. We are encouraged by the strong product profile of donidalorsen to date and look forward to reporting pivotal topline Phase 3 donidalorsen results in HAE in the first half of next year."

(Source: Ionis)

IONIS

19 December 2023

BioCryst announces publication of data from open-label extension of the APEX-2 pivotal trial of Orladeyo (berotralstat)

Biocryst Pharmaceuticals Inc. today announced that data from the open-label extension (OLE) of the APeX-2 trial of oral, once-daily, Orladeyo for the prophylactic treatment of hereditary angioedema (HAE) in patients 12 years and older have been published online by the Journal of *Allergy and Clinical Immunology: In Practice (JACI: In Practice)*.

William R. Lumry, MD, Clinical Professor of Internal Medicine at the University of Texas Southwestern Medical School, said: "As detailed in this manuscript, long-term prophylaxis with ORLADEYO has enabled patients with HAE to better manage their condition, including reducing the number of HAE attacks they experience and demonstrating clinically meaningful improvement in their quality of life. I have seen first-hand in my practice evidence that the longer patients remain on ORLADEYO, the better their outcomes are."

Dr Ryan Arnold, Chief Medical Officer of BioCryst, said: "We are pleased to share long-term efficacy and safety data from APeX-2 as published in JACI: In Practice. These data further illustrate the potential lasting outcomes that can be appreciated by patients who are treated with oral, once-daily ORLADEYO. We continue to see long-term safety and effectiveness data that reinforce ORLADEYO as an important treatment option for patients with HAE, and we look forward to sharing additional real-world evidence at upcoming medical congresses."

(Source: BioCryst)

20 December 2023

KalVista Pharmaceuticals announces publication of first oral Factor XIIa data in Frontiers in Pharmacology

KalVista Pharmaceuticals announced that data from the first report of an oral, potent, and selective FXIIa inhibitor has been published in the scientific journal, Frontiers in Pharmacology.

The article describes the pharmacology of a representative compound from KalVista's portfolio of structurally diverse, oral Factor XII inhibitors, including:

- Potent, selective inhibition of both FXII zymogen and FXIIa, thereby suppressing both the initiation and amplification of kallikrein-kinin system (KKS) activation
- Blockage of KKS-mediated edema in preclinical HAE models in vivo

Andrew Crockett, Chief Executive Officer of KalVista, said: "The publication is the first report of a potent and specific FXIIa inhibitor with high oral availability in multiple species and demonstrated efficacy in HAE models in vivo. This once again demonstrates our scientific leadership in medicinal chemistry and KKS biology. It also provides new insight on the inhibition of FXII zymogen."

(Source: KalVista)

4 January 2024

Intellia Therapeutics highlights its anticipated 2024 key milestones

Intellia Therapeutics today announced its strategic priorities through 2026 and key anticipated 2024 milestones that support the company's mission to transform the lives of patients and bring forth a new era in medicine.

Intellia anticipates initiating the global pivotal Phase 3 trial of NTLA-2002 in HAE in the second half of 2024, subject to regulatory feedback. The company also expects to present updated data from Phase 1 and new data from Phase 2 portion in 2024.

NTLA-2002 is an in vivo CRISPR-based investigational therapy designed to prevent potentially life-threatening swelling attacks in people with HAE.

John Leonard MD, Intellia President and Chief Executive Officer, said: "Looking ahead, we will continue to turn ground-breaking science into real-world medicines by capitalizing on our extensive experience and capabilities to edit disease-causing genes. We are rapidly expanding the potential to treat an even larger range of diseases, including those that originate outside of the liver, by deploying our novel delivery and editing technologies. With our comprehensive gene editing toolbox, we are well-positioned to harness the full potential of CRISPR-based medicines for patients."

(Source: Intellia)

5 January 2024

Pharvaris provides business update and outlines 2024 strategic priorities

Pharvaris provided business updates and outlined its strategic priorities for 2024.

Berndt Modig, Chief Executive Officer of Pharvaris, said: "Pharvaris enters the new year having demonstrated deucrictibant's potential to be the preferred option for both the prevention and treatment of HAE attacks." He continued: "We are operating from a strong financial position and anticipate 2024 will be an important execution year for Pharvaris as we transition into a late-stage clinical company with the initiation of RAPIDe-3 expected within the first half. We have submitted the results of the nonclinical study to the FDA for review with respect to the clinical hold on the long-term prophylaxis program in the U.S. We are also preparing to initiate the global pivotal study, CHAPTER-3, for the prophylaxis against HAE attacks. In parallel, we will be building on our foundation for Pharvaris' long-term strategy as we invest in our commercial and product infrastructure to support our commitment to provide deucrictibant to people living with HAE."

Jochen Knolle, PhD, co-founder of Pharvaris, transitions to strategic advisor to the CEO and Executive Committee commented: "It has been a privilege working with the HAE community throughout my career. The approval of icatibant as an acute treatment for HAE attacks shifted the paradigm of treatment for people living with HAE, and I anticipate that deucrictibant will have an equally important impact on the HAE community. I look forward to continuing to contribute to the strategic transformation of Pharvaris in my new capacity."

Business Updates and Company Highlights
Pipeline

- Anticipated initiation of RAPIDe-3 within first half of 2024. RAPIDe-3 is a randomized, double-blind, placebo-controlled, cross-over Phase 3 study designed to evaluate the efficacy and safety of oral deucrictibant immediate-release capsules (PHVS416) for the on-demand treatment of HAE attacks. During the treatment phase, participants will self-administer double-blinded study drug (20 mg deucrictibant immediate-release capsule or placebo, in a crossover fashion) to treat a total of two qualifying attacks. The primary endpoint is time to onset of symptom relief, defined as a Patient Global Impression of Change (PGI-C) rating of at least "a little better" for two consecutive timepoints within 12 hours post-treatment. Secondary endpoints include assessments of time to end of progression of attack symptoms, substantial symptom relief, and symptom resolution, as defined by PGI-C, Patient Global Impression of Severity (PGI-S) and Angioedema syMptom Rating scAle (AMRA), as well as use of rescue medication. Data from a real-world study in HAE with standard-of-care treatments suggest the median time to symptom relief is similar when measured by AMRA-3 ⩾20% reduction from pre-treatment and with PGI-C "a little better" on two consecutive timepoints. Safety outcome measures include incidence of treatment-emergent adverse events. After RAPIDe-3 completion, participants may continue treatment with deucrictibant in an open-label extension study. In the RAPIDe-1 Phase 2 study, deucrictibant significantly reduced the time to onset of symptom relief and to resolution of HAE attacks, reduced use of rescue medication, and was well-tolerated.
- Submission of non-clinical rodent toxicology data to the U.S. Food & Drug Administration (FDA) completed. Pharvaris has submitted the results from the 26-week rodent toxicology study to the FDA. The study was intended to provide additional data to address the clinical hold on the IND of deucrictibant for long-term prophylaxis, and Pharvaris believes the study met its objective. Neither the nature nor timing of the response from FDA is certain.
- Phase 2 CHAPTER-1 clinical study met its primary endpoint. The primary endpoint of the CHAPTER-1 study measured the time-normalized number of investigator-confirmed HAE attacks during the treatment period. The monthly attack rate was reduced by 84.5% (p=0.0008) compared to placebo in participants who received 40 mg/day of deucrictibant. In the analysis of the secondary endpoints, deucrictibant demonstrated clinically meaningful reductions in the occurrence of moderate and severe attacks and in the number of attacks treated with on-demand medication. Participants on deucrictibant treatment experienced a meaningful improvement in their quality of life as measured by patient global assessment of change (PGA-Change) and angioedema quality of life (AE-QoL) questionnaires. Throughout 12 weeks of treatment in CHAPTER-1, both the 20 mg/day and the 40 mg/day doses of deucrictibant were well-tolerated. The open-label portion of the CHAPTER-1 study is ongoing. Pharvaris is preparing to initiate CHAPTER-3, a global, pivotal study to evaluate deucrictibant for the prophylactic treatment of HAE attacks.

(Source: Pharvaris)

PHARVARIS

22 January 2024

Pharvaris announces FDA lifting of the clinical hold of deucrictibant for the prophylactic treatment of HAE attacks

Pharvaris today announces that the US Food and Drug Administration (FDA) has lifted the clinical hold on the Investigational New Drug (IND) application for deucrictibant for the prophylactic treatment of HAE attacks, following review of data from a 26-week rodent toxicology study.

Berndt Modig, Chief Executive Officer of Pharvaris, said: "The lift of the clinical hold in the US enables us to progress the global development of deucrictibant for long-term prophylaxis, including resuming the open-label portion of CHAPTER-1, our Phase 2 proof-of-concept study of deucrictibant for the prevention of HAE attacks, in the US.

"We are pleased to have worked collaboratively with the FDA to address the requests of the agency with the submission of additional nonclinical data, and we appreciate the agency's comments and recommendations regarding study conduct. We will request an End-of-Phase 2 meeting with the FDA to align on key elements of CHAPTER-3, the anticipated global Phase 3 study of deucrictibant extended-release tablets (PHVS719) for the prophylactic treatment of HAE attacks."

In August 2022, the FDA placed clinical studies of deucrictibant, including CHAPTER-1, on hold. Pharvaris notified ex-U.S. country-specific regulatory authorities of the clinical hold in the US, and the regulatory status of deucrictibant outside the US was not affected. In June 2023, Pharvaris announced the FDA's removal of the clinical hold of deucrictibant for the on-demand treatment of HAE in the US following FDA review of data from a pre-planned interim analysis of a 26-week rodent toxicology study. In December 2023, Pharvaris announced positive top-line clinical data from the Phase 2 CHAPTER-1 study of deucrictibant for the prophylactic treatment of HAE attacks.

(Source: Pharvaris)

PHARVARIS

22 January 2024

Ionis announces positive topline results from Phase 3 OASIS-HAE study of investigational donidalorsen, and prepares submissions to US FDA and European Medicines Agency

Ionis Pharmaceuticals Inc. announced positive topline results for the Phase 3 OASIS-HAE study of donidalorsen in people with hereditary angioedema (HAE). The trial met its primary endpoint of reduction in rate of angioedema attacks in patients treated with donidalorsen (80mg) via subcutaneous injection dosed every 4 weeks (Q4W) ($p<0.001$) or every 8 weeks (Q8W) ($p=0.004$), compared to placebo. In addition, the trial showed donidalorsen achieved statistical significance on all secondary endpoints in the Q4W group and key secondary endpoints in the Q8W group. Donidalorsen demonstrated a favorable safety and tolerability profile in the study, and there were no serious adverse events in the patients treated with donidalorsen.

Based on these data, Ionis is preparing to submit a New Drug Application with the US Food and Drug Administration. Otsuka, which has exclusive rights to commercialize donidalorsen in Europe, is preparing to submit a Marketing Authorization Application to the European Medicines Agency. Donidalorsen received Orphan Drug Designation in the US, and the Orphan Drug Designation procedure in the EU is ongoing.

Kenneth Newman, MD, Senior Vice President, Head of Clinical Development at Ionis, said: "We are very pleased with the positive topline results from the Phase 3 OASIS-HAE study of donidalorsen. Based on these results and the durable efficacy and favorable safety data seen in the ongoing Phase 2 open-label extension study, we believe donidalorsen, if approved, could be an attractive new treatment option for patients with HAE, many of whom continue to experience unpredictable, painful and severe breakthrough attacks despite currently available prophylactic treatments. We are grateful to the patients, caregivers, investigators and study teams who participated in the OASIS-HAE study."

Ionis plans to present the Phase 3 OASIS-HAE results at an upcoming medical congress by mid-year. Ionis also plans to share results from the Phase 3 OASIS-Plus study by mid-year, which includes both the open-label extension of the Phase 3 trial and a separate cohort of patients who have transitioned to donidalorsen from another prophylactic HAE medication (switch cohort).

(Source: Ionis)

IONIS

31 January 2024

Intellia Therapeutics announces publication in the New England Journal of Medicine of positive interim phase 1 data for NTLA-2002 in patients with hereditary angioedema

Intellia Therapeutics announced that interim results from the Phase 1 portion of the Phase 1/2 study of NTLA-2002 were published online in the New England Journal of Medicine (NEJM).

The reported data showed that a single dose of NTLA-2002 led to a 95% mean reduction in monthly HAE attack rate across all 10 patients in the Phase 1 portion. Nine out of 10 patients remained completely attack-free following the 16-week primary observation period through the latest follow-up. Further, all patients who discontinued concomitant long-term HAE prophylaxis treatment after NTLA-2002 administration (n=6) have reported no HAE attacks since discontinuation. NTLA-2002 has been well tolerated at all dose levels. The most frequent adverse events reported were mild, transient infusion-related reactions and fatigue.

Intellia anticipates initiating the global pivotal Phase 3 trial of NTLA-2002 in HAE in the second half of 2024, subject to regulatory feedback. The company also expects to present updated data from Phase 1 and new data from Phase 2 portion in 2024.

NTLA-2002 is an investigational in vivo CRISPR-based gene editing therapy in development as a single-dose treatment for hereditary angioedema (HAE), a rare genetic condition that leads to potentially life-threatening swelling attacks.

John Leonard MD, Intellia President and Chief Executive Officer, said: "The interim NTLA-2002 clinical data published suggest that a single dose of NTLA-2002 may eliminate angioedema attacks for people suffering from hereditary angioedema. We are highly encouraged by these data and look forward to presenting extended follow-up from the Phase 1 and results from the Phase 2 portion later this year. Additionally, we remain on track to initiate a global pivotal study for NTLA-2002 in the second half of 2024, subject to regulatory feedback."

(Source: Intellia)

5 February 2024

BioCryst to present new Orladeyo (berotralstat) real-world data at 2024 American Academy of Allergy, Asthma & Immunology annual meeting

BioCryst Pharmaceuticals Inc. today announced that the company will present five abstracts featuring new analyses of real-world use of oral, once-daily Orladeyo (berotralstat) for the prophylactic treatment of hereditary angioedema (HAE) in patients 12 years and older at the 2024 American Academy of Allergy, Asthma & Immunology (AAAAI) annual meeting.

The meeting will take place at the Walter E. Washington Convention Center in Washington, DC, from 23-26 February 2024. The five abstracts that BioCryst will present are:

- ***Berotralstat Prophylaxis Reduces HAE Attack Rates Regardless of Baseline Attacks: Real-World Outcomes***; Poster #012; Friday 23rd February, 3:15-4:15 p.m. ET; Convention Center, Level 2, Hall D
- ***Consistently Low Hereditary Angioedema Attack Rates with Berotralstat Regardless of Prior Prophylaxis: Real-World Outcomes***; Poster #008; Friday, 23rd February, 3:15-4:15 p.m. ET; Convention Center, Level 2, Hall D
- ***Assessment of the Tolerability and Effectiveness of Berotralstat for Long-term Prophylaxis in Hereditary Angioedema***: Berolife Study Interim Analysis; Poster #028; Friday 23rd February, 3:15-4:15 p.m. ET; Convention Center, Level 2, Hall D
- ***Evaluation of Adherence to Berotralstat in Patients with Hereditary Angioedema: A Prospective Survey in Community Pharmacies***; Poster #023; Friday 23rd February, 3:15-4:15 p.m. ET; Convention Center, Level 2, Hall D
- ***Real-World Effectiveness of Berotralstat in HAE With and Without C1-Inhibitor Deficiency***; Poster #281; Saturday 24th February, 9:45-10:45 a.m. ET; Convention Center, Level 2, Hall D

(Source: BioCryst)

13 February 2024

KalVista Pharmaceuticals reports phase-3 KONFIDENT trial meets all endpoints for sebetralstat as first oral on-demand therapy for hereditary angioedema

KalVista Pharmaceuticals today announced positive results from the phase 3 KONFIDENT clinical trial demonstrating statistically and clinically significant efficacy of sebetralstat as oral on-demand therapy for hereditary angioedema (HAE). KONFIDENT was the largest and most representative trial ever conducted in HAE, and included adolescents, patients using long-term prophylaxis, and all attack severities and locations.

The clinical trial met all primary and key secondary endpoints and demonstrated a favorable safety profile. HAE attacks treated with both 300 mg and 600 mg of sebetralstat achieved the primary endpoint of beginning of symptom relief significantly faster than placebo ($p<0.0001$ for 300 mg, $p=0.0013$ for 600 mg). The median time to beginning of symptom relief was 1.61 hours with sebetralstat 300 mg (CI 1.28, 2.27), 1.79 hours with sebetralstat 600 mg (CI 1.33, 2.27) and 6.72 hours with placebo (CI 2.33, >12).

Consistent with previous studies, sebetralstat was well-tolerated, with a safety profile similar to placebo. There were no patient withdrawals due to any adverse event and no treatment-related serious adverse events (SAEs) were observed. Treatment-related adverse event rates were 2.3% for 300 mg sebetralstat, 2.2% for 600 mg sebetralstat, and 4.8% for placebo.

Andrew Crockett, Chief Executive Officer of KalVista, said: "We are thrilled to announce positive phase 3 results for the KONFIDENT trial, which we believe position sebetralstat to become the first oral, on-demand therapy for the treatment of HAE. These clinically meaningful results represent a potentially significant advance for people living with HAE. If approved, sebetralstat may offer a compelling treatment option for patients and their caregivers given the long-standing preference for an effective and safe oral therapy that provides rapid symptom relief for HAE attacks.

"Most importantly, we want to thank the people living with HAE, their families, and the investigator teams around the world who supported KONFIDENT and made it the largest clinical trial ever conducted in HAE. We look forward to submitting a new drug application for sebetralstat to the U.S. FDA in the first half of 2024 and in the EU and Japan later this year."

The Company plans to present phase 3 data for the KONFIDENT trial at the annual meeting of the American Academy of Allergy Asthma and Immunology (AAAAI) on 25 February 2024.

(Source: KalVista)

16 February 2024

KalVista Pharmaceuticals to present phase-3 sebetralstat data at the 2024 American Academy of Allergy, Asthma & Immunology Annual Meeting

KalVista Pharmaceuticals today announced the acceptance of multiple abstracts at the 2024 American Academy of Allergy, Asthma & Immunology (AAAAI) Annual Meeting taking place in Washington, DC from 23-26 February 2024.

The following presentations will take place on Saturday 24 February 2024 from 9:45-10:45am Eastern Time (ET) in the Convention Center, Level 2, Hall D.

- Characteristics of Hereditary Angioedema Attacks Among Long-Term Prophylaxis Users (#215): Bob Geng, Vibha Desai, Julie Ulloa, Sherry Danese, Shawn Czado, Paul K. Audhya, Timothy Craig. Results shared as an oral poster presentation and Q&A
- Delayed On-demand Treatment of Hereditary Angioedema Attacks: Patient Perceptions and Associated Barriers (#216): Sandra Christiansen, Maeve O'Connor, Julie Ulloa, Sherry Danese, Vibha Desai, Shawn Czado, Paul Audhya, Paula Busse. Results shared as an oral poster presentation and Q&A Anxiety
- Associated with On-Demand Treatment for Hereditary Angioedema (HAE) Attacks (#257): James Wedner, Cristine Radojicic, Julie Ulloa, Sherry Danese, Vibha Desai, Shawn Czado, Paul Audhya, Sandra Christiansen. Results shared as an oral poster presentation and Q&A
- Anxiety Associated with Refilling On-demand Therapy for HAE Attacks Contributes to Treatment Delay and Non-Treatment (#258): Daniel F. Soteres, Anete S. Grumach, Sally van Kooten, Neil Malloy, Markus Heckmann, Julie Ulloa, Autumn Burnette. Results shared as an oral poster presentation and Q&A

- Characterizing the Negative Impact of Delayed On-Demand Treatment of HAE Attacks (#260): Princess Ogbogu, Hilary Longhurst, Sally van Kooten, Neil Malloy, Markus Heckmann, Julie Ulloa, Ricardo Zwiener. Results shared as an oral poster presentation and Q&A
- The Impact of On-demand Treatment on Quality of Life of People with HAE (#265): Paula Busse, Bob Geng, Sally van Kooten, Neil Malloy, Markus Heckmann, Julie Ulloa, Douglas H. Jones. Results shared as an oral poster presentation and Q&A
- Characterizing the Perspective of Patients with HAE on Prophylactic Treatment (#270): Stephen Betschel, Cristine Radojicic, Sally van Kooten, Neil Malloy, Markus Heckmann, Julie Ulloa, William Lumry. Results shared as an oral poster presentation and Q&A
- Treatment Patterns of Patients Requiring Redosing of an On-demand Treatment After the Return of an HAE Attack (#284): Constance Katelaris, Michael Manning, Sally van Kooten, Neil Malloy, Markus Heckmann, Julie Ulloa, William Lumry. Results shared as an oral poster presentation and Q&A

The following late-breaking presentation will take place on Sunday 25 February 2024 from 9:45-10:45am ET in the Convention Center, Level 2, Hall D.

- Sebetralstat for On-demand Treatment of Hereditary Angioedema Attacks: Results of the Double-blind, Placebocontrolled Phase 3 KONFIDENT Trial (L45): Marc Riedl, Emel Aygören-Pürsün, William Lumry, Henriette Farkas, Andrea Zanichelli, James Hao, Matthew Iverson, Michael Smith, Christopher Yea, Paul K. Audhya, Jonathan Bernstein, Marcus Maurer, Danny Cohn

(Source: KalVista)

16 February 2024

KalVista Pharmaceuticals awarded UK innovation passport for sebetralstat

KalVista Pharmaceuticals today announced the UK Medicines and Healthcare products Regulatory Agency (MHRA) has awarded the Innovation Passport for sebetralstat, an investigational novel, oral plasma kallikrein inhibitor for the on-demand treatment of hereditary angioedema (HAE).

The Innovation Passport is the first step in the UK's Innovative Licensing and Access Pathway (ILAP), which is designed to accelerate a product's time to market and facilitate patient access to innovative medicines.

Andrew Crockett, Chief Executive Officer of KalVista, said: "As a company which has its roots in the UK, we are pleased to receive the ILAP designation, which will enable us to further accelerate our regulatory submission for sebetralstat. We look forward to collaborating with the MHRA and other health regulatory agencies worldwide as we continue to work towards bringing the first oral, on demand treatment to people living with HAE."

About the Innovation Passport. Delivered in partnership by the All Wales Therapeutics and Toxicology Centre (AWTTC), the Medicines and Healthcare products Regulatory Agency (MHRA), the National Institute for Health and Care Excellence (NICE) and the Scottish Medicines Consortium (SMC), the Innovation Passport prioritizes innovative medicines in development for the treatment of diseases for patients with significant unmet need. Benefits of the ILAP include opportunities for enhanced regulatory and other stakeholder access with the aim of accelerating the time it takes for a product to reach the market, thereby boosting patients' access to innovative medicines.

(Source: KalVista)

19 February 2024

BioCryst launches Orladeyo (berotralstat) in Italy

Biocryst Pharmaceuticals Inc. today announced that the Italian Medicines Agency, Agenzia Italiana del Farmaco (AIFA), has finalized reimbursement and recommended Orladeyo (berotralstat) for the routine prevention of recurrent attacks of hereditary angioedema (HAE) in eligible patients 12 years and older.

With this recommendation, HAE patients in Italy now have access to the first oral, once-daily therapy for the routine prevention of recurrent HAE attacks.

Charlie Gayer, Chief Commercial Officer of BioCryst, said: "The positive AIFA recommendation of Orladeyo broadens access to modern prophylaxis in Italy and is great news for Italian clinicians and HAE patients."

The AIFA decision follows European Commission (EC) marketing authorization of Orladeyo in April 2021.

(Source: BioCryst)

20 February 2024

Astria Therapeutics to present at upcoming American Academy of Allergy, Asthma & Immunology (AAAAI) Annual Meeting

Astra Therapeutics today announced that it will present final STAR-0215 Phase 1a healthy subject data at the upcoming American Academy of Allergy, Asthma, & Immunology (AAAAI) Annual Meeting in Washington, D.C. on February 23, 2024 at 3:15pm Eastern Standard Time (EST).

Dr. William Lumry, M.D., Clinical Professor of Internal Medicine at the University of Texas Health Science Center at Dallas, will present a poster titled: "Updated Results from a Phase 1a Trial of STAR-0215 for Hereditary Angioedema" at 3:15pm EST in the Convention Center, Level 2, Hall D, poster number 032.

(Source: Astria)

22 February 2024

Intellia Therapeutics announces fourth quarter and full-year 2023 financial results and highlights recent company progress

Intellia Therapeutics today reported operational highlights and financial results for the fourth quarter and year ended December 31, 2023.

Intellia President and Chief Executive Officer, John Leonard MD, said: "We're off to a very strong start in 2024 as we execute against our strategic priorities to realize the full potential of CRISPR-based gene editing. We also remain on track to begin the Phase 3 trial for our second in-vivo CRISPR-based therapy, NTLA-2002 for hereditary angioedema, later in the year."

Recent Operational Highlights. NTLA-2002 is a wholly owned, investigational in-vivo CRISPR-based therapy designed to knock out the KLKB1 gene in the liver, with the goal of lifelong control of HAE attacks after a single dose.

Intellia plans to initiate the global pivotal Phase 3 study, including US patients, in the second half of 2024, subject to regulatory feedback.

As previously announced in January, Intellia completed enrollment and dosing in the Phase 2 portion of the Phase 1/2 study in adults with HAE. The Company plans to present updated data from the Phase 1 and new data from the Phase 2 portion in 2024.

In January, the Company announced that positive interim results from the Phase 1 portion of the Phase 1/2 study of NTLA-2002 were published in the New England Journal of Medicine (NEJM). The reported data showed that a single dose of NTLA-2002 led to a 95% mean reduction in monthly HAE attack rate across all 10 patients in the Phase 1 portion. NTLA-2002 was well tolerated at all dose levels. The most frequent adverse events reported were mild, transient infusion-related reactions and fatigue.

During the fourth quarter of 2023, Intellia received Priority Medicines (PRIME) designation from the European Medicines Agency and orphan drug designation from the European Commission for NTLA-2002.

(Source: Intellia)

22 February 2024

Positive Results from CHAPTER-1 Phase 2 Study of Deucrictibant for the Prophylactic Treatment of HAE Attacks to be Presented at AAAAI 2024 Annual Meeting

Pharvaris today announced the upcoming presentation of two posters at the American Academy of Allergy, Asthma, & Immunology (AAAAI) 2024 Annual Scientific Meeting, to be held in Washington DC from 23-26 February 2024, at the Walter E. Washington Convention Center. The abstracts have been published in an online supplement to The Journal of Allergy and Clinical Immunology (JACI).

A poster, titled "Efficacy and Safety of Bradykinin B2 Receptor Antagonism with Oral Deucrictibant in Prophylaxis of Hereditary Angioedema Attacks: Results of CHAPTER-1 Phase 2 Trial," will be presented by Marc A. Riedl, MD, MS, during the poster session on Friday, 23 February from 3:15-4:15 pm Eastern Standard Time (EST). CHAPTER-1 is a two-part, Phase 2 study evaluating the efficacy, safety, and tolerability of deucrictibant for long-term prophylaxis against angioedema attacks in HAE type 1 and type 2 (HAE-1/2). The study enrolled participants in Canada, Europe, the United Kingdom, and the United States. Eligible participants were between the ages of 18 and 75 years, diagnosed with HAE-1/2, were not receiving other prophylactic treatments, and experienced an average of at least one attack per month.

34 participants were treated with double-blinded study drug (placebo or deucrictibant, 20 or 40 mg/day) for 12 weeks of treatment. Analysis of the primary endpoint demonstrated that deucrictibant significantly reduced the monthly attack rate by 84.5% (p=0.0008) in participants dosed at 40 mg/day and 79.3% (p=0.0009) in participants dosed at 20 mg/day compared to placebo. Deucrictibant was well tolerated at both doses, and all reported treatment-related treatment-emergent adverse events (TEAEs) were mild in severity, and no serious TEAEs, no severe TEAEs leading to treatment discontinuation, study withdrawal, or death were reported.

Peng Lu, MD, PhD, Chief Medical Officer of Pharvaris, said: "The CHAPTER-1 study results show that deucrictibant is the first oral therapy with the potential to provide injectable-like efficacy with a favorable safety profile to prevent HAE attacks. These data validate the mechanism of deucrictibant to provide early and sustained protection from HAE attacks. Combined with the results of our Phase 2 on-demand study, RAPIDe-1, these results support the further development of deucrictibant, which could become the preferred option to both treat and prevent HAE attacks."

A second poster, titled "Understanding the Reasons not to Treat All HAE Attacks and Patient Satisfaction for On-Demand Treatment (ODT). Results from the HAE Wave II Disease Specific Program™ (DSP™) 2023," will be presented by Joan Mendivil, MD, during the poster session on Friday, 23 February from 3:15-4:15 pm EST.

(Source: Pharvaris)

PHARVARIS

23 February 2024

BioCryst presents new real-world data showing rapid, substantial and sustained HAE attack rate reductions after beginning Orladeyo (berotralstat) treatment

Biocryst Pharmaceuticals Inc today announced new analyses of real-world use of oral, once-daily Orladeyo (berotralstat) that showed patients who initiated Orladeyo experienced rapid, substantial and sustained reductions in attack rates through 18 months of treatment regardless of the severity of their disease, their history of prior prophylaxis or their C1-inhibitor (C1-INH) level and function.

The data are being presented in five posters at the 2024 American Academy of Allergy, Asthma & Immunology (AAAAI) annual meeting, which is being held at the Walter E. Washington Convention Center in Washington, D.C., from 23-26 February 2024.

Professor Jonathan Bernstein MD, Professor of Medicine at the University of Cincinnati and partner of the Bernstein Allergy Group and Bernstein Clinical Research Center, said: "These additional analyses of real-world use of Orladeyo show that any person living with HAE has the potential to experience a rapid, substantial and sustained reduction in their monthly attack rate with Orladeyo. From patients who live with severe disease to well-controlled patients and those who have a history of being treated with other long-term prophylaxis that carry a therapeutic burden, these data demonstrate that once patients begin oral, once-daily Orladeyo, they can experience attack control over the duration of their treatment."

Dr Ryan Arnold, Chief Medical Officer of BioCryst, said: "We are continuing to see strong disease control with Orladeyo in the real world, including in patients with HAE who report differing baseline disease severities. These findings further demonstrate that Orladeyo can help maintain disease control in patients with lower baseline attack rates and further reduce attack rates in patients with more active disease. We continue to be encouraged by the consistent, building body of real-world evidence demonstrating the significant benefit that our oral, once-daily prophylactic therapy can provide to people living with HAE."

The five posters being presented are:

- ***Berotralstat Prophylaxis Reduces HAE Attack Rates Regardless of Baseline Attacks: Real-World Outcomes***; Poster #012; Friday 23 February, 3:15-4:15pm eastern time (ET)
- ***Consistently Low Hereditary Angioedema Attack Rates with Berotralstat Regardless of Prior Prophylaxis: Real-World Outcomes***; Poster #008; Friday 23 February, 3:15-4:15pm ET
- ***Real-World Effectiveness of Berotralstat in HAE With and Without C1-Inhibitor Deficiency***; Poster #281; Saturday, 24 February, 9:45-10:45am ET
- ***Assessment of the Tolerability and Effectiveness of Berotralstat for Long-term Prophylaxis in Hereditary Angioedema: BeroLife Study Interim Analysis***; Poster #028; Friday 23 February, 3:15-4:15pm ET
- ***Evaluation of Adherence to Berotralstat in Patients with Hereditary Angioedema: A Prospective Survey in Community Pharmacies***; Poster #023; Friday 23 February, 3:15-4:15pm ET

(Source: BioCryst)

26 February 2024

KalVista Pharmaceuticals presents data on unmet needs in HAE from a Patient Perspective at the 2024 American Academy of Allergy, Asthma & Immunology Annual Meeting

KalVista Pharmaceuticals today announced that it presented real-world data from US patient surveys that assessed the experience of HAE patients using injectable on-demand treatments at the 2024 American Academy of Allergy, Asthma & Immunology (AAAAI) Annual Meeting that took place in Washington, DC.

The following presentations occurred at AAAAI 2024:

- ***Characteristics of Hereditary Angioedema Attacks Among Long-Term Prophylaxis Users***: Bob Geng, Allergy and Immunology, University of California, San Diego, California, United States (Poster Presentation)
- ***Delayed On-demand Treatment of Hereditary Angioedema Attacks: Patient Perceptions and Associated Barriers***: Sandra Christiansen, University of California San Diego, La Jolla, CA, United States (Poster Presentation)
- ***Anxiety Associated with On-Demand Treatment for Hereditary Angioedema (HAE) Attacks***: James Wedner, Washington University School of Medicine, St Louis, MO, United States (Poster Presentation)
- ***Anxiety Associated with Refilling On-demand Therapy for HAE Attacks Contributes to Treatment Delay and Non-Treatment***: Autumn Burnette, Division of Allergy and Immunology, Howard University Hospital, Washington, DC, United States (Poster Presentation)
- ***Characterizing the Negative Impact of Delayed On-Demand Treatment of HAE Attacks***: Princess Ogbogu, Division of Pediatric Allergy, Immunology, and Rheumatology, University Hospitals Rainbow Babies and Children's Hospital, Cleveland, Ohio, United States (Poster Presentation)
- ***The Impact of On-demand Treatment on Quality of Life of People with HAE***: Paula Busse, Department of Medicine, Division of Clinical Immunology, Mount Sinai, New York, United States (Poster Presentation)
- ***Characterizing the Perspective of Patients With HAE on Prophylactic Treatmen***t: Stephen Betschel, Division of Allergy and Immunology, Department of Medicine, St. Michael's Hospital, University of Toronto, Toronto, Ontario, Canada (Poster Presentation)
- ***Treatment Patterns of Patients Requiring Redosing of an On-demand Treatment After the Return of an HAE Attack***: Constance Katelaris, Department of Medicine, Campbelltown Hospital and Western Sydney University, Sydney, NSW, Australia (Poster Presentation)

Andrew Crockett, Chief Executive Officer of KalVista, said: "The results of these surveys clearly conveyed the challenges faced by patients trying to manage their HAE attacks with injectable on-demand treatments. The resulting non-compliance with treatment guidelines may lead to poor clinical outcomes, even among patients receiving LTP. We believe the efficacy and safety data from our phase 3 trial for sebetralstat show a potential path forward to address these persisting unmet needs."

(Source: KalVista)

26 February 2024

KalVista Pharmaceuticals presents additional phase-3 KONFIDENT data at the 2024 American Academy of Allergy, Asthma & Immunology Annual Meeting

KalVista Pharmaceuticals today announced that it presented additional data on its phase 3 KONFIDENT trial for sebetralstat, including a more in-depth analysis of sebetralstat efficacy and safety at the 2024 American Academy of Allergy, Asthma & Immunology (AAAAI) Annual Meeting taking place in Washington, DC.

The following late-breaking presentation occurred at AAAAI 2024:

Sebetralstat for On-demand Treatment of Hereditary Angioedema Attacks: Results of the Double-blind, Placebo-controlled Phase 3 KONFIDENT Trial: Marc Riedl, Division of Rheumatology, Allergy and Immunology, University of California San Diego, San Diego, California, United States

Among the additional efficacy analyses from KONFIDENT presented during the poster session were the proportions of attacks reaching the primary (time to beginning of symptom relief) and key secondary endpoints (time to reduction in attack severity and time to complete attack resolution) without the use of a second dose. Proportions of attacks that reached the beginning of symptom relief without a second dose were 93.9% and 95.8% with sebetralstat 300 mg and 600 mg, respectively, while the proportions of attacks reaching a reduction in severity without a second dose were 90.9% and 95.9% with sebetralstat 300 mg and 600 mg, respectively. These proportions were 91.9% and 84.8% for complete attack resolution. Additional safety analyses demonstrated that the safety profiles associated with one dose or two doses of sebetralstat 300 mg or 600 mg were comparable to placebo.

Marc A Riedl, MD, Professor of Medicine and Clinical Director, US Hereditary Angioedema Association Center at the University of California, San Diego, said: "Given the unrestricted use of a second dose of oral sebetralstat in KONFIDENT, it was important to understand the proportion of attacks that achieved the primary and key secondary endpoints without a second dose. What we observed was that the vast majority of attacks that successfully met the three endpoints did so with a single dose of sebetralstat."

Andrew Crockett, Chief Executive Officer of KalVista, said: "If approved, we believe that a single dose of sebetralstat 300 mg would appear to be appropriate for most HAE attacks. However, the ability to dose flexibly depending on the characteristics of a specific attack is supported by the safety and tolerability observed with repeated dosing at both 300 mg and 600 mg. We believe that these additional efficacy and safety data only strengthen the case for sebetralstat to become the first, oral on-demand treatment available to the HAE community."

(Source: KalVista)

KalVista Pharmaceuticals

26 February 2024

BioCryst reports fourth quarter and full year 2023 financial results and upcoming key milestones

Biocryst Pharmaceuticals Inc. today reported financial results for the fourth quarter and full year ended 31 December 2023, and provided a corporate update.

Jon Stonehouse, President and Chief Executive Officer of BioCryst. "The impressive growth we are seeing with Orladeyo has put us in a position to accelerate our path to profitability while continuing to invest in our diverse pipeline of first-in-class or best-in-class molecules that we believe will deliver our next marketed product."

Program updates and key milestones for Orladeyo (berotralstat)

- Oral, Once-daily Treatment for Prevention of Hereditary Angioedema (HAE) Attacks Orladeyo net revenue in the fourth quarter of 2023 was $90.9 million.
- The total number of U.S. patients on paid or long-term free product reached 1,104 at the end of the fourth quarter (+30 percent y-o-y) with 71.5 percent of those patients on paid product.
- Net U.S. patient growth totaled 321 in 2023, including new patients still on short-term quick start product at the end of the quarter.
- The number of new Orladeyo prescribers in the fourth quarter of 2023 (Q4 2023) was the largest number of new prescribers of any quarter in 2023.

Real-world data presented at the American Academy of Allergy, Asthma & Immunology (AAAAI) annual

meeting reinforced prior data showing patients switching to Orladeyo experience sustained attack reduction regardless of baseline attack rate or prior HAE prophylaxis treatment.

Access to Orladeyo continues to expand to more HAE patients around the world. In Q4 2023, Orladeyo was approved in Argentina, launched in Spain, and granted final pricing approval in Austria. In the first quarter of 2024, Orladeyo secured final reimbursement in Italy.

Charlie Gayer, Chief Commercial Officer of BioCryst, said: "As the real-world evidence with Orladeyo consistently underscores, patients are switching to Orladeyo because they can achieve outstanding HAE attack control and tolerability with an oral, once-daily therapy, leading to life-changing results."

Fourth Quarter 2023 Financial Results. For the three months ended December 31, 2023, total revenues were $93.4 million, compared to $79.5 million in the fourth quarter of 2022. The increase was primarily due to $90.9 million in Orladeyo net revenue in the fourth quarter of 2023, compared to $70.7 million in the fourth quarter of 2022.

Full Year 2023 Financial Results. For the full year ended December 31, 2023, total revenues were $331.4 million, compared to $270.8 million in the full year ended December 31, 2022. The increase was primarily due to $326.0 million of Orladeyo net revenue in 2023, compared to $251.6 million in 2022.

(Source: BioCryst)

4 March 2024

Astria Therapeutics reports fourth quarter and full year 2023 financial results and provides a corporate update

Astra Therapeutics today announced today reported financial results for the fourth quarter and full year ended 31 December 2023, and provided a corporate update.

Jill C Milne, Chief Executive Officer at Astria Therapeutics, said: "We have strong conviction in the potential for STAR-0215 to be the first-choice preventative therapy for HAE. Our final data from the Phase 1a trial demonstrate that STAR-0215, in addition to having a trusted modality and proven mechanism, has a favorable safety profile with low risk of injection site pain and has the potential to achieve rapid and durable protection against HAE attacks. We are looking forward to sharing initial proof-of-concept data in HAE patients this quarter and believe they could support dosing as infrequently as every six months."

STAR-0215

- The ALPHA-STAR Phase 1b/2 trial of STAR-0215 in people with hereditary angioedema (HAE) is on track. Initial proof-of-concept results are expected in the first quarter of 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 (PK), pharmacodynamics (PD), and quality-of-life assessments. The initial proof-of-concept results are expected to inform on three and six month dosing and include efficacy results in the form of attack rate reduction, safety and tolerability, PK, and PD. Additionally, the company expects to have data from single and multiple doses in patients.
- Pending proof-of-concept results from the ALPHA-STAR trial, Astria expects to progress directly to a pivotal Phase 3 program which is anticipated to initiate in the first quarter of 2025.
- A Long-Term Open-Label Trial, ALPHA-SOLAR, has been initiated and is enrolling participants from ALPHA-STAR, with data from participants who have received multiple doses of STAR-0215 now accruing. The trial is assessing the long-term safety, tolerability, and efficacy of STAR-0215. Participants are receiving STAR-0215 every three or six months.
- Final results from the Phase 1a trial were shared at the AAAAI Annual Meeting in Washington DC. These results confirm early proof of concept in healthy subjects for STAR-0215 as a potential preventative HAE therapy with a favorable safety profile, long half-life, and durable PD.

(Source: Astria)

8 March 2024

KalVista Pharmaceuticals to present HAE attack journey data at 2024 HAEi Regional Conference Americas

KalVista Pharmaceuticals today announced the acceptance of multiple abstracts at the 2024 HAEi Regional Conference meeting taking place in Panama City, Panama from 15-17 March 2024. KalVista is a Silver-level sponsor of the meeting. The presentations are:

- ***Relationship Between Time to On-demand Treatment and Quality of Life During Hereditary Angioedema Attacks***: Sandra Christiansen, Timothy Craig, Maeve O'Connor, Cristine Radojicic, Julie Ulloa, Sherry Danese, Vibha Desai, Tomas Andriotti, Paul Audhya, Paula Busse. Results shared as an oral presentation as part of Abstract Oral Presentations: Session 1 on Saturday, March 16 at 9:15 am EST in the Magnolia Room
- ***Anxiety Associated with On-Demand Treatment for Hereditary Angioedema (HAE) Attack***s: James Wedner, Cristine Radojicic, Julie Ulloa, Sherry Danese, Vibha Desai, Paul Audhya, Sandra Christiansen. Results shared as an oral poster presentation and Q&A
- ***Delayed On-Demand Treatment of Hereditary Angioedema Attacks: Patient Perceptions and Associated Barriers***: Sandra Christiansen, Maeve O'Connor, Julie Ulloa, Sherry Danese, Vibha Desai, Paul Audhya, Paula Busse. Results shared as an oral poster presentation and Q&A
- ***Characteristics of Hereditary Angioedema Attacks Among Long-Term Prophylaxis User***s: Bob Geng, Vibha Desai, Julie Ulloa, Sherry Danese, Paul Audhya, Timothy Craig, Maeve O'Connor (presenter-only). Results shared as an oral poster presentation and Q&A
- ***Treatment Patterns of Patients Requiring Redosing of an On-demand Treatment After the Return of an HAE Attack***: Constance Katelaris, Michael Manning, Sally van Kooten, Neil Malloy, Markus Heckmann, Julie Ulloa, William Lumry
- ***Anxiety Associated with Refilling On-demand Therapy for HAE Attacks Contributes to Treatment Delay and Non-Treatment***: Daniel F. Soteres, Anete S. Grumach, Sally van Kooten, Neil Malloy, Markus Heckmann, Julie Ulloa, Autumn Burnette
- ***Characterizing the Negative Impact of Delayed On-Demand Treatment of HAE Attacks***: Princess Ogbogu, Hilary Longhurst, Sally van Kooten, Neil Malloy, Markus Heckmann, Julie Ulloa, Ricardo Zwiener

(Source: KalVista)

11 March 2024

KalVista Pharmaceuticals reports third fiscal quarter results and provides operational update

KalVista Pharmaceuticals today provided an operational update and released financial results for the third fiscal quarter ended 31 January 2024.

Ben Palleiko, Chief Executive Officer of KalVista, said: "This has been an exciting and busy period for KalVista, as we achieved key milestones with the release of our phase 3 KONFIDENT data and the completion of a substantial financing. We remain on track to file the NDA with the US Food and Drug Administration in the first half of this year, and we look forward to presenting further phase 3 data for sebetralstat at upcoming patient and medical meetings. We also continue to grow the commercial organization to enable us to deliver on the promise of providing the first oral, on-demand treatment option to the HAE community."

Recent Business Highlights:

- In February, the Company announced positive topline data from the Phase 3 KONFIDENT clinical trial evaluating the safety and efficacy of sebetralstat as the potential first oral, on-demand therapy for hereditary angioedema (HAE). KONFIDENT was the largest and most representative trial ever conducted in HAE, enrolling a total of 136 patients from 66 clinical sites across 20 countries. Eligible participants included adolescents, patients using long-term prophylaxis, and the trial evaluated all attack severities and locations. The clinical trial met all primary and key secondary endpoints across both the 300 and 600 mg doses and demonstrated a safety profile similar to placebo.
- The Company announced that Benjamin L. Palleiko, the Company's prior President, CBO and CFO, was appointed as Chief Executive Officer and a director of the Company. This appointment is the result of a planned transition as KalVista prepares to become a commercial entity following the success of the KONFIDENT Phase 3 trial for its program sebetralstat.
- The UK Medicines and Healthcare products Regulatory Agency (MHRA) awarded the Innovation Passport for sebetralstat, providing entry to the UK Innovative Licensing and Access Pathway (ILAP), which aims to accelerate time to market and facilitate patient access to innovative medicines.
- Presented additional Phase 3 KONFIDENT data as well as data on unmet need in HAE from a patient perspective at the 2024 American Academy of Allergy

KalVista Pharmaceuticals

Asthma & Immunology Annual Meeting (AAAAI) in Washington, D.C.
- Announced publication of the first report of a potent and specific Factor XIIa inhibitor with high oral availability in a peer-reviewed journal. The Frontiers in Pharmacology article describes the pharmacology of a representative compound from KalVista's portfolio of structurally diverse, oral Factor XII inhibitors.

(Source: KalVista)

12 March 2024

KalVista Pharmaceuticals awarded UK Promising Innovative Medicine (PIM) designation for sebetralstat

KalVista Pharmaceuticals today announced the UK Medicines and Healthcare products Regulatory Agency (MHRA) has awarded the Promising Innovative Medicine (PIM) designation for sebetralstat, an investigational novel, oral plasma kallikrein inhibitor for the on-demand treatment of hereditary angioedema (HAE). The PIM is the first step in the Early Access to Medicines Scheme (EAMS) which would allow KalVista to treat patients with sebetralstat prior to receiving a Marketing Authorisation.

Ben Palleiko, Chief Executive Officer of KalVista, said: "We are proud to have sebetralstat designated as a Promising Innovative Medicine by the MHRA, which is similar to Expanded Access in the US. Receiving the PIM designation shows that the MHRA believes that we have a promising candidate for the EAMS to treat people living with HAE."

The PIM designation also gives companies the opportunity to have early in-depth discussions with both the National Health Service (NHS) and the UK's Health Technology Agencies.

About the Promising Innovative Medicine Designation
A Promising Innovative Medicine Designation is an early indication that a medicinal product is a promising candidate for the Early Access to Medicines Scheme (EAMS), intended for the treatment, diagnosis or prevention of a life-threatening or seriously debilitating condition with the potential to address an unmet medical need. The designation is issued after an MHRA scientific designation meeting on the basis of non-clinical and clinical data available on the product, in a defined disease area. Following designation, the applicant is expected to complete a clinical development program within a reasonable time period, in order to continue with an application under the EAMS (step II). A designation is a prerequisite to enter the EAMS scientific opinion assessment step.

(Source: KalVista)

14 March 2024

Pharming Group reports fourth quarter and full year 2023 results.

Pharming Group presents its preliminary (unaudited) financial report for the three months and full year ended 31 December 2023.

Sijmen de Vries, Chief Executive Officer, commented: "We are pleased to have delivered an excellent year in which we transformed Pharming into a multiproduct, commercial rare disease biopharmaceutical company."

Over 70 Ruconest new patient enrollments were achieved for four quarters in a row. Total enrollments in 2023 were up 25% vs. 2022. The Ruconest physician prescriber base increased by 13% during the year, in many cases adding previously unknown HAE prescribers.

(Source: Pharming)

21 March 2024

KalVista Pharmaceuticals Presents Real-World Data on Burden of Treatment and HAE Attack Journey at the 2024 HAEi Regional Conference Americas

KalVista Pharmaceuticals announced that it presented multiple analyses of the relationship between the time to attack treatment and the effects delays in treatment have on clinical outcomes of people living with hereditary angioedema (HAE) at the **2024 HAEi Regional Conference Americas** that took place in Panama City, Panama.

The following presentations occurred at the **2024 HAEi Regional Conference Americas**:

Relationship Between Time to On-demand Treatment and Quality of Life During Hereditary Angioedema Attacks: Sandra Christiansen, University of California San Diego, La Jolla, CA, United States (Oral Presentation)

- Treatment delays are associated with lower QoL and poorer general health during HAE attack, emphasizing the benefits of compliance with HAE guidelines and greater awareness of the impact of delayed treatment on QoL

Characterizing the Negative Impact of Delayed On-Demand Treatment of HAE Attacks: Ricardo Zwiener, Servicio de Alergia e Inmunología Clínica, Hospital Universitario Austral, Pilar, Buenos Aires, Argentina (Poster Presentation)

- The time to feeling in control of an HAE attack and time to feeling fully recovered were shorter for patients treating HAE attacks in <1 hour versus those who waited ≥1 hour
- Anxiety Associated with Refilling On-demand Therapy for HAE Attacks Contributes to Treatment Delay and Non-Treatment: Anete S. Grumach, Clinical Immunology, Faculdade de Medicina, Centro Universitario FMABC, Santo Andre, Brazil (Poster Presentation)
- Anxiety associated with not being able to refill on-demand treatment impacted treatment decisions, which contributed to treatment delay or resulted in non-treatment of HAE attacks
- Treatment Patterns of Patients Requiring Redosing of an On-demand Treatment After the Return of an HAE Attack: William Lumry, Allergy and Asthma Research Associates, Dallas, Texas, United States. (Poster Presentation)
- HAE attacks initially treated within 1 hour returned less frequently compared with attacks treated at 1 hour or longer

Anxiety Associated with On-Demand Treatment for Hereditary Angioedema (HAE) Attacks: Maeve O'Connor, Allergy, Asthma, & Immunology Relief of Charlotte, Charlotte, NC, United States (Poster Presentation)

- Both adults and adolescents with HAE reported moderate to extreme anxiety when anticipating use of parenteral on-demand treatment, irrespective of use of on-demand only or on-demand plus LTP

Characteristics of Hereditary Angioedema Attacks Among Long-Term Prophylaxis Users: Maeve O'Connor, Allergy, Asthma, & Immunology Relief of Charlotte, Charlotte, NC, United States (Poster Presentation)

- Among HAE patients who had treated a recent attack, the location and duration of the most recent attacks were similar between long-term prophylaxis (LTP) and on-demand only users

(Source: KalVista)

KalVista Pharmaceuticals

18 March 2024

Pharvaris announces Phase 3 clinical study design for recently initiated RAPIDe-3 study, and presents quality-of-life improvement and caregiver behavior data at two recent HAE congresses

Pharvaris presented at two recent congresses: the 3rd National Congress of the Italian Network for Hereditary and Acquired Angioedema (ITACA) and the 2024 HAE International (HAEi) Regional Conference Americas.

Berndt Modig, Chief Executive Officer of Pharvaris, said: "We are committed to the continued advancement of our clinical development program of deucrictibant to fulfil unmet needs of current HAE treatment. In collaboration with regulatory authorities, we have designed a robust global study to assess the efficacy and safety of deucrictibant, a molecule which we believe has the potential to be best-in-class for both the prevention and treatment of HAE attacks."

The design of the Phase 3 RAPIDe-3 study was showcased for the first time in two posters on Friday. One titled "Design of RAPIDe-3 Phase 3 Trial: Efficacy and Safety of the Oral Bradykinin B2 Receptor Antagonist Deucrictibant Immediate-Release Capsule in Treatment of Hereditary Angioedema Attacks" was presented by Mauro Cancian, MD, PhD, at the ITACA meeting and the second, titled "Efficacy and Safety of the Oral Bradykinin B2 Receptor Antagonist Deucrictibant Immediate-Release Capsule in Treatment of Hereditary Angioedema Attacks: RAPIDe-3 Phase 3 Trial Design" was presented by Anete Grumach, MD, PhD, at the HAEi Americas Congress. RAPIDe-3 is a randomized, double-blind, placebo-controlled, crossover study, which is designed to enroll approximately 120 adolescent and adult participants globally. The primary efficacy endpoint is time to onset of symptom relief, as measured by Patient Global Impression of Change (PGI-C) of at least "a little better" for two consecutive timepoints within 12 hours post-treatment. Other efficacy endpoints include time to End of Progression (EoP) in attack symptoms within 12 hours as measured

by PGI-C, and proportion of attacks achieving symptom resolution with one dose of deucrictibant as measured by Patient Global Impression of Severity (PGI-S).

In a poster titled "Prophylactic Treatment with Oral Deucrictibant Improves Health-Related Quality of Life of Patients with Hereditary Angioedema" presented by Andrea Zanichelli, MD, PhD, on Friday at the ITACA meeting, two health-related quality of life (HRQoL) outcomes were measured in participants from CHAPTER-1, a double-blinded, placebo-controlled Phase 2 study evaluating the efficacy and safety of deucrictibant for the prevention of HAE attacks. The data illustrates that HRQoL is negatively impacted, including functional and psychological impairment, in people with HAE. Analyses of CHAPTER-1 study data provide evidence that prophylactic treatment with oral deucrictibant for 12 weeks improved HRQoL for people living with HAE, in addition to significant reduction of attacks.

In a poster titled "Need for Caregiver Support for People Living with Hereditary Angioedema in European Countries," also presented by Andrea Zanichelli, MD, PhD, on Friday at the ITACA meeting, the Adelphi HAE Disease Specific Programme™ (DSP™) examined caregiver support requirements among people living with HAE in some European countries, as well as the impact of their condition on their HRQoL and ability to work.

(Source: Pharvaris)

10 April 2024

Pharvaris reports fourth quarter and full year 2023 financial results and provides business update

Pharvaris reported financial results for the fourth quarter and year ended 31 December 2023, and provided a business update.

Berndt Modig, Chief Executive Officer of Pharvaris, said: "2024 is off to a strong start, supported by the incredible momentum we built in an impressive 2023—driven by a second positive data readout of deucrictibant that was validated by the support of our investors. At the start of the year, the lift of the remaining hold on the prophylaxis program in the U.S. allowed us to progress with the global Phase 3 clinical development plans for deucrictibant for prevention of HAE attacks. As we move toward the initiation of CHAPTER-3, we hope to realize the promise of the proof-of-concept CHAPTER-1 data, which support deucrictibant's potential to be a best-in-class oral prophylactic therapy. We are pleased with the HAE community's excitement in RAPIDe-3, which is enrolling as planned; we will provide the anticipated timing of topline data as enrollment progresses and data is accumulated. Receipt of the Innovation Passport designation for deucrictibant in the UK reflects regulatory recognition of deucrictibant's innovation for better treatment options for people living with HAE."

Recent business updates and highlights:

Enrollment initiated in RAPIDe-3 - a global Phase 3 clinical study. Pharvaris is currently enrolling in RAPIDe-3, a global pivotal Phase 3 study of deucrictibant immediate-release capsule (PHVS416) for the on-demand treatment of HAE attacks.

End-of-Phase 2 meeting scheduled to align on prophylactic Phase 3 clinical development plan. Pharvaris continues preparatory activities for CHAPTER-3, a global Phase 3 study of deucrictibant extended-release tablets (PHVS719) for the prophylactic treatment of HAE attacks.

Deucrictibant awarded UK Innovation Passport. The UK Innovative Licensing and Access Pathway (ILAP) Steering Group, which consists of the All Wales Therapeutics and Toxicology Centre (AWTTC), the Medicines and Healthcare products Regulatory Agency (MHRA), the National Institute for Health and Care Excellence (NICE), and the Scottish Medicines Consortium (SMC), has awarded an Innovation Passport to deucrictibant for the on-demand and prophylactic treatment of HAE attacks in people 12 years and older.

Clinical hold lifted. Following review of data from a 26-week rodent toxicology study, the FDA lifted the clinical hold on the Investigational New Drug (IND) application for deucrictibant for the prophylaxis of HAE attacks.

Deucrictibant data presented at recent industry meetings. Data supporting the ongoing development of deucrictibant for both the on-demand and prophylactic treatment of HAE attacks were presented at many international scientific meetings.

(Source: Pharvaris)

17 April 2024

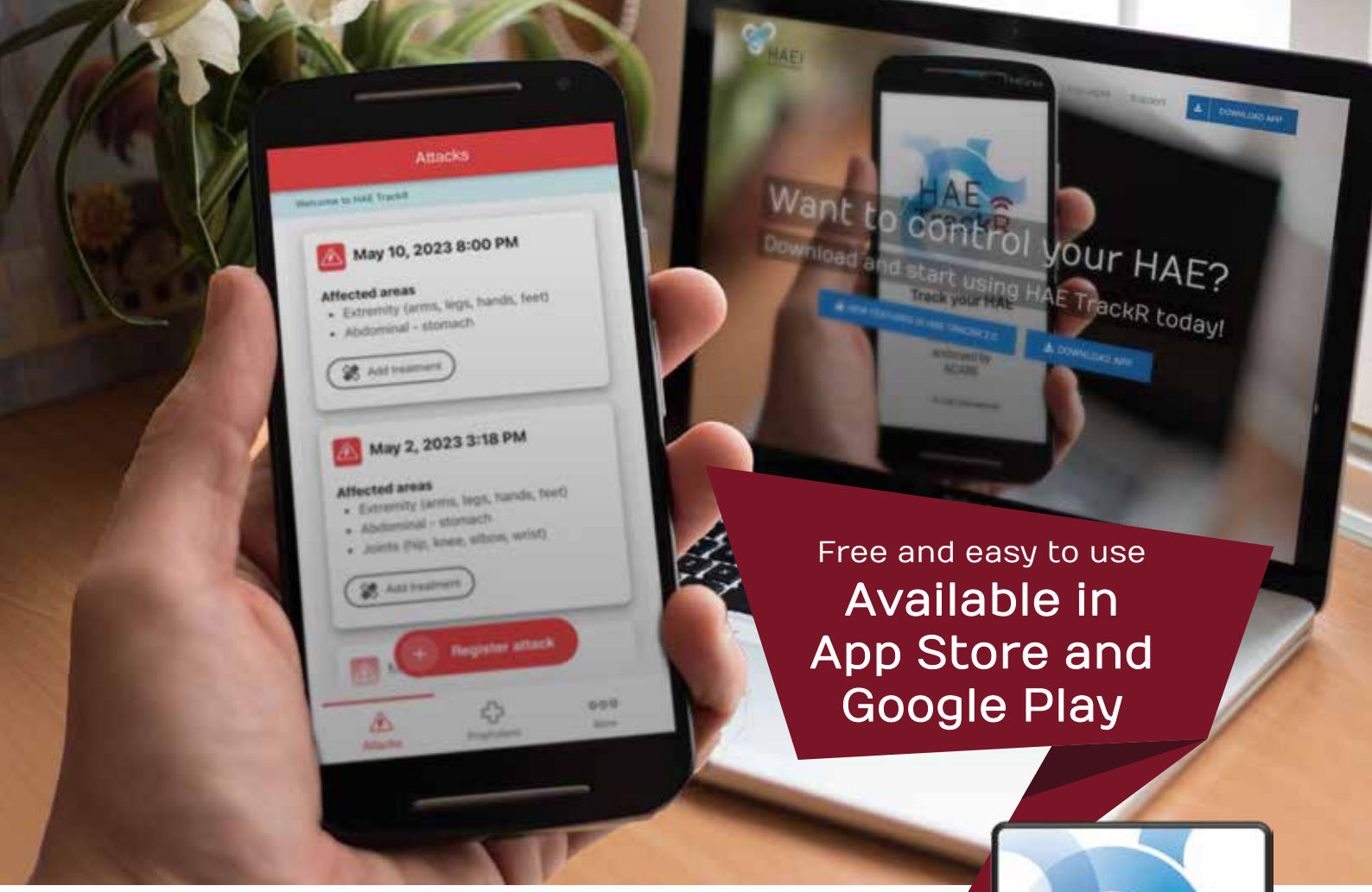
BioCryst Announces Approval of Orladeyo (berotralstat) by the Brazilian Health Regulatory Agency

Biocryst Pharmaceuticals Inc announced that the Brazilian Health Regulatory Agency (ANVISA) has granted approval for oral, once-daily Orladeyo (berotralstat) for the prophylaxis of hereditary angioedema (HAE) attacks in adults and pediatric patients 12 years of age or older.

Charlie Gayer, Chief Commercial Officer of BioCryst, said: "We are excited to announce that Orladeyo is now approved in the region's largest market, following the positive regulatory decisions we received in Chile and Argentina last year. This is an important moment for patients living with HAE in Brazil, as they will soon be able to access our oral, once-daily prophylactic therapy to help manage their condition. We look forward to working with our partner, Pint Pharma, to make Orladeyo available in Brazil and across LATAM."

BioCryst has an exclusive collaboration with Pint Pharma GmbH to register and promote Orladeyo in the pan-Latin America region. Under the terms of the agreement, Pint is responsible for obtaining and maintaining all marketing authorizations and for commercializing Orladeyo in the region.

(Source: BioCryst)



Get Your HAE under Control with the HAE TrackR App

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

Download on the App Store bit.ly/haetrackr-appstore

GET IT ON Google Play bit.ly/haetrackr-googleplay

Start using **HAE TrackR** today – download from **App Store** or **Google Play**!

HAEi AROUND THE WORLD

Currently there are HAE Member Organizations in **99** 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.