DECEMBER 2023

Global perspectives:

HAEi MAGAZINE · ISSUE 3/2023

99 Member countries

Global perspectives:

Global Perspectives
Issue 3/2023
December 2023

Cover photo
HAEi is truly a global organization with 99 member countries worldwide

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

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

DEAR HAEi FRIENDS,

On behalf of the Board of Directors and the entire HAEi team, we extend warm holiday greetings and wishes for a happy and healthy New Year. As 2023 comes to a close, we celebrate the amazing spirit and dedication of our global HAEi community. All HAEi Friends should be proud of what has been accomplished this year. Together, we will continue and even enhance our mutual quest for improving the lives of people with HAE in 2024.

In this final 2023 issue of Global Perspectives, we present highlights from our Regional Patient Advocates in a creative, engaging infographic format that visually captures each region's efforts and progress.

In the article "Advocacy is Proactivity," HAEi leaders share their thoughts about the importance of fierce advocacy that brings modern HAE medicines to all corners of the globe. The spirit of proactive advocacy is also reflected in the piece "A New Lease on Life," which turns the spotlight on HAE leaders from Chile and The Netherlands who relaunched, rejuvenated, and put their Member Organizations on the path to successful advocacy.

This issue also introduces Rachel as HAEi's new Coordinator, Angioedema Centers of Reference and Excellence (ACARE); an HAEi and GA^{2}LEN partnership and network. In addition, we hear from representatives from 3 Centers, who share their views regarding what it means to be accredited and operate as an ACARE.

In a new Global Perspectives feature, "TALKING TO ...", Professors Marcus Maurer and Bruce Zuraw offer invaluable perspectives on their hopes for the future as well as challenges faced by the global HAEi community.

The article "LEAP Class of 2023" reveals the remarkable impact of this unique global Youngster's Community educational program. We look forward to welcoming the 2024 HAEi LEAP Class in April.

The piece entitled "HAEi 2023 Research" provides a glimpse into the pioneering HAE with Normal C1-INH Symposium. We brought together 31 of the world's leading researchers who will publish a medical journal paper outlining the latest thinking regarding the diagnosis and treatment of this ultra-rare condition. This article also summarizes HAEi-sponsored advocacy research that is ongoing in various countries throughout the world.

This edition of Global Perspectives also includes "Countdown to **hae day :-)** 2024" with inspiring quotes from Member Organizations who remind us how this global awareness day can motivate involvement and strengthen advocacy efforts.

Following the successful **2023 HAEi Regional Conference APAC** and **2023 HAEi Regional Conference EMEA**, we look forward to welcoming HAEi friends from North, Central, and South America to the **2024 HAEi Regional Conference Americas** in Panama City, Panama, in March. Simultaneously, we cordially invite the leaders from our 99 member countries, our global scientific community, and industry representatives to join us for the trailblazing **2024 HAEi Global Leadership Workshop** in Copenhagen, Denmark, in early October next year.

You will also find updates from our Member Organizations around the globe, industry news, pivotal medical journal papers, and information on clinical trials.

Warm regards and Happy Holidays!

Tony
Anthony J. Castaldo
Chief Executive Officer, HAE International

Henrik
Henrik Balle Boysen
President, HAE International

IN THIS ISSUE OF GLOBAL PERSPECTIVES

HIGHLIGHTS FROM HAEi'S REGIONAL PATIENT ADVOCATES

HAEi Regional Patient Advocates – thank you for what you have been doing!

As always, time flies, and so it has this year. The December issue of the Global Perspectives magazine is a perfect opportunity to summarize the past 2023 year. I feel extremely privileged to be able to collaborate with the group of outstanding experts, Regional Patient Advocates (RPAs), who, in their daily activities, help HAE people in their respective regions.

Thanks to their tireless efforts, HAEi was able to initiate many interesting projects and achieve further goals. RPAs are in constant contact with HAEi Member Organizations, helping them implement available tools and resources that play a fundamental role on the advocacy path to access to and reimbursement of modern HAE treatment options.

Many of you had the opportunity to meet RPAs during this year's HAEi Regional Conferences: APAC in Bangkok and EMEA in Munich. Some of you will meet the RPAs at the 2024 Regional Conference Americas in Panama in March.

In the magazine's December issue, each of our RPAs has selected a few of the most important moments and successes from the past year. As you can probably guess, it was not easy due to the many activities RPAs were involved in. I am even more eager to know what they came up with. You will also be able to read their plans and goals for 2024 on these pages:

The RPA highlights showcase some of the work of the HAEi Regional Patient Advocates and our Member Organizations. Perhaps it will inspire you to start advocating more for HAE patients in your country. Taking this opportunity, I would like to express my gratitude to all HAE advocates for their invaluable work over the past year.

Michal Rutkowski
Director, Regional Patient Advocate Program

SOUTH AMERICA AND MEXICO

Region Member Organizations:

Argentina · Bolivia · Brazil · Chile · Colombia · Ecuador · Mexico · Paraguay · Peru · Uruguay · Venezuela

2023 HIGHLIGHTS

Identified HCPs with an interest in knowing more about HAE

ALL COUNTRIES IN THE REGION CONTRIBUTED

HAEi BURDEN OF ILLNESS SURVEY CONDUCTED IN MEXICO

Clear picture of the HAE burden for those living with HAE in Mexico. Presented at the Latin America Society for Immunodeficiencies (LASID) and to the Mexico Congress of Deputies.

FOCUS ON HAE IN CHILDREN

HAE Peru patient meeting: Conversatorio de pacientes de AEH de Peru

Increased

- SHARED EXPERIENCES
- SUPPORT NETWORK
- KNOWLEDGE OF HAEi TOOLS TO HELP MANAGE HAE IN SCHOOL

NEW MEMBER ORGANIZATION: CORPORACIÓN ANGIOEDEMA HEREDITARIO CHILE

RESCUE MEDICINE APPROVED FOR INCORPORATION

Submitted evidence to the public consultation

INTO THE PUBLIC HEALTH SYSTEM

HOPES FOR 2024

FERNANDA DE OLIVEIRA MARTINS

I hope, and I work for it, that every HAE patient in my region can have access to modern therapies and therefore can live life with no worries about HAE.

*At the recent **2023 HAEi Regional Conference EMEA**, we got to sit down with HAEi Leadership (from left), Henrik Balle Boysen, Tony Castaldo, Fiona Wardman, and Jørn Schultz-Boysen. We spoke to them about their passion for advocacy and their hopes and fears for the future.*

HAEi LEADERSHIP ROUNDTABLE INTERVIEW

ADVOCACY IS PROACTIVITY

Let's start by discussing what keeps you all working so hard. What excites you and fires you up to do more?

Tony: My passion is seeing a country or a person finally get access to treatment. The sense of community that we feel is another big driver. We see physician-scientists, patients, and caregivers come together and learn from each other. The fellowship we all feel is something magical. People getting together with a shared interest. When we hear: 'Gosh, you know, I've never met anybody else with this rare condition before,' or 'Wow, I've learned things that I didn't know about before.' That really excites me.

Fiona: I share that. Bringing people together, whether they are old friends or meeting for the first time, is an opportunity to share knowledge and learn more.

Henrik: Yes, seeing so many people with a 'can-do' attitude. When you get HAE patients together, they know that everybody there will understand where they come from and their situation. They don't have to explain everything from the beginning. So, there's a certain pre-understanding of everybody, which makes the meeting take off immediately.

The sight of HAEi continuing to grow inspires me, too. The strength that we see in each of our Member Organizations is really exciting. As an individual or a group begins to organize and grow, we see the difference our tools and support offer.

> As an individual or a group begins to organize and grow, we see the difference our tools and support offer.

Tony: It takes just one active person for HAE to get a seat at the table and for the situation to improve. The **2023 HAEi Regional Conference EMEA** took the slogan 'Take Action.' And that remains the case wherever you are in the world. We want to motivate people to go back and be more active. There is strength in numbers. When our community comes together, things improve.

Henrik: Every patient who Takes Action is a hero, advocating for their case to get access to and reimbursement for life-saving and life-giving HAE medication.

Fiona: Even the smallest Member Organization can act like a bigger one with the right tools. Whether it's one person, like Henrik said, or a group of people. No matter how they're organized, they can be organized, whether they have an hour or 10 hours a week.

Jørn: Being on the cusp of 100 Member Organizations is hugely exciting. I'm most inspired by the dedication of the many passionate individuals in the HAE community who work tirelessly every day to improve the lives of those living with HAE, striving to transform their situation for the better.

What is your biggest hope for the future?

Tony: My ultimate vision is that by doing our part collectively, we bring access to one or many forms of modern therapies everywhere in the world. Everything is focused on that. So, when I talk about our HAE patient-driven research, it is about giving HAE a seat at the table when decisions are made. Decisions about medicines are driven by data.

Henrik: Hope comes from seeing our young people come together. It's incredible to see all those youngsters wanting to be part of the future of their organization and HAEi.

Fiona: Every person who understands HAE, every correct diagnosis, every program of awareness, and ultimately, every new person accessing medication is a cause for hope. We know there is much to do. There are still countries where access is limited or non-existent, but every positive step shows change is possible.

> Every person who understands HAE, every correct diagnosis, every program of awareness, and ultimately, every new person accessing medication is a cause for hope.

Jørn: I want every person with HAE to have access to and receive reimbursement for modern HAE treatments and have the freedom to choose the option that best suits their needs. Before that happens, it is crucial to identify individuals with HAE, secure their diagnosis and treatment, and thereby provide the highest possible quality of life for every person with HAE and their families.

Tony: The continued advancement of HAE science and new companies coming in with more advanced therapies, are huge sources of hope and excitement. Together with patients, caregivers, physicians, and pharmaceutical companies, we can move the needle to bring treatments to individual countries.

You mentioned moving the needle. Can you give any examples of impact?

Tony: Dr. Philip Li in Hong Kong credits HAEi as a reason why there is now a Member Organization. Ultimately, patients in Hong Kong are starting to get access to medicines. Dr. Li is working on an exciting study to try and quantify how our work has made a tangible impact in that specific region.

Fiona: Hong Kong is a great example, and so is India. When we first started in India, there was nothing. We only had access to androgens, not even fresh frozen plasma, in the hospital. Now, we've got access to C1-INH products, with on-demand treatment potentially on the horizon. Adjusting for the years lost to COVID, they've moved so far in three or four years maximum.

Jørn: 2023 has been a year of remarkable impact. HAEi has collaborated across our community to reach more people than ever. Our meetings are over-subscribed. **hae day :-)** wraps the globe in solidarity and understanding, touching every corner of the HAE community. This day embodies the vast reach and unified spirit of our cause. As Henrik mentioned, our young people are such a beacon of a bright future, especially the LEAP class of 2023. Numerous young participants have completed the educational program and contributed significantly by undertaking and delivering projects for their member countries. This level of engagement and accomplishment is nothing short of extraordinary. Ultimately, success is achieved through our collective impact across the HAE community. Our joint efforts form a vibrant mix of hope, advocacy, and progress for the HAE community worldwide.

> Ultimately, success is achieved through our collective impact across the HAE community. Our joint efforts form a vibrant mix of hope, advocacy, and progress for the HAE community worldwide.

What concerns you or stands in the way of this hope for the future?

Tony: Economic realities exist when discussing access to and reimbursement for modern HAE medicines. Ours is a tiny population. We are working with our physician-scientists and pharmaceutical companies to make these clearly, life-saving, and life-giving medicines available.

Henrik: Bringing access to medicines and, most importantly, reimbursement is a fight we've not yet won. Things are improving, and the future for HAE is bright. We currently have eight approved medications, with nine more therapies being evaluated in ongoing clinical trials. It's unheard of within a rare disease to have that many choices. But there's so much more to do. Our work must be focused on creating a situation where the health minister of a country wakes up every morning, and the first thing they think about is HAE.

Jørn: The struggle of those who remain undiagnosed is particularly troubling for me, as we are acutely aware of the severe impact the disease can have when it goes unrecognized and untreated.

Fiona: Treatment availability is also closely linked with diagnosis. We often see that there are patients diagnosed following treatment access. And so, to make sure that we find and diagnose as many patients as possible, which is a core aim for HAEi, we must keep pushing for reimbursed treatment.

Jørn: Our work must be sustained for the long haul. There is no quick fix to getting people with HAE the care and support they need.

Tony: I like to tell a story of perseverance. There's a little country called the United States. Back in the day, we didn't have access to any medicines. We had heard that the Europeans had access to products like C1 inhibitors. And so, in 1999, we started an advocacy organization. Our first HAE product wasn't delivered until late 2008. It took nine years of intensive advocacy to reach where we need to be. We did it because we found other organizations that already had access to medicine. We found physicians who understood how all this worked. Then, we also found pharmaceutical companies and worked with them to see if we could convince them to come to our country to provide these medicines.

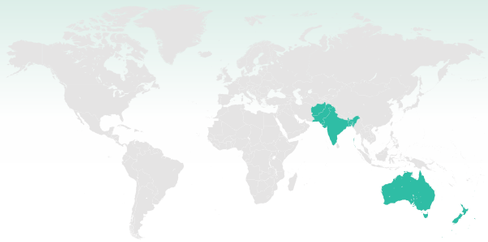
What would you say to people with HAE who aren't currently active?

Tony: Get involved. Get involved with your Member Organization. Get your family involved. Make sure that you're in contact with your Member Organization. Make sure you also get Global Perspectives magazine because that will tell you about everything happening in the world of HAE.

Henrik: Grab the information, grab the excitement, grab the motivation to do something. Please don't just sit back and wait for others.

Tony: Every individual, we call it the power of one. You gotta get out there!

> "Every individual, we call it the power of one. You gotta get out there!



ASIA PACIFIC AND SOUTH AFRICA

Region Member Organizations:

Afghanistan · Australia · Bangladesh · India · Japan · New Zealand · Pakistan · South Korea · South Africa

2023 HIGHLIGHTS

PHARMACO

AND SOCIOECONOMIC SURVEY IN AUSTRALIA AND NEW ZEALAND

New Zealand patients do not have access to prophylaxis at this time. With the data collected, the organization can show the government the costs and benefits for funding modern preventative therapies.

EDUCATIONAL WEBINAR

International speakers deliver an educational webinar to Bangladesh doctors

HAE INDIA OFFICIALLY REGISTERED

The group will be able to fundraise and run activities for patients and their families in India. Having a registered organization makes it easier to work with pharmaceutical companies directly.

2023 HAEi Regional Conference APAC

Increased activity

SOUTH AFRICA

Delivering

- More engagement
- More collaborations
- More information available
- More industry interest

PAKISTAN

Delivering

- More strength to the organization
- More education to doctors
- More recognition as force for advocacy!

All countries in the APAC region participated

Increased

- COLLABORATION
- PARTNERSHIP
- EXCITING PROJECTS

Renewed motivation to advocate for access to medicines and better quality of life

HOPES FOR 2024

FIONA WARDMAN

I want to assist the region to grow with more collaborations and bring more attention from pharmaceutical companies to this underserved part of the world. Patient meetings, awareness, and education sessions are already planned in some countries. I hope to increase the number of patient groups by fostering connections and continuing to open doors in more countries in the region. I look forward to the **2024 HAEi Global Leadership Workshop** in Copenhagen. Being able to bring all the region's Member Organization (MO) leaders together, to connect once again, and to meet with others worldwide will be of huge benefit to further collaborations and create stronger MOs.

A New Lease of Life

How Two Patients Rose to the Challenge of Leading Member Organizations

Across the globe, the HAEi family of Member Organizations (MO) has grown to 99. With such a large number of groups doing incredible work to support people living with HAE, it can take a lot of work to keep up. To help, we will hear directly from MO leaders on what's happening and how they are embracing change and helping their members live their best lives with HAE.

For this issue, we spoke with José Ignacio Contreras Silva in Chile and Marijk Beekman-Kortekaas in the Netherlands. Both have recently worked with other volunteers to rejuvenate and relaunch their MO following a period of inactivity. They tell us about how patients felt unsupported and how, by stepping up, patients could access vital information and see a strong local group advocating for them.

First, we asked the two leaders to explain more about the situation in their countries.

José: In Chile, patients were without support from an entity that cared about hereditary angioedema (HAE). They had no information or support for themselves and their families. They did not know about new therapies or how to improve their quality of life.

Marijk: In the Netherlands, the group is an organization for all types of angioedema. However, HAE needs a totally different form of advocacy from non-hereditary forms. There didn't seem to be any sense of urgency to advocate for people with HAE, and everyone was unhappy. The pandemic hit the group, and there was nothing organized and virtually no news. In 2020, they started to look for a new president. And at the time, I thought, well, no, it's not for me. I was trying to finish an education and was having three attacks a week. The news came that they would need to terminate the organization if they couldn't find anyone.

How did the situation impact people living with HAE in Chile and the Netherlands?

Marijk: We needed someone to advocate for people with HAE. There were new medicines that needed reimbursement. I enrolled in clinical trials through an ACARE center, so I had a lot of information. But, I discovered that other patients didn't have the latest information. People were asking questions on Facebook. The information there was outdated. Many still thought we could only treat throat swells or abdominal swells. If you're treated in a local hospital with little knowledge about HAE, you don't get the best or latest information.

José: The people were very sad and angry. They had lost hope that anyone could help them to improve the situation in Chile and continue to fight for them.

Marijk: The most significant thing was no peer support. For me, peer support is one of the most important aspects of living with HAE. If you have a large family with all kinds of people who live with HAE, you can talk to each other. But I'm the only one in my family. And at the time, most people in the Netherlands were just suffering alone.

> For me, peer support is one of the most important aspects of living with HAE.

Can you explain to me what changed?

José: We created a new organization and were immediately able to assist all those who needed information on the latest treatments available in Chile. Specifically, we could explain the protocol used in our country to administer Berinert. The Chilean organization now visits health centers where we show them what HAE is all about.

Marijk: I needed to step up. I told myself, 'I need to become president of this organization.' So I stepped up and told them, ' Well, okay, if no one else wants to do it, I'll do it.' I had absolutely zero knowledge about the organization.

José: We began to hold meetings with the Ministry of Health to inform them of the progress in HAE management. We are working on an assistance program where patients' emergencies are attended to by telephone 24 hours a day.

Marijk: We had to step up the pace of medication access, ensuring modern medicines get and stay reimbursed. We needed to professionalize because only professional organizations are invited to sit at the table and contribute.

We worked on the website, so now there's correct information in the right language so that it's understandable for everyone. HAE is a complex disease, and most information is difficult for large groups of people to understand.

José: We are working on a free psychological help program for all pediatric patients, adults, and caregivers. It will start in March of next year.

Marijk: We had a patient meeting last October. One of the doctors told the audience that some people with HAE experience anxiety or irritability as a prodromal symptom. The doctor described a situation where a patient was irritated and screaming in the car with her daughter. The daughter tells her mom, 'Mom, I think it's time to get your on-demand treatment because you're suffering from an attack.' I had to laugh as it sounded like me! That's a great case study example of the power of peer support.

How did HAEi support these changes?

Marijk: Michal [Rutkowski] introduced me to many people. We built a network of people and organizations to create more awareness in the Netherlands. I traveled to Frankfurt for the **2022 HAEi Global Leadership Workshop**. When I arrived, I was like, 'All these people know each other. They're all friends, and I'm the new kid.' Michal saw me struggling and said, 'I will introduce you to lots of people.' And he did. He introduced me to Fiona Wardman of HAE Australasia. Fiona connected me with Evelien de Bruin from HAE Australasia, who was also in Frankfurt. It turned out Evelien has a sister in the Netherlands, and her sister was one of the first board members of my organization. Their mother actually founded HAE Netherlands. Within a week of getting home from Frankfurt, I got an email from Evelien. We had an amazing brainstorm session. All because Michal introduced me to Fiona.

José: Fernanda was the best help we could receive to start working as a corporation. She was always there when we needed information, always concerned about the situation in Chile and how the patients were. Together with others, she and HAEi have provided us with tools and support. We are delighted to be part of the HAEi family.

> We are delighted to be part of the HAEi family

Marijk: There was so much support available. Our Regional Patient Advocate, Maria Ferron, traveled to Utrecht for our patient meeting. We had no translation service whatsoever. But she stayed the whole day. She was there for me, and that is what HAEi does. They always tell us we're one big global family. I thought, 'Is it really true? But you feel it. You just feel part of this big global family.'

And how have people living with HAE benefitted?

José: Now, patients are very happy and excited. They feel calm knowing that Corporación Angioedema Hereditario Chile is fighting for them and that HAEi backs us. Our members show their support every day by actively participating in meetings. They deeply believe in our organization's mission: 'to help and protect all patients with Hereditary Angioedema in Chile.'

Marijk: People were relying on doctors to provide them with information. Now we have information, and people are just stepping up for themselves. People who aren't even members tell me the information we share is working. At the pandemic's start, the ACARE center in Amsterdam had 100 patients. The last I heard, they have 150 patients. People see a strong organization that wants to advocate for everyone.

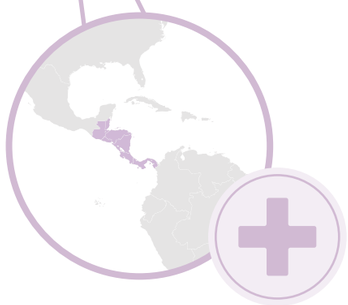
I speak to people worldwide, from Pakistan to South Africa, and we discuss what we can do. I learn from them; they learn from me; it's powerful and what we need. Step up and advocate for yourself. You can only do so much, but everything you do counts.

CENTRAL AMERICA AND CARIBBEAN

Region Member Organizations:

Costa Rica · Cuba · Dominican Republic · El Salvador · Guatemala · Panama · Puerto Rico

2023 HIGHLIGHTS



Central America

Pharmaceutical companies started the process to bring medicines to countries in this area.

PUERTO RICO

New HAE Law developed!

Patient rights guaranteed

Security in access to medicines

1ST HEALTH MINISTER HAE ADVISORY GROUP

A specific program to support HAE patients will be developed

MEETING WITH THE VICE MINISTER OF HEALTH

- **HAE is now on the agenda**
- **Future benefits for patients possible**

1ST ACCESS TO MODERN MEDICINE

Two medicines available

Result of meetings and continued advocacy with the Health Minister and Government Officials.

HOPES FOR 2024

JAVIER SANTANA

- We hope to continue identifying patients in countries in the region and to identifying doctors who can help them. We want to work together; patients, doctors, RPA, and HAEi.
- We will continue with the efforts towards governments and pharmaceutical representatives so that HAE treatments are secured in countries where they have not yet arrived.
- We will promote more programs to increase HAE awareness.

ACARE
Angioedema Centers of Reference and Excellence
A GA²LEN | HAEi NETWORK

ALL ABOUT ACARE

Introducing our new HAEi Coordinator, ACARE and an update on how accreditation benefits patients

Hello everyone. My name is Rachel. You might've met me. I've lived with HAE from a very young age, enduring unpredictable attacks and remaining undiagnosed during my childhood. I empathize with the emotions associated with living with a medical condition and the struggles that arise from a lack of sufficient support.

My long period without a diagnosis makes me passionate about better care. So, in August of this year, I was delighted to take on a new role as Coordinator, Global Advocacy and ACARE. But what is ACARE, and what could it mean for people like us with HAE?

ACARE, which stands for Angioedema Centers of Reference and Excellence, is a unique, equal partnership between HAEi and GA²LEN (Global Allergy and Asthma European Network). It aims to develop and accredit centers of great angioedema patient care, including HAE, fulfilling a longstanding HAEi goal of establishing a worldwide network of such centers. Both organizations work on developing ACARE, ensuring that it is an attractive partner for HAE treating physicians all over the world. Since its launch in 2019, ACARE has grown to 91 certified centers, with 13 more applicants undergoing certification across 37 countries worldwide. Ultimately, we believe ACARE will become an inclusive solution for everybody; wherever they live.

The role of Coordinator, ACARE is a bridge between HAEi and GA²LEN, ensuring that information flows smoothly and efficiently, disseminating updates, news, and opportunities and resources to help actively engage ACARE centers and members. By bringing together the largest network of specialists dedicated to treating angioedema globally, and HAEi, a global community of 99 Member Organizations, we can do more to reach and support people with HAE across the globe.

HAEi and GA²LEN share many objectives, including increased education about HAE. We are also dedicated to enabling individuals residing in more remote areas to gain access to a specialist center, vital HAE testing, and treatment options, consequently enhancing their quality of life.

I've been a part of the HAEi since 2014, including serving as a member of the Board of Directors. This new role excites me about opportunities to improve people's lives. I look forward to telling you more and sharing stories in the coming months. In the meantime, if you have any queries or ideas about ACARE, please don't hesitate to contact me at r.annals@haei.org

Rachel

ACARE ANSWERS

We asked ACARE centers from our Asia Pacific, Americas, and EMEA regions to tell us a little about their journey. This time, we hear from Professor Anete Grumach, University Centre FMABC Medical School in Brazil; Dr. Philip Li, Queen Mary Hospital, Hong Kong; and Ms. Lorena Lorenzo, Lead Immunology Clinical Nurse Specialist, from Bart's Health NHS Trust, United Kingdom.

Bart's Health NHS Trust

Questions answered by Ms. Lorena Lorenzo RN, INP, Lead Immunology Clinical Nurse Specialist from Bart's Health NHS Trust. Ms. Lorenzo holds a BSc Degree in Nursing and is a qualified Independent Nurse Prescriber. She authored the recently published Nurse Competencies for Nurse-led HAE and CSU clinics, which forms part of the Immunology Nursing Competency Framework for UK nurses.

What benefits have you experienced since becoming an accredited ACARE center? Being an ACARE member has opened the doors for collaborative opportunities with other accredited institutions, creating a network for sharing knowledge and best practices internationally. ACARE membership provides access to additional resources, including preceptorship training, podcasts, and webinars about recent improvements in diagnosis, management, and treatments in HAE and chronic spontaneous urticaria (CSU). This access can lead to advancements in treatment options and a better-equipped staff for a better patient experience. Being a member presents an opportunity to participate in several projects, audits, research, and development to improve patient care. As a center for reference and excellence, it promotes and encourages commitment to continuous improvement by staying updated with the latest research. And lastly, it comes with increased credibility and recognition in the global HAE community.

How has care improved for your patients since you became an accredited ACARE center? Since Bart's Health became an accredited center, we have adopted an enhanced patient-centered approach. We have incorporated the use of PROMs (patient-reported outcome measures) and specific questionnaires in our routine consultations to highlight patient's needs in relation to the burden of the disease and treatment. In this way, our patients take an active part in decision-making regarding the overall management of their condition, resulting in increased patient satisfaction.

What advice would you give to a hospital or clinic considering applying to become an ACARE center? I encourage aspiring centres to start by looking at the 32 ACARE standards. During this process, it will highlight the existing level and quality of service. The journey towards accreditation is an opportunity to look into the gaps in the service and identify areas for change. The accreditation exercise will encourage your team to work together to improve operational efficiency and processes. This can lead to more comprehensive treatment plans and better outcomes for patients. Ultimately, it will reassure you, knowing that compliance with the set standards provides a solid foundation for your service, ensuring that service is high-quality, safe, and ethical.

What has becoming an accredited ACARE center enabled you to achieve? As an ACARE center, we have established ourselves as the hub of high standards of practice and quality of service in managing HAE in the UK. It has expanded our patient cohort and research portfolio. Bart's Health Angioedema Center currently provides the largest HAE service and the only ACARE-accredited NHS service in the UK. We have approximately 200 patients (adult, adolescent, and pediatrics combined). We have ongoing clinical trials at national and international levels, including novel therapeutics. Our clinicians have leadership in clinical networks, patient organizations, and training. We can provide home therapy for all therapeutics within the specialty. The accreditation process has helped us streamline our operations, improve the quality of care, and build trust with patients and the HAE community. This further motivates us to improve our performance and patient safety.

University Center FMABC Medical School, Brazil

Questions answered by Professor Anete Sevciovic Grumach, Professor of Clinical Immunology and coordinator of the University's Center for Rare Diseases. Professor Grumach is also President of Honor and Founder of GEBRAEH, the Brazilian group of HAE researchers and physicians.

What benefits have you experienced since becoming an accredited ACARE center? What needs to be emphasized is the recognition of being capable of diagnosing and treating HAE patients. Additionally, as an ACARE, we have the opportunity to exchange experiences with other centers all over the world.

How has care improved for your patients since you became an accredited ACARE center? We have been working to provide modern therapies to our patients. Belonging to a group with the same aims helped us be representative and heard by our health entities.

What advice would you give to a hospital or clinic considering applying to become an ACARE center? I encourage all the HAE centers with a particular interest in collaborations to submit their centers to be an ACARE. Several projects are going on, and the opportunity to improve awareness and knowledge about angioedema is invaluable.

What has becoming an accredited ACARE center enabled you to achieve? It isn't easy to pinpoint one aspect. However, participating in preceptorships, specific symposia or discussions has certainly improved our support to patients. In addition, I am sure that including our Center in projects and clinical research represents a huge benefit to all of us.

Queen Mary Hospital, Hong Kong

Questions answered by Dr. Philip H Li, Specialist in Immunology and Allergy and Clinical Assistant Professor at the University of Hong Kong. Dr. Li serves as Chief of the Division of Rheumatology & Clinical Immunology of the Department of Medicine. He is also the Vice President of the Hong Kong Institute of Allergy and a Board Director of the Asia Pacific Association of Allergy, Asthma and Clinical Immunology.

What benefits have you experienced since becoming an accredited ACARE center? It has put us in close partnership and collaboration with other ACARE centers in Asia Pacific and beyond. It has also given our patients, staff, and peers more recognition and confidence. Becoming an ACARE is proof that our center really does strive to continuously improve ourselves when caring for our HAE patients.

How has care improved for your patients since you became an accredited ACARE center? Through collaboration with ACARE, we have worked with other centers to devise new strategies and share experiences in HAE management. One example is our recent publication - working with colleagues in India - of using high C1-INH levels as a screening method for Type II HAE patients!

What advice would you give to a hospital or clinic considering applying to become an ACARE center? The scale of the accreditation criteria might seem overwhelming at first, but it is worth the effort! The standards set to join ACARE helped push our center to improve further. Just the preparation and process of applying helped us take a step back to review our center's progress and achievements and restructure ourselves and our strategies.

What has becoming an accredited ACARE center enabled you to achieve? We have joined other ACARE centers in working towards an Asia Pacific HAE Consensus Statement, as well as establishing an upcoming new Asia-focused collaboration we call: 'HAE-ASIA (Angioedema Screening Initiative in Asia).'

TALKING TO ...

Quick Fire Questions in HAE

Welcome to a new, occasional feature of Global Perspectives, where we hear from some of the key people in HAE today to ask them what's really happening.

We met Professor Marcus Maurer and Professor Bruce Zuraw during the **2023 HAEi Regional Conference EMEA**. We asked them what impact they hope to have, what their hopes for the future are, and what barriers they see to further progress.

We start with Professor Maurer.

What impact do you want your words to have on people with HAE, their caregivers, and fellow healthcare professionals?
I want my words to tell everyone that we have to address disparities. We have to go and help those patients who live in settings where improvements in care and treatment options are key.

Ultimately, we want people to forget they have HAE. We want them to think: 'This is the party I will go to. This is the vacation I will take. This is the business trip I will be on.' All without the looming fear that an attack at the wrong time may make all of this impossible. Complete control means the patient is in control. They are the boss of their life, not their disease.

> They are the boss of their life, not their disease.

We hear a lot about the success of ACARE (Angioedema Centers of Reference and Excellence). Can you elaborate on how ACARE and accredited centers benefit people with HAE?
There are 32 criteria that centers need to complete to be in ACARE. One is that every center commits to patient and physician education and awareness raising.

As a result, ACAREs are contributing much more to patient education. The Middle East ACAREs in Oman, Abu Dhabi, and Kuwait are great examples. Because patients now know that expert care is close to them, they are coming forward. We have newly diagnosed entire families as a result.

Clinical trials are another vital way patients benefit from the ACARE accreditation process. Clinical trials offer modern treatments to patients who otherwise don't have access to them. Within the ACARE criteria is the ability and desire to do clinical trials. These centers are basically 'pre-vetted.' We know they can do it, which means patients at these centers already have increased opportunities to get on a trial. We now need to get trials in new centers, as there are still many countries where there are currently no trials.

Every center coming on board pledges to use patient-reported outcome measures as routine tools for all of their patients. As a result, we see that quality of care improves and is more finely tuned. In contrast, without these tools, it's difficult to say how well-controlled the disease is in individual patients and what the disease activity is.

As we reach the end of the year, what gives you a sense that there is a brighter future?
Thanks to the partnership between GA²LEN and HAEi, access to care will become available to many more patients. This includes expert care, expert tools, and modern treatments.

To make global improvements, we must look at a global patient population and work with ACAREs globally. Our partnership with HAEi means this is more possible than ever.

And is there one thing that concerns you?
Complacency. All of us know that we have to keep at it and let people know that this is a horrible disease. Never give up must be the mantra.

> All of us know that we have to keep at it and let people know that this is a horrible disease. Never give up must be the mantra.

Next, we hear from Professor Bruce Zuraw.

What impact do you hope to have on people with HAE, their caregivers, and fellow healthcare professionals?
I want people to have the tools to know what's right for them and advocate for themselves. I want to help people to have agency. In other words, to take control of their disease and ensure they're getting the right care.

How important is it for experts like yourself to reach out and educate patients and caregivers?
Mostly, what I can do is education: the ability to explain what HAE is, what the options are, and what the science suggests the future might hold.

> I take education very seriously, as without it, the best medications in the world aren't going to have their desired effects.

I take education very seriously, as without it, the best medications in the world aren't going to have their desired effects. HAE is always a series of decisions. What do you do when this happens? How might you prevent it? How do you prepare? You have to have knowledge to do a good job of caring for yourself, especially when your doctor is not at your side.

> You have to have knowledge to do a good job of caring for yourself, especially when your doctor is not at your side.

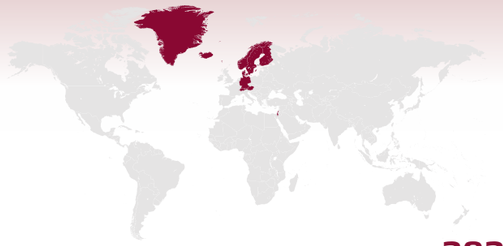
What do you learn from attending events like the 2023 HAEi Regional Conference EMEA?
I learn what's on people's minds and see the situation from a different perspective. Seeing the patients gather, hearing their stories, and wanting to help is a huge motivation. I want to continue, to push harder, and to achieve more.

What's your one hope for the future?
My hope for the future is that every HAE patient around the world lives a normal life free from the fact that they were born with HAE. I want HAE to become like your middle name. It's just part of you. But it doesn't influence how you live your life.

> I want HAE to become like your middle name. It's just part of you. But it doesn't influence how you live your life.

What's the one thing that stands in the way of that?
Lack of knowledge is one; the other is money. As you truly understand the disease and what the treatments do, it turns out that it is more expensive to ignore it and not treat it well than to treat it. It can be hard to get people who don't understand the problem to grasp that.

NORDICS, GERMANY, AUSTRIA, SWITZERLAND, AND ISRAEL



Region Member Organizations:

Austria · Denmark · Finland · Germany · Iceland · Israel · Norway · Sweden · Switzerland

2023 HIGHLIGHTS

PHARMACO AND SOCIOECONOMIC SURVEY LAUNCHED IN DACH

The data collected will significantly bolster our arguments for improved treatment options for people with HAE in these countries.

HAEi LEAP

HAEi LEAP PROGRAM GRADUATE AND PROJECT DELIVERY

HAE ON FILM & IN PODCASTS

- Understanding
- Dialogue about HAE

MEMBER ORGANIZATION MEETING

- Networking
- Insights
- Connections
- Engagement
- Advocacy efforts

2023 HAEi Regional Conference EMEA

8 of 9 countries in the region participated

KNOWLEDGE → CONNECTIONS → ADVOCACY

HOPES FOR 2024

JØRN SCHULTZ-BOYSEN

To aim for an even higher quality of life for individuals with HAE and their families, while ensuring ongoing access to and reimbursement for modern HAE medications. I look forward to witnessing the remarkable HAE communities in various countries, led with notable advocacy and empathy for those affected by HAE and their families. Lastly, I'm excited about many 2024 HAEi activities: **hae day :-)**, the **2024 HAEi Global Leadership Workshop** and HAEi LEAP, to name only a few, and to see how countries in my region will make the most of these opportunities.

HAEi GLOBAL LEADERSHIP WORKSHOP
COPENHAGEN
3-6 OCT 2024

SAVE THE DATE!

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

2024 HAEi Global Leadership Workshop
(covering all HAEi member countries)

3-6 October 2024 in Copenhagen, Denmark

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

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

Did You Know?

ALL ABOUT WOMEN WITH HAE: PUBERTY, PREGNANCY AND PEDIATRICS

Medical science is finally beginning to appreciate the unique ways women experience health conditions. Whether it is the way women experience pain differently, through to genetic illnesses like HAE, it is vital to have tailored advice and knowledge for women. For that reason, and with thanks to the US Hereditary Angioedema Association (US HAEA), HAEi will be introducing a brand-new guide called *Women With HAE*.

The guide focuses on the unique challenges faced by women with HAE, especially as studies indicate that women experience more severe symptoms than men. *Women With HAE* covers three life stages: childhood, family planning and pregnancy, and menopause and aging.

We'll launch the guide early in 2024, so look out for more news then.

Earlier this year, world-renowned HAE expert Professor Henriette Farkas spoke at the **2023 HAEi Regional Conference EMEA** about pregnancy and HAE. Prof. Farkas is Honorary President and Director of the Hungarian Angioedema Center at Semmelweis University in Hungary, and we took the opportunity to ask her more.

What do prospective parents with HAE need to know?

Having children is always an important and wonderful event in people's lives. In all cases, I encourage patients to have children.

Because of the way HAE is inherited, we should inform prospective parents that there is a 50% chance that their offspring will inherit the disease. At the same time, we must reassure parents that the disease is manageable, does not progress, and does not cause organ damage. Their children can live a normal life.

In people with HAE, there seems to be a lot of understandable fears about becoming pregnant. What are the risks, and what can be done to address these fears?

Family planning is a crucial issue. Thinking ahead about conception is recommended. If a woman does want to become pregnant, any androgen treatment should be discontinued because these drugs may cause masculinization of a female fetus. We can advise that the course of HAE may worsen during pregnancy, the postpartum period, and breastfeeding (and it may not). Plasma-derived C1-INH concentrate is the first line treatment during these times.

How can the healthcare professional community best support people with HAE who want children?

We can do this by providing information about possible changes in HAE and stressing the importance of family planning, working out a therapeutic plan, continuous follow-up while working closely with the obstetrician/gynecologist and providing ongoing consultation.

What does the future look like for children born with HAE in the coming years?

The future is bright. We can treat children with HAE with many drugs that are currently available. Several new medicinal products are being developed or evaluated in clinical trials. The introduction of oral medications for acute/ rescue treatment and prevention of HAE attacks, as well as long-acting medications for prophylaxis, will be a significant step forward.

How might genetic advances change the way people with HAE think about the prospect of having children?

The revolutionary progress of molecular genetics provides opportunities to diagnose and treat hereditary disorders like HAE. With this, we have a chance to discover new HAE types, to have a diagnosis during pregnancy (prenatal) or during IVF before the embryo is implanted in the uterus (preimplantation), and to examine young babies born to HAE patients through umbilical cord blood.

Using genetic methods, changing the defective gene to a healthy one, or eliminating a gene that causes disease could mean a long-term therapeutic solution. I do not know of any other rare disease where so many medications with different mechanisms of action and administration routes have been developed. I find this fantastic and really appreciate the work of drug-developing colleagues and companies.

> I do not know of any other rare disease where so many medications with different mechanisms of action and administration routes have been developed. I find this fantastic and really appreciate the work of drug-developing colleagues and companies.

MEDITERRANEAN, NORTH AFRICA, AND BRITISH ISLES

Region Member Organizations:

Algeria · France · Ireland · Italy · Libya · Morocco · Portugal · Spain · Tunisia · United Kingdom

2023 HIGHLIGHTS

8TH ASID PID CONGRESS FOR HEALTHCARE PROFESSIONALS

- Overview of HAE in the region presented
- HAEi point of contact established
- Emergency Room poster distributed in English, Arabic and French

HAE ITALY (A.A.E.E)

ITACA NATIONAL MEETING

- HAEi tools and resources presented
- HCP and patient relationships developed

1ST HAE Tunisia (ANAOH)

GENERAL ASSEMBLY HAE TUNISIA

- Main association roles allocated
- Patient identification card issued

NEW MEMBER ORGANIZATION LEAD

- Activity
- Relationships
- Awareness

1ST HAE PATIENT WORKSHOP

- Experiences shared
- Relationships developed

HOPES FOR 2024

MARIA FERRON

All patients in the countries of my region have access to lifesaving therapies, they can live their lives without fear and with the best standard of quality of life.

CENTRAL EASTERN EUROPE, BENELUX, AND MIDDLE EAST

Region Member Organizations:

Armenia · Bahrain · Belarus · Belgium · Czech Republic · Egypt · Estonia · Georgia · Hungary · Iran · Iraq · Jordan · Kazakhstan · Kuwait · Latvia · Lebanon · Lithuania · Netherlands · Oman · Poland · Qatar · Russia · Saudi Arabia · Slovakia · Syria · Ukraine · United Arab Emirates

2023 HIGHLIGHTS

2023 HAEi Regional Conference EMEA

18 of 27 countries in the region participated

NETWORKING → ENGAGEMENT → WE ARE NOT ALONE → AWARENESS

POLAND YOUNGSTERS' SUMMER WORKSHOP

6 PATIENT AND HCP MEETINGS

- shared experiences
- networking
- understanding of the power of patient advocacy

NEW PATIENT LEAD FOR ADVOCACY

Motivation is high to make a difference in 2024.

1ST HAE therapy included on the List of Essential Medicines pdC1-INH reimbursed from 2024

The result of 12 years of advocacy activity of the Member Organization.

HOPES FOR 2024

MICHAL RUTKOWSKI

- To help Member Organizations (MOs) in my regions to grow.
- To encourage more MOs and patients to use our resources, such as HAEi Connect, HAEi Advocacy Academy, **HAE TrackR** and the Understanding HAE patient guide.
- To support HAE Clinical Trials and to help patients understand the importance of taking part, and encourage clinical trials to be run in more countries.
- To support different hospitals/clinics to become members of the ACARE network.
- To help set up 1-2 new HAEi MOs from my regions and have representatives of all MOs from my regions present at the **2024 HAEi Global Leadership Workshop**.

Youngsters Community Review and Round-Up

LOOKING BACK AT 2023 AND FORWARD TO 2024

Hello everyone

What a year! The Youngsters' Community goes from strength to strength. Whether we are in person at the Youngsters' Track of the **2023 HAEi Regional Conference APAC** or **EMEA**, or staying connected through our awesome online resources, there's never been more ways to get up-to-speed and be supported.

As I look back on the year, I'm grateful for all the hard work, proud of our achievements, and in awe of the strength of all our young people.

This piece reflects on the year in more detail. We hear from three Member Organizations about their experiences working with their LEAP 2023 graduates, and three members of our Youngsters' Community Advisory Group tell us about their highlights.

We also look forward. There's huge excitement as LEAP 2024 is soon to start. We've selected a class from more than 20 fantastic applications that came in from across the world. We'll share more information soon, but you can follow the LEAP journey at our dedicated website: https://youngsters.haei.org/.

If you want to send a message to loved ones or friends, why not use our inspiring community postcards? https://youngsters.haei.org/community-postcards/

We'll be back in the new year and looking forward to seeing as many of you as possible in Panama at the **2024 HAEi Regional Conference Americas**.

Until then, the entire Youngsters' Community wishes you a wonderful, restful, festive season.

See you soon!

Nevena

Still haven't joined the HAEi Youngsters' Community?

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

IN THEIR OWN WORDS

Here, two of the Youngsters' Community Advisory Group give us their reflections on 2023 and share their excitement about what 2024 has in store. We hear from Dominika Blacharska, HAE Poland; and Kamila Moran Salaverry, HAE Peru.

How do you feel 2023 has been for the HAEi Youngsters' Community?

Dominika: 2023 has been a great year for the Youngsters' Community. I'm very proud of all the work we have done. Because of the conference in Munich, we have grown a lot! It was an amazing experience to see so many youngsters joining our family. For some Youngsters, it was their first time at a meeting, and it's really fantastic that they quickly made a connection and had the feeling of being understood.

Kamila: For me, it was very special to have the opportunity to meet others and learn about the different stories of the community. It has been a memorable year in which we were able to share projects and establish ideas for new goals and plans that will come in the future, so stay tuned!

What projects were you involved in this year?

Dominika: One project I participated in was an awareness month, where we created posts on our Instagram. It was a great opportunity to tell youngsters about the most important things in HAE. For example, we made a post for caregivers with advice on dos and don'ts with HAE.

Earlier this year, I took part in the LEAP Program. It was the best experience of my life! I could learn what and how to do something for youngsters in my country. Because of LEAP, I was able to make the first Summer Camp for Polish Youngsters. We also hosted our Youngsters' Track at the **2023 HAEi Regional Conference EMEA**! It was very stressful but such a proud moment!

Kamila: It made me very happy to be able to help with some projects for the advisory group. One was during the awareness month when I shared posts for "a week of our lives," there was the **2023 HAEi Regional Conference EMEA**, and without a doubt, one of my favorites was the "Youngsters' Spotlight" that we did in March. It was a nice way to learn more about community members around the world.

Compared to 2022, how do you feel about the Youngsters' Community in 2023?

Dominika: In 2023, the Youngsters' Community definitely got bigger with more youngsters, more projects, more engagement, and more memories! It was an important year because we could finally meet in person! Looking back on 2022, when we had online meetings, hugging the person (at last!) was amazing! We are happy to finish this year by launching our first-ever Social Media Internship Program. If you are passionate about social media, building a community, or want to make a positive impact – think about applying! You can find more information here: https://youngsters.haei.org/social-media-internship/

Kamila: Having the opportunity to join the group this year helped me to share the message of caregivers and how important it is to take care of our closeness to the patient. Even though I've only been a member for a short time, I can say that it has been incredible and even better than I expected. I feel grateful to Nevena and the other group members for giving me such a warm welcome. I've got the confidence to share my ideas, and I don't feel worried about asking for help or guidance in our activities. It's an amazing group!

What are you most excited about for 2024?

Dominika: I can't wait to see what 2024 brings. We have many ideas and even more amazing Youngsters who want to get involved. 2024 is our year!

Kamila: I am excited as a new year means the opportunity for new members to join the advisory group, to be able to listen to their ideas and share experiences ;)

LEAP CLASS OF 2023:

Projects with Member Organizations

Our successful LEAP 2023 Graduates are now well into the project phase of the program. Thanks to their hard work, a wide range of programs are underway to put what they learned into practice and support Member Organizations and local HAE communities. Three countries the US, Scandinavia, and Poland, tell us more about the projects and, where they are the MO Lead, gave us their impressions of what it was like to work with LEAP.

UNITED STATES

Lisa Facciolla is the HAE Advocate and Community Engagement Specialist at the US HAEA. The organization had two students accepted into LEAP 2023, and their projects are complete. Lisa tells us more.

"Overall, the process was very smooth. Once the students were accepted, we met, reviewed expectations, and set a timeline with milestones for their progress. After their training in Dubai, we scheduled check-in meetings to stay updated on their progress and reassure them that they had my support throughout the process.

Both students developed written support resources for different community groups. When complete, we worked on a communication and distribution plan to promote their newly created resources. We also include them in the care packages we send out to our community.

Overall, we're delighted that Ally and Jasmeen took part in LEAP. It gave us a chance to work at a closer level on a meaningful project. Everything was great. It's been a pleasure to work with them on their projects."

Ally crafted a caregivers' guide
https://bit.ly/leap-usa-ally

Jasmeen created a youth advocacy guide
https://bit.ly/leap-usa-jasmeen

SCANDINAVIA

Victoria Schultz-Boysen successfully graduated from LEAP in 2023 and is working on her project with the HAE Scandinavia organization. Victoria gives us an update on her project.

"My project is called 'Sums Up 👍' and is a progressive web app for patients and caregivers in Scandinavia. The philosophy behind the app is to provide a sense that you always have a friend in your pocket – someone who gives good advice, makes you smile and laugh, is honest, shares experiences, and makes you feel you are not alone. We have created a visual universe where content is creative through images, videos, and podcasts. We recently teased the project at the HAE Scandinavia conference in November 2023. The app is available in Danish, Swedish, Norwegian, and English, and it will be launched in 2024. Balancing university, life, work, and the LEAP project has been tough at times, but the project is close to my heart, and I am excited to see it come to life. My organization has supported me all the way and I want to say a big thank you to the fantastic HAEi LEAP team, Nevena and Debs for being supportive, advisory, and highly skilled throughout the journey."

LETNIE WARSZTATY HAE

POLAND

Michal Rutkowski is the President of HAE Poland. One youngster from Poland took part in LEAP 2023 and delivered their project - the first HAE Poland Youngsters' Workshop - earlier this year. Michal tells us more about how it went.

"I have known some of our young people for a long time, and it's fantastic to see how our Poland LEAP student, Dominika, has grown as an advocate. After the success of her project, Dominika is now the supervisor of the HAE Poland Youngsters' Community and is in contact with our younger members. LEAP is a wonderful opportunity, and I'm delighted that two more of our Youngsters have applied to be a part of LEAP 2024. Our goal is to create a vibrant Youngsters' Community in Poland. LEAP helps us do this by supporting our young people with life skills and advocacy skills."

SOUTH EASTERN EUROPE

Region Member Organizations:

Albania · Bosnia & Herzegovina · Bulgaria · Croatia · Cyprus · Greece · Kosovo · Montenegro · North Macedonia · Romania · Serbia · Slovenia · Turkey

2023 HIGHLIGHTS

1ST JOINT MEETING

- Improved communication and HAE knowledge
- Increase in members
- Physician collaboration
- Strengthened advocacy activities

- Emergency Room Poster
- Understanding HAE Booklet
- HAE Facts Social Media Cards

Resources available to MOs are attractive and requested for use

HAE KOSOVO NEW MO

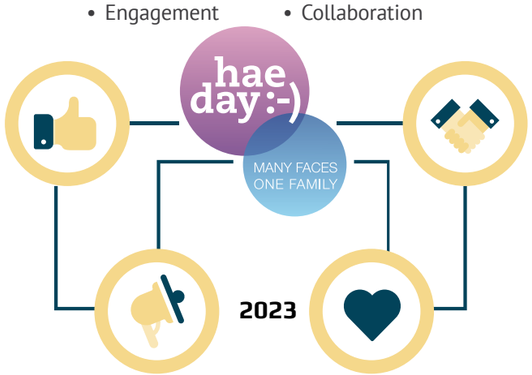
#97

The first HAE patient is officially diagnosed and added to the rare disease patient registry. There is more HAE awareness among physicians in Kosovo.

HAE DAY :-)

Patients, physicians, caregivers, and friends join together and celebrate hae day :-)

- Awareness
- Engagement
- Strength
- Collaboration



2023 HAEi Regional Conference EMEA

11 of 13 countries in the region participated

MOTIVATION

COOPERATION

ADVOCACY!

HOPES FOR 2024

NATASA ANGJLESKA

- To see modern treatment options available to HAE patients in ALL countries.
- To help more countries have access to preventative therapies according to individual needs.
- To organize an HAE meeting for all SEE countries, in Skopje, North Macedonia.
- To build stronger patient/caregiver communities advocating for improved quality of life for people with HAE.
- To help two more patient organizations officially register in their countries.
- To increase the use of available HAEi resources in all countries in my region.

SUB SAHARA AFRICA

Region Member Organizations:

Comoros · Kenya · Mozambique

2023 HIGHLIGHTS

HAE MOZAMBIQUE

#98

HAE advocacy has helped identify more patients in these countries and awareness campaigns amongst healthcare professionals have been initiated.

2 new HAEi Member Organizations

HAE COMOROS

#99

Zimbabwe HCP survey identified:

NEW HAE PATIENTS IDENTIFIED

Resources available to MOs are attractive and requested for use

- HAEi hosted websites
- HAE TrackR app

2023 HAEi Regional Conference EMEA

7 countries in the region participated

AWARENESS ACTIVITIES

HAE UNDERSTANDING

DOCTORS' KNOWLEDGE

HOPES FOR 2024

PATRICIA KARANI

To significantly enhance awareness amongst healthcare providers in the region and increase patient knowledge on available resources and medications for HAE. It is important to help patients eradicate their fears about living with HAE and let them know that they are not alone in their journey. We do this by bringing them into our larger HAEi family of people also dealing with the condition.

HAEI RESEARCH IN 2023

Debs Corcoran, Chief Scientific Officer

It has been a research and science-filled year for HAEi. We've been active with our research programs, have had more inquiries about our methodologies than ever before, delivered very successful scientific tracks at our Regional Conferences, and convened a unique healthcare professional Symposium.

Here, we focus on some highlights from 2023, including a first look into the HAEA/HAEi sponsored HAE with Normal C1-INH Symposium.

HAEi Research Methodologies: Baseline Burden of Illness

Our Burden of Illness survey has been conducted in Mexico and South Africa. This study provides data that opens the door to approaching health ministries with real-world evidence of a catastrophic unmet medical need.

Dr. Sandra Nieto from HAE Mexico has used the results of their Burden of Illness survey with great effect. Firstly, in a presentation to the Latin America Society for Immunodeficiencies (LASID) meeting in October in Mexico City. More recently, Dr. Nieto presented the data to the Congress of Deputies as part of an HAE Mexico event. The event was a great success, resulting in the Congress of Deputies declaring 16 May as National Hereditary Angioedema Day and agreeing on other important benefits for HAE patients.

Dr. Sandra Nieto said, "The study results mean we have a clear picture of the burden of people living with HAE in Mexico. We have been able to shine a light on this to doctors and, most importantly, to our Congress of Deputies, which makes decisions about healthcare in Mexico. The Burden of Illness study was taken into account by the Congress of Deputies when making their decisions."

For HAE South Africa, the final results from the Burden of Illness study were used to prepare an abstract submitted for the Africa Rare-X Conference in February 2024. Rare-X is Africa's leading conference on rare disease treatment and access, and brings together rare disease patients, dedicated healthcare professionals, and invested stakeholders. The abstract has been accepted, and Janice Strydom, CEO of HAE South Africa, will give an oral presentation at the conference.

Janice comments, "These results give us the baseline of the burden of HAE in South Africa right now. Before we had the results, we had a feel for what was happening, but now we have real-life numbers. The data helps show us where to target future awareness and education activities for the biggest impact. We're delighted to be a part of the Rare-X Conference and share the experience of people with HAE in South Africa. To be accepted as oral, rather than poster, is an honor."

HAEi Research Methodologies: Heat Map

HAEi designed our Heat Map Survey methodology for countries where there are few, if any, people diagnosed with HAE. The objective is to identify "pockets" of potential HAE so that we can pinpoint areas to concentrate HAE education and awareness campaigns. We have been working with several countries on the potential to implement Heat Map, and mention one example here.

Along with Michal Rutkowski, Regional Patient Advocate for Central Eastern Europe, Benelux, and the Middle East, we are working with Dr. Elham Hossny from Egypt to pilot the Heat Map project within her medical society in Egypt. There are currently only 44 people identified with HAE in Egypt, a country with a population of 110 million.

HAEi Research Methodologies: Pharmaco, socioeconomic, and quality of life assessment

These studies are targeted at countries that already have access to modern HAE therapies. The data collected can be used with Health Ministries to advocate for continued access to modern HAE therapies.

In 2023, this study was run in Australia and New Zealand. In New Zealand, patients do not have access to prophylaxis; HAE Australasia can use the data to show the Government the benefits of funding modern preventative therapies. In addition, this study is currently being run in Germany, Austria, and Switzerland.

The HAEA/HAEi HAE with Normal C1-INH Symposium 2023

The US HAEA and HAEi realized that a decade had passed since the last supported symposium on the subject. With Co-Chairs Prof. Bruce Zuraw, Prof. Konrad Bork, and Prof. Marcus Maurer, the HAEA/HAEi team convened a 1-day symposium before the **2023 HAEi Regional Conference EMEA** with the ambition of producing a new HAE with normal C1-INH scientific publication covering the latest science regarding this important subset of the HAE population.

At the start of the symposium, Anthony J. Castaldo, HAEi Chief Executive Officer and Chairman of the Board, thanked all the assembled experts, especially the three Co-Chairs, for agreeing to participate. He told the healthcare professionals present, "Well over a decade ago, the US HAEA began seeing a consistent number of patients joining after receiving a diagnosis of what was then called HAE Type-III. At that time, US HAEA had no way to answer the many questions we received from this subset of patients. Since then, thanks to HAEA and HAEi, multiple scientific meetings have addressed this lack of knowledge, and this new session will further advance the science to help this important subset of patients."

The project involves 31 HAE healthcare professional experts from 16 countries worldwide and is split into three task forces. Three critical areas of science were covered:

- Clinical features, diagnosis, differential diagnosis
- Genomics, biomarkers, and pathophysiology
- Management and treatment

Each task force is writing its own section, and the Co-Chairs will combine them into one manuscript. We hope to submit it for publication in 2024.

HAEI HELPS YOU SECURE DATA TO SUPPORT ADVOCACY FOR MODERN THERAPY AND IMPROVED QUALITY OF LIFE

HAEi conducts targeted, scientific-based research to provide data that our member organizations can use in their advocacy efforts to convince Health Ministries to approve HAE therapies.

HAEi has core methodological approaches for patient-centric HAE studies that we conduct for our member organizations.

>> Read more: https://haei.org/resources/advocacy/research/

Countdown to **hae day :-)** 2024

If it isn't in your diary already, mark 16 May 2024 for **hae day :-)**

This is our annual celebration of the HAE community and our focus on awareness-raising efforts. We are already thinking about the global campaign for next year. It will involve time spent on physical and well-being activities converted to steps - and more details will be available in early 2024.

In the meantime, our **hae day :-)** website has lots of practical information to help you plan your media engagement, community involvement, or social media, as well as lots of inspiration as you start to plan your **hae day :-)** activities – have a look at https://haeday.org/

Our Member Organizations (MO) raise the bar with their activities' creativity and their members' motivation. Here, we caught up with several countries to hear more about what **hae day :-)** means for them and why you should consider getting involved (if you don't already).

hae day :-) in a few words

What does **hae day :-)** help you achieve, that you can't do at any other time of year?

"It's easier to talk to people or raise awareness because there is a global awareness day, and activities are happening worldwide. It acts as a focus and boosts our influence when talking about HAE. As it is a global date, we can get people to help us raise awareness about HAE. hae day :-) helps us meet with our patients and their families, get to know each other, and share our experiences of living with HAE. Come together to commemorate our day."

Carla M. Goachet Boulanger, President, Asociación de Pacientes con Angioedema Hereditario del Perú (HAE Peru)

"**hae day :-)** helps us provide context where we can talk about HAE and raise awareness. We've traditionally celebrated **hae day :-)** and are now known for it. On the last **hae day :-)** media representatives contacted our MO advocates to inquire about our plans before HAE Macedonia announced anything. It felt good knowing that they have **hae day :-)** marked on their calendars."

Natasha Jovanovska Popovska, President, ХАЕ МАКЕДОНИЈА (HAE Macedonia)

Why should Member Organizations consider getting involved in **hae day :-)**?

hae day :-)

MANY FACES ONE FAMILY

"Participating in **hae day :-)** offers MOs a unique opportunity to amplify their impact. Organizing activities, large or small, can enable patient organizations to reach a wider audience, educate the public, and create a stronger sense of community among those affected by HAE. It is an opportunity to raise awareness, inspire support, and make a lasting impression on the individuals living with HAE who can contribute to positive change. Join **hae day :-)** activities to expand our organization's reach, discover more patients, and improve the treatment environment for patients around the world!"

Soojin Min, President,
유전성혈관부종환우회 (HAE Korea)

"For those not promoting **hae day :-)** you are missing a huge opportunity to bring your community together, which spurs change. We will be stronger with you; the more organizations that participate, the stronger our collective voice. It is a fun way to advocate, educate people, and engage your broader community - the more we get involved, the more we can improve HAE!"

Members of Board, HAE Canada

EASTERN ASIA

Region Member Organizations:

China · Hong Kong · Indonesia · Malaysia · Singapore · Taiwan

2023 HIGHLIGHTS

NEW EASTERN ASIA RPA APPOINTED

Increased

- HAEi representation at local meetings
- HAE patient voice
- Communication as Yong Hao speaks Mandarin

2 MEDICAL JOURNAL PUBLICATIONS ON HAE IN THE REGION

HAE HONG KONG OFFICIALLY REGISTERED

HAE Hong Kong can now raise funds and donations to support their activities.

1ST HAE MEETING

HAE Malaysia and physicians committed to improving the lives of people with HAE in Malaysia

2023 HAEi Regional Conference APAC

5 of 6 countries in the region participated

Increased

- INTEREST IN ADVOCACY
- CONNECTIONS
- PHYSICIAN COLLABORATIONS

HOPES FOR 2024

LIM YONG HAO

I hope to facilitate and coordinate the collection of high-quality data to:

1. better understand the experience of HAE patients,
2. demonstrate the impact of advocacy work,
3. identify areas for improvement, and
4. engage relevant stakeholders to improve the lives of HAE patients.

NEWS AND 2023 HIGHLIGHTS FROM HAEi COUNTRIES AROUND THE GLOBE

AUSTRIA

From Christian Müllner, HAE Austria

Report from the Annual Meeting in Graz on 30 September, 2023, at Hotel Weitzer.

Our chairperson, Heidi Huemer, welcomed 35 participants (patients, doctors, medical professionals, pharmaceutical representatives) to our annual meeting. In her introductory speech, she talked about the numerous tasks of our self-help group aimed at improving the quality of life for patients and their families:

- Information about treatment options, current research status, HAE experts, and HAE centers in Austria, new competence centers.
- Our telephone hotline 0677/63189739.
- Self-help in adjusting to life with HAE, exchange of experiences among those affected, networking of institutions helpful for HAE, and international cooperation with HAEi.
- Our public relations work in various media and social media at professional events.

During a year in review, Heidi provided numerous insights into our activities with a focus on Rare Disease Day on 28 February and hae day :-) on 16 May. Particularly important for us were the 2022 HAEi Global Leadership Workshop in Frankfurt in October 2022 and the **2023 HAEi Regional Conference EMEA** in Munich in early September 2023.

Prof. Aberer, one of the world's leading HAE experts, lectured on the history of HAE, the various types of HAE, and HAE in the past, present, and future. Of course, the development of treatment options was particularly important to us.

In the lecture by our board member Dr. Clemens Schöffl, the focus was on the challenges and fears in the daily life of HAE patients, at work, in leisure, during travels, and in family planning. He presented numerous study results on this topic.

Prof. Kinaciyan from AKH Vienna discussed new HAE therapies and developments. She also presented new studies conducted in Vienna, Graz, and Linz. She vividly demonstrated how these new medications intervene in the onset of an HAE attack and thus stop this cascade. These new therapies sound very promising.

At almost every meeting, there was an injection workshop with Health Care at Home and nurses from CSL-Behring, where participants could refresh and update their knowledge of self-administration or learn it for the first time, and as always, it was very well received.

In another workshop with Rene Schipits, there were interesting discussions on the topics of resilience and managing daily life. It was evident that, for many, there was a great need to talk about these issues.

During a joint lunch, further experiences were shared. The event concluded with a nice stroll through the city.

It was an extremely successful event with many interesting topics in a great setting.

BELARUS

From Viktar Lebedz, HAE Belarus

A year ago, in December 2022, updated official protocols for treating HAE attacks were approved. For the first time, C1 inhibitor drugs and lanadelumab were included in the treatment guidelines.

Starting from January 1, 2024, the updated document "List of Essential Medicines" comes into effect, which includes the C1 inhibitor drug CINRYZE®. The inclusion of CINRYZE® in this list means that patients with HAE can be provided with the medication at the expense of state funds.

Access to Effective HAE Medicine: Currently, in Belarus, there are two effective HAE medications registered: CINRYZE® and TAKHZYRO®.

Since 2020, purchases of the C1 inhibitor drug have been regularly made for pediatric patients with HAE: Berinert (2020, 2021), CINRYZE® (2022, 2023).

Starting in 2022, three adult HAE patients receive TAKHZYRO® prophylactically (monthly). The treatment is planned to be continued in 2024.

In 2023, a one-time purchase of CINRYZE® (a few doses for each patient) was made for two adult patients with HAE.

BRAZIL

From Raquel de Oliveira Martins, HAE Brazil (Abranghe)

Public hearing about HAE: HAE Brazil, Abranghe, was invited to participate in the public hearing at the Health Committee of the Chamber of Deputies on 16 May 2023 to debate access to treatment for hereditary angioedema.

At the Health Commission hearing, experts and diagnosed patients explained details about the disease. The date of 16 May was chosen for the debate as it is **hae day :-)** – the international awareness day for hae.

HAE Brazil has existed since 2010 with the main objective of demonstrating that HAE patients are not alone.

Watch the hearing on the Chamber of Deputies YouTube channel: https://bit.ly/brazil-gp3-23

Read more on the Chamber of Deputies website: https://bit.ly/camara-br and https://bit.ly/camara-br2

Access to new treatments: CONITEC (the body responsible for recommending the incorporation of the medicine into the list of the Basic Health System (SUS) has recommended the incorporation of intravenous C1 esterase inhibitor (Berinert) and icatibant (Firazyr) for the treatment of HAE attacks, meaning that patients will have both medicines available from the government.

Brazilian Allergy and Immunology Congress took place in Maceió from 17-20 November. HAE Brazil participated, handed out leaflets to doctors, and registered 12 new doctors for the medical network.

New drug approval: The Collegiate Board of the National Supplementary Health Agency (ANS) approved the incorporation of the drug lanadelumab into the List of Mandatory Procedures. The medicine is indicated for long-term prevention in patients with hereditary angioedema (HAE) from the age of 12.

The proposal to incorporate the technology was submitted directly to the ANS, having gone through the Agency's evaluation processes, including broad social participation and careful technical analysis, which uses the Health Technology Assessment (ATS) methodology, similar to countries such as England, Canada, Australia, and Germany.

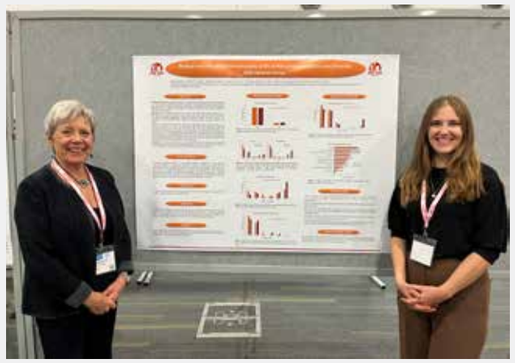
Lanadelumab will have mandatory coverage with the update of the Normative Resolution from 2 October, when it will be included in the List, in accordance with its usage guidelines.

CANADA

From Michelle Cooper (HAEC President and Ontario Regional Director), Jacquie Badiou, (Past President), and Daphne Dumbrille (COO), HAE Canada

In October, HAE Canada President, Michelle Cooper, along with COO, Daphne Dumbrille, were given the opportunity to attend BioCryst's first annual Patient Community Open House at their Discovery Center of Excellence Labs in Birmingham, Alabama. The open house brought Michelle and Daphne to their Lab, where they were treated to a guided tour, complete with a virtual reality experience to explain the molecules developed right there in the lab. Meeting BioCryst's staff, including the leadership team and scientists, allowed them to learn first-hand how BioCryst's patient-centered research truly drives the company.

Later in October, Michelle and the Atlantic Regional Director (Interim) joined Daphne in Ottawa to attend the Canadian Society of Allergy and Clinical Immunology (CSACI)'s Annual Scientific Meeting. Past President, Jacquie Badiou attended by taking advantage of the virtual option. HAE Canada presented a poster titled "Medical resource utilization and quality of life of HAE patients based on data from the 2020 national survey." During the conference, Michelle and Daphne were able to meet with fellow attendees, including representatives of some of HAE Canada's sponsors, as well as members of the Canadian HAE Network (CHAEN).



While in Ottawa for CSACI, HAE Canada board members and partners were pleased to attend the Canadian Allergy, Asthma and Immunology Foundation (CAAIF) fundraising gala at a table sponsored by the Canadian Angioedema Scholarship Program (CASP) foundation. CASP was founded by HAEC's Past President, Jacquie Badiou, along with Past Treasurer, Richard Badiou, and aims to fund research to find a cure for HAE and related angioedema. By attending CAAIF, we helped celebrate the first CAAIF-CASP HAE Research Grant. This grant was awarded to Dr. Stephen Betschel to help fund the Canadian Physicians HAE Practice Pattern survey, designed to better understand how physicians manage their HAE patients and learn of any potential care gaps.

On 4 November, HAE Canada hosted our second hybrid Patient Information Update. Patients, caregivers, family members, two HAEC Board members, Michelle and Kim Speiss (Central Regional Director), and Daphne gathered in Winnipeg to learn and meet fellow members of the HAE community. Kim began by presenting on HAE Canada's latest news and upcoming projects, and she then handed the microphone over to Stacy, who did an amazing job sharing her incredible patient journey. Attendees were treated to informative and helpful presentations from two local physicians. Dr. Nestor Cisneros discussed HAE and the burden of illness while also explaining the different available treatment options. Dr. Chrystyna Kalicinsky updated attendees on the latest clinical trials in Canada and the crucial research on HAE that is happening right here in Canada. Thank you to our members who joined us either in person (over 30 members!) or virtually, as well as our sponsor reps who were also able to attend.

Thank you, especially to our presenters who took the time out of their very busy schedules to spend the afternoon with us. We are extremely grateful that they shared their expertise and knowledge at our Patient Update. Finally, thank you to Heather Dow for providing IT support and allowing members across Canada to join us virtually.

Many HAE patients across the globe are fortunate to have access to Angioedema Centers of Reference and Excellence (ACARE), a network of clinics dedicated to educating medical professionals on angioedema. We are excited to report that the University of Alberta Hospital/Kaye Edmonton Clinic run by Dr. Bruce Ritchie and Dr. Adil Adatia recently received full accreditation status. Reviewers particularly noted the following strengths:

- a multidisciplinary team (physicians, nurses, social workers, and dentists),
- real-time blood product tracking,
- research productivity, and
- the electronic health system ConnectCare with myAHS direct patient connectivity.

It is clear Drs. Ritchie and Adatia are part of a committed and dedicated team who provide excellent care to their HAE patients. Congratulations to these amazing physicians.

For HAE Canada, fall means it's time for our Annual General Meeting (AGM). On November 14th, thanks to our wonderfully engaged membership, we held a successful virtual meeting that voted in our 2023-2024 Board of Directors. After the AGM, the Board then elected the executive positions as follows:

- Michelle Cooper: Ontario Regional Director/President
- Carmen Craciun: Director at Large/Treasurer & Secretary
- Kerstyn Lane: Pacific Regional Director/Vice President
- Jacquie Badiou: Director at Large/Past President
- Martine Paquette: Quebec Regional Director
- Kim Speiss: Central Regional Director

We are extremely pleased that these Board members are returning to lend HAE Canada their time, expertise, and enthusiasm to help ensure the success of our upcoming projects and events. Thank you to the Board for your dedication to HAE Canada; your contributions are invaluable.

HAE Canada is honored to be part of the I Am Number 12 campaign, organized and funded by Takeda Canada. This campaign brings together and elevates the voices of 12 individuals, or Changemakers, with different rare diseases from across Canada. The campaign includes patient journeys and beautiful portraits of each Changemaker in their hometowns. The campaign aims to increase awareness and highlight the importance of incorporating the Rare Disease Drug Strategy in Canada. We are extremely proud of Kerstyn Lane, HAEC's Pacific Regional Director and VP, who volunteered to be one of the campaign's Changemakers. Her powerful story is a wonderful contribution to this worthwhile campaign. Michelle was fortunate to attend the official launch of the campaign in Toronto on November 20th, 2023. Politicians from all political parties expressed support for a rare disease strategy, and there is currently a private members bill being tabled for the Province of Ontario to support its implementation. Michelle had the opportunity to meet the Parliamentary Assistant to the Minister of Health, Dawn Gallagher Murphy MPP, and will be following up with her about reimbursement and access to medication for HAE patients in Ontario.

Michelle and Kim will be attending the Canadian Organization for Rare Disorders (CORD)'s Fall Conference in person in Calgary, Alberta, on November 29-30, 2023. The theme of this conference is implementing a community-created Rare Disease Network and Drug Strategy across Canada. HAE Canada has been an active participant in CORD's advocacy efforts to implement a Rare Disease strategy.

To end this final newsletter of the year, HAEi asked if we could reflect on 2023.

Our top three highlights are:

1. Two successful hybrid Patient Information Updates - it was amazing meeting our members in person after a few years of only offering virtual Updates.
2. In May in Ottawa, the HAE Canada Board of Directors had the first board retreat since COVID. While in Ottawa, they were fortunate to have HAEi's Tony Castaldo and Henrik Balle Boysen join them while attending the Canadian Agency for Drugs and Technologies in Health (CADTH) Symposium.
3. A boom in our membership - partly due to patients and family members interested in attending our Patient Updates and applying to the 2024 HAE Regional Conference Americas in Panama City.

We hope 2024 will bring:

- Equal access across Canada to treatment for HAE patients with normal C1-INH and acquired angioedema.
- Standardized age testing for children who are at risk of having HAE.
- Access to reimbursement for Orladeyo across Canada.
- Inclusion of treatment recommendations for acquired and normal C1 INH patients in the Canadian Hereditary Angioedema Guidelines.
- Greater awareness in ER departments to recognize and appropriately treat HAE attacks. Specifically, that they listen to the patients and follow their treatment plans.
- Beginning implementation of a rare disease network and drug strategy coast-to-coast.
- More rapid approvals of new therapies for HAE.
- Access to more drug therapies for Pediatric patients.

CHILE

From HAE Chile

This year, HAE Chile has progressed in several areas:

1. The organization has contributed with a child's story to the Brady Club book.
2. The organization launched "Aeh Ayuda" in 2023 – a telephone system where calls for HAE emergencies are received 24 hours a day, seven days a week, and where patients are helped to receive better attention in the health systems of Chile.
3. The application of new therapies for patients with HAE in Chile has been submitted to obtain the first prophylaxis therapy in the country.

CHINA

From HAE China

In 2023, we promoted through official accounts and celebrities on multiple well-known social media platforms while organizing various offline activities to expand our organizational influence and continuously achieve good results. The organization currently has 267 members and has established volunteer service organizations in 25 provinces and cities in the Chinese Mainland. These numbers will continue to increase in the future.

Looking back on 2023, our work has three highlights:

Highlight 1: In April 2023, we held a three-day doctor-patient exchange conference in Beijing, which was attended by nearly 100 people, making it the largest conference in recent years. At the conference, patients shared their personal experiences, doctors gave professional explanations and answers, and doctors from Peking Union Medical College Hospital provided free consultation services for patients. Through this conference, more people have a deeper understanding of HAE, and even more than ten people have registered to become volunteers on the spot.

Highlight 2: We have established 25 HAE WeChat groups divided by provinces and cities, with a total of 28 volunteers providing services to facilitate better communication and mutual assistance among local people in various provinces and cities. To this end, we held a volunteer service incubation conference in Changsha in October to provide comprehensive training for volunteers to improve their service levels.

Highlight 3: We are committed to expanding the influence of HAE through various media platforms, official accounts, and celebrities. We have opened official accounts in TikTok, WeChat, Xiaohongshu, Bilibili, and other major social media to publish content and disseminate HAE knowledge continuously. In September of this year, the Illness Challenge Foundation, together with the Chinese HAE Care Center, launched the HAE theme activity "Urgent search for people, looking for a potentially fatal swelling" on TikTok and invited dozens of stars to participate, helping more potential patients to understand HAE as soon as possible, calling on all sectors of society to pay attention to HAE and care for HAE patients.

In 2024, we will continue to organize more activities online and offline, continue to speak out, expand the scope of organizational influence, and let more and more people know about the existence of HAE so that more and more patients can get effective help.

COMOROS

HAEi is delighted to have welcomed HAE Comoros as the 99 Member Organization to join the family.

The national patient contact is Fatoumia Said. You can find contact information on the country page on: https://haei.org/hae-member-countries/comoros/

GEORGIA

From HAE Georgia

Website Launch: We are thrilled to announce the launch of the HAE Georgia website. Leveraging the free templates and web hosting provided by HAEi, our community now has a dedicated online platform to connect, share information, and foster a sense of unity among individuals dealing with HAE in Georgia.

Diagnostic Advancements: In collaboration with the Centre of Allergy and Immunology in Tbilisi, Georgia, we have made strides in diagnosing HAE. Our efforts have identified more individuals grappling with HAE, ensuring they receive the necessary support and resources.

Regional Conference Participation: HAE Georgia was proudly represented at the **2023 HAEi Regional Conference EMEA** in Munich with the support of HAEi. This invaluable experience has enriched our knowledge, strengthened our network, and reinforced our commitment to advancing HAE awareness and care.

Local Language Resources: Recognizing the importance of accessible information, we have translated various informative sources about HAE into the local language. This initiative aims to empower individuals with accurate and comprehensible resources, fostering a deeper understanding of HAE within our community.

Hospital Outreach: We have created and distributed informative posters in Georgian specifically designed for surgical rooms. These posters, distributed to more than 20 hospitals across the country, serve as a vital resource for medical professionals and patients alike, ensuring a heightened awareness of HAE in critical healthcare settings.

Social Media Presence: As part of our ongoing efforts, we are actively working on launching local pages for HAE Georgia across various social media platforms. This initiative seeks to expand our reach, engage with a broader audience, and create a supportive online community for individuals affected by HAE.

These accomplishments underscore our unwavering dedication to improving the lives of those impacted by HAE in Georgia. We believe that by sharing our successes, we can inspire and motivate others facing similar challenges worldwide.

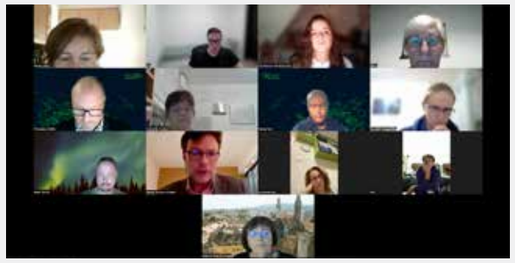
Thank you for your continued support and collaboration. Together, we can make a meaningful difference in the lives of individuals affected by HAE.

HUNGARY

From HAE Hungary

The Hungarian HAE Patient Association held its yearly patient meeting on 22 November in an online form. Thanks to HAEi, who helped to set up the Zoom meeting for the invited patients, caregivers, healthcare professionals, and the representatives of the pharmaceutical companies. The meeting was advertised on the association's homepage, on Facebook, and also in email. Despite not many patients participating in the meeting, the overall mood was very good and familiar. As part of the program, in the first section, the invited representatives of pharmaceutical companies had the opportunity to share their news. First, Tamas Soroncz-Szabó from Takeda informed the group about the latest studies they conducted. After that, Zoltán Vilimszky from ExCEEd Orphan shared news about the new packaging introduced for Ruconest in the last year and offered their help and support to continue the self-administration training for HAE patients.

Professor Farkas introduced her new colleagues Lili and Hanga to the group and gave an overview of the tremendous work they do at the HAE Center and Laboratory with Lilian Varga, who was also present at the meeting.



We also discussed the wonderful and, again, very successful 13th C1-inhibitor Deficiency & Angioedema Workshop in Budapest with about 400 participants. Then, Peter Strahl took the stage, and he told us about his experiences from the **2023 HAEi Regional Conference EMEA**. We also discussed the experiences from the Rare Disease Day from back in February. At the end of the meeting, all the patients talked about how their lives are affected by the opportunity to try out new, modern medications, and we heard wonderful stories about how they have been symptom-free for almost 1.5 years or more. Two hours just flew away and we had a very good time again! See you next year, and we hope to have a face-to-face meeting next year!

NEWS AND 2023 HIGHLIGHTS FROM HAEI COUNTRIES AROUND THE GLOBE

IRELAND

From Bettina Carty, HAE Ireland

It has been a quick year, and my first one as the national contact in Ireland. Our group is small but growing all the time, and with the help of immunology teams letting their patients know we are here, hopefully, that number will grow.

I had the pleasure of attending the 2**023 HAEi Regional Conference EMEA** in Munich in September, and it was fantastic to meet so many people and gain new information. The advancement in treatment has been so good in the last few years due to the hard work of the professors/doctors and drug companies that attended. So many of us now live the "normal" lives we wished for.

During 2023, I also had the opportunity to discuss HAE as a patient to the global staff of one of the larger pharmaceutical companies – the feedback was great, and many of the attendees said the fact they could see and hear the patients they create these drugs for was so helpful. This is being followed in 2024 by an information campaign to inform the public of the number of patients with Rare Diseases, and I will be representing the HAE patients – it is great to see these companies seeing and listening to a patient's perspective.

As we enter 2024, I am hoping to continue advocating for HAE patients and carers in Ireland and also to increase my social media platform to help make people aware of our disease.

"Great oaks from little acorns grow"

MOZAMBIQUE

From Daniella Assa, HAE Mozambique

Attending the **2023 HAEi Regional Conference EMEA** that was held this year in Munich, Germany, gave us support, encouragement, and the necessary tools to advocate for HAE in Mozambique.

We formed an HAE Mozambique patient support group.

We were also able to participate in the FNB Maputo 10k race 'Corrida das Acacias' that took place on Sunday, 5 November, with a record participation of more than 4,000 people. The competition is part of the City of Maputo month, celebrated annually on 10 November, with the aim of raising awareness of the health benefits of taking part in sporting activities to improve quality of life.

We successfully completed this competition swell-free due to better management of HAE.

HAE Patient Daniella Assa and her cousin Tainara pose together after successfully completing the 10k run.

THE NETHERLANDS

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

It's hard to believe that we're already wrapping up the year. So many great things have happened since January.

We worked hard to reinvigorate HAE The Netherlands. We managed to create a new website. We're grateful that our HAE specialists at the ACARE center in Amsterdam found time to help us by checking if the information on our website is correct and in understandable language. It's not completely finished yet, but it's online, and it provides the most important information on HAE for people living with HAE in The Netherlands. In 2024, we will continue working on improving the website.

On 7 October 2023, we hosted a live event for HAE and acquired angioedema (AAE) patients in the Netherlands. It was the first time since 2019 that we were able to meet in person. Besides that, it was the first event organized since changes in the leadership of our organization took place. It was a fantastic meeting! In the morning, we listened to presentations about the pathophysiology of HAE, the disease burden, personal and societal costs of HAE, and the latest developments in HAE science and therapies. The interaction between the speakers and the attendees was wonderful. A lot of good, important questions were being asked. We're grateful that our doctors took the time to answer all our questions. In the afternoon, we learned about what we as patients, can do to improve our quality of life. We learned a lot about the research that is currently taking place at the ACARE center in Amsterdam, where we, as patients, can take part. At the end of the day, we had the pleasure of listening to our special guest, Maria Ferron from HAEi. She told us all about the fantastic tools and resources of HAEi and encouraged us to use them. It was amazing that Maria traveled all the way to Breukelen to attend our meeting. Thank you, Maria. Next time you visit, we will make sure we have a translation service in place. I think it's safe to say that we all had a lovely day and that we were happy to meet old HAE friends and make new ones. We will definitely do it again next year. Preparations will start soon.

In the last few months, we have had a lot of meetings with our pharmaceutical company representatives about projects to help the Dutch HAE community move forward. It's nice to see that we share the common goal of improving the quality of life of people living with HAE. We have lots of exciting projects in the pipeline.

I want to finish by wishing you all a lovely holiday season and best wishes for 2024!!

PAKISTAN

From HAE Pakistan

HAE Pakistan has been busy in the country to educate and create awareness of HAE.

Mr. Shakeel Afridi has arranged productive awareness sessions in medical colleges and institutes. The first session on 28 September was with collaborators at the Rehman Medical College in Peshawar. We had lots of participants who showed great interest and enthusiasm.

The head of the Institute, teachers, and students participated in the session, learning about symptoms, treatments, early diagnosis, and management of HAE. Shakeel Afridi and Rahzeeb Khan spoke to the participants about how HAE International and HAE Pakistan are working together to save the lives of people suffering from this rare disease.

The second awareness session was at the Swabi Institute of Medical Sciences, Swabi. Shakeel held a successful awareness session on 24 October. Comprising mainly medical students and house officers from various hospitals, the dedicated members prioritized this occasion, temporarily stepping away from their academic and hospital responsibilities.

The next session was 285 km from Islamabad at Malakand College of Health Sciences, a Higher Education Pakistan-registered institute in Matta, Swat. Sir Sami Ullah Khan delivered a lecture on hereditary angioedema using simple and accessible language. Shakeel then shared his journey with HAE, followed by an engaging question-and-answer session. This session was attended by around 350 medical students, six PhD doctors, and faculty members.

The next awareness session held was at Jamrud Hospital Khyber, where staff discussed crucial matters about our organizational trajectory and affiliations with the community, government, and international collaborators.

HAE Pakistan appreciates the medical students who have volunteered to help raise awareness of HAE in Pakistan.

During all the awareness sessions, patient advocate Shakeel elaborated on the efforts and mission of HAE Pakistan. He talked about how rare and dangerous this disease is, and due to the lack of proper diagnostic facilities and information, many patients die without getting the appropriate medication. He said that while this disease has no cure, modern treatment methods can improve the quality of life.

HAE Pakistan is committed to advocating for all necessary treatments for patients.

HAE Pakistan would like to extend our appreciation for the sincere support and efforts of our HAEi Regional Patient Advocate, Madam Fiona Wardman, and Head of HAE Pakistan, Mr. Moazzam Farooq. Both have provided information about HAE in detail, passed on important information about the symptoms and life-threatening risks associated with HAE, and emphasized the importance of arranging accurate diagnoses and proper treatment in Pakistan.

HAE Pakistan would like to connect with as many HAE patients and family members living in Pakistan as possible. Please get in touch with us https://haei.org/hae-member-countries/pakistan/

PANAMA

From HAE Panama

During the celebration of the Central American and National Congress of Otorhinolaryngology, held in Panama, 16-18 November 2023, the topic of HAE was addressed by Dr. Olga M. Barrera, medical advisor of the Panamanian Association of Patients with HAE, which motivated a lot of interest among those attending that specialty.

PARAGUAY

From HAE Paraguay

In 2023, HAE Paraguay managed to contact the Health Sciences Research Institute, the Instituto de Investigaciones en Ciencias de la Salud (IICS) of the National University of Asunción (UNA), specifically with researchers from the Department of Immunology. A project entitled "Study of the C1 plasma inhibitor in patients with and without a diagnosis of hereditary angioedema for implementation of laboratory detection" was agreed. The reagents for the study of the quantitative and functional C1-inhibitor were financed by the University and also by the Binding Site LATAM – Scientific Competition. In Paraguay, diagnostic testing was not available, and very few patients had laboratory detection and covered its high cost outside the country. The objective of the study was to evaluate the behavior of plasma C1-INH in patients with and without a previous diagnosis of HAE for the implementation of laboratory detection. Currently, this project is underway, and 30 patients with clinical manifestations of HAE were studied, all of them in the age range of 5 to 67 years, and registered in HAE Paraguay. Only 6 of these patients already had a laboratory diagnosis of HAE. Of the remaining 24, 12 presented a decreased result of the quantitative C1-inhibitor, compatible with HAE type 1. With this study, it will be possible to develop and implement the laboratory test to diagnose HAE in Paraguay, making it available to the community at an affordable cost, which, over time, will strengthen the diagnosis and therapeutic management of patients with HAE at the local level.

PERU

From Carla M. Goachet Boulanger, HAE Peru

This year, we have participated in 2 rare disease campaigns in the interior of the country. In February, we were in the city of Piura at the Cayetano Heredia Regional Hospital of Piura, and recently, in October, in the city of Arequipa at the Honorio Delgado Hospital. In these two cities, we held medical talks on hereditary angioedema by Dr. Oscar Calderón and free medical consultations for patients, as well as the dissemination of HAE. Thanks to these campaigns, we have been able to find more patients with HAE.

NEWS AND 2023 HIGHLIGHTS FROM HAEI COUNTRIES AROUND THE GLOBE

POLAND

From Michal Rutkowski, HAE Poland

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

During the HAE Summer Workshops, psychologist Adrianna Sobol talked to young patients about how to deal with emotions caused by the disease. Remember that you are not alone; it is worth talking, sharing experiences, and supporting each other.

Michał Figurski (a partially paralyzed patient with chronic disease), music journalist, radio and television presenter, and producer shared his story with the participants and spoke about the importance of getting support from loved ones in illness and not giving up and believing in yourself! Thanks to determination and the support of our loved ones, we can achieve anything.

The meeting with HAE specialists Dr. Marcin Stobiecki and Dr. Małgorzata Bulanda gave young patients the opportunity to expand their knowledge about the disease and gave the opportunity to ask questions that may have previously been embarrassing or a taboo topic.

The youngest age group created their own Super-Hero that helps them with HAE. Most of them are parents who are always next to their little patients when they need help. The older group was working on different topics related to HAE. The work they have done will be the spark in our first Polish Youngsters Magazine. We finished the Workshops with a very important course of intravenous and subcutaneous self-administration. We want to make sure that every person feels comfortable with injecting the medicine.

We started September by taking part in the **2023 HAEi Regional Conference EMEA**. It was a huge event that gathered 650 HAE patients (including 18 delegates from Poland!), caregivers, physicians/scientists, and industry representatives from 61 countries. All representatives of the HAE Poland took an active part in three parallel sessions: scientific, general for patients, and a session dedicated to young patients aged 12-25. Dominika Blacharska, member of the HAEi Youngsters' Advisory Group, presented the project: HAE Summer Workshops, organized in cooperation with the Swelling Beautifully Association. Dr. Marcin Stobiecki, a member of the Scientific Committee of the conference, supervised the proper course of the scientific part and was engaged in the Youngsters track, and Michał Rutkowski, President of HAE Poland and Vice-President of HAE International, presented the current situation of patients in Central and Eastern Europe, the Benelux countries and in the Middle East.

October was a very special month for us. We held the 6th National HAE Conference in Krakow. It felt amazing to meet again after a 5-year break since the last Summit organized in 2018.

170 participants, including patients, caregivers, HAE expert physicians, and industry partners of the Swelling Beautifully Association, were celebrating 20 years of activity of the Patient Organization in Poland. Those days were full of great emotions and top-class specialist knowledge. We are left with the incredibly touching patient stories that moved every attendee and the fantastic keynote presentations by HAE expert physicians: Prof. Sandra Christiansen, Prof. Markus Magerl, Dr. Robert Pawłowicz, Dr. Marcin Stobiecki, and Prof. Bruce Zuraw.

Those who have helped HAE Poland spread their wings and began to help local patients with hereditary angioedema effectively also celebrated with us. There would be no successes of the Swelling Beautifully Association if it were not for HAE International, and especially its leadership team: Anthony J. Castaldo, Henrik Balle Boysen, Fiona Wardman, and Jørn Schultz-Boysen.

We are delighted to have the strong bond of patients and their enormous determination to ensure a better future for the next generations of HAE people.

During the Conference, Prof. Markus Magerl from the Charité University Hospital in Berlin presented the benefits and limitations of emergency treatment of HAE attacks and showed the benefits of preventative treatment for HAE people.

Dr. Marcin Stobiecki, from the National HAE Center at the University Hospital in Krakow, presented how, thanks to the current possibilities of effective long-term prevention and acute treatment of HAE attacks, patients can receive effective and individualized therapy. This is possible thanks to cooperation between the physician and the patient, consisting of "shared decision making". In his presentation, Dr. Stobiecki touched on the topic of ongoing clinical trials in HAE, as well as the criteria for qualifying patients for these trials.

Dr. Robert Pawłowicz from the University Hospital in Wrocław devoted his presentation to reviewing the therapeutic options available and reimbursed in Poland. The details of on-demand treatment, short-term prophylaxis, and long-term prophylaxis were discussed. Moreover, Dr. Pawłowicz emphasized the importance of patient education, as well as the ability of patients to self-administered.

Prof. Sandra Christiansen, from the Central HAE Center at the University of California San Diego (UCSD), presented topics that are close to all of us, family and family planning. Prof. Christiansen raised the issues of prenatal and postnatal diagnostics in a fantastic way and also presented the issues regarding HAE with normal C1 Inhibitor.

Prof. Bruce Zuraw, the Director of the US HAEA Angioedema Center at UCSD, presented a significant increase in interest in HAE over the last 20+ years. He also presented the mechanism of swelling and the most common triggers of a HAE attack. Prof. Zuraw emphasized how important it is to properly diagnose the disease and how important it is for all family members to undergo screening tests. We had the opportunity to hear about modern sequencing methods that help quickly diagnose the disease based on known and new pathogenic mutations and the development of accurate biomarkers.

As previously mentioned, the Polish HAE Youngsters created their first own Youngsters Magazine. It is the fruit of the work of young people during the workshop. There are stories of young people dealing with stress of the disease as well as photos from the workshop. Feel free to check it!

Furthermore, we are happy to announce another implementation of HAEi resource, 'Understanding HAE' Patient Guide is now available in Polish.

2H 2023 was extremely intense with making new connections, creating the Youngsters Community, taking part in amazing conferences and advocating for HAE. We can't wait to see what is coming in 2024!

If you want to see more about our advocacy activities, please take a look at our videos from the 2023 HAE Poland Summit at https://bit.ly/poland-gp3_23-1 and from the 2023 HAE Poland Youngsters Workshop at https://bit.ly/poland-gp3_23-2

PUERTO RICO

From HAE Puerto Rico

The Immunology and Allergy Association of Puerto Rico had an annual congress where the HAE topic was part of the agenda. Many doctors made some presentations and talked about the efforts, new treatments, and studies about HAE.

Prof. Marc Riedl, Clinical Director, US HAEA Angioedema Center in San Diego, California, was invited as a special speaker.

The Association extended its invitation to HAEi's Regional Patient Advocate Javier Santana and HAEA Senior Executive Vice President/COO, Michelle Cuevas.

The US HAEA had a booth to promote their efforts with HAE patients and doctors in the US and Puerto Rico.

RUSSIA

From HAE Russia

September: HAE Russia, together with the National Medical Research Center for Pediatric Hematology, Oncology and Immunology, named after Dmitry Rogachev, began a sociological study to study the quality of life and psychological state of patients diagnosed with HAE:

- The relationship between the intensity of swelling and the level of quality of life and psychological state of patients.
- The relationship between regular use of various medications and the level of quality of life and psychological state of patients.
- The need for psychological help and its relationship with the course of the disease.
- A comparison of the quality of life of patients taking different drugs.

October and November: A series of psychological thematic groups for patients and families with HAE has been launched. Each group is dedicated to a specific topic. Group members share their experiences, thoughts, and feelings with each other and also receive recommendations from a medical psychologist.

Some of the topics are:

1. Psychological stress as a factor provoking edema. Self-help methods for stress.
2. HAE and society – to talk or not to talk, and how do you talk about your illness at work to your friends?
3. Internal picture of the disease: The symptoms of HAE manifest themselves very differently in different people. Each person has his/her own perception of the disease.
4. Let's talk about what types of perception of the disease there are, what one or another type depends on, and how this affects the daily life of a person with HAE.
5. Psychological reactions to the disease: Which reactions help adapt to life with the disease, and which, on the contrary, interfere with adaptation?
6. HAE and self-esteem: How does the disease affect self-esteem, and what are the features of this influence in women and men?

November: Webinar. "Like wings behind your back" – patients need support. Our organization is constantly replenished with new members. Anyone who has gone through the path of a "newbie" knows how difficult it can be at first to find one's way, to understand the procedure for action in different life situations, how to achieve effective drug therapy, and to find the right doctors. Therefore, we always pay special attention to newcomers – we explain, advise, and support. On 5 November, we held a webinar, which was attended by many patients whose diagnoses were confirmed this year.

DENMARK, NORWAY, AND SWEDEN

From Jørn Schultz-Boysen, Vice President, HAE Scandinavia

"Stronger Together": Highlights from the 2023 Scandinavian Conference by HAE Scandinavia in Malmö: The welcoming city of Malmö, Sweden, played host to a memorable gathering from 10-12 November 2023, as HAE Scandinavia proudly presented the 5th Scandinavian Conference. Embracing the theme "Stronger Together," more than 100 participants from Sweden, Norway, Denmark, and a few from Finland convened to share insights, experiences, and hopes for the future.

A Warm Start to an Empowering Weekend: The conference began on a high note on Friday evening with a delightful tapas buffet, setting the stage for networking and exchange. The opening presentation, focusing on the strength of the mind and managing anxiety, was not only inspiring but also a fitting precursor to the enriching discussions that followed.

A Saturday Packed with Knowledge and Inspiration: Saturday's agenda started bright and early, featuring a presentation by HAE Scandinavia on the significance of being part of a member organization. The talk illuminated the myriad benefits for patients and caregivers, setting a tone of inclusivity and support.

The morning also showcased a live demonstration of HAE TrackR and HAE Companion, innovative tools designed to aid HAE management. The country-specific breakout sessions that followed allowed participants from the three Scandinavian countries to delve into issues uniquely relevant to them.

The emotional pinnacle of the day was a patient's personal story — a poignant, honest account that resonated deeply with everyone present. It was a powerful reminder of the human side of HAE, beyond statistics and medical discussions.

Afternoon Sessions: Motivation, Medical Insights, and More: Post-lunch, the conference's energy was reignited by motivation expert Mark Anthony. His practical advice on fostering motivation was further enriched by an engaging live interview with Nanna and Victoria, two youngsters and board members of HAE Scandinavia, who shared their experiences and insights.

Professor Markus Magerl from Charité University Hospital in Berlin graced the stage with his talk "HAE, the Future....and Everything." His expertise offered a hopeful glimpse into the advancements in HAE treatment and the promising future awaiting patients.

Innovative Conclusions and Leisurely Interludes: The conference's official program concluded with a segment titled "One More Thing." Here, Victoria presented her learnings from the HAEi LEAP 23 class and introduced "Sums Up" — an upcoming app designed to inform and uplift the HAE community, a testament to the innovation and commitment within our ranks.

Attendees also enjoyed some leisure time exploring one of Scandinavia's largest shopping malls, a welcome break before reconvening for a "hyggelig" dinner that facilitated further networking and fun.

Looking Ahead with Optimism and Unity: The conference wrapped up on Sunday with the General Assembly of HAE Scandinavia. It was heartening to

see familiar faces — patients, caregivers, healthcare professionals, and pharma representatives — all united in their dedication to the HAE cause.

As we reflect on the success of the conference, the theme "Stronger Together" resonates more than ever. It's in these gatherings that we find the strength to face HAE with renewed vigor and shared wisdom. We eagerly anticipate our next meeting, knowing that our collective efforts and connections are making a real difference in the lives of those affected by HAE.

SINGAPORE

From Lim Yong Hao, HAE Singapore

HAE Singapore's membership has recently broken into double digits with 11 members, consisting of patients and caregivers. We are slowly growing and hope to reach and engage the other HAE patients and their caregivers in Singapore. There are also more physicians in Singapore who are taking care of HAE patients and in more hospitals. At a recent conference for the Asia Pacific Association for Allergy, Asthma, and Clinical Immunology in Singapore, we met many interested physicians and were excited to see how HAE Singapore

can foster closer relationships with them to improve the care and management of patients in Singapore. HAE Singapore will also work with the physicians and the hospitals they practice in to become ACARE centers.

For 2023, for the first time in Singapore, HAE appeared on national television and in major newspapers where the condition received public limelight. This was an unexpected result of a feature of a HAE patient story on the morning news on Rare Disease Day. Interviews with HAE-knowledgeable doctors followed the feature in a podcast and coverage in major English and Mandarin newspapers.

HAE Singapore was also fortunate to have a three-person contingent attend the first HAEi Regional Conference APAC where we share and also learn from fellow patients and caregivers in the region. Seeing how HAE patient groups in other countries succeeded in getting access to modern treatment inspired us to want the same to happen in Singapore. The other patient groups were also very generous in sharing the lessons they learned so we can avoid the pitfalls they encountered on their journeys.

We also started a Facebook page to share news and events that are relevant to HAE in Singapore and the region. Hopefully, this page will mature and become a platform to share what is happening in Singapore for HAE to the public and for others to reach out to us.

We have many hopes for 2024. We hope to reach out to all HAE patients and caregivers in Singapore. We hope to be able to organize regular activities to build the HAE patient and caregiver community. We hope to work with the physicians to improve the current HAE diagnosis and management. We hope to kickstart the process to make more HAE treatments available. While we have many hopes, the most important would be to take the little steps that make these hopes possible.

SLOVENIA

From Teja Iskra, HAE Slovenia

After years of struggle and struggle, my family once again helped me so that I could fulfill my dreams. Finally, I can proudly say that the HAE Slovenia Patient Association was officially founded. I'm proud that I finally made it. And now the real work begins. First and foremost, the association will try to recruit members, of course, with the help of the medical staff and Dr. Zidarn, who provides me with maximum help in all these matters. Membership acquisition is currently underway. We are also in the phase of obtaining donations for the operation of the association. In this way, we will be able to provide printed educational and promotional material. The goal is to deliver this material to emergency centers across Slovenia so that our patients will no longer have unpleasant situations, something that each of us has certainly encountered. For now, I will not reveal future goals and wishes because, first of all, I would like to see these things implemented.

SOUTH AFRICA

From Janice Strydom, HAE South Africa

HAE SA started 2023 with the exciting announcement that icatibant, our first treatment for acute attacks of HAE, had been registered. This is a significant milestone for our organization and our patients, and we wish to thank everyone who contributed to this process for their dedication to making this possible. The official launch of icatibant took place at the Allergy Society of South Africa's annual conference on 28 September 2023. We currently have two medications on trial in S.A.

In the run-up to Rare Disease Day 2023, we joined Rare Disease S.A in their "#1in15" campaign, where we shared one "HAE fact" on our social media pages each day for the month of February. On 4 March, we joined 85 rare disease patients on the annual Denim Walk at the Walter Sisulu Botanical Gardens to show support for the 1 in 15 patients living with rare diseases around the world.

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28 February marked the official launch of the Sinovuyo South African Virtual Angioedema Centre, the first of its kind in Africa. The center is under the guidance of Prof. Jonny Peter and his team of angioedema specialists and is named in honor of Sinovuyo Nkelenjane, a 7-year-old patient who sadly lost her life to an HAE attack. Watch Sinovuyo's story here: https://youtu.be/RVn2r2dm4ko

The **HAE TrackR** app was made available in a further two of our official languages, Afrikaans and Zulu.

In the run-up to **hae day :-)** on 16 May, we shared a number of patient stories as well as features on the HAE physicians who dedicate so much of their time to the diagnosis and treatment of our patients. We were also able to share the news that C1-INH functional testing is now available in S.A.

On 26 July, we were joined by patients, representatives from pharmaceutical companies, and physicians for our HAE S.A Treatment Education webinar, where our guest speakers, Profs. Jonny Peter and Mike Levin shared some insights into current and new treatment options.

Later in the year, a CPD-accredited Advanced HAE Masterclass for healthcare professionals was hosted by The Allergy Foundation of South Africa.

We shared our three-part educational video series "Welcome to the World of HAE" – an easy-to-understand video series explaining symptoms, triggers, types, genetics, diagnosis, and treatments. You can watch the video here: https://youtu.be/27hIxPtLM88

Twenty patients and caregivers had the opportunity to attend the **2023 HAEi Regional Conference EMEA** in Munich in October – a record number of participants from South Africa, which included four youngsters for the Youngsters track.

Our HAE burden of illness survey was completed by 24 patients, and the results were compiled at the end of October. An abstract was submitted for presentation at the Rare -X 2024 Conference - Africa's leading conference on rare disease and patient access – and we will be presenting **"The burden of hereditary angioedema in South Africa in 2023: a patient survey"** as an oral presentation at the conference in February 2024.

HAE S.A has made significant progress over the past year and could not have done so without the dedication of our physicians and the support and guidance of our colleagues and friends at HAEi and within the greater rare disease community. We look forward to seeing what the 2024 year holds for our organization.

SOUTH KOREA

From Soojin Min, HAE Korea

Start of Welfare for the Weak! National Assembly Debate to Improve the Quality of Life of Patients with Rare Diseases in Medical Blind Spots: On Tuesday, 12 September, The Korean Society of Genetic Angioma held a policy discussion at the National Assembly Hall in Korea to find ways to support rare disease patients. Park Kyung-ja, Hwan-woo attended as a debater and announced the patient's difficulties, and Min Soo-jin, CEO of HAE KOREA, presented his opinion to introduce preventive drugs. Korea is making continuous efforts to introduce preventive treatments because the only emergency treatment available is 'Firazyr.'

HAE KOREA Patients Increase and Patient Friendship Membership Increase: We now have about 200 confirmed patients in Korea, and the 9 members at the beginning of the Korea Friendship Association in 2019 has increased to 73 members of the Korea Friendship Association as of 2023. The number of members increases steadily through SNS and promotion of each hospital.

Year-end meeting: On 9 December, HAE KOREA will hold the 2nd regular general meeting and year-end meeting in 2023 near Seoul.

SWITZERLAND

From Helene Saam, President, HAE Switzerland

The highlights in 2023 for the Swiss HAE Association were as follows:

- Production of the film 'HAE - Simply Explained'. Link to the film with German, English, French, and Italian subtitles: bit.ly/hae-simply-explained
- Swiss HAE Association goes Social Media
- HAE patient meeting with 55 participants and 5 HAE experts.

TAIWAN

From Chen Jyun-Ting and Lim Yong Hao

One of the biggest highlights in Taiwan in 2023 is the boost in the number of HAE-knowledgeable doctors and healthcare professionals. This happened because of training workshops organized and conducted by Dr. Shyur and colleagues, with the support of CSL Behring.

The workshops were conducted between May and September this year in four major cities, Taipei, Taichung, Yilan, and Kaohsiung, covering the north, central, east, and south regions of Taiwan, respectively. Each workshop covers a wide range of topics on HAE, including the epidemiology, pathogenesis, clinical manifestations, diagnosis & differential diagnoses, prognosis, latest management guidelines, including those of special populations like children and pregnant women, as well as practical training in the administration of subcutaneous and intravenous treatments and sharing of treatment administration experience.

Hundreds of healthcare professionals attended the series of training workshops, and those who attended the workshops were recognized with a certification of completion and were able to list their services on HAE Taiwan Facebook to allow patients to find HAE-knowledgeable doctors who are near them. This effort has made it more convenient for HAE patients and greatly improved access to HAE knowledgeable doctors, regardless of which part of Taiwan the patients' homes are.

HAE patients in Taiwan are fortunate to have readily accessible and affordable healthcare, and new and modern drugs and treatments are slowly being introduced, hopefully in the next few years. However, HAE patients are still not very willing to participate in activities, likely due to perceived social stigma and common misconceptions of the condition. For 2024, our hope and goal is for more patients to step out of this sentiment and be more active in HAE Taiwan's activities.

TUNISIA

From HAE Tunisia

The "ANAOH Tunisia" association officially began its activities in October 2022 with an executive office bringing together four patients and four doctors. The

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first general meeting was held in March 2023 in the presence of the executive office and 42 patients.

A board meeting was held in November to plan actions for the next three months, as well as to welcome two new board members: General Secretary Oueslati Mohamed and Communications Manager Barhoumi Arbia.

2023 was an important year as we increased the number of patients. The conferences in Egypt and Munich (the **2023 HAEi Regional Conference EMEA**) allowed the organization to connect with the HAEi family and get more information about HAE.

Highlights of 2023 are:

- All members (52) benefited from a medical card.
- Three media actions at regional and national levels took place, which made it possible to raise HAE awareness in the general public.
- Preparing in collaboration with medical teams from the Charles Nicolle hospital in Tunis the project of a reference center for angioedema to improve patient care.
- A medical caravan to two regions in southern Tunisia is planned for the end of December 2023.
- The objectives of ANAOH Tunisia in 2024 are:
- We plan to organize medical caravans to the central and southern regions of Tunisia every three months to diagnose symptomatic patients and help them join the patients' association.
- A national hereditary angioedema day is planned for the first half of 2024 (date not yet set).
- We will continue efforts with the Minister of Health and the competent authorities to finalize the reference center and create a national register.

UNITED KINGDOM

From Angela Metcalfe, CEO of HAE UK

Goodness, it has been a very busy year for HAE UK! As CEO, I was delighted to attend the **2023 HAEi Regional Conference EMEA** in Munich at the beginning of September, and very pleased to be joined by several other patients and Trustees of our charity, as well as Rachel Annals whom so many of you know. It was a very illuminating and educational conference, and on

behalf of HAE UK can we record our thanks to everyone involved in the organization, and, of course, to the many and varied speakers. The patient stories of diagnosis were particularly interesting.

We continue to have many surveys and research activities here in the UK and are also delighted that we now have a number of patients on clinical trials, which is extremely exciting. When we gather all this information it is carefully brought together to provide data with which we can hopefully use to implement appropriate change in the care, management, and treatment of HAE patients. It is both essential for us as the patient organization but, of course, also key to healthcare professionals to better understand the patient journey. Sharing the living experience of HAE, we hope supports, steers and guides others in being able to live with this condition, rather than just existing. There is some exciting stuff coming out soon which we will share on publication.

So, I wish you all a very merry Christmas wherever you may be celebrating, and a very happy and particularly healthy 2024.

USA

From Jess Myers, Communication and Advocacy Specialist, US HAEA

US HAEA Advocacy Update: The US HAEA advocacy team has been hard at work this year to fight against harmful policies that prevent Americans with HAE from accessing their lifesaving medications.

Recently, there have been some exciting steps forward for HAE advocacy including:

- On October 2nd, 2023, a US federal court struck down a rule that allowed health insurers to exclude certain types of charitable funding from counting towards insurance costs for patients. This court ruling will help to make HAE therapies more affordable for Americans with HAE.
- In Ohio, HAEA advocates have been hard at work asking lawmakers to support a state bill that would reduce out-of-pocket costs in healthcare.
- HAEA advocates in Connecticut, met with Senator Chris Murphy to ask for his support on two bills, the Safe Step Act and the HELP Copays Act, that would help people access affordable HAE treatments.

US HAEA Members Ally and Jasmeen Graduated from the HAEi LEAP Program:

US HAEA youth leaders, Ally Chacon and Jasmeen Aldaco, graduated as members of the first HAEi LEAP (Learn, Experience, Advocate, and Pave the way) program class earlier this year. HAEi LEAP is an educational program developed by HAEi that allows young people to learn new skills and develop as individuals and advocates.

During their time in the LEAP program, they completed a two-day training in Dubai and drafted valuable resources for the HAEA community. Ally crafted a caregivers guide, and Jasmeen created a youth advocacy guide.

Read more about Ally's and Jasmeen's projects on page 28 where you'll also find a link to their guides.

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

Sheena Discusses her First-Time Experience at the 2023 US HAEA National Summit:
https://www.haea.org/pages/bp/blog22_Sheena

Jasmine Finds Her Confidence through HAEA Advocacy and Youth Leadership:
https://www.haea.org/pages/bp/blog23_Jasmeen

Advances in Research:

The US HAEA Scientific Registry: Hereditary Angioedema Demographics, Disease Severity, and Comorbidities: The US HAEA Medical Advisory Board has published a paper in the Annals of Allergy, Asthma and Immunology highlighting the importance of the AdvanceHAE Scientific Registry as a robust tool to learn about the natural history of HAE, as well as potential comorbidities that affected patients, may experience. With the addition of new members to the registry, we anticipate that the power of the Scientific Registry to address the natural history questions and treatment outcomes for HAE will expand.

Access to the publication is free and can be viewed here: https://bit.ly/usa-gp3_23

HAE Caregiver Study: Most HAE research focuses on how individuals are affected by the condition, however, little attention has been paid to the impact on HAE caregivers. The US HAEA is initiating a study using real-world data on US caregivers for people with HAE, and our aim is to test and quantify the burden on caregivers for people with HAE, including monetary and non-monetary burden, socioeconomic burden, health economic cos, and the difference in burden when their loved one is on a modern prophylaxis medication. The HAEA launched the study with a pilot focus group and will continue the study with a survey of caregivers for people with HAE. We will publish the results in 2024.

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

You can view the updated CME program on the HAEA website by going to haea.org, clicking on "Resources", then "For Healthcare Professionals", and finally "CME".

VENEZUELA

From Wilfredo Bocaranda, President, HAE Venezuela

HAE Venezuela held meetings with the Authorities of the Military Hospital with Doctors Guillermo Boggiano, Rommy Boggiano, and their medical team to analyze the case of patients in Venezuela and offer an alternative treatment. The request for treatment is managed by the High Cost unit of Social Security in Venezuela and is currently in process.

We also held working meetings with the Institute of Immunology of the Central University of Venezuela to organize the procedures to begin testing Genetic Therapy studies in the experimental phase. The Bocaranda Family will be one of those selected to begin this process, and as far as possible, other families will be incorporated into the procedure.

HAE Venezuela also communicated via email to request information and follow up on the oral treatment found on the HAE treatment market. The organization has hopes for access to treatments for the Venezuelan population, who currently do not have any treatment, and even the option of fresh plasma is unattainable due to the existing hospital crises, considering that the largest population has very low resources.

HAE Venezuela was filmed in the city of Caracas, raising people's awareness about HAE.

HAE Companion

Travel safely with HAEi's free travel companion app, which includes translated emergency cards and local medical care options*.

Download on the App Store

GET IT ON Google Play

*In countries where this information is available. Please always check that the information is correct by contacting the physician/hospital first.

MEDICAL PAPERS

In addition to a wide range of case reports and small series, here are summaries of recently published hereditary angioedema (HAE) related scientific papers (data search undertaken 22 November 2023).

Hereditary angioedema and Wilson's disease during breastfeeding

Philip O Anderson.

The author highlights HAE as a genetic condition that clinicians may encounter in nursing mothers. The article provides information on specific drugs used to treat and prevent HAE attacks, and the implications during breastfeeding.

(Breastfeeding Medicine, 19 September 2023)

Patient-physician interactions in hereditary angioedema – key learnings from the coronavirus disease 2019 pandemic

Marcus Maurer, et al.

The authors consider the impact of the pandemic and reduced in-person contact in clinics on the experience of people with HAE. The authors report that the pandemic markedly affected patient-physician communication and that this is especially important in rare diseases such as HAE. The paper discusses the merits of technologies such as telemedicine and e-prescriptions, and how these should be individualized to the needs of patients.

(Clinical and Translational Allergy, 29 August 2023)

Evaluation of patient-reported outcome measures for on-demand treatment of hereditary angioedema attacks and design of KONFIDENT, a phase 3 trial of sebetralstat

Danny M Cohn, et al.

This paper focuses on the design of the KONFIDENT trial, a phase 3 randomized, placebo-controlled, three-way crossover trial evaluating the efficacy and safety of sebetralstat in patients 12 years or over with HAE-C1-INH. The authors sought patient feedback on ways to quantify the experience of treated patients. They concluded that the Patient Global Impression of Change (PGI-C) scale was supported by data and patient preference, and should be the primary outcome measure in the KONFIDENT trial.

(Clinical and Translational Allergy, 4 September 2023)

Could it be hereditary angioedema?—Perspectives from different medical specialties

Markus Magerl, et al.

The multi-faceted nature of HAE and the difficulties it poses for diagnosis are considered in this review. The views of various medical specialties are considered, and the authors conclude that it is a necessity to have cross-specialty awareness of HAE. They give the essential information any healthcare professional who may encounter a patient with HAE symptoms should know to diagnose and treat effectively.

(Clinical and Translational Allergy, 19 September 2023)

Sensitivity to change and minimal clinically important difference of the angioedema control test

Lauré M Fijen.

The Angioedema Control Test (AECT) is a patient-reported outcome measure for assessing disease control in patients with recurrent angioedema, including HAE. The authors analyze cases in which changes in AECT scores were measured, finding that the AECT is a valuable tool to assess changes in disease control in patients with recurrent angioedema, over time. The lowest AECT score change that reflects a meaningful improvement of disease control (MCID) in patients is three points.

(Clinical and Translational Allergy, 1 September 2023)

Gluten intolerance and its association with skin disorders: A narrative review

Vaibhav Vats, et al.

The authors indicate in this review that HAE has shown an association with gluten intolerance in some patients who had symptomatic benefits with a gluten-free diet. They call for additional clinical and observational trials to further expand on the pathophysiology and provide conclusive and comprehensive recommendations for possible dietary interventions.

(Cureus, 1 September 2023)

MEDICAL PAPERS

Hereditary angioedema: Patient journey approach in Mexico

María Eugenia Vargas Camaño, et al.

This research aimed to understand the patient journey with HAE and the impact of the disease on the lives of patients in Mexico. Analyzing the responses to a patient survey, the authors conclude a need to reinforce general practitioners' knowledge of HAE to promote an earlier diagnosis and awareness of rare diseases and their impact on quality of life among the general population and promote the removal of barriers to treatment.

(Revista Alergia México, 21 August 2023)

How does pregnancy and type of delivery affect the clinical course of hereditary angioedema?

Deniz Eyice Karabacak, et al.

The authors identified limited knowledge about HAE during pregnancy and aimed to evaluate the course of HAE in pregnancy and breastfeeding. The study evaluated 88 pregnancies in 48 HAE patients. The conclusion was that the course of HAE can be worse during pregnancy and breastfeeding. Normal vaginal delivery (NVD) is related to fewer HAE attacks.

(International Archives of Allergy and Immunology, 6 September 2023)

Characteristics and drug utilization of patients with hereditary angioedema in Italy, a real-world analysis

Elisa Giacomini, et al.

This real-world analysis investigated the characteristics and treatment patterns of patients with HAE in Italy, using the administrative data of health units across the country. Investigating patient treatment records, the authors found that 84% received a treatment for acute attacks. In terms of co-morbidities, 36.5% of patients had gastrointestinal disorders, 28.4% had high blood pressure, 11.5% had high cholesterol, and 9.5% were affected by depression. The authors conclude that these patients may have unmet therapeutic needs.

(Healthcare (Basel), 10 September 2023)

Hereditary angioedema patient experiences of medication use and emergency care

Amy Elizabeth Burton, et al.

This study aimed to gain an in-depth understanding of patient experiences using treatment and emergency care. 65 participants completed an online survey. The results indicated the life-changing nature of new treatment and benefits for quality of life, but also illustrated common barriers to treatment administration. Experiences of emergency care illustrated how throat attacks and the fear of future occurrence could be traumatic. The authors conclude that these findings indicate that HAE patients need psychological support to process fears and negative experiences. In addition, psychological barriers to treatment administration (such as needle phobia) must be addressed to ensure treatment is used effectively. Education for emergency practitioners is also needed to improve treatment in this setting and reduce the psychological burden of delayed emergency care.

(International Emergency Nursing, 14 September 2023)

Real-world experience of hereditary angioedema (HAE) in Mexico: A mixed-methods approach to describe epidemiology, diagnosis, and treatment patterns

Sandra Nieto, et al.

The authors identified a lack of data on epidemiology, diagnosis, healthcare processes, and treatment patterns of HAE in Mexico. A series of different sources of data and information were consulted during a literature review and meta-analysis. A prevalence rate of 0.9/50,000 was estimated for 2019. The authors conclude that despite attempts to raise awareness of HAE, the number of cases identified and treated in the national health service is just 16.6% of the estimated patient numbers in Mexico.

(World Allergy Organization Journal, 13 September 2023)

Acquired angioedema due to C1-inhibitor deficiency (AAE-C1-INH)- a bicenter retrospective study on diagnosis, course and therapy

Susanne Trainotti, et al.

Acquired angioedema with C1-inhibitor deficiency (AAE-C1-INH) is a rare condition resembling hereditary angioedema (HAE), but with late onset and low C1-inhibitor (C1-INH) due to consumption potentially caused by autoimmune diseases and mainly lymphatic malignancies. Being about 10-fold rarer than HAE, there is limited knowledge and no licensed therapy. By reporting retrospective data from 20 patients in southern Germany, the authors aimed to report clinical and biological data from a newly described patient population. The authors conclude that AAE-C1-INH is characterized by late-onset swellings mainly involving the face and low C1-INH levels. Delays to diagnosis are decreasing despite the rarity of the condition, and severely affected patients without indication for treatment could benefit from off-label therapy.

Lanadelumab in patients 2 to less than 12 years old with hereditary angioedema: Results from the Phase 3 SPRING study

Marcus Maurer, et al.

The authors report that there are no approved long-term prophylaxis treatments for children aged less than six years, and this study evaluates the safety, pharmacokinetics, and efficacy of lanadelumab and health-related quality of life in patients aged two to less than twelve years.

The authors conclude that their findings support the safety, efficacy and improved quality of life with lanadelumab 150mg, given either every two weeks or every four weeks, depending on the child's age.

(Journal of Allergy and Clinical Immunology: In Practice, 18 September 2023)

A comprehensive management approach in pediatric and adolescent patients with hereditary angioedema

Raffi Tachdjian, et al.

The authors state that the evidence informing the use of HAE medications in pediatric practice is limited. They conclude that HAE plans should address on-demand treatment and prevent potentially fatal laryngeal attacks. They indicate that such plans should take a holistic approach, considering nonclinical aspects of HAE, including quality of life and psychological issues.

(Clinical Pediatrics, October 2023)

Hereditary angioedema: A test of our progress

Sandra C Christiansen and Bruce L Zuraw.

In this editorial on a series of four separate papers, the authors summarize the latest data. They conclude that a unified approach to HAE may be beneficial, as, at present, the collective goal that all with HAE should live a better life is not being met. The authors present a future in which patients band together through the likes of HAEi to forge coalitions with healthcare professionals and the pharmaceutical industry. Such alliances could promote and improve the allocation of resources and provide effective care and treatment access for all patients with HAE.

(Journal of Allergy and Clinical Immunology: In Practice, October 2023)

Subcutaneous C1 inhibitor for the long-term prophylaxis of hereditary angioedema – a real life experience

K Baynova, et al.

This study reports on 22 patients with C1-INH HAE treated with subcutaneous C1-INH replacement treatment for long-term prophylaxis during the COVID-19 pandemic. The authors conclude that subcutaneous C1-INH is an effective prophylactic treatment in a series of patients with HAE treated with doses below 40 UI/kg. Although a small series of patients, the authors state that this is appropriate in rare diseases such as HAE, and the results confirm previously suggested clinical data.

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

Clinical evaluation of pediatric patients with hereditary angioedema

Ayşe Kırmızıtaş Aydoğdu and Gizem Ürel Demir.

This study concluded that HAE is diagnosed only when the patient requests examination following recurrent angioedema. Severe laryngeal edema attacks in patients without a diagnosis of HAE are fatal at a higher rate than attacks in patients with a diagnosis. The authors state that awareness of the symptoms of HAE is necessary, and correct diagnosis is essential to proper treatment.

(Tohoku Journal of Experimental Medicine, 5 October 2023)

Artificial intelligence-generated scientific literature: A critical appraisal

Justyna Zybaczynska, et al.

This paper asked an artificial intelligence (AI) chatbot to generate a review article on HAE. The authors conclude that while the language used was logical and articulate, there were apparent inaccuracies and fabricated information, such as references. The authors call for rigorous evaluation and validation of AI tools.

(Journal of Allergy and Clinical Immunology: In Practice, 11 October 2023)

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

M Branco Ferreira, et al.

The authors examined treatment guidelines for C1-INH-HAE from 2010 to 2022. The guidelines were found to have evolved with the availability of new medicines and therapeutic approaches, such as on-demand therapy and prophylaxis. An individualized, patient-centered approach is now central, along with home-based therapy and more flexibility and convenience. The authors note that treatment goals are increasingly ambitious, striving for total disease control.

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

Hereditary angioedema in older adults: Understanding the patient perspective

Alan P Baptist, et al.

To date, this paper notes that no studies have evaluated the impact of HAE on older adults. The authors identify seven core themes related to the experience of HAE amongst older people. They conclude that specific challenges and concerns in these patients may be unique compared with younger populations. These may include worsening of attack frequency and/or severity with aging, and the impact of comorbid conditions such as arthritis and memory loss. The authors call for healthcare providers to address these issues to provide optimal care.

(Annals of Allergy, Asthma and Immunology, 16 October 2023)

Pathophysiology of bradykinin and histamine mediated angioedema

Hermenio Lima, et al.

This review considers the types, triggers, and underlying pathophysiology of angioedema. The authors conclude that to accurately diagnose and manage angioedema, understanding the condition's complex and varied pathophysiology is necessary and critical.

(Frontiers in Allergy, 18 October 2023)

The role of anxiety in patients with hereditary angioedema during oral treatment: A narrative review

Alessio Rosa, et al.

This research investigated the clinical potential of managing anxiety during dental procedures to reduce acute attacks of HAE. The authors conclude that sedation, assessment, or anxiety control techniques could reduce attacks.

(Frontiers in Oral Health, 19 October 2023)

Evaluation of the retrospective LACE index in predicting the risk of readmission in patients with hereditary angioedema in an emergency department

Meltem Songur Kodik, et al.

The Length of stay (L), Acuity of admission (A), Comorbidities (C), and recent Emergency department use (E) or LACE index is a tool to predict premature death or unplanned readmissions after hospital discharge. It is used across many forms of illness. This study calculated the LACE index for HAE patients attending an emergency department. It found that the LACE index could predict outcomes for people with HAE.

(Emergency Medicine International, 20 October 2023)

Uncovering the true burden of hereditary angioedema due to C1-inhibitor deficiency: A focus on the Asia-Pacific region

Daisuke Honda, et al.

This paper concentrates on people with HAE living in the Asia Pacific region, especially lower-income and developing nations. The authors conclude an urgent need to improve HAE services and provide access to life-saving treatment in developing countries. Efforts should be made to increase awareness of guideline recommendations in high-income countries that do not currently provide long-term prophylactic treatments.

(Journal of Allergy and Clinical Immunology, October 2023)

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

Laura Barrett Ryø, et al.

This paper aimed to describe the effects of 28 disease-associated SERPING1 variants. The authors found that different SERPING1 variants cause disease through various and occasionally overlapping mechanisms. As a result, they define some types of HAE with C1-INH deficiency as 'serpinopathies.'

(Journal of Allergy and Clinical Immunology, November 2023)

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

Michihiro Hide, et al.

The authors evaluated the safety, efficacy, and pharmacokinetics of subcutaneous weight-adjusted icatibant for the treatment of HAE attacks in Japanese pediatric patients. Their results, the authors say, support the safety and efficacy of icatibant in Japanese pediatric patients.

(Journal of Dermatology, November 2023)

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

Robin Lochbaum, et al.

The authors reviewed existing literature on medicines with the potential to trigger HAE attacks. They indicate that there are several medications that should be avoided or administered with caution. Some triggering medications are well known, while others are suspected or of unknown significance. In particular, medications, such as tissue plasminogen activator, have a higher incidence of angioedema with potential airway compromise than ACE inhibitors, although this fact is widely underappreciated. The authors conclude that, in patients with diagnosed HAE, we consider all medication with the potential to increase bradykinin concentration as contraindicated unless it is a serious situation without equivalent alternatives.

(Journal of the German Society of Dermatology, November 2023)

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

Michihiro Hide, et al.

The safety and efficacy of lanadelumab for the prevention of HAE attacks had never been studied in Japanese patients. This study reports research from a Phase-3 trial in Japanese patients. Overall, the study supported two previous global phase 3 studies for lanadelumab use as a prophylactic therapy in Japanese patients with HAE.

(Journal of Dermatology, November 2023)

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

Hana Grombirikova.

The author screened 207 Czech HAE patients from 85 families to understand what SERPING1 gene defects were present. As a result, they were better able to understand the genetic landscape of C1-INH-HAE in the Czech population and identify some novel variants. Ultimately, the authors believe their findings highlight the importance of comprehensive screening strategies and functional analyses in improving the C1-INH-HAE diagnosis and management.

(Journal of Clinical Immunology, November 2023)

STAR-0215 is a novel, long-acting monoclonal antibody inhibitor of plasma kallikrein for the potential treatment of hereditary angioedema

Vahe Bedian, et al.

This study describes how STAR-0215 is created, along with its pharmacokinetic profile and indications of its suitability as a potential agent for long-term preventative HAE therapy. The authors conclude that STAR-0215 has been designed to be a long-lasting prophylactic treatment to prevent HAE attacks and decrease the disease burden for people with HAE.

(Journal of Pharmacology and Experimental Therapeutics, November 2023)

Hypersensitivity reactions amongst Hungarian patients with hereditary angioedema due to C1-inhibitor deficiency

Hanga Réka Horváth, et al.

This study aimed to assess the occurrence of hypersensitivity reactions in the Hungarian C1-INH-HAE population. Hypersensitivity reactions in HAE patients can be a result of a deficiency of C1-inhibitor. Questionnaires were completed by 106 patients, with 63.2% reporting hypersensitivity, most commonly to pollen. The authors conclude that this figure is much higher than Hungary's 19.3% currently reported for self-reported allergies. They consider there may be a possible connection between allergies and HAE, but further studies are needed.

(World Allergy Organization Journal, 23 October 2023)

PubMed

A retrospective analysis of long-term prophylaxis with berotralstat in patients with hereditary angioedema and acquired C1-inhibitor deficiency-real-world data

Felix Johnson, et al.

Berotralstat is a recently licensed long-term prophylaxis (LTP) and the first oral therapy for HAE patients. No approved therapies exist for AAE-C1-INH patients. This study is the first to report real-world clinical data of patients with AAE-C1-INH and HAE who received Berotralstat. The authors concluded that patients with HAE, HAE-nC1-INH, and AAE-C1-INH treated with Berotralstat showed reduced angioedema attacks and improved AE-QoL and AECT scores.

(Clinical Reviews in Allergy and Immunology, 2 November 2023)

Universal Access to On-Demand Treatment of Patients with Hereditary Angioedema, the Chilean Experience

Juan J Escobar, et al.

The authors of this paper indicate that Chilean HAE patients have been guaranteed access to on-demand plasma-derived C1-INH (pdC1-INH) since 2018. They aimed to analyze the first three years of this access program. The authors conclude that disease awareness must be improved to reduce diagnostic delays. They also call for long-term prophylactic medications to be included in the access program to treat patients with high attack rates and control the costs of frequent on-demand treatment with pdC1-INH.

(Pediatric, Allergy, Immunology and Pulmonology, 2 November 2023)

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

S Kent Dickeson

The authors describe a genetic mutation leading to HAE. They indicate that disease-causing mutations in at least 10% of HAE patients appear to involve genes for proteins other than C1-inhibitor. The research shows how a point mutation in the Kng1 gene encoding HK and low-molecular-weight kininogen was identified recently in a family with HAE.

(Blood, 22 November 2023)

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

Raffi Tachdjian, et al.

This paper aimed to characterize the real-world treatment burden associated with existing on-demand therapies. The authors analyzed US FDA records of adverse drug reactions associated with approved on-demand HAE therapies. The most commonly reported adverse reactions of administration sites were pain, skin redness, and swelling. The authors conclude that the real-world data suggests patients experience substantial treatment burden associated with FDA-approved, parenteral, on-demand therapies for HAE attacks.

(Allergy and Asthma Proceedings, 22 November 2023)

The analysis of the effect of the COVID-19 pandemic on patients with hereditary angioedema type I and type II

Dávid Szilágyi, et al.

The authors investigated the potential that C1-INH-HAE may worsen an SARS-CoV-2 coronavirus infection. The authors conclude that HAE patients did not experience more serious COVID-19 infections, and it did not aggravate the course of HAE. The authors also note that vaccines were safe in HAE patients.

(Scientific Reports, 22 November 2023)

PubMed

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 1 December 2023).

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

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

Poland

NTLA-2002 in Adults With Hereditary Angioedema (HAE)

Australia
France
Germany
Netherlands
New Zealand
United Kingdom

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

Spain
United States

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

China

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

United Kingdom

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

United Kingdom

A Survey of Lanadelumab in Participants With Hereditary Angioedema

Japan

A Survey of Icatibant in Pediatric Participants With Hereditary Angioedema

Japan

A Study of STAR-0215 in Participants With Hereditary Angioedema

Canada
United States

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

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

Israel
United States

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

Canada

Firazyr Patient Registry (Icatibant Outcome Survey - IOS)

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

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

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

Berotralstat Treatment in Children With Hereditary Angioedema

Austria
Canada
France
Germany
Israel
Italy
Poland
Romania
Spain
United Kingdom

Status of Dental Care Practices in Patients With Hereditary Angioedema

France

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

Australia

Identification and Characterization of Genetic Variants in Hereditary Angioedema

Spain

Global Registry to Gather Data on Natural History of Patients With Hereditary Angioedema Type I and II

Italy

The Role of the Coagulation Pathways in Recurrent Angioedema

France

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

France

Read more about these and other clinical trials at:

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

NEWS FROM THE INDUSTRY

24 Oct 2023

KalVista Pharmaceuticals Presents Data at the Asia Pacific Association of Allergy, Asthma and Clinical Immunology 2023 International Conference

KalVista Pharmaceuticals today announced the presentation of a novel analysis characterizing hereditary angioedema (HAE) attacks occurring in patients receiving long-term prophylaxis (LTP) treatments at the Asia Pacific Association of Allergy, Asthma and Clinical Immunology 2023 International Conference (APAAACI) in Singapore.

The following presentations occurred at APAAACI 2023:

- Hereditary Angioedema Attacks in Patients Receiving Long-Term Prophylaxis: A Systematic Review: Hilary J. Longhurst, Auckland City Hospital, Te Toka Tumai, and University of Auckland, Auckland, New Zealand
- KONFIDENT Phase 3 Trial Global Expansion: Sebetralstat Pharmacokinetics and Pharmacodynamics in Japanese and Chinese Adults: Daisuke Honda, Chiba University Graduate School of Medicine, Chiba, Japan

Andrew Crockett, Chief Executive Officer of KalVista, said: "Access to safe and effective on-demand therapy is essential for all people living with HAE, including those receiving LTP. Sebetralstat, an oral plasma kallikrein inhibitor in late-stage development for on-demand treatment of HAE attacks, has the potential to address this unmet need."

(Source: KalVista)

26 October 2023

Pharming Group reports third quarter 2023 financial results, including increased Ruconest revenue

Pharming Group presents its preliminary, unaudited financial report for the three months ended September 30, 2023.

The company reported 23% revenue growth in the third quarter of 2023, versus the same period last year. Pharming considered this growth attributable to increased Ruconest revenue, coupled with initial Joenja revenues of US$6.5 million.

Sijmen de Vries, Chief Executive Officer, comments: "Pharming delivered a strong third quarter, increasing quarterly revenues to US$66.7 million and putting the Company at US$164.1 million in revenues for the first nine months of the year. The 23% revenue growth seen in the third quarter of 2023, versus the same period last year, was attributable to increased Ruconest revenue, coupled with initial Joenja revenues of US$6.5 million."

(Source: Pharming)

26 October 2023

BioCryst to present new Orladeyo (berotralstat) data at annual scientific meeting of American College of Allergy, Asthma & Immunology

BioCryst Pharmaceuticals today announces that the company will present two abstracts on oral, once-daily Orladeyo (berotralstat) for the prophylactic treatment of hereditary angioedema (HAE) in patients 12 years and older at the Annual Scientific Meeting of the American College of Allergy, Asthma & Immunology (ACAAI).

The meeting will take place at the Anaheim Convention Center in Anaheim, Calif., from 9-13th November 2023.

The abstracts being presented are:

- Berotralstat Reduced Attack Rates in Patients with Hereditary Angioedema with Normal C1-Inhibitor: Real-World Outcomes; ePoster #P082; Friday, November 10, 5:30-5:45 p.m. PT; Monitor #13, Exhibit Hall
- Berotralstat Reduced Attack Rates Compared to Baseline in Patients with Hereditary Angioedema in APeX-S; ePoster #P064; Saturday, November 11, 12:05-12:20 p.m. PT; Monitor #12, Exhibit Hall

In addition to displaying in the exhibit hall at the noted times, ePosters will be accessible online and on demand to registered attendees on Thursday, November 9, beginning at 8:00am Pacific Time on ACAAI's website.

(Source: BioCryst)

31 October 2023

Pharvaris to present at the annual scientific meeting of American College of Allergy, Asthma & Immunology 2023

Pharvaris today announces the acceptance of two abstracts for ePoster presentation at the American College of Allergy, Asthma & Immunology (ACAAI) 2023 Annual Scientific Meeting, to be held from 9th-13th November, 2023, at the Anaheim Convention Center in Anaheim, California.

Presentation details:

- Title: Deucrictibant immediate-release capsule reduces time to end of progression of hereditary angioedema attacks' manifestations Presenter: Marc A. Riedl, M.D., M.S. Date/Time: Friday 10th November, 5:15-5:30pm Pacific Standard Time (PST)
- Title: Reasons not to treat HAE attacks and satisfaction for on-demand treatment Presenter: Joan Mendivil, M.D. Date/Time: Saturday, 11th November, 12:20-12:35pm PST

(Source: Pharvaris)

2 November 2023

KalVista Pharmaceuticals to Present Data at the 2023 Annual Scientific Meeting of the American College of Allergy, Asthma & Immunology

KalVista Pharmaceuticals today announces the acceptance of multiple abstracts at the 2023 Annual Scientific Meeting of the American College of Allergy, Asthma & Immunology (ACAAI), taking place in Anaheim, California from 9-13th November 2023.

The presentations are:

- Understanding Why Hereditary Angioedema Patients Often Do Not Carry Their On-Demand Treatment with Them #P078: Bob Geng, Sally van Kooten, Markus Heckmann, Sherry Danese, Ledia Goga, Cristine Radojicic. Results shared as an oral poster presentation and Q&A on Friday 10th November from 4:30 - 4:45 pm Pacific Time (PT) at Monitor 13
- Treatment of Hereditary Angioedema (HAE) with Normal C1-INH (HAE-nC1-INH) – A Real-World ACARE Survey #P059: Marcus Maurer, Markus Magerl, Marc A. Riedl, Sherry Danese, Julie Ulloa, Vibha Desai, Paul K. Audhya. Results shared as an oral poster presentation and Q&A on Friday 10th November from 5:15 - 5:30 pm PT at Monitor 12
- Treatment Patterns Among Individuals with Hereditary Angioedema in the United States - #P085: Raffi Tachdjian, Rose Chang, Maral DerSarkissian, Kristen A. Cribbs, Betsy J. Lahue, Shawn Czado, Vibha Desai. Results shared as an oral poster presentation and Q&A on Saturday 11th November from 11:50 am - 12:05 pm PT at Monitor 13
- Diagnosing Hereditary Angioedema (HAE) with Normal C1-INH (HAE-nC1-INH) – A Real World ACARE Survey #P069: Marc A. Riedl, Markus Magerl, Marcus Maurer, Sherry Danese, Julie Ulloa, Vibha Desai, Paul K. Audhya. Results shared as an oral poster presentation and Q&A on Sunday, 12th November from 12:05 - 12:20 pm PT at Monitor 12
- Refining Prevalence Estimates of Hereditary Angioedema (HAE) with Normal C1-INH (HAE-nC1-INH) - #P070: Markus Magerl, Marc A. Riedl, Marcus Maurer, Sherry Danese, Julie Ulloa, Vibha Desai, Paul K. Audhya. Results shared as an oral poster presentation and Q&A on Sunday 12th November from 12:20 -

12:35 pm PT at Monitor 12

- Anxiety Associated with On-Demand Treatment of HAE Attacks May Result in Suboptimal Attack Management #P092: Michael Manning, Sally van Kooten, Markus Heckmann, Sherry Danese, Ledia Goga, Princess U. Ogbogu. Results shared as an oral poster presentation and Q&A on Sunday 12th November from 12:20 - 12:35 pm PT at Monitor 13

All poster presentations will take place in the ePoster Section of the Exhibit Hall.

(Source: KalVista)

2 November 2023

BioCryst reports third quarter 2023 financial results and provides business update

BioCryst Pharmaceuticals today reported financial results for the third quarter ended September 30, 2023, and provided a corporate update.

Jon Stonehouse, President and Chief Executive Officer of BioCryst, said: "With nearly three years of launch data, we have excellent visibility into the pattern of growth for Orladeyo and we remain on track to achieve the no less than $320 million in Orladeyo revenues we have expected for 2023. Our solid base and the consistent addition of new patients each quarter, combined with the ongoing improvement in our percentage of paid patients and launches in new global markets, are all driving peak revenues to $1 billion."

Program Updates
The company reported the following regarding Orladeyo (berotralstat):

- Orladeyo net revenue in the third quarter of 2023 was $85.7 million (+29.8 percent year-over-year (y-o-y)
- Total growth in patients taking Orladeyo continued on a strong, linear trajectory
- The ongoing APeX-P trial in pediatric HAE patients who are 2 to <12 years of age continues to enroll as expected
- Sales from outside the U.S. contributed 12.2 percent of global ORLADEYO net revenues in the third quarter
- Austria has approved the reimbursement of ORLADEYO for the targeted prophylaxis of hereditary angioedema (HAE) in patients 12 years of age or older

Third Quarter 2023 Financial Results
For the three months ended September 30, 2023, BioCryst total revenues were $86.7 million, compared to $75.8 million in the third quarter of 2022 (+14.4 percent y-o-y). The increase was primarily due to an increase in ORLADEYO net revenue of $19.7 million, compared to the third quarter of 2022.

(Source: BioCryst)

6 November 2023

Astria Therapeutics to Present New STAR-0215 Data at the 2023 American College of Allergy, Asthma, And Immunology Annual Scientific Meeting

Astria Therapeutics, a biopharmaceutical company developing a potential treatment for hereditary angioedema (HAE), announced that it will share new STAR-0215 data in a poster presentation at the upcoming American College of Allergy, Asthma, and Immunology (ACAAI) Annual Scientific Meeting on 11 November 2023 at 12:35pm Pacific Standard Time (PST).

Dr. William Lumry, M.D., Clinical Professor of Internal Medicine at the University of Texas Health Science Center at Dallas, will present additional data from the Phase 1a clinical trial of STAR-0215 in a poster titled, "Support for STAR-0215 Administered Every Three- or Six-Months for Hereditary Angioedema: Phase 1a Results" at 12:35pm PST in the ePoster Section – Exhibit Hall, Monitor 12, presentation number P066.

(Source: Astria)

6 November 2023

BioCryst announces expected timescales for US filing of Orladeyo for pediatric use during R&D day

BioCryst Pharmaceuticals reported that it expects to submit a U.S. supplemental new drug application for the pediatric use of ORLADEYO in 2025. The announcement came during an update on the company's pipeline at a Research and Development Day at the company's Discovery Center of Excellence in Alabama, United States.

Jon Stonehouse, President and Chief Executive Officer of BioCryst, said: "The success we are achieving with ORLADEYO demonstrates that when we deliver a first-in-class or best-in-class medicine, we can change patients' lives. We believe the combination of ORLADEYO, our exciting pipeline and our financial strength, position us to continue to make a big difference in patients' lives, and that leads to sustainable growth for years to come."

BioCryst provided an update on clinical trials of its pediatric formulation of Orladeyo. It indicated that the ongoing APeX-P clinical trial is assessing an oral granule formulation of ORLADEYO in pediatric HAE patients who are 2 to <12 years of age.

The company noted that approximately 40 percent of HAE patients have their first attack by five years of age and there are no current targeted oral therapies available for prophylaxis in children <12 years old.

(Source: BioCryst)

7 November 2023

Pharming Group announces presentations at American College of Allergy, Asthma & Immunology Annual Scientific Meeting in November 2023

Pharming Group announces poster presentations by the Company or its collaborators regarding Ruconest at the American College of Allergy, Asthma & Immunology (ACAAI) Annual Scientific Meeting in November 2023.

Two poster presentations relating to Ruconest will be presented at ACAAI:

- Managing primary immunodeficiency immunoglobulin replacement therapy-related adverse events: Prophylaxis with recombinant human C1 esterase inhibitor; Poster #M234: Presented Friday 10 November 2023 at 15:00 Pacific Time (PT), Anaheim Convention Center Exhibit Hall
- On-demand treatment for hereditary angioedema attacks: Role of individualized management strategy; Poster #M150: Presented 12 November 2023 at 12:15 PT, Anaheim Convention Center Exhibit Hall

In addition to displaying in the exhibit hall at the noted times, ePosters will be accessible online and on demand to registered attendees on Thursday, November 9, 2023, beginning at 08:00 PT on ACAAI's website.

(Source: Pharming)

9 November 2023

Ionis presents positive two-year results from the Phase 2 open label extension study of donidalorsen in patients with hereditary angioedema

Ionis Pharmaceuticals Inc today announced positive results from an ongoing Phase 2 open-label extension (OLE) study evaluating the safety and efficacy of its investigational prophylactic medicine, donidalorsen, in patients with hereditary angioedema (HAE).

Over the two years, patients treated with donidalorsen via subcutaneous injection showed an overall sustained mean reduction in HAE attack rates of 96% from baseline, from 2.70 to 0.06 attacks per month, across all dosing groups. Furthermore, all patients treated with donidalorsen reported a clinically meaningful improvement in quality of life as measured by the Angioedema Quality of Life Questionnaire (AE-QoL) over two years. Treatment with donidalorsen was well tolerated in the studies, and there were no serious adverse events.

The study was presented at the recent American College of Allergy, Asthma & Immunology (ACAAI) Annual Scientific Meeting in November 2023.

Richard S. Geary, Ph.D., executive vice president and chief development officer at Ionis, said: "Hereditary angioedema is a significant healthcare challenge for which there is an ongoing need for long-term, sustained prophylactic treatment offering patients significant efficacy and tolerability that is easy to use. We are very encouraged by the demonstrated safety, efficacy, and quality of life profile of donidalorsen. The two-year OLE results further support donidalorsen as a potentially compelling prophylactic treatment option for patients with hereditary angioedema. We look forward to reporting pivotal topline Phase 3 results in the first half of next year."

In a subset analysis also presented at the ACAAI Annual Scientific Meeting, 62.5% of patients receiving

donidalorsen 80 mg every eight weeks remained attack-free over the two-year period and had a sustained mean reduction in HAE attack rates of 83% from baseline.

Injection site (IS) discoloration and IS reaction were the only study drug-related treatment-emergent adverse events (TEAEs) reported in more than one patient (n=2, 11.8% each). No serious adverse events were reported in the OLE study, and no TEAEs led to study discontinuation.

(Source: Ionis)

10 November 2023

Astria Therapeutics presents new Phase 1a data confirming potential for STAR-0125 at the 2023 American College Of Allergy, Asthma, And Immunology Annual Meeting

Astra Therapeutics presented new STAR-0215 data at the American College of Allergy, Asthma, and Immunology (ACAAI) Meeting, that demonstrated STAR-0215's profile as a potential HAE preventative therapy with robust attack suppression and low treatment burden in healthy adult subjects. These data confirm the potential for STAR-0215 to be dosed once every three months and every six months.

Chris Morabito, M.D., Chief Medical Officer at Astria, said: "These new data, including long-term follow-up from the original cohorts and initial data from new, higher dose cohorts, support our vision for STAR-0215 as a first-choice therapy for HAE. These data confirm our approach to administer STAR-0215 once every three and every six months in future trials. We intend to provide patients the option to choose what works best for their lives with a therapy that has a trusted modality and mechanism.

"Our Phase 1b/2 ALPHA-STAR trial in HAE patients is on track and enrolling the third and final cohort. We now expect to deliver initial proof-of-concept data for STAR-0215 as a long-acting preventative therapy for HAE in Q1 2024. Patients from the ALPHA-STAR trial are enrolling in the long-term open label ALPHA-SOLAR trial to continue receiving STAR-0215, with data now accruing in patients who have received multiple doses of STAR-0215."

(Source: Astria)

10 November 2023

BioCryst presents new real-world data showing reduced attack rates in patients with HAE with normal C1-inhibitor following long-term treatment with Orladeyo (berotralstat)

BioCryst Pharmaceuticals Inc today announced new analyses of real-world use of oral, once-daily Orladeyo (berotralstat) leading to a reduction in monthly attack rates in patients with hereditary angioedema (HAE) who have normal C1-inhibitor (C1-INH) level and function.

Dr Ryan Arnold, Chief Medical Officer of BioCryst, said: "There is a significant unmet need among people who live with HAE with normal C1-INH, and identifying optimal treatments has been challenging for these patients. These real-world observations suggest ORLADEYO can have a meaningful impact on the lives of people who have HAE with normal C1-INH. We look forward to continuing to evaluate our oral, once-daily prophylaxis as a treatment option for this subpopulation."

The company also announced a new post-hoc analysis from the APeX-S clinical trial that showed a sustained reduction in HAE attacks compared to patients' self-reported baseline attack rates.

The data were presented at the 2023 Annual Scientific Meeting of the American College of Allergy, Asthma & Immunology (ACAAI), which is being held at the Anaheim Convention Center in Anaheim, Calif., from 9-13 November 2023.

(Source: BioCryst)

13 November 2023

Orchard Therapeutics reports third quarter 2023 financial results and highlights recent business accomplishments

Orchard Therapeutics announced several business accomplishments along with its financial results for the quarter ended 30 September 2023.

Bobby Gaspar MD PhD, Chief Executive Officer of Orchard Therapeutics, said: "With strong execution across our key commercial, regulatory and clinical priorities, 2023 has been a transformational year for Orchard Therapeutics, culminating with the announcement of the planned acquisition by Kyowa Kirin."

Selected HAE 2023 Expected Milestones
Orchard Therapeutics has outlined that for the remainder of 2023 it expects to advance the company's other pre-clinical programs, which includes OTL-204 in the progranulin form of FTD and OTL-105 partnered with and funded by Pharming Group N.V. in hereditary angioedema (HAE).

(Source: Orchard)

13 November 2023

Astria Therapeutics reports third quarter financial results and provides a corporate update

Astra Therapeutics reported financial results for the third quarter ended 30 September 2023 and provided a corporate update.

Jill C. Milne, PhD, Chief Executive Officer at Astria Therapeutics, said: "We are realizing our vision of growing an allergy and immunology company with a pipeline of potential best-in-class therapies. The STAR-0215 program is in a strong position, and data from the Phase 1a healthy subject trial recently shared at ACAAI support the potential to provide patients with dosing options two or four times per year, without compromising on safety or efficacy. We now expect to deliver initial proof-of-concept results in Q1 2024 from the Phase 1b/2 ALPHA-STAR trial in HAE patients. We are excited about the future for Astria as we work towards important milestones for STAR-0215 and STAR-0310 next year."

STAR-0215

- Additional results from the Phase 1a trial were shared at the ACAAI Annual Meeting in Anaheim, CA. STAR-0215 was well-tolerated with no serious adverse events or discontinuations due to an adverse event, and low risk of injection pain. STAR-0215 achieved potentially therapeutic levels in less than one day and showed an estimated half-life of up to 127 days. Pharmacokinetic (PK) modeling of every 3 month and every 6 month clinical dose regimens predict concentrations that will continuously maintain drug levels believed to be sufficient for HAE attack prevention. Pharmacodynamic (PD) data showed statistically significant inhibition of plasma kallikrein for 140 to 224 days after single doses greater than 100 mg. These results demonstrate 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.
- The ALPHA-STAR Phase 1b/2 trial of STAR-0215 in people with hereditary angioedema (HAE) is on track and enrolling the third and final cohort. Initial proof-of-concept results are now 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, PK, PD, and quality-of-life assessments. For each dose cohort, efficacy will be assessed at 3 months and 6 months after the last STAR-0215 dose administered.
- A Long-Term Open-Label Trial named ALPHA-SOLAR has been initiated and is enrolling participants from ALPHA-STAR, with data now accruing in participants who have received multiple doses of STAR-0215. The trial is assessing the long-term safety, tolerability, and efficacy of STAR-0215. Participants are receiving STAR-0215 every three or six months.
- Pending proof-of-concept results from the ALPHA-STAR trial, Astria expects to progress directly to a pivotal Phase 3 trial which is anticipated to initiate in the first quarter of 2025.
- Additional preclinical results were shared in the Journal of Pharmacology and Experimental Therapeutics that support STAR-0215's potential as a best-in-class plasma kallikrein inhibitor.
- Astria presented at the Hereditary Angioedema Association (HAEA) National Summit in July, and the Hereditary Angioedema International EMEA meeting in September, sharing results from a research survey where patients prioritized attack-free status as their most important target for therapeutic efficacy in HAE clinical trials.

(Source: Astria)

13 November 2023

KalVista Pharmaceuticals announces Phase-3 KONFIDENT trial milestone achieved

KalVista Pharmaceuticals provided clinical trial and regulatory updates for its lead program sebetralstat, in development as a potential oral on-demand therapy for hereditary angioedema (HAE).

Clinical Trial and Regulatory Updates:

- KalVista has achieved the targeted number of on-treatment attacks required to complete the phase 3 KONFIDENT trial. The trial is a cross-over study in which patients are intended to treat a total of three attacks: one each with 300 mg sebetralstat, 600 mg sebetralstat and placebo, given in a randomized sequence.
- Topline data readout is expected in early 2024, remaining on track for a New Drug Application (NDA) submission to the US Food and Drug Administration (FDA) in the first half of 2024. The Company also expects to file for approval in the European Union and Japan later in 2024.
- KONFIDENT randomized a total of 136 participants from 66 sites across 20 countries, making it the largest clinical trial ever conducted in HAE based on number of subjects. The enrolled patients are representative of the global HAE population and include participants 12 years of age and above, with or without long-term prophylaxis, with all attack locations eligible for treatment, including the larynx.
- In addition, the KONFIDENT-S open label extension study continues to enroll, and the Company expects it will provide a robust safety database to support the planned NDA filing. In total, more than 600 attacks have been treated across KONFIDENT and KONFIDENT-S, and KONFIDENT-S includes numerous patients who have taken multiple doses for treatment as well as short-term prophylaxis.

Andrew Crockett, Chief Executive Officer of KalVista, said: "We are excited to have reached the number of on-treatment attacks required for completion of KONFIDENT. We have now initiated study closeout activities which enables topline data readout in early 2024, maintaining the timing of our planned NDA submission in the first half of 2024. We are grateful and highly encouraged by the overwhelming interest in this trial from people living with HAE and, if approved, we look forward to introducing a novel therapeutic that offers the potential to transform the treatment of this disease."

(Source: KalVista)

14 November 2023

KalVista Pharmaceuticals presents real-world data at the 2023 Annual Scientific Meeting of the American College of Allergy, Asthma & Immunology

KalVista Pharmaceuticals presented real-world data on anxiety associated with the use of injectable on-demand treatments for hereditary angioedema (HAE) attacks and patient perspectives on why people living with HAE don't often carry their on-demand treatment outside the home, at the 2023 Annual Scientific Meeting of the American College of Allergy, Asthma & Immunology (ACAAI).

Andrew Crockett, Chief Executive Officer of KalVista: "We continue to learn from people living with HAE. It is our hope that if sebetralstat is approved for the on-demand treatment of HAE, it may be able to address significant unmet need and help transform the management of the disease."

In addition to the data on persisting non-medication health resource utilization among patients receiving long-term prophylaxis, the Company presented data on the global prevalence of HAE-nC1-INH the approach to the presumptive diagnosis, and the highly varied treatment approaches that represent another significant area of unmet need in that population.

The following presentations occurred at ACAAI 2023:

- Anxiety Associated with On-Demand Treatment of HAE Attacks May Result in Suboptimal Attack Management: Michael Manning, Allergy, Asthma & Immunology Associates, Ltd., Internal Medicine, UA College of Medicine-Phoenix, Scottsdale, Arizona, United States (Poster Presentation)
- Understanding Why Hereditary Angioedema Patients Often Do Not Carry Their On-Demand Treatment with Them: Bob Geng, Allergy and Immunology, University of California, San Diego, California, United States (Poster Presentation)
- Treatment Patterns Among Individuals with Hereditary Angioedema in the United States: Raffi Tachdjian, University of California, Los Angeles, Los Angeles, CA, United States (Poster Presentation)
- Refining Prevalence Estimates of Hereditary Angioedema (HAE) with Normal C1-INH (HAE-nC1-INH): Markus Magerl, Angioedema Center of Reference and Excellence (ACARE), Institute of Allergology, Charité – Universitätsmedizin Berlin, corporate member of Freie Universität Berlin and Humboldt-Universität zu Berlin, Berlin, Germany (Poster Presentation)
- Diagnosing Hereditary Angioedema (HAE) with Normal C1-INH (HAE-nC1-INH) – A Real World ACARE Survey: Marc A. Riedl, Division of Rheumatology, Allergy and Immunology, University of California – San Diego, San Diego, California, United States (Poster Presentation)
- Treatment of Hereditary Angioedema (HAE) with Normal C1-INH (HAE-nC1-INH) – A Real-World ACARE Survey: Marcus Maurer, Angioedema Center of Reference and Excellence (ACARE), Institute of Allergology, Charité – Universitätsmedizin Berlin, corporate member of Freie Universität Berlin and Humboldt-Universität zu Berlin, Berlin, Germany (Poster Presentation)

(Source: KalVista)

14 November 2023

Intellia Therapeutics receives European Union orphan drug designation for NTLA-2002, an investigational in-vivo CRISPR genome editing treatment for Hereditary Angioedema

Intellia Therapeutics today announced that the European Commission (EC) has granted orphan drug designation to NTLA-2002 for the treatment of hereditary angioedema (HAE).

NTLA-2002 is an in vivo CRISPR-based investigational therapy designed to prevent potentially life-threatening swelling attacks in people with HAE.

John Leonard, MD, Intellia President and Chief Executive Officer, said: "The European Union orphan drug designation for NTLA-2002 represents another important milestone for Intellia as we continue to make rapid progress in the development of a novel, potential one-time treatment for people with hereditary angioedema. We are on track to complete enrollment of the Phase 2 portion of the study in the coming weeks, which will bring us one step closer to our goal of delivering a potentially life-changing treatment for people who suffer from this serious and debilitating disease."

Orphan drug designation in the European Union (EU) is granted by the EC based on a positive opinion issued by the European Medicines Agency (EMA) Committee for Orphan Medicinal Products. To qualify for orphan drug designation, a candidate therapy must be intended for the treatment, prevention or diagnosis of a life-threatening or chronically debilitating disease that occurs in not more than five in 10,000 people in the EU. The designation provides regulatory, financial and commercial incentives to develop therapies for rare diseases where there are either no satisfactory treatment options or significant benefit to those affected by the disease.

(Source: Intellia)

17 November 2023

European Commission approves label update for lanadelumab, for a broader group of pediatric patients with recurrent attacks of hereditary angioedema (HAE)

Takeda today announced the European Commission has approved Takhzyro (lanadelumab) for the routine prevention of recurrent attacks of Hereditary Angioedema (HAE) in patients aged 2 years and older1, expanding its initial approved use and making it the first long-term prophylactic treatment of HAE available in European Economic Area for patients under the age of six.2,3,4

The recently approved extension of the indication in pediatric patients was paired with an additional strength of 150 mg for lanadelumab solution for injection in pre-filled syringe. TAKHZYRO® 150mg should be used in patients aged 2 years and older and weighing less than 40 kg to prevent angioedema attacks in patients with Hereditary Angioedema (HAE).1

"Potentially fatal upper airway angioedema has been reported in patients as young as three years old5, presenting an acute unmet need in some of the most vulnerable of HAE patients." said Didier Relin, Head of International Regulatory at Takeda. "With this expanded label, Takhzyro offers a welcomed new preventative treatment option for the pediatric HAE patient population, and one that can be administered at home with the support of a trained caregiver."

(Source: Takeda)

21 November 2023

BioCryst Launches Orladeyo (berotralstat) in Spain

BioCryst Pharmaceuticals Inc today announced that the Spanish Ministry for Health (Ministerio de Sanidad) has granted marketing authorization for oral, once-daily Orladeyo (berotralstat) for the routine prevention of recurrent hereditary angioedema (HAE) attacks in HAE patients 12 years and older.

"We have an experienced and motivated team in Spain that is enthusiastic about the impact the availability of the first oral, once daily therapy can have for people with HAE in Spain," said Charlie Gayer, chief commercial officer of BioCryst. "Spanish patients and physicians have shared with us the need to expand treatment options and we are excited now to be in a position to support access to those who can benefit most."

(Source: BioCryst)

27 November 2023

Astria Therapeutics to present STAR-0215 data at the 2023 World Allergy Congress

Astra Therapeutics today announced that it will share STAR-0215 Phase 1a results in an encore presentation at the annual World Allergy Congress on Sunday 3 December 2023 at 10:00am ICT in Bangkok, Thailand.

Chris Morabito, M.D., Chief Medical Officer at Astria Therapeutics, will present a poster titled: "Support for STAR-0215 Administered Every Three- or Six-Months for Hereditary Angioedema: Phase 1a Results," in Convention Hall B, poster board number 246.

(Source: Astria)

29 November 2023

BioCryst announces approval of Orladeyo (berotralstat) by the National Administration of Drugs, Foods and Medical Devices (ANMAT) in Argentina

BioCryst Pharmaceuticals Inc today announced that the National Administration of Drugs, Foods, and Medical Devices (ANMAT) in Argentina 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 continue to make strides to bring Orladeyo to patients living with HAE in Latin America in collaboration with our partner, Pint Pharma. We applaud ANMAT's decision to grant approval to our oral, once-daily prophylactic therapy for HAE, which paves the way for patients living with HAE to receive Orladeyo to help improve management of their HAE attacks."

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)

6 December 2023

Pharvaris announces positive top-line phase 2 data from the CHAPTER-1 study of deucrictibant for the prophylactic treatment of HAE attacks

Pharvaris announced positive top-line data from the CHAPTER-1 Phase 2 clinical study meeting its primary endpoint, with deucrictibant demonstrating statistically significant and clinically meaningful results of deucrictibant as an oral preventative treatment for people living with HAE. Pharvaris plans to present data from the study at future medical meetings.

The study's primary endpoint 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.

Marc A Riedl, MD, MS, Professor of Medicine, Clinical Director of the US Hereditary Angioedema Association (HAEA) Angioedema Center at the University of California San Diego (UCSD), Clinical Service Chief for Allergy/Immunology at UCSD, and principal investigator in the CHAPTER-1 study, commented: "The HAE community is seeking highly effective, well-tolerated, and less burdensome therapies. The CHAPTER-1 data represent an important step forward in the evolution of HAE treatment. Given these encouraging results, deucrictibant has the potential to significantly improve clinical outcomes for people living with HAE."

Peng Lu, MD, PhD, Chief Medical Officer of Pharvaris, stated: "Deucrictibant is the first HAE treatment with the potential to combine injectable-like efficacy and a favorable safety profile with the convenience of an oral therapy. The study demonstrates, for the first time ever, that antagonism of the bradykinin B2 receptor can provide early and sustained protection from HAE attacks, including substantial reduction of moderate and severe attacks, with clinically meaningful improvement in health-related quality of life. We look forward to advancing the development of deucrictibant for the prevention of HAE attacks."

Berndt Modig, Chief Executive Officer of Pharvaris, added: "We sincerely thank the clinical trial participants and their caregivers, the site investigators and staff, the HAE community, and the Pharvaris team for their contributions to the CHAPTER-1 study. These study results, together with the compelling data from our on-demand program, further strengthens our confidence that deucrictibant can become the preferred option to treat as well as prevent HAE attacks."

(Source: Pharvaris)

7 December 2023

KalVista Pharmaceuticals reports second fiscal quarter results and operational update

KalVista Pharmaceuticals provided an operational update and released financial results for the second fiscal quarter ended 31 October 2023.

Andrew Crockett, Chief Executive Officer of KalVista, said: "We are in the process of completing the KONFIDENT trial and we look forward to advancing sebetralstat for an NDA submission in the first half of 2024. As we plan on several regulatory filings in the coming year, we are also continuing to build out our commercial organization in order to support a rapid launch upon FDA approval."

Second Fiscal Quarter and Recent Business Highlights:

Achieved the number of on-treatment attacks required to complete the phase 3 KONFIDENT trial. Topline data readout is expected in early 2024, remaining on track for a New Drug Application (NDA) submission to the US Food and Drug Administration (FDA) in the first half of 2024. The Company also expects to file for approval in the European Union and Japan later in 2024

Received Orphan Drug Status (ODS) approval from Switzerland for sebetralstat, allowing for shorter review time, priority authorization, and 15 years market protection and exclusivity

Presented real-world patient data at the 2023 Annual Scientific Meeting of the American College of Allergy, Asthma & Immunology discussing compliance challenges of currently available parenteral on-demand HAE treatments and evidence of the global prevalence of normal C1-inhibitor HAE (HAE-nC1-INH)

Reported data at the Asia Pacific Association of Allergy, Asthma and Clinical Immunology 2023 International Conference characterizing HAE attacks in patients receiving long-term prophylaxis (LTP)

(Source: KalVista)

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.