
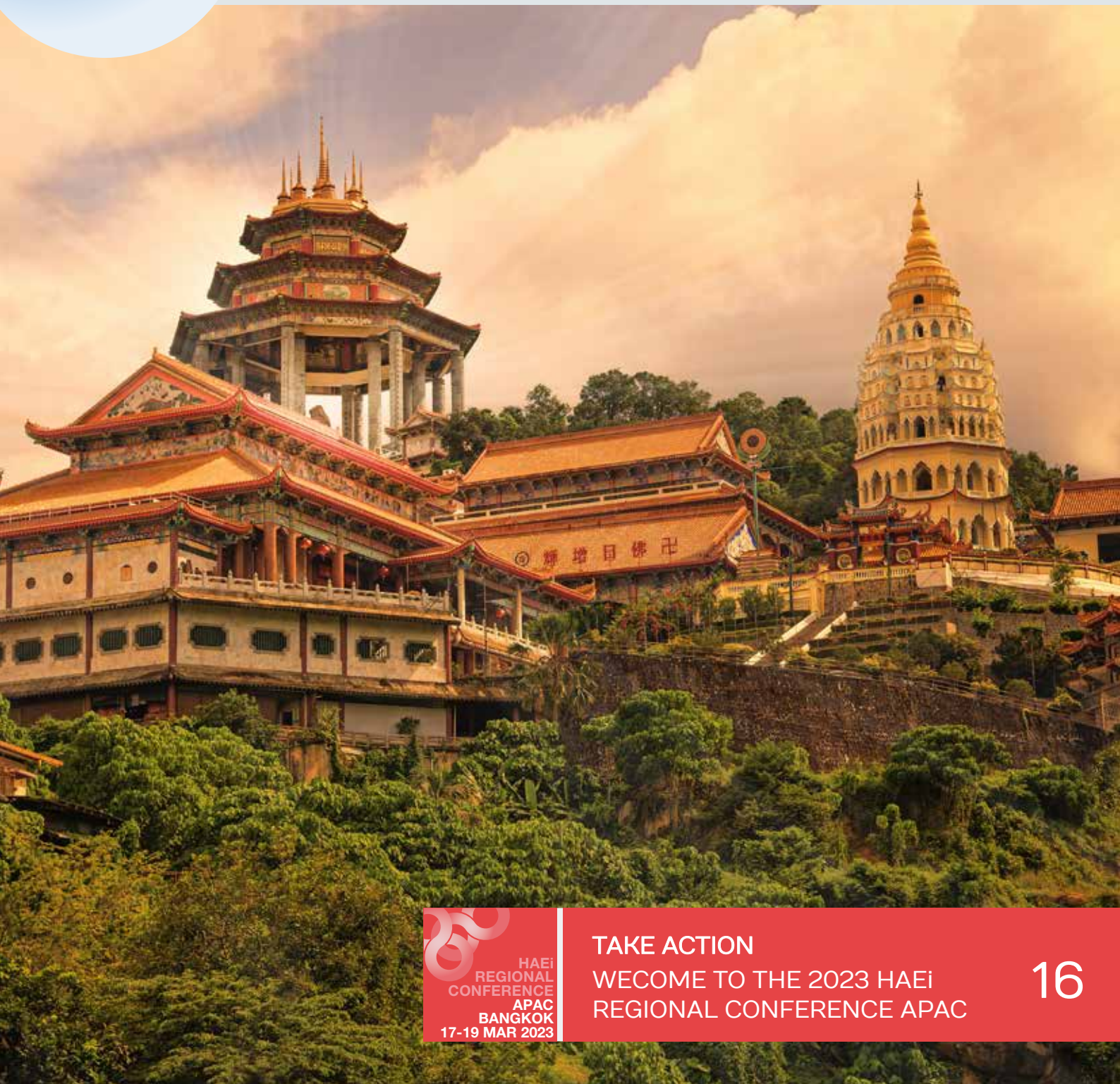


Global perspectives:

HAEi MAGAZINE · ISSUE 4/2022

 **95** Member countries




HAEi
REGIONAL
CONFERENCE
APAC
BANGKOK
17-19 MAR 2023

TAKE ACTION

WELCOME TO THE 2023 HAEi
REGIONAL CONFERENCE APAC

16

SUCCESSFUL 2022 HAEi GLOBAL
LEADERSHIP WORKSHOP

18

LET'S HANG OUT AT THE HAEi
YOUNGSTERS' HANGOUT

22

Cover photo

Take Action is the theme of the 2023 HAEi Regional Conference APAC. Read more on page13

Magazine staff

Henrik Balle Boysen, Executive Vice President and COO

Rikke Sørensen, Communication Design and Graphics Manager

Steen Bjerre, Editor-in-Chief

Subscription

If you would like to subscribe to our magazine, please send an email to info@haei.org or register directly at haei.org

HAEi 2022 activities are supported by:



CSL Behring



BiOMARIN IONIS



HAEi is registered as a non-profit organization in USA

HAEi is a global non-profit umbrella organization dedicated to working with a network of national HAE member organizations to raise awareness of HAE



DEAR HAEi FRIENDS,

On behalf of HAEi's Board of Directors, I wish you joy and good health during the holiday season!

2022 brought the easing of COVID-19 restrictions and, in most countries, the return of in-person gatherings. HAEi seized upon the world's reopening in October by conducting the first HAEi Global Leadership Workshop that brought Member Organization leaders together along with expert HAE physicians/scientists and the pharmaceutical industry. The feedback provided by many of the approximately 500 participants from 75 countries points to a highly successful Workshop, with over 98 percent of attendees rating the gathering as extremely useful or useful.

HAEi continues to focus its programs, activities, and services on helping our Member Organizations grow and sharpen their advocacy activities to win access to and reimbursement for approved HAE therapies. In addition, our Regional Patient Advocates (RPAs) are always working to help people form an HAE organization in countries where one doesn't already exist.

As we turn the last calendar page of 2022, I would like to highlight some key HAEi services that are available to our community:

HAEi TrackR – an easy-to-use electronic diary designed to record your HAE treatments, attacks, and the impact HAE has on your life.

HAEi Advocacy Academy – an online learning platform for the HAE Community that supports people living with HAE in their everyday life and offers training to help our HAEi member organizations sharpen their advocacy skills.

HAEi Connect – a user-friendly, yet sophisticated turnkey patient membership database and communications platform.

Website Hosting – HAEi helps Member Organizations design their website and provides secure hosting services.

HAEi Companion – ideal when traveling, the app contains digital versions of HAEi Emergency Cards in multiple languages along with a list of hospitals, physicians, and Member Organization contact information for whatever country the user is visiting.

HAEi Virtual Centers – an HAEi-designed platform that connects people with chronic swelling disorders (who may live in remote areas or otherwise cannot travel) to expert angioedema physicians.

ACARE Network – Angioedema Centers of Reference and Excellence (ACARE), a joint venture between the Global Allergy and Asthma European Network GA2LEN and HAEi, now has 75 certified centers in 34 countries and another 18 applicant centers in the process of being certified.

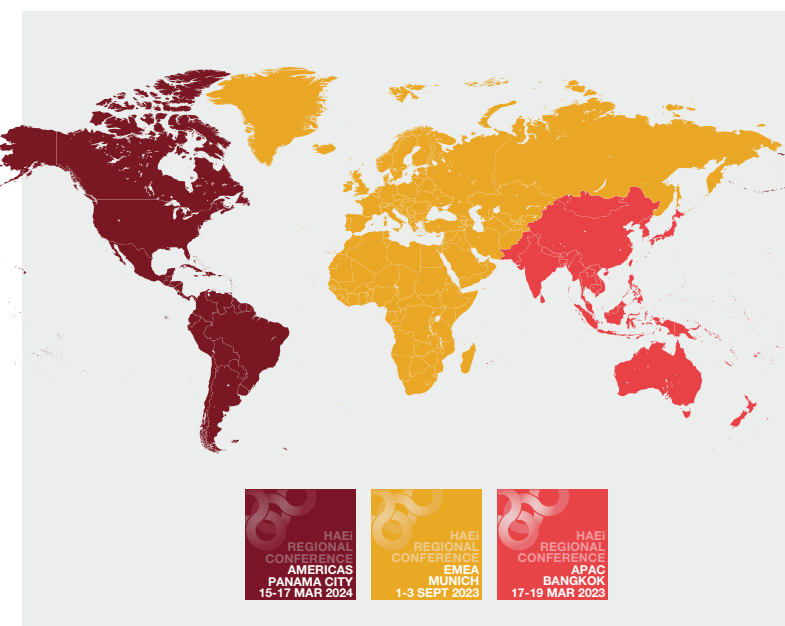
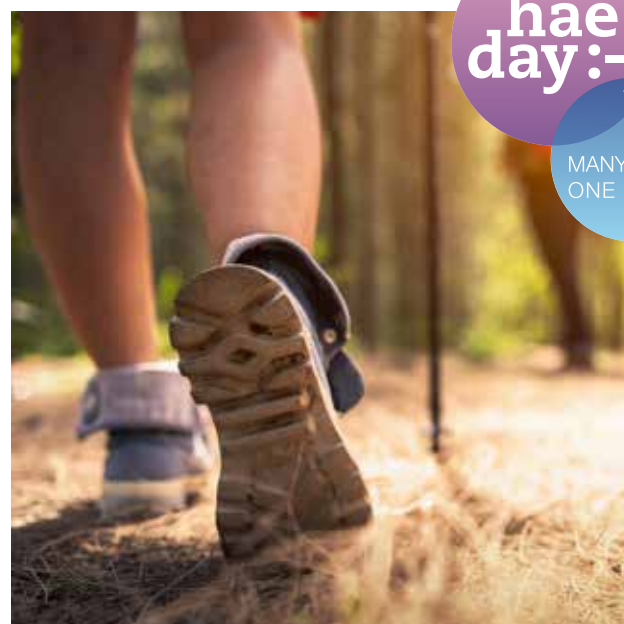
We encourage HAEi Friends to work with their member organization and RPA and take full advantage of these and other services provided by HAEi.

Let's continue to accelerate the global HAE patient movement in 2023!

Warmest holiday greetings,

Anthony J. Castaldo
President and CEO, HAEi

IN THIS ISSUE OF GLOBAL PERSPECTIVES



- 06 News from the HAEi Regional Patient Advocates
- 54 Follow HAEi on Social Media
- 13 TAKE ACTION. Welcome to the 2023 HAEi Regional Conference APAC
- 17 HAE Companion –Your Pocketsize Travel Partner

- 18 Successful 2022 HAEi Global Leadership Workshop
- 20 Celebrating 2022 with High Expectations for the New Year
- 21 One Small Step ... One Giant LEAP!
- 22 Let's Hang Out at the HAEi Youngsters' Hangout!
- 24 HAEi Goes Regional

- 26 ACARE Network Grows to 75 Centers
- 27 **hae day :-)** 2023 is Around the Corner
- 28 Have You Wondered How to Get Your HAE Under Control? **HAE TrackR** Can Help You! NEW: Version 2!
- 30 News for Member Organizations Around the World
- 40 Medical Papers

- 42 Clinical Trials
- 45 Understanding HAE
- 46 News from the Industry
- 55 HAEi Advocacy Academy – Your First Step to Successful Advocacy
- 56 HAEi Around the World

NEWS FROM THE HAEi REGIONAL PATIENT ADVOCATES

There was a buzz in the air in October, and for a good reason, as many of us were “Together Again” in Frankfurt, Germany!

It was an incredible feeling to be in one place with our global family, meet new friends, and catch up with those we haven’t seen in person for so long. The **2022 HAEi Global Leadership Workshop** was a great success, and we thank everyone who contributed and participated.

As shown in the updates from the Regional Patient Advocates (RPAs), the momentum that started at the workshop continues as our Member Organizations apply the lessons learned and implement programs and services offered by HAEi.

As we head toward the end of 2022, we can reflect on a year filled with uncertainty because of remaining COVID-19 concerns, but confidently start 2023 with a revitalized sense for the future. I look forward to working with my incredible RPA team, who have so much passion and commitment to assist the countries in their regions to strive for a better future for people with HAE.

Fiona Wardman
Chief Regional Patient Advocate



MARIA FERRON
THE MEDITERRANEAN, NORTH AFRICA
AND THE BRITISH ISLES



The 2022 HAEi Global Leadership Workshop held in October was an excellent chance for everyone to share their experiences, see all the new projects and activities HAEi has implemented, and meet face-to-face with the HAE community and stakeholders. The workshop gave the Member Organizations in the region I am dealing with the time to learn more and think about the next steps to take in the coming months. Here are some examples of the workshop outcome:

- Algeria took an interest in enrolling in one of the scientific-based research that HAEi conducts to provide data to Member Organizations that can be used in their advocacy efforts. They will also be investigating with their scientific committee the infrastructure and local regulations for pharmaceutical companies to do clinical trials.
- Spain and France will start using the HAEi project management team as the only channel to conduct market research and surveys in the country; and they will be using the new HAEi “Understanding HAE” patient guide as a source of information for their members.
- Morocco has shown interest in enrolling in one of the scientific-based research that HAEi conducts and in HAEi Connect.
- Portugal wanted to learn more about managing their website hosted under HAEi fully, so we set up a call with the operations teams to provide them with more information. They will also investigate with their doctor lead the infrastructure and local regulations for pharmaceutical companies to do clinical trials.



I am happy to announce that Ruconest is now available in Morocco, but at this point, with no reimbursement. A meeting took place in Casablanca to introduce the new treatment and the association with the distributor, Laprophan, and Pharming’s sponsorship.

And finally, I would like to announce that HAE Spain (AEDAF) is planning next year’s **hae day :-)** activity. You can find more information on this in the “News for Member Organizations Around the World” section.



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



The primary objective for the last three months was the 2022 HAEi Global Leadership Workshop in Frankfurt, Germany. September with the preparations, October with the actual workshop, and then the follow-up. The workshop itself was a very successful event – not only for HAEi but certainly also for the participants. Meeting people from the HAE Community from all around the globe for the first time in more than four years was genuinely unique. Meeting again, reconnecting, and sharing ideas and thoughts – I believe everyone took so many good ideas, input, and, not least, motivation home with them. I know for sure I did.

The workshop offered time to network, meet with the region I represent, hear the latest updates from the healthcare professionals and from HAEi, and meeting with our friends from the industry. All three “components” vital for the great collaboration in the HAE Community.

After the workshop, it is now crucial for all of us to remember the many good stories, the tools and services, and all the input given and select the next steps to take. Whether this is considering hosting the website via HAEi’s platform, building your member database via HAEi Connect as a secure, GDPR-compliant solution, or adapting HAEi Advocacy Academy. Maybe you would like to translate the new “Understanding HAE” patient guide giving precise and relevant information about HAE and leaving space for you to include information about your national organization as well – and to be used as a digital and/or printed tool. The benefits of HAE Companion and HAE TrackR are obvious – two apps that truly offer a clear advantage for all HAE patients and caregivers, whether it is when traveling or taking control of your HAE. Or it might be to involve youngsters from your country in the LEAP program introduced at the workshop.

The above mentioned are just some of the tools presented and discussed at the workshop – all great tools either to help the operation of an organization or to help patients and caregivers directly in their daily life.

Together with the renewed motivation gained at the Global Leadership Workshop, many things are still going on, also in my region.

We are still working on putting together our Regional Medical Advisory Panel for the region. Several participants are confirmed, and they are already feeding in very valuable information and ideas. Other countries have not yet been able to appoint a member of the panel. We are looking forward to continuing the dialogue with the panel to benefit even more from the collaboration.

Similarly, feedback from the Regional Advisory Groups has come in from many of the countries in the region. This will enable us to benefit from the experience of the organizations and to use the ideas in the tools and services provided by HAEi.

As mentioned, HAE TrackR continues to be a great tool and is now available in 30 languages – several of which are spoken in my region (Danish, Finnish, French, German, Norwegian, and Swedish). The HAE TrackR app is easy to use – and a safe way to store your data – and it will help the patient and physician in their dialogue on current and future treatment options. I can only urge everyone to check it out – and to start using it! And soon, a new release will be ready.

The HAE Companion app deserves another mention. It is available in both Apple App store and Google Play, and truly is a great tool. This app will help you find local HAE treatment centers and directions hereto, as well as you will find emergency cards available in more than 80 countries. You can download an emergency card in the local language of the country you are visiting to your smartphone, e.g., to Apple’s Wallet and Android’s Passbook. A great help when you are traveling to a country to have the emergency cards ready in the local languages. Or to have the emergency card in your own language available on your smartphone when being at home.

Sweden recently hosted two patient meetings, one in Malmö in October and one in Stockholm in November. Both with a fantastic show up from patients, caregivers, healthcare professionals, and industry. In December, Norway hosted a similar meeting in Bergen.

Norway, Sweden, and Denmark – united in HAE Scandinavia – are working on exciting projects for kids and youngsters. Soon a series of podcasts about being young with HAE will be launched as the recording has taken place, as has a series of podcasts in Switzerland about HAE.

As mentioned, the major priority during the last three months was undoubtedly the Global Leadership Workshop. It was an excellent opportunity to learn more about the advances within the research of HAE, what is

new on the horizon, and what to be aware of. Also, it was such a great opportunity to be able to present all the tools, services and programs that HAEi can offer all the Member Organizations. Both tools, services and programs that many Member Organizations already know and utilize daily and completely new announcements that will bring collaboration and advocacy to a new level. For me personally, to see all the people again was unbelievable. For the first time since COVID-19 shut down the world, we could get so many people together. I dare say I am not alone in that feeling. It was clear that both during the program sessions and the dinners, people really enjoyed being “Together Again”. Onwards to the next gathering, it will be exciting to see all again – and hopefully also many new patients and caregivers.



PATRICIA KARANI
SUB SAHARA AFRICA



In Mozambique, new HAE patients have been identified and connected to a local doctor for better care, while in Namibia, a new patient family has been located, and all are currently doing well on locally available medications.

Dr. Sadia Habiba is a new physician contact in Chad who has started sharing awareness materials with fellow doctors in the country.

HAE Kenya received its registration certificate as a community-based organization for patients with HAE.



JAVIER SANTANA
CENTRAL AMERICA AND THE CARIBBEAN



Much of my time has been concentrated on Costa Rica, where a young mother with HAE has been hospitalized several times due to severe crises. Unfortunately, the country's main hospital has refused to purchase and provide one of the two treatments already authorized in Costa Rica to counteract HAE attacks. HAEi and I, as Regional Patient Advocate, have maintained communications with government officials, doctors, and the media to make our opinion heard and demand that the medicine be provided to the patient who is still hospitalized. Together with HAE Costa Rica, the ANASOVI Association of Costa Rica, and legal representatives of the patients, we will continue our efforts until access to these drugs for patients with HAE in that country is achieved.

I have had conversations with local doctors and physicians from other countries to create a professional chain of reactions about the theme of HAE in Costa Rica. Also, I gave interviews to talk about the situation regarding medications and HAE patients. Furthermore, I communicated with government officials to help the specific patient. The government has informed me that it has authorized the purchase of the drugs, but at this point, none has been supplied to the patient.

If you would like to show your support to HAE Costa Rica in the efforts to help the young patient, please visit www.facebook.com/aehcostarica.

After the 2022 HAEi Global Leadership Workshop in Frankfurt, Germany, many Member Organization leaders in my region have been organizing new plans for their countries to share the workshop information with the members. I have offered to help them to present the different HAEi programs and explain them to other patients and doctors.

Recently I have been in contact with Rosa Maria Pérez, the national contact of HAE Cuba. Fortunately, Cuba has managed to acquire Berinert in case of severe attacks where the health and life of the patient is exposed. I

have put Rosa Maria and her doctor in contact with doctors from Colombia and Panama so the doctor could have more information from colleagues treating patients in their countries.

I have also been communicating with the HAE El Salvador national contact Raquel Fuentes. She has informed me about her new role as the group leader and how the workshop gave her more positive energy to get treatment in her country. She has coordinated a meeting in a hospital to speak with medical personnel about HAE, and she will give an interview to a national newspaper. Information about HAE and graphics designed by HAEi has been sent to her to use during the interviews and to share with the media.



FERNANDA DE OLIVEIRA MARTINS
SOUTH AMERICA AND MEXICO



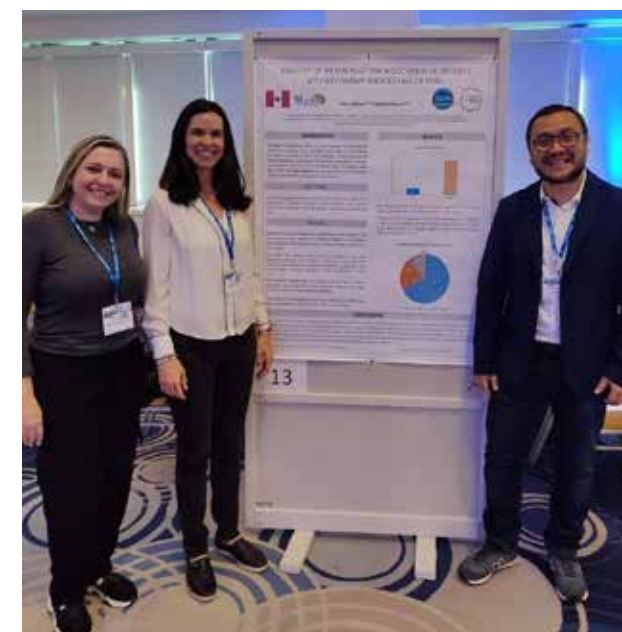
It was amazing to be "Together Again" in Frankfurt, Germany, for the 2022 HAEi Global Leadership Workshop. I thank all the Member Organizations and the physicians from South America and Mexico for the effort and enthusiasm taken to the meeting, as well as the HAEi community for the amazing event. These were intense days – full of energy, knowledge, hope, and joy.



Besides the global program, we had time for a regional meeting, a time for us to reconnect, celebrate and imagine the future! We are already looking forward to the Regional Meeting for the Americas in 2024 :-)

The Global Leadership Workshop definitely gave extra energy to the Member Organization leaders in my region. All came back from Frankfurt full of ideas and eager to implement more HAE tools in the countries. For the last few weeks, we have been working on implementing more Emergency Room posters and publishing new content in Spanish and Portuguese on social media.

The Burden of Illness survey being run in Mexico is almost done, and we aim to run it in other countries. Also, the LEAP program presented in Frankfurt has attracted much attention, and many youngsters from my region have applied for it.



Dr. Olivares from Colombia, Fernanda de Oliveira Martins, and Dr. Calderon from Peru



Representatives from HAE Brazil (ABRANGHE) with their poster



NATASA ANGJELESKA
SOUTH EASTERN EUROPE



According to the representatives from countries in my region, the most important event of 2022 is definitely the HAEi Global Leadership Workshop held in Frankfurt, Germany. There was a lot of positive energy, and it was terrific to meet face to face. All the sessions were excellent, and there has been a huge increase in activity and movements after the workshop. I would like to share a few words about the break-out session with participants from my region. First, they all appreciated being part of this event, and especially for those participating for the first time, it was a mind-blowing experience as they could feel the power of our global organization. They are delighted to be part of it. Another good opportunity was to discuss among ourselves the challenges and successes that have happened in the past two years after our latest regional conference in Skopje, North Macedonia. A lot has improved in terms of diagnosis and access to treatment, for example, in Serbia and Montenegro. Most importantly, having access to preventive therapies in these countries, as in Serbia, Greece, and North Macedonia, is very valuable and motivating for others. There were discussions and interest shown for different HAEi resources, and it was a busy period to meet the needs of all interested in translating and using the resources like the Emergency Room poster, social media cards, the “Understanding HAE” booklet, Event Zoo, etc.

I have written to the Member Organizations’ representatives about the HAEi LEAP program, encouraging them to find out if there were youngsters in their country who would apply and be part of this opportunity for young HAE patients, caregivers, family members, and the patient organization.

Furthermore, I have emailed all Member Organization representatives offering them to translate the “Understanding HAE” booklet. HAE Albanian informed me that they had it translated immediately, as it was helpful for them.

Ersan Sevinc from HAE Turkey has asked me to send

him templates for translating social media cards, Emergency Room posters, and “Understanding HAE”. He is also communicating with the HAEi Staff regarding redesigning the Turkish website and using Event Zoo for upcoming online and in-person patient meetings.

Teja Iskra has requested to translate the Emergency Room poster into Slovenian. She also shared the news that she is very encouraged to start the formal registration of the patient association.

In October 2022, our friends in Albania had their first activity as a patient organization advocating for therapies for HAE patients. The event was organized as a round table discussion with patients and representatives of the Ministry of Health. They also had media appearances on some national TV channels for the first time. At one of those hostings, I was invited and contributed to the discussion about the importance of treatment options for Albanian patients. Also, I shared experiences from other countries in the region.

President Mihaela Shogoric from HAE Croatia reports a very successful meeting held in Tuheljske Toplice. There were lectures from Croatian physicians and interactive discussions between patients, caregivers, and physicians. Mihaela also invited physicians and patient representatives from Serbia and Bosnia and Herzegovina to the event, and they all shared very positive impressions. Mihaela has submitted to me five new hospitals to be added where patients with HAE can ask for assistance and medication for HAE: Two in Split, one in Zagreb, one in Rijeka, and one in Osijek. All these places are known and visited by tourists, so their contact information can now be found on the HAEi website.

On 4-5 November 2022, HAE Macedonia organized a meeting for HAE patients in Skopje. The meeting held at Hotel Solun was attended by 20 patients and caregivers, around 80 percent of the HAE population in the country. Prof. Vesna Grivcheva Panovska, MD, took part in the meeting, sharing details about the ACARE

center in North Macedonia, which was certified in May 2022. She also elaborated on the future functioning of the center. Patients expressed their interest in the center, especially regarding genetic testing. HAE Macedonia President Natasha Jovanovska Popovska talked about the experience of the HAEi Global Leadership Workshop, explaining the treatment options and the new HAEi concept regarding future workshops and conferences. I shared with the patients the results from a quality-of-life survey I carried out for HAE patients in North Macedonia. This data will be instrumental in future advocating and lobbying with the Ministry of Health. The overall impression from this meeting is that patients could share their experiences in a relaxed and friendly atmosphere. They were able to learn about their rights and expectations. Some patients are too relaxed regarding their condition, while others are too terrified. I believe we were able to assure them that while they should take their situation seriously, they should also try to control it to the best of their abilities and diminish its negative effect on the quality of their lives.

Rushit Ismaili in Kosovo had a seat on the organizing committee of a conference for family doctors in Pristina and invited me to participate at the conference and present about HAE. As a mother and caregiver to an HAE patient, I told my personal story and gave facts about what HAE is, its symptoms, triggers, and medications. Also, I presented about HAEi, the support we provide to patients and caregivers, and our cooperation with physicians, researchers, and pharmaceutical companies to improve the quality of life of people with HAE. Dr. Ismaili will be in charge of the courses for the professional development of physicians in Kosovo, and he plans to have HAE as one of the topics for education. Therefore, I shared the “Understanding HAE” booklet with him so he could use it for educational purposes.



FOLLOW HAEI ON SOCIAL MEDIA

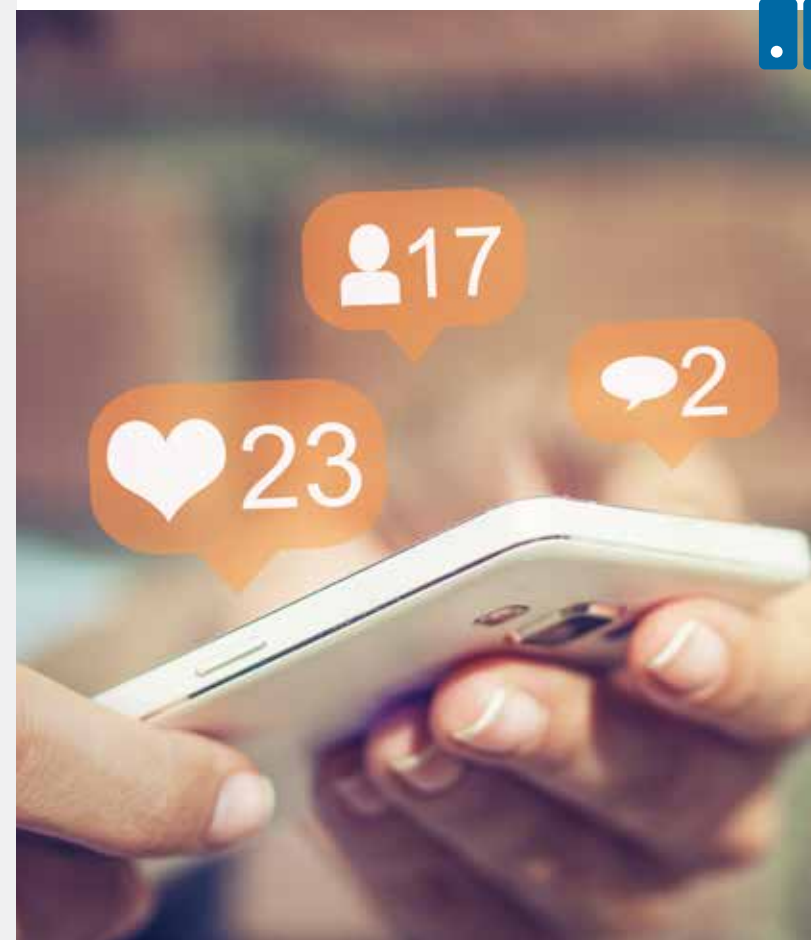
Are you aware that we post HAE-related news on our social media platforms almost daily?

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

You can find us on Facebook, Instagram, LinkedIn, and Twitter!

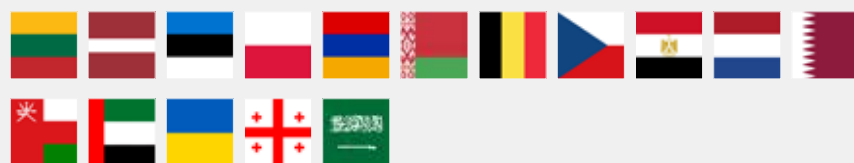
See more and find links on our website

>> [HAEI.ORG/SOME](https://www.haei.org/some)





MICHAL RUTKOWSKI
CENTRAL EASTERN EUROPE,
BENELUX AND THE MIDDLE EAST



It was a privilege to see you in person again in Frankfurt, Germany, at the 2022 HAEi Global Leadership Workshop! It's incredible how powerful and inspirational a patient gathering can be, and to see all the friends after such a long time is always additional value.

Much of my time in September was dedicated to the workshop, as many Member Organizations requested assistance regarding their attendance. Hard work pays off, and that's precisely what happened. The number of participants from the countries I am responsible for was significant.

As the global pandemic situation is better than a few months ago, I have had the opportunity to organize and participate in patient meetings on behalf of HAEi. The focus has been increasing awareness among patients and caregivers in the Baltic states. Therefore, HAEi held two educational meetings respectively in Vilnius, Lithuania, in early September and Riga, Latvia, in early November. It is important to say that both were very successful and were the first of this kind of meeting to be organized locally. I would like to express my gratitude to all the participants, including local HAE expert physicians: Prof. Laura Malinauskiene and Dr. Edita Gasusiene from Lithuania, as well as Prof. Natalja Kurjane, Dr. Signe Purina and Dr. Adine Kanepa from Latvia. The patient meeting in Tallinn, Estonia, is planned to be held in the first quarter of 2023, so stay tuned.

Also, over the recent months, I have supported HAE Poland in organizing local workshops dedicated to HAE families. In late September, a patient meeting was held in Wroclaw, and in mid-October, another meeting took place in Krakow. The total number of participants at these meetings was 62. In mid-December, one more gathering took place in Rzeszow dedicated to patients and their relatives.

As always, I have had numerous in-person and virtual meetings with HAE stakeholders:

- Member Organizations and patients from Armenia, Belarus, Belgium, the Czech Republic, Egypt, the Netherlands, Qatar, Oman, Poland, the United Arab Emirates, and Ukraine,
- HAE expert physicians from Armenia, Georgia, Latvia, Lithuania, Qatar, Oman, Poland, Saudi Arabia, and the United Arab Emirates,
- HAE pharmaceutical representatives from Astria Therapeutics, BioCryst, CSL Behring, KalVista, Pharming, Pharvaris, and Takeda.

Also, I am engaged in the HAEi LEAP project, the new educational program for youngsters, where I support and assist youngsters from Qatar, Oman, and Poland.

Furthermore, in the last months, I have been working intensively on increasing communication, especially in the Middle East. As a result, HAEi will soon hold awareness and educational meetings for patients and caregivers in collaboration with ACARE centers. We have a confirmed workshop in Oman in January and Qatar in February, and we expect more meetings: in Kuwait, Saudi Arabia, and the United Arab Emirates in the first and second quarters of 2023. I would like to express my gratitude to Dr. Iman Nasr from ACARE in Muscat, Dr. Mariam Ali Yousuf Al-Nesf and Dr. Hassan Mobayedh from ACARE in Doha, Dr. Hassan Alrayes and Dr. Rand Arnaout from ACARE in Riyadh for their support and ongoing communication.

Finally, I am pleased to introduce Mr. Mohamed Osman, the HAEi Advocacy Facilitator in the Middle East and North Africa. He will work with Maria Ferron (Regional Patient Advocate for the Mediterranean and North Africa) and myself and will use his expertise and knowledge to serve and assist Member Organizations, healthcare professionals, and the pharmaceutical industry and work with the Regional Patient Advocates to benefit the HAE patients locally.



FIONA WARDMAN
ASIA PACIFIC & SOUTH AFRICA



The 2022 HAEi Global Leadership Workshop in Frankfurt, Germany, was an excellent opportunity to bring together the Member Organization leaders. I was so pleased that many of the countries in my region had a strong presence – Australia, New Zealand, Japan, Pakistan, India, South Korea, Indonesia, and South Africa. Being face-to-face was something we have all missed on a global scale, and there is an extra buzz from everyone to keep progress going.

The leadup to the workshop was busy assisting the representatives with their visas and providing letters. Sadly, some countries and representatives were still affected by the pandemic and could not attend for various reasons. But I'm happy that off the back of the announcement of the HAEi Regional Workshop Asia Pacific to be held in Bangkok, Thailand, in March 2023, more countries like Hong Kong, Bangladesh, and Taiwan will have attendance from patients, caregivers, and doctors.

Following my visit to Singapore in July to meet with doctors, I held a meeting with patients, doctors and pharmaceutical company representatives in Singapore on 12 November 2022. We have a patient group passionate about kick-starting activities and a commitment from the Rare Disorders Society Singapore (RDSS) for local assistance. The HAE Singapore team has put together realistic goals and has highlighted the urgent needs. I will work with them to roll out their plans.

One of my roles as a Regional Patient Advocate is to ensure that pharmaceutical companies are reminded of the need for modern therapies in the various countries within the region. I am happy to report that the commitment is growing. We have already managed to bring either clinical trials or access to treatments for patients improving their quality of life immensely. In some cases, reimbursement is sadly a little way off, but the goal is in sight.

The end of 2022 is not far away now, and with the amount of achievement this year, I look forward to our progress in 2023.



**HAEi
REGIONAL
CONFERENCE
APAC
BANGKOK
17-19 MAR 2023**

TAKE ACTION

Welcome to the 2023 HAEi Regional Conference APAC

HAEi is delighted to announce our upcoming 2023 HAEi Regional Conference APAC, which will take place in Bangkok, Thailand, from 17 to 19 March 2023 and cover the Asia Pacific region.

We will welcome about 250 HAEi friends – including patients, caregivers, youngsters, HAE physicians/scientists, and pharmaceutical companies – to share experiences, meet others in the region, have fun, and learn how to improve quality of life for people with HAE.

Designed to fulfill the needs of the entire HAEi community, the conference features a Patient and Caregiver track, a Youngsters track (12-25 years old), and a Scientific track. We are finalizing the individual track programs, so please check the conference website at apac.haei.org for updates.

We look forward to welcoming you at the 2023 HAEi Regional Conference APAC in Bangkok.

Register now!

Registration for the 2023 HAEi Regional Conference APAC will be on a first come, first serve basis. We offer extremely attractive registration rates and conditions, so we advise you not to wait too long before registering.

Please register at apac.haei.org/registration



HAE Companion – Your Pocketsize Travel Partner

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

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



apple.co/33Qn4ZK



bit.ly/3osxkzm



Successful 2022 HAEi Global Leadership Workshop

The first HAEi Global Leadership Workshop held in October brought HAE Member Organization leaders, the global HAE scientific community, and the pharmaceutical industry together for the first time in over four and a half years.

“As the first event of its kind, we are very interested in our Member Organizations’ evaluation of the meeting,” says Henrik Balle Boysen, Executive Vice President and COO of HAEi. “We thank everyone who completed the survey and are happy to share highlights with HAEi friends.”

Your Feedback

87.8 % of those using the translation service Wordly said they could **understand all or the majority** of the content

98.4 % rated the workshop as either **extremely useful or useful**

97.6 % said the workshop provided them with **new information/skills** to use in the future

90.5 % said they would **do something differently** as a result of participating in the workshop

98.4 % would **attend a future** Global Leadership Workshop

A full write-up of the workshop is included in *Global Perspectives* issue 3/2022 and can be found on haei.org.



“It was an excellent workshop, very important for the physicians that treat HAE patients.”

– *HAE Scientific Track Participant*

“Very beneficial to be able to interact with the medical community and other attendees.”

– *Member Organization Leadership Program Participant*

“It was so good getting together after all this time and the difficult conditions of COVID-19; it gave me good ideas and a good boost to keep the work for our local association.”

– *Member Organization Leadership Program Participant*

“It was an inspirational event and truly impressive.”

– *Member Organization Leadership Program Participant*



The 2022 HAEi Global Leadership Workshop in numbers

- Just short of 500 participants representing +75 countries
- Almost 1,000 minutes of talks, presentations, and interactive sessions across the Member Organization Lead and Scientific Programs
- The scientific track was accredited by the European Accreditation Council for Continuing Medical Education (EACCME®) with five European CME credits (ECMEC®s)



Leaders from 75 Countries





Celebrating 2022 with High Expectations for the New Year

Hi everyone,

It's hard to believe we are already getting close to celebrating a New Year! Before we talk about our plans for 2023, however, let's reflect on the great things that happened throughout 2022.

We kicked the year off with so many goals that even the Youngsters' Community Coordinator, Nevena Tsutsumanova, thought we were a bit crazy! Yet, here we are at the end of 2022, and looking back, we (along with Nevena) are very proud of what has been accomplished.

The year started with a lot of hope and excitement as many countries began lifting COVID-19 restrictions. Everything felt a bit brighter as the world started opening and returning to at least some sense of normal.

Our 2022 began with a fantastic meeting between the HAEi Youngsters Advisory Group members and HAEi Leadership. The meeting truly revolutionized how we looked at the future of the HAEi Youngsters Community. During the meeting, we reflected on the goals, hopes, and dreams for our incredible community. Doing so led us to consider ways that we can help the Youngsters' Community grow, become more active, have more opportunities to learn from each other, and find ways to give something back to our local HAE communities.

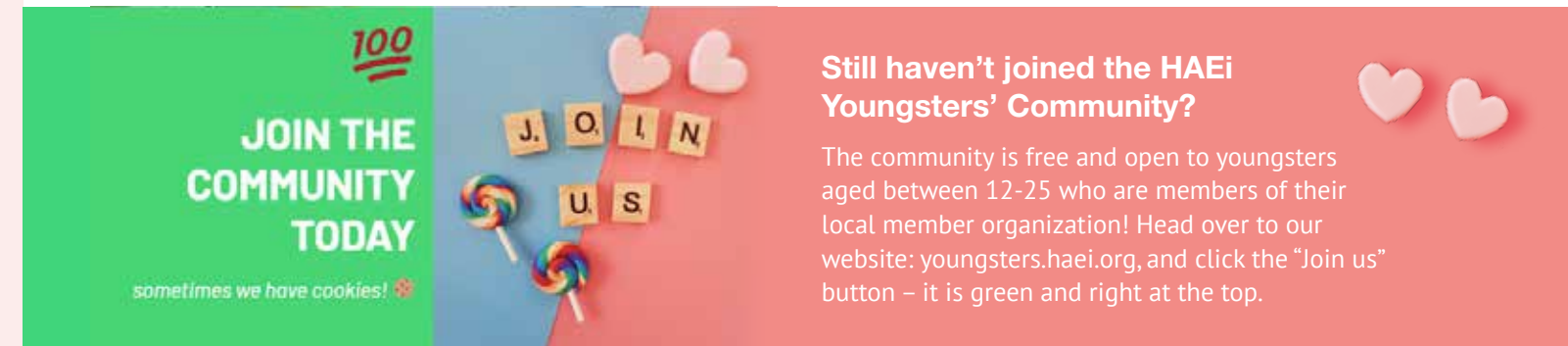
We revamped the **HAEi Youngsters Community logo** with our own unique design that reflects a theme that communicates our special message: The Youngsters Community is one family with many faces, and serves as a bridge that unites people of all ages and cultures.

The **HAE Friends Around the World** initiative began with the aim of connecting, sharing, and supporting our HAEi Youngsters. We are very happy to report that throughout the year, we managed to reconnect with many of our community members as well as welcome new ones!

We added a new feature to our website – **The Hangout**, which is a collection of short videos, blogs, and resources on HAE, motivation, and life in general. The content includes presentations from international HAE experts, stories from youngsters, caregivers, and parents, and tools developed by HAEi to help you manage HAE in your daily life. We will regularly add new content and encourage you to participate. This is your page, so please let us know if you have a story you would like to share or a question you would like answered.

The HAEi Youngsters Community was also presented at the **2022 HAEi Global Leadership Workshop** in Frankfurt, Germany. Such excitement and joy filled us when we got the chance to be there and meet with youngsters we hadn't met before but also with the friends we had missed seeing for a long time. It was really nice catching up with our HAEi Youngsters' friends, and amazing to see how we've grown up since the last time we met in person!

Last but not least is the 2022 launch of **HAEi LEAP**! This new educational program aims to train and motivate future HAEi advocacy leaders. It is designed to provide youngsters with new skills that will help them develop as individuals and HAE advocates. HAEi



One Small Step ... One Giant LEAP!

LEAP will offer participants the opportunity to gain invaluable experience and learn new skills. HAEi LEAP participants will work directly with their local Member Organizations on an advocacy project that will support the local HAE community.

Clearly, 2022 was a great year, but there are many exciting things planned for 2023!

We will kickstart the year by participating in a specially designed Youngsters track (ages 12-25) at the **2023 HAEi APAC Regional Conference** in March. That is a meeting that no HAEi Youngster in the APAC region should miss!

Just two weeks later, the **HAEi LEAP Class of 2023** will meet in Dubai to kick off the education program. And a few months after that, another regional conference for Europe, the Middle East, and Africa will be upon us with a Youngsters track of its own.

Those are just a few of the amazing things coming to you next year. So don't miss out, and stay tuned to the HAEi Youngsters website and Hangout.

Happy holidays everyone! And a Happy New Year!

Your HAEi Youngsters Advisory Group

In the most recent edition of *Global Perspectives*, we highlighted HAEi LEAP, our new educational program that helps youngsters learn new skills, develop as individuals, and work on an advocacy project that benefits themselves and their local Member Organization.

The LEAP rollout presentation at the 2022 HAEi Global Leadership Workshop in October produced such an overwhelming response from the Youngsters Community that we had to close the application process for the 2023 class. We are currently reviewing submissions and will soon get back to all who applied.

The HAEi LEAP team is busy putting the finishing touches on courses that will be added to the HAEi Advocacy Academy and planning for a Spring 2023 kick-off meeting.

A big thank you to everyone who showed interest in HAEi LEAP!

The HAEi LEAP team





Motivational Videos Now Available in the Hangout

We are very excited to announce that videos with Mark Anthony, one of Europe's leading motivational coaches, are posted on the Hangout! Mark is a highly regarded and sought speaker who has conducted well over 7,500 coaching and motivational sessions for 200,000 people. HAEi friends may recall that Mark was an invited speaker at the **2018 HAE Global Conference** in Vienna, Austria.

Find the videos on youngsters.haei.org/hangout/#health



Have a great day!

In this video, Motivator Mark Anthony gives a motivational talk about having a great day (4:48 min)



I am possible

'Impossible – how to make it possible!' is the title of this motivational talk from Motivator Mark Anthony for you (5:46 min)

Exciting
new Hangout
with lots of
resources

Let's Hang Out at the HAEi Youngsters' Hangout!

We are happy to introduce you to the HAEi Youngsters' Hangout – a collection of short videos and blogs featuring interesting and valuable content for all of our HAEi Youngsters, no matter where you are in your HAE journey.

The Hangout offers stories from youngsters, caregivers, and parents, presentations by HAE physician experts, and tools that HAEi designed to help you manage your daily life.

You can help expand the Hangout with fresh material by sharing your story or asking a question.

We invite you to the Hangout at youngsters.haei.org/hangout.

Your HAEi Youngsters Advisory Group

Mark graciously sat for an interview with the Hangout:

Where are you from?

Denmark. My father was from Cyprus, my mother is Danish (one-quarter Swedish ☺).

What is something interesting about you that will surprise us?

I am a former Champion in Latin American dancing.

What does living life to the fullest mean to you?

Being happy, free, and waking up with a smile on my face.

What would it be if you could eat only one thing for the rest of your life?

Good food ☺ Okay, Halloumi and watermelon (as I mentioned, my father is from Cyprus, so this is hereditary).

What is one thing that always brings a smile to your face?

Happy and playful people will always bring a smile to my face!

If you could have one superpower, what would that be and why?

Making people happy, that is why I chose to work with motivation and motivational coaching. I guess I do have a bit of a superpower.



HAEI GOES REGIONAL

In-person gatherings offer the HAEi Community inspiring and motivating opportunities to learn from fellow patients and caregivers as well as expert physician researchers. Following the highly successful 2022 HAEi Global Leadership Workshop in October, HAEi will conduct a series of Regional Conferences that focus on the unique needs of people with HAE and their caregivers in various regions throughout the world.

“Although the perspective of HAEi is global, we recognize and celebrate the diversity among the regions we serve. That is why we are organizing three HAEi Regional Conferences over the next two years.

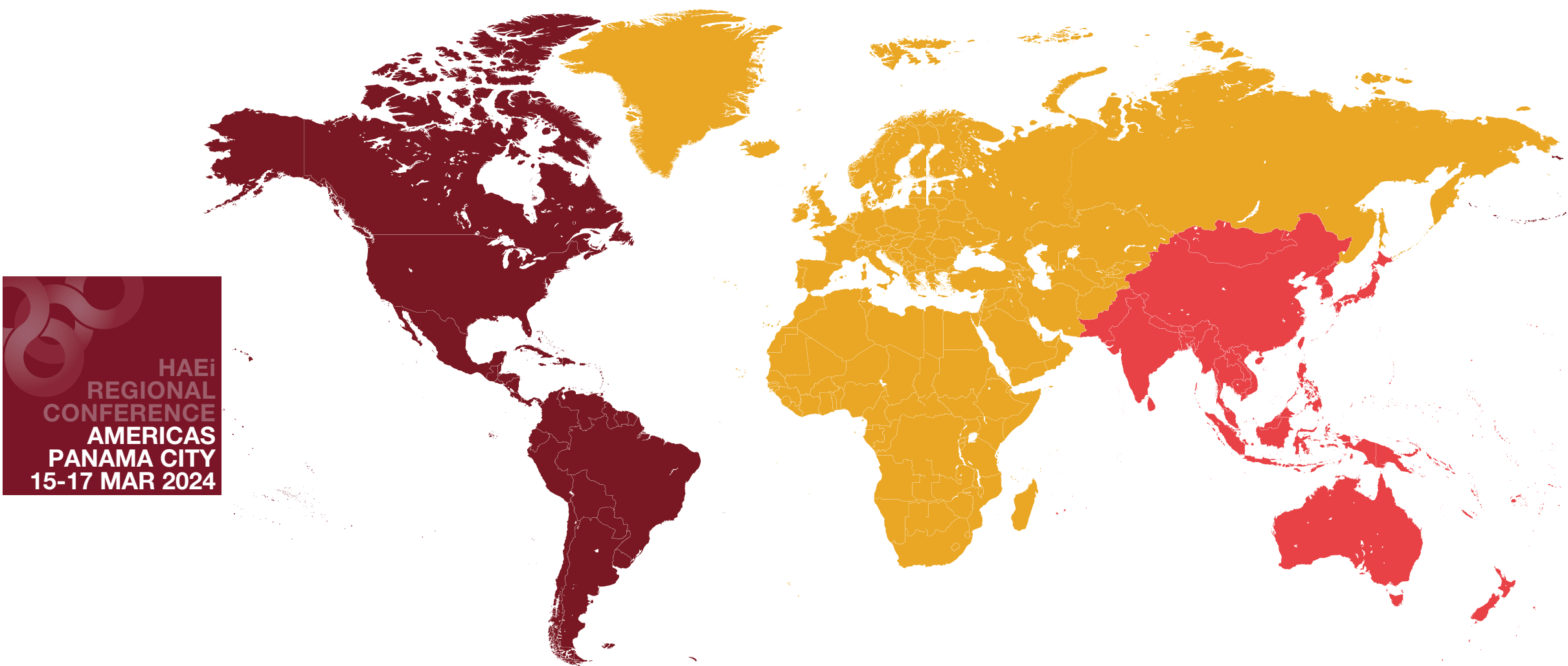
We look forward to welcoming representatives of our Member Organizations. And as always, we will do our very best to offer attendance at a very low price and will also work hard to secure travel grants for as many patients and caregivers as possible”, says Executive Vice President and COO, Henrik Balle Boysen.

We cordially invite you to mark your calendars and plan to attend the exciting HAEi Regional Conference that will be held in your part of the world!

2023 HAEi Regional Conference APAC
(covering Asia Pacific)
17 – 19 March 2023, Bangkok, Thailand
(Registration is open)

2023 HAEi Regional Conference EMEA
(covering Europe, Middle East, and Africa)
1 – 3 September 2023, Munich, Germany
(Registration will open in April 2023)

2024 HAEi Regional Conference Americas
(covering North, Central, and South America)
15 – 17 March 2024, Panama City, Panama
(Registration will open in October 2023)



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

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

**HAEi
REGIONAL
CONFERENCE
APAC
BANGKOK
17-19 MAR 2023**

ACARE NETWORK GROWS TO 75 CENTERS

The global network of Angioedema Centers of Reference and Excellence (ACARE) – a joint venture between the Global Allergy and Asthma European Network GA2LEN and HAEi – continues to grow!

“We now have 75 certified ACARE centers – and 18 applicants – as we proudly welcome Queen Mary Hospital/Grantham Hospital in Hong Kong, headed by Dr. Philip Li, to the ACARE network. In 2022, we have thus gained six new ACARE centers”, says Anthony J. Castaldo, HAEi’s President and CEO.

The ACARE leadership team attended the 2022 HAEi Global Leadership Workshop in Frankfurt, Germany, and took the opportunity to interact with Member Organizations as well as physicians/scientists. Rebecca Locke, ACARE Program Manager, comments: “We loved talking to everyone, hearing new perspectives from Member Organizations, and discussing what ACARE is all about. We were all impressed and inspired by the energy and commitment present at the workshop and are happy to be a part of the drive to offer the best possible care to patients with all forms of angioedema”.

In 2023 ACARE will launch an educational program called ACARE LevelUp that is designed to equip physicians with the knowledge and tools to provide top-notch care to people with angioedema. The ACARE LevelUp content will be delivered through webinars, podcasts, and preceptorships.

Physicians interested in becoming part of the ACARE network can get more information at acare-network.com/become-an-acare.

**We provide
excellence in
angioedema
management**



75 ACARE CENTERS:

By December 2022, the ACARE network consists of 75 accredited centers in Argentina (4), Australia, Austria (2), Brazil (10), Bulgaria (2), China (2), Denmark, France (3), Georgia, Germany (8), Greece, Hong Kong, Hungary, India (3), Italy, Japan (2), Kuwait, the Netherlands, North Macedonia, Oman, Peru (2), Poland (5), Portugal (2), Qatar, Russia (2), Saudi Arabia, Slovenia, South Africa, Spain (2), Thailand, Turkey (4), the United Arab Emirates, the United Kingdom (2), and the United States (2).

hae day :-)

MANY FACES
ONE FAMILY

hae day :-) 2023 is around the corner

If it isn't in your diary already, mark 16 May 2023 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 2023.

In the meantime, our **hae day :-)** website has lots of information and inspiration as you start to plan your hae day :-) activities.

Check out haeday.org to find

- Activity inspiration
- A toolbox to help plan media, community, or social media activities



New version 2!



Have you wondered how to get your HAE under control? HAE TrackR can help you!

Developed by fellow HAE patients at HAEi, HAE TrackR is an easy-to-use electronic diary designed to record your HAE treatments (preventative and on-demand), HAE attacks, and the impact HAE has on your life and the life of your loved ones.

HAE TrackR allows you to download a comprehensive report of your treatments and attacks which can be used as a tool for you and your physician to manage your HAE.

The app strictly protects your data and privacy and is fully EU-GDPR compliant – all data gathered is the sole property of the user.

Features of the app:

- ✓ Easy -to-use electronic diary
- ✓ Safe and secure – all data is the sole property of the user
- ✓ Store and share data about your HAE with your physician
- ✓ Product and company neutral with no commercial interests
- ✓ Endorsed by the ACARE network
- ✓ Accessible from anywhere at any time and in many languages

New features added in version 2:

- ✓ Smart reminder functionality for prophylactic treatments
- ✓ Improved reporting for easy sharing of data with physician
- ✓ Option to only list preferred/used treatment(s)
- ✓ Option to add clinical trial medication if needed
- ✓ Upload function for photo(s) of batch/LOT number for documentation

SCAN TO
START USING
HAE TRACKR



Start using HAE TrackR today
app.haetrackr.org

Get a full overview of your HAE.

NEWS FROM MEMBER ORGANIZATIONS AROUND THE GLOBE



UKRAINE

HAEi recognizes the horrific situation in Ukraine. HAEi members stand in support and solidarity with our fellow HAE brothers and sisters in Ukraine. HAEi understands that some Ukrainians are fleeing to neighboring countries for safety. HAEi advises those arriving in a country to reach out to the HAE Member Organization for advice on doctors and treatment centers for HAE assistance – please see <https://haei.org/about-haei/globally>.

People with HAE in Ukraine can also reach out to an HAEi Regional Patient Advocate for further assistance – you can find contact information on our website at <https://haei.org/about-haei/meet-the-rpas>.



JAPAN

*From President Beverley Yamamoto,
HAE Japan*

We have seen great improvement in the treatment environment in Japan over the last two years, and in September the third long term prophylactic treatment was approved by the PMDA. Since last reporting to *Global Perspectives*, we have also seen the indications for use of Icatibant expanded to include pediatric use.

On 3 September 2022 we held our first face-to-face patient meeting since COVID-19 changed all our lives. The meeting was held in Tokyo at the Juntendo University Affiliated Hospital. We had 29 participants, including 20 patients and caregivers, two physicians and seven industry partners. Dr. Inage, a pediatrician at Juntendo Hospital, spoke about his experiences with pediatric patients, focusing particularly on unmet need, the need to go beyond his medical training to think about the social aspects of HAE for children, and the difficulties with treating a new category of “suspected HAE with normal C1” that has emerged in Japan. Following on from Dr. Inage, I spoke about the long-term prophylactic treatments now available or shortly to be made available in Japan.

Over a year ago, key industry stakeholders in Japan

in consultation with patient leaders and physicians came together to create the Discovery Consortium, a charity to identify undiagnosed patients. HAE Japan has been working closely with the Consortium with representation on the Board of Directors and in one of the working groups, WG3. Although in Japanese, WG3 has created an up-to-date list of hospitals that can diagnose and treat HAE. This list is available at <https://discovery0208.or.jp/hae-info/search>.

Finally, a collaborative project between the Japanese Ministry of Health, Labour and Welfare and Takeda Pharmaceuticals has provided funding for building capacity around HAE physicians in Vietnam. Over the summer one of our medical advisory team, Dr. Honda, went out to Vietnam to talk with the doctors. In October the doctors came to Japan. On 29 October, I was invited as President of HAE Japan to give a talk to the doctors about the lived patient experience as well as patient reported outcome research that I have been involved in with some of the leading HAE physicians. Six doctors attended from Vietnam, and I was delighted at the wide range of questions that were asked.



SWITZERLAND

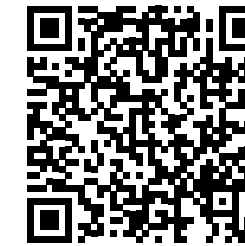
*From Vice President Ernst Greber,
HAE Switzerland*

Impressions from the 2022 HAEi Global Leadership Workshop in Frankfurt, Germany:

- We were represented by three board members
- The event was excellently organized
- The lectures were very interesting and gave us new insights
- The exchange with other organizations was also very interesting

11 November 2022 we held our 21st HAE Switzerland General Meeting in Olten. Twenty-two members took part, and the agenda items were carried out quickly. Afterwards HAE Switzerland offered dinner with drinks. This year, we have also published the 6th edition of our newsletter and we discussed interesting topics such as new drugs and where our HAE centers can be found and who the contact persons are. Also, the HAE Center in Basel introduced itself.

This year our President Helene Saam and our doctor Prof. Dr. med., Dr. phil. Walter A. Wullemmin have created various podcast films that are intended to show our life with HAE in small sequences. These films can be viewed at bit.ly/hae_switzerland_youtube or by using this QR code:



RUSSIA

*From President Elena Bezbozhnaya, HAE
Russia*

Over the past six months, HAE Russia has hosted various highlights, face-to-face meetings, and online webinars, with adults and children involved from all over the country.

Over the summer, we invited members living in the Moscow region to a webinar on “Long-Term Prevention and Screening of HAE Patients in Moscow”. The webinar was interactive, enabling HAE patients to discuss their concerns with the speakers, focusing on short-term and long-term prevention issues. Another webinar addressed the effectiveness of HAE preventive and therapeutic treatments prescribed to patients.

Since 2021, many Russian patients have started to receive biosimilar pathogenic drugs. Unfortunately, the experience revealed that analogue drugs do not always have the same effect as the original drugs. Therefore, at the end of the summer, we hosted the webinar “Drugs: Myths and Truth.” Doctors and lawyers presented participants with procedures for dealing with cases where the prescribed medications prove to be ineffective or cause negative adverse reactions.

Furthermore, in August, we organized a webinar titled “Litigation. What You Need to Know”, aimed at patients who need to pursue court-ordered medications.

In mid-September, representatives of HAE Russia had

a consultative meeting with the leadership of the federal “Circle of Kindness” Foundation and signed an Agreement on Cooperation. Since the beginning of 2021 “Circle of Kindness” has been engaged in supporting children with severe life-threatening and orphan diseases. Last October the Expert Council of the Foundation added HAE to the list of diseases curated by the Foundation, taking responsibility for providing juvenile patients with HAE with modern pathogenetic medicines. Now more than 20 juvenile patients are under the care of the Foundation.

On 24 September 2022 in Moscow we conducted the All-Russian School of HAE patients, the largest annual event, to discuss the most urgent and relevant issues. The participants traditionally exchange the latest information, news in the medical and legal fields, talk about their concerns and share their personal experiences. This time the conference was held under the motto “Life without fear”. HAE patients from all over the country – from Moscow and St. Petersburg, the Far East, Kamchatka Krai and other regions of Russia – participated in the school’s activities. Many of them came for the first time as families, with children, who have also been diagnosed with HAE. The speakers at the event represented the best experts and specialists of the largest medical centers, psychologists, lawyers, and members of public organizations. Opening the School, I presented the projects implemented by HAE Russia and noted that now our organization has about 300 members. Today, the Russian Federal Register contains data on 622 patients with HAE (including 93 children).

Within the conference, the experts discussed the practice of Russian patients’ use of Lanadelumab, a new long-term preventive agent registered in Russia in 2021. Also, they shared their experience regarding modern methods of detection and diagnosis, and treatment guidelines for the disease. The conference resulted in the definition of future objectives for HAE Russia, namely: improving the level of detection and diagnosis of HAE, implementing screening projects; introduction of educational programs to inform the medical community about the existence of HAE and its symptoms; promotion of the multidisciplinary approach to the treatment and medical support of patients with HAE.

Given that more than 20 children and adolescents (diagnosed with HAE) from 4 to 17 years attended the School for the first time, the organizers developed a special program for them, which included psychological games, film training, discussions, creative master classes, and even a legal interactive. Moreover, during the conference the young participants decided to form the HAE Russian Youth Community.

Throughout 2022, HAE Russia continued the “Call a Friend” project for children and adolescents diagnosed with HAE. At the online meetings, facilitators introduce underage patients to relaxation and stress reduction techniques, teach them how to deal with their own feelings, hold creative master classes, psychological games, and help teenagers decide on their future profession.

In October and November, we held several traveling HAE Patient Schools for residents of the Moscow region, St. Petersburg, and Nizhny Novgorod. In addition, patients from different federal districts of Russia were offered a series of online webinars focused on the creation and development of a multidisciplinary approach in the country's regions. Regional medical experts who specialize in treating comorbidities in patients with orphan diseases were invited to contribute to the discussion. This issue appears to be one of the most pressing, since not all medical institutions understand the specifics of treatment, premedication and preparation for all kinds of medical manipulations that people diagnosed with HAE need (including dental, surgical procedures, pregnancy support and safe delivery for patients with HAE). We aim at developing a multidisciplinary approach to treating patients with HAE at the regional medical centers.



HUNGARY

The dates of the 13th C1-inhibitor Deficiency & Angioedema Workshop in Budapest, Hungary, have been moved. It will now be held on 4-7 May 2023.



SINGAPORE

The first patient meeting has taken place in Singapore. Patients have connected and there are plans in place to advocate as a group and get things moving here.



COLOMBIA

“Mi vida con AEH” (“My life with HAE”) is a new podcast from HAE Colombia which seeks to create awareness through the stories and experiences of the members. The aim is to motivate, help and support others through their first-hand experiences.



AUSTRALIA & NEW ZEALAND

From CEO Fiona Wardman, HAE Australasia

HAE Australasia received ethics approval to run the HAE pharmaco and socioeconomic/quality of life survey. This critical survey is for patients and their family members in Australia and New Zealand. We encourage all our members to take the time to answer the questions to ensure their journey with HAE is included in the outcomes. The outcomes of this research-based survey will have significant gains in terms of unique insights into the lives of HAE patients that can be communicated to different stakeholders.

The 2022 HAEi Global Leadership Workshop held in Frankfurt, Germany, in October was an excellent opportunity to bring HAE Australasia's leaders to meet with other global leaders. Connections and friendships were made, and we learned more about HAE and HAEi tools and resources to assist our path forward with HAE Australasia. It was also the first opportunity for the Board Members across our two countries to meet face-to-face since before the pandemic.

Our HAE Australasia Youngster Jess Bogoyevitch has submitted a project to the HAEi LEAP program. We have our fingers crossed that her submission is successful so we can connect our youth in Australia and New Zealand.



NORTH MACEDONIA

On 4 November 2022, HAE Macedonia organized a meeting for North Macedonian patients in Skopje. The meeting was attended by 18 patients and caregivers and allowed patients to see each other for the first time after the pandemic and exchange their experiences.

HAE Macedonia's Vice President, Verche Jovanovska Jankovska, addressed the patients reflecting on the past and highlighting the patient association's accomplishments so far. Also, she expressed hope that the future will bring even more active and harmonious cooperation between the members. Natasha Angjeleska, the HAEi Regional Patient Advocate for South Eastern Europe, presented the various tools and applications HAEi has created and which patients in North Macedonia found useful. At the same time, Natasha Jovanovska Popovska, the President of HAE Macedonia, talked about the recent HAEi Global Leadership Workshop in Frankfurt, Germany. She also spoke of the experiences and novel treatments that were discussed there. Prof. Vesna Grivcheva Panovska also addressed the patients, explaining to them the functioning of the ACARE center in Skopje.

The patients were really inspired by the event, as they found it truly useful and got encouraged to be more active in HAE-related matters. They started by actively using the HAEi apps, and three youngsters took an interest in HAEi LEAP Academy.



UNITED STATES

From US HAEA

2022 HAEA Year in Review: Throughout 2022, the HAEA has continued to provide our 8,000 members with programs, services, and activities that engage and unite our community, and help people maintain the hard-fought access to and reimbursement for modern HAE medicines.



During the year, we developed and implemented a wide variety of new and exciting programs and resources and highlight a few below:

- The HAEA Round Table webinars and HAEA Community Blog facilitate communication among members so they can share challenges, successes, and lessons learned about HAE,
- The HAEA collaborated with HAE expert physicians to develop the Guide for Women with Hereditary Angioedema (HAE), an authoritative resource that

includes frequently asked questions, stories from patients, and relevant source material to help women with HAE navigate every phase of their life.

- We introduced the new information-sharing and communications platform Voter Voice to enhance the capabilities and reach of our grassroots network. Voter Voice dramatically improves the network's ability to engage with elected officials on public policy issues relevant to people with HAE.
- We continued to cultivate other valuable and effective services such as Meet and Greet events, Clinical Trial outreach, Advocacy and Awareness events, educational webinars, the HAE Speaks and #BeyondHAE podcasts, and the Pam King HAEA Scholarship Program.

As we look towards the new year, the US HAEA remains steadfast in creating innovative solutions that fulfill our ongoing quest to help everyone with HAE lead a life that is unhindered by the condition.



Original US HAEA Initiated Research Accepted for Poster Presentations at the 2023 Annual Meeting of the American Academy of Asthma, Allergy, and Immunology (AAAAI):

The results of three HAEA-initiated research projects have been accepted for presentation at the February 2023 AAAAI meeting.

1. Consequences of insurance coverage delays and denials for patients with HAE

Modern therapies have dramatically increased quality of life for people suffering from HAE. Nevertheless, insurance changes, delays, and denials are becoming more common. Twenty HAE patients (type 1 and

2) who recently experienced insurance delays or denials completed an online survey, and nineteen participated in a follow-up focus group. The survey and focus group addressed the impact of insurance challenges on the use of healthcare services, work/school attendance, and anxiety. Insurance delays and denials have significant impacts on individuals with HAE, including 1) increased urgent care and ER visits, 2) missed work/school days, 3) higher levels of anxiety, and 4) a negative impact on family life.

2. Comparison of Selected Health and Well Being Characteristics in Seniors with HAE and a Non-HAE Patient Matched Cohort

HAE requires lifelong treatment, disease course, management, and outcomes are rarely reported in older populations. This study compared US adult HAE patients ≥60 years to a non-HAE patient-matched cohort. Prior research revealed significant QoL and mental health differences between adults with HAE and the general population. With the exception of activity impairment, these data show relatively minor and statistically insignificant differences among the two groups of seniors. These results are likely associated with the significant advances in HAE therapeutic options resulting in improved outcomes.

3. Critical Analysis Highlights Methodological Flaws in a Study Using Insurance Claims Data to Assess Cost Effectiveness of Prophylaxis Therapies for the Rare Disease HAE

In a 2021 pilot study updating an earlier analysis of HAE prophylaxis medicines, the Institute for Clinical and Economic Research (ICER) concluded that these therapies are far less cost-effective than previously estimated. We evaluated ICER's methodology, including using and interpreting insurance claims data and the sources for utility values associated with Quality Adjusted Life Years (QALY) calculations.

Fundamental flaws in the base-level attack rate and utility used to calculate QALYs raise serious questions regarding the validity of ICER's methodology.

2023 US HAEA National Summit: We are pleased to announce the 2023 US HAEA National Summit that will take place in Orlando, Florida, from 20 to 23 July 2023.



The Summit theme is “Living Beyond Boundaries” and will bring the HAEA community together in person for the first time in three years. Our goal is to unite patients and caregivers, healthcare professionals, and industry partners once again for a transformative experience of learning and sharing.

The Summit will feature a highly relevant and engaging program that includes:

- a Professional-Scientific Track and poster viewing session,
- a Summer Youth Health Management and Advocacy Program,
- Brady Club Programs for the very young members of our community,
- General Sessions covering:
 - the latest information on HAE therapies,
 - treatments on the horizon,
 - results of HAEA advocacy research regarding insurance and other challenges,
 - grassroots legislative initiatives.

Registration will be open in January 2023 for residents of the United States, Puerto Rico, and the US Virgin Islands, so stay tuned for more details and make sure to Save the Date!



CROATIA

Five HAE-knowledgeable hospitals – KBC Osijek, KBC Rijeka, KBC Split, OHBP KBC Zagreb, and ORL Split – have been added to the HAEi world map. Please have a look at <https://haei.org/hae-member-countries/croatia> for contact information.



SPAIN

From HAE Spain (AEDAF)

HAE Spain (AEDAF) Camino Walk 2023: AEDAF is pleased to announce that it will be organizing another **hae day :-)** event on the Camino de Santiago: the HAE Spain (AEDAF) Camino Walk 2023, which will take place from 13 to 17 May 2023.

The aim of **hae day :-)** on 16 May every year is to raise awareness of HAE among the general public and medical community in order to create an environment in which there is better care, earlier and more accurate diagnosis, and recognition that people with HAE can lead a healthy life.

This time we will be walking on the Portuguese route of the Camino de Santiago, an extensive network of pilgrimage routes leading to Santiago de Compostela in Galicia, in the northwestern part of Spain. We will be walking an average of 15 km per day for three days and have a short walk on the fourth day to the shrine of the Apostle Santiago (St. James) in the Cathedral of Santiago de Compostela.

One of the main reasons for us walking the Camino is the publicity we can generate as an **hae day :-)** event. The Camino Walk will serve as a statement that people with HAE, many of them frequently incapacitated by their attacks, are overcoming the obstacles to leading normal and fulfilling lives and can now feel free to undertake a journey of this nature.

The Camino Walk will be open for registration in January 2023 on a first come, first served basis. If you would like to register, we encourage you not to wait too long after registration opens, as we have limited seats available on the bus. The price will be 400 EUR per person (staying in a shared double or triple room) and 500 EUR per person in a single room.

AEDAF is looking forward to welcoming you to the Camino Walk 2023 from 13 to 17 May 2023.

2022 HAEi Global Leadership Workshop: Along with Sarah Smith (President of AEDAF and HAEi Board Member) and Maria Ferron (Secretary of AEDAF and HAEi Regional Patient Advocate for the Mediterranean,

North Africa, and the British Isles), Spain was represented at the 2022 HAEi Global Leadership Workshop by eight members of AEDAF. Unfortunately, another two members who registered had to cancel at the last minute because of a severe health crisis in the family. Dr. Teresa Caballero of La Paz Hospital in Madrid was a member of the Scientific Program Committee. She gave an oral presentation in the scientific session on “Surveys on Access to Modern Medicines for Hereditary Angioedema (HAE)”, which summarized the results of the survey conducted by AEDAF in Spain, with a CSL Behring EU LEAD Grant (2021), on the availability of and access to modern HAE treatments in Spain based on place of residence.

We were excited to be “Together Again”, and we all found the workshop useful, motivating, and fun! Our congratulations to HAEi and all the fantastic people involved in making this a tremendous success!

AEDAF 25th General Assembly & Annual Meeting:

We are also pleased to announce that AEDAF will be celebrating its 25th anniversary with its 25th General Assembly and Annual Meeting, which will take place on 22 April 2023, in the venue where we have met every other year: La Paz Hospital in Madrid. To celebrate such a significant milestone, we hope to offer a very interesting program with national and international news about activities, innovations, and new initiatives for HAE patients.



IRELAND

Any people with HAE in Ireland who are interested in participating in a clinical trial or receiving news on new treatments, please get in touch with HAEi Regional Patient Advocate Maria Ferron at m.ferron@haei.org.



As the season ends, we must start with the most impactful event from fall 2022: the 2022 HAEi Global Leadership Workshop in Frankfurt, Germany. We'd like to deeply thank the HAEi team for giving HAE Canada's leadership the opportunity to participate in such a worthwhile experience. HAEi did a phenomenal job of bringing organizations from across the globe to meet, learn, reflect, and share. Thanks to the engaging and meaningful presentations, we are excited to put the tools we learned into practice.



Not only did we learn, but we proudly presented our poster “HAE with normal C1 esterase inhibitor (HAEi): Diagnosis, treatment and attack frequency from the 2020 Canadian patient survey”. We returned to Canada energized, motivated and grateful for having such an engaged and well-informed international umbrella organization.

Soon after returning from Germany, HAE Canada hosted a Patient Information Update in Hamilton, Ontario. We hoped to welcome our members in person, however, rising COVID-19 numbers forced us to hold the update virtually once again. After COO Daphne Dumbrille, highlighted key initiatives and items from HAE Canada, the Ontario Regional Director, Michelle Cooper,



presented about the Global Leadership Workshop. Michelle was followed by patients Jordyn and Danielle, who generously shared the journey they have taken. We all appreciated hearing what they have experienced as patients with a rare disease, and how they have become whom they are today after receiving their diagnosis and treatment. Dr. Paul Keith, an allergist and immunologist from Hamilton, shared a very informative presentation on the current HAE treatments in Canada while also explaining the importance of patients participating in clinical trials in Canada. Lastly, Dr. Keith was joined (virtually) by Dr. Sylvain Grenier from Canadian Blood Services, and they each answered pre-submitted and live questions from attendees. Thank you to our members who attended the update, and a special thanks to our presenters and speakers; their time and expertise are always gratefully appreciated.

Directly after the Patient Update, we hosted our 2022 Annual General Meeting. Thank you to Heather Dow for chairing this important event. Thanks to our engaged

membership, we held a successful meeting that voted in our 2022-2023 Board of Directors, who then elected the executive positions as follows:

- Jacquie Badiou: Director at Large / Past President
- Michelle Cooper: Ontario Regional Director; President
- Carmen Craciun: Director at Large / Treasurer and Secretary
- Kristen Lane: Pacific Regional Director / Vice President
- Martine Paquette: Quebec Regional Director
- Dwayne Semple: Atlantic Regional Director
- Kim Speiss: Central Regional Director

We are thrilled to announce that Michelle Cooper has generously agreed to take on the President position. This position is a natural next step for Michelle as she has been part of the HAEC Board since 2020 as the Ontario Regional Director, in 2021, she became Vice President and has remained on the Advocacy Committee throughout. After 12 years as President, Jacquie Badiou decided to step down to become our Past President. To say we are grateful for her guidance and leadership is an understatement. She helped HAE Canada become the well-organized and respected patient advocacy group it is today. Thankfully, Jacquie will remain a very active member of our Board of Directors, providing mentorship to Michelle and increasing her advocacy work internationally. On behalf of the entire HAE Canada membership and Board of Directors, we want to profusely thank Jacquie for her unwavering commitment to helping HAE patients and their caregivers in Canada.

Tina McGrath, who sat on the Board since 2018, decided not to renew her term as a Board member. We are grateful for her dedicated years as the Ontario Regional Director and as a member of the Governance Committee, and that she has agreed to remain as one of our Regional Director Assistants for Ontario. Thank you to our returning Board members, who are not only a joy to work with, but also bring their own perspectives and knowledge to the table.

Michelle and Daphne attended the Canadian Organization for Rare Disorders' (CORD) Fall conference in Toronto titled “Planning for Proof-of-Concept Programs to Transform Vision to Reality”. Canada is committed to setting up a federal Rare Disease Drug Strategy to provide Canadian patients access to treatments. The conference discussed the key elements of what makes up an effective and economic Rare Disease Drug Program. The two days covered a vast number of topics, including genome screening and sequencing, what role patients can play in establishing

the Drug Strategy and why it is important to invest in Canadian research. The days were packed with useful and interesting information, and it was great connecting with others in the rare disease community.



The CORD conference discussed the importance of establishing Centers of Excellence in Canada for rare diseases, which brings us to our next topic. We are proud to say that Yang Medicine, headed by Dr. William Yang in Ottawa, was designated as the first Angioedema Center of Reference and Excellence (ACARE) center in Canada. For many years, Dr. Yang has been a tremendous support to HAE Canada, while providing wonderful care to his HAE patients. He is dedicated and passionate about his work, making this designation from HAEi and GA2LEN very well deserved. We are very proud of Dr. Yang and hope his clinic will be the first of many ACARE centers in Canada.

ORLADEYO® (berotralstat) has been approved by Health Canada, and we have been working with the manufacturer, BioCryst, to help support a positive recommendation from the Canadian Agency for Drugs and Technologies in Health (CADTH). This is an

important step in Canada because for provinces and territories to fund treatment, the treatment first needs a positive CADTH recommendation. Thank you to HAEi President and CEO Anthony J. Castaldo and Executive Vice President and COO Henrik Balle Boysen for providing input to the patient feedback to CADTH; their contribution certainly made an impact. We hope CADTH will announce their decision early in the new year.

HAE Canada wants to help not-yet-diagnosed and diagnosed HAE Canadian patients who present at the Emergency Department during an attack. We are reaching out to stakeholders to accomplish key strategies, such as having HAE added to the Advanced Cardiac Life Support (ACLS) supportive text and adding HAE to first aid training materials. Fortunately, we have assistance from Canadian physicians, Drs. Susan Wasserman, Paul Keith, Allan DeCaen and David Barnes, American physician Dr. Marc Riedl, HAEi's Anthony J. Castaldo, HAE Canada Board members Jacquie Badiou and Dwayne Semple, and Kim Speiss contributed in Dwayne's absence. With their help, we are confident HAE will become better recognized by emergency health providers.

We will end with the great news that our recent abstract titled "Emergency room visits by patients with HAE based on data from the Canadian 2020 national survey" has been accepted to the American Academy of Allergy, Asthma & Immunology (AAAAI)'s 2023 Annual Meeting. Thank you to Dr. Suzanne Kelly from Red Maple Trials, and to the contributing authors, for developing this abstract. We are excited to share our data at this prominent event.



TURKEY

*From President Gökben Yurdakul,
HAE Turkey*

In response to the challenges posed by the COVID-19 pandemic, HAE Turkey took action to provide support for patients and their families. We held information meetings with doctors in a region where adult and pediatric patients were the focus, sharing their struggles and working together to develop solutions. They also organized an online meeting with patients to provide psychological support for those affected by HAE.

In addition to providing patient support, HAE Turkey participated in the 1st HAE Physician Symposium in

Turkey. We explained the organization's purpose and activities to the doctors and expressed our desire to work in partnership with them to support patient orientation. This collaboration allowed us to expand our reach and work with more doctors in different regions.

On 16 May, a significant day for HAE, we visited the Ministry of Health, the Social Insurance Institution, and the Grand National Assembly of Turkey. We met with officials face-to-face to share our demands and opinions, and as a result, some of the problems we were experiencing in medicine and treatment have begun to be resolved. This has encouraged us to continue our efforts and work even harder to address the challenges facing HAE patients in Turkey.

HAE Turkey also attended the 2022 HAE Global Leadership Workshop in Frankfurt, Germany, and shared the good news we heard and saw there with our members. Additionally, we have participated in a congress focusing on internal medicine. In Turkey, most patients first visit internal medicine doctors, so we believe that reminding these doctors about HAE can help improve diagnosis and treatment.

HAE Turkey is committed to solving all the problems experienced by our patients. We hope to continue our efforts to provide support and improve outcomes for those affected by HAE.

We wish all the HAE family a Merry Christmas and an amazing New Year!



ISRAEL

Israeli HAE patients have gained access to Orladeyo, so with Ruconest, Berinert, and Firazyr already on the market, there are now four modern HAE medications in the country.



UNITED KINGDOM

From HAE UK

Well, what a busy few months it has been for HAE UK! As CEO, Angela Metcalfe was delighted to attend the 2022 HAEi Global Leadership Workshop in Frankfurt, Germany, at the beginning of October, and very pleased to be joined by three other patients and Trustees of our charity, as well as Rachel Annals, our Executive Officer, 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.

We were planning on holding our annual patient day in November this year. Sadly, many of our members are still nervous about traveling and meeting in large groups, so we decided to postpone the event until spring 2023. We look forward to sharing information about this event with everyone soon.

We have had a number of patient-centered initiatives running in the UK recently, with several surveys and reference groups sharing their stories and HAE journeys. This kind of information gathering and research is vitally important for healthcare professionals, pharmaceuticals, and patients to understand current and new treatments and medications. By sharing the living experience of HAE, we hope that it supports, steers, and guides others in being able to live with this condition rather than just existing. Keep a lookout for some of these surveys being published in the new year, which we will share with you.

It just remains for us to wish everyone a very Merry Christmas, and we hope that you can spend it with loved ones for the first time in a couple of years for many of us. And looking forward, we wish you all a very Happy New Year and a healthy 2023.

MEDICAL PAPERS

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

Abdominal and pelvic imaging in the diagnosis of acute abdominal attacks in patients with HAE due to C1-inhibitor deficiency – by Piotr Obtułowicz, University Hospital Krakow, Poland, et al.:

Ultrasound or CT imaging facilitates the diagnosis of the patient suspected of having an abdominal attack due to C1INH-HAE. They allow to identify transitional presence of an abundant fluid in the free abdominal cavity and intestinal swelling which spontaneously disappear with a symptoms attack.

(Postepy Dermatol Alergol, August 2022)

A catalog of the genetic causes of HAE in the Canary Islands (Spain) – by Alejandro Mendoza-Alvarez, Hospital Universitario Nuestra Señora de Candelaria, Spain, et al.:

We have characterized for the first time the genetic causes of HAE in affected families from the Canary Islands (Spain). Whole-exome sequencing data was obtained from 41 affected patients and unaffected relatives from 29 unrelated families. We detected a total of eight different variants causing HAE in this patient series, affecting essentially SERPING1 and F12 genes. Despite the genetic causes of many patients remain to be identified, our results reinforce the need of genetic tests as first-tier diagnostic tool in this disease.

(Front Immunol, September 2022)

Altered Urinary Metabolomics in HAE – by Xue Wang and Yuxiang Zhi, Peking Union Medical College Hospital, China:

Uncovering the metabolic signatures of HAE may provide inspiration for a comprehensive understanding of HAE pathogenesis and may help explore potential new metabolic biomarkers. We performed a comprehensive metabolic analysis. Several metabolites associated with riboflavin metabolism, the citrate cycle, oxidative stress, and inflammation, including xanthine, oxypurinol, vitamin B2, and isocitrate, were significantly altered in HAE patients. No significantly different metabolites were found in HAE patients carrying different mutation types. The study highlights that metabolic disturbances in the purine metabolism, riboflavin metabolism, and TCA cycle may be involved in the pathogenesis of HAE. These findings may help to identify novel candidate metabolite biomarkers associated with HAE.

(Metabolites, November 2022)

A review of berotralstat for the treatment of HAE – by Henriette Farkas and Zsuzsanna Balla, Semmelweis University, Hungary:

The fundamental aim of the treatment of C1-INH-HAE is to ensure that patients can lead a normal life. The most effective way to do this is to prevent the onset of angioedema attacks. The availability of the first targeted oral prophylactic drug, the kallikrein inhibitor berotralstat, in 2021, is a milestone in the treatment of patients with HAE.

(Expert Rev Clin Immunol, November 2022)

HAE During Pregnancy: Considerations in Management – by Marc A. Riedl, University of California - San Diego, United States:

Pregnancy significantly influences the clinical symptoms of HAE in many women because of estrogen effects or other physiologic factors, and introduces important safety concerns related to HAE medications. Management of HAE during pregnancy requires clinicians to be familiar with the potential clinical course, triggers, and recommended treatment strategies to provide guidance and optimal medical management to women and families.

(Immunol Allergy Clin North Am, February 2023)

Long-term Prophylaxis with Androgens in the management of HAE in emerging countries – by Yinshi Guo, Renji Hospital, China, et al.:

Although androgens are not approved for HAE treatment in many countries, they are widely used in China and Brazil to reduce the frequency and severity of HAE attacks. The long-term adverse effects of androgen treatment are concerning for both physicians and patients. Virilization, weight gain, acne, hirsutism, liver damage, headache, myalgia, hematuria, menstrual disorders, diminished libido, arterial hypertension, dyslipidemia, and anxiety/depression are commonly observed during long-term treatment with androgens. These adverse effects can affect the quality of life of HAE patients and often lead to treatment interruption, especially in women and children.

(Orphanet J Rare Dis, November 2022)

Prospective Study on the Efficacy and Impact of Cascade Screening and Evaluation of HAE (CaSE-HAE) – by Jane C. Y. Wong, University of Hong Kong, et al.:

Cascade family screening is an effective approach to family screening in HAE, improving clinical and psychological outcomes, and reducing disease-related costs.

(J Allergy Clin Immunol Pract, November 2022)

Phenotypic and Genotypic Characterization of HAE in Saudi Arabia – by Farrukh Sheikh, King Faisal Specialist Hospital and Research Centre, Saudi Arabia, et al.:

HAE cohorts from several populations have been published but no large-scale study has been reported from the Arab world to date. We document the clinical and genetic findings of HAE patients from a single Saudi institution, which is a major referral center at the national level. A total of 51 patients across 17 unrelated families were recruited including two large multi-generational families. The predominant HAE subtype in our cohort was type I, at 76%. No type III (normal C1-INH) patients were encountered in the clinic, suggesting that this subtype does not play a major role in HAE pathogenesis in Saudi Arabia. Additionally, the existence of four patients with consistently normal complement 4 (C4) levels alongside abnormal C1-INH profiles highlights the utility of dual screening for both proteins in suspected patients.

(J Clin Immunol, November 2022)

Pregnancy in women with HAE due to C1-inhibitor deficiency: Results from the ITACA cohort study on outcome of mothers and children with in utero exposure to plasma-derived C1-inhibitor – by P. Triggianese, University of Rome Tor Vergata, Italy, et al.:

Through appropriate management and counseling, most women with C1-INH-HAE undergo successful pregnancy and delivery. For pregnant C1-INH-HAE women being treated with plasma derived C1INH, our findings are reassuring and might lead to an improvement of both the knowledge about treatments and the experience of HAE itself.

(Front Med (Lausanne), September 2022)



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 should be recruiting at this moment:

A Gene Therapy Study of BMN 331 in Subjects with HAE

– recruiting in the United States

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

– recruiting in France, the Netherlands, the United States

A Phase III, Crossover Trial Evaluating the Efficacy and Safety of KVD900 for On-Demand Treatment of Angioedema Attacks in Adolescent and Adult Patients with HAE

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

Assessment of the State of Health, Quality of Life and Expectations of Patients with HAE

– recruiting in France

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

– will be recruiting in the United Kingdom

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

– recruiting in China

A Study of Lanadelumab in Persons with HAE Type I or II

– recruiting in Austria, Germany, Israel, Italy, Kuwait, Portugal, Spain, Switzerland

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

– recruiting in Argentina

A Study of Lanadelumab in Teenagers and Adults with HAE

– will be recruiting in the United Kingdom

A Study of Long-Term Safety and Efficacy of Lanadelumab for Prevention of Acute Attacks of Non-histaminergic Angioedema with Normal C1-Inhibitor

– recruiting in the United States

A Study of STAR-0215 in Healthy Adult Participants

– recruiting in the United States

A Study of the Burden of Illness and Treatment Patterns in Teenagers and Adults with HAE

– recruiting in Austria, Belgium, Bulgaria, Canada, Croatia, Czech Republic, Estonia, Germany, Greece, Hungary, Ireland, Israel, Latvia, Lithuania, North Macedonia, Poland, Portugal, Romania, Serbia, Slovakia, Slovenia, Spain

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

– recruiting in Bulgaria, Canada, Italy, the Netherlands, Spain, the United States

A study to evaluate if different doses of KVD900 are safe and effective in treating attacks in patients with HAE

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

A Study to Evaluate the Real-World Long-Term Effectiveness of Lanadelumab in Participants with HAE

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

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

– will be recruiting in Canada

A Study with Lanadelumab in Persons with HAE in Poland

– recruiting in Poland

A Survey of Icatibant in Pediatric Participants with HAE

– recruiting in Japan

A Survey of Lanadelumab in Participants with HAE

– recruiting in Japan

Berotrastat Treatment in Children with HAE

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



EU Clinical Trials Register

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

– will be recruiting

C1 Inhibitor Registry in the Treatment of HAE Attacks

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

Dose-ranging Study of Oral PHA-022121 for Prophylaxis Against Angioedema Attacks in Patients with HAE Type I or Type II

– recruiting in Bulgaria, Canada, Germany, Israel, Poland, Spain, the United Kingdom, the United States

Efficacy and Safety of Lanadelumab (SHP643) in Japanese Subjects with HAE

– recruiting in Japan

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

– recruiting in Spain, the United States

FIRAZYR General Drug Use-Results Survey (Japan)

– recruiting in Japan

Firazyr® Patient Registry (Icatibant Outcome Survey - IOS)

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



National Institutes of Health
Turning Discovery Into Health



EU Clinical Trials Register

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

– recruiting in Italy

HAE multi-national survey study

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

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

– recruiting in France

Lanadelumab tested in patients suffering from hereditary angioedema with normal C1-Inhibitor

– recruiting in Germany

NTLA-2002 in Adults with HAE

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

OASIS-HAE: A Study to Evaluate the Safety and Efficacy of Donidalorsen (ISIS 721744 or IONIS-PKK-LRx) in Participants with HAE

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

Pathophysiological study for autoimmune dysregulation of HAE

– recruiting in Japan

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

– recruiting in France, the United States

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

– recruiting in United States

The Role of the Coagulation Pathways in Recurrent Angioedema

– recruiting in France

Read more about these and other clinical trials at:

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

New HAE resource



UNDERSTANDING HAE

During the 2022 HAEi Global Leadership Workshop, HAEi Executive Project Manager Jørn Schultz-Boysen presented the patient guide 'Understanding HAE' fresh from the print shop.

“Our new patient guide is a good example of ideas often coming from the bottom up as it is based on requests from Member Organizations looking for a comprehensive brochure with a full description of HAEi and HAE matters”, says Jørn Schultz-Boysen.

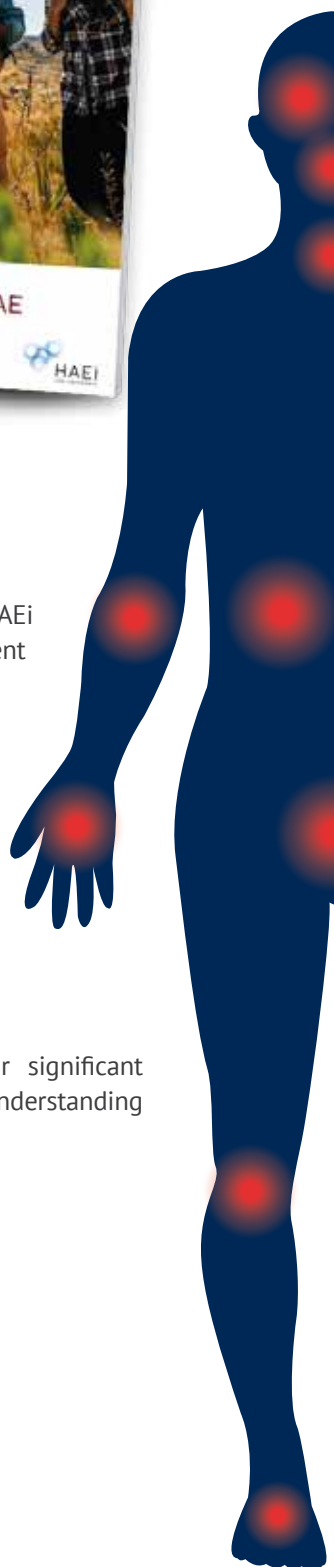
The patient guide contains a presentation of HAEi and, not least, the many resources offered by HAEi: HAE TrackR app, Emergency Cards, HAE Companion app, Global Access Program, website and social media, the **hae day** :-) global awareness day, the *Global Perspectives* magazine, HAE conferences and workshops, and the HAEi Youngsters' Community.

Currently, the patient guide exists in English, but the plan is to introduce more languages. If you want to translate the 'Understanding HAE' patient guide to your own language, reach out to your Regional Patient Advocate, who can help you with a template for translation.

In addition to a presentation of the HAEi resources, this is the content of the patient guide:

- HAE types
- HAE symptoms
- Attack triggers
- Diagnosing HAE
- Treatment options
- HAE and children
- Women and pregnancy
- Traveling

HAEi thanks the US HAEA for their significant contribution to the content of the 'Understanding HAE' patient guide.



NEWS FROM THE INDUSTRY

27 October 2022

At the presentation of the **Pharming Group N.V.** preliminary, unaudited financial report for the first nine months ended 30 September 2022, CEO Sijmen de Vries says:

“In the first nine months of 2022, we continued to perform well despite challenging industry conditions. Our commercialized asset RUCONEST® saw increased sales of 3%, compared to the same period last year, highlighting the continued need for a safe and effective acute HAE therapy.

As announced during the half-year financial results in August 2022, we initiated an internal review of our pipeline with a greater and renewed focus towards rare diseases. As a result, we will advance the development of our pipeline through OTL-105, a gene therapy for HAE.”

Pharming’s strategic collaboration with Orchard Therapeutics to research, develop, manufacture, and commercialize OTL-105, a newly disclosed investigational ex vivo autologous hematopoietic stem cell (HSC) gene therapy for the treatment of HAE, is ongoing. The program has made good progress in developing the lentiviral vector to enhance C1-inhibitor expression and is now testing in preclinical HAE disease models.

(Source: Pharming)



31 October 2022

KalVista Pharmaceuticals, Inc. announces positive phase 1 data for an orally disintegrating tablet (ODT) formulation for its lead compound sebetralstat. Sebetralstat is currently being developed in the phase 3 KONFIDENT clinical trial as a potential on-demand treatment for HAE attacks.

“This new ODT formulation reflects a next step in our constant efforts to provide people with HAE more options to help them manage this disease,” says Andrew Crockett, CEO of KalVista. “Orally disintegrating tablets are a standard in other disease areas such as migraine and would be of benefit to many people with HAE, particularly younger patients. We expect this formulation to become available in the US and rest of the world following the initial launch of sebetralstat tablets.”

KalVista recently completed a clinical trial to investigate the pharmacokinetics of the ODT. The phase 1, open-label, randomized, single-dose, 3-way crossover trial enrolled 36 healthy adult volunteers to compare the pharmacokinetics (PK) of sebetralstat following administration of ODT and the current film-coated tablets in healthy adult volunteers. Data from the study showed that the ODT tablet formulation has a similar PK profile to the film-coated version currently in development. Based upon these results, KalVista intends to continue to develop the formulation with the intention to make it available as soon as possible after the sebetralstat anticipated launch in the US. KalVista currently expects that data from the KONFIDENT study using the film-coated tablets will be available in the second half of 2023, to support a planned NDA filing in the first half of 2024.

(Source: KalVista)



1 November 2022

“With ORLADEYO on a trajectory to more than double sales in its second year of launch after a very strong first year, we continue to demonstrate that what we are offering HAE patients is unique and fills their need for a therapy that offers both low burden of disease and low burden of treatment. We believe that our ability to successfully pursue challenging targets like plasma kallikrein is just the start, and we expect we will bring rare disease patients many more oral therapies to offer these patients the unique treatment options they are waiting for,” says Jon Stonehouse, President and CEO of **BioCryst Pharmaceuticals, Inc.**, at the presentation of the financial results for the third quarter ended 30 September 2022.

ORLADEYO® (berotralstat) U.S. Launch

- The total number of patients on paid therapy grew by nine percent in the third quarter.
- New patient starts remained strong in the third quarter, in line with the running six quarter average.
- The ORLADEYO prescriber base grew by 11 percent in the third quarter.

These strong underlying growth trends were masked by accelerated direct shipments requested by patients late in the second quarter in June prior to the July 4 holiday weekend, plus a negative out of period reimbursement-related charge that was accounted for in the third quarter.

ORLADEYO® (berotralstat) Global Updates

In the third quarter, ORLADEYO was approved in Saudi Arabia. The company expects continued approvals and launches in additional countries.

“With the strong underlying growth fundamentals we continued to see from patients and physicians in the third quarter, and the further growth dynamics we expect to see in the fourth quarter, we are on track to more than double ORLADEYO revenue in 2022 compared to 2021,” says Charlie Gayer, Chief Commercial Officer of BioCryst.

(Source: BioCryst)



4 November 2022

At the presentation of operational highlights and financial results for the third quarter ended 30 September 2022, **Intellia Therapeutics, Inc.** President and CEO John Leonard, M.D., says:

“During the most recent quarter, Intellia has achieved significant clinical milestones. Interim data from our in vivo program, NTLA-2002, demonstrated robust reductions in both plasma kallikrein levels and the rate of swelling attacks in patients with HAE. These initial data support the potential for a single dose of NTLA-2002 to permanently prevent the debilitating and potentially fatal HAE attacks that characterize this lifelong genetic disease. We look forward to presenting additional data from NTLA-2002 later this month. The interim datasets demonstrate the modularity of our CRISPR-based platform, providing powerful evidence of our ability to apply this technology broadly to address a wide range of diseases.”

(Source: Intellia)



10 November 2022

BioCryst Pharmaceuticals, Inc. announces new real-world data demonstrating rapid, sustained reduction of patient-reported HAE attacks and consistently low attack rates among patients 12 years and older who started on oral, once-daily ORLADEYO® (berotralstat) for the prophylactic treatment of HAE, including patients who switched from other prophylactic therapies.

The data are being presented at the 2022 Annual Scientific Meeting of the American College of Allergy, Asthma & Immunology (ACAAI).

“These data are particularly exciting because consistent with our long-term clinical program data, they show that patients on ORLADEYO sustain, and even improve, attack control the longer they are on therapy,” says Dr. Ryan Arnold, Chief Medical Officer of BioCryst: “It is notable that this real-world evidence suggests that people living with HAE can maintain or improve control of their disease on ORLADEYO, regardless of prior treatment history.”

“Every HAE patient has a unique experience with their therapy, and these data demonstrate that ORLADEYO can be a very effective treatment option for patients regardless of their reported prior attack rates or prophylactic therapy history. The sustained, long-term attack rate reductions we are seeing illustrate the durability of this efficacy for patients who are looking to improve control over their HAE by switching to a therapy with a less burdensome route of administration than subcutaneous or intravenous prophylactic therapies,” says William R. Lumry, M.D., Clinical Professor of internal medicine at the University of Texas Southwestern Medical School.

The presentations at ACAAI are based on analyses from patient-reported results collected in the real-world clinical setting from BioCryst’s sole-source pharmacy, including HAE Type I and Type II patients in the United States who actively received ORLADEYO between 16 December 2020, and 20 May 2022.

BioCryst ACAAI 2022 Presentation Highlights **Consistently Low Hereditary Angioedema Attack Rates Observed with Berotralstat Regardless of Previous Prophylaxis: Real-World Outcomes:**

- This analysis assessed patient-reported HAE attack rates of patients on ORLADEYO 110 mg or 150 mg who were previously on another prophylactic therapy (n=129), including lanadelumab (n=53),

a subcutaneous (SC) C1 esterase inhibitor (n=31), danazol (n=15) and an intravenous (IV) C1 esterase inhibitor (n=21). Nine patients were on a combination of prophylactic therapies.

- Regardless of prior prophylaxis, a rapid reduction in median attack rates was observed early (1.67 attacks/month at baseline to a median attack rate of 0.33 attacks/month in days 1-90). The reduction of median attack rates was sustained throughout the 360-day treatment period.
- Upon initiating ORLADEYO treatment, the reduction in median attack rates from baseline over the 1–360 days period was consistent for patients regardless of their prior prophylaxis therapy. The reductions after starting ORLADEYO for each prior prophylactic therapy were:
 - 77 percent reduction for patients previously on lanadelumab;
 - 64 percent reduction for patients previously on SC C1-INH;
 - 70 percent reduction for patients previously on danazol; and
 - 72 percent reduction previously on IV C1-INH.
- The incidence of AEs reported was lower than the incidence reported in clinical trials.

Rapid and Sustained Reductions in Hereditary Angioedema Attack Rates with Long-term Berotralstat: Real-World Outcomes (Distinguished Industry Oral Presentation):

- This analysis assessed the efficacy of ORLADEYO 110 mg or 150 mg in patients (n=128) for a treatment period of more than 270 days.
- Patients reported a meaningful reduction in HAE attack rates when treated with ORLADEYO. An 80 percent average reduction from median baseline attack rate (1.67 median attacks/month at baseline vs. 0.33 median attacks/month during days 1-90) was observed during the initial 90-day treatment period. Regardless of baseline attack rate, patients reported a reduction in attack rates when treated with ORLADEYO.
- The incidence of adverse events (AEs) reported was consistent with the incidence reported in clinical trials.

(Source: BioCryst)



10 November 2022

“We are on track to report preliminary results from our Phase 1a trial of STAR-0215 by the end of this year. The trial is assessing safety and tolerability and will inform on dosing frequency and target engagement. We believe the results from this trial will validate STAR-0215’s differentiated best-in-class profile,” says Jill C. Milne, Ph.D., CEO of **Astria Therapeutics, Inc.**, at the presentation of the financial results for the third quarter ended 30 September 2022: “STAR-0215 is a long-acting monoclonal antibody inhibitor of plasma kallikrein that has the potential to reduce treatment burden for patients with HAE with dosing once every three months or longer.”

Phase 1a Clinical Trial of STAR-0215

- The Food and Drug Administration cleared Astria’s Investigational New Drug application for STAR-0215 in July 2022.
- The Phase 1a randomized, double-blind, placebo-controlled single ascending dose trial evaluating the safety, pharmacokinetics, and pharmacodynamics of STAR-0215 at a single U.S. center was initiated in August 2022. Astria has enrolled and dosed 24 evaluable subjects who were randomized to receive single subcutaneous administrations of one of three dose levels of 100 mg, 300 mg, or 600 mg of STAR-0215 or placebo. The subjects are now in the follow-up phase.
- The trial aims to establish the prolonged half-life and demonstrate inhibition of plasma kallikrein activity, which, if favorable, would provide proof of mechanism for STAR-0215 as a potential best-in-class treatment for HAE.
- The company plans to initiate a multi-center, global, single and multiple dose Phase 1b/2 proof-of-concept trial in people living with HAE, called ALPHA-STAR, or Astria Long-Acting Prophylaxis for HAE: STAR-0215, in Q1 2023, subject to favorable Phase 1a results.

STAR-0215 Highlights

- Astria presented new preclinical data at the European Academy of Allergy and Clinical Immunology 2022 Hybrid Congress that demonstrate STAR-0215’s rapid and durable inhibition of plasma kallikrein in cynomolgus monkeys, supporting the potential for once every three month or longer dosing in humans. The study demonstrated rapid inhibition of plasma kallikrein after subcutaneous administration. Inhibition of high molecular weight kininogen cleavage was rapid and sustained throughout the entire 84-day dose-free period in the extended portion of the study. These data confirm the long

half-life of STAR-0215 and demonstrate prolonged pharmacological activity of STAR-0215 in circulation in cynomolgus monkeys.

(Source: Astria)



10 November 2022

Pharvaris presents two in-person “ePoster – Meet the Author” at the American College of Allergy, Asthma & Immunology (ACAAI) Annual Scientific Meeting 2022.

“The data demonstrate the optimized pharmacokinetic and tolerability profiles of Pharvaris’ drug candidates that are in clinical development for the treatment of HAE,” says Peng Lu, M.D., Ph.D., Chief Medical Officer of Pharvaris. “PHVS719 is designed to be a once-daily prophylactic treatment for the prevention of HAE attacks, as supported by data demonstrating compound absorption in the colon and maintained exposure above predicted therapeutic levels. The cross-over pharmacokinetic data described in the second poster support the dosing regimen in CHAPTER-1, a Phase 2 study evaluating PHVS416 as a proof of concept of PHVS719 for the prophylactic treatment of HAE.”

Development of PHVS719: an Oral Extended-Release Bradykinin B2 Receptor Antagonist to Prevent Hereditary Angioedema Attacks

Pharmacokinetic properties of PHA121 were evaluated to support the intended therapeutic use of PHVS719 for the prophylactic treatment of HAE attacks using preclinical and clinical experimental models. Colonic absorption of PHA121 was investigated as a requisite for prolonged absorption of the extended-release formulation under development. In rodents, plasma concentrations following oral and intracolonic administrations of PHA121 were comparable, providing evidence that PHA121 can be systemically absorbed by colonic mucosa. In humans, high oral bioavailability and low fecal excretion further indicate almost-complete absorption in the gastrointestinal tract. Together, these data support clinical development of the extended-release tablet PHVS719 as a once-daily prophylactic treatment of HAE attacks.

Pharmacokinetics of PHVS719, extended-release tablet formulation of PHA121, a first-in-class oral human bradykinin B2-receptor antagonist

In the Phase 1 pharmacokinetic study of PHVS719, 10 healthy subjects received, in a randomized order, two different doses of PHVS719 extended-release tablets (XR1 at 20 mg and XR2 at 40 mg), in fasting and in fed conditions, and one dose of PHVS416 20 mg in fasting conditions. Measurement of time-to-reach-therapeutic-exposure-levels for PHA121 above the EC85 of 13.8 ng/mL showed that after administration of XR1 and XR2, therapeutic plasma concentrations were achieved within approximately two hours. Concentrations of PHA121 remained at therapeutic levels for at least 30 hours with XR2. Food intake did not have significant effects on the time to reach therapeutic exposure of PHA121 nor on the time at which concentrations remained at therapeutic levels. The 24-hour area-under-the-curve exposure of PHA121 after XR2 was comparable to that observed in Phase 1 studies with PHVS416 softgel capsules dosed at 20 mg bid with food. Administration of both PHVS719 and of PHVS416 were well tolerated. No severe nor serious treatment-emergent adverse events were reported, with no specific safety pattern or trend in number or type of events. The 10 treatment-emergent adverse events that were reported in 50% of the participating subjects were mainly of Grade 1 severity or of Grade 2 severity, occurred after only one of the administrations of study drugs, and all completely resolved. Two of these events, namely neck pain and post-procedural hypotension, were considered as not related to the study drug. PHVS416, a softgel capsule formulation of PHA121 is being evaluated for safety and efficacy outcomes in CHAPTER-1, a Phase 2 proof of concept clinical trial, which is currently ongoing in countries outside the U.S. The U.S. Food and Drug Administration has placed on hold on clinical trials of PHA121 in the U.S.

(Source: Pharvaris)

PHARVARIS

13 November 2022

At the American College of Allergy, Asthma & Immunology (ACAAI) Annual Scientific Meeting, **Ionis Pharmaceuticals, Inc.** presents positive results from a Phase 2 open-label extension (OLE) study evaluating the safety and efficacy of its investigational antisense medicine, donidalorsen, in patients with HAE. Interim data after all patients completed 1 year of treatment in the study showed a sustained reduction in HAE attacks and no new safety signals following treatment with donidalorsen. Treatment with donidalorsen resulted in an overall sustained mean reduction in HAE attack rates of 95% from baseline. For patients treated with donidalorsen, 99.6% of study days were HAE attack-free.

“The data further enhance donidalorsen’s profile and potential to provide significant sustained protection from attacks for people living with HAE,” says Richard S. Geary, Ph.D., Executive Vice President and Chief Development Officer at Ionis. “The positive results of the Phase 2 OLE are encouraging as we continue evaluating donidalorsen, a potential best-in-class medicine, in the ongoing OASIS Phase 3 program.”

Patients completing the Phase 2 study were eligible for enrollment in the OLE study. There were 20 Type 1 or Type 2 HAE patients in the Phase 2 study, and 17 (85%) entered the OLE. Following a 13-week fixed-dose period where participants received subcutaneous donidalorsen 80 mg every four weeks, eight patients switched to subcutaneous donidalorsen 80 mg every eight weeks. Patients who remained on donidalorsen 80 mg every 4 weeks had a mean reduction in attack rate of 95.3% and 98.3%, from Week 1 (after first dose) and Week 5 (after second dose), respectively. Patients receiving donidalorsen 80 mg every eight weeks experienced a mean reduction in attack rate of 75.6% from baseline and the mean monthly attack rate was 0.28. Five of these patients remained attack free over the one-year duration of this analysis, and three patients returned to 80 mg every four weeks.

No serious adverse events were reported in the OLE study and no treatment-emergent adverse events (TEAEs) led to study discontinuation. There were no clinically relevant abnormalities in any laboratory measurements.

(Source: Ionis)

IONIS

13 November 2022

At the American College of Allergy, Asthma & Immunology (ACAAI) 2022 Annual Scientific Meeting, **Intellia Therapeutics, Inc.** announces updated data from an ongoing Phase 1/2 clinical study of NTLA-2002 for the treatment of HAE. The data are from 10 adult patients with HAE in the Phase 1, dose-escalation portion of the study, with a data cut-off date of 28 September 2022.

“We see early evidence that our one-time CRISPR-based investigational therapy may offer patients suffering from HAE a functional cure for their disease,” says Intellia President and CEO John Leonard, M.D. “Based on the extended data across multiple dose cohorts, we are strongly encouraged that all patients who received a single dose of NTLA-2002 subsequently became attack-free. In the patients with the longest follow-up to date, their attack-free interval has been maintained five to ten months from their last attack. Importantly, the safety data from all patients are highly encouraging, further supporting NTLA-2002’s potential to change the future HAE treatment paradigm. As the second clinical program from our in vivo pipeline to demonstrate deep and consistent protein reduction following a one-time administration, the latest interim data further reinforce the enormous potential of our modular CRISPR genome editing platform to treat a host of genetic diseases.”

At all dose levels, NTLA-2002 was generally well-tolerated, and the majority of adverse events were mild in severity. The most frequent adverse events were infusion-related reactions, which were mostly Grade 1 and resolved within one day. There have been no dose-limiting toxicities, no serious adverse events and no adverse events of Grade 3 or higher observed to date. No clinically significant laboratory abnormalities were observed.

Intellia expects to select up to two doses to further evaluate NTLA-2002 in the Phase 2, placebo-controlled, dose-expansion portion of the study, which is expected to begin in the first half of 2023. Intellia anticipates expanding country and site participation, including U.S. clinical sites, as part of the Phase 2 study.

(Source: Intellia)

Intellia
THERAPEUTICS

14 November 2022

KalVista Pharmaceuticals, Inc. presents two posters at the 2022 meeting of the American College of Allergy, Asthma & Immunology. The first highlighted new data that reveals the significant impact of HAE attacks on the mental health, daily activities, and quality of life for people living with HAE receiving either parenterally delivered on-demand only treatment or prophylaxis with on-demand treatment for breakthrough attacks. More than half of people with HAE felt unable to participate in important events or activities, while nearly half felt 'less than 100% themselves' due to HAE.

The second presentation focused on injection- or infusion-related adverse drug reactions (ADRs) reported in the FDA's Adverse Event Reporting System (FAERS) for currently approved on-demand HAE therapies. The five most frequently reported ADRs were injection site pain, site swelling, site erythema, access site complications/malfunctions, and incorrect route of product administration.

"Both HAE patient feedback and FAERS data suggest that there is still a significant unmet need for on-demand treatments that have greater ease and less traumatic routes of administration," says Andrew Crockett, CEO of KalVista. "This is exactly why we are developing our pipeline of oral therapies, to improve the treatment experience and ultimately the lives of people living with HAE."

(Source: KalVista)



22 November 2022

TAKHZYRO® (lanadelumab), the first approved monoclonal antibody for the treatment of HAE, has been named the 2022 winner of the prestigious Prix Galien Canada Innovative Product Award. The most prestigious award in the field of Canadian pharmaceutical research and innovation, Prix Galien Canada recognizes the efforts and achievements of pharmaceutical research and development.

"We are honored TAKHZYRO has received the Prix Galien Canada 2022 Innovative Product Award, and are even more proud of the tremendous impact it's had on HAE patients in Canada," says Rute Fernandes, General Manager of Takeda Canada Inc. "This prestigious award is a reflection of Takeda's dedication and commitment to bring innovative treatments to patients."

(Source: Takeda)



28 November 2022

The Israeli Ministry of Health has granted marketing authorization for oral, once-daily ORLADEYO® (berotralstat) to prevent attacks of HAE in adults and pediatric patients 12 years of age and older in Israel.

"We continue to make significant progress in bringing our oral, once-daily prophylactic therapy to many HAE patients around the world," says Charlie Gayer, Chief Commercial Officer of **BioCryst Pharmaceuticals, Inc.** "With today's announcement, we look forward to working with our partner Neopharm to launch ORLADEYO in Israel."

"The approval of ORLADEYO is a major advancement for the HAE community in Israel. We are proud to partner with BioCryst to bring this oral therapy to patients who are in search of a less burdensome treatment option to help manage their disease," says Efi Shnaidman, General Manager of Neopharm Israel.

Neopharm Group has the exclusive rights to commercialize ORLADEYO in Israel.

ORLADEYO was safe and well tolerated in clinical trials. The most frequently reported adverse reactions in patients receiving ORLADEYO compared with placebo were back pain and gastrointestinal reactions. The gastrointestinal reactions generally occurred early after initiation of treatment with ORLADEYO, became less frequent with time, and typically self-resolved.

(Source: BioCryst)



2 December 2022

At its annual R&D investor briefing, **CSL** demonstrates how its growing, innovative pipeline is well-positioned to meet the current and future needs of patients and public health. Among the highlights:

Top-line Phase III results for garadacimab (CSL312, anti-FXIIa), an investigational first-in-class monoclonal antibody being developed as a long-term preventive treatment for patients with HAE, demonstrated that the study met its primary and secondary efficacy objectives and showed favorable safety and tolerability. CSL aims to begin filing for approval with global regulatory authorities next calendar year. Garadacimab was discovered and optimized by scientists at CSL's Bio21-based Research site, with formulation and manufacturing for the clinical programs completed at the CSL Broadmeadows Biotech Manufacturing Facility. (Source: CSL Behring)

CSL Behring

8 December 2022

Pharvaris reports financial results for the third quarter ended 30 September 2022, and provides a business update:

“The results of the RAPIDe-1 study affirm our confidence in our clinical development program in HAE,” says Berndt Modig, CEO of Pharvaris. “Pharvaris is fully committed to resolving the clinical holds on PHA121 in the U.S., and we appreciate the opportunity to meet with the FDA in the recent Type A meeting and to work with the agency on the next steps. To date, the regulatory status for our studies outside the U.S. remains unchanged. Top-line data from CHAPTER-1, a proof-of-concept study of PHVS416 for the prophylactic treatment of HAE, is anticipated in the second half of 2023. Pharvaris has a strong financial position and will continue to operate with a disciplined approach as we aspire to bring best-in-class oral therapies to the HAE community.”

(Source: Pharvaris)



8 December 2022

KalVista Pharmaceuticals, Inc. provides an operational update and releases financial results for the second fiscal quarter ended 31 October 2022:

“We made a very difficult decision this quarter in terminating our phase 2 KOMplete trial for KVD824, but the safety of patients is of utmost importance to our Company,” says Andrew Crockett, CEO of KalVista. “We remain committed to providing best-in-class oral treatments for the HAE community. We are particularly excited about our recent data for the orally disintegrating tablet (ODT) formulation of sebetralstat, which will enable people to take a dose without requiring chewing or swallowing. We are also pleased with the progress of our Phase 3 KONfident clinical trial for sebetralstat, which remains on our enrollment target for data in the second half of 2023.”

(Source: KalVista)



HAEi Advocacy Academy

Your first step to successful advocacy

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

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

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

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



HAEi AROUND THE WORLD

Currently there are HAE member organizations in **95** 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.

