


# Global perspectives

HAEi MAGAZINE · ISSUE 2/2022

 95 Member countries



STEPPING UP FOR THE GLOBAL  
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#### Cover photo

HAE Scandinavia Stepping up to the HAE Movement for **hae day** :-:) with a united baking activity. Read about HAEi's activity challenge for **hae day** :-:) on page 22.

#### Magazine staff

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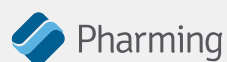
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HAEi is a global non-profit umbrella organization dedicated to working with a network of national HAE member organizations to raise awareness of HAE

## DEAR HAEi FRIENDS,

Welcome to the second 2022 edition of Global Perspectives, the quarterly HAEi magazine that offers a complete overview of just about everything that is happening in the world of HAE. We continue to expand our global footprint and are very happy to welcome Oman and Bolivia to HAEi's family, which now stands at 95 member organizations.

Excitement is growing as leaders from member organizations throughout the globe prepare for the first face-to-face HAEi event since the advent of the pandemic in early 2020. The 2022 HAEi Global Leadership Workshop (GLW)—that will take place from 6 to 9 October in Frankfurt Germany—will focus on helping local leaders implement HAEi's suite of technology solutions to manage and grow membership as well as build the capabilities of their advocacy organization. GLW will also offer sessions that address: Newly approved HAE medicines; the importance of participating in ongoing clinical trials; therapies in the pipeline; and the role of HAEi-Ga2len ACARE Centers in upgrading the quality of care available to people with HAE. There will also be a medical/scientific track that will focus on improving diagnosis and management of HAE, and normalizing HAE patients' lives. We will also welcome representatives from pharmaceutical companies who will participate in the medical/scientific track and have an exhibit space.

HAEi's commitment to uniting our global community and enhancing human interaction through innovative technology will be on display at GLW. We are very excited to announce that participants will be able to access real-time translation on their own personal devices in their preferred language! This cutting-edge simultaneous translation capability is powered by a cloud-based platform (called "Wordly"), which eliminates the need for special hardware and live interpreters working in sound booths.

We are also very pleased that our proprietary HAEi Virtual Center technology has been successfully implemented at the "The Elisabeth McArthur Virtual Angioedema Center" in Sydney, Australia. The virtual center solution



connects people with angioedema symptoms to expert physicians without regard to geographic location. We also see a possible role in broadening the reach of experts in centers that are running clinical trials by allowing experienced investigators to oversee clinicians in other locations. The platform features, among other things, a dedicated website, an appointment booking engine, an appointment notification/reminder feature, and a secure (peer to peer) video consultation room.

Please keep in mind that our kind, compassionate, and knowledgeable Regional Patient Advocates (RPAs) are always ready to help Member Organizations organize and implement a successful advocacy program. See page 6 for the latest reports from our RPA's.

Finally, I would like to highlight changes we are making to future HAEi sponsored gatherings. The steady growth in our community has made it impossible to find a venue large enough to accommodate the HAE Global Conferences we have held in the past. With that mind, we are now planning 3 in-person Regional Conferences that will allow maximum participation while best serving the diverse situations and needs faced by HAEi friends throughout the world:

- 2023 HAEi Regional Conference APAC (Asia Pacific),
- 2023 HAEi Regional Conference EMEA (Europe, Middle East, and Africa), and
- 2024 HAEi Regional Conference Americas (North and South America)

I wish all HAEi friends good health and happy reading of this Global Perspectives edition!

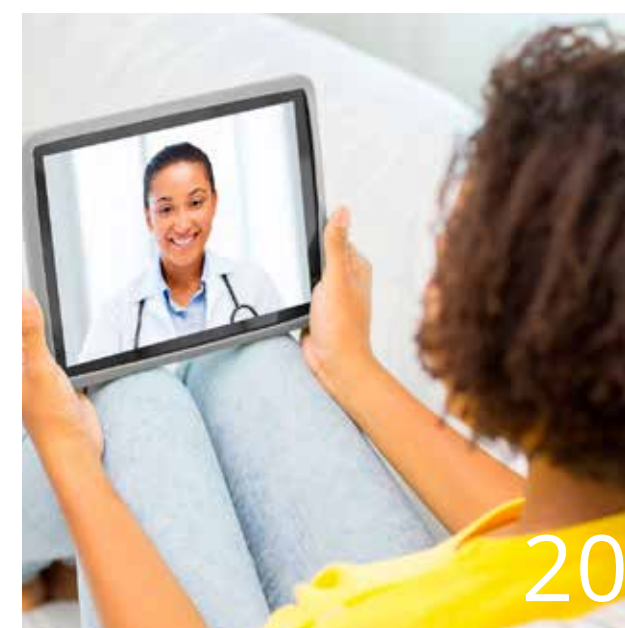
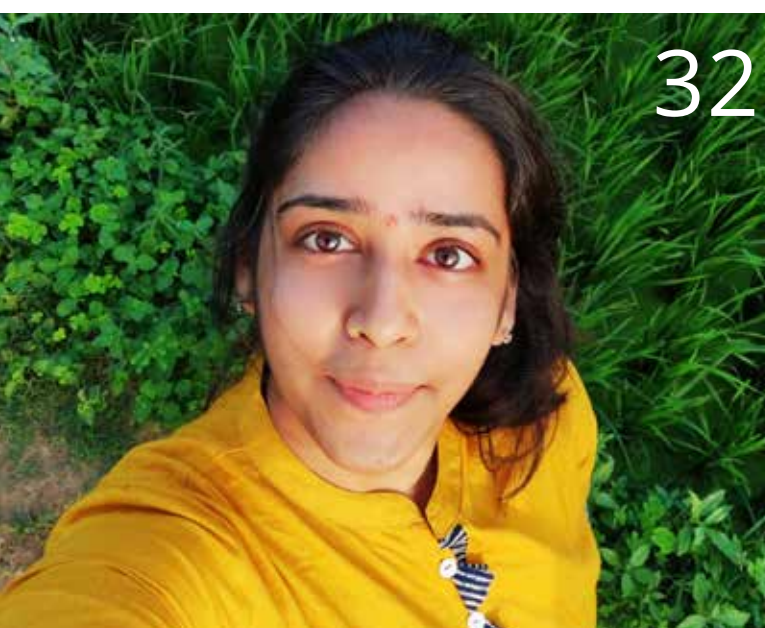
Warmest regards,

*Tony*

Anthony J. Castaldo  
President and CEO, HAEi



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hae  
day :-)

MANY FACES  
ONE FAMILY

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# NEWS FROM THE HAEi REGIONAL PATIENT ADVOCATES

Welcome to the latest news and updates from our Regional Patient Advocates (RPAs) around the globe. Their summaries below are full of information and exciting updates for HAEi's member organizations worldwide.

HAEi has two new member organizations bringing the total to 95! Please join me in welcoming Bolivia and Oman under the global umbrella.

Two significant events have taken the spotlight over the last few months – **hae day :-)** and the upcoming 2022 HAEi Global Leadership Workshop.

HAEi has sent out information and invitations to the leaders of member organizations, Regional Medical Advisors, and physicians inviting them to participate in the HAEi Global Leadership Workshop in Frankfurt, Germany, 6-9 October 2022.

This year, the **hae day :-)** events and festivities have been inventive, and it's great to see the raising of HAE awareness around the globe. Some of the awareness activities included social media posts, media interviews, special lighting projected on buildings, along with walks and other outdoor activities in exciting places. In addition, member organizations took advantage of relaxed COVID-19 restrictions by holding awareness raising face-to-face meetings and gatherings.

**HAE TrackR** and HAE Companion are two creative apps that demonstrate HAEi's commitment to supporting people with HAE and their families. With **HAE TrackR**, you can record your treatments (preventative and on-demand) and attacks to help you and your physician better manage your HAE. The app is currently available in 28 languages. If you would like the app to be available in your language, please reach out to your RPA. The HAE Companion app is an excellent resource especially when you are traveling. It provides an easy way to access and electronically store the popular HAE International emergency card, which is available for 84 countries. HAE Companion also offers contact information on HAE knowledgeable hospitals (including ACAREs) and physicians worldwide.

**Fiona Wardman**  
Chief Regional Patient Advocate



**MARIA FERRON**  
THE MEDITERRANEAN, NORTH AFRICA  
AND THE BRITISH ISLES



This period has been really exciting and active for several reasons. Firstly, due to changes in the pandemic situation, we are allowed to go back to more face-to-face meetings, and it is nice to see again members of our HAE community. Also, after more than two years, the HAEi Regional Patient Advocates met again in person in May 2022. And finally because many activities have been organized around the world celebrating **hae day :-)** 2022.

On 14 May, I had the pleasure of being invited to an HAE patient meeting held in Padova organized by A.A.E.E. (HAE Italy) and ITACA (the Italian Network for Hereditary and Acquired Angioedema). After two long years of pandemic shutdown, I was pleasantly surprised to see how many attendees joined the meeting (face to face and online). We were around 70 attendees on site. The ITACA team informed that they already have 21 centers around the country; they have been organizing training courses for pediatricians and planning to organize during September or October HAE Academy for doctors. Furthermore, there is contact with ACARE to maybe become a member of that network.

During the sessions dedicated to the association, President Pietro Mantovano encouraged the audience to talk to family members, caregivers, and the HAE community, in general, to become members of the association as the number of members is relatively low when compared to the number of patients diagnosed in Italy. This seems, by the way, to be a common thing in the Mediterranean countries. Also, Carlotta Cicardi and Martina Perera (A.A.E.E. secretaries) went to the stage to talk about the projects done last year and to encourage young Italians to join the HAEi youngsters' community because they believe that this is part of the organization's future. To finalize the meeting, I was invited on stage with Marco Castiglione (Responsible

for International Relations) to talk about HAEi, its tools, projects, activities, etc., and encourage patients and caregivers to participate in **hae day :-)** 2022. It was really nice to see some of my old Italian friends again and meet new ones.

Concerning **hae day :-)** 2022, there have been some exciting activities in my region. Not all of them could take place on the official date, but they still commemorated the very special HAE awareness day. Here are some of the activities:

- In Algeria, HAE Algeria decided to organize a city tour in the UNESCO world heritage city Casbah on 28 May. The organization wanted this year's event to be more diverse in content with both cultural and fitness activities. They hit a record of about four km walk (6,000 paces and more than 400 stair steps!).
- In Portugal, ADAH decided to launch a video on 16 May called "The faces of HAE", where Portuguese patients talk about their experiences living with HAE. Please check the Portuguese Facebook page to see the video: [shorturl.at/rsxN5](https://shorturl.at/rsxN5).
- In Spain, AEDAF decided to organize a walk in the Madrid region on 15 May. It took place in the Dehesa de Navalvillar in the town of Colmenar Viejo. Also, those members who could not join the walk were encouraged to send other activities and time spent to AEDAF to be added to [haeday.org](https://haeday.org) as a total time dedicated by the AEDAF team.
- In Italy, ITACA (the Italian Network for Hereditary and Acquired Angioedema), with the support of AAEE (HAE Italy), organized over several days an "open day" where all ITACA centers were open to the patients to enter and talk to the doctors at any time.



**JÖRN SCHULTZ-BOYSEN**  
NORDICS, GERMANY, AUSTRIA AND SWITZERLAND



The Regional Medical Advisory Panel (RMAP) for my region is still being put together. Yet another country has found its RMAP, which is great – for other countries, we are waiting for a confirmation on who should join. The first round of dialogue has already been going on. Still, it will be great to get the next countries involved as well to get the whole region activated in this activity, and thus benefit from the great experience from all the involved persons.

Similarly, feedback from the Regional Advisory Groups is also starting to come in, which is excellent. This will enable us to benefit from the experience of the local organizations in the region and to use the ideas present for HAEi.

The HAE app TrackR continues to be a great tool and is now available in 28 languages – several of which are spoken in the region. TrackR 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 start using it!

Approaching the summer period in the region – and a time when many decide to travel – another recommendation is to check out the HAE Companion app in the Apple App Store and Google Play. This app will help you find local HAE treatment centers and directions hereto, as well as you will find the emergency cards available in more than 84 countries. You can download the 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 traveling to a country is to have the emergency cards ready in the local languages. Or to have the emergency card in your language available on your smartphone when at home.

Germany continues to be very active, amongst other things, on social media with posts, podcasts on living with HAE, and many other activities. On 14 May 2022, HAE Germany hosted a virtual **hae day :-)** patient event to celebrate the awareness day.

HAE Switzerland held its annual patient meeting on 30 April, incidentally the first face-to-face patient meeting after the corona pandemic. A great success which you can read more about later in this magazine. Also, Switzerland has been busy being on air on the TV show "Gesundheit Heute" to mark **hae day :-)** and to speak more about everyday life with HAE as well as being covered in the magazine "Schweizer Familie" on 19 May. Furthermore, HAE Switzerland celebrated **hae day :-)** by sending all its members a gift for the awareness day.

HAE Austria celebrated its 15th anniversary and, at the same time, held the first patient meeting after corona. That happened on 30 April 2022. I had the pleasure to join the meeting and meet with both the leadership of HAE Austria, patients and caregivers, and physicians and pharma representatives. It was a very nice meeting with a good mixture of learnings and knowledge exchange as well as a social aspect – it was simply nice to meet people again.

It should also be mentioned that our Regional Medical Advisory Panel representatives from Austria and Switzerland have given great input to important questions, which HAEi is using to further enhance and develop the HAEi Advocacy Academy.

HAE Finland has held several online meetings over the past months. I had the pleasure to present at the meeting taking place on 31 May, where the focus was on some of the patient and caregiver-oriented services

HAEi can assist our Member Organizations with – among other things, Global Perspectives, HAE TrackR, HAE Companion, and news about the upcoming Global Leadership Workshop and regional conferences.

In Denmark, three patient meetings are currently ongoing. As with all other meetings, it's great to get out and meet the patients and their caregivers again. Norway and Sweden are planning two patient meetings in each country, all held in the autumn of 2022. The three countries are working on some exciting projects for kids and youngsters. Furthermore, in HAE

Scandinavia, **hae day :-)** was celebrated by sharing a lovely cake recipe for everyone to get busy baking – it is, of course, important to get some new calories after burning a lot of them during the **hae day :-)** activities.

A new HAEi patient guide is being developed, and it has now reached a stage where we have been able to share it with our contact at the University Hospital of Reykjavik, Iceland. We are currently looking for ways to translate the "Understanding HAE" guide to Icelandic so our patients, caregivers, and the whole HAE community in Iceland can benefit from it.



**PATRICIA KARANI**  
SUB SAHARA AFRICA



Every year patients living with HAE celebrate life and hope on (and/or around) the annual HAE awareness day. This year HAE Kenya participated in the 1st Stakeholders Workshop for Rare Diseases in Kenya. KEMRI, Cambridge Africa, and Rare Disease International supported Rare Disorders Kenya and started a Call-to-Action Campaign on rare diseases in Kenya. The agenda was to pave the way for rare diseases in the country. The Ministry of Health has put together a Technical Workforce Group for rare diseases in Kenya, which spells a lot of hope for patients with rare diseases. We highlighted challenges and ways to mitigate these challenges as I represented HAE and thus HAE Kenya. Indeed, this was a happy hae day :-)

The HAE Zimbabwe team of doctors has embarked on a patient identity campaign to ensure that suspected HAE patients receive free medical consultation as they do their medical workup to provide proper diagnosis of patients. Dr. Careen, our contact doctor, is spearheading this campaign which has seen a lot of response from the general public.

Finally, I am happy to report that HAE patients in Sudan now have access to correct prophylactic medication and also access to an HAE knowledgeable doctor.





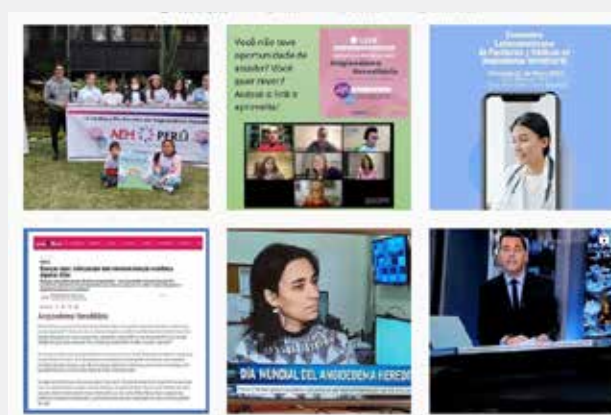
**FERNANDA DE OLIVEIRA MARTINS**  
SOUTH AMERICA AND MEXICO



Very recently, we welcomed Bolivia to our global HAE family as two Bolivian patients are working on establishing a patient group. A group has already been created on Facebook – please make sure to visit and like [shorturl.at/kCGQ1](https://www.facebook.com/kCGQ1). Furthermore, HAEi is in the process of creating a website for Bolivia. The patients in Bolivia have the support of two specialized physicians.

On 14 May, I joined with the Member Organization leaders for a video conference to align the activities the countries were organizing for **hae day :-)** 2022. We agreed to use the same template in the region for patients to include their pictures and share them on social media.

To further raise awareness of HAE, I have promoted the amazing activities being done in my region – have a look at our Instagram at [shorturl.at/lzRZ3](https://www.instagram.com/shorturl.at/lzRZ3).



**NATASA ANGJELESKA**  
SOUTH EASTERN EUROPE



I have had intense communication with the Member Organizations making sure that they were aware of the HAEi newsletter announcing the Global Leadership Workshop in October 2022. In addition, I had continued contact with some of them about the need for translation at the workshop. I'm happy that patient representatives from all countries in my region are interested in participating and have applied for travel grants. Their applications were approved, and registration was finalized by the end of May.

In Bosnia-Herzegovina, the patient representative got in contact with a patient group that is in the process of registering an alliance for rare diseases on a federal level in their country. The aim is to include HAE.

HAE Cyprus has informed me that Takzyro has been approved, but the health authorities have not yet brought it into the country. HAEi issued a letter supporting their request and submitted it to the health authorities.

I have had intense, almost daily, conversations with patients and caregivers from Kosovo in the past period. I recently had a more extended conversation with Dr. Rushit and explained the medicinal products recommended. Furthermore, I sent a link to papers describing HAE in general as well as medications and the influence of the disease on the quality of life of the patients.

I've sent information regarding the translation of the HAEi app TrackR to Bulgaria and reminded HAE Cyprus that the app is already translated into Greek, and their patients can start using it.

I recommended Natasha Jovanovska Popovska from HAE Macedonia to attend an ACARE webinar titled "Make a Difference – Mitigating Disparity – Patient and Physician Perspectives in the Treatment of HAE". During the preparation of the webinar, a pre-recorded video was required from patients participating, in addition to their participation. Natasha filmed a five-minute video on "My Life with HAE", where she talked about the dark past for patients from North Macedonia and a brighter looking future. She emphasized the need for prophylactic treatment for some patients as well as the need for an individualized approach to treatment for various patients.

More good news from North Macedonia as the first ACARE Center has been accredited: The Clinic for Allergology and Dermatology in Skopje.

One more thing from North Macedonia: Prof. Dr. Vesna Grivcheva Panovska prepared a webinar on "Coping with HAE in a Time of Pandemic". The first part of the webinar contained presentations by Dr. Laurence Boullet presenting the case in France and Dr. Mauro Cancian presenting the case from Italy. The second half was a panel discussion and a Q&A session with the participation of HAE physicians from Romania (Dr. Noemi Bara), Croatia (Dr. Ljerka Karagalapic), Serbia (Dr. Slagana Andrejevic), Albania (Dr. Eris Mesonjeshi) as well as patient representatives from North Macedonia (Natasha Jovanovska Popovska) and Turkey (Ersan Sevinc).

As for Albania, Silvi Bastri and Joanna Alkoci inform us that the process for registration of the patient organization is finalized, and HAE Albania has, among other things, made a logo. A letter has been sent to the health authorities on the consequences of non-available treatment for HAE in Albania. Based on the death of two patients, HAE Albania has requested immediate

access to therapy in all 12 regions of the country. Most recently, HAE Albania has met with a representative from the Ministry of Health and elaborated on the challenges and needs of HAE patients.

Regarding **hae day :-)** 2022, I was very active in promoting the activity challenge among patients and the general public and shared invitations for participation many times via SoMe. I am happy that a lot of patient representatives, caregivers, physicians, friends, and pharma representatives supported our global awareness initiative.

Many countries from my region organized activities to celebrate **hae day :-)** 2022:

- HAE Bulgaria prepared an extensive information campaign with two videos that can be watched on their website. In addition, Prof. Maria Staevska and President Danail Dimov from HAE Bulgaria participated in the program of BNT1 "100% Awake" dedicated to HAE.
- HAE Croatia held its annual patient-physician meeting in Shibenik. This gathering had great presentations from specialists covering topics like treatments, gender differences, and pediatric patients.
- HAE Greece held a press conference, announced its new website, and presented a short video produced to inform the public about HAE.
- On 14 May, HAE Macedonia organized an event to raise awareness among the general public. This event was supported by the dance studio EUREKA through performances in a newly opened shopping center frequented by numerous people. HAE Macedonia distributed T-shirts with the awareness day logo for the dancers as well as for people recording events on the HAEi activity challenge website.
- HAE Serbia organized a round table "Let's Get to Know Each Other" at the Crystal Hotel in Belgrade. They also had several buildings in Belgrade lit in purple.
- In Turkey, Prof. Nihal Mete Gökmen organized the 1st National Hereditary Angioedema Conference. It was a massive success, with more than 90 specialized physicians willing to learn more. No doubt, this will make it easier to access HAE specialized physicians for patients around the country.



**MICHAL RUTKOWSKI**  
CENTRAL EASTERN EUROPE,  
BENELUX AND THE MIDDLE EAST



I am excited to announce that the HAEi global family now comprises 95 Member Organizations. One of the most recent additions is HAE Oman, represented by Maryam Al Balushi. So, HAE Oman, welcome on board!

March, April, and May 2022 have been very busy with different advocacy activities. I was invited as a speaker to the ACARE Make a Difference webinar focused on “Mitigating disparity – patient and physician perspectives in the treatment of HAE”. I presented the patients’ perspective and the role of the patient organizations in reducing inequities in health resources worldwide, focusing on Eastern Europe. Together with other HAE patients, we shared our stories, achievements, and experiences in terms of international collaboration.

Also, I had the pleasure to present at the **hae day :-)** webinar for Ukrainian patients and health care professionals by Takeda. Moreover, I have been engaged in supporting HAE patients from Ukraine, who entered the neighboring countries, especially countries from my region, and were looking for healthcare assistance and access to HAE therapies.

Furthermore, I am happy to share with you that as of 1 May 2022, the Polish HAE patients gained access to and reimbursement for another therapy, recombinant C1-Inhibitor. There are currently six HAE medicines reimbursed in Poland, both on-demand treatment and long-term prophylaxis, which is unprecedented. It shows that everything is possible!

Over the last quarter, I organized and attended numerous individual virtual meetings with patient organizations from Belarus, Georgia, Hungary, Kazakhstan, Lithuania, the Netherlands, Poland, Russia, and Slovakia. Also, I organized regional zoom meetings for HAEi Member Organizations from Egypt, Jordan, Lebanon, Qatar, Saudi Arabia, and the United Arab Emirates. Apart from the virtual meetings, I was in email contact with patients

and caregivers from Estonia, Belgium, Iran, Iraq, Kuwait, and Oman.

Also, I participated in videoconferences with manufacturer representatives from BioCryst Pharmaceuticals, CSL Behring, KalVista Pharmaceuticals, Pharming N.V., and Takeda.

The HAE TrackR App is now available in two more languages from my region: Hungarian and Armenian – this is thanks to the great work of the Member Organizations. Furthermore, the HAEi Emergency Room poster has been translated into Armenian and Georgian.

However, the most time-consuming has been the activities regarding the 2022 HAEi Global Leadership Workshop. HAEi’s first in-person gathering after the pandemic gains a lot of interest from the HAE community. Therefore, the Regional Patient Advocates have lots of work communicating with the Member Organization to secure that everyone will register to attend the event. I keep close contact with all Member Organizations from my region, helping them with all the arrangements regarding the Global Leadership Workshop.

Finally, I would like to introduce you to the new President of HAE-QE (the Dutch patient organization), Marijk Beekman-Kortekaas, who has very recently been elected. We wish you all the best in your daily patient advocacy activities.



**FIONA WARDMAN**  
ASIA PACIFIC



The last few months have been busy in the countries in the Asia Pacific region and South Africa.

As the 2022 HAEi Global Leadership Workshop invitations have been sent out to the Member Organizations, there has been a lot of interest, and registrations have been completed. I’m looking forward to spending time with the Member Organization leaders from my countries in Frankfurt in October.

Clinical trials are being run in Taiwan, and Hong Kong, which brings the opportunity for patients who haven’t been on a prophylactic therapy to trial the modern therapies and (hopefully) give patients the experience of living attack free or at least a reduction in attacks.

New courses in Advocacy Academy were rolled out, including extra information like case studies for existing courses. The HAEi app TrackR is being downloaded by new patients in India and other countries to track attacks, and the emergency room poster has been translated into Mandarin.

The Asia Pacific region is seeing an increase in HAE presentations for education and awareness. The conference run by the Asia Pacific Association of Allergy, Asthma and Clinical Immunology (APAAACI) was the latest, bringing together international experts to talk about HAE.

**hae day :-)** 2022 was also a highlight during this period with meetings, interviews, gatherings, and media campaigns highlighting HAE and the burden of disease on our global awareness day: Australia, South Africa, South Korea, India, and China all had major events.

The HAE Society of India held its second in-person conference in Chandigarh, where HAEi presented the tools and resources useful for doctors. The results of the Indian Burden of Illness Study were also shown.

Lastly, I received the unfortunate news that one of the Patient Group Leaders in my region sadly passed away due to a throat attack. I want to stress the importance of seeking medical assistance for attacks in the face, throat, and neck – whether you have access to treatments or not. Please don’t delay getting help, and please keep advocating for modern therapies in your country.





**JAVIER SANTANA**  
CENTRAL AMERICA AND THE CARIBBEAN



In April, a follow-up was given to the survey that HAEi designed for my region in Spanish. The survey will help gather important information on the current situation related to HAE. All group leaders from the countries of the region participated in the survey.

Chile held a virtual conference on HAE to which all the leaders of the Central American countries were invited. As HAEi Regional Patient Advocate, I had the opportunity to speak to the participating patients and doctors about the work that HAEi does internationally and how we help patient groups.

During **hae day :-)** several groups in my region held events in their respective countries to educate and raise awareness about HAE. Several of the patient group leaders managed to get interviews in the media talking about **hae day :-)** and publications in some media. Furthermore, I have been invited to write several columns for newspapers of national circulation in the countries I represent to educate about HAE and explain the specific situation for which treatments do not reach the countries.

In May 2022, several events took place. For instance, HAE Costa Rica held a virtual meeting where new patients with HAE were invited, and I had the opportunity to talk about the work that HAEi does globally and how we help national patient groups. Also, HAE Puerto Rico held its annual conference where HAE specialist doctors talked about new treatments, research, and COVID-19 vs. HAE. As a representative of HAEi, I had the opportunity to address patients and give a presentation on advocacy and the process of getting HAE medicines to countries

through governments and their departments. Dr. Marc Riedl from US HAEA Angioedema Center in California was the main speaker and special guest. The president of the association of allergists and immunologists of Puerto Rico, Dr. Anardi Agosto, and the past president, Dr. Rafael Zaragoza, were also speakers. Furthermore, pharmaceutical representatives participated in the event.

New communications have been established with contacts in the Dominican Republic regarding my visit there soon to meet with government officials, doctors, and media representatives to discuss HAE.



## HAE Companion – free app for your travels

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](https://apple.co/33Qn4ZK)



[bit.ly/3osxkzm](https://bit.ly/3osxkzm)





## 'TOGETHER AGAIN'

### For the 2022 HAEi Global Leadership Workshop

We look forward to welcoming leaders from our 95 member organizations, HAE Physicians/Scientists, the HAEi Youngsters' Advisory Group, and industry sponsors to the 2022 HAEi Global Leadership Workshop, which will take place in Frankfurt am Main, Germany, from 6 to 9 October 2022.

We are excited to announce that we will offer on-site translation through an innovative advanced technology for the 2022 HAEi Global Leadership Workshop. This translation service called "Wordly" provides simultaneous translation for 20+ languages.

HAEi Executive Vice President & COO Henrik Balle Boysen says: "The workshop agenda offers learning opportunities and plenty of time for HAEi friends to interact. We are planning sessions to provide updated information on HAEi's suite of technology solutions that have been specifically designed to help our leaders manage and build their advocacy organizations. Other topics to be addressed include newly approved HAE medicines, clinical trials and the 14 therapies in the pipeline, and partnering with local HAEi-Ga2len ACARE Centers. In addition, the workshop's Scientific Track Co-Chairs, Professor Bruce Zuraw, Professor Konrad Bork, and Professor Marcus Maurer have developed a medical/scientific program that will address two areas of focus: Improving Diagnosis and Management of HAE, and Normalizing HAE Patients' Lives."

According to Deborah Corcoran, HAEi's Chief Specialist Projects and Research, the Scientific Track Co-chairs welcome abstracts on topics related to each area of focus as outlined below:

#### • Improving Diagnosis and Management of HAE

New/improved diagnostic approaches, prodromal symptoms, screening, markers of HAE, innovative treatment/management methods, and patient registry/real-world data.

#### • Normalizing HAE Patients' Lives

The impact of HAE on patient quality of life (work/education achievement, social life, and relationships), the psychological impact of HAE, the role of comprehensive HAE centers, the importance of a multidisciplinary approach to treating HAE, the importance of research networks, and real-world examples of normalizing patients' lives.

The abstract submission deadline is 15 July 2022. Email notifications regarding decisions on abstracts will be sent in mid-August 2022.

Please check <https://glw22.haei.org> frequently for the latest information on sessions, exhibitor information, and more. We look forward to seeing you in October!

## Supporters

The global HAEi family is grateful for the pharmaceutical companies that are supporting the workshop:

- BioCryst Pharmaceuticals Inc. (Diamond)
- Takeda Pharmaceutical Company Limited (Diamond)
- CSL Behring (Gold)
- Pharming Group NV (Gold)
- Pharvaris (Basic)
- KalVista Pharmaceuticals, Inc. (Basic)

## Register for the workshop

Registration for the 2022 HAEi Global Leadership Workshop will be on a first come, first serve basis. We recommend that you register early because the allotted slots are filling up fast!

>> [glw22.haei.org/registration](https://glw22.haei.org/registration)

## Attendee interpretation options

We will offer translation into 20+ languages during our 2022 HAEi Global Leadership Workshop. Languages currently supported by our translation partner "Wordly" includes:

- |                          |                       |                        |
|--------------------------|-----------------------|------------------------|
| • Arabic                 | • French (CA + FR)    | • Portuguese (BR + PT) |
| • Bengali                | • German              | • Romanian             |
| • Chinese (Simplified)   | • Hebrew              | • Russian              |
| • Chinese (Traditional)  | • Hindi               | • Spanish (ES + MX)    |
| • Czech                  | • Indonesian (Bahasa) | • Swedish              |
| • Dutch                  | • Italian             | • Tagalog              |
| • English (AU + UK + US) | • Japanese            | • Tamil                |
|                          | • Korean              | • Thai                 |
|                          | • Polish              | • Vietnamese           |



## 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 attacks, treatments and the impact HAE has on your life and the life of your loved ones.

- ✓ Is easy to use.
- ✓ Keeps track of both prophylactic and on-demand treatments and attacks if any.
- ✓ Is product and company neutral.
- ✓ Enables sharing data about your HAE with your physician.
- ✓ Can be accessed from a computer, tablet, or smartphone.
- ✓ Is available in more and more languages – right now 28 and still counting ...

Start using HAE TrackR today  
[app.haetrackr.org](https://app.haetrackr.org)

Get a full overview of your HAE.





# The HAEi Designed Virtual Center Concept is Now a Reality



## HAEi is excited to announce that its proprietary online, cloud-based, virtual center platform is now live!

According to HAEi Executive Vice President and Chief Operating Officer, Henrik Balle Boysen, "The Elizabeth Macarthur Virtual Angioedema Centre in Sydney, Australia is the first center to implement the HAEi Virtual Center technology, and we are working with other clinics interested in adopting the concept. Our virtual center solution connects people with chronic swelling disorders (who may live in remote areas or otherwise cannot travel) to expert angioedema physicians. In addition, we already envision many other ways that a virtual center can extend the reach of expert physicians."

Ole Frolich, HAEi's Chief Technology Officer says: "The platform includes a dedicated website per center, a sophisticated booking engine that allows setting an appointment with an angioedema specialist, a notification feature that reminds the guest and the physician about the agreed appointment, and a secure, peer-to-peer, video-based 'consultation room'. The HAEi Virtual Center platform also has an advanced administration module that allows each center to plan their time allocated to virtual services."

"We believe that virtual centers could also be used to broaden the reach of experts in Centers that are running clinical trials. Experienced investigators could oversee clinicians in other locations, conduct joint virtual visits with trial participants, and ensure that blood draws and other requirements are properly implemented," says Anthony Castaldo, HAEi's President & Chief Executive Officer.

Anyone interested in establishing a Virtual Center should contact Ole at [o.frolich@haei.org](mailto:o.frolich@haei.org).



## Who is behind the Elizabeth Macarthur Virtual Angioedema Centre

The Elizabeth Macarthur Virtual Angioedema Centre is the virtual leg of the Campbelltown Hospital, Immunology & Allergy Unit Dept Medicine, Therry St, Campbelltown 2560 NSW, Australia under the leadership of Prof. Connie Katelaris.

The center honors the name of Elizabeth Macarthur – an indomitable woman who combined being the mother of eight children with farming and pioneering the wool industry in Australia. She embodied courage and endurance – attributes displayed by many of those who live with a genetic disorder. Elizabeth Macarthur also displayed vision and innovation – attributes shared with the research community that has resulted in major advances in the management of HAE.



Read more about EMVAC here:

**>> [emvac.haei.org](https://emvac.haei.org)**



# Thank You for Stepping Up for the Global HAE Movement!

By Chief Specialist Projects and Research Deborah Corcoran

Throughout April and May 2022, our community came together to raise awareness of HAE with **hae day :-)** 16 May as the focus.

The outstanding HAE events and activities organized around the world included

- virtual patient days,
- educational events for doctors,
- dance and children’s events, walks, webinars,
- open days at medical facilities, and
- many people in our community sharing their HAE stories on multiple platforms, including social media, magazines, and television.

“It is always impressive to see the innovative ways our community steps up to raise awareness of HAE around our global awareness day,” says Henrik Balle Boysen, Executive Vice President & CCO, HAEi. “When we think we might have seen it all, our Member Organizations surprise and inspire us again.”

Our annual activity challenge saw participants regularly undertake and add activities – physical and wellbeing – to the **hae day :-)** website. The steps generated by the activities added between 1 April and 31 May created a virtual walk that took us around the world 2.8 times.

“The HAE awareness day provides a global opportunity to use the power of advocacy to positively influence the situation for all people with HAE. Every year I’m in awe of what we achieve when we come together. We truly are one family”, says Anthony J. Castaldo, President & CEO, HAEi.











**Since the first hae day :-) in 2012 we have generated a total of 554 mill steps; starting with 12 mill in 2012, a 10th-anniversary record of 169 mill in 2021 to 146 mill this year!**

## HOW DID THE COUNTRIES DO IN 2022?











### Total steps generated (mill.):

	United States	26,1 m
	Germany	18,6 m
	Sweden	17,8 m
	Denmark	13,5 m
	Canada	9,7 m
	Japan	9 m
	North Macedonia	8,7 m
	Finland	6,6 m
	United Kingdom	5,5 m
	Norway	5,4 m

### Number of activities:

	Canada	431
	North Macedonia	340
	Japan	330
	United Kingdom	293
	United States	253
	Denmark	181
	Australia	163
	Spain	129
	Brazil	125
	South Africa	118

### Number of participants:

	Canada	911
	North Macedonia	673
	United Kingdom	602
	United States	571
	Japan	429
	Denmark	387
	Australia	269
	Spain	246
	Germany	196
	Hungary	187



“**hae day :-)** is a good opportunity to continue to raise awareness about HAE all over the world, and in Spain, we have tried to do our part again this year: with group walks, a get-together with young patients and their parents, and a press release! Every little bit helps.”

Sarah L. Smith  
President, AEDAF (HAE Spain)



## 2022 ACTIVITY CHALLENGE IN NUMBERS

**145,828,745**  
steps taken in total

**5,538**  
activities

**43**  
countries

**111,150**  
kilometers

**203**  
photos



“With **hae day :-)** we feel we are not alone. We are motivated to celebrate the day and use it to raise awareness in the general public and share our HAE experiences with other patients.”

ADAH (HAE Portugal) Team







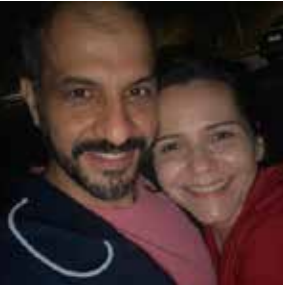
“More than ten years ago **hae day :-)** for HAE patients in Macedonia meant a sun on the horizon, a new hope that things would change for the better in their country. Little did they know, but their lives would be divided into a period before and after **hae day :-)** 2012. From that day, they became patient advocates and **hae day :-)** became a celebration of awakening the full potential of HAE patients in our country with the prospect of them becoming patient advocates.”

*Natasha Jovanovska Popovska  
President, HAE Macedonia*



“**hae day :-)** serves to create awareness and unity among patients. This year in Chile, many people sent their photos in support of the awareness day; they feel supported and united with other sister countries. It is a great activity.”

*HAE Chile Team*



“The annual event helps a lot. Above all to make people understand that we are not only people with HAE, but we are active people who make ourselves visible and help each other to improve our own lives and the lives of our children. It is also important for doctors to have their value and work on angioedema recognized. In short, **hae day :-)**, gives a voice to all of us!”

*Marco Castiglione, AAEE (HAE Italy)*



“**hae day :-)** also allows us to gather and involve our patients of the Association, motivating them to participate and carry out activities for the awareness day. This year for **hae day :-)** we launched the story ‘Motivating Lives – Hereditary Angioedema’, which allowed us to raise awareness about HAE by carrying out virtual and face-to-face activities to reach more families (kids and parents).”

*HAE Peru Team*



“**hae day :-)** motivates us to continue improving the quality of our patients’ lives and unites us more every day with very important expressions and feelings: ‘We are not alone,’ ‘We are not invisible,’ and ‘We are all united for the same reason.’ It is beautiful to come together, share experiences about when we have swelled, and work together to win the battle against HAE. We are stronger together and know that we will soon have skills and tools to make us stronger still. We will be successful as that is the reason our Association exists, to make life better for people with HAE in Venezuela.

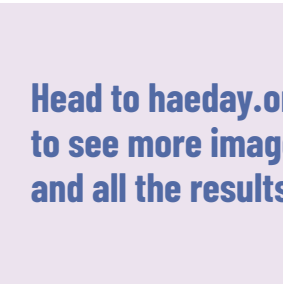
**hae day :-)** makes HAE stand out. It shows the world that we exist and are fighting to have our place on this beautiful planet.”

*HAE Venezuela Team*



“We are a group of HAE patients small in number and big in heart. For us, **hae day :-)** is a focus to come together as a family – adults and children with HAE – to share a positive and life-enriching experience. As well as participate in the activity challenge as a group!”

*HAE Algeria Team*



**Head to [haeday.org](https://haeday.org) to see more images and all the results!**





## Hacking Your Mood: Staying Motivated and Happy

*By Isabel Brunkan, member of the HAEi Youngsters Advisory Group*

Are you in need of a mood booster? Have you lost your motivation, and are you looking to get it back? As the end of the school year approaches for many youngsters, it's easy to lose motivation with the prospect of summer vacation just around the corner. Equally difficult can be regaining motivation after an attack and rebuilding the energy for exercise, schoolwork, and friends after having a flare.

Luckily, music can help! From triggering the brain's reward center and cueing learning to boosting your mood, neuroscience has shown how impactful music can be for us. Follow the link to learn more about how music can be used as a reward to help you learn: [shorturl.at/htEY2](https://shorturl.at/htEY2).

Additionally, the type of music matters! Stuck thinking of a topic for a final essay or on a hard math problem? You can trigger creativity by listening to specific types of music! A study investigating different types of music found that listening to Vivaldi helped trigger innovation in study participants. Have a look at the study here: [shorturl.at/divwP](https://shorturl.at/divwP).

The study found that listening to happy music resulted in a more diverse set of answers, thereby possibly enhancing mental flexibility and creativity!

Simply looking for a mood booster to get you energized and ready for the day?

A neuroscientist from the University of Groningen in the Netherlands worked with a British electronics brand to identify common traits of songs that reportedly made people "feel good". A mix of tempo and lyrics was found to be influential, and since publication, several playlists have been created with songs that fit these criteria. Read more about this here: [shorturl.at/empl3](https://shorturl.at/empl3).

Don't have Spotify? No worries – people have made YouTube playlists with similar songs as well. Have a look and listen at [shorturl.at/citY2](https://shorturl.at/citY2).

I hope you're enjoying the playlists.

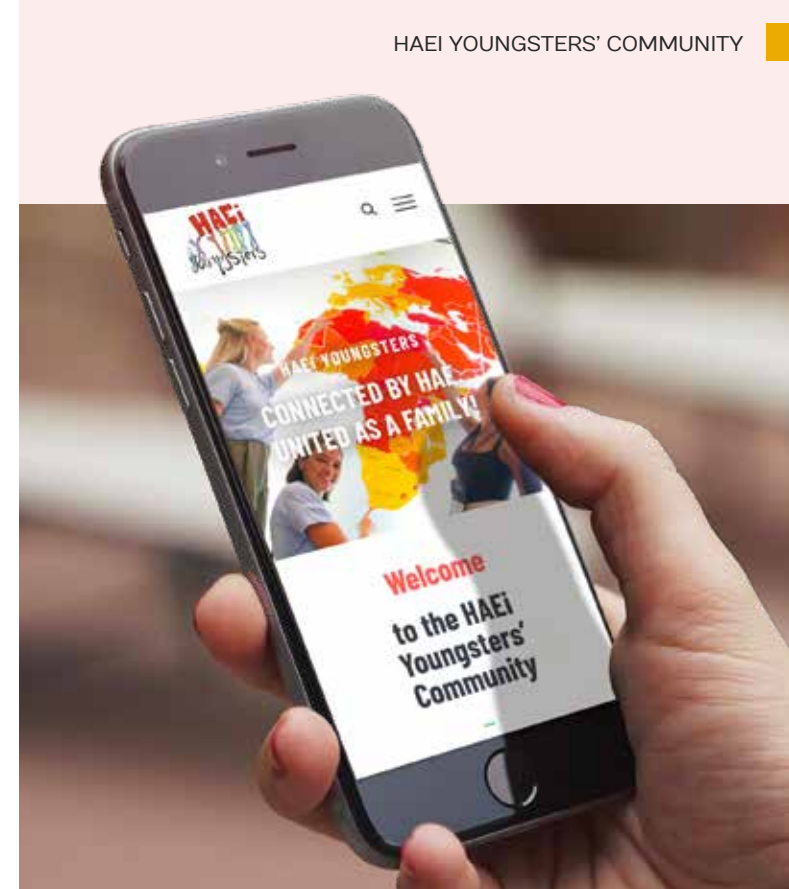
### Isabel



I'm Isabel from the US (though my mom is Chilean, I grew up in Costa Rica, and my family now lives in Victoria, Canada – so home, like the HAEi Youngsters Community, is a bit global).

I'm excited to be part of the community and hopefully help foster new connections.

Find more information and join the HAEi Youngsters' Community on our website: [youngsters.haei.org](https://youngsters.haei.org)



>> [youngsters.haei.org](https://youngsters.haei.org)

## HAEi Youngsters' Community website

The HAEi Youngsters' Community website is dedicated to young people, or as we like to call ourselves – youngsters – living with Hereditary Angioedema (HAE).

Whether you are newly diagnosed, starting school, university, moving abroad or starting a new job, this is the place for you. On our website you can find resources and tools that can help you learn more about HAE. Whether you want to build awareness about HAE in your community or become an HAE advocate, or simply need a way to explain what HAE is at school – we got you covered!

Our goal is to learn, stay connected, grow our global community, share our experiences and have fun together!

Join us and together we can make memories to last us a lifetime!

The Youngsters Advisory Group

>> [youngsters.haei.org](https://youngsters.haei.org)





## HAE Friends Around the World

*By the HAEi Youngsters Advisory Group*

The HAEi Youngsters Advisory Group (YAG) is delighted to introduce a wonderful new initiative they are very passionate about!

In March, HAEi's Operations Manager and Youngsters' Community Coordinator Nevena Tsutsumanova and Chief Specialist Projects and Research Deborah Corcoran led a two-day YAG meeting program filled with brainstorming sessions, presentations, guest speakers, and updates on exciting new projects. The meeting also included interactive discussion between YAG members and HAEi Leadership that focused on HAEi's overall mission, global footprint, and vision for the HAEi Youngsters Community.

The new initiative bring launched by YAG members is an outreach program – "HAE Friends Around the World" – that aims to (1) create connections and friendships around the globe, and (2) share the benefits of being part of the global HAEi Youngsters' Community.

Members of the YAG will be reaching out to youngsters internationally via social media to say hello and provide opportunities for youngsters to connect and learn from each other. The YAG hopes to build relationships, share experiences, and increase knowledge about HAE.

If you know of any youngsters in your country who might be interested in an international network of friends, please let them know they can get in touch with us via social media, email, or our website!

The HAEi Youngsters Community is for young people with HAE, their siblings, caregivers, and friends between 12 and 25!

We appreciate your support!

Don't forget to sign up for our newsletter  
 >> [youngsters.haei.org/newsletter](https://youngsters.haei.org/newsletter)

## TIME FOR SPRING CLEANING OF THE MEMBERSHIP REGISTER?

**MAKE GOOD USE OF  
HAE CONNECT – HAEi'S  
FREE AND CLOUD-BASED  
MEMBER DATABASE FOR  
NATIONAL ORGANIZATIONS  
TO MANAGE THEIR MEMBERS**  
 >> [HAEI.ORG/CONNECT](https://HAEI.ORG/CONNECT)

Good to know about HAEi Connect:

- Free and secure online membership database and communications platform
- User-friendly platform for collecting and storing member information
- Easy to use editor for creating emails to members as well as a template for saving and re-using emails
- Right-to-left language support
- Available to all HAEi member organizations for free
- Is used by 32 countries worldwide





**HAEI  
ADVOCACY  
ACADEMY**

## HAEI ADVOCACY ACADEMY SUPPORTING SUCCESSFUL ADVOCATES

*By Operations Manager Nevena Tsutsumanova and Chief Specialist Projects and Research Deborah Corcoran*

HAEi has many excellent tools and resources to support our growing number of Member Organizations and people with HAE. We designed HAEi Advocacy Academy with our global community in mind – a free, online, cloud-based virtual training platform – accessible from anywhere in the world.

“HAEi Advocacy Academy is an extremely useful resource for anyone. Whether you are an experienced advocate or just starting out, the courses are full of valuable information from skilled advocate leaders giving practical instructions, real-life examples, and case studies”, says Fiona Wardman, HAEi Chief Regional Patient Advocate.

Our virtual training platform has recently been expanded to include a range of case studies that showcase stories and experiences from HAEi Member Organizations. These add to the many educational courses on advocacy, disease-related information, and HAEi resources.

As a global organization, HAEi recognized the need for information and tools to help Member Organizations with every step of the advocacy journey.

“HAEi has a range of advocacy groups under our umbrella, from one person just beginning a patient group to much larger Member Organizations with lots

of experience. HAEi Advocacy Academy courses draw on global knowledge and expertise, and there are courses for everyone”, continues Fiona Wardman: “The most popular courses are ‘Building your Organization Part 1 - Getting Started’ to Part six in the series.”

HAEi Advocacy Academy allows you to access the courses in your own time. The progress bar shows you how much of a course you have completed, and you can leave the website and come back to finish the course another time – there is no expiry date. All modules are written in manageable ‘bite-size chunks’ that will allow you to start and finish a course in a timely manner.

We are very proud of HAEi Advocacy Academy and what it provides Member Organizations with. We would like to thank the Regional Advisory Groups (RAGs) for their on-the-ground insights, which are the inspiration behind all the courses. The courses bring to life the unmet needs and challenges highlighted by the RAGs from the first and second round of meetings with their Regional Patient Advocates (RPAs).

“HAEi Advocacy Academy is an excellent reminder of best practices and gives ideas on situations that may arise”, says Fiona Wardman.

Since the launch of the Member Organization’s Exclusive Access Courses, we have seen a growing number of people using HAEi Advocacy Academy and finding the courses very helpful:

- 35 of our member countries are using HAEi Advocacy Academy
- The most popular courses are ‘Building your Organization (Part 1) – Getting Started’, ‘HAEi Resources Summary for MOs’, and ‘HAE Management Plan’.

Courses are being released regularly, so make sure to check the HAEi Advocacy Academy website. If you are in doubt about how to register or, as a Member Organization, where to get your access code – contact your Regional Patient Advocate, and they will help you get started.

“

“We found the fundraising and working with pharmaceutical companies very useful, as it is something we are struggling to do right now. Being able to do this more effectively will help us run our Member Organization.”

*HAE Greece*

“HAEi set us up on HAEi Connect so we could better manage our member information. We had a lot of questions. HAEi Enterprise Technology Manager Ole Frolich directed us to the course in HAEi Advocacy Academy, and it answered all of them. The screen grabs were very helpful.”

*HAE Egypt*

”



**ADVOCACY ACADEMY CAN HELP  
EVERYONE BE A BETTER ADVOCATE!  
START NOW >> [ACADEMY.HAEI.ORG](https://academy.haei.org)**



# PATIENT STORY



*Pravalika Meduthuri, India:*

## NOT JUST TO SURVIVE BUT TO LIVE

I had my first HAE attack when I was around 17. It was an abdominal one, and it was very painful as well as confusing, as I guess would be the case for most people attacked by something they don't know what is. My parents and I didn't understand why it was happening, and initially, we thought it was food poisoning. Consequently, my diet was quite restricting, so both take-outs and eating out were very limiting at that point.

About four years later – after numerous attacks – I was in the hospital for three days straight. The swelling was way too much to handle, and this time it didn't respond to antihistamines or any kind of steroids. That raised suspicion among the medical staff, and eventually, I was diagnosed with HAE.

*How would you describe your adolescence as an HAE patient – did your condition keep you from doing things your friends did, or did you stick to your sport or other favorite activity despite having HAE?*

It was difficult initially, given that no prophylaxis treatment was available when I had my first attacks. But later on – after trying multiple medications and finding the one that works for me – I started doing all the things I love. I have a lot of hobbies now, and I like the fact that I'm able to keep up with my activities as things are nowadays.

As for hobbies, I like to try something new. I do different types of art, I try new workout techniques, I am a tech geek, I've tried amateur photography and short story writing, and it's been good so far. Also, I've killed enough plants to reach a level where I can finally raise some on my own.

*Are there any other HAE patients in your family?*

There are quite a few, actually. My father but also uncles and cousins. As for their condition, I'd say that it's much better than before. Given that everyone has their own set of medications and are keeping up physically as well.

### PRAVALIKA'S STORY IN BRIEF

- Born 1995 in Hyderabad, India – still living there. Married, no children yet.
- Batchelor in Computer Science; working as a full stack developer.
- First HAE symptoms in 2012; diagnosed four years later.
- Other HAE patients in her family: Father, uncles, and cousins.





*Would you say that you have met understanding from people around you?*

Understanding people around us is very important – also in general, I mean. Luckily people who are close to me have been very understanding and supportive of my decisions in life. I have doctors around for emergencies and on-call suggestions who respect my informed choices on the medications I take.

*Would you say that your condition influences your choices in life?*

Yes. That's a big yes. I got married recently, and when we decided to tie the knot, our biggest discussion was how we would handle my condition in emergencies. Obviously, it influences my daily life that I still have food restrictions. And when we are talking about the future – well, my condition will undoubtedly influence my decisions regarding having kids, the type of workouts I do, and the way I travel. All this and much more is and will be heavily influenced by my condition.

*Are you involved with HAE awareness work?*

I am. I volunteer for HAEi activities in India. I manage the patients' and doctors' database. I talk to new patients. I connect them with doctors in their area – in general, I help them as much as possible.

I was unable to attend the 2020 HAE Global Conference because I was affected by COVID-19 – and eventually, the conference ended up being canceled. All the more so, I'm looking forward to the next chance to meet with the international community.

My first involvement with the HAE Youngsters' Community was during Christmas 2020. The community is particularly important to me because I can connect and relate to everyone there. We do live in different countries but have similar goals, like, accepting HAE as a part of our lives and not letting anything affect our goals and ways of living. Not just to survive but to live.

*If you should advise a newly diagnosed patient from India, what your advice be?*

My major advice would be to reach out to the doctors and fellow patients. Get as much information as possible. Get all the questions answered. Keep checking for patient meets, online or otherwise. Talking to other patients – hearing how they handle HAE – will help a lot in understanding your own issues. Be transparent with doctors about the symptoms and get prophylaxis that would work for your medical history.

Also, I recommend keeping a doctor's prescription handy in case of emergencies. And your family should be very well informed about your condition and what to do in an emergency. Furthermore, you should note down all the numbers of nearby hospitals' ERs. Keep an ER bag handy with all the notes and stuff you need, such as daily medications. It'll help when you need to rush to the hospital.

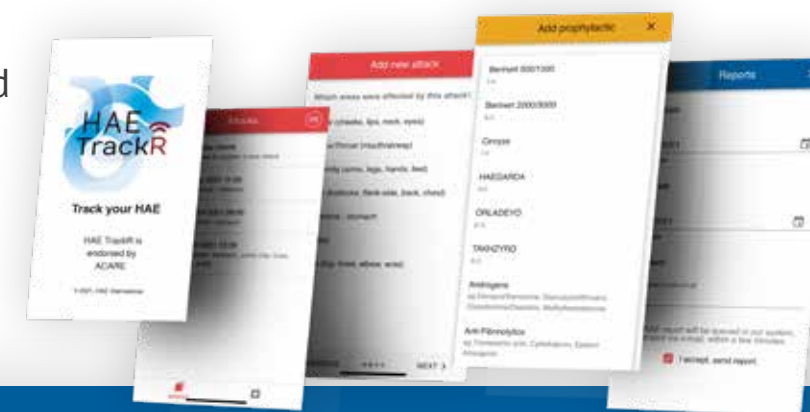


Free, secure and easy to use  
**app.haetrackr.org**

## Take control of your HAE!

### Cool facts about HAEi's HAE TrackR app

- ✓ Is an easy-to-use electronic diary to track your HAE and both prophylactic and on-demand treatments.
- ✓ Is developed by patients for patients and is product and company neutral.
- ✓ Enables inputting, storing, and sharing data about your HAE with your physician.
- ✓ Can be accessed from a computer, tablet, or smartphone.
- ✓ Is available in more and more languages – right now 28 and still counting ...



Start using **HAE TrackR** today: **app.haetrackr.org**



# HAEI ACARE CENTERS: A GROWING NETWORK TO SERVE PEOPLE WITH HAE

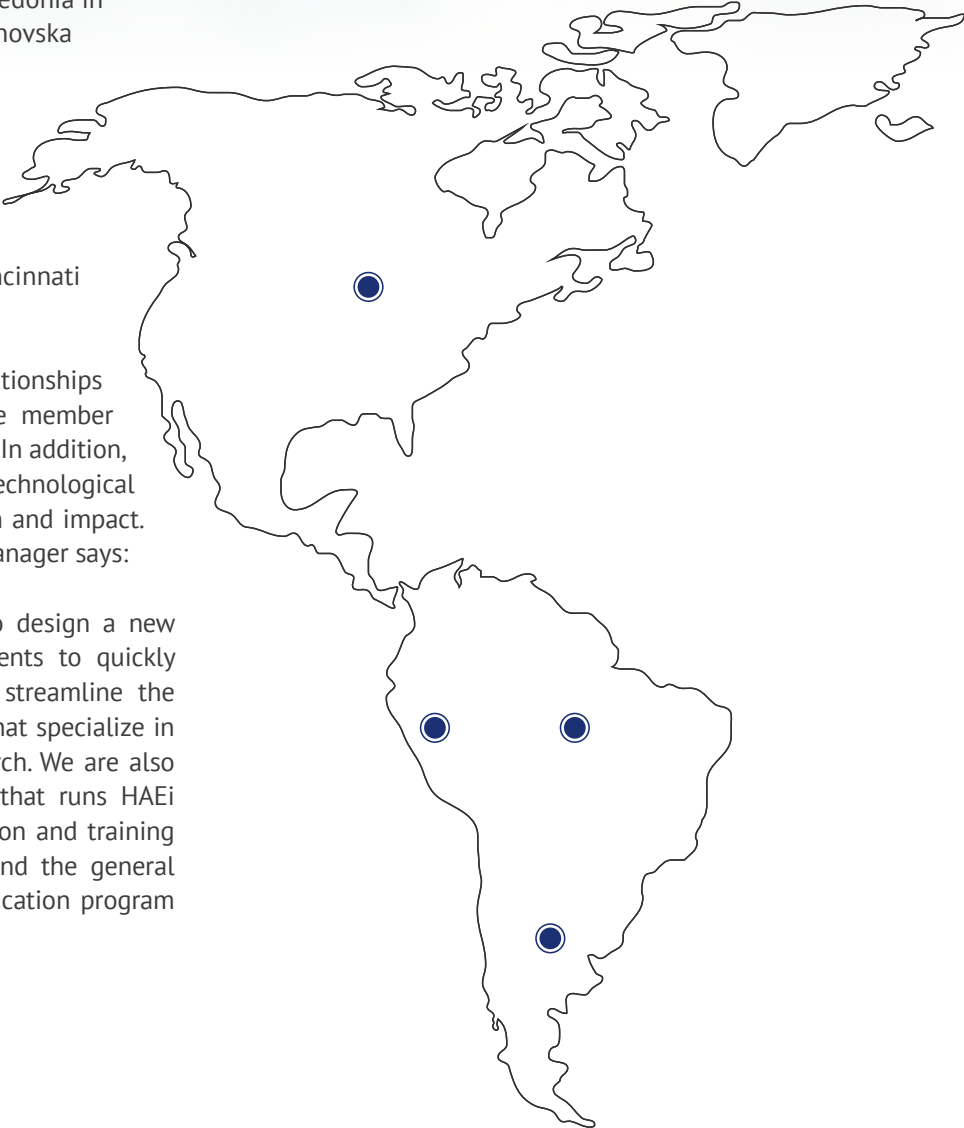
Membership in the global network of Angioedema Centers of Reference and Excellence (ACARE) – a joint venture between the Global Allergy and Asthma European Network GA2LEN and HAEi – continues to expand.

There are now 74 certified ACARE centers in 34 countries and another 17 applicant centers in the process of being certified. The most recent additions to the list are:

- **ITALY:** Allergologia e Immunologia Clinica nell'Università di Cagliari in Cagliari – Prof. Stefano Del Giacco
- **NORTH MACEDONIA:** HAE North Macedonia in Skopje – Prof. Dr. Vesna Grivcheva-Panovska
- **SPAIN:** The Allergy Department at Hospital Vall d'Hebron in Barcelona – Prof. Dr. Mar Guilarte
- **THE UNITED STATES:** The Division of Rheumatology, Allergy and Immunology at the University of Cincinnati College of Medicine, Cincinnati (OH) – Prof. Jonathan Bernstein

HAEi is working to build solid relationships with between ACARE Centers and the member organizations in their country or region. In addition, we are providing a high level of technological support to broaden each Center's reach and impact. Julia Föll, the ACARE Network Project Manager says:

“We are working closely with HAEi to design a new ACARE website that will enable patients to quickly find their closest ACARE Center and streamline the ACARE application process for clinics that specialize in angioedema diagnosis, care, and research. We are also working on adapting the technology that runs HAEi Academy – a platform housing education and training programs for member organizations and the general public – to host ACARE's physician education program called LevelUp.”

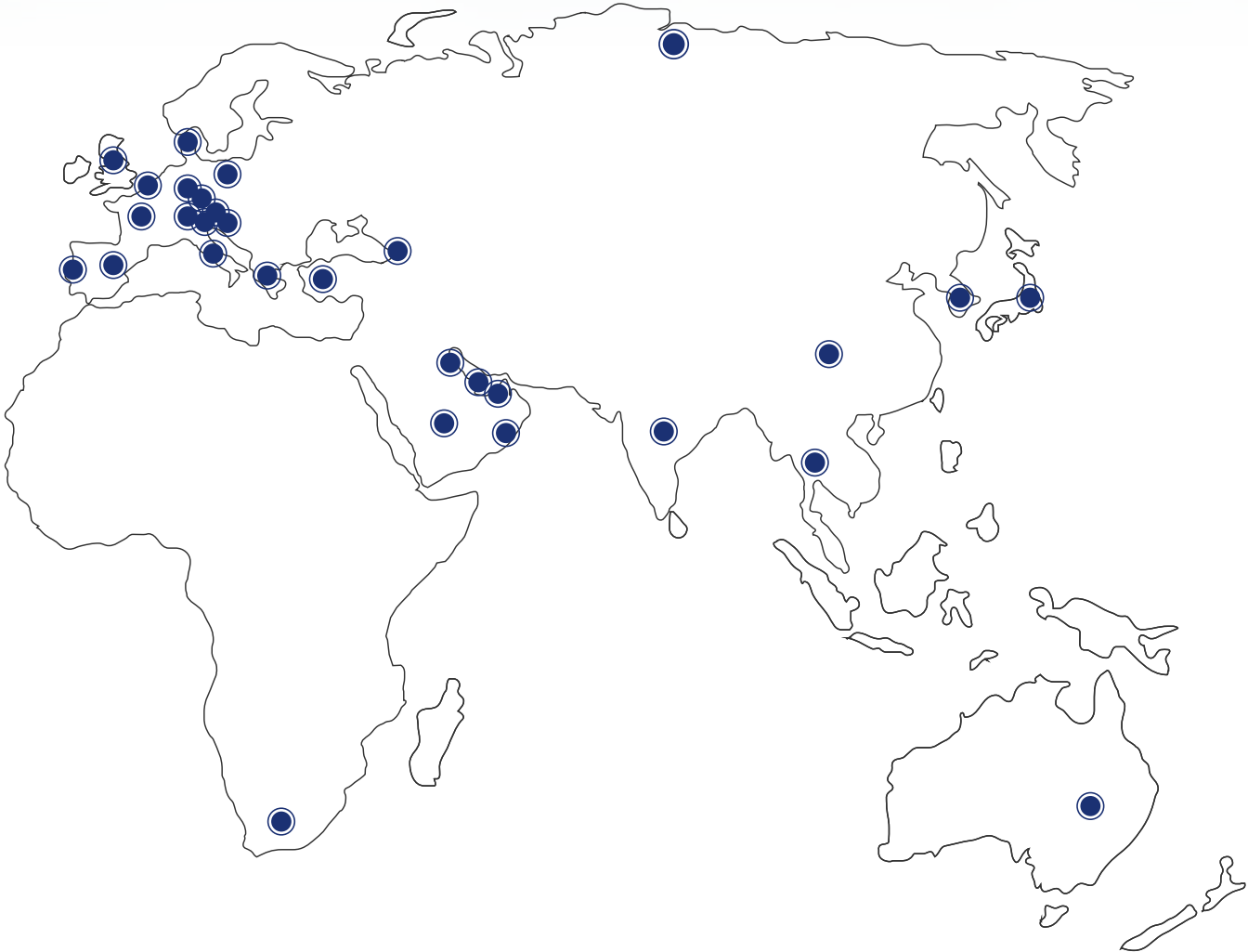


## 74 ACARE CENTERS:

Argentina (4), Australia, Austria (2), Brazil (10), Bulgaria (2), China (2), Denmark, France (3), Georgia, Germany (8), Greece, Hungary, India (3), Italy, Japan (3), Kuwait, the Netherlands, North Macedonia, Oman, Peru (2), Poland (5), Portugal, Qatar, Russia (2), Saudi Arabia, Slovenia, South Africa, South Korea, Spain (2), Thailand, Turkey (4), the United Arab Emirates, the United Kingdom (2), and the United States (2).

If you would like to become an ACARE center, please use this link for further information:

>> [haei.org/acare](https://haei.org/acare)





# 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's advice to those arriving in a country is to reach out to the HAE Member Organization for advice on doctors and treatment centers for HAE assistance – please see [haei.org/about-haei/globally](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 [haei.org/about-haei/meet-the-rpas](https://haei.org/about-haei/meet-the-rpas).



## BOLIVIA

We welcome Bolivia as member country no. 94 in the HAEi global family. At the same time, we have registered the first two HAE knowledgeable physicians in the country. That is Dr. Ronald Palacios Castrillo in Santa Cruz de la Sierra and Dr. Ariel Ramírez in La Paz. Please find contact information at <https://haei.org/hae-member-countries/bolivia>. At the same website you'll find contact information for the two national contacts, Jhosett Hinojosa and Kendra PeTerra. Bolivia has just launched its HAEi hosted website at <https://aehbolivia.haei.org> and if you want to have a look at HAE Bolivia's Facebook page, just go to [www.facebook.com/Angioedema-Hereditario-Bolivia-103697198999780](https://www.facebook.com/Angioedema-Hereditario-Bolivia-103697198999780).



## SINGAPORE

HAEi is very sorry to hear that the national contact in Singapore, Nurulannah Jamal, has passed away. For the moment being Regional Patient Advocate Fiona Wardman acts as the point of contact for Singapore.



## OMAN

Also, a warm word of welcome to Oman, the 95th member of the global HAEi family. National Contact is Maryam Al Balushi. At this point we have registered an ACARE center, an HAE knowledgeable hospital and an HAE knowledgeable physician in Oman. Contact information can be found at <https://haei.org/hae-member-countries/oman>.



## SOUTH AFRICA

*From HAE South Africa*

We started an awareness campaign running up to **hae day :-)** and began by filming three patient stories for inclusion on our social media platforms. We managed to secure seven interviews with various radio stations around the country in the run up to, and on, the awareness day. We had ten articles published in newspapers and magazines around South Africa, including three feature articles with interviews with some of our patients. Our stories appeared in both print and digital media.



We have recently launched a new website under the HAEi umbrella – please have a look at <https://southafrica.haei.org>. We have had numerous enquiries from patients via the new website, who we have directed through our testing and referral system to our ACARE Center.

Our patient database has increased from 104 to 116 patients in the last quarter.

Our chairperson, Janice Strydom, attended a press briefing in conjunction with Takeda and Rare Diseases S.A. for the “1 in 15” campaign, where we were able to share our patient stories and communicate our challenges in relation to access to healthcare, quality of life and time to diagnosis with various media outlets.



## THE NETHERLANDS

There has been a change in leadership of the Dutch HAE organization as longtime President Jan van Leeuwen has stepped down. New President is Marijk Beekman-Kortekaas – please see contact information at <https://haei.org/hae-member-countries/netherlands>.



## SPAIN

The second ACARE center has been accredited in Spain: The Allergy Department at Hospital Vall d'Hebron in Barcelona headed by Prof. Dr. Mar Guilarte. Please see <https://haei.org/hae-member-countries/spain> for contact information.



## NORTH MACEDONIA

*From HAE Macedonia*

HAE Macedonia organized an **hae day :-)** event on 14 May 2022 in order to raise awareness among the general public. Supported by the dance studio Eureka the event attracted a great audience, and we distributed T-shirts with the **hae day :-)** logo for the dancers and people who recorded events on the HAEi website. A beautiful story and a coloring book – ‘The Rare Boy and the Talkative Balloon’, authored by Natasha Angeleska, was distributed to passers-by. Furthermore, national Macedonian TV covered the event in the 8 o'clock news, presenting both stories of patients and information on the disease.

In the past ten years **hae day :-)** has for HAE patients in Macedonia not only been an opportunity to raise awareness among the general and medical public but it has also become a celebration of waking up the full potential in HAE patients with a prospect of them becoming patient advocates.

We are happy to inform you that North Macedonia has its first ACARE Center: The University Clinic of Dermatology in Skopje headed by Prof. Dr. Vesna Grivcheva-Panovska. You will find contact information at <https://haei.org/hae-member-countries/north-macedonia>.





## SERBIA

*From President Jovana Cvetkovic Lazic,  
HAE Serbia*

In order to celebrate **hae day :-)** 2022, HAE Serbia hosted the first in-person event in two years. This event was organized as a conference, where both HAE specialists and HAE Serbia were able to present. It was covered by all major media outlets in Serbia with more than 30 releases. The conference was attended by patients and their family members, representatives of other rare disease patient associations, representatives from RFZO (the Republic Health Insurance Fund) and of the Center for Rare Diseases at the University Clinical Centre of Serbia. The speakers were Dr. Sladjana Andrejevic, an HAE expert from the University Clinical Centre of Serbia, Dr. Gordana Petrovic from the Institute for Medical Care of Mother and Child of Serbia 'Dr Vukan Cupic', and myself.



I presented the results of a survey on the quality of life of HAE patients in Serbia, which was carried out by HAE Serbia prior to the awareness day. This survey allowed us to gather valuable and helpful information in an effort to improve the quality of life of patients with HAE.

We also organized a meeting that gathered patients from different cities and regions of the country where they could talk to HAE specialists, exchange experiences, and get all the necessary information.

Lastly, to raise HAE awareness, multiple landmarks in Serbia's capital, Belgrade, and in the cities of Nis and Novi Sad were illuminated in purple for **hae day :-)** 2022.



## ALGERIA

*From HAE Algeria*

For this second year of our existence and to celebrate **hae day :-)** 2022 HAE Algeria choose the casbah of Algiers to be its exclusive destination. This antic city, dating back to the 10th century, is a citadel overlooking the magnificent Algiers Bay and it has been classified by the UNESCO as world heritage.

We wanted this year destination to be diverse in content with both cultural and fitness activities, hence we organized a guided touristic tour where we hit a record of about 4 km walk (6,000 paces and more than 400 stair steps!). We also discovered many museums and historical places, while enjoying the overwhelming view of the Algiers Bay.

The tour was so rich and exciting and kids and adults enjoyed being together in a family atmosphere illustrating the **hae day :-)** slogan: many faces, one family.

We would like to thank the OnVoyage tourist agency that sponsored the guided tour.



## PARAGUAY

We have registered the first HAE knowledgeable hospital in the country: Hospital Pediátrico "Niños de Acosta Ñu" in San Lorenzo. Also, there are two HAE knowledgeable physicians in Bolivia: Dr. Manuel Ratti Sisa and Dra. Susan Portillo, both located in San Lorenzo. Another piece of good news: Patients in Paraguay now have access to Firazyr. You will find contact information at <https://haei.org/hae-member-countries/paraguay>.



## ALBANIA

What was a patient group founded in 2017 is now an officially registered patient organization. More information on HAE Albania can be found at <https://haealbania.haei.org>.



## SWITZERLAND

*From HAE Switzerland*

Our patient meeting on 30 April 2022 was a complete success. We were able to welcome 28 members and 16 guests representing patients and caregivers, HAE doctors and representatives of pharmaceutical and health care companies. The program included very interesting presentations on the medicines available in Switzerland.



In celebration of **hae day :-)** 2022 we surprised our members and guests with a present in the form of a key fob with a small flashlight and a shopping cart chip.

On 14 May 2022 the Schweizer Radio und Fernsehen (SRF) television program 'Gesundheit Heute' (Health Today), Dr. Jeanne Fürst discussed HAE. Patients showed how they cope with their everyday life

with HAE and together with the HAE expert, PD Dr. Urs Steiner from the University Hospital Zurich, they talked about their experiences, how their lives have changed with HAE and what has helped them.

Furthermore, in its 19 May 2022 edition the magazine "Schweizer Familie" published an interview with Dr. Jeanne Fürst and PD Dr. Urs Steiner.





## ITALY

From A.A.E.E. (HAE Italy)

**Joint General Assembly and Annual Meeting:** On 14 May 2022, HAE Italy (A.A.E.E.) and ITACA (Italian Network for Hereditary and Acquired Angioedema) held a General Assembly and Annual Meeting at the NH Hotel in Padua. For the first time, doctors and patients had a joint assembly that permitted them to better exchange information, news, and requests, all this possible thanks to the strong interaction between the two parties.



For A.A.E.E. members, the event was an excellent opportunity to celebrate the 40th anniversary of the Association. Actually, it was to be celebrated in 2020 but had to be postponed because of the pandemic. More than 150 people were present, and another 20 were online, which was a great success. We must thank HAEi Regional Patient Advocate Maria Ferron, who joined our meeting and presented the activities of HAEi – and we congratulate her for her good Italian.

The first part of the Assembly was an introduction by A.A.E.E. President Pietro Mantovano and ITACA

President Mauro Cancian. This was followed by doctors' reports from Andrea Zanichelli (Angioedema National Registry), Francesco Arcoleo (Available therapies and ongoing studies), Massimo Triggiani (Quality of Life), and Mauro Cancian (Angioedema in Italy and in the world).



The question time that followed the doctors' reports resulted in a widespread exchange of experiences between the attending patients, members of the Association, and doctors. This section was very well appreciated by patients for the good relationship with the scientific panel. By sharing doubts and questions, patients, regional patient advocates, and doctors could open new doors for improvement and shared solutions.



One of the topics of greatest interest was the National Patient Registry. Already developed a few years ago by Prof. Cicardi and currently under reconstruction and updating by ITACA, it is based on a new data culture to provide higher data quality and easier user access.

The second part of the Assembly was run by Pietro Mantovano, who presented the Association with an overview since its foundation in 1980 and how it improved over the years thanks to our unforgettable Prof. Marco Cicardi, without whom nothing would have been done. The A.A.E.E. President presented the different activities carried out and supported by the Association, such as support for ITACA.

The associative budget with the related expense and entrance notes were unanimously approved. For 2022-23, a scholarship in honor of Prof. Cicardi has been announced to be allocated to a professional/specialist dealing with angioedema in Italy.

The secretaries Carlotta Cicardi and Martina Perera contributed with realized activities supported by the Committee Board:

- Activation of the Telegram Channel
- Survey on Reference Centers
- The Christmas gift from the A.A.E.E., the novel "Dreaming Toronto", a love story written by a patient of ours whose protagonist is a girl suffering from HAE (photo)
- Personalized gadgets
- Participation in the second ITACA National Congress 16-18 December 2021 in Rome
- Participation in the UNIAMO project for the Rare Disease Day
- The UNIAMO Well-being project promoted by CSL Behring – a service of online fitness for people with rare diseases and their families



A Survey on the Reference Centres was sent to all 2021 and 2022 members. It consists of eight questions about the centers' degree of satisfaction/dissatisfaction. The responses were analyzed and collected in a single document that was then divided for each center and shared with the doctors of ITACA. The results have had substantial importance in improving the reference centers' individual services.

According to data, we have evidence that 24% of the patients have registered with the Association for the year 2022-23.

Following the answers to the survey's open question, a telematics psychological counseling service so whoever wishes can get in touch with Dr. Licata.

Maria Ferron and Marco Castiglioni Roffia, who is responsible for A.A.E.E.'s foreign relations, concluded the meeting by presenting the activities we have joined and the importance of being connected to HAEi.

**Awareness campaign:** For **hae day** :-) 2022 ITACA and A.A.E.E. organized an information campaign. Among other things this included that hospital departments such as specialized centers for HAE treatment all over Italy opened their doors for visits and laboratory analyzes.

**ACARE Center:** Italy has its first ACARE center. That is Allergologia e Immunologia Clinica nell'Università di Cagliari in Cagliari lead by Prof. Stefano Del Giacco. Please see <https://haei.org/hae-member-countries/italy> for contact information.

**Global activities:** The board of the Association has been invited to participate in the HAEi activities and the youngsters' program at the HAEi Global Leadership Workshop in Frankfurt, Germany, in October 2022.

## HUNGARY

Prof. Henriette Farkas, who is Chairperson of the Workshop, informs us that the 13th C1-inhibitor Deficiency & Angioedema Workshop will be held in Budapest, Hungary with the conventional in-person participation on 4-7 May 2023.





## CROATIA

From President Mihaela Šogorić, HAE Croatia

HAE Croatia successfully organized the second assembly and annual meeting with doctors on 14-15 May 2022. The meeting began with the submission of the financial report on the work of the Association in 2021. Agenda, plan, and program for 2022 have been successfully adopted.

At the end of assembly, seven doctors presented lectures on these topics:

- Prof. Primarius Asja Stipić Marković, MD, PhD: "How inheritance from parents causes swelling of the head and body"
- Primarius Ljerka Karadža Lapić, MD, PhD: "HAE – gender differences" and "The importance of treating acute HAE attacks"
- Daniel Šimac, MD: "HAE during pregnancy"
- Ana Marija Masle, MD: "Acquired deficiency of C1 inhibitors"
- Primarius Marko Barešić, MD, PhD: "Short-term prophylaxis of HAE"
- Boris Karanović, MD: "Experiences of the Clinical Hospital Center Zagreb in the treatment of acute attacks"
- Assist. prof. Draško Cikojević, MD, PhD: "Long-term prophylaxis of HAE"

In anticipation of **hae day :-)** 2022, our member Ante Prgin with his band El Gato organized a concert in the Šibenik Azimut during which members of HAE Croatia distributed information leaflets to raise awareness.

15 May 2022, the meeting continued with a lecture from Assist. prof. Dijana Perković, MD on "HAE from a psychological point of view" which was an introduction to a psychological workshop held by Univ. spec. clin. psych. Antica Petričić.

We were greeted by our guests Bojana Olujić, MD (Clinical Hospital Center Osijek), Nevenka Cigrovski, MD (Clinical Hospital Center Rebro), Dušanka Marković, MD (from Serbia) and Mihaela Zidarn, MD (from Slovenia). Dr. Zidarn invited us to a meeting of their patients next year to convey to them the importance of founding an association and a sense of belonging when you are among people like yourself.

At the end of the meeting and through lectures, patients gained better insight into the difference between types



of HAE and into the impact of HAE from a psychological point of view as well as the course and importance of HAE treatment protocols. What we would especially like to emphasize is the communication between the doctors and the patients and the interconnections between the patients created during and at the end of the meeting.

Everything that happened at the meeting was covered in the daily edition of Glas Slavonija and a show about the whole event was also made on Radio Đakovo.

There has been a change in the primary HAE Croatia contacts as Davorka Grbić is Vice President and Sasha Pavlić is Secretary while I am President. Our contact information is at <https://haei.org/hae-member-countries/croatia>. You might also want to have a look at the HAE Croatia Facebook page: [www.facebook.com/groups/498354757023217](https://www.facebook.com/groups/498354757023217).



## AUSTRALIA & NEW ZEALAND

From CEO Fiona Wardman, HAE Australasia

HAE Australasia has added three new 'Living Well with HAE' videos to the website library. The latest videos cover essential topics such as 'HAE Overview & Treatment' with Prof. Connie Katelaris, 'HAE & Gynaecology' with Dr. Andrew Pesce, and 'The Role of Genetic Counselling' with Rebecca McIntosh. HAE Australasia has complemented the videos with downloadable and printable documents for each video with time references to the content. View the videos at <https://haeausralasia.org.au/resources/video-resources>.



Embargoed until 12.01am Monday May 16, 2022

**Access to affordable treatment critical for rare swelling disease.**

**HAE Day, May 16, 2022, for Hereditary Angioedema Awareness**

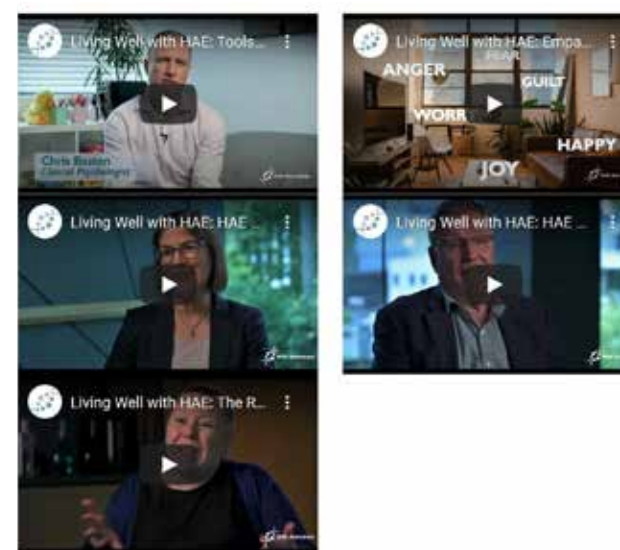
"There is a huge difference between just surviving and properly living – and access to critical treatment is the difference between the two," says Jess Bogoyevitch, a 22-year-old with hereditary angioedema (HAE), a very rare and potentially life-threatening genetic condition that leads to painful swelling in various body parts. Airway swelling is particularly dangerous and can lead to death by asphyxiation.

HAE Australasia has plans for new and exciting resources for youth and young adults, podcasts, a lifestyle and well-being pilot project and more!

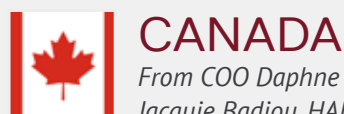
On **hae day :-)** 2022, the Australasian Society of Clinical Immunology & Allergy (ASCI) and HAE Australasia launched a media campaign to raise awareness of HAE and the importance of access to modern therapies. The media reach was through radio, print media, and online. Thank you to all the patients who took part and shared their stories.



Signifying swelling often brought Jess to tears, but for a time this was not enough to resolve the medical treatment she needed. Credit: Supplied







## CANADA

From COO Daphne Dumbrille and President Jacquie Badiou, HAE Canada:

Exciting news: Canadian HAE patients are one step closer to gaining access to the first oral HAE treatment. Health Canada has recently approved BioCryst's treatment, ORLADEYO® (berotralstat), for the routine prevention of HAE attacks in patients 12 years and older. HAE Canada will continue to help bring this oral treatment to Canada and we are looking forward to hearing the results from the patient and clinician submissions that we provided to the Canadian Agency for Drugs and Technologies in Health (CADTH).

This spring HAE Canada worked with Takeda Canada on multiple projects that amplified the patient voice to different audiences:

- In February, Jacquie Badiou was part of a panel at a Rare Disease Day event. The event gave Takeda employees a better perspective and appreciation for how rare diseases impact a person's life.
- In March, Board member Kim Speiss, along with a volunteer, participated in a panel discussion for healthcare providers in Saskatchewan. They each shared their patient journeys and advised on unmet needs for HAE patients in Saskatchewan.
- In April, alongside Drs. Marcus Maurer and Stephen Betschel, Jacquie Badiou participated in the HAE Expert Panel Discussion to healthcare providers. The event aimed to educate healthcare providers on treatment options while providing a patient's perspective.
- A recording was shown at the HAE Expert event of HAE Canada member, Anne, sharing her patient story. She described her childhood before she was diagnosed, her journey to receive a proper diagnosis and she ended by discussing how she lives her life to the fullest as a patient receiving proper treatment today. We want to extend our sincere gratitude to Anne for sharing her inspiring story.

HAE Canada was asked by HAEi to recruit members to complete CSL Behring's Patient Reported Outcome (PRO) Online Survey. Recognizing the importance of gathering real world evidence through surveys, HAE Canada was happy to help. We are grateful to our members who completed this survey and provided the Canadian patient's experience with long term prophylactic therapy.

We are both excited and proud to report that HAE Canada is presenting a poster (virtually) at the

European Academy of Allergy & Clinical Immunology (EAACI)'s Hybrid Congress in Prague titled "Type I/II HAE: Treatment and attack frequency improvements between 2017 and 2020 based on data from the Canadian national patient surveys". A huge thank you goes to Dr. Suzanne Kelly from Red Maple Trials who always does a fantastic job analyzing data and creating our abstracts. Thanks also to our Advocacy Committee and the HAE specialists who reviewed both our documents. We will be sure to post the abstract and poster on our website after it is presented at the conference in July. Our third abstract of 2022 was recently submitted for consideration at the next Annual Scientific Meeting for the Canadian Society of Allergy and Clinical Immunology (CSACI).



We recognize the importance of having a Canadian representative on the HAEi Youngsters Advocacy Group and we were thrilled when asked to be interviewed to express our reasons why. Over Zoom, the Canadian representative, Jacob Collins, expertly asked Jacquie Badiou and Daphne Dumbrille why it is important to have a Canadian part of HAEi's Youngsters community and why the youth voice is necessary to advocacy, and specifically to HAE Canada. Jacob did a fantastic job, and we are very proud to be part of this initiative. The interview will be part of their blog series and the recording is posted in our members-only HAE Canada Café portal.



The Canadian Organization for Rare Disorders (CORD) held their first in-person conference since March 2020! Daphne Dumbrille and HAEi Treasurer, Carmen Craciun, attended the conference titled "Building Canada's Smart Rare Disease and Rare Drug System 2022". The goal of the conference was to bring together

key stakeholders to discuss the importance of a holistic and comprehensive Rare Disease & Rare Drug System in Canada. The jam-packed agenda covered topics ranging from the importance of genetic testing to why it is essential that Canada's system considers a patient's full needs and not just their access to treatment. CORD invited a wide variety of panelists to discuss these topics, such as representatives from government and industry. Physicians and patients presented on their experiences of working and living with rare diseases. Daphne participated on a panel discussion titled "Patient Engagement and Patient Empowerment".

The end of COVID restrictions meant Jacquie Badiou was able to travel to Ottawa to meet with our sponsors who live in the city. During her visit, she and Daphne Dumbrille had productive meetings with representatives from BioCryst, CSL Behring Canada and Takeda Canada. HAE Canada is fortunate to have support from amazing companies.

Once again, HAEi hit it out of the park with their **hae day :-)** Global Walk initiative. We loved watching the numbers climb as participants from across the world contributed their steps. Canada respectfully finished fifth for the number of steps, but we ranked first in the number of participants, clocking in at 911 participants! Thank you to all Canadians, including our members, their friends and family, HAE physicians and their staff and the pharmaceutical companies in Canada who took the time to Step Up for the Global HAE Movement. Congratulations to the United States for their first-place finish.

It was a great spring, and we are looking forward to an even better summer!



## SOUTH KOREA

There are changes to HAE South Korea as Soo Jin Min is President while Raina Seonmi So servers as the daily contact. You'll find their contact information at <https://haei.org/hae-member-countries/south-korea>.



## DENMARK

During the last few months HAE Scandinavia in Denmark has launched a podcast series on HAE. If you are familiar with the Danish language you might enjoy listening to one or more of the podcasts at <https://haescan.org/ressourcer/podcast>.



## UNITED STATES

From Ianice Viel, HAE Advocate & Digital & Social Media Manager, US HAEA



**Guide for Women with HAE:** We have exciting news for women and young girls with HAE! Women and girls have extra concerns to acknowledge understanding their HAE diagnosis. While keeping this in mind, the US HAEA has developed a comprehensive guide for women and young girls with HAE to answer questions they might have about how puberty, pregnancy and family planning, or menopause may affect their bodies with HAE.

The US HAEA has also developed a three-part webinar series alongside expert HAE physicians who answer frequently asked questions about how women and young girls experience HAE through different life stages and provide tips on the management of the condition. These events are a great resource for women and young girls of all ages who are interested in learning more about how HAE impacts their lives. The webinars will be recorded and available on the HAE Café after each events' premiere.

*"This resource has allowed me to become more comfortable with how my HAE will affect me in the future. It not only answers questions that I have, but also provides stories from other women who have gone through similar situations."*

- Hannah (Person with HAE)



The Guide is available in English and Spanish. Read more about the Guide for Women with HAE and the webinars at [www.haea.org](http://www.haea.org).



**Advances in HAE Research:** The US HAEA continues its robust research program and is embarking on timely and relevant projects that will focus on the unique needs of the HAE community. Our ongoing projects include:

- **An HAE-Specific Instrument to Measure Quality of Life.** The US HAEA is working on a unique, ground-breaking research study that seeks to accurately show how HAE affects overall quality of life (QoL). The aim of the study is to develop and validate a broad-based HAE QoL instrument that more accurately depicts disease burden. This research will help us publish a QoL questionnaire that (1) truly captures the way HAE affects the everyday life of individuals and families, and (2) demonstrates to health insurers – and others – the value of life-changing improvements in health and QoL that result from modern HAE medicines. Based on concepts derived from behavioral economics, this tool will be more sensitive than any that are currently being utilized. We believe that this cutting-edge project could revolutionize QoL measurement of HAE and other chronic conditions.
- **Shared Decision-Making Tool.** We are currently developing a Shared Decision-Making tool that will enable physicians and people with HAE to work together when making healthcare decisions. This tool works to ensure that both the physician and the person with HAE have a voice in their treatment plan.
- **HAE and Aging Study.** The US HAEA study on HAE and Aging will help us better understand the demographic and clinical characteristics, HAE treatment patterns, HAE impact and burden, and perceived health-related quality of life of the aging HAE population. This study is being designed with a comparator population of people without HAE, and we plan to

have these results published in a manuscript when complete in the Fall of 2022. This research project is uniquely important to the HAEA community, as current data on HAE and Aging does not exist.

- **HAEA Study on the Number of People with HAE in the United States.** The US HAEA is dedicated to offering its unique programs, services, and activities to everyone in the United States that has HAE. We believe that past estimates underestimate the true prevalence of HAE. Estimates regarding the number of people with the condition in the United States have an extremely large range, which means we have no idea if we are reaching the entire population. We have initiated a study using sophisticated data mining techniques and expert HAE physician input to calculate the number of people with HAE in the United States. This survey involves a three-step process, first by completing a first initial survey in June, and completing two shorter follow-up surveys in July and September. The results of this study will help the US HAEA to target its outreach efforts to continue building the HAEA community.



**Clinical Trials:** Over the past two decades, the US HAEA has played a central role in supporting new drug development by recruiting patients for clinical trials by leveraging our unique reach into the HAE community. We handle all patient contact and referrals to clinical trial sites while never disclosing any personally identifiable patient information to the sponsor, and participate in regular update calls with the sponsor, sharing new information and recruitment status updates.

The US HAEA assists in clinical trial recruitment for individuals with HAE Type I, Type II, or HAE with Normal C1-Inhibitor and is currently assisting with recruitment efforts for an unprecedented eight HAE clinical trials. Clinical trial participation is crucial in medication development, as well as a critical part of expanding our knowledge of this rare disease.

*"I joined a clinical trial in an effort to assure my daughter would not have the same experience I had growing up with HAE."*

- Clinical Trial Participant

The HAEA has a proven and successful track record in clinical trial recruitment and continues to recruit for open trials whenever possible.

To view a list of active clinical trials please visit [https://www.haea.org/pages/p/clinical\\_trials](https://www.haea.org/pages/p/clinical_trials).



**HAEA Round Table:** Because HAE is so rare, it can feel isolating to deal with the everyday challenges of learning how to manage living with this rare chronic illness. However, when we come together as a community to talk about our struggles, it is easier to see that we are not alone in our efforts to live a normal life with HAE.

We also realize that at different life stages, HAE can affect individuals in various ways. That is why we have developed the HAE Round Table as a platform for members of our community to connect with their peers about the issues that currently affect them.

Through the HAEA Round Table we will discuss questions that affect each age group, such as:

- How do you explain your HAE diagnosis with others?
- How do you overcome a fear of needles?
- How did you go about obtaining insurance with your HAE diagnosis?

The HAEA Round Table will premiere every other month on the US HAEA official Facebook page Live. You can watch the next Round Table premiere for Young Adults with HAE on 9 August 2022 at 6:30 PM ET. Previous Round Table recordings can be accessed at <https://vimeo.com/user/115781635/folder/8930101>.



**US HAEA Podcasts:** The US HAEA is continuing to present community stories on both of our podcast platforms. These podcasts include:

HAE Speaks Podcast features adults with HAE or their caregivers who speak about various topics that affect the everyday lives of our community members. These topics include learning to come to terms with your diagnosis, mastering travel with HAE, how HAE can affect pregnancy, and gaining a sense of purpose through advocating for HAE. There are currently twenty-six informative episodes of the HAE Speaks podcast.

#BeyondHAE Podcast presents stories from the youngest members of our community. Young people with HAE can face a series of unique challenges when it comes to their diagnosis, so the HAEA has created the #BeyondHAE podcast to unite the youth community through shared experiences. Featured topics include understanding anxiety and how it relates to HAE, facing fear of needles, speaking to significant others or classmates about HAE, and creating a strong support system. There are now thirty-nine illuminating episodes of the #BeyondHAE podcast.

These podcasts are available on Spotify and Apple Podcasts!

**ACARE:** The second ACARE center has been accredited in the United States. The new center is the Division of Rheumatology, Allergy and Immunology at the University of Cincinnati College of Medicine, Cincinnati (Ohio), headed by Prof. Jonathan Bernstein. You will find contact information at <https://haei.org/hae-member-countries/united-states-of-america-2>.



# MEDICAL PAPERS

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

**Investigation of Mortality of Hereditary Angioedema in a Reference Center in Brazil** – by Fernanda Gontijo Minafra, Universidade Federal de Minas Gerais, Brazil, et al.:

HAE remains a threat to life in the studied population (a cohort of 433 patients from 46 families was evaluated). The large number of patients who do not receive a diagnosis makes the situation even more severe and is responsible for most deaths.

J Allergy Clin Immunol Pract, May 2022

**Prophylactic therapy with subcutaneous C1-inhibitor is associated with sustained symptom control in patients with HAE** – by Timothy Craig, Pennsylvania State University, the United States, et al.:

Prophylaxis with C1-INH (subcutaneous) provided sustained reductions in attack frequency and decreased rescue medication use, with a substantial proportion of patients being attack free.

Allergy Asthma Proc, May 2022

**Pregnancy in Patients with HAE and Normal C1 Inhibitor** – by Natalia Gabriel, Centro Universitario FMABC, Brazil, et al.:

The occurrence of abortion in HAE-nC1-INH was similar to the expected for not affected women. The 1st trimester of the pregnancy was more symptomatic for HAE-nC1-INH women. Considering the strong relevance of estrogens in HAE-nC1-INH, pregnancy could worsen the course of disease.

Front Allergy, February 2022

**Why does it take so long for rare disease patients to get an accurate diagnosis?** – A qualitative investigation of patient experiences of HAE – by Moeko Isono, Osaka University, Japan, et al.:

One of the most important factors related to the prolonged undiagnosed period is the lack of suspicion of a rare disease by patients and their medical professionals. While current policies tend to focus on the period from suspecting rare diseases to the time of a clear diagnosis, our results strongly suggest that measures are needed to facilitate patients and clinicians to become aware of rare diseases.

PLoS One, March 2022

**Inhibition of Prekallikrein for HAE** – by Lauré M Fijen, University of Amsterdam, the Netherlands, et al.:

Among patients with hereditary angioedema, donidalorsen treatment resulted in a significantly lower rate of angioedema attacks than placebo in this small, phase 2 trial (total of 20 patients).

N Engl J Med, March 2022

**Optimization of care for patients with HAE living in rural areas** – by Marc A. Riedl, University of California San Diego, the United States, et al.:

Challenges in managing HAE in the rural setting include obtaining a diagnosis of HAE, easy access to a physician with expertise in HAE, continuity of care, availability of telemedicine services, access to approved HAE therapies, patient education, and economic barriers to treatment. Ways to improve HAE patient care in rural areas include health care provider recognition of the patient with undiagnosed HAE, development of individualized management plans, expansion of telemedicine, effective care at the local level, appropriate access to HAE medication, and increased awareness of patient support and advocacy groups.

Ann Allergy Asthma Immunol, May 2022

**Long-term prophylaxis with lanadelumab for HAE: authorization for temporary use in France** – by Olivier Fain, Sorbonne Université, France, et al.:

Lanadelumab reduced attack rates, improved quality of life, and was generally well tolerated.

Allergy Asthma Clin Immunol, April 2022

**Gut microbiome alterations in HAE** – by Yang Cao, Peking Union Medical College Hospital, China, et al.:

Microbial richness and diversity were significantly reduced among patients who had recent HAE attacks, especially for those presenting with abdominal symptoms. Alterations of the gut microbiome in patients with HAE may provide new clues for the prediction of disease course, clinical treatment, and therapeutic evaluation.

Ann Allergy Asthma Immunol, April 2022

**Inheritance Pattern of HAE Indicates Mutation-Dependent Selective Effects During Early Embryonic Development** – by Konrad Bork, Johannes Gutenberg University, Germany, et al.:

There is a sex- and mutation-dependent selection during early embryogenesis, possible around the time of implantation, favoring male wild-type and female mutant embryos. It also appears that 20% to 25% of male embryos carrying the HAE mutation are lost specific in HAE with normal C1-INH. There is a potentially important role of the kallikrein-kinin system during early embryonic development.

J Allergy Clin Immunol Pract, April 2022



## FOLLOW HAEI ON SOCIAL MEDIA

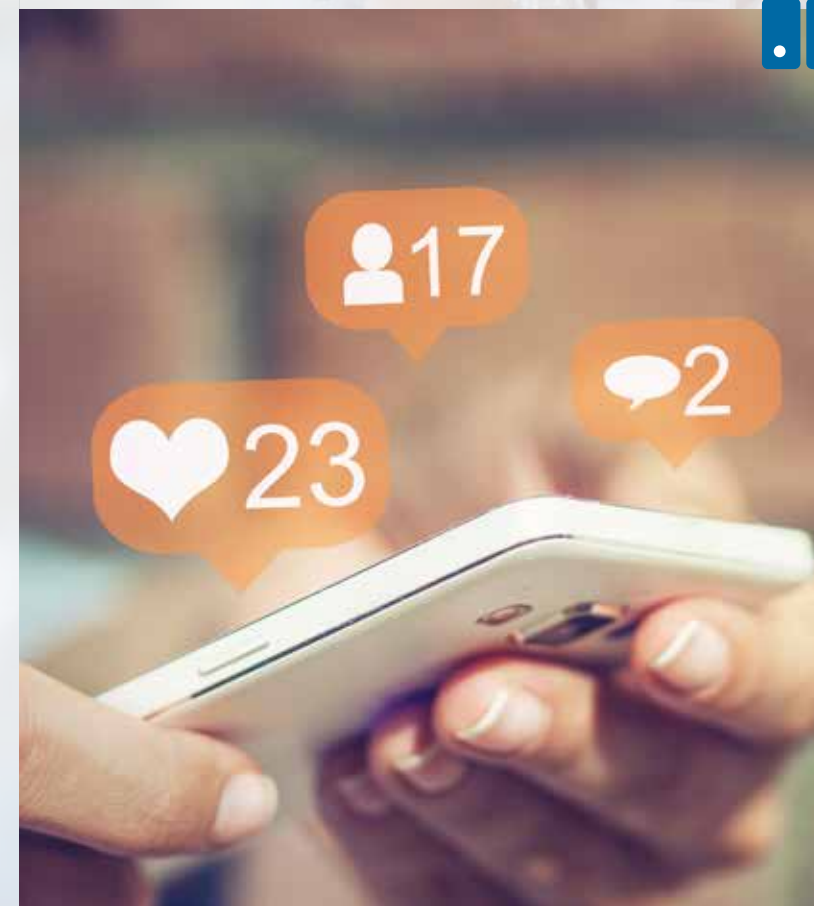
Are you aware that we post HAE-related news on our social media platforms almost daily?

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

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

See more and find links on our website

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





# NEWS FROM THE INDUSTRY

## 29 March 2022

**Takeda** has received approval from the Ministry of Health, Labour and Welfare (MHLW) for TAKHZYRO® (lanadelumab) subcutaneous injection 300mg syringes for prophylaxis against acute attacks of HAE in adult and pediatric patients 12 years of age and older in Japan.

This approval is primarily based on results of the global Phase 3 HELP (Hereditary Angioedema Long-term Prophylaxis) Study™ and the Phase 3 HELP Study Open-label Extension (OLE), in addition to results of a Phase 3 study evaluating the efficacy and safety of TAKHZYRO in Japanese patients. Combined, these studies have demonstrated the efficacy and safety profile of TAKHZYRO as a preventive treatment for HAE attacks.

“In addition to the burden of debilitating and potentially life-threatening HAE attacks, the unpredictable nature of this disease presents significant challenges to patients and their support networks,” says Naoyoshi Hirota, General Manager, Takeda Development Center, Japan. “We hope TAKHZYRO, a new treatment option for patients in Japan living with HAE, along with the efficacy and safety profile as a preventive treatment showcased across global studies and within a Japan-specific Phase 3 study, will contribute to HAE treatment.”

TAKHZYRO received its first approval for the prevention of HAE attacks in patients 12 years and older in 2018 in the United States and in the European Union and is now approved in more than 50 countries. TAKHZYRO is intended for self-administration or administration by a caregiver once trained by a healthcare professional. TAKHZYRO is supported by a robust clinical development program, which includes one of the largest prevention studies in HAE with the longest active treatment duration with additional regulatory submissions ongoing worldwide.

(Source: Takeda)



## 30 March 2022

Early in the pandemic, HAE patient Scott McCoy described his encounter with COVID-19 in a webinar for US HAEA. It was a bad news-good news story. The bad news was that McCoy had become infected with the novel coronavirus prior to vaccines being available. But the good news was that his infection did not cause an attack of HAE-related swelling, a symptom of the rare, genetic condition that can be life-threatening. In McCoy's words, “HAE didn't rear its ugly head.”

But new research suggests that McCoy might represent only half of the story. A study of 677 HAE patients in Italy found that 52 contracted COVID-19 and, of those, about half reported an HAE attack while they were infected. The Italian Network for Hereditary and Acquired Angioedema (ITACA) reported the data after collecting it for a year.

“51.9% of SARS-CoV-2 positive cases presented, during infection, at least one attack of angioedema, the severity of which correlated with that of COVID-19,” says Professor Mauro Cancian, President of ITACA on an Italian news hub about rare diseases, Osservatorio Malattie Rare. “Although the virus does not seem to affect patients with HAE more, it certainly has favored a greater onset and severity of acute episodes.”

Researchers were eager to investigate how COVID-19 affected HAE patients because the ACE-2 receptor, a gate of entry of the virus into human cells, is involved in the metabolism of bradykinin, the molecule that causes angioedema, according to the report in Osservatorio Malattie Rare.

The study from ITACA also found an increased prevalence of cardiovascular disease and diabetes among HAE patients. The results will be shared at an upcoming European Academy of Allergy, and Clinical Immunology Congress.

(Source: CSL Behring)

**CSL Behring**

## 30 March 2022

At the American Academy of Allergy, Asthma and Immunology (AAAAI) 78th Annual Meeting **Takeda** presents four abstracts including interim real-world data from the observational Phase 4 EMPOWER study of TAKHZYRO® (lanadelumab) as a treatment for people with HAE Type I or II in North America, as well as findings from a post-hoc analysis of the HELP Open Label Extension study of long-term safety and efficacy of TAKHZYRO in HAE patients 12 years of age and older.

“A big challenge for HAE patients is the unpredictability of attacks and the impact that the attacks have on quality of life. We are encouraged by the initial results of the EMPOWER study, along with the additional evidence presented in the HELP Open Label Extension,” says Associate Professor Paula Busse, MD, Division of Allergy and Clinical Immunology, Icahn School of Medicine at Mount Sinai in New York, USA. “Data from both studies show that lanadelumab had marked reduction in attack rates, and that angioedema control was sustained for 12 months.”

### Key findings from the data presentations include:

- Improvements among new users and sustained outcomes in established users were reported in scores of the Angioedema Quality of Life Questionnaire (AE-QOL), Angioedema Control Test (AECT), and the Treatment Satisfaction Questionnaire for Medication (TSQM-9) when collected every three months, as presented in the EMPOWER IA2 Patient-Reported Outcomes interim data presentation.
- An average of 1 in 5 established TAKHZYRO users were able to extend treatment from every 2 weeks to 4 weeks as shown in interim data shared in the EMPOWER IA2 Treatment Patterns and Subgroups presentation.
- Interim real-world data showed marked attack rate reduction of 83% and no new safety signals based on patient self-reporting reduced attack rates in the EMPOWER IA2 Effectiveness and Safety presentation.
- A post-hoc analysis of HELP and HELP OLE showed that reduction of attack rates with TAKHZYRO were similar for patients previously on androgen treatments as they were for the wider treatment population in these studies in the Switch from Androgens to TAKHZYRO in HELP 03 and HELP 04.

“We are pleased to see the real-world data from EMPOWER show improvements in angioedema control and treatment satisfaction. These interim results provide a better understanding of the overall patient experience with TAKHZYRO,” says Neil Inhaber, MD, Head, Rare Genetics and Hematology, Global Medical Affairs, Takeda. “With more than a decade of experience and innovation for patients with this devastating condition, Takeda remains committed to continuing our unwavering support for the HAE community.”

The observational Phase 4 EMPOWER study, evaluating real-world HAE attack rates before and after treatment with TAKHZYRO in patients with HAE types I and II, is ongoing. Full results of the EMPOWER study are expected to be published in 2024. HELP OLE is a completed Phase 3 study of the safety and efficacy of TAKHZYRO in patients previously treated with androgens and other therapies for long-term prophylaxis prior to transitioning to TAKHZYRO.

(Source: Takeda)



## 30 March 2022

At the presentation of the company's financial results for the fourth quarter and year ended 31 December 2021, **Pharvaris** CEO Berndt Modig says: “The time since our initial public offering in February 2021 has been transformational for Pharvaris, enabling us to reach clinical development milestones. We look forward to our next milestones and will continue to execute our strategy efficiently in 2022 supporting our mission to provide individual choice for managing HAE through potent and convenient oral on-demand and prophylactic therapies.”

### Pipeline

**Top-line Phase 1 data demonstrate extended-release PHVS719 suitable for once-daily dosing.** The Phase 1 pharmacokinetics (PK) study of PHVS719 included 8 healthy volunteers dosed in an open-label randomized five-period crossover single-dose study to assess bioavailability of two different extended-release formulations with and without food, in comparison to a single dose of PHVS416 without food. The pharmacokinetics of a single dose of PHVS719 (40 mg) under fasted conditions yielded exposure above 13.8



ng/mL (the EC85 determined in a Phase 1 bradykinin challenge in healthy volunteers) by the two-hour timepoint and maintained this exposure for at least an additional 28 hours. The overall exposure was not affected by food. The 24-hour area-under-the-curve (AUC24h) exposure of PHA121 using PHVS719 (40 mg) is similar to that observed in Phase 1 studies with PHVS416 softgel capsules dosed 20 mg twice a day with food (one of the doses used in the CHAPTER-1 prophylactic proof-of-concept study). The study showed that PHVS416 and PHVS719 were well tolerated. During the study, there were no severe adverse events (SAEs) or severe treatment-emergent adverse events (TEAEs) reported.

“We are thrilled to have achieved with the PHVS719 extended-release formulation for the first time the possibility of a once-daily oral bradykinin receptor antagonist for prevention of HAE attacks,” says Jochen Knolle, CSO of Pharvaris. “The versatile properties of PHA121, in this case the ability to be absorbed through the colon, combined with the appropriate slow-release technologies, has enabled a release profile well-suited to all-day exposure of compound. This single-dose study showed that PHVS719 (40 mg) maintained PHA121 exposure for a full day above the levels that prevented the effects of a surge of bradykinin as shown in our mechanistic study in healthy volunteers. We look forward to confirming these results in an upcoming multi-dose PK study, to support use of PHVS719 in a future pivotal clinical study when combined with the anticipated results of our CHAPTER-1 proof-of-concept study.”

**Orphan Drug Designation granted by FDA.** On 18 March 2022, the FDA granted orphan drug designation to PHA121, the active ingredient in our PHVS416 and PHVS719 product candidates, for treatment of bradykinin-mediated angioedema.

**Phase 2 on-demand study (RAPiDe-1) of PHVS416 ongoing.** RAPiDe-1, a Phase 2 clinical study of PHVS416 for the on-demand treatment of HAE attacks, continues enrollment and attack surveillance across 33 clinical sites in Canada, Europe, Israel, the UK and the U.S. Top-line data from the study is anticipated to be available in the fourth quarter of 2022.

**Phase 2 prophylactic study (HAE CHAPTER-1) of PHVS416 enrollment ongoing.** CHAPTER-1, a Phase 2 clinical trial of PHVS416 for the prophylactic treatment of HAE attacks, is enrolling patients across clinical sites

in Canada, Europe, Israel, the UK and the U.S. Top-line data from the study is anticipated to be available in the fourth quarter of 2022.

**RAPiDe-2 expected to initiate in 2022.** RAPiDe-2, an open-label extension study evaluating PHVS416 for the on-demand treatment of people with HAE, is expected to initiate in the second half of 2022.

**Preclinical data of PHA121 published in International Immunopharmacology.** In March 2022, Pharvaris announced the publication of preclinical data in International Immunopharmacology demonstrating the specificity and potency of PHA121, the active ingredient in our PHVS416 and PHVS719 product candidates.

(Source: Pharvaris)

**PHARVARIS**

### 31 March 2022

At the presentation of the financial results for the quarter and year ended 31 December 2021,

**Orchard Therapeutics** CEO Bobby Gaspar, M.D., Ph.D., says that a promising early-stage research program that apply a hematopoietic stem cell (HSC) gene therapy approach in HAE will remain an important part of the portfolio going forward given the promise in larger indications and as a possible source of future partnerships.

In the HSC approach, a patient's own blood stem cells are genetically modified outside of the body and then reinserted, with the goal of correcting the underlying cause of disease in a single treatment.

“In light of our experiences and knowledge gained in this current and rapidly evolving market environment for gene therapy, our plan is to concentrate resources on programs that have the potential to make a remarkable difference to patients while also providing sustainable value to the business to enable the achievement of our long-term vision,” says Bobby Gaspar.

(Source: Orchard)

**Orchard**  
therapeutics

### 19 April 2022

The Phase 3 SHP643-301 study evaluating the safety profile and pharmacokinetics of TAKHZYRO® (lanadelumab) in patients 2 to <12 years of age is complete and has met its objectives. The safety profile was consistent with that seen in the clinical program for patients 12 years of age and older; there were no serious adverse events and no dropouts due to adverse events.

The study also successfully reached the secondary objective evaluating the clinical activity/outcome of TAKHZYRO in preventing HAE attacks as well as characterizing the pharmacodynamics of TAKHZYRO in pediatric subjects 2 to <12 years of age.

“We are encouraged by these results, as each objective met instills further confidence in the potential to bring a treatment option to this vulnerable population,” says Ashley Yegin, Global Medical Unit Head, HAE, Global Medical Affairs, **Takeda**. “With more than a decade of experience and innovation in HAE, Takeda is committed to continued support for patients of all ages with this devastating condition.”

“I’m pleased that TAKHZYRO has met these key objectives and I look forward to Takeda sharing further insights with the HAE community in the near future,” says Dr. Marcus Maurer, Professor of Dermatology and Allergy Charité – Universitätsmedizin Berlin, Germany and principal investigator of SHP643-301.

Participants aged 2 to < 6 years received lanadelumab at a dose of 150 milligrams (mg) every 4 weeks (q4wks) over 52-week treatment period. Participants aged 6 to <12 years received lanadelumab at a dose of 150 mg every 2 weeks (q2wks) over 52-week treatment period. This study is complete, and full results will be presented at upcoming medical meeting(s).

(Source: Takeda)



### 19 April 2022

TAKHZYRO® (lanadelumab-flyo) injection single-dose prefilled syringe is now available in the U.S. to prevent attacks of HAE in adult and pediatric patients 12 years of age and older. Approved by the U.S. Food and Drug Administration on 8 February 2022, the TAKHZYRO prefilled syringe is ready to use and requires fewer preparation steps than the single-dose vial, enhancing the treatment administration experience.

“By introducing TAKHZYRO prefilled syringe to the HAE community, our aim is to continue to enhance the patient experience. With this advancement, those taking TAKHZYRO can now live their life with a ready-to-use option that requires fewer steps and less waste than when using the single-dose vial to receive their injection,” says Cheryl Schwartz, Senior Vice-President, Rare Disease Business Unit at **Takeda Pharmaceutical Company Limited**. “Over the coming months, we will be working with patients and their specialty pharmacies as they are ready to make the transition.”

With a proven efficacy and safety profile and real-world experience, TAKHZYRO has been available in the U.S. since 2018 helping to prevent HAE attacks in patients 12 and older. TAKHZYRO is supported by a robust clinical development program, which includes one of the largest prevention studies in HAE with the longest active treatment duration, HELP (Hereditary Angioedema Long-term Prophylaxis) Study™. Results from the HELP Study Open-Label Extension (OLE) found consistent safety and efficacy results with TAKHZYRO in a treatment period of up to 132 weeks.

“HAE attacks are unpredictable, debilitating and in some cases life-threatening, adding uncertainty and complexity to the daily lives of those living with this disease and their families,” says Dr. William Lumry, Allergy Immunology Specialist, Clinical Professor of Internal Medicine, University of Texas Southwestern Medical School. “After many years in practice witnessing the ups and downs of this disease, I welcome any opportunity to simplify the process of administering an effective prevention therapy for people living with HAE.”

(Source: Takeda)





## 29 April 2022

Héma-Québec has awarded a two-year tender for TAKHZYRO® (lanadelumab injection) as the sole option for prophylaxis subcutaneous treatment for type I/II HAE patients. The two-year term which began 1 April 2022, includes the option for Héma-Québec to extend the tender for an additional two years.

“My team and I took part in the clinical trials that led to the approval of TAKHZYRO®, and we are pleased that the medicine is now available for the treatment of patients with HAE,” says Dr. Jacques Hebert, Head, Immunology and Allergy Clinic, CHUL. “The treatment philosophy has evolved over the years. About ten years ago, prophylaxis was used to reduce the morbidity and mortality related to this clinical condition. With modern medicine that is proven to be safe and effective, such as TAKHZYRO®, prophylaxis aims to help patients lead normal lives without recurrence or attacks.”

“Patients with HAE have long been limited to acute attack treatments. Having access to safe and effective preventive treatment is a major advancement for our patients,” says Dr. Rémi Gagnon, Head of the Department of Allergy and Immunology, CHU de Québec. “Many of our Quebec patients have played a pivotal role in this treatment’s development by participating in the research protocols of the Clinique spécialisée en allergie de la capitale. Making Lanadelumab available improves patient care by giving them a better quality of life.”

“Patients with HAE constantly live in fear of their next attack, often disrupting day to day activities such as work, family and social interactions, and significantly impacting their quality of life,” says Charles St. Pierre, President, Hereditary Angioedema Quebec/Angio-Oedeme Héritaire du Québec. “The HAE community is delighted that TAKHZYRO® is now available for patients living in Quebec because having access to a treatment option that can prevent attacks would be life-changing for those living with HAE.”

The awarding of the Héma-Québec tender is the most recent milestone for TAKHZYRO® since receiving Health Canada authorization for routine prevention of attacks of HAE in adolescents and adults (12 years of age and older) in September 2018. Since then, Health Canada has issued the Notice of Compliance for a Prefilled Syringe (300 mg/ 2 mL) presentation in September 2020, as well as receiving a positive recommendation from CADTH’s Canadian Drug Expert Committee for the routine prevention of HAE attacks in November 2019,

and from the Institut national d’excellence en santé et en services sociaux (INESSS) in August 2020.

“We’re extremely proud of this agreement with Héma-Québec and what it represents for HAE patients,” says Rute Fernandes, General Manager, **Takeda Canada Inc.** “This collaboration is an important step to ensure patients with type I and type II HAE have to access TAKHZYRO® and the improvement of care that is now available to them. This is an example of how our commitment to meeting the needs of patients living with a rare disease has resulted in greater access for patients across the country.”

(Source: Takeda)



## 6 May 2022

From the **BioCryst Pharmaceuticals, Inc.** financial results for the first quarter ended 31 March 2022:

“We are now over a year into the ORLADEYO launch and are excited to see strong and continuing patient demand and steady expansion in our prescriber base among both new and existing prescribers. These trends continued in the first quarter of 2022,” says Jon Stonehouse, President and CEO of BioCryst. “We also have made substantial progress in our investigation with BCX9930. Based on our initial findings, we believe that both dose and dosing regimen could be contributing factors to the safety signal we have observed. By the end of the third quarter, we plan to discuss our proposed approach to resume the REDEEM trials, under a revised dosing protocol, with regulators.”

ORLADEYO has been launched in Denmark, France, Germany, Japan, Norway, Sweden, the United Arab Emirates, and the United Kingdom. The company expects launches in additional countries throughout the year.

(Source: BioCryst)



## 9 May 2022

At the **Intellia Therapeutics, Inc.** operational highlights and financial results for the first quarter ended 31 March 2022, President and CEO John Leonard, M.D., says:

“We continue to advance our second in vivo candidate, NTLA-2002, which benefits from the modularity of our platform. We look forward to another important clinical milestone in the second half of this year when we expect to present initial data from the NTLA-2002 first-in-human study. Finally, we remain well-funded to drive forward our robust portfolio and to support continued investment in platform innovation as we build upon our leadership position in genome editing.”

(Source: Intellia)



## 11 May 2022

**Pharvaris** presents its financial results for the first quarter ended 31 March 2022 and updates recent business highlights.

“We met our operational goals for PHVS416 for the on-demand treatment of HAE attacks and have reached our target patient enrollment in the RAPIDe-1 Phase 2 study,” says Berndt Modig, CEO of Pharvaris. “We sincerely thank the HAE patients and HAE community, investigators, and site staff across the globe who are participating in this trial, as well as our outstanding team. Pharvaris is also enrolling patients in CHAPTER-1, a proof-of-concept Phase 2 clinical trial using PHVS416 for the prophylactic treatment of HAE attacks. We continue to collaborate with the HAE community to further understand how our clinical research can address remaining unmet needs in HAE.”

*Recent Business Highlights and Updates*

- Target enrollment achieved for Phase 2 on-demand study (RAPIDe-1) of PHVS416. RAPIDe-1, a Phase 2 clinical study of PHVS416 for the on-demand treatment of HAE attacks, has reached its target enrollment and continues to assess HAE attacks across 33 clinical sites in Canada, Europe, Israel, the UK and the U.S. Top-line data from the study is anticipated to be available in the fourth quarter of 2022.

- Enrollment ongoing in Phase 2 prophylactic study (HAE CHAPTER-1) of PHVS416. HAE CHAPTER-1, a proof-of-concept Phase 2 clinical trial using PHVS416 for the prophylactic treatment of HAE attacks, is enrolling across clinical sites in Canada, Europe, Israel, the UK and the U.S. Top-line data from the study are anticipated in the fourth quarter of 2022.
- RAPIDe-2 expected to initiate in 2022. RAPIDe-2, a long-term extension study evaluating PHVS416 for the on-demand treatment of people with HAE, is expected to initiate in the second half of 2022.

(Source: Pharvaris)



## 12 May 2022

**Astria Therapeutics, Inc.** reports financial results for the first quarter ended 31 March 2022 and provides corporate update.

“We are looking forward to advancing STAR-0215 into the clinic this year with the Phase 1a trial, which we plan to initiate shortly after our anticipated mid-year Investigational New Drug filing. We expect initial results by year end,” says Jill C. Milne, Ph.D., CEO of Astria. “Pharmacokinetic modeling suggests that STAR-0215 could be effective at inhibiting plasma kallikrein and has the potential to prevent HAE attacks with subcutaneous administration with a self-injectable device dosed once every three-months or longer. This model informs our plans for the Phase 1a trial, which aims to evaluate safety, tolerability, demonstrate inhibition of plasma kallikrein activity, and establish the prolonged half-life of STAR-0215.”

### STAR-0215 for the Treatment of HAE

- Lead program STAR-0215 is a monoclonal antibody inhibitor of plasma kallikrein designed to provide long-acting, effective attack prevention for HAE with dosing once every three months or longer. The goal for STAR-0215 is to provide the most patient-friendly preventative treatment option for people living with HAE.
- Targeted plasma kallikrein inhibition can prevent HAE attacks by suppressing the pathway that generates bradykinin and causes excessive swelling.
- Astria expects to file an Investigational New Drug application for STAR-0215 in the middle of this



year and plans to initiate a Phase 1a clinical trial shortly thereafter with initial results anticipated by year-end. The Phase 1a clinical trial is planned to be conducted at a single center with healthy volunteers and evaluate several single ascending dose cohorts with subcutaneous administration. The goals of this initial proof of concept trial are to demonstrate safety and tolerability, establish prolonged half-life of STAR-0215, and to demonstrate inhibition of plasma kallikrein activity. We plan to initiate a multi-center, global Phase 1b/2 trial in patients with HAE in 2023.

- Astria presented preclinical data demonstrating how STAR-0215 binds to plasma kallikrein at the American Academy of Allergy, Asthma, and Immunology Annual Scientific Meeting in February.
- At the Fc Receptor and IgG Targeted Therapies Conference in April, Astria presented pharmacokinetic modeling data supporting that STAR-0215 can effectively inhibit plasma kallikrein and prevent HAE attacks with subcutaneous dosing volumes appropriate for a self-injectable device dosed once every three months or longer.

(Source: Astria)



## 16 May 2022

**KalVista Pharmaceuticals, Inc.**'s proposed name "sebetralstat" has been approved by the World Health Organization's International Nonproprietary Names (WHO-INN) Expert Committee and the American Medical Association's United States Adopted Names (AMA-USAN) Council for the drug candidate formerly known as KVD900. Sebetralstat is currently being evaluated in the Phase 3 KONFIDENT study as a potential therapeutic for on-demand treatment of HAE attacks.

"We are excited to announce this next important step in the evolution of sebetralstat as the first potential oral on-demand therapeutic for HAE," says Andrew Crockett, CEO of KalVista. "The KONFIDENT study continues to enroll on track with our expectations, and we have many other activities ongoing to prepare for the eventual NDA submission. We believe sebetralstat can be a transformational therapy for people living with HAE."

(Source: KalVista)



## 16 May 2022

**Pharvaris** announces the company-wide support of **hae day :-)** 2022. Pharvaris has supported the HAE community by partaking in the **hae day :-)** Activities Challenge for the 30 days leading up to awareness day. Pharvaris colleagues from across the globe participated in both individual and team activities to promote overall wellbeing and submitted more than 2,000,000 steps to help raise awareness of HAE.

"**hae day :-)** and the Activities Challenge contribute to HAEi's objective to unify individuals across the globe raising awareness of continuing medical need and innovative solutions to improve the lives of people with HAE. Our participation in these initiatives reflects our mission to improve outcomes for individuals living with HAE," says Berndt Modig, CEO of Pharvaris. "We are proud advocates of the HAE community and recognize the opportunity we have to influence the lives of those affected by HAE. At Pharvaris, we are focused on advancing our clinical pipeline of oral bradykinin B2-receptor antagonists to bring novel oral therapies to people living with HAE."

(Source: Pharvaris)



## 6 June 2022

Health Canada approves oral, once-daily ORLADEYO® (berotralstat) from **BioCryst Pharmaceuticals, Inc.** for the routine prevention of recurrent HAE attacks in patients 12 years and older.

"Today is a momentous day for the Canadian HAE community," says Jacquie Badiou, President of HAE Canada. "HAE carries with it a severe burden on patients and their families, much of which stems from the unpredictable attacks they experience even when actively managing their disease. We have seen important treatment advancements in recent years, and I believe the first oral preventive therapy for HAE will be a welcome option for many Canadians who are living with this rare condition."

"I believe this oral, once-daily prophylactic therapy will be a meaningful treatment option for patients who need a prophylactic therapy to help manage their HAE by reducing the frequency of attacks," says Stephen Betschel, HBSc, MD, FRCPC, FAAAAI, Chair of the Canadian Hereditary Angioedema Network.

"We appreciate Health Canada's thorough and timely review of ORLADEYO, and with this authorization we are one step closer to bringing this important treatment option to Canadians living with HAE," says Jared Rhines, General Manager of BioCryst Canada. "We look forward to working with stakeholders across the country to ensure these Canadians can receive timely and appropriate access to ORLADEYO. Further, this authorization follows approvals in the US, Europe and other markets, highlighting our ongoing commitment to individuals living with rare diseases regardless of geography."

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

(Source: BioCryst)



## 7 June 2022

Swissmedic grants marketing authorization for oral, once-daily ORLADEYO® (berotralstat) from **BioCryst Pharmaceuticals, Inc.** for the routine prevention of recurrent HAE attacks in patients 12 years and older in Switzerland.

"ORLADEYO offers people with HAE in Switzerland and their physicians the first orally administered non-steroidal therapy for preventing HAE attacks and provides the community with another vitally important treatment option," says Henrik Balle Boysen, Executive Vice President and COO of HAEi.

"We have made significant progress in making ORLADEYO available to many patients in Europe since we received European Commission approval of our oral, once-daily prophylactic therapy last year," says Waldemar Heiduk, CEO of BioCryst Pharma Deutschland GmbH. "With today's announcement, we look forward to launching ORLADEYO in Switzerland soon, pending finalization of our reimbursement plans."

ORLADEYO was safe and well tolerated in clinical trials. The most frequently reported adverse reactions in

patients receiving ORLADEYO compared with placebo were gastrointestinal reactions. These reactions generally occurred early after initiation of treatment with ORLADEYO, became less frequent with time and typically self-resolved.

(Source: BioCryst)



## 8 June 2022

**KalVista Pharmaceuticals, Inc.** presents data for its oral Factor XIIa (FXIIa) and plasma kallikrein (PKa) inhibitor programs at the KININ2022 conference in Annecy, France. Presentations for oral FXIIa inhibitors shows that the compounds block the initiation and amplification of the kallikrein kinin system (KKS) in preclinical models. Of note, small molecule FXIIa inhibitors were shown to suppress FXII zymogen enzyme activity, which has been recently implicated as a distinct initiator of KKS activation, and thus may contribute to disease.

"The oral FXIIa inhibitors we are evaluating show great promise by blocking the earliest steps of kallikrein kinin system activation," says Andrew Crockett, CEO of KalVista. "The discovery of potent, selective, and orally available FXIIa inhibitors may provide novel therapeutic opportunities to treat HAE and other KKS-mediated diseases."

(Source: KalVista)



## 9 June 2022

**BioCryst Pharmaceuticals, Inc.** enters into an exclusive collaboration with Pint Pharma GmbH to register and promote ORLADEYO®(berotralstat) in the pan-Latin America (LATAM) region.

"We are pleased to join forces with Pint Pharma to bring our oral, once-daily therapy to HAE patients in LATAM who are in need of a new treatment option. Pint Pharma is the ideal partner for BioCryst based on the team's deep experience in rare diseases that spans multiple aspects of commercialization and their



established network across this important region,” says Charlie Gayer, CEO of BioCryst.

“Our partnership with BioCryst is significant for Pint Pharma given the critical unmet need among HAE patients in LATAM who are seeking an innovative treatment option for this serious disease. ORLADEYO has successfully launched in multiple markets across the globe, and we are privileged to leverage our expertise to support BioCryst in introducing this prophylactic therapy to the region,” says David Munoz, CEO and co-founder of Pint Pharma.

Under the terms of the agreement, Pint Pharma will be responsible for obtaining and maintaining all marketing authorizations and for commercializing ORLADEYO in the pan-LATAM region.

Pint Pharma is an Austria-based pharmaceutical company that has extensive experience developing, registering and commercializing rare disease and specialty treatments throughout Latin America and Europe.

(Source: BioCryst)



# CLINICAL TRIALS

According to [clinicaltrials.gov](https://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

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

Recruiting in Argentina

**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 Puerto Rico, the United States

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

Recruiting in Poland, Romania, Slovakia, Spain

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

Recruiting in France

**A Study with Lanadelumab in Persons With HAE in Poland**

Recruiting in Poland

**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 Trial to Evaluate the Efficacy and Safety of Different Doses of KVD824 for Prophylactic Treatment of HAE Type I or II**

Recruiting in Australia, Bulgaria, Canada, Czech Republic, France, Germany, Hungary, Italy, New Zealand, North Macedonia, Puerto Rico, the United Kingdom, the United States

**A Study in Teenagers and Adults with HAE Type I or Type II Who Use Lanadelumab as Long-Term Prophylaxis**

Recruiting in Austria, France, Germany, Greece

**Biomarker for HAE**

Recruiting in Armenia, Egypt, Georgia, Germany, India, Peru, Poland, Romania, Turkey

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

Recruiting in Austria, Germany, Israel, Italy, Kuwait, Portugal, Spain, Switzerland

**CLOUD-R HAE Registry**

Recruiting in France





National Institutes of Health  
Turning Discovery Into Health



EU Clinical Trials Register

**C1 Inhibitor Registry in the Treatment of HAE Attacks**

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

**Dose-ranging Study of Oral PHA-022121 for Acute Treatment of Angioedema Attacks in Patients with HAE**

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

**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, Israel, Poland, the United Kingdom, 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, Czech Republic, Denmark, France, Germany, Greece, Ireland, Israel, Italy, Spain, Sweden, the United Kingdom

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

Recruiting in Italy

**Long-term Safety and Efficacy of CSL312 (Garadacimab) in the Prophylactic Treatment of HAE Attacks**

Recruiting in Australia, Canada, Czech Republic, Germany, Hong Kong, Hungary, Israel, Japan, the Netherlands, New Zealand, Russia, Spain, Taiwan, the United States

**Long Term Access to Berotralstat for HAE Subjects from Previous Clinical Trials**

Recruiting in Czech Republic, France, North Macedonia, Slovakia, South Africa, South Korea

**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 Canada, the United States

**Stopping Androgen Treatment in Patients with HAE – Characterization of Reasons and Protocols and Development of Advice for Patients and Physicians**

Recruiting in France, Germany, Hungary

**The Role of the Coagulation Pathways in Recurrent Angioedema**

Recruiting in France

**A Survey of Lanadelumab in Participants with HAE**

Will be recruiting

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

Will be recruiting

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

Will be recruiting

**Development of Diagnostic and Disease-Monitoring Biomarkers of Bradykinin-Mediated Angioedema with or Without C1-Inhibitor Deficiency in HAE**

Will be recruiting in Japan

**Efficacy and Safety of GNR-038 vs Berinert® in Patients With HAE**

Will be recruiting in Russia

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

Will be recruiting

**Open-label Berotralstat Access to HAE Patients Previously Enrolled in Berotralstat Studies APeX-A**

Will be recruiting

Read more about these and other clinical trials at:

- [clinicaltrials.gov](https://clinicaltrials.gov)
- [clinicaltrialsregister.eu](https://clinicaltrialsregister.eu)
- [trialsearch.who.int](https://trialsearch.who.int)





## 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.

