


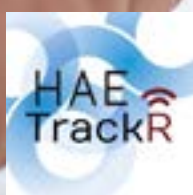
# Global perspectives:

HAEi MAGAZINE · ISSUE 2/2021

 **93** Member countries



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**NEW APP! HAE TRACKR  
BY PATIENTS FOR PATIENTS**

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**Cover photo**

HAEi launches new app: HAE TrackR  
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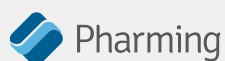
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organization dedicated to working with  
a network of national HAE member  
organizations to raise awareness of HAE



## DEAR HAEI FRIENDS,

A warm welcome to the second 2021 edition of *Global Perspectives*. I would like to start by congratulating our member organizations for the unprecedented level of participation in the 10th anniversary of **hae day**:-). The HAEi community broke all prior records by recording an incredible 169 million steps for raising HAE awareness! Clearly, the global HAE movement continues to gather momentum.

As usual, *Global Perspectives* provides our community with a comprehensive overview reflecting just about everything going on in the world of HAE. This issue includes very exciting information regarding the launch of HAE TrackR. This secure, easy to use app (designed and developed by your fellow HAE patients) helps you gather and record important information about your HAE that can be shared with your physician. I also invite you to read about the two highly successful HAEi Virtual Regional Workshops held in Central Eastern Europe and Benelux, and South America and Mexico, Central America and the Caribbean. We also provide an overview of the 12th C1-INH Deficiency and Angioedema Workshop.

Furthermore, I would like to highlight the importance of a very special group that comprise the future HAE advocates and leaders – our HAEi Youngsters. The spirit of this remarkable group is captured in a phrase that accompanies all of their social media and written materials:

### CONNECTED BY HAE. UNITED AS A FAMILY.

As you will read on pages 30 through 34, the HAEi Youngsters Community is an inclusive group of patients, caregivers, siblings and partners between 12 and 25. HAEi also offers the Brady Club for children between 5 and 12, including workbooks and fun activities helping the youngest members of the community better understand their HAE.

The programs and services we offer is part of a longstanding commitment to provide our HAEi Youngsters with the resources and assistance to help them interact with peers, better understand their HAE, and learn how to advocate for themselves and others in their respective countries. This full list of HAEi Youngster programs and activities is too long for this message, but here are a few highlights:

- A new youngsters webpage
- On-line meet-ups where HAEi Youngsters can interact
- Social media posts on living with HAE
- Youngster produced video recordings prepared for HAEi Regional Meetings
- Youngster's tracks at our Global Conferences
- A Special Youth Summer Workshop in Frankfurt, Germany
- A Global Youth Advocacy training and certification Workshop, and
- HAEi Advocacy Academy - an online training platform for HAEi Youngsters wishing to learn about advocacy

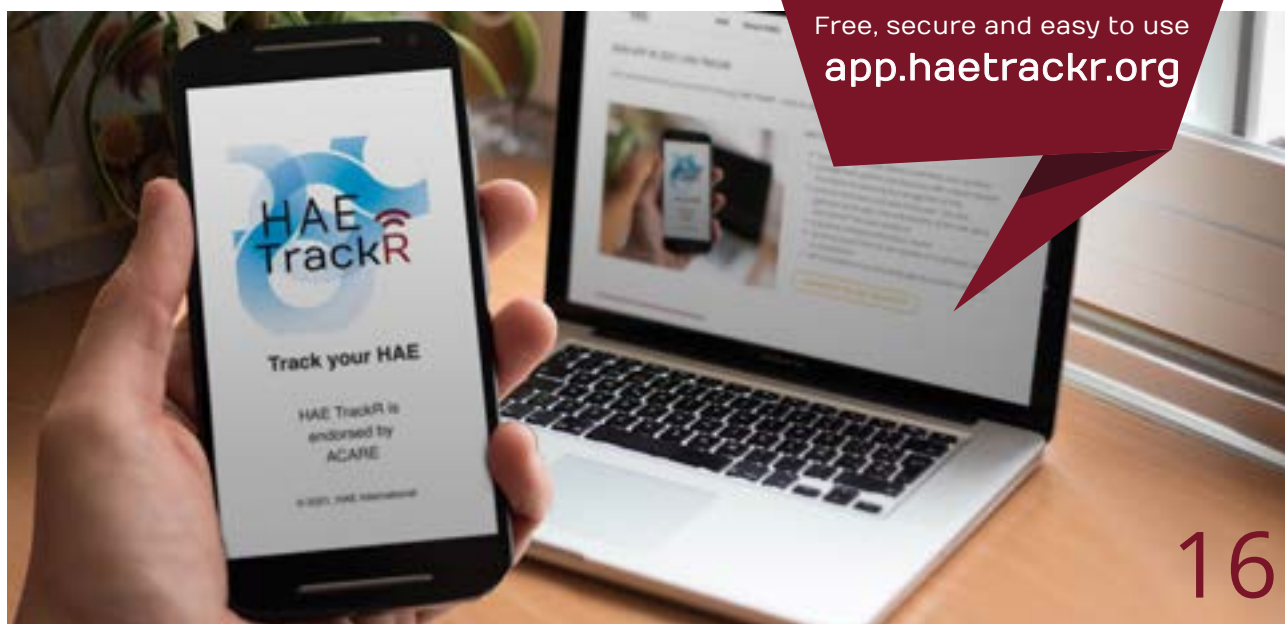
HAEi Youngsters continue to be a very high priority, as evidenced by our commitment to increasing investment in training, motivating, and encouraging our future HAEi advocates. Nevena Tsutsumanova, HAEi's Operations Manager, who works closely with the Youth Advisory Group and oversees the HAEi Youngsters program, reports that plans are well underway to significantly expand our youth-related programs and services. Future issues of *Global Perspectives* will highlight HAEi Youngster's activities and accomplishments.

Wishing all HAEi friends throughout the globe the very best, and looking forward to meeting in person again, hopefully in the very near future.

Warmest regards and please stay safe,

Anthony J. Castaldo  
President and CEO, HAEi

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# NEWS FROM THE HAEi REGIONAL PATIENT ADVOCATES

It has been truly amazing to see such a great variety of awareness activities and celebrations around the world for **hae day :-)** 2021! Congratulations to everyone for taking part and making the HAE awareness day such a huge success.

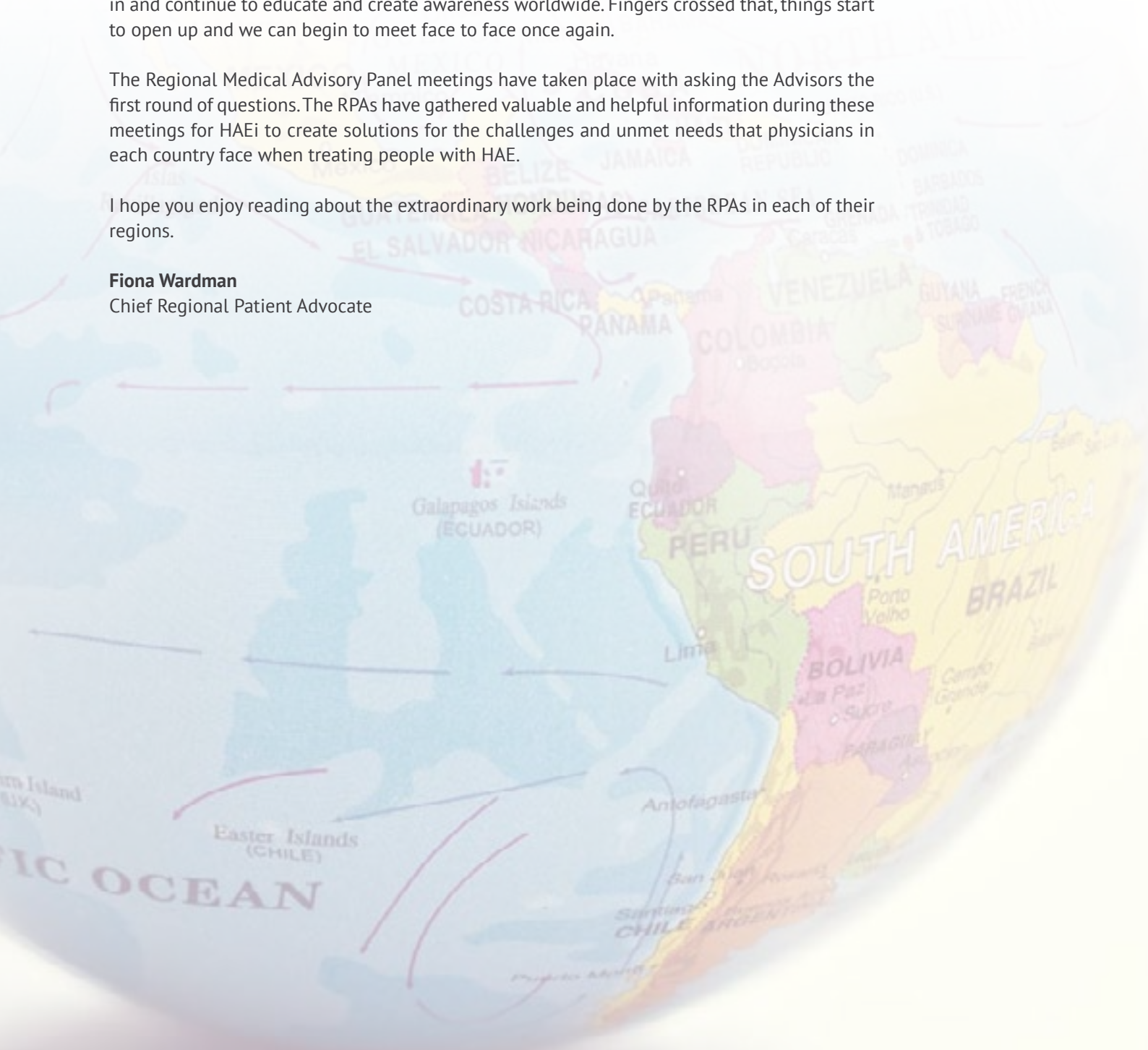
The Regional Patient Advocates (RPAs) have been involved in many virtual events over the last few months, and given the ongoing situation with COVID-19, we are thankful to still take part in and continue to educate and create awareness worldwide. Fingers crossed that things start to open up and we can begin to meet face to face once again.

The Regional Medical Advisory Panel meetings have taken place with asking the Advisors the first round of questions. The RPAs have gathered valuable and helpful information during these meetings for HAEi to create solutions for the challenges and unmet needs that physicians in each country face when treating people with HAE.

I hope you enjoy reading about the extraordinary work being done by the RPAs in each of their regions.

**Fiona Wardman**

Chief Regional Patient Advocate





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



A lot of things have been going on in my region in March, April and May. Much is, of course, related to the **hae day :-)** celebration that has kept so many of us busy with all kinds of activities. It's been amazing to see a great number of people from many countries join in and create awareness for HAE. Here are a few examples:

- In Scandinavia, youngster representatives worked hard to raise awareness by engaging as many youngsters from **Sweden, Norway** and **Denmark** as possible. Both online sessions and local individual activities were tracked.
- **Germany** hosted a wonderful virtual patient day with a lot of great information for the community.
- **Austria** created a TV broadcast centering around HAE, available online and presented during prime time on national television.
- **Switzerland** shared a gift with all its members, one that could be planted and, if treated correctly, would result in a lucky clover.

These are just some of the activities – all over the region, people were busy raising awareness step by step. **Finland**, for instance, started a little later but still managed to walk, run, cycle etc. a lot of steps!

Not to forget, building up to the **hae day :-)** campaign, a lot of time and hard work was invested from all the member countries to build content for the campaign website. All this content will remain and can be found under each country on [haei.org](https://haei.org). If you haven't already done so, this is a great chance to read about the fantastic countries in all regions.

Thank you to everyone who was involved in the **hae day :-)** campaign to make it such a great success and to be global yet still very local in all the different communities.

While all these activities took the main priority, we are currently working on building the Regional Medical Advisory Panel for the region. Some doctors have already agreed to take part, and we are very happy to start this together. In some countries, we are still talking with doctors about their participation.

HAE TrackR – the exciting new app from HAEi where you, amongst other things, can track your attacks, treatments and condition – is now ready. Currently, we are working on more language versions with the member organizations.

Last but not least, the Finnish website hosted on HAEi's environment is in the making and dialogue has started about the Austrian one.

This year, HAE Scandinavia (Denmark, Norway and Sweden) can celebrate its 20th anniversary. The 2021 Scandinavian Conference by HAE Scandinavia will take place 12 to 14 November 2021 as an in-person conference at the Clarion Hotel Copenhagen Airport.

Again, a callout: If you are a patient, caregiver or physician treating HAE patients in **Iceland, the Faroe Islands** or **Greenland** and would like to get in touch, please do not hesitate to reach out to me. It would be great to connect with you.





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



During March, the World Allergy Organization (WAO) and the Pan-Arab Society of Allergy, Asthma, and Immunology (PASAAI) organized the WAO Pan Arab Region Virtual Congress with several sessions, including one for HAE. Professor Douagui from Algeria invited me to join the HAE session named “HAE: Identifying & Managing a Potential Killer” that included these speakers:

- “Recurrent Angioedema: When to Suspect HAE” by Professor Iman Nars from Oman
- “Understanding and Navigating the Burden of HAE” by Professor Rand Arnaout from Saudi Arabia
- “Advance of HAE Biologicals” by Professor Marcus Maurer from Germany.

During May, the HAEi 10th **hae day :-)** was celebrated through all types of exciting activities. Many of the countries in the region were actively participating by adding steps on [haeday.org](https://haeday.org), where we could find interesting information from most of the countries of the Mediterranean, North Africa and British Isles region. Some of the countries had also organized local events to commemorate this day; **Morocco** and **Algeria** had local walks with HAE patients, family members and friends, and you can read about the activities done by the **United Kingdom** and **Spain** in the section with news from members organizations around the world later on in this magazine.

HAEi is starting to organize the 2021 Middle East, North Africa and Sub Sahara Africa Workshop. We expect to launch this exciting workshop after summer, so please stay tuned through *Global Perspectives* and our social media channels.

We are happy to announce that the patient group in **Tunisia** with the doctors’ support are in their final steps to set up an official association. It will be constituted by eight members; three doctors, one lawyer and four patients. At least one member of each HAE family will be represented in the association. The local doctors currently dealing with HAE patients have weekly follow-ups to keep record of the attacks.







I have had a call with one of the association members to introduce and explain all the resources that HAEi has available to help them run the association and provide guidance.

In **Libya**, the patient lead is planning an awareness campaign to increase knowledge and locate new patients because at the moment they only have around 10 HAE patients in their group. Based on the 1:50.000 ratio, the number should be closer to 132. For this campaign, the patient lead will be using the poster designed by HAEi and a brochure with basic HAE information that HAEi staff members and I will help them develop. The poster and brochure will be distributed in hospitals, clinics, emergency rooms and pharmacies; and the local doctors will be distributing the brochure to their current patients.

In Spain, the HAE organization AEDAF has submitted a proposal to this year's call for the CSL Behring EU Local Empowerment for Advocacy Development (LEAD) Grant applications, and I am happy to announce that one of the grants has been awarded to AEDAF.

The issue AEDAF addresses is this: Since the approval and commercialization in Spain of modern treatments for HAE, AEDAF has become increasingly aware of major differences in access and availability of these treatments, as well as in approval for patients to keep

these treatments at home and learn self-administration, depending on the Autonomous Region where they live, the hospital where they receive care and the physician who treats them. The uneven access and availability of treatments seem to be related to price, but also to differences in knowledge of accepted consensus and standard of care guidelines or inability or reluctance to observe them.

The objective of the project is to improve the equality of access to healthcare, diagnosis and modern HAE treatments in Spain and thus help HAE patients to take control of their disease and improve their quality of life.

To achieve this objective, AEDAF will:

- Conduct an online survey among AEDAF members and other patients being treated by the lead HAE physicians in Spain to ask about their personal experiences with access to and availability of modern treatments.
- Perform an online survey among physicians managing patients with HAE in different hospitals and Autonomous Regions regarding availability and access to the different drugs used for the treatment of HAE.
- Produce a short video or written document with testimonies of a select number of patients, including patients who do have proper access to modern treatments and others who are still heavily burdened by their disease due to lack of access, which would be used to accompany the survey findings in subsequent actions.
- With a prior press release, submit the survey results to the national Ministry of Health and Regional Ministries of Health in Spain to underline these differences and advocate for equality in healthcare and access for patients all over Spain.
- The survey's results will also be used to produce an improved, updated list of hospitals in Spain with knowledge of HAE and the availability of modern treatments to replace the outdated list posted on the AEDAF website.
- In the future, AEDAF could use the survey findings to focus on the regions where the situation is most inadequate to conduct workshops, produce online courses for patients and healthcare professionals and in general improve communication, information and advocacy efforts.



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



Hello everyone! I hope you had a fabulous **hae day :-)** and you did enjoy what HAEi had prepared to celebrate the 10th anniversary of the awareness day. The past few months have been incredibly busy and full of different activities in the regions I am responsible for.

Together with Maria Ferron, the Regional Patient Advocate for the Mediterranean, North Africa and the British Isles, and the HAEi Team, I have been working on the regional website for the Middle East and North Africa. The region's HAE patients and caregivers will have their own website in Arabic and French that will highlight the most important information from the HAE community. We expect to continue our work over the summer and have the website launched in September at the latest.

Another great piece of news for the HAE community in the Middle East, North Africa, and Sub Sahara Africa is the 2021 HAEi Virtual Workshop. I am delighted to inform you that the Regional Patient Advocates, respectively for each region, and the HAEi Team have started long term and difficult work on this project. By engaging HAEi member organizations, HAE patients, caregivers and HAE expert physicians, we wish to deliver another helpful event. Our previous experiences with virtual workshops enable us to make this particular project even more impactful, and I look forward to it.

Also, in the last months, I have had the pleasure of working closely with Takeda and the company's Middle East Team on different projects, increasing HAE awareness in the region, with an initial focus on projects to be launched in **Saudi Arabia** and the **United Arab Emirates**. We exchanged information and discussed how to make the project more valuable for patients. I was happy to present HAEi at the Takeda Gulf & Lebanon Patient Webinar held on 5 June 2021.

Furthermore, together with Rashad Matraji, the HAEi Patient Advocacy Contractor in the Middle East, I have finalized the HAEi Emergency Department Poster in Arabic and organized a Zoom meeting with the attendance of the member organizations from **Lebanon, Kuwait, Jordan** and **Qatar**.

In April, I organized Zoom virtual meetings with the member organizations in **Belarus, Georgia, Hungary, Kazakhstan, the Netherlands, Poland, Russia, Slovakia** and **Ukraine**. We discussed several topics, including the HAE TrackR app, the HAEi Advocacy Academy, the HAEA Brady Club and the **hae day :-)** anniversary. The meetings were very informative, and I received great feedback from the member organizations on current activities they have been doing separately or in collaboration with HAEi partners.

I have participated in person or virtually in several activities to celebrate **hae day :-)** 2021, including the Global Walk, the HAE Russia National Summit and the HAE Poland Virtual Meeting.

Furthermore, I had the pleasure of bringing another level of HAEi's decentralized approach to life through the organization of Regional Medical Advisory Panels (RMAs) for Central Eastern Europe, Benelux and the Middle East. In the two RMA meetings we have held so far, ten Advisors participated, all HAE expert physicians. We had a very productive discussion, and it was so great to hear the physicians' perspective on different HAE areas. Thank you all for your contribution; this is very much appreciated!

Finally, some news from a few of the countries I cover:

HAE Russia has produced and distributed the HAEi Emergency Department Poster among local hospitals

and clinics. Also, the organization has held seven awareness meetings in seven venues around Russia (e.g. Saint Petersburg) dedicated to HAE patients and health care professionals. On 15-16 May 2021, HAE Russia organized an HAE National Summit in Moscow. Finally, the organization has supported the successful registration of Lanadelumab (Takhzyro).

HAE Slovakia has actively supported Takeda's HAE awareness campaign resulting in the reimbursement for and access to long term prophylaxis (Takhzyro) for patients in Slovakia. The next step will be the recording of an HAE awareness video, possibly with patient participation. Currently, the organization is translating into Slovak the HAEi Emergency Department Poster and is interested in implementing the HAEA Brady Club Activity Book.

HAE Poland has developed the organization's own Regional Patient Ambassador project. Four such ambassadors are in constant contact with patients and support them on a daily basis. It is amazing how this project was needed by the Polish patient community and how much is yet to be done in this area.

Moreover, the spring edition of the HAEA Brady Club Activity Book has been translated, printed and sent to pediatric patients participating in this particular project. As if this was not enough, HAE Poland actively supports reimbursement processes regarding on-demand treatments and long-term prophylaxis.

Finally, it should be mentioned that the best of the previous electronic patient diary app used by Polish patients has been combined with the new HAE TrackR app from HAEi – and this new app is now available in Polish.



**JAVIER SANTANA**  
CENTRAL AMERICA AND CARIBBEAN



During the past months, groups of people with HAE in the Central American and Caribbean region have been quite active in seeking the support of citizens and the attention of government entities to achieve access to treatments for HAE.

Countries such as **Panama** and **Costa Rica** have held meetings with important government officials to discuss the problems faced by people suffering from HAE due to the lack of medical guidelines in hospitals and the lack of appropriate medications for HAE. In addition, the representatives of the groups in both countries have been present in important national media, discussing the problems they face. This has led to an increase in citizen support. As the HAEi representative in the region, I have been able to share and discuss with doctors from both countries the medical guidelines used in other countries to draft and educate new guidelines in their respective countries.

The patient groups in the **Dominican Republic**, **El Salvador** and **Cuba** have continued to identify more patients and physicians who know about the disease so that they can continue to increase the number of members in groups of people with HAE.

In **Puerto Rico**, the House of Representatives held a public hearing to discuss a bill aimed at helping people with HAE of limited resources. Doctors with knowledge of HAE in Puerto Rico participated in the public hearing to offer details about the disease and their experience caring for people with HAE.

During **hae day :-)** 2021 several groups held events in their countries to raise awareness about HAE. Among some of the events were lighting of private and government buildings with the **hae day :-)** colors, webinars, media interviews and peaceful protests.





**PATRICIA KARANI**  
SUB SAHARA AFRICA



I organized the first HAE doctors' training in **Ghana** with the collaboration of the Rare Disease Ghana initiative. It was overwhelmingly attended by 40 doctors and clinicians alike. It was a good interactive session with Dr. Priya Bowry giving a lengthy and comprehensive presentation on the clinical approach to angioedema and HAE with good case studies. Professor Konrad Bork presented treatment options in the case of availability and non-availability of modern treatment. He also presented research that has been done on danazol, fresh frozen plasma and other treatments around the globe.

I was nominated as a speaker for the Africa Health Agenda International Conference (AHAIC) 2021 organized by AMREF Health Africa and officially opened by the President of the Republic of **Kenya**. The conference theme was "Decade of Action: Driving Momentum to achieve Universal Health Coverage in Africa". It attracted 3,000 participants from 98 countries across Africa and beyond. I shared my journey to diagnosis and challenges that I have faced and continue to meet in the struggle to get rare diseases like HAE covered under Kenya's national health medical cover.

To celebrate the 10th anniversary of **hae day :-)** HAE Kenya created a video showcasing the burden of illness for a patient living with HAE and the difficulties an HAE patient faces at the workplace as well as in her social life.

We have held our first fruitful discussions with a focused team formed in **Tanzania** that comprises five medical doctors wishing to participate in creating awareness. We agreed to work on conducting HAE training soon. We have translated some awareness material into Swahili, which is widely spoken in Tanzania, and this will help raise more awareness.

I have established contact with the **Botswana** Organization for Rare Disease (BORDIS) founder Eda Selebasto, and we have discussed the current situation in the health sector in the country. We have also started conversations on the best way to create awareness.

I have been introduced to Dr. Martina Kawome, who is actively involved in supporting rare disease patients in **Zimbabwe**. She is ready and willing to create awareness through health care professionals.

Rare Disease **Lesotho** has linked me to a doctor contact to consider how we could initiate the HAE discussion amongst health care professionals.

I have been able to find a new contact with Rose Okoma, who is a rare disease advocate in the **Ivory Coast**, and in **DR Congo** connection has been established with Aime Lumaka, who is also a rare disease advocate. We are hoping to collaborate in raising awareness in both countries.

Finally, I should mention that I organized the first Regional Medical Advisory Panel meeting with lead doctors from **Rwanda, South Africa, Sudan** and Kenya. During the important discussions, we identified similarities and differences in the diagnosis and management of HAE in these countries.



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



First of all: We are moving towards adding **Bolivia** to the HAEi world map as Dr. Ariel Ramírez has been registered as an HAE knowledgeable physician. You can find his contact information on [haei.org](http://haei.org). Furthermore, I am in contact with a woman who has both a brother, a sister and a mother with HAE in Bolivia. Hopefully, they will be interested in setting up a patient group.

The big thing during the last few months has been the first Latin American HAE workshop. We made videos in Spanish and Portuguese or with subtitles in Spanish and Portuguese for the HAE community in Latin America so that it could be a genuinely inclusive meeting also for those not speaking English. For the workshop – held in a virtual format – we had engagement and participation of many member organizations and physicians from the region. You can find an article about the workshop later on in this issue of *Global Perspectives*.

For the first time, member organizations in South America and **Mexico** came together to celebrate **hae day :-)** 2021. We had a preparation meeting on 12 May – and on 15 May, we had the event itself. With the member organizations in **Argentina, Brazil, Chile, Colombia, Mexico, Paraguay, Peru, Uruguay, and Venezuela** joining in, we had more than 50 participants for a two-hour meeting. During the event, there were activities such as videos from patients and videos from doctors – and the participants split into eleven breakout rooms for group discussions. For this meeting, we developed a logo, which I am now using for the Instagram account.

Also, I have been working on collecting replies from physicians who are part of the Regional Medical Advisory Panel for my region. Furthermore, I have provided the Chief Regional Patient Advocate with information for CSL Behring to have Berinert in Latin America and Mexico.

As for the rollout of HAEi Connect, it will be coming to both Peru and Colombia soon.





**NATASA ANGJELESKA**  
SOUTH EASTERN EUROPE



On 6 March 2021, I was able via Zoom to address the participants at the annual patient meeting in **Croatia** and congratulate them on their engagement and activities even in a COVID year.

I held a meeting with the **Cyprus** patient group on 25 March 2021, during which I introduced the participants to the structure and functioning of HAEi as an umbrella organization. I also presented some of the HAEi resources available to member countries, such as hosted websites and the new apps HAE Companion and HAE TrackR. We discussed the advantages of having a registered patient organization, and it was agreed that the Cyprus group would investigate the necessary documentation and costs related to a registration.

In April, I received information from the physician from **Bosnia and Herzegovina** about five new confirmed patients with HAE type 1. Now all six patients are receiving therapy through donations, but our future goal is to make modern treatments available in this country for all patients with HAE.

In the same month, I held a meeting with the President of HAE **Serbia** and discussed the situation regarding access to preventive treatment for Serbian patients and their plans for marking **hae day :-)** 2021.

Leading up to the **hae day :-)** anniversary I was interviewed for two web media about HAE care and development in **North Macedonia** and the region. Also, I was a guest in a radio program where we discussed issues about living with an HAE patient, my advocacy work and the global and national initiatives for the awareness day.

Most of my time in May was dedicated to promotion and announcement of the **hae day :-)** anniversary. I posted on social media and motivated representatives from countries in my region to join the global initiative by registering activities on [haeday.org](http://haeday.org). Also, I encouraged them to initiate national events to mark this very important day for the HAE community. Almost all of the

countries in South Eastern Europe entered activities for the **hae day :-)** campaign 2021, and in the end, North Macedonia was among the top five countries entering activities. This was due to the excellent network of our friends and supporters who are always backing our awareness and advocacy activities for HAE.

For **hae day :-)** 2021 HAE Croatia initiated the illumination in purple of several buildings and monuments in cities like Zagreb, Split, Vukovar, Osijek, Zadar, Djakovo, Rijeka and Sisak. A similar activity was undertaken by HAE Serbia members who shared photos of the illuminated buildings and monuments across Serbia, and in **Romania**, the University of Medicine, Pharmacy, Science and Technology was illuminated in purple.

I initiated and participated in making a video recording from HAE Macedonia for **hae day :-)** 2021. The video received very positive feedback from the general public, our friends, supporters, media and physicians. It was also posted with a congratulation note to the Facebook page of the North Macedonian Minister of Health, Dr. Venko Filipce.

On 27 May 2021, I held a meeting with the nominated physicians for the Regional Medical Advisory Panel from the countries in my region. Representatives from **Albania, Bulgaria, Bosnia and Herzegovina, Croatia, Romania, Serbia, Slovenia, North Macedonia and Turkey** were present. The panelists were introduced to each other, and in a very pleasant atmosphere, we had a fruitful discussion on the first round of the questions that were sent to the participants before the meeting. Strong enthusiasm was shown from most of the doctors regarding the initiative. Some of the problems faced by physicians during the pandemic were highlighted, such as the locations where doctors treating HAE patients were turned into COVID-19 centers, as was the case in Albania and North Macedonia.





**FIONA WARDMAN**  
ASIA PACIFIC



Since the last magazine, there has been a focus on a few projects; a couple of these were the **hae day :-)** “Let’s Take the Next Steps” campaign and rolling out of the Regional Medical Advisory Panel project.

All the member organizations within the Asia Pacific region had their history and plans for the future highlighted on [haeday.org](http://haeday.org). It was also great to see the countries get involved with logging activities and photos on the campaign website.

Member organizations have nominated Advisors for the Regional Medical Advisory Panel project, and the first meetings have taken place. Due to the time difference within the region, four separate Zoom calls have been held so far. The discussions were very insightful, with the physicians sharing their challenges and unmet needs treating HAE patients within their countries, along with talking about diagnostics, HAE protocols, and a few other topics.

Over the last few months, meetings with member organizations have taken place to discuss various projects, tools, resources, and information translation. The Brady Club coloring in pages for children has been shared.

Together with HAE **Japan**, a webinar was held on HAE with normal C1-INH. We were very fortunate to have Professor Hide from Japan and Professor Riedl from the USA present. The entire webinar will be available on the HAE Japan website in English and Japanese very soon. In the meantime, Professor Riedl’s presentation can be found on the HAEi website, and you can find links on the HAEi social media channels.

On **hae day :-)** 2021, the Hereditary Angioedema Society of **India** held its first conference on HAE for physicians, patients, and their family members from around the country. The Society invited me to do a presentation; the event was attended by over 300 online.

An important paper titled “Mitigating Disparity in Health Care Resources Between Countries for Management of Hereditary Angioedema” was finalized and published recently.

Over the past few months in India, we have held Zoom meetings with patients, their family members, and special guest speakers such as an Obstetrician/Gynecologist, a Mental Health Therapist, and HAE experts from within India. On behalf of HAE India and myself, I would like to thank the guest speakers for giving their time and support so generously. These meetings have been beneficial, so we have invited patients and their family members from **Pakistan, Singapore, and Bangladesh**.

Dedicated discussions have been taking place for accessing treatments in India.

In partnership with the Chinese Organisation for Rare Diseases, HAE **China** invited me to take part in an **hae day :-)** patient and carers conference. The event was highly successful, with 60 people in the venue and over 450 online. Together with HAE China, HAEi is finalizing the questions for the HAEi Burden of Illness survey.

HAE **Korea** held an online meeting on **hae day :-)** where I was invited to give the opening address.

I was included as one of the three member organization leads from three points of the globe to take part in the HAE Connect **hae day :-)** initiative for Takeda. I presented on behalf of HAE Australasia.

A new pilot project in partnership with HAEi is currently being planned and finalized. I look forward to sharing more information about this exciting project in the next issue of *Global Perspectives*.

HAE Australasia was planning on holding the 2021 conference as a virtual event. The plans have been updated to hold a hybrid meeting to allow patients and their families to gather in a few states around **Australia**. A separate in-person meeting is being planned in **New Zealand**.



Track your HAE

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# HAE TrackR is now live!

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

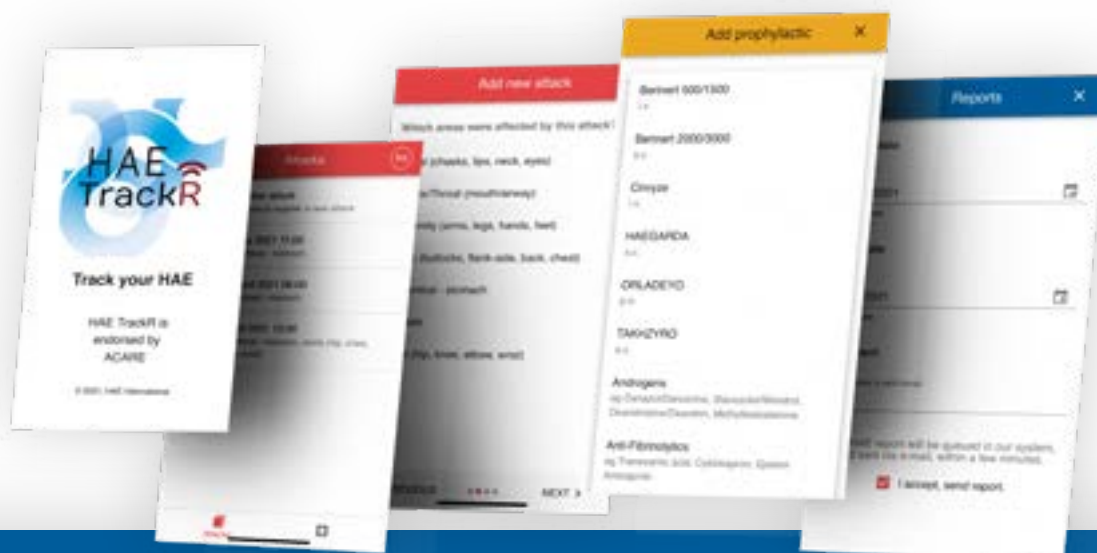
Developed by fellow HAE patients at HAEi, **HAE TrackR** is an easy-to-use electronic diary designed to record your HAE attacks, treatments and the impact HAE has on your life and the life of your loved ones.

- ✓ **HAE TrackR** is a secure, product and company neutral app, where all data gathered is the sole property of the user. Only you can share the data, if you want, with your physician.
- ✓ **HAE TrackR** allows you to download a comprehensive report of your attacks and treatments to be used as a tool for both you and your physician in managing your HAE.
- ✓ Endorsed by the ACARE network (Angioedema Center of Reference and Excellence), **HAE TrackR** will make managing your HAE easier.
- ✓ **HAE TrackR** strictly protects your data and privacy and is fully EU-GDPR compliant. HAE TrackR can be accessed from any device (smartphone, tablet, or computer) anywhere in the world.

In its first version **HAE TrackR** is available in the following languages: English, Polish, Norwegian, Swedish, and Danish. Very soon these languages will follow: Spanish, German, French, and Macedonian.

## Is your language missing?

Reach out to your member organization. **HAE TrackR** will continue to introduce new languages based on request from our member organizations.



**HAE TrackR** is developed by HAE International (HAEi) and is available as a Progressive Web Application (PWA), allowing you to access your secure and GDPR compliant electronic diary from any device with a standard internet browser.



## RECORD-BREAKING CELEBRATION FOR 10TH HAE DAY :- )

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As the HAE community knows, 16 May is the annual focus for raising awareness of HAE. In 2021, HAEi reached the milestone of the 10th **hae day :- )** and decided to celebrate with a very special campaign and an update to the activity challenge.

The HAEi team began 2021 with an extensive project plan and worked for months to bring everything together to ensure that this milestone was an exciting and unique opportunity for the HAE community. With the great efforts from and wonderful collaboration with the Regional Patient Advocates, member organizations, HAEi Youngsters and HAEi Leadership, the “Let’s Take the Next Step” campaign launched on 1 April 2021.

“To mark the 10th **hae day :- )** we planned something special. First, a campaign to showcase the power of advocacy and the impact member organizations and HAEi have had over the past ten years. Secondly, an activity challenge that everyone could take part in as they continued to live with restrictions to help manage the COVID-19 pandemic,” says Henrik Balle Boysen, HAEi Executive Vice President and COO.

The participants regularly added activities – both physical and wellbeing – to the **hae day :- )** website. The steps generated by the activities created a virtual walk around the world, unlocking content from the different regions and member countries – all in time for **hae day :- )** 2021.

Henrik Balle Boysen continues: “We’ve been overwhelmed by the response. Seeing the on-the-ground difference that our member organizations make and how our tools and resources are helping is so rewarding, as is seeing the amazing numbers from this year.”

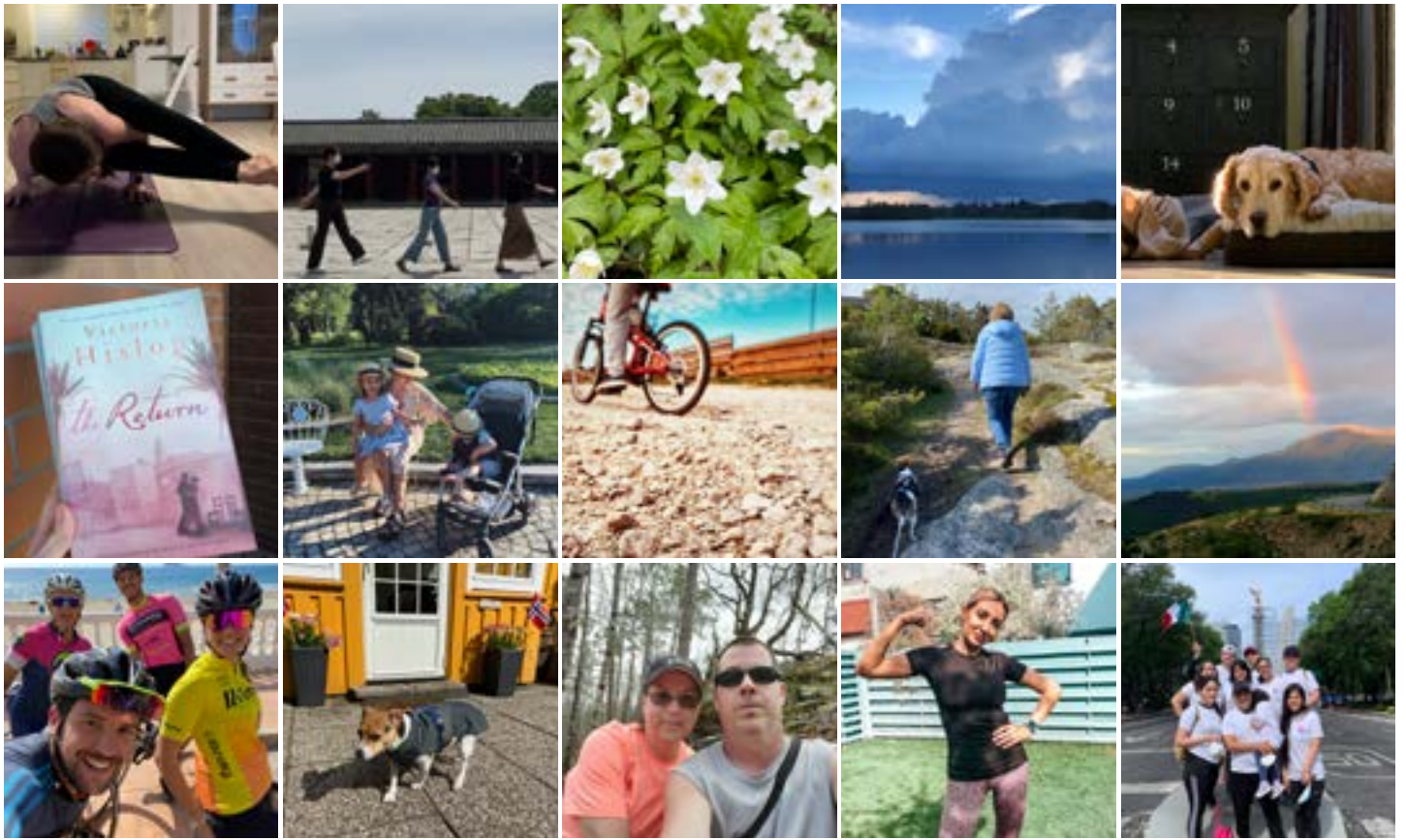
The “Let’s Take the Next Step” campaign ran until 31 May 2021. In that time, it had generated a record-breaking 168,787,911 steps from 7,414 activities in 61 countries – that is 128,649 km in total. Take a bow everyone – what a result.

“People participated on their own or in groups, as an HAE organization or as a company and walked, cycled, ran, rowed, read, painted, meditated, danced, “bingo-ed” and much more. HAEi says a huge thank you to each and every person who took part”, says Henrik Balle Boysen.

The generated steps took the participants on a virtual walk visiting each of the eight HAEi regions to showcase the 93 member countries’ achievements and spotlight the HAEi Youngsters and HAEi developments at the end. You can catch up with all this and more at [haeday.org](https://haeday.org).

“For **hae day :- )** 2021, we’ve been able to shine a light on incredible achievements that are a result of the power of advocacy. But we know our work is far from complete. We continue in our day-to-day efforts to achieve the goals of improved time to diagnosis and consistent access to lifesaving therapies for everyone with HAE around the world”, says Anthony J. Castaldo, HAEi President and CEO.

Anthony J. Castaldo adds: “There is no doubt that the annual **hae day :- )** offers a great opportunity as a focus for HAE awareness, and we see more and more engagement across our community each year. Although we won’t have an anniversary to spur us on next year, we look forward to opening our activity challenge again on 1 April 2022. Let’s see where we can get to then.”



## 2021 ACTIVITY CHALLENGE IN NUMBERS

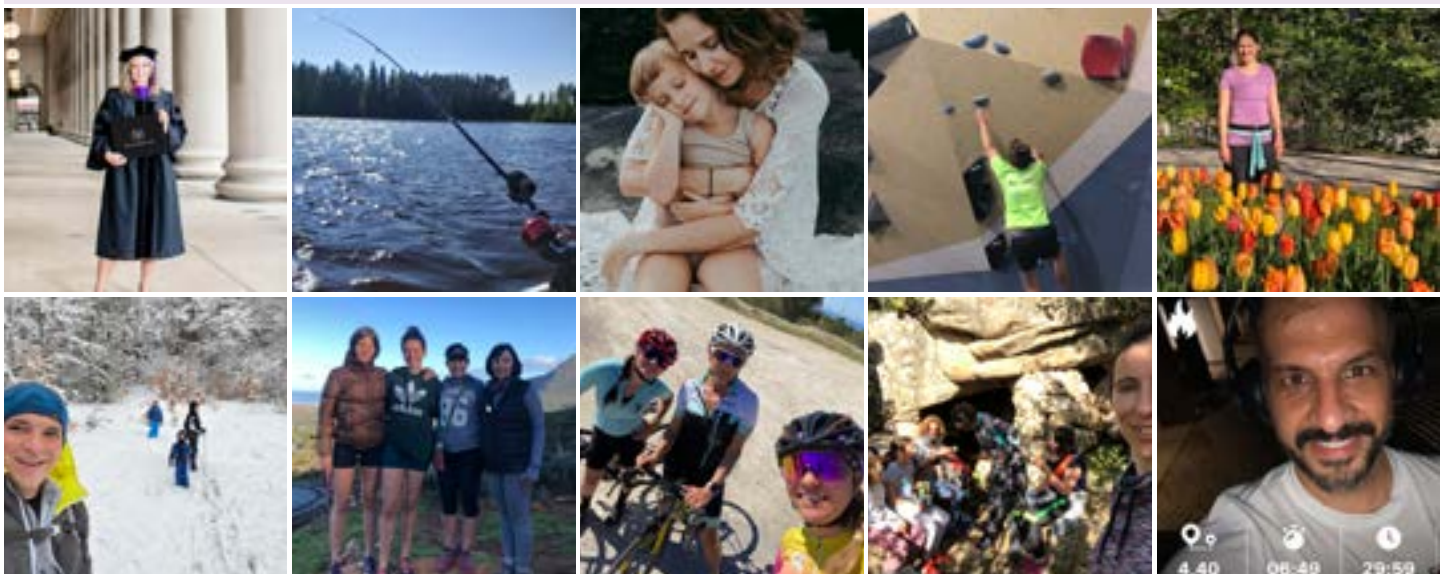
**168,787,911**  
steps taken in total

**7,474**  
activities

**61**  
countries

**128,649**  
kilometers

**79,939**  
miles







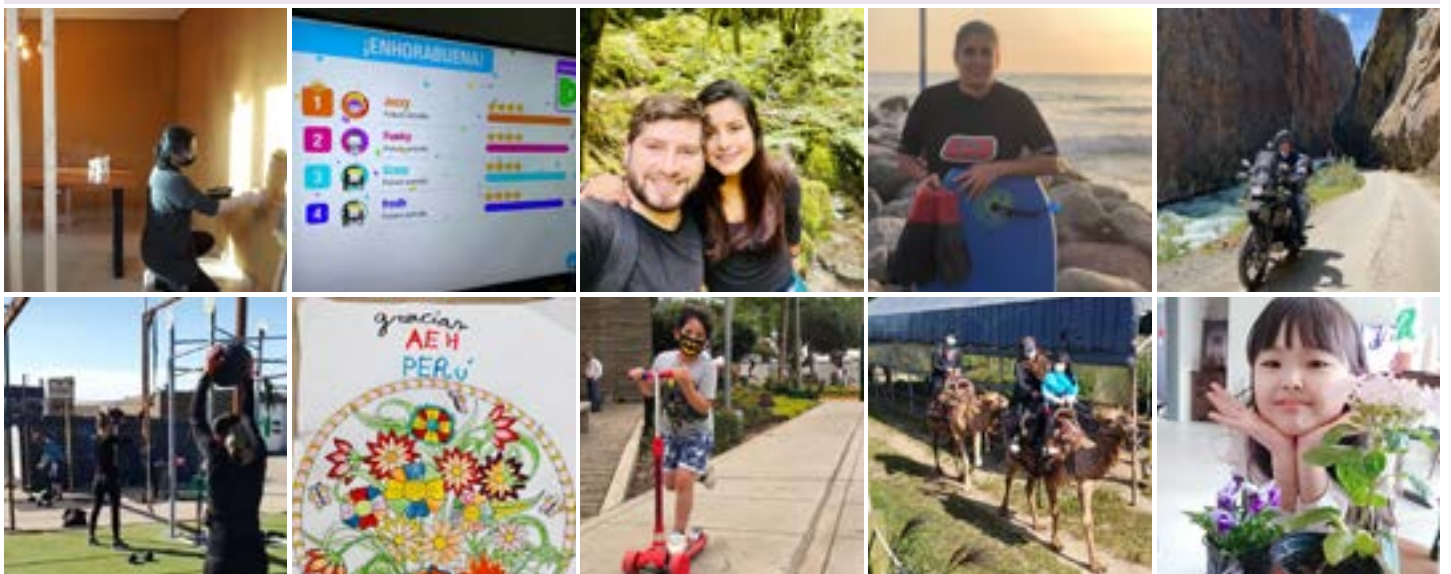
Showcasing the **WORK OF THE 93 MEMBER COUNTRIES** and learning a lot of fun and interesting things along the way

**341 PHOTOS** in the gallery to share activities –  
– visit [haeday.org](http://haeday.org) before 1 November 2021 to see them all!

Input from the **REGIONAL PATIENT ADVOCATES** supporting the regions;  
both in writing and in lighter hearted video interviews

News from the **HAEI YOUNGSTERS** about how  
their group and activities have changed

Overview of **HAEI ACTIVITES** over the past 10 years







# THE EXPANDED ACTIVITY CHALLENGE IN NUMBERS :-)

## HAE DAY :- ) DEVELOPMENT

To celebrate the 10th **hae day :- )** the global walk was extended to include all kinds of activities – physical and well-being. So people not only walked this year, but also cycled, ran, rowed, read, painted, meditated, danced, “bingo-ed” and much much more – and when converted into steps all these activities broke all records in 2021.



## HOW DID THE COUNTRIES DO IN 2021?

Total steps generated (mill.):		Number of activities:		Number of participants:	
	Canada 24.6		Canada 719		Canada 1,228
	Sweden 16.3		Japan 646		North Macedonia 836
	United States 14.2		North Macedonia 420		Japan 724
	North Macedonia 12.9		Denmark 301		Spain 550
	Denmark 12.6		Spain 294		Denmark 506
	Spain 12.4		United Kingdom 280		United Kingdom 470
	Norway 8.1		United States 162		United States 371
	Japan 7.2		Norway 152		Norway 250
	Finland 5.5		Brazil 140		Peru 242
	Poland 4.8		Netherlands 133		Germany 192

# 2030



## What do *you* want to achieve by 2030?

When preparing the **hae day** :-) 2021 campaign, HAEi asked the member organizations what they want to achieve by 2030. Here are the answers region for region:



Asia Pacific and Southeast Asia



### AUSTRALIA

Our next step is gaining access to more treatments for the patients.



### BANGLADESH

We want to connect more people, help guide them, create more awareness and education, and facilitate action for proper treatment.



### CHINA

Our aim is that all patients have specific drugs available and that there are HAE knowledgeable doctors in each area to improve the diagnosis rate of HAE. Also, we want to improve society's tolerance for HAE patients and improve their quality of life.



### HONG KONG

We want to see the approval of at least four more medications freely accessible to all patients as a special drug recognized by the government. We will continue to raise public awareness of HAE in terms of diagnosis and treatment.



### INDIA

Our next step is to get modern medication and create awareness throughout the country with the help from organizations such as the Organization for Rare Diseases India (ORDI).



### INDONESIA

Next for us is to find more patients and build the HAE Indonesia capacity to create more awareness of HAE – and for patients to get the right diagnosis and access to modern treatments.



## JAPAN

We have five aims:

- To build HAE Japan to better provide support to the patient community,
- To have correct diagnosing for above 80% of the theoretical patients in Japan,
- To ensure that patients have a high quality of life and are not limited by their condition given the current advanced treatment environment,
- To receive approval of self-medication for C1-INH,
- To ensure treatment options are authorized for patients so that they can choose the treatment that best fits their situation.



## NEW ZEALAND

We have no access to any funded prophylactic modern therapies, so we want to achieve this so all patients have a much better quality of life soon.



## PAKISTAN

We want to establish our association to provide modern treatments free of cost so all HAE patients would live a better life.



## SINGAPORE

Our aim is for all Singaporeans with HAE to get access to modern treatment via a medical fund or financial assistance.



## SOUTH KOREA

As we have only one HAE treatment, we will introduce additional drugs through our activities. Our organization will be activated as an official non-profit corporation. We will increase the diagnosis rate of HAE. We will promote and raise awareness of HAE so that there are no undiagnosed or untreated patients. We will focus on patient training to enhance HAE symptoms and awareness, and we will promote HAE to medical staff to help accurate diagnosis. Our overall aim is to put the safety of HAE patients first and minimize mental stress for the health of the mind and body.



## TAIWAN

In cooperation with Dr. Shyur, we hope to successfully introduce new drugs to the health insurance so that we can get away from steroids.



Central Eastern Europe and Benelux



## ARMENIA

We want to organize more seminars for patients and their caregivers with the involvement of HAEi, expand the number of members, enter clinical trials with new drugs, register modern drugs in Armenia, and initiate a process to establish an ACARE center.



## CZECH REPUBLIC

We want to make life with HAE as simple as possible for everyone. This could be with the help of a new treatment launched with the support of doctors, education of the specialist community and the raising of HAE awareness. We want to make sure HAE affects our lives as little as possible.



## GEORGIA

We want to be widely involved in clinical trials of HAE, find resources for the diagnosis and treatment of patients, and facilitate the registration and importation of modern medications.



## HUNGARY

We would like to achieve a status where children can self-administer medication. With the availability of modern medicines, we want each HAE patient to have the highest possible independence from the disease. We would also like all potential HAE patients to get an accurate diagnosis within one year after the first symptoms.



## NETHERLANDS

We would like to make the disorder known to all general practitioners, and that a definitive solution is found, we mainly think of genetic repairs. These are still in their infancy, but the reports are encouraging. We will continue to argue that HAEi will also represent the acquired angioedema forms.



## POLAND

By 2030, we would like HAE patients to have unlimited access to all therapeutic options registered in the European Union for on-demand treatment and long-term prophylaxis. We want each diagnosed patient to be able to administer the drug personally, regardless of the method of administration, to feel safe, feel independent and take complete control of HAE. We want all HAE patients in Poland to be diagnosed by 2030.



## RUSSIA

Our next steps are to strengthen, multiply and develop our achievements to date. We very much hope for great progress in the treatment of HAE over the next ten years.



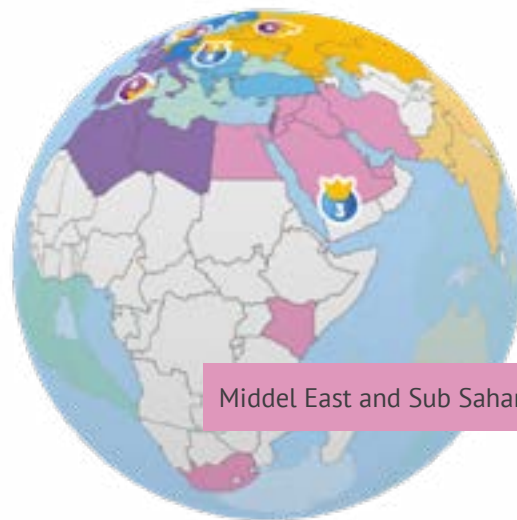
## SLOVAKIA

A campaign to raise the disease profile is currently underway, and sessions and articles are being prepared to help patients and professionals better understand the life of a person with HAE. We believe that the campaign will facilitate the demanding process of approving and registering effective medicines. Other activities will be aimed at making the lives of people with HAE more pleasant by sharing problems and experiences. We believe that the life of HAE patients in Slovakia will become simpler and better.



## UKRAINE

We want to find more patients, provide them with a sufficient amount of medicine and achieve the right to self-administration of HAE medicines. We plan to actively develop, share the experience with other countries, and participate in research on new methods of treatment.



Middel East and Sub Sahara Africa



## EGYPT

We want to ensure that the registration of medication for HAE treatment is completed and all HAE patients can access that medication. Also, we would like to increase the number of doctors familiar with HAE and ensure they know how to diagnose and treat HAE accurately. We will continue the journey of awareness to reach all parts of Egypt and provide full knowledge of the disease and how to diagnose and manage it.



## KENYA

We want to ensure that HAE patients can be recognized under disability and receive support as a result. We will work on getting emergency medications for HAE patients and increasing activities for our members and other stakeholders. We want to participate in public forums with key government institutions to ensure that our HAE voices are heard. This is to ensure inclusion in the Universal Health Care government scheme to make life-saving medications affordable for patients.



## LEBANON

We want to reopen the file with the Ministry of Health to secure the proper treatment for people with HAE and to grow the number of HAE knowledgeable and motivated physicians. We want to continue raising and increasing awareness of HAE, to help reduce the time that it takes for people to get an accurate diagnosis.



## SOUTH AFRICA

We want to achieve an improved response from the medical sector in better understanding HAE and ensuring that modern medication becomes available as a matter of priority.



## SYRIA

Our next step is getting access to modern HAE medications.





Mediterranean, North Africa and British Isles



## ALGERIA

Our main objective is the availability of emergency medicine, something we are actively working on with the health ministry.



## FRANCE

The next step is to find ways to decrease the prices of modern treatments that are today too expensive to cover the need of all HAE patients.



## ITALY

We would like to spread self-administration classes and the knowledge of HAE in the country, get fast diagnosis and access to the right medications and have more knowledge about other forms of angioedema.



## LIBYA

Our goals are first and foremost providing the right medication for HAE patients, providing an awareness platform in hospitals, private and public, and creating a specialized care center for HAE patients.



## MOROCCO

Our next step is that people with HAE are introduced to the required drugs, both for crisis treatment and for planned or urgent surgery.



## PORTUGAL

Our aim is to increase the number of members and their interaction with HAE Portugal. We want to get home treatment for the medicaments available in Portugal and increase the number of available medications as well as the number of hospitals with medication for HAE. We would like to see greater dissemination of HAE in society in general and among

health care professionals as many still demonstrate weak knowledge about what HAE is and how to act in acute attacks.



## SPAIN

We want people with HAE to have equal access to treatment and have greater consensus among treating physicians regarding the use and home availability of HAE medications all over the country.



## UNITED KINGDOM

We are working with HAE clinicians to recognize childhood attacks sooner. We hope that children with HAE will no longer have to miss school, and they can go on to college and university and have a life that is not limited by their condition. We have some great inspirational HAE patients who have achieved so much despite having HAE; they help us support our younger patients and encourage them not to let HAE stop them from achieving their dreams.



South Eastern Europe



## BOSNIA & HERZEGOVINA

Our next step is to continue establishing and registering the association, continuing testing for HAE, and raising awareness in the media. Our vision is to provide free testing and free therapy for all HAE patients in the shortest possible time. By receiving free therapy, we would restore dignity to our patients and improve their quality of life. We will continue to raise awareness amongst everyone about what HAE is, what the symptoms are, what therapy is appropriate and how to manage a patient with HAE. We want to help general practitioners to be able to promptly

identify and refer the patient to the right specialist to confirm the diagnosis and for them to receive therapy. The biggest challenge to deliver on our planned activities is only modest financial resources.



## BULGARIA

Our next important goal is to improve the access of HAE patients to therapies and, in particular, to HAE prophylaxis. Our goal is to collaborate more closely with the patient community, the authorities and health care professionals to improve access to treatments, especially in terms of prophylaxis with subcutaneous medication or any new oral therapy. We deeply believe that this will significantly improve patient compliance and will lead to positive lifestyle changes.



## CROATIA

Our aim is that we all have medicine available at home. Also, we want to secure disabled persons and benefited length of service status and faster diagnosis.



## CYPRUS

Our next steps are to make more medicines available and raise awareness among more people, especially doctors.



## GREECE

We want to get our organization recognized, make more social media postings to reach more people, and improve our website. We plan to hold meetings with doctors and patients. We strive to get more medicine approved for adults and children with HAE, find all HAE patients in Greece, and create awareness campaigns for medical professionals and the general public.



## MONTENEGRO

We would like to get several HAE medications approved for all patients through changes in the legal framework. We would like to work more closely with doctors and medical practitioners to treat HAE patients better and work with doctors on developing protocols for the treatment of HAE patients, especially children. We would like to create a database of HAE patients and increase public awareness of HAE.



## NORTH MACEDONIA

We have these aims towards 2030:

- Provide prophylactic treatments for patients who need it,
- Open an ACARE center with Dr. Vesna Grivceva Panovska where patients could come for periodic examinations and consultations,
- Disperse the distribution of medicines in cities where there are HAE patients, provide better information to primary care physicians in smaller towns on the specifics of HAE and the needs of HAE patients, and improve communication and collaboration between HAE patients and their primary care physicians.



## ROMANIA

Given that HAE is still underdiagnosed, our efforts will continue in the direction of raising awareness, both amongst doctors and the population. We will continue to advocate for the adoption of legislation recognizing HAE as a disabling disease so that patients can benefit from all legal rights. Unfortunately, there are also children under the incidence of this disease. That is why one of our goals will be to start psychological counselling programs for them.



## SERBIA

We want to raise HAE awareness to the level of recognition by most doctors. We would like to find as many undiagnosed patients as possible and provide prophylactic therapy for all those interested. Another mission is to decentralize therapy.



## SLOVENIA

We want to officially establish HAE Slovenia as a formal association that will make it easier to ensure emergency medicine is always available at emergency centers. Awareness and detection of potential HAE patients is also needed to prevent the worst. If we save one life with this, all the effort is rewarded.



## TURKEY

We want patients to be allowed to administer C1-INH and that home treatment is included within the scope of payment. We want to develop advocacy among our patients and reach more young patients enabling them to take a more active role inside HAE Turkey.

We want to increase advocacy activities in our adult patients to ensure that they take a more active part in HAE Turkey and to work together in all the activities we carry out throughout the country. We are always stronger together.

Nordics, Germany, Austria and Switzerland



## AUSTRIA

We will try to be more present in the media, in medical journals, etc., to draw attention to HAE. It is important to reach patients who do not know they have HAE, and for this, the clinical picture would have to become better known among doctors.



## DENMARK

Our focus is continued access to the wide range of HAE medicines, and that new HAE medicines will also be available to our patients. We want all patients to be offered prophylactic treatment so that no one has to wait for the “catastrophe” but can focus on living a life without fear of HAE attacks. Also, we aim to get ACARE implemented in Denmark and have strong treatment centers for HAE patients.



## FINLAND

We hope to get more information to our members about HAE as we believe there is a lot to be achieved by bringing HAE people together.



## GERMANY

We aim to make HAE even better known so that each affected person receives suitable therapy. We want anyone to be able to use medication at home and that all HAE patients have been found so there are no more unreported cases. Ultimately, our aim is that nobody has to die from HAE anymore.



## NORWAY

There is currently a possible change in the reimbursement policy, which might result in only a few options funded by the health care system. Thus, we want to achieve that all the approved HAE medications are still available to all patients.



## SWEDEN

We want to achieve that all patients have access to new and preventative treatments. We also want to hold many meetings, allowing us to exchange experiences and learn from each other. We want to increase awareness of HAE Scandinavia so that everyone who receives an HAE diagnosis or has a relative diagnosed with HAE is given the opportunity to join a strong and supportive patient organization.



## SWITZERLAND

We hope that by 2030, those affected by HAE will have a diagnosis much faster and that all HAE patients will become members of HAE Switzerland. That way, they can, as quickly as possible, get new information on HAE.



Start using HAE TrackR today and get a full overview of your HAE.

[app.haetrackr.org](http://app.haetrackr.org)



South America



Central America, Caribbean, Mexico and North America



## ARGENTINA

We would like to achieve a zero-attack pattern for our patients, as well as access to the greatest number of treatments available, tailored so they would allow our patients to lead an absolutely normal life. By 2030, why not also dream of the possibility of a cure?



## BRAZIL

We want to involve the patients more with our association, register all patients with HAE according to the Brazilian prevalence, better their quality of life with access to oral drugs for crisis control and a possible genetic modification in the defective gene.



## PERU

Our aim is that additional treatments enter Peru, including those suitable for children and pregnant women suffering from HAE. We want HAE drugs to be subsidized by the state and that patients get an early diagnosis. We want to achieve an adequate comprehension of treatment for HAE patients in the private and public sectors nationwide and to have HAE specialists in all departments of Peru.



## VENEZUELA

Our aim is that all patients have effective treatment with immediate availability. We would like to hold a world conference on Margarita Island, a place in the Caribbean anchored in our beautiful country. Here people from all over the world could come and share with patients with the same health situation.



## CANADA

The next steps are to welcome HAEi representatives to Canada to learn how to partner with upcoming projects, such as HAEi Connect. We want to help HAE patients with Normal C1 gain access to the variety of treatments that benefit patients with HAE Type 1 and 2.



## COSTA RICA

All of our efforts are focused on getting medication through social health and increasing the knowledge about HAE in the medical community.



## CUBA

The next step is to establish our organization legally. Then we will work on medical and social awareness about HAE and win the battle on the importance of guaranteeing access to advanced therapies for each patient.



## EL SALVADOR

By 2030, we expect to have more patients diagnosed with HAE and to be able to create a formal association. We want to have access to better treatments and raise awareness about the existence of HAE and the impact of misdiagnosis and ignorance. By having more patients with a registered diagnosis, we expect to hold meetings with doctors and patients at a national level to obtain information about the progress in the country and globally.



## GUATEMALA

The most important aim for us is to have a treatment for HAE patients.





## MEXICO

We aim to ensure that the majority of patients have access to specific HAE medications, whether they are entitled or not. We would like to have a national reference center for HAE. We want to see an HAE screening conducted at the national level to increase the percentage of people diagnosed with HAE since the disease is currently underdiagnosed.



## PANAMA

We hope that by 2030 all HAE patients have access to various treatments. We also hope that we have detected more patients because surely more people still do not know what they are suffering from.



## PUERTO RICO

We want people with HAE to have unrestricted access to modern therapies so they can experience life to the fullest and effectively manage their HAE. We want people with HAE to continue benefiting from HAEA's innovative programs and services.



## USA

By 2030, we want people with HAE to have unrestricted access to available modern HAE therapies so they can experience life to the fullest and effectively manage their HAE. We want people with HAE to continue benefiting from our innovative programs and services. We will expand our outreach, awareness and educational initiatives to narrow the gap between symptoms and proper diagnoses for people with HAE. Ultimately, we will continue to pursue a cure!

# 2030



**Access to medication**



**Identify HAE patients**

**Get better access to medication**  
**Identify patients for earlier diagnosis**  
**Organize seminars and events**  
**Establish member organization**



**Organize seminars/events**



**Welcome members**

**Welcome more members**  
**Enter clinical trials**

**Establish ACARE centers**

**Identify knowledgeable doctors/hospitals**

**Create awareness**

**Make use of HAEi resources**



**Create awareness**



**Establish ACARE centers**  
**Identify knowledgeable doctors/hospitals**



**Use HAEi Resources**



HAEi YOUNGSTERS

**CONNECTED BY HAE.  
UNITED AS A FAMILY.**

# It's time to meet the HAEi Youngsters Advisory Group

Hi everyone,

We hope you are all doing well and enjoying the fact that the world is starting to open up a bit, here and there and hopefully very soon everywhere!

We realize that some of you may be new to our global HAE community, and we thought it's a good time to tell you a bit more about the HAEi Youngsters' Community and Youngsters Advisory Group (HAEi YAG) and do a "round table" introduction of our current members.

The HAEi Youngsters Community is for both patients, caregivers, siblings and partners between the ages of 12 and 25.

Whether you are a newly diagnosed HAE patient looking for more information and support, starting at a new school and would like to know of other people's experiences or moving to a new country and looking for new connections, this is the place for you!

The Advisory Group comprises seven community members, and we all volunteer to be a part of it and carry on our responsibilities. Some of our key tasks are to oversee the direction and long-term objectives of the HAEi Youngsters Community, promote the work of the community, carry out different projects and social media campaigns, and last but not least, act as the contact point between the HAEi Youngsters Community and HAEi. We are happy to be working with Nevena Tsutsumanova, HAEi's Operations Manager, on the different activities and projects.

On the next two pages, you will find the current members of the HAEi YAG.

If you are interested in knowing more about the HAEi Youngsters Community, you can visit our webpage at [youngsters.haei.org](https://youngsters.haei.org).

We hope to see everyone soon! And until then, you are always welcome to reach out to us or send us an email at [youngsters@haei.org](mailto:youngsters@haei.org).

Stay safe, everyone!

Your HAEi Youngsters Advisory Group



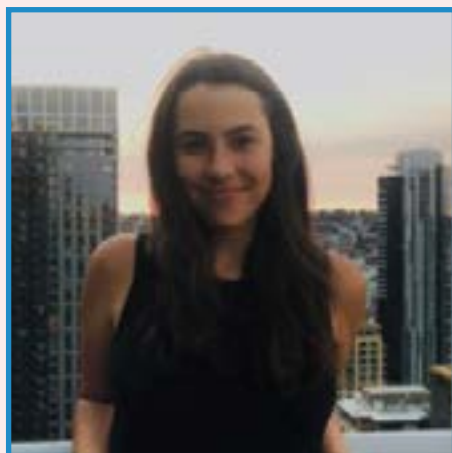
## Here we are, the current members of the HAEi YAG:



### Nanna, Denmark

Hi guys, my name is Nanna, and I am 19 years old, living in Denmark, Scandinavia. I am an HAE patient, and I was diagnosed when I was born, as my dad has HAE as well. This community really helped me having a positive view of HAE, and as a part of the advisory group, I really hope this community will do the same for you! I believe that we, in fellowship, can create a better quality of life for youngsters with HAE and young caregivers!

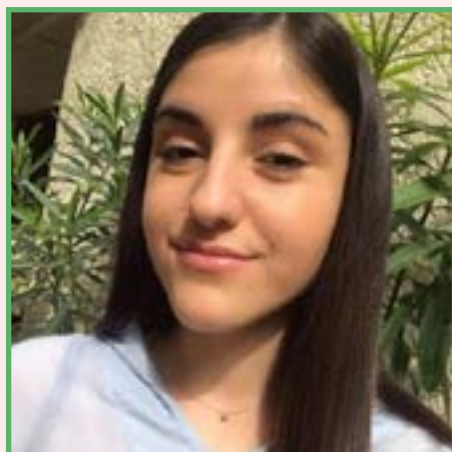
I would like to share my favorite quote with you: “Promise me you’ll always remember: You’re braver than you believe, stronger than you seem, and smarter than you think”, from Winnie the Pooh.



### Isabel, the USA

Hi all! I'm Isabel from the USA (though my mom is Chilean, I grew up in Costa Rica, and my family now lives in Victoria, Canada – so home, like this community, is a bit global). I really value the friendships I've made with the HAEi youngsters and am excited to contribute to making spaces to help you create some. I'm entering my 3rd year of university this fall (computer science/ data science).

Coronavirus-willing, I'll be living in Berlin, Buenos Aires, London and Taipei (or maybe San Francisco) in the next two years as part of my university degree, so if you're in or near any of those cities, I'd love to meet up!



### Eirini, Greece

My name is Eirini (or Irene in English). I'm an HAE patient from Greece, and I was diagnosed at the age of 12.

Being the only one in my family with HAE, it's been an amazing experience to get to meet other people and youngsters with the same disease. I have gained so much confidence and pride in myself, I found the courage to step up and raise awareness in my country and internationally.

I am part of the HAEi YAG, and I want to help create a strong community so no youngster feels alone or left out. HAEi is constantly working to create a better future for all HAE patients, and I'm proud to be a part of that.



## Nathan, Ecuador

Hi, I am Nathan. I am a patient from Ecuador. I recently graduated with a degree in marine biology and am interested in shark conservation and genetic research. Both my brother and father have HAE. I have many talents, including playing on the Ecuadorian national women's soccer team (before transitioning), teaching myself guitar, bass, ukulele and drums, and releasing a single in Ecuador, which was played on the radio. I am currently focusing on making art and composing music with piano.

I am greatly involved in the youngsters' community; after meeting so many youngsters at the summer camp in Frankfurt, Germany, I started appreciating HAEi as a family and seeing the importance of having an HAEi youngsters community, so I started advocating for it.



## Hana, South Africa

Hello HAE family! My name is Hana, and I'm from Cape Town, South Africa. I joined the HAEi community in 2018 after attending the HAE Global Conference in Vienna, Austria, where my life was forever changed by the amazing support and community. My struggles with HAE led me to study Medical Sciences to help research rare diseases and hopefully one day help future patients.

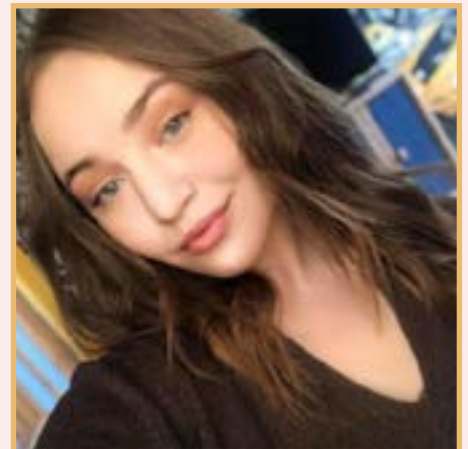
I love being part of this incredible and strong community, and I'm always inspired by all of you. Let's take control of our HAE together!



## Dominika, Poland

Hi, I'm Dominika, I'm an HAE patient from Poland, and I'm 23 years old. I was diagnosed when I was four. My dad, brother and sister also have HAE. I have been taking part in many national and international HAE meetings and conferences in my free time. HAEi helped me a lot in gaining confidence and in becoming a Regional Patient Ambassador in Poland.

Meeting so many youngsters in Frankfurt back in 2017 was an incredible experience. I was surprised to see that there were so many of us out there, but also very happy to know that there are people all around the world who know and understand what being a young person with HAE means. I knew right then and there that being an HAE advocate is what I want to do one day. I am very happy to start my journey by joining the HAEi YAG!



## Jacob, Canada

Hi, everyone – my name is Jacob Collins; I am a 14-year-old HAE patient from Canada. I am very excited to be a part of this global community, and I look forward to meeting you all in the future.

I am a high school student, and I love being active in sports, especially boxing! If I am not practicing my boxing techniques, I probably have my nose in a book. Some of my favourite books are: "Einstein his life and universe" and "Ali: A Life". Keep an eye out for all the exciting things coming on the website soon. Stay safe, everyone!





## Kick-starting the HAEi Youngster's blog



We are very excited to share with you that we are kick-starting our Blog again! We are so happy to present Pravalika from India. She is amazing, cheerful, and very passionate about photography. This is her story from the HAEi Youngsters Blog:

### **Where are you from?**

I am Pravalika from India :-)

### **What was your first involvement with the HAE Youngsters' Community, and why is the community important to you?**

My first involvement was during Christmas 2020. This community is particularly important to me because I can connect and relate to everyone here. We do live in different countries but have similar goals, like, to accept HAE as a part of our life and not let anything affect our goals and way of living. Not just survive but to live!

### **What is something interesting about you that will surprise us?**

One thing interesting about me is I have manyyyyy hobbies, and I always try something new. I do different types of art, I try new workout techniques, I am a tech geek, I've tried amateur photography, short story writing, and it's been good so far. I've killed enough

plants and reached a level that I can finally raise them on my own. And a few more (I really don't wanna bore you with stupid things I do in my week offs).

### **What does #BeyondHAE mean to you?**

#BeyondHAE means so much to me!!!

It's courage,  
It's motivation,  
and most of all  
It's us!

...going after the things we love, and not the condition we live with.

In spite of all the issues we have been facing and will in the future, we are so much more than that. After advocating so much for a rare disease at a young age, it becomes our identity, but #beyondHAE is anything but that!

### **If you could eat only one thing for the rest of your life, what would it be?**

The one thing I could eat all my life is anything that is cooked with a potato. It comes in all shapes and sizes!

### **If COVID-19 disappears tomorrow, what is the first thing you will do?**

I would run to my gym! I've been trying out kickboxing for a few weeks but had to stop because of rising COVID-19 cases!

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

The one thing that warms my heart and leaves a smile on my face is seeing a dog. I'm a huuuuge dog lover, but unfortunately, I don't have one.

**Wanna know  
more about the HAEi  
Youngsters and their  
Blog? Then have a look at  
[youngsters.haei.org](https://youngsters.haei.org)**

# 12TH C1-INH DEFICIENCY & ANGIOEDEMA WORKSHOP

— *yet another virtual event*



Due to the uncertain situation caused by the COVID-19 pandemic, the 12th C1-Inhibitor Deficiency and Angioedema Workshop was held online on 3-6 June 2021. The command center of the workshop was the studio of Diamond Congress, with Professor Henriette Farkas and Senior Research Scientist Lilian Varga once more as the main organizers.

As Professor Farkas said in the opening of the conference, “a virus that only has a couple of thousand bases and does not have a DNA cannot interrupt our 20-year-long tradition; we must keep our community, our HAE family, together”.

The organizers tried to see the positive side of the changed circumstances and use the opportunities modern devices present, with Diamond Congress providing professional help. In addition, the main sponsors CSL Behring, KalVista, Pharming, Pharvaris and Takeda, as well as the sponsors Biocryst and Biomarin, provided financial support, which made conducting the event in high quality possible.

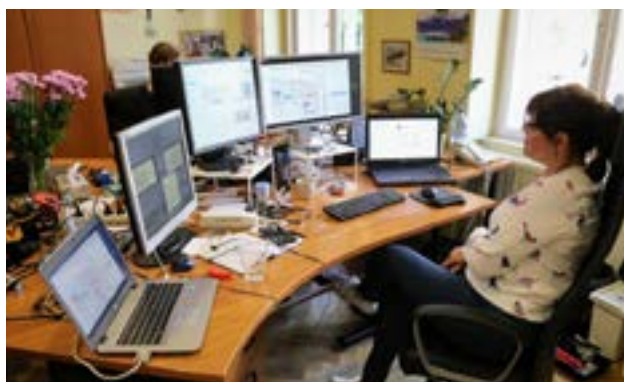
“The organizers believed in the mystic power of 12, and looking back, it seems like it was a good omen because a record number of 438 people connected to the conference, from 51 countries. We can declare that the four-day-long conference has lived up to the audience’s expectations, and just as in previous years, we could participate in a high-quality professional congress. The scientific program consisted of seven oral and two poster sections, where 35 lectures and

21 posters were presented. All the abstracts evaluated by the Scientific Committee will be published in the highly esteemed scientific journal *Frontiers in Allergy*”, says PhD student Zsuzsanna Balla from the Hungarian Angioedema Center of Reference and Excellence at Semmelweis University in Budapest, Hungary.

The conference was opened by Professor Attila Szabó, the Vice-Rector for clinical affairs, who emphasized that “the equation in the 21st century is science + information = power”. Under this context, basic, clinical, and translational research should be used to improve the health and quality of life of people around the world.

One of the opening day highlights was the awarding ceremony of the “for HAE Patients” award. The lifetime achievement award established in 2003 is given by the Scientific Committee to a colleague who has contributed to understanding the processes causing angioedematous diseases with their research and has done outstanding work for the patients. This year, the winner was Clinical Professor of Medicine Allen Kaplan, presented by HAEi’s President, Anthony J. Castaldo. After this, Professor Kaplan presented his work in an outstanding, impressive, and comprehensive presentation titled “Kininogen(s) structure and function”. It was a touching moment when Allen Kaplan logged in live, and the organizers welcomed him from the studio in Budapest with a surprise cake and champagne, which helped in reducing the social distance caused by the pandemic.





The complete recorded material of the 12th C1-Inhibitor Deficiency and Angioedema Workshop is available on the website of the conference until August 2021 – please see <https://2021.haenetworkshop.hu>

This time, six prominent invited experts talked about the different aspects of angioedemas:

- Daniel Jacobson, Chief Scientist for Computational System Biology of the Oak Ridge National Laboratory in the USA, spoke on a method based on systems biology and artificial intelligence that could prove that the bradykinin-storm is the cause of several symptoms of COVID-19, and therefore the medications already authorized for the treatment of C1-INH-HAE could potentially be effective in treating COVID-19 patients.
- Marcus Maurer, the Dermatologist and Allergologist Professor of Charité University Hospital in Berlin, Germany, presented the global initiative GA<sup>2</sup>LEN / HAEi ACARE with the purpose to treat and defeat the global challenges of HAE, to abolish global inequalities, and to review and update the current guidelines about HAE.
- Coen Maas, who is a Professor at the Department of Clinical Chemistry and Haematology at the University Medical Center Utrecht in the Netherlands, presented results according to which the closed conformation of FXII is critical to prevent detrimental activation. Any mutation that prematurely disrupts it has a chance to result in a thromboinflammatory disease.
- László Cervenak, a Senior Researcher at the Department of Internal Medicine and Haematology at Semmelweis University in Budapest, Hungary, emphasized that new factors, besides the old ones, surfaced in the pathogenesis of angioedema and that endothelial cells play a key integrative role in the development of angioedemas.
- Francois Alhenc-Gelas, Research Director of INSERM in Paris, France, presented evidence that indicates that the clinical development of kinin receptor agonists, especially of B1R, is warranted with the aim of cardiovascular prevention, particularly in diabetes.
- Konrad Bork, Dermatologist Professor at the Johannes Gutenberg University of Mainz, Germany, presented the developing constellation of genes involved in the pathogenesis of angioedema.

“The scientific program was very diverse. Regarding the treatment of HAE, we learned about the new bradykinin B2-receptor antagonist PHA-022121, a Factor XII inhibitor monoclonal antibody called garadacimab, which could be administered subcutaneously for long-term prophylaxis, and another new oral medication for the treatment of acute HAE attacks, the small molecule KVD900, which is a plasma kallikrein inhibitor. Colleagues presented about genetic studies and new gene therapy methods; the possibilities of measuring

patients’ quality of life, experiences with different treatments; prodromal symptoms; and we could continue listing the long line of interesting topics”, says Zsuzsanna Balla.

Being together in person cannot be replaced. Still, the organizers tried to add some items to the program that provided an opportunity to relax a bit and, casually, cheerfully be together. Making the event more colorful was a QR code hunt and virtual sightseeing in Budapest, where the participants could ask questions from the tour guide. For the virtual gala dinner, everybody prepared the “mutual” gala menu, and ate it “together”, and could compete for the title of “best chef”. A spectacular sand animation inspired by different melodies was performed by Ferenc Cakó, a multiple international award winner Hungarian graphic, who was also part of the opening ceremony. A short film about the history of the conference presenting how the first C1-inhibitor workshop has evolved into a world-class event evoked cheerful memories.

“HAE family members were saddened by the fact that three giant scientists of the HAE community have passed away. We remembered Michael Frank, Marco Cicardi and José Fabiani with sadness. It is a tremendous loss for the HAE community, but the memories in our hearts and souls and their scientific work will last forever and provide a solid base for further research”, says Zsuzsanna Balla.

True to the traditions of the previous years, four researchers younger than 35 – Aycan Aşık, Francisca Vílchez-Sánchez, Nina Rupar and Zsuzsanna Balla – received the “Grant For Young Investigator” award, which goes with a 2,500 EUR scholarship per capita.

Professor Anastasios Germenis summarized the essence of the conference in his excellent closing remarks. As Avner Reshef, the winner of the “Most active participant” prize wrote at parting: “Despite the pandemic, despite the critics, despite the sceptics, despite the distance, the workshop proved that science can unite people who are eager to work together toward a common goal.”

Zsuzsanna Balla emphasizes that it was very good to be together with the big HAE family, where the motto is: we are together, we remain together, we will be together:

“Let us hope that the number 13 will bring luck, and we can meet in 2023 in Budapest in person.”





# WELCOME TO THE 2020 HAEi VIRTUAL REGIONAL WORKSHOP CENTRAL EASTERN EUROPE & BENELUX



*By Michal Rutkowski, Regional Patient Advocate for Central Eastern Europe, Benelux and Middle East*

It has become a tradition that each year October brings the largest annual gathering for the HAEi community in Central Eastern Europe and Benelux. Part of the tradition is that Poland hosts a yearly regional advocacy event dedicated to patients, caregivers and physicians constantly working in awareness, diagnosis, access and reimbursement to make HAE patients' lives better and easier. These regional workshops have always been great opportunities to meet and greet the newcomers to the HAEi world and share experiences with other members of the global HAE family. Here unbreakable bonds have been forged, which continue to this day.

Due to the COVID-19 pandemic, HAEi was unfortunately forced to cancel the 2020 workshop in the form in which it had been held previously. However, the promise HAEi gave to the patients, and the importance of regional advocacy efforts made the global organization decide to organize a virtual edition of this event.

The virtual world gives us more opportunities, especially to reach those patients who have not been able to participate in such meetings or those who have encountered a language barrier. There is indeed absolutely no substitute for a face-to-face meeting, but at least with today's technology, we are able to continue our hard work. And as we all know, hard work pays off.

Therefore, we rolled up our sleeves and got down to keep the promise and deliver a virtual workshop. We started our activities in September 2020, and it turned out quite quickly that when it comes to organizing a virtual event is much more complicated than a traditional one. For many weeks several people from the HAEi team met regularly to discuss the work progress and jointly solve the obstacles encountered. The prepared program turned out to be extremely ambitious as well as very demanding in preparation. The assumptions made at the beginning of reaching as many patients as possible required the preparation of presentations and videos in 11 languages: English, Armenian, Czech, Dutch, French, Georgian, Hungarian, Lithuanian, Polish, Russian, and Slovak. Only in this way could we ensure that the prepared content would reach and be understood by all patients, caregivers and physicians, including those who only speak their native language.

The 2020 keynote speakers for the regional workshop were:

- HAEi Leadership members: Fiona Wardman (Chief Regional Patient Advocate), Anthony J. Castaldo (President and CEO) and Henrik Balle Boysen (Executive Vice President and COO);
- HAEi member organizations from Belarus, the Czech Republic, Georgia, Hungary, Kazakhstan, Lithuania, the Netherlands, Russia and Ukraine;
- HAEi Youngsters Advisory Group member Nanna Boysen;
- Professor Henriette Farkas, MD, PhD, DSc, HAE Center at Semmelweis University in Budapest, Hungary;
- Professor Markus Magerl, MD, Department of Dermatology and Allergy, Charité University Hospital in Berlin, Germany;
- Professor Marcus Maurer, MD, Clinic for Dermatology, Venerology and Allergology, Charité University Hospital in Berlin, Germany;
- Professor Marc Riedl, MD, MS, US HAEA Hereditary Angioedema Center at the University of California San Diego, USA;
- Marcin Stobiecki, MD, PhD, National HAE Center at the University Hospital in Krakow, Poland.

The workshop topics were divided into five sessions: Welcome, HAE International, Experts, Patients and Youngsters.



**Session 1 (Welcome)** was a message to all attendees from HAEi Leadership and the Regional Patient Advocate. Fiona Wardman, Anthony J. Castaldo, Henrik Balle Boysen, and I had a brief introduction and welcome to the followers of the workshop.

**Session 2 (HAE International)** was a presentation of the changes taking place within HAEi's structure, the aim of which is to create the best possible environment for patients, to accelerate a correct diagnosis process, and to reach access to modern HAE treatment options:

- Fiona Wardman introduced HAEi's decentralized approach, what it is, and why the organization has decided to develop it. Fiona acknowledged the

importance of the Regional Patient Advocate program, its values, and the general recipe for success. Also, she underlined what the Regional Patient Advocate mean for individual patients and their families. Furthermore, Fiona explained the Regional Advisory Groups (RAGs) and the Regional Medical Advisory Panels (RMAPs), practical solutions that that helps HAEi to understand the current needs and challenges in the countries from both the patients' and the physicians' perspective. She also introduced some of the tools available for HAEi member organizations.

- Anthony J. Castaldo and Henrik Balle Boysen explained how HAEi has built the foundation to ensure patients a better future. Tony highlighted that most of all HAEi is a company and product neutral non-profit organization working with anyone who steps up and makes an investment in developing a lifesaving HAE medicine. Tony and Henrik presented a few of the latest HAEi tools, including HAEi Advocacy Academy, HAE Companion, and ACARE, as well as HAEi Research tools: Pharmacoeconomic and Quality of Life Assessment, Baseline Burden of Illness Survey and Heat Map Survey. Finally, Henrik introduced the new approach to HAEi's meetings: HAEi Regional Conferences and HAEi Global Leadership Workshop.
- Anthony J. Castaldo and Marcus Maurer focused on Angioedema Centers of Reference and Excellence (ACARE), a joint venture of HAEi and GA2LEN, a non-profit organization of leading clinical and research facilities in the field of allergy and asthma.
- I had the privilege to present resources that HAEi offers to its members. I explained what the specific tool is about and how it can benefit both the organizations and individual patients.

**Session 3 (Experts)** was a true display of knowledge of four outstanding HAE expert physicians, who, in words understandable to patients and their relatives, explained important issues related to HAE:

- Marc Riedl presented the background of HAE, where he focused on explaining what HAE really is, what causes HAE, what the symptoms are, and why it is so difficult to diagnose it, and the burden of the disease. He also gave a pure history lesson introducing the pioneers in HAE, people who first described the symptoms.
- Marcin Stobiecki explained in detail how to approach the correct diagnostic process with HAE. He discussed the available diagnostic methodology: blood tests and genetics, reference ranges, as well as the importance of patients' family history.

- Marcus Magerl presents the recommendations from the international guideline for the management of HAE, which e.g recommends that all HAE attacks are considered for on-demand treatment, and that treatment is initiated as early in an attack as possible. Furthermore, he described on-demand and long-term prophylaxis treatment options.
- Henriette Farkas presented the specificity of the disease in children and women. She highlighted similarities and differences in symptoms, attacks frequency, and trigger factors. She also focused on the best available treatments for different types of patients (pediatric and pregnancy).

**Session 4 (Patients)** belonged to the leaders of HAEi member organizations who were interviewed, and answered questions on the current state of management of HAE in their countries, and advocacy activities they run to improve the situation for HAE patients. There are nine videos with patient leaders/supporters from Belarus, the Czech Republic, Georgia, Hungary, Kazakhstan, Lithuania, the Netherlands, Russia and Ukraine.

**Session 5 (Youngsters)** presented by Nanna Boysen was an introduction to the community dedicated to young people living with HAE. Nanna shared the information about the role and the responsibilities of the HAEi Youngsters Community and invited everyone to join and become part of the group.

The 2020 HAEi Virtual Regional Workshop Central Eastern Europe and Benelux was launched on 12 December 2020. To make it happen, a total number of 22 videos had to be recorded, and with the translation into 11 languages, 242 file versions were produced in just 12 weeks. This workshop would not have been possible if it were not for the long and hard work of the entire HAEi Team.

We hope that this solution introduced by HAEi has allowed many new patients, caregivers and health care professionals from the region to learn more about the disease from the top HAE scientists, experts and patient advocates. The interest that accompanied this project and the feedback from patients show that great work was done.

**You can watch the 2020 HAEi Virtual Regional Workshop Central Eastern Europe and Benelux at [haei.org/cee](https://haei.org/cee)**

# SEVEN MORE CENTERS HAVE JOINED THE ACARE NETWORK

The ACARE centers – a joint venture between the Global Allergy and Asthma European Network GA²LEN and HAEi to fulfill HAEi's longstanding goal of establishing a worldwide network of accredited angioedema care centers – continues to grow in numbers.

"In the March 2021 issue of *Global Perspectives*, we listed 56 ACARE centers in 28 countries. Since then, another seven centers have been added to the list – and two of them in new countries", says HAEi Executive Vice President and COO Henrik Balle Boysen.

With the addition of the most recent centers, the list now counts 63 ACAREs in 30 countries.

If you would like to become an ACARE center, please use this link for further information:  
[haei.org/acare](https://haei.org/acare)

The ACARE centers added most recently are – with the updated total number of centers in the country in brackets:

## **ARGENTINA (5)**

- Centro Médico Vitae, Buenos Aires
- Instituto de Asma, Alergia y Enfermedades Respiratorias, Corrientes

## **AUSTRIA (1)**

- Department of Dermatology at the Medical University of Vienna

## **BRAZIL (9)**

- Clínica Médica, Faculdade de Ciências Médicas at the Universidade Estadual de Campinas

## **GERMANY (7)**

- Department of Dermatology at the University of Erlangen

## **POLAND (4)**

- Department of Internal Diseases, Opole

## **USA (1)**

- Department of Asthma and Immunology at the Penn State University, Hershey (PA)





## RAPID DEVELOPMENT IN THAILAND

Known for its tropical beaches, opulent royal palaces, ancient ruins, and ornate temples displaying Buddha figures, Thailand is a wonderful country to visit in Southeast Asia. Thailand is also the home of HAE Thailand, a young organization that is working closely with HAEi as it rapidly expands its membership and advocacy efforts..

HAE Thailand is a great example of a newly established organization exploring and implementing HAEi's resources available for member countries.

"We are very excited to have launched a new website that was made possible by HAEi's web hosting services. HAE Thailand is now much easier to find on the Internet, and together with our Facebook group, we can now better maintain a relationship with our members, supporters and people interested in contributing to our work", says Suchitta Kengtanyagarn.

In today's online world, having a website for your member organization is crucial. It's a place where visitors can find out all they need about HAE and your organization's advocacy work and aims.

"Next on our "to do" list is implementing HAEi Connect, as it will help us keep our members' data secure and allow us to communicate with them in a faster and more efficient way. We are also interested in being part of the HAE Heat Map Survey, which will help us and our physician advisors estimate the number of people with HAE in Thailand", says Suchitta Kengtanyagarn.



Suchitta Kengtanyagarn is the international contact person for HAE Thailand. She lives in Bangkok and is happy to share the story of HAE Thailand and the young organization's goals for the future:

"HAE Thailand is currently in the process of being formalized as an organization, and we are very active in raising awareness among doctors, authorities, and the general public."



HAE is still a relatively unknown rare disease in Thailand, and the number of diagnosed patients is low. However, HAE Thailand is actively working on creating awareness, supporting patients in the country, and most recently helped translate the HAEi Emergency Card in Thai. The organization's first official meeting was held around **hae day :-)** 2021.

Thailand has an ACARE center and three hospitals with knowledgeable physicians, which will improve HAE diagnosing and treating in the future.

“There hasn’t been much advocacy work in Thailand”, says Suchitta Kengtanyagarn, “and it is hard to make patients and relatives understand the benefits of working together for more awareness. We do, however, believe that our cooperation with the medical team and the implementation of the tools and services from HAEi, will help us on the path for faster diagnosis, access to, and reimbursement for modern HAE therapies.”





# PATIENT STORY





*Patient story: Cecilia Ramirez, Argentina*

# I WAS SEARCHING FOR ANSWERS THAT MAY NOT HAVE HAD A QUESTION

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During my adolescence, I didn't have that many HAE attacks. My first episode was when I was 16 years old. One was in my face, and the others were confused for gastrointestinal problems, so in that sense, I guess I had quite a normal life at that point. Problems really began when I was about 24. I started to have more frequent attacks, and after I had given birth, I had attacks every week until I was diagnosed.

When giving birth, the doctors had to perform a cesarean operation because I had terrible abdominal pain. They found unusual liquid inside me and told me to look for information myself as they could not come up with a cause for this reaction. At a later point when I had a glottis crisis, I came across the website of HAE Argentina and thought that HAE might be what I was suffering from. I went to see Dr. Fabiani, who eventually diagnosed me. Actually, years before that, another doctor had asked for the same test that diagnosed me, but unfortunately, the lab results were wrong, so there was no diagnosis back then. During the 20 years or so when I wasn't diagnosed, doctors and health care professionals always said it was an allergy or nerves.

*Lacking the right diagnosis, would you say that at least you met understanding from people around you?*

Well, some people didn't believe me when I said my abdominal attacks were so painful, but they are not part of my life now. I must say that most people have at least tried to understand. At work, they never said anything when I was absent due to attacks, and my friends have indeed helped me a lot.

My family – that's another story. You know, without my parents, I wouldn't be on Earth, neither would I have had the experiences I had in life. They say your soul chooses your parents, and mine chose them both. Central to my life is also my sister, with whom I have shared my days since I was four years old. Above all, I must express my gratitude to my husband, whom I met in the 1980s and next to whom I have gone through life since then. He is the being who has made sure I always have my medication and who has spent many nights awake next to me, at home or in hospital, when I suffered the consequences of having HAE. He is also the man who let me fly free and with whom I have built a bond based on companionship and mutual respect.

*And then there is your son – the only other member of your family with HAE.*

Yes, Nicolas has inherited HAE, which makes me feel very sad as it is a legacy, I would have preferred not to pass on to him. But at the same time, my son, my



life, fills me with light day after day. He gives me the strength to go on when things do not go well, and I want to transmit to him the clearest and most positive message: I hope my story of personal growth helps him not go through the things I had to undergo. He is my greatest motivation and the one who has made me feel the most unconditional and purest love of all. I expect my message to be optimistic, and if someday he has to face the symptoms, I hope he can go ahead as I am learning to.

Talking about family, I will also mention my grandparents, especially Julio, who was the clearest example that one cannot change what happens, but you can change how you react to it. He didn't have HAE but had to fight with other health problems and gave me a great example of not quitting or ever being pessimistic.

All in all, I have the greatest wealth of all: Love and friendship. My chromosome may be broken, I may not produce human esterase, but I have a great capacity to give and receive unconditional affection.

*Tell me about your search for knowledge about HAE.*

Before I was diagnosed, I went to all kinds of workshops, looking for a cure. I see now that I was searching for answers that may not have had a question. That went on until one day I told myself I had to stop trying to escape and learn to live with what I had. It was not easy, but today I feel I may have achieved it. I do not promise that I will not feel weak sometimes, but some things are apparent to me. First of all, we have to accept

what happens and take care of our quality of life. And secondly: We should collaborate with others because together we always achieve more. HAE International, as well as national patient organizations around the world, prove that. Maybe one day, science finds a solution. Meanwhile, we should focus our energy on our requests to get every patient a diagnosis and a treatment without difficulties. We must also remember that although we cannot change what happens to us, we can transform our reactions and how we act by facing facts. To be successful, we need to be informed and look for a physical, psychological and spiritual balance.

*Is giving up the search part of the solution?*

No, that is not the point. It is not about giving up. On the contrary, really. I feel that I have learnt to live and value life, to see things from another perspective – and if HAE hadn't woken me up maybe today, I would be "only surviving" instead of "living life to the fullest".

*Could you elaborate on that?*

Every day I hear healthy people complaining, literally attaching themselves because of things that are easily changed, victimizing themselves for little stuff, not realizing the time they waste doing so. If I hadn't faced reality, I wouldn't have learnt so much about life, death, health, sickness, love, friendship and happiness.

One day I realized that what I learnt as a teacher couldn't be separated from what I learnt as a patient. Medicine and education have much in common. In psychology, we studied emotional intelligence and how it affects learning, and it was inevitable to see how it influence the health area. Working on emotions is crucial. In my case, it was essential to know how my emotions interacted with my disease.

*In 2016 you published a book in Spanish about your experiences with HAE. You have since written it in English. Why is the book called "Heroes Appear Everywhere"?*

When one has to live through extreme situations, hypervigilance grows. In the beginning, one lives life with so much uncertainty that it becomes very difficult to see things with clarity. You may spend whole days thinking "why" and "what for". As in a tale, the fantasy that a hero will come and take you out of your suffering appears. And there one remains sitting.

In tales, everything seems easy, but real life is another thing. It is not about kissing frogs to see if a cute prince turns up. It is about advancing along a labyrinth that sometimes is very dark and confusing, other times not so much. Unlike epic stories, there is no hero with a cape and sword but human beings who are common and go along life looking for answers. So, one day one wakes and realizes that the real heroes are each one of us and what we do with what happens to us. It is not about sitting around waiting for someone to find a cure or for a miracle to change our destiny. It is about facing the rare disease with what is called resilience.

but they are made all the same. A doctor taking care of a patient after a 24-hour-duty, a mother next to her son as he recovers from an illness. Perhaps there are no cameras on, but many people in their daily work display virtues and do deeds that maybe others do not recognize or understand.

Sometimes, ignorance is such that it takes you so long to find the right way that although you do not leave your country as an exile, you may possibly exile at home, on the road from clinic to clinic – and once you learn to deal with treatments and to face your own ghosts, you may

Feel free to download Cecilia's book in Spanish or English from [haei.org/cecilias\\_book](http://haei.org/cecilias_book) – please scroll to the bottom of the page to find the link.



### *In what way do you mean resilience?*

Yes, that capacity you have but do not fully know about until a crisis comes, and you discover that you can overcome physical and emotional pain as well as the fear of death. This leads you to concentrate on the positive capacities and attributes more than on the pathology you suffer.

Look up “hero” in a dictionary. It will most likely say that it is “a person admired for their deeds and virtues, a person who fulfills a heroic action”, but if we keep on researching, we can also read that on occasions, the word is used to refer to “a person who saves others from great danger, producing a noticeable or high-profile event”. I think all and each of us may be a hero in this sense; there are heroic actions that are not seen,

return strengthened. As regards morphology, those who suffer from HAE know that in our DNA, there is a “failing” chromosome, an uncommon one. Out of the ordinary. Maybe it is not like the heroes in movies, but when we make the decision, we start doing singular things in our search for better health and a better quality of life.

### *Do you have a personal experience with someone you'd call a hero?*

Often reaching a diagnosis is a slow and difficult process, above all, if there is no medical record of the disease in your family. That's where the hero might turn up. In my case, it was in the form of HAE Argentina and its President, Alejandra Menendez. To me, she





is a true heroine as she one day decided to form the patient association that she still presides to fight for a better quality of life for Argentinians with HAE. And even though she says that she only started it and that patients help her, those who collaborate with her as best we can know Alejandra is a pillar in this fight because she is working nonstop to help us all to live better lives.

*Being a hero – or heroine – also has to do with courage, hasn't it?*

For sure. The courage to face life is one of the most obvious characteristics of a hero, but you couldn't call someone a hero if everything scared you or if you didn't go through any adventure. The hero always has enough courage to face life problems and her or his own destiny. People with rare diseases know what fear is. Fear of a crisis, fear of not getting the medication or having enough money or proper insurance for the medication they need, fear of their kids inheriting

the disease, fear of the unpredictability they face day after day. That is why they learn to live a day at a time – and little by little, they find the courage that they need to face the present and have some adventures on occasions. They are afraid of going far from home or not getting someone to help them if they don't feel well. Other times, if they haven't found the cause of the swellings, they feel afraid of being hospitalized, not knowing what is happening. But, little by little, and with your diagnosis in hand and the medication in your refrigerator or bag, you find the courage. And then you risk going ahead step by step.

It is said that if, after the battle, the hero returns home, he doubts the pre-established values, the social order, and he can, directly or indirectly, change or break those rules to improve society. We can compare that to the time when patients get together to fight for their rights, to demand a better quality of life.

*So, patients resemble heroes?*

Not only do patients resemble heroes, but also their families as they stay by them and help them get through hard times. That shows love, patience, understanding and empathy.

Speaking of heroes, we mustn't forget the doctors who devote time to investigate and treat us. Those doctors who give their mobile phone number to their patients and are available 24 hours a day, seven days a week, 365 days of the year. HAE made me go through hard times but also led me to meet excellent doctors and nurses with great vocation – there is good reason to thank each of them for their work, support and understanding.

*Are you a heroine?*

Maybe I am an oyster? A colleague once said that she imagined I was like an oyster in which a grain of sand – here in the form of HAE – had entered, and it had become my pearl. I think she might be right. My life changed a lot and, despite what happened, I can say that I have learnt so much looking for a way out that perhaps I wouldn't change what happened to me if I had the chance. I suffered for a long time. I complained and asked “why me”, “why my son” – but I think that I do not need to know the reason as I found it. The pearl.



*You mentioned HAE Argentina. Are you involved with the organization?*

Yes, all the time, I try to help Alejandra as much as I can. I go to the meetings she asks me to attend, I help organize events, and if a patient needs to talk, I'm always ready to help.

Together with fellow patients from Argentina, I participated in the 2018 HAE Global Conference in Vienna, Austria. I learnt a lot, apart from meeting so many people from around the world who made me feel less alone with my condition. It was also great to meet and listen to so many professionals.

Indeed, the people of HAE Argentina – all the patients and their relatives I have met – have been important for my evolution when facing HAE. We share something maybe only we can fully comprehend, and I'd like to think that they know that they can always count on me.

## CECILIA'S STORY IN BRIEF

- Born 1966 in Buenos Aires, Argentina; living in San Antonio de Padua, Buenos Aires.
- Married, one child.
- Teacher of English from Institute Rojas in Moreno; Bachelor in Education from Universidad del Salvador, Buenos Aires – also a self-esteem practitioner and emotional intelligence coach.
- Working at a teacher training college teaching how to teach English, teaching private students, delivering conferences and workshops on methodology and emotional intelligence.
- First HAE symptoms: 16 years old. Diagnosed 20 years later.
- Other HAE patients in her family: Son.





# WELCOME TO THE 2021 HAEi VIRTUAL REGIONAL WORKSHOP SOUTH AMERICA & MEXICO CENTRAL AMERICA & CARIBBEAN



You can access all presentations at [haei.org/latam](https://haei.org/latam)



The biannual HAE Global Conference has been an excellent opportunity for people with HAE to meet, exchange experiences, and learn from each other over the years.

Following the 2018 HAE Global Conference in Vienna, Austria, organizing a workshop for fellow Latin American patients was a must. Originally the plan was to hold the first-ever HAEi Regional Workshop for South America and Mexico, Central America and the Caribbean as an in-person event. However, due to the global COVID-19 pandemic, it was decided to go with a virtual meeting instead.

The 2021 HAEi Virtual Regional Workshop for Latin America was launched in March and was a great success. The 34 videos available on the workshop website featured presentations from experts, member organizations, and youngsters, all recorded in the local languages.

“Our goal was to make this regional workshop a welcoming and inclusive event, which is why each video has subtitles in Spanish, Portuguese, and English for our global audience. That certainly was a fun challenge for the team”, says HAEi Regional Patient Advocate for South America and Mexico, Fernanda de Oliveira Martins.



The four tracks of the virtual workshop – HAEi, Experts, Youngsters, and updates from Member organizations – creates a brilliant overview of the current HAE situation in the region. The workshop is opened with welcome messages from the HAEi Leadership and the two Regional Patient Advocates, Fernanda de Oliveira Martins for South America and Mexico and Javier Santana for Central America and the Caribbean, who worked tirelessly in putting this event together.

In the HAEi track, viewers can find presentations from President and CEO Anthony J. Castaldo and Executive Vice President and COO Henrik Balle Boysen on the topic of “Building foundations for a better future”, while the Chief Regional Patient Advocate Fiona Wardman talks about “HAE International’s Decentralized Approach in Practice”. The track also introduces the topic of ACARE centers (Angioedema Centers of Excellence and

Reference) with the special participation of Professor Marcus Maurer and Anthony J. Castaldo. Member organizations and patients can also learn more about HAEi’s services and resources like Emergency cards, HAEi hosted websites, and HAEi Connect, that are available for member countries.

The Experts track contains four presentations from leading HAE physicians based in Latin America:

- “Diagnosis of HAE” by Dr. Olga M. Barrera from Clínica Hospital San Fernando INASA in Panama City, Panama
- “Clinical manifestations of HAE” by Dr. Margarita Olivares Gomez from Alergolica Clinica in Medellin, Colombia
- “Treating HAE” by Dr. Anete Grumach from the ACARE center Faculdade de Medicina do ABC in São Paulo, Brazil
- “The importance of ACARE centers – case of Peru” by Dr. Oscar Calderón from the ACARE center Clínica SANNA el Golf in Lima, Peru

Drs. Olga M. Barrera, Anete Grumach and Oscar Calderón also participates in a Q&A session.

In the Youngsters track, HAEi Youngsters’ Advisory Group members Nathan Galarraga and Isabel Brunkan introduce the youngsters community and talk about exciting upcoming projects to look out for.

In the final track, all 17 member organizations introduce their work and briefly talk about the current situation in their country:

- Argentina: President **Alejandra Menendez**
- Brazil: President **Raquel de Oliveira Martins**
- Chile: President **Lorena Merino**
- Colombia: President **Jessika Torres**
- Costa Rica: Leader **Angie Leiton**
- Cuba: Leader **Rosa Maria Perez González**
- Dominican Republic: Leader **Caroll Batista**
- Guatemala: Leader **Samantha Alvarado**
- Ecuador: President **Edison Galárraga**
- El Salvador: Leader **Raquel Fuentes**
- Mexico: President **Sandra Nieto**
- Panama: Leader **Muhammad Rawat**
- Paraguay: President **Nathalia Portillo**
- Peru: President **Carla Goachet**
- Puerto Rico: Leader **Ianice Viel**
- Uruguay: President **Natalia Vidal**
- Venezuela: Regional Director **Mary Bocaranda**

# NEWS FROM MEMBER ORGANIZATIONS AROUND THE GLOBE



## EGYPT

HAEi has registered two HAE knowledgeable hospitals for the world map. They are the Children's Hospital under the Ain Shams University in Cairo and the Alexandria University Children's Hospital. Also, there is an additional HAE knowledgeable physician in Egypt: Wala Mohamed Shoman, Consultant of Pediatric Immunology and Rheumatology at the Alexandria University Children's Hospital. For contact information please see <https://haei.org/hae-member-countries/egypt>.



## LITHUANIA

The Vilnius University Hospital – Santaros Clinic has been registered as an HAE knowledgeable hospital on the HAEi world map. Please see contact information on <https://haei.org/location/hospital-vilnius-lithuania>.



## ARMENIA

Three HAE knowledgeable physicians have been registered on the HAEi world map. That is Armine V. Hakobyan (Head of the Allergy and Immunology Clinics at the Heratsi University Hospital), Mariam Movsisyan (Assistant at the Allergy and Immunology Department at the Heratsi University Hospital) and Sevan Iritsyan (Head of the Laboratory Service at the Arabkir Medical Center). They are all located in Yerevan. Please see contact information at <https://haei.org/hae-member-countries/armenia>.



## ESTONIA

Krista Ress, MD, PhD, is the first HAE knowledgeable physician registered in Estonia. She is the Head of the Center of Allergology and Immunology at the East Tallinn Central Hospital. At the same time the East Tallinn Central Hospital has been registered as the first HAE knowledgeable hospital in Estonia. Please see contact information at <https://haei.org/hae-member-countries/estonia>.



## SOUTH AFRICA

*From HAE South Africa:*

We are planning a handover from Adrienne de Jongh to Janice Snyman and have restructured our committee to include Jane Rushton, who is in the legal field. Janice is in a new position as COO and will be handling day to day matters. We are still awaiting for our nonprofit company registration and plan to complete our restructure at that point.

We are also planning to initiate a program for training doctors in HAE awareness and current treatment protocols within a framework of angioedema. This program will be formulated by Professors Jonny Peter and Mike Levin and run from the ACARE center in Cape Town. This program will be fully supported by our Regional Patient Advocate Patricia Karani. We are planning to use the posters produced by HAEi in conjunction with this program.

Furthermore, we are active within the Rare Disease group and are participating in their programs as Janice is the official representative of HAE on that panel.



## SPAIN

*From President Sarah Smith, AEDAF:*

For the 10th anniversary of **hae day :-)**, in addition to taking part in the virtual walk around the globe, AEDAF, with the collaboration of Takeda, has produced a documentary titled “Impredicibles” which premiered on 16 May. The protagonists of the documentary are three members of AEDAF, real patients who have convincingly and emotionally portrayed the challenges of living with HAE and how it impacts their quality of life. With the documentary, we have intended to continue raising public awareness and communicating the realities of living with this rare disease. Dr. Teresa Caballero, a leading HAE expert in the allergy department of La Paz University Hospital in Madrid, also took part in the documentary.

Because of COVID-19 restrictions, the documentary was first shown in a cinema in Madrid to only a very small group of people, but the video went live at the same time and was seen “live” by 67 people. Since then, it has been viewed many times and the feedback has



been very positive. The link can be found on AEDAF's social media (Twitter, Facebook and Instagram) and on the AEDAF website <https://angioedema-aedaf.haei.org>.

Here is a link to the documentary in Spanish, which includes a short introduction by Carolina Zamora and Maria Ferron of AEDAF's Executive Committee and Dr. Teresa Caballero: <https://youtu.be/zNL3HaUrDUc>. And this is a link to the documentary with English subtitles and without introduction: [https://youtu.be/h\\_ch-ZB0i34](https://youtu.be/h_ch-ZB0i34).



## AUSTRALIA & NEW ZEALAND

*From CEO Fiona Wardman, HAE Australasia:*

HAE Australasia and its members (in Australia) welcomed the news that subcutaneous C1 is now indicated for children aged eight and older.

There have been quite a few questions from HAE patients around receiving the COVID-19 vaccinations. HAE Australasia has made available the information from HAEi on this topic.

A local survey for patients in South Australia was carried out to ascertain the complete picture of HAE in this state of Australia regarding patients' severity of their current condition and treatments.

HAE Australasia had a focus on the HAEi “Let's Take the Next Steps” campaign for **hae day :-)** 2021.

We have welcomed a few new patients into our membership from New Zealand and Australia over the last few months.

HAE Australasia engaged with patients on two different projects with the hope of broadening access to preventative therapies for patients who are currently ineligible for access and who are falling through the cracks. We hope that more patients will benefit from prevention therapies and enable them to live a much better quality of life.





## LATVIA

In the Latvian capital Riga, you will find the Pauls Stradins University Hospital which is the first HAEi registered HAE knowledgeable hospital in the country. Have a look at <https://haei.org/location/hospital-riga-latvia> for contact information.



## CYPRUS

HAEi welcomes the first HAE knowledgeable hospital registered in Cyprus: The General Hospital of Nicosia. Contact information can be found at <https://haei.org/location/hospital-nicosia-cyprus>.



## USA

*From Digital & Social Media Manager  
Ianice Viel, US HAEA:*



**2021 US HAEA Virtual Summit Series:** On 16 May 2021, the US HAEA launched its first ever US HAEA Virtual Summit Series. The Virtual Summit Series is being presented in one-day sessions through the summer and ends on 8 August. The virtual format combines engaging live and on-demand events that explore:

- challenges faced by our HAE community,
- exciting ongoing HAE research,
- special youth programs, and
- information that will help you evaluate your personal situation and explore alternatives to improve your quality of life.

HAEi friends interested in accessing the 2021 US HAEA Virtual Summit Series conferences and viewing all of the sessions (recorded and available on-demand), can register by visiting [www.haea.org](http://www.haea.org).

The 2021 US HAEA Virtual Summit Series kicked off with over 1,000 HAEA friends celebrating **hae day :-)** during a live session featuring US HAEA President, Tony Castaldo, who moderated discussions with:

- Dr. Bruce Zuraw regarding progress and challenges in the world of HAE, and
- HAEA leaders who shared details of available programs, activities, and services.

The June event features a live plenary session which will enable participants to ask US HAEA Medical Advisory Board members questions about care and treatment options for HAE. This program will include a live breakout session on women and HAE.

Additionally, a series of pre-recorded programs cover a diverse range of relevant topics that include:

- Coping during the COVID-19 pandemic,
- Grassroots advocacy efforts,
- US HAEA Medical Advisory Board 2020 Guidelines for the Management of Hereditary Angioedema, and
- Exclusive content for HAEA youth, with a particular focus on advocacy training and the publication of its third children's book, "Nico's Story".

The July and August events will focus on new developments with HAE therapies and insurance coverage of HAE medicines. Pre-recorded programming will include an HAEi presentation on access to HAE medicine around the globe.



**US HAEA Expands Research Portfolio:** 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 including:

- An HAEA/HAEi-initiated study, published on 1 March 2021, in a peer-reviewed medical journal demonstrated the clear economic value and improvement in quality of life offered by HAE medicines,
- The US HAEA Medical Advisory Board Guidelines for the Management of HAE are available and provide evidence-based recommendations for the diagnosis and treatment of HAE,
- An HAE Primer, published in a prestigious medical journal, that provides non-HAE expert medical professionals an overview on the condition,
- A study, submitted to a medical journal, that included 1,600 participants, and assesses how the COVID-19 virus affects people with HAE,
- Surveys focused on insurance challenges affecting the HAEA community. This important data is being used to prepare guidebooks on navigating insurance challenges. These guidebooks will be available for both physicians' offices and people with HAE in the fall of 2021.

Moving into 2021, the US HAEA plans to continue to actively pursue innovative and ground-breaking research. We are currently developing a Shared Decision-Making tool that will enable physicians and people with HAE to work together when making healthcare decisions, ensuring that both the physician and the person with HAE have a voice in their treatment plan. We are also launching an Epidemiological Study to establish the true prevalence of HAE in the US.

Additionally, we are making progress on a study on HAE in Aging, which will help to better understand the demographic and clinical characteristics, HAE treatment patterns, HAE impact and burden, and perceived health-related quality of life (HRQoL) 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 early 2022. This research project is uniquely important to the HAEA community, as current data on HAE in ageing does not exist.

Finally, we are collaborating with HAEi to develop a new questionnaire that offers a more comprehensive measurement of how dealing with a chronic condition like HAE affects quality of life.



**New US HAEA website:** We have recently unveiled our new website at [www.haea.org](http://www.haea.org). The newly redesigned format is user friendly and provides easier access to:

- Breaking HAE news,
- Educational materials, including successfully managing HAE,
- Information regarding FDA-approved treatments and ongoing clinical trials,
- Ways to connect to the broader HAEA community, and
- Information about the HAEA's programs, services, and activities.

The new website also provides sections tailored to specific groups, such as parents of children with HAE and people who are newly diagnosed. All content is available in English and Spanish.



**The 2021 HAE IN-MOTION® Virtual Challenge:** Hundreds of HAEA friends and families are counting their steps to raise awareness for HAE through the 2021 HAE IN-MOTION® Virtual Challenge.

Summer is the perfect time to get active. HAEA members are taking advantage of the warm weather to enjoy the fresh air while raising funds for three key US HAEA programs that directly impact community members:

- The Pam King HAEA Scholarship Program, which offers college tuition grants to HAE patients,

- The Chris Whalen HAEA Compassion Fund, which offers financial assistance for patients in need who must travel to see an HAE medical specialist, and
- The HAEA Research Fund, which supports a variety of research initiatives including the HAEA Scientific Registry.

The 2021 HAE IN-MOTION® Virtual Challenge started 14 June and goes on until 31 July 2021.

The Virtual Challenge is an event for participants from the United States and Puerto Rico who can register at [www.charityfootprints.com/HAEINMOTION2021](http://www.charityfootprints.com/HAEINMOTION2021).



**US HAEA launches Continuing Medical Education (CME) Program for Healthcare Professionals:** The US HAEA is committed to providing healthcare professionals with authoritative information that results in the best care and quality of life for the HAE community. That is why we collaborated with the Postgraduate Institute for Medicine, and RMEI Medical Education, LLC, to develop a CME Program course focused on the 2020 US HAEA Medical Advisory Board Guidelines for Managing HAE. The one-hour course is designed to meet the educational needs of healthcare professionals involved in the diagnosis and/or management of patients with HAE.



## COSTA RICA

In Costa Rica HAEi welcomes two HAE knowledgeable hospitals. That is Hospital de Niños and La Clínica de Odontología at Universidad Latina, both located in San José. Please see contact information at <https://haei.org/hae-member-countries/costa-rica>.



## CROATIA

*From President Mihaela Šogorić,  
HAE Croatia:*

On 6 and 7 March 2021, the HAE Croatia organized an assembly and a meeting in Rijeka. A report for the work of the association in the past year was presented to the participants. Doctors dealing with HAE were invited to the meeting and gave lectures. Ljerka Karađa Lapić, MD, from the General Hospital Šibenik had a topic on genetics while Srđan Novak, MD, PhD, from the University Hospital Centre Rijeka, covered the treatment of HAE, including therapeutic drugs which are currently being studied, and shared information about treatment options available in Croatia. Furthermore, Renata Vrsalović, MD, addressed the topic of management of HAE in pediatric patients. Younger doctors from the University Hospital Centre Osijek, Ana Kovač and Ana Marija Masle talked about the “my HAE” app. It is designed to help patients and their health care professionals jointly monitor the patients’ progress and make more informed treatment decisions together.

HAE Croatia celebrated the 10th anniversary of **hae day :-)** on 16 May 2021 with illuminating buildings in several cities throughout our beautiful country with the color purple in the hope of raising awareness about HAE. T-shirts with the association’s new logo and the HAEi logo accompanying the 10th anniversary were printed and distributed to patients and supporters.



## PUERTO RICO

From Humacao HAEi brings you the news that Hospital Ryder has been registered as an HAE knowledgeable hospital. Have a look at <https://haei.org/hae-member-countries/puerto-rico/>.



## SAUDI ARABIA

At <https://haei.org/hae-member-countries/saudi-arabia/> you will find the contact information for the National Center of Allergy, Asthma and Immunology NCAAI. The reason for mentioning this is that the hospital has been included on the HAEi world map as an HAE knowledgeable hospital.





## ROMANIA

*From Vice-President Ana-Maria Bălăţescu,  
HAE Romania:*

The celebration of the 10th anniversary of **hae day :-)** was a national premiere because for the first time an important building in Târgu-Mureş was lit in purple to celebrate the day and to raise awareness about HAE. The building we choose is the University of Medicine, Pharmacy, Science and Technology G.E. Palade, one of the most representative buildings in Transylvania.



## UNITED ARAB EMIRATES

While the United Arab Emirates already have an ACARE Center (The Immunology and Allergy Cleveland Clinic Abu Dhabi), HAEi has now registered the first HAE knowledgeable hospital in the country: The Mafrq Hospital located in Abu Dhabi. Contact information can be found at <https://haei.org/hae-member-countries/uae>.



## IRELAND

Ireland is yet another country to have its first HAE knowledgeable hospital on the HAEi world map. It is the Cork University Hospital, and you will find the contact information at <https://haei.org/location/hospital-cork-ireland>.



## SYRIA

The first HAE knowledgeable hospital in Syria is the Al Assad University Hospital in Damascus. This will take you to the contact information: <https://haei.org/location/hospital-damascus-syria>.



## BOSNIA AND HERZEGOVINA

Tuzla is yet another city where HAEi has registered an HAE knowledgeable hospital. It is the University Clinical Center – please go to <https://haei.org/location/hospital-tuzla-bosnia-and-hercegovina> for contact information.



## INDIA

The Department of Paediatrics and the Department of Dermatology, Venereology and Leprology, PGIMER, Chandigarh jointly celebrated **hae day :-)** by holding a virtual conference organized by the Hereditary Angioedema Society of India (HAESI). The e-conference included lectures on various aspects of HAE by national and international experts and faculty of Departments of Paediatrics and Dermatology PGI; case presentations by students; personal journey and struggle of patients suffering from the disease, and an open forum for patients and physicians.

Surjit Singh, Head of the Department of Paediatrics, PGI, introduced the proceedings of the e-conference. Dr. Ankur Jindal, Assistant Professor at the Department of Pediatrics, PGIMER and founder Secretary of HAESI, elaborated in his talk about the journey of HAE in India over the last 25 years. Prof. Sunil Dogra, Professor at the Department of Dermatology, PGI and founder President of HAESI, officially inaugurated the website of the Hereditary Angioedema Society of India. He elaborated on the need to increase awareness about this disease amongst physicians as there are likely to be more than 30,000 undiagnosed patients in India. He also highlighted that the society would aim to increase awareness about HAE in the community and bring better treatment options for patients in India.



The event was attended by more than 500 delegates across the country. Dr. Hilary Longhurst from New Zealand and the HAEi Chief Regional Patient Advocate Fiona Wardman were guest speakers. Dr. Longhurst talked about various clinical manifestations, diagnosis and treatment of HAE, while Ms. Wardman highlighted the activities of HAEi in improvising patient care in India.



## LEBANON

In Beirut you will find the Hotel Dieu Hospital, yet another HAE knowledgeable hospital to be registered on the HAEi world map. Contact information can be found at <https://haei.org/location/hospital-beirut-lebanon>.



## GEORGIA

Prof. Maia Gotua, MD, Ph.D., who is the General Director of the Center of Allergy & Immunology in Tbilisi has been registered as HAE knowledgeable physician on the HAEi world map. Please see contact information at <https://haei.org/location/physician-tbilisi-georgia>.



## BAHRAIN

HAEi welcomes the Al Kindi Specialized Hospital W.L.L in Manama as the first HAE knowledge hospital in the country. Visit <https://haei.org/location/hospital-manama-bahrain> and you will find contact information for the hospital.



## ALGERIA

HAEi has registered Centre Hospitalier et Universitaire de Beni Mesous as an HAE knowledgeable hospital on the world map. Please see contact information at <https://haei.org/location/hospital-beni-messous-algeria>.



## IRAQ

From Iraq HAEi has learned that the Allergy and Immunology Section at Bagdad University has been added as an HAE knowledgeable hospital. Visit <https://haei.org/location/hospital-bagdad-iraq> and you will find contact information.



## KENYA

In Nairobi you will find Dr. Priya Bowry, an HAE knowledgeable physician registered on the HAEi world map. She is located at the Allergy Clinic at the Upper Hill Medical Centre. For contact information please see <https://haei.org/location/physician-nairobi-kenya>.



## UNITED KINGDOM

*From CEO Laura Szutowicz, HAE UK:*

The run-up to **hae day :-)** is always fun, with us trying each year to find something that will be new and challenging for our members. This year we started with a quiz on Saturday night. Rachel is our indefatigable question setter and yet again came up with many brain teasers. The Easton family won the family group, June and Peter Cole the pairs, and Furkhanda won as an individual. As always, the feedback was “another one soon!” but it is hard work for Rachel, so we may have to look at making the winners of one set the questions for the next to give her a chance to join in!

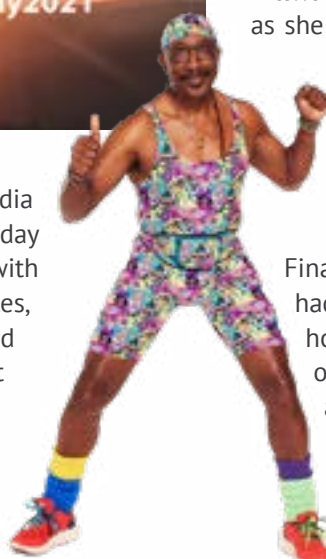
Rachel also set a “Scavenger Hunt” where people had to find various items ranging from “a leaf bigger than your hand” to “something to recycle”. We are still waiting for them all to come in to announce the winners.



Our big project for **hae day :-)** was to produce a collage of members photos showing them doing something they enjoyed – the result is stunning! It does just show that having HAE doesn't mean you cannot live your life.



Rachel manages our social media content and also posted every day in the week before **hae day :-)** with motivational and inspirational quotes, which all helps to lift our mood. And then this culminated in our great treat. Following up on an idea of June Cole's, I was able to make contact with "Mr. Motivator". He is an exercise coach famous for his exuberant style, both in his



clothing and in how he really does get people moving! We are so lucky that he has invited HAE UK to join in his Wednesday evening session, so look out for the reports in the next *Global Perspectives*.

That ties in nicely with a clinical study HAE UK has sponsored with the Immunology Center nurses from Cardiff. Twenty HAE patients have been recruited into this study, whereby they are issued with Fitbits and generally encouraged to raise their level of activity and fitness to see how this affects their HAE. Hopefully, the first results will be available fairly soon, but Emily Carne, who is the lead nurse, reports great enthusiasm from the participants. Depending on the interim results, we will see if the study should be extended to some other centers and more patients.

Another study HAE UK is assisting with is being carried out by the Psychology Department of Staffordshire University and Dr. Lavanya Diwaker, who is both a consultant immunologist and a health economist. The study was developed from an idea of Dr. Diwaker's to look into the lived experience of HAE and will be both qualitative, but more importantly, quantitative. We are still in the early days of developing the protocol for this study, but it is wonderful to have such enthusiasm from this very highly qualified team.

One of our members, Peter, who has two daughters with HAE, set himself a challenge of running four miles every four hours for 48 hours in aid of HAE UK! In doing this, he raised an amazing amount of money, as well as awareness of HAE in his local community. Peter's employers, Innospec, also more than doubled his fundraising by using us as their nominated charity. Thank you so much, Peter and Innospec!

Furkhanda again raised a large sum of money on her Kiltwalk! She also raised not a few blisters on her feet as she walked the equivalent of a half marathon and sent some gorgeous photos of the wonderful scenery in Scotland. We also have the wonderful Lucy Church, who is going to do a parachute jump in aid of HAE UK, very brave of her and much appreciated.

Finally, HAE UK has turned shopkeepers! We have had so much demand for the Percy the Pufferfish hoodies we awarded as prizes to the winners of our Christmas competitions that we have added a "shop" to our website. On this you can buy Percy sweatshirts, hoodies and T-shirts, also the branded supporter T-shirts, hoodies etc. in a variety of colors, all printed on good quality garments.





## COLOMBIA

HAEi welcomes the first HAE knowledgeable physician in Colombia to the world map. Please see contact information for Dr. Margarita Olivares Gomez in Medellín on the country page for Colombia at <https://haei.org/hae-member-countries/colombia/>.



## SWITZERLAND

*From Greber Ernst, HAE Schweiz:*

Unfortunately, this corona pandemic also affects the planning of HAE Switzerland. This year our association can celebrate its 20th anniversary and we would have loved to use this occasion for inviting our members and our HAE network to an excursion. We were looking forward to a great time and good conversations. However, since the health of all participants is very important to us and we do not want to take any risks, the board decided to temporarily cancel the anniversary excursion.

For this year's **hae day :-)** we wanted to make our members happy, so we sent them a lucky heart with seeds inside. A small meadow with fourleaf clover leaves emerges from these seeds. This small present should hopefully give our HAE members a healthy and virus-free time.



## LIBYA

If you visit Tripoli, you will find the first HAEi registered HAE knowledgeable hospital in Libya. That is the University Hospital of Tripoli – please visit <https://haei.org/location/hospital-tripoli-libya> for contact information.



## ALBANIA

Albania is yet another country to have its first HAE knowledgeable hospital registered on the HAEi world map. It is the University Hospital Centre “Mother Theresa” in Tirana – please see contact information at <https://haei.org/location/hospital-tirana-albania>.



## KUWAIT

The NBK Children's Hospital in Kuwait City has been registered as the country's first HAE knowledgeable hospital. Please go to <https://haei.org/location/hospital-kuwait-city-kuwait> for contact information. Previously, HAEi has registered the Al-Rashed Allergy Center – also in Kuwait City – as an ACARE center.



## THAILAND

*From National contact Suchitta Kengtanyagarn, HAE Thailand:*

We had our first virtual meeting on **hae day :-)** 2021. For the meeting, we had financing support by Takeda Thailand and the volunteer speakers were Dr. Gun and Dr. Chamas as well as the President of Hemophilia Thailand to share their experience about making a patient group and benefit. We had an amazing and empowering time together and HAE Thailand was officially established.



HAE Thailand can now be found on Facebook – please see [www.facebook.com/HAETHailand](https://www.facebook.com/HAETHailand). Furthermore, the organization has a website hosted with HAEi: <https://haethailand.haei.org>.



Canadian HAE patients have more to celebrate since the last edition of *Global Perspectives*.

After years of discussions and negotiations, we are excited to share that all eligible HAE patients across Canada are now able to access Haegarda, CSL Behring's subcutaneous C1-INH prophylaxis therapy for adolescents and adults, through Canadian Blood Services (CBS). Listing Haegarda on this federal formulary ensures equitable access for Canadian HAE patients. Thank you to the teams at CSL Behring and CBS who worked hard to ensure this treatment is available to Canadian patients. HAE Canada is proud to have played a major role advocating for access to Haegarda in Canada.

Additional jurisdictions have added Takeda's lanadelumab (Takhzyro), a subcutaneous monoclonal antibody inhibitor of plasma kallikrein, to their formularies. We are thankful to Takeda for successfully negotiating with five additional provincial governments, as well as the Non-Insured Health Benefits (NIHB) for First Nations and Inuit Program, for access to Takhzyro. We are on the road to equitable access to Takhzyro for eligible HAE patients across Canada.

We are thrilled to share that our second abstract of 2021, this one titled: "HAE in Canada: Preliminary Demographic and Treatment Results" from the Second

National Survey has been accepted at the 2021 EAACI Hybrid Congress. Once again, we need to thank Dr. Suzanne Kelly of Red Maple Trials who always does an amazing job developing our abstracts, and we are always grateful for assistance provided by our Board of Directors and Advocacy Committee.

On 15 May, HAE Canada hosted a virtual Patient Information Update for our membership. President Jacquie Badiou presented on HAE Canada's latest news and initiatives, while COO Daphne Dumbrille kept the meeting on task. We were grateful to have a fantastic line-up of expert speakers, which included:

- Dr. Chrystyna Kalicinsky, MD, FRCPC, Allergist/Immunologist,
- Dr. Sylvain Grenier, Director, Plasma Derived Products Formulary Program at CBS
- Anthony J. Castaldo, President and CEO of HAEA and HAEi
- Kim Speiss, RN, Regional Director, Manitoba & Saskatchewan

Dr. Kalicinsky spoke about HAE and the different treatment options available to Canadian patients, while Dr. Grenier spoke specifically about accessing treatment through CBS. Tony Castaldo discussed HAE treatments on the horizon, and Kim Speiss spoke about her patient journey. Members were grateful for the opportunity to have their pre-submitted questions answered by the speakers. We would like to thank our wonderful speakers who generously took time out of their busy Saturdays to help ensure our members remain informed.

During our Patient Update we announced that we are soon launching our **hae day :-)** 2021 initiative: the HAE Canada Cafe. This members-only online forum will allow our members to connect with other patients and caregivers to share stories and experiences in a supportive environment. We will also allow members to ask medical questions that will be answered by HAE specialists from across Canada. We are excited to offer this platform to the HAE Canada membership.

For the past five years, Jacquie Badiou has met with various stakeholders, including the Canadian Heart and Stroke Foundation, to work toward getting HAE added to the Airway Management section of the Advanced Cardiovascular Life Support (ACLS)/Pediatric Advanced Life Support (PALS) curriculum. This important initiative would ensure that first responders and front-line healthcare workers are educated on HAE and would learn the special treatment precautions necessary for

HAE patients presenting with a laryngeal attack. We are grateful that recently, Canadian physicians, including Drs. David Barnes, Susan Wasserman and Paul Keith, and US physician Dr. Marc Riedl and Anthony J. Castaldo of HAEi, volunteered their time to develop a PICO question to submit to the International Liaison Committee on Resuscitation (ILCOR). This is the first step, asking for a systemic review. While we have come far, we still have more steps to complete on this project.



## RUSSIA

From Chairman Elena Bezbozhnaya,  
HAE Russia:

In Russia, many restrictions imposed due to the spread of the COVID-19 pandemic have already been lifted. The activities of HAE Russia are gradually returning to the face-to-face format, although many events are still conducted both offline and online.

In Spring 2021, we held the fourth International Conference “Vector of the Future” (last year, we had to cancel due to prohibition on public gatherings). Besides, HAE Russia provided several informative webinars and online meetings for adults and children with HAE.

At the end of February, HAE Russia held the third series of online meetings from the “Call a Friend” cycle for underage patients with HAE. Webinar host Yulia Faikova, psychologist, the head of the Moscow branch of HAE Russia, an HAE patient, along with class administrator Dasha Bezbozhnaya (16 years old), organized a fun “Journey to the Land of Bubbles” for junior and middle age group children, during which they learned to make bubbles and shared their emotions. Throughout the meeting with teenagers, the host discussed self-help methods for stressful situations with the participants and introduced them to the activities to restore psychological balance and relieve anxiety and excitement. Participants tried to understand themselves and others better and interact with each other.

Mid-March featured the webinar “What an HAE patient needs to know”. The webinar gathered patients with HAE from many cities and regions of the country. I presented a report on the organization’s activities in 2020, noting that more than 250 people received information and legal assistance from HAE Russia during the year. Furthermore, I presented the HAE patient routing developed by HAE Russia. Doctor of the highest category, allergist-immunologist, head of the consultative-diagnostic department of the Center for Allergology and Immunology of the State Budgetary Healthcare Institution at the City Clinical Hospital No. 52 of the Moscow Department of Health, Elena Bobrikova described the etiology of the disease, clinical characteristics and specific features of the three types of HAE. Referring to localization of edema, the doctor stated that around 61% of Russian patients experience laryngeal edema during their life, causing the most life-threatening conditions leading to asphyxia. About 86% of patients experience abdominal edema, 90%



The 2021 HAE Global Walk illustrates why we are so proud of our amazingly engaged Canadian HAE community. Our membership, including the HAE Canada Board of Directors, continuously entered their steps to ensure Canada remained in the lead for a third time. Teams from our energetic sponsors, specifically, CSL Behring and Takeda Canada, as well as our contracted workers, especially our health policy consultant, Bob Bick, also logged many steps for Canada. We could not be prouder of the Canadian community who finds the time to keep HAE awareness alive. Thank you to all who participated, we appreciate the engagement and enthusiasm for this amazing initiative.



## ECUADOR

HAEi welcomes the first Ecuadorian HAE knowledgeable hospital to the world map. It is Hospital del Río in Cuenca – please look here for contact information: <https://haei.org/location/hospital-cuenca-ecuador>.



have limb edema, 79% suffer from facial edema, and 66% have edema of the genital organs. The physician reviewed the baseline therapies and short-term medications available to Russian patients, along with new long-term therapies.

Another seminar in March was devoted to methodological materials developed by HAE Russia for patients with HAE. Project coordinator Denis Bezbozhny presented an informational brochure prepared by HAE Russia. The brochure consists of three sections. The first part focuses on the medical aspect, while the second part summarizes the legal experience of HAE Russia. The third part deals with the psychological aspects of the disease. The guide also presents the routing standard, developed by HAE Russia together with the leading allergist-immunologists. Ilya Ushankov, PhD in Law, Associate Professor of the Graduate School of Law at the Institute of State Pedagogical University, Russian Academy of National Economy and Public Administration under the President of the Russian Federation, presented and commented on each block of the legal section. The lawyer emphasized that modern international practice provides for the cure of any edema for a patient with HAE. In Russia, so far, the practice is different – representatives of the medical community recommend people diagnosed with HAE to stop only life-threatening edema.

Ekaterina Viktorova, an allergologist and immunologist at the Dmitry Rogachev Scientific Research Center for Cardiovascular Surgery (Moscow), was the major speaker of the second part of the webinar. The doctor presented “Prospects of HAE therapy” and outlined promising therapies available to HAE patients in the near future. The speaker also detailed the methods and results of international trials of two new medicines, Lanadelumab and Berotralstat.

In April, HAE Russia held an on-site workshop in St. Petersburg for patients with HAE from St. Petersburg and the Leningrad region. During the event, Dr. Angelika Milichkina, the chief physician of St. Petersburg Pasteur Research Institute of Epidemiology and Microbiology, outlined the institute’s potential and interaction principles between its medical community and patients with HAE. Dr. Elena Bobrikova (see above) introduced aspects of a multidisciplinary approach in HAE patients’ treatment. In addition, the speaker shared the specifics of the disease caused by the gender factor. Raisa Kuznetsova, an allergologist and immunologist from the St. Petersburg Pasteur Institute of Epidemiology and Microbiology, an infectious disease doctor and candidate of medical sciences, presented “The current



approach and prospects in the treatment of HAE patients". The second part of the workshop addressed the issue of the legal literacy of HAE patients. Lawyer Ilya Ushankov delivered a presentation "Patient Navigator: Violated Law" and led an interactive "What if..." game. This game enables to simulate different legal situations, gives an effective algorithm of actions, and advises on how a patient should act when he or she is denied a recipe, advanced therapy appointment, provision of medical documents, and other situations.



On 15 and 16 May, the fourth annual International Scientific and Practical Conference of HAE Russia was held in Moscow. The "Vector of the Future" event coincided with the global **hae day** :-) 2021. The conference attracted representatives of HAE Russia from different regions of the country, leading scientists and specialists of medical centers – allergologists-immunologists, geneticists, clinical psychologists and lawyers. The best experts from Russia, the USA and a number of European countries acted as speakers at the event. Unfortunately, due to the restrictions imposed by the COVID-19 pandemic, foreign experts could not come to Moscow but participated online. The conference was addressed by Michal Rutkowski (Warsaw, Poland), Vice President and Regional Patient Advocate of HAEi as well as PhD, Professor of Allergology and Clinical Immunology, Department of Internal Medicine and Hematology, University of Semmelweis, Director of the Hungarian Center for Angioedema Recommendations and Excellence, Dr. med. Henriette Farkas (Budapest, Hungary). Furthermore, Clinical Director of the US HAEA Angioedema Center, Head of Clinical Service and Director of Training Program of the Department of Rheumatology, Allergology and Immunology, University of California, Dr. Mark Riedl (San Diego, USA) spoke along with the best specialists from Russian medical centers.

The event offered several modular panel discussions. The participants addressed current approaches to the diagnosis and therapy of HAE, shared their experience with patients in Russia and abroad, and commented on the prospects for pharmacological and genetic research in treating angioedema. Moreover, the conference agenda included a children's interactive art therapy session. On the first day of the conference, the Moscow Society presented a new animated film, "Tell me about HAE", which was prepared for the youngest patients with HAE.

At the conference, I reminded the participants that on 16 May, we could celebrate the fifth anniversary of HAE Russia, and I thanked the members, doctors, lawyers, psychologists and the official sponsors of the event – the companies Takeda and Medipal – for supporting our activities. Over the years, HAE Russia representatives have repeatedly participated in Russian and international events, organized scientific and practical conferences, actions and flash mobs, webinars, schools of patients, social exhibitions. Owing to the support of HAE Russia, many patients now receive effective therapy. Significant progress has been made over the years, but more needs to be done. HAE Russia will continue to defend the rights of its members – patients with HAE.



## SOUTH KOREA

Yeeun Yuk is the new daily contact at HAE South Korea while Soo Jin Min is President of the organization. Please see contact information at <https://haei.org/location/hae-south-korea>.



## NORTH MACEDONIA

*From President Natasha Jovanovska Popovska, HAE Macedonia:*

HAE Macedonia celebrated the 10th anniversary of **hae day** :-) by promoting a video that carried a message with the motto of the awareness day, "Many faces one family". The video was produced by the dance studio Eureka, which manifested great effort and enthusiasm.

The video featured young dancers who managed to convey a message through their dancing that regardless



of how alone and afraid an HAE patient feels, things will improve with the help of the HAE community. A poem was specially penned in Macedonian by Violeta Hristovska to metaphorically describe the struggle of HAE patients and how everything can change for the better when they get the help they deserve. Members of HAE Macedonia translated the poem to English and had it translated to Albanian. The video was very emotional and accepted very well by the public. It was also distributed on the official Facebook page of the Minister of Health. The video was viewed 1,000 times on YouTube, and there were more than 17,000 views on the Ministry of Health page.



T-shirts with the **hae day :-)** logo were distributed to the members of HAE Macedonia, the healthcare professionals at the Paediatric Clinic as well as the Clinic of Dermatology in Skopje, the Ministry of Health, and also to the participants that have supported HAE Macedonia with **hae day :-)** activities entries. Due to that kind gesture, the recipients of the T-shirts felt happy and appreciated, being glad that their efforts to help HAE Macedonia were cherished.



Here is the text of the English version of the video:

*When the stage is ready for the skies at night  
Thousands of stars come out basked in silver light.  
Each twinkling in peculiar splendor,  
Starting their dance ever so tender.  
Look at each star fly, hop and twirl!  
Oh, no! One of them stumbled and fell,  
Where did it curl?  
Friends are looking for her, there! She is found!  
Along comes help so unwavering and profound!  
And up stands the star, with the burden lessened  
Looking upward, to the moon beautifully crescent.  
I dance and feel cared-for  
I travel and always explore.  
I dream without a quaver  
I swell but defeat do not favor.  
Since I am rare, stronger I shall thrive  
Since I have HAE, I yearn to survive  
Since I am human, special I shall be  
My own person but so fond of thee.  
Each star is special, diverse and rare,  
Each twinkling and shining in its own magnificent flare.  
Still, they dance all together, nobody is alone  
And every night that scene is what we are shown.  
That we are one of many faces in a family of our own.  
Together we are more beautiful and brighter,  
Part of a web, so miraculously spun,  
Many stars, many faces in a family as one!*





## SERBIA

*From Sofija Popovic, HAE Serbia:*

In commemoration of **hae day :-)** 2021, HAE Serbia organized multiple happenings. Although we decided to cancel all in-person gatherings for the annual awareness day due to sanitary reasons and restrictions regarding group gatherings, we still found numerous ways to mark the day. HAE Serbia held its General Assembly with patients and HAE practitioners online, during which we discussed the association's progress, ways we raised awareness during the past year and announced the upcoming events. We were also very happy to announce that thanks to our collaboration with Takeda, all HAE patients in Serbia will finally have access to an app that will allow them to track HAE attacks, their length, intensity, pain levels as well as the medications used to treat them. All of the data will be easily transformable to be sent to an HAE specialist and further analyzed. HAE Serbia is grateful for Takeda's time and effort in making this app that will make tracking attacks so much easier for HAE patients. HAE Serbia has also designed HAE emergency cards which will soon be printed and sent to all Serbian HAE patients.

As part of our efforts to raise awareness on **hae day :-)** 2021, multiple landmarks in Serbia's capital, Belgrade, and in the city of Nis, were illuminated in purple: the National Assembly, the Avala Tower, the Palace of Albania, Slavija fountain, the Gazela Bridge, Branko's Bridge, the New Railway Bridge, the Ada Bridge and the National Theatre in Nis. We also created a hashtag **#osvetlimohae**, which translates to "Light up for HAE", which we used when sharing pictures of all the landmarks that glowed purple on social media. All of this was reported by multiple media outlets, as well as the interviews with the President of HAE Serbia, Jovana Cvetkovic. Even though we were unable to meet in person for the second year in a row, we still found ways to come together for HAE and raise awareness among the general public and are very thankful for everyone who participated.





## Track your HAE with HAEi's new app!

### Introducing HAE TrackR

- ✓ An easy-to-use electronic diary to track your HAE.
- ✓ Developed by patients for patients and is product and company neutral.
- ✓ Enables inputting, storing, and sharing data about your HAE with your physician.
- ✓ Fully EU-GDPR compliant, protecting your data and privacy – all data is the sole property of yourself.
- ✓ Endorsed by the ACARE network (Angioedema Centers of Reference and Excellence)
- ✓ Can be accessed from a computer, tablet, or smartphone.



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



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

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**Analysis of Heart-Rate Variability during Angioedema Attacks in Patients with Hereditary C1-Inhibitor Deficiency** – by Francesca Perego, IRCCS Istituti Clinici Scientifici Maugeri, Milan, Italy et al.:

Power spectral heart rate variability analysis may furnish early markers of an impending angioedema attack, thereby helping to identify patients at higher risk of attack recurrence. It could assist in the timing, titration, and optimization of prophylactic therapy and thus improve patients' quality of life.

(Int J Environ Res Public Health, March 2021)

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**Assessment and management of disease burden and quality of life in patients with HAE: a consensus report** – by Konrad Bork, Johannes Gutenberg University, Mainz, Germany et al.:

Patient-reported outcome measures for assessing HAE attack severity and frequency are available and valuable tools. However, attack frequency and severity are insufficient markers of disease severity unless evaluated in the broader context of the effect on an individual patient's quality of life. Assessments of quality of life should be individualized should minimally address the interference with work, school, social, family, and physical activity, along with access to and burden of HAE treatment. Advances in HAE therapies offer the opportunity for comprehensive, individualized treatment plans, allowing patients to achieve minimal attack burden with reduced disease and treatment burden.

(Allergy Asthma Clin Immunol., April 2021)

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**Clinical characteristics and burden of illness in patients with HAE: findings from a multinational patient survey** – by Joan Mendivil, Takeda Pharmaceuticals International AG, Zurich, Switzerland et al.:

A survey study of a broad international sample of HAE patients shows that despite the availability of on-demand treatment and long-term prophylaxis, patients across a wide geographical area continue to have high disease activity, likely due to restrictions in the availability of medications or incorrect use. Significant disease burden, including impaired quality of life and mental health and decreased productivity, is evident. Increased patient education and access to newer, more effective therapies are needed.

(Orphanet J Rare Dis., February 2021)

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**Consensus on treatment goals in HAE: a global Delphi initiative** – by Marcus Maurer, Charité-Universitätsmedizin Berlin, Germany et al.:

It is clear from the wide-ranging consensus statements of this Delphi process that the burdens of disease and treatment should be considered when assessing disease control and normalization of patients' lives. The ultimate goal for HAE treatment is to achieve no angioedema attacks. Over the last decade, the availability of improved medicines and disease management now makes complete control of HAE a realistic possibility for most patients.

(J Allergy Clin Immunol., May 2021)





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**HAE due to C1 inhibitor deficiency in Belarus: epidemiology, access to diagnosis and seven novel mutations in SERPING1 gene**

– by *Irina Guryanova, Belarusian Research Center for Pediatric Oncology, Hematology and Immunology, Minsk, Belarus et al.:*

C1-INH-HAE diagnosis and management in Belarus have improved, as seen from the high number of new diagnoses in the last three years. The next steps will be to reduce the diagnostic delay and to promote long-term prophylactic and on-demand therapy.

(Clin Mol Allergy, April 2021)

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**HAE: how to approach it at the emergency department?**

– by *Faradiba Sarquis Serpa, Santa Casa de Misericórdia de Vitória, Brazil et al.:*

HAE does not respond to the usual treatment with epinephrine, antihistamines and corticosteroids. Thus, if not identified and treated appropriately, HAE patients have an estimated mortality risk from laryngeal edema of 25 to 40%. HAE treatment has changed dramatically in recent years with the development of new and efficient drugs for attack management: plasma-derived C1 inhibitor, recombinant human C1-inhibitor, bradykinin B2 receptor antagonist (icatibant), and kallikrein inhibitor (ecallantide). In Brazil, plasma-derived C1 inhibitor and icatibant have already been approved for use. Managed correctly in the emergency departments, HAE patients can avoid unnecessary surgery and fatal outcomes.

(Einstein (Sao Paulo), April 2021)

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**Impact of anxiety, stress and depression related to COVID-19 pandemic on the course of HAE with C1-inhibitor deficiency**

– by *Deniz Eyice Karabacak, Istanbul University, Turkey et al.:*

Restriction measures during the COVID-19 outbreak cause an increase in the number of HAE attacks in relation to anxiety, depression, stress and fear of the pandemic. Therefore, it is important to provide psychological support to HAE patients during the pandemic.

(Allergy, March 2021)

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**Lanadelumab Efficacy, Safety, and Injection Interval Extension in HAE: A Real-Life Study**

– by *Thomas Buttgereit, Charité - Universitätsmedizin Berlin, Germany et al.:*

Gradual extension of injection intervals of lanadelumab can minimize the burden of therapy without losing efficacy.

(J Allergy Clin Immunol Pract., May 2021)

## Mitigating Disparity in Health-care Resources Between Countries for Management of HAE –

by Ankur Kumar Jindal, Postgraduate Institute of Medical Education and Research, Chandigarh, India et al.:

Using India as a paradigm for HAE management in lower-income countries, we reviewed the evidence for second-line and non-recommended practices reported by HAE experts. Results suggest significant inequities in the provision of HAE services and treatments. HAE patients in low-income countries do not have access to life-saving acute drugs or recently developed highly effective prophylactic medications. Most low-income countries do not have specialized HAE services or diagnostic facilities. There is an urgent need to improve HAE services, diagnostics and treatments currently available to lower-income countries. We recommend that all HAE stakeholders support the need for global equity and access to these essential measures.

(Clin Rev Allergy Immunol., May 2021)

## Optimal Management of HAE: Shared

Decision-Making – by Aleena Banerji, Massachusetts General Hospital, Boston, USA et al.:

Treatment decisions should be based on the individual patient's preferences and needs. A method for facilitating this is shared decision-making, a widely used methodology for making treatment decisions among multiple therapeutic options. We propose a three-phase "3D" model (Discover, Discuss, Decide) for shared decision-making in HAE. "Discover" focuses on improving the physician's understanding of the patient's needs and understanding of the available therapeutic choices. "Discuss" considers the alternatives, allowing a collaborative, informed treatment selection in "Decision". Uncovering the patient's therapy goals through appropriate questions during these phases can help reveal relevant information for treatment selection information.

(J Asthma Allergy, February 2021)

## Patient perspectives on the treatment burden of injectable medication for HAE – by Cristine

Radojicic, Duke University School of Medicine, Durham, USA et al.:

Based on an online survey of HAE patients in the USA, most are satisfied with their current therapies but desire novel medications with a simpler route of administration. Although most patients experience significant treatment-related burdens, they learn to cope with these challenges over time.

(Allergy Asthma Proc., May 2021)

## Patient-reported Outcome Measures for Angioedema: A Literature Review – by Anna Trier

Heiberg Brix, University of Southern Denmark, Odense, Denmark, et al.:

Patient-reported outcome measures (PROM) for use in monitoring and managing patients with HAE have been developed and validated but are not yet used on a regular basis in the management of HAE in the Nordic countries. The potential use of PROMs in the clinic setting has a wide range of benefits, both for patients and for the healthcare system. However, planning is required regarding how to implement PROMs optimally in the local routine care processes, preferably as an electronic version that is easily accessible for patients.

(Acta Derm Venereol, May 2021)

## Physician and patient perspectives on the management of hereditary angioedema: a survey on treatment burden and needs – by

Marc A Riedl, University of California, San Diego, USA et al.:

Patients and physicians need to discuss the risks and benefits associated with each treatment to develop an individualized approach to HAE management. A survey of HAE patients and physicians who treat HAE patients confirms that newer, subcutaneous therapies are prescribed for HAE prophylaxis more frequently than other therapies in the USA and that treatment burdens still exist for patients with HAE. Physicians and patients are not always aligned on how treatment choices affect patients' lives, which may mean that there are opportunities for enhanced patient-physician dialog and shared decision-making in HAE management.

(Allergy Asthma Proc., May 2021)



### **Randomized Trial of the Efficacy and Safety of Berotralstat (BCX7353) as an Oral Prophylactic Therapy for HAE: Results of APeX-2 Through 48 Weeks (Part 2)** – by *H. James Wedner, Washington University School of Medicine, St. Louis, USA et al.:*

Berotralstat (BCX7353) is a recently approved, oral, once-daily kallikrein inhibitor for HAE prophylaxis. In the APeX-2 trial, berotralstat reduced HAE attack rates over 24 weeks, with a favorable safety and tolerability profile. The safety, tolerability, and effectiveness of berotralstat were maintained over 48 weeks of treatment.

(J Allergy Clin Immunol Pract., April 2021)

### **Safety and Outcomes Associated with the Pharmacological Inhibition of the Kinin-Kallikrein System in Severe COVID-19** – by *Eli Mansour, University of Campinas, São Paulo, Brazil et al.:*

We tested two pharmacological inhibitors of the kinin-kallikrein system currently approved for the treatment of HAE – icatibant and inhibitor of C1 esterase/kallikrein – in a group of 30 patients with severe COVID-19. Neither icatibant nor inhibitor of C1 esterase/kallikrein resulted in changes in time to clinical improvement. We found evidence for safety and a beneficial role of pharmacological inhibition of the kinin-kallikrein system in two markers that indicate improved disease recovery.

(Viruses, February 2021)

### **Survey of actual conditions of erythema marginatum as a prodromal symptom in Japanese patients with HAE** – by *Isao Ohsawa, Saiyu Soka Hospital, Japan et al.:*

More than half of the Japanese patients with HAE-1 or 2 and one-third of those with HAE-unknown develop erythema marginatum – a visible prodromal symptom – as the prodromal symptom of an angioedema attack. Physicians should communicate the significance of erythema marginatum to HAE patients to prepare them for possible imminent attacks.

(World Allergy Organ J., February 2021)

### **The importance of recognizing and managing a rare form of angioedema: HAE due to C1-inhibitor deficiency** – by *Joshua Jacobs, Allergy and Asthma Clinical Research, Inc., Walnut Creek, USA et al.:*

HAE patients should be evaluated at least annually to assess the need for routine prophylaxis. HAE specific medications like plasma-derived and recombinant C1-INH products, kallikrein inhibitors, and bradykinin B2 receptor antagonists, have improved management of HAE. While the introduction of intravenous C1-INH represented a major breakthrough in routine HAE prophylaxis, some patients fail to achieve adequate control, and others have psychological barriers or experience complications related to intravenous administration. Subcutaneous C1-INH, subcutaneous monoclonal antibody (mab)-based therapies, and an oral kallikrein inhibitor offer effective alternatives for attack prevention and may facilitate self-administration. HAE management should be individualized, with quality-of-life improvement being a key goal. This can be achieved with the broader availability of existing options for routine prophylaxis, including greater global availability of C1-INH (subcutaneous), mab-based therapy, oral treatments, and multiple on-demand therapies.

(Postgrad Med., May 2021)



# 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:

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**A multicenter, double-blind, randomized, placebo-controlled, parallel-arm study to investigate the efficacy and safety of subcutaneous administration of CSL312 (garadacimab) in the prophylactic treatment of HAE**

– recruiting in Canada, Germany, Hungary, Israel, Italy, the Netherlands, Spain and the USA

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**A multicenter, randomized, placebo-controlled, parallel-arm study to investigate the efficacy, pharmacokinetics, and safety of CSL312 in subjects with HAE**

– recruiting in Australia, Canada, Denmark, Germany, Israel and the USA

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**A Phase II, double-blind, placebo-controlled, Randomized, cross-over, dose-ranging study of oral PHA-022121 for Acute treatment of angioedema attacks in Patients with HAE due to C1-Inhibitor Deficiency type I and II**

– recruiting in Belgium, Canada, France, Germany, Hungary, Israel, Italy, the Netherlands, Poland, Spain and United Kingdom

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**An Open-Label Extension Study of ISIS 721744 in Patients with HAE**

– recruiting in the Netherlands, United Kingdom and the USA

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

– recruiting in Australia, Canada, Czech Republic, Germany, Hong Kong, Hungary, Israel, Italy, Japan, the Netherlands, New Zealand, Russia, Spain, Taiwan, United Kingdom and the USA

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

– recruiting in France

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**A Study of Icatibant (TAK-667) in Japanese Children and Teenagers with Acute Attacks of HAE**

– recruiting in Japan

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**Biomarker for HAE Disease**

– recruiting in Armenia, Georgia, Peru, Poland, Romania and Turkey

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**Cloud-R HAE Registry**

– recruiting in France

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**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 and Sweden



**EU Clinical Trials Register**

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#### **CSL312 (Garadacimab) in the Prevention of HAE Attacks**

– recruiting in Canada, Germany, Hungary, Israel, the Netherlands and the USA

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#### **Contrast-Enhanced Ultrasound for the Evaluation of Changes in Tumor Blood Flow Surrounding HAE**

– recruiting in the USA

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

– recruiting in Canada, France, Germany, Hungary, Italy, the Netherlands, Spain and United Kingdom

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#### **Efficacy and Safety of Lanadelumab (SHP643) in Japanese Participants with HAE**

– recruiting in Japan

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#### **Epidemiological Analysis for HAE Disease**

– recruiting in Germany, Italy, Japan, Poland, Turkey and United Kingdom

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#### **Expanded Access Program with Lanadelumab for Japanese People with HAE**

– recruiting in Japan

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#### **Firazyr General Drug Use-Results Survey (Japan)**

– recruiting in Japan

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#### **Firazyr Patient Registry (Icatibant Outcome Survey - IOS)**

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

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

– recruiting in Italy

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#### **Hereditary Angioedema Kininogen Assay**

– recruiting in Germany

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

– recruiting in the USA

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#### **Patient Registry to Evaluate the Real-world Safety of Ruconest**

– recruiting in the USA

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

– recruiting in Italy

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**Study to Evaluate the Real-world Effectiveness of Lanadelumab in Participants with HAE**

- recruiting in Canada, Puerto Rico and the USA

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

- recruiting in Austria, Germany, Israel, Switzerland and United Kingdom

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

- recruiting in France

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

- will be recruiting in Austria, France, Germany and Greece

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

- will be recruiting in Russia

Read more about these and other clinical trials at:

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



# NEWS FROM THE INDUSTRY

## 30 March 2021

Data from a Phase 2 clinical study of IONIS-PKK-LRx from **Ionis Pharmaceuticals, Inc.** meets its primary and secondary endpoints, achieving significant reductions in the number of attacks suffered by patients with HAE compared to placebo. The study demonstrated a mean reduction of 90% in the number of monthly HAE attacks in weeks one to 17 of the study ( $p < 0.001$ ) and a mean reduction of 97% in the number of monthly HAE attacks in weeks five to 17 ( $p = 0.003$ ). In weeks five to 17, 92% of patients treated with IONIS-PKK-LRx were attack-free compared to 0% in the placebo group ( $p < 0.001$ ).

IONIS-PKK-LRx was developed using Ionis' advanced ligand-conjugated antisense (LICA) technology. IONIS-PKK-LRx is one of Ionis' wholly owned medicines to treat rare diseases.

IONIS-PKK-LRx is an investigational antisense medicine designed to reduce the production of prekallikrein, or PKK, which plays a key role in the activation of inflammatory mediators associated with acute attacks of HAE. Physicians have long prescribed prophylactic treatment approaches, including C1-INH replacement therapies and more recently inhibitors of plasma kallikrein, to prevent and reduce the severity of HAE attacks. Despite these available therapies, patients with HAE may experience breakthrough attacks.

"These topline Phase 2 study results support a profile for IONIS-PKK-LRx as a potential best-in-class prophylactic treatment for patients with HAE, with excellent efficacy, safety and tolerability along with the convenience of once per month low volume subcutaneous injections," said Kenneth Newman, M.D., M.B.A., Ionis' vice president of clinical development and leader of the pulmonology and immunology franchise. "These results highlight the potential benefits and advantages of IONIS-PKK-LRx for the treatment of hereditary angioedema and more broadly underscore the power of Ionis' antisense technology to target the root causes of rare diseases like HAE."

In the Phase 2 clinical study, 20 adults with Type 1 or Type 2 HAE were randomized and received either IONIS-PKK-LRx 80mg (n=14) or placebo (n=6) subcutaneously

once monthly for 17 weeks. The primary endpoint was the reduction of monthly HAE attacks compared to placebo. Secondary endpoints included the reduction of monthly attacks in weeks five to 17, reduction in the number of moderate or severe attacks in weeks one to 17, the number of moderate or severe attacks in weeks five to 17 and the number of attacks requiring acute therapy in weeks five to 17. The majority of adverse events during the study were mild with a frequency that was similar between groups. The most common treatment-emergent adverse events (TEAEs) were headache and nausea, which were seen more frequently in the placebo arm compared to the active treatment arm. Ionis expects to present a full analysis of its Phase 2 study of IONIS-PKK-LRx at a medical conference later this year.

(Source: Ionis)



## 14 April 2021

The Japanese National Health Insurance System (NHI) has approved the addition of oral, once-daily Orladeyo (berotralstat) from **BioCryst Pharmaceuticals, Inc.** to the NHI drug price list on 21 April 2021.

Oral, once-daily Orladeyo was approved in Japan in January 2021 for prophylactic treatment of HAE in adults and pediatric patients 12 years and older. Orladeyo is the first and only prophylactic HAE medication approved in Japan.

Orladeyo will be commercialized in Japan by BioCryst's partner, Torii Pharmaceutical Co., Ltd. Torii plans to launch Orladeyo following the NHI drug price listing.

"Our goal is to bring Orladeyo to HAE patients around the world who want a new oral, once-daily option to prevent their attacks. In Japan, Orladeyo is the first approved prophylactic HAE medication, which has the potential to significantly impact the lives of HAE patients," says Jon Stonehouse, President and CEO of BioCryst.

BioCryst received Orphan Drug and Sakigake designation for Orladeyo in Japan. The APeX-J trial in Japan met its primary endpoint ( $p=0.003$ ) of a reduction in HAE attacks from baseline for Orladeyo 150 mg compared to placebo, and Orladeyo was safe and generally well-tolerated in the trial. In APeX-2, Orladeyo also met its primary endpoint ( $p<0.001$ ) for Orladeyo 150 mg compared to placebo and was safe and generally well-tolerated.

(Source: BioCryst)



## 20 April 2021

The U.S. Food and Drug Administration (FDA) has notified **KalVista Pharmaceuticals, Inc.** in a letter that it has placed a clinical hold on the proposed Phase 2 clinical trial of KVD824.

KVD824 is KalVista's oral product candidate being developed for prophylactic treatment of HAE. An Investigational New Drug Application was submitted earlier in 2021 for a Phase 2 clinical trial to evaluate KVD824 as a potential prophylactic treatment for the prevention of HAE attacks. The FDA letter requests further information and analysis related to certain preclinical studies of KVD824 submitted to support the planned Phase 2 trial. Refinements were also proposed to the intended KVD824 Phase 2 study protocol. No new studies were requested nor was it suggested that new data be generated to initiate the Phase 2 trial.

"We intend to fully comply with the requests and recommendations provided by the FDA," says Andrew Crockett, CEO of KalVista. "Although we no longer can confirm that the KVD824 Phase 2 trial will initiate this quarter, we are working to resolve their concerns in a timely fashion. Importantly, this letter relates solely to KVD824, and does not impact our activities or expectations with regard to KVD900, for which we continue to prepare for an End of Phase 2 FDA meeting and commencement of our Phase 3 efficacy trial."

KalVista has previously reported data from first-in-human and formulation studies of KVD824 that were conducted in the UK. To date, a total of 121 subjects have received KVD824 as single doses up to 1280 mg and up to 14 days of twice-daily dosing of 600

mg and 900 mg. In both studies adverse event rates were similar in placebo and active arms, no subjects withdrew and no serious adverse events were reported.

KalVista will continue to work closely with the FDA on the overall development of KVD824 and will provide further updates as appropriate.

(Source: KalVista)



## 20 April 2021

**Terumo Blood and Cell Technologies** and **CSL Plasma** collaborate to deliver a new plasma collection platform at CSL Plasma U.S. collection centers. A clinical trial of the investigational plasmapheresis device began earlier in April 2021, and introduction of the new platform is subject to U.S. Food and Drug Administration device clearance, at which time the parties will provide further comment.

Plasma is a key ingredient for therapies crucial to treating patients around the world suffering from a host of life-threatening conditions, such as HAE, primary immune deficiencies, autoimmune diseases, hemophilia and inherited respiratory disease. Plasma is also commonly given to patients experiencing trauma, burns and organ transplant surgeries.

(Source: Terumo)



## 1 May 2021

The European Commission (EC) has approved oral, once-daily Orladeyo (berotralstat) for the prevention of recurrent HAE attacks in patients 12 years and older.

"As the first targeted oral prophylactic therapy approved in Europe, Orladeyo represents a major advance in treatment for HAE patients who have been waiting for a preventive therapy. Physicians will be delighted to discuss this new option with their patients," says Emel Aygören-Pürsün, M.D., Head of the HAE Center at the University Hospital in Frankfurt.

“Orladeyo offers people with HAE in Europe and their physicians the first orally administered non-steroidal option for preventing HAE attacks and represents a vitally important and most welcome step in making more treatment options available,” says Henrik Balle Boysen, Executive Vice President and COO of HAE International.

The EC approval of Orladeyo is applicable to all European Union member states plus Iceland, Norway and Liechtenstein.

**BioCryst Pharmaceuticals, Inc.** has its European commercial team in place and expects to launch Orladeyo this quarter in Germany, with launches in other European markets to follow. HAE patients in France currently have access to Orladeyo through an Autorisation Temporaire d'Utilisation de cohorte (cohort ATU). In the United Kingdom, HAE patients also currently have access to Orladeyo through an approved early access to medicines scheme (EAMS). A marketing authorization application (MAA) has been submitted to the Medicines and Healthcare products Regulatory Agency (MHRA). Under the new European Commission Decision Reliance Procedure, the MHRA will aim to complete the review of the UK MAA as soon as possible following the EC approval decision.

“Most European HAE patients today treat their disease with on-demand therapy or androgens and we believe the approval of oral, once-daily Orladeyo provides an exciting new opportunity for these patients to reduce their burden of therapy by moving to prophylaxis with Orladeyo,” says Jon Stonehouse, President and CEO of BioCryst. “We saw tremendous enthusiasm and participation from European HAE patients in our clinical trials and we have invested in an experienced European commercial team that is excited to bring Orladeyo to HAE patients across Europe.”

In the pivotal Phase 3 APeX-2 trial, Orladeyo significantly reduced attacks at 24 weeks, and this reduction was sustained through 48 weeks. HAE patients who completed 48 weeks of treatment (150 mg) saw reductions in their HAE attack rates, from a mean of 2.9 attacks per month at baseline to a mean of 1.0 attacks per month after 48 weeks of therapy. In the long-term open label APeX-S trial, patients completing 48 weeks of therapy (150 mg) had a mean attack rate of 0.8 attacks per month. Orladeyo was safe and well tolerated in both 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.

HAE patients note a significant treatment burden associated with existing prophylactic therapy. In addition to reducing HAE attack rate, data from APeX-2 show that patients reported meaningful improvements in both quality of life, overall patient-reported satisfaction, and significant reductions in their monthly use of standard of care on-demand medicine, while taking oral, once-daily Orladeyo (150 mg).

(Source: BioCryst)



## 6 May 2021

Announcing the **BioCryst Pharmaceuticals, Inc.** financial results for the first quarter ended 31 March 2021, President and CEO Jon Stonehouse says:

“Our commercial team is off to an outstanding start with the U.S. launch of Orladeyo. In this highly competitive market, we are demonstrating what we have known for some time now, HAE patients have been waiting to switch to an oral, once-daily therapy to reduce their attacks and burden of therapy. Our early launch performance is the latest piece of evidence that our differentiated strategy to discover, develop and, now, successfully commercialize unique oral medicines for rare diseases has the potential to create greater and greater value.”

The majority of the Orladeyo revenue in the first quarter of 2021 came from new patients who switched to Orladeyo from either injectable/infused prophylactic medications or from acute-only treatment. The remainder came from patients transitioning from clinical trials and the company's early access program.

(Source: BioCryst)





## 6 May 2021

Reporting the **Intellia Therapeutics, Inc.** operational highlights and financial results for the first quarter ended 31 March 2021, President and CEO John Leonard, M.D., says:

“Intellia continues to build momentum as we progress our full-spectrum pipeline towards key upcoming milestones this year. We plan on submitting first-in-human regulatory filings for NTLA-2002 for HAE.”

NTLA-2002 aims to prevent attacks for people living with HAE after treatment consisting of a single administration. Intellia is applying its modular LNP delivery system to develop NTLA-2002 to knock out the KLKB1 gene in the liver to permanently reduce plasma kallikrein activity. This approach is expected to provide continuous suppression of kallikrein activity and eliminate the significant treatment burden associated with currently available therapies for HAE patients.

Intellia expects to submit a regulatory application for NTLA-2002 in the second half of 2021. The first-in-human trial is expected to evaluate safety, tolerability and activity in patients with HAE.

In March, Intellia presented preclinical results confirming greater reductions in serum kallikrein protein levels and activity versus the current standard of care for HAE, sustained over 17 months following a single dose in an ongoing non-human primate study of its cyno-specific LNP formulation for NTLA-2002. Additionally, the Intellia presented data from a humanized KLKB1 mouse model of bradykinin-mediated vascular permeability, establishing that a single administration of NTLA-2002 prevented captopril-induced vascular leakage. These results, which affirm NTLA-2002's therapeutic hypothesis of preventing HAE attacks, were presented at the American Academy of Allergy, Asthma & Immunology (AAAAI) 2021 Annual Meeting.

(Source: Intellia)

**Intellia**  
THERAPEUTICS

## 11 May 2021

HAEGARDA (C1 Esterase Inhibitor Subcutaneous [Human]) from CSL Behring – a subcutaneous C1 esterase inhibitor (C1-INH) therapy indicated for routine prevention of HAE attacks in adolescent and adult patients – is now available in all of Canada.

“As a physician that treats this disease, I'm pleased to have HAEGARDA available for patients across Canada. HAEGARDA represents another needed therapeutic advancement for the prevention of HAE as the only Subcutaneous C1 Inhibitor for preventative therapy approved in Canada”, says Dr. Stephen D. Betschel, Clinical Immunologist and Allergist, St. Michael's Hospital, Toronto.

HAE Canada President, Jacquie Badiou, COO, Daphne Dumbrille, and the Board of Directors, are excited that at long last, HAEGARDA is now equally available, without restrictions, to adolescent and adult HAE patients across Canada. This represents yet another milestone for the advancement of care for Canadian HAE patients who need a subcutaneous C1 esterase inhibitor therapy.

**CSL Behring Canada** also assists patients to get access to training for the administration of HAEGARDA via the CSL Behring PLUS+ program that offers services and educational materials. Patients can ask their healthcare professionals to enroll them in the program at any time.

(Source: CSL Behring Canada)

**CSL Behring**

## 11 May 2021

At the presentation of the **Takeda Pharmaceutical Company Limited** financial results for the fiscal year 2020 (period ended 31 March 2021), President and CEO Christophe Weber says:

“Takeda remained resilient as we operated in new ways through the COVID-19 pandemic. We maintained business continuity, ensured patient access to our medicines and safeguarded the health and well-being of our employees. This enabled us to deliver on our full-year management guidance, with underlying revenue growth driven by our 14 global brands.”

During 2020 the growth in the HAE portfolio was driven by excellent performance from Takhzyro, which continues to expand the HAE prophylaxis market, as well as launching into additional geographies.

“As we celebrate Takeda’s 240th anniversary in June 2021, I am extremely proud of our progress and confident in our outlook for the future. We will advance on our growth trajectory, maximize value creation for all of our stakeholders, and continue to position the company for long-term success”, says Christophe Weber.

(Source: Takeda)



### 13 May 2021

The United Kingdom’s Medicines and Healthcare products Regulatory Agency (MHRA) has granted marketing authorization for oral, once-daily Orladeyo (berotralstat) for the routine prevention of recurrent HAE attacks in HAE patients 12 years and older.

“HAE UK welcomes the decision as HAE is an unpredictable and life-threatening condition which causes significant emotional and economic burdens on people with HAE and their families and careers. An oral therapy that shows effective control of HAE attacks will provide a different treatment choice for clinicians and patients and will assist in improving the quality of life of those living with the condition,” says Laura Szutowicz, CEO of HAE UK.

“This is a significant milestone in furthering our ability to meet the needs of more patients living with HAE. As the first oral, once-daily therapy proven to reduce the number of HAE attacks, today’s news has the potential to offer a convenient new treatment option to improve the lives and outcomes of patients with HAE,” says Dr. Sorena Kiani, consultant immunologist at Barts Health NHS Trust.

A decision from the National Institute for Health and Care Excellence (NICE) and Scottish Medicines Consortium (SMC) for use of Orladeyo under the UK’s National Health Service (NHS) is anticipated in the fourth quarter of 2021.

“With the approval of the first oral, once-daily treatment in the UK, BioCryst continues to bring Orladeyo to

HAE patients and their families around the world,” says Jon Stonehouse, President and CEO of **BioCryst Pharmaceuticals, Inc.**

In the pivotal Phase 3 APeX-2 trial, Orladeyo significantly reduced attacks at 24 weeks, and this reduction was sustained through 48 weeks. HAE patients who completed 48 weeks of treatment (150 mg) saw reductions in their HAE attack rates, from a mean of 2.9 attacks per month at baseline to a mean of 1.0 attacks per month after 48 weeks of therapy. In the long-term open label APeX-S trial, patients completing 48 weeks of therapy (150 mg) had a mean attack rate of 0.8 attacks per month.

Orladeyo was safe and well tolerated in both 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.

HAE patients note a significant treatment burden associated with existing prophylactic therapy. In addition to reducing HAE attack rate, data from APeX-2 show that patients reported meaningful improvements in both quality of life, overall patient-reported satisfaction, and significant reductions in their monthly use of standard of care on-demand medicine, while taking oral, once-daily Orladeyo (150 mg).

(Source: BioCryst)



### 16 May 2021

**Pharming Group N.V.** supports the 10th annual **hae day :-)**, the global awareness day for HAE. CEO Sijmen de Vries comments:

“We are proud to support the 10th annual **hae day :-)** to raise awareness of HAE and better quality of life for patients and their families around the world. Pharming remains dedicated to making a positive difference to patients with this rare life-threatening condition so they can receive an accurate diagnosis and appropriate medical care.”

(Source: Pharming)



## 16 May 2021

The pharmacy, distribution and patient management organization **Optime Care** is pleased to recognize **hae day :-)** and celebrate the 10th anniversary of an awareness campaign that unites the HAE community.

“Access to innovative therapies that can improve quality of life is critical for people with rare diseases like HAE,” says Donovan Quill, President and CEO, Optime Care. “We are proud to be partnering with BioCryst to help bring the first oral, once-daily prophylaxis medicine to members of the HAE community. This year, Optime Care is sharing care coordinator testimonials about their experience working with HAE patients.”

Testimonials:

“My life has been touched in so many ways by the patients I have had over the years, but never quite the way that my HAE patients have. They motivate and encourage me, and they have helped me grow into the best care coordinator I can be.” – Liz, Care Coordinator, Optime Care

“Hearing a patient say I’ve taken a burden off their shoulders or how much they appreciate the efforts I put in is amazing. Patients have reported being able to regularly see their facial features now or being attack-free for the first time in their lives. I am so grateful to be a part of this change.” – Rochelle, Care Coordinator, Optime Care

(Source: Optime Care)



## 24 May 2021

**Glenmark Pharmaceuticals Limited** has received final approval by the United States Food & Drug Administration (U.S. FDA) for Icatibant Injection, 30 mg/3 mL (10 mg/mL) Single-Dose Prefilled Syringe, the generic version of Firazyf Injection, 30 mg/3 mL (10 mg/mL) Single-Dose Prefilled Syringe, of Shire Human Genetic Therapies, Inc. This marks Glenmark’s first synthetic decapeptide injectable approval and will be manufactured in facility based in Monroe, North Carolina.

Glenmark’s current portfolio consists of 172 products authorized for distribution in the U.S. marketplace and 44 ANDA’s pending approval with the U.S. FDA. In addition to these internal filings, Glenmark continues to identify and explore external development

partnerships to supplement and accelerate the growth of its existing pipeline and portfolio.

(Source: Glenmark)



## 26 May 2021

At the presentation of the **Pharvaris** financial results for the first quarter ended 31 March 2021, co-founder and CEO Berndt Modig said:

“We continue to execute on a development strategy which we believe provides value to shareholders and patients, as demonstrated by the continued enrollment of our Phase 2 on-demand study of PHVS416, from which we plan to report data next year. In order to meet the unmet need of many HAE patients demanding oral alternative therapies, we plan to initiate our HAE CHAPTER-1 Phase 2 prophylactic study of PHVS416 this year. Pharvaris remains committed to providing access to medicines for patients in need.”

*From the pipeline:*

PHVS719 is an extended-release formulation of PHA121 intended for use in the prophylactic treatment of HAE. The company expects to initiate a Phase 1 pharmacokinetics study by the end of 2021.

(Source: Pharvaris)



## 3 June 2021

Oral, once-daily Orladeyo (berotralstat) from **BioCryst Pharmaceuticals, Inc.** was approved by the European Medicines Agency (EMA) on 30 April 2021 for the prevention of recurrent HAE attacks in patients 12 years and older - and now the product is available for patients with a prescription in Germany.

“We have an experienced team in place in Germany that is excited and honored to bring the first oral, once daily therapy to HAE patients in Europe,” says Charlie Gayer, Chief Commercial Officer of BioCryst. “Patients and physicians across Europe have told us about the significant need for a targeted oral therapy for HAE,



and Germany is the first of many upcoming European launches for Orladeyo.”

(Source: BioCryst)



#### 4 June 2021

**Pharming Group N.V.** has reached an agreement with the Spanish Ministry of Health to grant reimbursement for Ruconest (conestat alfa) in Spain. Ruconest is the first and only plasma-free recombinant human C1 esterase inhibitor (rhC1INH) protein replacement therapy approved for the treatment of acute HAE attacks in adults and children aged two years and over.

Clinical trial evidence has demonstrated that rhC1INH is efficacious and well-tolerated, and these results have been further confirmed in real-world observational studies.

Mrs Sarah Smith, President of the Spanish HAE Patient Association (AEDAF), says: “We welcome the approval of new treatments which might help alleviate the suffering of patients with HAE.” And Sijmen de Vries, CEO of Pharming, comments: “We are delighted with this positive reimbursement decision by the Spanish Ministry of Health, as it means patients in Spain in need of new treatment options for HAE will now be able to access Ruconest. We look forward to working with the Spanish healthcare community to ensure a rapid rollout of the product.”

(Source: Pharming)



#### 4 June 2021

At the 12th C1 Inhibitor Deficiency and Angioedema Workshop (held virtually 3-6 June 2021), Anne Lesage, Ph.D., Chief Early Development Officer at **Pharvaris**, presents bradykinin challenge data supporting the pharmacokinetic and pharmacodynamic profile of PHA121 (PHA-022121) for the treatment of HAE.

“Findings presented here from pre-clinical and clinical studies, particularly from in vivo bradykinin challenge

studies, show that PHA121 demonstrates faster onset than icatibant in head-to-head preclinical studies and, compared to published data, is consistently more potent showing longer duration bradykinin-BR2-antagonist activity than icatibant in human pharmacodynamic studies. These data position PHA121 as a potentially valuable treatment option for both on-demand and prophylactic treatment of HAE”, says Dr. Lesage.

Berndt Modig, CEO and co-founder of Pharvaris says: “Our data demonstrate a favorable pharmacokinetic and pharmacodynamic profile of PHA121 – providing strong proof of mechanism for PHA121 and a foundation for the dose regimens to be further evaluated for HAE as we continue progressing our clinical programs using the PHVS416 and PHVS719 product formulations.”

Pharvaris established a proof-of-concept model for HAE in non-human primates using bradykinin, an endogenous peptide known to mediate signs and symptoms of HAE. The model was validated utilizing icatibant, a marketed injectable B2 receptor antagonist, providing back-translation from human clinical experience with icatibant. The objective of the study was to investigate the ability of PHA121 to attenuate blood-pressure changes induced by bradykinin injection. In this model, PHA121 inhibited bradykinin-induced changes in blood pressure at all doses tested with a faster onset of action than icatibant and the duration of the effect was dose dependent.

PHA121 was also orally administered in two double-blind, placebo-controlled single-ascending-dose studies up to 50 mg, with pharmacokinetics (PK) and safety observed for 72 hours, in healthy volunteers. Pharmacodynamic (PD) effects were evaluated with a nonlinear mixed-effect PK/PD model using 12 mg and 22 mg doses and compared to historical icatibant data. PK/PD analysis showed significant inhibition of bradykinin-induced hemodynamic changes with an average composite EC50 of 2.4 ng/mL and EC85 of 13.8 ng/mL. Single-dose treatment of PHA121 demonstrated effective bradykinin inhibition. Quantitative modeling indicates that single oral doses of PHA121 will maintain pharmacologically active drug levels for a substantially longer time than 30 mg of subcutaneous icatibant.

(Source: Pharvaris)



## 5 June 2021

At the 12th C1-Inhibitor Deficiency & Angioedema Workshop, **KalVista Pharmaceuticals, Inc.** presents clinical data supporting KVD900 as an oral on-demand treatment for HAE.

“Our goal is to provide the best outcome for HAE patients experiencing an attack, and that means offering them the ability to treat early in the attack progression to shorten attack duration,” says Andrew Crockett, CEO of KalVista. “The data reinforce that attacks treated with oral KVD900 experience a rapid improvement in symptoms which is maintained for 24 hours, while also being generally safe and well tolerated. We look forward to providing updates on KVD900’s upcoming Phase 3 program.”

Following is a brief summary of the presentation “Fast improvement of hereditary angioedema (HAE) attacks with the oral on-demand plasma kallikrein inhibitor KVD900: an analysis of the pharmacokinetic and pharmacodynamic profile of KVD900 and attack symptom severity during a double-blind, randomized phase 2 cross-over trial in patients with HAE type I and II” given by Andrea Zanichelli, MD:

- KVD900 achieves rapid plasma exposures, with near complete plasma kallikrein inhibition within 30 minutes.
- Attacks treated with KVD900 experienced a rapid improvement in the most severe baseline symptoms both in absolute terms and relative, when expressed as change from baseline. This improvement was maintained for 24 hours.
- KVD900 significantly accelerated improvement in attack severity as assessed using a composite Visual Analog Scale (VAS). A 50% reduction in the composite VAS was reached for 50% of the attacks treated with KVD900 within six hours versus greater than 12 hours for attacks treated with placebo. By 24 hours, 74% of KVD900-treated attacks showed a 50% reduction in score compared to 38% for placebo-treated attacks.
- Adverse events were of mild or moderate intensity with no severe or serious adverse events reported. Overall, the single administration of KVD900 appeared safe and well tolerated.

(Source: KalVista)



## 16 June 2021

The Israeli Ministry of Health has accepted the regulatory submission of Orladeyo for the prevention of recurrent attacks in patients with HAE 12 years and older. The Ministry also has granted an accelerated review. In addition, **BioCryst Pharmaceuticals, Inc.** has entered into a distribution and supply agreement granting Neopharm Ltd., a corporation organized under the laws of the State of Israel, the exclusive rights to commercialize Orladeyo in Israel.

“Neopharm is the right partner to help us commercialize in Israel as we continue to bring oral, once-daily Orladeyo to HAE patients around the world. They have extensive rare disease experience and proven commercial success in Israel, and they understand the local regulatory environment,” says Charlie Gayer, Chief Commercial Officer of BioCryst.

“We are proud of our partnership with BioCryst and excited to deliver a new and innovative treatment option to HAE patients in Israel. The momentum gained from recent approvals of Orladeyo across the globe will support our commercialization efforts to provide access to this important treatment,” says Efi Shnaidman, general manager of Neopharm Israel.

Founded in 1941, Neopharm Israel is one of the leading pharmaceutical companies in Israel, providing the Israeli market with a wide range of products and integrated services for patients in need, with a proven track record of successful market access and launches. In addition to exclusive rights to commercialize Orladeyo in Israel, Neopharm Israel has exclusive rights to commercialize in the Palestinian Authority.

(Source: BioCryst)



# Have no fear – take the vaccine



“Have no fear – take the vaccine.” That is, in short, the main message from HAEi’s Chief Medical Advisor, Professor Marcus Maurer, when it comes to people with HAE getting the COVID-19 vaccine.

In a video recorded interview with Anthony J. Castaldo, President and CEO of HAEi, Professor Maurer says:

“Having HAE is not a risk to the vaccine not working or having side effects. It is important that HAE patients get vaccinated so as to avoid COVID-19 because, on the flip side, COVID-19 can induce attacks, worsen attacks, so protection from the disease and from attacks is important for HAE patients. Have no fear – take the vaccine.

Some of the questions we receive from the HAE community have to do with the treatment of HAE. We all know it is very important to have treatment – treatment for the attacks and probably more important prevention of the attacks. What patients want to know is whether this treatment can be continued while waiting for the vaccine and if it can be co-administered with the vaccine. The answer is yes. The treatment should be continued, and the treatment should be given together with the vaccine – not necessarily on the same day, but there is no harm in having good treatment and the vaccine. You should have both.”

Regarding the fear of side effects, Professor Maurer says:

“HAE is not a risk factor for the vaccine not working or having side effects but having HAE does not exclude you from also having allergies. Here we need to look a little bit closer. By and large, having an allergy, hay fever is not a problem when it comes to the vaccine, but having severe anaphylactic reactions in the past may be a risk factor. So, we want to make sure that when we administer the vaccine that patients are monitored for some time after receiving the vaccine and that we look closer when patients tell us that they’ve had serious allergic reactions previously, through drugs, through foods. This is a small fraction of all patients with allergies, but these are the patients we have an eye out for and where we look more closely and take precautions.

HAE is a disease where attacks can be triggered by viral infections; most HAE patients know this. If there is a way to prevent that – to not allow for an infection which is what a vaccine does – then that’s a good way to stay healthy. There is really no reason to be afraid of this vaccine as an HAE patient, and there are lots of benefits to be gained from this vaccine. So, when you get the chance, go out and get vaccinated.”

One HAEi’s website you will find information from HAEi and the organization’s medical experts about COVID-19 and issues especially relevant for people who have HAE. View videos and infographics and get information about COVID-19 and HAE at: [haei.org/covid-19-and-hae](https://haei.org/covid-19-and-hae)





## HAEi AROUND THE WORLD

Currently there are HAE member organizations in **93** 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.

