


# Global perspectives:

HAEI MAGAZINE · ISSUE 1/2020

 85 Member countries





**Global Perspectives**  
**Issue 1/2020**  
**April 2020**

**Cover photo**

HAE patient Noah Davis-Logan,  
the United States of America  
– read his story on page 22

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



## DEAR HAE INTERNATIONAL FRIENDS,

On behalf of the HAE International Board of Directors and the entire team, please accept our sincere hope that you and your families are remaining healthy and safe as you deal with COVID-19 Coronavirus pandemic. These are unprecedented and stressful times as governments and health care systems work to treat those who have been infected and control the spread.

Please know that you are not alone in dealing with the pandemic and that we care deeply about your health and well-being. The HAE International Team and the Regional Patient Advocates are available to support our member organizations and we will continue sharing information from credible sources. Page 6 of this issue of *Global Perspectives* contains a recent message to the community outlining the current understanding of HAE and COVID-19. Keep in mind that you can also stay connected on a personal level by sharing information and ideas with the extraordinarily kind and compassionate global community of HAE patients and caregivers on HAE International's Facebook page. See [www.facebook.com/groups/HAEinternational](https://www.facebook.com/groups/HAEinternational).

We canceled the 14-17 May 2020 HAE Global Conference, where 1,000 HAE International friends from 75 countries were set to meet, first and foremost, out of concern for your health and safety, and in light of border closings, flight limitations, and individual country restrictions. On page 10, we provide a "Frequently Asked Questions" feature that includes answers to inquiries on registration fee refunds and flight cancellations, and other related matters.

Challenging times like these can be made a bit easier when we are reminded of struggles that others have faced and overcome. This issue of *Global Perspectives* features the story of a Noah Davis-Logan, an extraordinary young man from a strong, loving family. Noah started having severe attacks at age three but did not initially have access to a modern HAE medicine. In spite of his illness and all of the limitations it imposed at such an early age, this impressive young man never let HAE keep him down. He has blossomed into an elite high school student athlete who thrives in numerous activities including acting. Notably, Noah is a passionate HAE youth advocacy leader who is frequently a featured speaker at HAE events throughout the globe. His story is an inspiration to all of us!

Finally, do not hesitate to call your HAE doctor if you have any questions. Please accept my most sincere hope that you and your loved ones stay healthy and safe by following the hygiene, social distancing and other guidelines offered by your government and the World Health Organization.

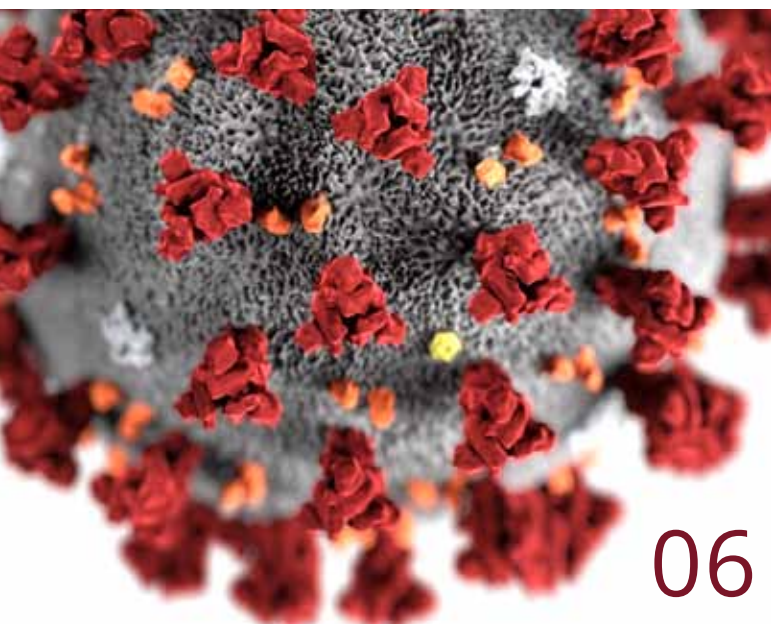
Warmest regards,

Anthony J. Castaldo  
President & CEO, HAE International



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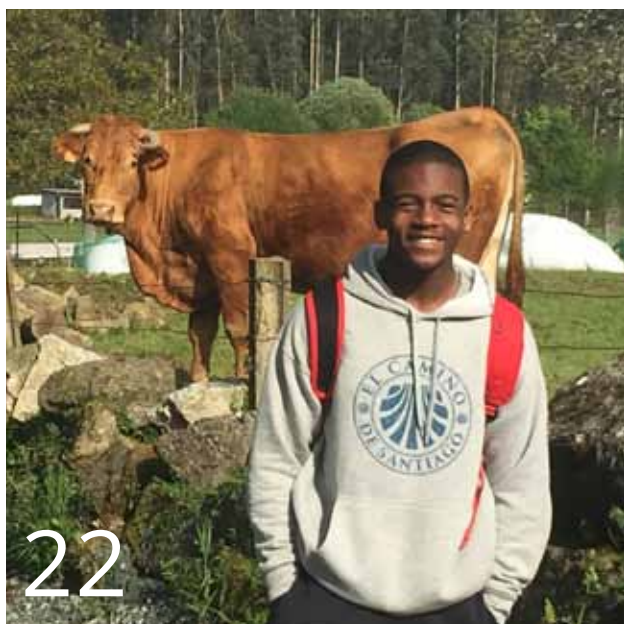
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# IMPORTANT INFORMATION ABOUT COVID-19 AND HAE

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The health and well-being of the global community is HAE International's top priority. With that in mind, the organization has asked its medical experts to provide their best thinking regarding the COVID-19 virus health risks that might be faced by people who have HAE and other forms of recurrent angioedema.

COVID-19 is a new virus and to this point the medical community has very little experience in relation to HAE and other forms of recurrent angioedema. Nevertheless, expert physicians have told HAE International that having HAE (types I/II or normal C1 inhibitor) or other forms or recurrent angioedema does not increase:

1. The risk of being infected with COVID-19, nor
2. The severity of infection if the virus is contracted.

Furthermore, infections of any type may be a trigger for attacks, so it is important to make sure medications are taken as prescribed and that prescriptions are kept current.

It is also important that everyone considers other non-HAE health conditions that may be specific risk factors of COVID-19.

Please follow instructions from governmental and local health authorities which includes frequent hand washing, social distancing, and avoiding travel.

HAE International recommends that you contact your HAE physician if you have specific questions.

Take good care of yourself and your family – stay safe!

## EMERGENCY CARD NOW IN 30 LANGUAGES

The HAE International Regional Patient Advocates have developed a template Emergency Card with clear and straightforward information about HAE and treatment required during an attack. It also contains space for patients to add personal information such as emergency contact details and their specialist treatment center.

At this point, the Emergency Card has been translated into 30 languages and is being used by HAE advocacy organizations around the world.

“Each card is designed to be printed at home, cut out and folded in half to create a format that can fit

within a wallet or purse. It is designed to be used by HAE patients from all countries and can be adapted by any HAE International member organization. If you are a member organization and have designed your own emergency card that could be displayed on the HAE International website, or if you would like help from us in adapting a card, please contact me”, says Chief Specialist Projects and Research Deborah Corcoran:

“If you are not a patient or part of an HAE International member organization and would like to use one of the cards, please contact the Regional Patient Advocate for your area and inform him or her that you plan to use the card.”

**Deborah Corcoran:**  
d.corcoran@haei.org

**Regional Patient Advocates:**  
<https://haei.org/about-haei/meet-the-rpas>









## CANCELLATION OF THE 2020 HAE GLOBAL CONFERENCE

HAE International has been carefully monitoring the developing situation regarding COVID-19 (novel coronavirus) as the health and well-being of everyone in the HAE community is of paramount importance.

“HAE International leadership has been meeting on a daily basis to carefully assess the evolving situation and has determined that we cannot ensure the health and safety of attendees at such a large global gathering. In addition, we are faced with boarder closings, flight limitations, and individual country restrictions. Based on these factors, we have decided to cancel the 2020 HAE Global Conference scheduled to take place 14-17 May 2020 in Frankfurt, Germany”, says Anthony J. Castaldo, HAE International President and CEO.

### **If you have received a travel grant (flight tickets paid by HAE International)**

HAE International will cancel your itinerary (air travel) at no cost for you. However, HAE International cannot refund purchases for upgrades, date changes or other services. HAE International will cancel your full hotel reservation booked through the conference website and will calculate and refund the total amount of the registration fees paid, which may include the Jakobusweg Walk, Sightseeing Tour of Frankfurt, and any additional room nights purchased through the HAE International registration system.

### **If you have purchased your own flight tickets**

You will need to reach out to your travel agency or airline for cancellation policies and procedures. HAE International cannot reimburse any individually purchased flight ticket nor any other travel related costs (passports, visas, additional room nights bought outside HAE International’s registration system, and other arrangements). HAE International will cancel your full hotel reservation booked through the conference website and will calculate and refund the total amount of the registration fees paid, which may include the Jakobusweg Walk, Sightseeing Tour of Frankfurt, and any additional room nights purchased through the HAE International registration system.

Given the uncertainties surrounding the transmission patterns of the COVID-19 (novel coronavirus), HAE International is not planning to reschedule the global conference in 2020.

When contacting HAE International regarding any conference related questions, please allow additional time for receiving a response.

Please, only contact HAE International through the dedicated email address: **covid-19@haei.org**.

# FAQ: CANCELLATION OF THE 2020 HAE GLOBAL CONFERENCE

## FREQUENTLY ASKED QUESTIONS



### **Why has the conference been cancelled?**

HAE International leadership has been meeting on a daily basis to carefully assess the evolving situation and has determined that we cannot ensure the

health and safety of attendees at such a large global gathering. In addition, we are faced with boarder closings, flight limitations, and individual country restrictions. Based on these factors, we have decided to cancel the 2020 HAE Global Conference scheduled to take place 14-17 May 2020 in Frankfurt, Germany.

### **What happens to my registration and hotel booking?**

HAE International will cancel all hotel rooms for attendees who booked within the HAE International registration system. If you booked through HAE International, there is no action you need to take to cancel your hotel room since HAE International will cancel your full reservation and issue a refund. If you made separate hotel reservations on your own (outside of the HAE International registration system) you will need to contact the hotel(s) directly to cancel your reservation. Keep in mind that any separate hotel reservations will be subject to the cancellation policy provided when you confirmed the reservation.

### **I have not received a travel grant, but I have registered for the conference, will I get my money back?**

HAE International will calculate and refund the total amount of the registration fees that you paid, which may include the Jakobusweg Walk, Sightseeing Tour of Frankfurt, and any additional room nights purchased through HAE International's registration system.

### **I have received a travel grant and planned to fly into Frankfurt only to participate in the conference. Will I get my money back?**

HAE International will cancel your flight itinerary (air travel) at no cost for you. However, HAE International cannot refund purchases for upgrades, date changes or other services. HAE International will cancel the full hotel reservation booked through the conference website and will calculate and refund the total amount of the registration fees paid, which may include the Jakobusweg Walk, Sightseeing Tour of Frankfurt, and any additional room nights purchased through HAE International's registration system.

### **Will I be responsible for any penalties associated with my cancelled travel grant flights?**

No. However, HAE International cannot refund purchases for upgrades, date changes or other services.

### **Will I be responsible for any penalties associated with cancelling my independently booked flight?**

Individuals will need to reach out to their travel agency or airline for cancellation policies and procedures.



**Will I be reimbursed for any extraordinary expenses (such as passports, visas, seat upgrades, transfers, additional accommodation costs, etc.) to travel to the Conference?**

HAE International is only able to reimburse participants for their registration fee, and the Jakobusweg Walk, Sightseeing Tour of Frankfurt, and any additional room nights purchased through HAE International's registration system.

HAE International cannot reimburse any individually purchased flight tickets nor any other travel related costs (passports, visas, additional room nights bought outside HAE International's registration system, and other arrangements). HAE International cannot refund purchases for flight upgrades, date changes or other airline services.

**Some of the people in my travel party have received a travel grant, others have funded the flight tickets themselves. Will I get my money refunded?**

HAE International will calculate and refund the total amount of the registration fees that you and your other party members paid, which may include the Jakobusweg Walk, Sightseeing Tour of Frankfurt, and any additional room nights purchased through HAE International's registration system.

*Those who received a travel grant (flight tickets paid by HAE International)*

HAE International will cancel their itinerary (air travel) at no cost for the participant. However, HAE International cannot refund purchases for upgrades, date changes or other services.

*Those who purchased their own flight tickets*

Individuals will need to reach out to their travel agency or airline for cancellation policies and procedures. HAE International cannot reimburse any individually purchased flight ticket nor any other travel related costs (passports, visas, additional room nights bought outside HAE International's registration system, and other arrangements).

**All the people in my travel party have received a travel grant, and we have an extended stay in Germany/Europe. Can we still travel?**

HAE International will cancel their itinerary (air travel) at no cost for the participant. However, HAE International cannot refund purchases for upgrades, date changes or other services. HAE International will cancel their full hotel reservation booked through the conference website and will calculate and refund the total amount of the registration fees paid, which may include the

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**What does a refund include?**

The refund includes the registration fee you paid and may also include the Jakobusweg Walk, Sightseeing Tour of Frankfurt, and any additional room nights purchased through HAE International's registration system.

**When and how will I receive my money back?**

The amount will be refunded to the credit card you have used for payment. Please allow approximately 6 weeks to process the refund, depending on country, credit/debit card and other local agreements.

**Will there be a new global conference?**

Given the uncertainties surrounding the transmission patterns of the COVID-19 (novel coronavirus), HAE International is not planning to reschedule the global conference in 2020.

# NEWS FROM THE HAEi REGIONAL PATIENT ADVOCATES

## ALL RPAs HAVE HIT THE GROUND RUNNING

Late last year, each Regional Patient Advocate (RPA) submitted their plans and budgets for 2020, and I have to say there is going to be a lot of work and progress in each region; it's an exciting year ahead.

There are also some new and innovative programs that the RPAs will be introducing for each country, one of them being the Regional Advisory Groups (RAGs), where a member from each country within each region will meet via teleconference or face to face. During these meetings, the RPAs will facilitate discussions to gain valuable feedback on challenges and how HAE International can be of further assistance.

One of the projects that the RPAs have worked on together is the 'HAEi Member Organization Code of Conduct'. The HAE International Board of Directors has approved the document, and each member organization will be receiving a copy. HAE International currently has 84 member organizations under the HAE International umbrella. While we recognize and celebrate the differences in cultures and business practices, there are certain fundamental principles and best practices that should guide HAE International Member Organization operations, and this was the impetus for the document. Please feel free to contact your RPA to receive your copy.

**Fiona Wardman**  
Chief Regional Patient Advocate

### IMPORTANT NOTICE

On the following pages on Regional Patient Advocate activities and throughout this issue of *Global Perspectives* you will find information on patient meetings, workshops and other activities during the upcoming months.

Please be aware that dates may have been changed and events cancelled due to the coronavirus COVID-19 outbreak.

You should always check the relevant national websites before planning to attend a scheduled event. You will find links to all national websites at [www.haei.org](http://www.haei.org).







**JAVIER SANTANA**  
CARIBBEAN AND CENTRAL AMERICA



I have held discussions with a pharma company from **Costa Rica** who wishes to introduce a generic HAE treatment to Costa Rica, Guatemala, and Peru. The company is currently engaged in discussions with doctors in these countries.

A European citizen will be temporarily relocating to **El Salvador** to participate in a student internship, and her parent inquired about available treatment and physician referral. The patient was directed to a knowledgeable physician in the country.

I am continuing to provide support to the member organizations and HAE patients in **Guatemala, Panama, El Salvador**, and the **Dominican Republic** through HAE International programs and services, and by helping to identify and refer more HAE patients.

It was exciting to see the patients in **Panama** getting involved in Rare Disease Day activities. Members of HAE Panama generated HAE awareness and educated the public by participating in media interviews to discuss the disease and joined in on special rare disease events along with 11 other rare disease groups. Events included sharing video testimonials for social media, joining in a human chain in front of key local buildings, and helping to coordinate the lighting of government buildings in rare disease day colors.

The first National Forum of Rare Diseases in **Costa Rica** was held on 29 February 2020. Doctors, as well as patients with rare diseases, participated in the event.



**MICHAL RUTKOWSKI**  
CENTRAL EASTERN EUROPE,  
MIDDLE EAST & GULF REGION



I organized and held Regional Workshops in Wroclaw and Lodz, **Poland**, during February.

Together with HAE Ukraine, I have been working on preparations for the national summit to be held in **Ukraine** – and along with HAE Belarus, I have worked on the concept and schedule for the immunology conference/workshops in **Belarus**.

Contact has been made with an immunologist in Riga, **Latvia** who is interested in HAE. And I am happy to report that Latvia has most recently joined HAE International as member country no. 85.

Some discussions on collaboration have taken place with Takeda in **Belgium** to help patients in this country.

The HAEi Middle East Regional Workshop took place in January in Muscat, **Oman** which brought together patients and caregivers, physicians, and pharmaceutical representatives. Keynote speakers, including knowledgeable physicians from the region, presented on the science of HAE. Patients had the opportunity to present their personal stories, which were well received and educational. Other sessions included focusing on advocacy and awareness of HAE. The meeting was a great success in helping to understand HAE in the Middle East.

I also attended and presented at the 2020 Emirates Allergy and Clinical Immunology Interest Group in Abu Dhabi in the **United Arab Emirates**.

Although the countries are not a member of HAE International, ACARE Centers have been registered in **Oman** and **Saudi Arabia**. Contact information can be found at [www.haei.org](http://www.haei.org).





**NATASA ANGJELESKA**  
SOUTHEASTERN EUROPE



In **Croatia**, I have been assisting and advising the national patient group, which was officially founded on 29 February. HAE Croatia drew some media attention and appeared on several TV shows to explain HAE. They also held a session on Rare Disease Day, which was attended by physicians and patients. HAE Croatia has teamed up with the Rare Disease Alliance in Croatia to build on its capacity to advocate.

I have been encouraging patients to take part in 'The Burden of Hereditary Angioedema (HAE) and Impact on Quality of Life: A National Survey,' a project by HAE International.

In **Bosnia & Herzegovina** as well as **Croatia**, the patient groups now have their websites under the HAE International umbrella.

I successfully worked on the preservation of annual Berinert treatments in **North Macedonia**.

Alongside the President and the Vice President of HAE Macedonia and members from the Rare Disease Alliance, I worked on organizing a public event with famous child actors, a singing group, band, and dancers on an event for Rare Disease Day. Unfortunately, the event had to be canceled under advice from the Macedonian Ministry of Health due to the first case of COVID-19 registered in Macedonia. We hope to hold the event in the future.



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



I have held calls with member organization leaders in my region, which have been very successful. So far, the participants have included leaders from **Brazil, Colombia, Mexico, Ecuador, Venezuela, Chile, and Paraguay**. During the calls, we initiated the Regional Advisory Group discussions and went through the Code of Conduct document. We also reviewed the activities for 2020 and the next steps for each country.

I have translated HAEi Connect into Portuguese for use in **Brazil**.

I attended a 'Training of Health Associations Workshop' promoted by Takeda and the CDD organization (rare disease group) on 6 February 2020.

I am providing support and materials to HAE Colombia for their Facebook page, and website hosting by HAE International.



**PATRICIA KARANI**  
SUB-SAHARA AFRICA



I am involved in an exciting HAE International pilot project in **Rwanda** with a dedicated doctor to locate patients with HAE who have been misdiagnosed.

I have established contact with a doctor in Harare, **Zimbabwe**, who has an interest in HAE.

During February, awareness campaigns were held in **Kenya**. A story was published in a free newspaper which reaches over 600,000 Kenyans daily. Dr. Priya Bowry held a one-hour training program presenting on the differences between angioedema and HAE. During the training program, I presented a patient's perspective and gave my story on my HAE journey to diagnosis. We also discussed how best to work with doctors to reduce the burden of disease. Furthermore, HAE Kenya joined with Rare Disorders Kenya to celebrate Rare Disease Day.





**MARIA FERRON**  
MEDITERRANEAN & MAGHREB COUNTRIES



During the last few months, my focus has been on the organizing of the 2020 HAEi Regional Workshop North Africa in **Algeria**, on 6-8 March. This meeting brought together patients, families, and physicians from **Libya, Tunisia, Mauritania, Morocco, and Algeria** for the first time. The agenda included a scientific track as well as a patient track to create further education and awareness of HAE in these countries.

I have been involved with meetings and follow up in **Spain** with pharmaceutical companies regarding access to HAE treatments.

I am assisting the organizations in **Morocco, Algeria, Libya, and Tunisia** with their national websites and will be helping some of these organizations with the rollout of HAEi Connect.



**FIONA WARDMAN**  
ASIA PACIFIC



I attended and presented at the Third Scientific Congress of APSID during February in Chandigarh, **India**. While there, I met with patients and the administrative team from HAE India. We had some useful discussions, including ways of getting access to modern treatment by conducting a survey, and how important it is to have all patients in India a member of HAE India with their details in HAEi Connect.

I am currently organizing the first HAEi Regional Workshop Asia Pacific in **Singapore**. As most of the countries in the region are new to HAE International, this workshop will bring together the leads from each country. During the workshop, they will learn about the HAE International tools and resources, get to know more about their neighboring country's challenges, learn about APARDO and the APEC Rare Disease Action Plan, and work on plans for each of their countries.



Please see <https://acare-network.com> for an updated list of ACARE Centers across the globe.

If you would like to become an ACARE Center please use this link for further information:  
<https://acare-network.com/index.php/become-an-acare-center>

# SPEEDY GROWTH OF NEW GLOBAL NETWORK OF SPECIALIZED TREATMENT CENTERS

In December 2019, HAE International together with GA<sup>2</sup>LEN – a non-profit European based organization of leading clinical and research facilities in the field of allergy and asthma – established GA<sup>2</sup>LEN/HAEi ACARE (Angioedema Centers of Reference and Excellence).

The joint venture with GA<sup>2</sup>LEN fulfills HAE International's longstanding goal of establishing a worldwide network of accredited angioedema care centers. Together with GA<sup>2</sup>LEN, HAE International work on developing ACARE, ensuring that it is an attractive partner for HAE treating physicians all over the world.

Establishing ACARE through cooperation with GA<sup>2</sup>LEN has given HAE International the certified accreditation program that the organization has been looking for as well as an inclusive solution where nobody will be left out as ACARE will be a worldwide network of specialized treatment centers.



Since the introduction in late 2019, the new global network of specialized treatment centers has grown at high speed. At this point, there are ACARE centers in Brazil, Bulgaria, China, Denmark, France, Georgia, Germany, India, Japan, Kuwait, Oman, Poland, Portugal, Russia, Slovenia, Saudi Arabia, South Africa, Spain, Thailand, Turkey, and the United Kingdom.

GA<sup>2</sup>LEN and HAE International have agreed that 32 requirements must be met for a hospital to become an accredited angioedema clinical care center. Among these requirements are multidisciplinary approach, structured and valid protocols of diagnosis and management, assessment of patient satisfaction and unmet needs, support of the ACARE network, a “Never give up” attitude, knowledge and use of current nomenclature and classification of angioedema, family screening, scientific and educational activities, and interaction with and support of HAE International and national angioedema patient organizations.





**90,189,232**  
**steps taken in 2019**

**69,376**

**kilometers**

**43,108**

**miles walked**

**5,190**

**participants**

**39**

**countries**

## WALKING APART – BUT STILL TOGETHER

Due to coronavirus COVID-19, the population in a large number of countries around the world experience harsh limitations to everyday life. Nevertheless, HAE International hopes that many people will find it possible to take part in this year's edition of the HAE Global Walk.

“Obviously the world is a very different place compared to last year due to the COVID-19 epidemic. Even though most countries have invoked some level of quarantine and social distancing, we notice that, now more than ever, people enjoy getting out for a walk – and can do so in compliance with the safety recommendations of their government. We therefore believe that the 2020 HAE Global Walk could be a positive activity in people's lives – because every step they take shows their willingness to raise awareness and advocate for the HAEi community. Bearing in mind that they must comply to local recommendations, we hope that many HAE friends across the world will find a way to take part in the walk,” says HAE International Executive Vice President & COO Henrik Balle Boysen.

The campaign website has been updated for this year's HAE Global Walk making it a lot easier to register your steps. All you need to do is

- Walk any distance you would like wherever you feel like it – on your own or together with others
- Report the distance (in miles or kilometers) in the form at <https://haeday.org>
- Wait for the website to add your distance – that usually happens once a day
- Walk some more and add that as well :-)

At the website, you can follow the development day by day – which countries have walked how much, how many kilometers/miles have been walked in total, and how many people have participated so far.

Please make sure to visit the website often during the campaign period from 1 April to 31 May 2020 – and add all the walks you would like. Remember: Even though many people will have to walk alone or in small groups, every step counts.



hae  
day:-)

MANY FACES  
ONE FAMILY

### IT'S EASY TO TAKE PART IN THE HAE GLOBAL WALK:

1. **Walk any distance** you would like wherever you feel like it – on your own or together with others
2. **Report the distance** (in miles or kilometers) in the form at <https://haeday.org>
3. **Walk some more** and add that as well :-)

## PATIENT STORY



NOAH DAVIS-LOGAN WISHES TO USE HIS INFLUENCE TO RAISE AWARENESS ON HAE AND TO MAKE THE WORLD A BETTER PLACE – NOT LEAST FOR HAE PATIENTS AROUND THE GLOBE.



*Patient Story: Noah Davis-Logan*

# NO HAE PATIENT DESERVES TO BE UNMEDICATED

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I had the first symptoms that I can remember at three with stomach attacks quite often. I was diagnosed the same year. Before I was born, my mother already knew that she had HAE, so she wanted me tested immediately. Despite her diagnosis, the doctors refused testing, and my attacks continued for several months before I was finally diagnosed.

*And you had proper medication from day one?*

No, unfortunately not. At the time I when I had my diagnosis, there was no medication approved for use in children, so the doctors had to administer androgens with hopes of preventing the attacks. At the age of six, I began to experience severe reactions to the androgens and was later diagnosed with Sixth Nerve Palsy, Encephalopathy, and Aseptic Meningitis as a result of the medication. Due to these severe side effects of the medication, I spent the better part of three months in and out of hospitals relearning how to walk and talk. As a result of what I had gone through, I have made it my mission to spread awareness of HAE and not least, the importance of proper therapy for children.

*How would you describe your childhood and adolescence as an HAE patient - did your condition keeps you from doing things your friends did or did you stick to your sport or other favorite activity despite having HAE?*

In the past, HAE used to stop me from playing sports, school and even going over to friends' houses. Now due to faith, support of family and friends and doctors who went above and beyond, I can do the things I love. Now I run track on a national level and a three-time AAU all American. I play street basketball whenever I can, and I refuse to let HAE stop me. I also have begun professional acting and have been cast for a major movie, two commercials, two web series and a documentary. I am involved in many clubs in my high school, and I am a leader in Student Government.

## NOAH'S STORY IN BRIEF

- Born 2004 in Atlantic City, New Jersey, USA – living at Sewell, New Jersey
- Student at Saint Augustine Preparatory School in Richland, New Jersey
- HAE symptoms started around three years of age - diagnosed shortly after
- Other HAE patients in his family: mother, grandmother



*Maybe it is not a sport as such, but you have also been walking part of the 'Camino' pilgrimage route to Santiago de Compostela in northwestern Spain. Why did you do that - and how was that experience?*

It was an honor to participate in the very first Camino Pilgrimage organized by HAE International and the Spanish HAE organization AEDAF. It was equally challenging and rewarding. This experience gave me the opportunity to meet many patients and overcome an extremely challenging hike. I was able to establish long-lasting relationships with patients and others from all over the world. Also, this helped us to show others that even though we have HAE, anything is possible; that was indeed the purpose of the Camino Walk in 2016 as well as for the subsequent walking events organized by HAE International.

*You mentioned your mother also having HAE. Are there any other members of your family with this condition?*

Yes, both my mother and my maternal grandmother were also born with HAE. Before they were diagnosed, they had no medicine, and no one would believe them whenever they had attacks that you couldn't see with the naked eye, because at the time no one had ever heard of HAE. The doctors also misdiagnosed them with allergies, so their attacks would continue and migrate. My mother lost a lot of opportunities in high school due to HAE and almost lost her life as an adult as a result of an airway swell. Now she is doing very well and hasn't had an attack in 18 months. My grandmother is doing better, as well.



*Would you say that you have met understanding from people around you - close family, other family members, friends at school, other friends?*

My family is very educated in HAE, and some of them even know how to administer my medicine. They support all my efforts to raise awareness. My community is also extremely supportive. Almost everyone who knows me closely knows that I have HAE. I believe that it is crucial for the people closest to you to know just in case of an emergency what to do. My old middle school would even throw a fundraiser and a walk for awareness.





*Talking about school: What are you studying at the moment - and what are your plans for the future?*

I'm currently in my second year of high school at Saint Augustine Preparatory School in Richland, New Jersey. I plan to graduate high school and attend a prestigious university where I will major in political science or social justice law and become a lawyer. I also wish to grow my platform and become a social influencer. I wish to use my influence to raise awareness and make the world a better place.

*I remember meeting you the first time at a US conference some five years ago. Already at that time, you were involved in raising awareness, and I guess that has evolved quite a bit since then?*

Yes, today I am involved with both HAE International and the national organization US HAEA. I actually started raising awareness when I was even younger than you mention as I spoke at the 2013 HAEA National Conference back when I was nine. Ever since then, I've had a passion for awareness, and I try my hardest no matter what to share my story. I've spoken at various conferences, colleges, sports events, and internationally. Among other things I have spoken at the International

Research Forum in Frankfurt, Germany - and last year I became the first keynote speaker under the age of eighteen to share my story with physicians and other healthcare providers at a meeting in Palm Beach, Florida.

I attended the HAE Global Conferences in Madrid, Spain in 2016 as well as the one in Vienna, Austria in 2018 and I loved it. I learned about other countries' struggles with access to therapy. Seeing what they go through motivates me to make a change, and I hope that one day I will be able to be a factor in getting these countries therapy. No HAE patient deserves to be unmedicated. I have also gained very strong friendships and bonds with the people I have met throughout conferences.

*And you are still involved with your national HAE organization?*

Yes, I am very involved in the US HAEA as I have spoken at their summits and other meetings a number of times, I am a member of the Youth Leadership Council, and I also am a US HAEA Youth Advocate. I just recently joined the HAEA internship program. I plan to do much more in the near future and hopefully change more than just my country but the world.



## 2020 HAEi REGIONAL WORKSHOP MAGHREB-NORTH AFRICA

*By Maria Ferron, Regional Patient Advocate Mediterranean & Maghreb Countries*

I was very worried that the 2020 HAEi Regional Workshop Maghreb-North Africa was bound for disaster after experiencing many problems and difficulties in the organization. Then at the last minute, I was advised that Dr. Laurence Bouillet and Dr. Teresa Caballero would not be able to attend the Workshop because of the COVID-19 situation, which then led me to worry that fear of the novel coronavirus would result in many cancellations. But in the end, it seems that the patients' fear of living with HAE without modern life-saving medication and the determination to come together and start solving their problems overcame all fear of the coronavirus and even of war. After the airport at Tripoli, Libya was bombarded, our participants from Libya had to travel 200 km to another airport to be able to leave the country!

In the end, the 2020 HAEi Regional Workshop Maghreb-North Africa took place 6-8 March at the Mercure Hotel in Algiers, Algeria. There were 80 attendees to the Workshop, 18 of whom were doctors. A representative of Takeda attended briefly on Friday afternoon.

I would especially like to thank Prof. Habib Douagui of the Algiers University of Medicine for his invaluable support and help in coordinating and organizing this meeting; he secured the High Patronage of the Minister of Health, Population and Hospital Reform for the Workshop. Many thanks also to Drs. Teresa Caballero (Spain) and Laurence Bouillet (France) who were finally able to address the Workshop via videoconference, and Professors Kamel Djenouhat (Algeria), Ahmed Elbousify (Libya), Agnes Hamzaoui (Tunisia), and Fatma Tritar (Tunisia) as well as Drs. Leith Belkahia (Tunisia), Khadija Moussayer (Morocco), and Miriam Kebe (Mauritania) for their lectures and their support and untiring efforts to help HAE patients in their respective countries. Finally, my thanks to the Board of Directors members Sarah L. Smith and Michal Rutkowski for attending in representation of HAE International.

After the Welcome on Friday afternoon, Michal Rutkowski gave a talk on "HAEi – Who we are and Global Overview". After saying that HAE is very personal and that every HAE patient is different from the next, he explained the HAE International structure, global

reach, the role of the Regional Patient Advocates and HAE International's objectives and goals. He concluded by saying that the most important goal is to find motivated patient advocates and establish patient associations around the world. Many thanks to Prof. Ahmed Elbousify for translating Michal's presentation.

Dr. Khadija Moussayer of Morocco then gave a talk on "Rare Disease Associations: What they do and benefits", with a definition of rare disease and an overview of the situation in Morocco. The Friday afternoon session concluded with a lecture by Prof. Habib Douaguie on "National Algerian Plan to Manage HAE"; although there "is a bad and difficult situation [in Algeria] like all African countries" (for example, only fresh frozen plasma is available for treatment in Algeria), there is reason for hope, including "significant engagement of HAE International with our movement" and creation of the National Association of Patients with HAE, and there are proposed plans for improvement of care, for a 2020 Algerian (and Maghrebian) Walk in April, and for a network of HAE support.

On Saturday morning, we had the following Country Presentations:

- Morocco: Dr. Othmane Boumaalif
- Tunisia: Prof. Fatma Tritar, Dr. Leith Belkahia
- Algeria: Prof. Kamel Djenouhat – "Current State of Bradykinin Angioedema in Algeria"
- Libya: Prof. Ahmed Elbousify
- Mauritania: Dr. Mariam Kebe



In general, the situation in the Maghreb is very deficient:

- **Morocco** is making some progress, although HAE is still confused with allergy; its patient association, AMMAO, was legally registered in late 2018 and now has 50+ members, and the doctors receive help for diagnosis via CREAK (Grenoble, France).
- In **Tunisia**, the patient association, TuAOH, is in the process of registration. Still, there are only 30 confirmed cases out of an estimated 120 patients in the country (based on a 1/100,000 prevalence), and the only available medications are fresh frozen plasma, tranexamic acid and danazol.
- In **Algeria** there are 144 diagnosed patients out of an estimated 850 (based on 1/50,000), the patient association should be legally registered soon (after an unsuccessful attempt in 2005), but the only available treatment is fresh frozen plasma since danazol and tranexamic acid have to be brought from abroad.
- **Libya** is in an even worse situation, with only six diagnosed patients out of an estimated 230 (based on 1/30,000), and there is a lack of everything in the country.
- Finally, in **Mauritania**, where there should be 80-100 patients, there are no patients diagnosed, but there are now implicated physicians, and Dr. Kebe hopes that a Tele dermatology pilot project they have in Mauritania to compensate for lack of specialists can be used as the basis for a similar project to identify HAE patients.

After a coffee break, we connected via teleconference with Prof. Laurence Bouillet in France and Dr. Teresa Caballero in Spain to hear their lectures on: "What is HAE & Types and Diagnosis Strategy", "HAE Modern Therapies", and "HAE in Pregnancy".

Saturday afternoon was divided into two tracks: The Patient Workshop and the Scientific Track for drawing up a Protocol for Northern Africa. During the patient workshop, all the participants, except for Sarah Smith and me, spoke in Arabic with no translation. After a short description by Othmane Boumaalif of what is being done in Morocco, Sarah gave a presentation on "Strength in Unity", in which she described the founding of the Spanish HAE organization (AEDAF), the beginnings of the C1 Inhibitor Workshop in Hungary and the creation of HAE International, stressing the importance of the HAE patient-physician relationship and of all stakeholders working together to make advances in the field of HAE. Before my presentation, I allowed some time for several patients to come up and talk about their worries, concerns and hopes for the future. In my presentation, I described all the resources,





Finally, the patients and the physicians came together at the end, and Prof. Agnes Hamzaoui of Tunisia presented the protocol drafted by the physicians during their meeting, including an Algorithm for the diagnosis of HAE and Management of HAE, both with their objectives and actions.

After the Workshop was over, I met with the member organizations' patient leads before dinner to discuss a local action plan.

projects and activities of HAE International. And finally, patients from the different countries (except Mauritania, of course, which so far has no patient representation) talked about the situation in their countries and the efforts to register patient associations.

The language barrier does make a meeting such as this one somewhat tedious and a lot slower. Still, despite this, the patients and their families were very happy to be there and couldn't thank HAE International and us enough for the chance to be able to come to this informative Workshop. Sarah and I thank Mohand Cherif Negrouche and Salah Alioui of Algeria for their excellent translation of our presentations.



# 2020 HAEI MIDDLE EAST AND GULF REGION WORKSHOP

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*By Michal Rutkowski, HAEi Regional Patient Advocate Middle East & Gulf Region, and Rashad Matraji, HAEi Contractor Patient Advocacy*

The intro into the new year was fascinating for the Middle East and Gulf Region HAE Community as the 2020 HAEi Middle East and Gulf Region Workshop took place 9-10 January at Crowne Plaza Muscat OCEC venue in Oman. The meeting was dedicated to patients and caregivers with the presence of HAE physicians from all the region, and the local media covered the workshop.

The keynote speakers were Dr. Fares Zaitoun, First President of the Pan-Arab Society of Allergy, Asthma & Immunology, Dr. Salem al Tamemi from Sultan Qaboos University Hospital in Muscat, and HAEi Executive Vice President and Chief Operating Officer Henrik Balle Boysen (online). Furthermore, we both had the pleasure of being keynote speakers.

Patient stories were delivered by Mubarak Alajami (from HAE Kuwait), Ellie Stott (from HAE United Arab Emirates), Ameera Alawad (from HAE Qatar), Husam Alhmooda (from HAE Qatar), Ahmed Lotfy (from HAE Egypt), Navin Goyal (from HAE United Arab Emirates), Nour Elchami (from HAE Lebanon), and Najlaa Alshaikh (representing Saudi Arabia).

The two days were divided into break-out sessions focusing on Advocacy, Science, and Awareness:

## FIRST DAY

- Henrik Balle Boysen welcomed the attendees and explained who HAE International is and what value the global organization brings to the HAE patients, caregivers and physicians
- Dr. Fares Zaitoun highlighted the scientific part and the awareness required among the physicians in order to identify and diagnose more HAE patients
- Michal Rutkowski spoke about the different roles the patient advocacy has taken in the region and explained about all HAE International's ongoing programs and projects
- Rashad Matraji gave a motivational presentation on how patients, together with physicians, can fight the burden of HAE and how successful it can become.

## SECOND DAY

In the general session for patients, caregivers and physicians, the stage was given to patients who spoke about their personal HAE journey. This part of the session was very impressive for everyone and significantly educational on many levels. The patient stories were followed by a presentation made by Dr. Salem al Tamemi, who shared the background of HAE, focusing on types of angioedema, diagnosis process, symptoms and treatment therapies.

In a break-out session for patients and caregivers, there was a regional perspective focusing on the role of patients' advocacy, implementation of HAE International's tools such as web hosting and HAEi Connect, and also a group discussion on challenges and opportunities in the region.

Another break-out session for health care professionals focused on topics like medications availability, patients' registry, diagnosis approach, awareness campaigns, patients' organizations, as well as regional studies and surveys.

All participants emphasized that the 2020 HAEi Middle East & Gulf Region Workshop turned out to be the best meeting of all organized so far; Motivated patients, caregivers and physicians speaking one voice in order to make a milestone step along the path to HAE medications availability. This years' workshop was also an opportunity to meet and exchanged experience with biotech representatives. One of the most active HAE pharmaceuticals companies in the region is Takeda, who took part in the event, listening to the patients' current needs and expectations.





# READY TO HOST NATIONAL WEBSITES – ALSO IN LIGHT VERSIONS

Still more national HAE organizations across the globe are letting HAE International host their websites. The reasons for this are pretty obvious if you ask Enterprise Technology Manager Ole Frölich:

“We have established an advanced system under the HAE International website allowing us to host national websites as well as provide them with templates for an individualized website – naturally all in the language of their choice. And not to forget: This service is for free for all our member organizations. At this point, there are numerous templates to choose from – and

in the backend, there are powerful editing tools for the website administrator. Furthermore, the HAE International solution supports the organization’s own domain name just as it supports https secure protocol.”

In the second half of 2019 HAE International introduced a light version of its website system. This actually allows national member organizations to come online within just a few working days.

To this date almost 40 countries have chosen to have HAE International host their website. They are:

- **Albania:** <https://haealbania.haei.org>
- **Australia:** <https://haeaustralasia.org.au>
- **Belarus:** <https://by.haei.org>
- **Bosnia & Herzegovina:** <https://haebosnia.haei.org>
- **Brazil:** <https://www.abranghe.org.br>
- **China:** <https://haechina.haei.org>
- **Costa Rica:** <https://costarica.haei.org>
- **Croatia:** <https://haecroatia.haei.org>
- **Denmark:** <https://haescan.org>
- **Ecuador:** <https://aehecuador.haei.org>
- **El Salvador:** <https://elsalvador.haei.org>
- **Greece:** <https://greece.haei.org>
- **Hungary:** <https://hungary.haei.org>
- **Hong Kong:** <https://haehk.haei.org/>
- **Iceland:** <https://iceland.haei.org>
- **India:** <https://haeindia.haei.org>
- **Kenya:** <https://haekenya.haei.org>
- **Kuwait:** <https://haekuwait.haei.org>
- **Lebanon:** <https://haelebanon.haei.org>
- **Lithuania:** <https://paelietuva.haei.org>
- **New Zealand:** <https://haeaustralasia.org.au>
- **North Macedonia:** <https://haemacedonia.haei.org>
- **Norway:** <https://haescan.org>
- **Panama:** <https://aehpanama.haei.org>
- **Peru:** <https://peru.haei.org>
- **Poland:** <https://pl.haei.org>
- **Qatar:** <https://haeqatar.haei.org>
- **Romania:** <https://romania.haei.org>
- **Serbia:** <https://rs.haei.org>
- **Slovenia:** <https://haeslovenia.haei.org>
- **South Africa:** <https://southafrica.haei.org>
- **South Korea:** <https://haekorea.haei.org/>
- **Spain:** <https://angioedema-aedaf.haei.org>
- **Sweden:** <https://haescan.org>
- **Taiwan:** <https://haetaiwan.haei.org>
- **Turkey:** <https://turkey.haei.org>
- **Ukraine:** <https://ua.haei.org>
- **United Arab Emirates:** <https://haeuae.haei.org>
- **Uruguay:** <https://uruguay.haei.org>

Ole Frölich is presently working on a handful of other national websites, hoping to be able to introduce countries like Chile, Georgia, Pakistan, Libya, and the Philippines later this year.





# NEWS FROM MEMBER ORGANIZATIONS AROUND THE GLOBE



## BOSNIA & HERZEGOVINA

The national HAE organization in Bosnia & Herzegovina now has its own website under the HAE International umbrella – have a look at <https://haebosnia.haei.org>.



## EGYPT

HAE International welcomes HAE Egypt as one more national organization with its website hosted with the global organization – please see <https://haegypt.haei.org>.



## ALGERIA

*From Salah Alioui, Vice-president of HAE Algeria:*

A popular Algerian idiom says, “There are only mountains that don’t meet”. Another way to say this is that, as long as you keep moving, you’ll meet people you never expected! This is precisely what happened years before when my old colleague Mohand-Cherif (now President of HAE Algeria) and I met for the first time during a night shift 700 km away from our homes and discovered while having a friendly chat that both our families were “rare”! The reason they were rare is that Mohand-Cherif wife and kids and my brother were diagnosed a few years before with a rare and potentially life-threatening disease, called HAE.

During that night, we talked about the importance of having a patient group to support each other in the daily struggle for medical care in a country where even the basic medication is not available. We decided hence to work together to achieve that ambitious project of a local HAE organization. We were yet aware of all the difficulties we were going to face since a few attempts were made before to create such an organization –

without any success due to the lack of commitment from doctors and the bureaucracy from the authorities!

A few years later, with a lot of determination and with the great help of the HAE International Regional Patient Advocate Maria Ferron we finally witnessed 28 December 2019 when we had our first meeting together with patients and doctors for the unique purpose of setting up our local HAE organization.





After an opening speech from doctors and organizers and discussing the technical issues for the creation of a formal organization (gathering 25 founding members representing at least 12 states and a lot of official authorizations), we invited the patients and their families to talk about their HAE stories. That moment was very emotional for patients and their families. There were a lot of tears and pain but also a lot of pride and dignity from both patients and their families. We finally came up with some suggestions to improve the care of HAE patients. Among the most important suggestions were:

- The creation of a reference center with a live phone line (24h/24) where trained doctors can back patients and local emergency doctors all around the country so that the patients don't all the time remain relating solely to their family doctor (who may or may not be reached for emergency assistance)
- Working with doctors to convince the medical authorities to provide emergency medicine and prophylaxis treatment to HAE patients
- Help in establishing the national HAE patient's registry
- Increasing awareness among doctors and authorities.

At the end of the meeting, patients and families were happy and grateful for this first meeting, and we understood that talking about their disease and sharing their stories were of great importance and a real relief for the patients and their families!

That credit from patients and their families gave us as a big push, and already on 1 February 2020, we gathered again for a General Assembly with about 80 peoples – patients, doctors and volunteers – for the official kick start of our local organization.



## CROATIA

HAE Croatia now has its website hosted with HAE International – please see <https://haecroatia.haei.org>.





## UNITED STATES OF AMERICA

*From Ianice Viel, Patient Advocate/Social Media Manager, US HAEA:*

**US HAEA Hosts Meet & Greet Events:** The US HAEA kicked-off 2020 with an expanded focus on the Meet & Greet program. This outreach initiative offers patients and caregivers the opportunity to interact directly with each other and the HAEA staff. The events are designed so that attendees can meet other people affected by HAE in their local areas, share experiences, learn about HAEA programs and services, receive guidance based on individual needs, and develop a sense of community. The events are well attended and create a positive atmosphere for participants, particularly for newly diagnosed patients who are learning to navigate life with HAE. There are ten events scheduled this year in the following major US cities: Atlanta, Charlotte, Cincinnati, San Juan, Boston, Denver, Phoenix, Seattle, Minneapolis, and New Orleans.



**US Clinical Trials Announced:** Throughout the years, the US HAEA has actively supported the research and development of new therapies for the benefit of HAE patients.

Currently, the search for improved treatments and services continues with multiple ongoing recruitments for clinical trials taking place. The future is bright when we all support future research through clinical trial participation.

There are over 30 active trial sites across the U.S. enrolling patients in the APeX-S study, a worldwide clinical research study to investigate the long-term safety of an investigational oral medication to prevent HAE attacks. Furthermore, a new study has been announced to evaluate the safety and efficacy of repeated subcutaneous administration of Lanadelumab in preventing angioedema attacks in adolescents and adults with non-histaminergic angioedema with normal C1-INH, and in adults with acquired angioedema (AAE) due to C1-INH deficiency. Trial sites are beginning to open and are ready to enroll patients across the country. Additionally, there is an ongoing, small scale study to evaluate the safety, pharmacokinetics, and pharmacodynamics of Lanadelumab to prevent HAE attacks in pediatric subjects ages two to twelve years of age.

If you are a U.S. patient interested in participating in any of these trials, please contact John Williamson at [john@haea.org](mailto:john@haea.org), to see if there is an active clinical trial site in your area.



**#BeyondHAE Podcast:** Each month, the US HAEA shares a story from a different youth host from the HAE community. Their stories are a true testament to the challenges and triumphs that we experience as a community.

Episode 6 is hosted by Ava, who is a caregiver to her little sister who has HAE. In this episode, Ava provides insight into how she has supported her sister during her HAE journey. While coping with a family member with a chronic health condition isn't easy, oftentimes there are important lessons that are learned along the way. It's important to remember that undiagnosed family members and caregivers often play a big role in supporting their diagnosed family member and that HAE truly affects the whole family. Listen to this and other HAEA Youth Podcasts at <https://anchor.fm/haeyouth>.

**Serving HAE Patients with Kindness and Compassion:**

The US HAEA Health team is dedicated to working closely with everyone in our community by offering personalized support to anyone with a chronic, hereditary swelling symptom who needs help in getting

a diagnosis and finding a treatment. While HAE is most commonly caused by a deficiency in the C1-Inhibitor protein, the HAEA Health team puts significant time, attention, and investment into helping people who suffer from chronic swelling but have normal levels of C1-Inhibitor. Every day, we aim to create long-lasting connections that unite us in the quest to help everyone with HAE attain wellness and happiness. Obtaining a correct diagnosis and having an optimal HAE treatment plan are the cornerstones of a better life. We have built a nationwide network of knowledgeable physicians who know how to diagnose and treat HAE. Our US HAEA Health team provides referrals to these physicians and can also arrange appointments with the highly skilled angioedema specialists at the US HAEA Angioedema Center at the University of California at San Diego.

Furthermore, many in our HAE family wish to connect with others to share life situations and challenges being faced when dealing with HAE. To meet this need, we also offer a virtual support group that provides a warm and caring environment conducive to supportive, compassionate discussions, and problem-solving.

We will not rest until all medical professionals have at least a basic understanding of HAE. We have therefore dedicated significant time and resources to our Physician Outreach program, which provides high-quality Continuing Medical Education courses to health care professionals through our special portal at [www.haeedu.com](http://www.haeedu.com):

- HAE Body Interact
- Consensus Guidelines on Improving the Management of HAE
- Angioedema in the Emergency Medicine Setting: Optimizing Management for Every Patient





## SPAIN

The 22nd General Assembly and Annual Meeting of HAE Spain (AEDAF) was set to take place on 18 April 2020 in La Paz Hospital Madrid. However, due to the COVID-19 situation the event is postponed. Please see <https://angioedema-aedaf.haei.org> for further information.



## CUBA

The HAE International map has been updated with an HAE Care Center as well as an HAE knowledgeable physician in Cuba – please see <https://haei.org/hae-member-countries/cuba>.



## KUWAIT

If you visit <https://haekuwait.haei.org> you can see the new website of HAE Kuwait, yet another hosting with HAE International.



## LATVIA

Latvia has been added to the HAE International map as country number 85. The national contact in Latvia is Natalja Kurjane, an associate professor at Pauls Stradins University Hospital in Riga. She is an immunologist looking after HAE patients in Latvia so there is now also the first registered HAE knowledgeable physician in the country.



## AUSTRALIA AND NEW ZEALAND

*From HAE Australasia CEO Fiona Wardman*

HAE Australasia held its strategy meeting during January 2020. This meeting brings together the HAE Australasia Board of Directors to discuss the projects and initiatives for the coming year. The Board is excited about the new projects the organization will be working on to bring more HAE education and awareness, and resources to patients in Australia and New Zealand. Stay tuned for more details :)



HAE Australasia, with the help of Jess Bogoyevitch, launched the Youth Facebook page in February. This useful resource gives 13 to 21-year-old HAE patients and their siblings an opportunity to have a private and closed Facebook group to discuss, share stories, ask questions, and make friends in a safe place with

their peers. Jess will be facilitating discussions and encouraging participation. The group is monitored to ensure everyone is kind and respectful. HAE Australasia encourages parents in Australia and New Zealand to have their children join the group.

The HAE Australasian brochures and resources such as “Someone in my family has been diagnosed with HAE” and “About Hereditary Angioedema for Patients, Carers, Families, Physicians” are now available online via <https://haeaustralasia.org.au/resources/>. If you are in Australia or New Zealand and you would like a hard copy simply send us a message.

HAE patients in Australia and New Zealand have an essential role in helping our organization make changes and improving our current situation. We are now working on changing the criteria for preventative treatments and gaining access to new therapies. HAE Australasia is encouraging all HAE patients who have family members, including children with HAE, to join our membership via <https://haeaustralasia.org.au/stay-in-touch/become-a-member>.





## FINLAND

HAE Finland had a patient meeting in Tampere in February 2020. Among the participants were Chief Physician Jaakko Antonen from Tays Central Hospital. In the photo you see the members of the board of HAE Finland.



## SOUTH AFRICA

*From President Adrienne de Jongh, HAE South Africa:*

HAE South Africa have successfully been registered as a non-profit company which will allow us to progress and collect some much-needed funds. We have four directors at present and plan to add a fifth soon so that we can fulfill all the roles we have set out to accomplish.

We have had a meeting with one of the drug companies so far and plan to follow up with the other relevant players in the near future. We have also established firm links with the Rare Disease Foundation in South Africa, and one of our members is an ambassador for HAE within their organization and has done a fair amount of PR for HAE.

For Rare Disease Day 2020, we made a few video clips used on social media. We also made a video which was posted by backabuddy, a South African crowdfunding company. This is an example of some of the media coverage we got, in this case in the Benoni Times: <https://benonicitytimes.co.za/369583/morehill-resident-proudly-a-rare-disease-ambassador>.

The annual patient and carer meeting was scheduled for 18 April 2020 in Cape Town. However, due to the COVID-19 situation the meeting has been postponed. Please check <https://southafrica.haei.org> for further information.

HAE South Africa has grown the patient list to 97 members.



## CANADA

*From President Jacque Badiou and Volunteer Coordinator Daphne Dumbrille, HAE Canada:*

It has been a few months since our last article in Global Perspectives, but not due to a lack of exciting news to report – it's hard to know where to begin.

We would like to start with an important announcement: We are pleased and proud to report that the new treatment, Takhzyro (lanadelumab), received a positive recommendation from the Canadian Agency for Drugs and Technology and Health (CADTH). This recommendation was supported by the patient submission HAE Canada provided, which illustrated the importance of this treatment to Canadians with HAE. A special thank you to our engaged Advocacy Committee and Robert Bick, Health Policy Consultant. This positive recommendation means Takhzyro is one step closer to becoming available to HAE patients across Canada. HAE Canada continues to work hard advocating for the provinces and territories to add Takhzyro to their drug plans. You can find a copy of the "Patient Group Input Submission" on CADTH's website at [www.cadth.ca/lanadelumab](http://www.cadth.ca/lanadelumab).

Fall 2019 was busy for the Regional Directors who organized and held Patient Update Events in their areas. Members from across the country attended events in Halifax, Ottawa, Calgary, and Vancouver to not only connect with other HAE patients but to also learn about a variety of HAE related issues. Each Regional Director arranged for professionals to speak on these issues, such as accessing upcoming HAE treatments in Canada. The Patient Events are always a positive and energizing experience for everyone – the HAE Canada Board enjoys meeting new members and reconnecting with those we have met along the way. Thank you to all these Regional Directors and especially Lorraine Coumont, Pacific Regional Director, for organizing two of these events and the upcoming event in Winnipeg in April.

Tina McGrath, HAE Canada's Ontario Regional Director and VP, and Daphne Dumbrille from the HAE Canada office attended the 2019 Canadian Society of Allergy and Clinical Immunology (CSACI) Annual Scientific Meeting in Montreal, in October 2019. A poster titled "Real-World Data of Canadians Living with HAE: Attributes of New Medications" was presented by Dr. William Yang on behalf of HAE Canada. The poster



was well received and generated many discussions surrounding the importance of providing Canadians with a variety of effective, current and convenient treatments.



HAE Canada understands the importance of partnering with different rare diseases in Canada. To help remain up to date, HAE Canada Treasurer Richard Badiou, President and Quebec Regional Director Jacque Badiou, a volunteer Tina, and contracted workers Heather Dow and Daphne were happy to attend the Canadian Organization for Rare Disorders (CORD) Access to Innovation Opportunities for Cell & Gene Therapies Conference in Toronto in November 2019. Presenters discussed many topics, from cellular and gene therapies to personalized healthcare in Canada. It was an incredibly informative and interesting conference, and we appreciated the opportunity to attend.



In December 2019, Daphne presented on behalf of the HAE Canada Board of Directors at the Canadian Blood Services (CBS) Open Board Meeting. She respectfully requested that CBS work toward fast-tracking products that have been approved by Health Canada, specifically HAEGARDA and Takhzyro, and asked CBS to consider adding them both to the CBS formulary, which would further improve the care of HAE patients. Soon after this presentation, Jacquie and Richard, with a fellow Board member, volunteers, clinicians, health policy experts, government officials and CADTH representatives travelled to Toronto to attend CBS's HAE Forum. The Forum aimed to engage all stakeholders in an open and honest discussion to analyze various key issues surrounding HAE treatment options, namely C1 esterase inhibitor (C1-INH) products. Over the two days, many topics were discussed, such as patient's perspectives and access, as well as trends in utilization. All discussions aimed to provide an understanding of the increase in demand for C1-INH products. HAE Canada would like to thank CBS for hosting this important and

valuable Forum that will hopefully see HAEGARDA, a C1-INH product, added to the CBS formulary; giving equal access to all Canadian HAE patients.

In January, Daphne, along with an HAE Canada volunteer, attended the Patented Medicine Prices Review Board (PMPRB) Forum organized by CORD. This is an independent Canadian agency mandated to keep the costs of patented drugs low. The goal of the Forum was to educate attendees of the impacts the PMPRB's proposed changes to drug pricing regulations will have on treatments for rare diseases. HAE Canada appreciated the opportunity to attend and learn about this complex issue and supports CORD in trying to ensure the proposed changes will not prevent Canadian patients from accessing treatments.

The start of March brought Board members and workers to Ottawa to attend CORD's conference "Bringing Canada's Rare Disease Strategy to Life".



## HUNGARY

For a number of years HAE Hungary has had a website. However, it has now become a website hosted by HAE International. At the same time, it has been revitalized quite a bit – please have a look at <https://hano.hu>.



## SERBIA

After four years as President of HAE Serbia Ivana Golubović has stepped down. New President is Jovana Cvetkovic.





## UNITED KINGDOM

*From Laura Szutowicz, Chief Executive HAE UK:*

**I am Number 17 Campaign:** HAE UK was one of 13 Rare Disease patient groups invited to propose a representative to be featured in the 'I am Number 17' campaign about Rare Diseases which is an initiative of Genetic Alliance and backed by Takeda. The basis of the title of the campaign is that one in seventeen people suffers from a rare disease in their lifetime, so they are actually not that rare!



The representatives designated as 'Changemakers' were paired with an artist who created artwork to symbolize the experience of living with a rare condition. Amongst other conditions featured were Haemophilia, Ehlers Danloss Syndrome, Tubercular Sclerosis, Sickle Cell and Ataxia. The campaign, the patient stories and the artworks can all be seen at <https://iamnumber17.geneticalliance.org.uk>.

Our Changemaker was Karen Owen. She was paired with artist Jazmin Parsons who created a portrait of Karen which was part of the 'I am Number 17' art exhibition which was at the Oxo Gallery in London.

Karen used the phrase 'Live with not suffer from' to sum up her experience of living with HAE and feels that Jazmin's portrait sums up the many faces of her life with HAE.

**Karen's Story:** I have lived with HAE all my life and was symptomatic with external swellings from about two years old. Though I was a "difficult" baby, so may have been having internal attacks from birth. Like many HAE patients and people living with other rare conditions,

I spent my childhood in and out of GP surgeries and hospital clinics. Even though I was born with the condition, mine was due to a genetic mutation, not heredity, which is even rarer, so there were no family clues to help with diagnosis.

My earliest memory is being about age four and seeing a child in an allergy clinic look at me delightedly, saying very loudly to my Mum's absolute horror "Look there's someone else with a face like me!"

I was seen by many doctors and consultants, who all thought I had allergies. I remember coming home from one appointment and seeing my dad's face when he was told by my Mum that he had to dig up all the primulas he had lovingly planted, "just in case".

The hardest time for me was in my early teens. My parents had a troubled marriage, probably not helped by the stress of a child who was constantly ill, and they separated shortly after I started senior school and whilst I was dealing with entering puberty. So, my stomach aches and swellings increased in effect and frequency thanks to the combined effect of stress and hormones, which I now realize were all known triggers. All the symptoms still weren't tied together, and at least one clinician implied I was 'an attention-seeking neurotic female'!

I was finally diagnosed with Hereditary Angio-Neurotic Oedema when I was about 14 years old. Thankfully it's no longer called that, as it created a certain amount of stigma, and I was often made to feel I was overreacting to period pains or being a hypochondriac. When I was diagnosed, I didn't know anyone else with HAE, which was rather challenging. That's why I'm so passionate about peer support! I didn't meet another person living with HAE until I was 50 when I attended my first HAE UK patient conference. My ability to manage my health confidently changed exponentially with that support. I wonder how different my life would have been if I, and my family, had that support from birth? As a result, I changed my career and now work in the NHS as a HealthMakers Peer Support & Volunteer Coordinator, which is a program I started in East Berkshire back in 2014. HealthMakers are there to help anyone living with health issues to take more control of their condition & improve their quality of life.

My self-management regime is based on being mindful of the three possible scenarios we face every day – well days, hiccups/change days and emergency mode. I follow a healthy lifestyle; try to ensure I get enough sleep that I eat well and have a good work/life balance. This does mean I have to make sacrifices from time to time, and it's important to pace myself by building downtime into my routines. I try not to let my condition stop me from being sociable with friends and family. However, I do see myself as lucky.

I was fortunate to grow up with a supportive family and friends. I have been able to work whilst living with HAE and hold down a job my whole life. I have many wonderful people in my life who I would never have met if I didn't have HAE. I do, however, have to be realistic with what I can and can't do. I have lost count of the number of events and holidays I've had to forgo or cancel at the last minute, and the £'s I've lost in deposits and tickets not used... but I still try to live my life to the full.

I also like to share what I can on social media and within the national and local health arena, raising general awareness of the number of people who contend with rare conditions on a daily basis. In addition to this, I also like to help the patients and their families and carers to have more understanding of the importance of gaining self-management skills and building appropriate peer support networks.



It's vital to have hope when living with rare diseases such as HAE. Research is still being conducted, and new treatments are still being developed. Things have changed a great deal in my lifetime but knowing that people are devoting their lives to improving the lives of those living with the HAE and other rare conditions gives hope of improved quality of life & maybe a cure. I hope that the advocacy and volunteer work I carry out continues to make a difference.

I believe patient involvement in research and health and care service delivery is vital and key to improving the care we receive. That's why I agreed to become a Changemaker, and I hope by being a part of the #IAmNumber17 campaign it can continue to raise awareness for HAE and all the rare conditions and ensure those who live with these conditions receive the peer support and self-management skills they need to live life to the full.





**HAE and sports:** Another member who does not let HAE get in the way of her life is our executive officer Rachel Annals. Many of you will know Rachel through her being the UK representative on the HAE International Board of Directors, and she is absolutely invaluable in the help she gives me in running HAE UK. Not content with having a full-time career in local Government and being the mother to Darcy, she has set herself challenges to complete this year. Her first was to take part in the New Year's Day sea swim from Woolacombe, and in February she had a go at archery. Plans for the next months are flying a plane, dry slope skiing and running in a 5k or 10k. We will report on these in future magazines!

**Patient Days 2020:** Already being planned are our Patient Days. 2020 sees the 10th anniversary of the founding of HAE UK, started by John Rixon, Ann and John Price. Aided by HAE International, they set up the initial patient support and advocacy group, which grew and grew, becoming a registered charity in June 2013. We are planning to celebrate this birthday with an extra-special Patient Day on 21 November so make sure this date is in your diaries!

**Fundraising for HAE:** We are very fortunate in having people who wish to raise money to help HAE UK carry out our various projects, and we have some particularly interesting fundraising efforts coming up soon:

- We have a runner for the 2020 London Marathon! Terry Mansfield, who is the manager of The Railway Hotel at West Hornden, has kindly suggested he donates all the sponsorship he receives to HAE UK. The Railway Hotel was where we ran the Family Fun Day last year and Terry and his team, aided and abetted by Trustee Ann Harding, made it a wonderful day for HAE UK. Now he is lacing up his running shoes and tackling the 26.2 miles of the big event. We hope that many HAE UK members will be able to support him and cheer him over the finish line. Sponsorship money can be donated through our Just Giving page at [www.justgiving.com](http://www.justgiving.com). Search HAEUK, and you will find Terry's page or click [www.justgiving.com/Terry-The-Railway-Hotel](http://www.justgiving.com/Terry-The-Railway-Hotel).
- We are so lucky to have people like Terry raising money for us, but we are also especially fortunate to have people who want to remember HAE UK at very sad times in their lives. Our thanks and condolences to Trisha North who set up a page in memory of her mother Grace Fleischer. It was wonderful of Trisha to support HAE UK in this way at such a sad time for her and her family. Also remembered is Christopher Mead, who sadly passed away at the end of 2019. His family and friends kindly made donations to HAE UK. We are enormously grateful to them.

**Contact to immunology centers:** In the UK we are very fortunate to have more than 30 immunology centers treating patients with HAE. Each center is registered and approved and will have at least one clinician who is expert in treating HAE. Several of the centers are specifically for children too. So if you are traveling to UK contact either [laura.szutowicz@haeuk.org](mailto:laura.szutowicz@haeuk.org) or [rachel.annals@haeuk.org](mailto:rachel.annals@haeuk.org) to find the center nearest to where you are traveling.

With best wishes to all our friends in the HAE community.





## HONG KONG

*From Sandy Chan, HAE HK national contact:*

HAE HK's main focus in 2020 is to raise public awareness about HAE in Hong Kong, and January 2020 has proven to be a big month for HAE in Hong Kong.



In January 2020, Radio Television Hong Kong (RTHK), a popular Hong Kong TV and radio public broadcaster, released two episodes about HAE for the health television series 《醫生與你》 ("Doctor and You") and 《精靈一點》 ("Healthpedia"). In the first episode, Miss Wong and Eliza shared their patient stories about their sufferings for many years as a result of frequent and severe swelling attacks. Dr. Philip Li from the Queen Mary Hospital elaborated on the science about HAE and treatments. In the second episode, Davy and myself from HAE HK shared that Hong Kong was treading far behind other countries in terms of diagnosing, treating and funding HAE. In addition to the lack of education in public about HAE, which made diagnosing HAE far more difficult and slower, modern medicine available elsewhere in the world was not available in Hong Kong. Even if patients gained access to modern treatments, the exorbitant price tag attached to these treatments made them highly impractical for the ordinary citizen.

In terms of education, representatives from HAE HK participated in the "Human Library Series" organized by the Hong Kong Alliance of Rare Disease (HKARD) where medical students from the University of Hong Kong, patients and doctors shared their experiences and understanding about HAE. This series gave students a good insight into HAE and the difficulties HAE patients faced in Hong Kong.

In addition, HAE HK presented to students of the University of Hong Kong about HAE as an example of a genetic disease at the "World Changed by DNA" seminar.



Other efforts in January 2020 included interview publications of doctors and patients on HKU Med, HKET and HKARD as well as a storytelling session to a class of Primary 1 Students about young patients living with HAE.

In February and March 2020, amid the coronavirus outbreak, HAE HK uses its best efforts to source and distribute surgical masks and sanitizing products to HAE HK members.

Stay tuned for more news to come from Hong Kong. In the meantime, good health and stay safe!



## SLOVAKIA

HAE Slovakia reports that still more patients are being located and that they need the support and help of the organization. Most communication takes place via e-mail and the national website assist the patients with basic information on HAE.



## LEBANON

HAE Lebanon is yet another national organization to have its website hosted with HAE International – please see <https://haelebanon.haei.org>.



## SLOVENIA

HAE Slovenia now has a website under the wings of HAE International. You will find it at <https://haeslovenia.haei.org>.



## PAKISTAN

Four HAE knowledgeable physicians - located in Faisalabad, Lahore (2) and Peshawar - have been added to the HAE International map. Please see contact information at <https://haei.org/hae-member-countries/pakistan>.



## LIBYA

HAE International has registered the second HAE knowledgeable physician in Libya. Please see <https://haei.org/hae-member-countries/libya/>.



## RUSSIA

*From Elena Bezbozhnaya, Chairman of HAE Russia (SPHA):*

Early 2020 HAE Russia held several events for the members and organized an educational exhibition at the museums of the two biggest cities of the Russian Federation.

In January we conducted a webinar “Effective Health Care: Diagnosis and Treatment of HAE” with members from Novokuznetsk, St. Petersburg, Ulyanovsk, Yekaterinburg, Voronezh, Murmansk and Krasnodar Krai taking part. The speakers at the online meeting were lawyer Ilya Ushankov and myself. The webinar began with a small talk about legal issues. The participants were given recommendations on how to use the right of getting preferential medicines coverage. We also discussed positions of International Classification of Diseases 11th revision (ICD-11) developed by the World Health Organization. ICD-11 has already been adopted in most European countries, and this year it is supposed to be put into effect in Russia. At the end of the meeting, the speakers answered the participants’ questions. Some of them were related to the problem of how patients with HAE can obtain disability status. Since 1 January 2020 Russia has a new procedure for obtaining disability status. The main criterion for getting such status for patients with HAE is the frequency of edema. Still, it is not clear how the patient’s condition will be monitored because the mechanism of fixation of seizures is not described anywhere.

At the beginning of February, a social photo exhibition “The HAE family” was held in The National Pushkin Museum in St. Petersburg. HAE Russia organized the event with the support of the Ministry of Culture of the Russian Federation. The exhibition – dedicated to the International Rare Disease Day on 29 February 2020 – consisted of photographs of children with HAE and their families. At the opening of the event the Director of The National Pushkin Museum Sergey Nekrasov spoke about social responsibility and society’s indifference: “The International Rare Disease Day, the idea of which has already been picked up by 85 countries, is necessary to draw public attention to those poorly known problems that people living among us have. Therefore, I consider it important and proper that such exhibitions now began to appear”.



On behalf of HAE Russia, I thanked the museum staff for their help in organizing the event and told about the current situation with rare diseases in Russia and abroad. A speech by Ekaterina Viktorova, allergist-immunologist at the Dmitry Rogachev National Medical Research Center of Pediatric Hematology, Oncology and Immunology in Moscow was dedicated to the features and problems of the diagnosis and treatment of HAE. In the final part of the ceremony, guests of the event were shown a cartoon about children with HAE, created on HAE Russia's initiative, and the information video "Hereditary angioedema. Life without Borders", from which visitors could learn how the disease proceeds and what problems people with such a diagnosis face.

After St. Petersburg the exhibition "The HAE family" moved to Moscow. At the end of February, the grand opening took place in the Armory Chamber of the Moscow Kremlin Museums. Dmitry Morozov, professor,

MD, Chairman of the State Duma Health Protection Committee, Olga Dmitrieva, Deputy General Director of the Moscow Kremlin Museum, and doctors from the primary federal clinics expressed their gratitude for the opening of the exhibition. All speakers mentioned the social significance of the event and expressed their readiness to expand cooperation with HAE Russia. In response, I thanked the Moscow Kremlin Museums' staff for their assistance and noted the importance of raising public awareness of the existence of rare diseases. Among the guests of the exhibition were patients with HAE, members of HAE Russia (including children) and representatives of the federal media.

As mentioned, the International Classification of Diseases 11th revision (ICD-11) developed by the World Health Organization will be adopted in Russia soon. In connection with these changes, HAE Russia is concerned about the status of patients diagnosed with HAE of the third type. It is an extremely rare form of the disease. There are only a little more than ten patients with such a disease registered in Russia. In anticipation of the new version of ICD, a discussion about the advisability of including patients with the third type of HAE in the federal register of patients with rare diseases began in the medical and expert community. That is because, unlike the patients of the first two types, they have no defect in the complement system. If codes in the new edition of ICD changed, the third type of HAE would be classified as an allergic disease. This, in turn, would lead to the fact that patients would be left without modern treatment that can relieve life-threatening edema. Representatives of HAE Russia have been actively participating in all stages of this discussion for several months and have sent a petition to the Russian Association of Allergologists and Clinical Immunologists (RAACI) to clarify the situation. In response to the petition, RAACI sent a letter explaining the position of the experts. It states that after an expert discussion, RAACI will recommend the Ministry of Health of the Russian Federation to combine patients with HAE of all three types with the assignment of a single code in the new ICD-11. This is very encouraging news. After assigning a single code to ICD, all patients with HAE would have the right to receive medical coverage from the state budget.

HAE Russia continues its actions to protect the rights of patients and provide them with legal, social and psychological support.



# STILL MORE COUNTRIES USING HAEi CONNECT





HAEi Connect – a cloud-based member database for national organizations to manage their members – is being rolled out to still more countries.

“First and foremost, HAEi Connect includes an easy to use editor for creating emails to members as well as a template for saving and re-using emails. Another addition to the system is the possibility of contacting members via text message. Other updated features are four new sections in the HAEi Connect dashboard to initiate contact to members as well as right-to-left language support”, says Enterprise Technology Manager Ole Frölich.

To date, HAEi Connect has been implemented in Australia, Brazil, Bulgaria, Denmark, Hong Kong, Hungary, India, Israel, Kenya, Mexico, New Zealand, North Macedonia, Norway, Peru, Poland, Slovenia, South Africa, Spain, Sweden, Turkey, and the United Kingdom.

“Furthermore, we are talking to six national organizations so it is my expectation that we will within the coming months be able to add countries like China, Croatia, Lithuania, Pakistan, Puerto Rico and the United States of America”, says Ole Frölich.



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

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**A novel functional C1 inhibitor activity assay in dried blood spot for diagnosis of HAE** – by Lai Y, Takeda Pharmaceuticals International Co., et al.:

Dried blood spot specimen collection for measurement of functional C1-INH activity in a physician's office is straightforward and not limited by logistic considerations. Therefore, it is appropriate for the diagnosis of HAE in high throughput diagnostic laboratories.

(Clin Chim Acta., 2020 May)

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**Parallel comparison of three methodologies for measuring functional C1-inhibitor in HAE patients** – by Kapoor A, Shire, et al.:

Functional binding immunoassay methods serve as reliable alternates for conventional chromogenic method to quantitate fC1-INH in human plasma samples with a better dynamic range of detection and ease of use. Of the two immunoassays used in the study, FXIIa-binding method gave better sensitivity, specificity, and correlation to the chromogenic method as a diagnostic method to distinguish HAE samples from healthy controls.

(Int Immunopharmacol., 2020 Mar)

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**HAE: a prospective study of a Brazilian single-center cohort** – by Alonso MLO, Federal University of Rio de Janeiro, Brazil, et al.:

The severity of HAE attacks, especially in females, highlights the need for an awareness of disease by gynecologists and obstetricians. Screening of familial members, including asymptomatic individuals, is critical for earlier diagnosis. Regional evaluation of patient profiles can be helpful to draw more attention about HAE and to improve quality of life.

(Int J Dermatol., 2020 Mar)

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**Angioedema without urticaria: novel findings which must be measured in clinical setting**

– by Veronez CL, University of California San Diego, the United States of America, and Grumach AS, University Center Health ABC, Sao Paulo, Brazil:

The validation of biomarkers for the contact system activation could be beneficial in differentiating bradykinin from histaminergic-mediated angioedema. Currently, the available laboratorial tests are still somewhat restricted to the evaluation of the complement activation and the mediators of nonhistaminergic and nonbradykinin-mediated angioedema remain to be identified.

(Curr Opin Allergy Clin Immunol., 2020 Feb)

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**Modeling Cost-Effectiveness of On-Demand Treatment for HAE Attacks** – by Bernstein JA, University of Cincinnati College of Medicine, the United States of America, et al.:

Accounting for patient well-being and additional cost components of HAE attacks generates a better estimation of cost-effectiveness than drug cost alone. Results indicate that rhC1-INH is the dominant treatment option with lower expected costs and higher calculated effectiveness than comparators. Further analyses reinforce the idea that low redose rates contribute to improved cost-effectiveness.

(J Manag Care Spec Pharm., 2020 Feb)



**Management of patients with HAE in dental, oral, and maxillofacial surgery: a review** – by Uzun T, Trabzon Oral and Dental Health Hospital, Turkey:

Dental procedures cause emotional stress and mechanical trauma and may also initiate HAE attacks. The most feared complication is asphyxiation as a result of laryngeal oedema. Cases that resulted in death after tooth extraction have been reported, so dentists and oral and maxillofacial surgeons should take maximum care in the treatment of patients with HAE, consult with the patient's doctor, and ensure that prophylaxis is given before the procedure. They should work as atraumatically as possible and use procedures to minimize stress. In the event of an HAE attack the procedure should be terminated immediately and treatment of the attack started as soon as possible. The first drugs for the treatment of acute attacks are C1-INH, ecallantide, or icatibant.

(Br J Oral Maxillofac Surg., 2019 Dec)

**Pediatric HAE: what the otolaryngologist should know** – by Bailey CE and Carr MM, West Virginia University School of Medicine, Morgantown, the United States of America:

Targeted biologic agents have gained acceptance in treatment of acute attacks, and their use as prophylactic agents is changing the focus of management from acute intervention to preventive management. While intubation or surgical airway management may still be necessary, early intervention or prophylaxis can decrease morbidity and improve quality of life.

(Curr Opin Otolaryngol Head Neck Surg., 2019 Dec)

**Biological therapy in HAE: transformation of a rare disease** – by Longhurst H, Addenbrooke's Hospital, Cambridge, the United Kingdom, and Farkas H, Semmelweis University, Budapest, Hungary:

Over the past 10 years, several evidence-based parenteral treatments have been licensed, including two plasma-derived C1-INH replacement therapies and one recombinant C1-INH replacement for acute treatment of angioedema attacks and synthetic peptides for inhibition of kallikrein or bradykinin B2 receptors, with oral small molecule treatments currently in clinical trial. Moreover, recent advances in prophylaxis by subcutaneous C1-INH to restore near-normal plasma function or by humanized antibody inhibition of kallikrein have resulted in freedom from symptoms for a high proportion of those treated. This plethora of treatment possibilities has come about as a result of recent scientific advances. Collaboration between patient groups, basic and clinical scientists, physicians, nurses, and the pharmaceutical industry has underpinned this translation of basic science into treatments and protocols. These in their turn have brought huge improvements in prognosis, quality of life and economic productivity to patients, their families, and the societies in which they live.

(Expert Opin Biol Ther., 2020 Jan)



# CLINICAL TRIALS

According to the International Clinical Trials Registry Platform under World Health Organization (WHO) and clinicaltrials.gov under the U.S. National Institutes of Health the following trials should be recruiting at this moment:

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## **A clinical study to assess the safety of Lanadelumab to prevent episodes of severe swelling in children**

– will be recruiting in Canada, Germany, Hungary, Spain, and the United States of America

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## **A clinical trial to assess 2 different doses of BCX7353 compared to placebo as an oral treatment for the prevention of attacks in people with HAE**

– will be recruiting Austria, Belgium, Bulgaria, Canada, Czech Republic, Denmark, France, Germany, Hungary, Ireland, the Netherlands, North Macedonia, Spain, the United Kingdom, and the United States of America

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## **A Long Term Safety Study of BCX7353 in HAE**

– recruiting in Australia, Austria, Denmark, France, Germany, Hong Kong, Hungary, Israel, Italy, Korea, New Zealand, North Macedonia, Poland, Serbia, Slovakia, South Africa, Spain, Switzerland, the United Kingdom, and the United States of America

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## **A Phase II, Cross-over Clinical Trial Evaluating the Efficacy and Safety of KVD900 in the On-demand Treatment of Angioedema Attacks in Adult Subjects with HAE Type I or II**

– recruiting in Austria, Czech Republic, Germany, Hungary, Italy, the Netherlands, North Macedonia, Poland, the United Kingdom, and the United States of America

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## **A Phase 3, randomized, double-blind, placebo-controlled, parallel-group study to evaluate the efficacy and safety of two dose levels of BCX7353 as an oral treatment for the prevention of attacks in subjects with HAE**

– recruiting in Japan

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## **A Safety, Pharmacokinetics (PK), and Pharmacodynamics (PD) Study of Lanadelumab to Prevent HAE Attacks in Pediatric Participants of 2 Years to Less Than (<) 12 Years of Age**

– recruiting in Canada, Germany, Hungary, Spain, and the United States of America

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## **A study to a) evaluate the tolerability and blood levels of KVD900 when given as a single dose to patients and b) to assess whether KVD900 is effective in treating attacks of swelling in patients with the genetic disease HAE**

– will be recruiting Austria, Germany, Hungary, the Netherlands, North Macedonia, and the United Kingdom

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## **A Study to Assess the Clinical Efficacy of IONIS-PKK-LRx in Participants with HAE**

– recruiting in the United States of America

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## **A Study to Investigate CSL312 in Subjects with HAE**

– recruiting in Australia, Canada, Germany, Israel, and the United States of America

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## **Biomarker for HAE Disease Type 1 (BioHAE)**

– recruiting in Egypt, Georgia, Germany, and India

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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



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**Determination of Specific Biomarkers of Acute Attack of Angioedema Within Pediatric Population**

– recruiting in France

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**Efficacy and Safety of Lanadelumab for Prevention Against Acute Attacks of Non-histaminergic Angioedema with Normal C1-Inhibitor (C1-INH) and Acquired Angioedema (AAE) Due to C1-INH Deficiency**

– recruiting in the United States of America

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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 Hereditary Angioedema Disease**

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

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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, Israel, Italy, Spain, Sweden, and the 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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**HAE Kininogen Assay**

– recruiting in Germany

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

– recruiting in France

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**Lanadelumab tested in patients suffering from HAE with normal C1-Inhibitor**

– will be recruiting in Germany

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

– recruiting in the United States of America

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**Single-arm pharmacokinetic phase 2a study of a single dose intravenous human plasma-derived C1-INH involving 20 HAE type I or type II patients, aged 18 years or older**

– will be recruiting in Belarus, Bulgaria, Czech Republic, Germany, Hungary, Poland, Russian Federation, Serbia, and Ukraine

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

– recruiting in Canada, Puerto Rico, and the United States of America

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

– recruiting in Austria, Germany, and Switzerland

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

– recruiting in France

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Read more about these and other clinical trials at <https://clinicaltrials.gov> and <http://apps.who.int/trialsearch>.

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# NEWS FROM THE INDUSTRY

## 13 January 2020

“Based upon the progress of the ongoing KVD900 Phase 2 clinical trial, we expect to announce data from that trial in the second quarter of this year,” says Andrew Crockett, CEO of **KalVista Pharmaceuticals, Inc.**

“We are also pleased to announce the selection of KVD824 for development as an oral prophylactic treatment for HAE. Based on preclinical formulation work conducted, we see evidence that KVD824 can achieve the properties we believe necessary for high efficacy as a twice-daily treatment for prevention of HAE attacks. After completing additional clinical work to optimize the exposure profile, we plan to commence a Phase 2 clinical trial in the second half of this year. KVD824 could be an excellent companion to KVD900's profile as an on-demand therapy to together serve all of the needs of HAE patients.”

In developing the strategy for its oral HAE franchise, KalVista has conducted extensive patient, physician and payer research to identify the key needs in the market. Oral therapy remains the highest unmet need according to all stakeholders, with 93% of patients surveyed by KalVista expressing a willingness to switch to oral therapy for both on-demand and prophylactic usage. Importantly however, the survey data shows that patients are not prepared to accept significantly reduced efficacy with a switch to oral therapy. The survey also indicated that twice-daily dosing would have little impact on willingness to switch compared to once-daily.

KalVista anticipates that KVD824 can meet the efficacy and safety needs of patients as an oral prophylactic treatment. KVD824 is a highly potent and selective plasma kallikrein inhibitor which achieved high exposures and a favorable safety and tolerability profile in a first-in-human study. KalVista intends to investigate twice-daily dosing in the planned Phase 2 trial to maximize efficacy while maintaining the convenience of an oral therapy.

(Source: KalVista)



## 13 January 2020

As first announced in July 2019, **Regenxbio Inc.** is developing a one-time gene therapy candidate to deliver a gene encoding a therapeutic antibody against plasma kallikrein, a key protein of the plasma contact pathway which is left unregulated in patients with HAE.

Preclinical animal studies conducted using NAV AAV8 indicate the potential for a sustained and safe delivery of biologically active antibody at therapeutic concentrations. REGENXBIO expects to select a lead product candidate in the first half of 2020 and provide a program update in the second half of 2020.

REGENXBIO's NAV Technology Platform, a proprietary adeno-associated virus (AAV) gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10.

(Source: Regenxbio)



## 21 January 2020

**Pharming Group N.V.** has received European Medicines Agency (EMA) approval of a Type II Variation for a new production facility for the Ruconest.

With the addition of this new facility, Pharming will significantly increase the production capacity of Ruconest as it becomes fully operational over the coming year. Pharming is now also able to release the product that was manufactured at the facility during the approval process for commercialisation in the EU. As previously announced, Pharming had identified a potential risk of short-term pressure on the supply of Ruconest for the European market due to increasing demand for the product. With the approval of this new facility, the Company believes the risk to supply is now greatly reduced.

The new facility's post-approval supplement (PAS) for the distribution of Ruconest in the US is still under review by the Food and Drug Administration (FDA).

Approval of the new facility for distribution in the US is expected in H1 2020.

Sijmen de Vries, CEO of Pharming, says:

“As we continue to see increasing demand for Ruconest in the treatment of HAE, we are pleased to announce the approval of our new facility, which will enable us to significantly increase production capacity for supply to patients in the EU. In addition, as a result of our recent re-acquisition of Ruconest’s European distribution rights from Sobi, this capacity expansion will allow us to reach an even greater number of EU patients.”

(Source: Pharming)



## 6 February 2020

HAEGARDA (C1 Esterase Inhibitor Subcutaneous [Human]) is now available in the province of Québec, Canada.

“The availability of a new treatment option to help health care professionals and patients prevent HAE attacks is an important milestone and great news for the HAE patient community,” said Charles St-Pierre, President of the AOHQ patient group.

**CSL Behring** Canada is also committed to help patients get access to training for the administration of HAEGARDA via CSL Behring PLUS+ program that offers a wide array of services, and educational materials. Patients can ask their Healthcare professionals to enroll them in the program at any time.

(Source: CSL Behring)



## 10 February 2020

**Intellia Therapeutics, Inc.** is presenting preclinical results for its CRISPR/Cas9-Based Therapy for HAE.

“The data from our HAE development program reinforce the modularity of Intellia’s non-viral delivery genome editing platform and how it is enabling independent, single-dose therapies for multiple monogenic diseases.

For HAE, we expect to nominate a development candidate in the first half of this year,” says Intellia President and CEO John Leonard, M.D.

Intellia’s HAE treatment hypothesis involves knocking out the kallikrein B1 (KLKB1) gene to reduce kallikrein activity, which is involved in the biological pathway for release of bradykinin. Intellia expects this reduction to correlate with a decrease in bradykinin activity, thus, preventing the activation of endothelial cells that causes vascular leakage and angioedema in HAE patients. The data shows that the knockout of KLKB1 produces in non-human primates (NHPs) a 90% reduction in kallikrein activity, a level that translates to a therapeutically meaningful impact on HAE attack rates. This kallikrein activity reduction was sustained for at least five months in an ongoing NHP study, in a highly reproducible manner observed across both rodent and NHP studies.

Intellia’s potential HAE therapy utilizes the company’s modular non-viral lipid nanoparticle (LNP) system to deliver CRISPR/Cas9. Intellia’s proprietary LNP-based delivery system includes two basic components: Cas9 messenger RNA (mRNA) and a guide RNA (gRNA). The gRNA is the only variable portion of the LNP delivery system and is the sole component that needs to be changed from the LNP-based delivery system that forms the foundation of NTLA-2001, Intellia’s development candidate for the treatment of ATTR for which the company intends to submit an IND application in mid-2020.

Intellia continues to evaluate several potential guide RNAs and expects to nominate a development candidate for HAE in the first half of 2020. Intellia’s KLKB1 HAE program is subject to an option by Regeneron to enter into a Co/Co agreement, in which Intellia would remain the lead party.

(Source: Intellia)



## 29 February 2020

**BioMarin Pharmaceutical Inc.**, a global biotechnology company that develops and commercializes therapies for people with serious and life-threatening rare diseases and medical conditions, has announced its financial results for 2019.

From the Key Program Highlights:

On 14 November 2019, BioMarin introduced its third gene therapy candidate, BMN 331, for the treatment of HAE. The company plans to build on its ever wider and deeper experience in developing gene therapies for severe hemophilia A and phenylketonuria to improve efficiencies in the development process and optimize capsid and transgene design. BioMarin expects to begin IND-enabling studies in early to mid-2020.

(Source: BioMarin)



## 5 March 2020

Announcing the financial results for the full year 2019, **BioCryst Pharmaceuticals, Inc.** President and CEO Jon Stonehouse says that “2020 is off to a strong start, with NDAs accepted and approvals lined up later this year in the U.S. and Japan”:

“We are hearing increasing excitement from both HAE patients and physicians about the availability of an oral option to manage their disease, and we are attracting outstanding commercial talent to bring this new medicine to patients,” Stonehouse added.

Upcoming Key Milestones for the HAE Program – Berotralstat (BCX7353):

- Submit Marketing Authorization Application (MAA) for oral, once-daily berotralstat for the prevention of HAE attacks with the European Medicines Administration (EMA) (Q1 2020)
- Approval and launch of oral, once-daily berotralstat in Japan (2H 2020)
- Approval and launch of oral, once-daily berotralstat in U.S. (December 3, 2020 PDUFA date)

Recent Corporate Developments for the HAE Program – Berotralstat (BCX7353):

- On February 27, 2020, the company announced it will present abstracts with new data on oral, once-daily berotralstat (BCX7353) at the upcoming annual meeting of the American Academy of Allergy, Asthma & Immunology (AAAAI) March 13-16 in Philadelphia.
- On February 18, 2020, the company announced that

the U.S. Food and Drug Administration (FDA) had accepted and filed its new drug application (NDA) for the approval of oral, once-daily berotralstat (BCX7353) for the prevention of HAE attacks. The Prescription Drug User Fee Act (PDUFA) date for the NDA is December 3, 2020. In the NDA filing acceptance letter, the FDA stated that they are not currently planning to hold an advisory committee meeting to discuss the NDA.

- On February 3, 2020, the company announced that it had submitted a new drug application (INDA) to the Japanese Pharmaceuticals and Medical Devices Agency (PMDA) for approval of oral, once-daily berotralstat for the prophylactic treatment of HAE.
- On January 12, 2020, the company announced that the APeX-J trial in Japan met its primary endpoint ( $p=0.003$ ) for prevention of HAE attacks, and berotralstat was safe and generally well-tolerated.
- On December 11, 2019, the company announced it had submitted a new drug application to the FDA for approval of oral, once-daily berotralstat (BCX7353) for the prevention of HAE attacks.

(Source: BioCryst)



## 10 March 2020

At the release of the financial results for the fiscal third quarter ending 31 January 2020 **KalVista Pharmaceuticals, Inc.** CEO Andrew Crockett said the ‘work to bring multiple best-in-class oral therapies to patients with HAE is advancing well’:

“First, we look forward to delivering data in the second quarter of this year from our Phase 2 clinical trial with KVD900, our on-demand therapy for HAE attacks. We believe KVD900 has the potential to offer patients an attractive option for control of their disease through a fast-acting tablet that can be taken at the earliest stages of an incipient attack. Second, we continue to make progress in our optimization of KVD824 as a potential oral prophylactic treatment. We still plan to initiate a Phase 2 trial of KVD824 for prophylaxis of HAE attacks in the second half of the year.”

(Source: KalVista)



## 10 March 2020

The US Food and Drug Administration (FDA) has approved **Pharming Group N.V.**'s Prior Approval Supplement to add the new Netherlands production facility of starting material to the Biologics License Application (BLA) to support its lead product Ruconest.

With the addition of this new facility for US supplies as well, Pharming can continue to expand sales in all markets in the coming year. Pharming is now also able to release the product that is manufactured with starting material from the facility for commercialization in the USA. As previously announced, Pharming had already received approval for output from the new facility from the European Medicines Agency for commercial use in the European Union.

CEO Sijmen de Vries says:

"We are pleased to announce this approval by the FDA of our new facility, which will enable us to meet increasing demand for Ruconest in the treatment of HAE for patients in the USA as well as for those in the EU. Following on from the EMA approval announced earlier this year in January, this gives us sufficient capacity for current demands as we continue to build for the future."

(Source: Pharming)



## 16 March 2020

New data from the APeX-2 trial shows that 150 mg of oral, once-daily berotralstat (BCX7353) for prophylaxis of HAE attacks reduced patients' monthly use of standard of care (SoC) on-demand medicine by 53.6 percent compared to placebo, and reduced the number of HAE attacks requiring acute SoC treatment by 49.2 percent compared to placebo. Additional data show that the percentage of HAE attacks requiring re-treatment with multiple doses of on-demand therapy was lower for patients receiving berotralstat (150 mg) than placebo.

"Patients receiving berotralstat had fewer attacks, treated fewer attacks, experienced less severe attacks

and used less on-demand medication compared to placebo. These data from APeX-2 provide further evidence that HAE patients are seeing significant benefits from oral, once-daily berotralstat," says Dr. William Sheridan, chief medical officer of BioCryst Pharmaceuticals, Inc.

In APeX-2, patients experienced a rapid and sustained decrease in their attack frequency over 48 weeks. Thirty patients who were randomized to 150 mg of berotralstat at the beginning of the trial and completed 48 weeks of therapy had a baseline mean attack rate of 2.9 attacks per month, which declined to 1.4 attacks per month after one month and to 1.0 attacks per month at month 12. APeX-2 patients who switched from placebo to 150 mg of berotralstat after week 24 saw dramatic and sustained reductions in their HAE attack rate. Their mean attack rate dropped to 0.5 attacks per 28 days at month seven and to 0.4 attacks per 28 days at month 12.

An integrated 48-week analysis across both the APeX-2 and APeX-S trials showed berotralstat was safe and generally well tolerated in a total of 342 patients with a total of 232 patient-years of daily oral dosing. The most frequent adverse drug reactions were mild-to-moderate gastrointestinal events that were brief in duration and self-limited.

APeX-2 is a randomized, double-blind, placebo-controlled, three-arm trial testing two dose levels of orally administered once-daily berotralstat (110 mg and 150 mg) for prevention of angioedema attacks. The trial enrolled 121 patients with Type I and II HAE in the United States, Canada and Europe. Following completion of the 24-week analysis period, patients continued on study drug in an ongoing extension phase of APeX-2 through 48 weeks. Patients randomized to placebo for 24 weeks were re-randomized to receive one of the two doses of study drug in the extension phase of the trial. Patients who complete 48 weeks may continue in the trial on open-label berotralstat.

(Source: BioCryst)



## 25 March 2020

The Committee for Medicinal Products for Human Use (CHMP) of the European Medicine Agency (EMA) has recommended approval of Ruconest for the treatment of acute angioedema attacks in children with HAE. This marketing authorization would expand the age range of **Pharming Group N.V.**'s lead product, a recombinant analogue of the human C1 esterase inhibitor produced by recombinant DNA technology in the milk of transgenic rabbits.

The positive opinion is based on the data from a Phase II clinical trial in 20 children. This recommendation will allow children aged 2 years and older to be treated with Ruconest for acute angioedema attacks. The European Commission's (EC) approval decision is expected in June 2020. In the European Union, Ruconest has been approved for this indication in adults since 2010 and in adolescents since 2016.

In January 2020 Pharming received EMA approval for a new facility. Together with this extension of the indication, Pharming believes that supply of Ruconest to the market is now further strengthened. Ruconest will be available for use in children patients later in 2020 throughout Member States of the EU, as well the European Economic Area in which Pharming currently has a license in the adult and adolescent population. Sijmen de Vries, CEO of Pharming, says:

"As we continue to see increasing demand for Ruconest in the treatment of HAE, we are pleased to announce this step forward in this paediatric label expansion. This will enable us to treat attacks of patients of all ages living with HAE in the EU. In addition, as a result of our recent re-acquisition of Ruconest's European distribution rights from Sobi, this extension of the indication will allow us to reach an even greater number of EU patients."

The open-label, single arm, Phase II clinical trial was designed in agreement with the EMA as part of a Paediatric Investigation Plan (PIP) to assess the pharmacokinetic, safety and efficacy profiles of Ruconest at a dose of 50 U/kg in HAE patients aged 2-13 years in support of the indication for treatment of HAE attacks in children.

A total of 20 children with HAE were treated for 73 HAE attacks at a dose of 50 U/kg (up to a maximum of 4200 U). The study reported clinically meaningful relief of symptoms assessed using a visual analogue scale (VAS) completed by the patient (assisted by their parent). The median time to onset of relief was 60 minutes (95% confidence interval: 60-653), and the median time to minimal symptoms was 123 minutes (95% confidence interval: 120-126). Only 3/73 (4%) attacks were treated with a second dose of Ruconest.

Ruconest was generally safe and well-tolerated in the study. No patients withdrew from the study due to adverse events. There were no related serious adverse events, hypersensitivity reactions, or neutralizing antibodies detected.

(Source: Pharming)



## 30 March 2020

The European Medicines Agency (EMA) has validated the marketing authorization application (MAA) submission for approval of oral, once-daily berotralstat (BCX7353) for the prevention of HAE attacks. With this validation, the EMA has begun their formal review of the MAA under the centralized procedure for all member states of the European Union, Norway, Iceland, and Liechtenstein.

An opinion from the Committee for Medicinal Products for Human Use (CHMP) is expected within approximately 12 months.

"Berotralstat would represent the first targeted oral therapy approved for HAE prophylaxis in Europe and would deliver a major advance in therapy to HAE patients. HAE treatment in Europe tends to be consolidated and we have developed excellent relationships with HAE-treating physicians through our clinical trials. This is allowing us to build an efficient and experienced European commercial team to bring our innovative medicine to patients," says Jon Stonehouse, CEO of **BioCryst Pharmaceuticals, Inc.**

BioCryst expects three regulatory approvals for berotralstat in 2020 and early 2021. The U.S. Food and Drug Administration (FDA) is currently reviewing a new drug application for berotralstat and has set an action date of 3 December 2020 under the Prescription Drug User Fee Act (PDUFA). In Japan, the Pharmaceuticals and Medical Devices Agency (PMDA) is reviewing a new drug application (JNDA) for berotralstat under the Sakigake timeline, and the company expects Japanese approval in the second half of 2020.

(Source: BioCryst)



### 30 March 2020

Sijmen de Vries, CEO and Chairman of **Pharming Group N.V.** in the 2019 Annual Report:

"During 2019, we have seen an increase both in patients who are badly affected by HAE and those with less severe symptoms who have been discovering the efficacy as well as the reliable and consistent response rates of Ruconest to treat their HAE attacks.

With the developments in the prophylaxis market (in which Ruconest is not approved) including a far greater dependence on products and potential products which only block the kallikrein pathway, one of several pathways identified as being complicit in attacks of HAE, we see an increasing need for patients to have a C1 esterase inhibitor on hand to treat attacks which break through their prophylaxis medication. This has led to increased demand for Ruconest as a fast, effective resolution therapy for acute attacks of HAE. We see this need for an effective breakthrough resolution therapy continue to drive Ruconest sales, even as new products enter the market and despite the convenience of prophylaxis therapies."

(Source: Pharming)







## HAE INTERNATIONAL AROUND THE WORLD

Currently there are HAE member organizations in 85 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 care centers, hospitals, physicians, and available medication.

The information on **haei.org** is being updated as soon as HAE International receives fresh data from the national member organizations.

