

May 2013

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## Message from the President

Dear HAEi Friends,

May is a very exciting month because on the 16th day, the world-wide HAE community including patients, caregivers, and family members has its own special day! Indeed, **hae day :-)** has already become a tradition and in 2013 patients throughout the globe are sponsoring events to raise awareness regarding the need for better diagnosis and treatment. We will provide details about some of these exciting events in our next newsletter.

It has been a busy first part of the year as HAEi set up a special **hae day :-)** website and coordinated activities with HAEi members throughout the world. During that period we also sponsored the first-ever patient meetings in Japan and met with success in efforts to form or further develop patient groups in Australia and New Zealand, the Gulf Region, Macedonia, and the Philippines. HAEi also has a full slate of important activities planned for the remainder of the year which will include (1) our day to day work with HAEi member organizations and



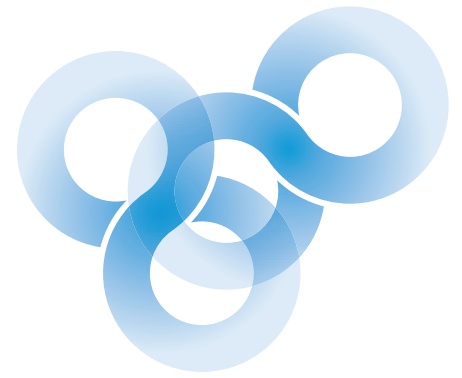
efforts to form new HAE groups, (2) Patient Advocacy Forums (which will result in publications on the state of HAE diagnosis and treatment) to be conducted in Europe and Latin America, and (3) planning for our 2014 Global Conference.

### Happy hae day :-)

The goal of **hae day :-)** is to (1) raise awareness of HAE among the general public as well as the medical community, (2) create an environment for earlier and more accurate diagnosis, and (3) spread knowledge that HAE patients can use to advocate for better care that will lead to a healthier life.

Since the beginning of the year, HAEi has been busily promoting hae day :-), through [www.facebook.com/haeday](http://www.facebook.com/haeday), [www.twitter.com/haeday](http://www.twitter.com/haeday), and various media sources.

The awareness of HAE has been growing during the first months of the year and we now we have close to 14,000 “likes” on our **hae day :-)** Facebook page. The number of smiles send via [www.haeday.org](http://www.haeday.org) has been growing. Our HAEi friends in Argentina, Belarus, Brazil, and Canada have made a significant contribution to the **hae day :-)** campaign by covering their respective countries



I wish everyone in the global HAE community a happy, healthy, and active **hae day :-)**.

Warmest regards to all,

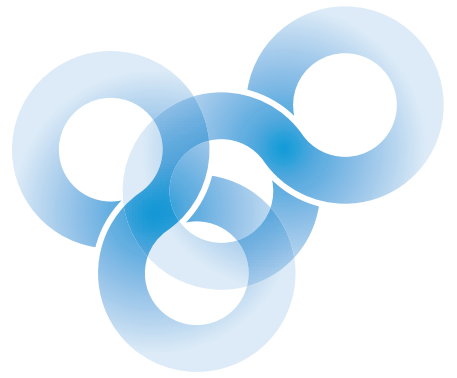
**Tony**

Anthony J. Castaldo  
President, HAEi

with smiles! North and South America as well as Europe are still the big contributors; however, we now see smiles come in from pretty much all over the world.

We would like to see many more smiles from all over the globe even after this year’s **hae day :-)** has passed. Therefore, we hope that each and every HAEi friend will launch initiatives in order to further increase the number of smiles from your country. Send a smile yourself if you haven’t already done so – and/or send an e-mail to your HAE contacts, asking them to post their smiles at [www.haeday.org/smile](http://www.haeday.org/smile). It is really easy to do – and it shows the world that HAE patients are united in improving diagnosis and treatment.

Please visit [www.haeday.org](http://www.haeday.org) to view hae day :-) events for 2013, and don’t forget to upload your own events on the website.



We are happy to announce that Swedish Orphan Biovitrum (Sobi) supports this year's **hae day :-)** as

Basic Sponsor, bringing us to a total of seven 2013 sponsors.

### From HAE Australia to HAE Australasia

HAE Australia has changed its name to HAE Australasia Ltd. At the same time the organization has appointed a new Director, Mrs. Olivia Willard from New Zealand.

The organization held its second Patient Meeting in Brisbane on 4th May 2013. The event provided participants with an opportunity to hear from HAE experts and to meet others sharing similar HAE experiences.

HAE Australasia will continue focusing on Australia but will also be helping HAE patients in New Zealand gain access to more treatments. Furthermore, the organization will be starting awareness activities in New Zealand to help educate the medical and general community about HAE.

It also proved to be a good opportunity for participants to meet the HAE Australasia board members and discuss how to improve HAE awareness in Australia and New Zealand.

### First patient/physician meeting in Japan

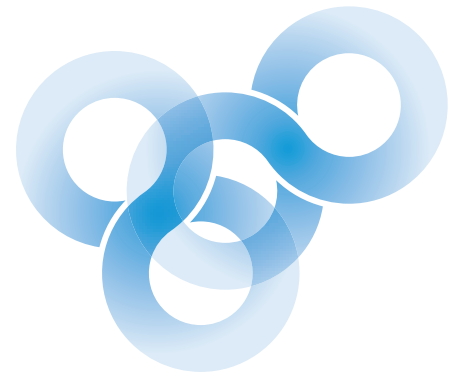
In mid April HAEi sponsored 2 inaugural patient/physician meetings in Japan, and provided Japanese HAE patients with their first opportunity to meet and interact.

served to motivate patients to begin the process of forming a patient group. We have been working with Professor Beverley Yamamoto – a HAE patient herself with a son diagnosed with HAE – and a Japanese doctor who is interested in helping angioedema patients.

HAEi delivered a presentation entitled “Patient Organizations are a Critical Force in Improving Diagnosis and Access to Life Saving HAE Therapies.”

We will continue to provide encouragement and guidance to Professor Yamamoto and Japanese patients as they take steps to organize a patient group.

Judging from the patient and physician feedback, the meetings were highly successful and have



## Gulf Region awareness meeting on its way

In January 2013, HAEi attended the Arab Health Conference that was held in Dubai.

During the meeting, HAEi met with a local HAE patient as well as an industry representatives in Dubai. The purpose of the meeting was to explore ways to improve HAE

awareness, diagnosis and treatment in the Gulf Region.

We are now assessing the feasibility of arranging a Gulf Region HAE awareness meeting conducted in late 2013, bringing together physicians from the entire region.

## New initiatives under way in Macedonia

In April HAEi attended the first HAE meeting in Macedonia and appeared on Macedonian national television to discuss problems faced by HAE patients. HAE Macedonia has been actively conducting an effective advocacy program, and their hard

work is about to pay off. During the meetings in Skopje, the Rare Diseases Committee of Macedonia said that it would “see to it” that Macedonian HAE patients will soon have affordable and on-the-shelf medicine throughout the year.

## WAO Global Guidelines for HAE

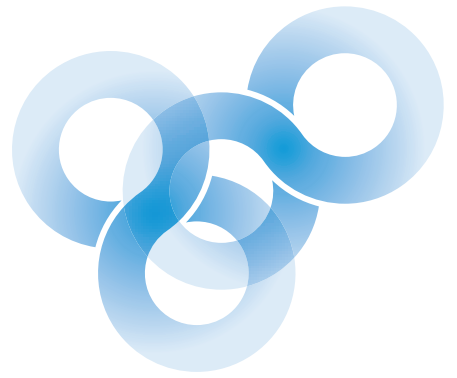
We would like to draw your attention to the “WAO Guideline for the Management of Hereditary Angioedema”, issued by the World Allergy Organization (WAO) as a part of the WAO Journal in December 2012.

WAO convened a group of HAE specialists from around the world to develop not only a consensus on diagnosis and management of HAE, but to also provide evidence-based grades, strength of evidence and classification for the consensus. The aim of the guideline is to improve

diagnosis and management of patients with HAE throughout the world and to help initiate uniform care and availability of therapies to all with a HAE diagnosis.

The first global guidelines for the management of HAE contain 20 evidence-based recommendations for clinical decision makers. These are just a few examples:

“All attacks that result in debilitation/dysfunction and/or involve the face, the neck, or the abdomen should be



considered for on-demand treatment. Treatment of attacks affecting the upper airways is mandatory.”

“The guidelines recommend that HAE attacks are treated with C1-INH, ecallantide, or icatibant.”

“The guidelines recommend that all patients should carry their on-demand treatment at all times.”

“The guidelines recommend that all patients who are provided with on-demand treatment licensed for self-administration should be taught to self-administer.”

The “WAO Guideline for the Management of Hereditary Angioedema” can be downloaded for free from:

<http://www.worldallergy.org/haealliance/pdf/wao-guideline.pdf>.

### First 2013 Executive Committee meeting

At the end of March we held the first 2013 Executive Committee meeting. This very fruitful meeting took place in Frankfurt am Main, Germany and was attended by the majority of the

committee members. A number of important issues were discussed and some of them will be the subjects of future newsletter articles.

### 2014 HAE Global Conference

Most of you probably still remember HAEi global conference in Copenhagen, Denmark in May 2012. Now we are excited to announce the venue and the dates for the 2014 HAE Global Conference.

Make sure that you already now save the date for this important global event bringing all HAE stakeholders

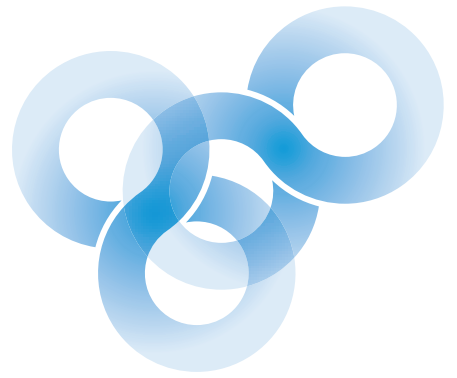
together. It will take place from 15 until 18 May 2014 – and we are extremely thrilled that the venue for 2014 will be **Washington DC Metro Area in the United States of America.**

More information will soon follow on [www.haei.org](http://www.haei.org) as well as in the HAEi Community and with our Facebook group.

### HAEi Patient Advocacy Forum in Europe

Representatives of the European HAE patient organizations will meet for

the third HAEi Patient Advocacy Forum on 14 - 16 June 2013 in



Frankfurt am Main, Germany. The meeting objective is to build on the important work that we started back in 2010 and continued the year after.

This time we will focus on updating the “State of Management of HAE in Europe – Facing up to Hereditary Angioedema” report using data

collected for the “European Burden of Illness” study.

HAEi believes that an updated version of the Patient Advocacy Forum report will provide patients with a powerful tool for enhancing the effectiveness of patient advocacy efforts.

## HAEi Patient Advocacy Forum in South America

We are planning to conduct the first HAEi Patient Advocacy Forum in South America later this year. Similar to the your objective is to create a report on the state of HAE manage-

ment in Latin America that can be used as a tool to advocate for better diagnosis and greater access to HAE medicines.

## HAEi’s global advocacy work – recent activities

HAEi is invited to participate in a variety of international meetings. Below is a brief overview of recent activities:

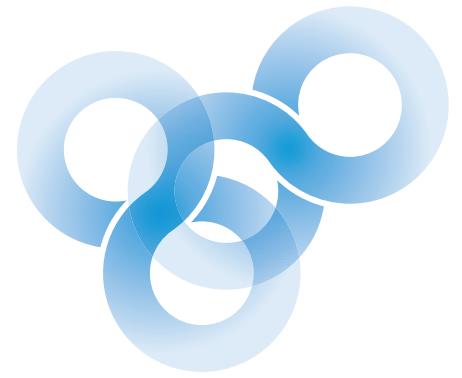
In February 2013 HAEi was present at the American Academy of Asthma, Allergy and Immunology (AAAAI) annual meeting in San Antonio, USA. During the meeting, we met with representatives from Dyax, Viro-Pharma, CSL Behring, and BioCryst to discuss HAEi global activities and funding.

In the beginning of March HAEi participated in the annual International Plasma Protein Conference

(IPPC) 2013 in Dublin, Ireland as well as in meetings with PLUS (Plasma Users). Issues addressed included regulatory matters as well as plasma safety and supply.

In late April, HAEi spoke at a meeting organized by Sanquin. The topic of the talk was “The role of patients and patient organizations in the management of HAE”. Other speakers at the meeting were Prof. Marcus Maurer, Prof. Marcel Levi, Prof. Teresa Caballero, and Prof. Anette Bygum.

Recently HAEi spoke at the annual UK Immunology and Allergy Nurses



Meeting in St. Mellion, UK – with

focus on the “Patient Perspective”.

## HAEi's global advocacy work – future activities

End May HAEi will be present at the 8th C1 Inhibitor Workshop in Budapest, Hungary. The conference organizers accepted an HAEi abstract that outlines the principles HAEi uses in helping patients and physicians form patient advocacy organizations.

In June HAEi will be involved in presentations at the European Academy of Allergy and Clinical Immunology (EAACI) & World Allergy Organization (WAO) World Allergy & Asthma Congress 2013 in Milan, Italy. The congress theme is “Allergy, A Global Health Challenge”.

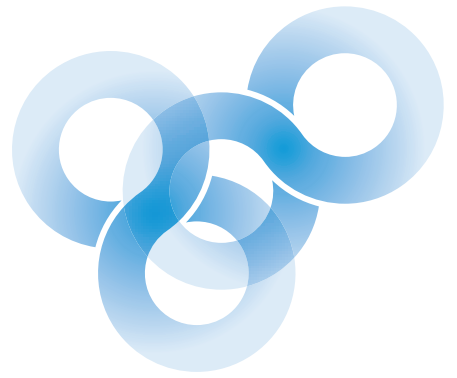
## News from the industry

At the 2013 American Academy of Allergy, Asthma and Immunology (AAAAI) Annual Meeting in Texas **Dyax Corp.** announced results from a preclinical study of DX-2930. The poster, titled “Discovery and characterization of a fully human monoclonal antibody inhibitor of plasma kallikrein for the treatment of plasma kallikrein-mediated edema”, highlighted results of preclinical work that was designed to evaluate the activity of DX-2930 as a long-acting inhibitor of plasma kallikrein (pKal). Dyax is exploring DX-2930 as a subcutaneous, long-acting, prophylactic treatment for HAE.

Based upon the attributes of selectivity, specificity and long half-life Dyax has chosen to develop DX-2930 as a self-administered, subcutaneous injection for chronic prevention of HAE attacks. Dyax plans to file an Investigational New

Drug (IND) application with the United States Food and Drug Administration (FDA) by the end of the second quarter of 2013 and expects to initiate Phase 1 clinical studies in the second half of 2013.

In January 2013 **ViroPharma Incorporated** announced the publication of data demonstrating that use of Cinryze® (C1 esterase inhibitor [human]) in pediatric patients provided relief from symptoms of HAE attacks and reduced the rate of attacks. The paper, entitled “Nanofiltered C1-Esterase Inhibitor for The Acute Management and Prevention of Hereditary Angioedema Attacks Due to C1-Inhibitor Deficiency in Children” by Dr. William Lumry et al. describes the efficacy and the safety profile of Cinryze in prevention and treatment of HAE attacks in the largest analysis of



pediatric data from prospective studies of patients with HAE ever reported in medical literature.

**CSL Behring** has announced the results of a cost-effectiveness analysis showing that on-demand treatment of HAE attacks with Berinert®, C1 Esterase Inhibitor (Human), provides cost savings to payers when compared with the use of two other therapies approved in the U.S. for the acute treatment of HAE. The cost study, which compared Berinert to Firazyr® and Kalbitor™, analyzed differential dosing and administration costs indirectly using current, publically available prices. Results were weighted by typical re-dosing frequencies. The study included no head-to-head comparison of product efficacy or safety.

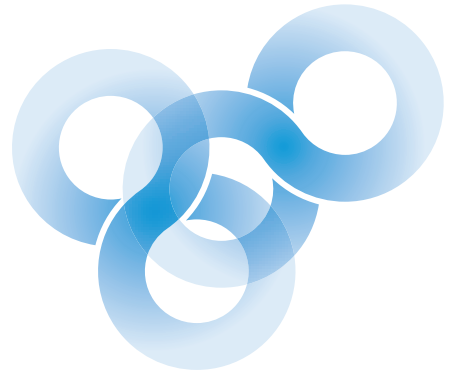
**Dyax Corp.** and CVie Therapeutics (CVie), a subsidiary of Lee's Pharmaceutical Holdings Ltd., has announced a strategic partnership for the development and commercialization of Kalbitor® (ecallantide) in the treatment of HAE and other angioedema indications in China, Hong Kong and Macau.

Together with Novellus Biopharma AG (Novellus) **Dyax Corp.** has announced a strategic partnership for the development and commercialization of Kalbitor® (ecallantide) for the treatment of HAE and other angioedema indications in select regions in Latin America, including Argentina, Brazil, Chile, Colombia, Mexico and Venezuela.

**CLS Behring:** European health authorities have approved an extended use of Berinert®, a C1-esterase inhibitor (C1-INH) concentrate, for pre-procedure prevention (short-term prophylaxis) of acute episodes of HAE in adult and pediatric patients undergoing medical, dental or surgical procedures. The approval supports the established treatment recommendations for the use of C1-INH concentrate as first-line therapy for the prevention of potentially life-threatening HAE attacks triggered by surgical or dental procedures.

Further information on these news items can be found at [www.haei.org](http://www.haei.org).





## Your feedback is very welcome

Please let us know what you believe should be included in future newsletters. You can do that by providing feedback to Communications Manager Steen Bjerre at [s.bjerre@haei.org](mailto:s.bjerre@haei.org) or Executive Director Henrik Balle Boysen at [h.boysen@haei.org](mailto:h.boysen@haei.org).

In addition, we invite you to submit articles on any topics that you believe would be of interest to other member organizations.

We look forward to your comments and working with you on future newsletters.



# Happy hae day :-) 2013!