EHC Newsletter

December 2014

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Editor: Amanda Bok
Editorial Committee: Jo Eerens, Radoslaw Kaczmarek, Brian O'Mahony, Olivia Romero-Lux, Laura Savini
AISBL EHC registered office: Rue du Marché aux Herbes 105 b14, 1000 Brussels, Belgium
Tel. +32-2 521 11 50, Fax +32-2-520 68 66, Email office@ehc.eu
No. 887.106.966

President and CEO Report

Thanking all who supported the work of the EHC in 2014


Brian O'Mahony, President of the EHC and Amanda Bok, EHC CEO

Approaching the end of another very productive year, we would like to begin this message by thanking all the volunteers, partners and sponsors who have made our work possible and especially who have enabled the growth that we achieved this year.

From 2013 to 2014 we increased our income by 40%, which enabled us to build on last year's work and activities, keeping the same programming but also adding two workshops – a Youth Leadership workshop and a New Technologies workshop; marking World Haemophilia Day with a high-level event and producing a well-targeted and effective advocacy video on the haemophilia recommendations of the European Directorate for the Quality of Medicines and Healthcare (EDQM) – an organisation part of the Council of Europe – coming out of the Wildbad Kreuth III consensus process; revising our website; hiring a Communications and Public Policy Officer; and overall elevating our level of work and impact.

HTA and Economics Workshop in Riga

We held the second Economics and Health Technology Assessment (HTA) workshop for National Member Organisations (NMOs) from central EU-Member States in Riga, Latvia, from 19-21 September (see article on pg 12), and have begun planning to hold the next one for Russian-speaking Eastern European NMOs in St Petersburg, Russia from 11-12 September 2015. These workshops are made possible by exclusive funding from Pfizer.

New Technologies Workshop in Dublin

We held the first EHC, multi-stakeholder workshop on New Technologies in Dublin, Ireland, from 20-22 November, which brought together patients, healthcare professionals and regulators to discuss the science behind innovative new products as well as the economics and patient access issues (see article on pg 14). This workshop was an ideal preparation for NMOs and clinicians in preparing for the probable availability of the new longer acting factor concentrates in Europe with the first product expected to be licenced in late 2015 and followed by further products in 2016. The workshop participants discussed probable treatment regimens with the products and were also forced to collectively evaluate theoretical treatment options and opinions in different clinical situations using an elicitation process. This workshop was made possible with an educational grant from Sobi. We look forward to continuing with this important series in 2015.

EHC Annual Conference and General Assembly in Belfast

We held our Annual Conference in Belfast, Northern Ireland, from 2-4 October and ran four well-received NMO workshops in parallel to a varied, state-of-the-art scientific programme and four stimulating symposia. With close to 300 participants in attendance, including a large youth contingent, the conference was lively, dynamic and full of energy and fresh perspectives. We extend our thanks to the UK Haemophilia Society for so warmly hosting our conference and look forward to collaborating with the Serbian Haemophilia Society on our next Annual Conference, to be held in Belgrade from 2-4 October 2015 (see article on pg 7).

In Belfast, we also held our General Assembly (GA) in which our members re-elected Jordan Nedevski to the position of Vice-President Finance on a renewed four-year term, updated the EHC's Statutes and gave the EHC a new visual face by voting on our new logo.

The EHC General Assembly voted on a new logo during its last meeting

The GA also approved the EHC's move into a new office space located in a non-profit building in the European Union quarter close to the European Parliament, the European Commission and key partner organisations. In December, following our last Round Table of the year, we hosted a cocktail in our new building and invited our partners to mark our 25th anniversary, our move into our new office space and the soft launch of our new website.

The revision of the EHC website has been a long process and we give special thanks to all those who worked hard on it over the past 1 ½ years. We are delighted to announce that the new website will be launched in January 2015. Some sections, including the News and Library pages, will remain a work in progress and we will be in touch with you, our members and stakeholders, over the next months and year to ensure that we develop these sections in accordance with your needs. The revision of the our website was made possible by an educational grant from Pfizer.

Round Table on: 'National Haemophilia Councils: From concept to reality'

The December Round Table (see article on pg 18) focused on National Haemophilia Councils (NHCs) and was held in the European Parliament on 2 December, co-hosted by MEPs Nessa Childers (Ireland) and Cristian Bușoi (Romania). It highlighted the importance of these statutory joint decision-making bodies and the effective role they can play in the management of haemophilia treatment, care and budgets nationally. Their establishment in all European countries is also an EDQM recommendation, which the EHC will continue to promote widely in all of our member countries in 2015. The success following the establishment of the NHC in Romania following the signature of a memorandum of understanding (MOU) between the Romanian NMO, the EHC and the Ministry of Health last year at the EHC Conference in Bucharest was highlighted. This has resulted in an 80% increase in the national haemophilia budget and is an excellent example of the increased visibility at Ministry level, which can be achieved by establishing an NHC (see article on pg 30).

Data collection

This year the EHC began and completed its data collection on national tenders and procurement systems. We would like to thank the 37 EHC members who responded to the survey and look forward to sharing the results with our community in early 2015. We will also ensure that the survey results are produced in a format, which will be of particular utility to those countries who responded. If your NMO is one of the few who have not responded, it is not yet too late. We can include late data received by January 10th in the analysis and report. This survey was particularly timely as the EU has published a new Procurement

Directive 2014/23/EU [1]– which will replace the existing Directive 2004/18/EC[2]. The new directive increases the efficiency of the process by shortening the time periods for submission, allowing for full electronic communication of tenders and specifies increased possibility for collaboration on tenders. In the course of 2014, we have been working with Latvia, Estonia and the European Commission to explore the possibility of a Baltic tender to improve access to care in those countries and to promote a more inclusive nature in the decision making process. This Directive may be of assistance. We will publish further details in later newsletters along with the results of the tenders and procurement survey. Next year our survey work will continue with a renewed look at the status of haemophilia care in Europe, when we will once again measure how the Principles of Haemophilia Care and the new EDQM recommendations are implemented in each of our member countries.

Update on hepatitis C treatment

Treatment for Hepatitis C took enormous strides forward in 2014 with the availability of several new direct acting antivirals. These therapies, which include sofosbuvir, ledispavir, simeprevir and daclastavir, are now being used with therapeutic success rates in excess of 90% in many cases. Of course the therapies are expensive and in advocating for access to these therapies, NMOs will find that the principles imparted in our HTA and Economics workshops will be very valuable. This will be an area of increased focus for the EHC in 2015.

Partnerships

We were very pleased in 2014 to increase our collaboration and positive working relationship with the European Association for Haemophilia and Allied Disorders (EAHAD). This included speaking slots for EHC at the EAHAD Conference and for EAHAD at the EHC Conference – a practice that will now become annual. Also, following on from the official MOU we signed with EAHAD we will have increased collaboration on administrative work, policy review and a joint visit to the Baltic countries together with the World Federation of Hemophilia (WFH) in 2015. We want to express our appreciation to EAHAD for the very positive and constructive ongoing engagement with the EHC.

Plasma-derived products' availability and safety

We had been greatly concerned by the Rome Declaration, initially supported by the World Health Organisation (WHO), which sought to greatly limit access to plasma-derived medicinal products (PDMPs) manufactured from the plasma of paid donors. This proposal, which sought to demonise paid plasmapheresis donors would have, if implemented, dramatically reduced the supply of safe and effective PDMPs worldwide and would have led to people dying. This proposal, which was far removed from reality, was vigorously opposed by the EHC in our collaboration with the Platform of Plasma Protein Users (PLUS) and by the counterparts of PLUS in the USA and Canada. We had productive discussions later in 2014 with the WHO, when we were assured that this illogical and impractical declaration did not in fact have the official support of the WHO. We will continue to monitor developments in this area, especially as the topic is now on the preliminary agenda for the WHO World Health Assembly in January 2015. We are also monitoring and responding as appropriate to other safety issues including the ongoing debate in many countries regarding MSM[3] blood donations. The issue of inhibitors came to the fore with the papers

[1] Directive 2014/23/EU of the European Parliament and of the Council of 26 February 2014 on the award of concession contracts Text with EEA relevance

[2] Directive 2004/18/EC of the European Parliament and of the Council of 31 March 2004 on the coordination of procedures for the award of public works contracts, public supply contracts and public service contracts.

[3] Males who have sex with males, are male persons who engage in sexual activity with members of the same sex, regardless of their sexual orientation and how they identify themselves.

published earlier this year in *Blood* from France and the UK, which followed the earlier RODIN[1] study showing an apparent increase in inhibitor risk in previously untreated patients with a second generation recombinant product. We will continue to monitor this and other issues together with our very active expert Medical Advisory Group. This will also be the topic for our first Round Table in 2015.

Looking ahead

In closing, we have exciting new work ahead of us in 2015, with a continuation of all of the above-mentioned programmes and activities and the addition of new initiatives, including a Leadership Conference pilot linked to our Annual Conference in Belgrade, in which we will bring together multi-generational leaders from within our NMOs to align on key issues and exchange best-practices; our joint project with EAHAD and the WFH in Eastern European countries; and other projects still under development.

We look forward to working together again in 2015 in these and other areas, but in the meantime, we take this opportunity to wish all of our members, partners and other stakeholders in our community a restful and rejuvenating holiday period with friends and family, a Merry Christmas and a happy New Year.

[1] Research Of Determinants of INhibitor (RODIN) Development among Previously Untreated Patients (PUPs) with haemophilia.

EHC News

EHC 2014 Conference in Belfast, Northern Ireland, UK

By Gordon Clarke and Liz Carroll***

Liz Carroll

Gordon Clarke

The UK Haemophilia Society had the pleasure of welcoming the European Haemophilia Consortium (EHC) 2014 Conference to Belfast on 3-4 October. The conference was a wonderful opportunity for National Member Organisations (NMOs), their members and health professionals to come together, learn, share ideas and discuss some of the challenges we all face in living with, supporting, campaigning for and treating everyone affected by bleeding disorders across Europe.

Workshops included discussions of the challenges of mild and moderate haemophilia, where we heard that for many, the most appropriate treatment and support is not always agreed upon and those living with mild or moderate haemophilia can sometimes feel as if they are not the priority for their medical team.

The workshop on youth-led strategies for engagement stimulated some lively debate as the panel were asked to role play the most extreme versions of the role they represented, bringing a fun but useful discussion alive. It seems there is no agreement on whether there should be specific youth committees to inform NMO thinking, or youth members should be included in the main governance structures of organisations, and your age doesn't seem to define which you think is best!

During the afternoon of day one the two parallel work streams on NMO activity and health focussed sessions allowed participants to think differently about their work or health as well as learn from experts and other participants. Almost every session highlighted both the differences and similarities between our experiences wherever you live in Europe.

Day one finished with a reception and dinner in the beautiful City Hall Belfast where Gordon Clarke from the UK Haemophilia Society, Brian O'Mahony, EHC President and Jim Wells, the new Minister for Health, Social Services and Public Safety spoke about the current challenges in care and support and how pleased we were to welcome the community to Belfast.

Day two focussed on new and emerging treatments and challenges in decision making. As with the first day the nosiest parts of the day were break times. The opportunity for long-standing friends to catch up, having not seen each other for a long time, as well as new friendships and relationships to be formed was as important as the workshop sessions. We were fortunate enough to be joined by two of the UK's top athletes, who both have severe haemophilia. The cyclist Alex Dowsett, who won a gold medal at the 2014 Commonwealth Games, celebrated his birthday on Saturday and encouraged everyone to have a go at cycling with him in his aim to get everyone clocking up the total miles for haemophilia as part of Pfizer's campaign to raise awareness. Jack Bridge also joined us and talked about his role in the UK para Olympic swimming team at the London 2012 Olympics. Both Jack and Alex are an inspiration to the UK bleeding disorders community, particularly the younger members and are working hard to encourage everyone to be fit and active despite their bleeding disorder.

The conference closed with a magnificent reception and dinner at Titanic Belfast, where Alain Weill President of the World Federation of Hemophilia (WFH), also presented Gordon Clarke with a WFH Lifetime Achievement Award for Outstanding Service. This award was announced at the WFH Congress in Melbourne in May, but as Gordon was not able to attend, Alain took the opportunity of being in Belfast to present the award. It meant a great deal to Gordon to accept this award in his home country surrounded by the community he has spent his lifetime working for. A fitting end to a fantastic conference. We hope you enjoyed your visit, and please do come back and visit us again.

*Gordon Clarke, The Haemophilia Society, UK Society UK

**Liz Carroll, Chief Executive, The Haemophilia Society, UK

Invitation to join the 2015 EHC Conference in Belgrade

By Vladimir Ilijin, President of the Board of Udruženje hemofiličara Srbije (the Serbian Haemophilia Society)

УДРУЖЕЊЕ ХЕМОФИЛИЧАРА СРБИЈЕ

I use this opportunity to thank you all for voting for the Serbian Haemophilia Society (SHS) to host the organisation of the 28th European Haemophilia Consortium (EHC) Conference and to invite you to Belgrade from 2 to 4 October 2015. It is a great honour for us to be chosen to host the biggest event in the European bleeding disorders community.

This 28th EHC Conference will crown the achievements of the SHS as the Society will celebrate its 15 years of existence in October 2015. Though SHS is a successor of the Centre for Haemophilia Care of Yugoslavia, it was re-established in today's form in 2000 and has 250 members out of around 700 patients with bleeding disorders in Serbia. All our members are excited and looking forward to host you next year in Belgrade.

Belgrade is a city of a long and turbulent history. It was always the crossroads of many nations, religions and empires whose legacies created a unique mixture, which is the biggest part of its charm. Today, Belgrade is a city, which is striving to be exciting, modern and European.

The program of the conference will be very interesting and it will keep up with the updates and news from the bleeding disorders community. Some of the topics that we will be covering include: gene therapy, women and bleeding disorders, ageing and cardio-vascular health, inhibitors, long-acting factors, orthopaedic surgery and haemophilia care in Serbia.

I sincerely hope that this invitation and our cheerful presentations in Bucharest and Belfast attracted your attention and that you will participate in the 28th EHC Conference in Belgrade.

Wishing you all a very warm welcome.

25 Years of EHC: Looking ahead: An interview with the next generation

By Laura Savini, EHC Communication and Public Policy Officer

To conclude the series of 25 years of EHC, Laura Savini speaks to four younger volunteers active in their own country and contributing to the activities of the European Haemophilia Consortium (EHC). She asks them what motivated them to first get involved in their National Member Organisations (NMOs) and what they perceive as future challenges for the work of their organisation's in the future.

Megi Neziri supports the advocacy activities of the Albanian NMO, liaising with the Ministry of Health, hospitals, the World Federation of Hemophilia (WFH) and the EHC.

Thomas Schindl is a board member of the Austrian NMO in charge of communications, public relations and international relations.

Aleksandra Ilijn is on the youth committee of the Serbian NMO and contributed to developing the proposal to host the EHC 2015 conference in her country.

Michael van der Linde is a volunteer with the Dutch NMO and the chair of the EHC Youth Committee.

It starts with a handshake…

This is how Michael describes the start of his journey into volunteering with the Nederlandse Vereniging van Hemofilie-Patiënten (NVHP – the Dutch Haemophilia Society), when, as a child, he took part in summer camps and other activities. Over time the NVHP provided him not only with more practical information on how to manage living with a bleeding disorder but also with friendships and a social network. At 17, he started to volunteer within the association. He took part in the NVHP's annual sailing trip and heard about the idea of developing a youth group within the NMO. He immediately decided to join the group, not only to volunteer but also as a social activity.

Like Michael, Thomas was also involved in the Austrian NMO since childhood, however during his teenage years he distanced himself from the NMO and the community. It was only during university, while he was on an exchange year in Paris, that he got back in touch with the NMO to ask for some support to access treatment abroad. It is following this contact that he got back in touch and became active with the Society.

Aleksandra explained that it was only in 2012 that carriers could officially get involved in the Serbian NMO, which her father is currently presiding. In 2011, she accompanied her father to the EHC Conference in Budapest where she had the opportunity to meet the people behind the organisation. "*I wanted to learn more and be more involved. I approached the board and asked them to send me to the conference. This is when I decided to become involved in the association's activities.*"

Megi followed in the steps of her mother, who has been working for many years in the Albanian NMO. Megi lived and studied in Italy, where her brother also currently lives. She noticed the difference in

treatment and quality of life for her brother compared to other people with haemophilia (PWH) in her own country and that pushed her to get involved.

…and continues with a WFH Congress

For all of them, the first international exposure to the global community came through the activities of the World Federation of Hemophilia (WFH) and through some learning programs such as Step Up Reach Out (SURO) developed by the pharmaceutical industry.

When Aleksandra got involved in her NMO's activities in 2012, it was the same year that she attended the WFH Congress in Paris, where she was awarded the Susan Skinner Memorial Fund Scholarship that supports the training and education of young women with bleeding disorders who demonstrate outstanding leadership potential in their home countries to improve the care of people with bleeding disorders.

For Megi, one of the first tasks she helped with while working with her NMO was to translate during WFH activities and visits in her country. Earlier this year, she attended the WFH Congress in Melbourne thanks to the WFH Youth Fellowship, where she also attended the NMO Training and the General Assembly.

This first congress had a big impact on Megi as it gave her the opportunity to communicate and share experiences with other carriers from around the world. It was also where she had the opportunity to witness, on a larger scale, how people with haemophilia live when they have access to treatment and that really encouraged her for her work.

It was in 2006 that Thomas attended his first WFH Congress in Vancouver, where he also took part in the NMO Training and General Assembly. In the same year, he attended the first EHC Round Table at the European Parliament. At the time, the late Dr Hubert Hartl, whom Thomas knew well, was the President of the EHC. Since then, Thomas has attended the EHC Annual Conference on a regular basis.

Michael also attended WFH conferences, where he had the opportunity to meet many youth from other European countries. It was at the WFH Congress in Argentina where the idea to develop a European youth training emerged. Although the WFH already had a youth programme as part of its NMO training, Michael felt that something more tailored to the needs of the European region was needed. "*From discussions I had with people from other European countries, I realised that we all struggled with the same problems, which may be very different in other world regions. So I decided to approach the EHC as it seemed the logical place to go to.*" He first approached the EHC at the WFH Congress in Paris and after a series of exchanges to finalise the project and to raise funding, the Youth project became a reality last July with the first EHC Youth Workshop held in Amsterdam.

Challenges for the haemophilia community

The main challenge in Michael's opinion is that many NMOs struggle to attract and retain volunteers for their activities.

"The number of volunteers is in decline as PWH do not feel any urgency any more. In Western Europe, the treatment facilities and treatment regimens are so good, that people do not feel compelled to join a patient group any longer," warns Michael van der Linde

The idea for Michael and others behind the youth workshop proposal was to provide training to develop skills and leadership to deal with these types of problems. "*The Amsterdam experience was very different from what I had in mind at the time we pitched the project. I only had my own perspective and what I had discussed with others, however the result was very pleasing.*"

Both Aleksandra and Megi were participants in the workshop, while Thomas was one of the speakers and moderators.

Megi explained that the workshop provided her with lots of ideas to work with for her NMO. Meanwhile, Aleksandra is currently setting up a youth committee in Serbia. The idea for a youth committee came to her while at the WFH Congress in Paris where she met with other volunteers. She then came back to Serbia and pitched the idea to her board. The process took time, as resources are limited in her NMO, but it's now underway with current selections for youth committee ongoing.

When asked about the future challenges for the haemophilia community and patients' organisation in general we see as a main issue the lack of volunteers in Western Europe, while for the Eastern non-EU countries, the main issue is to get access to proper treatment.

For Megi, for example, one of the biggest challenges is to improve treatment levels for patients both through the establishment of a proper Comprehensive Care Centre in Tirana as well as through the purchasing of additional treatment products. Also, inadequate treatment means that a lot of young PWH and other people with bleeding disorders miss out on school and work days, which has negative consequences on the social and financial welfare of patients: "*If we have good treatment we will have less social and financial problems,*" states Megi. In addition, involving volunteers seems to be difficult and her NMO is currently looking into strategies to recruit new volunteers, notably through social media.

For Aleksandra, a major issue for her NMO was to get established and to gain credibility with the medical profession and the government. Another challenge is to prove to stakeholders that patients' perspectives are as valuable as those of other stakeholders. In her opinion, one of the biggest struggles of her NMO was to prove their knowledge and expertise and to be considered as equals.

For Thomas, one of the major threats is the impact of the financial crisis on European healthcare systems. In his opinion, the effects of the crisis have not had their full impact yet on healthcare systems and patients will increasingly feel them. Although Austria is a country with generous healthcare coverage, there are some tendencies towards budget restrictions and towards being more cost-effective. For instance, Austria is going through a major reform of its healthcare system and it is not quite clear where this will lead. Another problem is the issue of succession in patients' organisations.

"There is a huge issue in motivating young people to join patients' groups and getting involved to take over responsibility. This is for sure an effect of the good treatment that PWH and people with other bleeding disorders are getting in Austria and other Western European countries. Patients' needs are different today compared to 20 years ago and this leads to a need to re-shape the mission statement of haemophilia societies, including the EHC. Although, there is still a legacy from the past 20 years that is still not resolved and also needs to be addressed," states Thomas Schindl

The value of patients' groups

These statements led to the interviewees being asked of what, in their opinion, is the value and role of today's haemophilia societies.

For Thomas, the patients' organisations' value lays in the work they do to educate and raise awareness about the disorder: "*Haemophilia is a rare disease and there is a need to educate and inform about this condition with the wider public but also to provide support to the parents of newly diagnosed patients and to patients' themselves. Patients' groups can train patients on how to take care of their condition, to understand the disease and how to deal with difficult situations. These issues will persist.*" Furthermore, the haemophilia community is quite small so there will always be a need for a strong advocacy group to make sure that patients' interests are looked after.

Michael has the same perception as Thomas regarding the reasons to maintain patients' groups. He believes that education, developing contacts, bringing people together and advocating on behalf of patients are the core objectives of patients' groups. Michael jokingly says that: "*One week of summer camp equals one year of therapy*." Also, patients groups' need to remain vigilant at all time. "*The world is never standing still, and so patients' groups need to evolve to keep up with changes in their environment.*"

For Aleksandra: "*It is important to have a clear strategy at the local level on how to improve access to care for patients and how to reach out to physicians and the government. The voice of the patients' society is stronger than single patients working alone*;" which is also a view reflected by Megi. In her opinion, patients' groups need to speak with a loud voice to governments and all other relevant stakeholders about what bleeding disorders are and to let them know that patients' groups have a presence and representation mission. Also, patients' groups are important to share experiences.

The value of the EHC

Sharing experiences is perceived by most volunteers as the main role of the EHC. For Megi, for example, attending EHC Conferences is a very enriching process. It is a place to meet people with similar problems from other countries, to talk about these problems and to find solutions.

For Michal, the EHC provides a knowledge basis for all different countries who are dealing with similar national issues such as access to treatment, reimbursement and so on. Also the EHC can directly advocate at European level by combining the strengths of various national groups. Finally, it can support individual countries that have problems by providing examples of what is happening in other countries and how problems are solved elsewhere.

For Aleksandra, the ability to share experiences is 'priceless'. The EHC provides a European network and someone to turn to, so that you know that you are not alone and you have a resource to learn from other countries.

For Thomas, the question is difficult to answer. On the one hand most European countries have better access to treatment than the rest of the world, concluding that the need for support would be more urgent for most countries and patients outside of Europe. On the other hand Europe, and the EHC respectively, has the chance of taking a leading role in the global haemophilia community when it comes to establishing common goals and standards of care for all haemophilia patients worldwide. The WFH also works with European countries on projects such as Twinnings and the Advocacy in Action (AIA) programmes, so there should be a careful distinction between the roles played by each organisation. One EHC initiative that Thomas finds distinctively positive is the European certification process for haemophilia treatment centres and he hopes that a similar project can be applied to other regions of the world. Nevertheless, in his opinion, there is still a big divide in treatment in Europe when he compares for example countries like Romania and Austria, which are worlds apart in terms of level of treatment. In his mind, the EHC must offer a base to tackle those differences and to reduce the division.

This concludes our special series on the '25 years of the EHC,' which we developed in commemoration of our 25th anniversary this year. We invite you to read our other articles in this series: Celebrating 25 years of EHC: the origins of the EHC (EHC Newsletter April 2014) and 25 Years of the EHC: A perspective from physicians (EHC Newsletter August 2014) and hope that you have found compelling the past, current and future perspectives that we have shared with you as part of this special series.

Report on the EHC Workshop on Economics and Health Technology Assessments

By Lino Holstetter and Lukas Zahrer***

We were delighted to be part of a delegation taught basics on health economics and Health Technology Assessments (HTAs) on haemophilia treatment during the second edition of this EHC workshop cycle held in Riga, Latvia.

It was during the beautiful weekend of 19-21 September 2014 that participants from 12 countries met at the Avalon Hotel in Riga, the beautiful capital city of Latvia. We were excited to get an overview of basic concepts of health economics, how HTAs are constructed, and what kind of process is followed for a new drug to get reimbursed by public insurance.

To be honest, we did not really know much about all this, but were well aware that a lot of the work (especially from the EHC) deals with these issues and many papers were published on these topics recently. The program kicked off on Saturday morning with a short questionnaire, where we were reminded that our level of knowledge in this area was not the best. The quiz was repeated at the end of the workshop on Sunday afternoon, so that all the participants could see improvements in their levels of understanding on their own – and it worked!

Participants at the HTA and Economics Workshop

After the first questionnaire, Keith Tolley gave a speech on concepts of basic economics, which went into the depth of the topic really quickly. Keith is an independent health economist, who works closely, amongst others, with the UK-based National Institute for Health and Care Excellence (NICE), which provides national guidance and advice to improve health and social care. We found out about important key metrics like quality adjusted life years (QALYs) and others that come up regularly in literature on health economics.

Brian O'Mahony's presentation followed and it gave us the natural link between principles of health economics and the haemophilia world. He gave us an overview of which HTAs have been performed in the area of haemophilia care and which advantages and disadvantages these HTAs may have. In the afternoon, HTAs were examined from three different perspectives: the payer's (with a presentation from Keith Tolley), the industry's (with a presentation from Adam Heathfield, Pfizer) and the Patient Organisation's (with presentations from Baiba Ziemele, Latvia; Radoslaw Kaczmarek, Poland; and Haluk Zülfikar, Turkey).

In the afternoon Declan Noone, chair of the EHC Data and Economics Committee, introduced us to six new controversial and fictional pharmaceutical drugs. We had to decide from a payer's perspective with a €100 million annual budget which drug we were going to choose to cover. The decision was not easy. "Who needs it the most? Which one is the most cost-effective one?" were some of the questions. We had a lot of discussion but finally we all agreed on our final choice.

From left to right: Prof Margit Serban, Zrinko Salek and Lino Holstetter

The first day of the workshop found an end with a dinner in the old city of Riga, which is a UNESCO world heritage site since 1997.

The second day of the workshop began with a presentation about HTA models for prophylaxis by Declan Noone. He showed us a cost-utility model for prophylaxis versus on-demand treatment. We learned about the components of an economic evaluation that includes cost effectiveness, cost utility, cost minimisation and cost benefit. This presentation was followed by case studies in which we took part. We were divided into two groups. Each group received a case study that included a regulatory measure from a national government. We had to prepare our arguments to advocate for the government to maintain its coverage of certain products or of a prophylaxis regime. Then the participants who represented the National Member Organisation (NMO) had a fictional meeting with the Health Ministers (played by Declan Noone and Radoslaw Kaczmarek). This exercise showed us that you have to be very well prepared when it comes to a meeting with the Health Minister and that data and sometimes affective empathy is necessary.

From left to right: Lukas Zaher, Egidijus Sliauzys, Uros Brezavscek and LIz Carroll

The workshop gave us a very good overview of HTAs and it helped us to better understand health politics and what phases new products have to go through. We now know about the main methods of economic evaluation and how we can defend the accessibility and coverage of the products.

We would like to thank the EHC for organising this interesting workshop and Pfizer who sponsored it.

**Lino Hostettler is a board member of the Schweiz. Hämophilie-Gesellschaft (the Swiss Haemophilia Society)*

***Lukas Zahrer is a board member of the Österreichischen Hämophilie Gesellschaft (the Austrian Haemophilia Society)*

Report on the EHC New Technologies Workshop

By Laura Savini, EHC Communication and Public Policy Officer

On 22-23 November, 40 participants from 17 countries attended the first EHC workshop on New Technologies, held in Dublin. Participants included patients' representatives, healthcare providers and regulators. The workshop followed on from last year's pilot organised by the Irish Haemophilia Society on the technologies behind the new generation treatments for haemophilia A and haemophilia B due to arrive on the European markets in the next couple of years.

The programme included scientific sessions explaining in detail the technologies behind novel treatments, including longer-acting products for haemophilia A and haemophilia B, bi-specific antibodies as well as the regulatory landscape in Europe and the potential pricing schemes and reimbursement mechanisms for these products. Participants also took part in two interactive workshops on the prioritisation of access and the economic access to medicines. The workshop's sessions and ensuing discussions were lively, interactive and enriching.

Participants at the New Technology Workshop

New Technologies

The novel technologies presented were PEGylation, Fc-fusion, albumin-fusion and bispecific antibody. The first three technologies extend the half-life of current coagulation factors VIII and IX, while the bispecific antibody replaces the factor VIII altogether in the coagulation cascade. The first key information that participants learned is that half-life enhancing technologies such as PEGylation and fusion can significantly prolong (up to 80-100 hours or up to a five-fold increase) the half-life of factor IX, while unfortunately only slightly increasing (about 18 hours or less than a two-fold increase) the half-life of factor VIII. This is primarily due to its strong dependence on von Willebrand factor. These results could allow a prophylactic regimen of an average of one infusion for patients with severe haemophilia B every one to two weeks; while patients suffering from severe haemophilia A would have to infuse once or twice a week. This may however change with the advent of bispecific antibody, which has so far proved very encouraging in terms of efficacy for patients with haemophilia A. For instance, the potential half-life of bispecific antibody could be of up to a month, although in clinical trials the product was still administered on a weekly basis. Besides a reduction in frequency of infusions, another exciting development is that these products can provide much higher trough levels than current products with some achieving around anywhere from eight up to 20 per cent. In addition the bispecific antibody offers a subcutaneous route of administration, hence preserving venous access. All of these developments are very exciting because they are perceived to considerably improve the quality of life of patients with severe haemophilia by reducing the number of injections and providing

better protection against bleeds. However, all presenters warned that these technologies are relatively new for long-term and intravenous use and therefore, once marketed, these products should be monitored closely for side effects. In fact, the data of the clinical studies alone is not enough to determine long-term side effects.

Regulatory Requirements

Participants were given a brief introduction and explanation on regulatory requirements for putting new medicines on the market in both Europe and in the US (where these products have been recently marketed). The European Medicines Agency (EMA) and the US Food and Drug Administration (FDA) are the major regulatory agencies issuing guidelines and enforcing rules and regulations that oversee the marketing of drugs. At the moment, pre-authorisation requirements for safety and efficacy differ for the EMA and the FDA. The differences lay in the number of previously treated patients exposed to the product and the paediatric studies. In Europe a paediatric investigational plan is required at the end of phase I clinical trial, while in the US a study in the paediatric population is required in the post-marketing phase. Furthermore, there are differences in defining low, high and cut-off inhibitor titers and assays. Currently, it is estimated that the paediatric requirements of the EMA increase the time to market access from two to three years, compared to the US. This is an area in which the EHC has conducted advocacy efforts since 2012 (see articles in EHC Newsletter from April and August 2013).

Speakers at the workshop

Another regulatory aspect is post-marketing surveillance. The new pharmacovigilance regulation calls for strengthened requirements for post-authorisation safety and efficacy studies, which would be especially welcome with these new therapies as data from pre-clinical studies is not sufficient to fully assess safety.

Finally, participants were informed about the Orphan Medicinal Product Regulation, which in Europe grants a series of advantages to promote the development of medicinal products for rare conditions that either do not have a treatment or that will bring a significant added value to existing treatments. One of these advantages is ten-year marketing exclusivity. Although this incentive is seen as a very positive motivation for other therapeutic areas, the EHC does not believe it should be applied to novel generation treatment for haemophilia A and haemophilia B as all these treatments use different technologies and also because in fact haemophilia is not a typical rare disease and has many competing coagulation products already existing on the market. The EHC has also carried out actions on this point as additionally it fears that marketing exclusivity could create a monopoly for one product preventing others from coming to the market and thus preventing prices for older generation products from adapting to the advent of newer generation products.

Prioritisation of Access

During this workshop, healthcare providers and patients actively discussed the perceived advantages and disadvantages of longer-acting products and explored what the ideal circumstances would be for patients

to switch to these new treatments. Participants also raised the question of what criteria should determine prioritisation of access.

The first remark was that there is no one-size-fits-all protocol when it comes to longer acting products. For instance, in the case of longer acting factor IX, the frequency can be anywhere between one infusion every two weeks to twice per week. The frequency of the infusions should be decided on an individual basis and considering a variety of factors such as, for example, the patients' activity level, venous access, level of joint damage or number of target joints. Reduced injections are also clearly perceived as being advantageous to improve adherence and to facilitate prophylaxis in children. However, physicians who have already worked with these longer acting products have noted that the primary concern of the parents of children with haemophilia is safety. In fact, physicians stressed the importance of being vigilant with these new products as the studies currently underway do not provide enough data to fully evaluate the safety of these new treatments.

Discussions at the event

Physicians agreed that the current trough level of 1% is barely sufficient to protect joints and avoid bleeds. Ideally, there should be an aim to reach a trough level of 12%, although this seems unrealistic due to the costs associated with it. The next question would then be what trough level should be achieved, which brings us back to the first statement that no one treatment protocol fits all and that trough levels, like treatment regimens, should be decided for each individual patient depending on his/ her medical history and lifestyle. Furthermore, less frequent infusions may create situations in which patients are at lower trough levels for longer periods, meaning that bleeds could more easily occur if physical activities or minor injuries occur during the lower peaks.

Participants were also asked to do an exercise with various case studies of hypothetical patients and ask them to determine which patients should be switched to novel treatments and which should be maintained on older generation products.

In conclusion, the discussions on the topic of prioritisation of access were extensive and participants were able to listen to three different patient perspectives from different geographical regions in Europe and North America. The broad conclusion is that defining clear cut parameters for prioritisation of access is difficult because it is in fact a very personal choice that should be made based on many different factors and in consultation with the patient and physician. Furthermore, should guidelines be set, it is a widespread reality that medicinal products are often used off-label and off-guidelines and so it would be difficult to implement these. Participants heard that these longer acting products will be officially available in Canada as of Q2/2015. It will be then interesting to draw expertise from the Canadian experience on how to prioritise access and how to manage patients with these new treatments.

Pricing and reimbursement

The next question discussed was how these products would be priced. A very comprehensive presentation was provided on the pricing strategy that companies are perceived to use to price these products for the US and Canadian market. The idea would be not to compare a unit with a unit but to price quantities used to achieve similar trough levels. This would mean that depending on the product, a unit could be priced as either times one and half or times two the price of an older generation product unit. This type of pricing would ensure that current budgets for haemophilia care are not exceeded by these new treatments. Participants discussed these strategies and also questioned patients' access to these new products, in particular in countries that rely on national tenders. Participants questioned how a tender commission would compare the new products with the older generation products. They also questioned whether, if a country switches to longer acting products, it could then switch back to older generation products if it wanted to. Furthermore, if a country would choose to provide its patients with both older and newer generation products, medical professionals will have to be more careful with dosing and potential medical errors if using both types of products for different purposes (e.g. prophylaxis vs treating a bleed). This point also led to the question of monitoring patients' levels and laboratory assays. Every assay requires reconstitution of specific standards, which could disrupt the work of busy laboratories and result in lack of round-the-clock availability of modified assays even in larger hospitals.

Participants during the event

The question of pricing also came into play for resource-limited countries that currently pay lower prices for recombinant products. At the moment, we note in Europe alone a 400% price difference[1] between the cheapest and most expensive unit of FVIII product. The question is whether governments will be willing to pay a higher price for novel generation products or if they will prefer to keep older generation treatments. The question will also be whether companies will adapt their pricing depending on the country's perceived ability to pay.

Again the discussions on the potential modalities for economic evaluation, pricing and reimbursement were extensive and lively and with no country currently fully using and reimbursing these products, they still remained somewhat hypothetical. The factors contributing to the setting of prices for reimbursement are many and one major factor is also the current wave of budget cuts in healthcare services and products. This is why it is expected that advocacy efforts from both patients and physicians will be necessary in order to ensure patients' access to these products.

[1] Based on preliminary results of the upcoming EHC survey on 'Tenders and Procurement.'

To be continued…

As seen from the aforementioned discussions, the question of how these products will work and how they will be made available is still evolving and this is why the EHC is planning to organise a second edition of this workshop next year from 20-22 November. Workshop invitations will be sent to targeted EHC national member organisations and other stakeholders in the new year, however, should your NMO have a particular interest in taking part in this workshop, please feel free to contact the EHC office to enquire for potential participation.

The main sessions of the workshop will be available to the public in the new year via webcasting on the EHC YouTube Channel. The EHC would like to thank Sobi for the educational grant, which made this workshop possible.

EHC Round Table on National Haemophilia Councils: From concept to reality

By Laura Savini, EHC Communication and Public Policy Officer

On 1 December 2014, the European Haemophilia Consortium (EHC) organised a Round Table on: "National Haemophilia Councils: From concept to reality" at the European Parliament in Brussels.

The event was co-chaired by Members of the European Parliament (MEP), Mrs Nessa Childers (S&D/ Ireland) and Dr Cristian Bușoi (EPP/ Romania) and was attended by over 40 participants.

Mrs Nessa Childers (center) talking with the Round Table participants

The event was opened by Mrs Childers, who explained that the effect of budget cuts had been discussed widely in the European Parliament as these often resulted in a limited access to treatment for more vulnerable populations, including people with chronic or rare conditions. Mrs Childers was pleased to be able to present a model for cost-effectiveness in organising healthcare services and in particular she was pleased to have speakers talking about a successful example of how this can be achieved through a National Haemophilia Councils (NHC) in her country, Ireland.

This introduction was followed by a video-message from Dr Cristian Busoi, who unfortunately could not attend the event as it was Romania national day and he had to attend other events in his country.

The talks were kicked off by Mr Brian O'Mahony, president of the EHC, who provided a quick overview of what NHC are and demonstrated their utility through practical examples. NHC allow healthcare to be coordinated nationally, thereby more appropriately distributing limited resources in accordance with

patients' needs. They bring together all the key stakeholders including patients' representatives, government officials, representatives from payers and insurance companies, and physicians. They allow patients' organisations to develop relationships with these key players and provide them with a better understanding of the disease and patients' needs. In terms of budgetary management, centralised budgets for bleeding disorders allow hospitals and physicians to work more freely as they will not have an impact on a single hospital budget but their costs will be absorbed by the haemophilia budget.

Furthermore, he added that NHCs are one of the seven recommendations from the European recommendations on haemophilia treatment agreed upon by the European Directorate for European Directorate for Quality of Medicines and Healthcare (EDQM), coming out of the Kreuth III initiative. So far, only Ireland, Romania and Georgia have formal haemophilia councils, which means that much work remains to be accomplished in other European countries to create these bodies. Many countries in Europe have an unofficial body to provide advice on haemophilia care, however making these bodies official and providing them with decision-making power is far more advantageous. In fact, during the discussions it appeared that whenever these bodies are informal, their input and advice is not always taken into account, which many feel limit the effectiveness of contributions.

The presentations continued with Dr Barry White, director of the Irish National Haemophilia Centre. He talked about the organisation of haemophilia care in Ireland and the benefits brought by the NHC. He explained that all patients with severe haemophilia are on home treatment, all children are on primary prophylaxis and all adults on secondary prophylaxis. Currently, recombinant products are used. He explained that one of the biggest advantages of NHCs is to receive patients' perspective directly as they are the direct users of the healthcare system and have the ability to point out shortcomings and ways for improvement. He stressed that NHCs should be based on true partnership and all those around the table treated as equal partners.

The key achievements of the NHC in Ireland were to define guidelines, infrastructure and performance reviews. They also improved the supply chain for home treatment with the introduction of a phone app to monitor product use and stock. The NHC also considerably contributed to cost control by establishing national tenders and it facilitated the organisation of data collection for co-morbidities such as hepatitis C.

Dr Barry White presenting during the Round Table

The next part of the event provided an overview from three very different countries and how healthcare services for bleeding disorders are organised there. Each presentation outlined the potential need for a NHC.

Mr Radoslaw Kaczmarek, a member of the EHC Steering Committee and a member of the Polish Haemophilia Society, presented the situation in his country. He started his presentation by re-stating the

benefits of patients' involvement in haemophilia care, citing that according to the published data from 'Haemophilia Care: a survey of 35 countries,' countries that actively involve patients in their decision-making process are also shown to provide them with better quality of care.

He then gave a quick overview of Poland, where following the end of the Cold War, treatment for haemophilia considerably improved. As key milestones, he noted that in 2008 there was a decision to put all paediatric patients on primary prophylaxis. In 2005, the country also established a comprehensive haemophilia program. This program was then renewed in 2012 with, as its main objectives, an improved access to prophylaxis for adult patients and the development of a network of comprehensive care treatment centres. However, after being agreed upon, the budget of this program was suddenly cut in half in 2014. This decision was taken under obscure circumstances and without the consultation of patients' representatives because that was considered unnecessary. Mr Kaczmarek then proceeded to point out that this would probably have been different if an official body with patients' representatives had existed.

Mrs Liz Carroll, CEO of the UK Haemophilia Society followed Mr Kaczmarek. She presented the history of the haemophilia alliance established in 1999 between patients and physicians to advance the care of people with bleeding disorders. In 2009 the alliance also included representatives from the four British nations: England, Wales, Scotland and Northern Ireland. As part of its main activities, the alliance informed all those responsible for the commissioning of haemophilia care services about state of the art guidelines for the care of patients with bleeding disorders, commented on the commissioning of proposals and took actions on variant Creutzfeld Jakob and Hepatitis C-related issues.

The current problem with the UK healthcare system lays in the fact that all services are decentralised and so there are now local commissions for specific services, which all include a separate aspect of haemophilia care. This means that it is almost impossible for anyone in charge of healthcare organisation to have a comprehensive view of the needs of haemophilia care because commissioning bodies not only work on specific services and products, but also act nationally and lack a big picture. This situation is particularly disadvantageous for the care of rare conditions, including haemophilia. The UK Haemophilia Society is trying to tackle this situation but this current fragmentation of healthcare services is particularly difficult to approach.

The last country experience was shown by Mr Daniel Andrei, President of the Romanian Haemophilia Association. He quickly gave an overview of haemophilia care in Romania, highlighting the great disparities in terms of access to treatment. He then continued by outlining the work carried out by the EHC and the Romanian Association during the 2013 EHC Conference that took place in Bucharest. During this conference, the EHC together with the Romanian Association signed for the first time a Memorandum of Understanding (MOU) with the Romanian Ministry of Health. Since then, the budget for haemophilia care increased by 80%. The MOU also established a NHC, which has so far met three times and, which is actively involving patients for the organisation in haemophilia care. So far, the feedback from this experience has been very positive (see article on page 30).

Participants during the event

The final presentation was from Mrs Camille Bullot from the European Patients' Forum, an umbrella organisation representing multinational patients' groups for chronic conditions and of which the EHC is a member. Mrs Bullot presented the differences between patient empowerment and patient involvement outlining that patient empowerment focus on the ability of patients to take care of their conditions and make choices regarding how they deal with their disease, while patient involvement is about having an active and meaningful role in the decision-making process that will affect the patient community. She stressed the fact that meaningful patient involvement can only take place when patients are considered as equal partners and are properly resourced to carry out their involvement.

She then provided a brief outline of the EU policy context and the actions promoting patient involvement. She outlined how patients are already actively taking part in activities of the European Medicines Agency (EMA) and how patient empowerment is promoted in several EU legislations such as the Cross-Border Healthcare Directive and the Medical Devices Regulation. She finished her presentation by stressing the importance of adequate patient representation throughout the policy life-cycle but also the importance of patients' groups being independent and unbiased by industry or other stakeholders.

The presentations were followed by discussions in which participants voiced their primary concerns regarding how in many countries patients' voices are either not taken into account or not taken seriously. Mr Brian O'Mahony stressed again that this is one of the main reasons to have a formal body so that patients are considered as equal partners and their opinion taken seriously.

In conclusion, the Round Table generated fruitful discussions and exchanges of best practices amongst different European countries. It came out clearly that patients provide an added value in the organisation of healthcare as they are the primary consumers and can point out shortcomings and ways in which the system can be improved. Nonetheless, it is also very important for patients to be appropriately trained in order to make valuable and relevant contributions with these decision-making bodies.

NMO News

EHC welcomes 45th member: Kyrgyz Haemophilia Association

By Sergey Filipenko, President of the Kyrgyz Haemophilia Society

Good day dear friends,

Kyrgyzstan has a population of approximately six million people with different ethnicities. It is a mountainous region with beautiful nature. The biggest natural landmark is the lake Issyk-Kul.

In our country, haemophilia patients are treated in hospital with plasma and cryoprecipitate. Our Society was created over 12 years ago and because Kyrgyzstan is not a rich country we have poor access to care. The amount of treatment for children up to 16 years is insufficient to cover their needs and adults do not receive any treatment from the state. Recently we lost a girl with haemophilia A because of the lack of diagnosis expertise. Amongst our members, we also have a patient with factor I deficiency. However, despite the adversity we do not give up and try to support each other the best we can. We have qualified surgeons, who are able to conduct surgeries and we sometimes ask neighbouring countries for assistance for these operations.

Every year, we organise activities around World Haemophilia Day. We ask our local blood transfusion centres to provide us with a room and we advertise our activities on social networks and on the radio. We try to raise awareness and to encourage people to come and donate blood. Our slogan is 'Giving blood saves lives' as in our country cryoprecipitate in an integral part of haemophilia care. In 2008, our government implemented a measure to provide free cryoprecipitate to haemophilia patients, however until this year we have still had to buy it.

This year we launched our new website: http://hemophilia.go.kg/ and our objective is to create a patient registry. We have many plans and together we will succeed!

Members of the Kyrgyz Haemophilia Society during World Haemophilia Day

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Добрый день уважаемые друзья. В Кыргызстана около 6 000 000 человек проживает. Разных национальностей. Больных гемофилией лечат в больницах крио, плазмой. Закупок не хватает на детей до 16 лет. Взрослые не получают препарат от государства.

У нас была 1 девочка с заболеванием гемофилия А. мы ее потеряли. Не смогли вовремя поставить диагноз.

Также у нас есть 1 пациент с формой не свертывания крови № 1

В одной семье есть отец больной гемофилии и родившийся сын тоже болеет гемофилией. Я думаю что они родственники кровные с женой!

В Кыргызстане очень красивая природа. У нас есть незамерзающее озеро, Иссык- Куль. Горы круглый год в снегу. Хотя Кыргызстан имеет 4 полноценных сезона. Летом температура доходит до 36 градусов.

Наше общество работает более 12 лет. Кыргызстан не богатая страна, и пациенты здесь очень плохо себя чувствуют. Но мы не сдаемся. Как можем поддерживаем друг друга, до сих пор у нас нет квалифицированных хирургов которые могли бы делать операции. Мы иногда просим страны ближнего зарубежья о помощи в проведении операций.

Каждый год мы проводим акцию к 17 апреля. Просим центр переливания крови дать нам помещение. В Соц сетях, иногда по радио, мы говорим о всемирном дне гемофилии и просим население не оставаться равнодушными, приходить и сдавать для нас кровь! Под лозунгом «Сдавая кровь спасаю жизнь» в нашей стране крои это неотъемлемая часть лечения пациентов. В 2008 году вышел приказ о бесплатном крио для больных гемофилии. До этого года мы покупали крои.

В этом году у нас появился сайт http://hemophilia.go.kg/ мы хотим сделать электронный регистр пациентов. У нас много планов! Вместе мы справимся!

Greek Haemophilia Society elects new President

By Dimitris Verikios, President of Σύλλογος Προστασίας Ελλήνων Αιμορροφιλικών (the Greek Haemophilia Society)

Dimitris Verikios

Earlier in October, the Greek Haemophilia Society elected a new President, Mr Dimitris Verikios. In this issue of the EHC Newsletter, Mr Verikios introduces himself and his vision and priorities for the Greek Society.

I was born on 12 June 1955 and my haemophilia A was detected at the age of six, following severe bleeding for almost one month after an adenoids surgery.

I was diagnosed by the late Prof Hippocrates Tsevrenis and his assistant at the time, Dr Titika Mandalaki; both were pioneers in haemophilia treatment in Greece. Following my diagnosis, I received the proper 'plasma' treatment and within a few days I was back at home.

My case is considered mild and therefore I did not need to follow any prophylactic treatment. Meanwhile, I have experienced the full evolution of treatment from traditional plasma-derived treatments to the latest recombinant factors.

During my youth I was active with the Greek Haemophilia Society (GHS). The best moment was my participation at the 13th World Federation of Hemophilia (WFH) World Congress in 1979 in Tel Aviv, Israel. The Greek delegation to this event included a group of six young people with haemophilia (PWH) from the GHS. We participated at a youth camp hosted in a 'kibbutz.' What an experience!!! During this camp, the

idea of the World Haemophilia Youth (WHY) was created and I had the honour to be elected as Treasurer of the WHY.

After my 30s, I became fully dedicated to my professional activities and therefore I was no longer involved with the GHS.

Now that I am a retiree, I have the time to get involved again in the GHS and work for the needs of the Greek PWH, as the situation in Greece is very difficult since the country is under hard austerity measures. Among others, these austerity measures affect the state's policy on public treatment. The budgetary reductions imposed on public hospitals result in limited provisions of factor concentrates, leading PWH not to be sure what will happen in the future.

My first objective, along with the other board members, is to maintain the status of haemophilia treatment conditions, and to ensure sufficient available factor concentrates for all PWH. In view of further cuts in social policy due to the current austerity measures in the country, our second objective is to maintain the existing welfare benefits for PWH at present levels. We know that it will be very difficult as these measures have been imposed by the European Union, the European Central Bank and the International Monetary Fund, the so-called 'Troika' but we will fight for them. Finally, we try very hard to have more members involved with the GHS through social events, which we are planning to organise.

It is going to be a very interesting experience for me to meet and exchange ideas and thoughts with PWH from all over Europe and the rest of the world.

The reality for haemophilia patients in Cyprus and the role of the Pancyprian Organization of Haemophilia (POH)

By Lefteris Constantinou, Chairman of the Παγκύπρια Οργάνωση Αιμορροφιλικών (the Pancyprian Organization of Haemophilia)

Lefteris Constantinou was recently elected President of the Pancyprian NMO

The history of haemophilia in Cyprus officially started in the late 1950's since no data on haemophilia patients exist from an earlier time. During the 1950's and 1960's the first patients were diagnosed as people with haemophilia (PWH) and treated mainly with blood transfusion and immobilization. Deficient diagnosis and improper medical care at the time led many patients to suffer severe disabilities and various health complications such as transmission of HIV and Hepatitis C, which changed their lives dramatically. Some patients were even forced to migrate to developed countries like the United Kingdom and the United States of America during the 1970's in order to seek better medical treatment.

In 1978 the Pancyprian Organization of Haemophilia (POH) was established by a group of people who were directly and indirectly involved with haemophilia. Among them was our honorary member Mr. Andreas Maratheftis who led the organisation as its Chairman. The purpose of the organisation was to promote the interests of haemophilia patients in Cyprus and provide support to their families in all aspects. One of the burning issues at the time the organisation was established, was the rehabilitation of all patients who suffered from various disabilities acquired from lack of adequate treatment. POH became a member of the World Federation of Hemophilia (WFH) in 1981 and a member of the European Haemophilia Consortium (EHC) in 1992.

Today it is estimated that circa 180 PWH live in Cyprus, most of them diagnosed with haemophilia A and haemophilia B. All patients receive medical care through the Haemophilia and Thrombophilia Unit of the General Hospital of Nicosia, which is under the supervision, guidance and support of Dr Marios Antoniades. Quality of treatment is of a good standard, based on European recommendations for haemophilia treatment.

POH has recently gone through a major restructuring as a result of the need to raise haemophilia awareness in Cyprus and to act as a pressure group to the Cypriot state for better medication and improved medical care for haemophilia patients. Due to pressures arising from the recent economic crisis and significant cost cutting in healthcare budgets, it is crucial for our organisation to protect our members' interests and ensure continuous supply of high quality medication and medical support by the healthcare system.

The primary objective of POH now is to help maintain the continuous improvement of medical care to the haemophilia patients of Cyprus. To do so, emphasis is given on the involvement of all relevant stakeholders. As such, daily activities involve organisational improvement, updating our databases and strengthening our relationships with other organisations that share similar interests and goals. Future events under consideration include: fundraising events, annual meetings and family gatherings of haemophilia patients mainly for networking and bonding.

Our long-term vision, as the Pancyprian Organization of Haemophilia, is to help establish a high-standard haemophilia centre, similar to what is typically found in most developed countries, which will be supported by specialised expertise from various disciplines providing comprehensive medical support to all haemophilia patients.

Conference for the future of Hungarian haemophilia care

By Gabor Varga, President of Magyar Hemofília Egyesület (the Hungarian Haemophilia Society)

Gabor Varga *during the conference of the Hungarian Haemophilia Society*

In September, the Hungarian Haemophilia Society (HHS) organised, with all concerned stakeholders, a conference to discuss the direction of haemophilia care in Hungary. The impact of the event was further increased by international guest speaker Prof Dr Paul Giangrande, former Vice President (Medical) of the World Federation of Hemophilia and Director of the Oxford Haemophilia Centre, and current chairman of the European Haemophilia Consortium (EHC) Medical Advisory Group (MAG).

The conference was well organised and 70 participants attended, including patients, patients' relatives, directors of haemophilia centres, different specialists from all over the country, representatives of the pharmaceutical companies and the media. The aim of the conference was to establish how haemophilia care in Hungary can be improved. This was done through ideas and suggestions given by different stakeholders.

As President of the HHS, I had the pleasure to deliver the opening speech. Regrettably the invited and confirmed speaker of the Ministry of Health stepped back

from the previously accepted invitation and could not take part in the consultation. I commended the results reached in factor supply, but stressed the importance of the quality and safety of the factor concentrates. Bad experiences in the haemophilia community increased an understandable distrust in products derived from human blood over the last decades. I highlighted four major challenges of today's haemophilia care in Hungary: 1) the lack of an up-to-date national registry to provide hard data for planning and control and healthcare delivery, 2) the lack of comprehensive care, 3) the lack of official and legal involvement of the HHS in the decision-making processes, and 4) the lack of transparency in the tender mechanism provided by the National Health Insurance Fund (NHIF).

Prof Dr Csongor Kiss, President of the Faculty of Transfusion Medicine and Haematology of the Hungarian Health Care Professional College, presented the improvements in Hungarian haemophilia care throughout the last decades to today's decent European level of factor supply, which is insured by NHIF, and prevents severe complications of the illness. He recommended some changes in the legislation so as to rebuild the progressiveness into care for patients afflicted with haemophilia. He said he expected haemophilia treatment centres to develop hard data on factor supply. He also pointed out that virtually all young haemophilia patients are on prophylactic care to prevent painful joint damage, allowing them to live a full life.

Prof Kiss stressed the downsides of recombinant products, which in his opinion were causing unexpected adverse events in some of the patients. He also pointed out that plasma-derived products must have their share in modern care because, at the moment, there is no clear-cut professional agreement on this issue. He noted that all infant patients are supplied with recombinant factors and they usually choose to continue the treatment after the age of 18. Therefore, a further continuous rise in the levels of synthetic factors can be anticipated. Unfortunately, Prof Kiss left the conference for personal matters, leaving him unable to reflect on Prof Giangrande's speech, which in contrast emphasized the UK experts' official stance in favour of recombinants. For the same reason, the time allotted for discussion after Prof Kiss' speech also had to be limited, leaving differing views on local treatment, available care and tender procedures unvoiced.

Dr Judit Bidló, Department Leader in the Price Support Department of the NHIF, confirmed that the 50-50% share of plasma-derived products and synthetic recombinant factors will be maintained. This practice is regarded as good and appropriate even at international level and NHIF does not want to change it in favour of more expensive drugs. Her presentation was generously illustrated with NHIF data shown in different comparison diagrams, time frames and also in international frames of reference. An interesting remark is that the 600 regular factor users out of roughly 1000 haemophilia patients have an annual cost of 10 billion Hungarian forints (roughly 32 million euros). She stressed that health insurance is committed to continuously providing modern medication to guarantee a decent quality of life for patients with haemophilia. She outlined that the budget of the NHIF was cut by 30% in the last five years due to the global and national economic crisis; there was only one exception: the haemophilia budget remained unchanged and this can be considered a great result. She stressed that although the healthcare insurance provider agrees with organising complex centres, this idea requires political decisions, which are out of their scope. In response to the recent and recurring suggestions of the HHS on cost-reduction, including on lengthening the time-frame of tenders from one year to two or three, she stated that the state budget legislation does not allow the NHIF to do so.

Prof Dr Paul Giangrande gave an excellent overview of current treatment protocols based on evidence in literature, speaking also as a co-author of some of the articles cited. As a head of a comprehensive care centre, he stressed the importance of complex centres that can manage the patients' route to a specialist with appropriate know-how and that are able to deal with specific problems in haemophilia. In many cases, an average general practitioner (GP), specialist or dentist with no special practice in haemophilia care does not want to deal with patients with haemophilia. Prof Giangrande emphasised that this philosophy of care does not require newly-built facilities, but a better management and organisation of all needed specialists to form a smoothly operating network. We were given practical examples of the functions of such centres including European-level requirements, data collection (with some compelling graphics of extracted data) as basic evidence of planning, and quality management with peer-reviews involving patient representatives.

Prof Paul Giangrande was one of the speakers of the conference

The UK relies mainly on recombinant factors for its haemophilia care. This choice is supported by experiences from the past, including HIV-, HBV- and HCV- transmissions as well as suspicions of transmission of variant Creutzfeld Jacob disease and, in his words: “Who knows what will come next”-agents. Furthermore, treating centres in the UK have gathered evidence that disproves the common belief that recombinant factor concentrates trigger inhibitors.

Prof Giangrande also shared the factor supply tender process with the audience, demonstrating their way of gaining the most cost-effective and still professionally excellent care. He highlighted the strict rules against any bias and the importance of the patients' organisation as an equal stakeholder in the process.

Prof Dr Hajna Losonczy, professor emeritus of the Department of Internal Medicine of Pécs University of Science also accentuated the importance of arranging the previously existing regional haemophilia centres. In fact, in her opinion having a hierarchy between different treatment centres will improve the quality of care. She suggested that Hungary should have five or six comprehensive care centres, selecting the National Haemophilia Centre in Budapest and the University Centres from the 19 existing local ones. In accordance with the European protocol presented by the previous speaker, she reiterated the requirements of a comprehensive centre: a laboratory, a network of specialists providing services in orthopaedic care and dental surgery, physiotherapy, dental care, hepatology, infectiology, psychology and social support. Prof Losonczy stressed that a patient with haemophilia should be operated in a centre where haematological consultation is available. Lacking the up-to-date data from a register, she presented the data from a recent survey on patients' care. The data was gathered from less than half the existing centres.

The conference was important in giving an opportunity to share views and find common ground for the future development in Hungarian haemophilia care.

Participants at the conference

Unfortunately, there is no consensus yet on the quality of factor supply and the HHS is still supporting recombinants due to safety issues. In the meantime, the Faculty President of Hungarian Health Care Professional College and the representative of NHIF do not seem to think that increasing the proportion of recombinant is justified. Even so, the clarification of opinions, motives and considerations can be beneficial in finding common ground to move forward. Patients' representatives played an important role in keeping the budget unchanged during the economic crisis, and still continue to secure for common ground regarding products as well.

As haemophilia is a lifelong condition, improving the patients' endeavours in all aspects of their lives is paramount. The participating stakeholders agreed that an improvement of haematological care is necessary, and that organising comprehensive care centres capable of providing complex care in haemophilia play an important role. We hope that the ministry – involving the patients' organisation – will ensure the legal and budgetary basis for this professional consensus in due time.

Nordic Meeting in Copenhagen

By Maria Christensen, Communications Officer at Danmarks Bløderforening (the Danish Haemophilia Society)

In the weekend from September the 5th-7th, The Danish Haemophilia Society hosted the annual Nordic Meeting in Copenhagen. Representatives from the five Nordic countries (Finland, Norway, Sweden, Iceland and Denmark).

Youth representatives from Austria and the Netherlands, and doctors, nurses and representatives from pharmaceutical companies came together to exchange experiences about bleeding disorders and the work of the societies across the Nordic borders.

This year the Danish Haemophilia Society also invited youth representatives from the Nordic countries as well as Austria and the Netherlands in order to bring the needs and well-being of youth into focus, and to improve the engagement of youth in the haemophilia societies. On the Saturday, doctors and nurses from the two Danish haemophilia treatment centres and representatives from the pharmaceutical industry participated in the meeting as well.

Similarities across the Nordic borders

On Friday night, the representatives from the Nordic societies met in order to update each other on the challenges and successes faced by their respective societies. The similarities were quickly identified, and it

was for example highlighted that all of the Nordic societies experience difficulties recruiting and retaining youth. Therefore there is an increased focus on youth work.

Nordic standards

On Saturday morning, Dr Margareta Holmström from Karolinska Institutet in Sweden and Dr Lone Hvitfeldt Poulsen from Aarhus University hospital in Denmark were the keynote speakers.

Dr Holmström talked about the European standards for treating people with haemophilia and the requirements that the haemophilia centres have to fulfil in order to be certified as either a European Comprehensive Care Centre or a Treatment Centre. Dr Holmström also talked about the upcoming Nordic standards, which will be released soon. These are compiled by the Nordic Haemophilia Council, a forum of physicians working in the field of haemophilia and other bleeding disorders.

The changing demographic among people with bleeding disorders

Dr Lone Hvitfeldt Poulsen spoke about the possibilities and challenges, which doctors, patients and haemophilia societies face as people with bleeding disorders live longer and thereby get older.

As people with bleeding disorders get older they experience the same age-related diseases as the rest of the population. Dr Hvitfeldt Poulsen stressed that this requires new thinking and new solutions. For example, it is expected that in the future doctors will have to pay extra attention to the interaction between the bleeding disorder and age-related diseases such as cancer, diabetes and cardiovascular diseases. Furthermore, they will have to realize that the need for support in the home changes with age. The increasing number of concomitant diseases demands an increasing focus on coordination between the haemophilia centres and other departments, which again highlights the importance of the haemophilia nurse playing a key role in the coordination.

New technologies in Hepatitis C treatment

Dr Jannik Helweg-Larsen from Rigshospitalet in Denmark continued the Nordic Meeting by speaking about the new technologies in Hepatitis C treatment. He stressed that new and better medications have been appearing on the market since 2011. The new products are by far more effective and have fewer side effects than the previous generation of treatment. Unfortunately, prices for this novel treatment are very high and this means that not everyone will have access to the new treatment.

In Denmark 70 people with evolving Hepatitis C have been treated with the new medications in 2014. The price for these patients alone was estimated at 50 million Danish kroner. It is estimated that circa 21,000 Danes are infected with Hepatitis C.

The Danish Quality of Life Survey

Theis Bacher from the Danish Haemophilia Society closed the session on Saturday afternoon by presenting the results from the Danish Quality of Life Survey among people with bleeding disorders from 2012.

The survey shows that despite the fact that people with bleeding disorders are generally living longer, they still face the same challenges as previous generations. For example, they face mobility limitations, even though these are delayed compared to previous generations.

The Youth Summit

While the participants at the Nordic Meeting listened to the presentations on quality of life and the new technologies in Hepatitis C treatment, 17 youth representatives met to talk about the possibilities and challenges in life with a bleeding disorder. They also discussed how to engage young people in the haemophilia societies' activities.

Niels Ulrik Sørensen from The Danish Centre for Youth Research, CeFu, gave an introduction on youth, identity and well-being. He then facilitated a workshop where young people discussed their needs and wishes. The Danish Haemophilia Society will prepare a document with the conclusions, which will be distributed later to the participating haemophilia societies.

Sunday morning the young representatives continued the debate on how to engage youth in the societies, and there was great interest in meeting up again.

Participants at the Nordic Meeting

Cooperation in the North

Sunday morning the participants in the Nordic Meeting updated each other on the organisation of treatment in their respective countries. Bleeding disorders are rare diseases and as a result the doctors and nurses with expert knowledge on bleeding disorders are also rare. Therefore it is important to bring the organisation of treatment into focus.

Overall, the Nordic countries are faced with decreased resources for haemophilia centres and this has an effect on, for example, the nurses' roster as they need to do additional work in the same amount of time. Furthermore, haemophilia treatment centres are faced with generational change amongst their personnel. This is a development, which haemophilia societies are watching carefully.

With a strengthened sense of community the participants set out to their respective countries to continue their work in the haemophilia societies before meeting up again in Norway in 2015.

More information about the Nordic Haemophilia Council can be found here: www.nordhemophilia.org

You can read the full results of the Danish Quality of Life Survey, online here: www.bloderforeningen.dk/QoL2012

One year on barricades for improvement of haemophilia care in Romania

By Daniel Andrei and Margit Serban***

In solidarity with the community of haemophilia patients from Romania, the European Haemophilia Consortium (EHC) successfully organised, for the second time, its annual conference in our country (in October 2013). The meeting was hosted by the Romanian Haemophilia Association (RHA) and representatives from 35 countries met in Bucharest, sharing with us their experience and their models of success for ensuring equal access to adequate treatment of haemophilia, better quality of life and life expectancy. This gesture was justified by the precarious situation of haemophilia care in Romania, where the consumption of factor VIII is less than 1 International Unit per capita. This includes a lack of prophylaxis, poor on-demand treatment and the absence of home-treatment, and the limited access to the treatment of inhibitors. All of these factors have a disastrous consequence on health and quality of life as well as devastating socio-economic impact.

On this occasion, the RHA, member of the World Federation of Hemophilia (WFH) since 1994, under the guidance of its president Daniel Andrei, supported by the EHC through its president Brian O'Mahony and the chair of the EHC Medical Advisory Group, Prof Paul Giangrande, succeeded in establishing a concrete and direct relation with key government officials: then Minister of Health, Mr Eugen Nicolaescu and then President of the National Health Insurance House, Dr Cristian Busoi. In fact, at the conference the Minister of Health, the EHC and the RHA signed a partnership in which it was specified, among others, that 'Haemophilia – is a priority for public health in Romania.' Therefore, at the end of November 2013 a National Working Group was established, which has so far met three times. The aim of this working group is to include haemophilia on the priorities list of health care in Romania. It proved to be an opportunity to form a single central formal body consisting of relevant clinicians, representatives of the national patient organisation, of the health ministry and the paying authority, responsible for concrete decision-making.

Press conference during 2014 World Haemophilia Day, when an 80% budget increase for haemophilia care was announced

The initiative 'Equal access to treatment for patients in Europe - issues in haemophilia and hepatitis' was hosted in February 20, 2014 by the Romanian Member of European Parliament (MEP) Mr Petru Luhan, co-organised at the European Parliament in Brussels by the European Development Platform and Alliance of Chronic Patients. The event warned about the dramatic situation of Romanian people with haemophilia (PWH).

Following these favourable events, the Health Committees of the Parliament and of the Romanian Senate discussed with the Haemophilia Working Group the proposed national health program with distinct chapters for 'on demand' therapy, prophylaxis, treatment of haemophilia patients who developed inhibitors, orthopaedic and non-orthopaedic surgery. The proposal also included increased financial support for the haemophilia chapters.

In these conditions, following sustained efforts, an increase of 80% in the Haemophilia National Health Program Budget for 2014 was achieved. This was announced by the current Minister of Health, Mr Nicolae Baniciouiu, during a press conference held on the occasion of this year's World Haemophilia Day.

As a result, for the first time in Romania, patients below 18 years of age with severe haemophilia are offered prophylactic treatment, which increases quality of life and the hope of a better future for these patients.

It has also to be mentioned that the first European Haemophilia Treatment Centre in Romania was accredited by the EUHANET.

Unfortunately, we are far away from the standards and from achieving the European Haemophilia Care recommendations as outlined by the European Directorate for the Quality of Medicines and Healthcare (EDQM) following the Kreuth III initiative.

The discussions and outcome of the meeting on 'HTA in the assistance of haemophilia' that was held in Copenhagen this year, offered us an important tool for the argumentation of our defence. With the further support of the EHC and the WFH, the RHA, which represents the voice of the entire country, will continue to work with utmost diligence in the future.

**Daniel Andrei is the President of Asociația Română de Hemofilie (the Romanian Haemophilia Association)*

***Prof. Dr. Margit Serban is the Honorary President of Asociația Română de Hemofilie and a member of Romanian Academy of Medical Science*

International children's summer camp "Barsukas" Lithuania celebrates its 5th anniversary

By Egidijus Sliauzys, President of Lietuvos Hemofiljos Associja (the Lithuanian Haemophilia Association)

Children at the Lithuanian Summer Camp. Egidijus Sliauzys is at the centre bottom row

In the past five years, the Lithuanian Haemophilia Society (LHS) achieved many improvements in the physical and mental healthcare of people with haemophilia (PWH). LHS organised many international meetings, conferences and camps for adults and children and invited patients together with specialists who work in haemophilia to discuss healthcare and treatment for PWH.

This year is the fifth anniversary for the children's camp that is called 'Barsukas.' This camp is organised every summer for six or seven days by the LHS. We invite boys with haemophilia from seven to 16 years of age and also their sisters and brothers. Sometimes this camp becomes international because organisers invite children from abroad. In 2010, 2011 and 2013 there were children from Latvia and Estonia. The number of participants every summer is usually 24 children and four supervisors including a nurse, a physiotherapist and three members from the LHS.

Every year the camp takes place in the same location: the Open Air Museum of Lithuania (http://www.llbm.lt/eng). It is a unique and the largest open-air ethnographic museum in Europe. Children really enjoy it.

The purpose of the camp is, amongst others, to learn how to be more self-sufficient, to try to live without parents, to learn about responsibilities. We typically invite a haematologist and often Dr Sonata Saulyte Trakymiene accepts our invitation and joins us at the camp. The haematologist is there to teach the participants about haemophilia and prophylaxis. Our physiotherapist gives advice on what exercises to do in different situations and every morning we start with exercising. The role of the nurse is to teach how to self-infuse factor. She reminds the boys how to do it correctly and encourages them to be brave. The members from the LHS give children general advice on how to live with haemophilia and explain in other words that factor is important for normal, healthy and active lives.

Children learning to self-infuse during the camp

Of course children also take part in lots of entertaining and fun activities during the camp. This summer they learned to recognize non-poisonous plants and berries in the woods. Children learned to bake gingerbread cookies, work wood, do pottery and ride horses. Moreover, every year, children become familiar with living history: they learn how medieval knights lived and fought. Together with the soldiers of the 'Napoleon reconstruction club' they learned to march and shoot from ancient muskets. A few years ago, the children taking part in the camp tried to live like in 1930, where they re-enacted the life of that time. They each chose a role to play like a school boy, farmer or shopkeeper and then they invited tourists of the open-air museum to see how people lived at that time. Naturally, whenever the boys get some free time their preferred activity is to play football.

Children learning to ride horses during the camp

Children showing the results of their wood work

The LHS would like to invite you to come and join us at our summer camp.

Children watching a medieval re-enactment

Children re-enacting life in the 1930s

Recent Publications of the Polish Haemophilia Society

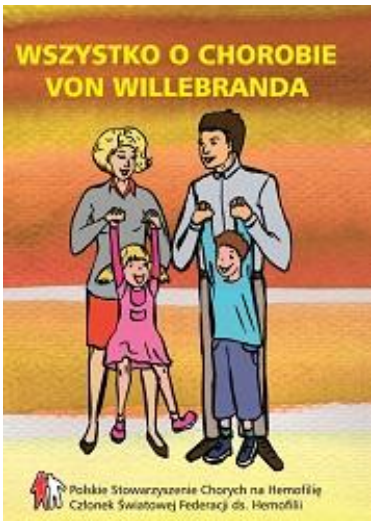
By Bogdan Gajewski, President of Polskie Stowarzyszenie Chorych na Hemofilię (the Polish Haemophilia Society)

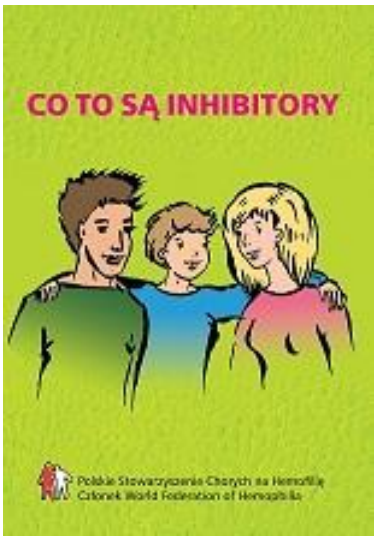
The Polish Haemophilia Society has been publishing materials for people with haemophilia and their families as well as for doctors and other medical staff for almost 16 years. Besides our quarterly newsletter, which not only informs about the Society's and its local chapters' activities but also aims at reporting new developments in the field of haemophilia care in the world, we also publish booklets (or even books) and DVDs. Almost all books and DVD publications are translations from English, with the necessary permission from the relevant original publishers (mostly haemophilia societies from Canada, Australia, Ireland, etc.). The translations and the editing are done by experienced volunteers. The printing is done by the father of a youth with haemophilia and the funds for printing are obtained from tax donations. In Poland tax payers donate one per cent of their income tax to a registered charity, so in fact it is at no cost to the tax payer.

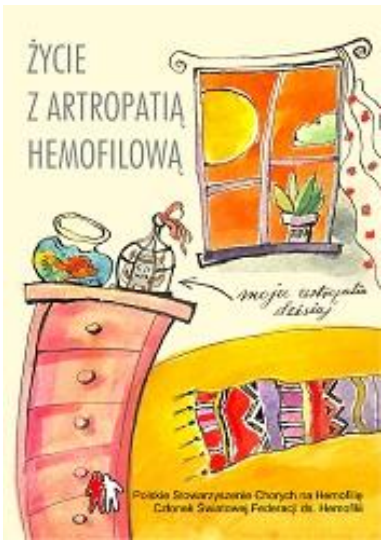
One of our recent publications is a translation of 'Aging & Haemophilia,' a booklet compiled by the Irish Haemophilia Society. Other titles published this year are translations of: 'Challenges, Choices, Decisions: A Guide on Orthopedic Surgery for People with Haemophilia' (by the Canadian Hemophilia Society), and 'Exercises for People with Haemophilia' (by the World Federation of Hemophilia - WFH). Forthcoming

books include the WFH ‘Guidelines for the Management of Haemophilia.’ This is a book that is meant first and foremost for doctors.

Our publications are available not only to people with haemophilia but also to the public; we send them to important libraries all over Poland, including those of medical universities, to hospitals and clinics, and to some individual doctors. For the Guidelines, our distribution list consists of 60 pages of various contacts.








Hemofilia
w wieku dojrzałym


CZYM JEST CHOROBA
VON WILLEBRANDA?


CZYM
JEST
HEMOFILIA?


JAK DBAĆ O NACZYNIA
KRWIONOŚNE?
MIEJSCA ZASTRZYKÓW
DOŻYLNYCH


Filmy o leczeniu
chorych na hemofilię
Polskie Stowarzyszenie Chorych na Hemofilię


USŁYSZCZIE NAS
FILM O KOBIETACH,
KTÓRE SĄ NOSICIELKAMI HEMOFILII
płyta bezpłatna
Polskie Stowarzyszenie Chorych ra Hemofilię

Feature Articles

Report on the conference: 'HTA 2.0 – teaming up for value'

By Declan Noone and Laura Savini***

Declan Noone is the chair of the EHC Data and Economics Committee

The European Network for Health Technology Assessments (EUnetHTA) organised earlier in October the conference: 'HTA 2.0: Teaming up for value.' The conference aimed at bringing all relevant stakeholders involved in health technology appraisal and reimbursement up to date with the achievements on health technology assessment (HTA) collaboration; what is the level of interaction between payers, HTA agencies, researchers and regulators; and what are the current challenges and opportunities for further collaboration. Declan Noone, chair of the European Haemophilia Consortium (EHC) Data and Economics Committee, attended the meeting as the EHC's representative.

The event was opened by Dr Andrzej Rys, head of the Directorate for Health Products and Services within the Directorate General for Healthcare and Consumers at the European Commission. Dr Rys stated that: "We are in a new phase of EU cooperation on HTA, which aims to assist in faster access for patients' to medicines, reduce duplication and promote better use of resources, improve and rationalise collection and requests of clinical evidence, and also improve the predictability in which medicines are reimbursed." He also stressed that all stakeholders need to be involved at both scientific and strategic levels and it is hoped that this will lead to a 'life cycle' approach to new health technologies and reduce the current fragmentation. In his opinion, this will ultimately improve the sustainability of the European healthcare systems.

The introduction was followed by Mr Finn Børlum Kristensen, Director of the EUnetHTA Secretariat, who outlined the timeline of development of the EUnetHTA and noted that EUnetHTA is now coming to the end of its second joint action. A third joint action will be starting after 2016, and with the deadline approaching there is an increased need to find a common position on how the programme will be continued in a sustainable manner. He pointed out that the current HTA landscape is still very complex due to so many national and regional entities carrying out their own assessments. However he hopes that EUnetHTA will be able to globalise the evidence so that local decisions can be taken. The EUnetHTA hopes to go one step further and locate what decision is being made at national level and with this information create a global evidence model that can be used as a base for local assessment.

Dr Guido Rasi, head of the European Medicines Agency (EMA), further added complexity to the discussions. In fact, for the marketing authorisation of pharmaceuticals, the EMA has standard procedures, which require one application, one assessment and it then provides one decision valid in 31 countries (28 Member States plus the European Economic Area (EEA) countries Iceland, Liechtenstein and Norway). However, with regard to HTA, there are over 30 different methodologies and over 30 different independent decisions on whether a particular technology should be reimbursed and if so, for how much. He then pointed out that in general it is neither the biology, pharmacology or the science that change across Europe but that the evaluation of a medicine depends on the healthcare system, the economics and politics, which vary greatly depending on the European country or region. He concluded by specifying that in the past, relative efficacy and relative effectiveness were both considered in series, now they need to be considered in parallel and adopt a life-cycle approach. He stressed that there is increased need for co-operation between regulators, HTA agencies and payers.

The first panel session was on: 'European cooperation on HTA – how does it make a difference at national/regional level.' Mrs Luciana Ballini from the Emilia Romagna region gave an overview of European co-operation and its impact at national and regional level. It was noted that the EUnetHTA had been used by HTA bodies from resource-limited countries to gain access to knowledge. Thanks to this mechanism these countries with less resources are able to develop tools and methodologies and eventually contribute to the joint assessment report. She also discussed the importance of the timeliness of the information. For example, in 2013, over 1,200 HTA projects were carried out in Europe, 16% (or approximately 200 HTAs) were overlapping so that individual agencies collaborated to reduce the strain on resources. Carrying out an HTA is a time and resource intensive exercise, hence the need for greater cooperation. Mrs Ballini also specified that the quality of the final product needs to be robust and trustworthy for other agencies to use it. She concluded by saying that this process had also allowed agencies to share further scientific information hence making assessments more robust.

The main outcomes of the first panel discussions were that the development of the EUnetHTA database and sharing mechanisms has allowed better information for national and regional agencies in carrying out the assessments quicker. This was even more apparent in regional agencies where resources are limited and the number of new technologies per year is beyond their ability to carry out full assessments on their own. The discussions followed on the problem of adding layers to the assessments, which may result in additional waiting time for patients to access the novel technologies. The panel clarified that only critical medicines should require a full assessment. Agencies also need to engage with companies in parallel much earlier so that assessment and licensing can occur at a similar time, hence providing all patients with a similar timing for access to new technologies. The second point made was the involvement of patients in the appraisals. There was a strong recognition that patient involvement was necessary moving forward. The final comment came from industry, who noted that the length of time required for new technologies to reach the market is an issue, in particular for Small and Medium size Enterprises (SMEs). Participants noted that this aspect does reflect in the final cost of medicines. In fact, there is an increased tendency for SMEs to develop the idea of a new product, which is then taken over by larger companies. This means that the costs for mergers and acquisitions of these smaller companies end up being absorbed in the final cost of the product so that consumers end up paying not just for the research, but also for these additional legal costs.

The second panel discussion was on: 'Getting an effective technology from the lab to the patient in Europe – challenges and opportunities.'

The panel discussion focused on the need for a common methodology and minimum standards for the approval of a new technology. The panel noted that one of the main shortcomings of HTA agencies is the lack of resources that allow them to process more assessments and to be able to answer questions from payers. In the opinion of several panellists, this is where the EUnetHTA can support the work done by HTA agencies in Europe. Panellists then approached the question of standards of care and how these vary not only from country to country, but also from region to region. Panellists also stressed the importance and need for early dialogue. Also, it was noted that agencies need to come to terms with observational data and use these in their models. In fact, the reliance on randomised controlled clinical trials (RCT) was defined as being a 'pipe dream' and the opportunity cost for patients, if observational studies are not included, is too big. Panellists also stressed the importance for a more heterogeneous and timely access to technologies across Europe as patients' needs remain the same irrespectively of the country they live in. Current disparities are unequitable and patients are increasingly aware of this. In terms of timeliness, panellists agreed that medicines should be reimbursed shortly after they are licensed. This underscored the point that joint assessment can be carried out, however agencies may still lack funds to purchase the new technologies. Panellists also agreed that treatments targeting rare diseases should not have a special procedure as rare disease patients can expect the same level of cost-effectiveness in a product than any other patient.

Patients' representatives in the audience pointed out that they were also willing to share their data in order to further research, however this data should not be used against them by, for example, cancelling insurance plans or raising premiums or supporting discrimination in the work environment.

The second day started with a plenary session on: 'Innovative tools for HTA – today and tomorrow' chaired by Dr Alric Ruether from the German Institute for Quality and Efficiency in Health Care (IQWIG). The session had presentations on various HTA-related projects including AdHopHTA, which covers HTAs for hospitals and PARENT, a registry of registries. Both presentations stressed the need for cooperation and harmonisation in the use of data. It was announced that guidelines on how to use data collected in registries for HTA will be released in May 2015. A third presentation was on multiple criteria decision analysis (MCDA) and its use. It was noted that patient experience and outcome was one of 20 criteria that are used. Presentations also included the Model for Assessment of Telemedicine (MAST) Project, which evaluates HTA in e-health. In this type of HTA, patients' perspective and experience is taken into consideration and added to the model because it impacts the effectiveness of the technology. Finally, the outputs of the joint work in EUnetHTA were presented. It appears that almost 90% of the agencies said the EUnetHTA is useful yet only 29% of them actually use the system.

The plenary session was followed by a round table on: 'HTA in Europe – from strategy to action: what's next?' This session focused on access and the need to work on reimbursement. The topics covered in this last session were quite diverse. A payer spoke on the fact that HTA does not take into account other non-clinical relevant facts. Furthermore, it does not take into account sub-populations needs.

A point was made regarding the EMA's adaptive licensing, which would bring faster access to novel treatment with relatively little data. Although, this system may work on the premise that patients and regulators are willing to take additional risk for conditions with no therapeutic options, the question still remains on whether payers will be willing to reimburse technologies with little clinical evidence. There needs to be fine-tuning to see how the reimbursement under adaptive licensing will be organised.

There was also a discussion about innovation and what true innovation is, with comments noting that often innovation is limited to the packaging or something irrelevant to the consumers.

Industry representatives and in particular those from SMEs noted that providing information for HTA assessment is a very resource-intensive activity that cannot be properly completed by smaller companies. This is why a centralised assessment would be welcome.

There were also talks on how to assess ultra-orphans, for which each country seems to have its own system. There have been discussions now for the past 15 years on how to assess these products but limited solutions have been found. There is a need to look at the entire life-cycle of these drugs and for payers to be involved in the very early stages of the drug development. Ideally, payers should also have access to independent research on a product with good comparators. The sole reliance on pharmaceutical data can make payers uncomfortable.

Another comment noted that HTA does not tackle transparency in pricing. To this day we note that larger and wealthier countries can better negotiate prices compared to countries with limited populations. This can have dire consequences, in particular when a system of pricing basket is used based on 12 specific countries.

The final comment of the session was that HTA was designed to assess the cost-effectiveness within a healthcare system. It is now being focused on, and keeps coming back to drugs or devices, which overall inhabit a relatively small place of the healthcare system. Going forward HTA needs to broaden its focus to the other 70-85% of the system and assess how we deliver a service to make room for advances in how we treat patients.

In conclusion, the event was very interesting and particularly well attended by patients' representatives, which is important due to the nature of the issues discussed.

*Declan Noone is the chair of the EHC Data and Economics Committee

**Laura Savini is the EHC Communication and Public Policy Officer

Ensuring efficiency in haemophilia factor concentrates: "First, do no harm"

Prof Albert Farrugia

By Albert Farrugia, Adjunct Professor at the University of Western Australia

Few of us in the field of haemophilia care could have confidently predicted the current therapeutic landscape ten years ago. Although recombinant products as the treatment of choice had become the major modality in the wealthy countries, they were very expensive and generally limited to young, previously untreated patients. Only three manufacturers supplied recombinant factor VIII, and only one supplied recombinant factor IX, giving them considerable market power. While research had indicated in 2004 that the treatment requirements then current were optimally met with a Factor VIII level of 6 IU/capita, only two countries supplied this amount (*Haemophilia* 2004; 10:18-26). The contrast ten years later is clear and one key feature, which is enhancing access, is the increase in the number of products, leading to greater competition and, as we all know, lower prices and affordability. This is allowing the enhancement of prophylaxis regimens and other therapeutic interventions. But some dark clouds occupy the horizon.

The approval of all haemophilia products is subject to scrutiny by regulatory authorities, and some doubts have been expressed about the appropriateness of their requirements, particularly in Europe, (*Haemophilia* 2014; 20:455-58; *Nat Med* 2014; 20:117) with which I concur. One aspect, however, appears to be attracting less attention. While much has been made on the issues of patient numbers in trials and on the need or otherwise of paediatric patients, the actual structure of the Randomized Clinical Trials (RCTs) used to approve the most recent products has been overlooked. This is what I wish to discuss.

A clinical trial is essentially an experiment done on human subjects in order to assess the effectiveness of a therapeutic intervention. Many factors can affect the validity of a clinical trial and over the past forty years a consensus has been established that, in order to prevent any such factors, clinical trials have to be randomized and controlled. Such randomized, controlled trials (RCTs) involve the random allocation of patients to one group ('arm') of the study, which administers the treatment under investigation, and another arm in which patients are administered a control, which generally involves an agent with similar appearance and administration but which does not include the drug in question.

Obviously, no one would propose that we demonstrate the overall efficiency of haemophilia products through an RCT, which would randomize patients to treatment and, as a control, to no treatment. The analogy of attempting to demonstrate the effectiveness of parachutes when jumping out of a plane has been used to highlight the absurdity of such a proposal (*J Thromb Haemost* 2006; 6:1226-7). However, the demand for RCTs to demonstrate the efficacy of prophylaxis compared to on-demand therapy, continued well into the era when prophylaxis was known to be superior in preserving patient health. Mostly as a result of the continuing pressure from the Cochrane Collaboration, trials have been carried out over the past ten years which have settled the issue (*N Eng J Med* 2007; 397:535-544; *J Thromb Haemost* 2011; 9:700-710). It is a sobering fact that this has been achieved at the expense of permanent and progressive joint damage in the patients 'randomized' into the 'control' or on-demand part of these trials.

And that, you would have thought, would have settled the question. The only issue should be the cost-effectiveness of prophylaxis versus on-demand, which has also been settled (*Haemophilia* 2013; 19:e228-38). However, a disturbing tendency is being established, primarily through a convergence of regulatory and industry perspectives, in the approval process for new haemophilia treatments. Scrutinising the literature and the other publicly available material on these processes, it emerges that for approving a claim for prophylaxis for specific products, an RCT between prophylaxis and on-demand treatments is being demanded and performed. As I survey these trials, I am struck by several features.

First of all, all the trials continue to demonstrate, as has been the case for decades, the superiority of prophylaxis. Plucking out one trial as an example, the US Food and Drug Administration's (FDA) reviewer declared that: "All 14 of the subjects in the on-demand cohort of the full analysis set (FAS) had bleeds. The mean annualised bleeding rate (ABR) was 33.87 (± 17.37). These results show that the mean annualised bleeding rates (ABRs) were lower in subjects who received prophylactic treatment than in subjects who received on-demand treatment."[1]

This is starkly shown on Fig 1. Behind these bland words and this graph is a group of patients who, in order to demonstrate, once again, the superiority of prophylaxis, had to undergo the pain and suffering of a bleeding rate ten times that which would have otherwise occurred.

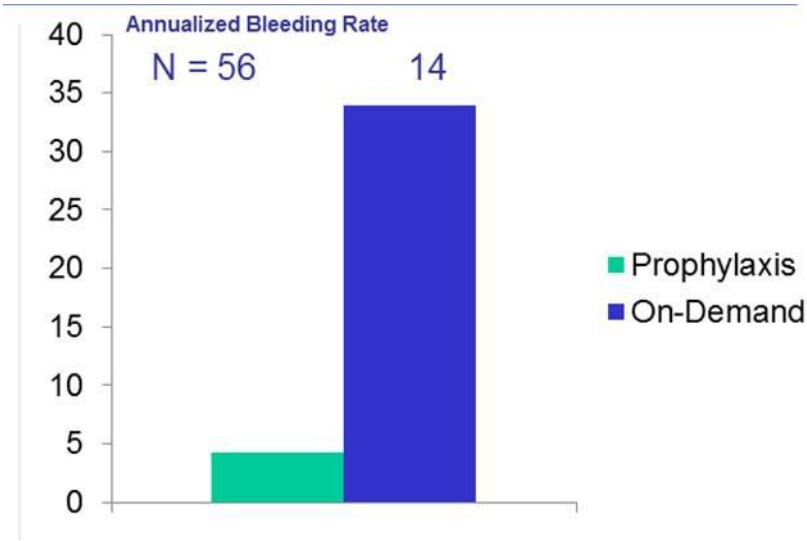


Figure 1: Annualised Bleeding Rate for patients on prophylaxis versus on-demand treatment for a recently approved recombinant FIX concentrate, as estimated by the FDA

Is this justified? This question needs to be addressed, firstly, through considering one of the first requirements for RCTs, which is that of Equipoise. This concept was developed by Dr Benjamin Freedman, a leading authority on evidence based medicine and clinical trials. Freedman proposed that for an RCT to be conducted, there has to be uncertainty within the expert medical community about the preferred treatment. He emphasized that this uncertainty was not necessarily on the part of the individual investigator, but was an 'honest, professional disagreement' among experts in the field about the relative merits of the competing interventions. Sadly, Freedman died at quite a young age (Fig. 2) but his ideas are accepted as the bedrock of RCTs (*N Eng J Med* 1987; 317:141-5). Can we truly state that there is a genuine division of opinion among haemophilia treaters about the relative merits of prophylaxis and on-demand treatment?

Figure 2: Dr Benjamin Freedman (1951-1997), one of the founders of modern Evidence Based Medicine

Regulators and industry argue that this may well be the case, but that each product is different and needs to be tested clinically; biosimilarity is not allowed for haemophilia products. Irrespective of the merits of this argument, there are other ways to demonstrate the enhanced efficiency of prophylaxis, which do not require a patient to be subjected to the inevitable increased bleeds resulting from on-demand treatment. For example, two prophylaxis regimens can be compared, one with an established (approved) product and one with the product under question. This will yield the same answer without harming patients. I am, however, apprehensive that the convergent interests of regulators

[1] http://www.fda.gov/downloads/BiologicsBloodVaccines/BloodBloodProducts/ApprovedProducts/LicensedProductsBLAs/FractionatedPlasmaProducts/UCM415248.pdf

reluctant to move out of their comfort zone and companies keen on getting a specific 'prophylaxis' label claim for competitive purpose will dissuade these players from such an approach.

I wish to raise the issue of ethics and, in particular, adherence to the Declaration of Helsinki on Ethical Principles for Medical Research involving Human Subjects[1]. This international standard for ethical conduct of clinical trials was shamefully abandoned by the FDA in 2008. The Declaration makes it clear that placebos/controls, which in our instance are presented by on-demand therapy, are only justified when there are compelling medical reasons and when the condition being investigated is a minor one. Are there compelling medical reasons for administering on-demand treatment instead of prophylaxis? Is haemophilia a minor condition?

One final point regarding ethics. Again, reading all the papers, I am stuck by the fact that all the trials done comparing prophylaxis to on-demand treatment are being reposted from poor countries. Clearly, such trials would not be possible in the established haemophilia therapeutic landscapes because, quite frankly, they would not be allowed. I am troubled that patients in poor countries, where treatment is scarce, may be subjected to harmful processes, which patients in rich countries are protected from. I recognise that the initiation of even on-demand treatment in these countries is an advance on what is available, and I salute the clinicians who are trying to treat people with haemophilia under very difficult circumstances. But I take recourse again to the Declaration of Helsinki, which states:

- Paragraph 30: At the conclusion of the study, every patient entered into the study should be assured of access to the best proven prophylactic, diagnostic and therapeutic methods identified by the study.
- "Clarification": It is necessary during the study planning process to identify post-trial access by study participants to prophylactic, diagnostic and therapeutic procedures identified as beneficial in the study or access to other appropriate care. Post-trial access arrangements or other care must be described in the study protocol so the ethical review committee may consider such arrangements during its review.

In other words, once these patients are started on the trials, their treatment must continue. Is this assured? And what is happening to the patients 'unfortunate' enough to not be on a trial?

I conclude with the Hippocratic Oath:

"I will prescribe regimens for the good of my patients according to my ability and my judgment and never do harm to anyone."

Most of the individuals involved in the activities described in this article have subscribed to this Oath. I look forward to their continued implementation.

The opinions expressed in this article are my own and not necessarily those of any of my past and current affiliations, although I could hope that they are.

[1] www.wma.net/en/30publications/10policies/b3/17c.pdf

ELPA 'HCV and Your Body' International Training Day

By Gerard O'Reilly, board member of the Irish Haemophilia Society

Gerard O'Reilly is the Treasurer of the Irish Haemophilia Society

This one day event in November was organised by the European Liver Patients Association (ELPA) and sponsored by Gilead Sciences Europe Ltd. The purpose of the day was to enhance dialogue between the people living with Hepatitis C (HCV), healthcare professionals, patient advocates and support groups. To do this, the day was a combination of plenary and workshop sessions. The plenary sessions gave an overview of the impacts associated with being diagnosed with HCV, stages of progression and treatments. Plenary presentations were delivered under three headings: medical, social and emotional impacts of HCV. They were followed by case-study-focused workshops where the attending participants interacted with each other on coping strategies for the medical, coping strategies for the emotional and coping strategies for the social impacts of HCV.

Attending participants included many non-government organisations, patient advocates and HCV Trusts from countries across Europe representing people who have been diagnosed with HCV and are members of the ELPA, which was founded in 2004. Under the ELPA, it is envisaged that liver patients' groups can share their experiences and the many different approaches adopted in different countries. It engages in lobbying with its members on many levels involving EU and national policy makers, liver specialist associations and public health experts.

The invited guest presenters were Mr Ashley Brown, Consultant Hepatologist from the UK who presented the plenary session on the medical/clinical impacts of HCV. The emotional impacts of HCV plenary session was presented by Mr Marko Korenjak, a person with haemophilia who had completed a year of HCV treatment. Mr Tato Marinho is a Gastroenterologist and Hepatologist from Lisbon in Portugal and he presented the final plenary session on the social impacts of HCV.

From the workshops and the case studies involved I gained a better understanding of the workings of other HCV patient groups in comparison to the haemophilia HCV community that I am a part of. The sessions showed that the impacts of HCV, the engagement with patients by the patient groups, healthcare professionals and the access to treatments differs greatly across Europe. To sum up the day, the ELPA hope to keep this process ongoing as it is a valuable way to gather views from across Europe, which will enable them to formulate an integrated approach to healthcare and treatments so as to advocate with EU policy makers.

News from Corporate Partners

Advancing to a new era of personalized care

Every bleed matters

While haemophilia itself cannot yet be cured, the community is increasingly able to help reduce and prevent its most damaging effect: bleeding episodes[1]. People with haemophilia live with the constant risk of spontaneous internal bleeds into the joints, muscles and soft tissues; even with the risk of life-threatening bleeding episodes like intracranial haemorrhages. Repeated and uncontrolled bleeds into joints can lead to long-term damage – leading to pain, immobility, lost school/work days and long-term joint damage[2]. Data show that joint function can be directly impacted by the number of bleeds an individual has experienced[3]. Irreversible and long-term damage that can compromise patient outcomes and quality of life can be caused by two to five bleeds a year[3]. Despite these impacts, few people with haemophilia are receiving comprehensive care due to persistent barriers including access to treatment and adherence[1 3]. A comprehensive care approach takes into consideration the full continuum of care in order to provide maximum benefit to people with haemophilia.

The evolution of individualized care

While improvements in diagnosis and access to care have helped to advance the treatment for haemophilia, comprehensive care is driving further innovations that better address the barriers to optimal treatment. For example, some approaches are focusing on a more individualized approach to dosing and disease management. Scientific advancements are driving an improved understanding of how each haemophilia patient responds differently and how physicians can personalise treatments to fit their patients' unique needs based on their physiology and lifestyle[1].

Lessons learned from the diabetes community show that individual blood glucose monitoring and adjustments to insulin injections better enables people to manage their condition. With advances in science and technology, we are learning that similarly targeted and actively monitored treatment may also help people with haemophilia achieve improved results[1].

Pharmacokinetic guided dosing represents one example of a more personalised approach in prophylaxis treatment. This approach is based on the considerable individual variations in the absorption and elimination of clotting factors (pharmacokinetics, PK) in the body. Prophylaxis can therefore be customized on an individual basis in accordance to the drug's PK in the individual's body and more efficiently prevent bleeds.

Baxter: Envisioning a life without bleeds

Speaking at Baxter's symposium at the EHC 2014 conference in Belfast, Dr Pedro Pina, Medical Director, Baxter for the region of Europe, the Middle East and Africa (EMEA), commented on the company's vision: "Together with our partners in the community, Baxter continues to drive innovations to improve global access and quality of care such as individualized treatment approaches. People with haemophilia should have access to the quality of care necessary to help prevent bleeding, thereby potentially reducing short-term pain and long-term disability."

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[1] Ewenstein B et al. Envisioning a World without Bleeds: Making a Real Impact on the Lifetime Burden of Bleeds for People Living with Hemophilia. April 2014. Accessed October 2014: http://www.baxter.com/downloads/press_room/press_releases/factsheets/baxter_hemophilia_position_paper.pdf

[2] Frequently Asked Questions About Hemophilia. World Federation of Hemophilia. Accessed October 2014. Available at: http://www.wfh.org/en/page.aspx?pid=637

[3] Gringeri A, Ewenstein B, Reininger A. The burden of bleeding in haemophilia: is one bleed too many? *Haemophilia*. 2014:1-5.

CSL Behring Continues to Advance New Approaches to Coagulation Therapy

As the original expert in bleeding disorders, CSL Behring has a broad portfolio of coagulation products, a long history of advancing coagulation therapy, and a thorough understanding of the challenges facing the bleeding disorders community. This commitment to innovation and understanding of the community has led the company to develop and advance its recombinant factor development programs.

The AFFINITY clinical trial program is studying CSL Behring's recombinant Factor VIII single-chain (rVIII-SingleChain) to treat haemophilia A. rVIII-SingleChain uses a strong, covalent bond that connects the light and heavy chains, thereby creating a stable single chain rFVIII molecule. Studies have shown that rVIII-SingleChain demonstrates a strong affinity for von Willebrand factor (VWF), resulting in a faster and more efficient binding to VWF. The FVIII/VWF complex plays an important role in the physiological activity and clearance of FVIII and has been shown to have an influence on the presentation of FVIII to the immune system.

The PROLONG-9FP clinical trial program is studying the company's recombinant fusion protein linking coagulation factor IX with recombinant albumin (rIX-FP) to treat haemophilia B. CSL Behring selected recombinant albumin as the ideal recombinant genetic fusion partner due to its long physiological half-life and ability to preserve the native function of the coagulation factor.

CSL Behring also continues to advance its recombinant fusion protein linking coagulation factor VIIa-FP with recombinant albumin (rVIIa-FP) to control bleeding episodes in haemophilia patients who have inhibitors as well as its recombinant von Willebrand Factor (rVWD).

In addition to its clinical programs, CSL Behring continues to make capital investments that support the recombinant factor development program. In 2014, the company opened the CSL Behring Biotechnology Manufacturing Facility in Melbourne, Australia which will produce these novel recombinant therapies on a large scale for international clinical trials. The new facility is the centrepiece of CSL's US $250 million expansion at its Broadmeadows site and is one of the largest and most advanced facilities of its kind in the world. The world-class facility reflects CSL Behring's commitment to providing better treatment options for people who are managing bleeding disorders and other rare and/or serious conditions.

For more information about CSL Behring, please visit www.cslbehring.com.

MILES FOR HAEMOPHILIA

Pfizer launches Miles for Haemophilia in Barcelona and Belfast

In close collaboration with Alex Dowsett, a professional cyclist and severe haemophilia A patient, Pfizer launched the campaign Miles for Haemophilia earlier this year to show support and raise money for haemophilia patients. The campaign was officially launched in Barcelona at the fifth Haemophilia Global Summit in September, and has since continued across Europe, including at the European Haemophilia Conference in October.

What is Miles for Haemophilia?
The idea behind the Miles for Haemophilia campaign is simple: participants can run, walk, cycle or swim and, for every mile completed, Pfizer donates money to haemophilia patient associations. With Alex leading by example, Pfizer invited the medical haemophilia community, haemophilia patients and their families and friends to 'raise miles' for haemophilia.

The UK campaign ran for the month of October and accumulated 15,063 miles in total. Pfizer UK donated £6,500 to the Haemophilia Society.

From Barcelona to Belfast: Miles for Haemophilia on tour
Miles for Haemophilia was officially launched in Barcelona on 26th September during the fifth Haemophilia Global Summit. All attendees were invited to take part in a one kilometer race on static bikes, with Alex leading by example. By the end of the Summit more than 160 people had taken part, raising a total of 5,000 EUR for FedHemo, the Spanish haemophilia patient association.

The campaign was continued enthusiastically in Belfast on 3rd–5th September at the European Haemophilia Conference. Alain Weill, President of the World Federation of Haemophilia, was one of a number of willing delegates to cycle alongside Alex during the conference, with over 70km cycled over the 3 days. Participants rode the static bikes along to a video depicting the streets of Belfast, taking a tour of the city without leaving the building. They were joined by Alex, who was well received by patients keen to share their experiences with him.

Alex Dowsett: a time trial phenomenon
Alex is considered a time trial phenomenon, and his career to date includes major international time trial titles including success at the Giro D'Italia 2013, where he beat Olympic gold medalist, and Tour de France Champion, Sir Bradley Wiggins. He is also the current national UK time trial champion – a title he has held for the past three years (2011, 2012 and 2013). Earlier this year Alex recorded a major career high when taking gold in the Commonwealth Games time trial event, producing a superb performance over the 40 km course to record a dramatic victory.

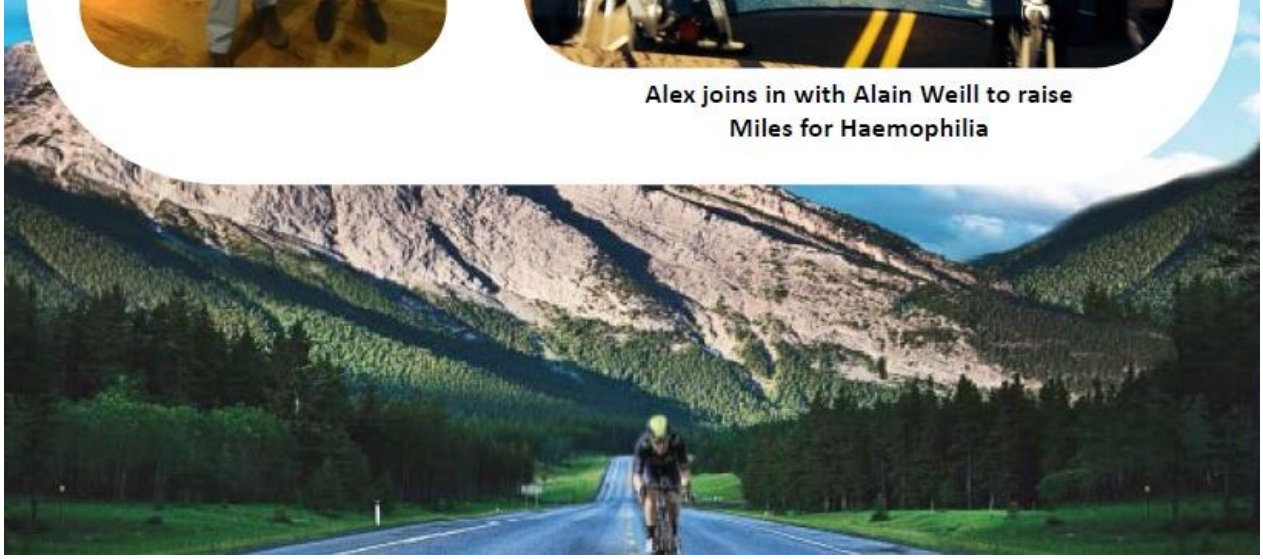
MILES FOR HAEMOPHILIA

Living with haemophilia

Despite having haemophilia, Alex has succeeded in finding the mindset necessary to pursue his goals without compromising both personal aspirations and professional ambitions. With careful treatment and self-care, Alex has been able to maintain an active lifestyle and achieve great success in his career to date.

He is open in offering his support and guidance to children with haemophilia and their families and works with many patient associations, speaking at schools and conferences. Speaking at the Summit in Barcelona, Alex said "*Everyone deserves the opportunity to do what they love. Haemophilia is a lifelong condition, but it should always be possible to realize your goals. Today, we present a campaign which symbolizes this message. I'm very proud to be part of this campaign, and I'm sure it will help people with haemophilia for years to come. That's what really counts.*"

Alex joins in with Alain Weill to raise Miles for Haemophilia



European Haemophilia Recommendations: A debate with a new generation, a report from the event

A focus on young leaders and the future leadership of the EHC

By Wills Hughes-Wilson, Senior Vice President, Chief Patient Access Officer; and Phil Wood, Vice President, Global Commercial Lead, Haemophilia, at Sobi

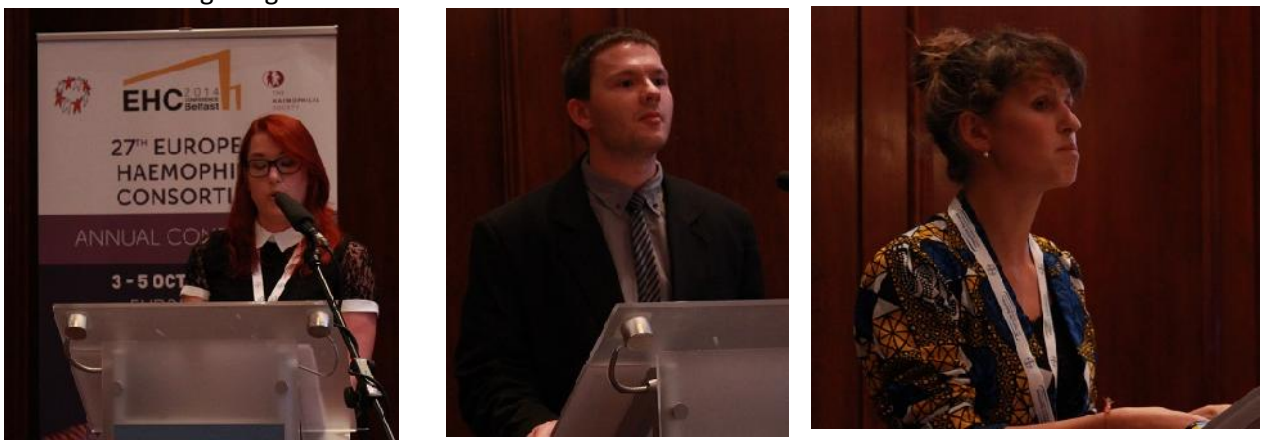
After a conversation with the European Haemophilia Consortium (EHC) leadership, Sobi decided to offer its company symposium at the Annual Conference in Belfast, 3-5 October 2014, to the EHC as a platform for a more youth-focussed activity during the Conference.

The goal was to provide an otherwise unavailable platform for haemophilia youth to gain public exposure and experience in a key area of focus for the EHC, and to work with the experienced leaders in the community, through presenting a persuasive argument on a key topic to a large group in, hopefully, an enjoyable setting. Getting younger EHC members involved in the organisation's work – including at the conference – has been identified by the EHC as a critical element for the future continued success of the community.

So, on the Friday evening at the Annual Conference, just before the official dinner, the EHC organised a debate entitled 'European Haemophilia Recommendations: a debate with a new generation.' Youth leaders from the haemophilia community were paired up with more senior leaders, to each prepare arguments for either supporting or opposing positions for a debate on three of the seven new European Recommendations for Haemophilia Treatment and Care resulting from the Kreuth III initiative.

The Kreuth recommendations were formally brought to the attention of the community by EHC on World Haemophilia Day this year. They are intended to capture the state-of-the-art approach for optimal clinical use of clotting factors; and are widely regarded as the new international reference for haemophilia treatment and care. Getting them implemented is a critical part of EHC's mission for the coming years and, for this reason, the EHC has been working hard to ensure that its members are strong advocates for their implementation.

The idea of the debate was that each younger person would pair up with a more senior leader, to defend an argument, while the other would oppose the proposition. The ability to advance an argument, talk convincingly in public and be persuasive before an audience is a critical element of being a successful advocate. This pairing-up arrangement also allowed the more experienced generation to pass on to a new generation of leaders the skills and knowledge that they have acquired over the years of representing the community. The situation was made even more challenging for the senior leaders, because they were the ones asked to argue against the recommendations.



From left to right: Sarah Gilgunn (Irish Haemophilia Society), Radoslaw Kaczmarek (EHC Steering Committee) and Naja Skouw-Rasmussen (Danish Haemophilia Society) representing the views of the new generation

This gave them the opportunity both to 'play the devil's advocate' role in the debate; and also an even bigger challenge to try to prepare and advance a persuasive case against something that is important for the community.

The symposium set-up created by the EHC was, indeed, a fun and engaging opportunity to dig into the opportunities and potential pitfalls of the implementation of the recommendations into national healthcare systems. Would governments use elements such as multi-stakeholder national haemophilia

From left to right: Prof Paul Giangrande (chair of the EHC Medical Advisory Group), Dr Gary Benson (Belfast City Hospital) and Dr Barry White (Irish National Haemophilia Centre) playing devils' advocates

committees or the setting of a minimum level of factor use to hide behind or as an excuse to cut treatment? Or are these recommendations a golden opportunity to further improve the treatment and care for people with haemophilia?

The debate, chaired by EHC President Brian O'Mahony, was lively, interesting, engaging and creative; with a broad range of references covering everyone from Descartes to the Wizard of Oz, Aristotle to Peter Pan. The entertaining nature of the approach did not detract from the seriousness and the relevance of the topics covered, however.

The phrase 'our young people are our future' is often heard and, in the case of patient advocacy groups, this is also very true. But the historical challenge for many advocacy groups can be that, when treatments become available, the interest of the younger generation to join can fall into decline. This is an ironic situation – that the existence of treatments and good care, which are often brought about thanks to the strong leadership of advocacy groups, can cause a lessening of interest by the very people that they exist to serve.

The EHC had identified that it is critical to secure that the more experienced generation pass on the knowledge and experience to the next generation of talented leaders, to secure that the community remains vigilant, so that the hard-won successes – such as access to treatment – are also secured in the future. Hopefully this event has contributed one step further to EHC's ability to continue to do this successfully and we look forward to being part of such future programmes with the whole community.

Bayer: Building the Future on a Solid Foundation of Investment in Europe

For more than 25 years, Bayer has been committed to the hemophilia community. "We're proud of our long-term involvement in this community," said Prasad Mathew, M.D., specialist in bleeding disorders and Head, Bayer Hematology Medical Affairs. "We have been listening to patients, advocates and healthcare professionals, and responding to the needs they express with a comprehensive portfolio of research activities, support programs and treatment options for people with hemophilia."

Prophylaxis: Answering the Key Questions

Part of our long-term commitment involves conducting the research to find scientific answers as to which treatment options lead to better patient outcomes. As the sponsor of the JOS (Joint Outcome Study), Bayer worked with leading physicians to demonstrate the benefits of routine prophylaxis in protecting against bleeds and joint damage in children with severe hemophilia A. Next, we undertook SPINART (Secondary Prophylaxis in Adults, a Randomized Trial), proving that prophylaxis is important for adults with hemophilia A as well.

A Pipeline of Innovative Treatments

Bayer continues to invest in research and innovation to fulfil the unmet needs of people living with hemophilia. Our pipeline in hemophilia currently in late-stage clinical trials is comprised of:

- BAY94-9027, an investigational, longer-acting recombinant factor VIII (rFVIII) that may offer prophylaxis through less frequent dosing
- BAY81-8973, an investigational rFVIII that is building upon the hemostatic control offered by Bayer's existing rFVIII product, with a new manufacturing process

At the same time, we investigate new paradigms in treatment for men and women with bleeding disorders. Bayer is currently conducting early investigations into plasminogen inhibiting modalities and earlier this year signed a collaboration agreement with Dimension Therapeutics for the development and commercialization of novel gene therapies in hemophilia A.

New Manufacturing Facility in Germany

Also earlier this year, we began one of the largest investments in Bayer HealthCare's history: the development of a new facility for manufacturing hemophilia treatments, to be based in Wuppertal, Germany.

"2014 has been a significant year for us, in terms of building on our history of commitment to the community, as we've made tangible progress toward being able to deliver new, innovative therapies to the marketplace," said Dr. Mathew.

Supporting Our Community

Bayer has also been working on enhancing our tools and support programs. They include FactorTrack™ – a free, personal, interactive mobile application that supports the tracking and recording of FVIII hemophilia infusions – and Hemophilia Joint Visualizer – an interactive, customizable web-based tool designed to help physicians teach patients the benefits of long-term prophylaxis.

The Bayer Hemophilia Awards Program (BHAP) is one of the largest awards programs of its kind. It provides grants to early researchers, clinicians and caregivers. BHAP has awarded more than 230 grants totaling 22 million euros to researchers and caregivers from 30 countries, contributing to more than 400 publications and other scientific communications by awardees.

Bayer's mission is "Science for a Better Life," and our hemophilia offerings aspire to that important goal.

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Novo Nordisk is committed to Changing Possibilities in Haemophilia®

It is our response to address the unmet needs in haemophilia care. We work towards a future where all people living with haemophilia, with or without inhibitors, have the opportunity to live the life they desire.

We work with partners across the haemophilia community - such as the European Haemophilia Consortium (EHC) and other global or local partners, in order to reach this goal. In this article we would like to briefly highlight two key initiatives we support in the pursuit of our commitment to Changing Possibilities in Haemophilia®: the HERO grants to advance psychosocial research in haemophilia and the funding of the Novo Nordisk Haemophilia Foundation to improve access to care in the developing world:

HERO Research Grant 2014 received numerous applications

The HERO Research Grant encourages and seeks to foster more in-depth, evidence-based understanding of the challenges faced by people living with haemophilia. The goal is to obtain evidence that can support advocacy aiming to improve care for people with haemophilia, with a focus on psychosocial care. The application round for the grant has now closed. Applications from a total of 13 countries have been received and are now being evaluated. The winners will be announced during December 2014.

Learn more about the HERO on www.herostudy.org

The activities of the Novo Nordisk Haemophilia Foundation (NNHF)

To answer the need for improved access to care in the developing world, the Novo Nordisk Haemophilia Foundation (NNHF) was established as a non-profit organisation in 2005 in Zürich, Switzerland. The NNHF is dedicated to define and fund sustainable programmes, which improve access to quality care benefitting people with haemophilia and allied bleeding disorders in the developing world. With local partners and renowned experts, the NNHF addresses three focus areas: capacity building, diagnosis and registry as well as education and awareness.

Since 2005, the NNHF has funded development programmes in 59 countries worldwide. In Eastern Europe, the NNHF has supported programmes in Bulgaria, Latvia, Lithuania, Macedonia, Poland, Romania and Serbia. These programmes help to improve multidisciplinary care, care infrastructure, quality of diagnosis and registries, and empower the medical and patient communities. Current programmes in Lithuania for instance focus on musculoskeletal (MSK) care and sharing expertise gained with neighbouring countries to strengthen regional networks and adjust standards of care.

For more detailed information about the NNHF and its programmes, visit: www.nnhf.org

Find out more about how Novo Nordisk is Changing Possibilities in Haemophilia® everyday on (http://www.novonordisk.com/about_us/improving_haemophilia/improving-haemophilia.asp).

Announcements

EHC 2015 Calendar of Events

Mar 2 Round Table on 'Adverse Events and Inhibitors' - open to NMOs and selected participants

Brussels, Belgium

Apr 16 or 17 World Haemophilia Day on: 'Tenders and Procurement' - open to NMOs and selected participants

Location to be determined

Jun 15 Round Table on 'Tenders and Procurement' - open to NMOs and selected participants

Brussels, Belgium

Jul 3-5 Youth Workshop - open to NMOs

Location to be determined

Sep 11-13 HTA and Economics Workshop - open to NMOs

St Petersburg, Russian Federation

Oct 1-2 Pilot Leadership Conference - open to NMOs only

Belgrade, Serbia

Oct 2-3 EHC Annual Conference - open to all

Belgrade, Serbia

Oct 4 EHC Annual General Assembly - open to NMOs only

Belgrade, Serbia

Oct 12 Round Table on 'Mild Haemophilia' - open to NMOs and selected participants

Brussels, Belgium

Nov 20-22 New Technologies Workshop - open to NMOs

Location to be determined

For more information on these events, contact Laura Savini (laura.savini@ehc.eu)

Other Events

Feb 11-13 8th Conference of the European Association for Haemophilia and Allied Disorders (EAHAD)

Helsinki, Finland - More information at www.eahad2015.com

Feb 24-27 59th Annual Meeting of the Society of Thrombosis and Hemostasis Research

Düsseldorf, Germany - More information at www.gth2015.org

Mar 10-11 International Plasma Protein Congress 2015

Rome, Italy - More information at http://pptaglobal.org/meetings-events/international-plasma-protein-congress/overview

Mar 25-27 Congress of the European Association of Hospital Pharmacists (EAHP): "The hospital pharmacists' agenda - patient safety first!"

Hamburg, Germany – More information at www.eahp.eu/congresses

Apr 22-26 50th International Liver Congress

Vienna, Austria – More information at https://ilc-congress.eu/

May 7-10 World Federation of Hemophilia Musculoskeletal Congress

Belfast, UK – More information at www.wfh.org/whd

May 20-21 IPFA/PEI 22nd International Workshop on 'Surveillance and Screening of Blood Pathogens'

Prague, Czech Republic – More information at www.ipfa.nl/events/ipfa-pei-22nd-international-workshop-on-surveillance-and-screening-of-blood-borne-pathogens-prague-czech-republic

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The International Plasma Fractionation Association (IPFA) and the Paul-Ehrlich-Institut (PEI) are pleased to announce the 22nd annual International Workshop on "Surveillance and Screening of Blood Borne Pathogens". The Workshop will be held in Prague on 20-21 May 2015 at the Corinthia Hotel Prague and will be hosted by the Czech Society for Transfusion Medicine.

Range of topics

This annual International Workshop will, as usual, address key issues concerning the availability, regulation and risk benefit of existing and potential new developments, designed to ensure the microbiological safety of blood components and plasma derivatives. The event will build on IPFA's traditions of open and inclusive discussions between all stakeholders who may have an interest in and/or responsibility for maintaining a safe and secure supply of blood components and plasma products.

Event details | Registration | Sponsorship

For event details, registration and sponsorship opportunities please visit www.ipfa.nl.
You may also contact the IPFA Secretariat for further information: info@ipfa.nl; tel. +31 20 512 35 61.

We warmly invite you to attend this meeting and look forward to welcoming you to Prague for what we expect to be another informative and productive Workshop!

Dr. Paul Strengers, President IPFA
Dr. Micha Nübling, Head Molecular Virology PEI
Dr. Vit Rehacek, President Czech Society for Transfusion Medicine
Dr. Petr Turek, Head of Blood Transfusion Dpt. Thomayer Teaching Hospital

Who should attend?

The workshop has evolved to become a highly regarded international event, regularly attracting contributions from recognized international experts and opinion formers and delegates from more than 30 countries from all regions of the world.

The workshop will be of interest to:

- Blood establishments
- Doctors and health care providers
- Industry
- Regulatory authorities
- Patient organisations
- Academia

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