Haemophilia 2010 World Congress

IHS Magazine Supplement

Welcome to WFH Wold Congress 2010 - Buenos Aires

The World Federation of Haemophilia 29th Congress took place in Buenos Aires from 11th to 15th July 2010. The Congress was attended by the largest ever attendees over 4,300. It was a fantastic experience to meet and interact with so many people from so many countries throughout the world.

The Opening Ceremony was excellent with nearly 3,000 delegates attending and enjoying Argentinean ballet and the Tango. Participants also were treated to a Cultural Evening of Argentinean folklore and dance, and some even received Tango lessons.

Ireland was well represented in that we had 4 board members of the Society, 3 staff members along with many clinicians from the NCHCD, and also some members of the Society. We also had at various times over the course of the conference 9 posters on display in the exhibition hall in relation to a variety of issues. You will find a copy of each poster on pages 12 to 20 of this supplement.

We also had a booth within the World Federation of Hemophilia Resource Centre which was manned by both board members and staff of the I.H.S. and was a huge success. We brought 4 suitcases of various publications, and we were delighted that we had nothing left at the end of the Congress.

Before we left for the Congress we looked carefully at the programme and allocated various sessions to board and staff members which seemed to work well.

Argentinean Ballet at the Opening Ceremony

I attended a very good session entitled "Sports: Dangerous, Beneficial or Luxury" which was chaired by Physiotherapist Nichan Zourikian from Montreal. The session discussed the pros and cons of sports for children with haemophilia. While we all know sports can help build physical strength and improve musculoskeletal structure it can be difficult for children and parents as they must weigh up the risks and benefits carefully. Sports can be classified in three categories said Andres Thomas a physiotherapist in Argentina: those that can be safely recommended, those with relative risks and benefits must be assessed for each individual, and those like boxing or rugby whose risks outweigh the benefits. He also said that families should consult with professionals while their children are young to find the sport that best suits their circumstances. Dr. Sylvia von Mackensen a medical psychologist from Germany showed the results of three studies assessing the impact of sports in children's quality of life. The children's own perceptions of their physical abilities significantly affected their quality of life.

Home Treatment for people with haemophilia leads to improved quality of life no matter what part of the world they live in. This is the topic for another talk I attended in which it was pointed out the drastic differences in the level of care in the developing world and the fact that many people still receive no treatment at all and some do not survive into adulthood. Carmen Rodrigues from Brazil spoke about a "Home Dose Programme" which seems to be working quite well however due to recurring shortages of factor it means that only one quarter of patients actually have access to home treatment. Due to the geography of Brazil it makes sense that patients infuse at home as many live hours away from the nearest treatment centre.

Debbie Greene

The Haemophilia Population are Living Longer

But the Challenge to Address Acute & Chronic Medical Problems is ongoing

One of the main issues at this years WFH Congress in Buenos Aires is that of longer ageing of people with haemophilia and the new challenges that this create for individuals, treatment centres and the National Haemophilia Societies.

Life expectancy of people with Haemophilia even in the 1960's was about 20 years but over time with the advances of factor concentrates, the development of comprehensive care programmes and despite the catastrophe of HIV and Hepatitis C, we are now seeing a generation of people with haemophilia living into their 60's and older. People with haemophilia living longer of course creates new problems that are common in the general population such as cardiovascular disease, cancers related, renal disease and musculoskeletal disorders among them. These related co-morbidities in the ageing haemophilia population have now to be managed just as is the case in the general population.

We were told that there are not sufficient results from what very few studies that have been done reflecting health status and quality of life among the older generation of people with haemophilia. The challenges are there for the National Haemophilia Societies and Care Centres. For example with regards cardiovascular disease it is understood that compared with the general population, it is increasingly becoming apparent that ageing people with haemophilia risk to cardiovascular disease could be a main contributor of mortality comparable to the general population going forward as they live longer. The complication facing the medical practitioners is the use of anti-thrombotic therapy like aspirin and wafarin, it is a balancing act with the use of these drugs and the need for factor concentrate. To date there is some but not enough evidence based data to guide management of acute and chronic medical problems including heart disease in older people with haemophilia.

In other areas like cancer and renal diseases, that are more problematic to manage in people with bleeding disorders, similar conclusions are made that evidence based results is necessary as guides to better management. Again they tell us that there is little data to guide the use of factor concentrate in conjunction with chemotherapy or radiotherapy in the treatment of cancer related problems. Currently guidelines are based on opinion rather than evidence.

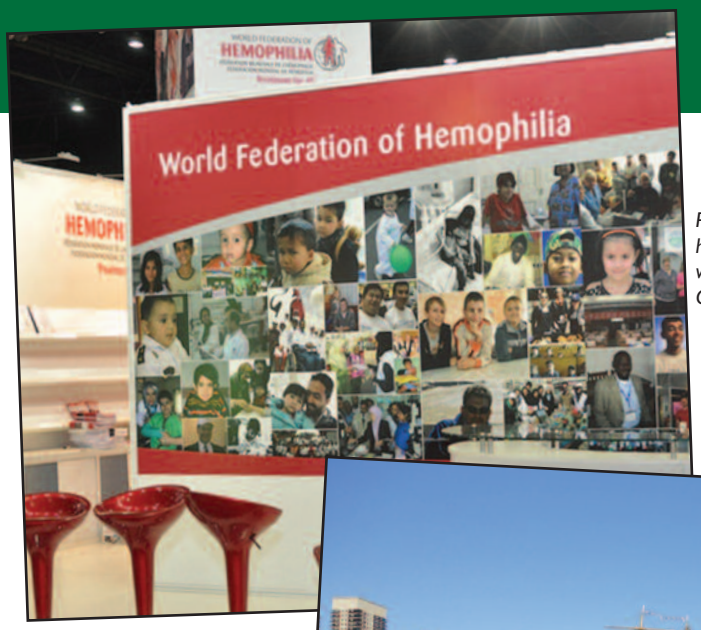
Most of the older population emerging have severe joint arthropathy and did not have the benefit of being treated with prophylaxis as children and now suffer from joint disease, poor mobility as a result of joint deformity and muscle weakness. This causes a lot of pain, risk of falls and lack of proper exercise and isolation. Over the years a physiotherapy programme was mostly effective at maintaining and strengthening the patients movements, quality of life, social aspects and fitness. With this older generation, a review of the forms of physiotherapy needed for the future also has to be addressed. In some studies that have been done, osteoporosis is shown to be on the increase in person with haemophilia. There is probably a need for screening for osteoporosis so as to re-evaluate the change in the physiotherapy programme for this group.

A programme that may change the way exercise is done and in the case of a joint replacement it would show how problematic it might be. It would show other skeletal issues that would be helpful in forming a physiotherapy programme.

Comprehensive Care Centres main focus over the decades was on managing and treatment of bleeding events, the risks of bleeding and also the complications associated, mainly Hepatitis C and HIV. Patients also relied on the haemophilia care centres for all of their medical care, that not enough attention to the other issues that can develop as they get older like utilising preventative programmes and screening for cardiovascular, prostate, lung, diabetes, hypertension ect.

Most aspects and studies of haemophilia discussed at this conference came around to the fact that ageing and living longer is here and will be a large group within the haemophilia population into the near future and that other health issues related to living longer, as is comparable to the general population have to be taken into account. More evidence based treatment and care is required to be put in place as a guide management of acute and chronic medical problems in older adults with haemophilia. Action now for more comprehensive and conclusive studies was the common agreement from the experts at the congress. People like Dr. Alison Street from Australia's "The Alfred Hospital", Melbourne, Dr. Gerry Dolan of Nottingham University Hospital, the Swedish, Dutch and New Zealand Societies amongst others are to the fore in addressing the ageing issues at present and I feel as a result of this congress, the message is clear and the attitude is to actively progress evidence based results to help guide the management of these age related issues.

Ger O'Reilly



Photos of people with haemophilia from across the world at the WFH Resource Centre

Ger O'Reilly taking in the sights of Buenos Aires

The famous monument Obelisco de Buenos Aires

Debbie Greene & Kevin Birkett at the I.H.S. Booth

Future Developments in Factor Therapy

There are many exciting potential developments in relation to longer acting factor concentrates. Many of the current companies and indeed several new companies are working assiduously on the development of factor concentrates which will have a longer half life or greater efficacy. This holds out the hope that in the coming years we will not only have a number of products available with longer half life but also that there will be more choice of products in each category where there is currently very limited choice. Dr. Claude Negrier in his plenary lecture talked about 4th generation recombinant products being those with an increased half life and 5th generation being those with an increased half life and decreased immunogenicity. It would be wonderful to have available recombinant products in the future which not only have a longer half life but which also would lead to a diminished risk of inhibitors. The development of 5th generation recombinant products are still some way into the future but companies are working hard at the development of the 4th generation products at the moment.

Established companies including Bayer, Baxter and Novo Nordisk are working on longer acting recombinant Factor VIII, recombinant Factor IX and recombinant Factor VIIa products. New companies including Biogen (who are working in conjunction with the established company Biovitrium) and Inspiration are working on recombinant Factor VIII and IX products. Octapharma are working on a human cell line recombinant Factor VIII product. Both Bayer and Novo Nordisk are working on longer acting recombinant Factor VIIa products.

The Irish Contingent at the conference centre

The fact that several companies are working on each product increases the competition between the companies. This is a good development as it should hasten the development of these much needed products.

There was also some discussion about an alternative approach or a complementary approach using a group of substances called Fucoidans.

These substances, including one which can be extracted from brown seaweed, are known to increase clotting in dogs and it has been found that oral treatment in dogs reduces bleeding. Basically they assist with clotting and they could possibly be used as an oral treatment in conjunction with (but not as a replacement for) factor concentrates.

In his lecture on recombinant and plasma-derived products, Dr. Alexander Gringeri, from Italy referred to a recent review article published on inhibitors in previously untreated patients (PUP's). This looked at 2,113 people with haemophilia from 24 different studies and showed that the inhibitor rate among those using plasma derived products was 14.86% and among those using recombinant products of 26.6%. This study does not provide clear evidence that there is a higher risk of inhibitors with recombinant products but there is certainly a trend to seeing higher inhibitor risk with recombinant products.

There is a study on going at the moment (SIPPET study), involving 78 centres from 18 countries. The outcome of this study should be instructive in the coming years in relation to this issue.

Brian O'Mahony

Prophylaxis

There were a number of symposiums on prophylaxis. Dr. Alok Srivastava from India stated that in his opinion on demand therapy does not change the natural history of haemophilia and that "the only way to treat haemophilia long term is prophylaxis". He pointed out that even in developing countries this may be more feasible than we think despite the limited resources that are available. This is because a large proportion of the people with haemophilia are not diagnosed. A percentage of those who are diagnosed with severe haemophilia will not have the severe bleeding phenotype and will not require prophylaxis and lower doses per kilogram could also be infused.

Dr. Marilyn Manco-Johnson, author of the Joint Outcome study, reiterated that prophylaxis in children allows normal development of joints and cartilage and prophylaxis is associated with an 84% reduction in the risk of joint damage. It also prevents possible sub clinical bleeding into joints. Some children with haemophilia who are treated with on demand therapy had significant changes using MRI in their joints, despite the absence of clinical bleeding. In relation to prophylaxis use in adults, she talked about using minimal doses, adjusting the frequency of treatment and individualising doses.

Dr. Tageliaferri from Italy, discussed the use of secondary prophylaxis in adults and the preliminary results of a long term study they are doing where they are following up 52 patients. Their conclusions were that secondary prophylaxis was a good investment for the management of patients with severe haemophilia. It did result in higher short term treatment costs but gave significant long term benefits in relation to reduced cost of treatment for bleeds, surgery and other procedures and also resulted in increased productivity compared to patients using on-demand therapy. For example, from the patients who stayed with on-demand therapy, the average number of bleeds per year was 10-17 and for those on prophylaxis this fell to 1.7-2.5 bleeds per year.

Dr. Victor Blanchette, from Canada reviewed the current prophylaxis regimens. The regimen pioneered by Malmo in Sweden where prophylaxis was started at 2 years of age, resulted in excellent joint scores. He also pointed out that joint damage is increased for every year that prophylaxis is delayed, with an average of 8% increase in joint damage per year. He compared the results from the Malmo regimen to the regimen used in the Netherlands (which uses a lower dose). The average number of bleeds per annum using the Malmo regimen was 0.2, whereas using the Netherlands regimen, the average number of bleeds per annum was 3.7. There was no difference in joint scores but scoring of the joints using X-rays did appear to show some more damage using the Dutch regimen.

The third prophylaxis regimen discussed was the Canadian Escalating Dose regimen. Rather than treating three times per week, this treatment regimen uses a high dose of 50IU/kg once per week escalating to twice a week in the event of bleeding or escalating to alternate days with further bleeding. The doses change from once a week to twice a week if there are more than 3 bleeds in the same joint in a 3 month period or 4 soft tissue bleeds in three months or 5 bleeds into a single joint. They have found that as the boys get older they tend to move in any case from treatment once to twice to three times per week. Using this regimen, 50% of the boys had some bone changes using MRI but the clinical significance of this is not clear.

You are here! Some young delegates looking at global figures

One of the many talks that took place over the course of the Congress. Attendances were very high.

He also discussed whether or not prophylaxis can be modified in adolescents or young adults. Those who stopped prophylaxis were generally of a mild bleeding phenotype. One study which looked at a group of 28 people with haemophilia between the ages of 16-24, 30% of them stopped prophylaxis and a further 22% decreased the frequency of prophylaxis. 50% of those who stopped had to restart prophylaxis and whether or not they had to restart seemed to depend on their particular bleeding phenotype or tendency. He also quoted a study from Italy, where 84 people with haemophilia were switched from on-demand treatment to prophylaxis. Their compliance was excellent as they knew from on-demand therapy what it was like to have a large number of bleeds. In this group, they had an average of 35 bleeds per annum using on demand therapy, and this fell to 4 bleeds per annum using prophylaxis. The tendency to bleed depends on the factor levels, the state of the underlying joints and the activity levels of the individual.

There was also an individual case study which was presented as a poster presentation by one individual with haemophilia from Germany. This gentleman who was 47 years old was treated with on-demand therapy in 2008. In that year he had 29 bleeds and used 164,000 IU's of factor VIII. In 2009, he switched to prophylaxis, with doses of 1,000 IU's on alternating days. In this year he only had 4 bleeds and used 191,000 IU's. The number of days he was sick and unable to attend work decreased from 31 in 2008 to 5 days in 2009 (all 5 days were due to one joint bleed). The result was that for a 16% increase in the amount of factor he used in the year on prophylaxis he had an 84% decrease in the number of bleeds. In a study from the Van Creveld Clinic in the Netherlands, where they compared the bleeding pattern and the quality of life of 27 people with severe haemophilia on prophylaxis with 37 who have moderate haemophilia, they found that the number of bleeds per annum were very similar and the quality of life was very similar. The conclusion was that prophylaxis effectively changed severe haemophilia to moderate haemophilia.

Brian O'Mahony

HIV and Hepatitis C

Dr. Margaret Ragni spoke about HIV and Hepatitis C co-infection. She pointed out that 10% of those who are co-infected develop end stage liver disease and the relative risk of developing end stage liver disease is increased 3.7 fold by alcohol use, and by 2.2 fold by each decade where the person is HIV positive or Hepatitis C positive. In contrast treatment with highly active anti-retroviral therapy is protective in a relative sense, in that the relative risk of developing end stage liver disease is reduced to 0.14. She discussed a study of 781 people with haemophilia of whom 37% were HIV positive. 25% of this group under went liver biopsy. Among those who were HIV positive, 46% had fibrosis. Among those who were HIV negative 32% had fibrosis. In those who were HIV positive, she stated that good predictors of fibrosis were alpha-feta protein and platelet count. In those who were HIV negative alpha-feta protein, ferritin and a liver index called APRI could be used. Measuring the ALT level (a liver enzyme) was also useful in both groups. Liver Transplants in those were co-infected with HIV, showed a 90% survival rate after 12 months. This is comparable to the group who are HIV negative. Post-transplant, in those who were co-infected, the CD4 count and HIV viral load were important predictors of outcome. She stated that people with haemophilia had a poorer outcome prior to liver transplant but their outcome post liver transplant was the same. Prior to transplant they had a higher mortality on the waiting list, preceded to end stage marginally faster and generally they were marginally longer on the waiting list.

Brian O'Mahony

Hepatitis C Update

Dr. Kenneth Karmen from Cinnicinati gave an update on Hepatitis C. There are more than 130 potential treatments or agents for treatment of Hepatitis C currently in development and two protease inhibitors have reached Phase III trials. These protease inhibitors have demonstrated a high degree of efficacy - 65-75% sustained virological response (SVR) in patients with Hepatitis C Genotype I. In trials using Telaprevir, one of the protease inhibitors has demonstrated a 67% SVR after 48 weeks of treatment in the so called Prove I trial. In the Prove III trial, where the treatment was given to previous non responders to pegylated interferon and Ribavirin, the addition of Telaprevir and 48 weeks of treatment achieved a 54% SVR. A specific gene polymorphism in one gene could also be used as a future predictor of outcome of treatment. This gene known as the IL28B gene has three possible confirmations, CC, CT or TT. In relation to predicting the probable success of Hepatitis C treatment in experimental studies, they demonstrated that those with a CC configuration at gene IL28B had a 75% SVR compared to a 35% SVR for those with CT or TT.

Brian O'Mahony

Why my son?

"Why my son," they were the first thoughts of César Garrido of Caracas, Venezuela when told that his son had been diagnosed with haemophilia. Who among us hasn't had similar thoughts when told that our son, or daughter least we forget that women can suffer too, had a bleeding disorder. Just think what it was like when we as parents were first told we had a child with a bleeding disorder. At best we felt challenged and at worst we were down-right frightened. Now think what it must be like to bring up a child in a country that doesn't enjoy the first class treatment that we enjoy in Ireland. Yet César's story is not one of despair but of hope. It is the story of parents overcoming the challenges that a bleeding disorder brings to help their son live a normal life.

Kevin Birkett with his wife Sheila, daughter Niamh and son Conor

César was speaking at a congress session entitled, 'Helping Children Cope with Bleeding Disorders' where he outlined the components to overcoming his own fears about haemophilia. "Early diagnosis, the knowledge that a comprehensive care team was available locally and the availability of safe treatment" were key factors in his opinion. He also acknowledged the work of the Asociación Venezolana para la Hemofilia and the peer groups it supports. "It was really important to know that you are not alone." César's philosophy is simple. "If we demand that people with haemophilia be 'normal' people for the rest of society, we must assume that they will face problems, norms, rules, activities, challenges, and opportunities that are also normal." He believes that children must develop tools for coping and "identify their limitations."

"The approach is that by offering children moments and scenes, where through sharing with others, they acquire awareness and realise that they can do anything but 'shouldn't do it'. Experience tells us in many cases that parents have too much fear, or are ignorant of what haemophilia is and what it means to live with it, and therefore they hinder bold and appropriate learning." He passionately believes that children should be encouraged to engage in activities that promote self-esteem and helps them feel more confident such as wall or mountain climbing, horse riding, biking, hiking, surfing, kayaking, dancing, camping, and swimming, amongst others. In closing his presentation César left us with this thought, "It is important for a father to feel useful, that the mother is not the sole carer. Haemophilia doesn't mean tears or crying, it's possible to enjoy haemophilia."

Gordana Stevanovic from Belgrade, Serbia gave a mother's perspective on bringing up a child with a bleeding disorder. In an at times emotional and heartfelt address to the delegates she outlined how finding out that her son had haemophilia had placed an, "enormous stress on her family." However like César she too believes that knowledge is the key when raising a child with a bleeding disorder.

"The ways in which families cope with it vary greatly. Individual differences, cultural and social background seem to play an important role in perceiving and dealing with the problem. However, the decades long experience of mothers from our local association shows that the more knowledge and understanding one has of the issue, the more efficiently one can build up a healthy environment for bringing up the child with a bleeding disorder." Knowledge she believes helps the family to overcome the initial shock of diagnosis and realise that with proper treatment the child can lead a normal and fulfilled life and need not be excluded from the society. "Knowledge builds a positive attitude", she maintains, which the child will learn from other family members.

Since in Serbia there is no organised service providing psychological assistance knowledge also enabled her and her family, with the assistance of the Serbian Haemophiliac Association, to provide appropriate psychological guidelines for her son. "Sharing experiences with other families is very important," Gordana acknowledged. Even with the help of the association there are still questions that each family has to find their own answers to such as when and how do I tell my child he or she has a bleeding disorder? The only advice Gordana would give to this vexed question was, "Always be truthful towards your child."

Both César and Gordana emphasised how important support from their national member organisation was to them. Christina Keilback, who is Executive Director of the Manitoba Chapter of the Canadian Hemophilia Society, was on hand to describe how her organisation assists its members.

"The chapter," she explained, "builds programming around the goal of helping children with haemophilia, and their families, to accept and normalise their bleeding disorder. Programmes like Family Camp, Member Meetings, Just the Guys Getaway, Sports Bursary Programme, presentations in a child's school, and social events reduce the isolation a family managing haemophilia can feel as a result of the rarity of the disorder."

Many of the programmes supported by Christina's chapter are quite familiar to us but given César's plea for fathers to become more active in the lives of their sons the 'Just the Guys Getaway' deserves special mention. The 'Just the Guys Getaway' allows sons and fathers, or a significant other male, time out together. During the getaway both will be given the opportunity to try various activities giving sons a chance to compete against their fathers. Interestingly the sons are often more adventurous than their fathers. All the children completed the high rope challenge but only one father attempted the task!

Christina's chapter works closely with the local psychosocial team member in the development and delivery of all its programmes, a luxury not afforded to Serbian parents. Ed Kuebler, a social worker at the University of Texas Gulf States Hemophilia and Thrombophilia Center, outlined his thoughts about helping children cope with a bleeding disorder.

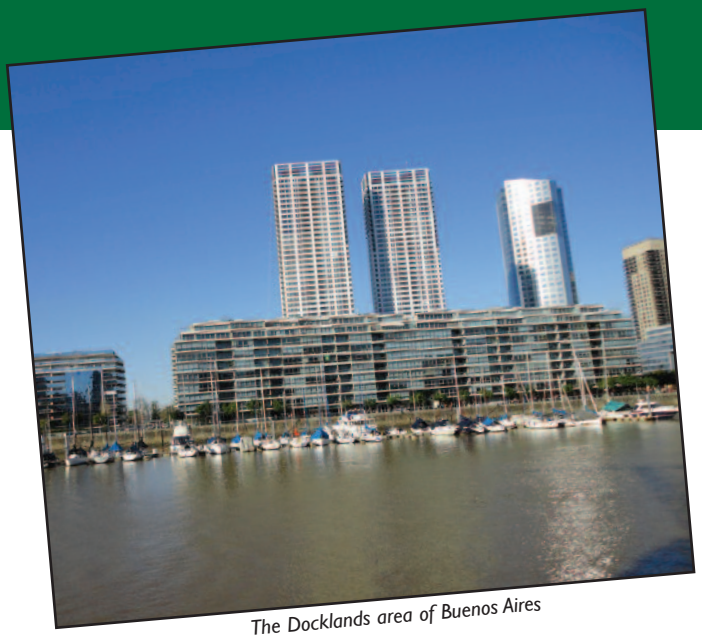
"In so many ways children are resilient and have the amazing ability to adjust to their surroundings and innately create coping skills to handle the immediate situation. Dealing with a chronic illness like a bleeding disorder requires the child to manage and adjust to their condition on a daily basis."

Unfortunately children can develop negative coping strategies. To avoid this Ed works with the family as a unit so the child who is receiving help has some consistency while gaining new, positive coping skills.

"If a strong, supportive family has positive coping skills then the child will learn them. Parents can teach impulse control, empathy skills and self awareness. Helping children understand and deal with their feelings allows them to handle the stress and issues facing them with their bleeding disorder."

While no two children, no two families and no two situations are exactly the same, one message came across loud and clear from all four speakers at this session; a caring, supportive family with a positive attitude towards haemophilia can have a hugely beneficial influence when helping children cope with bleeding disorders. As César said, "It's possible to enjoy haemophilia."

Kevin Birkett



The Docklands area of Buenos Aires

Mark Skinner (centre) President of the World Federation of Hemophilia at the WFH Resource Centre

Tango entertainment during Congress lunch

Reflections on the Congress

Today, as I sit down to write this report, I'm looking for inspiration by reading through the Hemophilia Newsletters, which were produced for each day of the Congress. I see one of our member's name as the Tuesday's winner of a draw to help a country win the WFH International World Cup! No, Ireland did not win any world cup, but during the Buenos Aires Congress, I certainly felt like a winner!

The buzz that I have come to associate with the WFH Congresses was certainly present in Buenos Aires. Even though, it was winter time in South America, the warmth and passion of the people I met there, touched my heart.

The sessions with the most interest for me were those in the psychosocial area, and in particular, the networking with colleagues from both developing and developed countries

I was pleased to chair an important session "Ageing and Haemophilia" at the WFH Congress. A Canadian colleague has called this topic 'a success story' and, while this is very true, it is one which will present many challenges for people with haemophilia and their health care professionals in the future. This session was an opportunity to bring together expert knowledge and experience from the different aspects of haemophilia care, and an opportunity to open an important dialogue on the future needs of people with haemophilia – physical, psychological, medical, financial and social. Most importantly, it gave a voice to the concerns and fears of senior people with haemophilia.

Dr. Alison Street from Australia, spoke on the medical aspects of ageing, from mobility issues to the age-related problems of kidney, heart and prostatic disorders, including osteoporosis, diabetes and cancer. Dr. Street introduced her preventative approach with the programmess she has developed in relation to: Balance & Falls; Vascular disease and Wellness Checks. The important Social Factors were also explored.

Zygmunt Gruszka, a member of the Swedish Hemophilia Society, spoke on lobbying for the future service needs of the Swedish senior members. The need of his organisation to formulate their future strategies based on the expressed needs of their senior members. He highlighted the importance of mutual understanding between the Haemophilia Treatment Centres and the needs of senior members.

Mike Carnahan from New Zealand, has been an active member within his haemophilia community where he was President for 5 years. He is a campaigner for the care of older people with haemophilia. Mike shared his personal experience of living, working and of taking early retirement in a developed country. Mike highlighted the adjustments he made to his living accommodation in terms of safety and easy access.

Anne Duffy & Pat Downey in the conference centre

Anil Lalwani from India who has degrees in chemistry and marketing and has severe haemophilia, and who has been involved with the Hemophilia Federation India since 1983 where he has worked in many voluntary capacities, including Executive Committee member and President. Anil shared his personal experience of living in a developing country with little resources for people with haemophilia.

Both Anil and Zygmunt recommended the establishment of an 'Over 50's Forum' To give a voice to this cohort within the haemophilia community.

Another interesting talk I went to was titled "Living with a rare bleeding disorder". Evelyn Grimberg, from the Netherlands shared her experience of living with this condition. It is a platelet disorder, where the platelet plug cannot be formed. The treatment is Tranexamic acid; Platelet transfusion; Factor 7A; and the Contraceptive Pill. Evelyn was diagnosed at 3 months with Glanzmanns disorder following a severe nosebleed. Her main difficulty was at puberty, where the haemorrhaging needed blood transfusion. She is currently on injections of hormones monthly to suppress menstruation. Evelyn was asked by the audience about what helped her cope with the adolescent period. Her answer was talking with fellow sufferers. She stressed the importance of sharing her feelings.

Gabriel Lottaz from Switzerland, who has factor I deficiency, which is the absence of fibrinogen, gave an interesting insight into this condition. Thrombosis can occur as well as bleeding, the mechanism is still not fully understood. In his early years, Gabriel bled from the umbilical cord. Spontaneous bleeds started when he was 14 years of age. When this developed into a chronic haemorrhage, he was started on on-demand treatment. Gabriel only got home treatment at the age of 28 years.

Anne Duffy

Musculoskeletal Updates

The Swedish Haemophiliac's Self Reported Activity and Function in Daily Life:
Study aimed at establishing a standardised scoring system for people with haemophilia, living in a comprehensive care environment with potential access to local community services. Should a need be identified in the scoring system an action plan would be sent in place locally based on the needs of the patient.

Physiotherapy Training Workshop in South Africa – Unchartered Territory:
Due to funding issues and the vast distances involved in South Africa this workshop was an attempt to disseminate best practice in the treatment of people with haemophilia by physiotherapists. In South Africa the vast majority of physiotherapists, work in the private sector and are based in the larger urban areas. The workshop also gave access to treatment for patients in rural districts that otherwise would not have the financial means to travel to cities to learn appropriate physical therapy regimes.

Haemophilia Joint Health Score Reliability Study in China:
The World Haemophilia Federation continues to attempt to reach agreement on a standardised "joint health score" that will satisfy the apposing requirements of both the developed world and the developing world.

The research continues in China with the ultimate goal of finding an agreed measurement system to quantify the minimum amount of factor required to prevent joint disease and to use this information in encouraging governments to fund prophylaxis programs in countries where it is not now available.

Is it a bird? Is it a plane? No its Anne Duffy, Kieran McHugh, Ger O'Reilly, Pat Downey & Sharon Farrelly taking in the sights of Buenos Aires

Musculoskeletal Health in Patients with Haemophilia in Colombia:
Excellent description of the use of minimal resources to provide long-term treatment for haemophilia patients by focusing on the basics of habitual poor posture, insufficient treatment/rehabilitation, imbalance between different muscle groups and appropriate management of bleeding episodes are essential to prevent long-term disability.

Surgical Management of Chronic Large Pusedotumour in Haemophilia Patients who presented late at Kuala Lumpur Hospital:
This is a reminder to the Haemophilia family in Ireland that we have access to adequate factor products and that as a group we have seen in the past the results of the late or inadequate treatment of devastating muscle bleeds leading to Pusedotumours. This should not be happening to people with haemophilia in the 21st century and we should be acting as an advocate for all people with haemophilia that currently do not have access to adequate levels of factor.

The famous Tango at the Cultural Evening

Effects of modified Tai-Chi Exercise Program:
In Korea they are seeing wonderful progress in post-operative patients following arthritis surgery by modifying the four styles and twelve basic movements of Tai-Chi. By modifying the program to the abilities of each patient into fluid flowing movements you can build up over time both the flexibility and the feel-good factor of the patient. I was delighted to state that in Ireland certain aspects of the principals of Tai-Chi are incorporated into exercise programs and that I had personal experience following a recent total knee replacement.

Influence of water activities programme on haemophilic children's physical condition and quality of life:
Long-term study by Valencia University on this subject, they will continue to collect data for the use of the World Haemophilia Federation.

Influence of an aquatic training protocol on the reduced risk of cardiovascular morbidity and mortality in a hemophiliac: Long-term study by Valencia University on this subject, they will continue to collect data for the use of the World Haemophilia Federation.

Pat Downey

General Assembly 2010

Looking to the future: Kevin Birkett, Michael Davenport and Brian O'Mahony with our prospective twinning partners 'Vietnam'

The General Assembly of the WFH is held the day after the Congress ends. This is the business meeting of the WFH and is a convenient time as most of the 118 WFH country members attend the Congress anyway. On this occasion, in the Sheraton Buenos Aires Hotel and Convention Centre, 75 countries were represented at the Assembly, with translation available in Arabic, French, Russian, Spanish and English.

A very interesting agenda item occurs at the beginning of the meeting when new countries are accepted into the WFH as either 'associate' or 'full' members. This follows a vetting procedure during the previous year to establish the level of organisation within the applicant country. Associate members accepted this year were Bolivia, Ethiopia, Ghana, Surinam and Tanzania and full members accepted were Botswana, Eritrea and Sudan. This brought the total number of countries in the WFH to 118. On reading the information supplied for each of the above countries it is apparent that they have an enormous task ahead of them to establish haemophilia care. For instance, Ethiopia has a population of 80.7 million people but only 53 people registered with haemophilia! Ghana has a population of 23.4 million people and only 23 people with haemophilia registered!

However, each of these new organisations has committed volunteers, and board members from the medical profession, and with NMO training and twinning programmes organised by the WFH, hopefully they will achieve their goals in the not too distant future. Mark Skinner's presidential address covered WFH activities since Istanbul in 2008. Referring to the 2006 strategic plan, among a list of items, he said the WFH have made progress in improving treatment in emerging countries, ensured continued development where treatment is already established and continues to promote access to safe and improved treatment. Among challenges for the future he included applications of new communications technology, such as podcasts, twitter, facebook etc. in order to distribute up to date information to as wide an audience as possible. The WFH is also well placed to take a leadership role in research for better treatments and eventually a cure for bleeding disorders and are currently exploring how best to use their unique position.

Gordon Clarke, from Northern Ireland, stepped down from the Executive Committee since joining it in 2002. Gordon worked tirelessly for many years on NMO Advocacy Training, Strategic planning, Accreditation and many other WFH programmes. Many of us in the I.H.S have met Gordon on many occasions while participating in training programmes and other meetings. We thank him for his work with the WFH and wish him well for the future.

An exciting part of each General Assembly are presentations by two countries wishing to host the Congress in four years time. Miami and Melbourne each displayed their conference facilities, tourist attractions, hotel and flight costs etc. and a secret ballot took place. The result was 55 to 20 in favour of Melbourne.

While many World Federation Congress' have been in far flung places that can be expensive and time consuming to attend, the next one is in Paris in 2012. This is very accessible and a great opportunity to attend the largest worldwide biennial haemophilia community gathering. The Congress is an eye opener to the bigger picture of haemophilia, the contrasts between developed and non developed countries is startling, the range of in-depth up to date information on any aspect of haemophilia you can think of is amazing. Experts in the fields of haematology, hepatology, virology, physiotherapy, psycho social, etc. are on hand to give talks and answer questions over four days. See you there mon ami!

Michael Davenport

Sightseeing in Buenos Aires

A survey of the outcome of prophylaxis, on-demand or combined treatment in 20-35 year old males with severe haemophilia in four European countries

Background

Prophylaxis has been widely accepted as the standard of care for children in the treatment of haemophilia. However there is a lot of discussion still on the requirement for prophylaxis to continue into adulthood. The aim of this study was to examine the effects of prophylaxis in adults with severe haemophilia.

Methods

National Haemophilia patient organisations in Ireland, UK, France and Sweden were asked to participate by randomly selecting 20 severe haemophilia patients between 20 to 35 years. The data collection was performed between August and November 2009. Out of the total 80 questionnaires, 58 (72.5%) responses were received either by mail or by phone interview. Ireland provided 19 responses, UK 9, France 10 and Sweden 20. Phone interviews were carried out by the relevant national haemophilia society. Sociodemographic data (age, country and work or college status) medical data and responses to an EQ-5D questionnaire were collected. Work status was categorised into 3 categories: employed, student and unemployed. The Medical data collected was type of haemophilia, severity of haemophilia, treatment regime (prophylaxis vs. on demand), bleeds per year, target joints, major bleeds and days missed from work per year. Examples given of major bleeds were ilio-psoas or inter-cranial bleeds. The EQ-5D questionnaire is a generic health-related quality of life measure consisting of five questions and it has been previously used in haemophilia patients. The primary analysis evaluated the differences between the four countries Sweden (N=20), France (N=13), Ireland (N=19) and the UK (N=9). Subsequently, based on time spent on prophylaxis, the sample was split into four groups: Group 1: patients on prophylaxis their entire life (N=18); Group 2: patients who spent 50 – 99% of their life on prophylaxis (N=22); Group 3: patients who spent 1 – 50% of their life on prophylaxis (N=16) and Group 4: patients who spent their entire life using an on-demand regime (N=7).

Results

	Reported Average Number of Bleeds per Year (n)	Presence of target Joints (%)	Occurrence of Major Bleeds (%)	Average Days missed per year (n)	Mean EQ-5D Utility Value	Median EQ-5D Utility Value
France	20.1	100%	80%	15.0	0.74	0.73
Ireland	16.5	94%*	68%	5.0	0.68	0.76
Sweden	3.2	25%	20%	0.5	0.93	1
UK	17.5	100%	44%	6.6	0.76	0.73
*Note: Of respondents from Ireland, UK and France only 1 person (20y.o) does not report target joints. He was on prophylaxis until he was 18 and has now switched to on demand						

Table 1: Individual country comparison

	Reported Average Number of Bleeds per Year (n)	Presence of target Joints (%)	Occurrence of Major Bleeds (%)	Average Days missed per year (n)	Mean EQ-5D Utility Value	Median EQ-5D Utility Value
Group 1 (Prophylaxis)	3.2	26.3	26.3	0.9	0.88	1
Group 2	11.5	81.0	59.1	3.6	0.77	0.8
Group 3	20.1	93.8	56.2	3.6	0.79	0.74
Group 4 (On Demand)	26.5	88.9	48.5	19.2	0.72	0.8

Table 2: Treatment comparison across all countries

Conclusion

Prophylaxis started at an early age and continued into adulthood results in less bleeding, less damage to joints and less time missed at work. Prophylaxis increases mobility and the ability to do everyday activities and improves the health related quality of life of people with severe haemophilia. It would be beneficial to extend this survey in the future to gather data on a larger number of people with Haemophilia from a larger number of countries and the authors plan to do this. It would be interesting to extend this survey to countries including the Netherlands and Canada where a distinctly different prophylaxis regime is used that that modelled on the Swedish regime. It would also be instructive to extend the survey to several countries which use low levels of FVIII per capita in order to assess what may well be, in effect, a baseline utility figure.

Authors: Declan Noone, Brian O'Mahony, Lucia Prihodova
Irish Haemophilia Society

Psychosocial Workshops Belarus 2009

Author: Anne Duffy – Irish Haemophilia Society

Psychosocial Committee Volunteer working with WFH GAP Program

Psychosocial Support of the Patient & his family

Life Long Challenges, Parenthood, Siblings, Grandparents, First Year, Toddlers, School, Adolescence, Sexuality, Relationships, Self-esteem, Ageing & Practical Ideas.

Two workshops

This provided an opportunity for both the adults with haemophilia and the parents of children with haemophilia, to ask questions about services in other countries and to express their own concerns.

Learnings

It is possible to have engaged discussions with people through a translator. The main concern of patients was lack of factor outside of the capital Minsk. I gained insight into their daily life, their plight and their desire to improve the services available to them. I was very impressed by the Belarusian Association of Haemophilia Patients Board Members – they are passionate, organised and determined individuals. They are not helpless – and with their support – neither are the people with haemophilia in Belarus.

Psychosocial One-Day Training

'Dare to Dream' Exercise

Let yourself imagine how you see your Association develop in the next 3 years.
Imagine there are no obstacles, no lack of funds and enough factor for all.
Imagine what you would like to see available for your members:-
What do you see yourself doing in the future? Who else is involved in this future scene?
What events have you for adults with haemophilia and for children & their parents?
Ask yourself, what do you need to reach this goal?
What skills will you will need? What people and resources will you need?
Pick 2 things which surprised you. Everyone is invited to share their experience with the group.

Comprehensive Haemophilia Care

The ideal way of caring for all the needs of the person with haemophilia.
Core team members are: Haematologist; Haemophilia Nurse Specialist; Physiotherapist; Psychologist; Social Worker/Counsellor; with access to - Orthopaedic Surgeon; Dentist; Hepatologist; Infectious Disease Specialist & Psychiatrist.

Psychosocial Support within Patient Organisation

Create a space where the person with haemophilia can share his thoughts, worries and feelings; Provide one-to-one counselling; Peer Support;
Home and hospital visits.
Importance of building a strong sense of community;
Knowing members and making direct contact through annual phone calls;
Providing current information on factor safety & supply, updates on Hepatitis C and HIV treatments and management through Newsletters and Website;
Meetings for members which are both informative and social.

Psychosocial Issues through the Life Stages

Strategies & Objectives for each stage:

0 to 5 years:
helping parents to accept the child's diagnosis, building trust with health care professionals and allow child to develop normally.

6 to 9 years:
empowering parents to help child understand and accept his haemophilia; importance of play and friends for child to develop self-confidence and positive self-esteem.

10 to 13 years:
empower child to take active role in his treatment; use of humour for self-acceptance; importance of body image and need to fit in with peers; support child to deal with his feelings about physical changes and health issues.

14 to 17 years:
independence & self-management; career guidance to maximise use of talents & minimise risks of injury; issues with compliance – ignoring bleeds without thinking of consequences;
need to understand importance of physiotherapy and self-care.

18 years & over:
transition towards independence; support with job interviews and disclosure;
importance of support from family, friends and peers; genetic counselling for young couples.

Managing your Pain:

Medical Treatment; Dependence; Exercise – Using Joints Wisely; Sleeping Better; Safe Massage; Hot & Cold Treatments;
Relaxation: guided imagery;
Family Issues: support, encourage, and avoid over-pampering;
Stress: tips for reducing stress; Coping with Depression & Anger; Acceptance & Coping;
Importance of Peer Support.

Managing bleeds without factor – RICE

Use of 2 hand Puppets in Education for young children with severe bleeding disorders:-
'Lucky Duck' is a bright yellow duck puppet who has haemophilia.
His friend 'Treatment Turtle' is a ninja turtle, who helps 'Lucky Duck' deal with his haemophilia. The dialogue with the puppets give positive reinforcement of the following messages:

- Use of RICE (Rest - Ice - Compression - Elevation) to manage bleeds especially in the absence of an adequate factor supply
- Importance of gentle exercises to prevent re-bleeds
- Importance of education, keeping up with lessons and homework while absent from school because of bleeds
- The need to care for teeth and gums through twice daily brushing
- Importance of swimming to build up muscles which protect joints from future bleeds

Psychsocial Issues & Hepatitis C – Stress & tension are natural reactions to any health and safety threat.
Concerns for family can make this experience worse.
Family Relations – importance of good communication; counselling may help
Telling the Children – timing is vital; affected parent has to decide and be prepared to answer questions honestly
Common Emotions Experienced – grieving process; denial and isolation; anger; bargaining; depression; fear and worry
Getting Emotional Support – important to talk to supportive family members, friends and health care professionals
Management of Fatigue – balancing daily activities; taking breaks to rest; asking for help from family and friends; exercise can increase energy and strength; eating small meals frequently; developing a before-sleep routine with quiet time and a warm drink.

Psychosocial Action Plan

Following discussion, the group decides what actions are required and when to do each of them – Now, Soon or Later.
Who - Names of people responsible to do each job
When - Dates when each action is due for completion
How - Resources available and what needs to be sourced.
Keep chart of action plan as a record of what was agreed. Give copy of plan to the WFH.
It is important to monitor the plan quarterly.

Individual Communication Strategy from a National Haemophilia Society

Authors: Anne Duffy, Declan Noone – Irish Haemophilia Society

Objective of communication strategy:

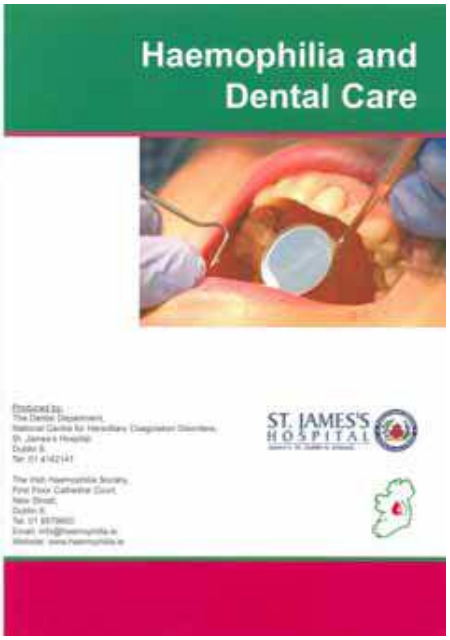
To make an annual telephone call to all Irish Haemophilia Society members by designated staff. This examines how the present Irish Haemophilia Society support services, activities and programmes meet the current and changing needs and requirements of our members.

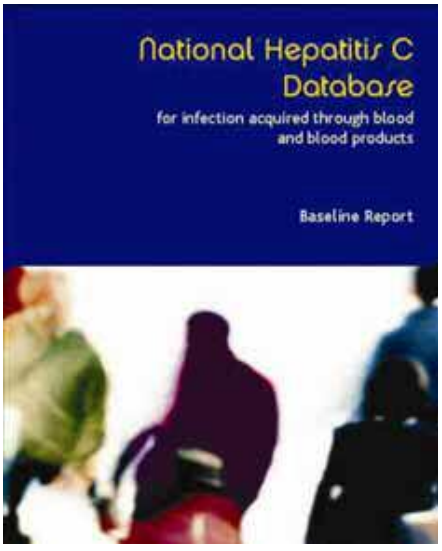
Communication strategy:

- **Gathers vital data.**
- **Updates contact information including mobile numbers and emails.**
- **Determines impact and usefulness of publications and educational materials.**
- **Identification of any barriers to this interaction.**
- **Identifies need for home and/or hospital visits.**
- **Targets resources based on real need, identifies gaps in services nationally.**
- **Identifies concerns of members and any problems they have with the health care professionals.**
- **In 2009 – 70% of members were contacted. Of these:-**
  84% were satisfied with the services in the Haemophilia Treatment Centres nationally.
  92% were satisfied with the services and support of the Irish Haemophilia Society.
- **Targets resources based on real need and identifies gaps in the services nationally.**
- **Identifies members' concerns and any problems with the health care professionals.**

Ascertained reason for non-participation of 30% of members living with Hep C and/or HIV in a National Hepatitis C Database.

This enabled the Society to take positive steps in order to reduce this to 10%. Therefore, we achieved a 90% participation rate with the Irish Haemophilia Society members.





Haemophilia in Central and Western Europe: A Comparison

Authors: Brian O'Mahony, Declan Noone - Irish Haemophilia Society

In 2009 a questionnaire was developed and sent out to 44 National Haemophilia Patient Organisations in Europe. Responses were received from 19 countries. The questionnaire was based on the extent of which the European Principles of care reflect the reality of Haemophilia care in these countries. The questionnaire consisted of 31 questions covering aspects of the ten basic requirements for Haemophilia care as set out in the European Principles. The 19 countries which responded including 16 EU countries and 3 non EU countries. They covered a total 28, 916 Patients with Haemophilia A, 5,545 patients with Haemophilia B and 17,396 patients with von Willebrands Disease**.**

Results:

- 12 of the 19 countries have a Central Organisation for Haemophilia Care
- 15 of the 19 countries have National Patient Registries, those who do not have a National registry are Latvia, Poland, Sweden and the Netherlands
- In terms of managing the registry, 6 countries have the National Organisation involved, in 3 countries the Government is involved, in 6 countries clinician's are involved and in 7 countries the National Patient Organisation is involved
- 15 of the 19 countries have comprehensive care centres. Those who state they do not have comprehensive care centres are Bosnia-Herzegovina, Bulgaria, Portugal and Hungary
- 16 of the 19 countries have Haemophilia Treatment Centres. Those that state they do not have Haemophilia Treatment Centres are Bosnia-Herzegovina, Russia and Sweden (in both Russia and Sweden all centres are categorised as comprehensive care centres)
- In the majority of countries surveyed, the clinicians, the Ministry for Health and the National Haemophilia Patient Organisation played a significant role in the decision making on National Haemophilia Care
- In relation to the choice of haemophilia treatment products, governments, clinicians and patient organisations played a role in the majority of countries
- Home Treatment is available in 17 of the 19 countries surveyed and is delivered directly to the patients home in 6 of the countries
- Home Treatment is not available in Bosnia-Herzegovina and Romania. These are the two countries who use less than 1 IU per capita of FVIII per annum
- Prophylaxis is theoretically available to all persons with Haemophilia in 8 countries and available to some children in 5 countries.
- Prophylaxis is limited or unavailable in 6 countries. In ten countries (Belgium, Czech Republic, Hungary, Switzerland, Netherlands, Portugal, UK, Germany, Ireland and Sweden) between 75% and 100% of children with severe Haemophilia avail of prophylaxis. In four countries (Poland, Slovakia, France and Russia) 50% - 75% of children with severe Haemophilia avail of prophylaxis
- There is limited or no availability of prophylaxis in Bosnia-Herzegovina, Bulgaria, Latvia, Romania and Lithuania
- Prophylaxis for adults with severe Haemophilia is widely available in 3 countries (Sweden, Netherlands and Hungary)
- Prophylaxis for adults with severe Haemophilia is available for up to 50% of adults in a further 6 countries (UK, Germany, Ireland, Portugal, France and Russia)

The major gaps in relation to the provision of elements of comprehensive care identified were as follows:

Pain management – not available in ten countries
Social and psychosocial support - not available in 11 countries
Dentistry – not available in five countries
Hepatology – not available in four countries
Urology – not available in eleven countries
Physiotherapy - not available in five countries
Rheumatology - not available in four countries

A Survey of Factor use in 19 European Countries

Author: Brian O'Mahony - Irish Haemophilia Society

In 2009, a questionnaire was sent to all European countries and responses were received from 19 countries. This included 16 EU countries and 3 non EU countries.

❑Choice of Haemophilia Treatment Products - 12 countries stated that their Health Ministry were involved, one country (Sweden) stated that their regional Government was involved, hospitals were involved in eight countries, patients in four countries and the National Haemophilia Patient Organisation was involved in three countries (Portugal, France and Germany), clinician's in eight countries and a National Procurement Committee in three countries (Bosnia – Herzegovina, Hungary and Ireland).

❑In the case of Ireland the National Haemophilia Patient Organisation are fully involved in the decision making as they have a formal role on the National Procurement Committee for factor concentrates.

❑In Hungary, Bosnia – Herzegovina, the National Haemophilia Patient Organisations are invited as observers but they do not have a formal role in the process of product selection.

❑The survey revealed enormous variation in relation to the availability of factor concentrates in the European countries surveyed (Fig 1). The country with the highest per capita use was Sweden and consumption was lowest in Romania.

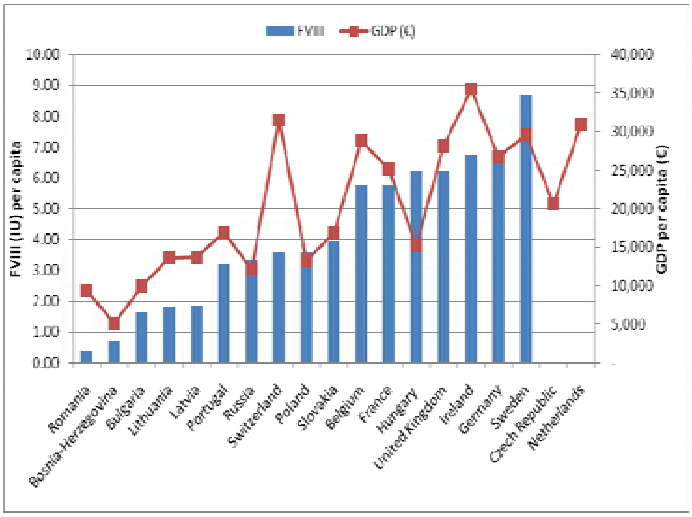


Figure 1

❑Seventeen countries reported figures for their FVIII per capita use for 2009 which ranged from 0.38 to 8.7iu per capita, Median was 3.6iu per capita and Mean was 4.1iu per capita.

❑Romania and Bosnia – Herzegovina both reported usage of less than 1iu per capita while Bulgaria, Lithuania and Latvia reported use of less than 2iu per capita. If we compare FVIII use to GDP use per capita, we can see that all five of the Eastern European countries who use less than 2 iu per capita significantly under perform in relation to their GDP per capita.

❑Of the Western European countries, Portugal and Switzerland use less FVIII per capita than you would predict from their GDP per capita.

❑In the case of Ireland there was a very rapid increase in GDP per capita up to 2008, which out stripped the high increase in per capita FVIII use which has increased from 1.9iu per capita in 1997 to 6.75iu per capita in 2009.

❑Hungary and Sweden out performed in relation to their FVIII per capita use compared to their GDP per capita.

❑It is worth nothing that between Romania and Sweden who are both EU countries, there is more than a 22 fold difference in FVIII use per capita.

❑Ten countries stated that Recombinant Factor Concentrates were always available but Plasma Derived Concentrates were rarely available.

❑13 Countries stated that Plasma Derived Concentrates were always available and Recombinant Concentrates were rarely available.

❑Four countries (Romania, Bosnia – Herzegovina, Lithuania and Russia) reported persistent or occasional use of cryoprecipitate.

❑Romania is the only country which uses fresh plasma on a regular basis although it is also infrequently used in Bosnia – Herzegovina, Lithuania and Russia.

❑The countries which primarily use recombinant factor concentrates (and the percentage) and rarely use plasma derived concentrates are: Ireland (100%) , Sweden (83%) , Germany (56%) , France (81%) , Switzerland (97%) , Belgium, Netherlands, Hungary and UK.

❑Those who use plasma derived (and the percentage) and rarely recombinant – Slovakia, Hungary, Czech Republic, Latvia, Lithuania, Poland, Russia, Portugal (55%) Sweden (17%) and Germany (44%).

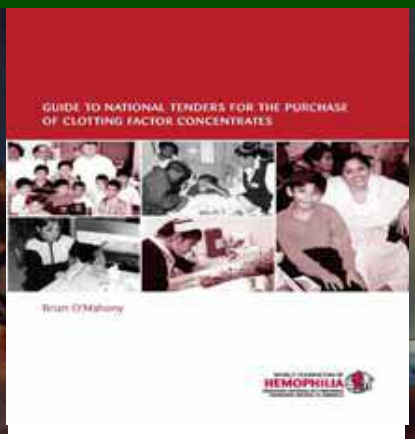
❑Recombinant Factor Concentrates were always available in all of the countries who have a FVIII consumption of 5iu per capita or greater.

❑In relation to treatment for von Willebrands Disease 15 countries reported that Plasma Derived concentrates are always available, 3 countries – Lithuania, Romania and The Netherlands reported that they are rarely available. One country did not supply an answer to this question. Ten countries stated that DDAVP was always available.

Country	Haemophilia				Von Willebrand disease (vWD)			
	Plasma	Cryoprecipitate	Plasma-derived Factor Concentrate	Recombinant Factor Concentrate	Plasma	Cryoprecipitate	Plasma-derived Factor Concentrate	DDAVP
Belgium	Never	Never	Rarely	Always	Never	Never	Always	Always
Bosnia-Herzegovina	Rarely	Rarely	Always	Rarely	Never	Never	Always	Never
Bulgaria	Never	Never	Always	Rarely	Never	Never	Always	Never
Czech Republic	Never	Never	Always	Rarely	Never	Never	Always	Rarely
France	Never	Never	Always	Always	Never	Never	Always	Always
Germany	Never	Never	Always	Always	Never	Never	Always	Always
Hungary	Never	Never	Always	Always	Never	Never	Always	Rarely
Ireland	Never	Never	Never	Always	Never	Never	Always	Always
Latvia	Never	Never	Always	Never	Never	Never	Always	Always
Lithuania	Rarely	Rarely	Always	Rarely	Rarely	Rarely	Rarely	Always
Netherlands	Rarely	Never	Always	Always	Rarely	Never	Rarely	Always
Poland	Never	Never	Always	Never	Never	Never	Always	Never
Portugal	Never	Never	Always	Always	Never	Never	Always	Always
Romania	Always	Rarely	Rarely	Rarely	Rarely	Rarely	Rarely	Rarely
Russia	Rarely	Rarely	Always	Rarely	Rarely	Rarely	Always	Never
Slovakia	Never	Never	Always	Rarely	Never	Never	Always	Rarely
Sweden	Never	Never	Rarely	Always	Never	Never	Always	Always
Switzerland	Never	Never	Rarely	Always	Unknown	Unknown	Unknown	Unknown
United Kingdom	Never	Never	Rarely	Always	Never	Never	Always	Rarely

A Training Course in Factor Replacement Therapy Concepts for Haemophilia Organisation Leaders

Author: Brian O'Mahony – Irish Haemophilia Society



National Haemophilia Patient Organisations are encouraged to seek representation on their tender commissions or procurement committees. In several countries including Ireland, Canada and Brazil, patient representatives play an integral part of the procurement process. The concepts can be intimidating for those with limited experience or scientific knowledge. There is no training pathway for key opinion leaders in National Haemophilia Patient Organisations to develop their knowledge in this area. The majority of patient leaders do not have a scientific background and may lack understanding of the basic concepts. The training course was organised as a means of educating participants in the concepts relating to Factor Replacement Therapy. The course was organised around two (basic and advanced) three day modules, ideally held one year apart.

Basic course

Focused on Introduction to the Basic Concepts in Replacement Therapy, Safety, Economics, National Tender Procurement Systems and Mock tenders. The lectures on the basic aspects are interactive and care is taken to ensure that the participants are encouraged to ask questions and receive clear answers in terminology that they can comprehend. The terminology is demystified and the concepts are explained clearly.

Mock Tenders

The third day of the course is primarily based around carrying out a number of mock tender evaluations.
Separate mock tenders are carried out for both plasma derived and recombinant factor concentrates.
The examples of products used and the specifications and characteristics are based loosely on real products but alterations have been made to make the choice more difficult and also to ensure that the knowledge imparted on the course from the previous two days is used during the selection process.
The mock tender process and the discussions during the mock tender process embed the information that people have listened to for the previous two days and demonstrate to the participants the practical benefits of the course.

Evaluation

Participants were given a multiple choice pre-evaluation questionnaire of 25 questions. This was completed at the beginning and at the end of the course. In the pre training course questionnaire the average number of correct answers was 12.5 of 25 (50%) The range of correct answers ranged from 9-19. At the conclusion of the course, correct answers from the same participants averaged 17.7 (71%) with a range from 13-21. The most notable change was from one participant who prior to the course answered ten out of 25 (40%) questions correctly and by the end of the course was able to answer 21 out of 25 (84%) questions correctly. This individual now participates in his countries tender process for factor concentrates.

Advanced course

Focused on Manufacturing and Regulation, Economics and Health Technology Assessments, Update on Safety, Current and Future Developments.

Mock Tenders

In addition to carrying out two further mock tenders participants were also asked to devise the selection criteria for these tenders prior to carrying out the mock tenders.

Evaluation

In an evaluation survey carried out 9 months following the completion of the advanced course: 6 of the 7 respondents categorised the course as extremely useful to them and one as very useful. The course directly assisted 3 participants from 2 of the countries to become formally involved in their National Tender Process. Other participants were assisted by the course in their involvement with their Ministries for Health and Regulatory Authorities in relation to issues including: vCJD, Health Technology Assessments and European Directives and Guidelines. Participants also greatly valued the networking opportunity and exchange of information with each other. Ongoing contact between participants has been encouraged and facilitated by the provision of information from the course organisers on a regular basis. In evaluating the course, participants stated they now had a more in depth knowledge of the relevant issues, increased confidence presenting the views of their organisations and they believe themselves now to be more effective advocates for their communities on these issues.

Attendees

The initial basic and advanced courses were carried out in 2008 and 2009 for participants from 6 European Countries; France, Sweden, UK, Ireland, Germany and Italy. The basic course has also been attended by program staff from the World Federation of Hemophilia and by members of the Haemophilia Patient Organisation in New Zealand.

A Consensus Statement on Key Global Issues Relating to Blood and Plasma

Author: Brian O'Mahony - Irish Haemophilia Society

There exists a broad range of differing opinion on key global issues relating to blood and plasma between stakeholders including patients, donors, blood authorities, industry and the not for profit sector. Under the auspices of the Plasma users coalition, PLUS, a consensus conference convened in Ireland in January 2010.

PLUS Member Organisations

•International Patients Organisation for Primary Immunodeficiency (IPOPI)
•World Federation of Hemophilia (WFH)
•European Haemophilia Consortium (EHC)
•Alfa Europe
•Idiopathic Thrombocytopenic Purpura Support Organisation (ITP)
•Hereditary Angiodema International (HAEI)
•Guillian Barre Syndrome Foundation International (GBS/CIDP).
Fifteen persons were in attendance representing the views of key stakeholders.

The agreed statement includes sections on patients, donors, sector relationships and global utilisation of donated blood and plasma. Specific points included:

Meeting the health needs of patients through a sufficient supply of safe and effective blood components and plasma products is the principal goal of the blood and plasma sectors.
•Patients whose continued health is dependant on the use of blood or plasma products have a right, through their representative organizations, to be consulted on any issue which may have an impact on the safety, efficacy or supply of the treatment they receive.
•The blood and plasma sectors and society in general should highly value all those who donate blood or plasma for the benefit of patients, recognize that donors perform a good action and treat donors with respect.
•There is a limit to the capacity of the blood and plasma sectors to ensure the safety of blood and plasma products through testing and processing alone. It is therefore important that measures to defer donors are based on a precautionary approach and underpinned by evidence based assessment where feasible. Donors must have donor deferral policies clearly explained to them.
•The blood product and plasma collection sectors should each take cognisance of the requirements of the other sector when collecting in a country or region and cooperation between the sectors should increase.
•The needs of patients should determine the optimal collection of blood or plasma.
•The feasibility of global utilisation of blood components, plasma and plasma intermediates collected and not required in the region where they are collected should be examined.


Vox Sanguinis

REPORT

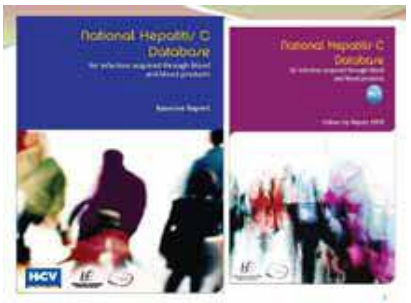
The Dublin Consensus Statement on vital issues relating to the collection of blood and plasma and the manufacture of plasma products

Endorsement

By May 31st, 2010
Statement had been endorsed by:
IPOPI, EHC, WFH, HAEI, GBS-CIDP, ITP Support Association, American Plasma Users Coalition (9 organisations) Canadian Hemophilia Society.
Supported in Principle with Qualification by:
PPTA, IPFA, IFRCRCS, Alliance of Blood Operators (Canada)

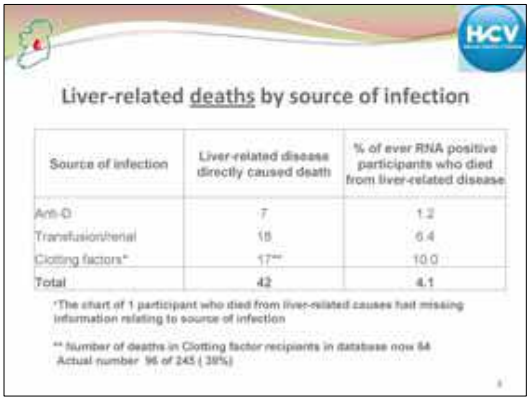
Not Endorsed by:
EBA, IFBDO
The agreed statement was an important step in establishing clear principles and in improving dialogue between sectors with competing interests but sharing a common purpose of providing safe, effective and sufficient therapy. During the Endorsement process, key areas requiring further dialogue were identified. These will be the subject of a follow up meeting in early 2011. The process and dialogue will continue.

Hepatitis C Progression and Treatment in the Haemophilia Population in Ireland



Approximately 1,700 people were infected with Hepatitis C through receipt of contaminated blood and blood products in Ireland; these include women infected through Anti D Immunoglobulin, recipients of blood transfusions, 245 people with Haemophilia and other blood clotting disorders and people who received treatment for renal disease. The Health Protection Surveillance Centre has set up a database to gather information on an ongoing basis on this group of people. The original database report was published in 2007 and a follow up report was published in 2009. Data is collected by looking through the medical charts and records of the individuals concerned.
Data was collected from 1,275 individuals:

- ❑ 787 (62%) were infected through contaminated Anti D
- ❑ 325 (26%) were infected through receipt of contaminated blood transfusions or treatment for renal disease.
- ❑ 157 (12%) were infected through contaminated blood clotting factors
- ❑ Of the 157 persons with Haemophilia or related bleeding disorders in the database 94% were male and 43% were co-infected with HIV
- ❑ The majority of people with Haemophilia were infected with Hepatitis C from the mid 1970's to the early 1980's. (if the year of infection was unavailable, the year that the patient first received clotting factors was used as a proxy). The median age of infection was 13.5 years; the median duration of infection was 25 years.
- ❑ On diagnosis 64% were RNA Positive and a further 13% had died prior to having a PCR Test for RNA.

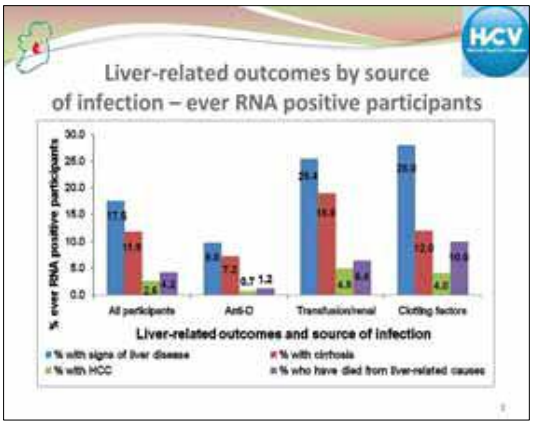


Mortality

- ❑ A total of 245 persons with Haemophilia were infected with Hepatitis C, including 106 who were co-infected with HIV – 96 of these have died as a result of HIV or Hepatitis C (39%)
- ❑ Two thirds of those who had died were co-infected with HIV, average age of death was 35.5 years for those with HIV co-infection and 54 years for those who were HIV Negative.
- ❑ 17 of the 157 (10.8%) persons with haemophilia on the database have died as a direct result of liver related disease. This is a higher percentage than the mortality in the transfusion / renal group (6.4%) or the Anti D group (1.2%)

Progression of Liver Disease

- ❑ Of those who were RNA Positive 28% had signs of severe liver disease, 12% had cirrhosis, 4% had Hepatocellular carcinoma (HCC) and 10% had died from liver related illnesses. This is a similar clinical picture to that seen in the transfusion and renal disease groups.
- ❑ The clinical picture is much less severe in the Anti D group
- ❑ 6 Patients with Haemophilia from Ireland have currently undergone liver transplants, 2 of these 6 have since died.
- ❑ HIV status and alcohol consumption were significant predictors of disease progression
- ❑ Of those with HIV co-infection, 31% had serious liver disease compared with 17% of those who were HIV Negative
- ❑ 45% of those who consumed more than 40 units of alcohol per week had serious liver disease compared to 18% of those who did not consume alcohol at that level



Treatment

- ❑ 12 of 14 (84%) individuals with Genotypes 2 or 3 achieved sustained virological response (SVR)
- ❑ 9 of 25 (36%) individuals with Genotype 1 achieved SVR
- ❑ The data is utilised in education programmes using newsletters, meetings and phone calls to persons with Haemophilia. They are informed of the factors with affect the progression of liver disease and especially the factors which they can influence including alcohol intake, weight and body mass index and decisions on treatment

Acknowledgements

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Education & Integration of the Entire Family into Programmes & Activities: The Irish Experience

Author: Debbie Greene – Irish Haemophilia Society

The family institution plays an immensely important role in the life of a person with haemophilia. The Irish Haemophilia Society endeavours to provide support and services to all members of the family from the youngest age. Our conferences and educational events include comprehensive specific programmes and activities for four separate age groups.

The separate programme age groups are:

Adults
Teenagers from 12 to 18 years
Children from 7 to 11 years
Children up to age 6

Three residential weekend conferences are organised annually incorporating specific programmes for all age groups. The adult programmes include lectures, debates and workshops. The teenage programmes include appropriate safe yet challenging activities with appropriate education sessions. The children's programmes include enjoyable group activities such as swimming and some educational elements introduced in an enjoyable context including the use of quizzes, competitions, puppets or short appropriate information sessions.

The leaders are generally those who have come through the programmes in the past. This approach involves the individual from early childhood. It increases the children's self esteem, enhances their personal growth and reduces any sense of isolation or difference they may feel by exposing them to peers with bleeding disorders from early childhood. It also gently introduces education about haemophilia in an enjoyable manner.

Total family involvement means that each member of the family benefits greatly from the interaction and contact with other families, which in turn builds community spirit, support and long lasting friendships.

For further information, visit our website:
www.haemophilia.ie