

HEMOPHILIA WORLD



Record attendance for Bangkok congress

Richard Andrews
WFH Communications Officer

2004 World Hemophilia Congress the biggest and most successful to date.

About 3,800 people from 120 countries gathered in Bangkok, Thailand, October 17-21, for the WFH's 26th World Hemophilia Congress. The record number

of participants included people with hemophilia, medical professionals, national hemophilia organizations, industry, and regulators. They gathered to share their

knowledge, exchange experiences and meet friends. "I feel like I'm here with my family," said a delegate from the Netherlands.

A royal opening

The five-day meeting was officially opened by Her Royal Highness Princess Mahachakri Sirindhorn, who welcomed delegates to Thailand. Participants were given a taste of Thai culture, including traditional dances and martial arts demonstrations, as well as a special tour of the Royal Palace and a visit to the revered Temple of the Emerald Buddha.

"The event was an historic occasion," says Congress President, Professor Parttraporn Isarangkura. "It was only the second time in 41 years that the congress was held in Asia."

"The Bangkok congress has opened up hemophilia awareness in Asia and will bring needed benefits to this part of the world," she says. "The meeting will lead not just to improvement in hemophilia care, but also to improvements in the treatment of all bleeding disorders and global clinical and laboratory practices."



Traditional Thai dancers.

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Record attendance for Bangkok congress

The latest hemophilia information

After hours of travel, delegates were warmly greeted by Thai smiles. At the high-tech congress centre, participants were offered an extensive program that included about 200 speakers, 700 submitted abstracts, and 55 sessions, as well as advanced medical sessions and multidisciplinary perspectives. "It fills me with delight to see so many people here dedicated to helping those with hemophilia," said a patient from southern India.

Session topics ranged from the latest developments on gene therapy and variant Creutzfeldt-Jakob disease to innovative education models and hands-on workshops for lab technicians.

Highlights of the program included:

- A symposium on vCJD risk for patients using U.K. clotting factor concentrates;
- Breakthroughs in non-invasive prenatal diagnosis of disease;
- Quality of life research in Europe;
- Assessment procedures for people recovering from bleeds.

In keeping with the needs of the region, a number of sessions also focused on the challenges faced by countries with limited economic resources. "The developing countries include most of the world's patients, but they lack adequate treatment to alleviate their suffering," says Professor Isarangkura.

Pre-congress sessions

A series of well-attended sessions was held in the day before congress for physiotherapists, laboratory personnel, nurses, psychologists, and social workers. In addition, patient leaders and youth from 83 countries participated in a three-day skills development workshop designed to help strengthen national hemophilia organizations. (See page 13.)

Positive responses

The WFH has received many positive responses from congress participants about the program and venue. "I received much valuable information that will help my son," said a mother from Mexico. "The organization, the sessions and the facilities were great," according to a US delegate.

"The biennial event has reached an important stage in its development," says Ross Robinson, WFH Director of Corporate Relations, Congress and Meetings. "The consistently high turnout for the last three congresses shows that if the facilities are there, we can now go anywhere in the world, be it Bangkok in 2004, Vancouver in 2006, or Istanbul in 2008."

"The world hemophilia community has demonstrated the strength of its commitment," says Miklos Fulop, WFH CEO and Executive Director. "Congress participants have proven they're willing to travel long distances and take time off work to get together and talk about improved treatment and care. The scene in the treatment centre summed it all up. Despite pain and other difficulties people showed that they were determined to participate in the sessions."

Tributes to volunteers

WFH volunteers were presented with awards at a ceremony honouring long term volunteers who have significantly furthered the WFH's mission to improve hemophilia treatment and care. (See page 9.) There was also a special tribute to Brian O'Mahony, who ended his 10-year term as WFH president in October.

The outgoing president received a standing ovation at a special farewell ceremony held during the congress, where friends and colleagues paid tribute to his achievements and commitment – while poking gentle fun at his Irish sense of humour. (See page 4.)

O'Mahony says he discovered a very strong bond in the hemophilia community globally that has been deeply motivating when faced with great challenges. "The realities and standards of care are so vastly different worldwide," he says. "Countries in the developed world are working to optimize care. Meanwhile in emerging countries, there is often no diagnosis and a much lower level of care and quality of life. There is so much work to be done."

As he stepped down from the helm, O'Mahony said his involvement with the world hemophilia community would not end. "I can close my eyes and still see the children and people with hemophilia that I've been privileged to meet in traveling to different countries. Improving hemophilia care everywhere will remain a major passion in my life."

U.S. attorney, Mark Skinner was elected the WFH's new president by representatives at the General Assembly on 22 October.

(See page 5.)

Princess Sirindhorn
opens Hemophilia
2004.



Congress delegates visit the
Temple of the Emerald Buddha.



The exhibit hall

Congress exhibitors showcased the latest hemophilia treatment products, services, and publications. Hemophilia associations from various countries set up their own booths in the WFH area, while a poster display featured numerous contributions from individuals and organizations on a wide range of hemophilia topics. A lively Thai marketplace offered everything from suits and scarves to carved elephants and carpets. The Thai patients group sold CDs of the congress theme song as well as other souvenirs.

Over the course of the congress, delegates added their signatures to the "Hemophilia Helping Hands" donation board located in the Wyeth booth. In response, the pharmaceutical company donated \$5,000 each to the WFH and the National Hemophilia Foundation of Thailand.



"Helping Hands" cheque presentation.

Congress delegates.



Goodbye Bangkok, Hello Vancouver

The congress ended with farewell banquet, a fireworks display, and a Thai cultural performance in a spectacular setting: the Royal Navy Convention Hall on the Chao Phraya River. Guests launched floral and incense offerings on the river in a traditional ceremony that signifies the floating away of negative influences.

With this special edition of *Hemophilia World* we focus on the highlights of Hemophilia 2004. Whether you attended the congress or not, we hope you are left with an impression of how the event unites the hemophilia community and works to improve hemophilia care worldwide.

Planning is underway for the next World Congress to be held in Vancouver in May 2006. (Visit www.wfh.org for details.) We hope to see you there. ■



Professor Isarangkura with budding Thai dancer, Clare O'Mahony.



Congress treatment centre.



Exhibition hall.

The State of the Art Book and the Book of Abstracts from the Bangkok congress are available on CD from the WFH and are posted on www.wfh.org

Tribute to Brian O'Mahony

Hundreds of well-wishers attended a farewell ceremony during congress for outgoing WFH president, Brian O'Mahony. Speakers praised his commitment, dedication, and achievements during the 10-year presidency. The following article is taken from the speech by Brian's friend and colleague, Dr. Peter Jones.

This tribute to Brian is a mix of pride, gratitude, and sadness. The pride of all those who have been privileged to work with him over the years. Gratitude, for what he has achieved for people with hemophilia and their families throughout the world. Sadness that his term of office comes to an end at tomorrow's General Assembly.

Let me try to give a flavour of the man. Now, Brian has two faults, or at least the only two faults that I know about. One, he makes the most appalling puns and jokes. Two, he is a superb manipulator.

But both faults have their good sides. He is always ready to make people laugh, even in the most harrowing of circumstances. Faced with his unfailing good humour it is easy to forget that he has hemophilia, and

that he suffers the bleeds, and the acute and chronic pain of that disorder.

Never, in the years that I have known him, have I heard him feel sorry for himself. Sure he'll shout and treat us to some choice Gaelic terminology, but he does not complain.

The good side of his skill at manipulation is that he almost always has the media at his mercy. Even the professionals succumb to his charm, especially the females, before realizing too late that it is Brian who has controlled the interview, not them.

Among the many friends who have written to us I have picked one to quote. You will see that I have done that for two reasons. Firstly, because of the eloquence of the language she uses in her tribute.

Secondly, because she is President of the Irish Republic.

Mary McAleese writes: "Brian's stringent, uncompromising professionalism and determination kept a small but unquenchable light burning through the darkest days that were faced by sufferers—a light that brought hope and healing to so many, which in turn deepened his vocation to protect victims and to advance medical research.

"Somewhere in all that research, campaigning and innovating treatments Brian fought for, he found time to develop the friendships and memories which have made the hard work, and at times heartbreaking work, so worthwhile."

But before we get too serious, another facet of Brian's life is that he's great fun to be with and to travel with. Enjoying a beer (or rather another beer) at two o'clock in the morning on a sidewalk in Sevilla; having a snowball fight on the way home from dinner in Montreal; reputedly making room for a goat to sit next to him on a flight somewhere to the east of Europe; searching for original film scripts on Hollywood Boulevard and only finding dubious women asking him for a light.

In conclusion let me quote Anne Morrow Lindbergh. She said "I do not believe that sheer suffering teaches. If suffering alone taught, all the world would be wise since everyone suffers. To suffering must be added mourning, understanding, patience, love, openness and the willingness to remain vulnerable."

Brian has all these qualities in full measure, and we are fortunate to have watched him use them all during his term as our president. ■

Brian O'Mahony and family at his farewell ceremony.



Commitment and experience drive new WFH president

Richard Andrews
WFH Communications Officer

More resources for hemophilia programs and cheaper treatment products are top goals for Mark Skinner.

"It's an exciting opportunity and the culmination of a lifelong passion to help people with hemophilia around the world," says Mark Skinner, the new WFH president.

The 44-year-old attorney from Washington, DC, was elected in October by the WFH General Assembly, during the Hemophilia 2004 World Congress in Bangkok. He replaces Brian O'Mahony who served as WFH president since 1994.

"The WFH enjoyed tremendous growth under Brian's leadership. The organization is now at a new stage. I'm confident that my experience will complement Brian's work," says Skinner.

A member of the WFH Executive Committee since 2002, Skinner was also president of the National Hemophilia Foundation (NHF) in the USA. The new WFH president has a strong background in public policy and advocacy for improved safety and supply of blood products. He belongs to the WFH Blood Products Safety, Supply, and Availability Committee, and has served on the NHF's Medical and Scientific Advisory Council (MASAC).

Skinner has been an active fundraiser, including a research campaign that raised \$5 million dedicated to finding a cure for bleeding disorders. He chaired the NHF Advocacy Committee that guided the Ricky Ray Hemophilia Relief Fund, which provided compensation for people with hemophilia infected with HIV from tainted clotting factor.

"One of the most significant challenges is the need to diversify the WFH's funding base and look outside the pharmaceutical industry for new sources of revenue," says

Outgoing WFH president, Brian O'Mahony, congratulates his successor, Mark Skinner (right).

Mark Skinner as a young boy (third from right) on a family outing.



Skinner. "I will use my background to secure more and diverse resources for the WFH."

"At the same time, we have to find ways to make safer treatment products more affordable for emerging countries. This will require greater collaboration with regulators and industry."

Growing up in rural Kansas during the 1960s with severe hemophilia A has made Skinner acutely sensitive to those with limited access to treatment. His early life experiences have had a lasting impact. In

1984, he began his advocacy involvement by helping to enact a state program for those without affordable treatment.

"When I was born, there was no treatment available apart from fresh frozen plasma. My hematologist was a four-hour drive away. I was wearing a leg brace by the age of six. Because of the knee damage, I slept in traction using a bucket of sand to keep my knees straight. I was told that I would never walk again unassisted.

"However, my parents instilled in me the idea that life is manageable and the world should be viewed in a positive light. They helped me to build a good life. I learned the importance of having a support group that included my immediate family as well as the extended family of a national hemophilia association. I have also been very fortunate to grow up in a country with access to advanced hemophilia care and treatment to correct my joints.

"I am pleased to be at a point in my life where I can give something back to the community. My passion runs deep. I remember the pain and frustration of my early life. There is no need for any child to go through that." ■

Highlights of Hemophilia 2004 medical

Carol K. Kasper, MD
Emerita Professor of Medicine
University of Southern California

Medical experts share knowledge of advances in hemophilia treatment.

The biennial WFH Congress in October was a resounding success. Congratulations to Professor Partraporn Isarangkura and her committee as well as to the WFH staff, all of whom worked very hard. It was difficult to choose among the many interesting simultaneous sessions.

Surgery in patients with inhibitors

Use of recombinant VIIa (rVIIa) has made "previously impossible surgery possible" said orthopedic surgeon Marvin Gilbert. Surgeons are "getting bolder," a large majority of results are good, but surgeons are unhappy with occasional poor results, blamed on inadequate hemostasis.

In the U.K., said Paul Giangrande, the dosage of rFVIIa for inhibitor surgery has been increased. So far, physicians are happier with the hemostasis afforded by higher doses.

Another approach, if rVIIa alone is inadequate, has been to use FEIBA and rVIIa together (for acute hemorrhages primarily, rather than for surgical operations). FEIBA contains prothrombin and factors IX and X. Harold Roberts explained that VIIa needs not only tissue factor but also factors V and X to convert prothrombin to thrombin. The addition of factor X, prothrombin, and VIIa to hemophilic plasma improves thrombin generation to near normal.

Coagulation testing

Dr. Trevor Barrowcliffe described the limitations of using only the one-stage assay in the clinical laboratory. Its accuracy in measuring very low levels of FVIII or factor IX, that is, in predicting disease severity, troubles him, as it does me. There are a great many different sources of each reagent used, as well as many different instruments. He is particularly concerned that many laboratories assay at only one dilution of the plasma sample. He is also concerned that many laboratories use duplicates instead of replicates (that is, two samples are taken from the same dilution, rather than from two separate dilutions),



Dr. Paul Giangrande presents an International Healthcare Volunteer Award to Dr. Carol Kasper.

and many laboratories routinely rely on standard curves stored in machines. (If a machine spews out a result, it has to be right, right? Wrong.) Many FVIII immuno-depleted plasmas may still contain a disturbing amount of FVIII, as demonstrated in thrombin generation tests. The thrombin generation test (TGT) was first described in 1953, and was standard in the laboratory in which I trained in the early 1960s. It has been made easier. Dr. Barrowcliffe likes to use activated factor IX as the activating trigger and also likes a fluorogenic substrate which does away with the need for subsampling. He uses continuous recording of thrombin generation so that one can observe the lag phase and peak thrombin produced. One can quantify thrombin generation by measuring the peak, the time to half-maximum, and area under the curve (AUC). The calibrated fluorometric method requires a special machine, which is easy to use.

One great advantage of the TGT, and the two-stage FVIII assay that was derived from it, is that very low levels of FVIII can be mea-

sured, as low as 0.1% says Dr. Barrowcliffe. He also notes that the recorded curve of thrombin generation becomes normal when a person with severe hemophilia A receives enough FVIII to raise his level to 30%.

Issues surrounding pregnancy

Jim Wainscoat of Oxford described progress in making diagnoses from fetal material in the maternal circulation. There are very few fetal cells in maternal blood, but free fetal DNA and RNA can be found and distinguished from maternal DNA and RNA, if the fetus is male. Maternally inherited fetal nucleic acids appear very early, by day 28 of conception in 80% of instances. The major utility of testing fetal material is to determine gender. In carriers of hemophilia, if the fetus is female, further tests of an invasive nature, such as chorionic villus biopsy, are avoided.

Stuart Lavery of London described pre-implantation genetic diagnosis, the process in which a very early embryo, formed by in-vitro fertilization, is biopsied. One can

Gene therapy session.



determine gender, look for chromosomal abnormalities and sometimes diagnose a gene disorder such as hemophilia, and then implant the healthy embryos into the uterus.

He emphasized the uncertainties of diagnosis with biopsy of only one or two cells from the embryo, and the uncertainties of the eventual effect of that biopsy on the children. (Although no ill effects have been seen so far.)

Dr. Lavery reported that sperm separation into X-bearing (female) and Y-bearing (male) is best with the flow cytometry method used in Fairfax, Virginia. Their outcomes are 76% success if a male is desired and 91% if a female is desired.

The role of plasma-derived FVIII-VWF concentrate

We heard again from German investigators that induction of tolerance in a patient with an inhibitor may be more successful on plasma-derived factor VIII (pdFVIII) with von Willebrand factor (VWF) than on purified pdFVIII or recombinant factor VIII (rFVIII) alone. The total number of patients is not large enough for a firm conclusion, but, if a patient is not responding to tolerance induction with a product containing only FVIII, then use of a pdFVIII with VWF might be considered.

The German-speaking countries have an ongoing previously untreated patient (PUP) surveillance project, and, as of June 2004, are following 278 hemophilia A and 40 hemophilia B patients. The 183 hemophilia A patients who have been treated are about evenly divided between pdFVIII and rFVIII. Of those on pdFVIII, about half use FVIII with VWF and half use FVIII without VWF. Of those on rFVIII, about 80% use a product with the full-length molecule.

Alessandro Gringeri of Italy reported on inhibitor experience with Emoclot,

the Italian FVIII-VWF concentrate introduced there some 15 years ago. He followed 70 patients with severe and 27 with moderate hemophilia A. The rate of inhibitors is 10% among patients with severe hemophilia and none have appeared in the moderately affected patients. The rate of inhibitors in true PUPs was 10% and in minimally previously treated patients was 6%.

Radionucleotide synovectomy

Radionucleotide synovectomy isn't new, but it has been slow to be applied around the world for hemophilic synovitis. I was excited to hear of the introduction of radionucleotide synovectomy over the past three years in new places. Sylvia Thomas reported on a series of 50 patients in Cuiaba, Brazil; Yangquiang Zhao on 25 patients in Beijing, China; and Kaan Kavakli on 65 patients in Turkey. All reported high rates of success in suppressing synovitis.

Whither gene therapy?

Don't give up. The future, says Thierry van den Driessche, includes new vectors including nano-particles containing genes, hepatocyte-specific targeting, site-specific genomic integration (to reduce the chance of exposing oncogenes), and new clotting factor molecules with improved secretion. The gene therapists haven't run out of ideas. Expense and regulations may be the limiting factors.

With the kind permission of the author, this article was excerpted from the November 2004 edition of The Hemophilia Bulletin. ■

Workshop demonstrates good laboratory practice

Laboratory experts conduct training session to improve diagnosis of bleeding disorders.

Biomedical scientist, Angus McCraw (U.K.) opened the packed workshop of more than 110 people by highlighting the importance of taking quality samples for blood coagulation in order to perform good tests.

Stacey Weston (U.S.A.) outlined some of the complications in the measurement of factor VIII assay. There is no globally used single reference standard for either the one-stage clotting assay or the chromogenic substrate assay. "It is important to be cognizant of the challenges in different settings," she emphasized. Therapies are beginning to be tailored to individual patients, which will be an improvement over the 'one size fits all' dosing regimen.

The workshop ended with demonstrations of the semi-automated coagulometer, factor VIII assay, and agglutination method for ristocetin cofactor. ■



Laboratory workshop with Angus McCraw (right).

Congress sessions focus on current safety and supply issues

Mark Brooker
WFH Public Policy Officer

VCJD highlighted in sessions dealing with safety, efficacy, and affordability of treatment for people with hemophilia.

Leading authorities at the Bangkok congress presented the latest findings on topics including variant Creutzfeldt-Jakob disease (vCJD) and risks to product safety.

In his plenary lecture on prion disease, Dr. James Ironside (U.K.) presented a comprehensive overview of the current knowledge about transmissible spongiform encephalopathies (TSEs), and their potential to be transmitted by plasma products. TSEs seem to be caused by an abnormally folded protein and can be transmitted from animals to humans, and humans to humans, in the form of vCJD. He continued the discussion later in the week at a special symposium on vCJD risk in U.K.-sourced plasma products.

Ironside explained that some people in the U.K. with vCJD were known to have been blood donors in the past. The recipients of those blood donations have been followed and two now

appear to have acquired vCJD through transfusion of packed red blood cells. One 69-year-old died of obvious vCJD in 2003. The second recipient died in 2004 of unrelated causes. An autopsy found signs of vCJD infection, although no symptoms.

Nine people in the U.K. who later also developed clinical vCJD had given a total of 23 blood or plasma donations that were pooled with other plasma to make factor concentrates. Many people with bleeding disorders received plasma products that included these donations. None has yet developed vCJD. For many years now, the U.K. has imported plasma from the U.S.A. to make U.K. plasma products. Recombinant products are now used in the U.K. as much as possible. The U.K. still makes plasma concentrates for rare disorders from U.S. plasma.

Dr. Paul Giangrande (U.K.) explained that the U.K. is now notifying all recipients

The absolute risk of vCJD transmission in plasma products is thought to be very low.



VCJD panel speakers (left to right) Dr. Bruce Evatt, Dr. Albert Farrugia, Dr. James Ironside, and Dr. Paul Giangrande.

of products derived from U.K. plasma from 1980 to 2001 that they are at risk of vCJD. The recipients are being told not to donate blood, tissues or organs and their general practitioners and dentists are to be notified. There is concern

that dental, endoscopic, and surgical instruments may be contaminated and there is no secure way to decontaminate them from the prions.

This has led to concerns about people with hemophilia being denied care.

Dr. Albert Farrugia (Australia) reviewed evidence about removal of prions from plasma during normal processing. Cryoprecipitation removes some infectivity, other precipitation processes such as the use of polyethylene glycol or glycine remove more, while chromatography or nanofiltration remove even more infectivity. The stickiness of prions likely contributes to the efficacy of the latter methods, but chromatography columns and filters must be changed often. Farrugia concluded that the higher-purity products, processed by

chromatography and/or nanofiltration, are safer than those produced with precipitation methods alone.

The absolute risk of vCJD transmission in plasma products is thought to be very low. All three experts at the symposium stressed that there is no known case of transmission of vCJD by a plasma product and no one with hemophilia has been diagnosed with vCJD.

Dr. Bruce Evatt (USA) chaired a broad-ranging session on the emerging and receding threats to product safety. This session included updates on inhibitors, risks in gene therapy trials, and the ongoing debate over the efficacy of national self-sufficiency policies.

At a session on the supply of safe products in the developing world, experts from Thailand, Malaysia, and Brazil described innovative methods used for hemophilia care despite economic constraints and the high prevalence of viral diseases such as human immunodeficiency virus (HIV) and hepatitis C (HCV). Thailand, for example, has developed a freeze-dried cryoprecipitate product that is manufactured locally for the hemophilia community. ■

New hemophilia care awards launched at congress

Paul Karwatsky

The WFH honours its volunteers and twinning partners.

New awards recognizing the contribution of volunteers to global hemophilia care were presented for the first time at the Bangkok world congress. The awards included the International Frank Schnabel Volunteer Award and the International Healthcare Volunteer Award.

The Frank Schnabel Award honours an individual living with hemophilia, or a family member, who has furthered the mission and goals of the WFH. The award was presented posthumously to Ashok Verma, who passed away earlier in 2004. As founder and Executive Director of Hemophilia Federation India, Verma was a torchbearer for the country's hemophilia movement. He campaigned tirelessly for improved hemophilia treatment and care, and motivated people to establish their own hemophilia societies. His efforts resulted in 56 patient chapters throughout India, as well as the establishment of hemophilia societies in Nepal and Bangladesh.

Dr. Carol Kasper and Professor Partraporn Isarangkura were jointly acknowledged for their work as healthcare professionals in the field of international hemophilia care. Isarangkura, a leading hematologist and medical pioneer, heads the Hemophilia Society of Thailand. She

established the country's first hemophilia treatment centre, which trains medical professionals from around the region. "Mother Pat" is known for more than 40 years of commitment to initiate and advance hemophilia treatment throughout Southeast Asia.

Kasper's award was also based on her volunteer work around the globe assisting patients and training medical professionals to help improve hemophilia diagnosis and care in their countries. The Californian specialist also shares her expertise in her extensive hemophilia related medical writing. Publications such as the *Registry of Clotting Factor Concentrates* and the *Hemophilia Bulletin* have benefited the hemophilia community for the past 20 years.

Other WFH volunteers were honoured with the previously established Inga Marie Nilsson Award, sponsored by Octapharma. Recipients included Dr. Koon-Hung Luke, Dr. Man-Chiu Poon, and Angus McCraw for their achievements in hemophilia diagnosis and treatment.

Luke and Poon were recognized for establishing the Hemophilia Treatment Centre Collaborative Network in China, a direct result of the twinning projects the

two Canadian doctors had pioneered in China. The two doctors also created a national medical registry, organized numerous hemophilia symposiums and training workshops, and expanded working relationships between hemophilia care workers across China.

Angus McCraw was recognized for decades of voluntary work around the world to improve the laboratory diagnosis of blood coagulation disorders. McCraw's recent achievements include the co-authoring of the WFH *Laboratory Manual* and the launching of a *Training the Trainers* program in 2001.

The WFH also recognized 2003's most exceptional hemophilia organization twinning and treatment centre twinning partnerships. A Twin of the Year Award was presented to the highly successful collaboration of the East Egyptian Hemophilia Centre and the Tennessee Hemophilia Clinic. The Jordan Blood Diseases Patients' Association's partnering with the Toronto and Central Ontario Regional Hemophilia Society was chosen as the hemophilia organization Twin of the Year. The Twinning Program is sponsored by Wyeth. ■



Professor Partraporn Isarangkura receives an International Healthcare Volunteer Award from Dr. Bruce Evatt.



Inga Marie Nilsson awardees, Dr. Man-Chiu Poon and Dr. Koon-Hung Luke.



Tribute to Ashok Verma, winner of the Frank Schnabel Volunteer award.

Extensive musculoskeletal program at Bangkok congress

Jerome D. Wiedel, MD
Kathy Mulder, physiotherapist
Co-Chairs, WFH Musculoskeletal Committee

New session format draws physical therapists, orthopedists and other specialists from around the world.

A new format for the musculoskeletal program consisted of four symposiums, each focusing on a particular topic. These included: The Ankle as a Target Joint; Functional Recovery of Bleeding Episodes of Muscle and Joint; Orthopaedic Surgery in Patients with Inhibitors; Joint Scoring Systems MRI and PT. Invited experts gave formal presentations followed by discussion

Orthopedist Marvin Gilbert (U.S.A.) observed that muscle bleeding is "the most undiagnosed and untreated injury associated with hemophilia." It's important to educate patients so they seek early treatment and to follow up with ultrasounds until the bleed is completely resolved, to avoid the risk of developing pseudotumours.



Physical therapy session participants.

Muscle bleeding is the most undiagnosed and untreated injury associated with hemophilia.

Ten sessions were devoted to musculoskeletal issues, including 43 paper presentations selected from abstracts submitted by the authors. The Henri Horowitz Award was presented to Goris Roosendaal (Netherlands) for his paper on Radiosynviorthesis Using Yttrium-90 Results in Adverse Direct Effects on Cartilage: A Human In Vitro Study.

A pre-congress workshop, conducted by Kathy Mulder (Canada) and Pamela Narayan (India), presented basic information

on physical therapy management of hemophilia to medical professionals new to this area. The workshop also featured case

presentations and discussions about the need for further research regarding safe and effective treatment methods.

Highlights of the musculoskeletal sessions included:

- An update on the Hemophilia Activities List by Frank von Genderen (Netherlands).
- An update on the International Joint Health Score. Therapists involved in this project include Nick Zourikian and Pam

Hilliard (Canada), Sharon Funk (U.S.A.), Britt-Marie Bergstrom (Sweden).

- A session on using functional milestones to guide rehabilitation during recovery from muscle and joint bleeds, chaired by Piet deKleijn (Netherlands).
- A reminder that not all pain is related to bleeds by Rebecca Dalzell (Australia).

Following the congress, Kathy Mulder was invited to give a lecture at the Faculty of Physical Therapy at Mahidol University in Bangkok. Physical Therapy for hemophilia is not well known in Thailand, and students and professors were keen to begin working with the Thai Hemophilia Foundation to develop treatment and education programs. ■

Multidisciplinary sessions extend their scope

Psychosocial, nursing, and new capacity building sessions benefit participants from both developing and developed countries.

Congress discussion group.



The importance of psychosocial, cultural, and religious issues in global hemophilia care was emphasized by patients, nurses, social workers, psychologists and other participants in 19 multidisciplinary sessions. The wide range of presentation topics included training programs in the Philippines, interactive clinics in Australia, a men's project in the U.K., sports camps in North America, and a Netherlands study showing the importance of peer contact among people with hemophilia.

"Psychosocial support for people with hemophilia is often overlooked, but is essential to hemophilia care," said Maureen Spilsbury, chair of the WFH Psychosocial Committee.

Two lively sessions involving the Up Front Theatre from the U.K. used drama to explore issues surrounding adolescents and hemophilia. Innovative approaches were also highlighted in a session on the universal challenges created by the transition of adolescents from pediatric to adult services.

Quality of life

During the pre-congress psychosocial workshop, Dr. Sylvia von Mackensen (Germany) focused on quality of life (QoL) research in Europe. She emphasized the importance of factors that influence physical, mental, emotional, social, and functional well-being, including treatment modalities, living conditions, and socio-economic status.

At a multidisciplinary session, presenters spoke about various tools for measuring QoL. Elena Lopatina compared QoL between patients with and without home treatment in Russia, while a U.K. study by Karen Beeton looked at the QoL of parents of children with severe hemophilia.

Nancy Young of Canada described QoL measures as "attempts to identify the gap between one's expectations of life and their actual experiences." She outlined the ways in which children played a role in the development of the Canadian Hemophilia Outcomes - Kids Life Assessment Tool (CHO-KLAT). "Children's participation in the design and testing process is really what gives this tool its value," she said.

A traditional approach

The multidisciplinary sessions also included presentations from various developing countries who presented their perspectives on a wide range of issues and shared valuable information. Presenters from Thailand offered one form of treatment for pain and stress management that could help

people in both developing and developed countries.

Evidence of the benefits of meditation was presented in the Mind-Body Connection session, which drew more than 100 participants, including doctors, social workers, and patients. At the end of the session, they joined in a 10-minute meditation with a Buddhist monk.

Pre-congress workshop for nurses

Speakers from around the world participated in a well-attended pre-congress nurses day, which opened with a plenary session on genetic counselling in hemophilia. The program was then divided for new nurses and advanced nurses. The new nurse program included a hemophilia overview, history taking, treatment options, inhibitors, and management of musculoskeletal bleeds. The advanced program covered case study presentations and studies in more unusual topics including combined von Willebrand disease and severe hemophilia B, and prophylaxis in a neonate with severe factor X deficiency. *(Please see page 12.)*

“The nurses day enables us to update on what our colleagues are doing and also allows the networking we need to support each other working within the specialty of hemophilia.”

Susan Hook (Scotland)



WFH Nurses Committee Chair, Lara Oyesiku, serves tea during a session break.

The afternoon program was split into an adult session and a pediatric session. Topics included complementary care, low factor VIII in women, desensitization of a factor VIII inhibitor, bridging the gap between childhood and adulthood, and compliance and understanding one's own disease.

Elaine Sandoval from Brazil presented her experiences of training children to self infuse factor concentrate from the age of three years. The benefits of starting self-infusion at this early age for the child and family included improved quality of life, and reduced hospital attendance. Photos showing a four-year-old self-infusing inspired many to consider a similar program.

Presenter Susan Hook (Scotland) says the long trip to Bangkok was worth it. “The nurses day enables us to update on what our colleagues are doing and also allows the networking we need to support each other working within the specialty of hemophilia.”

Capacity building

For the first time, the congress program included sessions designed to help organizations build sustainable hemophilia care around the world. In eight, well-attended sessions, speakers from patient and

medical groups shared their experiences of working with each other, strengthening their organizations, and securing greater access to treatment products.

Adolfina Berges described the importance of medical/patient cooperation to care delivery in Mexico, Magdy El Ekiaby spoke about obtaining government support for a

national hemophilia care program in Egypt, and Dr Flerida Galsim-Hernandez outlined the patient organization's role in shaping the Philippines national hemophilia care program. ■

Ruud Bos, Anna Farrell, Susan Hook, and Dr. Sylvia von Mackensen contributed to this article.

IN MEMORIAM

Douglas Piquinela

1924-2004



The hemophilia community was saddened to hear that Douglas Piquinela, president of the Hemophilia Association of Uruguay, passed away while attending the World Congress in Bangkok.

Known for his high energy, outstanding leadership, and lobbying skills, Piquinela helped develop national health care for hemophilia patients in Uruguay. He worked with the government to implement the WFH's Operation Access program in 1999, leading to greater access to factor concentrates and reduced hospital time for people with hemophilia.

Born with severe hemophilia, Piquinela overcame many obstacles to complete his schooling and lead a “normal” life with a family and employment. He loved to play basketball and quipped that his main obstacle was his small stature.

“Despite the challenges, it's a tribute to his commitment to the world hemophilia community that he was determined to travel so far to attend the congress,” says Cesar Garrido, from Venezuela.

Piquinela's daughter, Berenda, flew to Bangkok to be with her father in hospital. ■

Leading the Way

Laurie Blackstock
WFH Education Officer

More than 120 leaders of national hemophilia organizations boost skills and share development ideas at a pre-congress training event in Thailand.

Hemophilia leaders from 85 countries spent three days in October sharing questions and experience in building and sustaining a vibrant hemophilia organization. Sessions covered topics including pharmaceutical relations, developing a national patient registry, providing psychosocial support, and media relations.

The global training program is held every two years prior to the world congress to help leaders of WFH national hemophilia organizations improve their skills and knowledge in areas related to organizational development.

Bangkok participants met the WFH presidential candidates, received information on recent WFH activities, and swapped ideas while strolling through orchids and palm trees on the grounds of the Rose Garden Hotel just outside the city. At the youth meeting, outgoing WFH president, Brian O'Mahony, encouraged participants to make their voices heard at the national level.

So what did people learn? Ramana Murthy of the Hemophilia Federation of India says he will put some new information into action immediately. "I will talk to members and executive about going beyond the government and approaching philanthropists, other organizations, and public interest groups about helping to improve hemophilia care." Murthy feels that the session on promoting emotional health will help him facilitate a discussion on this sensitive topic with members of his own hemophilia chapter.

Several participants said they now understand the perspective of journalists and broadcasters through the workshop on media relations. Those who attended the workshop said they felt it better prepared them for interviews. Participants hope to generate more press coverage about hemophilia and build a larger network of connections based on tips from the media experts from Latin America, who acted as facilitators.

During the highly interactive workshop on media relations, participants examined press kits and practiced interview skills from the perspective of both the media and the hemophilia organization, each representative making sure that they requested or gave relevant information.

The workshop on preparing organizations for the future showed that some challenges for hemophilia leaders differed according to factors such as local customs, the organization's level of development, the country's level of national health care, and the extent of support from the ministry of health. However, several first-time attendees were comforted to learn that they shared many of the same obstacles and that, in some cases, the obstacles were eventually overcome. Facilitators emphasized the need to plan for leadership change by expanding the membership base with people of all ages, providing training, and assessing whether any of the organization's policies or practices limited growth.

Even the facilitators benefited from the training event. "These workshops help me put order to ideas about issues that are of great importance to our organizations, and thus it becomes easier for me to put them into practice," says Héctor Beccar Varela, a facilitator from Argentina. "We will be able to apply the concepts we shared and learned about the participation of youth, volunteers, and the orderly incorporation of new members on the board."

Declan Noone, a youth delegate from Ireland, says that the information about succession planning was especially useful for getting younger members actively involved through camps. "The highlight for me was the youth meeting and the ideas that came out of that, but also the chance to speak at my limited level of knowledge without feeling like I was interrupting. I have returned home with many new ideas and full of energy and enthusiasm for the future." ■



The training program begins with a getting-to-know-you exercise.

General Assembly highlights WFH growth and change

Miklos Fulop
WFH CEO and Executive Director

The WFH General Assembly brings together national hemophilia organizations from around the world to set future directions.



Tamara Grigorievna Rubalova of Kazakhstan accepts WFH membership on behalf of her national hemophilia association.



Outgoing WFH President, Brian O'Mahony, congratulates Yefdi Amlak on Eritrea's new membership.

The United Nations Conference Centre in Bangkok was a fitting locale for this international gathering. It was my first Assembly, and as I looked out at all the delegates, I was struck by the size and strength of our organization.

Our network grew significantly with the election of seven new member organizations from Botswana, Eritrea, Iraq, Kazakhstan, Moldova, Oman, and Qatar. The total number of WFH member organizations is now 107.

Safety and Supply

Key issues raised at the assembly included recent concerns over treatment products, including the decision by health authorities in the United Kingdom (U.K.) to consider people with hemophilia and other congenital bleeding disorders "at-risk" for variant Creutzfeldt-Jakob disease (vCJD). Delegates urged the WFH to ensure that the rights of people with hemophilia are not violated.

Another key issue was the maintenance of factor VIII and factor IX on the World Health Organization's (WHO) Essential Medications list. The factor concentrates may be deleted from the list in March, and delegates signed a letter against the removal of these treatment products.

Congress 2008

The Turkish and Italian hemophilia associations both gave compelling presentations to host the 2008 world congress. The vote was close, but Turkey won and Istanbul will be the site of the 28th WFH International Congress. "We're very happy," said a jubilant Turkish delegate.

New Executive Committee

Another highlight of every General Assembly is the election of new Executive Committee members. This year was no exception, as there were seven positions up for renewal, including that of president.

Mark Skinner was elected the WFH's new president, and two vice-president positions

were won by acclamation. Dr. Paul Giangrande (U.K.) was re-elected VP medical, and Rob Christie (Australia) was elected VP finance.

Four other executive committee members were also elected. Dr Alison Street (Australia) and Dr Paula Bolton-Maggs (U.K.) were elected as medical representatives, and in the lay member category, David Page (Canada) was re-elected and Aliakbar Tchupan (Iran) was elected.

At the executive committee meeting following the general assembly, Gordon Clarke (Northern Ireland) was appointed vice-president for NMOs, Dr. Bruce Evatt (U.S.A.) was re-appointed VP developing world, and Mohamed Aris Hashim (Malaysia) was co-opted as a lay member.

Departing executive members

A fact of life of any organization is the coming and going of various board members. This year, the terms of a number of key executive committee members ended. I would like to acknowledge and thank them for their hard work and dedication. They are: VP finance, Ken Poyser, Dr. Peter Jones, and Dr. Sam Schulman.

In his 10 years as president, Brian O'Mahony has used his incredible enthusiasm, boundless energy, and great leadership qualities to help transform the WFH into the successful organization it is today.

Throughout the General Assembly many delegates and staff thanked Brian for his contributions, and some even asked him to stay on. Brian reminded the Assembly that he will continue to volunteer for the organization and work with the new president to make the transition a smooth one.

"It has been a privilege and an honour to serve you as president," he told the Assembly before formally handing over the presidency to Mark Skinner. ■

THANK YOU to congress volunteers

Ross Robinson

WFH Director Corporate Relations, Congress, and Meetings

Organizing a WFH congress requires hundreds of dedicated volunteers, who contribute valuable time and expertise. Their commitment to our goal of improving hemophilia care is what drives them. The volunteers bring ideas from around the world, to reflect the interests, strengths and diversity in the world of hemophilia.

Everyone can be proud of the final result, which by all accounts was a great success. The Bangkok program was informative and educational. Comments have been very positive.

In this big group of volunteers (numbering over 300) there are many people who should be named. Many of them are listed in the final program and on our web site. At the risk of not including everyone, I would like to mention several people who made enormous contributions.

Dr. Paul Giangrande and David Page recruited key opinion leaders (doctors, nurses, physiotherapists, patient group staff, etc.) to construct a very thoughtful congress program. After that they recruited 200 resourceful speakers to freely share their ideas and expertise with delegates.

Professor Parttraporn Isarangkura worked many, many hours and traveled to numerous meetings. Imagine her shock to be caught in the biggest snowstorm Philadelphia had seen in decades. Her pride in

Thailand and desire to see improvement in hemophilia care inspired many, including myself, to work very hard for the success of the congress. By bringing this world event to her country she has shown the importance of hemophilia to Thailand's government, patients, and medical professionals. Hemophilia care will advance in Thailand, as well as in the region, as a result.

Within the WFH, volunteers and staff banded together to professionally organize one of the best ever WFH congresses. Despite his numerous commitments, Brian O'Mahony devoted considerable time to work on WFH congress. He has been doing so since 1994. He was a great proponent to make the changes necessary to take this very complex event outside the usual congress venues of North America and Europe.

The congress was also made possible with the generous support of our industry partners. Our very generous congress sponsors were: Baxter, Bayer, Novo Nordisk, Octapharma, RAAS, Wyeth, and ZLB Behring.

See you in spectacular Vancouver May 21-25, 2006. ■

Professor Parttraporn Isarangkura's pride in Thailand and desire to see improvement in hemophilia care inspired many.

Thai hemophilia association volunteers gather at their congress booth.



Calendar of Events, 2005

International Symposium on Women's Health Issues in Thrombosis and Haemostasis 4-6 February 2005 -

Budapest, Hungary
Information: Kenes International
Tel: +41 22 908 0488
Fax: +41 22 732 2850
whith@kenes.com
www.kenes.com/whith

Taller de Hemofilia en el V Congreso Nacional y VII Jornada Latinoamericana de Hematología, Immunología y Medicina Transfusional 16-20 Mayo 2005 -

Cuba, La Havana
Palacio de las Convenciones
Información:
Dr. Jose M. Ballester
Correo electrónico:
ihidir@hemato.sld.cu

10th Congress of the European Hematology Association 2-5 June 2005 -

Palexpo, Geneva, Switzerland
Information: Eurocongres Conference Management
Tel: +31 20 679 3411
Fax: +31 20 673 7306
eha2005@eurocongres.com
www.eurocongres.com/eha2005

34th Annual Scientific Meeting of the International Society for Experimental Hematology 30 July - 2 August 2005 -

Glasgow, Scotland
Information: ISEH
Registration
Tel: +1 202 367 1173
Fax: +1 202 367 2173
iseh@smithbucklin.com
www.iseh.org/meetings/2005.cfm

XX Congress of the International Society of Thrombosis & Haemostasis (ISTH) 6-12 August 2005 -

Sydney, Australia
Tel.: +61 2 9241 1478
Fax: +61 2 9251 3552
isth2005@icmsaust.com.au
<http://www.isth2005.com>

13th National Haemophilia Conference - Integrating Knowledge and Practice 30 September - 2 October 2005

Melbourne, Australia
Tel: +61 3 9885 7800
Fax: +61 3 9885 800
hfaust@haemophilia.org.au
www.haemophilia.org.au

Congreso Latino Americano Hemostasia y Trombosis 3-6 November 2005 -

Viña del Mar, Chile
Tel.: +56-2-274-6714
congresoclhtchile2005@terra.cl

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The activities of people living with hemophilia and their organizations are important to everyone in the global hemophilia community.

We welcome stories, letters, and suggestions for articles. Please send them to:

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