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Hemophilia 2008 WORLD CONGRESS
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HEMOPHILIA DAILY
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Changing the history of hemophilia

Yildirim Çelik

WFH president Mark Skinner addresses delegates at the opening plenary

WFH president Mark Skinner highlights 45 years of progress

WFH founder Frank Schnabel's first words to the delegates assembled in Copenhagen in1963 still ring true today, WFH president Mark Skinner said during Monday's opening plenary. "The threat to the life of just one hemophiliac would be sufficient reason for us to travel to this meeting."

Forty-five years later, Skinner said, WFH has established itself as the cornerstone of global development. Its flagship program, the Global Alliance for Progress (GAP) addresses all five areas of the WFH Development Model: ensuring accurate diagnosis; getting government support; improving care delivery; increasing the availability of treatment product; and empowering patients by building a strong patient organization.

WFH's success in bringing about long-term, sustainable improvements in care for people with bleeding disorders is supported by data from the organization's annual Global Survey. According to the survey, the number of people with bleeding disorders identified worldwide has increased from 103,000 people in 65 countries to 219,000 in 101 countries. The availability of factor concentrates has increased to over 1.25 IU per capita worldwide (with GAP countries reporting a total cumulative increase of 467 million IU in the past five years). Over 130 million IU of factor have been distributed to 69 countries since the WFH launched its humanitarian aid program in 1996 and hundreds of healthcare professionals have received training through the WFH fellowship program.

"Daily, the work of the WFH is making a difference in the lives of individuals with bleeding disorders around the world," Skinner concluded. "Let us pause today and take great pride in what we have achieved by working together these past 45 years, and realize that there is great hope in what we will achieve together in years ahead."

Early treatment of target joints reduces arthropathy

The primary goal for all musculoskeletal health practitioners is to prevent joint bleeds and subsequent damage to synovial tissue, to ensure that children with hemophilia reach adulthood with optimal joint health, said Adolfo Llinás (Colombia). However, there is some controversy about when a joint has become a target joint and how long it takes for a joint to suffer irreversible damage.

"There is now a lot of data that shows that joint injury happens a lot earlier on than previously thought," he stated. "It is clear that musculoskeletal joint bleeds must be kept to a minimum to prevent permanent inflammation of the synovial tissue… We should be treating target joints expeditiously, but in reality we have a tendency to wait and to delay considering the fact that it has become a target joint."

There is generally the expectation that when fluids are removed from a joint, it returns to normal volume. In fact beneath the epidermal layers the synovial lining remains thickened and inflamed, adding volume to the joint. A joint that does not return to normal between bleeds has become a target joint and is in a state of chronic synovitis, he said.

Synovitis is very harmful to joints as it produces large deposits of iron that cause synovial proliferation, induces angiogenesis and releases inflammatory cytokines, contributing to articular cartilage destruction and injury to bone and ligament.

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von Willebrand disease still underdiagnosed

von Willebrand disease is the most frequent bleeding disorder but also the most undiagnosed, Ann-Marie Nazarro (US) told this morning's concurrent session, von Willebrand Disease: Issues for Men and Women. The WFH's Global Survey has identified 50,000 people with VWD, she said, but estimates are that between 300 and 400 thousand actually have it. "That tells us how much is still to be done in terms of identification, diagnosis, and connection to appropriate care."

Both men and women are affected by VWD but women tend to be more symptomatic because of the challenges of monthly menstruation, ovulation, childbirth, and various gynaecological pathologies, according to Rezan Kadir (UK). "If VWD is not diagnosed, each of these regular events in a woman's life can be complicated by bleeding."

But women aren't the only ones who experience severe VWD. Martyn Cooper (UK) is a 57 year-old man with Type 2A VWD. His condition causes him to have recurrent GI bleeds; he requires frequent transfusions and many investigative and intrusive medical procedures – most of which have been unsuccessful. In 2007 and 2008, he was admitted to hospital over 40 times.

Cooper became very emotional describing the huge impact his illness has had on his life. His first wife left him because she didn't want to "care for an invalid." His employer of many years dismissed him because he had such frequent bleeding episodes. "I have little income and most frequently can't work; retirement is likely to be pretty bleak... I ask myself do I keep trying the new treatments? Do I keep pushing? Unless I give myself a chance, I don't have too much left. So I keep pushing uphill, whether it works or not."

Sylvia von Mackensen (Germany) presented studies underscoring the importance of having good tools to assess quality of life issues for people with VWD. "It should not be left out because it's a soft issue; it's a must-have... If we understand health-related quality of life, then we can positively impact people like Martin and that positively impacts treatment outcomes."

Early treatment of target joints reduces arthropathy

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There is evidence to support the concept that secondary prophylaxis limits but does not abolish the progression of bleed-related arthropathy. Furthermore, it appears that incremental joint damage occurs with delays in the initiation of treatment. A Dutch study by Fischer et al demonstrated an 8% deterioration in the Pettersson score in children for every year prophylaxis initiation was delayed after the first joint bleed.

Llinás noted that individuals with hemophilia are a heterogeneous group, and the severity of synovitis and resistance to treatment will depend on a number of variables, such as presence of inhibitors, age, the stage at which articular bleeds develop and when treatment is started.

Program Updates

Tuesday

B.1.4 Going Beyond: Models to Promote Education, Training, and Employment
Michael M. Schneider will be presenting *Hemophilia and the ability to work, self confidence, finding a job, and a partner in Germany.*

Wednesday

C.1.5 How Elderly People With Bleeding Disorders Manage Their Condition
June Ward will chair in place of Susan Hook.

C.1.6 Global Impact of Twinning
Susan Cutter will present *Emerging Medical Centre Twin.*

Poster Exhibit

Manuela Carvalho's poster, Elective total hip replacement under rFVIIa cover in a patient with severe Hemophilia A and inhibitors, although not in the Final Program, will be displayed at the Exhibit Hall until the end of the day.

Yildirim Çelik

Wyeth presented the WFH and the Hemophilia Society of Turkey with cheques for USD 10,000 each as part of the Helping Hands initiative

On his bike to raise hemophilia awareness

It took László Kiss a little longer than most delegates to travel to Hemophilia 2008. That's because he covered more than 650 kilometres through three countries by bicycle to raise awareness of hemophilia and advocate for improvements in access to products and treatment.

Kiss lives in Sfantu Gheorghe, Romania, and his six-year-old son has severe hemophilia. "There are many problems trying to live with hemophilia in my country," he says. "The factor supply is only about 0.8 units per capita, which is not adequate."

"I wanted to do something to bring attention to the seriousness of the problem, not just for my family but for all the families living with hemophilia in Romania."

The problem of inadequate factor supply is compounded by public misunderstanding of hemophilia and a medical community that is often not up-to-date on the latest science and treatment developments, he said.

Kiss left his hometown on May 17 and travelled through Bulgaria to Turkey. Along the way, he was accompanied by many cyclists, who wanted to show support for his campaign. In Bucharest, he met with the Romanian Minister of Health and presented him with a petition demanding improved access to products and treatment. Kiss described the discussion as constructive and said the minister was open to more "bilateral communication" with the hemophilia community about their needs and unique challenges.

Kiss is secretary of his local chapter of the Romanian Hemophilia Association (RHA), which he says is "at the crest of a wave of activity and success." He enthusiastically describes many projects and campaigns aimed at extending treatment, improving product supply, and enhancing public and medical awareness of hemophilia in Romania.

The struggle to raise awareness and improve the lives of people with hemophilia is very personal for Kiss. "Adequate access to factor would change our lives. With factor my son can go to kindergarten; without it, he must stay home. The scarcity of treatment products reduces my son's worlds and limits his experience."

Lásló Kiss on his cross-country trip from Romania to Istanbul

Announcements

Please note that lunch and light snacks are available during Congress hours at the **Food Court**, located on the lower level of Rumeli Hall (one level below the Exhibition Hall).

To accommodate people with limited mobility:

- A row of accessible seats has been reserved in the main plenary hall (Anadolu Auditorium)
- There is elevator access to the Exhibition Hall
- An elevator is available from the sidewalk in front of the ICEC to the Military Museum walkway
- Please give people with limited mobility priority use of the elevators

Can we count you in?

Show your support of the WFH and its work in three easy steps:

1. **Stop by the WFH Resource Centre in the Exhibit Hall to make a donation in support of Treatment for All.**
2. **Add your name and wish to our Treatment for All donor wall.**
3. **Pick up a sticker and wear it to show your support.**

All donations will be used to support WFH programs and activities to improve care for people with bleeding disorders worldwide.

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Treatment for All

Prophylaxis beneficial but clinical challenges persist

The benefits of prophylaxis in preventing bleeds and the development of arthropathy in patients with severe hemophilia has long been demonstrated. But questions and challenges remain concerning its initiation, optimal frequency, and dosage regimen, stated Victor Blanchette (Canada).

Prophylaxis offers tremendous results for patients with severe hemophilia. Left untreated, patients suffer pain, swelling, limitation of movement, and eventually joint damage, he noted.

In 2006, the European Paediatric Network for Haemophilia Management devised definitions for primary and secondary prophylaxis. Primary prophylaxis consists of regular continuous treatment started after the first joint bleed and before the age of two, or before the age of two without previous joint bleed. Secondary prophylaxis consists of regular continuous long-term treatment started after two or more joint bleeds or after age two, or intermittent short-term treatment for frequent bleeds.

Blanchette reported that studies from Sweden and elsewhere endorse initiation of primary prophylaxis with target trough factor levels above 1% between doses. Compliance with the prophylaxis protocol is essential for positive musculoskeletal outcome, he asserted.

Kathelijn Fischer (the Netherlands) noted that hemophilia requires lifelong treatment, which makes prophylaxis difficult to study and manage. "In the progression from child to adult, patients experience physical changes with growth, susceptibility to cartilage damage, changes to bleeding pattern, and mental changes."

Manuel Carcao (Canada) described treatment complications that can arise with individuals who develop high titer inhibitors. Some patients can be successfully tolerized with immune tolerance induction therapy, but others continue to have persistent recurrent bleeds and joint damage that are difficult to treat, resulting in diminished quality of life. He described how bypassing agents such as FEIBA and rFVIIa can bring a significant reduction in the number of bleeds in inhibitor patients.

Sessions to watch for

PL04 New scientific and technical advances regarding hemophilia and other bleeding disorders continue to arise at a staggering pace. In a world of limited resources several significant ethical issues, relatively new to hemophilia-related clinical research, arise. These will be explored in today's second plenary session.

The presenter is Donna DiMichele and the plenary will be chaired by Paul Giangrande.

It takes place in the Anadolu Auditorium at 9:45.

B1.5 Planning for emergencies like a pandemic or natural disaster requires a great deal of time, cooperation, and coordination between all levels of governments, the community, multiple agencies, and families. Learn about the particular challenges that emergency preparedness presents to the global bleeding disorders community.

Presenters are Pam Wilton, Gillian Kernaghen, and Mateus Haun.

The session takes place at 11:00 in Topkapi A.

B2.1 The management of carriers and babies with hemophilia can be complex. New developments make preimplantation genetic diagnosis possible but it's extremely important that counselling and medical support accompany the testing. Treating young babies also poses particular challenges. These issues will be explored at 3:00 in the Anadolu Auditorium.

Presenters are Alison Street, Stuart Lavery, and Rolf Ljung.

B2.4 The benefits of activity, sports, and exercise are well-documented for people with bleeding disorders, but the psychosocial impacts of limitations to physical activity are also significant. These will be explored from the perspective of both consumers and therapists.

Presenters are Angela Forsyth, Stephen Weisser, Pamela Narayan, and Susan Cutter.

The session takes place at 3:00 in Topkapi A.

In the Halls

We hope to learn a lot here. We are nurses, so it always helps to get practical hands-on information. The physiotherapy sessions are always very interesting and useful. It's always a pleasure to learn new things that help in our daily lives and meet new people who work in this field.

-the Netherlands

Even within Europe, there are really different levels of treatment from country to country. I want to hear about how people are doing in other countries and how they have managed to improve things. The science is important, but figuring out how to use that science for patients in all countries is important too.

-Romania

As a scientist, I am anxious to hear about the amazing advances in genetic diagnosis and potential therapies. At the same time, as a clinician, I am interested in more practical approaches to treatment that are feasible for patients in developing countries.

-India

There's no history of hemophilia in our family, but we have two sons with hemophilia A via a sporadic genetic mutation. We want to hear as much as we can about treatment, but also to learn more about the genetics. We are active in our national hemophilia organization so it just makes sense that we are here.

-Italy

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