

# HEMOPHILIA WORLD

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[www.wfh.org](http://www.wfh.org)



## WFH appoints John E. Bournas as new CEO/ Executive Director

*Elizabeth Myles*

WFH COMMUNICATIONS AND PUBLIC POLICY DIRECTOR

The World Federation of Hemophilia (WFH) is pleased to announce the appointment of John E. Bournas as CEO/ Executive Director. Bournas brings over fifteen years of senior managerial experience in the healthcare and not-for-profit sectors and international experience as a diplomat. His areas of expertise include management, business development, healthcare advocacy, and cultivating relationships with stakeholders at the highest level of government. Bournas started his new position on January 25.

"The WFH is delighted to welcome John Bournas to our team," said WFH president Mark W. Skinner. "He brings a unique combination of skills and an international perspective that will be integral to achieving our strategic goals in the coming years."

Prior to joining the WFH, Bournas was senior director of international affairs with the American College of Cardiology Foundation, the largest international professional society for cardiologists. He was responsible for global strategic planning, international memberships, partnering with

Bournas brings over fifteen years of senior managerial experience in the healthcare and not-for-profit sectors and international experience as a diplomat.

national healthcare societies, global advocacy, and diversifying the organization's revenue stream through engagement with its corporate partners. Before that, Bournas was senior director-international with Cardinal Health in the United States, a Fortune 19 company that specialized in improving the cost-effectiveness of health care.

Bournas has also worked and travelled throughout the world developing relationships and bridging gaps between developed and developing countries. He worked as a diplomat in Chile, Australia, and Japan, overseeing international trade and development projects. The son of Chilean and Greek parents, he was raised in Chile and the United States. He speaks Spanish, French, Portuguese, and some Japanese and Greek.



John E. Bournas

Bournas holds a Master of Business Administration degree from Macquarie University in Australia, a Master of Arts in Political Science from Fordham University in the United States, and a Bachelor of Arts in International Studies from Fairleigh Dickinson University in the United States. He is married and has four children. ■

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**WFH** 50 YEARS OF ADVANCING TREATMENT FOR ALL

WORLD FEDERATION OF HEMOPHILIA  
Fédération mondiale de l'hémophilie  
Federación Mundial de Hemofilia

# A tribute to Mark W. Skinner

*Elizabeth Myles*

WFH COMMUNICATIONS AND PUBLIC POLICY DIRECTOR

Since he was first elected as the World Federation of Hemophilia (WFH) president in 2004, Mark W. Skinner has become the face of the WFH. His term as president ends in July 2012, so it is fitting to highlight some of his accomplishments.

Mark's focus, first and foremost, is on the patient and he works tirelessly to make things better for people with bleeding disorders. As a child growing up in the 1960s, Mark experienced the pain and despair of bleeds with little treatment, and it became his mission to make life better for others. "No one should have to suffer the pain I endured as a child," he said. Over the last eight years Mark has travelled to every region of the world, visiting 58 countries, and has met with thousands of people with bleeding disorders, bringing the message of hope for a better life.

One of Mark's first major initiatives as president was to undertake a strategic planning exercise in 2005, which led to the identification of a new vision for the WFH: Treatment for All. One day, treatment will be available for all those with inherited bleeding disorders, regardless of where they live. Treatment for All means more than just treatment products; it means proper diagnosis, management, and care by a multi-disciplinary team of trained specialists. It means safe, effective treatment products are available for all people with inherited

bleeding disorders. It means expanding services beyond hemophilia to those with von Willebrand disease, rare factor deficiencies, and inherited platelet disorders.

Mark's personal experiences with the AIDS pandemic in the 1980s raised his awareness of the need for patient involvement in government oversight and the role of the patient advocate to protect oneself. This continued at the WFH, as he played a key role in promoting the need for patient participation on national hemophilia committees and in introducing, together with Gordon Clarke, a new advocacy training initiative to help WFH national member organizations make the case for better care to governments.

Mark's extensive fundraising experience helped strengthen the WFH's financial position and diversify its funding. During Mark's two terms as president, six new corporate partners have been added and, excluding Congress revenue and Humanitarian Aid donations, the WFH's annual revenues have nearly doubled from Can\$2.5 million in 2004 to Can\$4.9 million projected for 2012. He also set up the Susan Skinner Memorial Fund, in memory of his mother who was a symptomatic carrier, to support the education and training of young women with bleeding disorders.

Mark's hope for Treatment for All extended not only to treatment, but to the hope for a cure. He has also played a lead role in developing the WFH's new research program, to be launched at the WFH 2012 Congress in July.



Mark attending a WFH training session in the Philippines, 2010.

During Mark's tenure, the WFH has become a powerful voice for the bleeding disorders community, and he has prepared the WFH to embark on an exciting new phase as we prepare to commence the WFH's 50<sup>th</sup> anniversary activities. Like the WFH's founder, Frank Schnabel, Mark had a dream to "alleviate the pain and plight of the world's haemophiliacs," and through his work, he has succeeded. On behalf of the global bleeding disorders community, we say thank you. ■



Mark with a young boy with hemophilia in Damascus, Syria.



Mark visiting an artificial limb manufacturer in Dakar, Senegal.



# Invaluable contributions to the WFH

Long-time WFH volunteers Professor Alison Street, WFH vice-president medical, and Rob Christie, WFH vice-president finance, will be completing their terms at the WFH 2012 World Congress.



**Prof. Alison Street** (Australia) has served as an Executive Committee member since 2002 and as vice-president medical since 2008. As VP medical, Alison chairs the WFH Medical Advisory Board (MAB) and guides the medical activities of the WFH. A strong supporter of the multidisciplinary approach to care, she reinvigorated the WFH's Medical Advisory Board to include the various key disciplines. In addition, Alison restructured the WFH World Congress to better meet the needs of the multidisciplinary care team.

Alison visiting the National Institute of Hematology and Blood Transfusion in Hanoi, Vietnam.

**Rob Christie** (Australia) has overseen the financial aspects of the WFH as vice-president finance since 2004. Drawing on his professional experience in business management, he plays a key role in improving the financial position and stability of the WFH and in improving management practices to reflect the growing complexity of the WFH. Rob chairs the WFH Congress and Meeting Standing Committee from 2004 until 2012 and also co-chairs the Multidisciplinary Program Committee for the WFH 2012 World Congress.

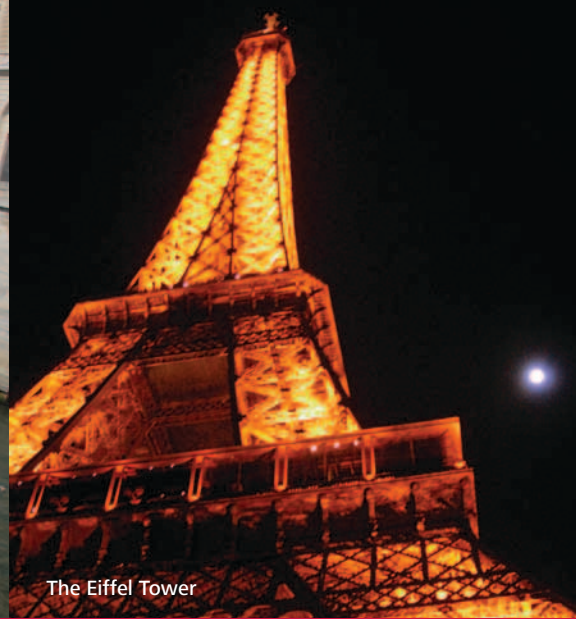
Rob presenting the financial report at the WFH 2010 General Assembly in Buenos Aires, Argentina.



In addition to their invaluable volunteer contributions to the WFH, Alison and Rob have shown leadership in donating each year to the WFH's annual appeal, nurturing the culture of philanthropy that will support future development. Thank you.



Cruise along La Seine.



The Eiffel Tower

# Bienvenue à Paris

*Sarah Ford*

WFH COMMUNICATIONS MANAGER

Come and discover the beauty of Paris while attending the WFH 2012 World Congress. For those wishing to combine an exciting vacation, along with hearing about the latest developments in bleeding disorders treatment and care at Congress, the opportunities are endless.

A number of captivating full- and half-day tours are available to delegates before or after Congress. Explore beyond Paris with tours to the Loire Valley, Normandy, and Champagne regions.

Visit the famous sites and monuments, including the Arc de Triomphe, the Chailot Palace, the Eiffel Tower, the Louvre Museum, the Military Academy, and L'Hôtel national des Invalides, the immense Place de la Concorde, the Opera House, and the famous Champs-Élysées. Take a walking tour of Montmartre, which has retained its village charm in the middle of the busy city. For those who wish to learn about the art of French cooking, participate in a cooking class at Le Cordon Bleu.

All this and more is available to Congress delegates. Consult [www.wfhcongress2012.org](http://www.wfhcongress2012.org) for detailed information on the Congress programs and sessions, workshops, daily tours, hotel information, social events, a very special Awards Ceremony, and more.

**We look forward to seeing you in Paris!**

## 50<sup>th</sup> ANNIVERSARY CAMPAIGN

During Congress, the World Federation of Hemophilia (WFH) will launch its 50<sup>th</sup> anniversary campaign (see page 5). We encourage everyone to participate in these activities as this 50<sup>th</sup> anniversary is a reflection of both the hard work of the WFH and also the global bleeding disorders community.

## TRAVEL INFORMATION



All major airline carriers fly to either of the two international airports in Paris: Roissy/Paris-Charles de Gaulle (CDG) and Paris-Orly Airport (ORY). The Eurostar train is another option for international travellers, with direct connections to London, U.K., and Brussels, Belgium. Congress participants should verify their visa requirements with their French Embassy as soon as possible. Further information regarding visas may also be found at [www.diplomatie.gouv.fr](http://www.diplomatie.gouv.fr).

## 2012 CONGRESS SPONSORS

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# WFH 50<sup>TH</sup> ANNIVERSARY

## 50 years of advancing Treatment for All

Antonietta Colavita  
WFH ANNUAL GIVING MANAGER

As part of the WFH 2012 World Congress experience this year, the World Federation of Hemophilia (WFH) will launch its 50<sup>th</sup> anniversary. Come join the thousands of Congress participants and mark the 50 years of the work of the WFH and the global bleeding disorders community.

The WFH 50<sup>th</sup> anniversary is an opportunity to advance our journey towards Treatment for All. The WFH invites all members of its global family to become a driving force for the future. Your support and passion will help lay a solid foundation for the next decade of global development to help Close the Gap. The reality is that most people with inherited bleeding disorders do not receive adequate diagnosis, treatment, and management for their conditions.

At the Congress Opening Ceremony, the WFH will premiere the 50<sup>th</sup> anniversary video entitled "Close the Gap", the first in a series of anniversary videos to be released over the next two years. Filmed on location in Senegal, it highlights the work that the WFH has done thanks to the tireless commitment of this community.

During the WFH Awards Ceremony at Congress, there will be a special tribute to the extraordinary vision and leadership of the WFH volunteers who have championed the vision of Treatment for All.

Every Congress delegate is also invited to visit the WFH Resource Centre in the exhibition hall to make

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Close the Gap.

an anniversary wish and a contribution to help Close the Gap in care around the world. Be sure to make your mark on the WFH world map. Donors will be eligible for the daily prize draws and a WFH World Cup will be awarded to the country that donates the most. One Grand Prize, a trip to the 2014 Congress to be held in Melbourne, Australia, will be announced at the Farewell Dinner. All donors at Congress and Sustaining members who sign up or renew during Congress will be eligible.

Our 50<sup>th</sup> anniversary is an opportunity for action as well as reflection. While the WFH's successes have been impressive, there is much work left to be done. Approximately 75 per cent of people with bleeding disorders do not receive adequate care. In areas of the world where it is a struggle to access even the most basic medical care, bleeding disorders can be debilitating and life-threatening.

As we reflect on the past 50 years of advancements, we are constantly looking forward. We will continue to work toward the day where treatment will be available for all around the world.

**Together, we will Close the Gap.**



WFH founder Frank Schnabel.

### 50<sup>th</sup> ANNIVERSARY HONOURARY COMMITTEE SPONSORS

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Sanquin

# Extraordinary volunteers

Supporting research through participation and outcomes analysis

Mark W. Skinner  
WFH PRESIDENT

Earlier this year, I was sitting in a waiting room preparing to enroll in yet another clinical research study. This time it was to participate in a study to improve diagnostic techniques and treatment for those living with hepatitis. As I waited, I took note of a quote on the wall, "Behind every treatment and every cure is a group of extraordinary volunteers."

Over the past 50 years, we have seen enormous advances in approaches and therapies to treat bleeding disorders. It is well worth remembering that none of these advances would have been possible without the heroic and altruistic generosity of thousands of patients. Each of them voluntarily consented to participate in medical research, or to allow the collection of personal medical data for study.

Within my lifetime, treatment options have advanced from whole blood and fresh plasma, to fresh frozen plasma, cryoprecipitate, plasma-derived factor concentrates, and, most recently, recombinant clotting factors. The promise of even newer treatments, such as long-lasting therapies and gene transfer, are on the horizon.

Similarly, the delivery of care has evolved from a fragmented system with few specialists, to an understanding that care delivered in a comprehensive multidisciplinary framework, with trained healthcare professionals, produces optimal results.

Today, through continuing research, we are developing tools and knowledge to further tailor and deliver treatment personalized to the individual patient rather than generally treating the disease. Concepts such as personalized prophylaxis, the identification of individuals at risk of developing an inhibitor, and health indicators unique to women with bleeding disorders are moving into clinical care.

Those of us living with a bleeding disorder, as well as the families that support us, look forward to the day when there is a cure or, at the very least, treatments that are easily accessible and affordable for all, as well as being minimally invasive in our daily lives. If we are to achieve these goals,

we must support and participate in research to bring about the next great advance, and at the same time sustain the many gains already achieved.

The challenges of answering government and payer demands for evidence-based medicine and cost justification for the introduction and enhancement of new treatments and care models are ever-present and growing. To sustain and continue the expansion of access to care globally, it is critical to build the body of outcome data for individual patients, within hemophilia treatment centres, nationally, regionally, and globally. Doing so will not only improve clinical practices and support the allocation of scarce resources, but also, most importantly, improve the overall well-being of patients.

To do so, the World Federation of Hemophilia (WFH) recommends the development of national patient registries through collaboration between national patient organizations, healthcare professionals, treatment centres, and ministries of health. Registries are beneficial for patients and their quality of life, to doctors and nurses for the identification of changing care needs, and to public health authorities for a better management of care requirements and resources. All countries, developed or developing, have a limited amount of resources to spend on health care and they typically allocate resources based upon what they assume is the highest priority and produces the greatest value.

A properly designed registry that tracks outcomes of treatment, including complications, will allow the vital analysis of where resources are expended and inform future health planning. This is important for two reasons. First, successful healthcare outcomes (such as reduced days of hospitalization, absence from school, or lost days from work) will increase the support and enthusiasm of patients. Secondly, the successes will demonstrate to health authorities the usefulness of care program or therapeutic interventions.

Other examples of outcomes data include the number of infections with blood-borne viruses (HIV, HCV, etc.), the number of patients with joint disease or



the degree of joint disease in the patients, the number of patients that have inhibitors, liver disease, are hospitalized, or die. This information is critical in identifying the changing needs of both men and women with bleeding disorders, identifying particular problems that need addressing, or assessing and documenting the effect that changes in health care delivery have made for the population.

We as patients have an incredibly important role to play. Our participation in clinical research both for the development of new therapies and optimization of standards of care is essential. Without our participation, we will not realize our hope and vision of achieving Treatment for All. If we do not participate in clinical research, if we do not implement the necessary framework to capture and analyse data, it will not happen. Over the years ahead, the WFH is committed to developing a global initiative to educate patients and assist clinicians in improving the global research capacity and addressing these challenges. ■

# The age of assessment

Alison Street, MD  
WFH VP MEDICAL

As my tenure as World Federation of Hemophilia (WFH) vice-president medical draws to a close, I am heartened to see all the advances that our community has made in recent years. This is indeed a most exciting time for hemophilia research and treatment.

At the 2011 annual scientific meetings of the International Society on Thrombosis and Haemostasis (ISTH) and the American Society of Hematology, prominent clinician-researchers in hemophilia gene transfer therapy were invited to discuss their work in the plenary sessions.

Topics for plenary presentations are selected by peer review for their exceptional science and also recognized for the importance of their clinical application. We congratulate and thank everyone involved in these presentations.

In our bleeding disorders family, we are looking forward to having many of the new treatments in areas of coagulation factor replacement and gene transfer therapies, which are in clinical trial status presently, registered within the next few years. Achieving the goal of registration takes trust and collaboration between researchers, clinicians, and most importantly patients, for without patients, there can be no studies of new products. Hemophilia treatments must be trialed in humans, as in the cases of people with hemophilia, as studies in tissues or other animal species do not sufficiently predict their safety and effectiveness in humans. Learn more about the latest developments in gene transfer therapy on page 8.

During the WFH 2012 World Congress in July, we will be discussing and saluting the role of patient volunteers in the conduct of clinical trials. We also will be working with ISTH to provide support and training to clinicians, encouraging them to participate in a broad range of clinical research, in addition to product evaluation and clinical outcome analysis.

It is the dawning of the age of assessment in our community. This includes the assessment of the physical and social well-being of the person with a bleeding disorder, the determination of the extent and quality of their care, as assessed by patients

Advocacy for recognition and treatment for patients with bleeding disorders is increasingly strong and effective in many parts of the world.

and their clinicians, and from the funding perspective, the assessment of value for the money invested in treatment. To do this properly we need robust data collection systems with sufficient provisions of human and technical resources to permit capture of meaningful and reproducible data. The data should be able to be analysed serially in time, its longitude and in comparison with validated studies being conducted elsewhere, its latitude.

For example, we can measure in an individual patient over many clinical consultations, the number of clinically apparent joint bleeds against product usage, joint function and appearance on imaging studies, time lost from school or work, and measures of self-assessed quality of life including social participation. Ideally these parameters should be recorded at each visit. Less frequently performed measurements, such as pharmacokinetic profiles in response to factor infusion, are also important to collect and analyse. These measures provide not only product information but also permit individualized dosage recommendations.

Building up individual patients' data sets permits comparison within clinics and more widely between centres, between regions within a country, and between countries. The annual WFH Global Survey Report, for example, represents a "big picture" analysis of the number of persons with bleeding disorders and treatment availability. However, the number of data elements is small and improved data collection at the centre and country level will increase the quality and applicability of our analysis.

Hemophilia care, because of its complexity and expense, is a natural target disease for study by funders. It is very important that when a formal clinical effectiveness review or health technology assessment (such as the one recently published from



Sweden<sup>1</sup>) is planned, that the local hemophilia community of patients and healthcare professionals participate from the beginning. This is to ensure that there is maximum cooperation and transparency of the process and that data are appropriately interpreted by the collectors and reviewers. The more relevant data that are available, the better the quality of the assessment can be made. From this review, the stronger the case can be made for improving and maintaining care.

This is my last column for *Hemophilia World* as vice-president medical. The world outside has changed enormously in the time since I was first co-opted and then elected to the WFH board nearly 10 years ago. Advocacy for recognition and treatment for patients with bleeding disorders is increasingly strong and effective in many parts of the world. To continue these gains, given the many wider crises in global financial systems, our community has a critical obligation to support and inform patients and clinicians everywhere of the importance of data collection and clinical outcome research. Encouragement of a research climate stimulates and benefits us all and leads to a flow of novel and exciting ideas. ■

<sup>1</sup> <http://onlinelibrary.wiley.com/doi/10.1111/j.1365-2516.2011.02723.x/abstract>



# Gene therapy trial shows promising results

Mark Brooker

WFH SENIOR PUBLIC POLICY OFFICER

Scientists have made a remarkable breakthrough in the development of gene transfer treatment for hemophilia. A team of researchers based in London, led by doctors Amit Nathwani and Edward Tuddenham, published unequivocal evidence of success of this technology in the treatment of hemophilia B.<sup>1</sup> An early abstract, hinting at success in the study, was first reported at the WFH 2010 World Congress.

A total of six patients with severe hemophilia B were treated with injections of the adeno-associated virus (AAV) vector carrying the normal factor IX gene. All six patients showed therapeutic response to the factor IX gene administration because post-injection they showed higher measurable clotting factor levels and required either less clotting factor concentrate or none at all in their daily lives. One patient receiving the highest dose of the vector has maintained factor levels between 8 per cent and 12 per cent for beyond 6 months. The study is ongoing.

We have known for years that treating hemophilia through gene transfer is possible because it has been demonstrated to work long-term in animals, including mice and dogs. In dogs with hemophilia, therapeutic levels of clotting factor production have been achieved for more than eight years after a single gene transfer administration. The challenge has been in reproducing that success in humans.

The technique employed in this study was simple, with the normal factor IX gene being delivered into a peripheral vein in a tiny carrier called a vector. These are small viruses that infect humans and some

We have known for years that treating hemophilia through gene transfer is possible because it has been demonstrated to work long term in animals, including mice and dogs. The challenge has been in reproducing that success in humans.

other primate species but do not cause any disease. There are different types of AAV with attraction to different human tissues, such as the liver. The strategy is that the modified AAV with the factor IX gene inside it is injected into the vein of a patient and travels to the liver where it delivers the normal factor IX gene into liver cells. These liver cells then begin to produce factor IX and the patient's factor level rises.

Previous attempts to use AAV vectors in humans with hemophilia B did show some success in raising factor levels. In these early trials, researchers used the AAV2 virus particles in very large numbers to overcome inefficiency in entering liver cells. However, the response was short-lived (four to six weeks) and factor production in the liver eventually stopped as the human immune system destroyed the liver cells that contained the virus. Furthermore, as most humans have been previously exposed to this form of the virus and therefore carry antibodies to it, they cannot be treated effectively with this vector.

This most recent trial is exciting because it used a redesigned AAV8 vector that has greater efficiency in entering liver cells and requires lower doses which avoids the major immune response seen in earlier trials. Furthermore, knowing that an immune response to the vector could

be a problem, the patients in this study were carefully monitored. When two of them showed signs that their bodies were attacking the vector, they were given a short course of steroids to suppress this immune response and their factor levels remained elevated.

Gene therapy for hemophilia B will move forward in a process much like that for the development and approval of a new clotting factor concentrate. The six patients from this trial will continue to be monitored. A further clinical trial, under the supervision of regulatory authorities in the U.S.A. and Europe, will be conducted to show the safety and efficacy of the treatment and to determine the optimal dose. If that trial is successful, the regulators would then approve it for use in patients.

The success in treating hemophilia B suggests that hemophilia A could also be treated using this strategy. However, the factor VIII gene is much larger than the factor IX gene and it remains to be seen if the vector used for hemophilia B gene therapy could carry the larger factor VIII gene. It may be that a different and larger vector is necessary to effectively deliver the factor VIII gene. In addition, the immune response to factor VIII (factor VIII inhibitor development) is also a potential challenge for factor VIII gene transfer studies.

The patients who volunteered for this and previous gene therapy trials for hemophilia have made an incredible contribution in the advance towards a cure for hemophilia. The WFH has been an early supporter of gene therapy, including this trial. "We support clinicians and researchers working in the field to achieve this goal," said WFH president Mark W. Skinner. "We are most grateful to the researchers who pioneer these studies and to the patients who have participated in clinical trials." ■

<sup>1</sup> Nathwani AC, Tuddenham EGD, Rangarajan S, et al. Adenovirus-associated virus vector-mediated gene transfer in hemophilia B. *N Engl J Med* 2011. DOI: 10.1056/NEJMoa1108046



# Strengthening and building connections in South Africa

Tony Roberts

SOUTH AFRICAN HAEMOPHILIA FOUNDATION

With only 30 per cent of the estimated men and women with bleeding disorders presently diagnosed worldwide, the World Federation of Hemophilia (WFH) works with countries, such as South Africa, to increase diagnosis and treatment of people with bleeding disorders. Through its Global Alliance for Progress (GAP) program, South Africa will work with the WFH to bring bleeding disorders care throughout the country to a higher level.

One of the first steps in improving treatment is to locate those people with bleeding disorders who live in the most remote parts of this beautiful country. Bradley Rayner, chairperson of the South African Haemophilia Foundation (SAHF), has a dream for “improved diagnosis – to find our missing brothers and sisters. We know that they are out there.” He proposes the establishment of a national working group, with the aid of geneticists to guide those involved to focus on this task.

The South Africa GAP initiatives include the formation of a national hemophilia committee, along with the National Department of Health, with the aim of reinforcing the South African government’s vision of equal

“The hope is that GAP will enthuse, focus, and excite the work already started in building a more improved and strengthened hemophilia care system in South Africa.”

access for all, including access to health. In addition, the SAHF is looking to open up care in the rural communities by providing nurses with netbooks and modems. There will also be the creation of a web resource portal for the SAHF to share information and deliver reports. Other initiatives include improving and expanding the patient address list and the patient registry; outreach and development to under-serviced areas; strengthening psychosocial, physiotherapy, and nurses training; and creating more awareness about people with bleeding disorders.

“The hope,” according to Rayner, “is that GAP will enthuse, focus, and excite the work already started in building a more improved and strengthened hemophilia care system in South Africa.”

Anne-Louise Cruickshank, hemophilia nurse coordinator for the Western Cape province and secretary of the Medical and



Abdul Barends with his two sons who have severe hemophilia A and his daughter.

Scientific Advisory Council (MASAC) of the SAHF, believes that the South African GAP program will contribute toward comprehensive hemophilia care for all across the country. In addition, the hope is that there will be increased diagnosis, prevention of untimely deaths, care for carriers and family members of people with bleeding disorders, more expertise in the rural areas, and succession planning.

Dr. Johan Potgieter, pathologist and chairman of MASAC, has no illusions about the task when stating that, “we find ourselves, once again, at a watershed. I have no doubt that engaging in the GAP program constitutes nothing less than a daunting endeavour. Closing the gap between the amounts of replacement product required versus what is available poses perhaps the biggest challenge. This will require committed and sustained government support to increase resources. Without this, we may be unable to drastically reduce mortality and morbidity amongst people with hemophilia, as the organization of care makes a difference. No doubt, the SAHF will be strengthened through the GAP program.”

Ultimately, the SAHF hopes that the GAP program will improve the quality of life of everyone effected and affected by hemophilia and related bleeding disorders across South Africa, especially those that are the most vulnerable, the young. ■

*The WFH is grateful for the support of the GAP program by founding sponsor Baxter; sustaining sponsors the André de la Porte Family Foundation, Bayer, and CSL Behring; supporting sponsors Biogen Idec Hemophilia, Biotest, Grifols, and Pfizer; contributing sponsor the Irish Haemophilia Society; and collaborating partner the World Health Organization (WHO).*



Young men with hemophilia attending camp.



# WHAT WILL IT TAKE TO CLOSE THE GAP?

World Hemophilia Day 2012  
April 17

An estimated 1 in 1,000 women and men has a bleeding disorder. However, 75% still receive very inadequate treatment or no treatment at all.

Together, we can work towards a day when treatment will be available for all around the world.



Help us Close the Gap  
[www.wfh.org/whd/en](http://www.wfh.org/whd/en)





# WORLD HEMOPHILIA DAY 2012—APRIL 17

# Close the Gap

Sarah Ford

WFH COMMUNICATIONS MANAGER

An estimated 1 in 1000 women and men has a bleeding disorder. However, 75 per cent still receive very inadequate treatment or no treatment at all. What will it take to Close the Gap?

On World Hemophilia Day 2012, help us spread the message to “Close the Gap” in care around the world. Together, we can work toward a day when treatment will be available for all globally.

The reality is that most people with hemophilia or another bleeding disorder do not receive adequate diagnosis, treatment, and management for their conditions. This is important where good treatment is already established but needs to be protected, or where treatment needs to be improved.

World Hemophilia Day began in 1989 and has grown into a truly global event. Hemophilia organizations and treatment centres around the world take part each year to increase awareness of hemophilia and other bleeding disorders.

Connect with your online communities to help send out the message about the need to sustain and improve care. The World Federation of Hemophilia (WFH) will be posting weekly Facebook and Twitter messages leading up to World Hemophilia Day. We encourage you to visit the WFH Facebook page at [www.wfh.org/facebook](http://www.wfh.org/facebook) and see how orga-

nizations around the world have marked World Hemophilia Day this year.

Many people will be wearing red to help raise awareness about World Hemophilia Day. The WFH will be collecting photos of you, your family, and your friends wearing red. Post your photos on the World Hemophilia Day Facebook page or send them to [sford@wfh.org](mailto:sford@wfh.org). The WFH will then select photos from your submissions for a poster that will be displayed at the WFH 2012 World Congress.

Join the international bleeding disorders community on April 17 to mark World Hemophilia Day. Together, we will Close the Gap. ■

*The WFH is grateful for the support of World Hemophilia Day by Baxter, Bayer, Biogen Idec Hemophilia, CSL Behring, Inspiration Biopharmaceuticals and Novo Nordisk.*



Give a gift of membership on World Hemophilia Day, visit [www.wfh.org](http://www.wfh.org)

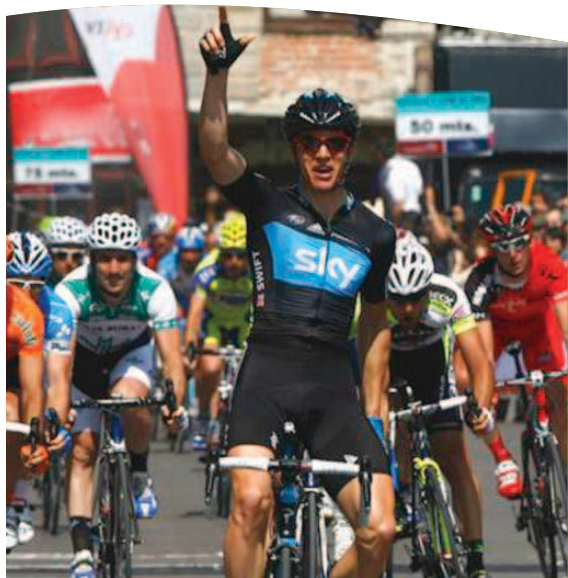
The WFH would like to hear how you and your organization or treatment centre mark World Hemophilia Day. Please send an update, along with a few high resolution photos to [sford@wfh.org](mailto:sford@wfh.org) by May 18.



## The journey towards gold

The career of an elite athlete can be quite a journey. Alex Dowsett, a professional cyclist with Team Sky Britain, has achieved great success which has continued into 2012.

Dowsett has severe hemophilia A and is an excellent example of how treatment and care has advanced to a level where elite athleticism is indeed feasible for a person with hemophilia. The World Federation of Hemophilia wishes Dowsett great success in his 2012 racing season. Follow Dowsett's quest for gold at [www.trainingscapes.com/alex-dowsett](http://www.trainingscapes.com/alex-dowsett).



# NEWS FROM AROUND THE WORLD

WFH regional update



LEFT: Symposium on hemophilia in Santa Cruz, Bolivia. RIGHT: Patient examination facilitated by physiotherapist Natasa Jankovic and orthopedist Dr. Luigi Solimeno, at the musculoskeletal workshop in Belgrade, Serbia.

## AFRICA AND EASTERN MEDITERRANEAN

### Jordan

The Minister of Health of Jordan approved the home treatment concept to be applied in all Ministry of Health hospitals in October. As a result, patients will receive one to two doses to start treatment as soon as bleeding occurs without waiting until they reach the treatment facility. This will have a positive impact on treatment outcome. The Minister of Health thanked the World Federation of Hemophilia (WFH) for its continued support to the hemophilia cause in the country and expressed the Ministry's desire to maintain close cooperation with the WFH in the future.

### Kuwait

In cooperation with the WFH, the Kuwait Hemophilia Committee organized a two-day activity in November. The first day was a scientific symposium that attracted health professionals from major treatment facilities in Kuwait. The second day was a patient/family educational session that covered home

therapy and self-infusion. The event was widely covered in Kuwait public media.

### Lebanon

In October, the Lebanese Hemophilia Association (LHA) and the WFH organized two sessions on hemophilia and other bleeding disorders during the annual meeting of the Lebanese Society of Hematology, in Beirut. The WFH and the LHA delegation also met with the Minister of Health and discussed the ministry's future role and support for hemophilia care in the country, particularly in the field of provision of treatment products. It was agreed that the LHA will submit a proposal to the Ministry of Health on future cooperation in this field.

### Morocco

With cooperation from the Moroccan Minister of Health, the Moroccan Hemophilia Association and the WFH organized a regional meeting for the Middle East region in Casablanca, in October. Representing Syria, Lebanon, Jordan, Kuwait, Bahrain, Qatar,

Oman, United Arab Emirates, Saudi Arabia, Egypt, Sudan, Tunisia, Algeria, Morocco, Mauritania, Senegal, and Turkey, 40 health professionals and people with hemophilia attended this meeting. The meeting focused on several topics related to challenges faced by hemophilia communities.

## AMERICAS

### Bolivia

In November, a two-day hemophilia symposium was held in the city of Santa Cruz that brought together patients, families, and medical professionals from different areas of the country. The main objectives were to provide an opportunity for learning and exchange amongst health professionals, medical students, families, and patients and to provide information on comprehensive care. This was the first time that the Bolivian Federation of Hemophilia (*Federación Boliviana de Hemofilia*) organized this type of event. It was a great success with over 100 participants. Representatives of the Ministry of

Health also attended and helped to inaugurate the event.

### Brazil

The Ministry of Health is promoting primary prophylaxis and immune tolerance induction for patients and helping to support the implementation of this new regime in the hemophilia treatment centres. The Brazilian Federation of Hemophilia (*Federação Brasileira de Hemofilia*) worked arduously and lobbied the Ministry of Health to begin offering this form of treatment for patients. This was done by demonstrating the benefits that this treatment provides for the patients in the short and long term with regards to quality of life.

### Venezuela

The Venezuelan Association of Hemophilia (*Asociación Venezolana para La Hemofilia*), after having organized 30 camps for children, youth, and adults, decided to organize a camp specifically for nurses. Over a weekend, 18 nurses from 10 different regions participated in various sessions that covered the role



of the nurse in the multidisciplinary team, the administration of factor, and how to treat patients depending on their bleed. This was a pilot project and after all the positive feedback, it was decided to organize a nurses' camp annually.

## ASIA AND WESTERN PACIFIC

### Australia and New Zealand

Twenty-five people gathered in October for the first ever joint Australia and New Zealand Inhibitors Workshop in Sydney that focused specifically on the challenges faced by families with hemophilia and inhibitors. Participants included parents of children with inhibitors, as well as men with inhibitors of all ages, and their partners or a support person. The idea for the workshop grew out of consultation with local hemophilia nurses and was a combined effort between Haemophilia Foundation Australia and Haemophilia Foundation of New Zealand.

### China

In Yangzhou, in September, the nursing group held a one-day national nursing forum attended by over 100 nurses and healthcare professionals. Nurses from 28 provinces participated. An afternoon concurrent session was held for over 70 patients and families on

home care. Hemophilia Home of China (HHC), in cooperation with the One Foundation, began a three-year project providing assistance to rural, low-income children around the country. HHC also signed an agreement with the Children's Hope Foundation China to set up another fund to assist children with hemophilia.

### India

In October, a WFH delegation carried out an assessment visit to acquire a broad understanding of the situation in India and the issues and challenges facing Hemophilia Federation India (HFI) and its 70 chapters. Activities included chapter meetings with patients, families and volunteers in Mumbai, Chennai, and Delhi. In addition, there were visits to treatment centres in Delhi and to the WFH International Hemophilia Training Centres in Mumbai and Vellore. There were also regional roundtable conferences with HFI chapters, meetings with representatives from government officials, and formal discussions with HFI regarding future cooperation.

### Mongolia

A clinical workshop covering a wide range of comprehensive care topics was held for two days in Ulaanbaatar, in September. It was followed by a one-day workshop with the Mongolian Association of



Attendees of the clinical workshop in Ulaanbaatar, Mongolia.

Haemophilia during which patients and families were educated on basic concepts about the disorder, looking after joints, replacement therapies, inhibitors, and managing the disorder. In October, a workshop was held for family physicians from Ulaanbaatar and another workshop for doctors from eight provinces.

### Caucasus

A two-and-a-half day regional dental workshop was organized in Tbilisi, Georgia, for 26 participants from Georgia, as well as Armenia and Azerbaijan. Dentists, hematologists, and patient leaders learnt about prevention, hygiene, and dental procedures. Plans are now underway to bolster dental care in the comprehensive HTC services of Baku, Yerevan, and Tbilisi.

## EUROPE

### Balkans

Close to 50 orthopedists, psychiatrists, hematologists, and physiotherapists attended a day-and-a-half regional musculoskeletal workshop in Belgrade, Serbia. Topics discussed ranged from the referral of patients by the comprehensive care team, arthropathy, synovitis, physiotherapy, and orthopedic preventions. Participants came from Serbia as well as from Macedonia, Bosnia and Herzegovina, and Montenegro.

### Moldova

A board training was held in Chisinau for 20 participants from all regions of the country. The workshop was facilitated by the WFH delegation and a Polish Hemophilia Society representative that is twinned with Moldova. The topics discussed were lobbying, media relations, good governance, and succession planning. This activity was followed-up with a visit to Warsaw by three Moldovan patient leaders to attend the Polish Hemophilia Society's elections and general assembly. ■



Meeting with the Jordanian Minister of Health.

# Global giving from our national member organizations on the rise

Eric Stolte

WFH FUND AND RESOURCE DEVELOPMENT  
COMMITTEE CHAIR

Like any community, our strength lies in our members and the vital work we do together to improve treatment and care for people with bleeding disorders around the world. As we prepare for our 50<sup>th</sup> anniversary, we are especially grateful to the national member organizations (NMOs) of the World Federation of Hemophilia (WFH) that have gone beyond the national scope of their remarkable work and we wish to highlight these extraordinary contributions to the WFH's global mission.

For several years, a small and growing group of NMOs have begun to incorporate financial contributions to the WFH as a core organizational commitment. These are recognized in *Hemophilia World* and in WFH annual reports. Most recently, the National Hemophilia Foundation (NHF) in the U.S.A. has shown tremendous leadership by making a contribution that matches the total donated by all NHF chapters in 2011. This in turn encourages a culture of philanthropy, solidarity, and global outreach throughout the American bleeding disorder community. The total donated by all NHF chapters and the NHF itself reached close to US\$40,000 for 2011. This is leadership indeed.

Also it is with great pride that we welcome the Venezuelan Association for Hemophilia (*Asociación Venezolana para la Hemofilia*) into our global giving club. *Muchas gracias y bienvenidos.*

In an unprecedented gesture, the Egyptian Society of Hemophilia (ESH) redirected its portion of revenue from the 20<sup>th</sup> Musculoskeletal Congress to the WFH as a donation. This substantial gift was more than six times the amount of the ESH's usual annual donation.

No contribution is too modest. For the second year in a row we were humbled to receive a gift from the Angamaly Chapter of the Hemophilia Society of India. Their ongoing generosity is a testament to the power and the joy of participation as we continue in our mission to provide Treatment for All.

Here are some more examples of how some NMOs have chosen to partner with us in philanthropy: the Canadian Hemophilia Society contributes two per cent of its unrestricted revenues each year; the Egyptian Society of



We are especially grateful to the national member organizations of the World Federation of Hemophilia that have gone beyond the national scope of their remarkable work and we wish to highlight these extraordinary contributions to the WFH's global mission.

Hemophilia has made a multi-year pledge; the Haemophilia Foundation of New Zealand has pledged a segment of its annual membership dues; and the Irish Haemophilia Society has supported our GAP program with an annual contribution since 2007.

As we approach our 50<sup>th</sup> anniversary launch and embark upon the WFH's first multi-year fundraising campaign, we invite all NMOs to be inspired by these examples and to engage in philanthropy with the WFH and with one another. Together, let's Close the Gap between those who have access to care and those for whom care remains out of reach. ■

In 2011, NMOs and chapters combined contributed over US\$75,000 to help Close the Gap in care, an increase of 30 per cent over 2010. **THANK YOU to:**

Asociación Venezolana para la Hemofilia  
Canadian Hemophilia Society  
Egyptian Society of Hemophilia  
Haemophilia Foundation of New Zealand  
Irish Haemophilia Society  
National Hemophilia Foundation (U.S.A.)

#### CHAPTERS India:

Angamaly Chapter of the India Hemophilia Society

#### U.S.A.:

Bleeding Disorder Foundation of Washington  
Delaware Valley Chapter of the National Hemophilia Foundation  
Hemophilia Foundation of Greater Florida  
Hemophilia Foundation of Michigan  
Hemophilia of Georgia  
Hemophilia of North Carolina  
Virginia Hemophilia Foundation

Special recognition will be accorded to all NMOs and their chapters who make a contribution to the 50<sup>th</sup> Anniversary Close the Gap Campaign.



# Calendar of Events 2012



## World Hemophilia Day 2012

April 17, 2012

World Federation of Hemophilia

Tel.: +1-514-875-7944

Fax: +1-514-874-8916

Email: sford@wfh.org

www.wfh.org/whd

## XXXIV World Congress of the International Society of Hematology (ISH)

April 25-28, 2012 – Cancun, Mexico

International Society of

Hematology (ISH)

Tel.: +52-55-55-24-11-12

Email: contacto@amehac.org

www.hematology2012.com/en/inicio.aspx

## World Blood Donor Day

June 14, 2012

World Health Organization

www.who.int/worldblooddonorday

## Thrombosis and Hemostasis Summit of North America (THSNA)

May 3-5, 2012 – Chicago, U.S.A.

Tel.: +1-617-638-4605

Tel.: +1-800-688-2475

Email: cme@bu.edu

www.thsna.org

## IPFA/PEI 19<sup>th</sup> International Workshop on Surveillance and Screening of Blood Borne Pathogens

May 23-24, 2012 – Budapest, Hungary

IPFA/PEI

Tel.: +31 20 512 3561

Fax: +31 20 512 3559

Email: ipfa@sanquin.nl

www.ipfa.nl

## PPTA Plasma Forum

June 21-22, 2012 – Washington, D.C., U.S.A.

Plasma Protein Therapeutics Association (PPTA)

Tel.: +1-202-789-3100

Email: ccash@pptaglobal.org

www.pptaglobal.org

## 58<sup>th</sup> Annual SSC Meeting (Scientific & Standardization Committee of the ISTH)

June 27-30, 2012 – Liverpool, United Kingdom

The International Society on Thrombosis and Haemostasis

Tel.: +41 22 33 99 588

Fax: +41 22 33 99 631

Email: ssc2012reg@mci-group.com

www.ssc2012.org

## 32<sup>nd</sup> International Congress of the ISBT

July 7-12, 2012 – Cancun, Mexico

International Society of Blood Transfusion (ISBT)

Tel.: +31 20 679 3411

Fax: +31 20 673 7306

Email: mexico@isbtweb.org

www.isbtweb.org/mexico/welcome

## 2012 Global NMO Training

July 5-7, 2012 – Paris, France

World Federation of Hemophilia

Tel.: +1-514-875-7944

Fax: +1-514-874-8916

E-mail: azavagno@wfh.org



## WFH 2012 World Congress

July 8-12, 2012 – Paris, France

World Federation of Hemophilia

Tel.: +1-514-875-7944

Fax: +1-514-874-8916

Email: info2012@wfh.org

www.wfhcongress2012.org

## World Hepatitis Day

July 28, 2012

World Hepatitis Alliance

www.worldhepatitisalliance.org

## ATHN Data Summit 2012

September 20-21, 2012 – Chicago, U.S.A.

The American Thrombosis and Hemostasis Network (ATHN)

Tel.: +1-800-360-2846

Fax: +1-847-572-0967

Email: info@athn.org

www.athn.org

## EHC Conference 2012

October 26-28, 2012 – Prague, Czech Republic

European Haemophilia Consortium

www.ehc.eu

## HAA-APSTH 2012

October 29-31, 2012 – Melbourne, Australia

Haematology Society of Australia (HAA)

Tel: +61 8 8363 1307

Fax: +61 8 8363 1604

Email: haa@fcconventions.com.au

www.fcconventions.com.au/HAA2012

## NHF 64<sup>th</sup> Annual Meeting

November 8-10, 2012 – Orlando, U.S.A.

National Hemophilia Foundation

www.hemophilia.org

## World AIDS Day

December 1, 2012

National AIDS Trust

www.worldaidsday.org

## ASH Annual Meeting

December 8-11, 2012 – Atlanta, U.S.A.

American Society of Hematology

Tel.: +202-776-0544

Fax: +202-776-0545

www.hematology.org

# Thank You

In recognition of the organizations that have committed or contributed to the WFH's mission so far in 2012

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Jan Willem André de la Porte

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Baxter  
Bayer  
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Training Program  
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International Hemophilia  
Training Centre Fellowship  
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Website  
WFH website: Bayer  
HemoAction e-games: Novo  
Nordisk

HTC Passport Web Directory: Biogen  
Idec Hemophilia  
Inhibitors web section: Grifols, Novo  
Nordisk  
Online Laboratory Manual: Grifols  
von Willebrand Disease web sec-  
tion: CSL Behring, Grifols, LFB,  
Octapharma

Publications  
Medical Advisory Board  
E-newsletter: Biogen Idec  
Hemophilia

Other programs  
Inga Marie Nilsson Award:  
Octapharma  
Susan Skinner Memorial Fund: The  
Hemophilia Alliance  
von Willebrand Disease Outreach  
Project: LFB

World Hemophilia Day  
Baxter, Bayer, Biogen Idec  
Hemophilia, CSL Behring,  
Inspiration Biopharmaceuticals,  
Novo Nordisk

## ORGANIZATIONS

Canadian Hemophilia Society\*  
Egyptian Society of  
Hemophilia\*  
Haemophilia Foundation of  
New Zealand\*  
National Hemophilia  
Foundation (U.S.A.)\*

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# HEMOPHILIA WORLD WOULD LIKE TO HEAR FROM YOU!

The activities of people living with hemophilia and other inherited  
bleeding disorders, and their organizations, are important to everyone  
in the global bleeding disorders community. We welcome stories, letters,  
and suggestions for articles. Please send them to:

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