

HEMOPHILIAWORLD

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A diverse and innovative program awaits: WFH 2016 World Congress



The World Federation of Hemophilia (WFH) 2016 World Congress will be held in Orlando from July 24-28, 2016. This unique global platform provides the bleeding disorders community with the opportunity to share experience and expertise on the treatment and management of hemophilia and other inherited bleeding disorders. The WFH is pleased to be organizing this event with the National Hemophilia Foundation (NHF) acting as gracious hosts.

/Helene Fasciano Lussier, CONGRESS AND MEETINGS MANAGER

There has been an outstanding level of cooperation at all levels of the WFH 2016 program committees to ensure that the program represents the global bleeding disorders community. The program caters to a wide range of interests and expertise, from patients to expert clinicians.

In all, there are 10 professional development sessions, 10 plenaries, two late breaking trial sessions, 52 congress sessions, and 12 abstract-based free papers sessions.

The Congress will begin on Sunday, July 24, with full- and half-day professional development sessions and workshops. In addition to the customary nursing, psychosocial, orthopedic, and physiotherapy sessions, the WFH committees have developed some brand new and exciting presentations this year.

The WFH Nursing and Psychosocial Committees will begin each of their respective sessions with a combined two-and half-hour

session on genetic counselling. The WFH Laboratory Science, the von Willebrand disease (VWD), and bleeding disorders Committees have also combined their efforts in developing a three hour workshop on the current issues in the diagnosis and management of VWD. New topics also include complimentary workshops on clinical research and data collection.



"The WFH 2016 Congress Program committees have again strived to devise an innovative and dynamic program with broad appeal to clinicians, basic scientists, allied health professionals and patients for the four main days of the Congress. "



Each day during the Congress, from Monday to Thursday, will open with a general plenary. The WFH is pleased to announce that to have former NASA International Space Station (ISS) Risk Manager, Michael Lutomski, as this year's

special guest plenary speaker on Wednesday, July 27, 2016. As Risk Manager, Lutomski was responsible for defining and implementing the qualitative and quantitative risk management processes across the organizations and international participants of the ISS Program. He retired from NASA in 2013 and joined Space Exploration Technologies, or SpaceX, where he works as the Director of System Safety and Risk.

For the first time at a WFH World Congress, the general plenary will be followed by two concurrent plenaries: Medical and Multidisciplinary. The medical plenaries include a presentation by Dr. Rodney Camire, associate professor of pediatrics at the University of Pennsylvania and the Children's Hospital of Philadelphia entitled *Primary Hemostasis*.

The multidisciplinary program committee is delighted to include their first plenary sessions: *Women and bleeding disorders*, presented by Dr. Michelle Sholzberg from St. Michel's Hospital in Toronto; *Patient partnerships: the next step in hemophilia care*

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presented by Mr. Vincent Dumez, Co-director of the Office of Collaboration and Patient Partnership, Faculty of Medicine at University of Montreal; and *Empowerment through self-care* with Patrick James Lynch, creator, executive producer, and star of the award-winning hemophilia comedy web series, "Stop The Bleeding!". Each of these multidisciplinary plenaries will be followed up with 90 minute comprehensive congress session, elaborating on the plenary's main topic.

The final plenary on Thursday, July 28, will be a combined effort from the medical and multidisciplinary program committees that will highlight 'what is a bleed' as defined by a patient and 'what is the response to treatment' as defined by healthcare professionals.

The WFH 2016 Congress program committees have again strived to devise an innovative and dynamic program with broad appeal to clinicians, basic scientists, allied health

professionals and patients for the four main days of the Congress. One of the highlighted sessions entitled, *Get Net-Smart: Privacy Protection, Assessment of Online Information, and Using Web-Based Tools in Bleeding Disorders Care*, will have an interactive component that will engage the on-line community with online surveys and live tweeting.

The WFH multidisciplinary committees have also developed special track sessions for the patient and healthcare professional in musculoskeletal, laboratory sciences, dental, psychosocial, and nursing disciplines on challenges and solutions to improve treatment and care for the bleeding disorder patient.

The WFH and NHF look forward to welcoming you to Orlando for the next World Congress. Early registration and call for abstracts is open until January 22, 2016, at www.wfh.org/congress.

WFH 2016 WORLD CONGRESS

10 PROFESSIONAL DEVELOPMENT SESSIONS

10 PLENARIES

2 LATE BREAKING TRIAL SESSIONS

52 CONGRESS SESSIONS

12 ABSTRACT-BASED FREE PAPERS SESSIONS

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Addressing the need for a comprehensive and integrated approach to healthcare development

Earlier in the fall, I joined a delegation travelling to Senegal where we marked the expansion of the WFH Humanitarian Aid Program. While talking to patients, it became evident that the expansion of the WFH Humanitarian Aid Program will allow for more sustainable and predictable flow of treatment products into countries most in need. This will now allow for outlying communities to also benefit, not just in the major cities or centres.

/Alain Weill, WFH PRESIDENT

The other point that was made clear to our delegation was that outreach and education were paramount to identifying those patients in need. Treatment products alone cannot solve this challenge. It is up to healthcare professionals, patient organizations, and volunteers to educate people in their communities to what daily life is like for people in inherited bleeding disorders. However, this often falls to already strained and resource-stretched volunteer networks.

When we look at the WFH's strategic goal of improving access to safe and effective products through advocacy and product donations, we cannot forget the other areas that are important to those in developing countries. It is imperative as a global community that we work to improve diagnosis and access to treatment for all people with inherited bleeding disorders. The WFH will continue to help build capacity of our national member organizations (NMOs) to better serve the inherited bleeding disorders community.

In many developing countries governments do not have the resources to purchase treatment products, let alone provide basic hemophilia care. The reality for the people with hemophilia in those countries is to face life with severe disabilities or tragically shortened life expectancies.

To address the education and training for countries that have yet developed basic hemophilia care systems, the WFH launched the Cornerstone Initiative in 2013.

Developing countries may be considered for a Cornerstone Initiative when they, or their regions, demonstrate initiative, motivation and potential to develop basic standards of care. In addition, the WFH has endorsed the French African Alliance for the Treatment of Hemophilia (AFATH) program which works within French-speaking sub-Saharan Africa. This program is coordinated by the French Hemophilia Society (AFH) and the ultimate aim is for these countries to join the WFH and become a WFH NMO.

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“In many developing countries governments do not have the resources to purchase treatment products, let alone provide basic hemophilia care. The reality for the people with hemophilia in those countries is to face life with severe disabilities or tragically shortened life expectancies.”

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Once basic care standards are established in these countries and there is the ability to work more in-depth on improving hemophilia care, countries may be considered for WFH Country Programs. When a Country Program demonstrates the potential for establishing a national hemophilia care program that is integrated within the health system and covers all areas of hemophilia care development, it may be considered for the Global Alliance for Progress (GAP) Program. The GAP Program aims to identify an additional 50,000 people with inherited



bleeding disorders by 2022, with 50 per cent of them living in the world's poorest regions.

For WFH NMOs, we carry out our work through country-specific, global programs, and activities. This includes working with NMOs to implement national care programs to achieve sustainable comprehensive care, and targeted country development action plans. Expanding training for healthcare professionals as well as training to build capacity to achieve accurate laboratory diagnosis is achieved through training workshops. Programs such as Advocacy in Action and the WFH Twinning Program help implement NMO leadership skills training nationally, regionally and globally. This is complemented with promoting WFH NMO development through events like the WFH Global NMO training that always takes place a few days before a Congress.

WFH country-specific programs and healthcare development programs play an essential role in combination with the WFH Humanitarian Aid Program. The ability to identify, diagnose, train, and then treat those most in need are all part of advancing our vision of Treatment for All. As the WFH works towards improving access to safe and effective products through advocacy and product donations, the continued work through our programs will help to improve and sustain care for people with inherited bleeding disorders around the world.

Historical perspective on the treatment of hemophilia: Understanding the value of experience

/Marijke van den Berg, VICE-PRESIDENT MEDICAL

As we enter into an era of increased scrutiny over healthcare costs and the associated benefits of providing certain treatment regimens, it has become quite evident that health technology assessments (HTA) may make recommendations on how hemophilia treatment is managed. The question that arises is whether or not the correct evidence is used to justify financial decisions that could significantly impact health outcomes for those living with hemophilia. The unfortunate outcome are assessments derived from data that does not take into consideration decades of real-life treatment experience and the significant quality of life improvements that treatment regimens, such as prophylaxis, provides for patients.

In 1958, a historic paper was published by Biggs and Farlane, who proposed a diagnosis of hemophilia based on the missing amount of factor VIII or IX. Severe hemophilia was characterized by < 1% FVIII or FIX, moderate hemophilia by 1-5%, and mild hemophilia by >5%.

These early laboratory diagnoses were correlated with clinical observation in well-defined patients groups in Sweden. In 1964, Ahlberg published a paper which showed a clear correlation between the severity of hemophilia and the age bleedings started as well as the number of joint bleedings.

This was the main background for healthcare professionals to consider regular infusion with plasma component, later with cryoprecipitate, in order to raise the factor level in severe hemophilia to the level present in moderate hemophilia. This was first offered to patients who had the most frequent bleedings and it clearly was demonstrated to reduce bleeding.

An obvious further development in the treatment regimen of prophylaxis was decreasing the age at which this therapy was started, in order to raise the pre-infusion level above 1%. This led to the effective prevention of bleeding and in fact changed the patient's diagnosis from severe hemophilia to moderate hemophilia.

Many observational studies have demonstrated with excellent results that this strategy is effective and we now see that patients with

severe hemophilia can have a normal life expectancy, reach adulthood with normal or near-normal joints and are fully engaged in activities similar to their peers.

For the hemophilia community, especially in Europe, it is inconceivable that this basic treatment regimen based on a well-defined pathophysiologic concept needs additional proof through randomized controlled trials. However, this discussion is beginning to be raised in several countries. The challenge of performing randomized controlled trials in hemophilia is that the bleeding patterns between patients, even with severe hemophilia, is variable. Disabilities due to complications from bleeding would only become evident after many years of monitoring and follow-up, meaning that patients are put into inevitable situations of additional health complications of joint damage and suffering.

The most significant clinical trial, leading at last to the provision of prophylaxis for children within the United States, was performed by Manco-Johnson and her colleagues (NEJM 2007). In a direct comparison between prophylactic and intensive on-demand therapy, after a six year follow-up period, it was concluded that children who did not experience bleeding were healthier than children who had to suffer bleeding. The latter group experienced more joint abnormalities identified on MRI. In this study no clear correlation was found between the number of bleeds these children had experienced and the severity of joint damage as observed with MRI. This shows the weakness of using only bleeding as a clinical measure to determine outcomes for the patient, particularly over such a relatively short period of time. More randomized controlled clinical trial studies have been published and all show a beneficial outcome for prophylaxis, including the last Cochrane Evaluation of the concept of prophylaxis, concluding that prophylaxis provided beneficial outcomes for patients with hemophilia.

Even considering the overwhelming evidence from both these trials and real-life experiences of healthcare professionals providing prophylaxis to their patients, the reality is that we now live in the era of health technology assessments (HTAs), designed to determine the correlation between cost of treatment and outcome. This is especially prevalent when



there needs to be a determination of paying for treatment for a growing population of patients with hemophilia.

The bleeding disorders community has to take an active role in the discussion, but has also has to actively encourage additional data collection and assist in providing them, especially in countries that do not have access to treatment at the same levels as developed countries. Well-defined registries, collecting data in a comparable way, from the diagnosis to the provision of treatment, provide the opportunity to demonstrate that without sufficient treatment, patients with severe hemophilia still struggle with their condition and have a shorter life expectancy. This is not only crucial for individual countries working to improve access to treatment, but also for the collection of prospective data on outcomes in many parts of the world.

Within this area of hemophilia care, the World Federation of Hemophilia (WFH) is investigating how to deliver a framework for data collection that will help the global community gather valuable data on diagnosis, bleeding scores, treatment regimen, and results of validated outcome assessment tools. Choosing too narrow a focus, as happened in some of the more recent HTAs, negates the many years of real-world experience of those working directly with patient populations who have significantly benefited from prophylaxis. It is our responsibility not to place patients in situations where their quality of life will be put at risk for many years because the main focus is on financial analyses.



Ninth WFH Global Forum on Research and Treatment Products for Bleeding Disorders

Access to safe and effective treatment products continues to improve each year due to the tireless advocacy from the global bleeding disorders community. This strategic priority for the WFH, coupled with the continued support of clinical research which provides evidence to make the case for better care, were integrally linked throughout the WFH Global Forum.

/Mark Brooker, WFH SENIOR PUBLIC POLICY OFFICER

For the first time, the WFH combined our Global Forum on Treatment Products for Bleeding Disorders with our Global Research Forum, covering the latest developments in both areas. The speakers addressed issues related to the safety and supply of treatment products, as well as discussing and debating critical issues and challenges in the areas of research and clinical trials related to bleeding disorders and their treatment products. More than 170 people from over 30 countries attended the 9th Global Forum, which was held October 22-23, bringing together patient groups, regulators, representatives from industry, not-for-profit fractionators, and researchers as well as doctors who treat people with bleeding disorders.

The first day of the Global Forum focused on safety and supply of treatment products. A session on inhibitors development in hemophilia highlighted the need for continued efforts to develop global surveillance to monitor and understand inhibitors. That session was followed by a detailed look at various supply issues affecting our global community including WFH efforts to increase the world supply of clotting factor concentrates in a sustainable way.

The second day covered research, including novel treatment products that are not clotting factor concentrates and updates on various trials of gene therapy for hemophilia A and B.

There was also discussion of how much gene therapy might cost and how it could eventually be rolled out around the world. The WFH Clinical Research Program was also featured and two presenters gave updates on the work they are doing with the support of WFH Clinical Research Grants.

During the manufacturers session that closed the Global Forum, a record 10 companies presented data on newly licensed or about to be licensed treatment products as well as sharing data from ongoing clinical trials.

The WFH wishes to thank the sponsors for supporting the Global Forum: Baxalta, Bayer, Biogen, Canadian Blood Services (CBS), CSL Behring, Hema Québec, Novo Nordisk, Octapharma, Ministère des Relations Internationales du Québec (MRI), and SOBI.

The proceedings of the Global Forum will be published later this year. Many presentations from the meeting are already available on our website.

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The expansion of the WFH Humanitarian Aid Program: Transforming the lives of those most in need

In the regional hospital of Thies, Senegal, about twenty families sat around a large boardroom table. Many had travelled over an hour by bus to attend this event. Mothers with their teenage sons, fathers holding their small children, all looking for the same thing; treatment for their children which has proven so hard to come by.

/Sarah Ford, WFH COMMUNICATIONS AND MARKETING DIRECTOR

Each shared their experiences and each recounted how they are determined for things to change. What was exceptionally heart-breaking were the stories of their children who were not there – the ones that died in infancy from complications due to bleeding, who died before they were even diagnosed.

This gathering was the final event in a three-day visit to Senegal during which the World Federation of Hemophilia (WFH) marked the commencement of the delivery of humanitarian aid donations from global pharmaceutical companies Biogen and Sobi. Patients from the region surrounding Thies had gathered together to tell their stories and to explain what is desperately needed—diagnosis, education, and treatment.

Just prior to this visit with patients and their families, the WFH co-hosted a luncheon with Prof. Saliou Diop, WFH Board Member and Director of the National Center of Blood Transfusion in Dakar. Through testimonial by members of the bleeding disorders community, both from the patient and healthcare

professional perspectives, this event highlighted the reality of hemophilia care in developing countries and the need for predictable and sustainable WFH humanitarian aid donations. Attendees included those leading the donation efforts to this program, along with leading representatives of inherited bleeding disorders care in Senegal.

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“Expanding the WFH Humanitarian Aid Program has been part of advancing the WFH vision of Treatment for All, through many years of strategic planning and outreach to global partners. Improving diagnosis and treatment in developing and emerging countries has been one of the highest priorities for the WFH.”

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A poignant moment during this event was the announcement by the representative of the Ministry of Health that factor replacement therapy for hemophilia was now being added onto the Essential Medicines List

for Senegal. This accomplishment was due to many years of advocacy work by the national patient organization Association Sénégalaise des Hémophiles and healthcare professionals within the country such as Prof. Diop. In addition, through the many years of humanitarian aid donations from the WFH, the government was able to recognize the successful outcomes of providing factor replacement therapy. Within countries such as Senegal, the WFH Humanitarian Aid Program serves as a catalyst for the development of sustainable national care programs for the treatment of bleeding disorders, as well as becoming leverage to encourage local health authorities to invest in this necessary treatment.

Expanding the WFH Humanitarian Aid Program is part of advancing the WFH vision of Treatment for All, through many years of strategic planning and outreach to global partners. Improving diagnosis and treatment in developing and emerging countries has been one of the highest priorities for the WFH and the recognition that humanitarian aid plays an integral role within this strategy has been paramount. In recent years, it has



WORLD HEMOPHILIA DAY 2016

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APRIL 17
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Treatment for All is the vision of all

On April 17, the World Federation of Hemophilia (WFH) is inviting our community to come together and work towards reaching the shared vision of Treatment for All. World Hemophilia Day is a perfect opportunity to raise awareness about hemophilia and other inherited bleeding disorders, but also to consider how each of us can contribute to improving the lives of those living with one.

Globally 1 in 1,000 people has a bleeding disorder but most are not diagnosed and do not receive treatment. Together we can change that.

By marking World Hemophilia Day on April 17 and encouraging those in and outside of the bleeding disorders community to provide support and information to those who are in need, we can all play a part in reaching the shared goal of seeing every person with a bleeding disorder diagnosed and treated.

World Hemophilia Day provides an opportunity to talk to your family and friends, colleagues, and caregivers to raise awareness and increase support for those living with an inherited bleeding disorder.



become evident that there are some developing countries where, for the foreseeable future, governments do not have the financial resources to provide treatment products at the current prices for their bleeding disorders populations. The need for a sustainable and predictable humanitarian aid stream is the only chance for these groups of patients and their families to receive access to diagnosis and then treatment.

An increasing number of partners within the global bleeding disorders community have accepted this challenge and have helped provide humanitarian aid treatment for those most in need. Through the donation to the WFH by Biogen and Sobi of 500 million IUs over five years, along with the continued efforts of the Canadian Blood Services, Biotest, and Grifols with Project Recovery, and the work by the Italian National Blood Services through Project Wish, there will now be a more predictable and sustainable flow of humanitarian aid donations to the global community. In addition, CSL Behring and Grifols have signed multi-year commitments of treatment products which will contribute to the expansion of the WFH Humanitarian Aid Program.

To ensure that this program is sustainable and that the channel of aid is predictable, a collaborative effort is needed among companies, countries, and the global hemophilia community. With increased capacity, the development of expertise still needs to continue and outreach to those who remain undiagnosed is still a priority for the WFH. Continued investment in healthcare development programs, educational resources, training for healthcare professionals, and raising awareness are all needed to realize the vision of Treatment for All.

When the WFH delegation visited the hemophilia treatment centre during their visit to Dakar, Senegal. In Dakar, they came across a young boy named Bachir. This young boy had started receiving treatment products donated through the WFH over five years ago. Without this treatment and care he received through the healthcare team in Dakar, Bachir would have faced many more obstacles throughout his young life. He is one of many children that will see a drastic change in how they will live their lives now that treatment will become more predictable.



Project WISH provides vital support to reduce the gap in access to treatment

Representatives of the World Federation of Hemophilia (WFH) and the Centro Nazionale Sangue (Italian National Blood Centre, CNS) signed the project WISH agreement on June 29, 2015, to continue to help reduce the gap in access to clotting factor concentrates.

/ Assad Haffar, WFH HUMANITARIAN AID DIRECTOR

The WISH project (World Federation of Hemophilia and Italian National Blood Centre for a Sustainable Supply for Haemophilia patients) is a humanitarian program consisting of a not-for-profit distribution, over a five-year period, of up to 150 million IUs of factor VIII. This will be derived from voluntary non-remunerated blood donations in Italy, produced under toll-fractionation agreements and exceeding the treatment needs of Italian hemophilia patients.

The partnership between CNS and the WFH aims at providing recipient countries with medium- to long-term products supply. This will be based on specific agreements to be settled between Italian Regions and recipient countries.

The WISH project is based on the commitment by the CNS to the WFH humanitarian aid mandate, ensuring that patients will have a source of treatment. Italian biopharmaceutical company Kedrion Biopharma, as the current partner of the Italian Regions in the toll-fractionation production of plasma-derived medicinal products, has promoted the development and success of this agreement. They have undertaken the regulatory and logistics costs of the shipping of the products to the recipient countries, thus providing a valuable contribution to broaden access to clotting factor therapies worldwide.

Project WISH demonstrates the commitment of these partners to the mission of the WFH, to improve and sustain care for people most in need around the world.

Over 2.9 million IUs of humanitarian aid donated to date through Project Recovery

The World Federation of Hemophilia (WFH) is leading the effort to change the lack of access to treatment products in developing countries for people with inherited bleeding disorders.

/ Assad Haffar, WFH HUMANITARIAN AID DIRECTOR

Project Recovery, an innovative humanitarian aid project, has played an integral role in providing consistent and predictable access to life-saving treatment to those most in need.

Originally conceived and supported by the Canadian Hemophilia Society (CHS), Project Recovery turns unused cryoprecipitate paste from Canadian Blood Services' plasma into factor VIII concentrate with a full shelf life. Partnering with Biotest, for their role in the manufacturing and shipping of the final product, along with the harvesting of the cryoprecipitate and testing done by Grifols, over 2.9 million IUs of humanitarian aid has been shipped to treatment centres around the world since the project began in late 2013.

This reliable source of humanitarian aid will allow the WFH to provide sustainable and predictable channelling of factor VIII treatment products. Over the next year, another 4.3 million IUs will be distributed to additional countries, providing a steady flow of treatment products to the WFH network.

Project Recovery has contributed to the expansion of the WFH Humanitarian Aid Program and the support of the Canadian Hemophilia Society, the Canadian Blood Services, Grifols, and Biotest demonstrate their global commitment in supporting those most in need in the bleeding disorders community. The leadership from these partners will help to continue to serve as a model of cooperation and dedication to other countries in similar situations to Canada.

Homemade jam, Pepaw, and hemophilia

Dylan DeMatteis, already at 15 years of age, is an aspiring filmmaker from Ohio who is an avid fan of rock and roll; he collects vintage vinyl and plays the drum with the same tenacity that he takes to the basketball court. In his downtime, he follows the exploits of his favorite ice hockey team—the Lake Erie Monsters.



/Roddy Doucet, INTERIM WFH ANNUAL GIVING MANAGER

The idle moments though are rare for this busy teenager as he pursues his dream of going to film school and traveling Europe to share his love of music and acquire the necessary life experience to write and direct great stories for the big and small screen. If that is not enough, he also finds time to manage his hemophilia.

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"After settling upon homemade jam as their product, Dylan's classmates voted to donate all proceeds to the hemophilia community. Dylan was thrilled with their choice, recognizing that education and engagement are the best way to move towards the WFH goal of treatment for all."
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While in middle school he noticed that his classmates did not understand hemophilia, a condition he has managed with the help of family and a dedicated healthcare team for as far back as he can recall. One of his earliest memories is receiving his "magic button", a med-port he renamed much to the merriment of his family, who then marveled at his irrepressible spirit shortly after at a wedding where he danced the night away sporting a cast to allow a spontaneous bleed the chance to heal.

With a zeal for raising awareness and community engagement, fostered by the memory of his grandfather "Pepaw" Charles Carmen (WFH president, 1988-1993), he decided to bridge this gap by taking to the stage of his school to talk about his experience with hemophilia. He admits to believing his classmates would be ambivalent at best. To

his pleasant surprise, his friends, old and new, all displayed a new level of understanding and care about blood disorders after his presentation.

Shortly thereafter, their teacher tasked them with a project where they would manage all aspects of a small business from concept to production and marketing to sales. The students would choose one local charity where they would donate all their profits. After settling upon homemade jam as their product, Dylan's classmates voted to donate all proceeds to the hemophilia community. Dylan was thrilled with their choice, recognizing that education and engagement are the best way to move towards the WFH goal of treatment for all.

He is indeed an inspiration for the blood disorder community; "My advice to someone else living with hemophilia is to think of it as a gift, making you stronger than most people, not as a curse that makes you weaker and don't use it as an excuse for self-pity. My advice to a friend of someone with hemophilia is to support them and to help them by being a friend and listening."

WFH donors support education and outreach programs across the globe. To find out how you can join the growing list of people who are choosing Treatment for All through financial support of the WFH, e-mail Interim Annual Giving Manager Roddy Doucet at rdoucet@wfh.org.

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WFH 2015 ANNUAL APPEAL



A fond farewell

/ Jay Poulton, WFH EDITOR/EDITORIAL SERVICES COORDINATOR

The World Federation of Hemophilia (WFH) has to say goodbye to a long-time member of our family. Elizabeth Myles, COO, has decided to move on to new adventures, leaving her post behind at the end of October.

In 1998, Elizabeth joined the WFH as its Publications and Information Officer. In the time that she has been with the WFH, she has been to eight WFH World Congresses, countless international events, and a seemingly endless amount of meetings, but by far the thing that she remembers most are the people she has met along the way.

"I have been very privileged to be a part of this organization and this community. I am very proud of the contributions I have made and the work of the WFH, but what I will remember most are the people. The WFH staff over the years, the board and volunteers, the NMO leaders, the doctors, the people with bleeding disorders; I have been inspired by their commitment," said Elizabeth, "and they have given me the memories of that I will continue to cherish for years to come."

During her seventeen years with the WFH, the organization has grown by leaps and bounds, not just the internal operations but with the amount of programs dedicated to helping the bleeding disorders community.

On behalf of the entire bleeding disorders community, the WFH extends its deepest thanks to Elizabeth and wishes her the best of luck in the future.

Leadership in training: One story from South Africa

When Dr. Nomawethu Tonjeni, a physician from South Africa, applied to the International Hemophilia Treatment Centre (IHTC) Fellowship Program in 2012, she wanted to learn everything there is to know about the diagnosis and treatment of patients with bleeding disorders.

/ Stephanie Pineda, WFH PROGRAM OFFICER

Having previously attended educational workshops in California, Sendai, as well as the Hemophilia 2010 World Congress in Buenos Aires, Dr. Tonjeni was keen to learn more. She was awarded a fellowship that year, and undertook her training at the Manchester Haemophilia Comprehensive Care Centre, U.K., under the guidance of Prof. Charles Hay.

"My first impression was that of a huge, first-world, well-equipped hospital and the largest laboratory I have ever seen," said Dr. Tonjeni. "The wards, clinics, and weekly programme were well organized". The IHTC had a multi-disciplinary team that included doctors, nurse clinicians, laboratory scientists, physiotherapists, and social workers. She felt that the staff provided her with world-class quality training because for her they were "excellent and knowledgeable teachers."

During Dr. Tonjeni's four-week fellowship in Manchester, she had the opportunity to learn about various aspects of hemophilia treatment. "I learned a lot about the management of pregnant women with thrombosis or bleeding disorders; a service which is not offered in my country." She also attended pediatric hematology clinical sessions, learned about the UK national database, home therapy, using smart phones as tools and received a tutorial about various laboratory investigations relating to hemophilia and hemostasis. Her schedule included observing the co-management of patients with other disciplines, such as Obstetrics and HIV clinic. One of the highlights of her stay at the Manchester Royal Infirmary hospital was the national U.K.

accreditation team visit of the Manchester Haemophilia Comprehensive Care Centre. "It was a lesson on how to establish a hemophilia centre and what needs to be in place" said Dr. Tonjeni.

Nowadays, Dr. Tonjeni visits peripheral hospital and clinics to train other doctors and nurse clinicians on how to diagnose and treat patients with bleeding disorders. She also helps raise awareness in schools, and gives talks on the radio. As a chief specialist physician and head of the Haemophilia Clinic at the Nelson Mandela Centre Hospital in Mthatha, Dr. Tonjeni has noticed an increase in the number of patients who attend the clinic. She also reports that the senior management of her hospital is fully supportive and provides the budget so that the clinic has continuous supply of factors. Their laboratory services have also improved, physiotherapists have received up to date training, there is an urologist for medically supervised circumcision, and there are now regular dental check-ups for patients.

Dr. Tonjeni is now part of a network of more than 600 trained IHTC fellowship alumni who are trying to improve care of patients with bleeding disorders around the world. She is grateful for her IHTC Fellowship training. "It has opened my eyes to a lot of things: how to set up a clinic, what needs to be in place, the organization of the clinic, research, database, home therapy, prophylaxis, use of smartphones, importance of support services (multidisciplinary team) and much more."

The WFH would like to thank Bayer for their exclusive support of the IHTC program.

Global Forum speakers highlight need for inhibitor surveillance

Inhibitors—antibodies to infused factor VIII or factor IX produced by the immune system, making treatment ineffective—are the foremost treatment complication faced by people with hemophilia today, associated with increased morbidity, increased mortality, and a greatly increased financial burden, said Dr. Mike Soucie of the U.S. Centers For Disease Control And Prevention (CDC) in the inhibitors session at the 2015 WFH Global Forum.

/Debbie Hum, WFH CONTRIBUTING AUTHOR

For clinicians and researchers, inhibitors are a multi-factorial event. To investigate inhibitors in a rare disease large international collaborations are required in the future. Following U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) inhibitor workshops in 2003 and 2005, the regulatory agencies stated that clinical trials are inadequate to assess risk and recommended long-term surveillance using registries.

INHIBITOR SURVEILLANCE IN THE U.S.

The U.S. Hemophilia Inhibitor Research Study (HIRS) was initiated in 2006 as a pilot project for prospective national inhibitor surveillance. Key findings of the HIRS study reported in 2012 confirmed that all hemophilia patients are at risk; the largest risk is in severe hemophilia A with an incidence rate of 25-30% during the first 50 exposure days. Inhibitors occur also in patients with mild hemophilia, patients with low-risk mutations, and patients with over 150 exposure days; although the risk for inhibitors after 150 exposure days is very rare. National inhibitor surveillance requires a standardized protocol specifying the patients to be tested, testing intervals, and standardized, centralized testing, Dr. Soucie emphasized.

FDA IMMUNOGENICITY WORKSHOP

Inhibitors present numerous challenges to different stakeholders, noted Dr. Glenn Pierce, medical member of the WFH Board of Directors. For patients, caregivers, and the healthcare system, inhibitors present economic costs as well as human costs in terms of increased morbidity and possibility of life-threatening circumstances leading to mortality. For industry, inhibitors development during the production of new drugs present added risks to drug development costs. The lack of predictive tools means antibodies are detected only in late Phase 3 trials after significant expenses have accrued. For regulatory agencies, novel bioengineered products are becoming the norm, however, the immune consequences of neo-epitopes are difficult to evaluate and need large patient populations.

Dr. Pierce presented a summary report on the FDA Public Workshop on New Methods to Predict the Immunogenicity of Therapeutic Coagulation Proteins, held in September 2015. The workshop focused on the genetic determinants of immunogenicity and highlighted the importance of well-characterized clinical samples. It also looked at resources such as hemophilia registries and databases, genotyping initiatives and biological samples and highlighted the importance of integrating data for better patient outcomes.

Novel engineered coagulation proteins emerging from the drug development pipeline present new concerns and greater risks have been identified for subsets of patients, Dr. Pierce said. “Virtually all biotechnology drugs provoke immune response in some patients, though usually just tiny fractions. However, the reactions are becoming of greater concern as the number of protein drugs increases.”

EMA WORKSHOP ON HEMOPHILIA REGISTRIES

The key challenge with hemophilia registries is that there is no overarching structure for how to manage, design or host data collection,

said Dr. Anneliese Hilger of Germany’s Paul-Ehrlich Institute and the EMA. She presented a summary of the EMA workshop on hemophilia registries which was held in London in July 2015.

The workshop addressed a number of crucial questions, including whether the current number of hemophilia registries improve patient safety and lead to a better research in the field of hemophilia. Workshop participants reached a number of important consensus points on what is needed to improve hemophilia data collection. Ideally, every patient should be in a disease registry, and patients enrolled in clinical trials should remain in registries. Patient identifiers are needed to avoid overlap between registries and reduce double-counting. There is a need for collaboration among all stakeholders, and need for agreement with regulators and other stakeholders on a minimum protocol or dataset. Furthermore, it is important to link with the initiatives of other rare disease registries as there will be common issues. Finally, there is a need to harmonize national registries and promote and support more national registries and quality assurance.

WFH VOLUNTEER AWARDS

DO YOU KNOW SOMEONE WHO HAS MADE AN OUTSTANDING CONTRIBUTION TO GLOBAL HEMOPHILIA CARE?

The WFH is now accepting nominations for the International Frank Schnabel Volunteer Award and the International Healthcare Volunteer Award.

Visit www.wfh.org/awards for more information and to submit your nomination.



WFH Clinical Research Grant supports new clinical investigations

Lize van Vulpen knows that without the assistance of the World Federation of Hemophilia (WFH) Clinical Research Grant she might never have been able to undertake her research into joint distraction in the treatment of ankle arthropathy in people with hemophilia.



/Christine Herr, WFH DATA AND RESEARCH COORDINATOR

“Besides the financial support, the grant also was an acknowledgement by the WFH that our research was important and worthwhile investigating. This helped me convince all the people I needed to collaborate with to make this project possible,” said van Vulpen.

Van Vulpen, recipient of a 2014 grant along with principle investigator Roger Schutgens from the University Medical Center Utrecht, Utrecht, the Netherlands, wanted to look into the value of joint distraction from a patient oriented point of view with the primary objective being symptoms and functionality.

“The grant requires a direct clinical objective. This fitted perfectly with the proposal I was writing,” said van Vulpen.

The direct clinical objective provides a means for researchers to directly influence the treatment of people with inherited bleeding disorders and potentially provide the groundwork to improve the lives of patients.

“By designing my research I try at first to formulate what the problem is for the patient. From that point of view I try to formulate a research question that in the end adds to an improvement of the life of the patients. Sometimes this link is difficult to see, as

I am also performing basic research. But for instance the research focusing on the identification of biomarkers of joint damage, I’m convinced that this ultimately leads to better and more sensitive trials investigating the impact of our interventions on joint outcome.”

Since the grants launch in 2013, the WFH has awarded a total of eight grants / projects funded. A total of \$363,770 has been distributed. Please note that part of this will be distributed at the beginning of 2016 as the second year of funding for the 2015 winners.

WFH Board of Director Nominations

Members of the World Federation of Hemophilia (WFH) Board of Directors play a key role in setting the strategic direction and ensuring that the organization represents the needs of the global bleeding disorders community. Board members are elected at the WFH General Assembly, following the WFH World Congress. WFH national member organizations (NMOs) who are fully accredited and who have paid their subscriptions may nominate people to stand for election to the board of directors. Nominations for candidates for the Board of Directors will be accepted as of March 1, 2016. Individuals interested in running for a position on the board of directors should contact the NMO in their country to express their interest in being nominated.

The following positions will be elected, President, two lay members (people with a bleeding disorder or a parent)

and two medical members. These positions are for a four-year term. Each NMO may submit one nomination for president. In addition, each NMO may nominate two medical nominees (medical doctors), and two lay nominees (people with a bleeding disorder, or a parent), one from their own country or region, and another from anywhere else in the world. All nominations forms must be signed by the president/chair of the nominating NMO. After the nomination deadline WFH headquarters will count the number of nominations and ascertain if the nominee has received the required number of nominations. Sixty days prior to the General Assembly, WFH headquarters will circulate the final list of candidates together with their curriculum vitae (CV) to all NMOs for their consideration. At the WFH General assembly, all eligible NMOs will vote for the candidates of their choice for the respective positions.

Nominations must be received at WFH headquarters no later than:

Friday, April 30, 2016, at 17:00 Eastern Standard Time.

For more information about the WFH board positions and the nomination and election process, or to submit a nomination form, please send an e-mail to WFHElections2016@wfh.org.

Thank You

In recognition of the organizations that have committed or contributed to the WFH's mission

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Jan Willem André de la Porte

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von Willebrand Disease web section: Octapharma

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*WFH is proud to acknowledge the support of our national member organizations

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:

The Editor, Hemophilia World
Fédération mondiale de l'hémophilie
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Montréal, Québec H3G 1T7 Canada
E-mail: jpoulton@wfh.org

Calendar of events

9th Annual Conference of the European Association for Haemophilia and Allied Disorders

Feb 3-5, 2016
Malmö, Sweden
Tel: +44 (0)1355 244 966
Fax: +44 (0)1355 249 959
www.eahad2016.com

International Plasma Protein Congress PPTA

March 22-23
Barcelona, Spain
www.pptaglobal.org

60th Annual Meeting of the Society of Thrombosis and Haemostasis Research

17-20 February 2016
Münster, Germany
Tel.: +49 (0)30 20 45 936
Email: gth@mci-group.com
www.gth2016.org

IPFA Asia Pacific Workshop on Plasma Quality and Supply

March 8-9, 2016
Taipei, Taiwan
Tel.: +31 20 512 3561
Email: info@ipfa.nl
www.ipfa.nl

13th International Hemophilia Congress of Turkey

April 17-19, 2016
Istanbul, Turkey
www.turkiyehemofilikongresi.com

IPFA/PEI 23rd International Workshop on "Surveillance and Screening of Blood Borne Pathogens"

May 25-26, 2016
Lisbon, Portugal
Tel: +31 20 512 3561
Email: info@ipfa.nl
www.ipfa.nl

WFH 2016 World Congress

July 24-28, 2016
Orlando, USA
Tel: +1 514-394-2834
Email: info2016@wfh.org
www.wfh.org/congress/en

Spanish Hematology Meeting

April 20-21, 2017
Buenos Aires, Argentina
www.acamedbai.org.ar/iihema.php

TREATMENT FOR ALL THE VISION OF ALL



WORLD
HEMOPHILIA DAY
2016 | APRIL 17

Globally 1 in 1,000 people
 has a bleeding disorder
 most are not diagnosed and
 do not receive treatment




TOGETHER WE CAN CHANGE THAT



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WFH

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