

# HEMOPHILIA WORLD

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## WFH launches new strategic plan

/Elizabeth Myles, INTERIM WFH CHIEF EXECUTIVE OFFICER / WFH CHIEF OPERATING OFFICER

In 2014, the World Federation of Hemophilia (WFH) underwent a comprehensive process to develop its strategic direction for the next three to five years. After consultation with key stakeholders, and an assessment of opportunities and challenges facing the global bleeding disorders community, WFH Board and staff developed a new strategic plan to ensure the continued success of the WFH in our mission to improve care for all people with inherited bleeding disorders.

Over the next three years, we will continue our work to improve care for all those with inherited bleeding disorders, and this plan includes a number of enhancements to address the opportunities and challenges of our changing environment, including access to treatment products.

### IMPROVING ACCESS TO SAFE AND EFFECTIVE PRODUCTS

With new treatments poised to enter the market, and gene therapy likely to become a reality in the near future, the bleeding disorders community is entering a new era of access to care. While there are many unknowns around access, safety, and pricing, one thing that seems sure is

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# COVER STORY

that there will be greater supply and variety of products with which to treat those in need.

Because of this, improving access to safe and effective products is an important focus of the new strategic plan. Through our Humanitarian Aid Program and our role monitoring safety and supply issues, we have been involved in product supply and safety for many years. Now thanks to increased humanitarian aid donations and the success of Project Recovery, the WFH is focusing on providing long-term access to treatment in developing countries.

## IMPROVING DIAGNOSIS AND TREATMENT IN DEVELOPING COUNTRIES

Establishing sustainable care in developing and emerging countries continues to be a core focus of the WFH, using our development model and program tools to bring about long-term sustainable improvements in the delivery of care. In the next three years, we will ensure our programs include people with von Willebrand disease, those with rare factor deficiencies, women with bleeding disorders, inhibitors, and other under-recognized populations, so that the needs of *all* people with inherited bleeding disorders are addressed. It is also the goal of the WFH to enhance our regional approach in our programs, where appropriate, to identify regional priorities, develop opportunities for countries facing similar challenges to work together, and maximize impact and effectiveness of our programs and activities.

## BUILDING CAPACITY OF OUR NATIONAL MEMBER ORGANIZATIONS

Our role is to empower patient leaders to serve their community effectively. We will support WFH national member organizations' (NMO) organizational capacity to represent the needs of all the people with inherited bleeding disorders. We will promote leadership development for youth and people with inherited bleeding disorders within NMOs, and support NMOs and patients to better advocate for the improvement of care for people with inherited bleeding disorders in their country.

## SHARE KNOWLEDGE AND BUILD AWARENESS

Information is a powerful tool, and one of the important ways we empower our community is through increasing knowledge



## Our Strategic Goals 2015–2017

Over the next three years, our strategic goals are to:

1. Improve diagnosis and access to treatment for all people with inherited bleeding disorders.
2. Build capacity of the WFH national member organizations to serve the inherited bleeding disorders community.
3. Share knowledge and build awareness globally through information exchange, education, and training.
4. Define and promote practice standards, collect data, and support clinical research to provide evidence to make the case for better care.
5. Improve access to safe and effective products through advocacy and product donations.
6. Expand our financial base and enhance operational excellence to advance our mission.

and awareness. For healthcare professionals we help deliver effective training and produce relevant educational resources to improve clinical management and patient outcomes. We will develop and share information to help educate people with bleeding disorders and their families. As our community is small and sometimes isolated, it is our goal to capitalize on digital strategies, such as improving online platforms and social media contributions, to increase awareness, provide networking and other learning opportunities.

## MAKING THE CASE FOR BETTER CARE

Our aim is to continue to define and develop standards of care, such as the WFH Treatment Guidelines, to document where the best evidence exists to support clinical practice. Similarly, through our research program, we will fund research to improve clinical management and standards of care. We will continue to enhance and expand our

data collection to support advocacy efforts and build the evidence to make the case for better care.

## MAXIMIZING OUR RESOURCES

We shall ensure the financial health of the organization and develop sustainable and increasing sources of funding. We will function in an effective and agile way, by assessing our current resources and continuously improving efficiency and effectiveness. We will also capitalize on our digital and online platforms to support all aspects of our work.

## OUR VALUES

During this process, we identified the values that shape our organizational culture and guide our decision-making. People with bleeding disorders are at the centre of everything we do and every decision we make, so **patients first** is our core value. Our other values are collaboration, integrity, respect, solidarity, and excellence.

# United to achieve Treatment for All

As the World Federation of Hemophilia (WFH) continues its mission in 2015, I am reminded of all that we have accomplished. At the same time, I am continually aware of all that is left to do for the global bleeding disorders community. The past will always be with us but we must keep pushing forward.

/ Alain Weill, WFH PRESIDENT

The WFH Strategic Plan provides a framework for our organization, determining what the most important goals are to accomplish. There is continuity with our previous plan's direction, towards building sustainable care for bleeding disorders, but this plan includes a number of enhancements to address new opportunities and challenges. I encourage you to read more about our new Strategic Plan on pages 1 and 2 of this issue.

The 2015-17 plan focuses on the following areas:

- Improving access to safe and effective treatment products through advocacy and product donations;
- Building the evidence base, through data collection, our research program, and other tools, to advocate for better care;
- Incorporating underrepresented populations (including VWD, rare factor deficiencies, and platelet disorders) in all we do;
- Building capacity and empowering our national member organizations (NMOs) and patients to serve their community successfully;
- Adapting our healthcare development approach to regional realities; and
- Capitalizing on digital technologies to amplify our activities and connect our network.

It is an exciting time for the WFH as we set out to expand our Humanitarian Aid Program. The WFH's vision of Treatment for All is at the forefront of our minds as we intend to reach out to populations within our community that have not yet received adequate treatment and care.

In addition, we will further expand our digital capabilities to the global bleeding disorders community. Effective communication between

all participants within our global community is indeed attainable and the WFH places high importance on extending our reach to those who want the latest information about our community, our programs, and scientific innovation. The WFH will look to provide opportunities for our community to reach out online and through social media outlets to connect with people from different parts of the world. This will help bring together people facing similar challenges and at the same time help educate the world about all that is left to accomplish.

While these WFH initiatives will indeed open new possibilities, we are constantly reminded of the challenges facing our organization and our NMOs. Global economic markets are constantly changing, for the better or the worse. Many governments in developed and developing countries are also demanding more clinical evidence to justify the costs associated with treatments. We must remain vigilant to address these new economic realities and advocate effectively ensuring adequate treatment will be the reality for any person with a bleeding disorder.

Our community is indeed global but each region has different and varied needs. A 'one size fits all' approach is neither realistic nor attainable. Through evidence and the effective use of data, each country can provide the necessary information to their own government to justify appropriate spending on treatments and care for their own community.

In addition, when we focus too narrowly within each region, we run the risk of isolating these communities. It is only through a global network that we can remain united during these challenges. The WFH provides the solid foundation to support all our national member organizations to achieve their own objectives. The WFH will continue to adapt our healthcare development approaches to accommodate these regional realities while at the same time



“Effective communication between all participants within our global community is indeed attainable and the WFH places high importance on extending our reach to those who want the latest information about our community, our programs, and scientific innovation.”

providing global support based on experience and expertise from around the world.

We cannot lose sight of all the immense work that has already occurred. Indeed, all the struggles that our community has faced with regards to the tragedies during the tainted blood crisis and the work to secure safe and effective treatments, reminds us that we have indeed worked hard to improve the lives of all people with bleeding disorders. We must never forget all members of our community who have died; the many that are still struggling, along with their families; and that safety of treatments will always be a top priority for the WFH. Now is our time to push forward and recognize that we are stronger united. We look forward to 2015 and beyond as we continue to work towards achieving Treatment for All.





## BUILDING A FAMILY OF SUPPORT

Join us on April 17 to raise awareness about bleeding disorders and the need to build a family of support for those living with them.

Families come in many forms but they all share the ability to support and advocate.

# WORLD HEMOPHILIA DAY 2015 | APRIL 17



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# Gene therapy in hemophilia

/Marijke van den Berg, WFH VICE-PRESIDENT MEDICAL

When I became involved in hemophilia in the early nineties, I was asked why I chose a career in this field. At this point, there was a sufficient amount of clotting factors available in many developed countries and it seemed that it was only a matter of a few years before gene therapy would become a reality. In recent years, this breakthrough appears to be within reach.

In November 2014, a very important milestone regarding gene therapy and clinical trials for people with hemophilia B occurred when the New England Journal of Medicine (NEJM) published a paper entitled "Long-term safety and efficacy of factor IX gene therapy in hemophilia B" written by Amit Nathwani Ph.D. et al.

Significant work and interest in gene therapy started with the cloning of the factor genes in the early 1980s. Hemophilia was a very interesting model for gene therapy as only a small increase of factor level can dramatically decrease the number of bleedings and prevent athroopathy. Through this type of treatment, patients with severe hemophilia could attain moderate hemophilia clotting levels, thus leading to an increased reduction in bleeding. The clinical principle of changing severe hemophilia into moderate hemophilia is also the basis for the use of prophylaxis on patients with severe hemophilia. However, high treatment costs and lifelong injections would limit access to prophylaxis for many patients, making gene therapy likely a preferred option.

## THEORY AND PRACTICE COULD NOT BE FURTHER APART

Why did it take so long for gene therapy to become available? The reality was that it took many years until it was possible to produce safe recombinant adeno-associated virus (AAV) vectors that would also reduce the risk of inducing cancer through the infusion of viral vectors. A dramatic event happened in the 1990s when leukemia occurred after gene therapy was given to a child with primary immune deficiency. This event caused regulatory authorities to develop guidelines for the production of safe recombinant AAV vectors and all clinical trials for gene therapy were stopped at that time.

The process to develop safer and more reliable vectors took many years and also the concern that gene therapy could induce immunogenicity hampered much of the initial enthusiasm. The perception that gene therapy has many unpredictable risks is still on the minds of many patients and physicians today.

It is therefore promising that an article on the long-term safety results was published in the NEJM, showing the results of 10 patients with severe hemophilia B who received gene therapy.

The first six patients were enrolled between 2010 and 2011 and they received a low dose of vector. After the low dose was well tolerated, another four patients were enrolled in 2012 with a higher dose in order to increase the overall effect on the factor IX level. In all, 10 patients experienced the effect of the vector infusion which was regular when measured with the analysis of their factor IX level. Also, anti-bodies against factor IX and liver function tests were regularly tested. Since this was the first trial of gene therapy in patients with hemophilia, safety was of utmost concern. All enrolled patients were HIV negative, but 7 out of 10 patients tested positive for hepatitis C virus. The infusion of the vectors was well tolerated with no signs of fever, symptoms, or other general complaints. After a median follow up period of more than three years, the factor IX activity measured in the patients was related to the amount of vector that was initially infused. Patients that received a higher dose have had a higher stable factor IX level than the patients that received the lower dose.

## WHAT WAS THE EFFECT ON THE REGULAR TREATMENT OF THESE PATIENTS?

In four out of seven patients, prophylaxis could be stopped and these patients did not report spontaneous bleedings. A large decrease of additional factor IX infusion was observed for all patients and their annual consumption in IU/kg dropped from a median dose of 2613 IU to 206 IU/kg per year after gene therapy was started. This meant an overall reduction of factor level consumption by 90 per cent.



## ADVERSE EVENTS?

The most reported adverse event was an increase in liver enzymes (ALT levels), this occurred 7 to 10 weeks after the infusion with high dose vector. This adverse event was treated with steroids and during the increase of the ALT levels also a decrease of factor IX activity was found. With steroids the increased levels of the liver enzymes normalized after five days. No factor IX neutralizing antibodies were demonstrated; however a general increase in antibodies against the AAV vector were detectable.

## WHAT CAN WE EXPECT NOW FROM GENE THERAPY IN THE COMING YEARS?

Currently there are several trials ongoing of patients with hemophilia B. However, there is a slow recruitment of patients into these trials, which is caused by both the limited number of patients with no antibodies against AAV vectors and also concern that there is a limited number of patients willing to participate in these trials.

We have come a long way and success in this area of research is evident, as highlighted in the NEJM paper. We continue to look towards the day that all people with bleeding disorders can benefit from the availability of this life-changing treatment.





## WFH launches new Hemophilia World online magazine

/Jay Poulton, WFH EDITOR/EDITORIAL SERVICES COORDINATOR

In 2015, the World Federation of Hemophilia (WFH) has decided to take *Hemophilia World* to a new and visually exciting platform online as part of its mission to reach out to its global community and help increase awareness of people living with a bleeding disorder. An online version of the WFH's popular newsmagazine would provide a greater opportunity for more people to learn about the WFH's programs and news from the global bleeding disorders community.

*Hemophilia World*, as we now know it, began in 1994 and has steadily evolved since then to deliver the latest news and updates from our global community. For the WFH, this move to an online format helps improve how we communicate globally, "We want to be able to better reach out to our community and keep them informed about the latest news related to them," said WFH communications and marketing director Sarah Ford. "This online version is just a natural progression of that."

*Hemophilia World* will now be able to reach people 24 hours a day, around the world. The latest news, stories from our community, and newest developments from the scientific world will be accessible to more people than ever.

The online version will still contain all the stories from the print version but it will now allow the reader to get a more in-depth understanding of what the WFH is doing to further its mission; stories from WFH national member organizations (NMOs), the latest emerging breakthroughs in medical science and research, and the latest information of WFH programs and fundraising activities. Make sure to visit the new online platform at [www.hemophiliaworld.org](http://www.hemophiliaworld.org) and see what it has to offer.

## World Hemophilia Day 2015 focuses on Building a Family of Support

Every year on April 17, World Hemophilia Day is observed around the world to increase awareness of hemophilia and other inherited bleeding disorders. This is a critical global effort with an ultimate goal of promoting better diagnosis and access to care for the millions who have a rare bleeding disorder yet remain without treatment.

/Vanessa Herrick, WFH COMMUNICATIONS MANAGER

World Hemophilia Day in 2015 is focused on the global theme of Building a Family of Support; embracing and promoting the idea that family, in all forms, is critical to the care and support of those with a bleeding disorder.

The family is easily extended beyond those who are related by blood. Family can include all of those who participate in the care and support of a patient; caregivers, both institutional and individual, the global community of those with a bleeding disorder, and each individual who steps up to provide support and encouragement when needed. Most importantly, families all share the ability to encourage and advocate for people with bleeding disorders.

This support is an invaluable resource of strength and stability for the community but these connections are also critical in raising awareness and encouraging proper access to care.

World Hemophilia Day provides an opportunity to talk to extended family, friends, colleagues, and caregivers to raise awareness and increase support. It also provides an opportunity to approach new communities with information about what it is to live with a bleeding disorder. It will be easier than ever to connect with the global bleeding disorder family on the World Federation of Hemophilia (WFH) social media network this year and encourage your online community to join the global community.

World Hemophilia Day is also a good time to work with your children and relatives, to put together a family tree. It is a fun and educational process that encourages a

discussion about the genetics of hemophilia and other bleeding disorders. The WFH has posted a guide on how to make a family tree at [www.wfh.org/whd](http://www.wfh.org/whd).

On this World Hemophilia Day, "We are all working together to raise awareness and improve care," said Alain Weill, WFH president. "Join the WFH family and be a part of the worldwide effort to improve the diagnosis and standards of treatment for those who need it."

The World Federation of Hemophilia will be hosting online activities for our community on our website and social media accounts, to follow Like us at [www.facebook.com/wfhemophilia](http://www.facebook.com/wfhemophilia).





# A decade of change in Africa

Since the early 2000s, the World Federation of Hemophilia (WFH) has made a concerted effort to reach out to sub-Saharan Africa and in a few short years has helped make a dramatic impact on the lives of people with bleeding disorders.

/ Assad Haffar, WFH DEPUTY PROGRAMS DIRECTOR  
AND REGIONAL PROGRAMS MANAGER

Before the turn of the new millennium, there were only five WFH national member organizations (NMOs) from sub-Saharan Africa (Nigeria, Kenya, South Africa, Zimbabwe, and Senegal). Subsequently, the WFH has made a concentrated effort to improve care for people with bleeding disorders and have brought in 13 new NMOs from the region, thereby enabling the WFH to further support to those in need.

## BARREN BUT FERTILE SOIL

The situation in sub-Saharan Africa posed a particular challenge. Prior to the WFH's involvement on the continent, hemophilia was a rarely heard of or spoken about condition, let alone treated. Children often die young without any real explanations. Without the proper tools or knowledge, medical professionals in these countries simply did not know what the patients were dying from. While many other countries around the world needed to improve diagnosis and care, the situation for people with bleeding disorders in sub-Saharan Africa was dire.

"I have seen only four cases of hemophilia in the last 20 years. Diagnosis was mainly clinical and they were treated by fresh whole blood and fresh frozen plasma. The equipment to prepare cryoprecipitate is not working," said a Tanzanian hematologist.

In order to properly contribute to improving this situation, it was necessary to build a

network of care from the ground up. Those working in the region knew that despite this lack of infrastructure, there was a great need from the bleeding disorders community.

## BUILDING UP THE SUPPORT

The key to building a community of support in these countries begins with identifying key individuals that wants to affect proper change for bleeding disorders community. In many cases of the sub-Saharan African countries, it is actually just one individual that is the point of contact between the WFH and the patient. With a concerted effort from this one person, along with assistance from the WFH, the right people can come together to form a organization. From there, they can begin to educate and train the right people on the ground so that help gets where it is needed most. This is the optimal model to begin to establish care in a country. However, in several countries that have since become WFH NMOs, a primary contact could not be identified and someone had to be brought in to establish a foundation from which to build upon.

In order to facilitate the process of establishing care on the ground and maximize results, the WFH split sub-Saharan Africa into three sectors, West Africa, East Africa, and South Africa, with the cities of Dakar, Nairobi, and Johannesburg serving as the centres of each sectors respectively. Within each of these sectors a centre, usually in an established city, was selected to train and give resources to organizations that were just starting out.

## TOWARD COMPREHENSIVE CARE

To make a difference in the lives of people, the first thing that must be done is to begin identifying the patients. Although this may sound like a simple task, diagnosing patients starts with organizing a group of lab technicians who know how to properly test for bleeding disorders and then identify people that may have hemophilia. Not having proper functioning laboratories to do the coagulation tests means that blood had to get sent to Europe or to South Africa to be analyzed, which was costly and time consuming.

The next phase is to build a comprehensive care team consisting of a hematologist, nurse, physiotherapist, social worker, orthopedist, dentist, and a lab technician within each country that can monitor and care for the new patients. In 2008, the WFH began by holding a series of training workshops with members of the comprehensive care team through each of the sub-Saharan sectors. This approach assisted in providing the knowledge professionals required and in turn could put into practice.

## A MODEL FOR CHANGE

Development work within Cameroon illustrates how the efforts of a small group can improve the lives of people in need. Prior to joining the WFH in 2008, Cameroon had a basic level for care of people with bleeding disorders.

*continues on page 12*



# Remembering a lost generation

Over thirty years ago, the global bleeding disorders community faced a tragedy that made an indelible impact on the lives of many who were left with lasting health consequences.

/ Sarah Ford, WFH COMMUNICATIONS AND MARKETING DIRECTOR

In the early 1980s, the human immunodeficiency virus (HIV) entered into the blood supply and in turn, many people with hemophilia developed acquired immune deficiency syndrome (AIDs), along with hepatitis C.

Prior to this, there was a sense of optimism emerging regarding hemophilia care. The hopefulness over the availability of new and effective treatments ended as more and more countries experienced HIV contamination within their blood donation supplies. Globally, tens of thousands of people with hemophilia contracted HIV, along with hepatitis, from their treatment products. Since this crisis occurred, great effort has gone into monitoring the blood donation supply to ensure that treatment products are safe and secure through donor screening, testing of plasma pools and the implementation of manufacturing steps that eliminate or inactivate viruses.

Advocacy continues globally to obtain justice for those that were lost and for those that continue to face multiple hardships due to this tragedy. Around the world, many countries have held inquiries, ceremonies, passed legislation, and set up organizations to protect future generations.

As recently as this past November, the French Hemophilia Society (AFH) held a commemoration ceremony marking 30 years since the blood contamination crisis hit France. Together with the French Ministre de la Santé, Marisol Touraine, the AFH reaffirmed its determination not to let this occur again.

The global bleeding disorders community will continue to ensure that this is a chapter from our history will not be forgotten. WFH founder Frank Schnabel was one of the victims of this crisis. His reaffirmation when reflecting on what had happened continues to be inspirational: "We are going to emerge victorious."



## THE 9TH WFH GLOBAL FORUM on Research and Treatment Products for Bleeding Disorders

Montreal, Canada  
October 22–23, 2015

For more information go to  
[www.wfh.org/en/globalforum](http://www.wfh.org/en/globalforum)



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Advocacy in Action Prague workshop group

# Albanian Advocacy: Improving care one step at a time

Advocating for change is not an easy process. All World Federation of Hemophilia (WFH) national member organizations (NMOs) face a series of roadblocks when it comes to improving the care for people with bleeding disorders. However, with the right tools and a lot of persistence, changes can be made.

/Marianna Balahknina, WFH PROGRAM COORDINATOR

The Albanian Hemophilia Association (AHA) has faced many challenges in its campaign to improve care in the country but also many victories. To show how far they have come and to help develop their advocacy knowledge, the AHA recently participated in the Advocacy in Action workshop in Prague in December 2014.

The shifting political and economic climate in Albania's recent history meant that getting the government to listen about the needs of a small community of people was never simple or next to impossible. However, with the stabilizing of the political and economic situation in the country, and with its recent application to join the European Union, advocacy efforts are now reaching decision makers so that progress for the hemophilia community has come a long way.

The AHA has set itself some ambitious goals. Their aim has been to increase the clotting factor concentrates (CFCs) usage by requesting the government to increase its purchasing budgets, establish a hemophilia treatment centre, develop a sustainable medical infrastructure to achieve a basic level of care throughout the country, and create a unified national patient registry.

To achieve these goals the AHA started by sitting down with the current Minister of Health, the Health Insurance Institute, and the Ministry of Education to inform them of

the current situation people with hemophilia face and where the governments can step in to help. This was the biggest step for the AHA as the constant changing political climate of the country has meant that the department ministers change on a frequent basis.

"Compared to years ago, the situation has changed a lot, first of all we have a higher public awareness, better and more medical staff, less treatment with fresh frozen plasma and cryoprecipitate and much more treatment with factor," said Megi Neziri, active member of AHA.

The AHA also faced a situation where it needed to provide people with the correct information and skill set to be able to help in the right areas. This meant setting up training

workshops and information sessions for the doctors, nurses, lab technicians, and therapists that are involved in administering care to people with bleeding disorders. Despite this lack of knowledge in the health care community, the AHA has managed to bring in enough skilled professionals and has managed to open up a hemophilia treatment centre in the capital city Tirana.

The success that the AHA has shown in recent years is testament to what a group of dedicated individuals can achieve, with the right advocacy training, to move towards the end goal of improving access to proper treatment and care.

*The Advocacy in Action Program is supported by exclusive funding from Baxter.*





Jan Willem André de la Porte speaking at the WFH 2014 World Congress



NHF CEO Val Bias donating to Campaign at Congress

# Close the Gap Campaign a resounding success

The Close the Gap Campaign of the World Federation of Hemophilia (WFH) officially came to a close on December 31, 2014. This was the WFH's first multi-year campaign, raising US\$5.4 million to help improve care for those who need it most. The corporate portion of the campaign raised nearly US\$3.5 million from existing and new WFH corporate partners. The community portion of the campaign raised an outstanding US\$1.9 million.

/ Antonietta Colavita, WFH ANNUAL GIVING MANAGER

The global bleeding disorders community demonstrated an unprecedented level of support during the three-year fundraising drive with a total of 991 donors, from 96 different countries, that made contributions ranging from \$1 to \$150,000 in a meaningful show of solidarity within the community. To honour this solidarity, WFH patron Jan Willem André de la Porte pledged to donate an additional \$2 for every \$1 donated to the campaign. Be sure to read the enclosed campaign bulletin for a full list of campaign supporters and key volunteers.

The Campaign was inaugurated at the WFH 2012 World Congress in Paris to honor the WFH's 50th anniversary. This special anniversary was marked by a renewed commitment from the WFH to expand the care and services available to people with a bleeding disorder in some of the world's poorest regions. Often these regions lack the medical and economic resources to fully benefit from established WFH programs. The Close the Gap Campaign was dedicated to raising funds to finance this new phase of programs. Special support from a number of WFH national member organizations (NMOs) during the Campaign enabled the WFH to introduce the Cornerstone Initiative. This project was developed to support those

countries that may not have been able to adequately participate in WFH country programs and potentially the Global Alliance for Progress (GAP) Program.

This remarkable support for the Cornerstone Initiative was provided by the twelve countries that had representatives at the very first meeting of the WFH, just over fifty years ago. The NMOs from Argentina, Australia, Belgium, Canada, Denmark, France, Germany, Japan, the Netherlands, Sweden, the United Kingdom, and the U.S.A. are considered to be the "founding members" of the WFH and showed tremendous leadership in giving during the campaign. The National Hemophilia Foundation (NHF) of the U.S.A. also took the additional initiative to match all contributions made by their chapters throughout the campaign. This resulted in nearly US\$120,000 of contributions made directly from NHF chapters, in addition to NHF's contribution.

However, NMO generosity went far beyond the founding members' contributions, a total of 31 NMOs made a contribution to the Close the Gap Campaign, up from seven NMOs during 2009-2011. Martha Monteros, Secretary of the *Federación de Hemofilia de la República Mexicana* and Close the Gap Campaign regional co-chair said, "Whether attending the WFH Global NMO Training,

downloading the new treatment guidelines, watching a conference webcast or participating in a Twinning or GAP Programs, we have all benefited from WFH programs. This is our chance to ensure these resources will continue to be available - to the *Federación de Hemofilia de la República Mexicana* and to anyone else around the globe who needs them. Too many patients still lack access to the most basic treatment and diagnosis."

The Campaign regional co-chairs were a vital part of the campaign's success, inspiring donors and leading grassroots initiatives in their respective regions, from bicycle races to "pass the hat" initiatives. Well-respected in their regions for having already made remarkable contributions to improving care for people with bleeding disorders, each of the regional co-chairs was instrumental in maintaining the global perspective of the Close the Gap Campaign. Please see the Campaign bulletin for a full list of its volunteers.

The Close the Gap Campaign has helped enable the WFH to continue its work and reach more patients than ever before. It's not too late to be part of this legacy. You can contribute to help the WFH meet its ongoing needs and help those around the work gain access to the care they need and deserve. Because you care, we can accomplish so much together. Thank you for all your support.



# One Ugandan boy

Growing up in a rural area of Uganda, Segawa Wasswa knew very early on that he was different from the other kids in his community. Wasswa was born with severe hemophilia A but he never knew it. In fact, his brother and two uncles had all died from complications related to hemophilia but none of them had even heard of the condition let alone received a diagnosis.

/ Maria Carolina Arango, WFH HUMANITARIAN AID OFFICER

The situation in Uganda for people with a bleeding disorder has always been challenging, but in 2012, the Ugandan Haemophilia Foundation (UHF) joined the World Federation of Hemophilia (WFH), taking an important step to improving care in the country.

Since then, the UHF has taken steps to improve care within the country by attending several workshops, as well as a productive Twinning partnership with the Great Ormond Street Hospital from London, United Kingdom. The UHF has worked very hard towards improving diagnosis and care for people with bleeding disorders but there are still many challenges ahead for this African nation.

These challenges are perfectly illustrated by the case of young Segawa. It was not until the age of 12, after suffering a painful knee bleed in 2013 that he was brought to a rehabilitation care unit for children with bone disabilities.

Doctors at this centre misdiagnosed him with a bone disease and proceeded to perform orthopedic surgery on his knee without any clotting factor concentrates. Luckily he survived the surgery but his condition only worsened.

Fearing the worst, the rehabilitation centre transferred Segawa to Mulago Hospital in Kampala, where he was tested and finally received the proper diagnosis of severe hemophilia A.

Segawa's condition was reported to the UHF, who was able, through the WFH Humanitarian Program, to provide him with the factor concentrates he needed to survive. The only problem that remains for him and his family is getting enough money to pay to travel from his rural village to the city where the treatment is available.

The situation on the ground for people with a bleeding disorder in Uganda is less than perfect, but every day that passes it improves. With assistance from the WFH, the UHF is



Segawa Wasswa

providing proper training to people in the field and the WFH Humanitarian Aid Program ensures that in cases like Wasswa, that there is treatment available where there was none before.

The WFH Humanitarian Aid Program channels donations of life-saving treatment products to people with bleeding disorders who need them all around the world. This program is one of the many ways in which the organization supports its vision of Treatment for All.

## Calendar of events

### World Hemophilia Day 2015

April 17, 2015  
World Federation of Hemophilia  
Tel.: + 1-514-875-7944  
Email: [vherrick@wfh.org](mailto:vherrick@wfh.org)  
[www.wfh.org/whd](http://www.wfh.org/whd)

### 14th WFH Musculoskeletal Congress

May 7 – 10, 2015  
Belfast, Northern Ireland  
World Federation of Hemophilia  
[www.wfh.org/en/msk](http://www.wfh.org/en/msk)

### World Blood Donor Day

June 14, 2015  
World Health Organization  
[www.who.int/worldblooddonorday](http://www.who.int/worldblooddonorday)

### 2015 PPTA Plasma Protein Forum

June 16-17  
The Plasma Protein Therapeutics Association  
Washington, D.C., U.S.A.  
[www.pptaglobal.org/meetings-events/plasma-protein-forum/overview](http://www.pptaglobal.org/meetings-events/plasma-protein-forum/overview)

### ISTH World Congress

June 20-25, 2015  
Toronto, Canada  
International Society on Thrombosis and Haemostasis  
[www.isth.org](http://www.isth.org)

### NHF's 67th Annual Meeting

August 13-15, 2015  
Dallas, Texas, U.S.A.  
National Hemophilia Foundation  
[www.hemophilia.org/Events-Meetings/Annual-Meeting](http://www.hemophilia.org/Events-Meetings/Annual-Meeting)

### The 9th WFH Global Forum On Research and Treatment Products for Bleeding Disorders

October 22-23, 2015  
Montreal, Canada  
World Federation of Hemophilia  
[www.wfh.org/en/globalforum](http://www.wfh.org/en/globalforum)

### EHC Annual Conference 2015

October 2-3, 2015  
Belgrade, Serbia  
European Haemophilia Consortium  
[www.ehc.eu/events/ehc-annual-conference-2015/](http://www.ehc.eu/events/ehc-annual-conference-2015/)

# WFH Twins Khartoum and Basingstoke: Partnering for a better future

In order to understand the World Federation of Hemophilia (WFH) Hemophilia Centre Twinning partnership between Khartoum (Sudan) and Basingstoke (United Kingdom), as part of the WFH Twinning Program, it is important to reflect on and consider the context.

/Christine Herr, WFH INTERIM PROGRAM COORDINATOR, TWINNING AND GLOBAL NMO TRAINING

Sudan has a long history of conflict and turmoil: from gaining independence, to the first civil war from 1955–1976, the second civil war from 1983–2005, and the crisis in the Western region of Darfur. It is estimated that 1.5 million people died during the decades of fighting in both civil wars.

When the Twinning partnership between Khartoum and Basingstoke began in 2011, the nation was divided in two, with South Sudan becoming an independent country. War and conflict has left repercussions across every sector of the country. Political tensions, sanctions, and economic recessions have also left its lasting marks on the country. Any health system does not effectively work in isolation from politics and economics.

It is within this setting that this Twinning partnership began. The original goals were to improve the standard of hemophilia care in Sudan, spread the services to areas outside of Khartoum, and increase the number of identified patients. After four years of working in collaboration, Khartoum and Basingstoke have made great strides. Effective lobbying and advocacy was an immediate priority. Working with the Sudanese Ministry of Health and the

Ministry of Social Welfare has allowed the partners to promote the needs of patients with bleeding disorders, in addition to advocating for an increased budget for hemophilia care and improved access to treatment. Capacity building has been another area of focus with the completion of physiotherapy workshops and improved laboratory diagnosis.

There are elements of the Twinning partnership that have left a lasting impression on both treatment centres. The Twins have recognized each other's strengths and how this can provide learning lessons, from collaboration with patient organizations, to the effective use of resources and innovative ways of operating on patients with complex pseudo-tumors.

According to the WFH 2013 Global Survey, there were 780 identified people with hemophilia in Sudan, 199 with von Willebrand disease, and 216 with other bleeding disorders. With a population of nearly 38 million, the work continues in Sudan to identify more people with bleeding disorders. Equipped with skills and knowledge from the WFH Twinning Program, the Khartoum Teaching Hospital is dedicated to improving care within their community.

*The Twinning Program is supported by exclusive funding from Pfizer.*

## Change in Africa *continued from page 7*



“Thank you and WFH for the support with factor VIII. It is like a dream come true because for so many years we struggled. Now we can even administer factor VIII prophylactically for patients who have the moderate and severe hemophilia or are developing complications,”

said Dr. Pauline Musukwa Sambo  
Consultant Paediatrician Hemato-  
oncology unit Department of  
Paediatrics University Teaching  
Hospital Lusaka, Zambia.

Medical professionals had very limited knowledge of hemophilia or how to treat it. The country, in terms of care for people with a bleeding disorder, was starting from the bottom.

The first step was identifying a proper laboratory technician and a hematologist. With Cameroon, there was a good pool of eager people to work with and from there it was a matter of getting those people the right training. Within a few short years, Cameroon went from very basic care and diagnosis to being to identify patients and take proper care of them.

### ROOM TO GROW

Sub-Saharan African countries have had to face many hurdles when developing hemophilia care within their own communities but by providing support and expertise to these countries, they are put on a path to build upon their own successes and improve access to care and treatment.

## The Foundation of Hemophilia Argentina celebrates 70 years

On October 26, 2014, the Foundation of Hemophilia in Argentina (FHA) celebrated 70 years of continued work improving the lives of people with a bleeding disorder.

The World Federation of Hemophilia (WFH) president Alain Weill and vice-president medical Marijke van den Berg attended a series of meetings and events and helped celebrate the milestone anniversary in Buenos Aires, in November 2014.

Weill thanked the Foundation for the invitation and congratulated them for all the hard work and commitment to the hemophilia and other bleeding disorder's community not only in Argentina but around the world.



# WFH expands website to include Russian and Arabic



/ Jay Poulton, WFH EDITOR/ EDITORIAL SERVICES COORDINATOR

The World Federation of Hemophilia (WFH) website is now available in Simplified Chinese, Russian, and Arabic, reaching out to new linguistic communities, beyond the existing English, French, and Spanish. This expansion of its web presence is part of the WFH strategic goal of using new technologies to reach out its community.

These localized websites are not intended to replicate the full English, French, and Spanish website, but to provide all essential information in Simplified Chinese, Arabic, and Russian, to people accessing the WFH website who might not have been able to access it otherwise. With more than an estimated 11,200 people with a bleeding disorder in China, 14,000 in Russian-speaking countries, and 17,000 in Arabic-speaking countries, these

localized websites are essential in helping to ensure that current information gets out to as many people as possible.

“When it comes to someone’s health and well-being it is essential that they have the proper information. Often things can get lost in translation, so that is why, as an organization, we are expanding our web resources reach these regions,” said Elizabeth Myles, Interim CEO and COO.

To access the localized sites please visit [www.wfh.org](http://www.wfh.org) for more information.

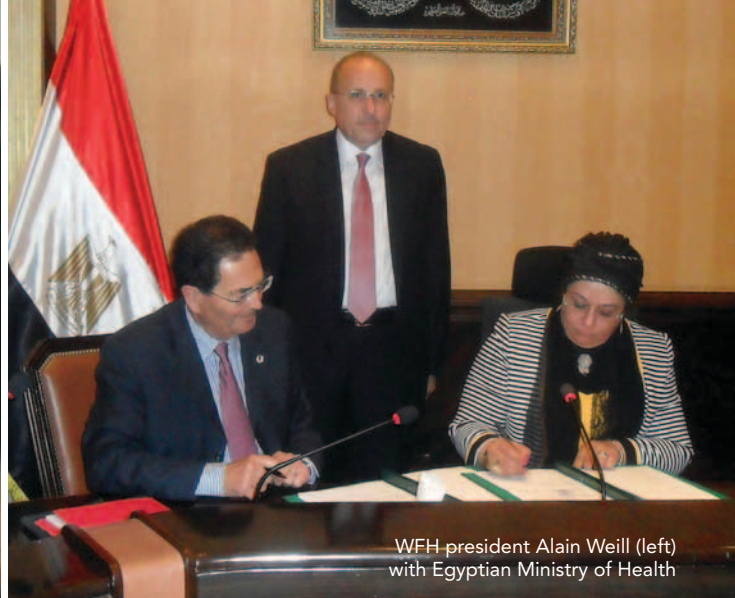


## THE 14TH INTERNATIONAL MUSCULOSKELETAL CONGRESS

Belfast, Northern Ireland  
May 7-10, 2015



VP medical Marijke van den Berg with patient during a 2014 visit to Egypt.



WFH president Alain Weill (left) with Egyptian Ministry of Health

# Egypt enters second decade of GAP Program

In October 2014, the Egyptian Ministry of Health signed the World Federation of Hemophilia (WFH) Global Alliance for Progress (GAP) Program Second Decade agreement. This significant event occurred during a visit to Egypt by Alain Weill, WFH president, and Marijke van den Berg, WFH vice-president medical.

/Assad Haffar, WFH DEPUTY PROGRAMS DIRECTOR AND REGIONAL PROGRAM MANAGER

This GAP agreement is a continuation of the work started in 2003, when Egypt joined the GAP First Decade Program and became the first GAP country in the Middle East region. During this period, the bleeding disorders community in Egypt witnessed many positive steps towards improvement in hemophilia care in the country.

Among the most important events were the establishment of a national hemophilia committee that had representation from different health sectors and the establishment of a full coagulation lab at the Ministry of Health Central Health Laboratory. In addition, they managed the organization of eight multidisciplinary symposiums on hemophilia and other bleeding disorders in Cairo and other major cities; the organization of more than eight multidisciplinary training workshops for nurses, physiotherapists, laboratory technicians, and social workers; training of ten Egyptian health professionals through WFH International Hemophilia Training Centre (IHTC) Program; and the donation of two million units of factor VIII and factor IX which helped in treating patients and perform many surgical interventions.

At the end of the first decade of the GAP Program, Egypt served as a model within the Middle East region, helping guide other GAP

projects in other countries, such as Jordan, Lebanon, Tunisia, and Syria.

However, even with all these positive achievements, Egyptian health professionals felt that there is still room for further improvement for bleeding disorders care in Egypt, particularly in Hemophilia Treatment Centres (HTCs) outside of Cairo.

## MOVING INTO PHASE TWO

“Since the establishment of the Global Alliance for Progress project in Egypt, we have seen a remarkable improvement in care where hereditary bleeding disorders have been included in the national health care system. Yet the second decade of the GAP Program will focus on harmonization of comprehensive care across the country which will reflect on improved diagnosis, registry, and management. The Egyptian GAP project will help ensure access to care reaches all areas of the country,” said Magdy El-Ekiaby, MD, head of the Egyptian Hemophilia Society and WFH board member.

The current GAP project is going to bring many parties together in order to consolidate previous achievements and maximize these results. Work will begin between the Egyptian Hemophilia Society, Ministry of Health, General Commission of Health Insurance, National Blood Transfusion Services, and

health professionals across different sectors to address the most pressing needs.

For the bleeding disorders community in Egypt, the hope is that there will be a harmonization of hemophilia management in all HTCs across the country; provision of training on laboratory diagnosis in HTCs outside Cairo, and the establishment of a National External Quality Assessment Scheme (NEQAS) in the country.

In addition, there will be training provided for healthcare professionals in treatment centres outside of Cairo on the implementation of the WFH Treatment Guidelines. Issues will be addressed related to women with bleeding disorders and support VWD outreach, with a focus on corrective surgeries. The WFH Humanitarian Aid Program will also contribute to supporting treatment and care in Egypt by the donation of two million IUs of clotting factor concentrates annually over the period of the GAP project.

These new achievements will lead the way to improving bleeding disorders care in Egypt that would be positively reflected on the life of all people with bleeding disorders in Egypt.

*The WFH is grateful for the support of the GAP Program from Baxter, Bayer, Biogen Idec Hemophilia, Biotest, CSL Behring, Grifols, Kedrion, Novo Nordisk, Pfizer, and SOBI.*



# Thank You

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

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## Website

Prophylaxis web section: Biogen Idec Hemophilia  
Rare Bleeding Disorder web section: Novo Nordisk  
von Willebrand Disease web section: Octapharma

## Website localization projects:

Arabic: Pfizer  
Russian: SOBI  
Simplified Chinese: Bayer

## Other sponsored programs

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WFH African Initiative: Pfizer  
Workshop on Hemophilia Care: Kedrion, Susan Skinner Memorial Fund: Hemophilia Alliance Foundation  
von Willebrand Disease Workshop: LFB

## World Hemophilia Day

Baxter, Bayer, Biogen Idec Hemophilia, CSL Behring, Novo Nordisk, Precision BioLogic, SOBI

## ORGANIZATIONS

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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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