WFH 2016 WORLD CONGRESS
Orlando, USA · July 24-28

THE CONGRESS DAILY

THE OFFICIAL NEWSPAPER OF THE WFH 2016 WORLD CONGRESS

WEDNESDAY, JULY 27, 2016 • ORLANDO • WWW.WFH.ORG

WFH VICE PRESIDENT MEDICAL: THE FUTURE OF HEMOPHILIA TREATMENT IS VERY PROMISING

Hemophilia treatment has entered an exciting era, with new products making diagnosis and treatment available for a far larger population than ever before, said WHF Vice-President Medical Marijke van den Berg during her Tuesday morning VP Medical Plenary address.

The session covered a variety of studies in people with severe hemophilia A that demonstrate how early prophylaxis can prevent bleeding and is key for joint outcome. This replaces episodic therapy, which has been a frequently used hemophilia treatment regimen since the 1970s.

Early prophylaxis can prevent bleeding and is key for joint outcome.

"Modern hemophilia treatment has completely changed the phenotype—but not in countries where early treatment is not available," she said.

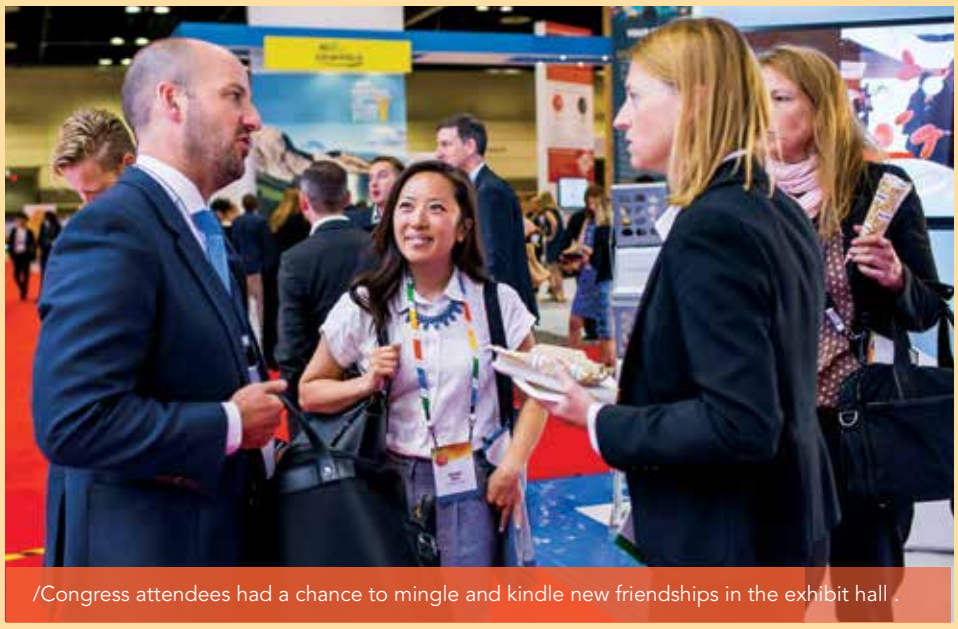
van den Berg cited a very large US study of patients with severe hemophilia A, divided into four birth-date cohorts. Even in the age group born in the 1980s, disability was too high, she said, with more than five joint bleeds over six months, despite very high clotting factor consumption.

The large, international Musculoskeletal Function in Hemophilia (MUSFIH) study of children with severe hemophilia A showed that even very high factor dosing resulted in substantial bleeding, van den Berg said. The study also showed that the number of bleeds—but not the dose of episodic treatment—is responsible for joint outcome. This is a key understanding because joint function deteriorates after age 12.

She cited a small, randomized study showing that low-dose prophylaxis, rather than episodic treatment, reduces bleeding by 80 percent. Research also shows that early diagnosis is crucial. "Remember, more than 50 percent of those with severe hemophilia A have a negative family history," van den Berg said.

Continued on page 8.

/WFH Vice-President Medical Marijke van den Berg discusses new breakthroughs in hemophilia treatment



/Congress attendees had a chance to mingle and kindle new friendships in the exhibit hall .

OPTIONS IN DELIVERY OF GENE THERAPY EXPLORED

Scientists involved in cell-based therapy for bleeding disorders provided updates on this exciting discipline of gene therapy Tuesday morning. David Lillicrap, Canada, the chair of the session, "Gene Therapy", said the momentum of clinical gene therapy is remarkable.

Christopher B. Doering, Atlanta, Georgia, USA, took the audience through the process of using stem cells in gene therapy of hemophilia A. Stem cells were first applied to T cells in the 1990s, but safety concerns led the research back into academic laboratories, said Doering.

"Stem cells are rare populations of unspecialized cells that are self-renewing and can become other cells," began Doering. "Donor cells from a non-affected individual (from the blood) are transplanted into the patient. In order to apply to hemophilia, this may require gene transfer."

In order to implant stem cells, some of the patient's cells must be negated to make room for the new cells. Doering said that it is possible to use the patient's own cells harvested peripherally or from bone marrow.

Continued on page 7.

IN THIS ISSUE

TODAY

16:30 – 18:00

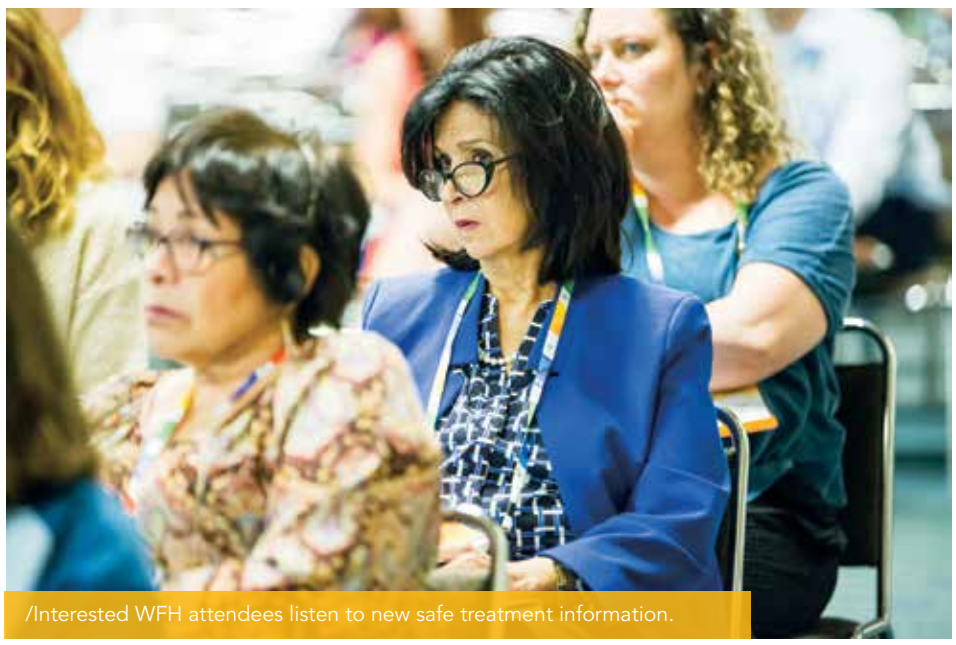
Von Willebrand Disease (Hall A4)

Inhibitors –
What have we Learned (Room 206)

Remember to stop by the WFH Resource Center.

Remember to stop by the WFH Resource Center.

/(l to r) Mark Skinner chaired the panel session, which included presentations from David Page and Brian O'Mahony.



/Interested WFH attendees listen to new safe treatment information.

WFH LAUNCHES SEVERAL **NEW SAFE TREATMENT** PRODUCT INITIATIVES

The HIV/AIDS epidemic in the 1980s was a shock that made citizens and governments throughout the world scrutinize blood-supply safety. Since then, the WFH has been a leader in blood-safety and treatment-access initiatives.

During the Tuesday afternoon session "Leading Global Surveillance and Collaboration: The WFH's Role in Ensuring Safety and Supply of Treatment Products," presenters discussed new WFH programs, including a comparative database of factor products, a collaborative genotyping project using 5,000 blood samples from people with hemophilia A and B and a Worldwide Bleeding Disorders Registry.

David Page, the first chairman of the WFH's Blood Safety Committee, detailed the WFH's stance on blood safety, including factor manufacturing.

"Today, many governments and blood establishments are doing their best to be more discriminatory" after the HIV/AIDS and hepatitis C crises, Page said. This includes instituting donor referrals for high-risk populations, such as men who have sex with men (MSM). WFH recognizes that MSM donor-referral policies are discriminatory, but leaves specific policies to individual countries, he said.

In terms of blood products, Page said the WFH lists safety steps for manufacturing factor concentrates: Use only healthy donors, screen each donation with antibody and nucleic acid tests for multiple viruses, institute a plasma hold to allow time for a concern to be reported, and institute purification steps and viral reduction/removal steps.

Based on new research over the last 15 years, the Blood Safety Committee has had a clear shift in focus from pathogen effects to immunogenicity, Page said.

Brian O'Mahony, current chair of the Blood Safety Committee, discussed the committee's involvement in worldwide regulations. The WFH's main goal is to ensure adequate supply and access to treatment.

O'Mahony said key initiatives the WFH is involved in include:

- The World Health Organization's (WHO) Essential Medicines List. More than 130 countries use this list to develop their essential medicines. The WFH has helped ensure that FVIII, FIX and desmopressin are on the list.
- The European Medicines Agency's (EMA) Committee for Orphan Medicinal Products. More than 1,200 therapies have orphan status in Europe. The WFH has successfully fought against orphan drug designation for factor concentrates, arguing that it could hinder the development, licensing and marketing of similar products for hemophilia.

Continued on page 4.

VWD: ONE SIZE DOES NOT FIT **ALL**

The most common type of bleeding disorder in the world is von Willebrand disease (VWD). There are three main types of VWD. Within each type, the disorder can be mild, moderate, or severe. Bleeding symptoms can be quite variable within each type depending in part on the von Willebrand factor activity. It is important to know which type of VWD a person has, because treatment is different for each type.

This important topic will be addressed on Wednesday during the "VWD" medical session in Hall A4. Chair and speaker Andra James, Duke University Medical Center, will address women's issues associated with VWD. Diagnosis will be addressed by Jeroen Eikenboom, Leiden University Medical Center, Netherlands. Augusto Federici, University of Milan, Italy, will speak about acquired VW.

16:30–18:00, Hall A4

A **NIGHT** TO REMEMBER, IN FULL **COLOUR**

Yesterday's host country networking reception truly was a night to remember. The event was held at Epcot's® World ShowPlace Pavilion, which is part of the Walt Disney World Resort. The setting was beautiful, with colourful glow tables and projectors showing images of the illuminated buildings from World Hemophilia Day.

The evening began with delicious food, delightful wine—and a totally unexpected visit by Mickey, Minnie, Donald, Pluto and Goofy. Guests were able to mingle with the Disney stars and even get their picture taken by professional photographers. Colleagues and friends—both old and new—had the chance to socialize, share their WFH Congress experiences and enjoy life music from GenNext, a six-piece live band.

IN THE **WORLD** OF BLEEDING DISORDERS, **CHOICE IS NEVER SIMPLE**

Debate over products—plasma derived (PD) versus recombinant—has been raging for a long time. A panel assembled on Tuesday afternoon to discuss this issue for the session, "Making a Choice: How to Determine the Optimal Choice to Treat PUPs."

Cedric Hermans, Belgium, began by providing an overview. "Globally, severe hemophilia A patients only develop antibodies if exposed to FVIII. In less developed countries, patients are exposed later and less frequently and there are less options available as well as less research."

The evolution of FVIII/FIX concentrates has mainly been driven by the need to improve infectious safety. Improvements have been successful and there have been no infection transmissions since 1985. But he cautioned that safety should remain a concern.

> Risk factors for inhibitors are driven by genetics and type of treatment. Family history is critical since the type of mutation is clearly important.

"Improvement in immunological safety has been much less successful."

Risk factors for inhibitors are driven by genetics and type of treatment. "Family history is critical since the type of mutation is clearly important," said Hermans. "The intensity of treatment and type of concentrate also play a role."

Strategies to reduce inhibitor development include starting prophylaxis at a young age to prevent bleeds and to provide exposure to factor.

Alok Srivastava, India, spoke to the use of PD in previously untreated patients (PUPs). "No concerns have ever been expressed about the efficacy of PD product as long as you use the recommended dosage. In the last 20 to 25 years there hasn't been any transmission of documented infection. The manufacturing industry has greatly reduced the chance of transmitting any know infectious agents."

"The first priority is the adequate quantity of safe clotting factor concentrate (CFC). Once adequate quantity is achieved, consider options for type of CFC," he said. "The cost of recombinant is nearly double of PD."

Srivastava said that there is little experience in the world regarding the use of recombinant products with PUPs. "Using any safe product is better than using none."

"The rationale for developing recombinant products was that they would be safer than PD counterparts," opened Steven Pipe, USA. "Consistent manufacturing and processing liberated us from the uncertainty of securing source plasma. There is also a potentially unlimited supply."

/Julie Malan and Steven Pipe discuss treatment choices during the PUPs panel.

Pipe stated that over the last 20 years of clinical use of recombinant factor, there has been no infectious pathogen transmission and no evidence of increased rate of inhibitors in previously treated patients (PTPs). "Inhibitors in PUPs are common and are considered a natural immune response to a foreign protein," he said. "It is commonly accepted that product-related immunogenicity is best assessed by using PTPs."

He said that there are conflicting reports from pivotal studies and other forms of data

Continued on page 6.

The Congress Daily is the official newspaper of the WFH 2016 World Congress.
Editor: Michel Semienchuk
Scientific Advisor: Magdy El Ekiaby
Editorial, design and production: CustomNEWS Inc., Tim Mercer, Vicky Uhland, Deb Nerud
Photography: Benjamin Peña and Denisse Springer

INHIBITORS – WHAT HAVE WE LEARNED?

A medical session this afternoon chaired by Brigitte Keller-Stanislawski, of the Paul-Ehrlich-Institute and Mike Soucie, of the Centers for Disease Control and Prevention will ask the question, "Inhibitors, what have we learned?" Inhibitors to FVIII are today the most serious complication of treatment for hemophilia but there is contradictory information about how best to avoid inhibitors, especially in previously untreated patients (PUPs), the most vulnerable group.

Most inhibitors develop within the first 50 exposure days but questions remain about whether the choice of treatment product during that period has an effect on the risk of developing inhibitors. The recently published SIPPET paper suggests that, in PUPs, the risk of developing an inhibitor when using certain recombinant FVIII products is higher than when using plasma-derived FVIII concentrates that contain von Willebrand factor. The study was the first prospective, randomized, multicenter open-label trial in this area. It compared PUPs treated with recombinant factor VIII (rFVIII) to those treated with plasma-derived factor VIII concentrate containing von Willebrand factor (pdFVIII/VWF). The results showed a higher rate of inhibitor (neutralizing antibody) development in the rFVIII-treated subjects compared to pdFVIII/VWF-treated subjects.

However, other datasets do not support the conclusion that the class of recombinant products carries a higher risk. This session will survey these contradictory studies and continue the debate. Dr. Keller-Stanislawski will describe an individual patient meta-analysis of the France coag, UKCHDO and PedNet/ RODIN studies on recombinant products. This analysis involves a very large cohort of PUPS all followed out to 50 exposure days or more. Other speakers will discuss the clinical and laboratory aspects of the immunologic basis of inhibitor development and further data related to the roles of product class and von Willebrand factor. Come to hear the latest thinking on this controversial and important topic.

16:30-18:00, Room 206

Safe Treatment

Continued from page 2.

• Paid plasma bans by organisations like WHO and the Canadian government. WFH and seven other patient organisations formed a plasma-users commission in 2010, and in 2011, released a consensus statement that plasma products made from both non-remunerated and remunerated donations are currently essential to meet global health needs.

But despite these efforts, "The debate goes on—it's endless fun," O'Mahony said sarcastically. "The problem is that there are some people whose views haven't changed since the tragic events of the 1980s."

Mark Brooker, WFH, discussed regulatory questions members ask about products:

• Label indications for pediatric use. Most products, particularly in Europe, are licensed for adults only, Brooker said. Members have concerns that this delays getting products on the market.

• SIPPET study fallout. This study found that recombinant FVIII users were more likely than plasma users to develop an inhibitor. "This is tricky for us because we've been using those recombinant factors for years," Brooker said. The WFH is very clear that not treating is the worst choice, he added, meaning it's better to use recombinant factor than to not treat at all.

• Products not approved by the EMA and Food and Drug Administration (FDA). EMA and FDA approvals are accepted by most countries. But some countries can require testing for non-FDA and EMA products, which can be frustrating for small manufacturers.

Brooker also announced the WFH's new Online Registry of Clotting Factor Concentrates. This registry was originally created by the International Society on Thrombosis and Haemostasis, and transferred to the WFH in 2008.

The registry is now online at elearning.wfh.org. Brooker said it's a comprehensive clearinghouse for product information and also allows users to compare products. In the next few months, the WFH is planning to add regulatory status for products, with future plans to include data for products in the pipeline.

Glenn Pierce, board member of Global Blood Therapeutics, closed the session with a look at product data gaps and exciting new initiatives being created through the WFH and private-public collaborations.

"We could be doing a much better job in the global hemophilia community to provide evidence to support bleeding-disorder products," he said.

First of all, Pierce said there's an efficacy-effectiveness gap in bleeding-disorder product trials. Specifically, patient populations tend to be more homogenous than they are in real life, and the studies tend to focus more on patient outcomes than other data. Pierce said providing more data will appeal to payers, who want head-to-head comparisons of products, utilization of healthcare resources, and final versus surrogate endpoints.

> "The question is should we use the PUPs for product analysis, or to determine how to prevent inhibitor development?"
>
> —*Glenn Pierce*

For instance, he said, previously untreated patient (PUP) studies on the risk of inhibitor development may look at the same small numbers of PUPs. But only about 750 PUPs are born in the U.S. and Europe each year, so "We're dealing with a small, precious commodity," Pierce said. "The question is should we use the PUPs for product analysis, or to determine how to prevent inhibitor development?" Pharma really has no impetus to do the latter, he said.

Trough levels are another issue, Pierce said. The concept of a trough level of 1 percent stems from historical product shortages and consistently high per-unit pricing. "But inadequate trough levels are the biggest single cause of morbidity and mortality in prophylactic treatment," Pierce said. Troughs should be at 10 to 15 percent factor activity, which is the minimum required for a bleed-free existence.

The good news is that there are some products in development for low trough levels: Emicizumab, Fitusiran, AAV-FIX and AAV-FVIII. "This should be changing treatment paradigms as we go forward," Pierce said.

He also discussed a new, collaborative genotyping project to collect 5,000 blood samples from people with hemophilia A and B. The goal is to create a research repository to look for risk factors for the complications of hemophilia. So far, 2,000 samples have been sent to the U.S. National Institutes of Health for full genome sequencing.

The repository will be open to researchers within the next six months. "We are very fortunate to have this number of samples to be able to answer a number of significant research questions," Pierce said.

The WFH is also creating a Worldwide Bleeding Disorder Registry (WBDR). The pilot phase is underway with 31 hemophilia treatment centers, with completion scheduled for this December.

"High-quality observational data is being collected," Pierce said. "This facilitates research by sharing data with the scientific community to address gaps in evidence to improve diagnosis and care, support advocacy initiatives and allow the scientific community to address unresolved clinical questions."

WFH SIGNS MEMORANDUM OF UNDERSTANDING WITH THE **ASIA PACIFIC HEMOPHILIA WORKING GROUP** TO SUPPORT HEALTHCARE PROFESSIONALS IN THE REGION

The World Federation of Hemophilia will collaborate with the Asia Pacific Hemophilia Working Group (APHWG) to help improve care for people with hemophilia and other hereditary bleeding disorders in the Asia Pacific region. The APHWG consists of clinicians managing hemophilia from countries across the regions and is led by Alok Srivastava.

The pilot phase for this collaboration will begin this fall, with both parties sharing work plans for 2016 and 2017. This will ensure that development work done in the Asia Pacific region will be complementary and will support existing WFH program activities. The primary focus of the APHWG will be on education, training and research.

The WFH will continue its outreach efforts in the Asia Pacific region and believes this partnership will benefit those most in need, furthering our vision of ensuring Treatment for All.

DONORS OF CONGRESS: NAJA SKOUW-RASMUSSEN

/Naja at the WFH Resource Centre with WFH Patron Jan-Willem André de la Porte

Naja Skouw-Rasmussen—who lives with Von Willebrand disease (VWD)—is a kite-surfing yoga practitioner who works with the Council of Volunteering in Copenhagen, Denmark. When not braving the waves she helps civil society organizations develop their democratic and engagement capacity. Naja strongly supports the mandate of the WFH Humanitarian Aid Program to invest in education and other resources that will ensure patients have a bright future. She notes that, "You can give a person a fish and they will eat for one day, but if you teach them to fish they will feed themselves for a lifetime."

Thank you Naja! Please join her and our growing list of supporters by visiting us and making your mark on our World Map at the WFH Resource Centre today!

DON'T MISS THE MODERATED POSTER SESSIONS

WFH World Congress offers attendees the rare opportunity to meet leading experts and researchers face-to-face during the moderated poster sessions. Speakers will give short presentations about their poster, and they will also be by their posters during the afternoon break at 15:30 to answer any questions you may have about their research. 36 experts will be giving presentations on a wide range of topics, so the moderated eposter sessions promise to be an interesting experience to everyone who participates.

For more information on titles and presenting authors, please look for the moderated poster sessions in the daily Congress program or on the official Congress app. The poster area is sponsored by Baxalta, now a part of Shire, and is located at the far end of Exhibition Hall B1.

SPECIAL NOTE: For those who want to familiarize themselves with what will be at the poster area before going, just open the WFH Congress App and tap on "Poster Index."

/Dilli Raj Adhikari traveled from Nepal to speak about his FX deficiency during the Tuesday afternoon session "I Will Take My Bleeding Disorder Rare."

PUPs

Continued from page 3.

collection. Some suggest no difference between PD and recombinant forms. You need to look further into the research to really see what was being assessed. "Some studies are non-homogenous, the length of the study is different, some include only severe populations, gene mutation types are not aligned, what is the ethnicity and therapy regimen and are they prophylactic or on demand use."

"The SIPPET study looked at PDFVIII/VWF versus rFVIII," Pipe said. "There was a significant increase in inhibitor development in the rFVIII treated patients. But a large proportion of patients had gene mutations associated with increased inhibitor risk and half the patients were treated on demand."

He also pointed out that only 16 percent used third generation rFVIII. "The data doesn't address any of the newer recombinant products being used in the US. It is not a head to head product comparison."

Julie Malan, South Africa, spoke from a mother's point of view about the choice her family made to select a recombinant product.

With no known family history of hemophilia, her 6 day-old son received a diagnosis of severe hemophilia. "We needed to get him FVIII and only plasma derived product was available in South Africa. We weren't given any choice," Malan said.

The majority of patients are state patients in South Africa and do not have a choice. "All plasma products have an intermediate purity," she said. "Even on medical insurance, not all patients are guaranteed the option of using a recombinant product. And the one recombinant product available is double the price of plasma."

After a scare that her son was Hepitis C positive—later finding out he wasn't—she asked her hemophilia treatment center about recombinant product. "My choice was made by the fear I experienced," she said. "I still fear the unknown viruses. Changing to recombinant gave me peace of mind and since we have insurance the choice was there. It is not a choice most South Africans have."

Speakers on both sides of the issue supported their stance with data. The debate of this complex issue is not yet settled.

DID YOU KNOW THAT...

The oldest tree in Orlando is estimated to be nearly 400 years old?

RECHARGE YOUR BATTERIES AT THE INTERNET HUB

Conveniently located in the exhibit hall right next to the WFH Resource Centre and by the poster area, the WFH internet hub is the place to be if you want to take a break and get online. The area will have several stations where attendees can check their email, browse the internet and charge their mobile devices. It's also a good spot to just take a moment and sit down and relax between sessions, or between visits to exhibition booths.

HELP US SAY **FAREWELL** THIS THURSDAY EVENING

They say all good things come to an end. Alas, the 2016 WFH World Congress will be wrapping up tomorrow. Fortunately, there's no reason to hang your head low, since the WFH has planned an exciting event to cap your Congress experience. On Thursday, July 28, the Farewell Dinner will take place in the Grand Ballroom in the WFH headquarters hotel—the Rosen Centre—from 19:00 to midnight. The event will feature fine cuisine and wine, live music, dancing—and a friendly, fun atmosphere. Be sure to wear cocktail dress and your very best smile.

Gene Therapy

Continued from page 1.

Challenges to using stem cells include the optimization of transgene expression and producing biosynthesis, and also safe and effective pre-transplantation conditioning and clinical vector manufacturing, said Doering. "Stem cells can last for the life time of an individual so we have a potential cure. We need only to target a few cells as each stem cell will produce hundreds of daughter cells."

A pilot clinical trial design has been approved by the US Food and Drug Administration with a single site trial at Emory University, USA, to start.

Matthew Porteus, Stanford, California, USA, said that genome editing is a method to correct disease causing variants. "This is a precise, controlled mutagenesis of the genome. Creating a break in the DNA will cause the cells look for this and make a repair. So we can stimulate mutations at the site of the break."

The repair could change the DNA sequence to one that already exists in the genome or to something novel using synthetic biology. This second option "Creates a new therapeutic phenotype in the cell. In hemophilia it might be used to overly express a clotting factor," said Porteus.

/Christopher Doering discusses stem cell possibilities.

Using homologous recombination to change single nucleotide variants can be delivered on an adeno-associated virus (AAV) nanoparticle. "In research with sickle cell disease there is about a 20 percent success rate. We can also insert a gene cassette into a safe harbor or single location in the genome," said Porteus.

"Targeting transgene addition without knocking out the target gene or knocking it into a highly expressed gene has some exciting applications." — *Matthew Porteus, Stanford, California, USA*

"Targeting transgene addition without knocking out the target gene or knocking it into a highly expressed gene has some exciting applications," noted Porteus. "There are opportunities and challenges for in vivo gene editing for hemophilia. There would be no need to give patients conditioning agents and it is a potential method to edit cells that naturally make clotting factors." One drawback however, is a lesser ability to monitor efficacy and off-target effects, he added.

Porteus noted that an ethical concern of genome editing is equity and distribution and how to take it to the parts of the world where most people with hemophilia live.

Brigit E Riley, Sangamo BioSciences, USA, delivered new data on FVIII using AAV delivery. "Using AAV in clinical and preclinical trials for FIX has been successful, however there is a lag in the clinic for FVIII."

She said liver-directed AAV FVIII cDNA gene therapy is being explored as liver cell DNA is separate from transgene DNA. Recombinant AAV is efficient and stable long-term in tissues that do not divide such as the liver, brain and muscle.

FVIII is not an ideal gene for AAV as it is constrained by gene size and low efficiency of transcription/translation. Thus, it requires multi-factorial modifications. "With the modifications, virus yield was improved 8 to 10 fold," said Riley.

She noted that data from in vitro design show good correlation between FVIII activity and levels along a range of doses. In vivo wild type mouse data showed FVIII level 2 times normal. In vivo hemophilia A mouse model FVIII activity was 3 times normal and levels were stable over time. A reduced bleeding time was also observed. In vivo non-human primate data FVIII levels were 4 to 6 times normal levels. "Follow-up dose finding studies are aimed at determining minimal dose," said Riley.

The challenge of financial incentives is one area to still be addressed. Porteus pondered, "With no established reimbursement model for a one time gene therapy, it begs the question, 'Are stakeholders willing to take a chance on experimental curative therapies that have a different conceptual basis when the current paradigm has transformed the lives of hemophilia patients?'"

An impromptu conversation begins in the halls of the Orange County Convention Center on Tuesday.

2016 WFH AWARDS CEREMONY: HELP US CELEBRATE THOSE WHO HAVE MADE A DIFFERENCE

Every day, thousands of people around the world help make a difference in the field of bleeding disorders. Making a difference comes in many forms, from a modest gesture of kindness, to someone in need, to lifelong dedication to research. Every two years, the WFH has the immense privilege of recognizing some of the men and women who have helped—and continue to help—push the vision of "Treatment For All" forward.

Tomorrow, at 12:15, the 2016 WFH Awards Ceremony will be held in room 205 of the convention centre. The 45-minute event will recognize people who have made a significant impact in the field of bleeding disorders. Many of these people are professional researchers and physicians who have found their calling in helping people with bleeding disorders. Others are volunteers who have graciously given time to the WFH and our affiliates. Each of them is deserving of being recognized for their efforts. We hope you'll be there tomorrow and join in on the applause for these generous individuals.

WFH ANNOUNCES MULTI-YEAR HUMANITARIAN AID AGREEMENT WITH GREEN CROSS

The World Federation of Hemophilia and the Green Cross Corporation signed a three-year agreement on Monday July 25, 2016, to contribute a total of six million international units (IU) of its recombinant FVIII product to the WFH Humanitarian Aid Program.

Dr. Eun Chul Huh, Green Cross President, and Alain Weill, WFH President, signed this agreement during the WFH 2016 World Congress in Orlando, Florida. With multi-year donations—such as this commitment from Green Cross—and a steady flow of treatment products to the WFH network, it will also be possible for people with bleeding disorders in the developing world have access to treatment for emergency situations, acute bleeds, corrective surgeries, and also prophylaxis for young children.

/(l to r) Dr. Eun Chul Huh, Green Cross President, and Alain Weill, WFH President

"We are honoured by this commitment by Green Cross to the WFH Humanitarian Aid Program," said Weill. "This donation will help to improve the lack of access to treatment products in developing countries by providing consistent and predictable access to Treatment for All."

NEXT STOP: GLASGOW, SCOTLAND IN 2018

Your WFH team works hard for years to make sure your Congress is an exceptional experience. From choosing the venue to developing the program and the social calendar, we take pride in giving you something to look forward to every two years. And while we may take a small break once the Congress is done to recharge our batteries—the break will be a short one indeed because the next Congress is only two years away!

In 2018, Glasgow, Scotland will proudly welcome our next Congress. To be held from May 20 to 24, our next congress will once again feature the latest scientific breakthroughs — many of which still haven't been discovered yet. The event will also feature the rich cultural background of Glasgow, a city known for its friendly people, walkable neighbourhoods, colourful history, fine dining… and of course, world-famous whiskey. Glasgow is also very well located in regard to flights, making it easy to get to for our members.

It's not too early to pencil the 2018 WFH World Congress into your calendar—and we'll be sure to keep you posted on what you'll have to look forward to in the coming months.

/Georgina Hernandez of Mexico, the proud winner of the 2016 WFH World Congress draw, and her son Enrique Preza Hernandez—who has hemophilia—pose for the WFH Congress photographer.

VP Medical

Continued from page 1.

But when and how do you start prophylaxis? van den Berg said research suggests that the key is to start earlier than age 3 because physical examination scores increase with treatment delay. Other research shows that low-dose prophylaxis should be done a minimum of once a week.

There is a correlation between joint scores and dosage of factor replacement. "With a 1,000-1,500 dose, there's a lot to gain," van den Berg said. The good news for people in developing countries, where factor supply is limited, is that data show that lifetime prophylaxis with 1,000 IU per kilo is much more effective than episodic treatment. "You can significantly improve outcome with limited factor consumption," she said.

However, to implement low-dose prophylaxis, comprehensive care centres are crucial, van den Berg said. She recently toured two international hemophilia training centres that are excellent examples of this: the centre in Campinas, Brazil, led by Margareth Castro Ozelo, and the centre in Johannesburg, South Africa, led by Johnny Mahlangu. The Johannesburg centre serves 1,200 patients, with an impressive 35 percent on prophylaxis and home therapy.

Unfortunately, these centres are the exception. Recent data from Africa show that not even 5 percent of hemophilia patients are diagnosed. "The main reason is because limited or no treatment is available," van den Berg said.

The WFH Humanitarian Aid Program will substantially address that deficit. From 2016 to 2020, the program plans to provide a predictable supply of 500 million IUs of factor, van den Berg said. Availability of products will lead to more diagnosis, and that will lead to more training and, in some cases, corrective surgery.

The good news for people in developing countries, where factor supply is limited, is that data show that lifetime prophylaxis with 1,000 IU per kilo is much more effective than episodic treatment.

In conclusion, van den Berg said that the evidence shows that only primary prophylaxis can prevent joint disease, and episodic treatment is not an appropriate regimen for severe hemophilia A. After joint bleeds, signs of arthropathy appear even with very high-dose prophylaxis. And signs of loss of joint function are often visible at puberty due to growth spurts.