



# THE CONGRESS DAILY

THE OFFICIAL NEWSPAPER OF THE WFH 2016 WORLD CONGRESS

MONDAY, JULY 25, 2016 • ORLANDO • WWW.WFH.ORG

## OPENING CEREMONIES HIGHLIGHT THE PROGRESS OF THE BLEEDING DISORDERS COMMUNITY



/(l to r) Moderator Dennis Da Costa, NHF Chair of the Board Jorge de la Riva and WFH president Alain Weill speak at the Opening Ceremony.

On Sunday evening, hundreds of guests from around the world gathered to participate in the opening ceremonies of the 2016 WFH World Congress. The atmosphere was upbeat in the hall of the Orange County Convention Center's West Concourse as speakers applauded the efforts of everyone in the bleeding disorders community who were striving to provide "Treatment For All."

NHF Chair of the Board Jorge de la Riva, WFH president Alain Weill, WFH CEO Alain Baumann, and Val Bias, CEO of NHF, warmly welcomed guests and spoke at a panel discussion about the exciting developments being made in the world of bleeding disorders. The overall theme was clear: progress is being made, and lives are being impacted in a positive way.

Jorge de la Riva spoke about the importance of teamwork in the world of bleeding disorders, saying, "I realized long ago that whatever happens in the hemophilia world is because we took action or neglected to do so. Whatever happens will be, because, in the aggregate, together, with our great community fighting tirelessly, we made it happen." Alain Weill, added to this statement when he said, "You have to be optimistic—to be fully engaged as a member of our community—whether you are a volunteer, staff member, researcher, healthcare professional, or industry partner."

The evening also showcased the commitments the WFH and the NHF have made to

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## THE WFH CONGRESS ARRIVES IN ORLANDO

The WFH is very proud to host the WFH 2016 World Congress in Orlando, Florida. This marks the first time in over 25 years that a WFH Congress has been held in the United States. The Congress is also expected to be best attended of all time, with more than 5,000 people from over 130 countries expected to participate over the next few days.

Most activities will take place in the superb Orange County Convention Center (OCCC). The OCCC is an extremely manageable venue for the congress, boasting world-class services, convenient hotel transportation, and, above all, a compact, convenient setting that is conducive to meeting, listening, learning and sharing.

As with all WFH Congresses, top researchers, scientists, clinicians, doctors, nurses, patients, and other people from the bleeding disorders community will come together to help advance knowledge and techniques—and ultimately, improve the lives of millions of people around the world who live with a bleeding disorder.

Take a moment to look at your Final Program to see what's in store for you over the next four days. There will be something to satisfy every interest, including sessions, free papers, plenaries, crossfires and workshops presented by leading experts in the bleeding disorder community.

After a productive day at the Congress, the WFH encourages you to explore the Orlando area and see what the City Beautiful has to offer, from great food to exciting shopping to entertaining nightlife and theme parks. And, of course, don't forget to participate in one (or more) of the many social events your WFH team has organized for you and your colleagues.

The best way to keep on top of everything that will be going on this week is to read *The Congress Daily*. We'll be keeping you abreast of important events, and offering suggestions on what not to miss every day.



/The Orange County Convention Center West Building.

### IN THIS ISSUE

#### 02 Data Collection

Good data can support research and advocacy, but there are challenges as well as opportunities. See what the experts have to say.

#### 03 Genetic Counselling

There is increased recognition for the need of genetic counselling in hemophilia. Speakers give an overview of this critical part of the healthcare team.

### TODAY AT A GLANCE

#### 09:30 – 10:00

Multidisciplinary Plenary – Patient Partnerships: The Next Step in Hemophilia Care (Hall B3)

#### 10:45 – 12:15

WFH: Humanitarian Aid: Treatment for All is the Vision of All (Room 205)

#### 14:15 – 15:45

Building Capacity to Address Global Need – WFH and ISTH Fellowship Programs (Room 202)  
Evolution of Patient Centred Care (Hall B3)

#### 16:30 – 18:00

Alternative Therapies in Inhibitor Management (Approach of Hemostasis) (Hall A4)



/Kathelijn Fisher was one of the 10 speakers who discussed the importance of data collection in hemophilia research and advocacy.

## BUILDING A BETTER DATABASE CAN IMPROVE ADVOCACY, RESEARCH EFFORTS

There is no one-size-fits all approach in data collection, but there is a credo: quality data needs to be trustworthy, appropriate, understandable and powerful, said Alfonso Iorio, McMaster University, Canada, as he kicked off the Sunday morning session “Show Me the Data! Challenges and Opportunities in Data Collection.”

“The more we move toward better treatment, the more data we will need,” Iorio said. “What was enough data a few years back will not be enough a few years down the road. It’s our responsibility to set up data collection we can follow for generations.”

The session featured a series of panelists addressing topics ranging from the keys of data collection to how to build a national patient registry.

Randall Curtis, Hemophilia Council of California, United States, said when building a database for advocacy purposes, it’s important to know your target audience. Decision makers often have scarce resources, and hemophilia advocates usually ask for many resources for a small population. “So your data has to be very compelling,” he said.

Legislators have narrow attention spans, so Curtis said it’s important to show data that’s of value to them, and make it short, easy to read and understand. In some countries, reduction of pain and suffering is very compelling. In other countries, it’s compelling that a young man with hemophilia can’t start a family.

“I always try to compare my data to the normal population,” Curtis said. For instance, the benefits of better care for people with hemophilia include reduced emergency room visits and shorter hospital stays, and

“What was enough data a few years back will not be enough a few years down the road. It’s our responsibility to set up data collection we can follow for generations.”

improving underemployment rates and costs to society through things like lower graduation rates. And remember, “Cost avoidance is almost as good as cost-effectiveness,” he said.

In terms of the ethics of data collection, Margareth Ozelo, University of Campinas, Brazil, said participants should be fully informed of the way their data is going to be used and managed. She also advised that when forming a patient registry for research usage, you can reduce risk by getting patient consent and emphasizing the patient’s confidentiality, privacy and security.

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## THE WFH MEMBERSHIP SURVEY

### GET A CHANCE TO WIN AN IPAD MINI!

Giving our members the best possible experience and access to meaningful resources is a key objective here at the World Federation of Hemophilia. As a next step toward shaping the future of the WFH Membership experience we are very excited about launching our first member survey at the 2016 World Congress. We hope that everyone will take the survey so that we can better understand which benefits are important to keep or improve and identify new ones to offer.

Did we mention you also have a chance to win an iPad Mini? Everybody who takes the survey will be entered into a drawing that will take place Thursday July 28 for a new iPad mini!

The survey only takes a minute to complete and you will be helping us to help you and all future WFH members. All you have to do is fully complete the survey questions and we will automatically enter your name for a chance to win. Input from the global community in attendance at the Congress allows the WFH to tap into a vast vein of knowledge and experience, and will help us develop membership opportunities that will engage and inspire everyone in the community. We want you to be part of this initiative, so please drop by and see us at the WFH Resource Center to take the survey while you are at the Congress.

The entire Philanthropy and Resource Team—Paula, Andreina, Amelie and Roddy—wish all of you a very productive Congress experience and we look forward to meeting all of you. Please drop by the WFH Resource Center and let us know why membership matters to you!



/Andreina Tovar and Paula Curtis – WFH membership coordinator and director of philanthropy

## Community\*

\*Your opinion matters! Complete the WFH Member Survey today at the WFH Resource Centre (all participants have a chance to win an iPad)

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



## GENETIC COUNSELLOR VERSUS GENETIC COUNSELLING

Genetics is one of the most predictive tools in medicine. A genetic counsellor (GC) is a specifically trained healthcare professional with credentials in areas such as genetic testing, medical genetics or developmental anatomy. The aim of genetic counseling is patient education for informed decision making. This topic was discussed in a panel session Sunday morning.

The GC gathers both clinical and nonclinical information about the client. Family history—charted with standard pedigree forms—helps provide a picture for the counselor to see possible risks as well as psychosocial issues. Using these standard forms allows them to be read across disciplines and providers.

After actual testing, one of the most critical pieces of counselling is noticing how patients

Michelle Alabek, Hemophilia Center of Western Pennsylvania, acknowledged that GCs may not be available in all areas. “Ideally, the patient will be in contact with a genetic counsellor. However, it may be more realistic that other disciplines are providing genetic counselling at your site,” she said.

She suggested the following for sites that do not have a genetic counsellor:

- Assess skills of existing staff
- Designate who will be responsible for each aspect of genetic counselling (disease education, risk assessment, testing, psychosocial, reproductive decision making)
- Provide staff with specific education in genetics
- Identify genetic counsellors to use for support

“But when you do have a genetic counselor on your team, the other staff can concentrate on their own areas of expertise,” she suggested.

Worldwide, genetic counseling resources may be limited, said Richa Mohan, India. To maximize the impact of what is available, she suggested seeking local, regional, national and international resources.

Factors that drive genetic counselling needs worldwide include: the severity of hemophilia; the incorrect perception that genetic counselling is directive; healthcare priorities in the country; marriage and consanguinity; sexual taboos; as well as religious and cultural beliefs.

“Genetic counseling provides information without bias; nondirectiveness is the gold standard. The first step is the goal-setting process. Simply ask your patients why they are there.”

— Jennifer Lemons, University of Texas



/ Richa Mohan poses considerations for genetic counselling in less developed countries.

“Genetic counseling provides information without bias; nondirectiveness is the gold standard,” said Jennifer Lemons, University of Texas. “The first step is the goal-setting process. Simply ask your patients why they are there.”

emotionally respond to information. “GCs are specially trained to recognize and address the potential psychosocial impact of the news you provide after testing is done,” said Lemons, noting that one should evaluate both verbal and nonverbal cues.

Regina Butler, Children’s Hospital of Philadelphia, described a program developed for women held in her region. Topics included an overview of hemophilia in the 2000s, progress since earlier generations, case studies, the difference between genetics testing and factor levels and resources available to those attending the program.

Genetic counselling continues to grow in importance and should be part of the patient’s comprehensive care plan.

## NEW INHIBITOR THERAPIES HAVE THE POTENTIAL TO CHANGE HEMOPHILIA TREATMENT

Considering that the treatment of hemophilia with inhibitors is still problematic, and inhibitors will also be a future issue in gene therapy, it’s particularly relevant that a Monday afternoon session will examine alternative therapies in the management of inhibitors.

Midori Shima, Nara Medical University, Kashihara, Japan, will chair the session, which is designed to help attendees understand the next generation of treatments for patients using inhibitors. “The session is aimed at summarizing the current concepts of inhibitor development and eradication from the immunological aspect, and presenting emerging novel therapies,” Shima said.

David Lillicrap, Queen’s University, Ontario, Canada, will present new concepts of inhibitor development and eradication. Rebecca Kruse-Jarres, University of Washington, United States, will discuss how recombinant porcine FVIII is a potent hemostatic therapeutic.

Shima will give details of clinical trials of three novel therapeutics: anti-FIXa/X bispecific antibody (Emicizumab)—in which he is directly involved—anti-antithrombin si RNA (Fitusiran) and anti-TFPI antibody (Concizumab).

16:30–18:00, Hall A4

## STAY CONNECTED



Download the WFH2016 Mobile App on app stores or online at [www.wfh.org/congress/app](http://www.wfh.org/congress/app)



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#WFH2016



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# HOW THE HEMOPHILIA COMMUNITY MODEL OF CARE IS TRANSFORMING MODERN MEDICINE

GROWING UP IN THE 1970S, VINCENT DUMEZ LEARNED THROUGH HIS LOCAL CLINIC ABOUT HOW TO BE ACTIVE WITH THE CARE OF HIS BLEEDING DISORDER, INCLUDING DIAGNOSING HIS BLEEDS AND TRANSFUSING HIMSELF. AND SO DID OTHER PEOPLE WITH HEMOPHILIA IN HIS HOME COUNTRY OF CANADA AND THROUGHOUT THE WESTERN WORLD.

“Because of the desire to move transfusions from the hospital to the house, so patients could stay at home as much as possible, hemophilia was one of the first communities to empower patients to be engaged members of their care teams,” said Dumez, MSc, co-director, Office of Collaboration and Patient Partnership, Faculty of Medicine, University of Montreal, Canada.

During the Monday morning plenary “Patient Partnerships: The Next Step in Hemophilia Care,” Dumez will discuss how the hemophilia community has been a role model for a variety

of health-system leaders and stakeholders who are advocating for medical systems that fully engage patients as active partners in their care.

The Faculty of Medicine at the University of Montreal is a leader in promoting an outpatient-centred vision of medicine in which patients, families, and healthcare professionals work together to create care plans. Caregivers in areas such as cancer, dialysis, and mental health are embracing this vision, but a gap exists between rhetoric and reality, Dumez said.

During his plenary presentation, Dumez will address those challenges and give examples of how the hemophilia community has overcome them. For instance, he said, in the hemophilia community, treatment is not only about new drugs or cures, but also about building bridges between patients, researchers, and healthcare providers.

The WFH conference is an example of this. “It’s a very unusual congress in which patients, families and scientists are united,” Dumez said. “But this very special partnership model has been a reality in hemophilia from the start.”

0930–1000, Hall B3

## PATIENT-CENTRED CARE EXPLORES NEW PARTNERSHIPS BETWEEN RESEARCHERS AND PATIENTS

Patient-centred care is an emerging trend in healthcare today. But like any new initiative, there can be challenges with implementation, said Vincent Dumez, MSc, co-director, Office of Collaboration and Patient Partnership, Faculty of Medicine, University of Montreal, Canada.

During the Monday afternoon session “The Evolution of Patient-Centred Care,” Dumez and a panel of experts will discuss the best ways to promote this type of care. “Traditionally, the medical establishment has been very paternalistic in pushing care,” Dumez said. “One of our

main challenges is determining what we need to do to make sure a patient-centred approach is deployed.”

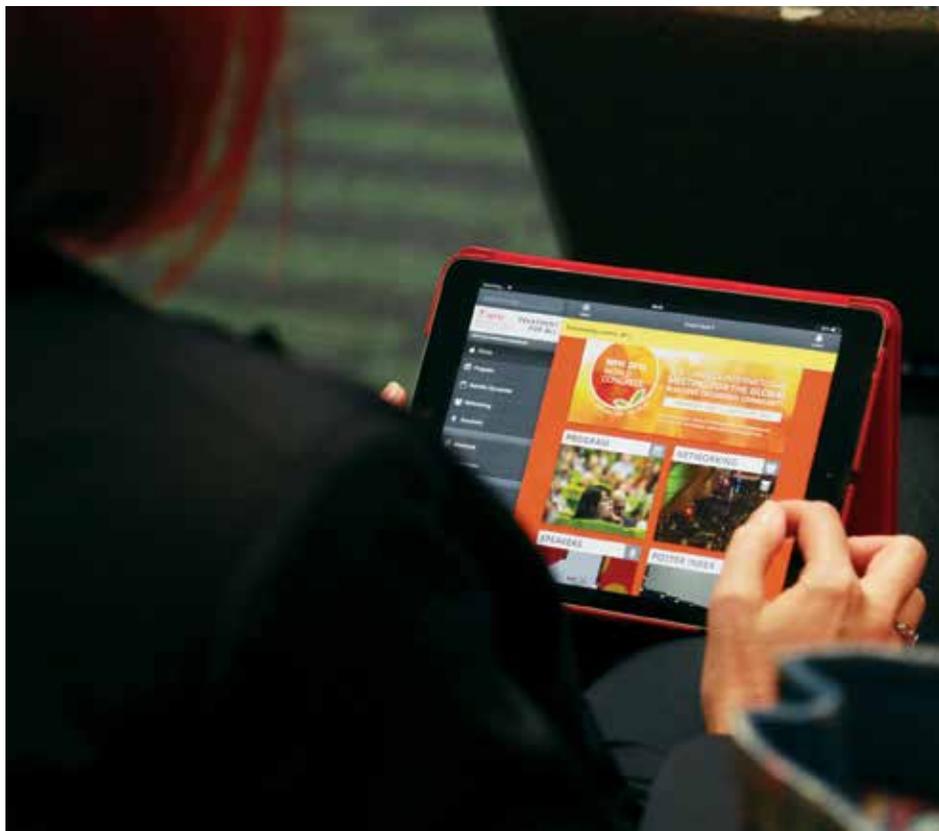
One way to engage patients is by including them as partners in research. “Usually, scientists think from their side and patients think from their side,” Dumez said. “The challenge is how can we get closer and learn to work together to generate new ideas, new research and new medical education.”

Speakers will discuss their experiences of engaging, selecting, and working with people with hemophilia, and how that informs research models and results. Patients can even influence what types of research should be prioritized, Dumez said.

For instance, Dumez said consulting patients about a study on a new blood-transfusion product can influence how the product is developed. Patients can discuss side effects, the impact of the product on their lives, and how the product is deployed.

“Patients have a huge added value for scientists,” Dumez said. “We have an obligation to have patients as partners in research.”

14:15–15:45, Hall B3



### ARE YOU MOBILE? SEARCH AND FIND USING THE WFH 2016 WORLD CONGRESS APP

What’s better than having the WFH 2016 World Congress Final Program in one hand and a Congress map in the other? Why, having both on your mobile device of course! The WFH 2016 World Congress app is currently available on all major app stores, including the Apple App Store and Google Play.

Download it now and get instant access to a wealth of content, including up-to-date program information, maps of the entire Congress venue, abstracts and poster indexes, and the *Congress Daily News Online*. You can also use it to manage your experience at the congress, including creating your own schedule of events, taking notes before or during sessions and contacting other attendees instantly right from the app. What’s more, the app is fully searchable, so if you’re looking for the location of a session you want to attend, the room number—and even where it is on the Congress map—is just a few keystrokes away.

## PROGRAM CHANGES

### CANCELLED

Free Papers M-01:  
Hemophilia: Clinical  
Monday, July 25  
14:35-14:45

### TOPIC CANCELLED

Non-Neutralizing  
Antibodies Against  
Factor VIII and The Risk  
of Inhibitor Development  
in Untreated and  
Minimally Treated  
Patients with Severe  
Hemophilia A (SIPPET  
Study). On Behalf of  
SIPPET Investigators  
Group. Presenter:  
Flora Peyvandi

### ADDED

HTC Psychologists  
Monday, July 25 –  
12:30-14:00  
Room 303A

This is a wonderful  
opportunity for  
psychologists working with  
people with hemophilia to  
meet with colleagues  
around the world in order  
to examine the current  
need for psychological  
services as well as various  
models of integrating  
psychological services in  
hemophilia care.

IHTC Alumni Event  
Monday, July 25 –  
14:15-15:45  
Room 202

## ADDRESSING GLOBAL NEED

A special event organized to showcase and discuss raising awareness of the value of

capacity building and leadership development in hemophilia, thrombosis and hemostasis will be held Monday from 14:15-15:45 in room 202 of the Convention Center. A moderated panel and roundtable discussions will actively engage the audience during Building Capacity to Address Global

Need: WFH and ISTH Fellowship Programs.

Sponsored by Bayer, the International Society on Thrombosis and Hemostasis (ISTH) and WFH, the discussion will provide tools for participants to take home that will enable them to implement programs in their home

countries to improve patient engagement and outcomes. Alumni Fellows and mentors will provide their perspective on challenges, accomplishments, new skills gained and how hemophilia care has changed in their country due to participation in the program.

14:15–15:45, Room 202

## QUICK FACTS ABOUT THE ORANGE COUNTY CONVENTION CENTER

- Second largest convention center in the United States (McCormick Place in Chicago is #1)
- 650,000 square metres (7,000,000 square feet) of total space
- 200,000 square metres (2,100,000 square feet) of exhibit space
- 3 full-service restaurants and 8 food courts
- 1 million delegates hosted every year



/ Attendees pause for a selfie on Sunday.



## Satellite Symposium PedNet Study group

Thursday July 28th 2016

Time 7:00 - 8:30 AM

Location Room 308

## Program

- The PedNet Registry and satellite studies – an overview  
*Marijke van den Berg & Rolf Ljung*
- 5 years after the RODIN study; an update on 913 PUPS with severe haemophilia A  
*Kathelijn Fischer*
- The REMAIN study; follow up of 260 severe haemophilia A patients with inhibitors  
*Maria Elisa Mancuso*
- Mode of delivery and neonatal bleeding; results on 994 children with severe and moderate haemophilia A and B  
*Hervé Chambost*

### Participating centers in the PedNet Registry

Austria: Vienna; Belgium: Leuven; Canada: Montreal, Toronto; Denmark: Århus; Finland: Helsinki; France: Le Kremlin-Bicêtre, Marseille, Toulouse; Germany: Bonn, Bremen, Frankfurt Goethe & Mönchhof, Würzburg, Munich; Greece: Athens; Ireland: Dublin;

Israel: Tel Hashomer; Italy: Genova, Milan; The Netherlands: Utrecht; Spain: Barcelona, Madrid, Seville, Valencia; Sweden: Malmö, Stockholm; Switzerland: Wabern; United Kingdom: Birmingham, London, Edinburgh, Glasgow

## MILLIONS OF PEOPLE BENEFIT FROM WFH HUMANITARIAN AID

In developing countries, the cost of treatment for people with bleeding disorders can be prohibitively expensive. The WFH has been a leader in humanitarian aid efforts to help ensure consistent and predictable access to treatment for millions of people around the globe.

Since the Humanitarian Aid program was created in 1996, the WFH has distributed more than 322 million international units of clotting factor to 90 countries—helping more than 100,000 people. In 2015 alone, the Humanitarian Aid program donated nearly 53 million IUs of factor to people in 63 countries.

During the Monday morning session “WFH Humanitarian Aid: Treatment for All is the Vision for All,” speakers will discuss the evolving global needs for basic care for bleeding disorders and how the WFH addresses those needs.

Assad Haffar, the WFH director of humanitarian aid and four panelists will discuss details of the WFH program. Megan Adediran, Haemophilia Foundation of Nigeria, will focus on the WFH Cornerstone Initiative, which provides support, expertise and training to countries with minimal levels of care for people with bleeding disorders.

Thomas Sannie, French Association of Hemophilia, will highlight the Franco-African Alliance for the Treatment of Hemophilia (AFATH), which helps bring WFH Humanitarian Aid initiatives to hemophilia patients in 18 countries in sub-Saharan Francophone Africa.

10:45–12:15, Room 205

*The Congress Daily* is the official newspaper of the WFH 2016 World Congress.

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# WFH CONGRESS BLEEDING DISORDER TREATMENT ROOM

The WFH is once again setting up a treatment room for patients who are not able to bring their own clotting factor treatment to the United States. Clotting factor concentrates have been generously donated by Baxalta, Bayer, Biogen, CSL Behring, Grifols, Novo Nordisk, Octapharma and Pfizer. Supplies for the treatment rooms have been donated by Bleeding and Clotting Disorders Institute (Peoria, IL), Option Care and Cascade Hemophilia Consortium.

The treatment room will be managed by the Congress host society, National Hemophilia Foundation, and staffed by volunteer nurses, specialized physicians and physiotherapists.

**Specialized physicians and nurses** will be able to assess emergency and semi-urgent situations related to your bleeding disorder and assist with infusions

**Physiotherapists** will be available to perform all appropriate physical support and give advice to patients who are having an acute or sub-acute bleeding episode

Treatment room hours of operation and services offered			Services	
Day	Location	Hours	Nursing	Physiotherapy
Sunday, July 24	Room 207A	14:00 - 20:00	✓	
Monday, July 25		08:00 - 18:00	✓	✓
Tuesday, July 26		08:00 - 18:00	✓	✓
Wednesday, July 27		08:00 - 18:00	✓	✓
Thursday, July 28		08:00 - 15:00	✓	✓
Friday, July 29		08:00 - 18:00	✓	

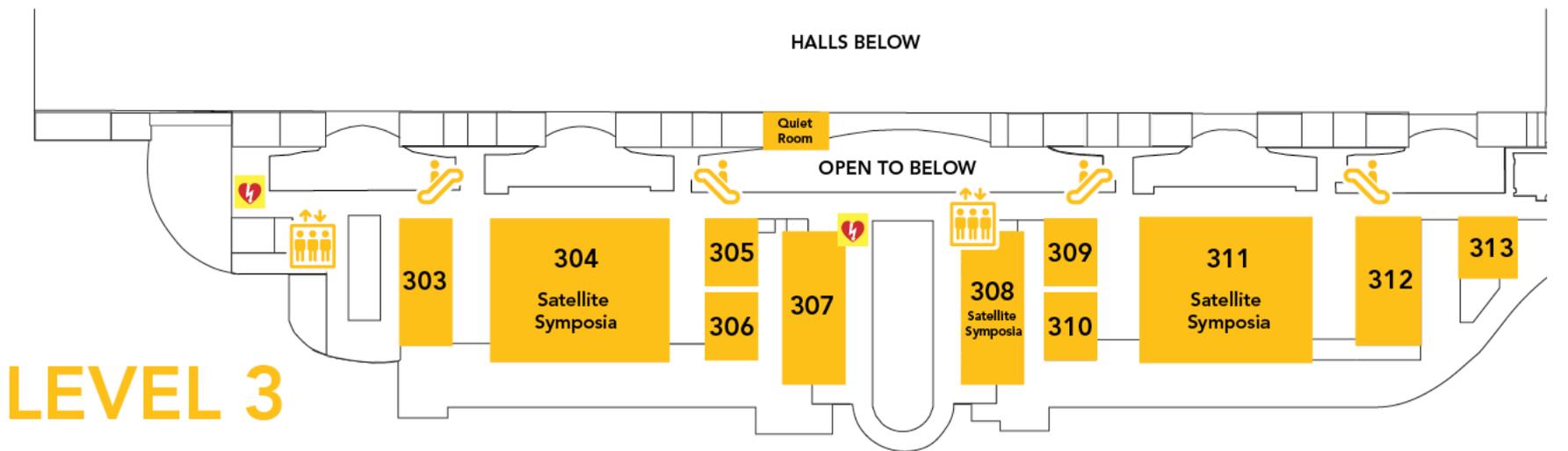
A limited number of wheelchairs, crutches and walkers will also be made available. Also, please note that donated treatment products will not be offered to those patients attending

from the US and Canada. **Important note:** The treatment room is for those seeking treatment related to a bleeding disorder. If your medical problem is not related to a

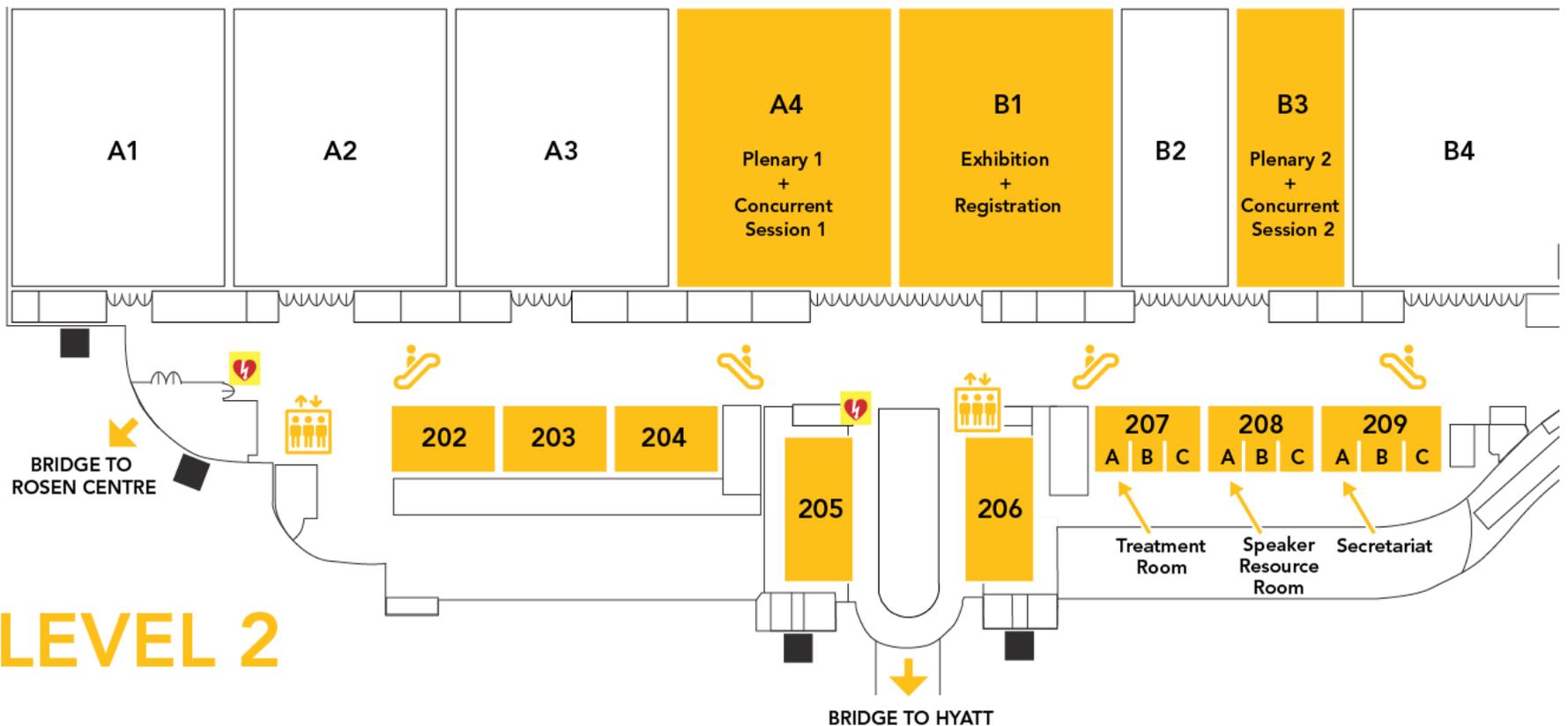
bleeding disorder, we recommend that you either contact a doctor through your hotel or visit the emergency department of the local hospital.

## ORANGE COUNTY CONVENTION CENTER FLOOR PLANS

HALLS BELOW



LEVEL 3



LEVEL 2

## HEMOPHILIA ALLIANCE SUPPORTS US HEALTHCARE PROFESSIONAL CONTINUED TRAINING

WFH USA is pleased to announce the first installment of Hemophilia Alliance Travel Grants which will begin this year in time for the WFH 2016 World Congress. This grant will advance the art and science of bleeding disorders nursing, psychosocial work, dentistry, physiotherapy, and laboratory sciences. Funded through the generous grant support from the Hemophilia Alliance, a \$15,000 grant per year is committed to support scientific exchange and global learning through attendance of one American member, from selected WFH multidisciplinary committees, at international meetings of the WFH.

## Opening

Continued from page 1.

positively impact the bleeding disorder community now and in the near future. Val Bias spoke enthusiastically about the WFH Humanitarian Aid Program, saying, “These new realities provide us with a unique opportunity to work together to make today’s therapies available to more people around the world with bleeding disorders—many of whom were previously undiagnosed or lacked access to treatment.” Alain Baumann commented on one of the WFH’s major

objectives for the next five years: “By 2020, we’ve committed to working towards identifying 50,000 new individuals with bleeding disorders—50 percent of whom are from the world’s most impoverished nations. We’re off to a great start and we’re up to the challenge.”

The evening concluded on an enthusiastic high note from all those involved with the opening of the Exhibition Hall. Attendees enjoyed canapés and refreshments and had the opportunity to reconnect with colleagues and friends and talk about all the exciting events the Congress will be offering in the coming days.

## DONORS OF CONGRESS: SHELLY REED

Shelly Reed – WFH USA Board Member and Monthly Donor

Shelly resides in Murfreesboro, Tennessee, USA with her new husband Corey and their “fur baby”—a rescue dog they recently adopted. As a WFH USA Board member Shelly knows the difference the WFH Humanitarian Aid Program makes in developing nations. She supports these efforts through a monthly donation. “For less than the price of lunch per month, which adds up by the end of the year, you can make a big difference in people’s lives all over the world.” —Shelly Reed

Join Shelly and our growing list of supporters today at [www.wfh.org/donateusa](http://www.wfh.org/donateusa).

## Data

Continued from page 2.

David Page, Canadian Hemophilia Society, focused on the role of patient organizations in data collection. The WFH Global Survey and the European Haemophilia Consortium Annual Survey both include patient data, and Page said many countries’ health technology assessment bodies also require data from patient organizations.

Page said patient organizations have an advantage in collecting data because they provide easy access to patients and caregivers. They also have a lot of credibility. “Unlike market research firms, a patient organization is seen to have an unequivocal interest in patient welfare,” he said. Patient organizations can also efficiently collect data because they can bypass onerous research ethics board approvals.

Page said the disadvantage is that patient organizations can lack the scientific expertise to collect good-quality data. Data collection is also not seen as a role for patient organizations.

Kathelijin Fisher, University Medical Center Utrecht, the Netherlands, listed a hierarchy of patient data collection in order of need: number of patients according to diagnosis; treatment, including prophylaxis and clotting factor consumption, surgeries and number of annual joint bleeds and location; joint status, including a physical exam and imaging; and activities, participation and quality of life.

Jeff Stonebraker, North Carolina State University, United States, said there’s no perfect data, but there are sources for collecting the best data possible. These include the WFH Annual Global Survey, scientific literature, national registries, The Marketing Research Bureau, the World Bank, the United Nations, the World Health Organization, the U.S. Centers for Disease Control and Prevention, survey questionnaires, subject-matter-expert interviews, and the Immune Deficiency Foundation.

Christine Herr, WFH’s data research coordinator, said data is being collected for the 2015 Annual Global Survey, which will be released in October and can be viewed at [www.wfh.org/GlobalSurvey](http://www.wfh.org/GlobalSurvey). This comprehensive survey can be downloaded and used in advocacy efforts, Herr said.

The 2014 AGS includes data from 106 countries, validation against humanitarian data, and new sections on hemophilia severity and data sources.

New topics on the 2015 questionnaire include: percentage of the patient population being

reported on per country; more information about HIV and hepatitis C infection, common doses of factor administered and frequency, immune tolerance induction, and amount of donated factor concentrates.

The session closed with a look at the successes and challenges of building nationalized, web-based patient registries.

Anne-Louise Cruikshank, South African Haemophilia Foundation, said challenges in building her country’s web-based patient registry included patient underdiagnosing and under-reporting, accuracy problems with data collection, and synchronizing data sent in Excel spreadsheets to a web-based system. Security was also an issue — “Data was only as secure as the office it was locked in,” she said. And healthcare personnel charged with collecting data often became disheartened.

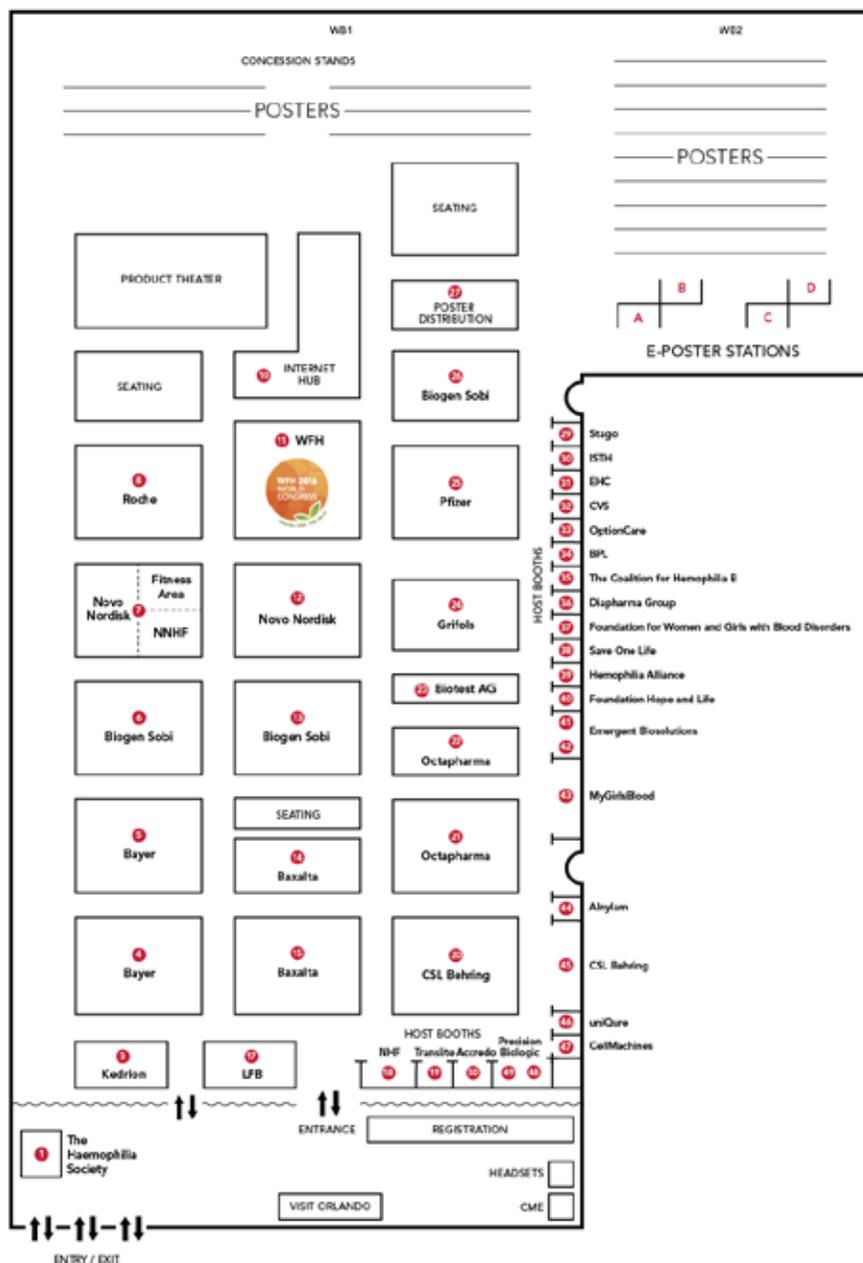
As a result, the foundation came up with a new approach to obtain better data. The University of Cape Town Research Centre, in conjunction with a local hematology centre, will develop a web-based software that will be hosted on the university servers.

Saeed Ul Hassan, Pakistan Hemophilia Patients Welfare Society, said In 2013, the society submitted an initiative to the WFH to build a national patients registry. The initiative was approved and funded by Novo Nordisk.

The registry, which was launched in June 2015, groups data by diagnosis, age group, chapter/area of country, incidence of hepatitis C and B, joint deformity and more. Hassan said the data is used to monitor trends in health, allocate resources, distribute mechanisms for humanitarian aid and ensure better global data.

In Canada, Page said a lack of financial resources and clinician time to gather data gave the Hemophilia Society the idea of using an already-existing registry. As a result, Canada is using Israel’s registry and adapting it to fit Canada’s needs.

## EXHIBIT HALL FLOOR PLAN



**DID YOU KNOW THAT...**

Orlando is the most visited destination in the US and welcomed over 66 million visitors in 2015?



## UNMET NEEDS REQUIRE UNMATCHED COMMITMENT.

The combined talents and energy of Shire and Baxalta are uniting in common cause: to make a difference in the lives of those living with and affected by rare diseases.

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